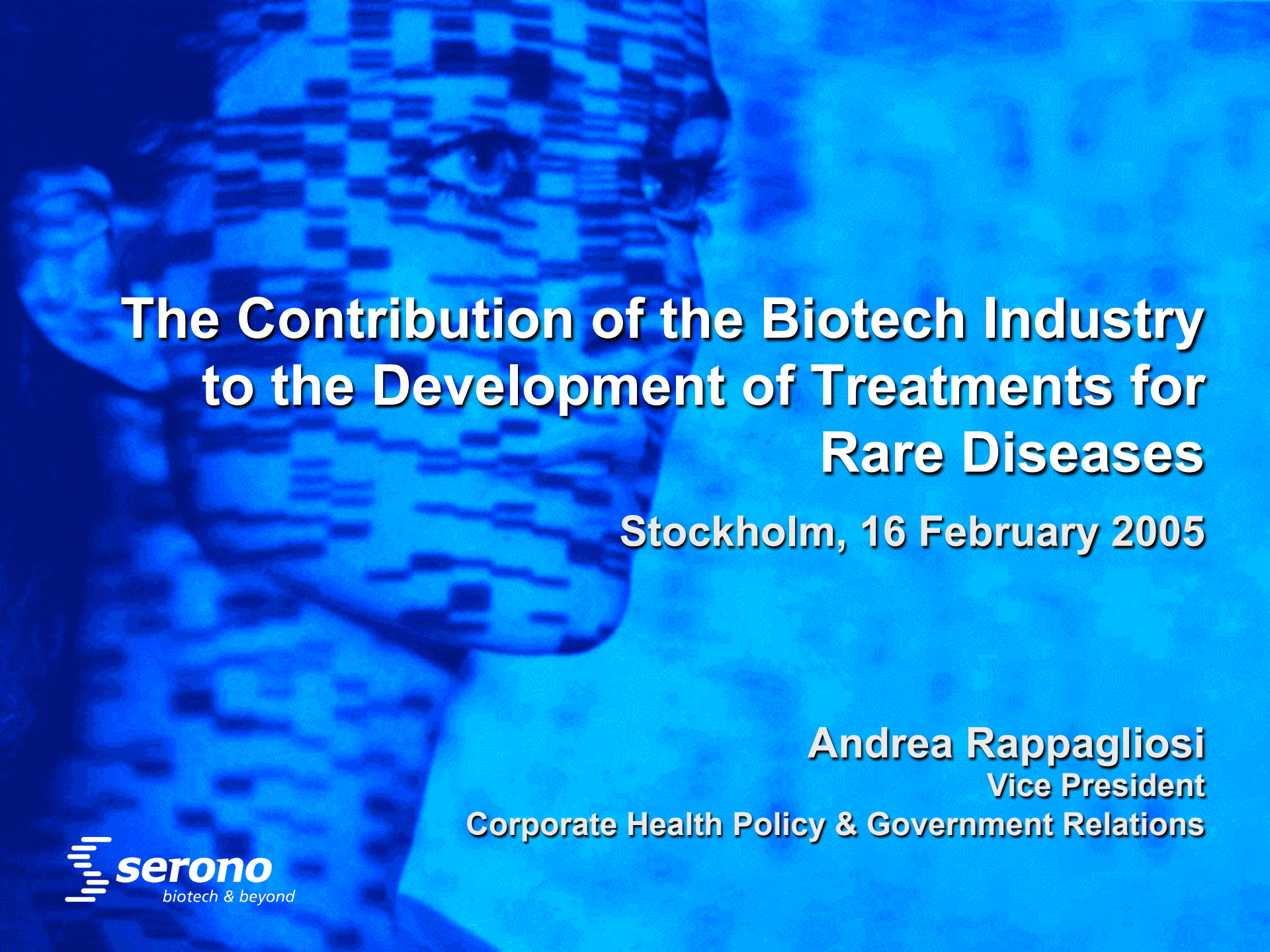

The Contribution of the Biotech Industry to the Development of Treatments for Rare Diseases

Stockholm, 16 February 2005

Andrea Rappagliosi
Vice President
Corporate Health Policy & Government Relations

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Disclaimer

« Human beings: created at the 7th day of a long week when God was very tired. »

Mark Twain

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Gaps related to the development of treatment of rare diseases

- Still a great need for a basic molecular understanding of the diseases and the identification of possible pharmacological targets

- In view of the rarity of the diseases, it is recommended that public money should be used to fund basic research

- Serious gap in clinical evaluation even though genomic and proteomic technologies provide useful clues for both diagnosis and treatment

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Source: WHO EU – NL Presidency – Nov. 2004
"Priority Medicines for Europe and the World"

Gaps contd.

- **Lack of long-term epidimiological data on both medicine safety and effectiveness and on the course of the disease**

- **Translational research too difficult even though fundamental research has already been done**

Source: WHO EU – NL Presidency – Nov. 2004
"Priority Medicines for Europe and the World"

Biotech: a Remedy?

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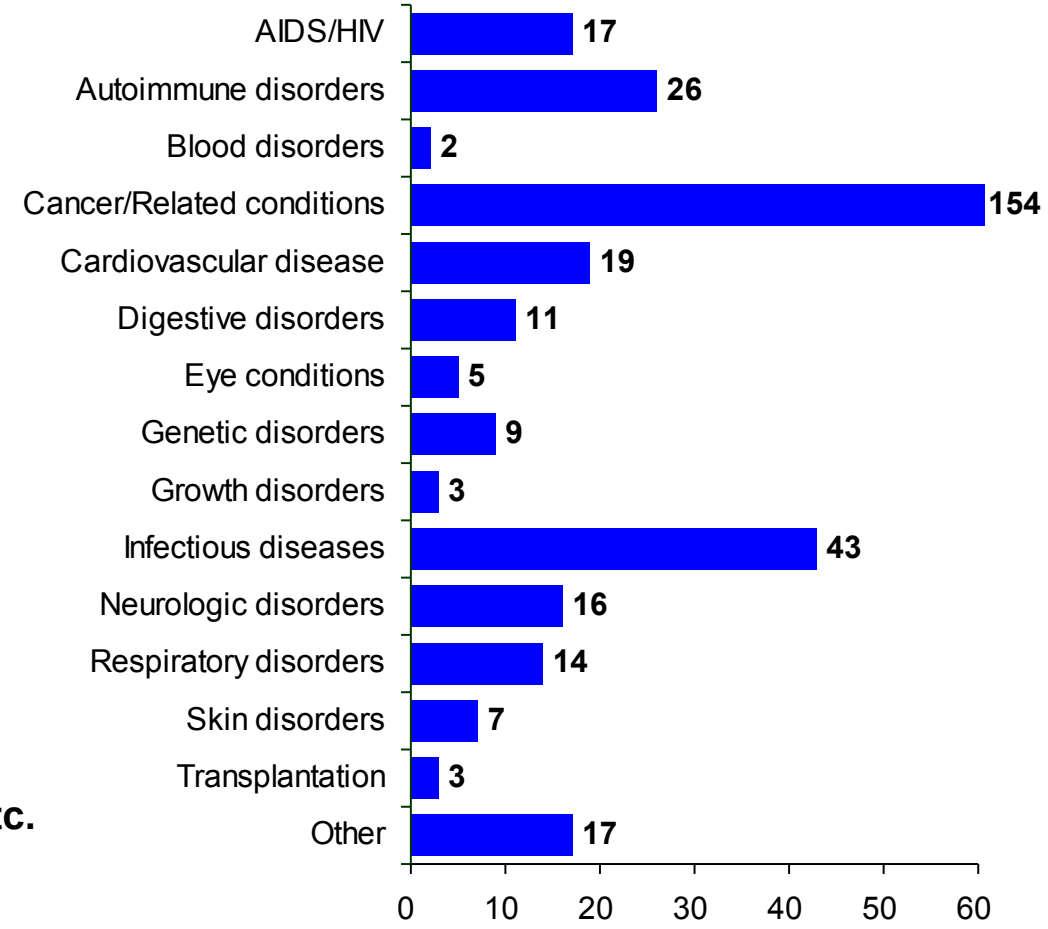
Biotech Today

- **324 Biotech new medicines and vaccines today being tested for nearly 150 diseases**
- **154 to treat cancer, 43 to treat infectious Diseases and 26 to treat autoimmune disorders**
- **More than 250 million patients have benefited from already approved biotech medicines to treat or prevent heart attack, MS, breast cancer, cystic fibrosis, leukemia, etc.**



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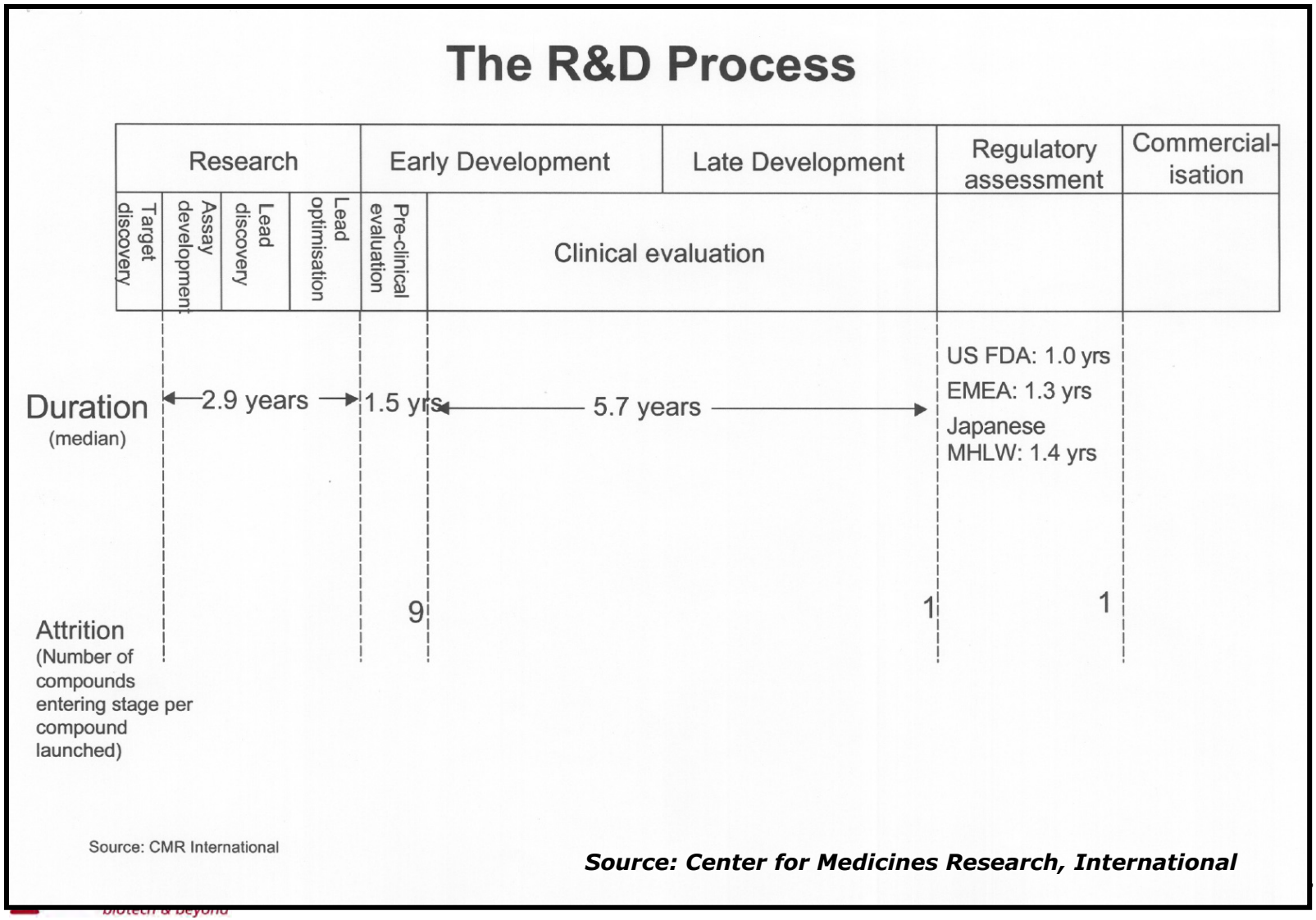
Source: PhRMA – 324 Biotechnology Medicines in Testing Promise to Bolster the Arsenal Against Disease – October 2004

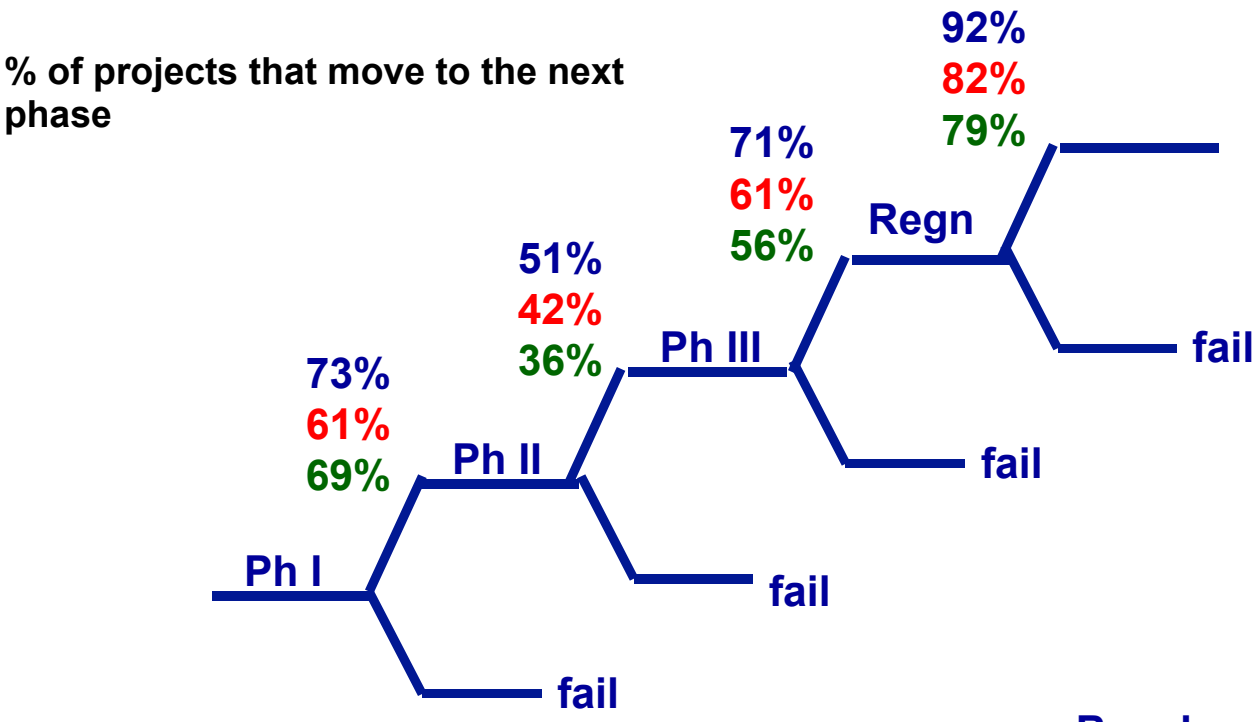
Developing a Biotech drug: from Research to Commercialization



Source: Center for Medicines Research, International

Success Rates in Drug Development

1996 –1998:
11% of NCEs that enter Phase I are marketed



Benchmark: 94-96
Benchmark: 95-97
Benchmark: 96-98

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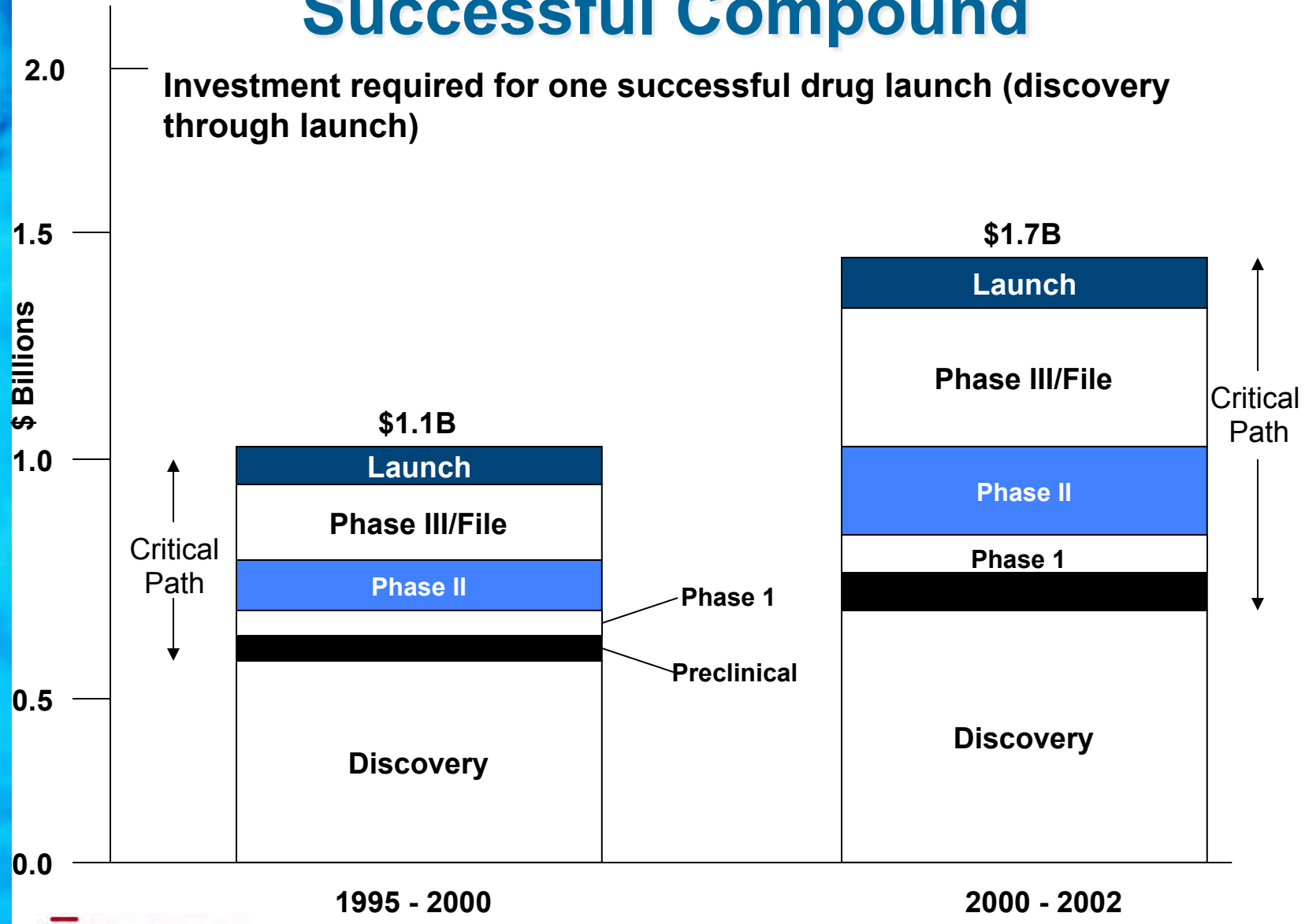
Source: Center for Medicines Research, International

Investment Escalation per Successful Compound



Source: Windhover's In Vivo: The Business & Medicine Report, Brain Drug Economics Model, 2003
FDA : Challenge and Opportunity on the Critical Path to New Medical Products, March 2004

Promoting Public Health by Enhancing Innovation

«Our capacity to meet unmet medical need should be great. We are, though, in danger of jeopardizing this potential if we do not make every attempt to reduce the cost of drug development. *It will not be easy; nor will it be uncontroversial.* There will be political, social and legal challenges to be addressed. But if we do not work towards this goal, we will fail future patients, their families and society as a whole. »

Source: Sir Michael D. Rawlins: « Cutting the cost of drug development? » - Nature Publishing Group April 2004

“The State we’re in”

Will Hutton

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The State we are in: Designated and Approved OMPs in the EU and the US

	EU	US
Designation	242	1200
# of Biotech	~61%	~50%
Approved	16	238
# of Biotech	~20%	~20%

Source: EMEA website – updated February 2005; BIOTECanada International Comparison of OD Programs – April 2004

Orphan Drugs and Biotech: meeting unmet medical needs…

Patients suffering from rare conditions should be entitled to the same quality of treatment as other patients with more frequently occurring disorders.

Regulation EC 141/2000, preamble 7, article 3.1b

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…through tailor - made medicines

- **Genetics and Pharmacogenetics**

➢Allow for a better understanding and diagnosis of the disease

➢Widen the numbers of diseases that can be treated effectively as well as limits the occurrences of adverse drug reactions on patients

➢Increase both the safety and efficacy of treatments by diminishing the trial and error by finding the optimal dose and treatment

➢Provide new ways to match medicine doses and medical treatments to individual groups of patients

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Source: EuropaBio Healthcare Biotechnology 2005

Difficulties to Overcome

- Lack of scientific understanding of the disease (know-how)
- Number of patients with a specific rare disease is low
- Lack of the interest of society
- Lack of infrastructure and exchange of information
- Lack of translational research

Source: Priority Medicines for Europe and the World _ A Public Health Approach to Innovation – van Weely and Leufkens - November 2004;

Rare disease and orphan medicinal products, Helen Crompton, October 2002

Difficulties cont'd.

- From concept to patient, translational research is a key element in the product development in emerging technologies

- « Massive investments in one part of the network are likely to be partly wasted unless the other links are strengthened as well »

Source: Baumann M, SM Bentzen, W Doerr, MC Joiner, N Sauders et al: « The Translation Research Chain: Is it Delivering the Goods? »

Difficulties cont'd.

- "Controversies appear when the distribution of expertise during the innovation process does not take into account some potentially interested actors.
- The controversies begin when some actors who claim the right to participate to the definition of risks, cost and benefits are not included in the management of innovation"

Senker et al(1999) EC TSER Project

Source: Baumann M, SM Bentzen, W Doerr, MC Joiner, N Sauders et al: « The Translation Research Chain: Is it Delivering the Goods? »

The Way Forward

- **Better Science**
  - Boosting translational research (e.g., EIH, public-private collaborative work)
- **Better Rules**
  - A paradigm shift in the tools to assess safety and demonstrating medical utility (e.g., Clinical Trials)
- **Better Access**
  - Improving timely access to Orphan Drugs in Europe

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"Knock on the sky and listen to the sound!"

Zen Saying

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