



European Federation of Pharmaceutical
Industries and Associations



4th Nordic Conference on Paediatric Medicines

How to make treatments for rare diseases less rare?

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Presentation



A stage set with several spotlights shining down on a grid structure. The text "Setting the Scene" is overlaid in white.

Setting the Scene



Pharmaceutical Strategy for Europe

- Adopted in November 2020
- 4 main objectives

To ensure access to quality and safe medicines, while boosting industry global competitiveness



What to expect in the next five years?



- More than 50 different legislative and non-legislative actions are proposed

- Review of:

- The 2001 Pharmaceutical Directive (2001/83)
- The 2004 EMA Regulation (746/2004)
- The 2000 Orphan Drugs Regulation (141/2000)
- The 2006 Paediatric Regulation (1901/2006)



Basic pharmaceuticals legislation, so called "Pharma Acquis", proposals to be adopted by Q4 2022



Commission proposals expected by Q4 2022





Intellectual property is the key driver of medical innovation

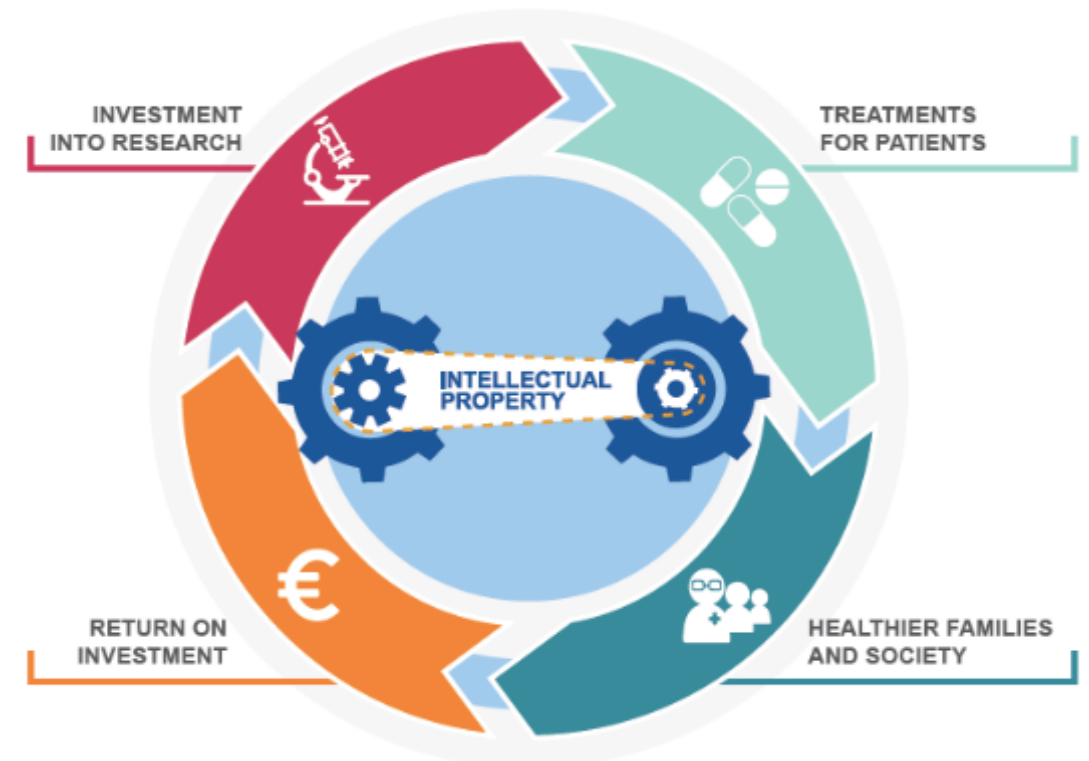
Without IP, there would be no medicines

It generally takes 12-15 years and costs more than 2 billion EUR to develop a new drug + the development time has been increasing over the years

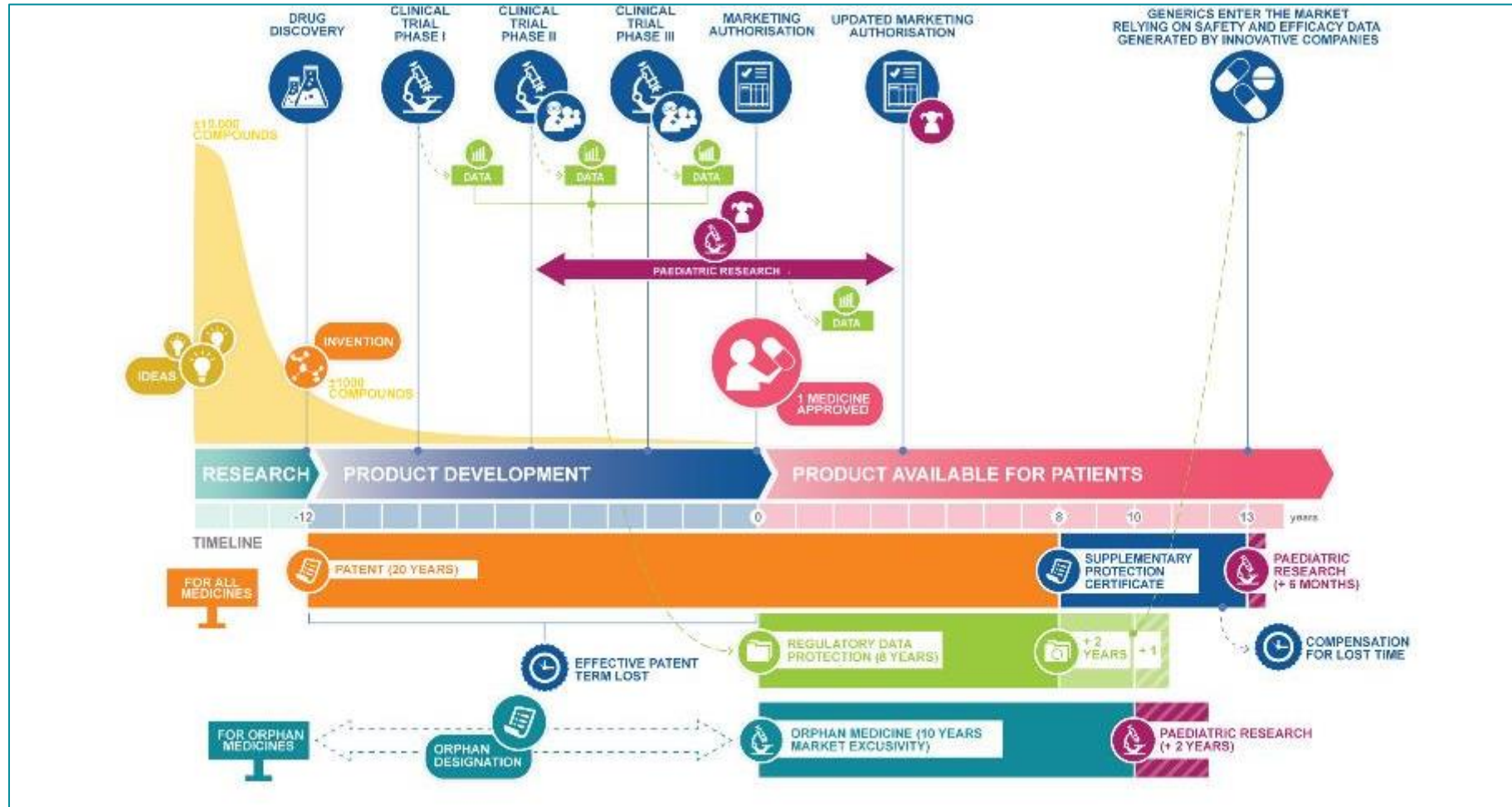
IP allows pharmaceutical companies to take the risk to invest in the long, complex, risky and costly R&D process

Without IP protection, there is no incentive to invest in medical innovation, and without innovation, there will be no new therapies

INTELLECTUAL PROPERTY DELIVERS ACCESS TO TODAY'S MEDICINES AND INVESTMENT INTO TOMORROW'S CURES



How the EU is fostering research and development today



The EU Orphan Legislation introduced in 2000 aimed at addressing the significant scientific and market failures inherent to rare diseases

Investments in rare diseases represent an uncertain economic case, as a result of their poorly understood pathophysiology, difficulty in conducting clinical trials, and low volume of sales

Before the OMP Legislation, economic incentives in the rare disease space were **scarce**



Low levels of R&D activity in the orphan space, as a result of scientific hurdles and market failures¹



Therapies approved in **only 8** of the **6-7,000** known rare diseases¹



Major **unmet medical need** for patients suffering from rare diseases¹

The OMP Legislation introduced **incentives** to support the development of orphan medicines¹

Market exclusivity, which grants the MA holder an additional temporary exclusivity right

Protocol assistance, which allows the sponsor to request scientific advice from the EMA

Fee waivers, which offers total or partial exemptions from the payment of application fees

Aid for research, which creates incentives to stimulate the R&D of OMPs

1. European Commission, 2020. EMA: European Medicines Agency; EU: European Union; MA: Marketing authorisation; OMP: Orphan medicinal product; R&D: Research and development

Orphan Regulation is an EU success story: delivering treatments for up to 6.3 million patients with rare conditions

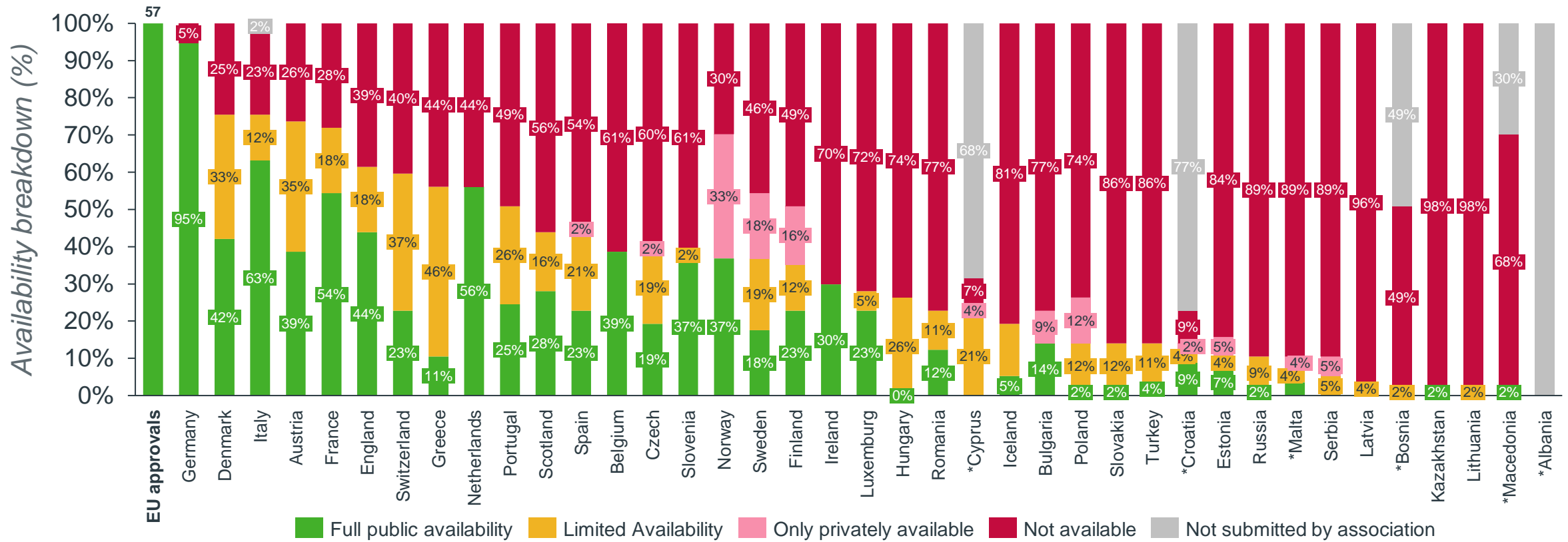


- Since 2000: 160+ new treatments authorised
- By 2019: 2100+ designations
- The Regulation raised the profile of rare diseases, built a community and networks
- EU Reference Networks (ERNs) improve the sharing of knowledge

What is driving the debate: Availability of Orphan medicinal products in Europe

Number of medicines available to patients in 39 European countries (point at which the product gains access to the reimbursement list)

95% in Germany vs. 2% in Poland and Slovakia

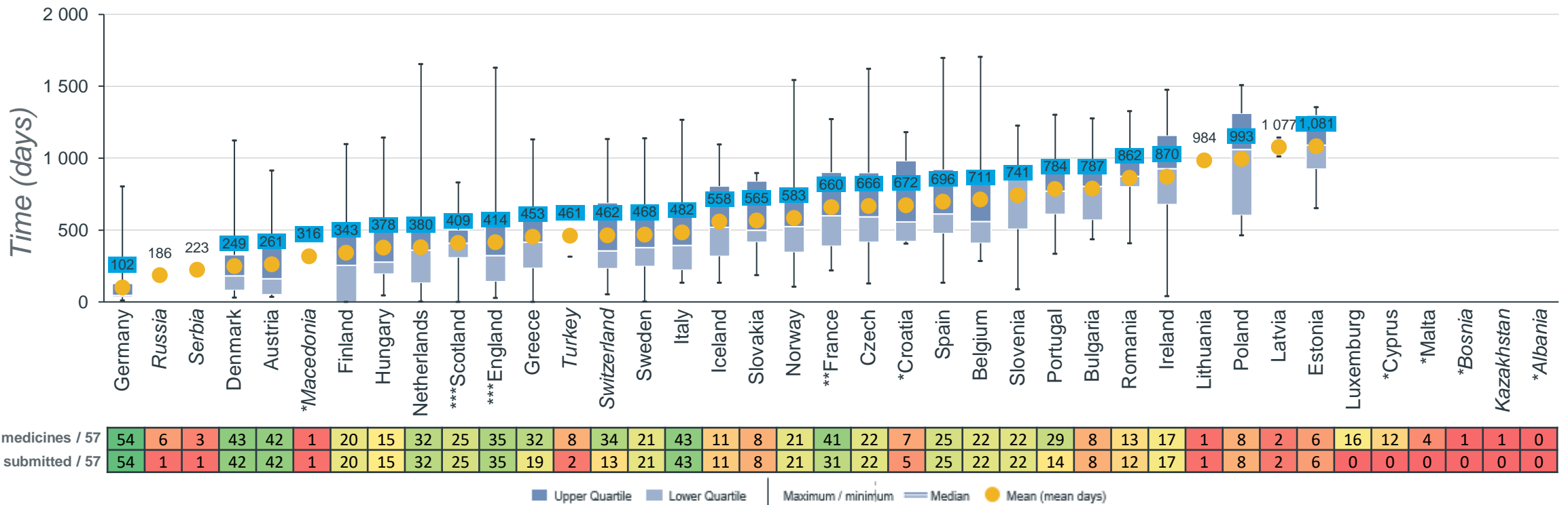


Source: EFPIA/IQVIA, Patients W.A.I.T. Indicator, April 2022

Delays and time to availability of Orphan medicinal products

Days between marketing authorisation and the date of availability to patients in 39 European countries (point at which the product gains access to the reimbursement list)

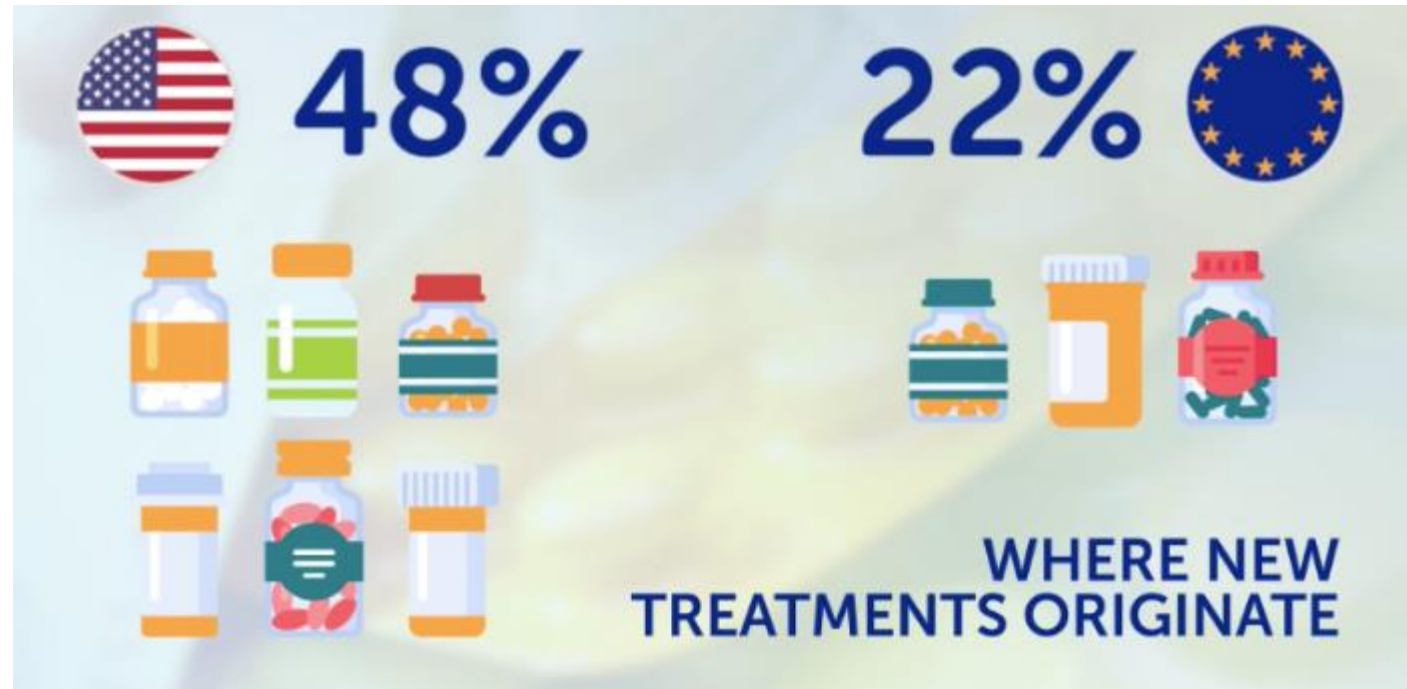
Eight-fold difference: 102 days in Germany vs. 862 Rumania, over 2 years more



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| Available medicines / 57 | 54 | 6 | 3 | 43 | 42 | 1 | 20 | 15 | 32 | 25 | 35 | 32 | 8 | 34 | 21 | 43 | 11 | 8 | 21 | 41 | 22 | 7 | 25 | 22 | 22 | 29 | 8 | 13 | 17 | 1 | 8 | 2 | 6 | 16 | 12 | 4 | 1 | 1 | 0 | |
| Dates submitted / 57 | 54 | 1 | 1 | 42 | 42 | 1 | 20 | 15 | 32 | 25 | 35 | 19 | 2 | 13 | 21 | 43 | 11 | 8 | 21 | 31 | 22 | 5 | 25 | 22 | 22 | 14 | 8 | 12 | 17 | 1 | 8 | 2 | 6 | 0 | 0 | 0 | 0 | 0 | 0 | 0 |

Source: EFPIA/IQVIA, Patients W.A.I.T. Indicator, April 2022

Simultaneously: Europe's research and development base is gradually eroding



Only through a future-proof regulatory framework, and a robust and predictable intellectual property and incentives ecosystem, can Europe become a true world-leader in medical innovation.

A holistic set of solutions to address unmet medical needs in orphan and paediatric diseases

Orphan and paediatric regulations are EU success stories, enhancing paediatric-specific expertise and delivering treatments for up to 6.3 million patients with rare conditions



Unmet medical needs

are failing to be addressed by innovation and Regulations may not be future-proof

Access

is incomplete and inequitable, preventing widespread patient benefit

Incentives

are not appropriately balanced



Need holistic solutions to advance clinical and economic viability

Progress on access only possible with significant Member State commitments and P&R reforms

Existing incentives remain essential. Potential to refine the incentive system to encourage investment in areas of unmet needs

A holistic set of solutions: Unmet medical needs



1. Unmet medical need: EFPIA calls for a 'Moonshot' rare and paediatric diseases

- Limited understanding of disease pathophysiology and of potential drug targets precludes any investment
- The *Moonshot* aims to establish a mindset of concerted effort towards developing the basic science and accelerating translational R&D as prerequisites for clinical development

2. Tackling unmet medical need in children – Mechanism of Action Paediatric Investigation Plan

- EFPIA proposes a framework in which the PIPs can evolve in a more scientific manner to address UMN
- PIPs can be proposed by companies based on the **Mechanism of Action (MoA)** of the product, if there's sufficient scientific evidence that a product could meet the UMN in a paediatric condition that is different than the adult condition.

A holistic set of solutions: Definition of Condition & Incentives



3. Harmonisation in understanding is key - Update to the way “a condition” is defined

- The concept of a “condition” is critical to define Orphan Designation and Orphan Market Exclusivity, and to define paediatric investigation plan (PIP) obligations, waivers and deferrals.
- EFPIA proposes to update this **Definition of Condition** in tune with current science on the cause(s) for the disease and the organ(s) in which it can occur.

4. Incentives: Existing incentives remain essential

- Potential to refine the incentive system to encourage investment in areas of unmet medical needs
- EFPIA has developed a proposal for OME modulation based on clear and predictable criteria
- EFPIA proposes to retain 10 years as the baseline OME duration, to be modulated up or down based on certain product attributes

Several EFPIA concrete proposals to improve patient access to innovative medicines and reduce inequalities across Europe

1. **A commitment from the industry to file pricing and reimbursement applications in all EU countries no later than 2 years after EU market authorisation.**
2. **The creation of a European Access Portal** where marketing authorisation holders 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.
3. **A conceptual framework for Equity-Based Tiered Pricing (EBTP)**, to ensure that ability to pay across countries is considered in the prices of innovative medicines, anchored in a principle of solidarity between countries, to reduce unavailability of new medicines and access delays.
4. **Novel payment and pricing models**, when used appropriately and tailored to the situation, can accelerate patient access, allowing payers to manage clinical uncertainty, budget impact and sustainability of the healthcare system, whilst providing sufficient incentives for innovation.
5. Contributing to achieving an **efficient system of European assessments of relative efficacy at time of launch** in the context of the implementation of the Health Technology Assessment (HTA) Regulation.

Now is the time.





Thank you!



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