EMA - London
10 years of EU
OMP Regulation
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The next 10 years: Focal Points from the Industry.

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Congratulations

to the rare disease patients' associations,
the Commission, the Parliament,
the EMA / COMP & CHMP
the scientific community, and
the EU industry associations

for the **10th anniversary** of
Regulation EC 141/2000

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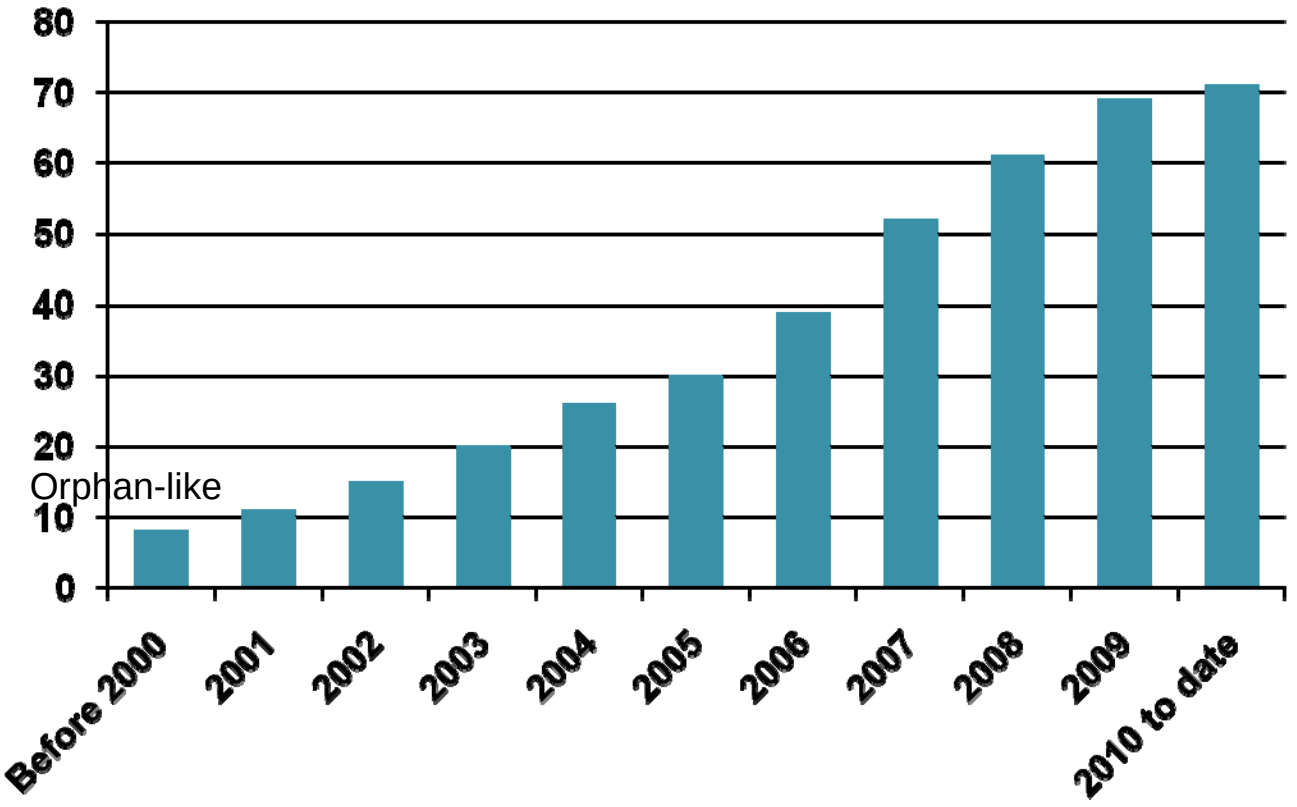
10 years and more…

1983: Actions by NORD and allies	US **Orphan Drug Act**: rare diseases become a health priority, worldwide.
1999/2000: Actions by EURORDIS and allies	Unanimous approval of the **EU Orphan Medicinal Products Regulation**.
Coordinated EU health policy actions	**EU taking the global policy lead** on rare disease policies!

Growing number of orphan drug designations and approved orphan drugs also in EU

EU Orphan Medicines with Positive Opinion



10 years but more work to do…

- Only 10-20% of rare diseases have treatment available + available treatments can still be improved → **medical need remains high**.
- Commonalities between rare and common diseases researched → rare diseases as **models for common diseases** (oncology, neurology, auto-immune and infectious diseases….).
- 10 years mark the **end of the market exclusivity** period for the first orphan drugs approved under the Regulation → competition will enter if there is a market...

10 years is not long .?.

- 10 years is not long in **developing medicines**: average development time > 12 years.

- 10 years is **long for the evolution of business**:
  - Since 2000: large R&D cost increases; attrition; generic competition; major consolidation;
  - Rarer diseases now also more appealing for research in large pharma companies;
  - Rare diseases and orphan drugs now linked to innovation in healthcare.

Contradictions.?.

- **Contradictory perspectives in society** :
  - (1) orphan drug regulations have not (yet) lived up to their promises: much more to do & time is of the essence

→ revise regulation to provide more and better incentives for industry, with a stricter regulatory regime, and priority setting;

  - (2) orphan drug regulations have provided too many economic incentives to industry

→ revise or abandon regulation, companies should not be allowed to create monopolies by combining market exclusivity (and multiple indications) with high pricing.

Misconceptions .?.

- Both perspectives include misconceptions!
- For industry, most important is **to implement the policies developed over the past years**. ..
- Industry's role is creating **critical questions** about pricing, access and development priorities.
- Industry will have to play a **much clearer role** in explaining and needs to take responsibility.
- All other stakeholders also need to double their efforts to better implement the **spirit of the regulation** and communicate better.

- **Rare cancers** are also rare diseases!

Communication .?.

- Industry needs to better explain the **development process of drugs**.
- *And* the difference between basic research and industrial development → to build **societal consensus** and support.
- First priority is **shorter time to diagnosis:** average time to diagnosis between 5 and 30 years is no longer acceptable → without timely diagnosis, no care plan can be effective.
- Rarity factor, work **at EU level**!
- More and better **physician education** in rare diseases.

Definitions, Data …

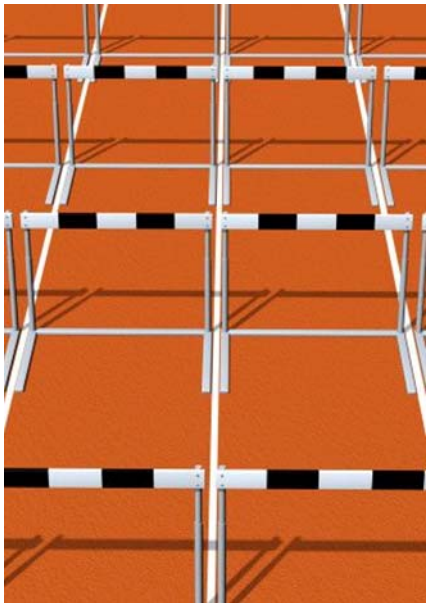
- A clear **definition of "orphan drug"** would avoid misunderstandings → if definition as "*a product to treat a life-threatening or serious and chronic disease that meets a high medical need and has no suitable alternative*" is also used in member states, it would facilitate access discussions and help patients.

- Clarify whether **compounding ingredients** used as treatment should be considered as "an existing treatment" to avoid loss for sponsor and patients.

- Support such definition by a system of **conditional reimbursement**

→ grant patient access to orphan drugs upon EU approval + collection of further **clinical added value data** at EU or even international level.

Access and …



- Current orphan drug regulations deal with **regulatory aspects** of development and approval of orphan drugs and provide economic incentives for such development.
- Diagnosis, research priorities and access issues for patients (and how to best organize such access) are **not covered** in the regulation but gaining in importance.

Transparency!

- The definition of **price transparency** for orphan drugs needs to be agreed upon
  - Society may request more transparency in return for the economic incentives → take into account that **companies are operating globally**, and depend on investors in a globalized finance system.
  - **Misconceptions** about profits, sufficient profitability, high pricing, etc …

  → concepts are not well understood.

EU Orphan Regulation 141/2000

OMP Regulation : Can the EU go any further?

Criteria – which diseases (prevalence, significant benefit to patients if other treatment approved)?

Safety & efficacy rules – rare disease patients deserve quality drugs!

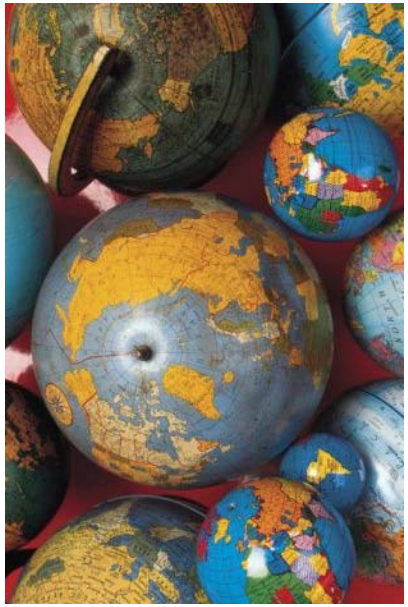
EU Marketing Authorisation valid in all EU countries

The Regulation calls on EU Member States to design programs for patients' access

Don't change a winning horse…

- There is no need to change the Regulation at this time, rather **to better implement it, as well as all other EU policies** prepared.
- EMA to focus on new medicines for the patients' benefit → not on balancing the health care budgets in the Member States.
- Industry **applauds** ongoing discussions with payers about needs for data to result from early drug development stages.
- Not much activity in the Member States in terms of incentives → we hope that **national plans** will change this.

Globally.?.



- More **international regulatory AND health policy collaboration** and exchange of information → to avoid duplicate work and allow best use of expertise.
- Cost savings for the sponsors and authorities alike + earlier access to patients.
- **EU and US should take even more the lead** in sharing experiences and expertise.
- The field of rare diseases is a '**societal laboratory**' and predictor of future trends → model for personalized healthcare.

Collaboration as the solution!

- **Multi-stakeholder platforms**, including policy makers, regulators, patient groups, treating physicians, researchers, industry and payers and administrators → work on sustainability and solve complex issues.
- Issues to work on include more data, improved communication and more awareness.
- The **models** of multi-stakeholder platforms which exist in the EU and in some Member States (Netherlands, Belgium,…) should be cherished and nurtured.

So, **congratulations**

- to all, for the success of making rare diseases a health priority in Europe
- to the EU for taking the global policy lead in the field

And to the EMA to organize a conference to recognize and consolidate this, for the **benefit of the patients.**

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Nagyon Köszönöm

Efharisto Poli

Dêkuji

Tänan

Labai Achiu

Multumesc

Grazie!

Merci Beaucoup!

THANK YOU!

Dziekuje

Liels Paldies

Tack så Mycket

Obrigado

Gracias!

Dakujem Vám

Mnogo Blagodarya

Danke Vielmahls!

Dank U!

Mange Tak

Paljon Kiitoksia

Hvala Vam

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