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# Gaining Access to Orphan Products: Sustainability

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# The Spirit of Orphan Drug Legislation

- To provide timely and equitable access to therapies for rare disease patients
- To balance the risk by providing economic incentives to industry to develop therapies
- Patients with rare disorders deserve the same care as patients with common diseases
- However, successful development and regulatory approval do not automatically mean access

# Diagnosis of rare diseases

- Time delay from first doctor's visit to diagnosis (Genzyme surveys)
  - Fabry……..16 Years
  - Gaucher……..4-13 Years
  - Scheie……7.5 Years

- Prevalence of late or misdiagnosis
  - Fabry……78%
  - Gaucher……...74%
  - Pompe……..67%
  - MPS 1……...60%

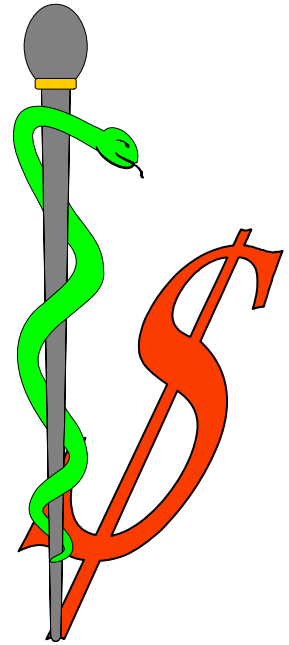
- Consequences : 16% unnecessary surgery, 33% unadapted therapy, 10% unnecessary psychological treatments (Source: Préscire, France)

# Challenge of developing drugs for Orphan Diseases

- Challenge of Rarity: Continuum with increasing complexity
  - Disease affecting 500 citizens much more challenging than a disease affecting 200.000
- Issues are becoming more obstructive with rarity
  - Disease awareness: low
  - Patients diagnosed: Heterogeneous and low number
  - Availability of reliable testing: low
  - # of Experts: low
  - Availability of natural history data: low
  - Prior clinical trial experience: low (endpoints, biomarkers,… )
  - Regulator experience: low
  - Priority in health care system: now higher, was low

# Pricing challenges

- 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

# Responsibility, does not stop at approval

- Potentially life saving therapy without satisfactory alternative
- Responsibility starts at first demonstration of safety and efficacy
- How do you deny therapy?
- If the market is too small, can you quit?

# Key to Sustainability = Market for these therapies

- Large companies → greater ability to absorb risk than small companies, charity may work
- Small companies may have only 1 orphan drug
- However, NO company or investor will support the development of a drug for the western world if they know there is no market

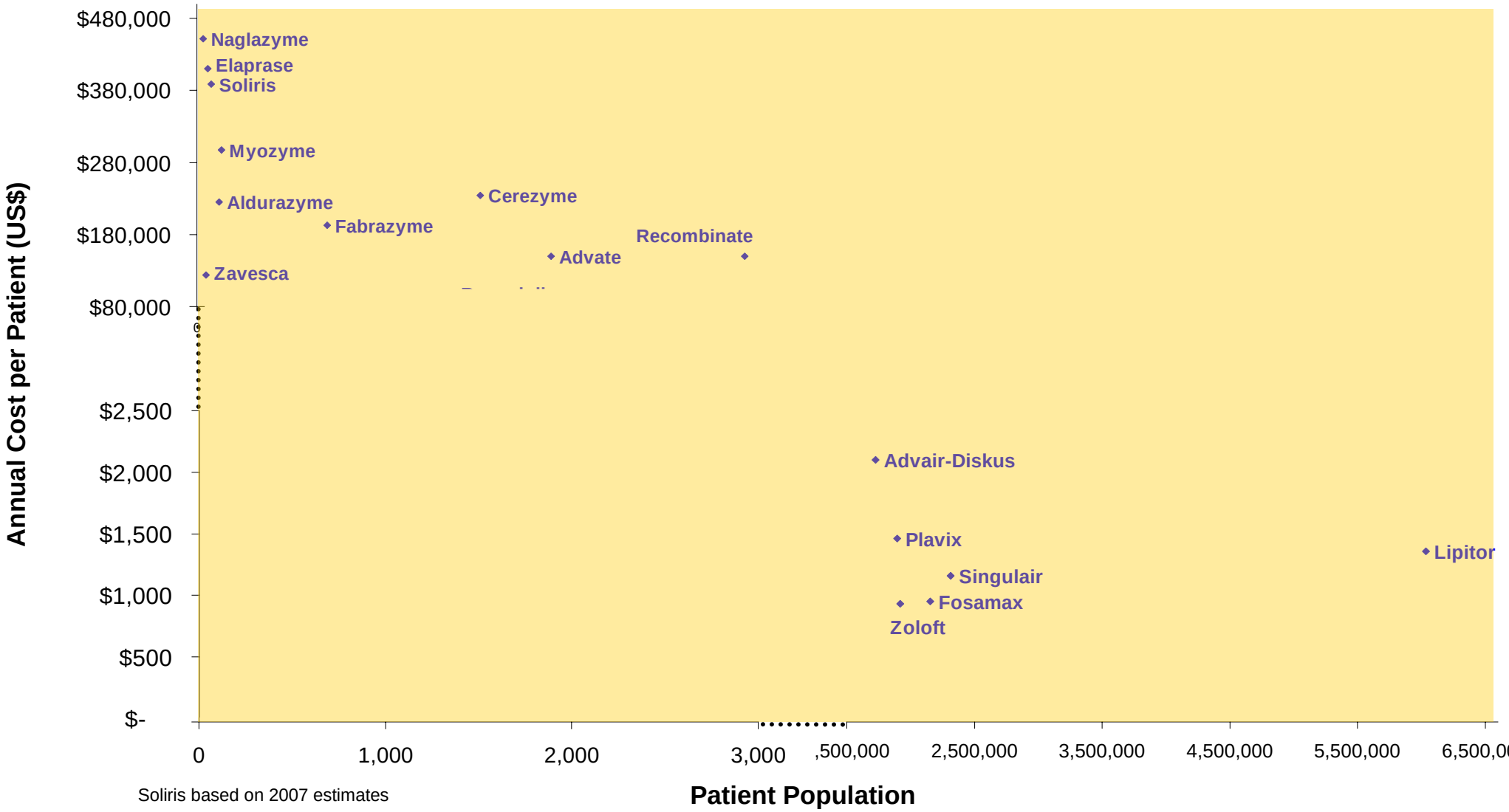
=>Business Model Must Be Sustainable

# Cost of Therapies: we are early in the life cycle

- Smaller disease = higher per patient costs
- Total cost to the system remains low if total number of patients treated is small
- If market becomes larger: competition will enter (EPO, HIV, …) ànd better therapies developed
- If market is small, little or no competition will enter because problem has been addressed and market is not large enough

=> Overall cost to society should remain small

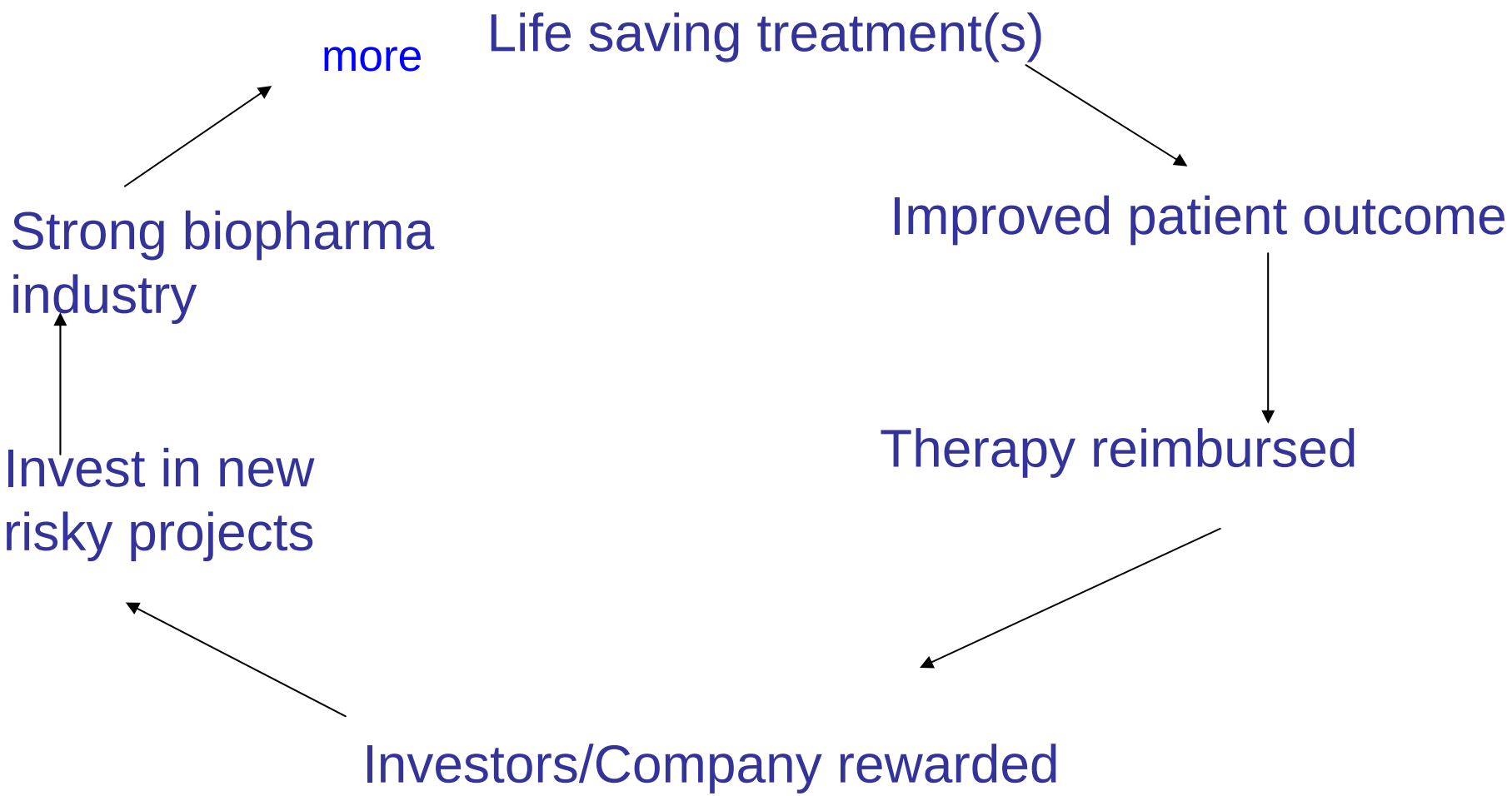
# Price per Patient Function of Patient Population




Soliris based on 2007 estimates

# Pricing Challenges

# Circle of Sustainability



# THANK YOU