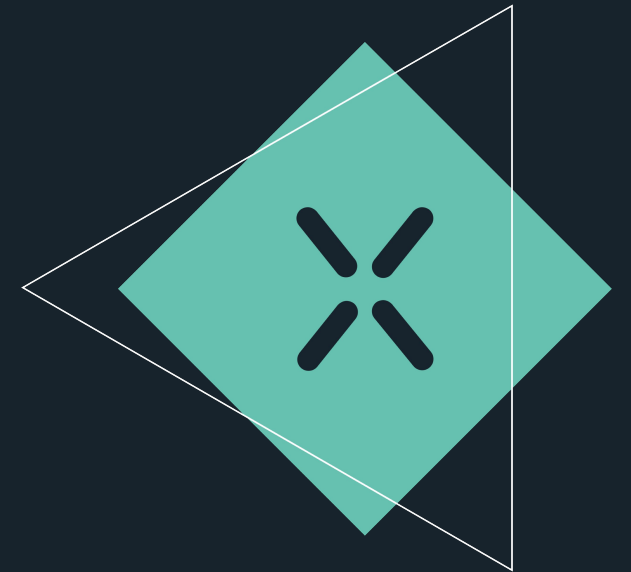




Use of AI in Rare Diseases - The Healx Experience

IPLS conference September 16th 2020

Dr. Bruce Bloom, Chief Collaboration Officer
Dr. Clara Tang, Business Manager



The problem

Rare disease patients



7,000 rare diseases



400m patients worldwide

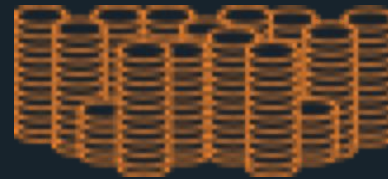


50% are children



Pharma companies rely on blockbuster drugs

R&D costs \$2-3bn



Takes 12-14 years



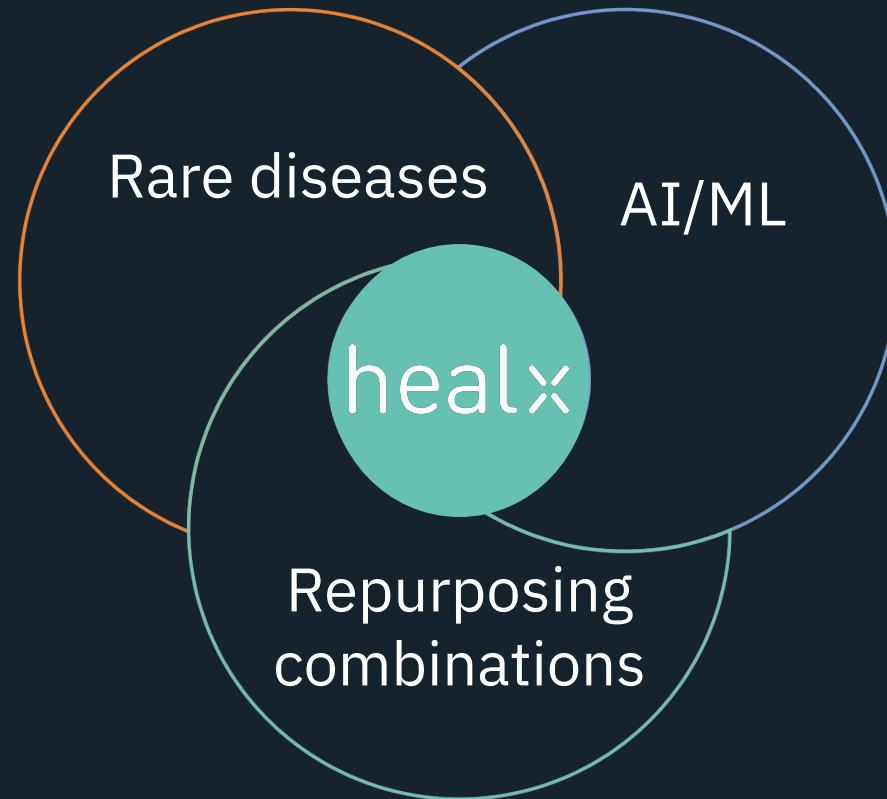
Success rate of 5%



95% of rare diseases don't have an approved treatment

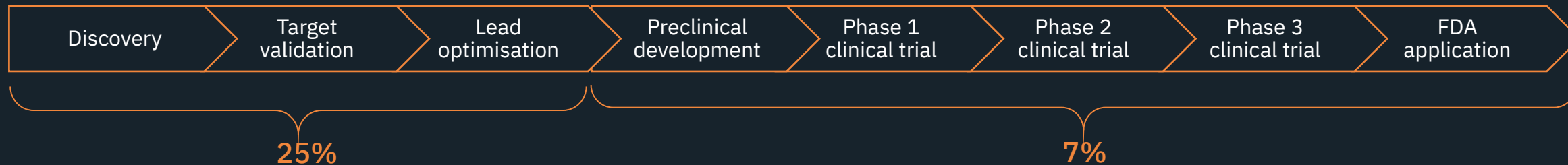
Our sweet spot

Using AI, Healx repurposes and combines existing drugs to find novel rare disease treatments at scale

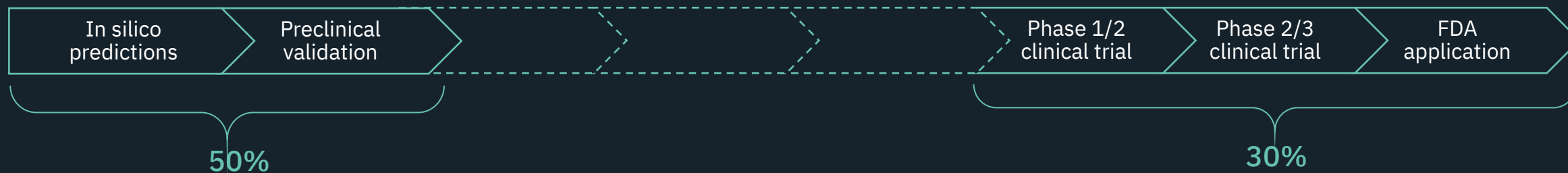


Healx AI redefines and de-risks drug discovery

Traditional drug discovery process



Drug repurposing with Healx



Healx therapeutic pipeline has 10-fold lower attrition rate

Solution: Drug repurposing is faster, cheaper and safer

Healx scales and automates rare disease treatment discovery



Faster



Cheaper



Safer

AI-powered drug repurposing

3-5 years



\$50m



25%



Conventional drug discovery

12-14 years



\$2-3bn

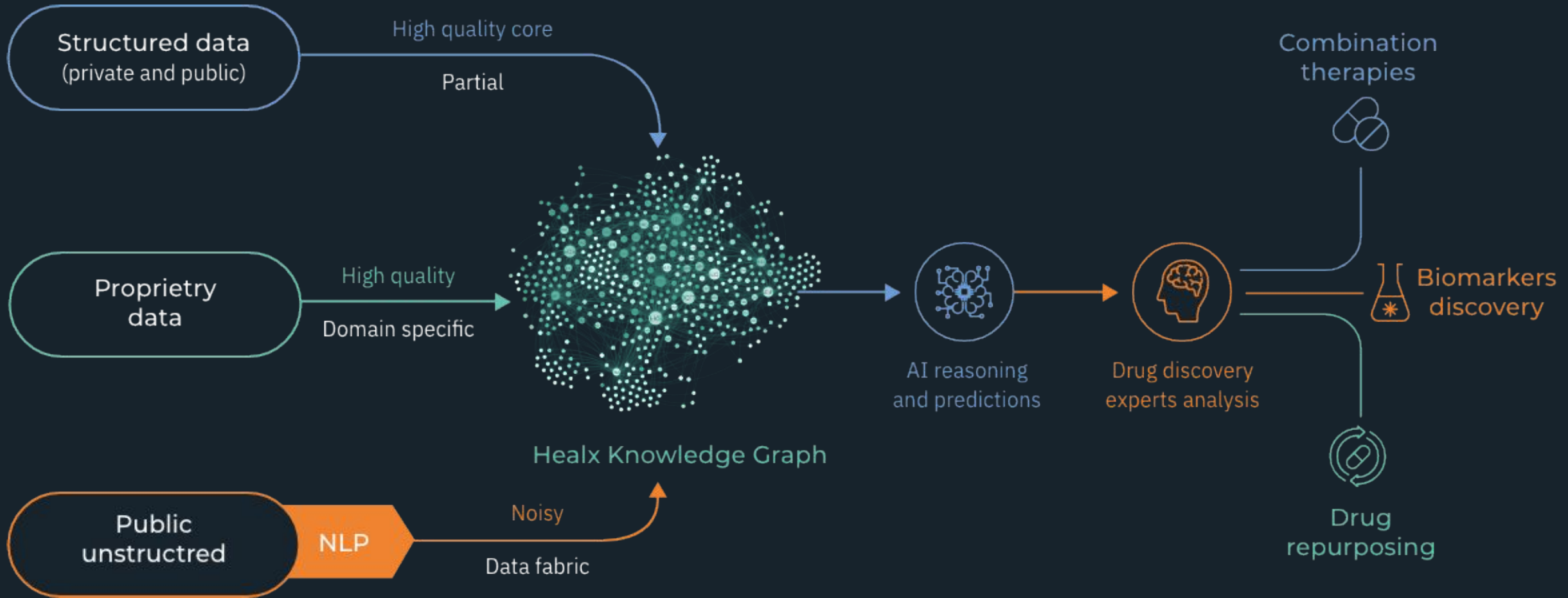


5%

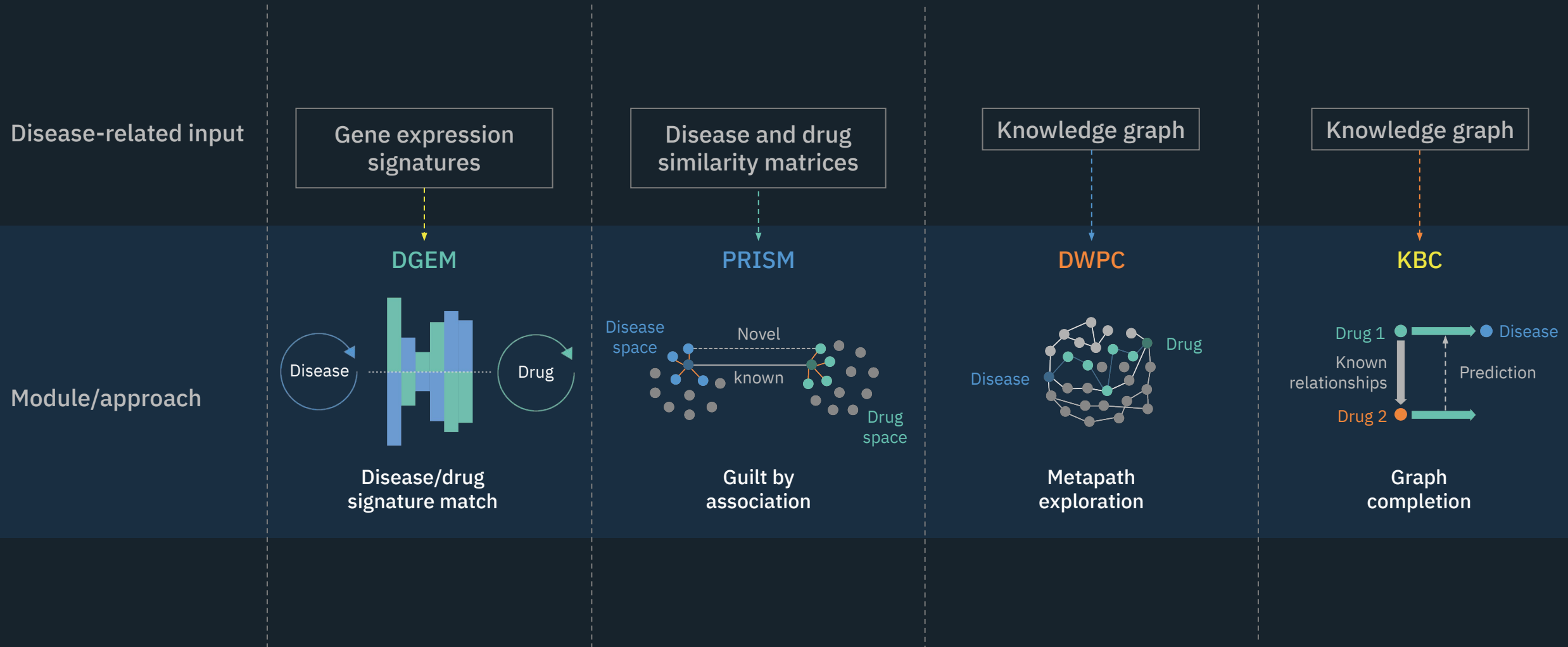


Millions of rare disease patients will have an approved treatment for the first time

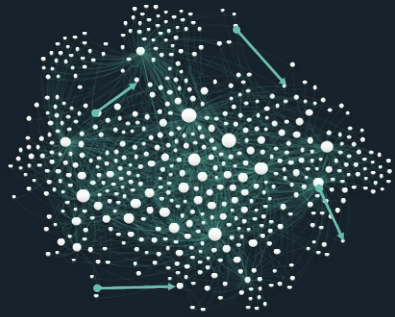
Healnet AI platform data discovery workflow



Rare diseases require multiple AI drug matching approaches



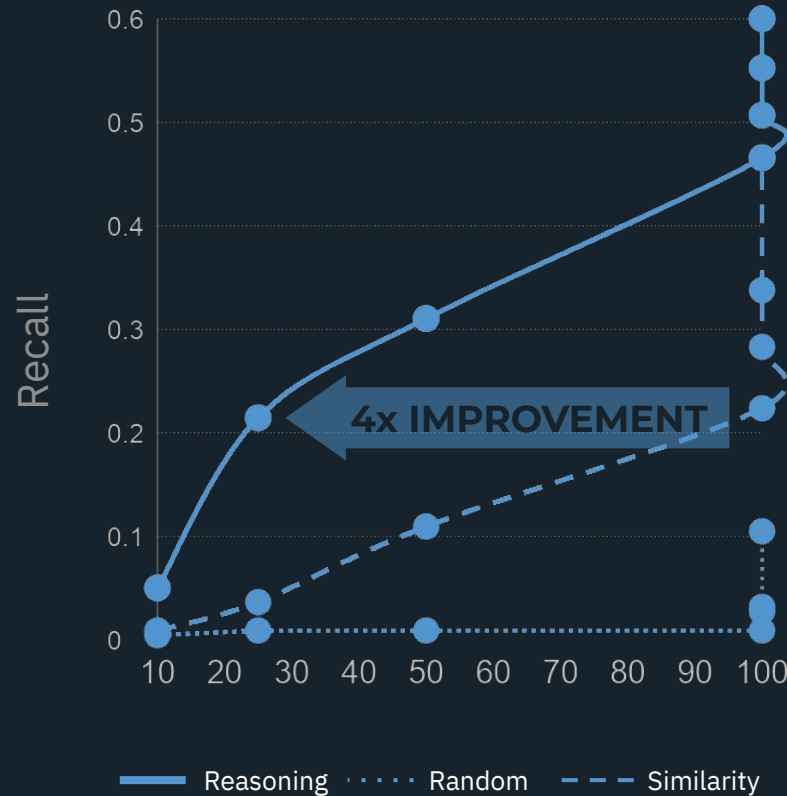
AI gives 4-fold improvement in prediction precision



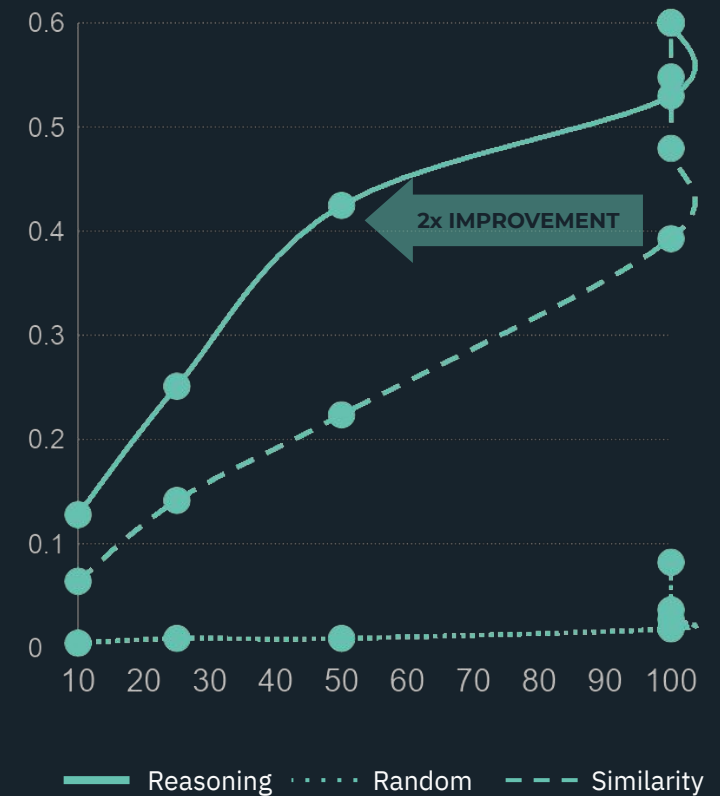
AI Reasoning achieves higher precision



Indication expansion

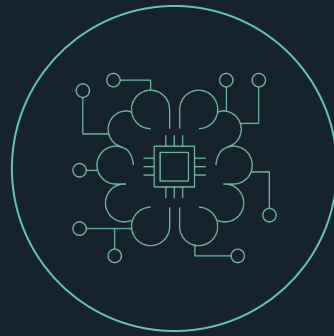


Drug repurposing



Hits@k (# top predictions)

healx



AI Case Study: Healnet for Fragile X treatment discovery

Fragile X Syndrome



Symptoms

- Intellectual disability
- ADHD, hyperactivity
- Anxiety, sensory issues
- Autistic behaviours
- Seizures (~25%)



**DNA repeat in
FMR1 gene**
(chromosome X)

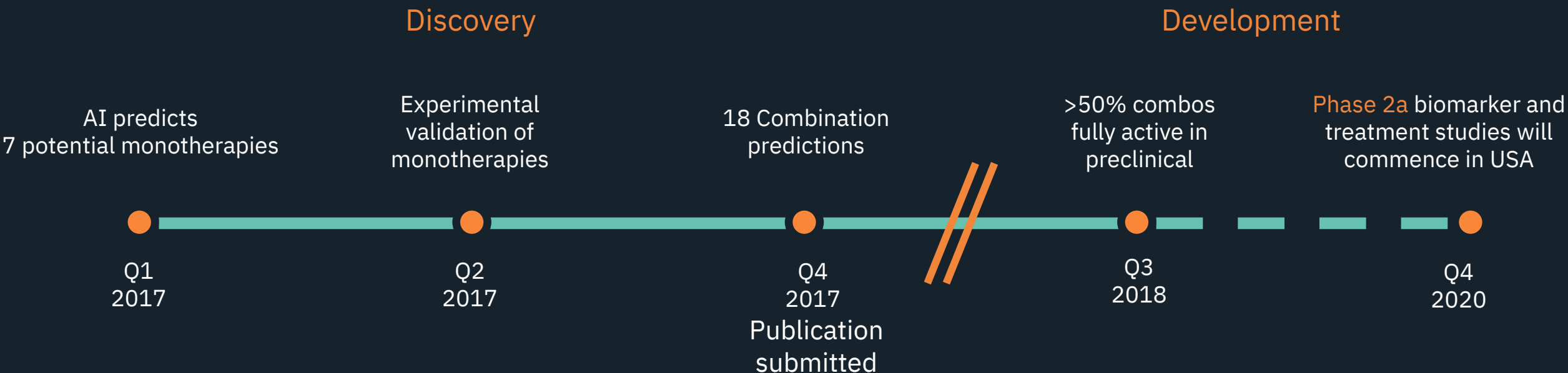


**Affects
1/6,000
people**



Image by: Peter Saxon

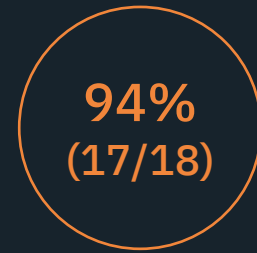
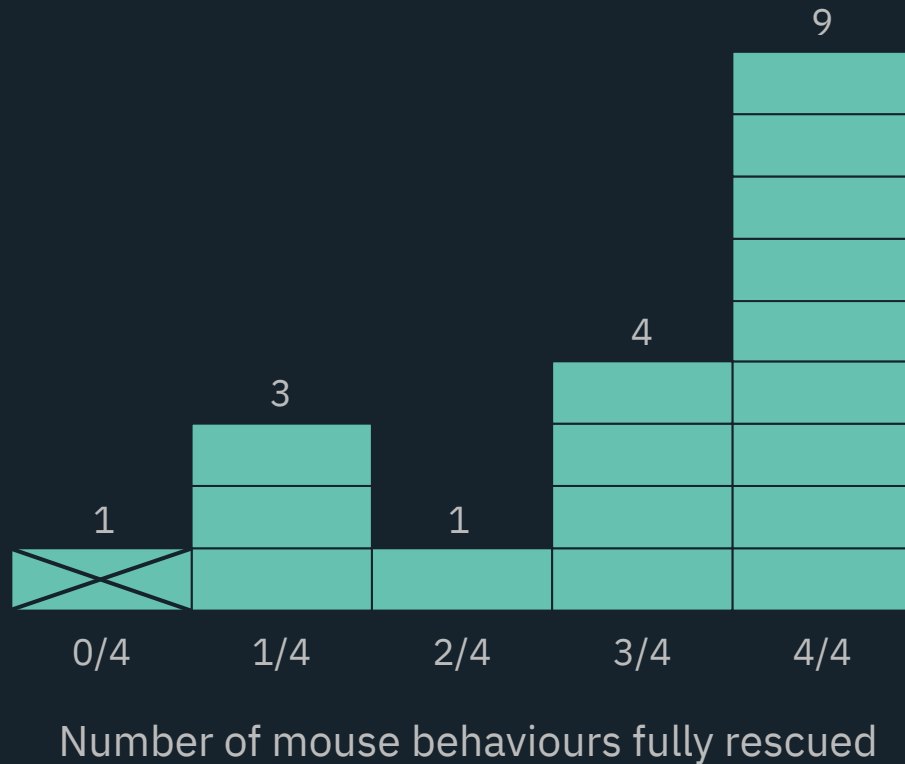
AI drug discovery and development: Fragile X roadmap



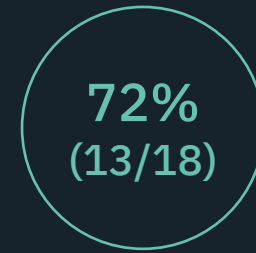
36 months from project start to be clinic ready

Preclinical testing combinations predictions in Fragile X

Combinations shown to fully rescue four key mouse behaviours in disease model



Full rescue of at least one behaviour

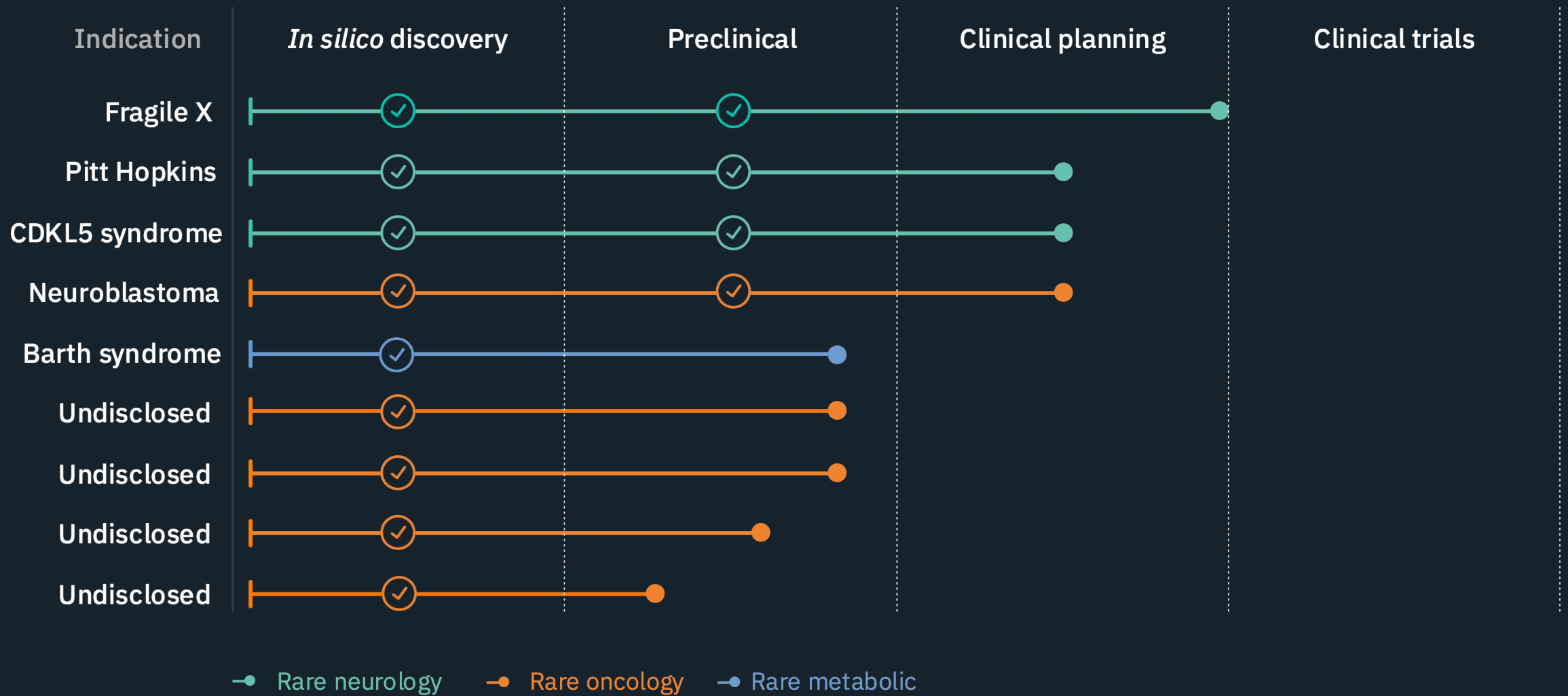


Full rescue of at least 3 behaviours



Fully rescued all 4 behaviours tested

What we have achieved so far



Creating rare disease treatments at scale

healx

AI-powered
Patient-inspired

healx



healx

healx

healx

healx

healx