

Dear sir/madam,

HollandBIO (the Dutch biotech industry association) and the Association Innovative Medicines (the Netherlands Association for Innovative Pharmaceutical Companies) would like to take the opportunity to hereby respond to the 'Commission notice on the concept of 'similar medicinal product' in the context of the orphan legislation: adaptation to technical progress'.

We welcome the clarification of certain provisions of Regulation (EC) No. 141/2000 on Orphan Medicinal Products, as fifteen years have passed since the adoption of this milestone regulation and significant technical progress has been achieved. Nevertheless, we stress the importance of the original goal of the Regulation, which aimed at stimulating the research, development, marketing and delivery of orphan medicinal products (OMPs) to patients suffering from rare diseases.

The Orphan Regulation is beginning to have positive effects, but more efforts are needed

Until 2000, research in the field of rare diseases was limited. Due to the complexity of rare diseases (small, heterogeneous, and highly dispersed patient populations) and the lack of commercial attractiveness, (bio)pharmaceutical companies were generally unable to successfully develop suitable medicines. Patients with a (severe) rare disease could therefore not benefit from appropriate treatments, since only a few medicines were available for them. Nonetheless, patients affected by a rare condition deserve access to the same high quality of treatment as other patients with more common conditions.

Since then, the Regulation (EC) No. 141/2000 has successfully stimulated the research and development of OMPs to treat rare conditions. And with positive results: today more than 110 OMPs are approved in Europe, providing patients with continuous treatment improvements in various disease areas.

However, the unfortunate reality is that many rare diseases do not have a treatment available yet. The number of OMPs actually getting to the market remains flat if compared to the more than 1100 orphan designations granted by the EMA. A granted orphan designation is not a guarantee for the successful development of an effective treatment. With a total of more than 6000 existing rare conditions, there is still a long way to go to efficiently tackle all these unmet medical needs.

HollandBIO and the Association Innovative Medicines therefore would like to stress the importance to maintain a favourable and predictable environment to defend and further encourage the research on innovative therapies treating rare diseases, especially in those areas where there is high unmet medical need.

Definition of similar active substance for biological medicinal products needs further consideration

We agree that the requirements for demonstrating similarity will differ from product type to product type. The changes proposed for chemical medicinal products and radiopharmaceutical medicinal products are appropriate. However, the changes proposed for biological medicinal products require further consideration and revision. Since the regulation was developed, there have been major developments in the field of biological medicines. These developments are still rapidly progressing. Therefore three issues should be taken into account:

- Further defining and agreeing on the similarity criteria for ATMPs is untimely. Therefore, we agree with the use of broad points in the text.
- The definition for monoclonal antibodies would benefit from greater clarity.
- The potential similarity for medicines with more than one active substance should be addressed in this document too.

For specific comments, as well as for the line-by-line comments, we would like to refer to the document drafted by the EFPIA – EuropaBio Joint Task Force on Orphan Medicinal Products and Rare Diseases that we fully support.

Despite the first important successes of the Regulation, still too many patients suffer from rare diseases. Only if we strive together for an environment that supports innovation, development of and equal access to OMPs we are able to eventually improve the lives of *all* patients with rare diseases.

With kind regards,

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Response by:

HollandBIO: the Dutch biotech industry association. HollandBIO is the Dutch biotech industry association connecting, supporting, and representing approximately 140 medical, agro-food, and industrial biotech companies. Our members are active in all phases of research and development and include all company sizes: start-ups, SMEs, listed companies and multinationals. Together, we strive for a society taking full advantage of the power of biotechnology in health, food and sustainability.

The Association Innovative Medicines: the Association for innovative medicines in The Netherlands is the industry association for the Dutch branches of innovative pharmaceutical companies. The association is not active in the field of generic or non-prescription drugs but is strongly involved with companies focused on innovative (bio)pharmaceuticals. We currently represent 43 companies.