Towards an optimal framework for rare disease therapies in Europe

1st Eastern European Conference on Rare Diseases and Orphan Drugs

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2005 Industry White Paper

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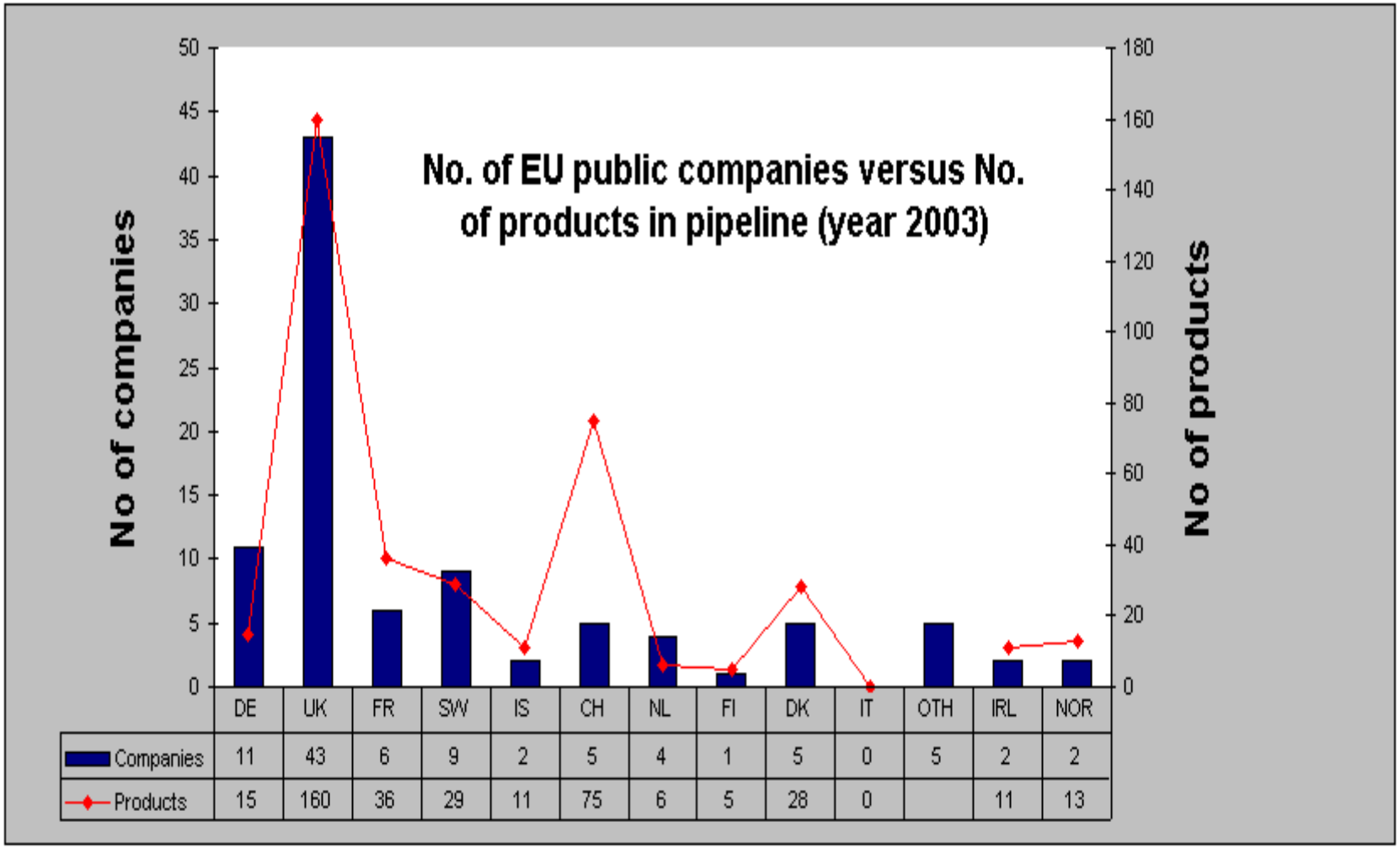
What is EuropaBio ?

- 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



	DE	UK	FR	SW	IS	CH	NL	FI	DK	IT	OTH	IRL	NOR
Companies	11	43	6	9	2	5	4	1	5	0	5	2	2
Products	15	160	36	29	11	75	6	5	28	0		11	13

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 scarse**

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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