

Towards an optimal framework for rare disease therapies in Europe

2005 Industry White Paper

1st Eastern European
Conference on
Rare Diseases and
Orphan Drugs

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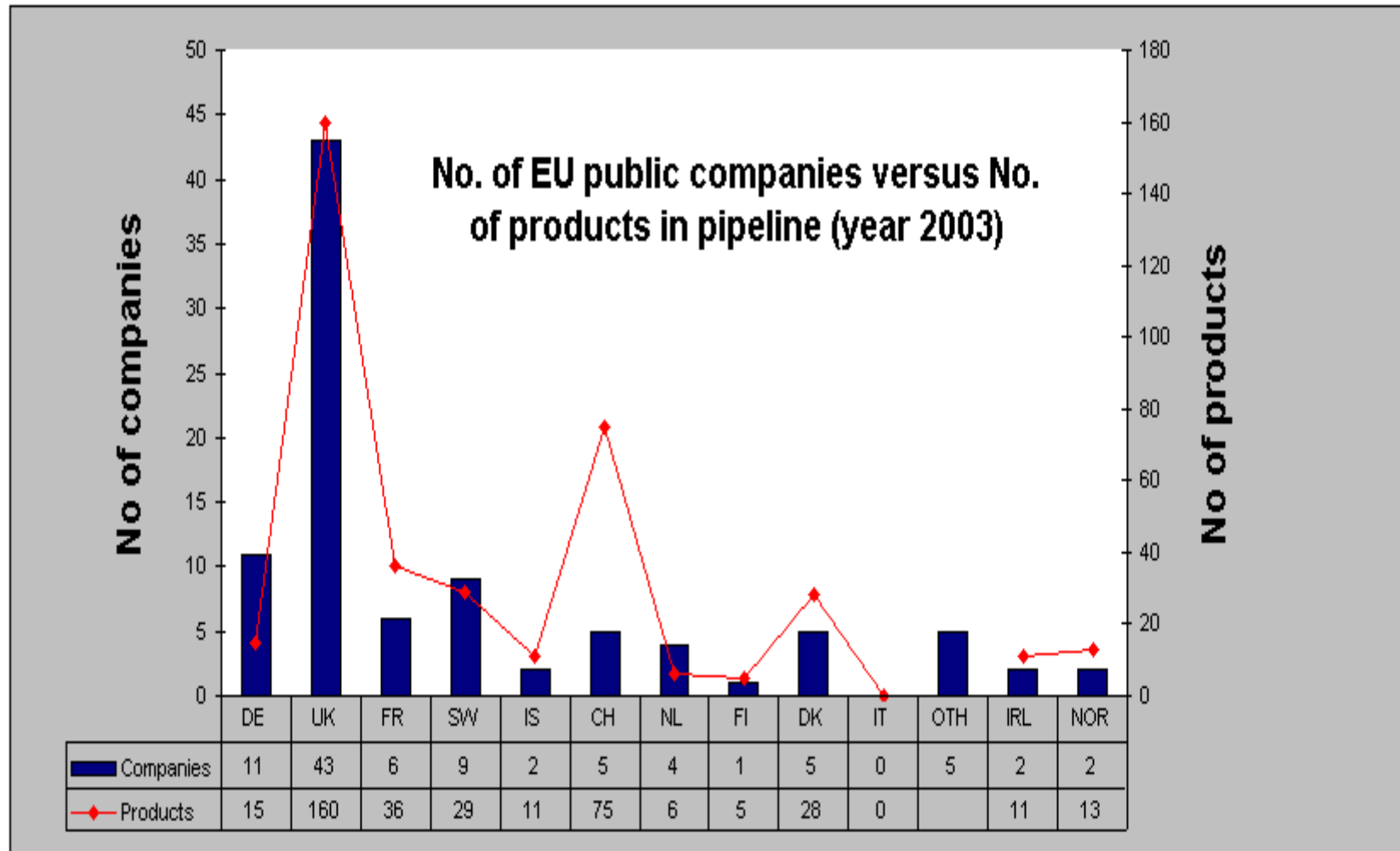
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What is **EuropaBio** ?



- EuropaBio is the **European Biotechnology Industry Association** representing over 50 globally operating biotechnology companies and 25 national associations, representing more than 1500 small and medium-sized companies
- It aims to be a **promoting force for biotechnology** and makes proposals to industry, politicians, regulators, non government organisations, and the public at large
- EuropaBio's **Core Ethical Values** (CEV) are available since 1998 in 11 languages
- White Paper developed jointly with **EBE: association of Emerging Biopharmaceutical Enterprises**

1.900 biotech companies in the European Union 80,000 employees and 6,5 billion € in R&D



Source: E&Y Global Biotechnology Report 2004

Biotech looks at causes and develops new treatments

- **Improving disease knowledge and diagnosis**
 - Genomics, genetic testing, proteomics, RNA and DNA
 - Underlying mechanisms of disease
- **Addressing unmet medical needs**
 - Rare genetic diseases, cancer, biosurgery, HIV/hepatitis treatments, anemia, diabetes
- **Targeted and tailor-made medicines**
 - **Pharmacogenetics** + fewer side-effects
 - Monoclonal antibodies
 - Diagnosis and therapy
- **New technologies in development**
 - Cell therapy and tissue engineering
 - Gene therapy
 - RNA

Rare disorders? Orphan drug?

- Over **6000** rare disorders - 70-80 % genetic
- **25-30 million** Europeans: 4-5% of population
- An **orphan drug** is a medicine for a life-threatening or serious rare disorder → no sponsor or “parent” to develop it
- Cost of medicine not recovered by expected revenues → no development of therapies without industry **incentives**
- **35-50 % biotech** orphan drugs

The EU developed the Orphan Medicinal Products (OMP) Regulation



Its purpose is to provide:

- **Effective therapies** for patients with rare diseases, and
- **Incentives** to industry

The core of the OMP Regulation are **non-economic societal values** representing the desire to provide **equitable access** to therapies independent of rarity of disease

Situation analysis

- The Regulation has had a **successful start**: **254** designations since 4/2000 (compared to nearly no EU-developed products before) and **20** Orphan Medicinal Products granted Marketing Authorisation
- It is too soon to judge results - but the outlook is promising - **we all support this Regulation**
- The Regulation does not concentrate on **access or research**
- A Study by Alcimed for the Commission states that the price for an OMP in the EU is related to the **rarity** of the disease and to the **health systems** of the member states

The general public's view?

- **United Kingdom:** 20/27 participants in a Citizen's Council believe the NHS should pay premium prices for effective therapies to treat patients with very rare and severe diseases
- **France (1002 persons over 15 questioned) :**
 - 85 % of the population is ready to support rare disease research
 - 25 % is directly or indirectly in contact with persons affected by rare diseases
 - Research into rare diseases is considered as important as road safety or effects of smoking tobacco

White Paper

Recommendations

1. Awareness and Education

- Address issues **at EU or national level**
- Work on **awareness** regarding rare diseases
- **Education** of health professionals
- **Eliminate inequalities** in diagnosis and access
- Lots of work in the **member states!**

2. Accurate & Timely Diagnosis

- Need for **network of diagnostic centers**
- Rare disease patients are **diagnosed too late**
 - Rarity and heterogeneity of the disease
 - Late diagnosis often associated with poor prognosis
 - Screening or diagnosis not well-established
- Individuals need **timely treatment** → accurate diagnosis is key, especially if therapy exists
- Diagnostic and population and/or appropriate **newborn screening**

3. Compassionate Use (CU): a shared responsibility



- CU needs **definition**
- A **shared responsibility** between the clinician, the developer of the product and the authorities
- France (ATU), Italy and Belgium fund CU of OMPs before approval or before reimbursement
- **Sustainable, appropriate systems** in Member States: many OMPs are developed by SME's
- CU may create **dilemma's** when product is scarce

4. Increase application of Regulation



- **Access** to approved OMPs: lack of timely and equitable access – see Eurordis Survey
- Price of OMPs defined by rarity and by health care systems
- Understanding the spirit of the regulation
- Predictable regulatory climate is key

5. Incentives

- **Tax incentives** impossible in EU Regulation
→ MS action to improve EU competitiveness
(Few countries provided OMP incentives)
- More **awareness** and explanation are incentives
- **Earlier access** for OMPs are most important incentives both for patients and for industry
- **Paediatric medicines**: good proposal for 2 years extra market exclusivity

6. Market exclusivity (ME)

- ME is **strongest incentive**
- Confusion is eroding incentive
- ME does **not lead to monopolies** → rarity of the disease does
- ME provides **partial exclusivity** only
 - in respect of similar products
 - In respect of clinical superior products
- Review of ME only to be based on **designation criteria**

7. Value and health economics

- **Cost-effectiveness** for rare disease therapies: **social values** versus health economic methods
- Determination of value: **what data are needed** to get reimbursement
- **Eliminate double work** on clinical and cost effectiveness

8. Clinical trials

- The Clinical Trial Directive is making clinical **trials for rare diseases more complex**
- Post-approval commitments and additional trial requests should be **ethical and feasible** under national rules
- **Facilitation** of cross-border clinical trials and of small protocol changes for OMPs

9. EU Research Priorities

- More **coordination** within the Commission plus link with the OMP Regulation
- Link with the objectives of the **Lisbon treaty** (“EU → leading knowledge-based economy in the world”)
- Many OMPs developed by **SME's**
- **Link** research with regulatory framework
- **Harmonize** regulatory requirements between EMEA and FDA

Perspectives for Eastern Europe



- **Develop innovation** using existing research and clinical strengths
- **Orphan drugs are good pilot projects** for healthcare innovation: trendsetters for change in healthcare
 - regulatory framework
 - patient care, and
 - preventive medicine
- **Foster entrepreneurship** in industry, academia and patient groups as well as partnerships

Thank you for your attention!

Questions?

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