EURORDIS Membership Meeting 2015 Madrid

Access to orphan and rare disease treatments

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EURORDIS OBJECTIVES

To achieve the quickest access to
as many safe, efficient
and affordable medicines
with a real therapeutic added value,
for all rare disease patients
in the European Union.

The European Regulation for Orphan Drugs: a corner stone

Strong advocacy movement from the patients through EURORDIS
Partnering with health authorities and industry

16 December 1999: Adoption of the European Regulation on orphan medicinal products

- 2000: Creation of the COMP - Committee for Orphan Medicinal Products at the EMA: 3 patients' representatives for the first time
- **Market exclusivity for 10 years after MA, 12 years if paediatric studies have been performed**
- Fee waivers for orphan designation and reduced fees are granted by EMA
- **EMA provides protocol assistance & scientific advice to developers**
- Pharmaceutical companies developing orphan drugs may be eligible for specific grants from EU and Member State programmes as well as initiatives supporting research and development.

This includes the EU Community framework programmes

The European Regulation for Orphan Drugs: achievements & challenges

Since 2000:

- 1469 Orphan Drugs designations by EMA/EC

- 103 Orphan Drugs received an EU Marketing Authorisation by EMA/EC, including 78 with active market exclusivity of 10 to 12 years

- **A successful regulation to incentivize R&D of medicines in rare diseases but:**

→ **No optimal development of innovative medicines in particular for diseases for which there is no medicine yet approved**

→ **Poor and inequal patient access**

→ **Challenge of sustainability for healthcare systems**

→ **EU Market Exclusivity to an EU Market which doesn't exist**

A new sustainable business model is needed for innovative rare disease therapies

- Times are changing:
  - **Economic pressure**
  - **Demographic pressure on healthcare budgets**
  - **RD scientific opportunities from translational research**
  - **Stratified therapies**
  - **Growing investors expectations**
  - **Society sustainability & values**
- **The current business model of ODs is not sustainable**
- **An evolution not a revolution + risks of not acting now**
- Look at essential & long term common interest at stake across patients, companies, competent authorities, rather than antagonising the short term & short take diverging interest
- Corporate responsibility & leadership & policy innovation

5 KEY CONCEPTS*

- **RD Treatments Evidence Generation is a Continuum**
- **Medicine Adaptive Pathways to Patients and Flexibility of Regulators & HTA & Payers should become an Official Policy**
- **Focus on Effectiveness beyond Quality, Safety and Efficacy**
- **Bridging the Gap Between EU Centralised Regulatory Decision and National Decisions on Pricing & Reimbursement in a seamless process**
- **Enhancing the Dialogue Between all Stakeholders all Along the Product Development & Life Cycle**

*EURORDIS Position Paper 2009; EU Pharma Forum on Improving Access to Orphan Medicinal Products 2009; Commission Expert Group on Rare Diseases, Recommendation on the Clinical Added Value of Orphan Medicinal Products (CAVOMP) in 2012 ; Process on Corporate Social Responsibility in the Field of Pharmaceuticals Platform on Access to Medicines in Europe, Working Group on Mechanism of Coordinated Access to Orphan Medicinal Products (MoCA-OMP) in 2013.

MEDICINES – A continuum of evidence generation

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- The journey to outcomes is recognized - and promoted - as a continuum of evidence generation

- The challenge to outcomes has to be addressed with a new mind-set to risk trade-off and to innovation appetite

MEDICINES - Bringing patients' voice at the heart of scientific innovations:

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Ensuring that new medicines coming to the market respond to patients' need

Making clinical development "patient-relevant" : design, endpoints, measurement of endpoints, informed consent, selection of centres, recruitment, interpretation of data, dissemination of results

Patients relevant outcomes and Patients reported outcomes

How much harm or risk patients are willing to accept for a given benefit?

Preferred treatment option

Providing evidence based opinion, quantitative as well as qualitative, in an independent manner

DECISION MAKING PROCESS – The goal should be: patients access + sustainibility

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The opinion & decision making processes should be streamlined between regulators, HTA and payers. No more vertical silos. A series of rational decisions made by rational people using rational methods doesn't make a rational outcome if they are disconnected from each other. **No more gaps between EU centralised and national levels.**

A dialogue all along the medicines development pathway, with the same aim to patients' health outcomes, with streamlined evidence generation requirements

We cannot separate these decision making process from market access. Focusing on outcomes means focusing on real life world, on real patients life (actual holistic care, treatment options, real access), on capturing real world evidence.

We cannot separate the R&D model from the business model and from market access, **they are conditioning patient access & affordability.**


European
Reference
Networks

HEALTHCARE SYSTEMS: toward a European system focused on outcomes

Hospital specialised services / Centres of Expertise — no fit for all EU MS

European Reference Networks: New from 2016 and will focus on patients' health outcomes. This is where the "**evidence**" are being generated by the interface patients-doctors, where clinical trials take place, and where real life treatments is prescribed (the real use of a medicine not always being the defined good use of this medicine) and experimented in real medical practice.

Large scale EU Data Collection and registries: inter-operability, e-prescriptions, patient summaries, electronic patients' health records, EU pooling and analysis

Multi-centric European Clinical trials

Good Practices Guidelines on Diagnosis & Care

Increasing access to essential medicines: WHO framework for collective action

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World Health Organization

EURORDIS Call
to
National Competent Authorities
on Pricing & Reimbursement

Released: May 2015
Expected Impact: Fall 2015 & Year 2016

Call to scale-up the specific early dialogue between payers and industry (MOCA)

- The **Principle of Mechanism of Coordinated Access to Orphan Drugs** has been elaborated by the **EU Pharma Forum** and by the **EU Corporate Responsibility Platform**

- **MOCA** pilots have been implemented since 2014 by MEDEV, involving patient representatives (EURORDIS) and industry (EFPIA-EuropaBio Task Force on OMPs & RDs)

- **EURORDIS Call to NCAPR to support MOCA as the specific early dialogue with payers on Orphan Drugs**

- **Examples of early dialogue**

Bridging the Gap Between EU centralised regulatory decision and National decisions on Pricing & Reimbursement

- One way or another, EMA and HTA and Payers need to be involved in their respective process at key points of time to be well informed about the reality of medical needs, the potential and reality of the product, the uncertainties and the pathway to generate additional evidence for well targeted patients and good medical practices

- Time points:
  - **Scientific Advice / Protocol Assistance at EMA**
  - **Scientific Advice Parallel EMA & HTA (or EUnetHTA) at EMA**
  - **SEED : Scientific Early European Dialogue at EUnetHTA**
  - **Risk-benefit assessment at CHMP EMA – as Observer**


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50
100

Call to establish of a new "Table for Price Negotiations" with a core group of volunteer EU MS

- EURORDIS Call to NCPAR most important message
- **Championed by Belgium (INAMI) together with the Netherlands, Austria. Possibly Luxembourg, Italy and Portugal**
- **Value-based pricing discussion linked to the volume of patients and to post-marketing evidence generation**
- **Certain level of transparency on cost is expected**
- **Price**: a) Price will have to fluctuate over time based on additional generated evidence, b) conditional pricing, managed entry agreements, payment on outcomes, and other innovative pricing schemes, c) a reference for basket of price

Conclusion

- *Access & Sustainability* rather than *Access vs Sustainability*
- We need to stop looking at things in isolation and contemplating the problems. We need to stop being restrictive on Treaty and Regulations interpretation as an excuse for not doing, and be daring trying to make sense of it, to address multi-facet issues
- We need to use new scientific, regulatory and policy knowledge to improve the overall system (mostly based on current regulations) with the two aims of achieving public health objectives (provide access to all patients, improve patient health outcomes) and supporting the growth & jobs (industry innovation, EU attractiveness for investment in medicine developments, Sustainability for the NCA)

THANK YOU
OBRIGADO