Challenges to Orphan Drug Development

Lack of an animal model

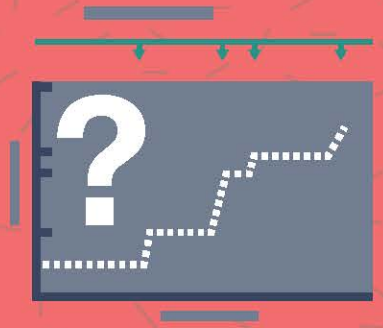
that shows similar signs/symptoms to a human with the disease (or, at minimum, shows signs/symptoms that can be measured and tested)

Control Group not possible or ethical

For very rare, rapidly progressive, fatal diseases, there may not be sufficient patients for controlled trials, and there can be ethical concerns with having placebo controls in clinical trials. In these cases, data on the natural course of the disease that are collected outside the trial may be used for comparison.

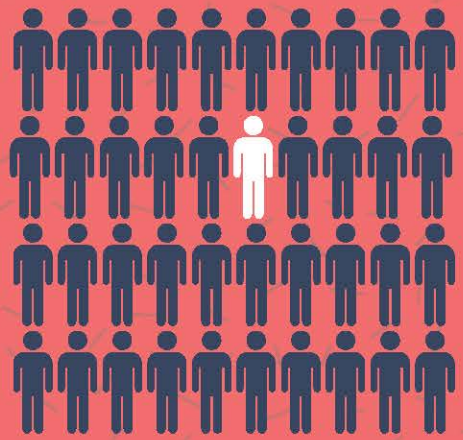
Poor understanding of the natural history

of many rare diseases (e.g. clinical features, progression, signs and symptoms) means it is difficult to develop new therapies, design clinical studies and determine an appropriate trial duration



Lack of appropriate endpoints

(e.g. lab values, patient-reported outcomes (PROs), clinical exams/ assessments, etc) available for a clinical trial to show that a therapy is effective



Very small numbers

of people with each orphan disease leading to challenges recruiting enough patients to participate in trials

Challenges securing Government & Insurance Company Reimbursement

for very expensive therapies

Cost of Development

Cost per patient of orphan drugs is high because there are fewer patients being prescribed orphan drugs when compared to "standard" therapies, meaning that fewer patients are available to cover the cost of development.

(Jayasundara et al, 2019)

Additional Resources

Cost of Orphan Drugs vs Standard Drugs
https://ojrd.biomedcentral.com/articles/10.1186/s13023-018-0990-4

Global Genes Resources
https://globalgenes.org/resource-hub/

Why do Discovery and Development Take so Long?
https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5725284/

Standard Drug Development
https://www.fda.gov/patients/learn-about-drug-and-device-approvals/drug-development-process

the orphan disease center

Penn Medicine