Business Models for Orphan Drugs

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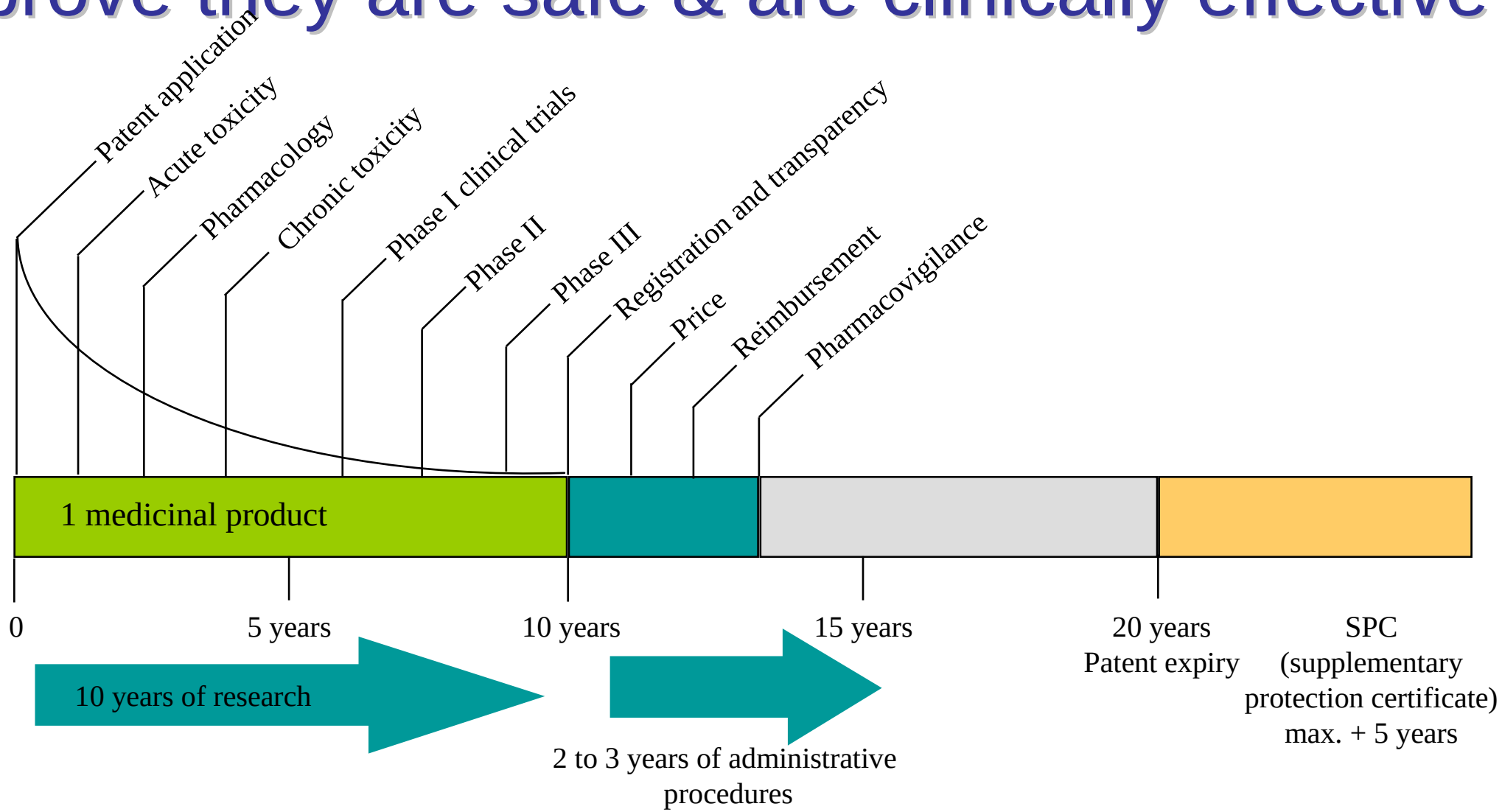
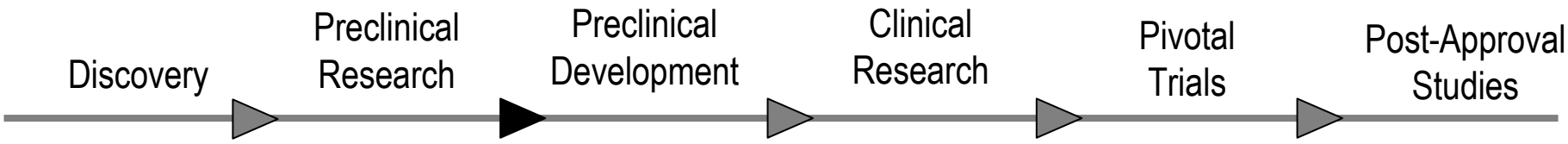
Like any other drug, orphan drugs must prove they are safe & are clinically effective



Developing a new therapeutic is ….



regulatory review

4.3 yrs — 6.8 yrs — 1.3 yrs

12.4 Years

Lengthy

w/o failures **$170M**

w/ failures **$560M**

w/ failures **$1.2B** (11.5% Discount Rate)

Costly

Risky

preclinical success rate **very low**

<25% clinical success rate

85% ph1 to ph 2

45% ph2 to ph3

70% ph3 to approval

Post - Approval

73% need additional studies

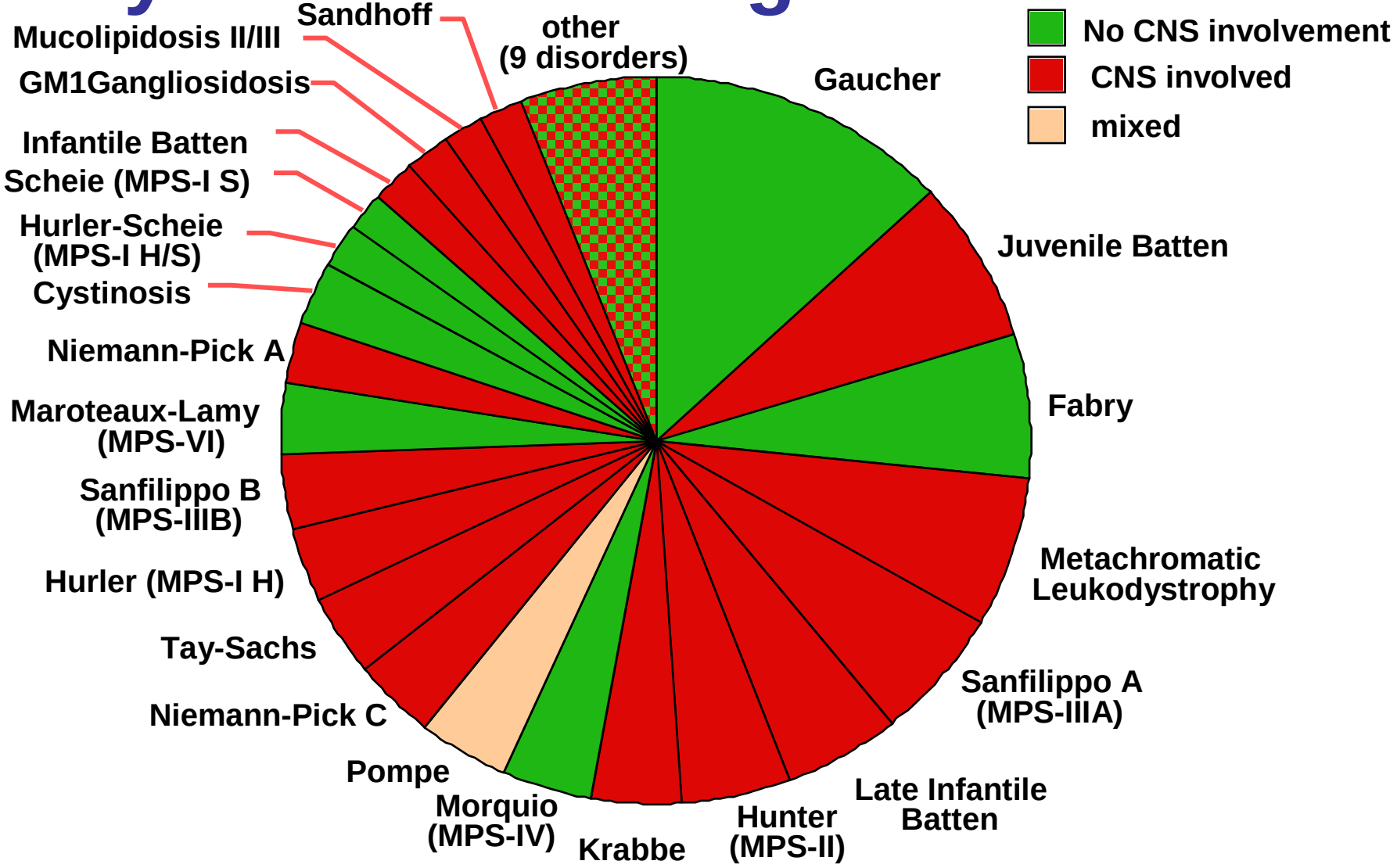
30% recoup develop. costs

Success is very rare!

*Recent Biotechnology industry metrics, small molecule drug metrics are similar

Center for Study of Drug Development, Tufts University, 2006

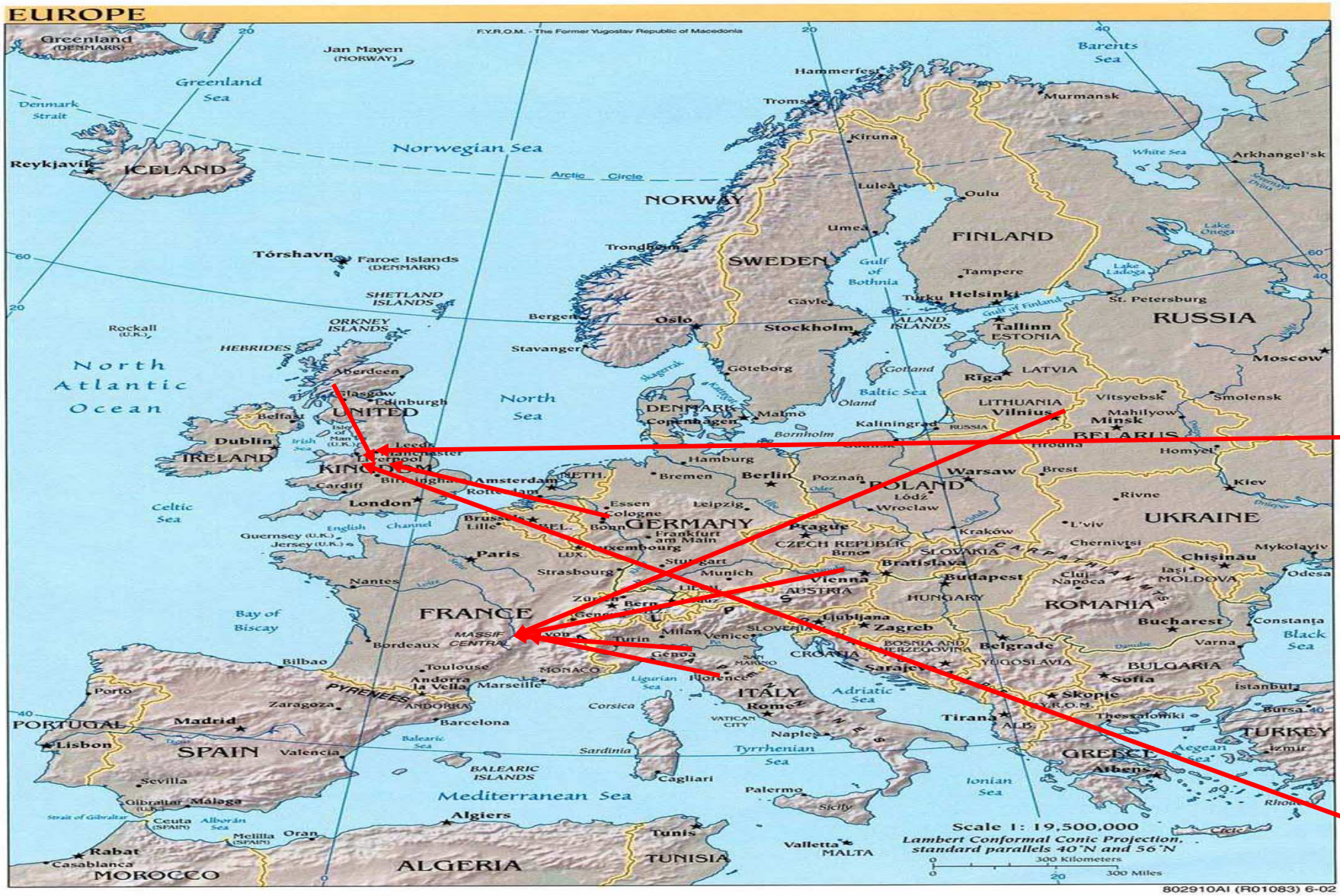
Examples of very rare diseases
Lysosomal Storage Diseases



from Meikle et al, JAMA 281:249-254, 1999; Batten data mapped from worldwide estimates

Clinical Trials for a Rare Disease

Relocation of very young infants to EU sites for trials in Pompe Disease



Development of Orphan Drugs

- On the basis of innovative basic research
- As additional indications of approved medicines that are still in use
- From raw materials unofficially used for treatment
- Based on off-label use information of approved orphan or non-orphan drugs
- Based on old / abandoned products (mostly no longer patented so market exclusivity is needed)
- Based on rescue of products with adverse effects in some other indication
- A mixture of the above

Understanding Risk

- Developing OMPs is an important risk
  → R&D or approval *doesn't* mean reimbursement
- About 33% of orphan drugs are the first treatment in history → into uncharted territory
- The sustainability of the business is highly important: for investors, but even more so for patients
- Prevalence and incidence data not well studied (overestimated?)
- Finding the patients : diagnosis (screening?)
- Undeveloped "infrastructure" and Need for Centers of Excellence

10 yrs after in the EU … New business models

Business model examples	Key activities	Average company size
Development of new RD indications for "old", off-patent compounds	*Clinical development*	*SME, Medium size (in future "Big Pharma"?)*
Development of innovative products for one/very few RD → no marketed products*	*R&D, pre-/clinical, development*	*Start Up* *SME*
Development and marketing of targeted innovative products for one/few RD	R&D, pre-/clinical, development, marketing	SME Medium size
Development/marketing of innovative RD products in diverse healthcare product portfolio	R&D, pre-/clinical development, marketing	Medium size, "Big Pharma"

* "products" includes drugs, diagnostics and vaccins

Access to ODs means understanding of

- The needs of patients with rare diseases
- The biology of the disease
- Rarity and its impact on diagnosis, clinical trials, manufacturing, price, infrastructure…
- Role of industry and the strategic risks involved
- Regulatory approval
- Reimbursement approval
- The ethical aspects

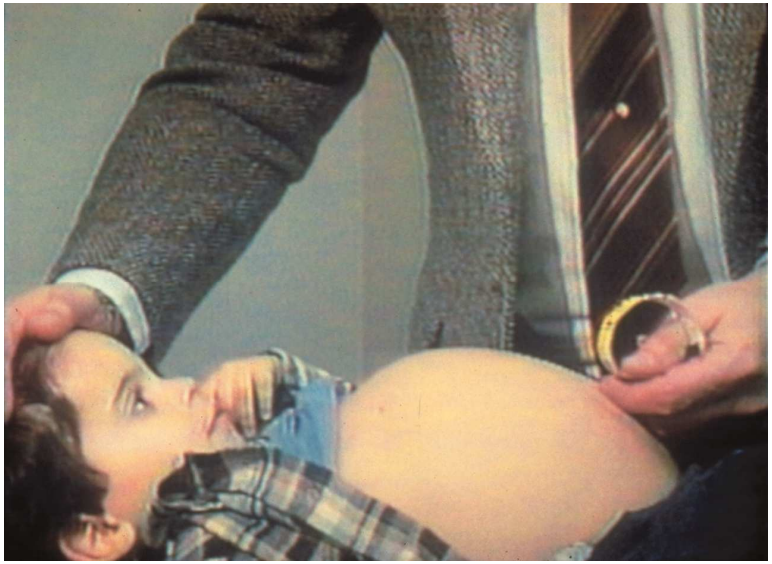
Other factors influencing “access or availability”

- Understanding the different national/regional reimbursement: small companies cannot tackle all at same time
- Are there patients to treat?
- Price not accepted for reimbursement & Price/Benefit ratio
- Clinical trials, special programs …

Contributions to solutions:

- Cross-country collaboration of all stakeholders
- Registries and post-marketing data
- Timely diagnosis: develop systems and tools
- Centers of reference: the infrastructure
- Support for early availability of diagnosis and treatment

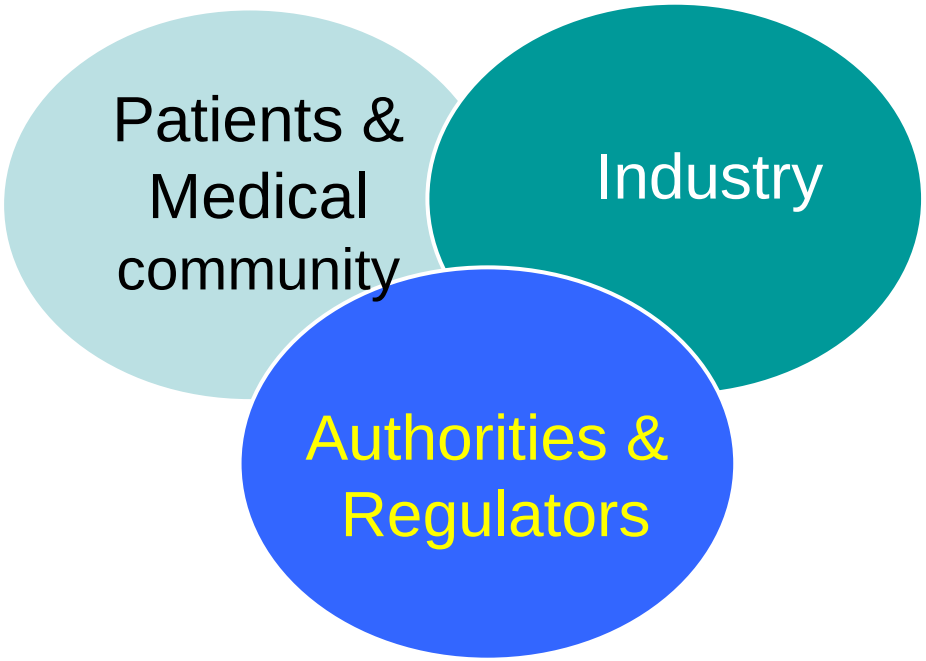
The Ultimate Reward



Industry part of the solution in next 10 yrs

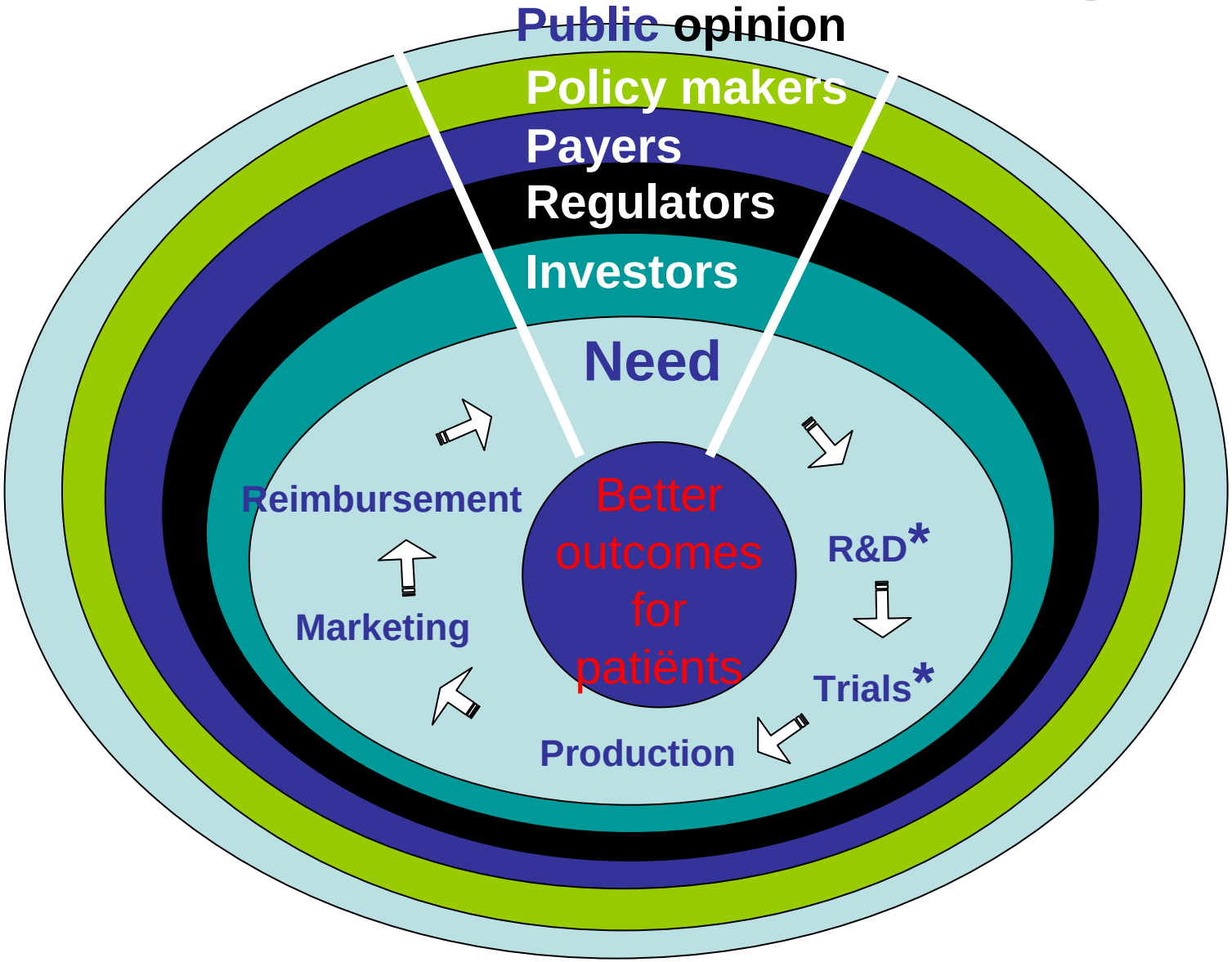
- Create awareness for medical need and patient rights
- Provide and request information
- Establish networks and communities

- R&D for new, safe&effective drugs
- Create competitive environment
- Compassionate use programs
- Share knowledge



- Provide incentives → foster innovation
- Create framework for R&D
- Grant timely and equal access to treatment
- Adapt regulations to high and different needs
- Ensure sustainability of healthcare systems

The wheel of Sustainability



* Collaboration of academia and industry

Hvala lijepa

Efharisto poli

Dankon al vi

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

genzyme