



**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%

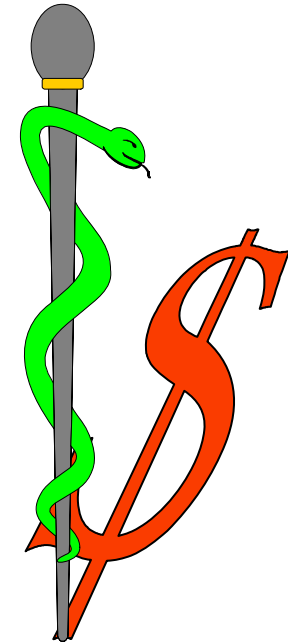
- Consequences : 16% unnecessary surgery, 33% unadapted therapy, 10% unnecessary psychological treatments (Source: Préscire, France)

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?
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- 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

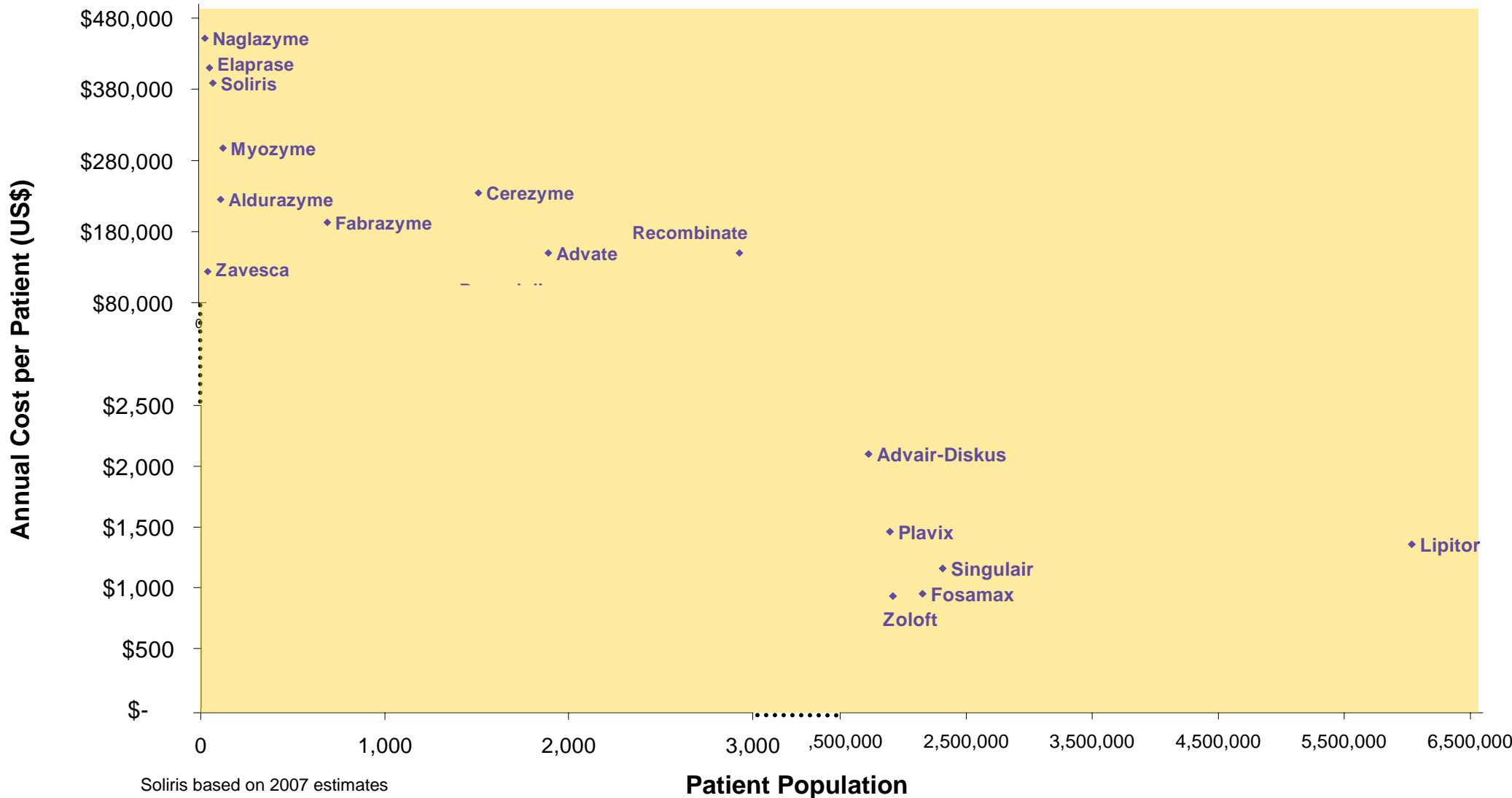
=>Business Model Must Be Sustainable

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, ...) and 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

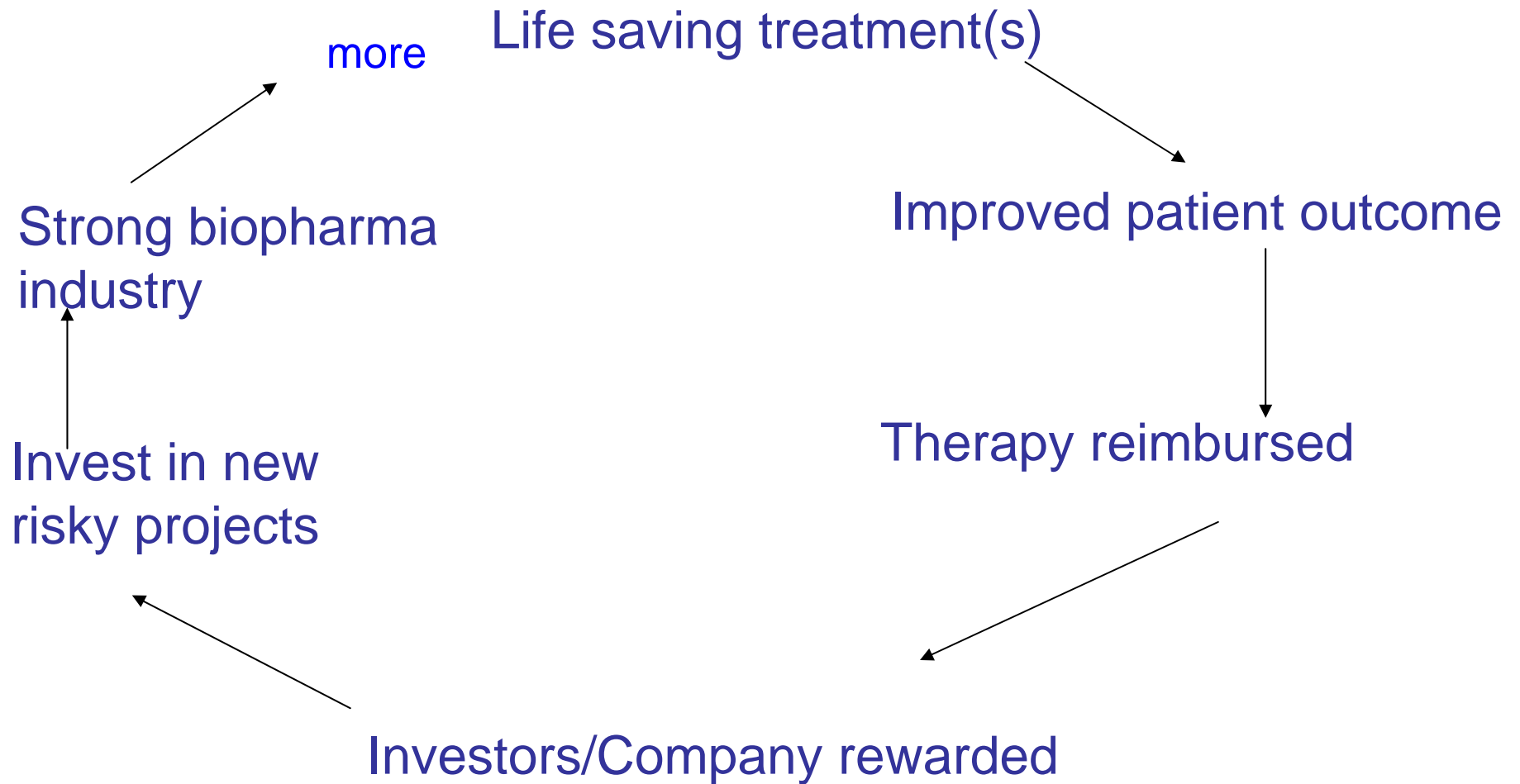
Price per Patient Function of Patient Population



Pricing Challenges



Circle of Sustainability





THANK YOU