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Subject:	COMMISSION STAFF WORKING DOCUMENT EXECUTIVE SUMMARY OF THE IMPACT ASSESSMENT REPORT Accompanying the documents 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 Proposal for a Regulation 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

Delegations will find attached document SWD(2023) 193 final part 2/2.

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PART 2/2

COMMISSION STAFF WORKING DOCUMENT

EXECUTIVE SUMMARY OF THE IMPACT ASSESSMENT REPORT

Accompanying the documents

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

Proposal for a Regulation 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

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A. Need for action

What is the problem and why is it a problem at EU level?

The evaluation of the legislation on medicines for children and for rare diseases has shown that the two regulations have effectively fostered the development of medicines over the past 20 years. However, since their adoption there have been wide-ranging developments and discoveries in science and a globalisation of the pharmaceutical sector. These changes, which have focused attention on unmet medical needs, patient access and the budgetary impacts of medicines, call for a review of policy intervention in the field of rare diseases and medicines for children.

The evaluation of the two Regulations, published in 2020, identified the following problems:

1. medical needs of children and patients with rare diseases and are not sufficiently met;
2. the price of medicinal products poses a challenge for healthcare systems (affordability);
3. patients have unequal access to medicines across the EU;
4. the system does not accommodate innovation well enough and creates unnecessary burdens.

1) Of the over 6 000 recognised rare diseases, 95% still have no treatment option. The development of medicines for children is still driven by the development of adult medicines. Where therapeutic needs for adults diverge from children's needs, the number of treatments available is limited.

2) Pricing and reimbursement decisions and pharmaceutical expenditure are national competences, outside the scope of EU pharmaceutical legislation. The average list price of new medicines is increasing, especially for orphan medicines. High prices impact the affordability and sustainability of health systems. The incentives provided by the legislation delay the market entry of cheaper versions of products (generics and biosimilar) which could otherwise improve the affordability for the health systems.

3) Only about half of the orphan medicinal products on the market are currently accessible to patients in a majority of Member States and overall access varies considerably between Member States. This is worse than for standard medicines. Access to medicines for children is often linked to the launch of the corresponding adult product.

4) Advances in science, such as advanced therapy medicinal products and personalised medicine approaches, have already improved target treatments for patients suffering from rare diseases. These new products have challenged the current system of orphan designation, which specifies the criteria a product must meet to receive an orphan designation. Moreover, the Paediatric Regulation relies on certain procedures (agreement on a paediatric investigation plan at an early stage of development) that have sometimes proved to be burdensome and inefficient.

What should be achieved?

The overall objective of this initiative is to ensure a high level of health protection for all EU citizens and ensure that children and patients with rare diseases have access to affordable high-quality medicines and to safe and effective therapies to address their medical needs.

What is the value added of action at EU level (subsidiarity)?

The initiative is expected to bring significant efficiencies by improving the system of incentives, rewards and obligations related to research and development of orphan medicines and medicines for children, as well as helping to make them more affordable and accessible to all patients across the EU. In addition, this revision could enhance the competitive functioning

of the market through the review of other measures to facilitate market entry of generic and biosimilar medicines. This would improve patient access and affordability. As the market for medicines for rare diseases and children is small even in larger EU Member States, only a harmonised approach at EU level is likely to succeed. The proposed intervention will be consistent with other European and national measures.

B. Solutions

What are the various options to achieve the objectives? Is there a preferred option or not? If not, why?

The Orphan Regulation. All options are complemented by a set of common elements ensuring faster introduction of generic medicines, measures to keep orphan medicines on the market, measures creating the necessary flexibility to cater for technological and scientific advances and simplification of procedures.

Option A: keeps the 10 years of market exclusivity and adds a voucher for products addressing a high unmet patient need. The voucher allows for a 1-year extension to the period of regulatory protection and can be sold to another company or used for a product in that company's portfolio. **Option B**: abolishes the current 10-year period of market exclusivity. **Option C**: provides for a *variable* duration of market exclusivity of 10, 9 or 5 years, based on the type of orphan medicine (addressing high unmet need; new active substance; well-established use application respectively). If a medicine addressing high unmet need or containing a new active substance is made accessible in all relevant Member States, market exclusivity is extended by 1 year for those products.

The preferred option is **Option C.**

The Paediatric Regulation. All options are complemented by a set of common elements supporting the development of products addressing the unmet medical needs of children, streamlining and, where necessary, simplifying procedures to agree on which clinical studies have to be conducted in children. The intention is to better accommodate innovation in science and to speed up the procedures, to make products accessible to children faster.

Option A: the 6-month extension of the intellectual property right (supplementary protection certificate) will be kept for all medicines for which use in children is studied. For medicines addressing unmet needs of children, an additional reward in the form of *either* an additional 6-month extension of the supplementary protection certificate *or* of a voucher for a 1-year extension of the length of regulatory protection, which can be sold to another company or used for a product in that company's portfolio. **Option B**: the 6-months extension of the supplementary protection certificate will be abolished. **Option C**: the 6-months extension of the supplementary protection certificate will be kept.

The preferred option is **Option C**.

What are different stakeholders' views? Who supports which option?

The Orphan Regulation. All stakeholders agree on the need to continue supporting the development of medicines for rare diseases with specific incentives, as otherwise market failure could reoccur. Public authorities and patient organisations opt for a differentiation of the current main incentive, as reflected in Option C. The pharmaceutical industry could support the modulation of the incentives but not their overall shortening. Industry proposed the introduction of additional incentives compared to the current situation or a novel incentive like a transferable exclusivity voucher. Industry has also highlighted the need for the current Regulation to remain stable, and for predictability in the form of the current orphan

designation criteria, as investment decisions are taken long before the incentives for a successful development can be acquired.

The Paediatric Regulation. The need to keep the existing obligation to study all new medicines for use in children is generally supported by all stakeholders. The pharmaceutical industry and academia generally welcome the improvements to the procedures. The shortening of the supplementary protection certificate extension is not supported by any stakeholder group. Industry favours the maintenance of the existing system but has also called for the introduction of additional rewards to support specific developments in areas of unmet medical need for children. Public authorities recognise the need to better identify unmet paediatric medical needs and recognise that the existing system of obligation and rewards has worked relatively well, but were concerned about the introduction of novel rewards and their subsequent impact on the sustainability of national health systems.

C. Impacts of the preferred option

What are the benefits of the preferred option (if any, otherwise of main ones)?

The Orphan Regulation. The preferred option C will improve the quality of life for patients suffering from these diseases and for their families, as patients will on average benefit from an additional 1-2 new medicines a year, in particular in areas where no treatment is currently available. Originator companies will profit due to extended market exclusivity for medicines addressing high unmet medical need. Generic companies profit from earlier and more predictable market entry. Furthermore, predictability for generics producers should increase as they can place their product on the market on the day market exclusivity expires. Patients will have increased access due to earlier market entry for generics, which will also reduce costs for Member States' health systems. Simplification measures would save EUR 3.3 m a year in administrative costs. The incentives specifically for medicines addressing high unmet medical need will promote innovation and should redirect research investment to those areas where it is most needed and will hence boost competitiveness.

The Paediatric Regulation. The preferred option C will increase the number of medicines for children, which will improve their and their families' quality of life. This option will foster the development of products addressing unmet needs in children through clear criteria for identifying such products. It also introduces a requirement for products developed only for adults to be studied in children too where, based on scientific evidence, they could be effective against a disease in children. Procedural simplifications and adjustments will make the system more innovation-friendly, and lead to swifter completion of the paediatric investigation plan and authorisation of medicines. It will only be possible to defer studying the use of a medicine in children for up to 5 years (currently there is no limit), thus products will reach children quicker than today.

What are the costs of the preferred option (if any; otherwise of main ones)?

Faster market entry for generics will result in some losses for originator companies. Overall, however, the system will be more balanced, ensuring better access for more affordable medicines while directing incentives where they are most needed and thus promoting innovation.

What are the impacts on small and medium-sized enterprises (SMEs)?

The procedural improvements (such as simplification and increased support from the European Medicines Agency) and the reduction of the administrative burden will be particularly important for SMEs, given the small scale of their businesses. Moreover, SMEs will continue to benefit from fee reductions for Agency procedures. Considering that SMEs in the field of rare diseases often start innovative concepts (orphan designations), they should benefit from the modulation of the market exclusivity duration, rewarding products addressing high unmet medical need.

Will there be significant impacts on national budgets and administrations?

No significant costs are expected for national health systems. As more products are developed, national health systems are expected to incur additional costs linked to reimbursements, but this will be outbalanced by savings from faster market entry for generics.

Will there be other significant impacts?

This initiative is expected to have a positive impact on public health and society as a whole. It should ensure that new therapeutic solutions become accessible to patients through an increased number of products for rare diseases and for children, in particular in areas where no treatment exists. Under the umbrella of the pharmaceutical strategy for Europe, the revision of orphan and paediatric legislation and of general pharmaceutical legislation will have a cumulative positive impact on access to and affordability of medicines for all patients and on the sustainability of health systems.

Proportionality?

None of the options for the revision of the Orphan or Paediatric Regulations goes beyond what is necessary to achieve the objectives. The initiative is limited to those aspects that Member States cannot achieve satisfactorily on their own, and where the EU can do better. The preferred options are proportionate, given that they build on and maintain the pillars of an established system and propose targeted adjustments, and given the expected benefits for patients, health systems and industry.

D. Follow up

When will the policy be reviewed?

The development of new orphan medicines can be a long process and the completion of a clinical development plan for medicines for children can take up to 10-15 years. Incentives and rewards therefore exert their effect many years after the marketing authorisation date. The benefit for patients also needs to be measured over a period of at least 5-10 years after a medicine is authorised. The Commission intends to review the initiative periodically. However, a meaningful evaluation of the results of the revised legislation will only be possible at least 15 years after its entry into force.