Orphan Drugs

FDA approves first AI drug for rare disease

What is an Orphan Drug?

Orphan Drug Status is a designation given by the U.S. Food and Drug Administration (FDA) to drugs and biologics intended to treat rare diseases or conditions. A rare disease, according to FDA guidelines, affects fewer than 200,000 people in the United States.

- **Incentives:** Companies that develop orphan drugs are eligible for various incentives, including tax credits for clinical research, reduced filing fees, and potential for seven years of market exclusivity upon approval.
- **Development Grants:** FDA may offer grants for clinical trials and research specifically aimed at developing orphan drugs.
- **Accelerated Review:** The approval process for orphan drugs can be faster than that for other drug categories, although it still needs to meet the FDA's safety and efficacy standards.
- **Protocol Assistance:** The FDA can provide scientific advice and guidance to help companies navigate the development and approval process for an orphan drug.
- **Orphan Drug Act:** Established in 1983, this U.S. law aimed to encourage the development of drugs for rare diseases by providing these benefits and incentives.

First AI Orphan Drug

- **Insilico Medicine**
    - INS018_055 is a small molecule targeted at idiopathic pulmonary fibrosis (IPF)
    - Disease has no apparent cause and no cure
    - Prognosis: median survival is 2 to 5 years
    - Primarily affects adults aged 50 to 70
- **Drug candidate target selected by AI**
    - Details about their process are somewhat closed
    - Was generated by their in-house Pharma.AI platform
    - Will be entering Phase II clinical trials
    - "discovered and designed using artificial intelligence"
- **Pictured at right:**
    - Alex Zhavoronkov, PhD
    - CEO of Insilico Medicine

So what?

- **Proof of Concept:** This opens the door to more investment and trust in AI drug discovery.
  - De-risking investment means more VC
  - Where the money goes, so too goes research and results
- **Serving Everyone:** Drug discovery is ludicrously expensive, hence why "orphan drug" laws exist.
  - Anyone with rare diseases knows how hopeless it feels
  - There are 17,000 unique codes in ICD-11
  - Another 120,000 total possible permutations
- **Practical vs Theoretical:** The benefits of AI and drug discovery is no longer pure theory, now it is real.
  - For instance, "New study uses AlphaFold and AI to accelerate design of novel drug for liver cancer" earlier this year
  - It's one thing to hypothetically design a drug in a lab
  - It's entirely another to get it to the FDA and clinical trials

Takeaways

- **More Diseases Treated:** As long as this pans out (or another one does) we should expect to see an uptick in AI-discovered and designed drugs.
- **More Research, More Funding:** Businesses are always seeking new revenue streams, getting 7 year exclusivity on an Orphan Drug is a pretty big incentive.
- **Possible Gold Rush:** If AI can drastically reduce the time and cost associated with discovering orphan drugs, we might see a huge surge due to latent, unmet demand.
    - Not financial or investment advice
    - I personally avoid speculative penny stocks like the plague
- **Cheaper Medicine:** This is a long shot, but I hope that AI driving the price of drug discovery down means that medicine will soon become much, much cheaper (including rejuvenation and regeneration)

Call to Action

- **Support Open Source:** Lots of companies benefit from open source tools, research, and coding examples
- **Spread the Word:** This is really great news. Tell people about it, build consensus around AI and medicine
- **Help Me Out:** Support me on Patreon, like and subscribe, comment and share
- **Vote Accordingly:** Pay attention to politicians anywhere who understand the importance of balance and nuance where AI is concerned
  - It's about balancing public good versus private interest
  - Look out for regulatory capture, monopolization

Thank you