EPSCO Council (Health) Policy Debate on the Pharmaceutical Package

EFPIA Considerations on the proposed Questions for Discussion

Question A | Modulation of regulatory data protection periods: proposed way forward.

→ ***Can you agree to a modulated incentives system?***

A modulated incentives system could ONLY be acceptable IF regulatory data protection duration is NOT conditioned on market access and IF the other conditions (e.g. unmet medical need, comparative clinical trials, new indication) are proportionate and fair to stimulate research and development into novel therapies that fulfil public health objectives.

Regulatory data protection is an important form of intellectual property protection (because of the type of data collected for the marketing authorisation process) that is critical for R&D investment decisions. Modulating regulatory data protection duration based on market access raises concerns about predictability and impact on investment decisions. Other proposed modulations relating to unmet medical needs and comparative trials are under-powered in terms of the incentive they offer relative to the costs and time incurred to reach the objectives, and/or have narrow, impossible-to-predict success criteria.

Bold incentives are needed to attract investment and improve Europe's competitiveness. Reducing existing incentives and providing for a limited clawback linked to measures that are partially outside of industry's control is not only not going to improve access, but it will also worsen the existing trend of innovation leaving Europe thereby ultimately further reducing access in the long run. The proposal should not connect the incentives system for research to market access, which occurs 15 years after research has been incentivised.

Since incentives have the role to stimulate investment in R&D, any proposal offering additional incentives based on conditions requires that those conditions:

- Are proportionate and reasonably in the control of the marketing authorisation holders that make se of the incentive,
- Avoid one-sided subjective notions (such as "reasonableness" or "in good faith"),
- Can factually be measured as having been satisfied (or not) at a given fixed point in time,
- Do not penalise manufacturing authorisation holders for inaction or due to process failings at the level of national competent authorities,
- Do not disproportionately increase the risk of litigation.

→ ***Do you agree with the conditions (register, capping, modulated one year with market protection, reduction of basic RDP) mentioned in this paper?***

Public register

Yes. We could support a public register collecting **genuine, factual, and publicly available information with protection periods**.

11 years cap

No. Europe has lost ground as a location for R&D investment to the US and China over the last 20 years. **As requested by EU leaders in March 2023, this trend must be reversed by strengthening, not weakening, incentives for medical innovation. This means we should increase the cap.**

Modulated one year market protection

Yes. We support the **allocation of one year of market protection instead of one year of RDP** for an additional therapeutic indication.

Reduction of RDP baseline

No. Reducing the data exclusivity baseline would be a step in the wrong direction, diminishing the EU's attractiveness for R&D investment. According to published studies Commission's RDP proposal could lead to a decrease in investments for products that are more susceptible to replication, irrespective of the benefits they offer patients and even more so in complex products that require lengthier clinical trials (estimated loss of 50 out of the 225 products in development in the next 15 years). A blanket reduction of the baseline by a full two years – and making its recovery unpredictable and in practice not feasible to achieve, further dependent on factors outside of a company's control, i.e., the release and continuous supply of a given medicine in all 27 Member States within two years of marketing authorisation – will only erode confidence needed to support reliability of investing precious R&D resources in the EU.

OME: *with regard to orphan medicinal products the Presidency considers that the proposal, to have a basic market exclusivity period of nine years, with a further one year for 'high unmet needs' strikes the right balance.*

No, rather than reducing OME, additional incentives should be proposed to encourage further investment in rare disease and therapeutic areas with particularly acute scientific challenges. This means:

- Ensure a strong OME baseline,
- Offer pathways for OME modulation (underserved areas, further indications, additional OME or the current six-month SPC extension for completion of their obligations under the paediatric section),
- A maximum period of exclusivity is proposed to provide certainty to other stakeholders. This is at par with the maximum protection provided by SPC,
- The duration of OME could be reduced for well-established use products to 7 years, as there will already be existing knowledge and data about these products.

The EC proposes to introduce a Global (Orphan) Marketing Authorisation, which would eliminate concerns around "salami slicing" by awarding marketing authorisation an orphan incentive at the product level rather than indication level. **This proposal could be supported, as long as the development and approval of additional therapeutic indications is encouraged.**

Question B | Market access incentive: four options for a way forward.

→ ***Do you agree that incentives should be used as the way forward to improve access?***

No. The proposal to link regulatory data protection with measures to launch and continuously supply products in all Member States will hinder rather than foster market access of innovative products and disregards the root causes of access discrepancies. The root causes are multifactorial and

complex, and a one-size-fits-all EU regulatory measure will not address the challenges that mainly fall under the P&R remit of Member States. The speed of health technology assessments, different reimbursement processes or additional layers of regional and local decision making are all factors affecting barriers to access. Duplicative or inconsistent evidence requirements can also cause delays. For example, different countries, HTA bodies and payers may require different endpoints or some accept real world evidence where others do not. The use of external reference pricing also leads to unavailability. Many Member States do not start their national pricing and reimbursement processes until they have access to the reimbursement decisions from at least 5 other countries. **An evidence-based multistakeholder dialogue should devise value-based solutions addressing access challenges holistically and not a linkage between incentives for R&D such as RDP and access conditions no single stakeholder can fulfill alone.**

Moreover, the legality of Article 114 TFEU as the legal basis for this is highly questionable. Article 114 is the correct legal basis for legislation intended to overcome divergences in national laws that result in geographic fragmentation, obstructing the fundamental freedoms and the proper functioning of the internal market. The goal of improving access to medicines tied to national patient needs is inherently not an internal market concern and usurps the exclusive competence of EU Member States embedded in Article 168 TFEU.

→ ***Which option (set of conditions) described in this paper could you support? If none, under what conditions could you agree to a possible solution for the access-issue?***

None. Access issues cannot be solved through EU legislation. EFPIA calls for a revised approach to achieve a better balance in improving patient access to new medicines, while incentivising innovation and maintaining international competitiveness. In recent years, other regions of the world such as the US and China have become more competitive and attractive for innovators. We are seeing companies increasingly shifting resources such as research and development, clinical trials, advanced manufacturing and scientific and academic skills to areas with more ambitious life science strategies. We have witnessed a 25% fall in our share of global pharma R&D investment over the past two decades. Considering the sector contributes more to the EU balance of trade than any other, the stakes could not be higher.

EFPIA and its members have worked on a series of concrete proposals to improve patient access to innovative medicines and reduce inequalities across Europe. These include:

- A commitment from the industry to file pricing and reimbursement applications in all EU countries no later than 2 years after EU market authorization. This commitment reflects the joint ambition of industry and society to make innovation for unmet health needs available for patients and health systems across Europe as soon as possible.
- A framework for Equity-Based Tiered Pricing (EBTP), to ensure that the price of innovative medicines can vary between countries depending on their economic level and ability to pay, anchored in a principle of solidarity between countries. EFPIA developed a conceptual framework for how EBTP could be implemented in the EU in a way that is workable and pragmatic and in respect of the national prerogative for pricing & reimbursement policies, in order to benefit patients across Europe and create win-win solutions for all parties.
- The creation of a portal where marketing authorisation holders (MAH) can provide timely information regarding the timing and processing of pricing and reimbursement (P&R) applications in the various EU-27 countries, including the reasons why there is a delay in the P&R decision or why the MAH has not filed in a particular market. The portal enables an improved understanding of the functioning of the market for innovative medicines in Europe, and cast light on access delays and barriers that should be addressed by policymakers and economic operators at the appropriate level.

We stand ready to play our part in reducing the time it takes for patients to access the medicines we discover, develop and deliver. However, we cannot do this on our own. **Solving the access issue requires action from multiple actors, including Member States.** The **WHO Novel Medicines Platform** where EFPIA is participating together with Member States, NGO's and other industry stakeholders to co-create solutions for improved access to innovative medicines in Europe is an example of best practice.

Question C | Unmet medical need incentive and high unmet needs for Orphan Medicinal Products: proposed modification.

→ ***Can you agree to a UMN incentive (for normal and orphan medicines) under certain conditions? Do you agree with the conditions set out in this paper for such a system and what possible additional conditions would you like to see?***

Yes, but only under specific conditions (less restrictive criteria and with the involvement of all interested stakeholders). An excessively narrow definition of unmet medical need risks excluding the development of important therapies for patients. It will lead to the unintended consequence of disincentivising companies to invest in R&D that may have addressed patients' unmet medical needs. Furthermore, individual patients value the impact of new treatments differently than society, which may place a higher value on incremental improvements of diseases with a high societal burden or that help avoid future pandemics. **Therefore, prioritising at disease level is not adequate and should be avoided, while introducing a relative assessment at product level could be prioritised. The effect criterion has to include mortality, morbidity, and quality of life.**

It is of critical importance that the appropriate stakeholders are involved in identifying unmet medical needs from different perspectives. Collaborations need to be established to get an aligned understanding of UMN. These multi-stakeholder collaborations should involve representatives from diverse patient groups, broader societal and health care system stakeholders as well as industries.

In the case of orphan diseases, having a distinct category of High Unmet Medical Need is challenging for many reasons, not least because it raises ethical concerns: defining an UMN as "high" implies that other UMNs are of less importance, either to patients or society, which would be inappropriate. Therefore, additional incentives to encourage further investment in areas with particularly acute scientific challenges are important. **It is suggested to condition an enhanced orphan market exclusivity protection to objective criteria, which would provide sponsors with sufficient certainty to undertake investments in particularly challenging and high-risk conditions, without suggesting a scale or gradation of different levels of UMN.**