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INFORMATION

From:	General Secretariat of the Council
To:	Working Party on Pharmaceuticals and Medical Devices (Attachés) Pharmaceutical package
Subject:	Proposals for a Directive and a Regulation on the General Pharmaceutical Legislation - Summary of the feedback

Delegations will find enclosed a summary report of the feedback received on the proposals for a Directive and a Regulation on the General Pharmaceutical Legislation provided by the Commission services.

EUROPEAN COMMISSION
DIRECTORATE-GENERAL FOR HEALTH AND FOOD SAFETY

The Director-General

Brussels
SANTE.D.1/AR/ko (2023)12419695

Honourable Chair,

Your Excellency,

From 26 April to 8 November 2023, the Commission collected feedback from stakeholders received in the context of the Commission's better regulation agenda on the proposals revising the general pharmaceutical legislation (COM(2023)192 final and COM(2023)193 final).

Please find attached for your information the summaries of the feedback received during this period.

Your faithfully,

Sandra GALLINA

Mr Pascal Canfin
Chair of the Committee on the Environment, Public Health and Food Safety of the European Parliament
[redacted]

Mr Pierre Cartuyvels Ambassador - Deputy Permanent Representative to the EU (Coreper I)
Permanent Representative of Belgium to the European Union
[redacted]

Copies to:
[redacted]

[redacted]

[redacted]

Enclosures: Summary report of the feedback received on the proposal for a Directive of the European Parliament and of the Council on the Union code relating to medicinal products for human use, and repealing Directive 2001/83/EC and Directive 2009/35/EC

Summary report of the feedback received on the proposal for a Directive of the European Parliament and of the Council laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing rules governing the European Medicines Agency, amending Regulation (EC) No 1394/2007 and Regulation (EU) No 536/2014 and repealing Regulation (EC) No 726/2004, Regulation (EC) No 141/2000 and Regulation (EC) No 1901/2006

c.c.: [redacted]

ANNEX I

Summary report of the feedback received[1] on the proposal for a Directive of the European Parliament and of the Council on the General Pharmaceutical Legislation.

The feedback period ran from 26 April to 8 November 2023 and aimed at gathering stakeholders' views on the Commission proposal for a Directive on the General Pharmaceutical legislation[2]. Feedback was provided by 321 respondents[3], of which 7 were moderated[4]. 314 responses were hence valid. 193 respondents attached a document to their feedback. The majority (31%, 97 out of 314) of respondents were EU citizens, followed by business associations (19%, 59 out of 314), non-governmental organisations (18%, 56 out of 314), companies/businesses (14%, 44 out of 314), academic/research institutions (8%, 27 out of 314), others (8%, 26 out of 314), public authorities (1%, 4 out of 314), trade union (1 out of 314) and consumer organisation (1 out of 314), according to respondents' self-identification.

There was one **campaign** from Germany (115 respondents, including 75 EU citizens, 18 academic/research institutions, 10 companies, 6 NGOs, 5 other organisations, 1 business association). This contribution has been analysed separately from the rest of the responses. Respondents were positive towards the pharmaceutical revision. There were some recommendations with regards to the production of radiopharmaceuticals in Nuclear Medicines departments. The contribution proposes some revisions to the existing definitions and recommends adding the definition of 'kit-radiolabelling'. According to the submission, this should clarify the different interpretations and practices across the EU Member States. Lastly, this campaign calls for a specification regarding kit-based radiopharmaceutical compounding, suggesting an exemption from the requirement of a manufacturing authorisation when they are intended for in-house use, which is covered by national legislation and such products are not intended to be placed on the market.

Overview of contributions received outside campaigns

EU citizens (22 replies). Diverse concerns have been raised regarding the proposed revision, highlighting the need for additional safeguards within the legislative texts. Specifically, there is a strong emphasis on the necessity of maintaining and enhancing an incentive scheme to appropriately remunerate pharmaceutical companies. In this regard, one respondent voices the importance of giving more attention towards patients suffering from ultra-rare diseases, as the pharmaceutical revision would not be adequately addressing their needs.

With regard to SoHO derived-medicinal products, 3 EU citizens welcome the introduction of this new category in the Directive, expressing some concerns about the interplay with SoHO preparations.

Lastly, there is a call for the development of an online portal that ensures transparency regarding available medicinal products and provides a convenient platform for ordering them.

Business association (57 replies), including pharmaceutical and healthcare industries, professional associations, research-based pharmaceutical companies, and medical

[1] Revision of the EU general pharmaceuticals legislation (europa.eu)

[2] COM/2023/192

[3] Two feedbacks were received slightly after the deadline, but they were considered for the purpose of this report however they were not included in the statistical report of the feedback.

[4] The reasons for moderation were various, e.g. duplications, off-topic content, and offensive or aggressive language.

technologies industries. Respondents strongly emphasise concerns about the proposed revisions, fearing adverse impacts that could significantly diminish incentives for research and innovation in the EU. More than half of feedbacks point that the reduction of the regulatory data protection period (RDP) is deemed to hinder innovation and make the EU less competitive on the global stage. Additionally, mostly the same respondents specify how the proposed system of offering additional incentives based on launching a product in all Member States within a specified time frame is considered impractical, especially for SMEs, as it would be beyond the control and influence of manufacturers, increasing administrative burden for companies and regulators. The situation would be even more complicated for vaccines. In fact, 1 organisation emphasises that the fulfilment of the condition "release and continuous supply a product", required to obtain an additional two years of RDP, would be impossible for a vaccine manufacturer to meet as it would depend on local governments' decision to include the vaccine in the National Immunisation Programmes.

Similar concerns were raised regarding the extension of the Bolar exemption to encompass pre-commercialisation activities, including studies and trials required for Marketing Authorisation (MA), Health Technology Assessment (HTA), pricing, and reimbursement. In fact, based on more than 20 received feedbacks, extending the Bolar exemption could pose a threat to the enforcement of intellectual property rights. This extension would challenge the original rationale of the exemption and could undermine innovative research activities. As regards the Unmet Medical Needs (UMN) concept, a high number of replies (around 25 feedbacks) calling for greater clarity to define its boundaries and provide companies with certainty for investment decisions. Furthermore, there are debates about the transition from paper to electronic product information, with 13 respondents advocating for a smoother transition to digital leaflets complement paper ones, while a few others (4 contributors) push for a complete replacement of paper.

There is near-unanimous opposition to considering an inadequate environmental risk assessment as grounds for refusing a marketing authorisation. Such a move would jeopardise patient access to new medicines without being based on health benefit-risk considerations.

Finally, as regards SoHo-derived medicinal products, 2 associations welcome the introduction of the new category within the scope of the Directive. However, they emphasise the need to careful consider the interplay between SoHO preparations and SoHO-derived medicinal products, as when both products share the same SoHO as the starting material and are produced to treat or prevent the same disease, their coexistence in the market may result in unfair competition. They, therefore, call on competent authorities to refrain from granting an authorisation to a SoHO preparation for the same indication, in order to ensure a fair and secure marketplace.

Non-Governmental Organisations (NGOs) **(50 replies)**. The NGOs that send feedback represent medical professional groups, disease groups, patient and citizen groups. The revision is supported by almost all the contributors, who also positively emphasise the efforts to streamline authorisation process and the pivotal role of the environmental risk assessment (ERA) for marketing authorisations. However, around 10 respondents express concerns regarding the reduction of regulatory data protection period and the introduction of incentives for orphan and paediatric medicines developments. Additionally, half of the contributors call for a clear harmonisation of key provisions across Member States, such as those pertaining to UMN, considering the availability of alternative treatments, disease severity and patients' quality of life.

Significant emphasis is placed on patients' involvement in pharmaceutical processes, safety and awareness. In fact, around 20 NGOs advocate for greater patient access and engagement within EMA committees, working groups, and at the national level, especially

for patients with specific medical conditions. As regards the paediatric sector, 8 respondents call for programmes allocating public funds to research projects addressing UMN in paediatric indications and specific incentives for first-in-child development and marketing authorisations, as this would increase the interest in developing specific medicines for paediatric cancers and rare diseases. In addition, respondents suggest accelerating assessment procedures and use of conditional marketing authorisation to address paediatric and orphan drugs issues. Moreover, 5 organisations endorse the coexistence of both electronic and paper product information, suggesting a transition from paper to electronic formats only within healthcare facilities (e.g. hospitals) equipped with the necessary digital tools. Finally, to ensure uninterrupted access to care, 2 respondents suggest that the duration of the hospital exemption should not be shorter than the granted indication duration.
As regards SoHO-derived medicinal products, 4 NGOs welcome the introduction of the new category within the scope of the Directive. However, they manifest the same concern as previously explained.

Company/business (34 replies), including respondents coming from pharmaceutical and biopharmaceutical companies, medical technology companies, multinational consumer healthcare companies. Similarly to NGOs, although the majority of responding stakeholders endorse the legislative measures designed to fortify and streamline the regulatory process, they urge some improvements, such as a broader definition of UMN. In fact, half of the contributors highlight the importance of addressing inequities faced by patients with UMN, life-threatening or highly debilitating conditions. Moreover, 6 contributors favour electronic product information, stressing also the importance of standardised layouts, content, and terminology to enhance readability.
The prevailing majority (almost all the respondents) has concerns about the reduction of regulatory data protection period, that is feared to introduce greater unpredictability for the industry and elevate risks associated with investments in R&D and pharmaceutical operations. According to the respondents, the suggestion to tie part of regulatory data protection period to launch across all 27 Member States ignores the complex and fragmented market access dynamics and puts the burden entirely on companies. For non-prescription medicines, 2 companies propose to extend from 1 to 3 years the data protection and market exclusivity in case of change of prescription status, to ensure continued stimulation and attractiveness of EU innovation.

Academic/research institutions (9 replies). Half of the respondents highlight the difficulties in implementing the reduction of data protection period, especially for SMEs and stress the need for innovative and patient-centred package leaflets. More specifically, 3 respondents emphasise that the existing leaflets are deemed overly lengthy and challenging to read. The current proposal would lack a clear direction, prompting these research institutions to suggest recommendations based on best practices and international standards, such as patients' participations during information development process, evidence-based practices, visual design.

Other organisations (21 replies), including medical associations, disease organisations, organisation of pharmaceutical professionals, and non-for-profit organisations. Whilst expressing a positive stance on the proposed Directive, the majority of contributors deem some adjustments necessary to optimise outcomes for public health, safety and effective medicine management. 2 respondents advocate for systematic dialogue with healthcare professionals and patients, particularly concerning real-world data collection and UMN to identify research priorities. Around 10 organisations call for reconsidering the Highly Unmet Medical Need (HUMN) category to include rare cancers and ultra-rare diseases and

seeking clarity in the incentives system. Other 2 organisations stress the importance of redefining and clarifying provisions related to antimicrobials and crisis-solving incentives. For paediatric medicinal products, 3 respondents make a call for tailored obligations and incentives, including those for first-in-child marketing authorisation to boost commercial interest in developing these medicines.

Public authorities (4 replies) from Sweden and Germany. 2 respondents advocate for more precisely tailored incentives in underserved areas, introducing appropriate margins to counteract anticompetitive protection mechanisms. 2 authorities support the reduction of the regulatory data protection period and emphasise that an extension should be limited to real treatment gaps, where a clinical benefit is foreseen for patients. In addition, besides agreeing with the other categories of respondents about broadening the definition of UMN, this group supports the expansion of the Bolar exemption. They emphasise that such expansion ensures timely competition among interchangeable medicines, thereby stimulating the availability of new and substitutive medicinal products within therapeutic areas.

Trade union (1 reply). The respondent stresses the vital need to support the initiative, promoting collaboration between the scientific community and the pharmaceutical industry. This includes streamlining medication access, promoting transparency in public funding, and encouraging initial investments through public funds, as crucial elements for improving the overall quality of healthcare in the EU. In fact, the respondent emphasises that coordinated extensions of regulatory protection periods can ensure uniform access to medicines across Member States. Moreover, ambitious national action plans for issues like antimicrobial resistance and digitalization would enhance the EU pharmaceutical sector competitiveness.

Consumer organisation (1 reply). Diverging from other stakeholders, the respondent supports a broader extension of the Bolar exemption, aiming to accelerate the market introduction of new medicinal products after the expire of intellectual property protection. However, in line with the prevailing sentiment, the respondent agrees that electronic product information should complement the traditional paper leaflet rather than replace it.

ANNEX II

Summary report of the feedback received[5] on the proposal for a Regulation of the European Parliament and of the Council on the General Pharmaceutical Legislation.

The feedback period ran from 26 April to 8 November 2023 and aimed at gathering stakeholders' views on the Commission proposal for a Regulation on the General Pharmaceutical legislation[6]. Feedback was provided by 124 respondents[7], of which 1 was moderated and 2 were duplications 121 responses were hence valid. 75 respondents attached a document to their feedback. According to the respondents' self-identification, the majority (28%, 34 out of 121) of respondents were business associations, followed by company/business (21%, 26 out of 121), non-governmental organisations (18%, 22 out of 121), EU citizens (17%, 20 out of 121), others (7.5%, 9 out of 121), academic/research institutions (6.5%, 8 out of 121), public authority (1 out of 121) and non-EU citizen (1 out of 121).

There was one **campaign** from Germany with regard to radiopharmaceuticals. The majority of respondents were EU citizens (15 out of 20), followed by companies (2 out of 20), academic/research institution (1 out of 20), business association (1 out of 20) and NGO (1 out of 20). This contribution has been analysed separately from the rest of the responses. Respondents were positive towards the pharmaceutical revision. However, some recommendations are suggested. Some of the recommendations provided do not concern the proposal for a Regulation but the proposal for the directive (COM(2023)192). This is the case on the request for more sector specific provisions to radiopharmaceuticals.

Overview of contributions received outside the campaigns

Business associations (33 replies), including associations of bioindustries, pharmaceutical industries, professional associations, and associations of medical technologies industries. Respondents positively welcome some provisions such as those related to shortening the approval timelines, streamlining the EMA structure and the creation of a regulatory sandbox. Nevertheless, some topics are more contentious. Transferable Exclusivity Vouchers (TEV) reveals conflicting views, with 8 respondents advocating in favour of them as incentives against antimicrobial resistance, while others (5 respondents) note limitations, particularly implications on the development and authorisation of medicinal products and, therefore, undermining patients access to medicines. Improvements are sought for the voucher's long-term application, suggesting the removal of the 10-voucher and 15-year limitations. On the other hand, one respondent suggests a multi-country pull incentive as more suitable option to tackle the antimicrobial resistance (AMR), as TEV could potentially prolong monopolies on lucrative high-volume medicines, deviating from the traditional connection between innovation and reward.
Contributors oppose some measures under the EU prevention and mitigation system for medicines shortages, advocating a risk-based approach not to strain resources of both industry and regulators. 20 respondents propose a pragmatic strategy, maintaining a 2-months mandatory notification period and relying on the European Medicines Verification System for shortage prevention and monitoring of marketing authorisation holders' supply to wholesalers and pharmacies. Moreover, solutions to address shortcomings should be proportionate to risks. Consequently, 20 business associations call for Shortage Prevention

[5] Revision of the EU general pharmaceuticals legislation (europa.eu)
[6] COM/2023/193
[7] One feedback was received slightly after the deadline, but it was considered for the purpose of this report, however it was not included in the statistical report of the feedback.

Plan (SPP) only for critical products, included in a Union List of Essential Medicines, to avoid redundant and confusing national lists that weaken the regulatory system's coherence.
Similar resistance is expressed by 10 contributors toward changes in the market exclusivity system and reductions in IP incentives for orphan medicinal products, which will slow the progress, undermining the orphan drug ecosystem and the future investment in rare disease resources. Rules linking exclusivity to addressing a HUMN face criticism for creating barriers without promoting innovation, with calls from 8 respondents for the removal of HUMN from the Regulation. In the context of orphan medicinal products, developers must already demonstrate significant benefit and clinical superiority of a therapy before they receive orphan designation. Thus, the 'test' of HUMN creates additional hurdles for developers, requiring additional clinical data to meet a 'nebulous' concept.
Regarding the environmental risk assessment, although it is common knowledge that many pharmaceutical companies already have well-developed environmental responsibility plans, the refusal of a product's marketing authorisation based on environmental impacts is deemed inappropriate, as the protection of public health should always prevail in such a comparison. In 11 responses, the proposal is seen as potentially diminishing Europe's appeal as an innovation hub, leading to the relocation of production to other regions.

Company/business (24 replies), with most respondents coming from the pharmaceutical, healthcare and biopharmaceutical sectors. The contributors positively welcome the legislative revision regarding reconstructing EMA, shortening the approval timelines (from 210 days to 180 days), the use of real-world data and the regulatory sandbox.
On the issue of medicine shortages, half of contributors emphasise the need for targeted and proportionate policy solutions, stressing the importance of shortage mitigation and prevention plans tailored to the risks and vulnerabilities. They argue against imposing one-size-fits-all constraints to prevent medicine shortages because ineffective and burdensome; whereas they advocate for Shortage Prevention Plans (SPPs) for critical products identified on a risk-based approach along medical need, availability of adequate alternatives and resilience of supply chain. In this regard, to streamline reporting and avoid misinformation about shortages, respondents propose for an obligation to report shortages via the SPOR database, arguing that the 6-months advance notification would have a detrimental effect on their effective mitigation due to the increased administrative burden it could generate.
Significant concerns emerge regarding new provisions on orphan drugs, with around 10 companies fearing erosion of EU competitiveness and a risk to growth and patient access to innovative medicines in the EU. Opposition is voiced against the gradation of UMN and the introduction of HUMN, deeming it ethically questionable and limiting incentives for crucial therapies. Lastly, as stressed by 4 business associations in their feedback, another criticised change is the reduction of the orphan market exclusivity (OME). These respondents mention that this is a significant deterioration compared to the current legislation, which risks jeopardising investment attractiveness and development, due to the uncertainty about final market exclusivity and, hence, product protection. The basic rule for OME should remain 10 years, as today. 1 respondent also emphasises the detrimental effect of the seven-year limitation on orphan designation, due to its impact practicality for SMEs to complete development projects.
Opinions vary for Transferable Exclusivity Vouchers (TEV). In this regard, the opponents call for alternatives such as guaranteed revenues, market entry rewards and the use of regulatory leeway to reduce development costs, emphasising the opportunity to develop antimicrobial resistance (AMR) in a future-proof regulation.

Non-Governmental Organisations (NGOs) **(21 replies)**. The NGOs that send feedback represent patient and citizen groups, disease groups and medical professional groups.

While the revision is embraced by all respondents as a unique opportunity to render the EU framework for the authorisation of medicines more patient-centric, with the goal of ensuring fair access to medicines, three main concerns persist. First, 3 respondents oppose the attempt of stimulating research and development of antimicrobials via Transferable Exclusivity Vouchers (TEV). In particular, concerns relate to the limited evidence of the effectiveness and the potential costs for healthcare systems associated with the voucher.
Although the opinion on the provisions addressing staff shortages is somewhat less negative, concerns persist. Despite half of respondents support the prevention of shortages by tracking critical medicinal products and calling for clearer provisions on transparent and timely communication of shortages; one contributor stresses the lack of patient involvement in the shortage management process. In fact, patients' engagement is seen as crucial for improving data collection, understanding the societal impact of shortages and addressing the needs of the affected population. The importance of patient participation is also stressed and considered essential in the research and development of orphan drugs. In this regard, there is opposition by 2 respondents to the proposed deletion of two essential EMA committees for paediatric and orphan medicinal products.
In the paediatric sector, there is also a recommendation for a clear obligation to submit Paediatric Investigation Plans (PIPs) after the end of Phase I of clinical studies in adults, rather than before the start of Phase II clinical studies in adults. Penalties for delays in submission are recommended. Moreover, it is suggested to include a marketing authorisation incentive, to increase the commercial interest in the development of medicines specific to paediatric cancer and to undertake specific studies in underserved population, such as neonates.
Specific considerations are provided by a research institute regarding the implementation of provisions for innovative products such as SoHO-derived medicinal products and any microbiome-based medicinal product. The respondent appreciates the simplification of committees to address the demand for specific multidisciplinary expertise. However, the contributor emphasises the importance of the EMA to recruit experts specialised in microbiome regulatory science.
Lastly, 3 respondents express appreciation for the provisions aim at reducing the use of non-animal methods. They urge the Commission to invest in a comprehensive plan to end reliance on animal research for the development of safer and effective medicines.

EU citizens (5 replies). While some feedback pertains to general concerns beyond the scope of this report, others specifically advocate for amendments to provisions concerning orphan and paediatric medicinal products. It is suggested to include marketing authorisation incentives, to increase the commercial interest to invest in these niche sectors. For orphan medicinal products, a respondent advocates the establishment of a market exclusivity period of 20 years together with a data exclusivity period of 18 years. Concerns are also raised on the HUMN concept, which lacks a clear definition in relation to orphan drugs. As regards paediatric medicinal products, besides calling for new incentives, it is recommended to foresee a clear obligation to submit Paediatric Investigation Plans (PIPs) after the end of Phase I of clinical studies in adults, rather than before the start of Phase II clinical studies in adults.

Other organisations (9 replies), including medical associations, disease organisations, organisation of pharmaceutical professionals, a medical journal, a collective of academics, businesses, NGOs, EU citizens and non-EU citizens. Some contributors stress the importance of recognising pull incentives for antimicrobials to boost investments in their development and accessibility. In this regard, significant considerations are made by 1 respondent towards paediatric medicines, suggesting specific provisions in the EU pharmaceutical package to address the needs of children and babies regarding

antimicrobial resistance. This should include commitments incorporated in a Paediatric Investigation Plan, material support to both SMEs and not-for-profit organisations developing antibiotics for children and support from the EMA, along with a waiver of relevant fees.
Regarding orphan medicinal products, the importance of repurposing as a method of innovation is mainly stressed by a rare disease working group hosted by a foundation working in the field of rare disease, citing it as a faster and cost-effective means of making treatments available to patients. In fact, drug discovery is a complex, lengthy, and expensive process. Repurposing existing generic drugs to target new indications is less risky, as the behaviour of existing drugs in humans is already known and research is much faster and cheaper. For these reasons, the respondent proposes a Union push and pull incentives scheme, which can potentially incorporate market-entry rewards, 'play or pay' fees, or subscription payment mechanisms.
Another note concerns the rejection of the reduction of the EMA scientific assessment period from 210 to 180 days and the removal of the 5-years renewal of the Marketing Authorisations. One respondent advocates the potential detrimental effects on patients' access as the scientific assessment of the Marketing Authorisation Application (MAA) is considered an essential task that requires expertise, time and independence from both the EMA and national agencies. Lastly, a call is made for the application of risk-based approaches in Drug Shortage Prevention and Mitigation Plans. Ensuring the availability of necessary medicinal products for patients across all markets is considered paramount.

Academic/research institutions (7 replies). 3 respondents raise concerns on the proposed changes in the duration of market exclusivity, as they are unlikely to significantly invigorate generic or biosimilar competition, reducing innovation and EU competitiveness. A research study referenced by one respondent on enhancing Europe's appeal to pharmaceutical companies for the submission of innovative medicines suggests that the lack of incentives for innovation and insufficient regulatory simplification may contribute to companies preferring to submit medicines first in the US. In fact, the proposed reduction of the Orphan Market Exclusivity (OME) is criticised for potentially discouraging industry investment in Europe. Ethical concerns are raised about the gradation in the UMN classification as it leads to discrimination among patients not affected by a HUMN. Nevertheless, even in relation to HUMN, the proposed additional incentives are deemed to be insufficient to stimulate investments in those highly risky projects.
Conversely, another respondent calls for recalibrating the market exclusivity landscape by shortening the standard market exclusivity duration to 5 years, due to the limited competition on orphan medicinal products (OMPs) post-exclusivity. This approach would align incentives with broader accessibility goals and stimulates competition.
2 respondents focus their attention on the paediatric sector, stressing the backward step made with the inclusion of the paediatric regulation into the general framework. The abolition of paediatric legislation is considered unjustified, and concerns are raised about the inclusion of relevant parts in a directive. Strong concerns are then expressed about the disappearance of the paediatric committee within EMA and, therefore, about how a non-paediatric working group will deal with children needs without any specific competence.

Public authority (1 reply). The respondent advocates for a unified procedure, common to the SoHO Regulation, for seeking clarification on the regulatory status of borderline products of human origin. The respondent proposes a one-stop-shop mechanism, enabling stakeholders to address a single body comprising representatives from both EMA and SoHO Coordination Committee (SCB). Furthermore, the respondent hopes that, due to the specificity and complexity of advanced therapy medicinal products, the scientific expertise

within EMA working groups and in the Committee for Medicinal Products for Human Use (CHMP), will be upheld through effective and systematic coordination.

Non-EU citizen (1 reply). The feedback focuses on compassionate use, suggesting that in exceptional cases it may cover indication that are not subject to ongoing clinical trials and to a Marketing Authorisation Application. Furthermore, the respondent asks for a more comprehensive harmonisation of the existing compassionate use guidelines, to overcome issues related to different local interpretation which compromise patient access to the treatment.