History and Commitment from industry in the field of rare diseases and orphan drugs

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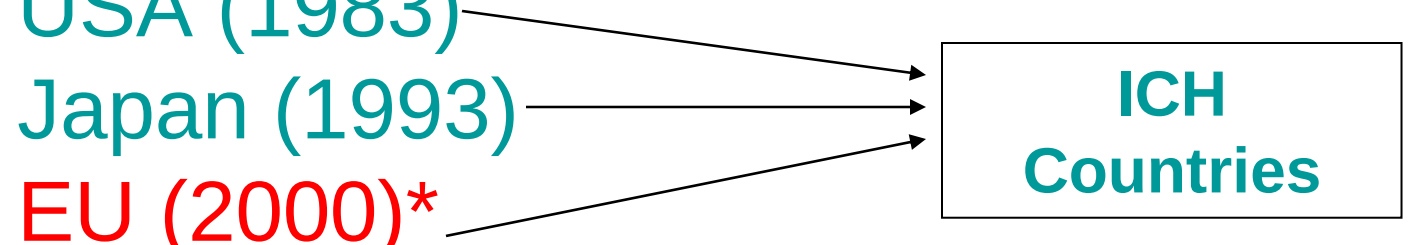
The Impact of the US Orphan Drug Act

- 10 products approved before the ODA (1983).
- Now > 2000 orphan drug designations, > 350 orphan drugs approvals
- *The most successful piece of US healthcare legislation so far*

- **Long term influence on other public health policies in other regions of the world**.

Orphan drug policies

USA (1983)
Japan (1993)
EU (2000)*



Australia (1998)

Korea

Taiwan

*** Unanimous approval in December 1999 by European Parliament**

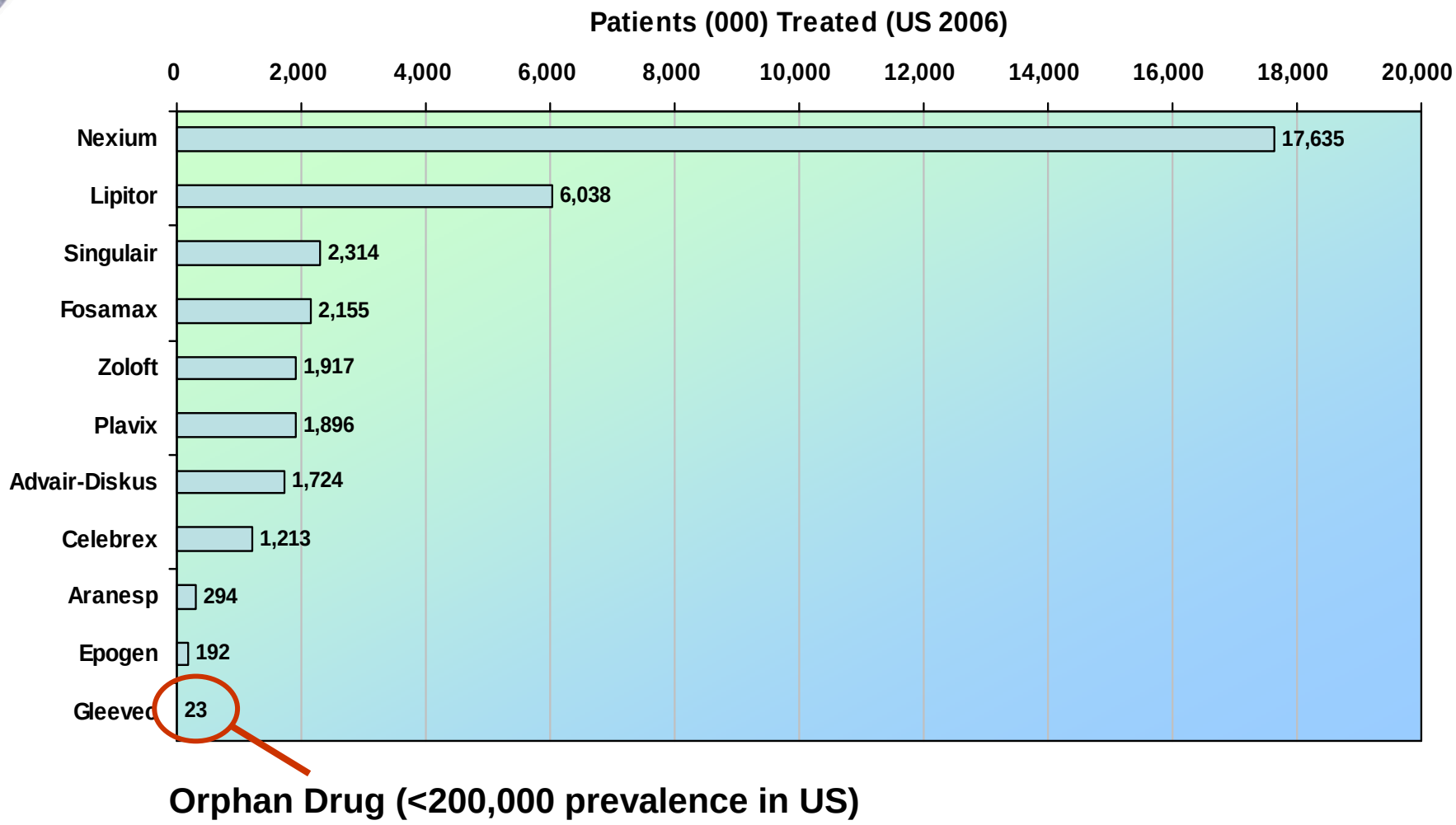
The Definition of Rare in the world

- Rare (orphan) as defined in orphan drug regulations:
  - US : prevalence <200,000 (300 million people)
  - Japan : prevalence <50,000
  - European Union (EU-27): prevalence <5/10,000, i.e. < 250,000 patients (500 million)
  - Australia : prevalence <2000 people

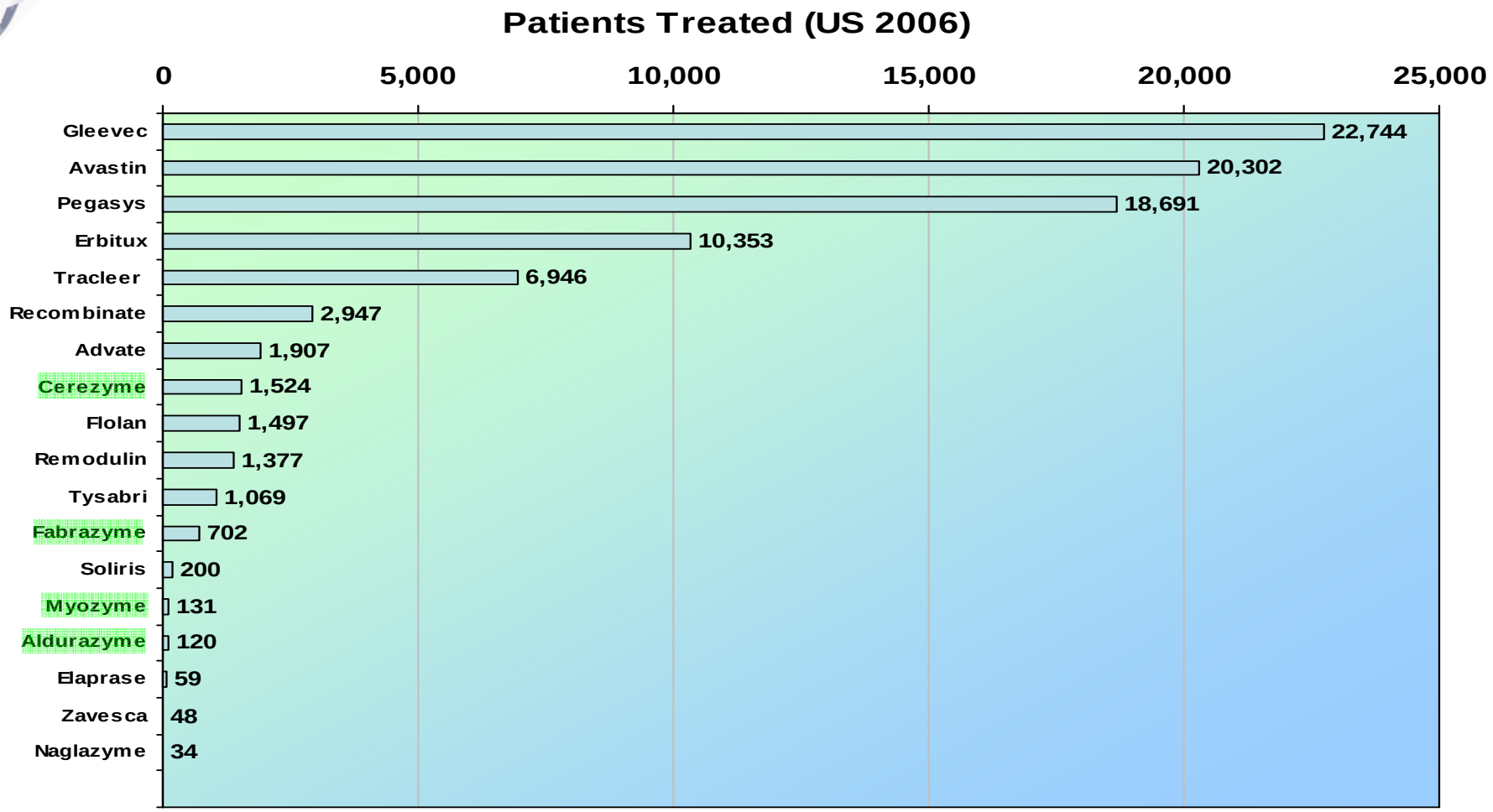
Note: ultra-rare (ultra-orphan) is defined by NICE as 1000 patients in UK population

The reality is a continuum with research & treatment complexity increasing with disease rarity

Understanding Rarity



Understanding Rarity



Soliris based on 2007 estimates

Industry was and is involved - 1

- Industry took a very active part in the discussions on the draft European Union Orphan Drugs Regulation
- Industry from the start supported patients representation on the COMP
- Industry collaborates with and supports rare diseases patient groups
- R&D and investments by industry in rare diseases in Europe grew tremendously

Impact in the EU

- Before 2000: **8** orphan drugs (OD) avant la lettre
- After 2000 :> **1000** OD designations filed, **> 720** granted by EC

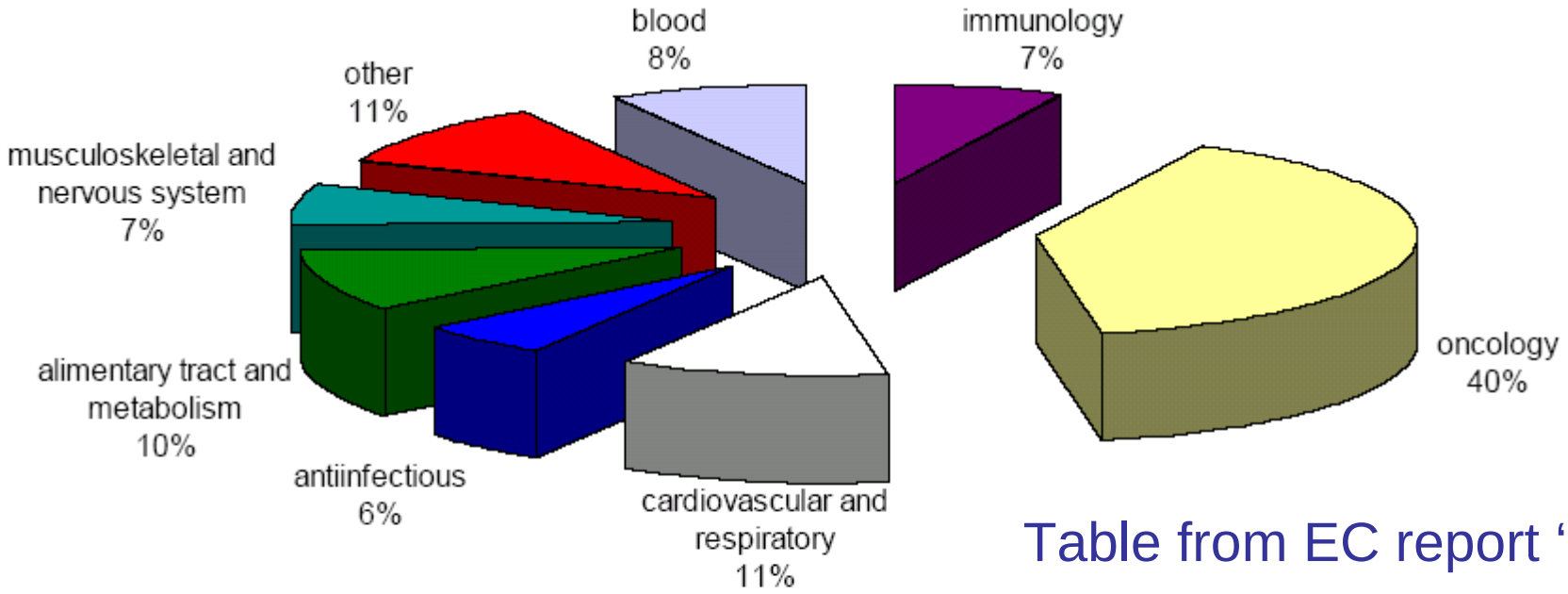


Table from EC report '06

immunology | oncology | cardiovascular and respiratory
antiinfectious | alimentary tract and metabolism | musculoskeletal and nervous system
other | blood

Industry was and is involved - 2

- In the beginning, mostly small companies were developing Orphan Drugs
- Gradually more interest from large companies
- The development of Orphan Drugs is not only for the most frequent rare diseases…
- Compassionate and expanded use programs
- Helping patients in emerging nations

10 years after… a success story!

- From only 8 before the regulation to now + 60 approved orphan drugs
- 2001-08, average 21% per year increase in approved OMPs
- 2008: > 15,000 private or public RD-resear ch projects* and about 2,530 clinical trials on OMPs**
- Growing number of new rare disease indications explored in other areas (e.g. due to the pediatric regulation, advanced therapies regulation and to pathway-driven R&D efforts)

* Source Unpublished data Office of Health Economics study for the industry task force
** Source: OHE/Orphanet data

Challenges in development

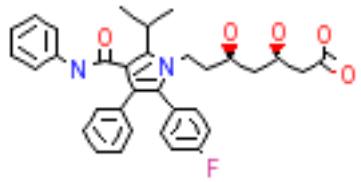
- Challenge of **rarity**: a disease affecting 500 citizens is much more challenging than one affecting 200.000
- Issues are becoming more obstructive with rarity
  - Disease awareness: low
  - Patients diagnosed: heterogeneous and low number
  - Availability of reliable testing: low
  - Number of experts: low
  - Availability of natural history data: low
  - Prior clinical trial experience
  - Regulator experience: low
  - Priority in health care system: depending on the region

Challenges in Manufacturing

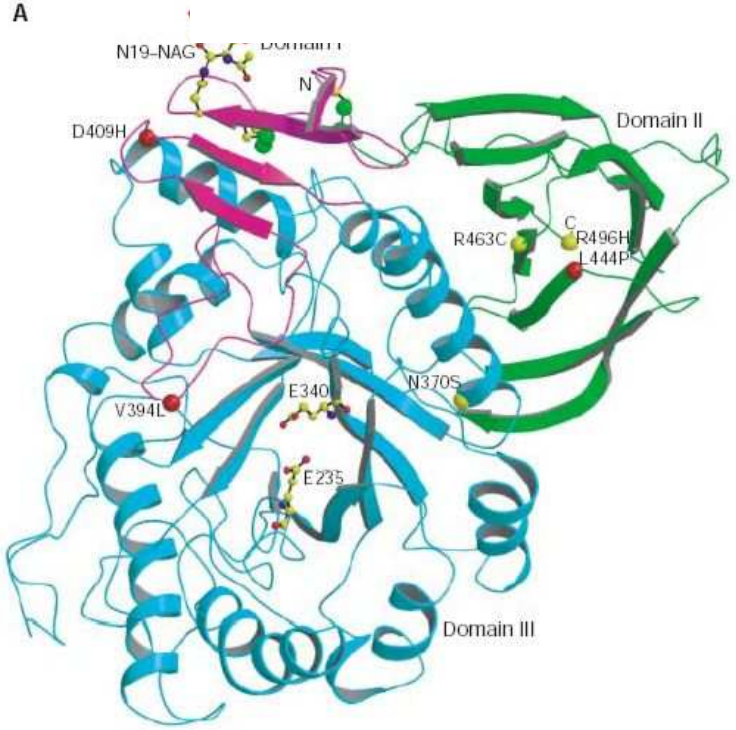
- Production of biopharmaceuticals is complex, resource-intensive and time-consuming

- Production capacity for biologicals costs hundred of millions of euro's, even for rare diseases

Lipitor® (Atorvastatin)



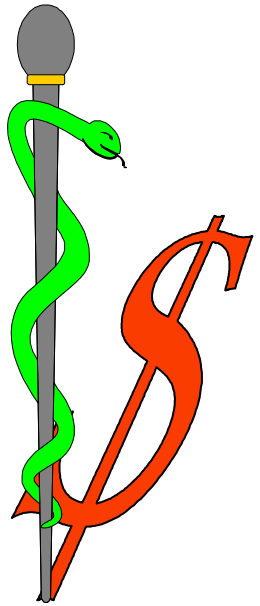
Cerezyme® (Gaucher's disease)




BUSINESS
ETHICS
The Economist

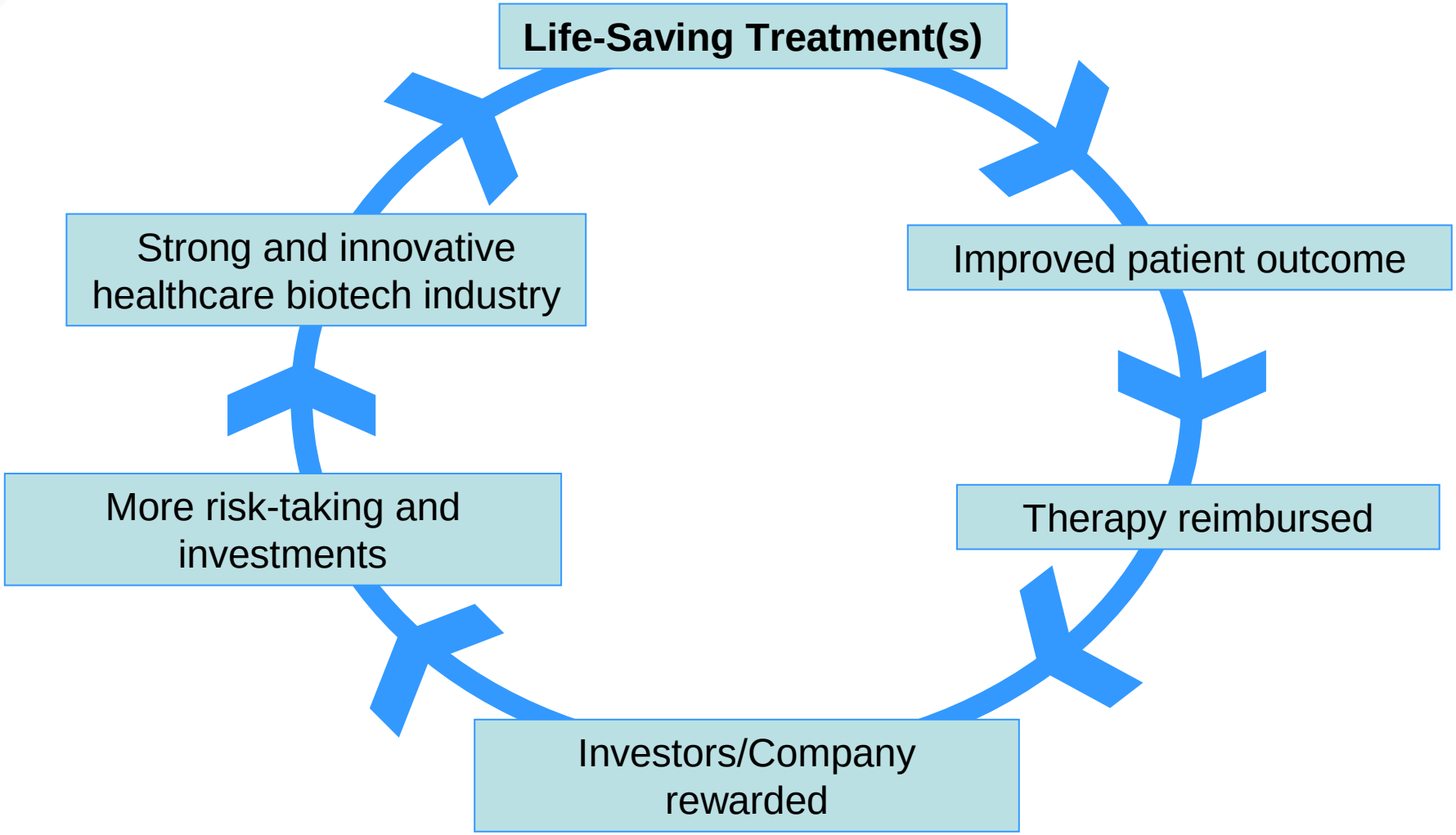
Challenges in Pricing

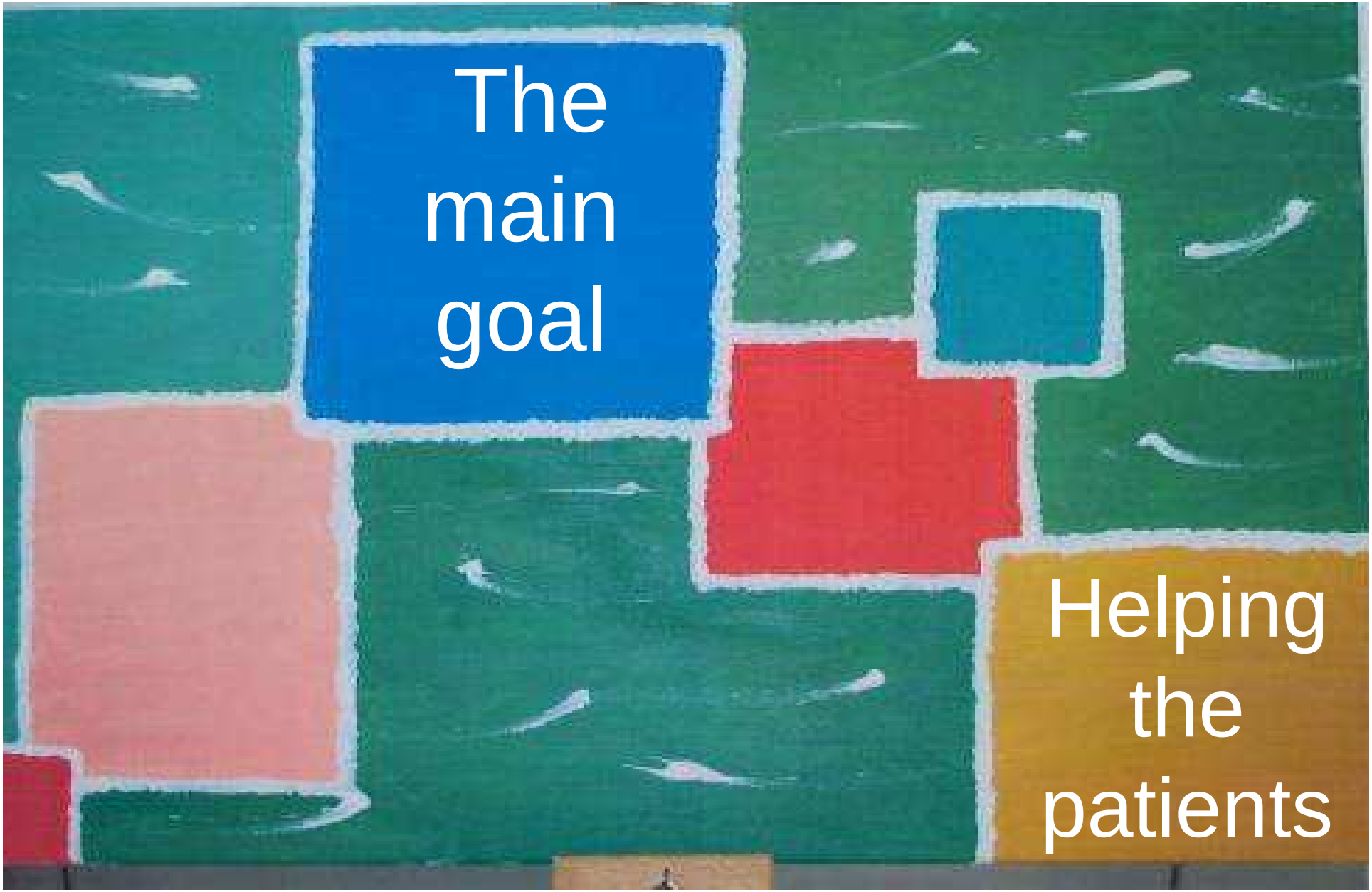
- Degree of innovation?
- Therapeutic value?
- Rarity of the disease treated?
- Uniqueness of the products?
- Degree of competition?



- Pricing is very important to ensure that a profit is made, and profit is very important to have a sustainable company, also for the treated patients
- Key to sustainability is a Market

The Wheel of sustainability





Yolanda Santos, Pompe disease, *Expression of Hope*

genzyme

Hvala lijepa

Dankon al vi

Efharisto poli

спасибо

Grazie!

Merci beaucoup

Dziekuje

Thank YOU

Tack så mycket

Tusen takk

Gracias

Obrigado

Xie Xie

Mange tak

Dank u

Danke vielmahls

Çok tesekkür ederim

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