EMA REGULATORY SCIENCE TO 2025

The point of view of patients

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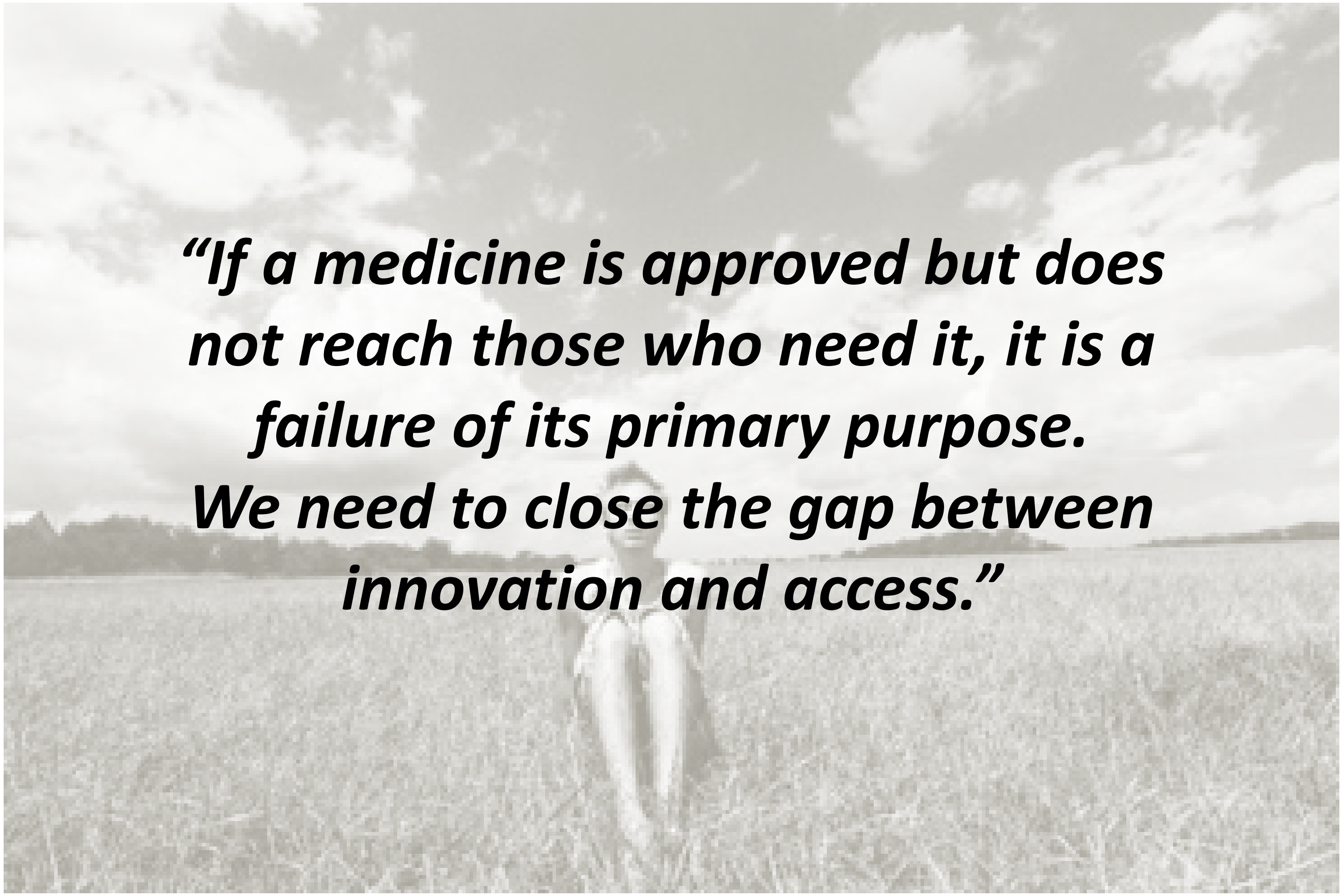
IN THE NEXT 10 MINUTES

OUR VIEW IN A NUTSHELL



"If a medicine is approved but does not reach those who need it, it is a failure of its primary purpose.
We need to close the gap between innovation and access."

THE CORE OF THE MATTER

Rare Barometer Voices
A EURORDIS INITIATIVE

Rare Barometer Voices survey on access to treatment
(February 2017 – 1350 respondents)

24% of rare disease patients surveyed could not get the medical treatment they needed in 2016 because **the treatment was not available where they live**

VS. 7% in the general population

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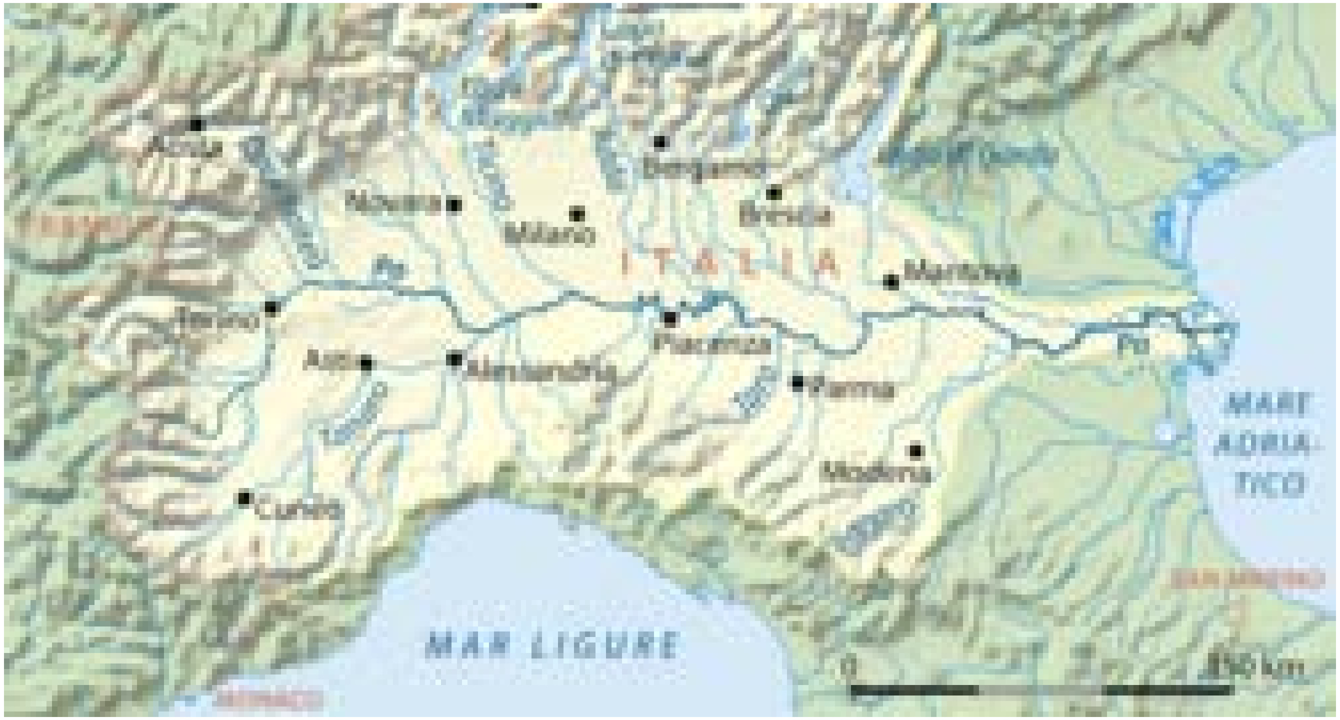
RARE DISEASES EUROPE

THE ROLE OF THE EUROPEAN MEDICINES AGENCY

The EMA should become an active enabler to address public health challenges and medical needs

IN PRACTICE, WHAT WOULD IT MEAN?

Upstream ⟷ **Downstream**



IN SUMMARY: A LOOK AT THE FUTURE – WHAT WE COLLECTIVELY NEED TO DO

1. **Access** - Bring the right treatment to patients at the right time
2. **Sustainability** – being able to afford the treatment and budget impact
3. **Horizon shaping** - Focus on unmet needs & curative treatments
4. **Investment** – Europe must continue to be attractive for R&D

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THANK YOU

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RARE DISEASES AT A GLANCE

OVER
6000
distinct rare diseases

Each one affects fewer than
1 IN
2000
PEOPLE

Affects between
6 % AND
8%
of the population in the course of their lives

NO
CURE
for the vast majority of diseases and few treatments available

All together, an estimated
30
MILLION PEOPLE
are living with a rare disease in Europe and
300
MILLION
worldwide

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810

member patient organisations

70 countries (28 EU countries)

41 National Alliances of RD Patients Organisations

58 European Federations of specific rare diseases

Founded in **1997**

Outreach to over

1800

patient groups

Over **440** Volunteers

+80 patient advocates

+250 moderators

40+

Staff members, with offices in Paris, Brussels, Barcelona

WHAT WE DO

ADVOCATING FOR PATIENTS

We advocate to ensure that healthcare and social policies and services, research, and the development of medicines take into account the real needs of people living with a rare disease and their families. Our advocacy is fuelled by real-life experiences of the rare disease community.

EMPOWERING PATIENTS

By uniting the rare disease community and building the capacities of patients, we empower them to become advocates equipped with the knowledge and skills needed to fight for better lives.

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ENGAGING PATIENTS

We make possible the engagement of patients in decision-making processes so that the patient voice is active, amplified and meaningful in research and medicines development, as well as in both healthcare and social policies and services.

PATIENTS FIRST