AmCham EU
SPEAKING FOR AMERICAN BUSINESS IN EUROPE

Consultation response

Revision of the EU legislation on medicines for children and rare diseases – Inception Impact Assessment

AmCham EU speaks for American companies committed to Europe on trade, investment and competitiveness issues. It aims to ensure a growth-orientated business and investment climate in Europe. AmCham EU facilitates the resolution of transatlantic issues that impact business and plays a role in creating better understanding of EU and U.S. positions on business matters. Aggregate U.S. investment in Europe totalled more than €3 trillion in 2019, directly supports more than 4.8 million jobs in Europe, and generates billions of euros annually in income, trade and research and development

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Representing American companies operating in the EU, the American Chamber of Commerce to the EU (AmCham EU) is well aware of the differences and respective strengths of innovation ecosystems on both sides of the Atlantic. The options laid out in the inception impact assessment (IIA) risk undermining an EU framework that has proven largely successful, rather than preserving the EU's strengths and incorporating learnings from other regions.

In this context, it is important to consider the economics of innovation: the Technopolis/Ecorys report[1] shows that 50% of orphan medicinal products (OMPs) make less than €10m a year and just 14% make more than €100m. Recent studies demonstrate that investments in treatment of rare diseases remain marginal.[2] Overcompensation must not be overstated in the context of a research environment that has a high risk of failure and often incurs a loss of investments. Currently these risks are partially offset by providing incentives and stability for investors – eg 10 years of additional market exclusivity (ME) for OMPs, 6 month supplementary protection certificates (SPC) for completed paediatric investigation plans (PIPs) and 2 additional years of ME for paediatric OMPs.

The frameworks in the US and the EU share a similar philosophy establishing pull incentives to encourage R&D. The US environment has led to higher numbers of approved indications and designations due to significant advantages when compared to the EU:

- As a federal state the US grants tax incentives;
- Additional regulatory incentives through the 'fast track' and 'breakthrough therapy' designations;
- Higher willingness to pay, hence higher price incentives on average than in Europe; and
- The US is able to garner higher levels of private investment in innovation, despite the EU's production of high-quality public research.

The EU partially balances these differences by providing a stronger market exclusivity incentive.

At a time when China is improving its intellectual property (IP) system, it is surprising to see Europe caveating or reducing IP and regulatory incentives when it has the ambition for its industry to be a 'world leader' and 'innovator'. Reducing average market exclusivity or rewards for research in paediatrics, narrowing the orphan designation criteria, or limiting incentives and rewards to areas where no other treatments exist will not improve R&D or address the challenges identified by the Commission. It may however erode Europe's long-term ability to provide leadership in innovation and, in a worst-case scenario, its ability to provide access to cutting-edge treatment to patients.

AmCham EU encourages the Commission to explore proposed novel regulatory rewards that complement the current system – eg, adopting well-functioning features from the US system, such as transferable vouchers or access to automatic priority review for medicines obtaining an orphan designation or a subset of them. On the Paediatric Regulation a number of measures would improve implementation, including better definitions of unmet medical needs and improved integration of regulatory and scientific dialogue for PIPs.

When considering the measures laid out in the IIA, in the Pharmaceutical Strategy and in the previously enacted SPC manufacturing waiver together, (US) investors and biopharmaceuticals companies perceive a strong imbalance in favour of the generic industry. This raises the question of the concrete actions the EU plans to undertake to strengthen Europe's attractiveness for investments in innovative medicines development.

[1] Thyra de Johng et al., 'Study to support the evaluation of the EU orphan regulation' (July, 2019).

[2] Emilie Neez et al., 'Estimate impact of the EU Orphan Regulation on incentives for innovation' (October, 2020).

'The joint evaluation of the two Regulations has shown that both legislative instruments have stimulated research and development of medicines to treat rare diseases and of medicines for children'.[3] We believe the Commission, if it wants the EU to remain an attractive location for investment, should seek to stimulate and not stifle R&D by incentivising the efforts of industry and creating the necessary environment to innovate and tackle unmet medical need rather than pursuing the potentially restrictive actions present in all options of the IIA.

[3] European Commission,'Inception Impact Assessment - Revision of the EU legislation on medicines for children and rare diseases', 1.