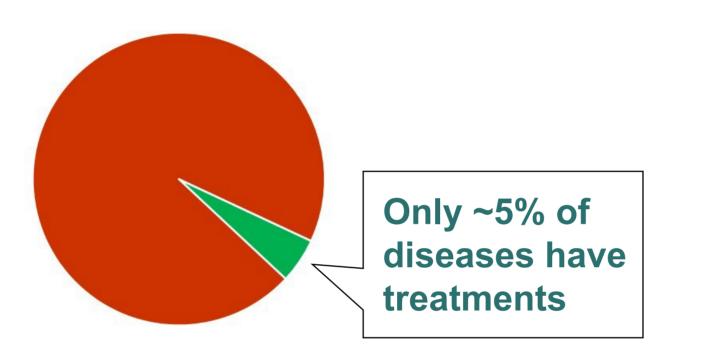


European infrastructure for translational medicine





- 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