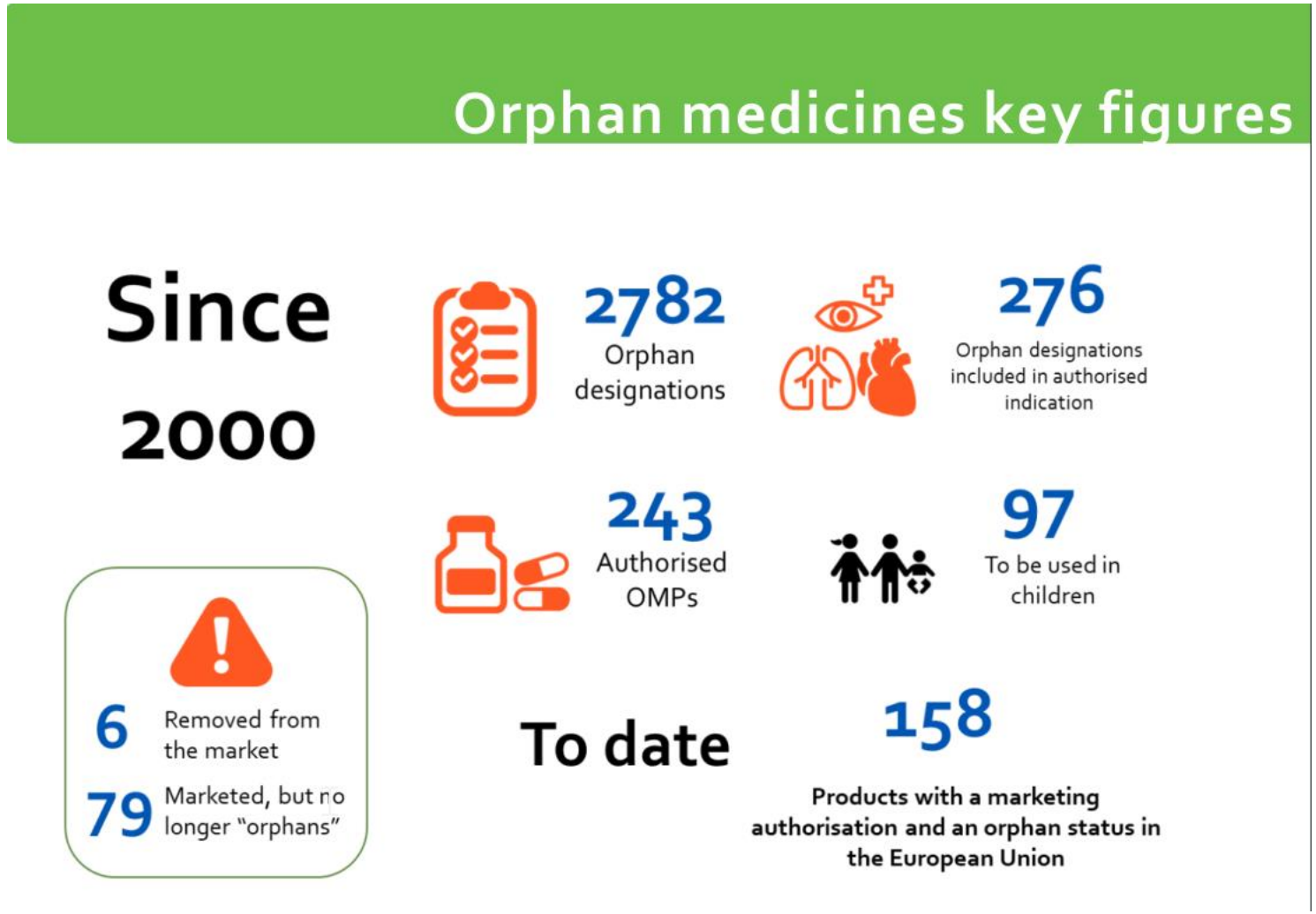
The EU Pharmaceutical Reform - orphan and paediatric medicines

Open Academy Alumni Meet Up

28 September 2023

Treatment authorisation in Europe



But …

Only 5% had received a transformative treatment approved for the entire European Union, with 69% of rare disease patients having received only symptomatic treatment for their rare disease

22% of people with rare diseases could not get the treatments they needed because it was not available where they live, reflective of the fragmentation of the market across the 27 Member States

Part of our strategic goals

By 2030, EURORDIS will have made contributions to the goals of (Based on the Foresight Study Rare 2030):

- Earlier, faster and more accurate **diagnosis** – goal of diagnosis within 6 months

- High-quality national and European **healthcare pathways**, including cross-border healthcare – a goal of improving survival by 3 years on average over 10 years and reducing the mortality of children under 5 years of age by one third

- **Integrated medical and social care** with a holistic life-long approach and inclusion in society – a goal of reducing the social, psychological and economic burden by one third

- **Research and knowledge development** that are innovative and led by the needs of people living with a rare disease

- Optimised **data and health digital technologies** for the benefit of people living with a rare disease and society at large

- Development and availability, accessibility, affordability of treatments, particularly transformative or curative **therapies** – goal of 1000 new therapies within 10 years

Five overarching principles for a responsible *evolution* of the RD incentives framework

To **transform** the European Research & Development for the rare disease ecosystem building upon advances of the past 20 years, for the next 20 years

To situate **Europe as a global leader in research, development and access**, through a regulation that is attractive for developers, and competitive globally.

To define a **model that is centered on the unmet needs** of people living with a rare disease, and includes **patient participation** in its establishment and implementation.

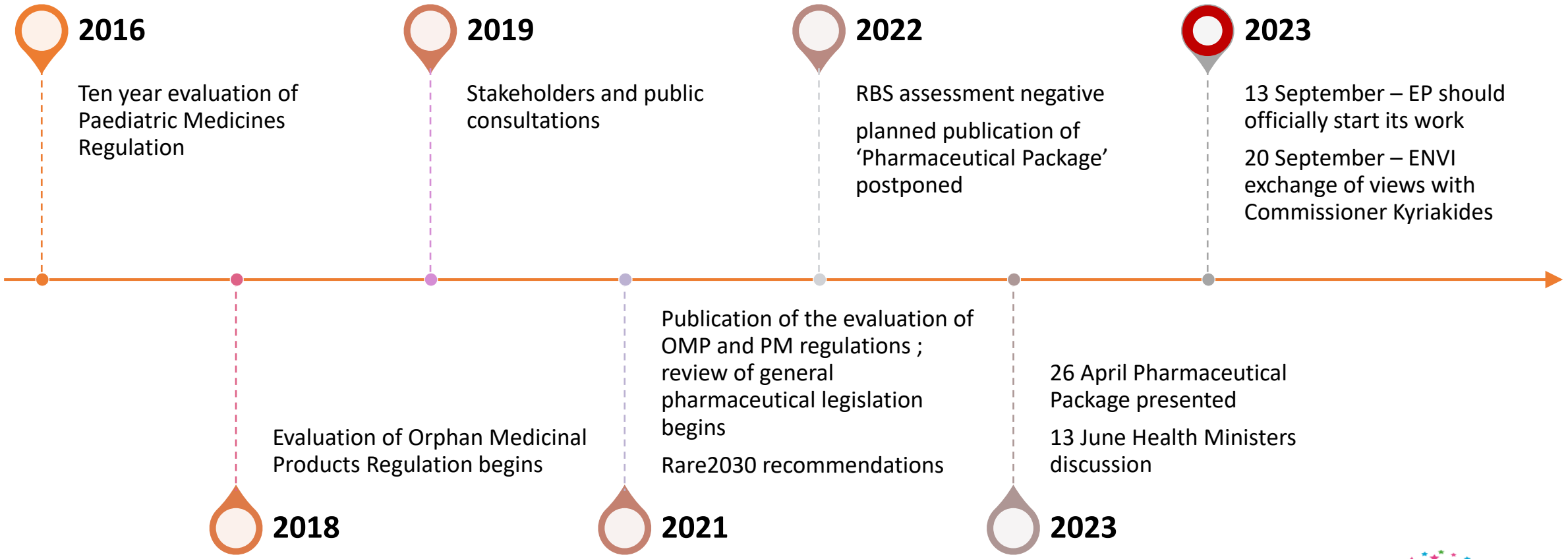
To establish a **European pathway**, from development to access, to ensure innovation coupled with **affordability** and to gain that crucial **strategic autonomy** in research and development.

To ensure **convergence** and **coherence** between different relevant legislations

What happened on 26 April?

- One ‘chapeau’ communication
- A new Directive and a new Regulation
- Council Recommendation on Antimicrobial Resistance

Where are we now? A long road so far



THANK YOU