Drug Development: Regulatory Pathway

Biotechyst
BIOTECHNOLOGY INSIGHTS

0-1 year

Research, discovery & prototyping

2-4 years

Preclinical testing & trials approval

- Toxicology studies (non-human: *in vitro; in vivo* - animal)
- FDA Investigational New Drug (IND)(AW1) application filing
- Potential pre-IND FDA meeting - human trial initiation requirements & regulatory pathway discussion

4-6 years

Phase 1 Clinical Trials

63.2% success rate

- First human testing
- ~20-80 trial participants (small group)
- Evaluate safety, dosage & sides effects, etc.

6-8 years

Phase 2 Clinical Trials

30.7% success rate

Highest failure rate (69.3% fail) - if phase 2 trials are successful, risk begins to decrease

- Larger trial participant group (100's)
- Safety & efficacy evaluation
- Pre-end FDA meeting to determine whether adequate safety & efficacy data exists to move to phase 3 trials

8-10 years

Phase 3 Clinical Trials

58.1% success rate

- FDA engagement: trial design, proposed protocols & identification of supporting information for New Drug Application (NDA) or Biologics License Application (BLA) (AW1)
- Large trial participant group (100's - 1000's)
- Efficacy & side effects tested; comparison with other treatments or interventions

10-12 years

Regulatory approval

85.3% success rate

- Successful completion of phase 3 trials: pre-BLA/NDA submission FDA meeting - discuss data in support of the NDA
- BLA/NDA submission
- FDA review of the NDA:
  - Data, safety, effectiveness, labeling & production facility review
  - `approval' letter issued - deemed suitable to enter market
  - 'complete response' letter issued - not deemed ready – may include list of correctable deficiencies and post-approval study requirements

12-14 years

Phase 4 Monitoring

- Post-launch general population efficacy and effectiveness study
- Monitoring of adverse reactions/side effects associated with continued or prolonged use
- Additional potential use identification for the intervention (different condition or combination with another intervention)

References and further details

- AusBiotech. Guide to Life Sciences Investing. https://www.ausbiotech.org/documents/item/451 (2018).
- U.S. Food & Drug Administration (FDA). FDA's Drug Review Process: Continued. U.S. Food & Drug Administration (FDA) https://www.fda.gov/drugs/information-consumers-and-patients-drugs/fdas-drug-review-process-continued (2015).
- Thomas, D. et al. Clinical Development Success Rates and Contributing Factors 2011–2020. (2021).