It is time to move mountains

Third Multi-Stakeholder Symposium on Improving Patient Access to Therapies for Rare Diseases

Yann Le Cam
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In the next 15 minutes
The ambition of EURORDIS and its members

Breaking the Access Deadlock to Leave No One Behind

A Position Paper by EURORDIS and its Members

The ambition of EURORDIS is to have 3 to 5 times more new rare disease therapies approved per year, 3 to 5 times cheaper than today by 2025
In line with international ambitions

1. All patients coming to medical attention with a suspected rare disease will be diagnosed within one year if their disorder is known in the medical literature; all currently undiagnosable individuals will enter a globally coordinated diagnostic and research pipeline.

2. 1000 new therapies for rare diseases will be approved, the majority of which will focus on diseases without approved options.

3. Methodologies will be developed to assess the impact of diagnoses and therapies on rare disease patients.
Access remains a challenge

*Value in Health* 2018 21, 553-560 DOI: (10.1016/j.jval.2018.01.007)
Looking beyond the headlines: growth of OMPs spending in Europe has declined
High price medicines represents a fraction of OMPs

Source: IQVIA (2017)
Expenditures is focused only in specific diseases areas

Source: IQVIA (2017)
System failure and misconceptions are hampering access to treatments to those who need it the most

Calls for action on patients denied £100,000 cystic fibrosis drug

Exclusive: Ministers urged to consider revoking patent on life-extending Orkambi, which NHS cannot afford

The patients facing shorter lives due to drug’s expense
Where are we coming from

- **2016 – First Multi Stakeholder Symposium**
- **2017 – Second Multi Stakeholder Symposium**
- **2018 EURORDIS Paper ‘Breaking the Access Deadlock to Leave No-one Behind’**
- **2019 – Third Multi Stakeholder Symposium**

Highlighted challenges, areas of agreements, and proposed solutions

Proposing structured approach and future actions
What do we aim to achieve in the next two days?

Let’s make a pact to ensure patients’ sustainable access to rare disease therapies
Breakout sessions and related recommendations

- A New Blueprint to Spend Efficiently and Fast-track R&D
- Improving Multi-Stakeholder Early Dialogues to Optimize Determination of Value
- A Continuum Approach to Evidence Generation Linked to Healthcare Budget Spending.
- A Transparent European Cooperation Framework for the Determination of Fair Prices and of Sustainable Healthcare Budget Impacts
Moving forward – what we are suggesting

A structured collaborative approach between ALL players involved
Time for action is now

*The man who moves a mountain begins by carrying away small stones*  
(Confucius)
THANK YOU

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