



- There are 7.000 to 10.000 disorders with know molecular basis
- But only ~500 with approved therapies
- At a rate of 10-20 new approvals each year it will take **centuries before most diseases will have treatments!**

Ethical considerations will be paramount in new clinical trial designs to accelerate the drug development process

Key factors include:

- **Randomized Clinical Trials (RCTs) always required?**
- **Small patient populations/N=1 or a few**
- **Role of historical controls/natural history studies**
- **Continued access to drugs after trials**
- **Reliance on animal studies to show preclinical efficacy**