The European Commission's Pharmaceutical Strategy

With the European Commission's new pharmaceutical strategy announced in 2020, legislative changes are on the horizon for the European Life Science market. From orphan drugs to paediatric medicines, staying abreast of crucial updates and proposals is key for pharma companies

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On 25 November 2020, the European Commission announced a new pharmaceutical strategy for Europe (the EU Pharma Strategy), as part of its vision to build a European Health Union. This is stated to be a patient-centred strategy that aims to ensure the quality and safety of medicines, while boosting the sector's global competitiveness. As part of this, the Commission is considering a number of legislative reforms that are likely to affect the regulation of the entire life cycle of medicinal products and medical devices.

We provide an update on the current position below, with a focus on the changes relating to orphan (rare) and paediatric medicines, and in particular the rewards and incentives available to companies who develop such products.

Background and Focus of the Strategy

The EU Pharma Strategy was introduced following the impact of the COVID-19 pandemic and its effect on access to medicines in the EU. The global context at the time highlighted the issues of unequal access to, and shortage of, medicines, as well as demonstrating that innovation was not meeting needs nor being efficiently harnessed.

Moreover, new technology and scientific developments (such as AI, gene therapy, and personalised medicines) and environmental risks (such as antimicrobial resistance) meant a change in approach was required to meet modern healthcare needs.

With this in mind, the EU Pharma Strategy focuses on three areas:

1. The main area of focus concerns the availability, accessibility, and affordability of medicinal products. This aims to ensure patients' timely access to, and the availability of, innovative medicines, especially orphan and paediatric medicines. There is a large amount of data to show that access to medicines across the EU is highly varied, and that innovative medicines are not available to patients in a timely manner. This means, amongst other things, that scientific and technological developments cannot be fully exploited

2. Linked to this, the second area of focus is streamlined innovation and enhanced competition. This involves fostering research and the development of medicines for rare diseases and for children, especially in the areas of highest unmet need

3. The third area of focus is securing supply and controlling shortages of medicinal products. This has been addressed through, among other strategies, EU-level reporting of critical shortages and mandatory shortage risk management plans

Orphan and Paediatric Legislation

The Orphan Regulation (EC) No 141/2000 and Paediatric Regulation (EC) No 1901/2006 were introduced to provide incentives for the development of medicines for rare

diseases or for children, where there may not be sufficient return on investment given the patient numbers and difficulties in conducting relevant research. Under these regulations, for orphan products, as a reward for developing a medicine for a rare indication, 10 years of marketing exclusivity is available if certain conditions are met.

For paediatric medicines, there are obligations to conduct paediatric research as part of an application for a new marketing authorisation, but rewards are available for such research if completed in line with the agreed investigation plan, including an extension to the supplementary protection certificate (SPC) on the patent that may cover the product.

Despite the marked advance in medicines for children and the treatment of rare diseases since the regulations were introduced, there remain significant areas of unmet need: an estimated 95% of rare diseases still do not have a treatment option. The Commission therefore believes that the legislation is not sufficiently addressing the highest unmet needs, particularly for children.

With this in mind, the Commission published an Impact Assessment in November 2020 in which it proposed various options to amend these regulations, and invited citizens and stakeholders to provide feedback. The possible options included changing the current rewards system, and introducing an 'unmet need' criteria or additional definitions to better identify rare diseases.

Current Proposals

Reports suggest that the Commission is currently working to finalise the proposed changes to the regulations following internal review and consultation with key stakeholders. In general, the Commission intends to streamline marketing authorisation procedures to better support companies bringing new products to market. This could include mechanisms such as shorter review timelines; the use of new concepts like real world evidence, rather than a full randomised controlled clinical trial; use of electronic submissions; and the optimisation of the working methods of the European Medicines Agency (EMA) and the regulatory network.

In relation to rewards and incentives specifically, the Commission is considering introducing a shorter base period of protections for products, and the likely capping of extensions to these base periods. Criteria for unmet medical need based on the severity of the disease and the characteristics of the product is likely to be developed, as well as for high unmet medical need (HUMN), which are medicinal products intended for conditions with no available treatment. These products may benefit from additional or better exclusivities and regulatory support from the EMA.

In relation to orphan medicines, the Commission is considering a limitation on the validity of orphan designations to try to reduce the number of such products – there is a view among Member States that the high number of orphan products leads to high prices and therefore a reduced access for these products. For example, when calculating the prevalence of a disease in order to determine if it will be considered as orphan, there could be a concept of cumulative prevalence for all orphan conditions targeted by the same product, and a review of the orphan designation criteria to account for technological and scientific developments.

Regarding orphan exclusivity, one scenario proposed is for variable exclusivity periods being available depending on the orphan product, for example:

- The longest exclusivity being available if the product addressed HUMN (between 8 years to 10 years)
- Shorter period of exclusivity for new active substances not previously on the EU market within a medicinal product (between 6 years to 8 years)
- Shortest exclusivity period for all other orphan products (e.g., for development of existing products – around five years)

In addition, an extra period of exclusivity (of around 1 or 2 years) is being considered for orphan medicinal products made available in all EU Member States in order to try to

increase access across all Member States and patients.

In relation to paediatric medicines, the Commission proposes to reform and simplify the paediatric investigation plan mechanism, including changes to waivers (for which companies can avoid conducting paediatric research for certain products if justified by the characteristics of the product or disease) and a limitation of deferrals (where paediatric research can be deferred so the product is not delayed on the market), and to encourage research through additional funding and early-access mechanisms.

The Commission is also considering various options for incentivising innovation in paediatric products. That could include an additional SPC extension for medicinal products that address a paediatric unmet medical need. Medicinal products that address a paediatric unmet medical need may also get additional exclusivity if placed on the market in all EU Member States within a set time period.

Current Status of the Proposals

The current understanding is that the initial proposals were rejected through the internal final quality review process and that revised proposals are undergoing intense discussion within the Commission and with key stakeholders. The original intention was for the draft legislation to be released in December 2022, but it is now understood that this will be delayed into 2023.

Industry has highlighted a number of concerns with the proposals under the EU Pharma Strategy, and whether they will meet the aims and issues identified. For example, one of

> *"Aiming to reduce the period of market exclusivity, narrow development incentives, promote faster generic competition and cap the aggregate number of patients that can benefit from a designated orphan product, is likely to drive research investment away from meeting the needs of patients and weaken a sector at the very heart of EU competitiveness and service to citizens."*

the key issues identified by the Commission is the variation in access across the EU. However, a significant factor in this variation is the pricing and reimbursement processes within each country (that is not under the control of the Commission), not with the authorisation of medicines or the rewards or incentives for product development.

Further, restricting or limiting incentives may reduce or even prejudice innovation, thereby further reducing access. Indeed, EFPIA's response to the consultation stated that:

Therefore, there are concerns about whether the proposals will adequately address the key concerns identified.

Based on current drafts that have been made public, it is expected that the system of incentives and obligations in the pharmaceutical legislation will be revised, which will involve the potential shortening of protection periods for some, if not many, medicinal products, and more tailored incentives depending on need. It is also likely to set out new regulatory expectations or obligations, such as the obligation to market or to file for pricing and reimbursement status in many or all EU Member States, or to comply with new definitions of unmet medical need and/or high medical need.

In the longer term, an aim is for the creation of a less fragmented IP system (introducing a unitary patent and system of centralised litigation before the new Unified Patent Court, and possibly a unified SPC grant system), and the improvement of access to generic and biosimilar medicines, as well as to gene therapy and personalised medicines.

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