Council of the European Union

Brussels, 1 June 2026
(OR. en)

9833/26

Interinstitutional Files:
2025/0405 (COD)
2025/0406 (COD)

SAN 359
PHARM 97
COMPET 639
CODEC 1042
RECH 247
BIOTECH 66
ENV 582
PI 68
FOOD 73
FEED 30
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AGRILEG 141
DENLEG 55

COVER NOTE

From:	Secretary-General of the European Commission, signed by Ms Martine DEPREZ, Director
To:	Ms Thérèse BLANCHET, Secretary-General of the Council of the European Union
No. Cion doc.:	SWD(2026) 450 final
Subject:	PART 1/2 COMMISSION STAFF WORKING DOCUMENT European Biotech Act Accompanying the documents Proposal for a Regulation of the European Parliament and of the Council on establishing a framework of measures for strengthening Union's biotechnology and biomanufacturing sectors particularly in the area of health and amending Regulations (EC) No 178/2002, (EC) No 1394/2007, (EU) No 536/2014, (EU) 2019/6, (EU) 2024/795 and (EU) 2024/1938 (European Biotech Act) and Proposal for a Directive of the European Parliament and of the Council amending Directives 2001/18/EC and 2010/53/EU as regards the placing on the market of genetically modified micro-organisms and the processing of organs

Delegations will find attached document SWD(2026) 450 final.

Encl.: SWD(2026) 450 final

EUROPEAN
COMMISSION

Brussels, 26.5.2026
SWD(2026) 450 final

PART 1/2

Addendum to
COM(2025) 1022 and COM(2025) 1031 adopted on 16.12.2025

COMMISSION STAFF WORKING DOCUMENT

European Biotech Act

Accompanying the documents

Proposal for a Regulation of the European Parliament and of the Council on establishing a framework of measures for strengthening Union's biotechnology and biomanufacturing sectors particularly in the area of health and amending Regulations (EC) No 178/2002, (EC) No 1394/2007, (EU) No 536/2014, (EU) 2019/6, (EU) 2024/795 and (EU) 2024/1938 (European Biotech Act)

and

Proposal for a Directive of the European Parliament and of the Council amending Directives 2001/18/EC and 2010/53/EU as regards the placing on the market of genetically modified micro-organisms and the processing of organs

{COM(2025) 1022 final} - {COM(2025) 1031 final}

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Glossary

Term or acronym	Meaning or definition
AI	Artificial Intelligence
ATMP	Advanced Therapy Medicinal Product
BMWP	Biosimilar Medicinal Products Working Party
CAGR	Compound Annual Growth Rate
CAPEX	Capital Expenditure
CES	Comparative efficacy study
CfE	Call for Evidence
CHMP	Committee for Medicinal Products for Human Use
CMA	Critical Medicines Act
CRO	Contract Research Organization
CT	Clinical Trial
CTR	Clinical Trials Regulation
DNA	Deoxyribonucleic acid
EEA	European Economic Area
EFSA	European Food Safety Authority
EIB	European Investment Bank
EMA	European Medicines Agency

ERA	Environmental Risk Assessment
EU	European Union
FTE	Full-Time Equivalent
GDP	Gross Domestic Product
GDPR	General Data Protection Regulation
GMM	Genetically Modified Micro-organism
GMO	Genetically Modified Organism
GMP	Good Manufacturing Practices
GVA	Gross Value Added
HTA	Health Technology Assessment
IMP	Investigational Medicinal Product
IP	Intellectual Property
IVDR	Regulation (EU) 2017/746 on in vitro diagnostic medical devices
MAA	Marketing Authorisation Application
mAB	Monoclonal antibody
MDR	Regulation (EU) 2017/745 on medical devices
MFF	Multiannual Financial Framework
MSC	Member State Concerned
NGOs	Non-Governmental Organisations

OPEX	Operating Expenses
R&D	Research and Development
R&I	Research and Innovation
RMS	Reporting Member State
SDG	Sustainable Development Goal
SMEs	Small and Medium-sized Enterprises
SoHO	Substances of Human Origin
SPC	Supplementary Protection Certificate
STEP	Strategic Technologies for Europe Platform
TEP	Tissue Engineered Product
TFEU	Treaty on the Functioning of the European Union
VMP	Veterinary Medicinal Product
VNRAs	Variations Not Requiring Assessment

1 INTRODUCTION

This staff working document summarises the analysis supporting the proposal for a Regulation[1] (the 'proposed Regulation') and the proposal for a Directive[2] (the 'proposed Directive') which were adopted by the Commission on 16 December 2025. These two proposals (collectively referred to as the 'Biotech Act') aim to strengthen the EU's biotechnology and biomanufacturing sectors, particularly in the area of health. They amend key legislation relevant to these sectors.

Biotechnology and biomanufacturing, supported by artificial intelligence (AI) and digital tools, could help modernise entire parts of the EU economy. They hold huge potential to boost competitiveness and innovation, while delivering for patients, public health, food safety, security and other areas. Despite its world-class scientific base, the EU is trailing its competitors when it comes to translating biotechnology research into development, market deployment and manufacturing at scale. Fragmented ecosystems, financing constraints, complex regulatory pathways, and a lag in adapting regulatory frameworks to technological advances, hinder EU biotechnology companies' potential to innovate, bring innovation to market, produce and grow in the EU. As a result, the EU's biotechnology sector does not meet its full potential to tackle major societal challenges and faces a widening global competitiveness gap.

The proposed Regulation and Directive present a series of measures primarily for the health biotechnology sector, with targeted actions addressing other biotechnology areas (food and feed, industrial and agricultural biotechnologies). The two proposals aim to accelerate the way biotechnology innovations, products and services are developed and placed on the single market, while maintaining the highest safety standards. The proposed Biotech Act is one of the flagship initiatives of the Competitiveness Compass[3], designed to unleash the EU's innovation potential. It also contributes to regulatory simplification and administrative burden reduction goals.

The Biotech Act is in line with the United Nations' Sustainable Development Goals (SDGs), in particular SDG 3 'Good health and well-being: Ensure healthy lives and promote well-being for all at all ages'; SDG 9 'Industry, innovation and infrastructure: Build resilient infrastructure, promote inclusive and sustainable industrialisation and foster innovation'; and SDG 12 'Responsible consumption and production: Ensure sustainable consumption and production patterns'. More details on the relevant SDG targets and indicators are available in Annex 3. Regarding specialisation in terms of scientific output, the EU leads *inter alia* in SDGs 9 and 12.[4]

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[1] COM(2025) 1022 final.
[2] COM(2025) 1031 final.
[3] COM(2025) 30 final.
[4] European Commission: Directorate-General for Research and Innovation, *Science, research and innovation performance of the EU, 2024 – A competitive Europe for a sustainable future*, Publications Office of the European Union, 2024, https://data.europa.eu/doi/10.2777/965670 (see Chapter 3: Scientific knowledge production).

2 BACKGROUND

2.1 Political and legal context

A 'European Biotech Act' was announced by the President of the Commission in the **2024 - 2029 Political Guidelines of the European Commission**[5], to make it easier to bring biotechnology products from the laboratory to the factory and then onto the market, while maintaining high safety standards. It was further reflected in the mission letter of Commissioner Várhelyi[6].

In the **Commission Recommendation on critical technology areas for the EU's economic security**[7], biotechnology was recognised as having "an **enabling and transformative nature** in areas such as agriculture, environment, healthcare, life sciences, food chains or biomanufacturing". The Commission Communication on Biotechnology and Biomanufacturing[8] highlighted the high **growth potential and labour productivity** of biotechnology and biomanufacturing. This makes the sector pivotal to the **competitiveness and modernisation** of the EU's economy, as well as conducive to its **strategic autonomy and resilience**. While the EU's strengths were recognised (i.e. research and innovation base and capacities), the Commission stressed the need to address the challenges faced by companies, users and consumers. It called for both a **supportive regulatory environment and a coordinated approach**, particularly for developing infrastructures, fostering the use of AI, and encouraging private and public investments. Additionally, the **EU's Economic Security Strategy**[9] identified biotechnology as one of the 10 technology areas most likely to present sensitive and immediate risks related to technology security and technology leakage. This strategy launched an EU joint risk assessment with Member States on biotechnology, which identified relevant biosecurity risks that require mitigation measures.

The seminal reports by **Enrico Letta**[10] and **Mario Draghi**[11] also underscored the need to take action in the biotechnology and biomanufacturing sectors. They identified the need for a stronger, more dynamic European industrial policy, including measures for the biotechnology and biomanufacturing sectors to scale up and get products on the market faster. The report by **Sauli Niinistö**[12] also pointed to the need to strengthen biological defence capabilities and preparedness against emerging biological threats, including synthetic pathogens.

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[5] See footnote 1, page 5.
[6] Ursula von der Leyen, Mission Letter – OlivérVárhelyi – Commissioner-designate for Health and Animal Welfare, 17 September 2024.
[7] Commission Recommendation (EU) 2023/2113 of 3 October 2023, OJ L, 2023/2113, 11.10.2023, ELI: http://data.europa.eu/eli/reco/2023/2113/oj.
[8] COM(2024) 137 final/2.
[9] JOIN(2023) 20 final
[10] Enrico Letta, Much more than a Market. April 2024.
[11] Draghi, Mario. The future of European competitiveness: A competitiveness strategy for Europe, European Commission, 9 September 2024.
[12] Niinistö, Sauli Safer Together – Strengthening Europe's Civilian and Military Preparedness and Readiness, 30 October 2024.

In line with this growing political impetus, reducing the **EU's strategic dependencies in sensitive sectors has become a priority of the European Council**[13], including in health and food, as identified in the **Versailles Declaration** of 2022[14]. The Council[15] has urged the Commission to unlock the potential of biotechnologies for the EU's competitiveness through the European Biotech Act, while maintaining environmental and safety standards. In particular, the Council has encouraged actions to advance the use of regulatory sandboxes, promote research and development (R&D) in advanced therapy medicinal products (ATMPs) and address the falling global share of EU clinical trials (CTs). It also supports the greater use of AI and the development of appropriate skills. In parallel, three Important Projects of Common European Interest (IPCEI) in the biotechnology domain are currently under design[16], reflecting Member States' interest in coordinated, large-scale investment approaches.

The **European Parliament's recommendations** in its resolution 'Future of the EU biotechnology and biomanufacturing sector' **are in line with these priorities**, asking to facilitate "a fast and efficient uptake of biotechnology and biomanufacturing through clear regulatory frameworks"[17]. Recommendations include facilitating the uptake of biotechnology and biomanufacturing; enabling more efficient scale-up and commercialisation of innovations; streamlining and simplifying the time-to-market for biotechnology throughout their life-cycles; streamlining the clinical trials framework by minimising administrative burden and delays; and using regulatory sandboxes. It also recognises the need for a skilled workforce, dedicated support for SMEs to access funding, and biosecurity screening standards. The European Parliament has also prepared an own-initiative **report on the 'Public health aspects of biotechnology and life sciences'**[18], where it puts forward similar recommendations. It has called for the Biotech Act to review, simplify and optimise the regulatory framework for biotechnology in healthcare to foster innovation, as well as for structured EU support to help excellent biotechnology innovation districts in the EU grow and ensure that they have sufficient capacity, resources and the scientific edge to promote new groundbreaking biotechnology discoveries, innovation and commercialisation.

2.2 Related EU legislation

2.2.1 Existing EU legislation

Health biotechnology products are subject to several EU legislative frameworks, in particular **Regulation (EU) No 536/2014 on clinical trials on medicinal products for human use** [19], **Regulation (EC) No 1394/2007 on ATMPs**[20], **Regulation (EU) 2024/1938 on standards of quality and safety for substances of human origin intended for human**

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[13] European Council, Special meeting of the European Council (17 and 18 April 2024) – Conclusions, EUCO 12/24.
[14] European Council, The Versailles declaration, 10 and 11 March 2022.
[15] Council of the European Union, A call for action on life sciences for the EU's competitiveness - Council conclusions, 13323/25, approved on 30 September 2025.
[16] https://competition-policy.ec.europa.eu/state-aid/ipcei/design-support-hub_en#ipcei-candidates-in-the-design-support-hub.
[17] European Parliament, 2025/2008(INI), 10 July 2025.
[18] European Parliament: 2025/2087(INI).
[19] Regulation (EU) No 536/2014, OJ L 158, 27.5.2014, pp. 1–76. ELI: http://data.europa.eu/eli/reg/2014/536/oj.
[20] Regulation (EC) No 1394/2007, OJ L 324, 10.12.2007, pp. 121–137. ELI: http://data.europa.eu/eli/reg/2007/1394/oj.

application (SoHO)[21], **Directive 2010/53/EU on quality and safety of human organs intended for transplantation**[22], and **Regulation (EU) 2019/6 on veterinary medicinal products (VMPs)**[23]. These legislative frameworks govern different stages of the product's lifecycle, including research, clinical development, authorisation and the placing on the market of biotechnology products and therapies. Accordingly, the EU biotechnology sector can only flourish if they are efficient, coherent and innovation-friendly.

For food and feed safety, the placing on the market of biotechnology products must comply with requirements set out in the **General Food Law**[24] and with specific legal frameworks, where applicable[25]. Placing products on the market that contain or consist of genetically modified organisms (GMOs), other than food and feed, falls within the scope of the **legislation on the deliberate release of GMOs**[26]. These legislative frameworks ensure high levels of human and animal health, and environmental protection. To foster biotechnology innovation, it is important to keep these objectives front and centre and maximise procedural efficiency and speed, because risk-assessment and authorisation procedures directly affect the time and cost for biotechnology products to reach the market.

The proposed Biotech Act reviews and seeks synergies with other legislation to ensure coherence with the overall EU regulatory system. It complements the **Critical Medicines Act (CMA)**[27] in strengthening EU-based biotechnology research and manufacturing. It is in line with the **pharmaceutical strategy for Europe**[28] and complements the recent **revision of the EU pharmaceutical legislation**[29]. It is also complementary to the Commission's proposal to revise the **EU medical device legislation**[30], sharing the same overall objectives and including common measures such as the single authorisation process for combined device-medicine clinical studies. It also aims to **improve coordination and consultation** between relevant competent authority groups established under other frameworks, such as medical devices[31], medicines, SoHO, health technology assessment[32], etc.

2.2.2 Aligning with the broader priorities of the Commission

As a flagship initiative under the **Competitiveness Compass**[33], the Biotech Act aligns with the EU's broader innovation and competitiveness agenda, turning its priorities into actions in the strategic sector of biotechnology. In particular, the Biotech Act aims to reduce the administrative burden, improve coordination, simplify rules and streamline procedures, in line with both the Competitiveness Compass and the Commission's **Communication on**

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[21] Regulation (EU) 2024/1938, OJ L, 2024/1938, 17.7.2024. ELI: http://data.europa.eu/eli/reg/2024/1938/oj.
[22] Directive 2010/53/EU, OJ L 207, 6.8.2010, p. 14, ELI: http://data.europa.eu/eli/dir/2010/53/oj.
[23] Regulation (EU) 2019/6, OJ L 4, 7.1.2019, pp. 43–167. ELI: http://data.europa.eu/eli/reg/2019/6/oj.
[24] Regulation (EC) No 178/2002, OJ L 31, 1.2.2002, pp. 1–24. ELI: http://data.europa.eu/eli/reg/2002/178/oj.
[25] E.g. genetically modified food and feed is subject to Regulation (EC) No 1829/2003, OJ L 268, 18.10.2003, pp. 1–23. ELI: http://data.europa.eu/eli/reg/2003/1829/oj.
[26] Directive 2001/18/EC, OJ L 106, 17.4.2001, pp. 1–39. ELI: http://data.europa.eu/eli/dir/2001/18/oj.
[27] European Commission website, Critical medicines Act.
[28] COM(2020) 761 final.
[29] European Commission website, Reform of the EU pharmaceutical legislation.
[30] European Commission website, Simpler and more effective rules for medical devices.
[31] Regulations (EU) 2017/745, OJ L 117, 5.5.2017, pp. 1–175. ELI: http://data.europa.eu/eli/reg/2017/745/oj and (EU) 2017/746, OJ L 117, 5.5.2017, pp. 176–332. ELI: http://data.europa.eu/eli/reg/2017/746/oj.
[32] Regulation (EU) 2021/2282, OJ L 458, 22.12.2021, pp. 1–32. ELI: http://data.europa.eu/eli/reg/2021/2282/oj
[33] see footnote 4, page 5.

implementation and simplification[34]. The Biotech Act forms part of the **Commission's Life Sciences Strategy**[35] which recognises biotechnology as a strategic cross-sectoral technology and seeks to strengthen the EU's biotechnology ecosystem, streamline regulatory pathways and boost the EU's competitiveness more broadly in life sciences.

The Biotech Act complements other policy initiatives announced in the Competitiveness Compass. For example, it aims to improve access to risk-tolerant capital for biotechnology firms, aligning with the **Savings and Investments Union**[36] and the **Start-up and Scale-up Strategy**[37], which establishes the Scaleup Europe Fund[38]. Its biosecurity provisions reflect the link between competitiveness and security by strengthening safeguards for biotechnology products of concern. They address the risks identified by the biotechnology joint risk assessment launched under the **EU's Economic Security Strategy**[39] and complement **Regulation (EU) 2022/2371 on serious cross-border threats to health**[40]. The provisions aim at helping to prevent and prepare for such threats and to ensure a coordinated EU-level response, also for arising public health emergencies (including any arising from the misuse of emerging biotechnologies). They also complement the **Medical Countermeasures Strategy**[41] for preparing and responding to health threats, including human-made biosecurity threats.

The Biotech Act reflects the emphasis on talent and contributes to the **Union of Skills**[42]. It aligns with actions under the **European Strategy on Research and Technology Infrastructures**[43]. It aligns with EU funding and investment initiatives that support biotechnology research, innovation and industrial scale-up. It takes into account EU financial support available, including cohesion policy programmes, InvestEU, the European Investment Bank (EIB) Group's TechEU programme[44,45] and the European Innovation Council. The strategic importance of biotechnology is also acknowledged in preparations for the next Multiannual Financial Framework (MFF), namely the next iteration of the **Horizon Europe programme (2028-2034)**. It is acknowledged in the section on collaborative research activities in the Competitiveness component, as well as the proposed **European Competitiveness Fund**, which includes a dedicated 'Health, Biotech, Agriculture and Bioeconomy' policy window.

The Biotech Act is designed to be coherent with related digital policies and pursue **digital transformation** objectives. It aims to support secure, data-driven biotechnology ecosystems and contribute to the EU's technological sovereignty by promoting greater data

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[34] COM/2025/47 final.
[35] COM(2025) 525 final.
[36] COM(2025) 124 final.
[37] COM(2025) 270 final.
[38] Scaleup Europe Fund - European Innovation Council - European Commission.
[39] JOIN(2023) 20 final.
[40] Regulation (EU) 2022/2371, OJ L 314, 6.12.2022, pp. 26–63. ELI: http://data.europa.eu/eli/reg/2022/2371/oj.
[41] COM/2025/529 final.
[42] COM(2025) 90 final.
[43] COM(2025) 497 final.
[44] European Investment Bank. "Tech EU. The EU's largest ever financing programme to support Europe's innovators from idea to IPO and from lab to leadership." European Investment Bank website accessed 12 March 2026, https://www.eif.org/flagship-initiatives/techeuhttps://www.eif.org/flagship-initiatives/techeu.
[45] European Investment Bank. "European Tech Champions Initiative. Overview." European Investment Bank website accessed 12 March 2026, https://www.eif.org/flagship-initiatives/european-tech-champions-initiative/overview.

use and AI integration in the biotechnology sector. Its emphasis on AI is consistent with the **AI in Science Strategy**[46], **the Apply AI strategy**[47], the **AI continent action plan**[48], the **European AI Act**[49], the **Data Union strategy**[50], as well as the **EU Cybersecurity framework** [51]. The Biotech Act is also consistent with the objectives of the **European Health Data Space Regulation** (EU) 2025/327[52] and of the **Data Union Strategy** [53], in particular with regard to data and metadata semantic interoperability and quality accelerators, data labs, and AI factories. It should also be read in coherence with ongoing interoperability and standardisation work for common European data spaces under Article 33 of Regulation (EU) 2023/2854[54], including the European Trusted Data Framework, insofar as these initiatives are relevant to metadata, discoverability, semantic interoperability and the cross-border re-use of data.

The Biotech Act is consistent with the **Vision for Agriculture and Food**[55], in particular because it amends the **General Food Law** to make the regulatory environment more supportive of innovation and competitiveness in the agri-food sector. The Biotech Act is also consistent with the Commission proposal on plants developed by certain **new genomic techniques** [56] as it continues to adapt the GMO framework to ensure that innovative products are regulated proportionately. The Biotech Act is also designed to build synergies with initiatives such as the recently-adopted **Bioeconomy Strategy** [57].

Climate change has highlighted the need to prioritise the EU's resilience, so the Biotech Act reflects this priority too. It is in line with the Commission's climate neutrality objectives set out in the **EU Climate Law**[58] and the **EU's Strategy on Adaptation to Climate Change**[59]. It also ensures alignment with the **'do no significant harm'** principle. Biotechnology products have the potential to support adaptation to climate change, contribute to health and food security through sustainable biomanufacturing, and protect biodiversity. They may also replace products potentially more harmful for the environment and provide benefits for consumers and users.

The Biotech Act will not affect EU legislation on the **Registration, Evaluation, Authorisation and Restriction of Chemicals (REACH)**[60], **Regulation (EU) 2024/1735 on sustainable biogas and biomethane technologies and biotechnology climate and energy solutions**[61] or the **Directive 2010/63/EU on the protection of animals used for scientific purposes**.

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[46] COM(2025) 724 final
[47] COM(2025) 723 final.
[48] COM(2025) 165 final.
[49] Regulation (EU) 2024/1689, OJ L, 2024/1689, 12.7.2024, ELI: http://data.europa.eu/eli/reg/2024/1689/oj.
[50] COM(2025) 835 final.
[51] Regulation (EU) 2019/881, OJ L 151, 7.6.2019, pp. 15–69. ELI: http://data.europa.eu/eli/reg/2019/881/oj.
[52] Regulation (EU) 2025/327, OJ L, 2025/327, 5.3.2025. ELI: http://data.europa.eu/eli/reg/2025/327/oj.
[53] COM/2025/835 final.
[54] Regulation (EU) 2023/2854, OJ L, 2023/2854, 22.12.2023, ELI: http://data.europa.eu/eli/reg/2023/2854/oj.
[55] COM/2025/75 final.
[56] COM/2023/411 final.
[57] COM/2025/960 final.
[58] Regulation (EU) 2021/1119, OJ L 243, 9.7.2021, pp. 1–17. ELI: http://data.europa.eu/eli/reg/2021/1119/oj.
[59] COM/2021/82 final.
[60] Consolidated text: Regulation (EC) No 1907/2006, ELI: http://data.europa.eu/eli/reg/2006/1907/2025-09-01.
[61] Consolidated text: Regulation (EU) 2024/1735, ELI: http://data.europa.eu/eli/reg/2024/1735/2025-08-17.

2.3 Background on the sector

For the purposes of this document, and as per the proposed Regulation, biotechnology[62] means "the application of science and technology to living organisms, as well as parts, products and models thereof, to alter living or non-living materials for the production of knowledge, products and services". On this bases, health, food and feed are the main scope of the proposals.

Biotechnology and biomanufacturing are major **contributors to the EU's economy**. In 2022, the overall biotechnology sector accounted for **EUR 38.1 billion of EU GDP (ca. 1.58% of the European industrial sector),** with health biotechnology being the dominant contributor. Additionally, it contributed to **913,160 jobs**, with around 77%[63] of those jobs coming from the health biotechnology sector. The sector in the EU is characterised by a **large base of small and medium-sized enterprises (SMEs) (47%) and micro enterprises (35%),** complemented by a smaller number of large companies (18%)[64]. The **sector's dynamism** is illustrated by the **5,000 R&D-active biotechnology companies** in the EU[65]. Furthermore, there were **3,232 unique biotechnology startups founded** since 2015 that are still operative in 2025, largely in health[66] (See Annex 5, Figure 1).

The sector's strategic importance is underscored by its rapid **expansion**. Over the last decade, the EU biotechnology industry has almost doubled to the share of GDP it generates, growing by around **7% per year** on average between 2015 and 2024[67] (See Annex 5, Figure 2 for details on the sector GVA comparison).

From a **global perspective**, the EU has significant strengths in biotechnology, but its overall competitiveness position is under increasing pressure (see section 3 on problem definition). The EU is the **largest global exporter** of biotechnology products, demonstrating the sector's strong industrial base and integration into global value chains (see Annex 5, Figures 3 and 4)[68]. It is also a leader in biotechnology **science**, reflected by a publication record comparable to that of the US and China, with all three regions having between 20 – 25% of the top 10% most cited publications across biology, biomedical research and clinical science[69]. The EU also represents 15% of global **patent families** in the biotech field (see Annex 5, Figure 5 for details on the distribution across the EU)[70]. At the same time, other major jurisdictions are accelerating policy and investment efforts to strengthen their biotechnology ecosystems, as shown by recent initiatives in the US at both

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[62] COM(2025) 1022 final, Article 2 (1).
[63] Andreas Haaf and Vera Sale, Measuring the Economic Footprint of the Biotechnology Industry in the European Union, Research Report March 2025, WiFOR (Prepared for EuropaBio – The European Association for Bioindustries).
[64] Landscape analysis study.
[65] Landscape analysis study; Biotech R&D firms are firms that perform biotechnology R&D.
[66] Landscape analysis study.
[67] Landscape analysis study.
[68] Landscape analysis study.
[69] European Commission: Directorate-General for Research and Innovation, *Science, research and innovation performance of the EU, 2024 – A competitive Europe for a sustainable future*, Publications Office of the European Union, 2024, pp. 151-229 https://data.europa.eu/doi/10.2777/965670. https://ec.europa.eu/assets/rtd/srip/2024/ec_rtd_srip-report-2024-chap-03.pdf.
[70] Landscape analysis study.

congressional and federal level, which frame biotechnology as a core economic and national security priority requiring coordinated action to sustain global leadership[71].

In terms of **projections**, biotechnology and gene technologies are some of the most **impactful technology trends expected to influence business models** over the next five years[72] (i.e. the second most impactful technology in the medical and healthcare devices industry, with similar trends expected in agriculture, forestry, and fishing). In terms of job creation, a strong positive trend has been observed[73]. In the public consultation on the European Biotech Act stakeholders agreed that biotechnology and biomanufacturing products could positively impact the EU's economy[74].

Finally, biotechnology applications have a significant potential to generate **positive societal and environmental outcomes**, making this sector a pillar of the EU's societal wellbeing in key areas in particular in health and food. This potential is positively perceived by stakeholders, recognising in the public consultation the contribution of biotechnology and biomanufacturing products **to society (90%) and to the environment (79%)** and considered that biotechnology and biomanufacturing products that reach the single market are **safe and secure (77%)**[75]. Moreover, around a third of respondents **(38%)** agreed or strongly agreed that the EU regulatory environment **ensures a higher level of safety and security** than in some other countries[76].

Health biotechnology encompasses a wide range of applications, from the development of innovative medicines and new medical devices addressing unmet medical needs, to combating epidemics and transforming the treatment of rare diseases. **Gene and cell therapies**, for example, hold great promise for treating genetic disorders[77] and restoring biological functions. Although the timeline for developing conventional vaccines is lengthy, current biotechnology tools allow **scientists to develop new vaccines** or adapt **existing vaccines** much faster than before[78].

Agri-food biotechnology innovations support farmers and food producers. **Environmental and industrial** biotechnology solutions contribute to the sustainable transformation of our economy. **Genetically modified micro-organisms** (GMMs) play a decisive role in the development of industrial, environmental and agricultural biotechnology, both as a tool for manufacturing and in products. Products containing

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[71] U.S. House of Representatives (2025), *Congressional Biotechnology Caucus announcement*, June 2025; National Security Commission on Emerging Biotechnology (NSCEB) (2025), *Report to Congress*, April 2025; The White House (2022), *Executive Order on Advancing Biotechnology and Biomanufacturing Innovation for a Sustainable, Safe, and Secure American Bioeconomy* (establishing the National Biotechnology and Biomanufacturing Initiative).

[72] Landscape analysis study. Based on World Economic Forum. (2025, January 7). The Future of Jobs Report 2025. https://www.weforum.org/publications/the-future-of-jobs-report-2025/digest/.

[73] Andreas Haaf and Vera Sale, Measuring the Economic Footprint of the Biotechnology Industry in the European Union, Research Report March 2025, WiFOR (Prepared for EuropaBio – The European Association for Bioindustries).

[74] European Commission, *Open Public Consultation on a European Biotech Act*, 2025, available at: https://ec.europa.eu/info/law/better-regulation/have-your-say/initiatives/14627-Biotech-Act/public-consultation_en.

[75] Public consultation, see footnote 74.

[76] Public consultation, see footnote 74.

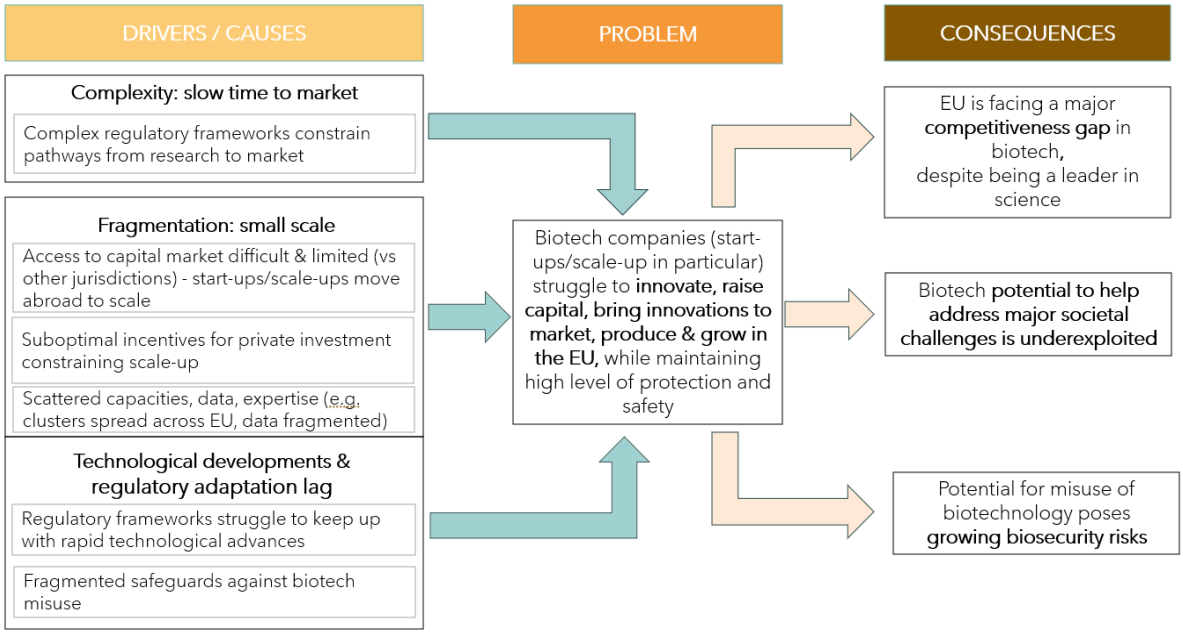
[77] Qie B, Tuo J, Chen F, Ding H, Lyu L. Gene therapy for genetic diseases: challenges and future directions. MedComm. 2025;6:e70091. https://doi.org/10.1002/mco2.70091.

[78] Aida V, Pliasas VC, Neasham PJ, North JF, McWhorter KL, Glover SR and Kyriakis CS (2021) Novel Vaccine Technologies in Veterinary Medicine: A Herald to Human Medicine Vaccines. Front. Vet. Sci. 8:654289. doi: 10.3389/fvets.2021.654289.

GMMs often yield innovative and beneficial product characteristics that other products cannot achieve or can achieve but with a higher cost or environmental impact. Examples of products containing GMMs already on the market outside the EU or in development include novel biofertilisers, biopesticides, and bioremediation products (i.e. to remove hazardous and toxic substances from the soil or wastewater).

3 PROBLEM DEFINITION

Figure 1. Problem tree



The Commission has refined and clarified the problem tree and intervention logic (see Section 4.4) from the version presented in the Explanatory Memorandum of the Biotech Act. These adjustments do not alter the underlying problem definition or policy rationale but provide a more structured and detailed explanation of the issues and causal pathways.

3.1 Problem: biotech companies struggle to innovate, raise capital, bring innovations to market, produce and grow in the EU, while maintaining high level of protection and safety

Although the EU benefits from a sizeable biotechnology sector and strong scientific capabilities (see section 2.3), it does not meet its full potential for innovation in biotechnology and biomanufacturing. The EU lags behind other regions when it comes to **translating its world-class science and innovation into commercially viable products in the EU**, and even more so **in manufacturing such products at scale**. As a result, **biotech companies often end up investing, growing, employing, creating value and placing their products on markets abroad**. This pattern weakens the EU's ability to retain value creation and industrial capacity from its research base[79].

[79] See also Landscape analysis study, forthcoming, for further details on the aspects summarised here.

The EU faces a **substantial gap in venture capital** with limited investment by pension funds and other institutional investors. Biotech is especially affected by this market failure due to the particular risk profile of biotech start-ups. Long delays between the initial innovation and first revenue generation due to lengthy regulatory process, massive upfront investments needed for CTs, and a high risk of failure of individual projects deter venture capital investors.

In addition to the fact that the EU accounts for 22% of biotech start-ups, against 49% in the US, the **EU's scale-up capacity also remains limited.** Only a small share of EU biotech firms reaches significant funding levels (12% of agricultural biotechnology start-ups, 10% of health biotechnology start-ups, and 8% of industrial biotechnology start-ups secure over EUR 10 million[80]). Very few exceed EUR 200 million[81]. As many companies struggle to scale in the EU, they increasingly rely on mergers and acquisitions as a common exit route. Around 40% of acquisitions of EU biotech start-ups are executed by US acquirers, highlighting how EU biotech innovation is frequently integrated into global pharmaceutical and life-science value chains instead of growing into large firms in the EU.

AI and data are now foundational to competitiveness in biotechnology, driving rapid advancements in discovery, development and manufacturing. While 20% of EU enterprises with 10 or more employees adopted AI by 2025 - up from 13.5% in 2024 - adoption in EU pharma and biotech manufacturing has surged even further, with large companies integrating AI at scale and shifting from pilot projects to system-level transformation[82]. However, **in-house AI development** and **scaling remain constrained by data and compute bottlenecks - particularly for SMEs.** AI biotech platforms are inherently capital-intensive, relying on costly model training and large-scale data generation, and depend on access to high-quality data and advanced compute infrastructure. As the industry matures, the gap between large pharma industry and smaller players is widening, underscoring the need for equitable access to technology and robust regulatory frameworks.

3.2 What are the problem drivers?

The problem is caused by three main related factors. Firstly, the regulatory and administrative operating environment for biotechnology in the EU is complex which in turn impacts ***speed***.

Secondly, the EU's biotechnology ecosystem operates in a fragmented manner, failing to tap the full ***scale*** of the EU research or testing infrastructure, markets, access to capital or production capacities, thus limiting companies' ability to grow and compete internationally.

Thirdly, ***technological advances*** in biotech can result in obsolete frameworks to govern this fast-moving sector.

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[80] Landscape analysis study.
[81] Landscape analysis study: 12 health biotech firms, 5 industrial biotech firms, one in agricultural biotechnology and none in marine biotechnology have raised more than EUR 200 million.
[82] https://ec.europa.eu/eurostat/web/products-eurostat-news/w/ddn-20251211-2.

3.2.1 Driver 1: the complexity of the EU regulatory framework, leading to slow time-to-market

A first set of challenges relates to the **regulatory environment**, which stakeholders consistently perceive as complex, time-consuming and difficult to navigate. In the EU, 60% of companies see regulatory burden as a key obstacle to long-term investment[83]. This finding applies to the biotechnology and biomanufacturing sector, as reflected in responses to the public consultation. **Complex regulatory pathways and divergent implementation of EU legislation** were identified as barriers to the expansion of EU companies, particularly start-ups, spin-offs and other SMEs[84]. Respondents **largely agreed that EU rules create regulatory barriers, particularly when products move closer to market entry and formal approval processes**[85].

In addition, the perception of the EU regulatory environment compared to other countries showed a **limited agreement about the level of predictability of the EU regulatory environment[86]**. Also, a small number of respondents agreed or strongly agreed that the EU regulatory environment accelerates access to the market, is less complex and clearer, or leads to lower compliance costs than in some other countries[87].

Such issues linked to the regulatory environment are confirmed across a variety of biotechnology areas. Detailed descriptions are presented in **Annex 5.**

With regards to the **authorisation of clinical trials**, the EU faces growing challenges in maintaining its competitive edge due to (i) lengthy regulatory timelines (particularly for the authorisation of multinational trials), and (ii) higher administrative requirements and costs in the EU/EEA associated with the authorisation and conduct of clinical trials, compared to other regions. As a result, sponsors[88] increasingly favour jurisdictions that offer faster regulatory timelines, simpler and more streamlined approval processes, and improved access for the recruitment of patient populations. This contributes to widening the competitiveness gap. On **ATMPs**, delays and increased costs are caused by administrative complexity and overly stringent requirements for GMO-based gene therapies. This stems from dual regulatory compliance requirements i.e. under the Clinical Trials Regulation and with the fragmented implementation of GMO legislation by Member States, further complicated by a blanket requirement for environmental risk assessment that does not take into account the specificities of low-risk gene therapies.

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[83] See footnote 10.
[84] Public consultation, see footnote 74.
[85] Public consultation: Three-quarter of the respondents indicated regulatory barriers in the assessment and market authorisation (77%). About 70% indicated pre-commercial testing or clinical trials, 68% noted impediments in commercialising products and 67% in scaling-up production or manufacturing, while 64% signalled regulatory barriers in product development matters.
[86] Public consultation: 21% agreed or strongly agreed and 43% disagreed or strongly disagreed that the EU regulatory environment is more predictable in comparison with some countries outside of the EU.
[87] Public consultation: About 9-10% agreed or strongly agreed and between 64% and 68% disagreed or strongly disagreed that EU regulatory environment enables products to reach the market faster, is less complex and clearer, or leads to lower compliance costs in comparison with some countries outside of the EU.
[88] Sponsor as defined in the CTR, Article (2) `means an individual, company, institution or organisation which takes responsibility for the initiation, for the management and for setting up the financing of the clinical trial'.

In the EU framework for **VMPs**, two main challenges are observed: (i) duplicative, not-fit-for purpose regulatory framework applicable to VMPs that contain or consists of GMOs and (ii) disproportionate administrative burden in the handling of variations not requiring assessment.

There is an increasing focus in the EU and globally on the authorisation of biosimilar medicines, where approval is increasingly based on analytical and functional characterisation to demonstrate similarity to the reference product[89]. However, the EU's regulatory framework can still lead to the conduct of lengthy and costly multi-country comparative efficacy studies, even when robust analytical methods may demonstrate comparability and where a more tailored, risk-proportionate approach could therefore be justified. In the food area, EU law requires pre-market authorisations/approvals for several categories of **food and feed products and related food chain inputs**, the so-called 'regulated products'[90]. In those cases, the European Food Safety Authority (EFSA) must conduct a scientific risk assessment under the applicable sectoral legislation before risk managers at EU and/or national level decide to grant a pre-market authorisation/approval. Delays have been observed in both validation and risk assessment phases as also confirmed by the findings of the ongoing evaluation of the Commission on EFSA's Performance covering the period 2017-2024. These have been linked to: (i) dossier deficiencies and limited effectiveness of pre-submission advice, (ii) procedural consequences linked to non-compliance with study notification requirements; and (iii) governance constraints relating to EFSA's Scientific Committee/Scientific Panels.

As a cross-cutting element of complexity, developers of **AI applications** in biotechnology face a rapidly evolving regulatory landscape due to sectoral legislation and AI-specific requirements, including validation, documentation, explainability and risk-management obligations. The increasing integration of AI across the biotechnology lifecycle requires clarity on how existing EU frameworks apply across different development stages. Limited capacity, especially among SMEs and start-ups, to navigate applicable regulatory frameworks, further constrain the uptake of AI-enabled solutions in biotechnology. Public consultation responses[91] confirm that stakeholders see a need for greater clarity in the practical application of AI-related requirements across the biotechnology lifecycle (58% agreement). Stakeholders report uncertainty regarding how AI-related requirements interact with existing medicinal product and clinical research frameworks, particularly in areas such as model validation, documentation and lifecycle monitoring[92] and highlight the importance of guidance. Taken together, these patterns show that the EU biotech companies' difficulty to thrive is not a science deficit, but rather a **lack of translation and**

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[89] Reflection paper on a tailored clinical approach in biosimilar development: "Based on the advancements in analytical technology and the regulatory experience gained, a tailored approach for clinical development of biosimilar candidates is possible. CES are no longer expected to be required for approval of biosimilars that can be thoroughly characterised using state-of-the-art analytical methods and have demonstrated similarity in physicochemical and functional properties. Comparative clinical PK studies are still essential elements in the biosimilar development and can provide supportive safety and immunogenicity data. This tailored clinical approach is expected to be applicable for the majority of biosimilar candidates. A regulatory option that, under certain conditions, allows authorisation of biosimilars based on demonstrated comparability at the analytical level with a limited clinical data package streamlines the development process without compromising efficacy and safety".

[90] E.g. substances used in food and feed (such as additives, enzymes, flavourings, and nutrient sources), novel foods, food contact materials, genetically modified organisms, plant protection products etc.

[91] Public consultation, see footnote 74.

[92] Based on Landscape analysis study.

a scale-up deficit shaped by long, complex and capital-intensive pathways from research to market.

3.2.2 Driver 2: Fragmentation of enabling ecosystems and suboptimal incentives for private investment constraining the scale-up of EU biotechnology and biomanufacturing

The EU's ambition to scale and industrialise biotechnology is currently limited by challenges in key enabling factors, in particular fragmented thus insufficient access to finance, development of infrastructures (e.g. clusters), availability of specialised talent and the deployment of AI and use of data.

First, with regards to **access to finance**, the EIB estimates the **sector investment gap at EUR 40 billion annually.**[93] US biopharma start-ups received around nine times more late-stage funding than EU biopharma start-ups, with around EUR 219 billion of venture capital focused on health biotechnology invested in the US compared to EUR 25 billion in the EU, between 2015 and June 2025.[94] **Even larger gaps in access to late-stage capital** have pushed biotech companies to find funding abroad, notably in the US. Between 2015 and 2021, 67 EU biotech companies going for a public listing have targeted the US NASDAQ rather than the European stock markets, with 60% of them only listing on NASDAQ without any listing on an EU exchange.[95]

Similarly, in earlier stages of capital, **although the EU biotech venture ecosystem has expanded materially in recent years, it remains a second-tier venture capital market in global biotechnology**. Annual investment increased from EUR 0.84 billion (2015) to a peak of EUR 5.47 billion (2021) during a rapid expansion phase, before partially normalising to EUR 3.89 billion (2025) (around 29% below the 2021 peak) consistent with post-pandemic slower deployment (see Annex 5, Figure 7). As a comparison, the US operates at a different order of magnitude across the cycle (peaking at around EUR 45 billion in 2021)[96]. In the public consultation[97], stakeholders reported **difficult access to private and some public investment instruments** in the EU in equity, debt, and commercialisation support (for more information, see *synopsis report* in Annex 2). In their answers, companies also noted that public funding mechanisms, while supportive for early R&D stages are also insufficiently tailored to the needs of biotech scale-ups. Specific challenges of start-ups and SMEs were also underlined from both representatives of **business associations and public authorities**. Respondents from **academic and research** also pointed the fragmentation in funding rules and tax incentives across Member States, limited availability of large venture capital funds, and a lack of banking expertise in biotechnology. **Non-Governmental Organisations (NGOs)** also reported challenges related to fragmented and insufficient public funding and a lack of significant grant-making institutions. This shows a need to reinforce de-risking financial instruments and budgetary guarantees in the EU.

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[93] The EIB Group uses industry data of EUR 69.7 billion investment for the US vs EUR 26.5 billion in the EU in 2021, resulting in a gap of ca. EUR 40 billion.
[94] Landscape analysis study.
[95] Kempen & Co analysis, Liquidity slides for dual listed companies, 2021.
[96] Landscape analysis study.
[97] Public consultation, see footnote 74.

Biological VMPs benefit from two protection mechanisms: regulatory data protection (Articles 39-40 of Regulation 2019/6) and intellectual property (IP) protection (patents and SPCs) under Regulation (EC) No 469/2009[98]. Nevertheless, the veterinary pharmaceutical market presents specific challenges for innovation, such as fragmented species-specific markets, low price levels, and a small overall market size. In this context, there is a need for enhanced incentives to support the development of biotechnological VMPs to diagnose, treat or prevent zoonotic diseases.

Second, with regards to **biotechnology clusters,** their **outreach in the EU remains fragmented**, with significant disparities in scope and resources. Biotechnology clusters foster collaboration across entities at different stages of the biotechnology value chain and across biotech sectors. They enable the transformation and evolution of, for example, university spin-offs into companies ready for the commercialisation of their product[99]. While clusters compete for investment, talent and projects, there is substantial scope for collaboration, exchange of best practices allowing the realisation of scale effects that cannot be achieved at regional level. The few clusters that have an EU-wide relevance provide networking and coordination support, yet the landscape remains fragmented, risking duplication of efforts and limiting impacts. As a result, EU biotechnology clusters cannot fully leverage their collective potential to compete with clusters in other regions, and the EU as a whole underperforms in the field of biotechnology.

Respondents to the public consultation[100] identified **five main barriers** to clusters: insufficient **financial support** (58%), insufficient **public support** (54%), **incapacity to reach a critical mass of stakeholders** (46%), insufficient **collaboration among existing clusters** (46%) and insufficient **start-up incubators or business support infrastructure** (45%). Other barriers were identified, with representatives of companies pointing to the limited involvement of end-users (patients, healthcare providers) and public authorities underlining the insufficient support for product development. In the area of ATMPs, the lack of innovation hubs that combine emerging ATMP specialisation with infrastructure capabilities, regulatory expertise and collaboration among clinical centres, research bodies, industrial participants and commercialisation services, obstructs development in the EU.

Third, productivity gains, acceleration of innovation and efficiency improvements associated with the **use of AI and big data remain below their potential in the EU's biotechnology ecosystem**[101]. Fragmented, heterogeneous, and high-dimensional data is one of the challenges, as datasets generated in healthcare or research contexts are often not readily usable for the training, testing and validation of AI systems without significant curation and standardisation efforts. For health data, ongoing EU initiatives, such as the European Health Data Space, are expected in the coming years to facilitate access and cross-border use; at the same time ensuring that such data are sufficiently curated, annotated and interoperable to support AI development remains a key challenge. Data access challenges affect non-health data relevant to biotech, such as datasets on chemical

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[98] Regulation (EC) No 469/2009 OJ L 152, pp. 1–10 ELI: http://data.europa.eu/eli/reg/2009/469/oj.
[99] Landscape analysis study.
[100] Public consultation, see footnote 74.
[101] Submissions to the Call for Evidence and Public Consultation, as well as stakeholder workshop insights, as analysed in the study supporting the rapid assessment of policy scenarios to strengthen innovation and competitiveness in the field of biotechnology in the EU (Rapid Assessment Scenario Study forthcoming); Landscape analysis study - Annex 2 – Case Studies (forthcoming).

reactions used in drug discovery and biomanufacturing processes[102]. Public consultation[103] results confirm these challenges. Companies, business associations and academic or research institutions report the highest levels of difficulty. Around two-thirds of companies and research institutions rely on data sourced from outside the EU/EEA, primarily due to higher reliability and quality of available datasets, as well as clearer legal frameworks for data access and lower perceived access costs. This suggests that improving the quality, usability and interoperability of datasets available within the EU could significantly strengthen the competitiveness of the European biotechnology ecosystem.

Fourth, the EU's ambition to scale and industrialise biotechnology also depends on the **availability of specialised talent**. While the EU maintains a strong scientific base, stakeholder evidence points to persistent gaps in the workforce segments critical for scale-up and deployment.

In the EU's biotechnology sector, stakeholders have identified **gaps in the workforce segments** needed for scale-up, progression to commercial scale and manufacturing[104]. There is a significant shortage in the total number of **higher education and vocational education and training graduates entering the biotechnology sector**[105], with shortages particularly observed in the following competences: biomanufacturing and bioprocessing, regulatory and compliance (e.g. ATMPs and pharmacovigilance), and digital skills[106] (e.g. bioinformatics, AI-enabled processes, data governance). Furthermore, for some profiles, stakeholder consultations indicated that technical skills would need to be complemented by additional competencies[107]. This challenge can be explained by several factors[108], such as constrained hands-on training capacity due to the scarcity and high cost of specialised facilities, the fragmentation of qualification systems across Member States, limiting **skills portability and talent mobility** and the **lack of entrepreneurial skills** in the EU (less than 50% of EU students have access to entrepreneurial education in secondary and higher education[109]). Barriers to the **upskilling** of workers are also relevant.

3.2.3 Driver 3: Rapid technological developments & regulatory adaptation lag

EU biotechnology companies also face barriers to manufacture, place products on the market and grow due to EU regulatory frameworks not always keeping up with rapid technological advances. This perception was confirmed in stakeholder consultations, as regulatory frameworks that insufficiently account for the innovative and rapidly developing nature of the biotechnology sector were also identified as a barrier to the expansion of EU companies[110].

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[102] Landscape analysis study - Annex 2 – Case Studies.
[103] Public consultation, see footnote 74.
[104] Landscape analysis study.
[105] Landscape analysis study.
[106] In 2023, only 55.6% of the EU adult population had at least basic digital skills. Digital Decade DESI visualisation tool DESI 2025, DESI indicators, Indicator: At least basic digital skills. https://digital-decade-desi.digital-strategy.ec.europa.eu/datasets/desi/charts/desi-indicators.
[107] Landscape analysis study based on Biotechnology Jobs. (2025, February 12). Building the Ultimate Biotech Skill Set: Technical and Soft Skills Employers Want in 2025.
[108] Landscape analysis study.
[109] EU Startup and Scaleup Strategy, based on European Commission's Entrepreneurship 2020 Action Plan.
[110] Public consultation, see footnote 74.

One major reason brought forward by stakeholders and some Member States is the complex, not innovation friendly and in part ill-fitting EU legislation on GMOs, in particular as regards GMMs. The EU's legal framework for GMOs (including GMMs[111]) originates from the 1990s and was primarily tailored to genetically modified plants. The framework as it currently stands is **less suitable to handle GMMs given the biological differences compared to plants and the variety of micro-organisms and their applications[112]**. In a recent scientific opinion, EFSA concluded that the potential risk of a microbial product relates to the changes introduced in the micro-organism itself regardless of the method used to introduce them, supporting a more holistic approach to the assessment of micro-organisms based on the characteristics of the final product.[113] It further concluded that for certain GMMs less assessment requirements would be sufficient to ensure safety. In this respect, considering the risks of typical GMMs and derived products and the variety of risk profiles, the current GMO regulatory procedures are considered by many stakeholders and some Member States as **disproportionate, overly rigid and time consuming**, rendering the EU unattractive for investing and bringing such GMMs to the market as products. In addition, the particularly short development cycles of GMMs, with one product frequently being based on a previous product, mean that a quick, efficient authorisation path is very important.

There is also a regulatory gap between the existing EU legal framework on the **quality and safety of human organs** intended for transplantation and current medical practice on organ preservations, leading to **diverging interpretations across Member States and hence legal uncertainty**. The current EU framework was designed for preservation rather than processing and does not provide a clear, harmonised approach for these activities. It does not include a particular framework for authorising processing operations, nor a mechanism for benefit-risk assessment of these interventions, and no provisions for oversight of transplantation centres that seek to apply a specific processing technique. Furthermore, the current framework does not contain a mechanism for coordination between the organ transplant competent authority and the competent authorities operating under other legislative frameworks (e.g. on medical devices, medicinal products, or on SoHO). In 2024, although over 32,000 organ transplants were performed across the EU, the gap between the supply of transplantable organs and clinical demand remained, with over 52,000 patients registered on transplant waiting lists across the EU (as of 31 December 2024)[114].

Similarly, in the **ATMP** area, outdated definitions and technological lag in the regulatory framework are leading to discrepancies between the legal framework (classification

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[111] In line with the definitions provided in Directive 2001/18/EC, GMO means an organism, except for human beings, in which the genetic material has been altered in a way that does not occur naturally by mating and/or natural recombination. In line with the definition provided in Directive 2009/41/EC (OJ L 125, 21.5.2009, p. 75-97. ELI: http://data.europa.eu/eli/dir/2009/41/oj), a GMM means a microorganism in which the genetic material has been altered in a way that does not occur naturally by mating and/or natural recombination.

[112] Regulatory Framework Study (forthcoming, see Annex 1).

[113] EFSA GMO Panel (EFSA Panel on Genetically Modified Organisms), Mullins, E., Bresson, J.-L., Dewhurst, I. C., Epstein, M. M., Firbank, L. G., Guerche, P., Hejatko, J., Moreno, F. J., Naegeli, H., Nogué, F., Rostoks, N., Sánchez Serrano, J. J., Savoini, G., Veromann, E., Veronesi, F., Cocconcelli, P. S., Glandorf, D., Herman, L., Jimenez Saiz, R., Ruiz Garcia, L., Aguilera Entrena, J., Schoonjans, R., Kagkli, D. M., Dalmay, T. (2024). New developments in biotechnology applied to microorganisms. EFSA Journal, 22(7), e8895. https://doi.org/10.2903/j.efsa.2024.8895.

[114] SANTE-SoHO/D2 - European Commission. (2026). *Introducing "organ processing" into directive 2010/53/EU* [Unpublished slideshow]. See Annex 5 for more details.

uncertainty) and progressing science, hampering market access for these products (e.g. affecting in vivo tissue generation, acellular therapies, and bio-synthetic hybrids).

The rapid technological advances characterising the biotechnology sector challenge existing regulatory frameworks. While these frameworks ensure high safety standards, they offer limited regulatory flexibility in a context of rapid technological advances. As a result, innovative biotechnology applications may struggle to progress efficiently from development to market deployment across these sectors when regulatory frameworks are not sufficiently adapted to emerging technologies. In the health area, existing regulatory frameworks are not fully adapted or adaptable to **complex and hybrid emerging health biotechnology products**, leading to barriers such as a lack of legal certainty on **products classification and evaluation**. In the **VMPs sector**, regulatory uncertainty also represents a serious hurdle to the marketing or use of **novel technologies, methods or products**. In the **food chain sector**, EU legislation requires pre-market authorisations for several categories of food, feed and related food chain inputs but also provide for limited regulatory flexibility.

Biotechnologies are critical for the **Union's defence and security**. And while rapid advances in biotechnology over the last decades have brought considerable benefits for healthcare, research and the economy, such advances also make biological agents more widely accessible, increasing risk of their misuse. For example, the price of synthetic nucleic acids (such as DNA or RNA) has decreased more than 100-fold over the past 20 years[115], making it increasingly feasible to synthesise potentially dangerous genomic sequences, such as the DNA or RNA of viruses, at limited cost.

While some commercial nucleic acid synthesis providers screen orders to identify potentially dangerous sequences and verify the legitimacy of their customers, there are currently no EU-level rules on this. National rules diverge[116], failing to offer a level playing field, fragmenting the single market and undermining the EU's prevention and biosecurity efforts.[117] In stakeholder consultations, several industry actors, including SMEs, have therefore called for harmonised EU biosecurity rules.

Finally, the **potential of data and AI** remains largely untapped in the EU. Despite new regulatory frameworks (European Open Science Cloud, AI regulatory sandboxes mandated in the AI Act, EuroHPC/AI factories/gigafactories), some challenges remain. **AI biotech platforms** are inherently capital-intensive, relying on costly model training and large-scale data generation, and depend on access to **high-quality data** and advanced computing infrastructure, which remain comparatively limited in the EU[118]. Furthermore, the **scaling of AI**[119] in biotechnology requires sophisticated experimentation environments capable of integrating high-performance computing, biological laboratories and advanced

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[115] DNA Synthesis and Sequencing Costs and Productivity for 2025, Rob Carlson, 2025 https://www.synthesis.cc/synthesis/2025/5/dna-synthesis-and-sequencing-costs-and-productivity-for-2025.
[116] https://ibbis.bio/policy-spotlight-french-leadership-defining-and-regulating-genetic-fragments/
[117] See IBBIS mapping of explicit and implicit rules on NA synthesis screening across countries: https://globalsynthesismap.bio/policy?country=FRA.
[118] Landscape analysis study - Annex 2 – Case Studies.
[119] COM(2025) 30 final based on Eurostat data: EU survey on ICT usage and e-commerce in enterprises (January 2025).

data infrastructures.[120] However, the availability of such integrated environments remains limited in the EU. Stakeholders underlined the importance of collaborative environments that facilitate the responsible testing, validation and deployment of AI-enabled biotechnology solutions while ensuring compliance with EU legislation[121].

3.3 Consequences

3.3.1 EU is facing a major competitiveness gap in biotech, despite being a leader in science

The problem presented in section 3.1. results in a **gap between the EU and the US in terms of entrepreneurial dynamism**. The EU only represents 22% of the global biotech start-up activity (i.e. start-ups founded since 2015) while the US accounts for nearly half (49%) of them (see Annex 5, Figure 6)[122].

This competitiveness gap is observed across the health and agri-food biotechnology sectors:

The EU is becoming less attractive to sponsors for **conducting clinical trials** in global comparison, affecting the economy, public health, and skill capacity building. The number of clinical trials conducted worldwide has increased substantially over the past decade, rising from approximately 13,000 in 2013 to 22,000 in 2023. Over the same period, the geographical distribution of clinical trials has shifted markedly. While the **EEA's share of global clinical trials declined** from 18% in 2013 to 9% in 2023, China's share increased from less than 10% to nearly 30%[123]. A similar trend is observed in cell and gene therapy trials: between 2013 and 2023, Europe's global share steadily declined, whereas China experienced a dramatic rise over the same period, emerging as the leading region.[124] This growth also reflects a different portfolio structure, with China placing stronger emphasis on early-stage and post-marketing development, particularly mononational Phase I and Part IV trials, compared to the EEA's stronger focus on multinational Phase II and Phase III trials[125]. Despite the strong growth in global clinical trial activity, the level of activity in terms of number of clinical trials submissions in the EU remained broadly stable between 2013 and 2025[126]. Consequently, the EU did not capture a proportional share of this growth, leading to a widening competitiveness gap relative to other regions with

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[120] Breakthroughs in in silico, AI-driven and digital-twin approaches can transform biomedical research, diagnostics, and personalised medicine. In silico modelling leverages computer simulations increasingly integrated with AI and can potentially replace some traditional animal testing and clinical trials. AI-driven analytics can enable precise, individualised therapies and accelerating tissue repair (*Healing the Future - Horizon scanning for emerging technologies and breakthrough innovations in the field of cell and gene therapies* https://publications.jrc.ec.europa.eu/repository/handle/JRC141934).

[121] See Annex 2, Synopsis report for more details.

[122] Landscape analysis study.

[123] EFPIA report: assessing-the-clinical-trial-ecosystem-in-europe.pdf.

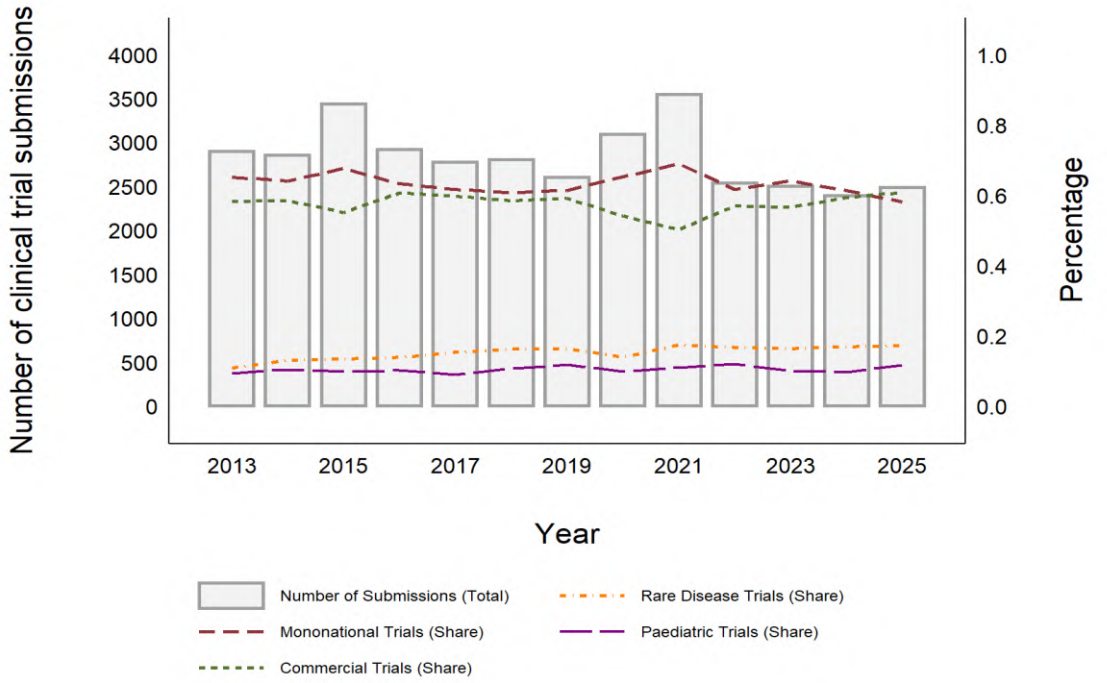
[124] EFPIA report: assessing-the-clinical-trial-ecosystem-in-europe.pdf.

[125] Fast-track landscape analyses to assess the regulatory clinical trial eco-system in the EU/EEA and in other relevant regions (forthcoming)

[126] The annual number of clinical trial applications submitted in the EU, as well as the shares of mononational, commercially sponsored, and pediatric trials, exhibited little variation over this period, aside from temporary changes during the COVID-19 pandemic. Between 2013 and 2025, the annual average number of clinical trials application is 2,895 with a standard deviation of 303.

potential implications for economic performance and timely access by patients to innovative treatments[127].

Figure 2. Clinical trial activity in the EU[128]

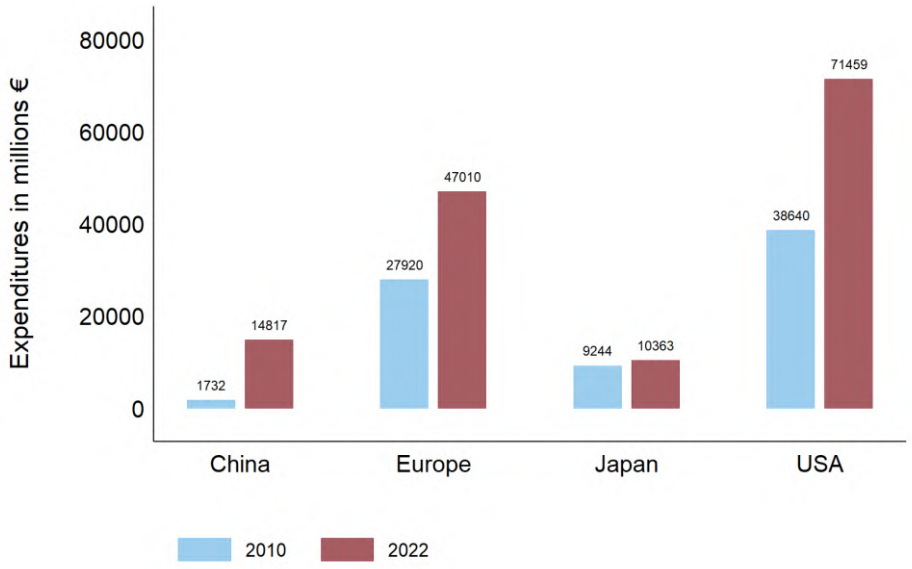


***Note**: Figure 2 describes the trend in the submission of clinical trial applications in the EU between 2013 and 2025. The bars illustrate the total number of applications per year, while the remaining lines show the share of the applications for mononational, commercial, paediatric and rare-disease trials.*

The gap in clinical trial activity between the EU and other markets, particularly China, is also reflected in **changes in annual pharmaceutical R&D expenditure** of which approximately 60% can be on average allocated to clinical trials[129]. Using overall pharmaceutical R&D expenditure as a proxy, China experienced a significantly larger increase in R&D expenditure compared to the other regions[130].

[127] The decline in the EEA share of global clinical trials stems from multiple, interlinked factors, with enhanced regulatory complexity being one of the driving factors.
[128] Calculations done by the European Commission based on data from EudraCT and CTIS.
[129] EFPIA report (2024) The Pharmaceutical Industry in Figures. Key Data. According to the report, 48.4% of the pharmaceutical R&D expenditures are allocated to Phase I to III trials and additional 11.5 % to Phase IV trials. The data is based on a survey with Members of PhRMA and collected in 2022. See further information here: PhRMA_membership survey_single page_70523_es_digital.pdf.
[130] China experienced an increase by a factor of 8.55 compared to a factor of 1.68 increase in Europe.

Figure 3. Pharmaceutical R&D expenditure in selected regions in 2010 and 2022[131].



Note: *Figure 3 illustrates annual pharmaceutical R&D expenditures in 2010 and 2022 for China, Europe, Japan and the USA.*

These trends show that Europe's position in global pharmaceutical R&D, including its clinical trial activity, is facing increasing challenges in maintaining its competitive edge[132].

The relative decline in clinical trial activity is likely to come along with adverse effects on the public healthcare systems, the economy and the development of skills, and capacity within the Union. Although estimating the economic impact of clinical trial activity is inherently complex and dependent on multiple model assumptions, recent studies based on national and EU-wide data indicate that each euro invested in clinical trials generates between EUR 1.60 and 2.50 of added value for the economy, along positive effects on employment[133]. A recent report estimates that commercial trials, which account for 53.1% of the trials authorised in the Union[134], create annually about EUR 35.7 billion in gross value added to the economy in the EEA and support the creation of over 165,000 jobs[135].

In addition, clinical trial activities are estimated to reduce costs for national healthcare systems. For example, two studies conducted in Italy indicate a leverage effect of avoided costs between 2.2 and 3.5; that is, for every euro invested in clinical trials or disbursed by

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[131] EFPIA report (2024) The Pharmaceutical Industry in Figures. Key Data.
[132] Tan et al. (2024) Current landscape of innovative drug development and regulatory support in China | Signal Transduction and Targeted Therapy.
[133] Walter et al. (2020) Economic impact of industry-sponsored clinical trials of pharmaceutical products in Austria - PubMed; Battelle Technology Partnership Practice (2015) Biopharmaceutical Industry-Sponsored Clinical Trials: Impact on State Economies (battelle-2015-study.pdf); Fast-track landscape analyses to assess the regulatory clinical trial eco-system in the EEU/EEA and in other relevant regions (forthcoming).
[134] See EU clinical trials during the 3-year CTR transition period
[135] EFPIA report (2026) the-economic-impact-of-industry-clinical-trials-across-europe.pdf.

sponsoring companies to health facilities, the Italian national health system saved more than two euros[136]. Finally, clinical trial activities also affect the health of European citizens. For example, patients participating in clinical trials can access innovative medicines up to 5 to 10 years before commercial launch in the EU.[137]

The innovation and competitiveness gap in the EU's ATMP sector is also becoming increasingly evident. Although Europe currently holds a **22.1% share** of the global gene therapy market (2025) and the sector is projected to grow at a compound **annual growth rate of 14,6%** (2026–2033), the region **risks falling behind North America and the Asia-Pacific region**. Forecasts indicate that North America will generate the highest revenue in the sector by 2033, while the Asia-Pacific market is expected to reach USD 4,193.1 million in the same year[138]. This trend, also highlighted in the Draghi Report[139], underscores the EU's struggle to strengthen its R&D capabilities and address the **innovation and competitiveness** gap in the region's ATMP market. Slow progress in ATMP development threatens the EU's position in the global healthcare and biotechnology sectors, as innovators may increasingly choose to operate in jurisdictions with more agile regulatory frameworks.

When it comes to **biosimilars**, the EU's biomanufacturing capacity can be further utilised. The EU has significant untapped potential in biosimilar manufacturing. The EU retains the largest cumulative authorisations of biosimilars (151 products vs. 72 in the US and 66 in Canada) and leads the global market in revenue valued at approximately EUR 25.6 billion in 2024, representing around 6% of the total pharmaceutical industry's contribution to GDP. Sales grew at 15% annually between 2020 and 2024 across five core therapeutic areas, and the sector supports around 81,000 jobs in Europe: 16,000 direct (20%), with the remainder split between indirect roles such as clinical research (30%) and induced employment (50%). The sector depends on a highly skilled workforce, with 40% of direct employees (~7,000) in technical roles and 70% holding university degrees.[140] However, manufacturing activity is increasingly migrating to Asia, particularly South Korea and India[141]. While the EU remains a key innovation hub — **supplying over 75% of active ingredients** for innovative biologics — it must develop more dedicated and cost-efficient manufacturing infrastructure to keep pace with international competitors.[142]

Similarly, challenges to bring **novel health biotechnology products** to the market have been observed[143], and are generally expected to increase given the increasing complexity and hybrid nature of novel products. These challenges further weaken the EU's position in

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[136] Cicchetti et al. (2020) Valorization of clinical trials from the Italian National Health Service perspective: definition and first application of a model to estimate avoided costs - PubMed; Polignano et al. (2022) Economic impact of industry-sponsored clinical trials in inflammatory bowel diseases: Results from the national institute of gastroenterology "Saverio de Bellis" - PMC.
[137] EFPIA report (2024) Assessing the clinical trial ecosystem in Europe: Final report, p.37
[138] https://www.grandviewresearch.com/horizon/outlook/gene-editing-market/europe.
[139] See footnote 11, page 6.
[140] Medicines for Europe, European Biosimilar Medicines Sector: Delivering Impact Beyond Health – Economic, Scientific & Strategic Contribution, Biotech Act Factsheet Series, March 2026, Pillar_1-2-3-4-FOOTPRINT-SPC-Biotech-Act-facsheets-ppt.cdr.
[141] Cohen, H. P., Turner, M., McCabe, D., & Woollett, G. R. (2023). Future evolution of biosimilar development by application of current science and available evidence: The developer's perspective. *BioDrugs*, *37*(5), 583–593.
[142] BioPlan Associates (2024). Annual Report and Survey of Biopharmaceutical Manufacturing Capacity and Production
[143] Maxwell, A. (2025, August 20). *Why EU innovators face growing barriers with EU combination product regulation*. Medtech Insight.

the global innovation race, delaying the availability of these products to patients across the EU.

Regulatory uncertainty also has practical implications for cross-border exchange of processed **organs** and for the scaling of machine perfusion technologies, contributing to uneven uptake and fragmentation within the EU[144], and eventually reducing the potential for optimal matching of organs and recipients. Over the last three years, the cross-border exchange rate of allocated organs fluctuated between 20-23%[145].

In the field of **GMMs**, recent evidence shows that there is significant activity in the development of innovative GMMs by both private and public/academic entities. However, the majority of the identified GMMs originates from the US or China, while **only a limited number were developed within the EU**[146]. Notably, there are **currently no products on the single market that contain or consist of GMMs**, except for medicinal products which are, however, not authorised under the GMO legal framework but under the EU framework on pharmaceuticals. This is particularly affecting the competitiveness of SMEs in the GMM field, since they are typically among the most innovative companies in the biotechnology sector but may lack the regulatory expertise and financial resources to navigate complex legal frameworks that may not adequately fit their innovative products. At the same time, this lack of products on the markets, or prospect to bring products efficiently to the market, is **discouraging investments in R&D, thus negatively impacting the wider science community in the EU**. EU economic sectors, foremost the agricultural, industrial and environmental sectors, are negatively impacted as they cannot easily make use of novel tools available in third countries that make operations more efficient.

As a consequence of the issues outlined under drivers 1 and 3, a competitiveness gap is also observable for biotechnology products in the food chain (**food and feed products)**, including substances (e.g. additives, enzymes etc), and other food chain inputs (e.g. food contact materials).

Finally, as **AI** continues to expand across the biotechnology lifecycle in the health and food sectors, the bottlenecks innovators face when attempting to develop, validate and deploy AI-enabled biotechnology solutions in the EU slow the transition from scientific discovery to real-world deployment. As a result, the Union risks failing to fully capture the productivity gains, efficiency improvements and innovation opportunities associated with the large-scale use of AI in biotechnology. This under-utilisation of AI may therefore translate into a growing competitiveness gap, as other jurisdictions more effectively integrate AI into biotechnology research, development and production across multiple biotech sectors.

[144] See Annex 5 for more details.
[145] See Rapid Assessment Scenario Study
[146] Ballester, A.R., Roqué, M., Ricci-Cabello, I., Rotger, A., Malih, N. 2023. Horizon scanning on microorganisms and their products obtained by new developments in biotechnology. EFSA supporting publication 2023:EN-8503, 65 pp. doi:10.2903/sp.efsa.2023.EN-8503.

3.3.2 Biotech's potential to help address major societal challenges is underexploited

Despite its innovation and growth potential, and expected benefits for patients and society at large, the potential of biotechnology remains largely underexploited in the EU, across the different biotechnology areas.

In the health sector, **ATMPs** – such as gene therapies – offer a transformative potential for treating a broad spectrum of diseases, from rare genetic disorders to common chronic conditions. Despite this promise and the innovative potential in the EU, patient access in the EU remains severely limited.

Organ transplantation also increasingly involves *ex-vivo* organ processing, such as machine perfusion to assess and recondition donor organs prior to transplantation. These techniques can expand the pool of transplantable organs, improve matching of organs and recipients, improve clinical outcomes and strengthen health-system resilience. However, there is limited cross-border exchange and slow EU-wide uptake of innovative processing technologies[147].

In the **VMP sector**, the potential of biotechnology to improve animal health and strengthen food supply chain resilience remains underdeveloped. Favourable regulatory conditions for the use of biotechnology in animal health will not only benefit the biotech pharmaceutical industry, but also farmers, animal health, the food supply chain and human health (zoonotic diseases). When disease spreads in food-producing animals, the impact on farmers can be dire and food security can be put at risk. Vaccination is a powerful tool to prevent and control animal diseases and contributes to reducing the use of antimicrobials. As a large share of emerging infectious diseases in humans has a zoonotic origin, preventing animal diseases also has positive effects on human health. Novel biotechnology tools enable the development of safer and more effective vaccines, as well as the distinction of vaccinated animals from those infected, and allow vaccines to be developed or adapted much faster than with conventional approaches, which is critical to prevent and control animal diseases.

When it comes to **innovative GMMs[148]**, their non-deployment in the EU market, in addition to the loss of competitiveness opportunities outlined under the driver 3, result in an unfulfilled potential for GMMs to contribute to other social and environmental objectives. For example, GMMs can now be used as or in biofertilisers, biostimulants, biopesticides, biocides, bioremediation, wastewater treatment, biomining and bioleaching, offering benefits in the wider agri-food, industrial, marine and environmental sectors. As GMM-based products that would be more efficient or environmentally friendly than the alternatives are not being used, EU society and consumers are negatively impacted as they are being deprived of innovative solutions to important challenges linked to climate change, environmental pollution and food and feed security.

Moreover, in the **food and feed sector**, the potential of biotechnology remains underexploited and experimentation in real conditions (e.g. real-world food production and

[147] See Rapid Assessment Scenario Study.
[148] See Annex 7, Section 5.2. for examples.

processing environments) is constrained. Biotechnology tools can support more efficient production processes across the food chain. These technologies can improve the safety and quality of food products, for example by reducing pathogens and toxins and enhancing nutritional properties[149]. Enzymes and biocatalysts can reduce environmental impacts by replacing chemical processes and improving process efficiency[150]. As regulatory drivers constrain the deployment of innovations in such areas, the single market as well as the contribution of biotechnology to food safety and security, resource efficiency, environmental sustainability and the EU's strategic autonomy, are not fully realised.

Furthermore, the **rapid advancement of technology, including AI, alongside increased data collection and use** are cross-cutting enablers with huge potential across the different areas of the biotech sector, yet to be fully utilised. AI and advanced data use act as key enablers of biotechnology innovation across health and agri-food applications. AI-driven tools support target identification, molecular design, optimisation of biological processes, CT design and manufacturing efficiency, while in the agri-food sector they enable advanced fermentation, strain optimisation and resource-efficient production. These applications can accelerate innovation cycles, reduce development costs and strengthen resilience in areas such as medical countermeasures, disease detection and sustainable food systems. However, the full potential of AI-enabled biotechnology remains underexploited in the EU. The effective deployment of these technologies requires clear lifecycle guidance, as well as access to integrated environments combining experimental and computational capabilities for testing and validation, and high-quality, well-curated datasets that allow AI systems to be trained and deployed reliably. The absence of these enabling conditions limits the EU's ability to fully harness biotechnology to address major societal challenges and strengthen its strategic autonomy in health and food systems.

Regarding the application of biotechnology to defence and security, the primary challenges identified by stakeholders in the public consultation were the **risks to strategic autonomy** in biomanufacturing and availability of countermeasures (50%), **cybersecurity risks** (e.g. biotechnology infrastructure, AI tools used) (45%), vulnerabilities in the **resilience of biotechnology supply chains** (44%), and threats related to biosecurity and biosafety including **misuse of biotechnology** (40%). Finally, **opportunities** in biosecurity were also particularly underlined in the public consultation[151]: the development of new innovative medical countermeasures (45%), detection of biological threats (45%), as well as increased food security (43%).

3.3.3 Potential for misuse of biotechnology poses growing biosecurity risks

In addition, the rapid progress in and increasing accessibility of biotech innovations also increase the likelihood and impact of biosecurity incidents, as certain applications of

[149] Siddiqui SA, Erol Z, Rugji J, Taşçı F, Kahraman HA, Toppi V, Musa L, Di Giacinto G, Bahmid NA, Mehdizadeh M, Castro-Muñoz R. An overview of fermentation in the food industry - looking back from a new perspective. Bioresour Bioprocess. 2023 Nov 28;10(1):85. doi: 10.1186/s40643-023-00702-y. PMID: 38647968; PMCID: PMC10991178.
[150] Palanisamy Vasudhevan, Zhang Ruoyu, Hui Ma, Subhav Singh, Deekshant Varshney, Shengyan Pu, Biocatalytic enzymes in food packaging, biomedical, and biotechnological applications: A comprehensive review, International Journal of Biological Macromolecules, https://doi.org/10.1016/j.ijbiomac.2025.140069.
[151] Public consultation, see footnote 74.

technologies and products may have the potential for misuse, posing threats to public health and safety.

The exponential decrease in the cost of synthesising DNA and RNA has made the physical material needed to create biological agents, including dangerous pathogens, increasingly accessible. Studies by RAND Europe[152] and CLTR[153], two independent non-profits, estimate the current annual probability of a large-scale biological attack using synthetic nucleic acid at 1%, and accidents from their use at 1.5%, with expected economic harm to the EU of almost EUR 20 trillion for a large-scale event.

The EU joint biotechnology risk assessment carried out under the EU's Economic Security Strategy argues that the likelihood and impact of such incidents is further amplified by advancements in AI and computational tools in biological applications, which can lower the technical barriers to the design, optimisation and misuse application of biological agents.

These advances could significantly expand the pool of individuals capable of conducting technical tasks with biotechnology products that could be misused for malicious purposes, such as synthesising dangerous pathogens. Modelling published in 2025 suggests that, under a set of assumptions about near-term AI capabilities, could increase the annual probability of an epidemic from biological misuse by a non-state actor roughly sixfold, from 0.15% to 1%[154]. This reflects broader concerns that AI tools developed for beneficial applications in life sciences may also present dual-use risks if misapplied.[155]

3.4 What is the baseline from which measures are assessed?

The baseline trajectory through 2038[156] is **expected to show a widening of the competitiveness gap between the EU and its main competitors.** This is driven by the compounding interaction of the identified drivers. Regulatory complexity deters investment; market fragmentation, in particular capital scarcity, constrained innovation; and the expanding pipeline of frontier technologies amplifies administrative burdens and regulatory barriers, within an ecosystem that is not positioned to absorb them. The thematic sections that follow detail these dynamics across five interconnected dimensions[157].

Regulatory landscape and administrative burdens

Under the baseline, biotechnology developers will be confronted with an EU regulatory landscape characterised by three mutually reinforcing deficiencies: the proliferation of overlapping and partially incompatible regulatory frameworks, the persistent national

[152] Zakaria, S. *et al.* (2026). Cost–benefit analysis for synthetic nucleic acid screening in the European Union. Santa Monica, CA: RAND Corporation, 2026. https://www.rand.org/pubs/research_reports/RRA4805-1.html.
[153] Fady, P. *et al.* (2025). Cost-Benefit Analysis of Synthetic Nucleic Acid Screening for the UK. The Centre for Long-Term Resilience. doi.org/10.71172/kyey-h0ya, p.18.
[154] Righetti, 2025 (https://www.governance.ai/research-paper/dual-use-ai-capabilities-and-the-risk-of-bioterrorism-converting-capability-evaluations-to-risk-assessments).
[155] Urbina, F., Lentzos, F., Invernizzi, C. *et al.* Dual use of artificial-intelligence-powered drug discovery. *Nat Mach Intell* **4**, 189–191 (2022). https://doi.org/10.1038/s42256-022-00465-9
[156] Some interventions have been assessed over a different baseline. See Annex 4 for more information on the timeline and baseline.
[157] See Rapid Assessment Scenario Study for more information including on assumptions used.

heterogeneity in the implementation of procedures, and the absence of structured pathways for innovations that do not fit neatly within existing legislative frameworks.

The most pervasive source of regulatory burden is **procedural duplication across intersecting frameworks**. Under the baseline, developers of **GMO-containing ATMPs** will continue to incur in up to 50 additional days in CT authorisation timelines beyond conventional medicines, with further extensions for substantial modifications. For **VMPs, GMO-containing products** will continue to face approval timelines two to three times longer than non-GMO equivalents despite the fact that no GMO-containing VMP has ever received a negative opinion on the basis of its GMO component, triggering an 'innovation tax', estimated at EUR 269,000-840,000 per year in FTE burden. Such a burden is set to compound, reaching EUR 403,000-1,119,000 by 2040, as the pipeline is projected to encompass approximately 100 GMO-containing VMPs in various stages of development at any point through 2040. In addition, **CT applications** subject to dual-track requirements will increase from 8-15 to 12-20 per year and annual GMO-containing marketing authorisation applications are expected to rise from the current 3-5 to approximately 5-8 per year by 2040. Meanwhile, the administrative burden from variations not requiring assessment (VNRA) is projected to scale proportionally with the expanding marketing authorisation base: the number of active veterinary Marketing Authorisations is expected to grow to approximately 55,000-60,000 by 2040, with annual VNRA volumes scaling accordingly. The aggregate annual VNRA fee burden is projected to grow in proportion.

In the **biosimilar** domain, the **disproportionality of certain regulatory requirements** will also persist. Between 2012 and 2022, 100% of all 36 monoclonal antibody and fusion protein marketing authorisation applications included a Phase III comparative efficacy study, yet in no case did the clinical data determine the regulatory decision. Thus, the most costly component of biosimilar development (EUR 19-26 million per study, representing 20-50% of total expenditure and adding 12-24 months to timelines), is likely to function as a ***de facto*** **procedural requirement** rather than a scientifically decisive evidentiary input. Indeed, there is increasing experience with tailored clinical efficacy and safety approaches for biosimilars at the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA). Based on EMA data, in 2025 more than 70% of adopted scientific advice procedures for biosimilars incorporated a tailored clinical efficacy and safety approach. In addition, among all biosimilar marketing authorisation applications submitted last year under Article 10(4) of Directive 2001/83/EC, approximately 8% proposed a tailored clinical efficacy and safety approach. While there is a gradual shift toward more tailored approaches by EMA[158], a slow uptake of these changes risks putting Europe at a competitive disadvantage compared with other regions that operate under more agile frameworks.

The EU's regulatory framework will be marked by persistent fragmentation in the implementation of clinical trials across Member States. The EU's share of global clinical trial initiations has halved, declining from 18% in 2013 to 9% in 2023. Without fundamental reforms, particularly in streamlining authorisation procedures and simplifying regulatory requirements, the EU risks a further decline in its clinical research

[158] EMA, 2026 (https://www.ema.europa.eu/en/documents/other/reflection-paper-tailored-clinical-approach-biosimilar-development_en.pdf-0).

competitiveness. Meanwhile, the more centralised markets of the US and China continue to outperform the EU, further consolidating their dominance. China's share of global clinical trials, for instance, has surged from 8% to 29% over the last decade, while the US maintains its strong position. **Current projections suggest that the EU's growth in clinical trial activity will remain sluggish compared to these faster-expanding regions, risking a further relative decline in its standing as a key hub for clinical research.**

However, this does not imply a decline in the absolute number of EEA trials as the Clinical Trial Regulation (fully applicable to all clinical trials since January 2025) is expected to generate a 'learning effect' as sponsors and national authorities gain experience with the Clinical Trials Information System (CTIS). This will lead to moderate improvements in median authorisation times and greater procedural stability over 2025-2030. Between 2025 and 2030, AI tools are projected to achieve 60-70% routine adoption, e.g. in trial design and conduct, and digital trial components are expected to become increasingly integrated into standard protocol design. However, national heterogeneity in ethics review, contracting, insurance, and site activation will persist. Clinical trial activity concentrates further in a few Member States, large firms widen their advantage over SMEs, and clinical translation of the EU's strong scientific output stagnates. SMEs and academic innovators increasingly rely on partnerships with larger companies, slowing EU-based evidence generation and eroding Europe's leadership in emerging technologies. In absolute terms, the number of EU clinical trials has remained broadly stable over the past decade at approximately ~2,400–2,500 trials per year, with no structural upward trend expected under the baseline[159].

For **SoHO**, 50 national competent authorities will continue to oversee blood and tissue establishments (currently at 1,400 and 3,258, respectively), with recurring administrative costs to businesses estimated at EUR 4.14 million over ten years under the incoming risk-based authorisation model. While Regulation (EU) 2024/1938 introduces a more unified SoHO Preparation Authorisation (SPA) model, regulatory uncertainty will persist for novel, complex, and cross-framework SoHO innovations. Meanwhile, **permitting for strategic health biotechnology projects** is projected to remain fragmented and slow under the lack of EU-level priority-project recognition or fast-track governance mechanisms.

These burdens are further aggravated by the **absence of structured innovation support**. In the **food and feed domain**, over 90% of application dossiers assessed by EFSA in 2021-2023 contained administrative or scientific errors leading to the suspension of the legal deadlines for risk assessment (the so-called stop-the-clock procedure) resulting in delays in the overall authorisation process, which are likely to persist under the baseline scenario. For **AI-enabled methodologies**, EMA's Qualification Procedure for Novel Methodologies costs EUR 89,000 per request, takes 160-250 days, and produces confidential, non-reusable outcomes, meaning each new entrant faces identical navigation costs with no cumulative learning. The scale of regulatory demand on the EMA is itself accelerating. It received 635 scientific advice requests in 2024, a figure that grew 16% year-on-year in the first half of 2024 alone, this mismatch between the pace of technological innovation and

[159] Rapid Assessment Scenarios study, forthcoming

the capacity of existing regulatory infrastructure to absorb it is expected to grow under the dynamic baseline.

Competitiveness and investment attractiveness

The capital base underpinning the biotech sector shows a **pronounced investment gap.** As presented under section 3.2.2, EU biotech venture capital stood at approximately EUR 4.8 billion across 243 rounds in 2024, roughly six times below US levels. The average EU late-stage financing round (approximately USD 49 million) is around half of the US equivalent, and the EIB estimates the structural annual investment gap at approximately EUR 40 billion. This capital shortfall is not merely quantitative; it is structurally embedded (see sections 3.1 and 3.2.2). **Despite the existence of significant public financial engagement, this gap shows no prospect of autonomous closure under the baseline.** The deep dependence on external capital, which weakens strategic autonomy over the direction and location of value creation, is expected to grow. The median time gap between funding rounds has increased from 17 to 20 months, with higher incidence of down rounds at Series C stage (growth stage), signalling that investor confidence in the EU's late-stage pipeline remains fragile. Without sufficient public investment to bridge the late-stage capital gap and catalyse private co-investment, the structural investment deficit is projected to persist through 2038.

The **exit asymmetry** is equally stark and is expected to continue. Only 138 EU biotech healthcare companies completed an IPO compared to 451 in the US (over the period 2013-2023). In 2024, no European biotech raised more than EUR 58.1 million in IPO proceeds.

The commercial viability of biotechnology investments is further conditioned by the existing **IP protection framework**. Health biotech innovators reinvest a large portion of their revenue back into R&D. By ensuring a viable commercial timeframe, the SPC is one of the measures that enables innovative companies to re-invest their revenue in additional research and development, improving patient access to innovative therapies, and generating broader economic benefits. However, intellectual property, including SPC, is just one part of innovators' investment decisions. Without policy change, innovators' decisions on investment location continue to reflect expected lifecycle revenues under protection, alongside market size, access conditions, regulatory predictability and operational considerations. In the immediate baseline period to 2030, effective protection durations are expected to remain broadly stable, with the revised general pharmaceutical legislation not expected to produce observable effects within this period. In the longer term to 2038, effective regulatory protection outcomes are expected to become more heterogeneous across medical products, reflecting the continued shift towards biologics and advanced therapies with longer and more variable development timelines. The EU share of earliest biotech patent filings is projected to remain stable at low single-digit levels (around 3-5%), while global biotech patenting activity continues to expand.

This capital deficit compounds a broader **erosion of the EU's global competitive position** across several dimensions that collectively determine where biotechnology innovation is located, financed, and commercialised. The **global clinical trials market** is expected to grow at approximately 5.7% annually, compared with approximately 4.8% in Europe, indicating a gradual but persistent loss of relative competitiveness for the EU as a location for clinical research in the absence of improvements to the clinical trial ecosystem. In

biosimilars, the EU's first-mover advantage has declined. While 71% of biosimilars are approved in the EU before the US, there has been a rising share of biosimilars that get approved in the US first, or only received US approval, which indicates the growth of the US market. At the same time, the European biosimilar medicines sector makes a significant contribution to European gross domestic product, contributing EUR 25.6 billion in 2024 and representing approximately 6% of the total pharmaceutical industry contribution.[160] The number of the CHMP of EMA scientific opinions on biosimilars has itself increased markedly, from 8 in 2023 to 25 in 2024 and 40 in 2025, reflecting a growing pipeline that amplifies the stakes of regulatory competitiveness. **Cumulative biosimilar authorisations** are also projected to rise from 151 in 2025 to 250-350 by 2030 and 450-650 by 2038, with new reference product classes entering biosimilar development expanding from approximately 27 to 35-45 by 2030 and 55-75 by 2038. However, 79% of the approximately 100 biologics losing exclusivity by 2032 have no biosimilars in development, representing a 'biosimilar void' valued at approximately EUR 122 billion in global forecast sales at loss of exclusivity. This indicates that a substantial proportion of the projected market value will not materialise without a reduction in per-product development costs.

The competitive erosion is reinforced by the fact that **no single-entry point** exists for health biotechnology innovators to navigate the regulatory and funding landscape. **Investor-innovator** connections will likely remain *ad hoc* and geographically concentrated in a few leading ecosystems. Disparities in innovation capacity and ecosystem maturity across Member States are expected to persist, with Central and Eastern European Member States lacking the **cluster** foundations that sustain leading ecosystems in some Member States. These fragmentation effects are compounding: without a coordinated ecosystem support mechanism, the navigational burden for SMEs is expected to increase further as new regulatory frameworks layer additional complexity and the pipeline of frontier biotechnologies expands. Meanwhile, other **major jurisdictions** are actively accelerating policy and investment efforts to strengthen their biotechnology ecosystems[161], suggesting that, in the absence of intervention, the EU's relative competitive position is likely to continue to deteriorate over time.

Innovation and research

The baseline reveals a **structural and persistent translation deficit** at the centre of the EU biotechnology innovation system. Under the baseline, the EU innovation system's upstream scientific base will remain internationally competitive, while the downstream deficiency in infrastructure for converting that science into products, trials, and market-ready applications will grow.

One of the key innovation-side deficits is the **absence of structured pathways for technologies that do not fit neatly within existing regulatory categories**. Developers of **novel health biotechnology** products will continue to operate simultaneously across two

[160] Medicines For Europe factsheet: Pillar 1-2-3-4-FOOTPRINT-SPC-Biotech-Act-facsheets-ppt.cdr.
[161] See for example US initiatives which frame biotechnology as a core economic and national security priority requiring coordinated action to sustain global leadership: U.S. House of Representatives. (2025). *Congressional Biotechnology Caucus announcement*, June 2025; National Security Commission on Emerging Biotechnology (NSCEB). (2025). *Report to Congress*, April 2025.

to three regulatory frameworks, with no structured cross-framework anticipatory dialogue in place. Forecast analyses project that up to 30% of such products could be stalled by regulatory uncertainty between 2030 and 2038, even as the volume of novel, hybrid health technologies is expected to increase by approximately 20% between 2025 and 2030. In **ATMPs**, as the current definitions fail to capture emerging modalities, this is projected to widen progressively as in vivo genome editing and cell-device combinations advance over 2030-2038. Without clarification of tissue-engineered product and related definitions, more products are likely to require case-by-case classification by EMA and bespoke scientific advice from 2030 onwards, increasing regulatory workload and amplifying unpredictability for the SME dominated ATMP developer base (over 60% of ATMP developers are SMEs). For **GMMs** used outside food and feed, the EU's process-based framework has resulted in no market authorisations for deliberate release under Directive 2001/18/EC, effectively excluding the EU from the specific parts of global markets in biofertilisation, biocontrol, bioremediation, and bioleaching using GMMs. Taking into account GM and conventional products, these markets have a combined value exceeding EUR 29 billion and growth rates of 9-14% annually. These global GMM-relevant markets are projected to grow significantly under the baseline: biofertilisers from EUR 1.18 billion (2024) to EUR 2.42 billion by 2030; microbial biocontrol from EUR 5.55 billion (2025) to EUR 14.2 billion by 2032; bioremediation from EUR 14.1 billion (2024) to EUR 32.9 billion by 2033; and bioleaching from EUR 8.66 billion (2024) to EUR 18.2 billion by 2033. Under the baseline scenario, the EU's partial exclusion from these markets persists indefinitely, and European companies developing GMM products will increasingly serve non-EU markets or relocate R&D and commercialisation activities to jurisdictions such as the US, where a product-based regulatory approach has already enabled commercial deployment. Similarly, under the baseline, **organ processing practices** would be driven by local institutional arrangements rather than a coherent EU standard. Under the baseline, the share of EU transplantation programmes with active processing capability is projected to rise from an estimated 27% to approximately 35-38% by 2030 and 42-48% by 2035, before plateauing at approximately 48-55% by 2040. This plateau reflects the absence of an EU-level framework.

Regulatory sandboxes, which could provide structured testing environments for such innovations, are either absent or fragmented and underused across the relevant domains. No EU-level sandbox exists for veterinary innovation. Similarly, the EU food acquis does not provide for the operation of regulatory sandboxes; any existing food and feed sandboxes remain weakly connected to food law and unevenly available across Member States. The incoming SoHO risk-based authorisation model does not incorporate a sandbox mechanism under the baseline. In the SoHO domain specifically, expert estimates indicate that sandbox-eligible cases represent 5-10% of overall SoHO activities, translating to 90-250 eligible cases per annum; based on comparable sandbox acceptance rates observed in other regulatory domains (7-16%), between 6 and 40 sandboxes could be established per annum, illustrating both the latent demand for structured innovation pathways and the scale of innovation potential that remains unexploited under the current baseline.

The **innovation investment consequences** of these constraints are illustrated in the biosimilars domain. Under the current framework, the sector’s contribution to European CTs for biosimilar medicine candidates amounts to EUR 4.9 billion. Under the baseline, the near-term biosimilar MAA volume is projected at 25-45 per year, with the lower bound reflecting persistence of the biosimilar void and the upper bound assuming gradual

spontaneous adaptation; the medium-term range rises to 35-55 per year. However, without formalised regulatory streamlining, per-product development costs remain at EUR 90-280 million over 6-9 years, with the CES representing the single largest component (EUR 19-26 million per product, approximately 57% of total clinical development costs being clinical), constraining the commercial viability of biosimilar development for lower-value reference biologics

Public health and safety

Under the baseline, while the EU maintains among the highest regulatory safety standards globally, aspects of the current regulatory architecture - particularly complexity, fragmentation and procedural duplication - can constrain the pace at which patients, consumers, and citizens benefit from biotechnological innovation. This tension will continue to manifest across the following interconnected dimensions:

First, delayed and uneven patient access to biotechnology-derived treatments will continue. The most consequential public health gap in the baseline concerns the widening distance between the availability of advanced biotechnology therapies and the speed at which EU patients can access them. The EU's share of global **clinical trial** initiations has halved from 18% in 2013 to 9% in 2023, with trial authorisation taking 100-120 days compared to approximately 30-60 days in the United States and China. For **ATMPs**, the current provisions of the CTR will continue to add up to 50 additional assessment days. In addition, foresight analyses projecting that up to 30% of **novel health biotechnology products** could be stalled by 2030-2038.

- Under the baseline, annual healthcare system savings from **biosimilars** are projected to rise from approximately EUR 13 billion in 2024 to EUR 16-22 billion per year by 2030 and EUR 22-35 billion per year by 2035-2038, but the upper bounds of these projections are constrained by the biosimilar void, meaning that the full savings potential depends on whether per-product development costs can be reduced sufficiently to make lower-value biologics commercially viable targets for biosimilar competition.
- In **organ transplantation**, the absence of a harmonised EU-level authorisation framework for organ processing means that advanced ex-vivo machine perfusion techniques diffuse unevenly, with no systematic mechanism for capturing processing-related safety data. Under the baseline, with a transplant growth trajectory of 1.54% CAGR annual transplant volumes are projected to rise to approximately 35,350 by 2030, 38,200 by 2035, and 41,200 by 2040. However, demand-side growth will outpace supply-side gains: waiting lists are expected to grow at approximately 0.5% per year to approximately 55,000-56,500 active patients by 2035 and 57,000-58,000 by 2040, meaning waiting-list mortality is projected to decline only marginally from approximately 3,366 deaths per year to approximately 3,100-3,200 by 2035 and 2,900-3,100 by 2040. The EU's dialysis-dependent population is projected to rise from approximately 310,000 to 330,000-340,000 by 2035 and 350,000-365,000 by 2040, with demand-side growth driven by population ageing, rising diabetes and hypertension prevalence, and widening dialysis initiation criteria outpacing the supply-side gains from transplant volume increases. The organ discard rate is projected to decline from approximately 12-13% to 10–11% by 2035 and 9-10.5% by 2040 under organic, centre-led adoption,

but these gains will be concentrated in liver and lung organs in a small number of advanced Member States and will remain below the levels achievable with wider processing adoption.

Second, in the food and feed area, although safety standards will be maintained, innovation will remain constrained. EFSA's rigorous risk assessment framework continues to underpin world-leading food safety standards. In the area of nutrition, biotechnology can enhance nutritional properties. However, the current legislation faces limitations in addressing the scientific aspects given the limited mandate of EFSA to deliver scientific advice in relation to nutrition matters. This may become even more relevant in the future considering the expected increase in the prevalence of diet-related health issues in the coming years. Similarly, regarding the **One Health risks from veterinary regulatory design,** the dual-track system for GMO-containing VMPs generates an 'innovation tax' of 2-3 times longer approval periods without commensurate safety benefit: no GMO-containing VMP has ever received a negative opinion on its GMO component. The absence of a legal 'firewall' clarifying that animals treated with **GMO-derived VMPs** are not themselves classified as GMOs will continue to create commercial risk. It could deter farmer uptake of next-generation biotech vaccines, sustaining reliance on antimicrobials and impeding progress towards the EU's target of halving antimicrobial sales by 2030.

Third, the baseline presents a structural gap in the EU's capacity to detect and prevent biological threats. Wastewater-based surveillance beyond SARS-CoV-2 and influenza remains limited (only 11 Member States monitor antimicrobial resistance, six monitor emerging pathogens), with no agnostic detection layer for novel or engineered threats. Concurrently, synthetic nucleic acid screening remains voluntary and fragmented: only 69 of over 700 global custom synthesis providers are confirmed to screen. The RAND analysis estimates that the expected monetised annual loss due to biological threats across all event categories amounts to approximately EUR 184 billion for large-scale events (1% annual probability; EUR 19.6 trillion expected harm), supplemented by small-scale events (22% annual probability, EUR 72 million expected harm), agricultural events (4% probability, EUR 75.5 billion expected harm), and non-transmissible events (5–25% probability, EUR 121 million expected harm). For attacks specifically, the probability that they involve synthetic nucleic acid ordered from providers ranges from 30% to 70% depending on event type, while for accidents the corresponding probability ranges from 6% to 17%.

The biosecurity and biodefence baselines expose a separate category of public health vulnerability. In the domain of synthetic nucleic acid synthesis, the EU market (estimated at approximately EUR 850 million in 2024, representing approximately 0.2% of the European biotechnology market[162]) is expected to grow at an annual rate of 10-20%, but competitiveness is shaped by the absence of a level playing field: companies that voluntarily screen their customers face competitive disadvantages against providers that do not screen, and as DNA synthesis prices continue to decline, the cost of screening represents an increasing share of the final order price. Without a change in biosecurity

[162] Based on Grandview Research estimates of the DNA synthesis market https://www.grandviewresearch.com/horizon/outlook/dna-synthesis-market/europe and the Biotech market more generally https://www.grandviewresearch.com/horizon/outlook/biotechnology-market/europe

policy, the annual probability of a large-scale biological attack or accident is estimated at 1%, with expected economic harm to the EU of a large-scale event of ca. EUR 20 trillion. The risk is set to increase further as synthetic DNA and RNA continues to become cheaper and more widely available. Meanwhile, pathogen surveillance remains reliant on clinical surveillance networks and targeted wastewater-based monitoring for known agents, with no systematic agnostic detection capacity for novel or engineered biological threats.

Environment and sustainability

The baseline reveals a tension at the intersection of the EU's biotechnology regulatory architecture and its environmental policy ambitions: while these frameworks uphold high environmental and safety standards, certain features – such elements of their process design and limited risk differentiation - can delay or constrain the deployment of biotechnology applications with significant potential to advance the environmental and sustainability objectives. This tension manifests most acutely across three intervention areas.

The most consequential environmental baseline deficit concerns **GMMs**. No GMM has been authorised for deliberate environmental release under Directive 2001/18/EC. As innovations utilising genetic engineering are not brought to the market in the EU, the EU is therefore entirely absent from this important segment of rapidly growing global markets in biofertilisation (EUR 1.18 billion in 2024, growing at 12.8% CAGR), microbial biocontrol (EUR 5.55 billion in 2025, 14.3% CAGR), bioremediation (EUR 14 billion in 2024, 9.9% CAGR), and bioleaching (EUR 8.65 billion in 2024, 8.9% CAGR), sectors with a combined global market exceeding EUR 29 billion and projected growth rates of 9-14% annually. In contrast, a product-based regulatory approach in the US has already enabled commercial deployment. Under the baseline scenario, this partial market exclusion persists indefinitely, and European companies developing GMM products will increasingly serve non-EU markets or relocate, while the environmental benefits of GMM-based microbial solutions for resource-efficient agriculture, pollution remediation, and sustainable extraction remain unrealised within the EU.

In the **veterinary domain**, the dual-track regulatory system for GMO-containing VMPs results in a regulatory friction that can slow the uptake of next-generation biotech vaccines in food-producing species, with potential ramifications for animal health, farmers and food security.

The **food chain sandbox landscape** is characterised by fragmentation and lack of coherence with Union law; only a few Member States operate sandbox-type mechanisms, none anchored in a harmonised EU framework under the General Food Law. As a result, findings and best practices cannot be systematically integrated into the evolution of the existing legal framework. This limits structured, pre-market testing of sustainable food production technologies, leaving innovation potential for the food system's environmental transition partly unexploited.

4 APPROACH TO THE BIOTECH ACT

4.1 Objectives of the proposal

4.1.1 General objectives

The **general objective** of the proposed Regulation and Directive is threefold: (i) to improve the functioning of the internal market by establishing a framework to strengthen the competitiveness of the health biotechnology sector, from research to production, (ii) to create the conditions for the development and timely placing on the single market, of biotechnology innovations, products and services, (iii) while safeguarding high standards for the protection of human health, animal health, patients and consumers, food and feed safety, the environment, and biosecurity.

4.1.2 Specific objectives

This general objective translates into the following **specific objectives,** as presented in the proposed Regulation and Directive: (i) strengthen the biotechnology sector and reinforce the EU's research, development and production capabilities; (ii) support funding of, investments in, and access to capital for, biotechnology companies and projects; (iii) improve the EU manufacturing capacity of, and expertise in biosimilars, including through international cooperation; (iv) facilitate the application of AI into the EU's biotechnology and health technology manufacturing ecosystems and frameworks, in line with the AI Act[163]; (v) ensure a legislative framework that encourages innovation and takes account of technological and scientific developments and progress; (vi) prevent the misuse of biotechnologies and strengthen biodefence capabilities and (vii) enable the effectiveness of the above specific objectives through a legislative framework conducive to the use of biotechnology innovations.

4.2 Choice of the legal instruments and legal basis

4.2.1 Choice of the legal instruments

A **Regulation** is the most suitable for measures requiring uniform application across the EU, such as those concerning recognition and support for heath biotechnology strategic projects and high impact health biotechnology strategic projects, and measures to boost EU biodefence and biosecurity and prevent biotechnology misuse. The Regulation is also justified to amend existing EU regulations in the area of health and food law. Its directly applicable nature ensures consistent implementation without national transposition. A **Directive** is the appropriate instrument for amendments to Directive 2001/18/EC and Directive 2010/53/EU.

4.2.2 Legal basis

The appropriate legal bases are therefore as follows:

- **Article 114 TFEU** is the basis for the adoption of measures that increase harmonisation and remove fragmentation and, therefore, seek to create a level playing

[163] Regulation (EU) 2024/1689, OJ L, 2024/1689, 12.7.2024, ELI: http://data.europa.eu/eli/reg/2024/1689/oj.

field within, and fully exploit the scale of the single market for biotechnology products and biomanufacturing. The proposal also seeks to achieve the objective of a high level of health and safety protection as per **Article 114(3)**.

– **Article 168(4) TFEU** is the basis for the adoption of certain measures that contribute to achieve a high level of human health protection, i.e. standards of quality and safety of organs and SoHO, blood and blood derivatives; measures in the veterinary and phytosanitary fields, and standards of quality and safety for medicinal products and devices for medical use.

- **Article 173(3) TFEU** is the basis for measures in support of Member States' action for the competitiveness of the EU's industry, in particular the provisions regarding the EU Health Biotechnology Investment Pilot.

4.3 Subsidiarity: necessity for EU action and EU added value

Under the subsidiarity principle, the Union may act in areas of non-exclusive competence only where the objectives of the proposed action cannot be sufficiently achieved by the Member States.

4.3.1 Necessity for EU action

The objectives of the proposed Biotech Act cannot be achieved by Member States acting alone, as the identified drivers are cross-border and systemic, affecting the functioning of the single market and the global competitiveness of EU companies. These challenges may differ in intensity across Member States and regions, but their nature is Union-wide. Access to finance is fragmented and EU biotechnology companies lack the capacity to access private finance at a competitive scale, including at later stages of development. European biotechnology clusters are dispersed and lack continental scale to compete globally. The development and deployment of AI in biotechnology remains limited, also due to the insufficient cross-border availability and sharing of relevant data.

While several Member States have taken action, these bottlenecks persist and improvements would remain slow and uneven. National measures alone cannot provide the scale, coordination and speed required, although in certain areas (e.g. access to finance, development of regional innovation ecosystems and clusters, and support to research infrastructures, etc.), Member States may still take complementary action alongside Union measures.

Important regulatory barriers stem from existing EU legislation, such as lack of legal clarity, complex or outdated rules, disproportionate administrative burden and additional national requirements. As these drivers originate in Union law, they cannot be effectively addressed by Member States acting individually. Union action is therefore necessary to facilitate innovation, enhance legal clarity and improve market access.

4.3.2 EU added value

The proposed actions focus on areas where there is a demonstrable value added in acting at EU-level due to the scale, speed and scope of the efforts needed.

A harmonised but simplified EU regulatory framework, supported by strengthened collaboration in selected policy areas (access to capital, AI and data) is expected to ensure patients, users and citizens across the EU can benefit from biotechnology innovations.

A large market with a streamlined, fit-for-purpose regulatory framework ensures a level playing field and reduces compliance costs in the single market, thereby supporting biotechnology market uptake. Coordinated EU action in the field of biotechnologies will generate economies of scale, reduce duplication of efforts, increase legal certainty for cross border entrepreneurs, and unlock investments, infrastructures and skills development that Member States alone could not achieve. Union-level coordinated action is expected to yield higher benefits than fragmented national measures.

Regulatory streamlining and clarification measures will reduce administrative burden, accelerate time-to-market and improve the functioning of the internal market, also by addressing the observed diverging national interpretations and additional requirements. The proposed Biotech Act also seeks to reinforce the EU's strategic autonomy in a critical technological area while fostering adequate biosecurity safeguards and biodefence capabilities.

4.4 Intervention logic of the proposal

Figure 4.[164]

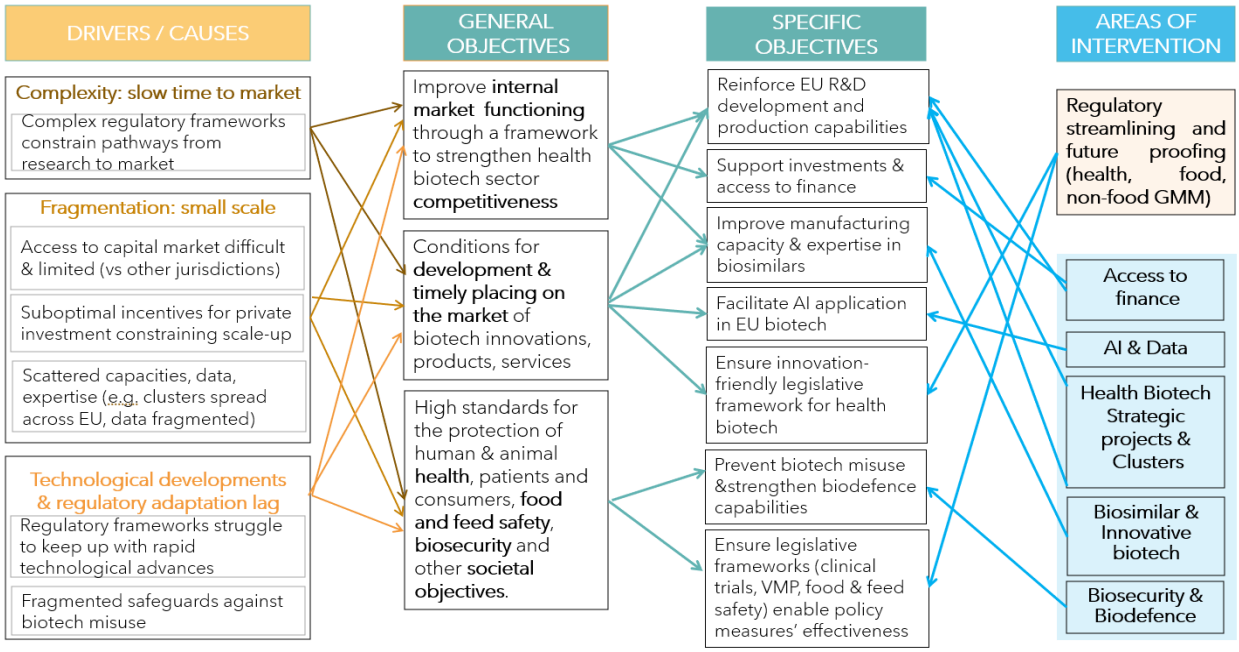
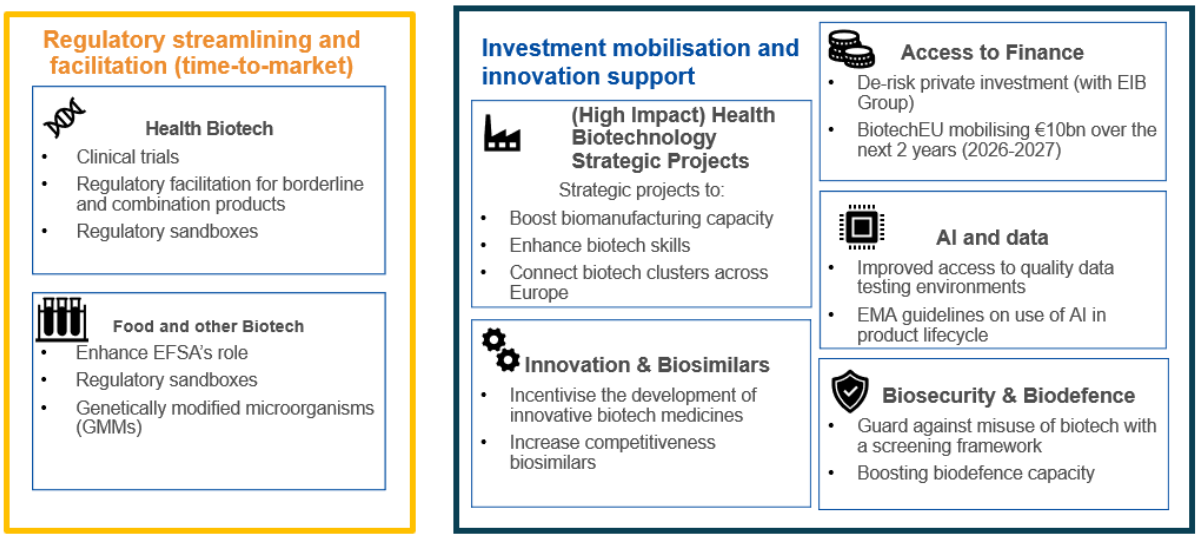


Figure 5 illustrates the overall architecture of the Biotech Act, highlighting the two complementary pillars of regulatory streamlining and facilitation to accelerate time-to-market and industrial enablers to strengthen the EU biotechnology ecosystem.

[164] Revised version of the intervention logic (see first version in COM(2025) 1022 finalhttps://eur-lex.europa.eu/legal-content/EN/TXT/?uri=CELEX:52025PC1022).

Figure 5. Biotech Act architecture



5 DESCRIPTION OF THE PROPOSED MEASURES AND ANALYSIS OF THEIR MAIN IMPACTS

The measures described below are outlined in the proposal for a Regulation and the proposal for a Directive[165]. They are presented in 17 packages of interventions, which fall under two categories: eight interventions related to regulatory streamlining and facilitation measures aiming at regulatory simplification (addressing drivers 1 and 3), and nine interventions linked to industrial enablers (addressing drivers 2 and 3). They are presented in more detail in Annex 7.

The summary assessment of measures[166] below includes tables capturing the key impacts across standard impact categories[167]. Where sufficiently robust evidence is available, key impacts are quantified and expressed using numerical estimates. Where quantification is not feasible, impacts are assessed qualitatively using a standardised rating scale[168], indicating the direction and relative magnitude of effects. For certain measures, tables are not used where the nature of the impacts or the available metrics are better suited to alternative forms of presentation (e.g. narrative analysis or visual representations), to ensure a more accurate and meaningful representation of the expected effects.

5.1 Interventions for regulatory simplification: measures and expected impacts

Chapter IX of the proposed Regulation and Directive include amendments to EU legislative frameworks in the areas of health, food and feed safety as well as GMMs, which aim to simplify existing procedures and accelerate time-to-market. These amendments are

[165] Annex 6 presents a mapping of the policy measures and the corresponding articles in the proposed Regulation and proposed Directive respectively.
[166] The assessment of impacts draws on the supporting studies presented in Annex 1.
[167] COB = conduct of business; Admin = administrative costs on businesses, including SMEs; CTI = competitiveness, trade and investment flows; Int Mar = functioning of the internal market and competition; I&R = innovation and research; PA = public authorities; H&S = public health and safety.
[168] ++ significant positive; + moderate positive; 0/+ marginal positive; 0 negligible/neutral; 0/– marginal negative; – moderate negative, -- significant negative.

needed to ensure the effectiveness of the self-standing measures on industrial enablers laid down in the proposed Regulation.

5.1.1 Intervention n°1: Regulatory facilitation for novel health biotechnology products

The Regulation proposal sets out the provision of **regulatory assistance or support by the EU Health Biotechnology Support Network** to developers. This targeted, early-stage support will be particularly valuable for SMEs, which often lack extensive in-house regulatory expertise. The support will consist of facilitating information on, and access to, applicable legislative frameworks, including procedures for seeking guidance on regulatory status, rules applicable to products combining different technologies or regulatory frameworks, and access to regulatory sandboxes.

Further, the Regulation proposal establishes a **publicly available regulatory status repository** containing decisions, opinions and scientific recommendations regarding the regulatory status of health innovations across the EU. This repository aims to ensure transparency and consistency in regulatory determinations. It also aims to support advisory bodies mandated under sectoral legislation to determine the regulatory status of health products promptly and without undue delay, as well as to assist developers in shaping their regulatory strategy based on existing practice.

In addition, the proposal establishes a **foresight panel for emerging health innovation** comprising scientific and regulatory experts from the SoHO Coordination Board ('SCB'), the Medical Devices Coordination Group (MDCG), the Coordination group on Health Technology Assessment, the EMA, and the competent authorities of the Member States. The panel would provide early recommendations to the Commission, relevant EU agencies, and Member State competent authorities.

Moreover, a **cross-framework coordination for regulatory sandboxes in the health area** aims to allow for dialogue and information exchange among authorities operating regulatory sandboxes for health biotechnology products across different Union legislative frameworks (MDR, IVDR, SoHO Regulation, Pharmaceutical Regulation). Member State and Union-level authorities operating sandboxes under different frameworks would have to consult with each other and with the foresight panel regarding sandbox design and implementation, ensuring the potential integration of cross-framework aspects, which is needed given the increasing cross-cuting nature of health product innovations. The Commission, the EMA, the MDCG and the SCB facilitate cross-framework exchanges through the above-mentioned foresight panel, focusing on knowledge sharing (regulatory approaches, technological challenges, best practices) and regulatory learning (identifying implications for future legislative adaptations).

Finally, the proposal includes the possibility for **regulatory sandboxes for novel health biotechnology products**. These Commission-led sandboxes are designed for products that, due to their very early stage of development cannot be accommodated in existing sectoral sandboxes for novel products and whose development is hindered by challenges in identifying suitable regulatory procedures. They offer a framework – in the form of a subsidiary sandbox – for continued engagement with regulators and eventually a recommendation on the applicable existing framework for marketing authorisation. Upon

substantiated request from developers, the Commission would assess applications and establish such a regulatory sandboxes through implementing act. These sandboxes would operate under time-limited sandbox plans specifying objectives, scope, participants, risk mitigation measures, and supervision arrangements. When assessing the applications for the establishment of such a subsidiary regulatory sandbox, and when developing and implementing the sandbox plan, the Commission may consult the EMA, the SCB, the MDCG and the Foresight Panel. Upon termination of the regulatory sandbox, the Commission would, upon a request from a developer, provide a recommendation on an existing appropriate regulatory procedural pathway for authorising the placing on the market and post-market surveillance and vigilance of the products concerned under an existing framework and may publish reports on lessons learned from the regulatory sandboxes.

Expected impacts:

It is crucial for research, business, investment and patients that the EU regulatory system can efficiently and adequately assess highly innovative health biotechnology products. With the above proposed measures, the Regulation proposal aims to introduce a comprehensive set of regulatory tools designed to make the EU regulatory system more predictable, efficient, and innovation-friendly without compromising safety requirements.

Conduct of business: These tools are expected to generate positive effects, as the repository would provide guiding documented regulatory interpretations for similar products, enabling firms to benchmark approaches. Navigation assistance by the Support Network will deliver targeted, early-stage support particularly valuable for SMEs, which often lack extensive in-house regulatory expertise and often rely on costly external consultancy. The foresight panel for emerging innovation would increase the regulatory readiness of the legislative frameworks in the health area by allowing them to integrate approaches to new technologies in a timely way. The panel, offering a forum for structured dialogue across the frameworks, also aims to ensure a cross-framework perspective on emerging technologies, which is needed as innovation increasingly develops across the silos of the different frameworks. This would make the existing frameworks more conducive to processing products based on new biotechnologies.

Regulatory sandboxes offer a controlled environment where developers of truly disruptive innovations are supported by regulators to identify suitable authorisation routes. Evidence from analogous sandboxes already applicable in other sectors, notably the UK Financial Conduct Authority study[169], suggests that **sandbox participation can reduce time-to-market by 20-40%, increase funding probability by 15-50%, and improve firm survival and patenting by 20-30%, with benefit-to-cost ratios ranging from 3/1- 10/1 over three to five years**.

The measures are also expected to have a positive impact on **administrative costs for businesses (including SMEs)**, through reducing administrative burden and costs by eliminating duplicative work and regulatory unpredictability for businesses and

[169] "Regulatory Sandboxes and Fintech Funding: Evidence from the UK" by G. Cornelli S. Doerr L. Gambacorta O. Merrouche (2020) act 2025: FCA Strategy.

innovators. The measures aim to avoid undue delays and inefficiencies in regulatory pathways at an early stage of development.

R&I: The measures are also expected to generate positive impacts. Clearer pathways for researchers would reduce the chilling effect that uncertainty currently has on frontier research, encouraging more high-risk, high-novelty projects being developed and retained in Europe. The repository and regulatory assistance would help research institutions and firms plan development activities with confidence, while the sandboxes would ensure that truly disruptive research in novel products is still accommodated by the regulatory system. The foresight panel aims to connect the research world, where innovation emerges, with regulators. This would allow to reflect collaboratively on how to process products based on new technologies in the best way. These measures combined would guide funding bodies and researchers toward promising areas likely to be facilitated in a system conducive to innovation, eventually unlocking the potential for increasing EU gross domestic expenditure on research and development and better translating scientific breakthroughs into clinical applications.

Competitiveness, trade and investment: The Biotech Act as a whole is expected to address structural weaknesses that currently deter investment and early-stage scaling in Europe. Greater regulatory clarity and predictability, through the repository and navigation assistance, should reduce perceived regulatory risk, making the EU more attractive for biotech investment. The sandbox might signal to global investors that tailored regulatory tools exist for disruptive innovations, providing viable pathways to market and reducing uncertainty. The foresight panel demonstrates that the EU actively incorporates emerging trends into regulatory planning, aligning long-term policy with cutting-edge innovation and supporting confidence that governance and rules will adapt to technological progress. These measures have the potential to reverse declining trends in EU biotech and partially close the investment and commercialisation gap with the US and China.

Functioning of the internal market: Its functioning is expected to improve through greater harmonisation and transparency, as the repository promotes convergence by making consistent regulatory interpretations visible across Member States and reducing divergent classifications that fragment the market and distort competition. Navigation assistance and structured guidance minimise interpretive disputes and streamline cross-border compliance. For public authorities, the Biotech Act is designed to improve efficiency and preparedness by reducing duplication through the shared repository reference base, streamlining pre-authorisation interactions through navigation assistance, and building anticipatory capacity through the sandbox and foresight panel. Although establishing the sandbox and operating the foresight panel require upfront investment, these costs are limited given the expected low number of such sandboxes, borne at EU-level with no additional burden on Member States, and are expected to be offset by efficiency gains, reduced uncertainty, and scale effects. For public health and safety, the combined regulatory tools aim to offer a more innovation-conducive regulatory system, bringing more breakthrough products with potential major patient benefit to the market through a more efficient and adapted regulatory process – without compromising safety standards.

Public authorities: The proposed measures will entail only limited costs, while delivering long-term efficiency gains, particularly through streamlined authorisation processes that reduce administrative workload. Regulatory assistance via the support network will leverage existing national structures, adopting an antenna approach to better coordinate and deploy assistance providers at the national level. The regulatory status repository, though requiring initial development, will build upon existing or planned resources—such as the SoHO compendium or the Medical Devices Manual on Borderline and Classification—or simply reference national publications, thereby minimising duplication and resubmissions by developers. This, in turn, will further alleviate the burden on public authorities by reducing uncertainty and improving procedural efficiency. The foresight panel, while incurring marginal costs for logistical support, staff allocation, and expert compensation, is designed to strengthen anticipatory governance, ensuring greater preparedness for emerging technological shifts and ultimately lowering the future regulatory burden for complex, innovative products. Though no data exists on the cost-benefit ratio of a regulatory sandbox for novel health biotech products, its use will be highly targeted, keeping expenses minimal. Importantly, investments to establish the sandbox and operate the foresight panel will be fully funded by the Commission, imposing no additional financial obligations on Member States.

On **public health and safety**, no impacts are expected as safety standards remain untouched by these provisions.

Table 1. Summary assessment of the effects due to the intervention n°1

Policy measure	COB	Admin	CTI	Int mar	I&R	PA	H&S
Union regulatory status repository	+	+	+	+	+	n/a	0
Foresight Panel for Emerging Health Innovation	+	0	+	+	+	n/a	0
Regulatory sandboxes	+	+	+	+	+	n/a	0

5.1.2 Intervention n°2: Targeted regulatory reform of the General Food Law[170]

First, the Regulation proposal includes measures to accelerate and improve EFSA risk assessment processes for products subject to pre-market authorisation under Union food and feed law. These include:

- the **broadening of the scope of general pre-submission advice** (currently limited to administrative and regulatory requirements) to cover scientific matters, such as study design and testing strategies, while merging it with the renewal-related advice into a single, unified and simplified procedure;

[170] Regulation (EC) No 178/2002, OJ L 31, 1.2.2002, pp. 1–24. ELI: http://data.europa.eu/eli/reg/2002/178/oj.

- a shortening to **three months** of the **procedural delay for non-compliance with the study notification requirements at pre-submission phase**;
- a targeted and limited **revision of the Scientific Committee/Scientific Panel governance** while maintaining the composition of the Scientific Committee;
- the **expansion of EFSA mandate** to provide scientific advice on all aspects of human nutrition.

Secondly, the proposal establishes a **comprehensive regulatory sandboxes framework within Union food law**, enabling Member States to create controlled environments for testing innovative technologies, products and substances at pre-market stage, data requirements and alternative regulatory requirements for food production, processing and distribution, feed produced for food-producing animals, food contact materials, and GMO-containing products. The proposal, however, **does not allow** the establishment **of regulatory sandboxes** for the following categories of products: **(a) Novel foods:** Experience has shown that certain types of novel foods involve highly innovative processes and complex scientific considerations, which require thorough oversight even at testing/experimental phase. This makes such foods less suited to the flexible, experimental nature of sandboxes. Furthermore, experience has shown that certain types of novel foods trigger ethical or cultural concerns among various consumer segments regarding their acceptability. Therefore, all aspects of novel foods in general are best addressed solely within the applicable rigorous regulatory framework established by Regulation (EU) 2015/2283 of the European Parliament and of the Council and should not be subject to regulatory sandboxes[171]; **(b) Recycled plastic food contact materials:** As regards innovations concerning novel plastic recycling technologies for plastics intended to come into contact with food, chapter IV of Commission Regulation (EU) 2022/1616 already establishes a framework that is meant to encourage the development of such novel technologies without prior authorisation. To ensure uniform rules on the development of novel recycling technologies that safeguard the health of the consumers, it is appropriate to exclude the development of recycling technologies from the possible use of regulatory sandboxes and rely instead on the procedure established in chapter IV of Regulation (EU) 2022/1616;[172] and, **(c) GMOs regulated under Part B of Directive 2001/18/EC:** For certain GMOs legal pathways already exist to allow testing of innovations, such as under Part B of Directive 2001/18/EC on the deliberate release of genetically modified organisms (GMOs) for purposes other than placing on the market, and therefore there should not be a duplication of paths in the interest of legal certainty. For this reason, regulatory sandboxes should be restricted to products containing or consisting of GMOs subject to authorisation under Part C of Directive 2001/18/EC.

[171] Regulation (EU) 2015/2283 of the European Parliament and of the Council of 25 November 2015 on novel foods, amending Regulation (EU) No 1169/2011 of the European Parliament and of the Council and repealing Regulation (EC) No 258/97 of the European Parliament and of the Council and Commission Regulation (EC) No 1852/2001 (OJ L 327, 11.12.2015, p. 1, ELI: http://data.europa.eu/eli/reg/2015/2283/oj).

[172] Commission Regulation (EU) 2022/1616 of 15 September 2022 on recycled plastic materials and articles intended to come into contact with foods, and repealing Regulation (EC) No 282/2008 (OJ L 243, 20.9.2022, p. 3, ELI: http://data.europa.eu/eli/reg/2022/1616/oj).

Regulatory sandboxes established in the context of Regulation (EC) No 178/2002 would not replace or circumvent existing pre-market authorisation procedures, as any product subject to pre-market authorisation – for example a new feed additive tested within a sandbox – would still need to undergo the full authorisation process before being placed on the EU market.

Expected impacts:

Conduct of business: The proposed extension of the scope of EFSA's general pre-submission advice to cover also scientific aspects is expected to significantly improve application dossier quality, reduce validation timelines and limit 'stop-the-clocks' during the risk assessment phase, thereby contributing to shorten time-to-market. By enabling applicants, especially SMEs and first-time applicants, to clarify study design, endpoints and methodologies in advance, with EFSA staff and external experts, the measures should increase predictability without undermining the 'non-committal' character of the pre-submission advice and without altering the applicant's burden of proof or EFSA's independence. Shortening the procedural delay for non-compliance with study notification obligations from six to three months further reduces procedural delays at pre-submission phase. In parallel, the introduction of a framework for regulatory sandboxes allows companies to test innovative technologies, products and processes in controlled environments before pre-market authorisation procedures, helping them to understand data requirements and regulatory expectations while maintaining oversight and safety. Overall, the conduct of business along the food chain is expected to become more innovation-friendly and less encumbered by avoidable procedural obstacles.

Administrative costs on businesses (including SMEs): the reform is expected to reduce unnecessary burdens linked to incomplete or poor-quality application dossiers. The enlarged scope of pre-submission advice should help applicants tailor their data generation more precisely to EFSA's requirements, avoiding mis-designed studies and decreasing the likelihood of repeated information requests and related delays. This is particularly important for SMEs and one-time applicants, for whom administrative and compliance costs weigh more heavily and who currently underuse existing advice instruments. By clarifying notification obligations and other procedural requirements at an early stage, the reform can streamline compliance and reduce the cumulative administrative effort over the life cycle of an application.

Competitiveness, trade and investment flows: The targeted amendments are expected to strengthen the EU's competitive position by making the regulatory environment more conducive to innovation while preserving high safety standards. Faster and more predictable risk assessment processes, fewer unnecessary administrative hurdles and reduced non-compliance penalties should lower the cost of bringing new products to market and make the EU a more attractive location for R&D and investment. By facilitating the development and approval of innovative products and related technologies, the reform supports the ability of EU firms to compete globally, leveraging the existing reputation of EU standards as a quality benchmark and reinforcing the EU's capacity to shape international norms.

Functioning of the internal market and competition: the reform is not expected to materially alter the baseline. The measures would apply horizontally and uniformly to all

operators and Member States, and do not change the core harmonised framework established by the General Food Law and sectoral legislation. The level playing field across the internal market, underpinned by common risk-analysis principles and centralised EFSA risk assessment, would therefore remain intact.

Innovation and research: The new legislative framework for regulatory sandboxes is expected to be potentially transformative. By allowing supervised experimentation with technologies, products and substances in real-world conditions before pre-market authorisation, sandboxes significantly reduce both technological and regulatory risks for innovators, particularly start-ups and SMEs. Shorter learning cycles, direct feedback on safety, usability and acceptance, and clearer insight into data and evidentiary expectations should accelerate development and derisk investment in more ambitious, less incremental innovations. Participation in regulatory sandboxes established by Member States in accordance with the proposed amendment of the General Good Law, with visible regulatory engagement, is also likely to signal credibility to investors and industrial partners, facilitating access to finance and strategic collaborations. Overall, the measures are expected to improve the innovation climate in the EU food and feed sector, while keeping full pre-market authorisation requirements in place for products entering the market.

Public authorities: Concerning public authorities, notably national competent bodies, as well as EFSA, the reform is expected to improve the efficiency and the quality of scientific and regulatory outputs. Enhanced pre-submission advice, including scientific aspects and delivered by EFSA staff and external experts who may also support subsequent risk assessment, should improve dossier quality, reduce the frequency and duration of stop-the-clock procedures, and allow better allocation of EFSA's resources along the application process. Adjustments to EFSA's scientific panel governance – having EFSA staff chair panels and the Scientific Committee – are expected to reinforce coherence, methodological consistency and timeliness across assessments. While the expanded advice function may require some additional staff and coordination efforts, these are expected to be offset by savings from fewer procedural delays and less remedial work on weak-quality dossiers. For national authorities, participation in regulatory sandboxes offers a vehicle for systematic regulatory learning, enabling them to test data requirements and alternative regulatory approaches in a controlled setting and to feed robust evidence into EU-level scientific guidance and regulatory standards.

Public health and safety: the targeted amendments are designed to preserve, and potentially enhance in the longer term, the high level of safety standards currently afforded by EU food law. The streamlining of EFSA procedures and the extension of pre-submission advice aim to ensure that risk assessments are supplied with more relevant, better structured data, thereby supporting robust scientific opinions within shorter timelines. The expanded mandate of EFSA on nutrition matters, enabling EFSA to provide advice on all nutrition matters aim at further equip EU regulators with the necessary scientific knowledge to address challenges relating with the increasing prevalence of diet-related health issues. The regulatory sandbox framework, under strict safeguards, is expected to function as a regulatory learning tool, generating richer real-world evidence on exposures, hazards, patterns of use and the effectiveness of risk-management options. This should enable more accurate risk characterisation, more proportionate and effective risk-management measures, and earlier detection of potential safety issues in a controlled

environment. Provided that capacity is available to analyse and use sandbox generated data, the overall effect is expected to be at least the maintenance, and potentially the strengthening, of the EU's high standards of public, animal and plant health protection and environmental safety.

Table 2. Summary assessment of the effects due to the intervention n°2

Policy measure	COB	Admin	CTI	Int Mar	I&R	PA	H&S
Improved timeliness and coherence of EFSA risk assessment processes	++	++	++	**0**	**0/+**	++	+
Regulatory framework for sandboxes in relation to food, feed, and GMOs	++	**0**	+	**0**	++	+	+

5.1.3 Intervention n°3: Targeted regulatory reform of the Advanced Therapy Medicinal Products (ATMPs) framework[173]

The proposed regulatory simplification and future-proofing of ATMPs includes two interconnected changes. First, it establishes a **risk-proportionate approach for clinical trials involving ATMPs containing or consisting of GMOs**. Under this approach sponsors would be exempted from submitting environmental risk assessments (ERAs) for products presenting no or negligible risks. Second, it aims at **future-proofing the regulatory framework** by amending the definition of tissue-engineered products to reflect technical and scientific advances, without extending the scope of the definition.

Expected impacts:

Overall, the reform is expected to strengthen the EU's attractiveness for high-risk biotech investment and support the growth of a rapidly expanding ATMP sector across Member States. The proposed amendments are expected to contribute to the EU's global competitiveness in biotechnology. Predominantly positive impacts are expected for businesses (in particular SMEs), research and innovation, the internal market, and public health.

Conduct of businesses, innovation and research: The introduction of a targeted exemption from ERA requirements **during the CT application phase** for clearly defined categories of low-risk investigational ATMPs directly addresses a key barrier to conducting clinical trials in Europe. Even where the recently agreed pharmaceutical legislation reform addresses the fragmentation of ERA requirements across Member States by centralising/harmonising the ERA procedure, requiring sponsors to obtain ERA approval prior to, or alongside, a CT authorisation, imposes a disproportionate regulatory

[173] Regulation (EC) No 1394/2007, OJ L 324, 10.12.2007, pp. 121–137. ELI: http://data.europa.eu/eli/reg/2007/1394/oj.

burden at an early stage of development where the risk profile of the product is already well-characterised and low. This deters sponsors –particularly SMEs – from choosing the EU as a trial location, diverting early-stage innovation to jurisdictions with more streamlined processes. This competitive disadvantage is evident when comparing with the US: the Food and Drug Administration grants an exclusion from ERA for the vast majority of gene therapy Investigational New Drud (IND) applications at the clinical trial stage. That regulatory efficiency accelerates development timelines and explains why a significant share of ATMP clinical trials are conducted in the US rather than in the EU. While an ERA will still be required at the marketing authorisation stage, removing this requirement during clinical development removes a critical friction point. This provision is expected to attract clinical research on certain categories of ATMP in the EU even if it is not possible to quantify the precise impact at this stage.

An empowerment which would allow the Commission to amend the ATMP Regulation via delegated acts, in order to amend the definition of tissue engineered products will future-proof the ATMP framework by allowing to take into consideration **technical and scientific advancements** in the field of ATMPs. The need for such adaptations is underscored by concrete cases. For example, an acellular tissue-engineered vascular graft intended for urgent revascularisation in severe arterial injury has faced 18 years of classification uncertainty in the EU. Its acellular nature excluded it from the existing tissue-engineered product (TEP) definition. Meanwhile, the FDA approved the same product, demonstrating how regulatory rigidity can drive innovation away from Europe.

Both measures are expected to **lower operational costs** by **removing non-value-adding regulatory steps**, potentially removing a burden of 0.15-0.3 FTE-years per clinical trial application, and **reducing uncertainty.** Together, they **speed the transition** from research to clinical development, particularly for SMEs, while making the EU a more competitive and predictable environment for ATMP innovation.

Functioning of the internal market and competitiveness: The impact of streamlining Regulation (EU) No 536/2014, in conjunction with modifications to ERA requirements for investigational GMO-ATMPs and amendments to ATMP definitions, is expected to be profound and multifaceted concerning internal market dynamics and EU competitiveness. By exempting certain investigational ATMPs containing GMOs from full ERA submissions, this change not only facilitates faster progression from trial initiation to product development, but also significantly influences sponsors' decisions to conduct clinical trials in Europe. Consequently, the EU could emerge as a leading destination for biotech investment, accelerating the development of innovative therapies. Future-proofing ATMP definitions allows EU to enhance its scientific and technological leadership in biotechnology and regenerative medicine, while further cementing its reputation as a regulator pioneer. The reform is also expected to attract more high-risk capital, support the growth of more than 580 ATMP companies across over 20 Member States, and help maintain EU competitiveness.

Public health and safety: The Biotech Act targets procedural simplification rather than substantive relaxation of safety standards. The ERA exemptions only apply to ATMPs which fall into clearly defined categories with no or negligible risks. **Safety oversight** is maintained through the review of sponsor declarations within the clinical trial assessment process, comprehensive risk–benefit evaluation and long-term follow-up requirements.

By reducing unnecessary procedural delays and shortening assessment timelines, the measures are expected to facilitate **earlier initiation of CTs** and potentially earlier **access** for EU patients to safe, effective and often transformative advanced therapies.

Furthermore, the ability to adapt ATMP definitions to scientific progress helps reduce the risk of regulatory blind spots for **emerging technologies**, ensuring that innovative therapies remain subject to the most appropriate regulatory oversight.

Table 3. Summary assessment of the effects due to the intervention n°3

Policy measure	COB	Admin	CTI	Int mar	I&R	PA	H&S
1. targeted ERA exemption	+	0	+	+	+	0	0
2. revised definition	+	+	+	+	+	0	0

5.1.4 Intervention n°4: Targeted regulatory reform of clinical trials[174]

The amendments to the CTR aim to shorten authorisation timelines, strengthen collaboration between Member States, and enhance regulatory efficiency without compromising safety, quality, or ethical standards.

The proposed amendments articulate around five pillars:

(i) Faster authorisation procedures

Authorisation timelines for initial clinical trial applications are reduced from 106 to 75 days, or from 75 to 47 days, where no additional information is requested from the sponsor. For substantial modifications[175], timelines are reduced from 96 to 47 days or from 64 to 33 days where there is no additional request for information. The additional 50-day assessment period for ATMPs is removed.

(ii) More predictable and efficient authorisation procedures

The shorter authorisation timelines are supported by procedural changes to increase efficiency, cooperation, and consistency across Member States. The reporting Member State (RMS) leads the evaluation of Part I[176] of the trial application, with other Member

[174] Regulation (EU) No 536/2014, OJ L 158, 27.5.2014, pp. 1–76. ELI: http://data.europa.eu/eli/reg/2014/536/oj.
[175] Substantial modifications as defined in the proposed amendments to Regulation (EU) No.536/2014, Art. 2 mean '*any change to any aspect of the clinical trial which is made after the notification of a decision referred to in Article 8 in at least one Member State concerned and which is likely to have a substantial impact on the safety or rights of the subject or on the reliability and robustness of data generated in the clinical trial*.'
[176] The application dossier for the authorisation of a clinical trial in the EU is split into Part I and Part II. Part I contains overarching scientific, medical, and technical information about the investigational medicinal product (IMP), the clinical trial design and participants' safety. The RMS leads the assessment of Part I. Part II contains the national, ethical, and national information (e.g., informed consent, recruitment related information, site and investigator suitability, insurance) assessed separately by each Member State concerned.

States concerned providing complementary considerations. For initial applications, the validation of Part I will be carried out solely by the RMS without coordination and consolidation steps with the other Member States concerned. It is also allowing the RMS to initiate the assessment already during the validation phase, shortening the overall assessment time by two weeks. The ethics committee of the RMS will be mandatorily engaged in the review of Part I to ensure that the ethical review is fully integrated with scientific and regulatory assessments. The scope of the ethical review and the mandate of the Member States concerned in the assessment are clarified. Maximum timelines are designed to allocate seven day-periods per assessment step, ensuring deadlines do not fall on weekends and thereby avoiding automatic extensions. This approach ensures predictability in timelines for sponsors and regulatory bodies. Substantial modifications to Part I and/or Part II for changes that may significantly affect participants' rights, safety, or data reliability can be assessed in parallel, with significantly shorter assessment times.

Efficiency is further improved through the establishment and re-use of a single investigational product core dossier containing documents common to all trials with the same investigational medicinal product (IMP), a mandatory use of harmonised EU templates, and by shifting translation checks from Part I to Part II. An upgraded EU portal enhances communication between sponsors and assessing Member States during assessments.

The amendments also encourage participation of vulnerable populations in clinical trials, by carefully weighing the potential risks and benefits of their exclusion versus inclusion in trials. Additionally, women who become pregnant or start breast-feeding during their trial participation will not be automatically excluded from the trial. Their continued participation will instead be evaluated by assessing the risks and benefits involved.

(iii) Context-specific clinical trial authorisation procedures

The Regulation proposal sets out three tailored authorisation procedures to streamline regulatory requirements to the specific context and risks of the clinical trial.

1. It introduces a single assessment procedure for combined studies involving both medicines and medical devices or medicines and *in vitro* diagnostic medical devices, rationalising the relevant provisions of three EU regulations (i.e. CTR, MDR, and IVDR).
2. It introduces a fast-track authorisation procedure with simplified requirements for multinational clinical trials to ensure availability of crisis-relevant medicines to prevent/contain health threats, provide timely treatment options, or facilitate diagnosis in the context of an emerging or a recognised serious cross-border threat to health at Union level[177]. The procedure will be automatically applicable in a recognised serious cross-border threat to health at Union level. The Commission would be further empowered to define, through implementing acts, transparent criteria and a process for declaring the procedure applicable in the context of an emerging serious cross-border threat to health.

[177] In accordance with Article 23 of Regulation (EU) 2022/2371 (see footnote 40, page 9).

3. It strengthens the risk-proportionate approach by more precisely aligning regulatory requirements with the level of risk associated with a clinical trial. A new risk category, minimal intervention trials, is introduced to cover post-marketing authorisation trials, which will require ethical review only for the authorization itself.

(iv) Innovation and Harmonised Data Governance

The proposal facilitates the integration of AI and digital tools (e.g. e-informed consent), reinforces direct shipment of IMPs to patients and improves trial design, execution and oversight. It introduces regulatory sandboxes to test innovative approaches and establishes a single legal basis under the requirements of the GDPR for processing of personal data to provide legal clarity and streamline multinational trials.

(v) Governance and Enforcement

The proposal strengthens EU governance by improving cooperation among Member States' authorities and Ethics Committees by expanding the role for the Clinical Trials Coordination and Advisory Group. National competent authorities and ethics committees must have sufficient personnel and resources to perform assessments. National competent authorities gain stronger inspection powers, including unannounced and joint inspections, which may be delegated to other Member States or agencies. Harmonised guidelines standardise IMP distribution oversight. The Commission can verify compliance within the EU and assess whether clinical trials outside the EU meet good clinical practice and ethical standards.

Expected impacts:

The policy measures outlined above aim to make the EU more attractive to sponsors, by reducing administrative burden and shorten time to market, thereby increasing the number of clinical trials conducted within the EU. The impact of the regulatory changes is assessed both quantitatively and qualitatively through a two-step procedure (Figure 6). First, the expected effect on the total number of EU-based clinical trials resulting from the simplification and acceleration of the authorisation process and conduct of clinical trials is examined. Second, the broader economic implications and public health benefits arising from the increased number of clinical trials conducted across Member States are assessed.

Figure 6. Schematic illustration of the assessment of possible impacts of the proposed regulatory changes to the Clinical Trials Regulation

Step 1 - Expected impact on the number of clinicals run in the EU

Faster, more efficient and better coordinated procedures, as well as the uptake of AI and digital tools are expected to increase the number of clinical trials conducted in the EU/EEA[178,179] and encourage participation by a wider set of Member States. This can reduce current concentration in a few jurisdictions, level the playing field for SMEs and support a more integrated EU clinical research market.

According to one survey with sponsors[180], the anticipated increase of clinical trial authorisations for selected individual policy changes lies between 5% and 28%, and by 32% for a bundled sub-set of policy measures included in the proposed Biotech Act[181]. Another study, based on limited empirical evidence, suggests that the proposed revision of the CTR could increase the number of clinical trials by on average of 10%[182]. In addition, sponsors believe that the regulatory change will foster multinational trials in the EU with a broader geographic scope (see Figure 7).

To quantify the economic and public health impacts of the regulatory change, we assume that the number of clinical trials will increase by 10% to 30%. These estimates draw on two separate studies[183]. The lower bound reflects the average projected increase in clinical trials of 10%. This estimate also aligns with the ACT EU target of having additional 500 multinational clinical trials over the next five years[184]. Meanwhile, the upper bound is derived from survey responses collected from sponsors as part of the second study[185]. The range was selected to reflect the differing findings of the two studies, while accounting for the respective strengths and limitations of each. The wide disparity in these estimates highlights the challenges in assessing the reform's impact on clinical trial activity, given data limitations and inherent uncertainties about future developments.

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[178] *Regulatory Framework Study (forthcoming)*

[179] Between January 2022 and December 2025, on average 2,484 new trial applications were submitted per year. This includes the transition period to the Clinical Trial Regulation which went from 31 January 2022 to 31 January 2025. Source: EU clinical trials during the 3-year CTR transition period.

[180] A survey with 48 sponsors, among those commercial sponsors, was conducted. It is to note that not all respondents replied all questions. For further information, see *Regulatory Framework Study (forthcoming)*

[181] In the survey, sponsors were asked to assess the impact of selected policies individually on the expected change in clinical trials. For the policies comparably to the changes included in the proposal of the Biotech Act the impact of the single measure lies between 5% and 28%. It is to note that the survey replies are subjective assessment from the respondents considering the difficulties that come along with projecting the future. Nevertheless, these figures can be used as indicative evidence. For further information see *Regulatory Framework Study (forthcoming).*

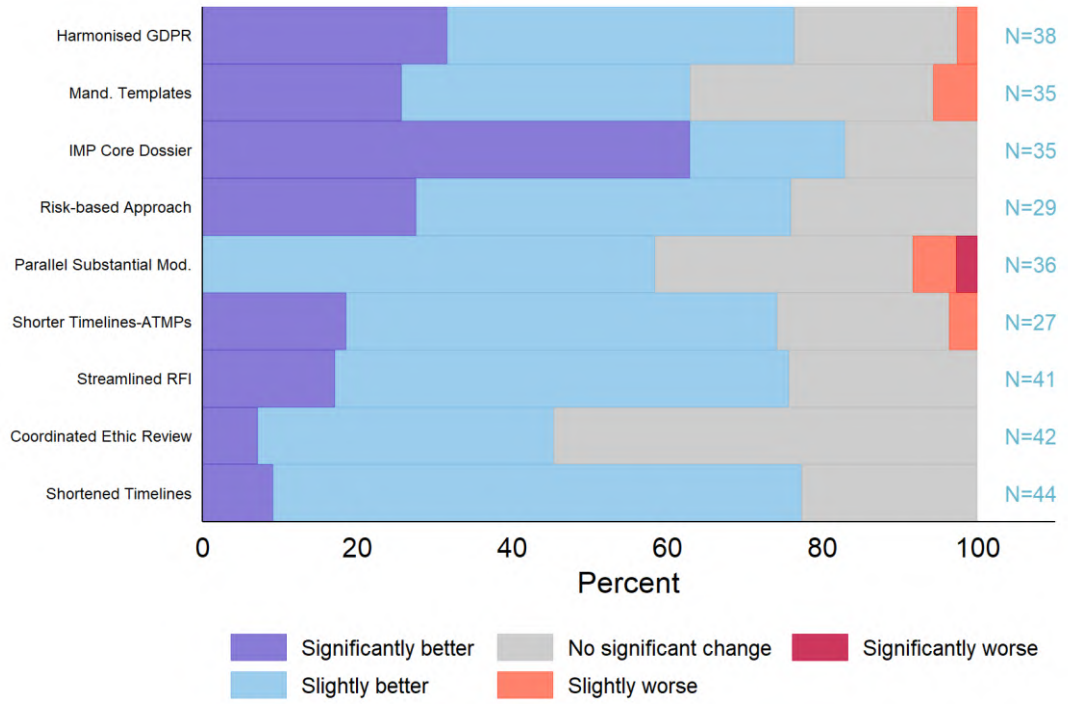
[182] *Rapid Assessment Scenario Study (forthcoming).* The study, based on limited empirical evidence, distinguishes between two hypothetical scenarios regarding the potential impact of the BioTech Act on the projected number of clinical trials in the EU. In the first scenario, a moderate increase in the number of clinical trials by 4% to 8% is anticipated. This is attributed to improved efficacy and attractiveness of performing clinical trials in the EU, driven by increased domestic uptake among sponsors already operating within the region. The second scenario assumes that, in addition to increased domestic uptake, sponsors relocate clinical trial activities from other regions to the EU/EEA as a result of the reforms in the Biotech Act, leading to an estimated increase of 8% to 16%. As the Biotech Act is expected to simplify authorisation processes and the conduct of trials for sponsors, it is considered plausible that some sponsors may relocate their clinical trials to the EU following the amendments of the Regulation. Therefore, the average of both scenarios is used as a benchmark for the lower bound.

183 Studies *Regulatory Framework Study (forthcoming)* and *Rapid Assessment Scenario Study (forthcoming).*

[184] For further information, see: New targets for clinical trials in Europe | European Medicines Agency (EMA). For the purposes of this analysis, the same 11.1% increase is also assumed for mononational clinical trials. For Q1/2026 the KPI has been evaluated for the first time. This quarter, 19 additional multinational trial applications have been submitted. If extrapolated annually, this would represent an 8% increase in trials without any changes to the regulatory framework.

[185] *Regulatory Framework Study (forthcoming)*

Figure 7: Survey replies from sponsors to the following question: "*To what extent would each of the following policies expand the geographic scope of multinational clinical trials in the EU, by increasing the number of participating Member States in a clinical trial in the EU?"*[186]



Step 2 – Economic and Public Health Impact

The subsequent section assesses the expected impact of the increase in the number of authorised clinical trials on economic and public health outcomes. Economic impacts are evaluated along the following dimensions: competitiveness, gross-value added (GVA), work productivity, employment, R&D spillovers, a simplified cost-benefit analysis for sponsors, and relevant EU and national actors involved. The public health impact is measured by the expected change in number of enrolled participants and the composition of the participant populations.

(a) Economic impacts

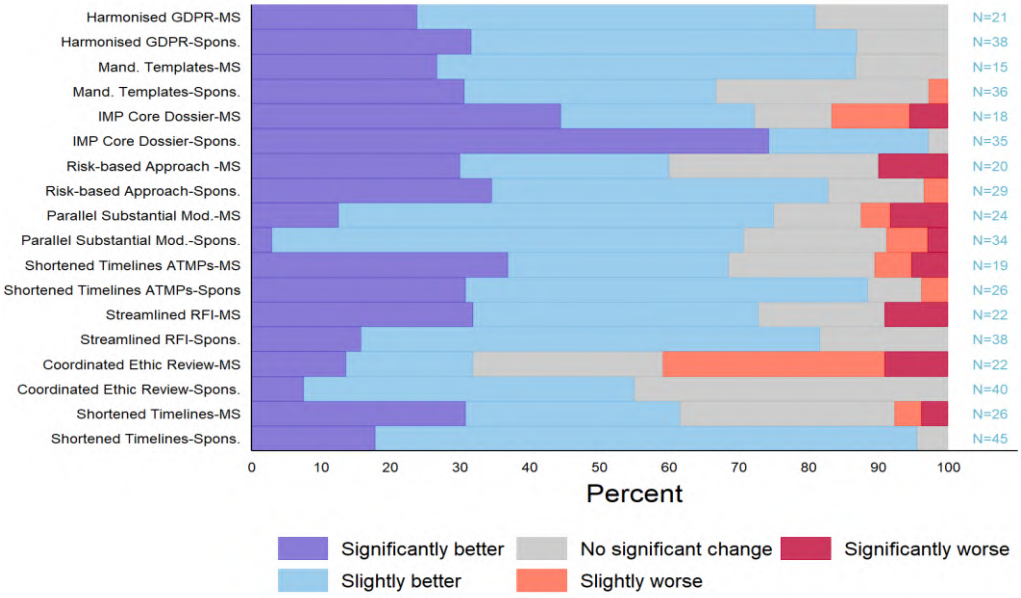
Survey responses from sponsors and Member State representatives[187], including national competent authorities, ethics committees and ministries, suggest that a subset of policy

[186] *Regulatory Framework Study, forthcoming.* In the survey, sponsors were asked to assess the impact of selected policies individually. The policies presented in this figure are those most closely to the ones included in the Commission proposal adopted on 16 December 2025. Please note that there is still some slight deviation between the described policy scenarios assessed in the survey and the policy changes proposed in the Commission proposal.

[187] For further information, see report *Regulatory Framework Study, forthcoming*. The policies presented in this figure are those most closely to the ones included in the Commission proposal adopted on 16 December 2025. Please note that there is still some slight deviation between the described policy scenarios assessed in the survey and the policy changes proposed in the Commission proposal.

measures introduced in amendments to the CTR are expected to strengthen the EU's **competitiveness** (see Figure 8).

Figure 8: Survey replies to the following question "*To what extent would the policies increase/decrease the competitiveness of the EU compared to the baseline in terms of, e.g. R&D expenditures in the pharmaceutical sector including revenue for healthcare providers, number of marketing authorisations and patents granted in the EU/EEA, Gross Value Added in the pharmaceutical sector*?[188]



The **economic impact** for commercially-sponsored clinical trials is likely to differ from that of non-commercial/academic ones due to systematic differences in their characteristics. In particular, 71.5% of applications submitted by commercial sponsors involve multinational trials, whereas 85.6% of applications submitted by academic sponsors relate to mono-national trials.[189] Academic sponsors are also more likely to conduct late-phase clinical trials, particularly post-marketing authorisation trials[190], which may entail different cost structures[191]. These differences suggest that the economic impacts of commercial and non-commercial clinical trials should be assessed separately.

Between January 2022 and January 2025, commercial trials accounted for 53.1% of all clinical trials authorised in the EU. Figure 9 illustrates the incremental benefit for an increase in clinical trials across three economic dimensions, *i.e.* **GVA, R&D spill-over**

[188] *Regulatory Framework Study, forthcoming.* In the survey, sponsors and Member State representatives were asked to assess the impact of selected policies individually. The policies presented in this figure are those most closely to the ones included in the Commission proposal adopted on 16 December 2025. Please note that there is still some slight deviation between the described policy scenarios assessed in the survey and the policy changes proposed in the Commission proposal.

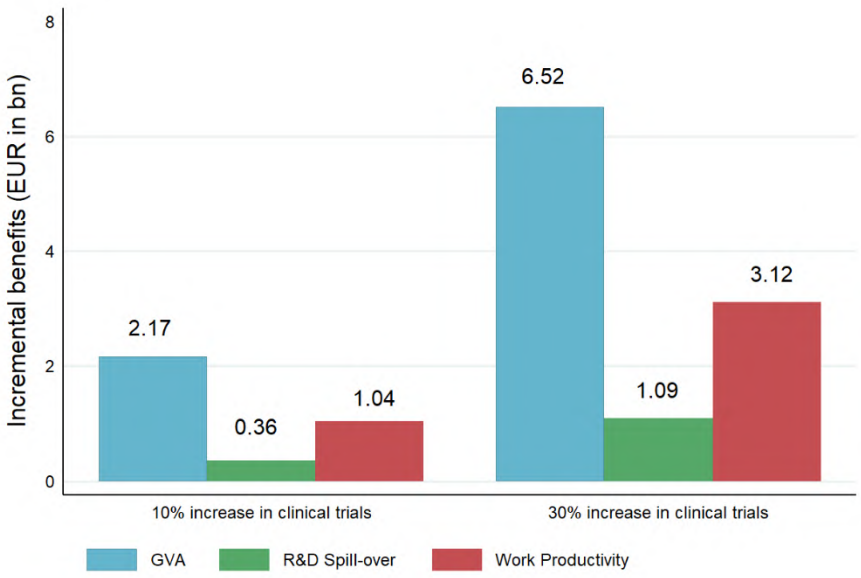
[189] EU clinical trials during the 3-year CTR transition period (see p.9).

[190] EU clinical trials during the 3-year CTR transition period (see p.26).

[191] Fast-track landscape analyses to assess the regulatory clinical trial eco-system in the EU/EEA and in other relevant regions (forthcoming)

and work productivity[192] assuming an increase of 10% or 30% in commercial clinical trials as a result of the legislative changes proposed in the amendments to the CTR. A 10% increase in clinical trials is estimated to generate approximately **EUR 3.6 billion** in economic gains and create roughly **16,500 additional jobs** across the EU, while a 30% increase could result in approximately **EUR 10.7 billion** in economic gains and **49,500 additional jobs**[193].

Figure 9. Economic impacts assuming a 10% and 30% increase in commercial clinical trials[194]



The value of non-commercial trials on the economy has been recently demonstrated for some regions and countries, such as the UK[195]. Due to a lack of sufficiently robust and comparable data for the EU, this analysis does not attempt to quantify the economic impacts of the proposed regulatory changes for non-commercial trials. Therefore, the estimated economic impacts presented for commercial trials should be considered a lower bound estimate of the overall economic gains associated with the increase in the number of authorised cinical trials.

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[192] The GVA measures "*the value of output less the value of intermediate consumption; it is a measure of the contribution to GDP made by an individual producer, industry or sector*" (OECD; National Accounts at a Glance 2009 (EN)). R&D spill-overs measure clinical research activities creating knowledge, products, and processes which can be used by other companies. Workforce productivity measures how improved treatment options enhance people's health and thereby reducing sick days (EFPIA report 2026; the-economic-impact-of-industry-clinical-trials-across-europe.pdf).

[193] The values are calculated based on the following assumptions/parameters: (1) between 2022 and 2025 the annual number of authorised industry commercial trials was 1,509 (EU clinical trials during the 3-year CTR transition period); (2) The reported values in EFPIA 2026 the-economic-impact-of-industry-clinical-trials-across-europe.pdf are used to calculate incremental effects per trial. (3) There is a linear relationship between the increase in clinical trials and the incremental benefits.

[194] Commission own calculations based on information from EFPIA (2026) the-economic-impact-of-industry-clinical-trials-across-europe.pdf and EU clinical trials during the 3-year CTR transition period.

[195] Frontier Economics The value of non-commercial clinical research | Frontier Economics.

The proposed regulatory changes are expected to encourage more non-commercial trials, particularly minimal-intervention and low-intervention trials, by simplifying authorisation requirements and streamlining the conduct of trials typically conducted by academic sponsors[196]. Survey replies support the assumption that these amendments would reduce administrative barriers and make academic research easier, including increased availability of data from comparative trial [197] or the Health Technology Assessment (HTA) procedures. The primary advantages extend beyond the authorisation stage, encompassing risk-proportionate safety reporting, more targeted monitoring, and flexible models for the supply and distribution of investigational medicinal products.

To assess the efficiency of the amendments proposed to the Clinical Trial Regulation, a **simplified cost-benefit analysis** has been carried out as part of two supporting studies. These analyses provide a broad indication of the range of expected cost savings based on different methodologies.

One study evaluates the impacts using estimates from sponsors and public authorities, collected through targeted surveys, on expected changes in costs and the number of clinical trials[198]. Based on these estimates annual cost savings for setting up and the conduct of trials are approximately EUR 1.24 billion for a subset of policy measures included in the Commission proposal. However, it should be noted that response rates for some survey questions have been low (N<10), which may limit the representativeness of conclusions on cost patterns. The policy measures assessed are largely aligned with, but not identical to, those in the Commission's proposal. Additionally, not all the proposed changes are captured therein, and those are expected to have a positive effect on clinical trials in Europe. Important policy changes, such as the introduction of a single assessment procedure for combined studies, the integration of artificial intelligence, and new structures for governance and enforcement measures, are not part of the assessment as the survey was designed prior to the finalisation of the proposed amendments. Therefore, the calculations can be used as suggestive evidence rather than precise estimates of the cost savings.

The assessment of a second study[199] considers the total set of measures included in the Commission proposal. It estimates direct annual cost-savings for sponsors of approximately EUR 1.5 billion to EUR 3.1 billion through more effective administration and faster authorisation procedures and indirect annual savings of equal amounts through an accelerated conduct of clinical trials. The calculations are based on the assumptions that the amendments reduce the total costs of a clinical trial by 5% due to more effective administration, a clinical trial runs on average 2 to 2.5 years, the average costs of a clinical trial amount EUR 30-50 million and the number of clinical trials remains unchanged. It is to be noted that the limited data availability on the average costs of a clinical trial may substantially impact the estimated cost reductions due to the proposed regulatory changes.

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[196] Over 90% of low intervention trials are conducted by non-commercial sponsors (EU clinical trials during the 3-year CTR transition period).
[197] *Regulatory Framework Study (forthcoming)*
[198] See *Regulatory Framework Study (forthcoming)*
[199] See *Rapid Assessment Scenario Study (forthcoming).*

(b) Expected Impact on Public Health

Clinical trials provide early access to innovative treatments for European patients, up to five to ten years before the commercial launch. In the case of rare diseases, clinical trials often present the only available treatment option for patients[200]. In this context, a crucial indicator is not only the number of clinical trials conducted but also the number of participants enrolled in those trials.

Between 2023 and 2025, the estimated average annual number of newly enrolled participants was 163,643 for commercial trials and 341,632 for non-commercial trials[201]. Assuming a 10% to 30% increase in the number of authorised clinical trials, with the median number of participants per trial remaining constant, this would result in an additional 7,847[202] to 23,540 newly enrolled participants included in commercial trials and 10,381 to 31,145 in non-commercial trials per year.

The increase in the number of participants is expected to contribute to improved patient health outcomes and quality of life, while at the same time generating more robust evidence to inform Europe's treatment guidelines and HTA decisions, and potentially facilitating faster market authorisation of the medical products under investigation.

In addition to increasing access to innovative and improved treatment options through clinical trials, the new provisions should also support efforts to reduce health inequalities. For example, pregnant and breastfeeding women would no longer be automatically excluded from trial participation, as is the case under the current regulatory framework. Instead, their participation would be evaluated based on the associated risks and benefits. Furthermore, the inclusion of vulnerable populations, such as minors and pregnant or breastfeeding women, is encouraged, provided that the potential benefits of participation are carefully weighed against the associated risks.

Finally, the anticipated increase in clinical trial activity is expected to foster science, innovation, and skill development. Conducting clinical trials generates knowledge, products, and technologies that stimulate further innovation, drive economic activity, and contribute to the development of a more highly skilled workforce across the sector. These spillover benefits from R&D extend beyond the returns captured by private investors, creating broader societal gains. The associated economic impact of these spillover effects for commercial trials is quantified and presented by the value of the R&D spillover in Figure 9.

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[200] See *Regulatory Framework Study (forthcoming)* and EFPIA (2024) assessing-the-clinical-trial-ecosystem-in-europe.pdf

[201] The data are retrieved from CTIS and represents the estimated mean number of participants by year of clinical trial authorisation for all initial applications between 2023 and 2025.

[202] The calculations rely on the **median** number of participants in commercial trials (52) and non-commercial trials (78) for 2023–2025, rather than the **mean**, to mitigate bias from outliers. This approach yields a more conservative estimate than using average participant numbers (108 per commercial trial and 257 per non-commercial trial).

Table 4. Summary assessment of the effects due to the policy measure

Policy measures	COB	ADMIN	CTI	INT MAR	I&R	PA	H&S
Accelerated and streamlined clinical trial authorisation procedures	++	++	++	+	0/+	0/+	+
Clinical trial regulatory sandboxes	0/+	+	+	+	++	++	0/+
Digital innovation and harmonised data governance for clinical trials	+	++	++	++	+	+	+
Combined Studies	+	+	+	++	++	++	0/+
Governance and enforcement	+	+	+	++	0/+	+	++

5.1.5 Intervention n°5: Targeted regulatory reform of Veterinary Medicinal Products (VMPs)[203]

First, the regulatory framework for **VMPs containing or consisting of GMOs is streamlined** by exempting from EU GMO legislation VMPs containing or consisting of GMOs authorised or manufactured in accordance with VMP Regulation, which are to be assessed solely under the VMP Regulation. Additionally, it is clarified that animals treated with VMPs do not become, for this sole reason subject to the GMO legislation.

Second, the **handling of variations not requiring assessment is simplified:**

- For variations not requiring assessment with no impact on the product information, submissions can be consolidated on a yearly basis.
- For all variations not requiring assessment, confirmation by the competent authorities is no longer required.

Third, the **SPC** for biotechnology VMPs treating zoonoses is extended under certain conditions:

- the product must be developed by means of a biotechnology process as defined in Article 42(2)(a) of Regulation 2019/6;
- it must be intended to diagnose, treat, or prevent zoonotic diseases;
- it must contain a new active substance distinctly different from any authorised medicinal product in the EU;
- it must have a mechanism of action distinctly different from existing products for the same zoonotic disease, with at least equivalent safety and efficacy;

[203] Regulation (EU) 2019/6, OJ L 4, 7.1.2019, pp. 43–167. ELI: http://data.europa.eu/eli/reg/2019/6/oj.

- and at least one manufacturing step (excluding packaging, quality testing, and certification) must be performed in the EU.

Fourth, the Regulation proposal establishes a **regulatory sandbox framework** for innovative technologies, methods or products related to animal health.

Expected impacts:

The measure related to the **VMPs containing or consisting of GMOs** delivers procedural simplification (1-3 month CT application saving; cumulative EUR 5-14.5 million in CTA administrative savings; EUR 560,000 in marketing authorisation dossier savings over the period 2026-2040) and cross-Member States harmonisation, with a high-value, zero-cost legal clarification on GMO status of treated animals eliminating a latent risk. While standard marketing authorisation timelines are unaffected, the benefit materialises under accelerated/emergency pathways (estimated at 30-60 days for 1-3 products/year). While no measurable increase of marketed products is expected, the measure contributes to preventing the progressive offshoring of the innovation process to other jurisdictions where next generation veterinary biotechnology would be developed and trialled.

The proposed SPC extension for VMPs is a low to medium impact signalling instrument. No near-term commercial benefit is expected due to the virtual non-existence of veterinary biosimilar competition and the fact that existing data protection periods already outlast patent/SPC terms. Therefore, the impact of this measure is not appropriately captured through quantification of direct commercial or cost effects, because the primary function of the SPC extension is not to deliver measurable near-term revenue protection but to serve three signalling and future-proofing purposes:

1. **Policy signalling:** The measure communicates EU institutional commitment to veterinary biotechnology innovation addressing zoonotic diseases, potentially influencing long-term R&D allocation decisions at the margin.
2. **Future-proofing:** Should veterinary biosimilar competition eventually materialise at scale (plausibly from the mid-2030s onward), the mechanism would already be in place, avoiding the need for reactive legislative intervention.
3. **EU manufacturing incentive:** The requirement that at least one manufacturing step be performed in the Union introduces an explicit industrial policy lever.

There is no centralised EU database publicly listing how many VMPs have obtained SPCs, and national patent office data are not often broken down by human vs. veterinary use. However, based on data provided by the EMA, out of the 28 biotechnology VMPs for zoonotic diseases approved between 2010 and 2025, only 8 fulfilled all eligibility criteria for the SPC extension. On average, this is 1 product for each 2 years. A forward-looking estimate would foresee **an average of 3 eligible products per year over 2026-2040**, based on the retrospective eligibility analysis (8 out of 28 over 16 years ≈ 2/year) and adjusted upward modestly to reflect the growing share of biotech vaccines in the pipeline. The targeted SPC extension is designed to be cost-neutral for the EMA. Given the narrow scope of the measure, the EMA is expected to handle a very low volume of applications.

The possibility of using a regulatory sandbox for animal health innovation provides a structured framework to facilitate the development and marketing or use of products, methods or technologies that are not regulated under EU legislation. Expected utilisation is modest (~1 sandbox per five years; 2-3 established over 2026-2040). Its primary value is qualitative: pathway creation, evidence generation for adaptive regulation, and investment signalling. The proposed measure creates new EMA/Commission responsibilities (application assessment, implementing acts, reporting).

Finally**, the reduction of burden for the handling of variations not requiring assessment** delivers the most quantifiable and immediately realised benefits with a central estimate of EUR 22.5 million present-value cumulative staff cost savings (2025-2040) and 45-55% reduction in submission events. This will result in approximately EUR 2.5 million/year in fee savings for applicants. The removal of the confirmatory step reduces the national competent authorities workload. It creates a one-off IT cost for EMA of EUR 150,000-300,000. The measure is a purely administrative reform and has no impact on safety or efficacy. The proposed measure has received positive feedback from industry.

Table 5. Summary assessment of the effects due to the intervention n°5

Policy measure	COB	Admin	CTI	Int mar	I&R	PA	H&S
1. GMO exemption	+	+	+	+	+	**0**	+
2. Variations revision	+	++	**0**	**0**/+	**0**	+	**0**
3. SPC extension	**0**/+	**0**	**0**/+	**0**	**0**/+	**0**	**0**
4. Regulatory sandbox	**0**/+	0	**0**/+	**0**	+	-	**0**/+

5.1.6 Intervention n°6: Targeted regulatory reform of the substances of human origin (SoHO) framework[204]

First, the **SoHO regulatory status consultation procedures** is streamlined by empowering the Commission to adopt implementing acts establishing **binding time limits** for regulatory status consultation processes within the SoHO framework.

Second, the Regulation proposal establishes a **regulatory sandbox framework for SoHO,** enabling Member States to create time-limited controlled environments for developing and

[204] Regulation (EU) 2024/1938, OJ L, 2024/1938, 17.7.2024. ELI: http://data.europa.eu/eli/reg/2024/1938/oj.

testing innovative SoHO products, services, processes, or substances that cannot yet fully comply with standard regulatory requirements.

The new regulatory sandbox framework allows a Member State (or several jointly) to set up a regulatory sandbox upon a substantiated request from a SoHO entity, providing a time-limited controlled environment to facilitate the development and testing of innovative SoHO products, services, processes or substances under the supervision of one or more competent authorities. Two eligibility conditions should be met: (a) the innovation is expected to contribute distinctively to SoHO safety, quality, including effectiveness, or to significantly improve patient access to treatment; and (b) applying the SoHO Regulation's requirements would impede or significantly delay development due to scientific or regulatory challenges inherent to the innovation.

The regulatory sandbox is an evidence-generating mechanism: it should enable assessment in a real-world environment under strict regulatory supervision, producing the data needed to demonstrate safety and quality, including effectiveness, in view of subsequent distribution decisions. Derogations from the SoHO Regulation may be included, but only if clearly described, strictly necessary, and justified in a sandbox plan. Derogations may take the form of adapted, enhanced, waived or deferred requirements. Sandboxes cannot derogate from the SoHO Regulation's standards on voluntary and unpaid donation.

Operationally, activities must follow a sandbox plan developed by the SoHO competent authorities, informed by data and consultations with the innovation's developers, and specifying participants and roles; which SoHO requirements cannot be complied with and the resulting derogations and adaptations; risk-mitigation measures; the sandbox duration; and the monitoring framework. The competent authorities must consult the SoHo Coordination Board (SCB) where appropriate, and the SCB is tasked with supporting and fostering a common approach. The SCB may also collaborate with the Foresight Panel for Emerging Health Innovation established by the Biotech Act, linking sandbox practice to upstream horizon-scanning and cross-framework learning.

The proposed amendments create a structured route for SoHO innovations, which cannot yet be developed in full compliance with the SoHO Regulation, or if compliance would impede or significantly delay development due to inherent scientific or regulatory challenges. The proposed amendments also formalise cross-authority collaboration and information exchange, which improve transparency and comparability across Member States while retaining core safeguards.

A SoHO preparation resulting from an innovation developed as part of a regulatory sandbox may be distributed for human application only where authorised in accordance with Article 38(1) of Regulation (EU) 2024/1938. The purpose of the regulatory sandbox is therefore to make authorisation feasible by enabling supervised testing and evidence generation in novel or complex cases where applying the current regulation's requirements would impede or significantly delay development. These are, for example, new technologies for which there are currently no fitting technical safety and quality requirements by the Expert Bodies (EDQM, ECDC); new technologies that are only partially covered by SoHO rules.

Sandbox-eligible cases would be a minority of cases which could not be authorised under the SoHo Preparation Authorisations (SPA) model established in the 2024 SoHO Regulation, but would bring in high extra value. Expert interviews suggested that SoHO products eligible for the sandbox route likely represent 5–10% of overall SoHO activities, i.e. **90-250 eligible cases per year**[205]. Sandbox use cases demonstrate **acceptance rate of between 7-16%** of sandbox applications, demonstrating great interest from developers in the sandbox instrument but limited capacity of the regulators to establish sandboxes[206]. Considering the high resource requirements of a regulatory sandbox, we can further assume that only a share of eligible cases will be pursued. If all eligible cases apply for the sandbox tool, between **6 and 40 regulatory sandboxes can be established pear year**.

Expected impacts:

Since the regulatory sandboxes are a first-of-its-kind instrument, the initial take-up could be slow, also as, in the first years, most innovations are expected to be well regulated under the new model for SPA in the 2024 SoHO regulation.

Conduct of business: Regulatory sandboxes offer a pathway for addressing complex cases that would otherwise not be pursued. We estimate a slowly growing interest in the regulatory sandbox from SoHO entities, leading to regulatory sandbox requests, particularly in strong innovation nations and for SoHO innovations where no safety and quality requirements are prepared by the expert bodies.

Administrative costs on businesses, including SMEs: Regulatory sandbox is expected to have similar costs to high-risk authorisations (between EUR 1,200 and EUR 6,000 per patient) due to the need to involve a certain number of data subjects and a thorough assessment process[207]. Therefore, while there would not be any extra cost for high-risk cases, if low- or medium-risk cases are pursued using the sandbox route, the direct cost of each case will increase. We assume that half of the cases directed to the sandbox route will be high risk cases and half of the cases will be medium risk cases. Under the low estimate, the additional cost of regulatory sandbox route for SoHO entities will be EUR 718,500 (under the assumption that six sandboxes are established per year) and EUR 4,790,000 (under the assumption that 40 sandboxed are established per year).

As a positive secondary effect this would allow SoHO entities to engage with authorities early in an environment that tolerates iterative learning, prototyping, and co-development of evidence strategies, thereby lowering the volume of redundant work and iterative back-and-forth that currently inflates administrative burdens.

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[205] Rapid Assessment Scenario study, assuming that currently, a share of SoHO activities is not pursued for authorisation due to exceedingly high costs of evidence generation and/or difficulty reaching full compliance. However, this share could not be estimated.

[206] Rapid Assessment Scenario study based on: Medicines and Healthcare products Regulatory Agency. (2025). *AI Airlock sandbox pilot programme: Report*. UK Government. https://assets.publishing.service.gov.uk/media/68ee1fb88427701993d5e02c/AI_Airlock_Sandbox_Programme_Report._Final.pdf and Commission nationale de l'informatique et des libertés. (2022). *Dossier de presse : Bilan 2021 et enjeux 2022.* https://www.cnil.fr/sites/default/files/atoms/files/dossier_de_presse_cnil_bilan_2021_et_enjeux_2022_vf.pdf.

[207] Rapid Assessment Scenario Study, based on assumptions provided by the European Commission.

Competitiveness, trade and investment flows: By providing a flexible, but coordinated and proportionate pathway for novel SoHO activities, regulatory sandboxes will reduce uncertainty and transaction costs for innovations. This could improve EU attractiveness for certain R&I projects. Particularly strong competitiveness effects can be expected in the forerunner countries with strong innovation potential. Such effects will come from two sources. First, projects that are authorised thanks to sandbox evidence, which would not be pursued otherwise.

Second, under the assumption that sandbox route allows for a responsible approach with limited or more flexible types of appropriate evidence, and for less authorisations to be obtained, innovative SoHO activities will become more attractive for SoHO entities.

Functioning of the internal market and competition: The regulatory sandboxes might have an indirect effect if the outcomes obtained in one Member State are reused in other Member States when assessing similar innovations. This can in particular be expected given the central role of the SoHO Coordination Board in supporting and monitoring sandboxes, including joint sandboxes undertaken by two or more Member States. The overall approach of open access innovation in the largely public SoHO sector will also contribute to a fast and harmonized roll-out of novel technologies.

Innovation and research: The regulatory sandboxes provide a pathway to authorisation and a signal to the R&I stakeholders. As the immediate response to the sandbox measure, we can therefore expect to see an increase in the actor activity in this segment. These changes in risk and incentives and the reduction of the administrative burden could attract more public entities into innovation and stimulate innovation activity in novel, borderline cases and cases requiring cross-framework coordination.

Innovation in SoHO is continuous, often incremental and largely driven by activity in public organisations, with knowledge diffusion through publications and professional societies rather than patents[208]. In leading Member States, major public hospitals treat research as a regular part of their activity and are linked to universities and research institutes. This creates a favourable ecosystem for SoHO innovation, which is nevertheless dependent on the availability of public and private research grants. However, high administrative costs deter these actors from innovative and novel SoHO activities.

Controlled testing environments created through the regulatory sandboxes would enable experimentation under protection from disqualification due to a mismatch with the current framework. By allowing for adapted standards of regulatory evidence generation, regulatory sandbox pathway can enable innovative research that might otherwise be abandoned for fear of regulatory rejection. Therefore, these proposed amendments support the 'throughput' phase of the innovation process. Innovation and research impacts at Member State level are expected to be positive mainly via risk reduction, faster evidence generation, increase in academic research, and regulator capability-building, but uneven across Member States depending on capacity and ecosystem maturity.

[208] Rapid Assessment Scenario Study, based on SWD(2022) 190 final.

Regulatory sandboxes could be expected to shorten the path from innovation to safe clinical use, especially where the baseline suffers from uncertain borderlines and divergent evidence thresholds. In the financial technology area, firms that went through the regulatory sandboxes had, on average, a 40% shorter time from engagement with the regulator to market authorisation than similar firms using the standard process. In SoHO, therefore, gains can also be expected. However, no baseline data is available for quantification as these gains would be realised in the cases that otherwise would not be pursued due to difficulty gathering evidence for authorisation.

Public authorities: The addition of the sandbox instrument is expected to expand the mandate and increase the workload of public authorities in the short and medium term for the benefit of significant cost reduction in the longer term due to achieved system efficiency.

To quantify the operational costs of the regulatory sandboxes on public authorities, person-day cost of the sandbox should be compared with person-day cost of the SPA model, taking into account both the costs of learning by doing and the cost reduction expected in the medium term due to cross-Member State learning leading to improved efficiency of operations when novel SoHO activities are concerned. Furthermore, OECD guidance indicates that investments are needed for regulators in terms of time, expertise, and organisational capacity when instituting a sandbox instrument.

These costs would be borne primarily by national competent authorities but also by the Commission due to (i) the role in supporting the regulatory sandbox activities and hosting the EU SoHO Platform, and (ii) the engagement of the SCB as a key operational and consensus-building mechanism. Experts interviewed for this study assessed the role of the EU-level guidance as high for organisation of SoHO activities on the Member State level[209]. Commission-defined principles and formal mandates are essential to stimulate cross-body collaboration among relevant public authorities within Member States and to harmonise common national procedures for multi-framework cases.

We calculate the cost of regulatory sandbox for public authorities as follows: 1) assessing request (no change per risk category), 2) workload of regulatory sandbox (50 person-days) and 3) assessment of clinical evidence (reduced by 40%). This brings the workload on a medium-risk case to 75-94 person-days per case (EUR 22,575 – EUR 28,294); and on the high-risk case to 98-149 person-days per case (EUR 29,498 – EUR 44,849).

Public health and safety: The sandbox instrument grants earlier access to therapies for patients involved in the clinical evidence collection, before the activity is approved. As illustrated above, regulatory sandbox has similar evidence requirements as a high-risk authorisation process. In total, regulatory sandboxes are expected to involve 450-3,000 patients p/a. The impact of such access could be significant in the case when sandboxes provide evidence generation for SoHO activities that would otherwise not be feasible to authorise, for example, in the cases of rare diseases or niche treatments. Follow-up monitoring and assessment should consider whether these patients would have been served at all if the sandbox route did not exist, since authorisations for sandbox-eligible cases

[209] Rapid Assessment Scenario Study, based on stakeholder consultation (interview).

would be very difficult to obtain ordinarily. A key condition of sandbox eligibility is a significant expected public benefit, which further implies that sandboxes will address the needs that would otherwise not be met.

Longer-term effects are expected to arise due to institutionalisation of cross-Member State learning and publication of sandbox reports on the EU SoHO Platform. The proposal constrains derogations to standard SoHO procedures to what is strictly necessary and requires explicit risk mitigation and monitoring and therefore disallows derogations on voluntary or unpaid donation standards.

Table 6. Summary assessment of the effects due to the intervention n°6

Policy measure	COB	Admin	CTI	Int mar	I&R	PA	H&S
Regulatory sandbox	++	+	0/+	0	+	++	+

5.1.7 Intervention n°7: Targeted regulatory reform of the Directive on the deliberate release into the environment of GMOs[210]

The proposal introduces specific provisions in Part C of Directive 2001/18/EC addressing the placing on the market of GMMs as or in products other than food and feed with the aim of creating a tailored, more efficient and streamlined regulatory framework for the placing on the market of GMMs as or in products, while maintaining a high safety level for human and animal health and the environment. The proposed provisions relate to the risk assessment, to the validity of the consent granted for their placing on the market, and to detection methods applicable to all GMMs. In addition, the proposal introduces the concept of low-risk GMMs and a specific set of rules for adapted information requirements and a streamlined authorisation procedure for this subgroup of GMMs to be detailed by way of Commission delegated and implementing acts. The requirement for post-market environmental monitoring of low-risk GMMs is equally adapted in the proposal, providing the possibility to a notifier, based on certain conditions, to propose to omit post-market environmental monitoring.

Expected impacts:

Conduct of business: The proposed regulatory framework for GMMs as or in products other than food and feed is expected to improve some framework conditions for companies being active in the GMM field. By shifting towards more product-specific risk assessment criteria, introducing specific modalities for detection methods, granting unlimited validity for market consents and creating an accelerated pathway for low-risk GMMs, where certain data requirements would be waived in consideration of an existing Qualified Presumption of Safety delivered by EFSA, the intervention should shorten time-to-market and reduce procedural rigidity. This is expected to pave the way for GMM-related consents for placing on the EU market of GMMs as or in products. Earlier market entry would

[210] Directive 2001/18/EC, OJ L 106, 17.4.2001, pp. 1–39. ELI: http://data.europa.eu/eli/dir/2001/18/oj.

mitigate income losses from regulatory delays and improve the business case for investment, particularly for SMEs and start-ups that are more sensitive to regulatory timelines and uncertainty.

Administrative costs on businesses (including SMEs): The impacts are expected to be very positive. While quantitative estimates are lacking for non-food GMMs, a qualitative assessment indicates that a more product-focused risk assessment and a dedicated low-risk GMM pathway should substantially reduce the burden associated with preparing extensive dossiers under the current GMO regime. Streamlined, more predictable procedures, the unlimited validity of consents and the possibility to waive post-market environmental monitoring where justified would lower recurring costs, especially for GMMs considered to be low risk. This is expected to make compliance more manageable for resource-constrained SMEs and start-ups, to stimulate exploration of novel products and partnerships between large firms and smaller innovators, and to reduce the incentive to relocate development activities to jurisdictions such as the US or China with more flexible GMM frameworks.

Competitiveness, trade and investment flows: The new regulatory framework is expected to act as an enabler and accelerator. By reducing regulatory costs and uncertainty and clarifying a route to market for GMMs, the intervention could render more projects investable, thereby supporting an expansion of the innovation pipeline in sectors such as biofertilisation, biocontrol, bioremediation, and bioleaching. Over time, this is expected to translate into a larger portfolio of authorised GMM products and more robust EU-based value chains in these areas. The more predictable pathway from laboratory to market would likely attract additional venture capital and corporate investment and may encourage non-EU companies with established GMM products to enter the EU market. The number and resilience of biotech start-ups and SMEs operating in GMM-related domains is likewise expected to increase, as founders and investors gain confidence that commercialisation in Europe can realistically be achieved within acceptable timeframes. However, all these effects would be gradual and path-dependent, unfolding over several years and dependent on how the regulatory scrutiny process works in practice.

Functioning of the internal market and competition: There are no adverse impacts expected. The new regulatory framework for GMMs modifies a horizontal framework and will apply across all Member States and to all economic operators, so no distortions of intra-EU trade or competition between Member States are anticipated. The analysis notes that competition and displacement may arise between GMM-based products and their non-GM counterparts; however, in the absence of concrete evidence and given that many firms are likely to offer both types of products, no material changes in internal market functioning or competition patterns are expected as a direct consequence of the new GMM framework.

Innovation and research: The proposed intervention is projected to have a positive and potentially substantial effect. By reducing administrative barriers, shortening authorisation timelines and providing regulatory clarity, the framework could stimulate R&D investment by universities, research organisations and companies. This is particularly important in the EU context, where excellent early-stage biotech research has historically struggled to translate into scaled-up applications and competitive firms. A more innovation-friendly regulatory environment is expected to improve the EU's relative position vis-à-vis other

major innovation regions such as the US and China, increase private funding for GMM-related research, and help retain and attract talent in this domain. Over the longer term, the biotechnology innovation system around GMMs could therefore become more dynamic, better leveraging Europe's strong scientific base.

Public authorities and their administrative workload: The effects of the new regulatory framework for GMMs are difficult to assess. The average effort for national competent authorities per GMO authorisation of a non-food/non-feed GMM would fall. However, the extent to which burden will be reduced will fall in a wide range, resulting from the fact that the adaptation of risk assessment (applicable to all GMMs, and expected to go further for low-risk GMMs) will be case-by-case and may lead to considerable variability of data requirements. In the most conservative scenario, risk assessment would require roughly the same amount of data as today, and only a small fraction of products would qualify for the low-risk procedure (interviews suggested that national authorities may be cautious in designating GMMs as low-risk, based on a precautionary approach and no practical experience with non-medical GMM releases), translating in very limited gains for competent authorities. In less conservative scenarios, the tailoring of risk assessment to GMMs is expected to deliver efficiency gains for national authorities. In addition, the harmonisation of criteria for low-risk products will allow the effective use of that procedure, delivering further efficiency gains and reduction of administrative burden. Further savings, in the phase following the initial authorisation, would result from the unlimited authorisation validity and waivers of post-market environmental monitoring. These were not quantified and would accrue mainly at national level. At EU-level, the mechanisms regarding EFSA's role in resolving disagreements between Member States is not modified, and the considerations above apply in those instances where -following a disagreement- EFSA would need to conduct an assessment. Both at national and EU-level, if application numbers rise significantly under the new framework, total administrative effort may increase or decrease depending on how many of these applications would have occurred under the existing regime and on the effective use of the low-risk pathway.

Public health and safety: The proposed measures are designed to maintain the high protection standards of the current EU GMO regime while making their implementation more proportionate to the specific characteristics of GMMs. The intervention does not lower safety standards or abandon mandatory risk assessment but rather tailors information requirements and allows for more flexible approaches where risks are low and well understood. This is expected to reduce unnecessary regulatory burdens without compromising the high level of protection of human and animal health and the environment.

At the same time, by enabling the deployment of beneficial GMM applications that are currently discouraged or displaced to other regions, the new framework could help realise **environmental and sustainability benefits**, for instance by supporting more resource-efficient agriculture, cleaner industrial processes or enhanced bioremediation. In doing so, it may contribute to broader EU policy objectives such as the European Green Deal and the 2030 Agenda for Sustainable Development, while preserving robust safeguards for health and the environment.

Table 7. Summary assessment of the effects due to the intervention n°7

Policy measure	COB	Admin	CTI	Int mar	I&R	PA	H&S
Regulatory framework for GMMs as or in products other than food and feed	+	+	+	**0**	+	+/**0**	**0**

5.1.8 Intervention n°8: Targeted regulatory reform of the Directive on human organs intended for transplantation[211]

The proposal amends Directive 2010/53/EU to reflect technological developments in organ preservation and processing and to provide EU-wide legal clarity and standards for innovative practices which aim to improve organ quality and expand transplantation opportunities.

First, the scope of the Directive is extended to explicitly include the processing of organs prior to transplantation, with a definition of "processing" being introduced by the proposal, encompassing any operation involving the handling of organs performed to maintain or improve the functional status of an organ prior to transplantation.

Second, a specific authorisation regime for organ processing per processing technology and per transplantation centre is established. A transplantation centre is to submit an application for authorisation to the competent authorities including a benefit-risk assessment where scientific evidence and clinical data are available. In the absence of such evidence and data, e.g. when no other EU transplantation centre has been authorised to apply the same organ processing technology, a clinical outcome monitoring plan must be submitted for approval. A clinical outcome monitoring plan must also be submitted for approval where the benefit-risk assessment identifies a significant risk. The extend of the clinical monitoring will be proportionate to the risks identified. Detailed rules on the authorisation procedure are to be established through Commission implementing acts and guidance on the benefit risk-assessment is to be established by the competent authorities.

Transplantation centres must request the written agreement of competent authorities prior to making significant changes to authorised processing steps. The competent authorities have the prerogative to suspend an authorisation where there is reasonable ground to suspect non-compliance. A central EU list of authorised processing techniques or with an approved clinical outcome plan is to be established by the Commission. This will serve as reference point to simplify later authorisations of similar processing technologies used in other EU transplant centres, and hence to facilitate the roll-out and uptake of the same processing technology for other centres and authorities.

[211] Consolidated text: Directive 2010/53/EU, ELI: http://data.europa.eu/eli/dir/2010/53/2010-08-06.

Third, the proposal clarifies coordination with other Union health legislation. Where organ processing involves medicinal products, medical devices or SoHO preparations, national competent authorities must verify compliance under the relevant Union frameworks and cooperate with the corresponding national competent authorities under Directive 2001/83/EC, Regulation (EC) No 726/2004, Regulation (EU) 2017/745 and Regulation (EU) 2024/1938 to ensure coherent and more efficient oversight.

These amendments aim to provide EU-wide and cross-sector legal certainty, support innovation in transplantation, and ensure high standards of quality and safety while facilitating timely clinical uptake of advanced processing technologies.

Expected impacts:

Measure 1 – the enlarged scope is textually modest but **legally consequential** as the foundational enabler of Measures 2 and 3. On its own, however, it generates no direct operational requirements, administrative obligations or supervisory mandates. Its independent effect operates through a single channel: the provision of legal certainty by formally recognising organ processing as a regulated activity within the EU legislative framework. This **resolves the legal ambiguity** identified in the problem definition and sends a **regulatory signal to transplantation centres and technology developers**, but the substantive operational, administrative, and health impacts materialise only through Measures 2 and 3.

Measure 2 – on organ processing authorisation regime - is crucial and derives in nearly all quantified impacts for this intervention area.

The conduct of business is assessed as moderate positive: the framework relates to the operational model of transplantation centres by introducing a prior authorisation obligation per centre and per processing technology. However, the **net effect is positive because the certainty channel** (removing the legal ambiguity that functions as a structural drag on adoption) outweighs the compliance channel, yielding an **estimated 70-110 additional programmes** with active processing capability and approximately **1,435 additional transplants** per year by 2035.

Administrative costs on businesses are assessed as **marginal negative**: the per-application cost is estimated at **EUR 11,500-16,750** (Track 1/Track 2), with a cumulative EU burden of approximately **EUR 2.5 million over 2029-2035**, costs that are real but proportionate and partially offset by replacing fragmented and duplicative baseline compliance arrangements. Moreover, only the first centre in the EU has to complete the full pathway at the full cost, since subsequent centres can apply a shortened pathway by using evidence from the central list. This leads to significant administrative savings for the overall EU transplant community. Further, transitional measures are envisaged to avoid the authorisation of practices already in place, so that these are not to be re-authorized.

Competitiveness, trade and investment flows are **marginally positive**: the transparent authorisation pathway reduces the regulatory risk premium for private investment in organ processing technologies, but the effect is indirect given the niche character of the market. The **internal market** benefits from standardised authorisation procedures that create mutual trust for cross-border exchange of processed organs, with an estimated **120-250**

additional cross-border exchanges per year by 2035, while the published list of authorised operations generates de facto EU-wide convergence of practice.

Innovation and research are **moderate positive**: the dual-track system provides a structured pathway for next-generation technologies (Track 2 conditional authorisation), an **estimated 15-36 active monitoring plans** generate structured EU-level evidence, and the "Processing" data field in the Annex creates the taxonomic infrastructure to disaggregate processing-attributable outcomes.

Public authorities face a **marginal negative** impact: the transition-phase assessment cost is approximately **EUR 850,000 over three years** across the EU, declining to approximately **EUR 60,000-85,000 per year** in steady state; the net incremental cost is moderated by the replacement of ad hoc national arrangements but represents a genuine, if manageable, expansion of the supervisory mandate.

Public health and safety is the strongest effect, assessed as significant positive: **1,435 additional transplants per year by 2035; 570-945 fewer organ discards; 57-72 waiting-list deaths averted annually; approximately EUR 158 m in cumulative dialysis cost savings** over 2028–2035; and a structural improvement in safety oversight through the mandatory benefit-risk assessment and clinical-outcome monitoring mechanisms.

Measure 3 addresses the **multi-regulatory-domain nature of organ processing**, which require competent authorities to verify that products used in processing (medical devices, medicinal products, SoHO preparations) are duly authorised under their respective EU frameworks, and mandate cross-authority collaboration for the exchange of clinical outcome data. Its impact profile is narrower and more targeted than Measure 2.

The **conduct of business** is marginal positive: the cross-framework verification adds a structured step to the centre's processing workflow but also brings EU harmonization and reduces the ad hoc legal uncertainty that centres currently face regarding the permissibility of specific product combinations.

Administrative costs are marginal negative: the verification dossier imposes a modest additional documentation requirement, functionally embedded within the Measure 2 application process. However, there is a significant simplification in administration by other centres, aiming to implement an organ processing step already authorized in other EU transplant centres, facilitating uptake of novel processes across the EU.

Competitiveness, trade and investment flows are marginal positive: the clearer multi-domain regulatory landscape marginally improves investment predictability for manufacturers of perfusates, gene therapy vectors, and other products used across regulatory frameworks.

The **internal market** is the principal beneficiary of this measure: the mandatory cross-authority coordination directly addresses the regulatory silo problem identified in Section 3.2.3 and creates legally binding cooperation obligations that facilitate the acceptance of processed organs involving multi-domain products across Member State borders.

Innovation and research are marginal positive: the mandated exchange of clinical outcome data between regulatory frameworks generates research-valuable evidence on multi-domain processing outcomes, contributing to a more coherent EU-level evidence ecosystem.

Public authorities face marginal negative costs from establishing inter-authority communication protocols, though Member States with existing cooperation infrastructure will face limited marginal effort.

Public health and safety benefits indirectly but materially: the cross-framework verification ensures that all products used in processing meet their respective EU safety standards, and the mandated data exchange improves pharmacovigilance and device vigilance for processing-related adverse events, closing a surveillance gap that currently exists between regulatory silos.

Table 8. Summary assessment of the effects due to the intervention n°8

Policy measure	COB	Admin	CTI	Int Mar	I&R	PA	H&S
1. Enlarged scope	**0/+**	**0**	**0/+**	**0/+**	**0/+**	**0**	**0/+**
2. Organ processing authorisation regime	+	**0/–**	**0/+**	+	+	**0/–**	++
3. Cross-border and cross-framework coherence	**0/+**	**0/–**	**0/+**	+	**0/+**	**0/–**	+

5.2 Interventions on industrial enablers: measures and expected impacts

5.2.1 Interventions n°9, 10 and 11: Strategic projects, high-impact projects and ecosystem support framework

The proposed Regulation establishes a comprehensive framework to structure and coordinate the selection, development and scaling-up of strategic health biotechnology capacities and projects, encompassing:

- **(i) the recognition and the support of health biotechnology strategic projects (Intervention n°9),**
- **(ii) a targeted EU-level framework for high-impact strategic projects (Intervention n°10)[212], and**
- **(iii) an ecosystem support framework (Intervention n°11).**

[212] The proposal sets out five types of high impact projects. For the purpose of the analysis in this section, two of these project categories (biotechnology development accelerators - trusted testing or demonstration facilities with state-of-the-art equipment and staff – and centres of excellence for advanced therapies) are taken into consideration, while three others (concerning access to finance, AI-enabled biotechnology innovation and biodefence) are considered under the respective intervention areas, namely Interventions 14, 15 and 17.

Together, these measures aim to boost the EU's competitiveness and resilience in health biotechnology, by strengthening biomanufacturing capacity, value chains, research and technology infrastructures in the sector, accelerating innovation and technology deployment and addressing ecosystem fragmentation through coordinated support mechanisms.

Strategic projects (recognised by Member States) would benefit from administrative support (including single points of contact at Member State level), accelerated procedures (including for permitting, subject to a maximum ten-month deadline, as well as for dispute resolution and litigation), a public interest status and measures to facilitate access to funding at national and EU-level. Provisions also proposed support to pro-competitive collaboration between projects, networks and clusters. High-impact projects (recognised at EU level) represent a limited flagship portfolio of systemic importance, including biotechnology development accelerators and centres of excellence for advanced therapies. These projects benefit from similar support but with priority status, including an accelerated eight -months permitting timeline and stronger signalling for Union financial support.

These project-level measures are underpinned by ecosystem support instruments:

(i) a strategic mapping of the EU biotechnology ecosystem to identify capacities, gaps and investment needs;
(ii) an EU health biotechnology support network with national and regional antennas to assist innovators in navigating regulatory and funding pathways and accessing scaling and networking opportunities; and
(iii) a European Health Biotechnology Steering Group to coordinate Member States and the Commission, facilitate exchange of best practices and ensure effective implementation for strategic and high impact health biotechnology projects.

The EU Health Biotechnology Support Network is not intended to duplicate existing EU-level SME support structures, such as the Enterprise Europe Network or future horizontal instruments under the European Competitiveness Fund. Rather, it operates as a sector-specific coordination and support layer, building on existing structures where appropriate, and providing health biotechnology-specific navigation, ecosystem coordination and cross-border integration functions. In particular, while existing networks may support firms on general business, innovation or internationalisation aspects, they are not designed to provide structured support for navigating complex, multi-framework health biotechnology regulatory pathways. The design of the Support Network is therefore intended to remain compatible with and complementary to future EU-level SME support architectures, including the envisaged EU for Business Network, notably by leveraging existing capacities while ensuring the availability of specialised expertise.

Expected impacts:

Conduct of business and administrative costs: Taken together, the measures are expected to generate significant positive impacts on conduct of business and administrative costs for firms, with positive but more incremental contributions to competitiveness, internal market functioning, innovation and, indirectly, public health and safety. Impacts on public authorities are expected to be front-loaded but moderate overall due to reliance

on existing structures. The principal transmission channels operate at two complementary levels. At project level, impacts arise from reduced execution risk through more predictable and coordinated administrative handling, including time-bound permitting (ten months for strategic projects, eight months for high-impact projects), structured single points of contact model and facilitated access to finance. At ecosystem level, impacts arise from reduced transaction and navigation costs, improved access to investors and infrastructures, and strengthened cross-border collaboration through network and cluster effects.

Uptake is a key uncertainty across all components, therefore the scale of impacts should be interpreted as proportional to the number and maturity of projects applying for recognition and to the utilisation of ecosystem support services. This reflects the voluntary nature of the mechanism and the fact that promoters will weigh the administrative effort of application against the expected benefits of more predictable administrative pathway and improved access to support, consistent with observed dynamics in comparable strategic-project regimes[213].

To illustrate the potential order of magnitude, three uptake scenarios can be defined for each of the project categories. As a calibration point, other EU-level priority-project frameworks[214] have operated at volumes ranging from several dozen to over one hundred projects, providing a useful order-of-magnitude reference (rather than a direct comparator) for the potential scale of project pipelines under coordinated policy frameworks. Against this backdrop, an indicative range for health biotechnology strategic projects could be framed as: (i) low uptake: around 25-30 recognised projects by 2038, (ii) medium uptake: around 60-70 projects, and (iii) high uptake: around 100-120 projects. For high-impact projects, reflecting a limited flagship portfolio, ranges would be: low uptake (six to –eight projects), medium uptake (12–15), and high uptake (20–25).

Conduct of business: Significant positive impacts are expected through greater predictability, shorter effective administrative lead times for strategic and high-impact projects, alongside reduced coordination frictions. Greater clarity and time-bound procedures are expected to improve planning horizons, milestone sequencing and time-to-revenue, reduce volatility in project schedules and strengthen firms' capacity to align procurement, construction and staffing decisions with a clearer administrative pathway. Delay and uncertainty remain economically material for capital-intensive investments, with an indicative benchmark suggesting that regulatory delays of more than 12 months beyond initial projections have been associated with an average 34% increase in total development costs, alongside a high incidence of additional funding rounds and some programme abandonment.

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[213] See Commission SWD on establishing a framework of measures for strengthening Europe's Net Zero Industry Act: COM(2023) 219 final (https://single-market-economy.ec.europa.eu/publications/net-zero-industry-act_en).

[214] E.g. projects recognised under Union frameworks with explicit strategic project provisions, such as those established by the Net Zero Industry Act and the Critical Raw Materials Act, as well as earlier coordinated frameworks such as the first Union list of energy Projects of Common Interest (PCIs) and Projects of Mutual Interest (PMIs) comprising 166 projects and approved IPCEIs in the microelectronics value chain comprising 100 projects across 14 Member States. While the scale of such projects is influenced by Member State participation, fiscal capacity and policy priorities, they remain informative as to the potential scale of coordinated project pipelines. The ranges presented here are therefore illustrative and adapted to the specific design and incentives of the present measure.

For high-impact projects, these effects are reinforced through access to promoter-led shared late-stage translation infrastructures with cross-border relevance, supporting more efficient development and scale-up. Impacts can be evidenced through utilisation indicators such as the number of firms served, including SMEs, the number of service engagements (e.g. testing, validation and small-batch GMP runs), and utilisation rates, reflecting improved coordination and access to critical capabilities.

Ecosystem support measures contribute indirectly by improving visibility of strategic projects, facilitating collaboration and enhancing firms' ability to navigate the EU regulatory and funding landscape. While these effects are not directly attributable in isolation, they reinforce the overall improvement in business conditions generated by the project-level measures. Overall, once implementation stabilises, a significant share of recognised projects is expected to complete permit granting within the applicable timelines.

Administrative costs on businesses, including SMEs: Significant positive impacts on administrative costs are expected, driven by reduced transaction costs, simplified administrative interactions and more predictable, time-bound procedures. For strategic and high-impact projects, reductions arise from fewer parallel interfaces, fewer iterative information exchanges and greater reuse of existing evidence, supported by single points of contact and structured administrative facilitation. These effects are expected to lower compliance costs and administrative burden, with avoided delay costs for capital-intensive investments constituting the main economic benefit.

For high-impact projects, impacts are more targeted, reflecting prioritised handling and faster decision-making for a limited number of catalytic projects, reducing schedule uncertainty rather than generating system-wide burden reductions. Ecosystem support measures further reduce navigation and search costs, particularly for SMEs and start-ups, through a single-entry point and improved preparedness for regulatory interactions. Overall, impacts are expected to be proportionately stronger for SMEs lacking in-house administrative capacity.

Competitiveness, trade and investment flows: Positive impacts on competitiveness, trade and investment flows are expected through improved bankability, stronger investment signalling and reduced time-to-market. For strategic and high-impact projects, more predictable delivery and prioritised handling are expected to lower risk premia and improve conversion from planning to financial close, supporting higher investment mobilisation and leverage, particularly for mature and bankable projects. High-impact projects are expected to generate stronger effects per project due to their scale, visibility and systemic relevance.

Ecosystem support measures reinforce these effects by improving access to investors through structured matchmaking and by reducing delays linked to incomplete or immature submissions, thereby shortening time-to-market[215]. Overall investment mobilisation will

[215] Evidence from the EIC Business Acceleration Services demonstrates that structured matchmaking can generate measurable outcomes (over 20,000 meetings, 595 deals, EUR 350 million raised since 2021). On time-to-market, EMA data indicates that applicant-side clock-stops represent a substantial portion of total procedure time (198 days average vs

depend on project maturity and uptake, but the combined framework is expected to strengthen investment attractiveness and facilitate greater mobilisation of both public and private capital in the Union health biotechnology sector. Existing Union financing instruments can also amplify these effects through leverage: where support is backed by an EU budget guarantee, this can reduce risk for implementing partners and crowd in additional public and private investment beyond the direct Union contribution.

Internal market: Positive impacts on the functioning of the internal market are expected through a more visible and comparable pipeline of strategic projects and reduced information asymmetries for promoters, authorities and investors. Cross-border recognition for projects located in two or more Member States is expected to reduce duplicative processes and improve consistency of treatment, supporting more contestable investment conditions across Member States, while take-up patterns will remain sensitive to underlying administrative capacity and clustering effects. Internal market effects are expected to strengthen as uptake increases and the pipeline becomes sufficiently broad and geographically distributed to improve comparability and transparency across Member States.

These effects are reinforced by improved cross-border accessibility of infrastructures and shared capabilities in the context of high-impact strategic projects, facilitating resource pooling and multi-country collaboration. The Support Network is expected to reduce fragmentation of support services by providing comparable[216] antennas across Member States. Cluster networks further contribute by structuring cross-border ecosystems, enabling access to biomanufacturing and research infrastructures and supporting the formation of cross-border consortia[217].

Innovation and research: Impacts are expected through faster and more efficient translation of research and innovation into industrial deployment, supported by strategic projects and reinforced by a limited number of high-impact catalytic assets addressing late-stage bottlenecks. Strategic projects contribute through strengthening biomanufacturing infrastructures and innovation assets, while high-impact projects further enhance the **number and speed of projects progressing through late-stage development**, particularly for advanced therapies and other complex applications.

These effects are complemented by ecosystem measures, notably cluster networks and support structures that strengthen research-industry linkages and facilitate cross-border collaboration, thereby enhancing firm-level innovation capacity and access to shared infrastructures. Overall, innovation impacts are expected to scale with uptake and portfolio composition, with the main effect being improved translation and deployment rather than system-wide shifts in innovation performance.

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204 days assessment time in 2023), with 42% of applicants requesting extensions due to immature data. By helping SMEs come better prepared to interactions with regulatory bodies, the Support Network could address submission readiness gaps that contribute to these delays.

[216] This comparability is ensured through common selection criteria set by the Commission, standardised service functions, and EU-level coordination via the Steering Group and strategic mapping, which together support consistent service delivery across Member States. See Rapid Assessment Study – forthcoming – for further elaboration.

[217] Evidence from the ECCP Cluster Panorama and sector comparators (e.g., Silicon Europe with 2,500+ companies) supports the potential of cluster structures to facilitate cross-border consortia.

Public authorities: They are expected to be front-loaded mainly due to process design, workload reallocation and coordination needs to operate single points of contact and provide structured support, as well as participation in governance and coordination structures. In many Member States, single points of contact may be established by designating or adapting existing administrative coordination mechanisms rather than creating entirely new structures, which may reduce upfront costs. Over time, efficiency gains are expected as procedures standardise, and administrative experience accumulates. Incremental impacts attributable specifically to the high-impact designation are expected to be limited and concentrated in selection, monitoring, coordination and reporting for a limited flagship portfolio, without duplicating cost drivers associated with general permitting operations and routine administrative facilitation. Administrative workload will scale with the number of recognised projects and monitoring cycles required.

The intervention also introduces additional obligations for Member States, including participation in the Steering Group, data provision for ecosystem mapping and facilitation of support network activities, but these are expected to build on existing structures, resulting in marginal set-up costs. Comparable initiatives (NZIA, CRMA) estimate 1–2 FTEs for one-stop shop functions and around two FTEs for governance participation. These initial obligations are expected to yield efficiency gains over time, as Member States gain access to a systematic evidence base and coordination forum that should improve national decision-making and reduce duplicative investments. At EU-level, technical assistance appropriations of approximately EUR 2.6 million annually (EUR 18.4 million over the MFF period) cover relevant coordination functions.

Public health and safety: Impacts are expected to be positive but indirect, mediated by the realised project portfolio and the extent to which faster delivery translates into earlier EU-based capacity for strategic products and a contribution to supply resilience outcomes. The scale of both administrative workload and resilience-relevant capacity effects is expected to increase with uptake, subject to project mix and implementation performance. For high-impact projects, the core impact pathway will be potentially faster, safer and more efficient development of innovative therapies, notably advanced therapies, supported by improved access to specialised testing, validation, small-batch GMP and quality control services and, where relevant, links to data and digital infrastructures.

Impacts are expected to materialise over the medium term and remain conditional on uptake by developers and manufacturers, as well as on broader regulatory, reimbursement and health system conditions affecting downstream patient access. Ecosystem support measures are not expected to have direct effects but may indirectly reinforce these outcomes by facilitating collaboration and improving system preparedness.

The tables below provide an illustrative, scenario-based quantification[218] of the expected impacts by 2038 under three uptake cases for the recognition and support framework for strategic health biotechnology projects (Table 9) and for high impact health biotechnology projects (Table 10) for each impact category. Impacts should be interpreted as orders of

[218] See Annex 7 and relevant sections of the Rapid Assessment Study – forthcoming for further elaboration on the calculations of impacts

magnitude and as broadly proportional to the number, maturity and operationalisation of recognised projects over the period.

Table 9. Illustrative uptake scenarios and order-of-magnitude impacts for strategic health biotechnology projects

Impact category	Summary assessment of the effects due to the policy measure	Key indicators expected evolution under different project uptake scenarios		
		Low uptake (25-30 projects)	Medium uptake (60-70 projects)	High uptake (100-120 projects)
COB	++	Projects completing permit granting within 10 months: ~18-26	~42-60	~70-102
Admin	++	Avoided delay costs (cumulative): ~EUR 90-410 million	~EUR 214-952 million	~EUR 357-1,632 million
CTI	++	Total investment mobilised (cumulative): ~EUR 6-12 billion	~EUR 15-28 billion	~EUR 25-48 billion
Int mar	+	Cross-border projects (≥2 Member States): ~3-6	~6-14	~10-24
I&R	+	Projects with material R&I or innovation infrastructure outputs: ~5-9	~12-21	~20-36
PA	0/+	Recognition decisions per year (average): ~2-3; indicative single point of contact capacity: ~0.5-1 FTE	~5-6; ~1-2 FTE	~8-10; ~2-3 FTE
H&S	+	Capacity-relevant strategic deployments: ~8-15	~18-35	~30-60

Table 10. Illustrative uptake scenarios and order-of-magnitude impacts for high impact strategic health biotechnology projects

Impact category	Summary assessment of the effects due to the policy measure	Key indicators expected evolution under different project uptake scenarios		
		Low uptake (6-8 projects)	Medium uptake (12-15 projects)	High uptake (20-25 projects)
COB	++	Shared-capability throughput: ~60-120 firms/year, ~120-480 engagements/year	~120-360 firms/year, ~240-1,440 engagements/year	~240-600 firms/year, ~480-2,400 engagements/year
Admin	++	Avoided delay costs (cumulative): ~EUR 20.4-85 million	~EUR 51-204 million	~EUR 91.8-340 million
CTI	+	Total investment mobilised (cumulative): ~EUR 2-6 billion	~EUR 4-12 billion	~EUR 6-20 billion
Int mar	+	Cross-border users supported: ~12-48 firms/year	~24-144 firms/year	~48-240 firms/year
I&R	+	Clinical trial applications linked to capability layer: ~4-8/year	~8-24/year	~16-40/year
PA	0/+	Incremental workload: ~6-8 recognition decisions plus monitoring cycles, limited multi-country coordination	~12-15 decisions plus monitoring, moderate coordination workload	~20-25 decisions plus monitoring, higher coordination workload but still bounded by portfolio design
H&S	+	Therapy programmes supported with improved development pathway: ~2-4/year	~4-12/year	~8-20/year

Table 11. Summary assessment of the effects of Intervention n°11: Health biotechnology ecosystem support framework

Policy intervention	COB	Admin	CTI	Int Mar	I&R	PA	H&S
Ecosystem support measure	0	++	+	+	+	-	0

5.2.2 Intervention n°12: Biosimilars competitiveness framework

The proposal includes two measures focusing on biosimilars:

First, it proposes the development and update of **EMA guidelines** on tailored and risk-proportionate regulatory approaches for biosimilar development, reflecting manufacturing and analytical testing advances. Most importantly, the guidance will set out the criteria and provide for the potential reduction of clinical data requirements for biosimilar development and approval without affecting quality, safety and efficacy. The revision would be guided by the assessment and conclusions of the reflection paper on tailored clinical approach in biosimilar development by EMA and the CHMP Biosimilar Medicinal Products Working Party (BMWP), published on 27 March 2026[219]. The reflection paper concludes that "a tailored approach for clinical development of biosimilar candidates is possible. Comparative Efficacy Studies (CES) are no longer expected to be required for approval of biosimilars that can be thoroughly characterised using state-of-the-art analytical methods and have demonstrated similarity in physicochemical and functional properties". Comparative clinical pharmacokinetics/pharmacodynamics (PK/PD) studies can in this context provide supportive safety and immunogenicity data. Over the past three years, applications concerning monoclonal antibodies and fusion proteins have represented more than 90% of all biosimilar marketing authorisation applications (see Annex 5 - additional background on the sector and problem definition). The proposed measure is therefore expected to have a broad practical impact, as it applies to the majority of current and future biosimilar development programmes.

Second, it proposes the establishment of a framework for the **recognition and the support of health biotechnology strategic projects focused on biosimilar** research, development, manufacturing and marketing authorisation. The support package outlined in intervention n°9 applies to these projects. Furthermore, the proposed measure promotes international cooperation between economic operators and biotechnology clusters active in the area of biosimilars, including in the context of health biotechnology strategic projects.

The two measures are complementary and the intervention logic goes as follows: The EMA guidelines are expected to reduce the evidentiary burden and associated cost and time of biosimilar development, thereby lowering barriers to market entry. Simultaneously, biosimilar strategic project recognition aims to provide administrative, financial, and permitting support for biosimilar manufacturing and development projects within the EU.

[219] Reflection paper on a tailored clinical approach in biosimilar development.

Furthermore, eligibility criteria for biosimilar strategic projects enable projects developing analytical methodologies that reduce clinical data needs to qualify for support under the measure, creating a feedback loop between regulatory modernisation and industrial investment.

Expected impacts of measure 1: update and development of EMA guidelines

Conduct of business: If, following EMA guidelines, CES requirement was waived for biosimilar mAb products where analytical similarity is comprehensively demonstrated, developers could reduce direct Phase III clinical trial costs by an estimated EUR 19–26 million per product[220], with a broader range of EUR 14–46 million across all trial types[221]. Clinical development accounts for approximately 57% of total biosimilar development costs[222], with the CES representing the single largest component.

Of the 27 biosimilar marketing authorisation applications (MAAs) resolved in 2024[223], approximately 24 concerned monoclonal antibodies or fusion proteins, the product classes for which CES have historically been required (see Annex 5)[224].

On this basis, **three scenarios** are considered regarding the uptake of a tailored development approach including CES waivers.

An estimated CES cost of EUR **19–26 million** per product for monoclonal antibody and fusion protein biosimilars is used to estimate the **gross annual cost savings** for industry.

Residual clinical requirements are expected to persist. Comparative PK/PD studies (estimated at EUR 5.7–7.8 million per product) will remain necessary for all biosimilars, and immunogenicity assessment will continue to be required, particularly for antibody-based products. Accounting for these residual costs, **total net savings** are assumed to be reduced by approximately 30%[225].

In the near term (2025–2030), assuming 25–45 biosimilar marketing authorisation applications (MAAs) per year, with approximately 90% relating to monoclonal antibodies and fusion proteins, and that 50–75% of these would benefit from a tailored development approach including a CES waiver, the **gross annual cost savings** for industry are estimated at EUR 214–790 million.

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[220] Ranbhor, R., & Kulkarni, P. (2026). Net present value impact of FDA's Phase 3 waivers on monoclonal antibody biosimilar development programs. *Biosimilars and Targeted Therapy*. https://www.dovepress.com/net-present-value-impact-of-fdas-phase-3-waivers-on-monoclonal-antibod-peer-reviewed-fulltext-article-BTT.

[221] Moore, T. J., Mouslim, M. C., Blunt, J. L., Alexander, G. C., & Shermock, K. M. (2020). Assessment of availability, clinical testing, and US Food and Drug Administration review of biosimilar biologic products. *JAMA Internal Medicine, 181*(1), 52–60. https://doi.org/10.1001/jamainternmed.2020.3997.

[222] Ranbhor, R., & Kulkarni, P. (2026). Net present value impact of FDA's Phase 3 waivers on monoclonal antibody biosimilar development programs. *Biosimilars and Targeted Therapy.* https://www.dovepress.com/net-present-value-impact-of-fdas-phase-3-waivers-on-monoclonal-antibod-peer-reviewed-fulltext-article-BTT.

[223] Of the 27 MAAs 25 were authorised and 2 withdrawn or refused; source: EMA Medicines Database, 2026.

[224] European Medicines Agency. (2026). *Medicines output report* [dataset]. Retrieved 2 March 2026 from https://www.ema.europa.eu/en/medicines/download-medicine-data; European Medicines Agency. (2025). *Annual report 2024.* Publications Office of the European Union. https://doi.org/10.2809/7433636.

[225] The share of European investment into biosimilar development, which is not allocated to CES, which in turns makes up for the 70% as outlined above.

After accounting for residual clinical development requirements—primarily comparative PK/PD studies and immunogenicity assessments **net annual savings** are expected in the range of EUR 150–552 million.

Over the **five-year period (2025-2030), cumulative net savings** are therefore estimated at:

Scenario	Uptake	Gross annual cost savings for industry	Total net annual savings	Five-year period, cumulative net savings
Low scenario (50%)	12 products per year transition to CES waivers	EUR 222–312 million	EUR 155–218 million	EUR 0.75 billion
Central scenario (≈60–65%)	14–15 products per year	EUR 266–390 million	EUR 186–273 million	EUR 1.5–1.8 billion
High scenario (75%)	18 products per year	EUR 342–467 million	EUR 239–326 million	EUR 2.8 billion

The projected annual range of 25–45 marketing authorisation applications (MAAs) reflects a normalisation following the exceptional peak observed in 2025, notably linked to denosumab-related applications. At the same time, projections remain constrained by the 'biosimilar void' [226, 227].

The lower bound (25/year) assumes the void persists; the upper bound (45/year) assumes that the guidance (issued by 2027) begins attracting developers. Under current conditions, it is estimated that only around 10% of the approximately 100 biologics expected to lose exclusivity in the EU by 2032 will face biosimilar competition[228].

In the medium term (2030–2038), assuming that the tailored approach to CES waivers is widely implemented, and that complementary investment support measures partially mitigate the 'biosimilar void', it is projected that 35–55 biosimilar MAAs may be submitted annually. Of these, approximately 90% are expected to concern monoclonal antibodies and fusion proteins, with 70–80% benefiting from a tailored development approach including a CES waiver.

On this basis, the **gross annual cost savings** for industry are estimated at EUR 418-990 million.

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[226] Approximately 100 biological medicines are expected to lose exclusivity in Europe by 2032. However, 79% currently have no biosimilars in development, and only 10% of biologics nearing loss of exclusivity are likely to face biosimilar competition in Europe when accounting for the geographic footprint of clinical trials (IQVIA, 2026). The void is concentrated in biologics with projected global sales below USD 100 million, where current development costs remain prohibitive.

[227] IQVIA, The Impact of Biosimilar Competition in Europe, 2026, iqvia-the-impact-of-biosimilar-competition-in-europe-2026-01-26-forweb.pdf.

[228] IQVIA, The Impact of Biosimilar Competition in Europe, 2026, iqvia-the-impact-of-biosimilar-competition-in-europe-2026-01-26-forweb.pdf.

After accounting for residual clinical development requirements **net annual savings** are expected in the range of EUR 293–700 million.

Over a five-year period within this timeframe, cumulative **net savings** are therefore estimated at:

- **Low scenario**: EUR 1.5 billion
- **Central scenario**: EUR 2.3–2.8 billion
- **High scenario**: EUR 3.5 billion

These projections reflect a scenario in which reduced development costs, increased regulatory predictability, and targeted policy support measures contribute to higher biosimilar uptake and increased market entry. As such, the economic impact of CES waivers is expected to be significantly amplified compared to the near-term period.

Overall, the medium-term scenario suggests that the full implementation of a tailored, risk-based regulatory approach could generate substantial and sustained cost efficiencies for industry, while supporting improved competition and access to biological medicines in the Union.

According to the Biosimilar Medicines Group[229], a sector group of the Medicines for Europe organisation, under the current framework, the sector's contribution to European clinical trials for biosimilar candidates amounts to EUR 4.9 billion over five years, with approximately 71% allocated to CES. The implementation of CES waivers could free an estimated **EUR 3 billion over five years** in clinical spending. These resources could be reallocated to European research and development and manufacturing, including the development of next-generation biosimilar medicines, new technological platforms (e.g., antibody-drug conjugates, cell and gene therapies), and modernisation of manufacturing operations).[230]

While the cost impact of biosimilars has been evaluated, the potential time savings are expected to be limited, as many development streams run in parallel rather than sequentially. A CES waiver would accelerate one stream, while the remaining streams continue to constrain the overall development timeline. However, shorter development pipelines would allow developers to start later, once the originator's market trajectory—including geographic penetration, therapeutic positioning and real-world outcomes—is clearer, thereby reducing commercial risk without affecting the ultimate timing of market entry.

Administrative burden: The dominant cost saving is associated with the CES waiver and not with the reduction in dossier preparation labour. According to reported estimates, the preparation of a full CES-based dossier requires approximately 328 person-days (EUR 131–197 thousand), compared to 253 person-days (EUR 101–152 thousand) for a tailored package. This represents a reduction of approximately 75 person-days (EUR 30–45 thousand) per application. Applied to 12–18 affected MAAs annually, this yields an aggregated administrative savings of **EUR 0.36–0.81 million per year at current**

[229] https://www.medicinesforeurope.com/biosimilar-medicines/who-we-are/ .
[230] Medicines For Europe factsheet: Pillar 1-2-3-4-FOOTPRINT-SPC-Biotech-Act-facsheets-ppt.cdr.

conditions. While measurable, these savings are secondary relative to clinical trial cost reductions (see Annex 5 for reported detailed dossier preparation calculations).

Innovation and research: The proposed intervention is expected to reallocate innovation incentives toward advanced analytical science. As outlined in the section 'conduct of business' above, savings generated from the elimination of superfluous comparative efficacy studies (CES) are estimated at **EUR 717 million- 2.76 billion in five years,** as regulatory reliance shifts from clinical trials to analytical comparability. According to another recent industry report[231], approximately EUR 3 billion in R&D expenditure will be freed up over five years out of the current EUR 4.9 billion —to be reinvested in the development of additional biosimilar products, new technology platforms, and the modernisation of manufacturing operations.

Public health: The tailored approach is expected to accelerate patient access without compromising safety. Therapeutic coverage is expected to expand from approximately 27 reference products to 35–45 by 2030 and to 55–75 by 2038. The cumulative number of authorised biosimilars is expected to grow from 151 in 2024 to 250-350 between 2025-2030[232] and to 450-650 between 2030-38[233]. This expansion is projected to generate significant system-level savings, increasing from approximately **EUR 13 billion in 2024 to EUR 16–22 billion annually by 2030 and EUR 22–35 billion/year by 2035–2038,** driven by increased competition and broader treatment availability. The introduction of a tailored regulatory approach is not expected to alter the underlying safety standard, as it modifies evidentiary requirements rather than regulatory thresholds for approval[234]. The safety outlook therefore remains unchanged, with **no increase in withdrawals on safety grounds anticipated**.

However, the upper bounds of these projections are tempered by a structural gap in the market. Of approximately 100 biologics expected to lose exclusivity by 2032, 79% have

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[231] Medicines for Europe, Biosimilar Medicines Sector Group. (2025). Reforming Regulations, Market Entry and Competition for Biosimilar Medicines (Pillar 2) [Factsheet]. Brussels: Medicines for Europe. https://www.medicinesforeurope.com/wp-content/uploads/2026/03/FOOTPRINT-Biotech-Act-factsheet.pdf.

[232] 151 biosimilars already authorised in 2024. To this, we add the expected new approvals between 2025 and 2030 i.e. between 25 and 45 new applications, of which ~89% will be successful over 5 years

(i) Low estimate: 25 × 5 × 0.89 = ~111 new products → total ~262

(ii) High estimate: 45 × 5 × 0.89 = ~200 new products → total ~351

[233] ~300 authorised products in 2030, to this we add 35–55 annual applications between 2030 – 38 × 8 years ~89% success rate

(iii) Low estimate: 35 × 8 × 0.89 = ~249 new products → total ~549

(iv) High estimate: 55 × 8 × 0.89 = ~392 new products → total ~692

[234] *Risk based tailoring of clinical evidence requirements for biosimilar marketing authorisations does not lower safety standards; it reflects the maturity of the EU regulatory framework for biosimilars and advances in analytical science used to demonstrate comparability with the originator product. The EU has the longest global experience with biosimilars, with the first EU biosimilar approved in 2006, and since then over 100 biosimilars authorised across around 30 reference products, with no withdrawals on safety grounds. This long-standing experience confirms the robustness of the comparability-based regulatory approach.*

At the same time, analytical methods have significantly evolved. High-resolution structural characterisation, advanced functional assays, and improved immunogenicity testing now allow very sensitive detection to demonstrate comparability with the originator medicinal product.

This is complemented by strong post-authorisation safety monitoring through the European Medicines Agency. In this context, streamlined clinical requirements do not weaken safety; they align regulatory expectations with current scientific capability while preserving continuous real-world pharmacovigilance.

no biosimilar versions in development and only 10% are likely to face biosimilar competition in Europe[235].

Expected impacts of measure 2: biosimilar strategic projects recognition and support

The projects this proposed measure would target benefit from the same framework described in intervention n°9 *i.e. recognition and the support of health biotechnology strategic projects***:** administrative support (including through single points of contact at Member State level), accelerated procedures (including for permitting, subject to a maximum ten-month deadline, as well as for dispute resolution and litigation), a public interest status and measures to facilitate access to funding at national and EU-level.

Administrative costs on businesses: As for the projects described in intervention n°9, the greatest efficiency gain is expected to be the reduction of execution risk through more predictable and coordinated administrative handling, notably through a structured single point of contact model and a maximum 10-month permit-granting timeline, complemented by facilitated access to finance.

Conduct of business: The measure is expected to mobilise additional investment in EU biosimilar manufacturing and development infrastructures. The magnitude depends on Member State implementation. To estimate the order of magnitude, three uptake scenarios are used, consistent with the approach applied in the strategic projects and high impact projects assessments. Biosimilar projects are framed as a subset of the total strategic project pipeline:

- **Low uptake**: 3–5 biosimilar projects recognised by 2038 (~0.3 per year)
- **Medium uptake**: 8–12 biosimilar projects (~1 per year)
- **High uptake**: 15–20 biosimilar projects (~1.5 per year)

See annex 7 for an overview of the illustrative order-of-magnitude impacts for biosimilar strategic projects derived by proportional scaling from the Strategic Projects of chapter II (intervention n°9).

Competitiveness, trade and investment flows: The effectiveness of the proposed measure is expected to scale with its uptake, with higher levels of recognised projects leading to greater mobilisation of investment in EU-based biosimilar manufacturing. The investment mobilisation estimated at **EUR 0.9–7.6 billion cumulative by 2038** reflects the total capital deployed in biosimilar manufacturing and development projects. This includes private investment, STEP-supported instruments, and any national co-financing. The per-project investment range of **EUR 200–400 million** is consistent with the capital requirements for dedicated biosimilar biomanufacturing facilities, as documented by industry sources.[236]

[235] IQVIA, The Impact of Biosimilar Competition in Europe, 2026, iqvia-the-impact-of-biosimilar-competition-in-europe-2026-01-26-forweb.pdf.
[236]Alira Health. (2025, November 6). 2025 global biosimilars report: Market size, growth drivers, regional dynamics. https://alirahealth.com/biosimilars-market-2025-market-size-growth-drivers-regional-dynamics/

Further, the proposed measure supports EU based manufacturers' competitiveness in biosimilars, by promoting **international partnerships** among economic operators as a mean to share expertise and diversifying and strengthening supply chains. Recent studies identified an increasing number of partnerships involving ex-EU biosimilar companies that collaborate with European manufacturers to launch products in Europe. Historically, these partnerships were relatively uncommon, but their prevalence has increased. Between 2006 and 2013, ex-EU partnerships represented 30% of all approvals, but more recently this figure increased to 45% (2014–2024).[237] The proposal aims to leverage this trend by encouraging promoters of biosimilar projects to establish or strengthen cooperation with **international partners**, including with a view to fulfilling the conditions for the recognition of biosimilar strategic projects.

When it comes to **research and innovation (R&I)**, as per specific eligibility criteria proposed in Article 29, the recognised projects could contribute to R&I through setting up or extending infrastructures for biosimilar analytical testing; strengthening the use of platform technologies for research, development and marketing authorisation; and developing analytical methodologies that would reduce the need for clinical data for the development and approval of biosimilars. The impact of the measure on R&I will depend on how many recognised projects will focus on material research or analytical innovation outputs. As mentioned in the conduct of business section above, biosimilar projects are framed as a subset of the total strategic project pipeline. Out of all 3-5 biosimilar strategic projects in a low uptake scenario, we assume 1-2 to be R&I focused; 2-4 out of 8-12 in a medium uptake scenario; and 3-6 out of 15-20 in a high uptake scenario (see Annex 7 for full table of scenarios uptake).

The administrative costs for public authorities are part of the overall cost analysed in chapter II for strategic projects. The initiative does not create a separate approval architecture for biosimilar strategic projects specifically. Efficiency gains are expected to emerge over time as procedures standardise, and administrative experience accumulates. By 2030–2038, as the recognition process matures and uptake increases (estimated at 8–12 biosimilar projects under the medium scenario), the net effect is expected to be decisively positive for the sector.

Table 212. Summary assessment of the effects due to the policy intervention

Policy measure	COB	Admin	CTI	Int Mar	I&R	PA	H&S
1. Tailored regulatory approach (Art. 28)	++	+	+	+	+	-	+
2. Strategic project support (Art. 29-30)	+	+/-	+	+	+	-	0/+

[237] Troein, P., Newton, M., Stoddart, K., Travaglio, M., & Arias, A. (2025, January). *The impact of biosimilar competition in Europe*. IQVIA. http://www.iqvia.com/-/media/iqvia/pdfs/library/white-papers/the-impact-of-biosimilar-competition-in-europe-2024.pdf.

5.2.3 Intervention n°13: SPC extension for biotechnology medicines

Supplementary protection certificates (SPCs) are an intellectual property right. They serve as an extension to a patent right and aim to offset the loss of patent protection for pharmaceutical that occurs due to the compulsory testing these products require prior to obtaining regulatory marketing approval. An SPC can extend a patent right for a maximum of five years[238].

The Regulation proposal introduces an extension of 12 months of the **SPC for medicinal products developed by means of biotechnology processes and for Advanced Therapy Medicinal Products.**

Eligible medicinal products would have to contain a new active substance which is effective via a mechanism of action distinctly different to that of any other product already authorised in the EU for preventing or treating the same disease. The clinical trials evaluating the efficacy of the product and supporting its marketing authorisation would need to have been conducted in more than two Member States and at least one manufacturing step, excluding packaging, quality and testing certification, would need to be performed in the Union.

This provision is intended to target highly innovative biotechnology-based products that are expected to deliver significant therapeutic advantages to patients, particularly those who currently have limited or no treatment options.

The proposed SPC extension does not affect in any respect the existing legal framework in Regulation (EC) No 469/2009[239] or related measures, nor the ongoing reform of that framework. In accordance with the principle of legal certainty, this includes how the SPC extension will be applied in the future. The SPC extension has a different scope. Its purpose is not to replace the existing SPC legal framework but to complement it.

Intended objective:

The SPC, by prolonging IP protection, provides a very robust shield from competition for the relevant product[240]. Additionally, on average, products for which SPC is the last form of protection to expire have higher revenues than medicines that rely on regulatory protection (RP) or patent (see Annex 7 for details on protection length). This further underscores the attractiveness and effectiveness of this incentive.[241]

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[238] Six-month additional extension is available in accordance with Regulation (EC) No 1901/2006 if the SPC relates to a medicinal product for children for which data has been submitted according to a Paediatric Investigation Plan (PIP). PIPs are required to support the authorisation of medicines for children. They ensure that enough data is collected on the effects of the medicine on children.

[239] Regulation (EC) No 469/2009 of the European Parliament and of the Council of 6 May 2009 concerning the supplementary protection certificate for medicinal products, OJ L 152 16.6.2009, p. 1 httphttp://data.europa.eu/eli/reg/2009/469/2019-07-01.

[240] Max Planck Institute for Innovation and Competition (2018), Study on the Legal Aspects of Supplementary Protection Certificates in the EU.

[241] Impact assessment accompanying the revision of the pharmaceutical legislation https://health.ec.europa.eu/publications/impact-assessment-report-and-executive-summary-accompanying-revision-

Innovation and patenting behaviour in biotechnology are closely linked to firms' expectations around the future commercial value and appropriability of inventions. However, intellectual property is just one part of innovators' investment decisions. The Biotech Act proposal includes explicit conditions to link the SPC extension to conducting late-stage clinical trials and manufacturing in Europe, because the incentive is precisely meant as a stimulus to modify sponsors' behaviour at the margin, thereby helping generate economic and societal gains for Europe.

Analysis and limitations:

It is acknowledged that modelling the costs and benefits of this measure necessitates certain assumptions and it is subject to inherent limitations, such as the need to work with small samples of medicines given the medicine characteristics being studied, and simplified representations of complex real-world health biotech innovators' behaviour. These limitations have been mitigated by using robust data sets reflecting observations of actual product lifecycles and market entry patterns (market data from the IQVIA MIDAS® databases) and assessments on products eligibility (data provided by the European Medicines Agency which will be in charge of assessing eligibility), to ensure that the resulting estimates of the measure's potential remain grounded and reliable rather than being based on a more theoretical model.

To make an estimation of the costs of the SPC extension, an analysis was performed of the 198 innovative medicines which lost patent or regulatory protection between 2016 and 2024 and that have not so far been withdrawn from the market. Of those medicines 31 were found to be biologics, 12 of which rely on SPC as the last protection to expire (40%). Of these 12, 10 fulfil the proposed Regulation's SPC eligibility criterion of being a medicine developed with a biotechnology process. In order not to limit the sample size unduly, the other SPC eligibility criteria have not been used to exclude medicines from the sample as they should not a priori affect the market characteristics of the medicine. The sample studied was restricted to these 12 products in order to ensure that the medicines considered are representative of those for which the SPC extension would be relevant[242]. The model specifically looked at how sales volumes and prices shift when a product loses patent protection and biosimilar versions enter the market. On this basis, the model made it possible to calculate how much additional profit accrues to the originator and how much additional cost is borne by the healthcare system and patients when an extra year of protection is added.

Expected impacts:

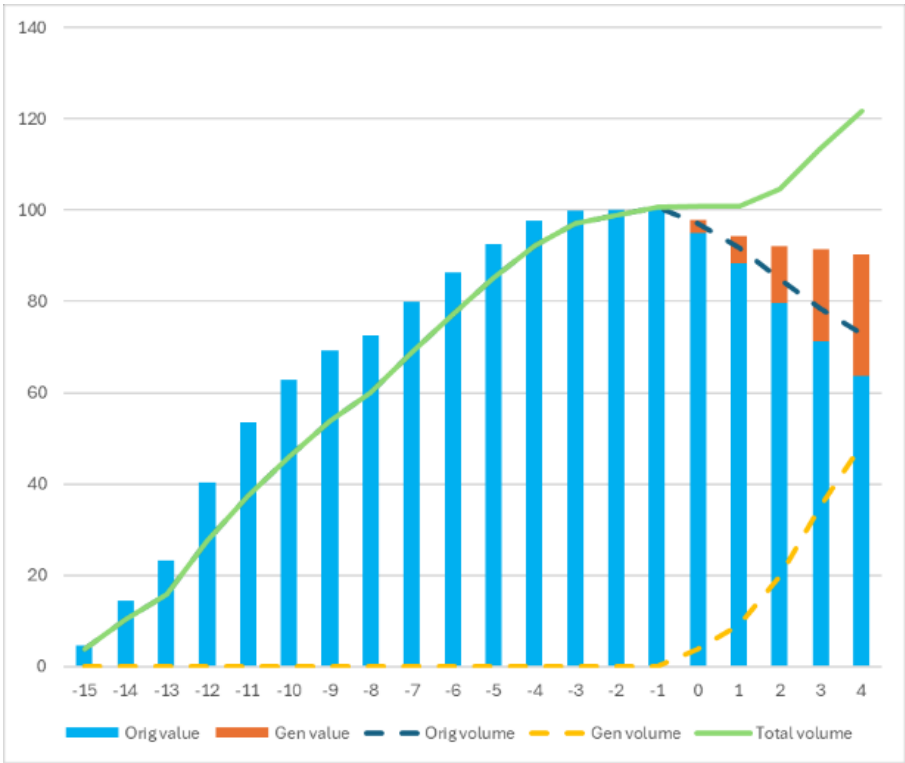
Figure 10 charts the course of revenue and volume for the average medicine in the sample across 20 years: 15 years before expiry of protection and 5 years after expiry. This represents our baseline in the absence of SPC extension. As biosimilar entry drives prices

general-pharmaceutical_en supports this statement, furthermore data analysed for the impact of the SPC extension measure confirm the same trend. Of the 31 biological products in the nine-year cohort studied, 12 had SPC as last protection to expire and 10 had RDP. While the SPC-reliant products averaged EUR 740 million during their last year of protection collectively, the 10 RDP-reliant medicines averaged EUR 83 million.

[242] To accurately measure economic impact, we shouldn't just count all eligible products. We must isolate those where the SPC extension is the sole factor preventing biosimilar entry (last layer of protection to expire). If other protections remain in place after the SPC expires, the extension has no real-world economic effect.

down, it is associated with an accelerated increase in volume, effectively allowing for a higher number of patients to be treated (green line). Because prices are lower, overall revenues fall, despite the higher volumes. This process is somewhat restrained in the case of biologics by the higher barriers to entry for biosimilars as compared to generics for small molecules, including due to stricter regulatory pathways and more complex production processes, resulting in a relatively shallow decrease in overall revenue.[243]

Figure 10. Normalised sales and volume for SPC-reliant biological medicines with current duration of SPC

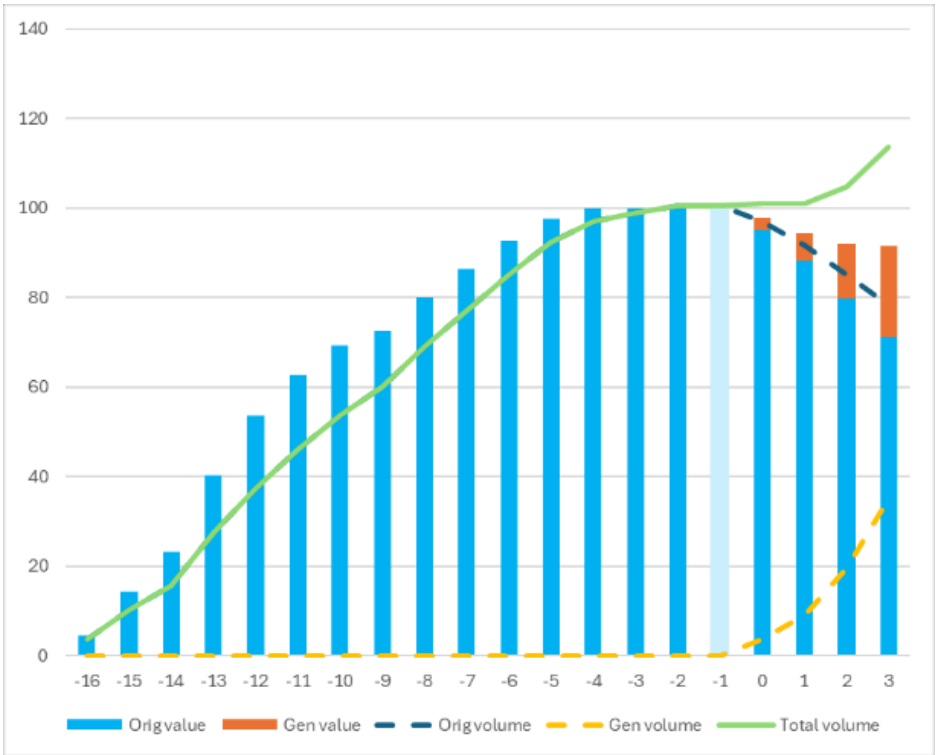


Author analysis based on IQVIA MIDAS data

To assess the impact of an additional year of protection, the same twenty-year reference period was maintained, but with an additional year of uncontested sales with revenues and volumes equal to those in the year before expiry and one year fewer of contested sales. The effect is depicted in Figure 11 (light blue bar). The result is a fall in the number of patients treated accompanied by an increase in spending.

[243] For the purposes of this graph, one medicine from the sample, with particular outlier characteristics was excluded. The product is a moderately high revenue medicine with no biosimilar entry even after several years and a far shorter lifecycle than all the other SPC-reliant biologicals in the sample.

Figure 11. Normalised sales and volume for SPC-reliant biological medicines products with a 12- months SPC extension



Author analysis based on IQVIA MIDAS data

To assess how many products per year would be eligible for the measure, data from EMA was analysed covering the period from 2011 to 2025. If the SPC extension existed, over the past ten years, there would have been on average **5 products per year** among all those centrally authorised that fulfil all the criteria to obtain the SPC extension, and **6** per year over the past 5 years, albeit with considerable variation per year. As stated above, out of the 31 biologics we analysed, 40% (i.e. 12 products), relied on SPC as their last effective protection[244]. Thus, assuming the proportion of products relying on SPC remains constant, the SPC extension would have an economic impact on **2-3 products per year**.

Conduct of business: The average revenue of a product in the sample in the year prior to protection expiry is EUR 740 million. Impacts in the model are calculated as a percentage of revenues in this last protected year. Taking into account the expected impact of the measure, as shown in Figure 11, an additional year of protection results in an additional direct cost to public payers in the EU of approximately EUR 70 million per medicine, corresponding to approximately EUR 210 million annually at aggregate level (considering three medicines per year). The relatively **moderate increase in spending** reflects the shallow post-expiry revenue decline characteristic of SPC-reliant biologicals.

[244] To accurately measure economic impact, we shouldn't just count all eligible products. We must isolate those where the SPC extension is the sole factor preventing biosimilar entry (last layer of protection to expire). If other protections remain in place after the SPC expires, the extension has no real-world economic effect.

Functioning of the internal market and competition: For the reasons set out above, biosimilar competition produces slower price reductions than generic competition, limiting the price differential between the extension scenario and the baseline. Instead, the main effect of the extension relative to the baseline is the temporary postponement of additional product entry, implying fewer available treatment options and delayed expansion of supply for patients during the extension period. By postponing competitive entry, the extension maintains higher average prices for an additional year and delays the expansion of supply associated with follow-on products.

Public health: In addition to the direct cost impact of approximately EUR 70 million per medicine, there is a delay in the expansion of coverage (the upward pivot of the green line is delayed by a year). This is expressed in a patient monetised loss of EUR 135 million per product and EUR 405 million for three products (see table below) calculated as the additional spending required to achieve baseline coverage at policy scenario prices. Additional details in Annex 7. Additional protection for originators results in a transfer of revenue and profit from biosimilar makers to originators as well as a transfer of economic surplus from payers and patients to the pharma industry, as set out in the table below[245].

Table 13. Impact of change of +1 SPC extension for biotechnology medicines

1 year increase in protection	Per medicine	Annual (3 meds)
Originator gross profit	**230 m**	**690 m**
Biosimilar gross profit	**-80 m**	**-240 m**
Cost to public payer	**70 m**	**210 m**
Patients monetised gains/losses	**135 m**	**405 m**
Patients + payer monetised gain/loss	**205 m**	**615 m**

***Source**: Author analysis based on IQVIA MIDAS data*[246]

Due to data availability and confidentiality around price negotiation, this calculation is based on list prices and cannot capture discounts and clawback mechanisms that apply at national pricing and reimbursement stage. If it were possible to use net prices, the cost calculation would be lower.

Competitiveness, trade and investment flows: According to EMA data from 2016 to 2025, in addition to the 5-6 products that would have met all the criteria each year, a further five products would have met all the criteria except the "geographic" ones, i.e. conducting trials in more than two Member States and having part of their manufacturing in the EU.

[245] As set out in Annex 11, this latter transfer can be approximated by the combined total of the monetised loss to patients and the additional costs to the public payer of EUR 205 million per medicine.
[246] Internal analysis by the authors using IQVIA MIDAS® quarterly sales data 2008-2024. Geographical coverage: EU27 without Cyprus, Malta and Denmark. which were obtained under license from IQVIA and reflect estimates of real-world activity. Copyright IQVIA. All rights reserved. The statements, findings, conclusions, views, and opinions contained and expressed herein are not necessarily those of IQVIA.

It was assumed again that about 40% of the products are SPC-reliant. Thus, the SPC extension could potentially be awarded to 4-5 products annually (rather than 2-3) if developers changed their behaviour in response to the incentive i.e. conduct clinical trials in more than one Member State and part of the production of the medicinal product in the EU.

This measure is designed to incentivise late-stage clinical trials as part of the product's marketing authorisation. Clinical trials generate sizeable economic benefits in several ways as outlined in the economic impact section of intervention n°4 (Clinical Trial Regulation Revision). A further potential benefit of more clinical trials is avoiding the healthcare expenditure which would normally be associated with sponsor-funded medicines, diagnostics and monitoring. As an illustrative benchmark, Polignano et al. (2022)[247] estimate a leverage ratio of **3.67**, implying **EUR 3.67 of avoided healthcare expenditure for every euro of sponsor investment** in industry-sponsored clinical trials. While the SPC extension conditionality of conducting clinical trials in the EU can only impact behaviour at the margin of a limited set of products, the combined impact with measures proposed in the Biotech Act on streamlining clinical trials can be non negligeable[248].

The measure is also designed to encourage sponsors to incorporate an EU-based manufacturing step as part of their manufacturing and scale-up strategies. As underscored by public consultations, literature and empirical evidence, intellectual property, including SPC extension, is an important part of the broader set of elements that drive biotechnology investment decisions. While the decision to invest in a biotechnology manufacturing facility cannot be attributed to any single product or regulatory incentive, similarly to the conditionality concerning the conduct of clinical trials in the EU, the positive impulse created by the SPC extension could contribute to broader investment and employment effects at the margin.

The effect of these measures is expected to be **amplified and completed by other measures** set out in the Biotech Act, especially those aimed at supporting and derisking investment in manufacturing in the EU (e.g. high impact biotechnology health strategic projects and biotechnology health strategic projects) and the amendment to the Clinical Trials Regulation to facilitate the conduct of multi-national clinical trials in the EU. These changes will make the incentive to conduct clinical trial and manufacturing activity to Europe introduced by the SPC extension more effective. It must also be noted that the SPC extension complements other measures in the Regulation proposal and in the reform of the general pharmaceutical legislation[249] that are aimed at facilitating development of innovative products and reducing time to market (e.g. use of AI and regulatory sandboxes, combined trials and shortening marketing authorisation timelines).

Research & Innovation: Between 2004 and 2011, just over one product per year would have been eligible for the SPC extension, according to EMA's data. This number grew to five in the last decade and to six in the last 5 years. While the long-term trend reflects a

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[247] Polignano, M. G., et.al. (2022). Economic impact of industry-sponsored clinical trials in inflammatory bowel diseases: Results from the national institute of gastroenterology "Saverio de Bellis". Frontiers in pharmacology, 13, 1027760. https://doi.org/10.3389/fphar.2022.1027760.

[248] Refer to the relevant section of the SWD on the Clinical Trials Regulation for a full assessment of costs and benefits

[249] COM/2023/193 final and COM/2023/192 final.

growing health biotech pipeline, we observed high variability in product eligibility year-over-year, reflecting the unique risk profile of the health biotech sector. With the **development of biotechnology pipelines** and scientific advances, we can expect this measure's economic impact to increase, but in parallel it is also expected to bring new transformative products to the market.

Administrative costs on businesses, including SMEs: Under the current regulatory framework, companies applying for SPCs incur **administrative costs** associated with preparing and submitting applications, assessing eligibility, and interacting with patent and regulatory authorities. SPC applications require legal interpretation of patent and marketing authorisation scope and prior use of products protected by the patent and the medicinal product which has been authorised. They require internal legal and regulatory staff time and, in many cases, recourse to external legal advisors. For SMEs, SPC-related administrative and advisory costs can weigh more heavily, given their more limited in-house legal and regulatory resources and greater dependence on outsourced expertise[250]. Evidence from the impact assessment of the Commission proposals for a unitary SPC[251] shows that overall administrative and advisory costs for filing SPC extensions across multiple Member States typically range between approximately EUR 80,000 and EUR 150,000 per product.

Public Authorities: The proposed SPC extension does not introduce new standalone administrative procedures beyond those already required for obtaining SPC protection under the baseline framework. Companies seeking to benefit from the SPC extension would follow the same application process as for a standard SPC, with the addition of the documentation provided by EMA demonstrating that the eligibility criteria for the extension are fulfilled. As these elements are largely based on information already provided during the marketing authorisation process and associated regulatory documentation, the additional administrative effort required from companies is expected to remain negligible. Therefore, overall costs would remain between EUR 80,000 and 150,000 per product.

The SPC system is subject to **disputes over eligibility, duration and scope**, which generate administrative and legal costs for companies, public authorities and courts[252].

The proposed SPC extension, by introducing novel eligibility criteria, especially in the early years of application might also entail the same litigation risks which currently characterise the existing legal framework with increased risk of administrative costs and burdens. This is because the generics manufacturers are well placed to contest any form of SPC extension. In addition, the Court of Justice interprets the conditions for grant narrowly

[250] European Commission (2023), Commission Staff Working Document – Impact Assessment accompanying the proposals on supplementary protection certificates, SWD (2023) 118 final, Brussels, 27 April 2023, Section 2.3.2 and Annex 6 (SME Test).
[251] European Commission (2023), Commission Staff Working Document – Impact Assessment accompanying the Proposal for a Regulation of the European Parliament and of the Council on the supplementary protection certificate for medicinal products (recast) and the Proposal for a Regulation on the unitary supplementary protection certificate for medicinal products, SWD (2023) 118 final, Brussels, 27 April 2023, Section 2.3.2 and Annex 5B.
[252] Technopolis Group (2018) Effects of supplementary protection mechanisms for pharmaceutical products. Final report, May 2018. Amsterdam/Vienna: Technopolis Group.

as it considers that all the interests at stake, including those of public health, in a sector as complex and sensitive as the pharmaceutical sector should be considered.

Conclusions

Providing an additional year of protection for innovative biotech medicines rewards the **delivery of transformative treatments** for high-burden conditions, ranging from oncology to metabolic and neurological disorders, while sending an important **signal to companies** regarding investment priorities. The geographical conditions ensure that this additional incentive also favours investment in the EU and helps strengthen the EU's health biotech sector, which is crucial both for European patients and for the competitiveness of one of Europe's most valuable industries.

Table 14. Summary assessment of the effects due to the policy measure

Policy interventions	COB	Admin	CTI	Int Mar	I&R	PA	H&S
SPC extension	++	0/-	+	-	+	0	-

5.2.4 Intervention n°14: Facilitating Access to Funding

The proposed Regulation establishes an **EU Health Biotechnology Investment Pilot** in order to mobilise substantial private investment into the sector. This is a partnership with the European Investment Bank and European Investment Funds (together the "EIB Group") and other implementing partners, bringing together equity instruments and venture-style debt tailored to biotechnology-specific risk profiles. In synergy with existing EU investment initiatives, the Pilot would support the full lifecycle of health biotechnology companies and projects. As early as 2026 and 2027, the EIB Group will aim to mobilise EUR 10 billion of total investment into the sector through the BioTechEU initiative.

Projects that contribute to an **EU late-stage Capital Booster Pilot** will be recognised by the Commission as high-impact health biotechnology strategic projects. These projects are expected to strengthen cross-border investment, mobilise long-term and institutional capital, improve investor access and issuer visibility, and enhance biotechnology-specific investment expertise.

More broadly, health biotechnology is recognised as a strategic technology eligible for Union and national financial support in line with applicable rules, including State aid rules. High-impact health biotechnology strategic projects may receive particular consideration under relevant Union programmes and may combine Union funding with financing from the EIB Group, national promotional banks and private investors, in line with applicable

rules[253]. These measures aim to address structural financing gaps, particularly at late development stages, and to crowd in private capital.

Expected impacts:

Over the last 10 years, health biotechnology was a strategic financing priority for the EIB Group, underpinned by close cooperation with the European Commission through EU budgetary resources[254] and complementary EIB Group own resources. Over the period 2015-2025, the Group has scaled its activity across the full financing chain, combining (i) EIF-backed investments into specialised venture capital and growth equity funds ('fund of funds' approach) to support more than 1000 health biotechnology projects and emerging startups with (ii) direct EIB loans to more than 145 later-stage innovative SMEs and midcaps ('venture loans') or (iii) established pharma companies ('corporate loans').

With all these investments, the EIB Group mobilised more than EUR 100 billion for the benefit of the health biotech sector over this period. Table 15 summarises this activity across instruments, including the EU budget guarantee backing those investments and how much funding the original EIB Group investment mobilised by crowding in private capital. Figures are reported as annualised run rates over the 2015-2025 period. **These are indicative averages only and should be interpreted with caution: actual annual volumes vary significantly from year to year, driven by market conditions, pipeline timing and the availability of EU budgetary guarantees.**

Table 15. Average annual EIB Group contribution to health biotech financing and investment mobilisation (2015–2025)

Instrument	EU guarantee/year	EIB Group Investment/year	Total Investment mobilised/year
Venture debt	EUR ~150 million/yr	EUR ~350-400 million	EUR ~3.1-3.5 billion
Corporate loans	EUR ~70 million/yr[255]	EUR ~730 million	EUR ~1.9 billion
EIF fund investments	EUR ~100 million/yr	EUR ~520 million	EUR ~5.5 billion
Total EIB Group	EUR ~320 million/yr	EUR ~1.6 billion	EUR ~10.8 billion

However, the EIB estimates that an annual investment gap of EUR 40 billion remains between the EU and US Biotech sectors[256]. To address this gap, the Biotech Act proposes the creation of a dedicated EU Health Biotech Investment Pilot. The pilot would deploy a toolbox of financial instruments, tailored to the specific risk and maturity profile of health

[253] Regulation (EU, Euratom) 2024/2509 of the European Parliament and of the Council of 23 September 2024 on the financial rules applicable to the general budget of the Union, OJ L, 2024/2509, 26.9.2024.
[254] InvestEU guarantee in the current MFF, EFSI and COSME-EFG in the previous MFF.
[255] Over the time period 2015-2025, out of the EUR 7 billion EIB corporate life-sciences loans (730/year), only EUR 2 billion (EUR 200 million/year) were backed by a guarantee of EUR 700 million (EUR 70 million/year). The remaining EUR 5 billion were financed from EIB own resources.
[256] The EIB Group uses industry data of EUR 69.7 billion investment for the US vs EUR 26.5 billion in the EU in 2021, resulting in a gap of ca. EUR 40 billion.

biotech companies. With the support of these instruments, the pilot aims to crowd in substantially more private capital and respond more effectively to unmet market needs across the financing chain.

Table 16 outlines three scenarios for the pilot across different ambition levels. The scenarios assume a positive review after an initial two-year pilot period, and initial evaluation of crowd-in ratios, after which it will continue for a total of seven years. A longer time horizon helps with attracting substantial private investment, particularly from institutional investors such as pension funds, insurers and corporates, and reflects the long investment and value-creation cycles characteristics of the health biotechnology sector. In addition, sustained and predictable public support is critical for investor confidence, while sufficient time is needed to deploy and test the different instruments in the toolbox and adjust the mix in response to market uptake, geopolitical shifts and observed outcomes. The first scenario assumes a continuation of the level of EIB Group's average investment level over the last 10 years, with the second and third illustrating the additional level of EC guarantees and investment by the pilot needed to mobilise sufficient investment to close, respectively, 25% or 50% of the EUR 40 billion annual investment gap in the Health Biotech sector.

These scenarios are illustrative sensitivity cases rather than forecasts and depend on strong assumptions: linear scalability of deployment by the investment pilot over seven years, with the same distribution of investment across instruments as the EIB Group over the last 10 years, sufficient absorption capacity in the EU health biotech pipeline, no material displacement of private finance and no binding implementation constraints (including risk appetite, deal flow, administrative throughput and the availability of EU budgetary guarantees).

Table 16. Scenarios for the EU Health Biotechnology Investment Pilot

Scenario	EC guarantee over 7 years	Direct investments by the pilot over 7 years	Total investment mobilised over 7 years
Continuation scenario	EUR 2.2 billion	EUR ~10.4 billion	EUR ~70 billion
Closing 25% of EU-US investment gap	EUR 4.5 billion	EUR ~20.7 billion	EUR ~140 billion
Closing 50% of EU-US investment gap	EUR 6.7 billion	EUR ~31.1billion	EUR ~210 billion

5.2.5 Intervention n°15: Use of Artificial Intelligence and Data

The Regulation proposal provides for the publication and regular update of **EMA guidance, in agreement with the Commission**, on the use of advanced technologies, including AI, in the lifecycle of medicinal products. This guidance is expected to establish overarching principles to enhance clarity on the use of advanced technologies across the lifecycle, including development, manufacturing, regulatory evaluation and approval, and post-authorisation monitoring. The proposal also provides for the recognition of high-impact health biotechnology strategic projects in the form of **trusted AI-enabled**

biotechnology testing environments (to facilitate integrated experimentation and translational validation) and **biotechnology data quality accelerators** to support the curation, standardisation and annotation of high-quality datasets required for the training, testing and validation of AI systems used in biotechnology applications.

Expected impacts:

The effects of the proposed measure are driven by several mechanisms:

- the establishment of common principles for the clarity of the use of AI across the medicinal product lifecycle, supporting consistent validation approaches and alignment of regulatory expectations;
- the complementing and structuring of case-by-case regulatory interactions through publicly available guidance, improving predictability and supporting the consistent deployment of AI across the medicinal product lifecycle;
- the provision of shared AI-enabled biotechnology testing environments combining experimental, computational and data-driven capabilities, reducing firm-level constraints and facilitating faster validation and scale-up of innovations;
- the improvement of data quality for AI applications, through the curation, annotation, standardisation and provenance of datasets to support the training, testing and validation of AI systems in biotechnology.

These mechanisms are interdependent and reinforce each other, with outcomes scaling with the number and maturity of recognised projects in the form of testing environments and data quality accelerators.

By combining improved regulatory clarity, enhanced validation infrastructure and higher-quality datasets, the measures are expected to help address key structural bottlenecks that currently limit the translation of Europe's scientific excellence into scalable innovation and thereby facilitate the translation of research into scalable innovation and contribute to strengthening the EU's competitiveness in AI-enabled biotechnology[257].

Uptake of high-impact projects (testing environments and data quality accelerators) is a key variable, and the scale of effects should be interpreted as proportional to the number, capacity and maturity of recognised projects. This reflects the project-based nature of the intervention and the reliance on public and private co-investment, which make different uptake scenarios possible.

Conduct of business: These impacts include less regulatory uncertainty and better access to validation and scale-up capacity. The guidance introduces a shift from case-by-case regulatory interactions to a publicly available reference point, replacing confidential and non-reusable exchanges and reducing transaction costs and internal deliberation time. This allows firms to take development decisions earlier and with greater confidence. Testing environments and data quality accelerators address the limitations in terms of access to shared pilot and scale-up infrastructure, with time-to-access effects being material. Under

[257] See further findings in the *Study to support the rapid assessment of policy scenarios to strengthen innovation and competitiveness in the field of biotechnology in the EU*, Rapid Assessment Scenario Study.

baseline conditions, firms must independently finance or secure validation capacity, which entails high fixed costs, long lead times and coordination challenges. Shared infrastructure converts firm-level capital expenditure into pooled capacity, reducing delays in accessing pilot-GMP capacity and limiting the risks of overall project delay. Providing access to such infrastructure operates as an enabling condition for commercialisation.

Administrative costs on businesses, including SMEs: These costs are expected to evolve in a balanced manner. While guidance can reduce repeated clarification processes and improve predictability, care may be needed to ensure sufficient flexibility in its application. Participation in testing environments and data quality accelerators may entail certain administrative steps, such as application, reporting and knowledge-sharing; however, these are expected to be proportionate and to support transparency, collaboration and the generation of shared evidence. Appropriate design of support frameworks, including streamlined procedures, can help facilitate participation and ensure that administrative processes remain aligned with the pace of technological development.

Competitiveness, trade and investment flows: These reflect the availability of validation infrastructure and AI-ready data. Testing environments contribute by addressing key R&D constraints, providing integrated capacity for validation and supporting development of such activities within the Union. Data accelerators contribute by reducing 'data friction', improving data usability, interoperability and quality, and thereby lowering costs associated with fragmented datasets. Both interventions require sustained capital and operational support to ensure effective utilisation and maximise their impact on innovation and investment decisions.

Functioning of the internal market and competition: Changes in data readiness and interoperability across Member States are expected, with fragmentation and heterogeneity in data readiness remaining significant. Legal access to data does not ensure technical usability, due to differences in formats, metadata and validation criteria. Data accelerators help enlarge the effective market for data-driven development by improving standardisation, curation and interoperability. At the same time, differences in absorptive capacity and digital maturity across Member States may lead to uneven uptake in certain instances. This underlines the importance of ensuring coordinated implementation over time, so that these measures support convergence and avoid the emergence of a multi-speed internal market.

Innovation and research: Impacts are expected to reflect changes in regulatory uncertainty, validation capacity and data usability. The guidance helps reduce the 'option value of waiting', thereby accelerating investment decisions in R&D. Testing environments contribute through addressing limitations in access to integrated validation capacity, enabling validation activities under more efficient and coordinated conditions, and supporting improved validation timelines, regulatory submissions and standardisation, including platform effects. Data quality accelerators contribute through improving data quality and reducing dataset fragmentation, and interoperability, reducing the data preparation burden, generating research productivity effects, and enabling discoveries that fragmented datasets cannot support.

Public authorities: These stakeholders may need to support supervision, coordination, infrastructure maintenance and the development of specialised human capital in relation to

testing environments and data quality accelerators. This includes ensuring appropriate technical capacities and skills of their staff, as well as the ability to adapt to evolving technological developments. Data quality accelerators require sustained investment in high-quality data curation and annotation capacities. This highlights the importance of ensuring continuity and coordination of funding mechanisms to support their long-term operation and maximise their impact.

Public health and safety reflect changes in the validation of AI-enabled biomedical tools prior to deployment. Testing environments contribute by reducing the real-world performance gap, improving reliability through structured multi-system validation, and supporting the use of more diverse datasets. In combination with the guidance, this supports the consistent application of validation and risk management principles across the lifecycle of AI-enabled medicinal products. These developments align with evolving regulatory validation frameworks and support safer deployment of AI-enabled tools.

Table 17. Summary assessment of the effects of Intervention n°15: Use of Artificial Intelligence and Data

Policy intervention	COB	Admin	CTI	Int Mar	I&R	PA	H&S
Use of Artificial Intelligence and Data	++	0/+	++	+	++	0/-	+

Scenario-based quantification of the expected impacts under different uptake cases has also been developed[258] to illustrate such potential impacts, which should be interpreted as proportional to the scale, maturity and utilisation of recognised projects.

Table 18. Illustrative uptake scenarios and order-of-magnitude impacts and estimation of associated impacts

Impact Category	Low uptake	Medium uptake	High uptake
What this scenario entails	2–3 recognised testing environments (Art. 32) and 2–3 data quality accelerators (Art. 33); upgrade-focused investment in existing facilities; primarily public funding with limited private co-investment	5–8 recognised testing environments and 5–8 data quality accelerators; mix of upgrades and new greenfield investments; mixed public-private funding on PPP model	10–15 recognised testing environments and 10–15 data quality accelerators; industrial-scale greenfield and upgraded facilities; public-private leverage at the largest nodes
Direct employment created	160–420 FTE (testing environments + data accelerators)	1,000–2,640 FTE	3,300–7,500 FTE
Companies and data users served per year	120–495 (20–45 companies; 100–450 data users)	1,100–3,400 (100–200 companies; 1,000–3,200 data users)	4,250–12,600 (250–600 companies; 4,000–12,000 data users)

[258] See Annex 7 and relevant sections of the Rapid Assessment Study – forthcoming – for further details.

Total infrastructure investment (public and private CAPEX combined)	EUR 85 million –EUR 205 million	EUR 650 million– EUR 1.3 billion	EUR 2.4 billion–EUR 7.2 billion
Annual operational funding required	EUR 6–18 million/year (data accelerators) + testing environment OPEX	EUR 40–120 million/year + testing environment OPEX	EUR 150–450 million/year + testing environment OPEX
EU-level supervisory burden	1–3 FTE	2.5–8 FTE	5–15 FTE
Regulatory interaction cost avoided per firm (Article 31)	Reduction in regulatory interaction costs and timelines through publicly available guidance; effects consistent across scenarios	same across all scenarios	same across all scenarios
SME infrastructure barrier addressed	82% of SMEs currently unable to self-finance validation; shared access reduces per-firm fixed costs	same direction; scales with number of environments	same direction; scales with number of environments
Data usability gap addressed	87% of biomedical datasets currently not readily usable for ML; accelerators begin conversion to AI-ready	scales with number of accelerators	scales with number of accelerators
Key implementation risk	Limited number of environments and accelerators reduces coverage and competitiveness signal	Need to ensure efficient access conditions and interoperability alignment; OPEX sustainability	Need for continuous technological upgrading and balanced. access conditions across users

5.2.6 Intervention n°16: Prevention of biotechnology misuse

The proposed Regulation sets out a regulatory framework for customer screening of biotechnology products of concern, focused on custom-made and potentially dangerous sequences of synthetic DNA or RNA (synthetic nucleic acids, synthetic 'NA'). Custom NA synthesis is a small but crucial part of the biotech sector, representing around 0.1% of the European Biotech market[259]. The EU is home to around 100 companies in scope of the Chapter's provisions (ca. 20% of global NA synthesis companies) [260], with companies outside the EU selling custom nucleic acids to the Union market also in scope.

[259] Estimations based on GrandViewResearch estimates of the overall EU DNA synthesis market https://www.grandviewresearch.com/horizon/outlook/dna-synthesis-market/europe and EU Biotech market https://www.grandviewresearch.com/horizon/outlook/biotechnology-market/europe, assuming 50% of the overall EU DNA synthesis market (0.2% of the Biotech sector) is custom DNA synthesis, based on the number of companies – see footnote 260 and Annex 4 *Additional methodological information on specific measures* for details.

[260] IBBIS has identified 215 companies in the NA synthesis market in the EU, of which 66 companies performing custom synthesis, including oligos and data storage. The EU also has 4 benchtop manufacturers and 40 third-party vendors. The

The exponential decrease in the cost of synthesising DNA and RNA has transformed biotechnology and made innovation more accessible. However, it has also increased the risk of misuse. A particular concern is that, given information on the genomes of viruses and other pathogens is publicly available, access to the physical DNA or RNA is an increasingly eroding chokepoint to create and weaponise pathogens to carry out biological attacks. **These could have consequences similar to, or worse than, those seen during the Covid-19 pandemic**, with economic costs of EUR 20 trillion for the EU alone. In 2024, MIT researchers demonstrated the ability to order synthetic genetic fragments sufficient to reconstruct the 1918 pandemic influenza virus, with their order fulfilled in almost all attempts[261].

Williams et al. (2025) estimate the near-future annual probability of biological attacks at 1% and accidents from their use at 1.5%. In recent years, multiple individuals in the EU attempted to carry out biological attacks by weaponising biological agents[262]. As synthetic NA continues to become cheaper and more widely available, this increases the risk that future biological attacks could be much more damaging, using transmissible viruses instead of toxins.

Table 19. Estimated risk from accidents with or misuse of Synthetic Nucleic Acids[263]

Type of events	Annual likelihood	Expected monetised harm to EU if event occurs
Large scale event[264]	1%	EUR 19.6 trillion
Small scale event[265]	22%	EUR 72 million
Agricultural event	4%	EUR 75.5 billion
Non-transmissible events	5-25%	EUR 121 million

To reduce this risk, the Biotech Act introduces a mandate for custom NA synthesis companies to verify the identity and legitimacy of customers purchasing potentially dangerous NA sequences. Companies that make benchtop NA synthesis devices available would also be obligated to implement a screening mechanism to prevent the synthesis of biotechnology products of concern with the device.

In the Biotech Act Call for Evidence[266], several industry representatives were supportive of such harmonised rules on NA synthesis screening at EU-level, given the significant

share of EU synthetic DNA providers is calculated following IBBIS number, namely 215 over 1,023 globally identified providers.

[261] Esvelt K. (2024), https://drive.google.com/file/d/1hNUnU8i2yubt5uesmmV17aTJXhYYDgTY/edit?pli=1

[262] https://jamestown.org/ricins-round-two-germany-prevents-another-islamic-state-motivated-bioterrorism-attack/, https://www.dw.com/en/germany-teens-home-searched-over-suspected-ricin-plot/a-72272711, https://health.ec.europa.eu/document/download/1be3e462-bd50-4c64-b147-add9d1d5a580_en?filename=com_2025-529-1_act_en.pdf on page 5.

[263] RAND 2026.

[264] Large-spreading events, global pandemics on the scale of COVID-19 or the 1918 influenza

[265] Expected to result in 65 deaths in case the event happens.

[266] https://ec.europa.eu/info/law/better-regulation/have-your-say/initiatives/14627-Biotech-Act/F3565340_en

heterogeneity in security practices[267] and the difficulty in complying with diverging rules across Member States[268]. Customer screening for potentially dangerous NA synthesis orders is frequent industry practice, with companies representing over 50% of global NA synthesis capacity being members of the International Gene Synthesis Consortium[269], an industry association that supports its members to follow its harmonised screening protocol.

However, companies implementing such biosecurity measures risk being undercut on price and turnaround time by providers without such measures. While the cost of screening for companies is currently minimal, as the cost of NA synthesis will continue to decline, the 'fixed' cost of screening could represent an increasing share of the final order price[270], further worsening this dynamic.

The provisions in the Biotech Act would aid to prevent such a 'race to the bottom' on biosecurity practices and enable competition by removing burden for companies that screen[271]. The RAND study assumes that a mandate would result in companies screening 98% of EU synthesis orders, while also indirectly increasing screening rates for non-EU orders by around 10%. **This is modelled to reduce misuse risk by 35% and accident risk by 86%[272]. This risk reduction would result in expected annual monetised benefits[273] of EUR 2.9 billion in 2027, increasing to EUR 5.5 billion in 2036.** Around EUR 1.2 billion of the benefits in 2027 stem from accident prevention, while around EUR 1.8 billion stem from attack prevention. This risk reduction would indirectly also benefit the biotech sector itself, avoiding severe regulatory backlash or a research pause as a consequence of such an event, which RAND estimates could represent EUR 10 billion in lost future research value.

These benefits significantly outweigh the estimated costs of the measure. Screening measures represent a minor cost for NA synthesis companies. In a Commission workshop on biosecurity[274], an EU NA synthesis SME who screens all their orders and customers highlighted how screening is practical and efficient for them, not hindering innovation or commercial timelines. Industry interviews suggest that SMEs of 40-50 staff dedicate around 0.5-1 FTE to biosecurity screening measures[275]. For cost calculations, we assume 30% of the ca. 100 custom NA synthesis companies in the EU already have customer

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[267] https://pmc.ncbi.nlm.nih.gov/articles/PMC11319849/

[268] See IBBIS mapping of explicit and implicit rules on NA synthesis screening across countries: https://globalsynthesismap.bio/policy?country=FRA.

[269] Harmonized screening protocol v2.0 (2017). International Gene Synthesis Corporation.

[270] If not matched by a similar decrease in the cost of screening with advancements in AI, as investigated in Acelas *et al.* (2026) cited below.

[271] https://www.weforum.org/publications/biosecurity-innovation-and-risk-reduction-a-global-framework-for-accessible-safe-and-secure-dna-synthesis-582d582cd4/

[272] Zakaria, S. *et al.* (2026). Cost–benefit analysis for synthetic nucleic acid screening in the European Union. Santa Monica, CA: RAND Corporation, 2026. https://www.rand.org/pubs/research_reports/RRA4805-1.html; The analysis is conducted over **ten years** and assumes a mandatory screening policy for all synthetic nucleic acids longer **than 50bp** bought and sold in the EU.

[273] The benefits quantify the reduction in the probability of biological events, such as another pandemic or an agro-terrorism attack, and the resulting reduction in expected public health and economic costs.

[274] Held on the 18th February 2026 in the context of stakeholder consultations for the Rapid Assessment Scenario Study (forthcoming).

[275] Evidence from SME expert interviews.

screening policies in place[276]. We estimate the direct costs for the remaining 70% of EU companies in scope to hire an additional biosecurity FTE, implement IT tools to screen sequence orders[277] and direct compliance costs for the regulatory introduction and adjustment efforts are estimated at EUR 10.5 million annually, compared to a market size of roughly EUR 500 million[278].

The RAND Europe study estimates costs on a per-order rather than per-company basis, taking the full NA synthesis market as a basis. Adjusting their model to only cover the custom NA synthesis market in scope of the regulation and EU wages, direct legitimacy screening costs are estimated at EUR 22.2 million[279]. However, per-order costs are expected to significantly decrease with AI tools becoming more powerful and more widely available, with Acelas *et al.* (2026) estimating their cost to decline from EUR 1 to EUR 0.20 per customer as the verification step becomes increasingly automated[280].

For researchers and other customers, mandatory screening introduces additional administrative costs linked to documentation, procurement adjustments, and compliance procedures. For custom NA synthesis orders, these costs are estimated at approximately EUR 16.4 million annually. Public sector implementation costs should remain relatively small at around EUR 15 million (for EU and MS together), as the provisions do not rely on a licensing regime and costs for both the EU and Member States would be mostly limited to monitoring and inspection activities.

Table 20. Annual direct costs of screening measures[281]

EU enforcement cost	EUR 1.3 million
MS enforcement cost	EUR 14 million
Direct costs to companies	EUR 10.5 - 22.2 million
Direct cost to customers	EUR 16.4 million
Total	**EUR 42.2 – 53.9 million**

[276] Estimation based on the average between minimum (15%) and maximum (45%) values of the proportion of EU gene companies that voluntarily screen now, from the RAND analysis. The number is also in line with the global results of the survey conducted by Sentinel Bio on major screening tool providers and Response to the European Biotech Act - Call for Evidence. See details on methodology in Annex 4 *Additional methodological information on specific measures* for Intervention No 16.
[277] Assumed to be relatively moderate at EUR 1.5 million for all companies, given the availability of free tools to screen for sequences of concern: https://github.com/ibbis-bio/common-mechanism; and Response Call for Evidence for EU Biotech Act.
[278] Estimations based on https://www.grandviewresearch.com/horizon/outlook/dna-synthesis-market/europe. Assuming 50% of the overall DNA synthesis market is custom NA synthesis.
[279] Estimations based on RAND analysis, but adjusted for EU average labour costs, number of custom providers, and orders by the share of the market belonging only to custom providers (110 custom providers/215 total companies in the synthetic DNA= 0.51).
[280] Acelas, A., Palya, H., Flyangolts, K., Fady, P. E., & Nelson, C. (2026). Evaluating AI-Assisted Customer Verification for Synthetic Nucleic Acid Screening. *bioRxiv*. https://www.biorxiv.org/content/10.64898/2026.02.27.708645v1?ct=
[281] Calculation based on 2027 numbers. Costs may rise in the long run as the market also continues growing.

In addition to these direct costs, a screening mandate may also result in different types of indirect costs which are more difficult to estimate. It might produce a "chilling effect" on research activities if some organisations delay or abandon projects due to concerns about delays or rejected orders. RAND Europe estimates potential research losses at EUR 97 million annually under very conservative assumptions. It assumes around 10% of researchers shifting to in-house synthesis, which carries significantly higher costs per order. In addition, introducing an additional regulation, even if not very costly to follow, may result in productivity loss for custom NA synthesis companies due to higher 'regulatory intensity', estimated at EUR 12.3 – 24.8 million.

Table 21. Comparing annual costs and benefits

Estimated benefits	EUR 2,900 million
Estimated costs (conservative estimate[282])	EUR 176 million

Even taking those conservative estimates of indirect costs into account, **the estimated benefits are 16 times higher than estimated costs.** This reflects the expected reduction in the probability of a biological attack with catastrophic public health and economic consequences, achievable through a targeted intervention with minimal costs for businesses and innovation.

5.2.7 Intervention n°17: Biodefence

The proposed Biotech Act establishes two categories of high-impact strategic projects for biodefence. Article 42 covers biodefence capability projects with a broad mandate, supporting activities from misuse prevention to surge capacity for diagnostics and countermeasures. Article 41 creates a more targeted instrument: the EU Biothreat Radar, focused on detection, characterisation, identification, analysis and assessment of biological threats, including novel, unknown and engineered pathogens, and on pathogen-agnostic cross-border surveillance.

These measures respond to a deteriorating threat environment. The ongoing Russia invasion on Ukraine[283] and war in Iran has raised concerns about the risk of pathogen release from biological weapons programmes[284], while advances in AI and biological engineering lower barriers to designing or modifying pathogens[285]. The assessment of impacts focuses on the EU Biothreat Radar, which provides a sufficiently detailed project scope for structured impact analysis.

Expected impact:

[282] Taking the upper bound of all cost estimates and including the relatively high RAND estimate of indirect costs.
[283] Russia has expanded site of past bioweapons research, satellite images show - Washington Post.
[284] Nelson, C., The Threat No One is Talking About in Iran, RUSI Commentary, 23 March 2026.
[285] Williams *et al.* (2025), Forecasting LLM-Enabled Biorisk and the Efficacy of Safeguards, Research Forecasting Institute.

Deploying a surveillance system geared towards novel and engineered pathogens would represent a significant change from current systems focused on surveillance of known communicable diseases, with significant upfront and operational costs. By building on existing **EU surveillance capacities and initiatives**, ECDC estimates that coordinating Biothreat Radar activities at EU-level over seven years would require an operational budget of EUR 25 million for coordination, governance, and information-sharing platforms and 10 FTEs for the full period. A possible EU Reference Laboratory for Biothreat Security is estimated to cost EUR 20 million for the 7-year period.

The larger cost lies in updating and integrating existing **surveillance infrastructure**: syndromic, clinical, laboratory and environmental surveillance combined with metagenomic sequencing. The Biotech rapid assessment scenario study uses a model by SecureBio Detection (2025)[286] for a US pathogen surveillance system[287] at ~USD 52 million/year as a basis. Adapting this to the EU[288] yields two scenarios: Scenario 1 scales proportionally to EU transport and wastewater networks as complementary surveillance activities (~EUR 46.5 million/year), while Scenario 2 expands environmental collection and sequencing capacity as investing in traditional public health surveillance (~EUR 110 million/year).

Table 22. Estimated annual costs of pathogen surveillance systems

Cost component	US model (EUR)	EU Scenario 1 (EUR)	EU Scenario 2 (EUR)
Traveller genomic surveillance	19,516,259	22,635,734	7,543,733
Sequencing, processing & analysis	23,967,648	23,470,428	101,883,439
Environmental detection	425,610	416,374	832,748
Total (annual)	**43,909,518**	**46,522,536**	**110,259,919**

Traveller Genomic Surveillance (aircraft wastewater + nasal swabs). Sequencing/processing covers metagenomic sequencing, bioinformatics and analysis. Scenario 1 scales the US model proportionally. Scenario 2 expands environmental sampling and sequencing while discontinuing passenger swabbing. US figures from SecureBio Detection (2025) converted to EUR.

Benefits are harder to quantify but potentially substantial. Despite gaps in EU-wide standardised evaluation frameworks analysing the benefits of surveillance, studies exist that have quantified the number of infections prevented, deaths avoided, and costs saved in specific hospital and outbreak settings[289]. The advantages of early detection (through modelling) and the impact of infection control interventions informed by pathogen agnostic sequencing have been documented. However, whereas genomic and epidemiological surveillance are likely to deliver greater real-world value, they are much

[286]SecureBio Detection (2025), Scaling US Pathogen Detection. See details on methodology in Annex 4 *Additional methodological information on specific measures* for Intervention No 17.
[287]Based on swabs of airport travellers, municipal monitoring and aircraft wastewater analysis.
[288]See Methodology Annex for scenario descriptions and cost calculations.
[289] Nascimento de Lima, P. *et al.* (2024), The value of environmental surveillance for pandemic response, Scientific Reports, 14, 28935.

harder to measure in economic terms. On the other hand, environmental surveillance is population and ecosystem-wide by design, produces early signals and is easy to plug into economic models. There are thus more quantified studies available proving the value of this type of surveillance.

Empirical evidence from European wastewater surveillance studies documents lead times of 5 to 19 days for SARS-CoV-2 detection relative to clinical identification.[290] Modelling by Nascimento de Lima *et al.* (2024) shows that an environmental surveillance system providing a five-day early warning relative to clinical detection could reduce deaths from 149 to 134 per 100,000, lower illness costs from ~EUR 1,271 to ~EUR 1,118 per person, and shorten lockdowns from 121 to 89 days in the first year of a COVID-19-type pandemic.[291]

Set against the broader costs of pandemics, these gains are substantial. Global viral epidemics cause estimated annual mortality of ~3.3 million people[292], and COVID-19 alone caused economic losses in the trillions across the EU. For a pathogen-agnostic system capable of detecting novel and engineered threats, the marginal benefit could be substantially higher, as limited surveillance currently exists for novel threats.

Beyond public health, the Biothreat Radar would generate indirect gains in research capacity and technological sovereignty, through investments in metagenomic infrastructure, bioinformatics pipelines and EU-based data sharing via the European Nucleotide Archive.

6 CUMULATIVE ECONOMIC, SOCIAL, ENVIRONMENTAL AND OTHER IMPACTS OF THE PROPOSAL

While many of the measures included in the proposed Biotech Act are technical and targeted in nature, their combined effect has the potential to be transformative. Together, they create a framework for competitiveness where regulatory simplification, investment mobilisation and innovation all support and reinforce one another.

This interaction is expected to generate impacts that are materially greater than the sum of each measure assessed in isolation, because it addresses the key structural bottlenecks affecting the EU biotechnology sector simultaneously. The measures also ensure that the sector contributes more effectively to societal objectives, such as economic growth, job creation and improved health outcomes.

The proposed Biotech Act's impacts materialise across four interconnected areas. The first three: **regulatory simplification**, **competitiveness and investment attractiveness**, and **innovation and research**, capture the primary economic impacts. They also generate indirect social impacts through employment creation, skills development and better access to innovative treatments driven by faster innovation. They address, respectively, the cost of operating within the EU regulatory environment, the EU's capacity to retain and attract

[290] ECDC (2025), Framework for integration of wastewater-based surveillance; Viviani *et al.* (2025).
[291] Nascimento de Lima, P. *et al.* (2024), The value of environmental surveillance for pandemic response, Scientific Reports, 14, 28935.
[292] Bernstein, A. S. *et al.* (2022), The costs and benefits of primary prevention of zoonotic pandemics, Science Advances, 8(5).

productive capital, and the conditions needed to translate scientific excellence into marketable products. These areas are causally sequenced: regulatory simplification lowers the cost base, which strengthens the investment proposition, which in turn accelerates innovation throughput. The fourth area, **public health and safety**, captures the Act's ultimate societal return in terms of health outcomes, safety and resilience, complemented with an assessment of the expected **environmental impact**. The combined effects of the preceding areas materialise in benefits such as earlier patient access, supply resilience, and proactive biosecurity governance. The following assessment draws on 17 intervention-level assessments of impacts presented above, synthesising quantitative estimates and directional findings into a coherent account of the Act's expected effects. The estimates presented below should be interpreted in light of several **cross-cutting** factors that may influence their magnitude, while not changing the overall direction of the effects identified. First, the magnitude of investment mobilisation critically depends on the availability of EU budgetary guarantees under the next MFF, with estimates for the EU Health Biotechnology Investment Pilot and strategic projects frameworks assuming sufficient budgetary headroom. Second, the realisation of benefits depends on consistent implementation by Member States, particularly for clinical trials, the organ processing authorisation regime, strategic project permitting, and biosecurity screening, among others. Third, uptake-dependent measures, such as strategic and high-impact project recognition, regulatory sandboxes, and the AI and data framework, are presented under illustrative scenarios (low, medium, high). Actual impacts will be determined by the number, quality and maturity of projects. Finally, estimates assume broadly stable global market conditions; significant shifts in competitor jurisdictions' regulatory frameworks could alter the EU's relative competitive position.

6.1 Regulatory simplification and administrative burden

The Biotech Act delivers its most immediate effects in two distinct but mutually reinforcing ways: by eliminating procedural duplication across intersecting regulatory frameworks, and by shortening authorisation timelines. Where the Act removes entire procedural layers, it also shortens the remaining ones. Because these savings compound over multiple applications, modifications, and jurisdictions, the cumulative reduction across a full clinical development programme is substantially larger than any individual measure assessed alone[293]. These effects complement other simplification efforts in related Union frameworks. For instance, the pharmaceutical and medical devices legislation, in combination with the Biotech Act, are expected to generate significant simplification effects for the biotechnology sector[294].

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[293] The cumulative programme-level impact results from a combination of a) measure stacking within a single application (e.g., a single clinical trial application can benefit from several reforms simultaneously); and b) lifecycle accumulation (i.e., the same per-application saving recurs across many transactions and jurisdictions in one clinical development programme.

[294] The indicated complimentary simplification effect stems for example from: a) the revised Pharma Package, which has already transferred ERA for investigational medicinal products from the GMO legislation to the CTR, and which the Biotech Act builds directly on by introducing risk-proportionate ERA derogations for specified categories of low-risk GMO-ATMPs; b) the Commission's proposal of 16 December 2025 on the MDR and IVDR, which the Biotech Act's combined-studies measure complements by replacing parallel CTR-MDR/IVDR tracks with a single coordinated assessment; and c) for novel biotechnology products that currently span pharmaceutical, medical devices, and SoHO frameworks simultaneously, the Biotech Act's regulatory status repository and Foresight Panel resolve cross-framework classification ambiguity that neither the pharmaceutical nor the medical devices reforms can address in isolation.

Significant burden reduction is expected in **clinical development**, one of the most cost-intensive stages of the biotech value chain[295]. For **human medicinal products**, the **proposed amendments to the CTR** are expected to reduce authorisation timelines from 106 to 75 days (47 without a request for information) and to simplify and streamline the authorisation procedure, as well as the conduct of clinical trials. Key improvements include a new single assessment procedure for combined studies involving medicinal products and medical devices or in-vitro diagnostics, a new single investigational product core dossier, and the possibility of submitting applications for substantial modifications to the clinical trial in parallel after the notification of the authorisation decision. It is expected to yield direct cost savings in EU sponsor expenditure of approximately 5% (EUR 1.5-3.1 billion per year). Indirect savings of a similar order are anticipated through shorter time-dependent costs, yielding aggregate **annual financial gains for sponsors potentially exceeding EUR 5 billion**. Applying the Standard Cost Model (SCM), each multinational trial stands to save approximately EUR 2,700-4,500 in sponsor administrative costs through alone a reduction in the staff time for regulatory interactions by 15-25%. Additionally, electronic submission and digitalisation measures are estimated to generate EUR 112-225 million in industry savings over fifteen years. A simplified version of the cost-benefit analysis, considering a subset of the policy measures included in the Biotech Act, suggests an overall annual cost-saving of EUR 1.24 billion[296]. The Biotech Act proposal is expected to result in an increase in commercial clinical trials by 10% to 30%, estimated to generate approximately **EUR 3.6 billion to 10.7 billion in economic gains and 16,500 to 49,500 additional jobs.**

These gains extend into adjacent product domains, where parallel reforms are addressing sources of procedural duplication there. In the **biosimilars** domain, the rationalisation of Comparative Efficacy Studies requirements removes EUR 19-26 million in direct trial costs per product and shortens development timelines by 12-24 months, translating into sector-level savings of EUR 222-467 million annually for the 12-18 affected MAAs per year. Thus, **combined annual regulatory cost relief for health biotech sponsors across clinical trial and biosimilar reforms approach (and potentially exceed) EUR 5.2-5.5 billion per year.** Regarding **ATMPs**, eliminating the additional 50-day assessment period and granting risk-proportionate ERA exemptions for qualifying GMO-ATMPs is expected to reduce regulatory timelines and remove non-value-adding procedural steps, potentially removing a burden of 0.15-0.3 FTE-years per clinical trial application, a reduction that disproportionately benefits the SME-dominated ATMP developer base, and is also expected to significantly influence sponsors' decisions to conduct clinical trials in Europe.

For **VMPs** containing GMOs, the single regulatory pathway eliminates 30-90 days of process delay per CTA, aggregating to 240-1,350 product-days of delay removed annually at current pipeline volumes, with cumulative administrative cost savings of EUR 5-14.5 million till 2040. The uniform handling regime for variations not requiring assessment adds a further EUR 22.5 million in present-value savings over the same period.

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[295] Farid et al. (2020) Benchmarking biopharmaceutical process development and manufacturing cost contributions to R&D - PMC.

[296] See *Regulatory Framework Study (forthcoming).* The simplified costs-benefit analysis focus on operational costs, not considering costs arising from transitioning to new regulatory requirements.

Beyond clinical development, the Act addresses the **regulatory fragmentation** that so often compounds costs. Divergent national practices in risk classification, product categorisation, and procedural interpretation currently force applicants to navigate several different Member State-specific requirements for what is nominally the same regulatory procedure. The Act responds by establishing shared infrastructure, harmonising regulatory pathways, reducing legal ambiguities, and offering regulatory and procedural guidance.

Within this context, the Act's benefits are structurally tilted toward **SMEs and start-ups**, for whom the reforms remove barriers to participation rather than merely reducing costs of ongoing operations. This happens in two distinct ways. First, regulatory simplification measures reduce fixed-cost compliance burdens (e.g., legal support, regulatory affairs staffing, dossier preparation, external consultancy, multi-jurisdictional coordination) that are largely invariant to firm size and therefore represent a substantially higher share of total expenditure for smaller firms. This is particularly pronounced in ATMPs, where SMEs constitute the majority of developers, and in GMMs, where SMEs and start-ups represent the predominant developer base. Second, the Act addresses infrastructure and data access barriers that structurally exclude smaller firms from the innovation pipeline, most pronouncedly in the AI and data domain.

These regulatory simplification gains for businesses come with new **governance obligations for public authorities and Member States**. The Act introduces an expanded public sector role in certain areas, which includes new coordination and project permitting responsibilities, more clinical trial governance and enforcement, biosecurity screening and inspection obligations, and ecosystem coordination through the Steering Group and Support Network. The resulting costs are expected to be front-loaded with activities such as designating competent authorities, establishing or adapting single points of contact, resource permitting functions, and developing sector-specific expertise. However, the Act’s provisions are also designed in a way that limits the incremental burden to the extent possible by designating or adapting existing administrative coordination mechanisms rather than creating entirely new institutions. Over time, efficiency gains are expected as procedures standardise, digital handling matures, and coordination platforms reduce the unit cost of multi-authority interactions, partially offsetting the initial burden. Thus, the net balance is structurally positive: by removing procedural layers, the Act generates savings for businesses and healthcare systems that substantially exceed the incremental institutional costs and administrative burdens borne by public authorities.

Aggregated across the full package of simplification interventions, the Act delivers **direct annual cost savings** for businesses in the order of **EUR 5.2-5.5 billion**, driven overwhelmingly by the clinical trials reform and biosimilars CES rationalisation, and supplemented by cumulative electronic-submission savings and VMP-related administrative and variation savings. Under the One-In-One-Out (OIOO) accounting, new administrative burdens on businesses of approximately EUR 16.6 million/year (dominated by biosecurity customer-side compliance) are partially offset by removed burdens totalling approximately EUR 4.3-8.0 million/year, yielding a **net administrative increase** of approximately **EUR 8.6-12.4 million/year**. In parallel, **adjustment costs on businesses** amount to **EUR 8.16-27.32 million/year**, with a further **EUR 48.6-112.6 million/year** in recurrent monetised costs falling on public administrations, mainly stemming from biodefence collection infrastructure (EUR 46-110 million/year).

Even at the upper bound of identified incremental costs, and before accounting for the Act's downstream health, supply-resilience and innovation-enabling benefits, the **direct business-level savings from regulatory simplification far exceed the full sum of monetised countervailing items**, confirming that, in cumulative terms, the Biotech Act operates as a substantial **net simplification instrument** for the EU biotechnology sector, with the residual OIOO increase concentrated in a single, security-motivated intervention whose justification rests on a distinct benefit-side case (biosecurity risk prevention monetised at EUR 2.9 billion/year in 2027, rising to EUR 5.5 billion/year by 2036).

Distributional impacts

While the aggregate cost-benefit balance of the package is firmly positive, the Act produces material intra-EU transfers whose direction warrants explicit recognition (see Annex 3). The principal redistributive vector is the **SPC extension**, which is explicitly designed to transfer approximately EUR 690 million/year in additional originator gross profit from biosimilar developers (around EUR 240 million/year in foregone profits) and from payers and patients (combined social cost of around EUR 615 million/year), in exchange for conditional EU-based manufacturing and multi-Member State trial commitments. A second, structural transfer runs **across firm size**: because fixed compliance costs are largely size-invariant, simplification gains in clinical trials, ATMPs, GMMs and the AI and data framework accrue disproportionately to SMEs and start-ups, for whom they typically remove barriers to participation rather than merely reduce operating costs. A third runs **across the public-private interface**: businesses and healthcare systems capture the bulk of regulatory and efficiency savings, while Member State authorities absorb front-loaded governance costs, which nonetheless remain orders of magnitude smaller than the corresponding business-side relief and associated prevention benefits. Crucially, the combination of measures generates additional distributional effects not visible at intervention level: CES rationalisation and strategic-project eligibility partly offset the SPC-driven loss to biosimilar developers; the SPC geographical conditionalities recycle part of the payer-to-originator transfer back into EU clinical and manufacturing activity; and the Support Network and cluster infrastructure narrow the capacity gap that would otherwise leave smaller firms less able to exploit complex regulatory architectures. On balance, patient welfare is strongly positive, as biosimilar-driven healthcare savings outweigh the SPC-related delay costs.

6.2 Competitiveness and investment attractiveness

The competitiveness rationale for the Biotech Act rests on a well-documented structural deficit[297], as evidenced by the EU's limited capacity to scale innovative firms, and a persistent investment gap (estimated at EUR 40 billion annually) in the EU biotechnology sector. Regulatory simplification is a necessary condition for closing this gap, but it is not enough on its own. The Act therefore deploys a set of instruments that collectively alter the return profile for biotech investments in the EU by targeting the specific stages and segments where market failures are most acute. The Act's competitiveness provisions are designed to address a structural erosion of the EU's position relative to its principal global

[297] See section 3 for further elaboration.

competitors and to reverse specific competitive disadvantages on the dimensions that drive investment and location decisions.

The **project recognition frameworks** (strategic and high-impact) turn what is currently an opaque, variable permitting landscape into a bounded, time-limited pathway, directly improving the bankability of industrial-scale deployments. The combined potential for investment mobilisation is substantial. Strategic project recognition alone could mobilise EUR 15-28 billion cumulatively by 2038 (under medium uptake; 60-70 recognised projects), while the high-impact flagship tier adds a further EUR 4-12 billion (medium, 12-15 projects) targeting catalytic infrastructure with cross-border reach, such as integrated biotech testing and scale-up facilities and centres of excellence for advanced therapies. Together, these frameworks could mobilise EUR 19-40 billion in total investment by 2038, depending on uptake and project composition, and avoid combined delay costs of EUR 265 million-1.16 billion (under medium uptake). Critically, these project pipelines also serve a signalling function; creating a structured 'investability' channel that did not previously exist, facilitating faster financial close and more crowding-in of private capital.

The **financing instruments** reinforce this logic. The proposed EU Health Biotechnology Investment Pilot targets the sector's structural EUR 40 billion annual investment gap, as estimated by the EIB. The already launched BioTechEU initiative backed by InvestEU is expected to mobilise up to EUR 10 billion in biotechnology investments in 2026-2027 alone. Under the medium-ambition scenario (closing 25% of the EU-US investment gap), the Pilot would result in approximately EUR 6.2 billion in direct investments over two years, supported by EC budgetary guarantees. It is expected to raise total investment mobilisation to approximately EUR 41.6 billion over the same period (compared with approximately EUR 21.6 billion under the baseline), consistent with reducing the annual investment gap by approximately EUR 10 billion per year.

A distinct competitive dimension concerns **biosimilars**, where the EU holds the world's largest market (approximately EUR 17 billion at list prices[298]), but is experiencing manufacturing migration to Asia. Streamlining CES requirements serves a competitiveness objective: by reducing the cost threshold for biosimilar development, it accelerates market entry by 12-24 months per product and strengthens EU-based developers against jurisdictions already formalising such flexibility. Strategic project eligibility for biosimilar manufacturing reinforces this supply-side effect. While the share of EU-headquartered companies in EU biosimilar MAs is 49% in 2025, projected to fall to 40-50% without intervention, it can be potentially maintained at 45-55% with the proposed measures. On the other hand, the **SPC extension** incentivises a very targeted set of innovative biotechnology medicines. The SPC extension could potentially be awarded to about three products annually[299]. According to EMA's data from 2016 to 2025, the three products could grow to **about five** per year if developers changed their behaviour in response to the incentive (i.e. conducting part of their clinical trials and manufacturing in the EU), thus bringing **new transformative products** to the market and a **marginal positive impact** on the **conduct of clinical trials** and **manufacturing in the EU**.

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[298] IQVIA Institute for Human Data Science (2026), *The Impact of Biosimilar Competition in Europe*, estimating the EU biosimilars market at approximately USD 18 billion (list prices).
[299] The average is 2-3 products per year rounded to 3 for ease of illustrating the systemic impact.

The extension comes with an additional gross economic gain of approximately EUR 230 million per qualifying medicine per year, and a direct payer expenditure and monetised cost of delayed patient access which together amount to approximately EUR 205 million per medicine. This corresponds to a cost of approximately EUR 615 million every year at aggregate level, based on an average of three qualifying medicines per year.

The extension focuses specifically on rewarding innovations that bring the **greatest value to patients**[300], sending an important **signal to companies** regarding investment priorities. The geographical conditions ensure that this additional incentive also favours investment in the EU and helps strengthen the EU's health biotech sector.

Clinical research is equally critical to the competitiveness proposition. Despite significant global growth in clinical trials between 2013 and 2023, the EU's share of global commercial trials halved from 22% to 12%, failing to capture the proportional share of the growth. Improved regulations, including simplified and more predictable procedures, as well as sandboxes work in concert to make the EU/EEA more attractive for sponsors as a location for conducting clinical trials. Consequently, a better regulatory environment is expected to increase the number of clinical trials in the Union and possibly, depending on the development in other regions, reverse the structural decline of the EEA share of global trials. The increase in trials is expected to range between 10% and 30% based on two studies supporting the assessment[301]. Together, these interventions form a coordinated competitiveness package.

6.3 Innovation and research

The EU's core innovation challenge in biotechnology is not insufficient scientific output. European research organisations rank among global leaders in biotechnology publications, but there are persistent barriers to translate that output into clinical and commercial development. This is reflected in significantly weaker late-stage financing and a persistent investment gap in the EU compared to global peers[302]. While the competitiveness measures described above address the conditions under which capital is deployed in the EU, the innovation provisions target the conditions under which scientific discoveries are developed, validated and translated across the full R&D continuum, including clinical research and scale-up activities, ensuring that innovation can progress effectively from early-stage research to market deployment in the Union. The Act addresses this structural translation gap through three mutually reinforcing channels.

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[300] Example of the value for patients of biotechnology medicines include: Jönsson B, Hampson G, Michaels J, Towse A, von der Schulenburg JG, Wong O. Advanced therapy medicinal products and health technology assessment principles and practices for value-based and sustainable healthcare. Eur J Health Econ. 2019 Apr;20(3):427-438. doi: 10.1007/s10198-018-1007-x. Epub 2018 Sep 18. PMID: 30229376; PMCID: PMC6438935.
Ohashi T. The impact of monoclonal antibody drugs on healthcare economics in the treatment of multiple sclerosis and neuromyelitis optica spectrum disorders. Clin Exp Neuroimmunol. 2022;13(3):166–171. https://doi.org/10.1111/cen3.12718.

[301] According to one study (*Regulatory Framework Study forthcoming*), the number of clinical trials is expected to increase by 32% considering the outcomes of a survey with sponsors. A second study (*Rapid Assessment Scenarios study, forthcoming*) suggests an average increase of 10 %. Additionally, the lower bound aligns with the ACT EU target of an increase of 11.1% of multinational clinical trials in the EU in the next five years.

[302] US biotech firms have raised approximately 870% more Series C capital over the past decade and 945% more in IPO proceeds (USD 53 billion versus USD 5 billion), while EU venture capital in biotech and healthcare fell from 34% of global share in 2013 to 18% in 2022.

First, introducing **regulatory sandboxes** across clinical trials, food and feed, SoHO, and veterinary products creates, for the first time, structured pathways for innovations that currently face regulatory uncertainty or do not fit neatly into existing EU frameworks. In the absence of such mechanisms, highly novel products risk delays, fragmented assessments or the absence of a clear route to market, constraining their development and deployment in the Union and delaying or preventing the Union from capturing benefits in terms of patient outcomes, innovation and economic activity.

Second, the Biotech Act addresses late-stage attrition by combining **dedicated financing instruments** with catalytic infrastructure. The EU Health Biotechnology Investment Pilot targets the sector's EUR 40 billion annual funding gap, as estimated by the EIB, building on the EUR 10 billion in mobilisation expected in 2026-2027 through the BioTechEU initiative and illustrative scenarios projecting a gap reduction of EUR 10-20 billion per year at full scale. Simultaneously, high-impact project infrastructures are expected, under illustrative uptake scenarios, to support 4-40 additional clinical trial applications per year and serve 60-600 firms annually. These ranges reflect very low to very high uptake scenarios, with impacts driven by de-risking translation services and compressing development cycles. Together, the sandboxes are expected to resolve pathway uncertainty for novel products, while financing instruments and catalytic infrastructure ensure that developers of products with a clear regulatory route can access the capital and facilities needed to reach the market.

Third, the **AI and data framework** addresses a cross-cutting enabler that conditions the effectiveness of the first two channels. Depending on uptake, the recognised testing environments and data quality accelerators could serve approximately 1,100-3,400 companies and data users annually (under medium uptake projections), and trigger infrastructure investments of EUR 650 million to EUR 1.3 billion. Along with the harmonised guidance on deployment and use of systems based on advanced technologies, including AI in the lifecycle of medicinal products, these measures are expected to deliver an 14-month average reduction in development timelines versus fragmented multi-vendor outsourcing, with best-case savings of up to 34 months across Phase I–III[303] , with potential trial timeline reductions of 18% as AI integration matures, collectively positioning the EU to capture a greater share of the rapidly expanding AI-enabled drug discovery market.

Together, these three channels constitute the most comprehensive intervention the EU has undertaken to address the structural disconnect between its scientific output and its capacity to translate that output into clinical and commercial innovation. The Act strengthens the **EU's capacity to innovate** in biotechnology by simultaneously lowering the three barriers that currently determine whether a viable scientific discovery acquires a path to market in the EU: regulatory pathway uncertainty (addressed by sandboxes, the regulatory status repository, and the Foresight Panel), capital scarcity at the translation stage (addressed by the Investment Pilot and high-impact project infrastructure), and the absence of shared enabling infrastructure for AI-enabled development (addressed by testing environments and data quality accelerators). These interventions could also create

[303] Evidence from integrated shared-facility models, based on DiMasi, J., Dirks, A., & Getz, K. (2025). "The Net Financial Benefits of Single Vendor Integrated CDMO and CRO Drug Development Services." Tufts Center for the Study of Drug Development. Announced June 16, 2025.

synergies with other Union initiatives, including potential Important Projects of Common European Interest (IPCEIs[304]), where relevant.

6.4 Public health and safety

The regulatory and economic reforms described in the preceding sections are not ends in themselves; their ultimate justification lies in their capacity to deliver measurable public health benefits. The Biotech Act's public health impact comes through three reinforcing channels: accelerating patient access to biotechnology-derived treatments, strengthening supply resilience for essential medicines, and establishing a preventive framework against biosecurity risks.

The most quantifiable **patient-access gains** come from biosimilars and organ transplantation. Accelerating and widening biosimilar competition is projected to increase annual healthcare system savings from approximately EUR 13 billion in 2024 to EUR 22-35 billion per year by 2035-2038 (cumulative EUR 300-450 billion), while preserving an unblemished safety record; no biosimilar has been withdrawn on safety grounds in 19 years. In parallel, reforms to organ processing standards are estimated to yield approximately 1,435 additional transplants per year by 2035, set against a waiting list exceeding 52,000 patients and approximately 3,366 waiting-list deaths annually, generating cumulative dialysis cost avoidance of approximately EUR 158 million. Beyond these gains, the organ processing reforms are expected to prevent 570-945 organ discards per year and avert 57-72 waiting-list deaths annually by 2035 (248-311 cumulatively over 2028-2035, valued at approximately EUR 285-537 million using the Commission's standard value of a statistical life year). Thus, the total monetised public health benefit of organ processing reforms over 2028-2035 is approximately EUR 443-695 million.

The reforms to **clinical trial authorisation and the ATMP framework** compound these effects. Given that around 5% to 10% of clinical trials result in a medicinal product reaching the market (an average of 8%), the proposed amendments to the Clinical Trial Regulation could enable roughly 100 of the 2,500–3,000 products tested annually to launch up to six months earlier, while ATMP reforms accelerate access to potentially curative therapies in high-unmet-need areas where the EU already lags behind competing jurisdictions. The biosimilars framework further strengthens both access and affordability: by reducing the cost threshold for biosimilar development, the Act addresses a 'biosimilar void'; some 79% of the approximately 100 biologics losing exclusivity by 2032 have no biosimilars in development.

On **supply resilience**, the strategic projects framework is expected to deliver 18-60 capacity-relevant EU-based biomanufacturing deployments by 2038 (depending on uptake), directly countering the structural vulnerability created by growing import dependence, currently increasing at 12% annually for biosimilars alone.

Supporting these four impact areas is a set of **ecosystem-level enabling measures**, comprising the EU Health Biotechnology Support Network, networks of health

[304] E.g. three Important Projects of Common European Interest (IPCEI) in the biotechnology domain are currently under design (https://competition-policy.ec.europa.eu/state-aid/ipcei/design-support-hub_en#ipcei-candidates-in-the-design-support-hub).

biotechnology clusters, the strategic mapping of the EU's biotechnology ecosystem, and the European Health Biotechnology Steering Group, which provide the coordination, advisory, and connectivity infrastructure needed to translate individual regulatory reforms into system-level outcomes. The Support Network provides a single, locally accessible entry point for health biotechnology developers (particularly SMEs and start-ups) to navigate regulatory pathways, identify funding instruments, and connect with investors, while cluster networks facilitate cross-border collaboration, infrastructure access, and knowledge transfer. Together, these mechanisms **reduce the transaction costs** that would otherwise limit the uptake and cumulative impact of the substantive reforms.

Finally, the **biosecurity framework** introduces a screening framework for synthesised nucleic acids, addressing the specific risk that arises as biotechnology products become more accessible and AI lowers barriers to misuse.

6.5 Environmental impacts

Beyond its primary economic and public health objectives, the Act is expected to generate material environmental and sustainability co-benefits across several interventions. The GMM regulatory reform enables the deployment of microbial applications in biofertilisation, biocontrol, bioremediation, and bioleaching (sectors with a combined global market exceeding EUR 29 billion and projected growth rates of 9-14% annually), which are currently entirely blocked in the EU, where no GMM has been authorised for deliberate release under the existing framework. The veterinary medicines reform facilitates uptake of next-generation biotech vaccines in food-producing species, reducing reliance on antimicrobials in support of the EU's target of halving antimicrobial sales by 2030; empirical evidence associates a 10% reduction in livestock disease levels with an 800 million tonne decrease in greenhouse gas emissions, equivalent to the annual emissions of 117 million Europeans. The biosecurity screening framework provides a direct environmental safeguard: mandatory nucleic acid screening, modelled to prevent 35% of deliberate attacks and 86% of accidental releases, yielding expected benefits of EUR 2.9 billion in 2027, rising to EUR 5.5 billion by 2036, set against implementation costs that are orders of magnitude smaller. In addition, the biothreat radar and biodefence provisions address the extreme tail of biological risk where market mechanisms cannot ensure preparedness. Meanwhile, the food chain sandbox framework creates structured pathways for pre-market testing of sustainable food production technologies, such as GM food, food enzymes, as well as methodologies thereof, such as new approach methodologies for risk assessment. These provisions collectively position the Act as a contributor to the EU's broader sustainability commitments under the European Green Deal and the 2030 Agenda for Sustainable Development.

Taken together, these effects position the Biotech Act as a structural enabler of a more competitive, innovative and resilient EU biotechnology ecosystem, while contributing to societal objectives and embedding safeguards against emerging biosecurity risks, with system-level benefits materialising over time. While the magnitude of these benefits will depend on uptake, implementation and broader market conditions, the assessment indicates that the cumulative benefits of the package are expected to significantly outweigh the associated costs.

6.6 Digital by default principle

The Biotech Act supports the digital transformation of the EU biotechnology ecosystem by embedding a digital-by-default approach across regulatory, scientific and administrative processes. Through measures dedicated to AI and data, but also other measures embedded across the act, digitalisation operates as a horizontal enabler that amplifies the effectiveness of the regulatory simplification, competitiveness and innovation interventions described in the preceding sections.

Measures under the Biotech Act promote the use of electronic submissions and coordinated approaches to data governance. This contributes to reducing administrative costs for businesses and streamlining interactions with public authorities.

The AI and data framework (Intervention n°15) establishes a coherent structure for the development, validation and regulatory use of advanced technologies, including AI, across the lifecycle of medicinal products. The provision of non-binding guidance at Union level reduces uncertainty around data requirements and validation standards, enabling developers to design evidence generation strategies more efficiently and with greater regulatory certainty. In parallel, trusted testing environments and biotechnology data quality accelerators are bound to create shared infrastructures that allow companies, particularly SMEs and start-ups, to access high-quality datasets and computational resources that would otherwise remain out of reach.

Taken together, these contribute to strengthening innovation and research capacity and shortening timelines for innovations to reach the market, reinforcing the competitiveness and investment effects described in Section 6.2 by improving the risk-return profile of data-driven biotechnology projects and increasing the attractiveness of the Union as a location for AI-enabled development.

For public authorities, the transition to digital-by-default entails upfront adjustment costs related to the development of digital infrastructures, data governance frameworks and technical expertise. These costs are expected to be largely offset over time as procedures standardise, digital handling matures and coordination across authorities improves. As systems scale, the unit cost of regulatory processing is expected to decline, contributing to greater administrative efficiency and more consistent decision-making.

Overall, through measures touching upon both regulatory and innovation aspects, the digital-by-default approach therefore acts as a systemic multiplier across the Biotech Act.

7 MONITORING AND EVALUATION

Five years after the Regulation proposal's entry into application, and every five years thereafter, the Commission will evaluate its implementation, effectiveness and impact. As regards the proposed Directive, it will also be evaluated in accordance with Better Regulation policy.

Progress towards the objectives of the proposed Regulation would be monitored using a set of quantitative and qualitative indicators. This assessment will draw on the **strategic mapping of the EU's biotechnology ecosystem** that will be established and periodically

updated by the Commission. It will also be based on continuous data and other information available through the implementation of existing legislation and initiatives in the Commission and the European Medicines Agency.

Such monitoring shall be based on key performance indicators:

Indicator	**Source**
Venture-capital investment flows in biotechnology in the Union[305]	Invest Europe Annual activity statistics
Number of strategic projects and high-impact strategic projects	European Commission
Number of products granted a supplementary protection certificate	National patent offices
Number of additional multinational clinical trials authorised in the Union over the 5-year period of the reporting, compared to the average number of such clinical trials authorised per year in the Union as of 2025	Clinical Trials Information System (CTIS)
Number of clinical trials concerning ATMPs authorised in the EU	Clinical trials information system (EMA)
Number of ATMPs with EMA authorisation in the EU	EMA annual report
Number of GMM products authorised under Directive 2001/18/EC to be placed on the EU market	European Commission

[305] Calculation methodology to ensure that short-term volatility and year-to-year fluctuations do not distort the assessment of underlying investment trends, such as by using three-year moving average of annual investment flows.