EURORDIS
Rare Diseases Europe

Collaborative research: Rare disease patients' perspective

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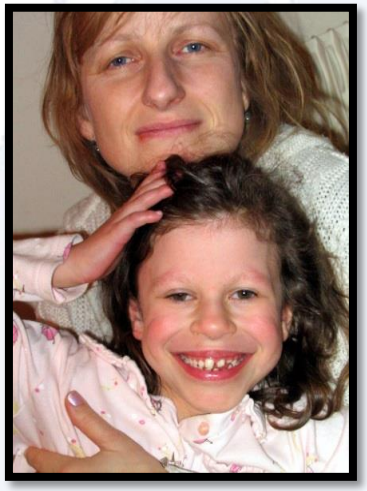
Brief presentation

- What is a rare disease?
- What is EURORDIS?
- Three main messages to improve collaborative research towards responding to UMN.

What is a rare disease?



A rare disease is defined in Europe as a disease affecting less than 1 in 2,000 citizens

- Many are of genetic origin
- Over 50% affect children
- Between 6000 – 8000 distinct rare diseases
- 6 to 8% of the EU population
- Estimated30 million people living with a rare disease in Europe

Characteristics of Rare Diseases

- Chronic, progressive, degenerative, heavily disabling and frequently life-threatening
- Patients & experts are few and geographically scattered → lack of data & expertise
- Research is fragmented
- Reliable information is scarce
- Resources are limited
- Sustainability is an issue

Who are we?

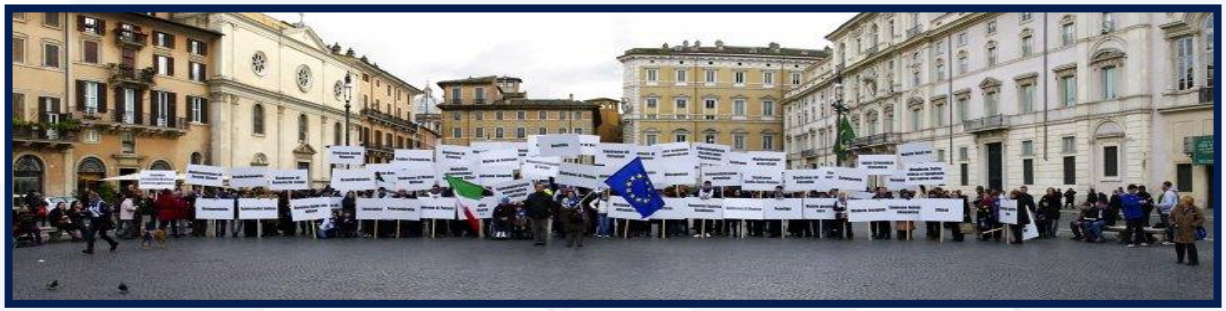
EURORDIS is an international not-for-profit, non-governmental umbrella organisation of rare disease patients' groups representing an estimated 30 million citizens in Europe.

Our Mission

- To build a strong **pan-European community** of patient organisations and people living with rare diseases
- To be **their voice** at the European level
- To directly or indirectly **fight against the impact** of rare diseases on their lives

EURORDIS in brief

- Founded in 1997
- 646 member patient organisations
- 60 countries (26 EU countries)
- 35 National Alliances of RD Patients Organisations
- 45 European Federations of specific rare diseases
- 30 staff
- Offices in Paris, Brussels, London, Barcelona, Geneva



Key factors for success

How to improve collaborative research ?

First message

1. Need for very early dialogue
   between patients and industry, as well as other stakeholders to discuss UMN and/or early development of a MP.

- UMN: symptoms, RD, patients & medical experts, EMA, HTA, payers, several companies, pharma & biotech, m-health industries, MDs → what strategy to respond to UMN, what tool, structure, process is needed, data, registries, 1ary + 2ary endpoints, PROs to be defined.

- MP: with 1 Company → safe harbour at the EMA or within the MoCA process (EU TVF to be discussed with all stakeholders).

Second message

2. Incentives:

They exist. Created since 15 years → 10 years (+2 for Paediatrics) of Market exclusivity and patent extension for Paediatrics. Fee waivers and reductions. All of this works fine.

Incentives for private Investment through tax reductions work well (US and some EU countries).

What does not work: incentives to bring products to patients.

The ME is an exclusivity to a market which doesn't exist.

Way forward: Single Market for pharmaceuticals? ➔ need smarter Europe, EU-wide mechanism of collaboration between MS.

Third message (1)

Context:

- increasing scientific opportunities and breakthrough innovation = enabling technologies
- Increasing economic pressure

→ More incentives towards high UMN

The system of 'Priority Review Voucher': to reward the developer of a product in proportion of its response rate to an UMN.

→ If a company gets the MA for a product responding to a High UMN (tropical neglected diseases), it gets a Voucher from the FDA granting expedited review to use for another (whatever) product. Even sell it to another company.

Third message (2)

- Breakthrough therapy designation for highly innovative therapies → fast track review of the therapy (only in the US)
- Why not a 'fast track' at the EMA ? Not even costly ! Just a shift in priorities management for the evaluation of different dossiers.

➔ Either an accelerated assessment procedure or some type of incentive for another product (voucher).

- Why does the public policy in the US give advantages for the UMN of NDs and not for the RDs?

… It seems like policy responses currently address more competition concerns rather than PH objectives…

“Innovation is not about market timing. It is about creating something that fulfils an unmet need”.

Jeremy Gutsche