



Expectations and Contributions from Industry

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7 Years EU Orphan Medicines Regulation

- ◆ >500 EU Orphan medicine designations
- ◆ 42 EU marketing authorisations
 - 32% Oncology
 - 29% Metabolic diseases
- ◆ Similar annual number of designations and approvals in EU and US
- ◆ Parties, including industry, are delivering on their promise

But we need to address perceptions with clearer messages

- ◆ Orphan medicines are treatments for **life-threatening or serious and chronic** diseases for which no alternative exists, or which has a significant benefit over existing products: **no choice of treatment**
- ◆ Common, rare (orphan), ultra-rare (ultra-orphan) diseases: the reality is a **continuum** with research and treatment complexity **increasing with rarity**
- ◆ There is **no avalanche of orphan drugs** coming, but rather a steady annual increase
(Source: EPPOSI workshop Copenhagen, 2007)

The *Spirit* of Orphan Drug Legislations

- ◆ To provide **timely and equitable access** to therapies for rare disease patients,
and
- ◆ **To balance the risk** by providing economic incentives to industry to develop therapies
- ◆ Patients with rare disorders **deserve the same care and the same safety, efficacy and quality of products** as patients with common diseases



Further EU Support for Rare Diseases

- ◆ Rare diseases a **public health priority** (DG SANCO Public Health Programs '03 – '08 and '08-'13)
- ◆ **DG Sanco's public consultation on Europe's challenges regarding rare diseases**
- ◆ **Advanced Therapies Regulation**
- ◆ **Paediatrics Regulation**
- ◆ **Policy continuity** through EC guidelines on regulation EC 141/2000 and the remarkable work by the COMP and EMEA
- ◆ **EuroGentest** project including rare disease genetic testing

Industry Makes Substantial Contributions

- ◆ **Industry innovates** for unmet medical needs: *“the need for innovation is constant”* (Dr. Margaret Chan, WHO)
 - ◆ Industry is a **major source** of healthcare products, including orphan drugs
 - ◆ Creating **access/compassionate use** programs
 - ◆ Industry creates **societal wealth & employment**
- **We need to better communicate about this**

Industry Strategic Focus on Orphan Drugs (1)

- ◆ **Access**: our top priority
- ◆ The definition and **application of HTA** to orphan drugs
- ◆ Regulatory issues
 - The EU **Clinical Trial Directive's** impact on rare diseases
 - **Work on understanding** of trial complexities and results by the regulators
 - **International harmonisation** of regulatory requirements
 - The **guidelines** on article 8.2 of Regulation EC 141/2000 and on similarity and clinical superiority
 - **Off-patent products**

Industry Strategic Focus on Orphan Drugs (2)

- ◆ The **economic and social Value** of orphan drugs
- ◆ Economic **incentives**
- ◆ Key communication messages
 - An orphan drug is a product for which **no alternative** exists in the EU or is of significant benefit to patients above and beyond what exists
 - More **awareness** about impact of rare diseases needed
 - Collect data about all rare disease initiatives and orphan drugs in Europe: **models and best practices**
 - Market exclusivity doesn't mean **monopoly**, and gets **eroded** by delays in access
 - The need for **more research** including on understanding **risk**
 - **Building partnerships** with other stakeholders



The Issue of Access to Orphan Drugs

- ◆ **Long** discussions on price and reimbursement in some countries
- ◆ The smaller the country, the more difficult the access (Eurordis)
- ◆ **Regionalisation** of healthcare
- ◆ Is Health Technology Assessment (HTA) possible for (ultra-)orphan drugs? For those orphan drugs for which it is possible: **can we assess at EU level, appraise locally?**

Understanding Risk

- ◆ Developing orphan drugs is **a strategic risk**
- ◆ Many therapies **are first in history** and **go into uncharted** territory
- ◆ **Sustainability** of the business is highly important: for investors, but even more so for patients
- ◆ **Finding the patients: the role of diagnosis and screening**
- ◆ **Undeveloped** care“ infrastructure “ for rare diseases

Need for More Research Overall

- ◆ Research on **health outcomes** and on impact on the healthcare budget
- ◆ Impact of orphan drugs or the denial of access on **families**
- ◆ **What does Value mean? For whom?**
- ◆ **Epidemiology and natural history**
- ◆ **Optimal** communication to create awareness
- ◆ Clinical **registries** as tools for payers and reimbursement discussions
- ◆ The **definition and applicability of transparency** from different stakeholders' perspective on issues such as pricing

Trust Building by all Stakeholders

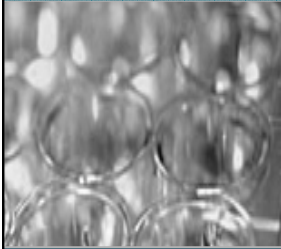
- ◆ Awareness and **correct** information
- ◆ Models of success generate interest in the field
- ◆ **Failure** and its costs – products or companies who fail are no longer in the equation
- ◆ Expectations from shareholders play a role
- ◆ Understanding that a product is one element in a company strategy/portfolio
- ◆ **We should not shy away** from controversial issues as they don't disappear that way: see EPPOSI Workshop discussions

Summary: What can Industry do?

- ◆ Strategically focus even more on rare/neglected diseases
- ◆ Be proud of what we achieve
- ◆ Continue to innovate
- ◆ Continue programs on access, community relations, patient support
- ◆ Communicate more clearly about our commitment, focus and difficulties, and our prices

Summary: What can all Stakeholders do?

- ◆ Focus on the needs of the **patients**
- ◆ **Be transparent** about issues, good and bad
- ◆ **Promote dialogue** with other stakeholders **as partners** to find solutions for issues
- ◆ Work on **communication** messages together
- ◆ Get **politicians** on board
- ◆ Engage **personally**



THANK YOU