

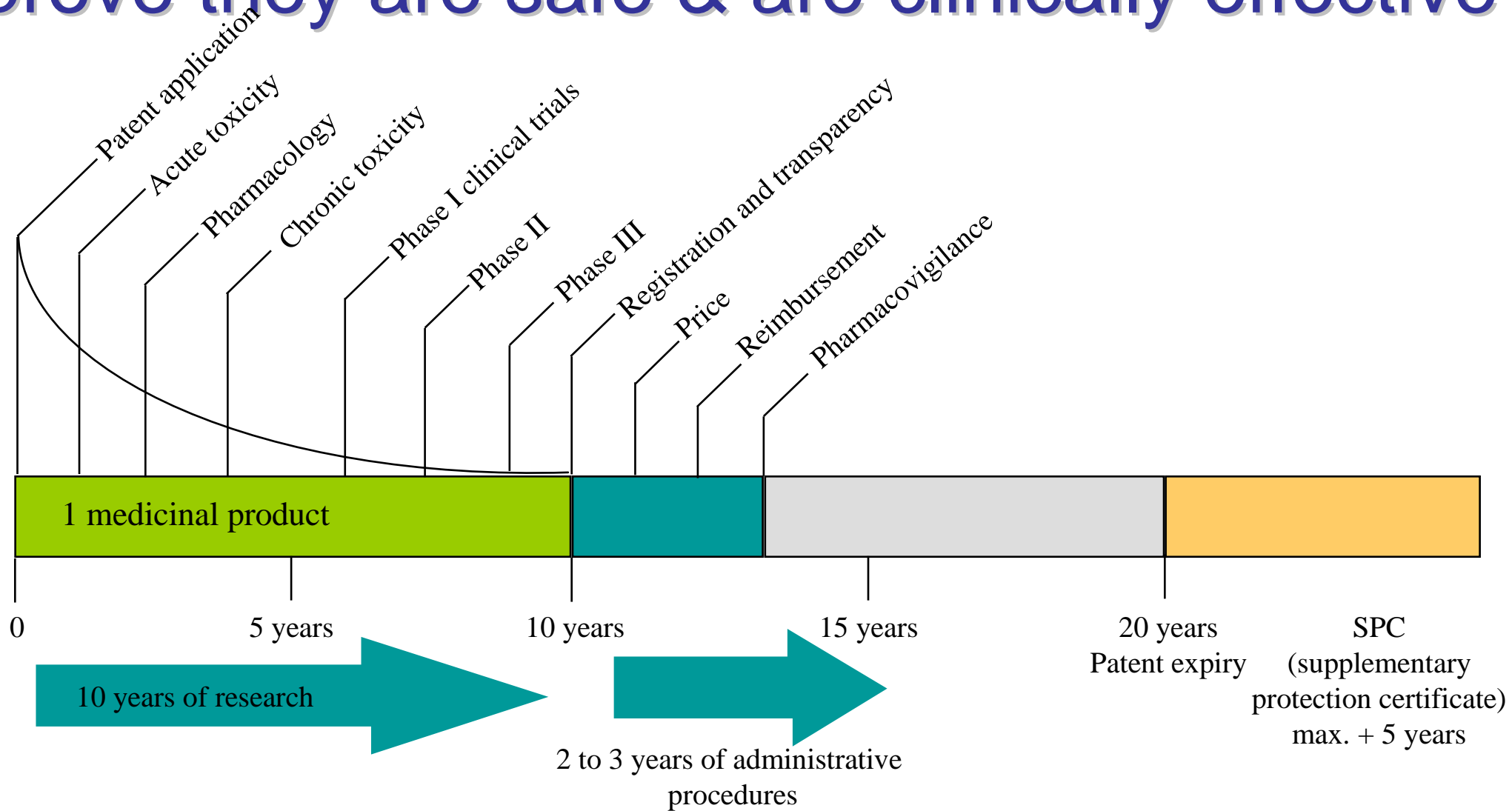
Business Models for Orphan Drugs

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



Developing a new therapeutic is



Lengthy

4.3 yrs (Preclinical Research to Preclinical Development)
 6.8 yrs (Preclinical Development to Pivotal Trials)
 1.3 yrs (Pivotal Trials to Post-Approval Studies, including regulatory review)
12.4 Years (Total duration)

Costly

w/o failures \$170M
 w/ failures \$560M
 w/ failures \$1.2B (11.5% Discount Rate)

Risky

preclinical success rate very low

<25% clinical success rate

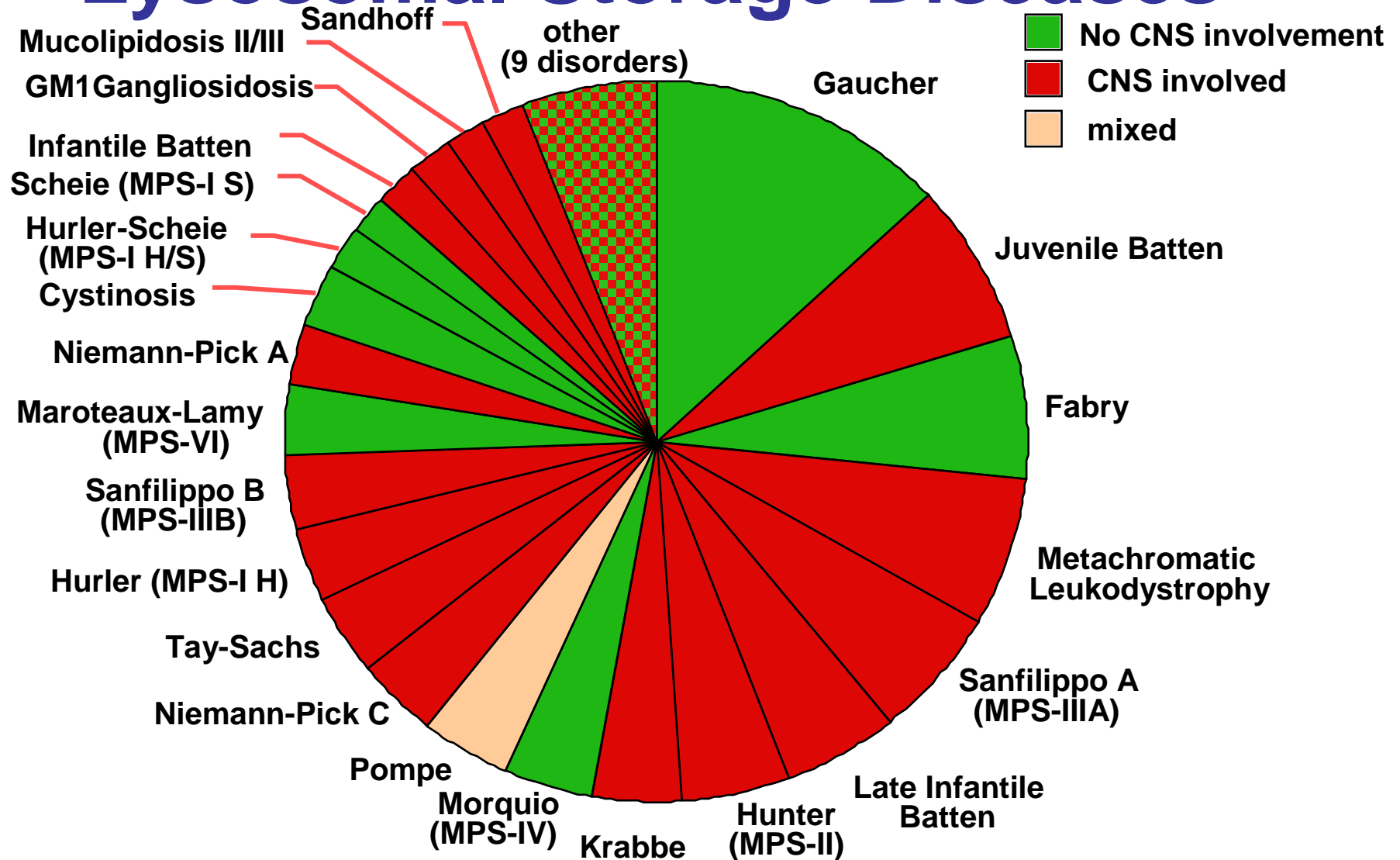
85% ph1 to ph 2 45% ph2 to ph3 70% ph3 to approval

Post - Approval
 73% need additional studies
 30% recoup develop. costs

Success is very rare!

Examples of very rare diseases

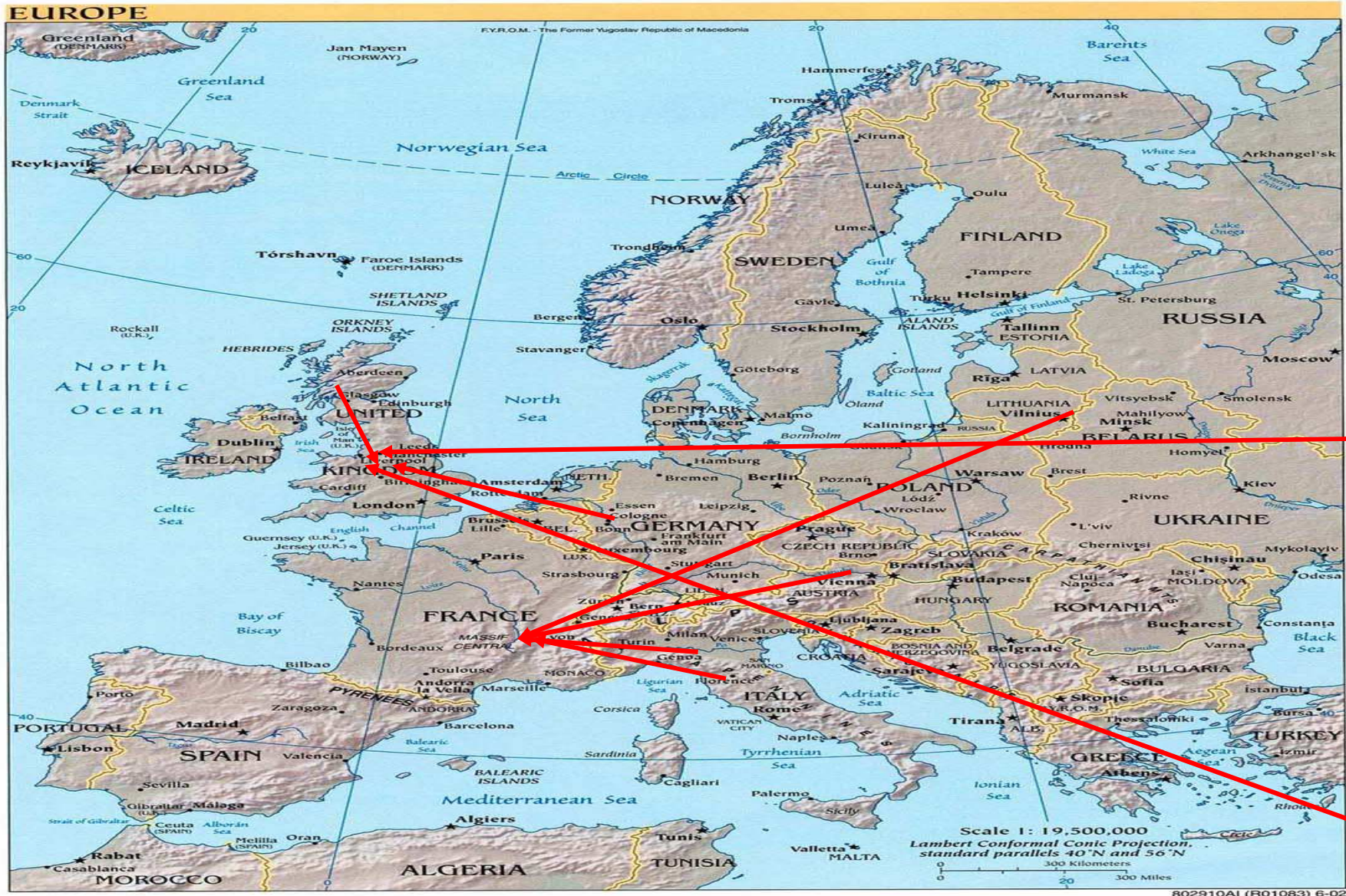
Lysosomal Storage Diseases



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



Japan

Israel

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 |
|---|--|---|
| <i>Development of new RD indications for “old”, off-patent compounds</i> | <i>Clinical development</i> | <i>SME, Medium size (in future “Big Pharma”?)</i> |
| <i>Development of innovative products* for one/very few RD → no marketed products</i> | <i>R&D, pre-/clinical, development</i> | <i>Start Up SME</i> |
| 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 vaccines

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:

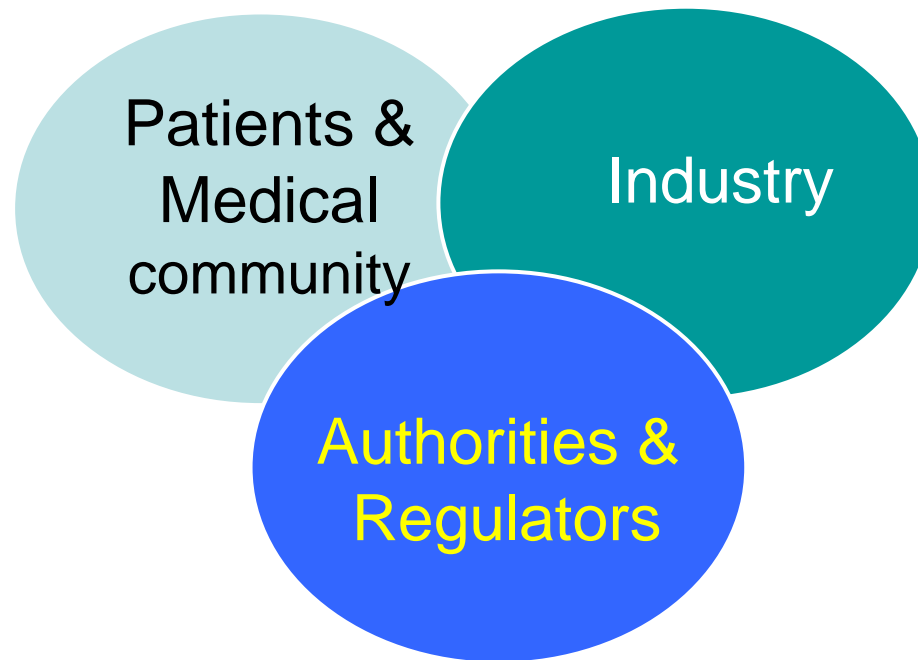
- Cross-country collaboration of all stakeholders
- Registries and post-marketing data
- Timely diagnosis: develop systems and tools
- Centers of reference: the infrastructure
- Support for early availability of diagnosis and treatment

The Ultimate Reward



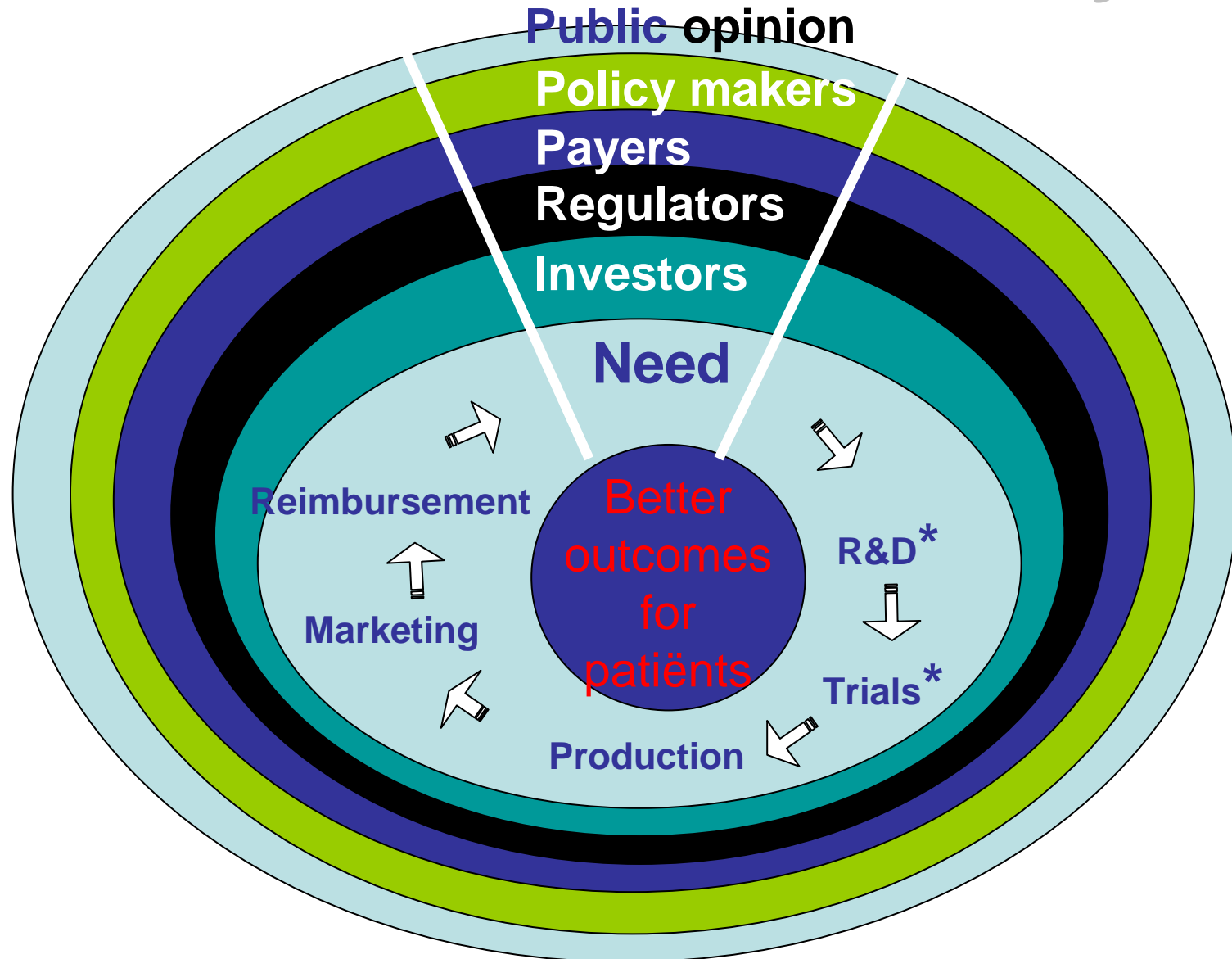
Industry part of the solution in next 10 yrs

- 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

Grazie!

спасибо

Thank
YOU

Merci
beaucoup

Dziekuje

Tack så mycket

Tusen takk

Gracias

Obrigado

Xie Xie

Mange tak

Dank u

Danke vielmahls

Çok teşekkür ederim

genzyme