Why are rare diseases of interest to industry

Brihad Abhyankar MS FRCS MBA FFPM
Executive Medical Director
Takeda Development Centre Europe

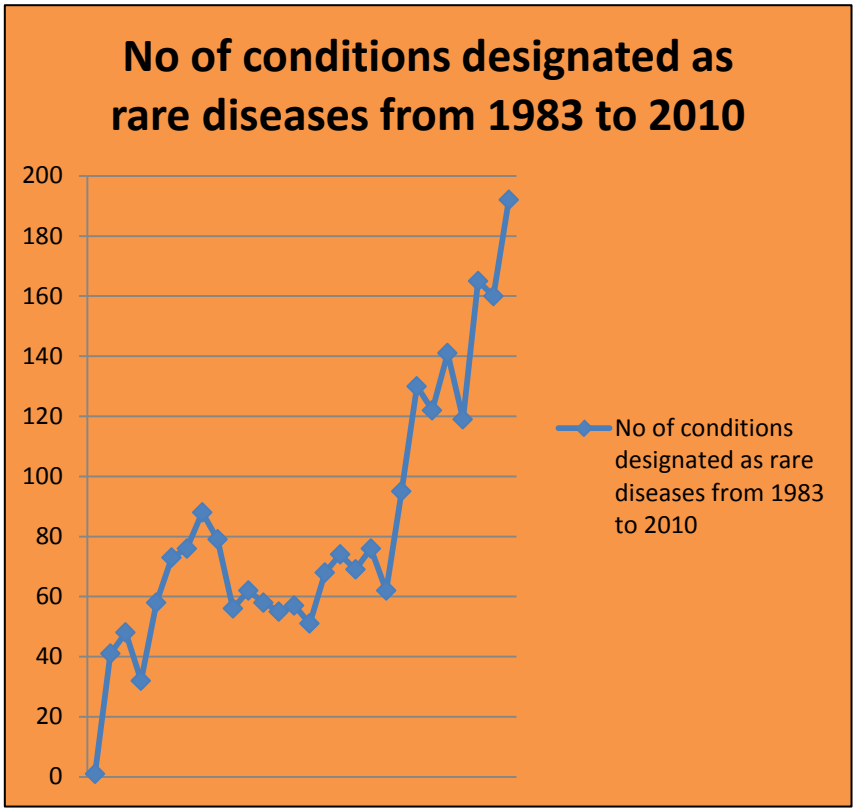
Note: The views expressed here are entirely my own and not of my employer or any pharmaceutical industry association

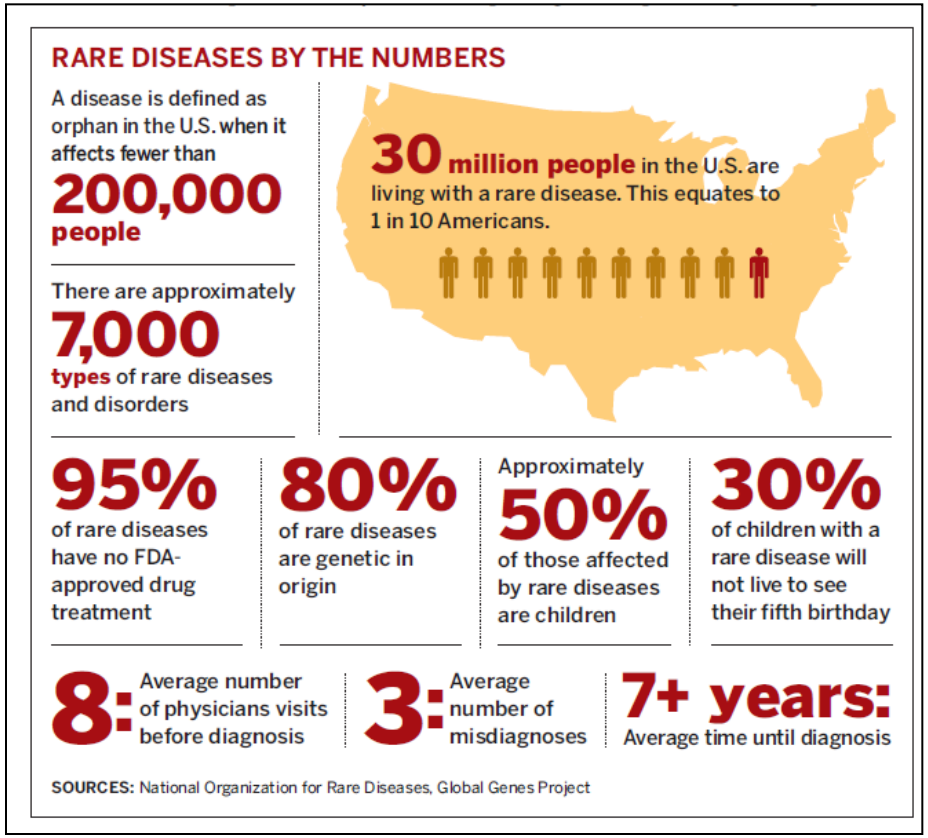
Environmental

- End of ‘blockbuster’ model
  - Increased pipeline attrition
  - Increased R&D spending
  - R&D productivity is becoming challenging
- Desire for personalized medicine
- Advances in science offering clues to pathophysiology
- ‘educated’ patient community

Scientific

- Significant advances in understanding of diseases





Scientific

- Many rare conditions are genetic in origin and therefore manifest early in life.
- Industry attempts to address pathology
  - Re-purpose current product and pursue new indication
  - Screen novel compounds for activity against rare disease targets
- Many phenotypic syndromes or conditions increasingly identified as distinct identified diseases

Regulatory

- Regulatory path and incentives from FDA, EMA and others.
- Many factors involved.
- Higher probability (93%) of regulatory success compared with 88% for non-orphan drugs ($p < 0.05$).
- Phase II to launch timelines
  - orphan drugs 3.9 years
  - Non-orphan 5.42 years

Meekings KN, et al Orphan drug development : an economically viable strategy for biopharma R&D. Drug Discov Today 2012;17:13-14; 660-664

Commercial

- Increasing strategic choice made by some companies to develop new drugs for rare diseases.

Selecting a rare disease for development

Commercial consideration	R&D/ scientific consideration
Prevalence	Understanding of biology and pathophysiology
Geographic and demographic distribution	Availability of clinical and scientific experts
Available treatments	Unmet need and available patient population

Summary

- These are generic perspectives from a clinician in the industry but not from a specialist in ‘rare disease’ development.
- Patients with a rare diseases have an equal right to medicines as do other patients with a well-known disease
- For many rare diseases there is still no treatment available
- But many advances are being made and likely to grow
- Industry, Regulators, Scientists and Clinicians need to work even closer together