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European Federation of Pharmaceutical Industries and Associations

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The European Association for Bioindustries

Comments on the Commission's Roadmap on the Evaluation of the legislation on medicines for children and rare diseases (medicines for special populations).

EFPIA and EuropaBio welcome the opportunity to comment on the European Commission's roadmap on the evaluation of the legislation on medicines for children and rare diseases (medicines for special populations).

It is to note that both the Orphan Medicines and Paediatric Medicines Regulations have successfully stimulated the development of medicines for special populations, with each addressing specific development challenges. As such, bearing in mind that the aim is to increase the number of therapeutic choices for children and patients suffering from rare diseases, we would caution against too much generalisation. EFPIA and EuropaBio would like to emphasise the vital importance of maintaining a favourable regulatory environment to maintain the momentum and continue to achieve progress in the fields of paediatric and orphan medicines.

We would welcome clarification of the Commission's intentions regarding the ultimate objective of this joint evaluation, which should be more precisely stated in the roadmap. In this respect the anticipated scope of the additional study on orphan medicinal products legislation that will be launched in 2018 should be explained, in particular the remaining research questions this study is intended to address.

The analysis of the impact of the legislation on the availability of treatment options to patients should be put in a broader context, aiming to identify all drivers of investment in research and development in both the areas of orphan and paediatric medicines. It should be driven by the overall objective to improve access of patients to new treatments. Payers, HTA bodies, investors and all other stakeholders involved in the financing, development, manufacturing, reimbursement and commercialisation of orphan and paediatric medicines will provide useful input to help understand the dynamics across the medicinal product's life. SMEs, often helped by capital provided by specialised investors, have proven to be important players in the development of orphan medicines and understanding the factors conducive to their success in this field is another important consideration. Since development is global, it is very welcome that the Commission considers the experience and developments in the United States. US FDA metrics could be added to the potential data sources.

Overall the roadmap constitutes a good starting point but questions remain on the methodology and on the sources that will be used in line with the stated objective. Regarding the financial and cost/benefit aspects of the planned evaluation, it will be important to understand the overlap with the work done in preparation for the 10 year Report on the Paediatric Regulation. In particular, how will data be gathered in an appropriately objective way, and duplication of the paediatric work avoided?

More specifically, EFPIA and EuropaBio would welcome further details and clarity on the following:

- Whether a separate consultation process is foreseen on the scope of the 'additional study on orphan medicinal products legislation'

- Methodology and source of data gathering envisaged for this new study
- Role of and relationship with the existing Technopolis study on the Paediatric Regulation
- The targeted consultation involving Member States and specific interest groups – for example, which aspects the Commission plans to explore more in depth?
- In addition to the listed stakeholders, to fully capture the picture of enablers and barriers to address unmet needs in special population, we recommend consulting with pharma investors.

We are looking forward to further engage as more information and detail on the roadmap become available.

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The European Federation of Pharmaceutical Industries and Associations (EFPIA) is committed to researching, developing and bringing to patients new medicines to improve health and quality of life. Its membership are 33 national associations and 40 pharmaceutical companies.

EuropaBio, the European Association for Bioindustries is committed to the use of biotechnology to improve quality of life, to prevent, diagnose, treat and cure diseases, to improve the quality and quantity of food and feedstuffs and to move towards a biobased and zero-waste economy. Represents 78 corporate and associate members and bio regions, and 15 national biotechnology associations in turn representing over 1800 biotech SMEs.