



Rethinking rare

Bringing promising drugs to market
... quickly



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The problem: 10,000 rare diseases and only 5% have an approved drug treatment



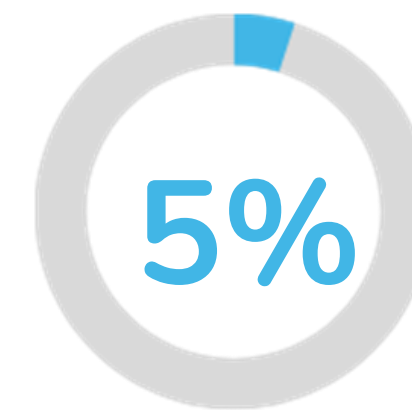
known rare diseases¹



people affected globally²



people have a rare disease³



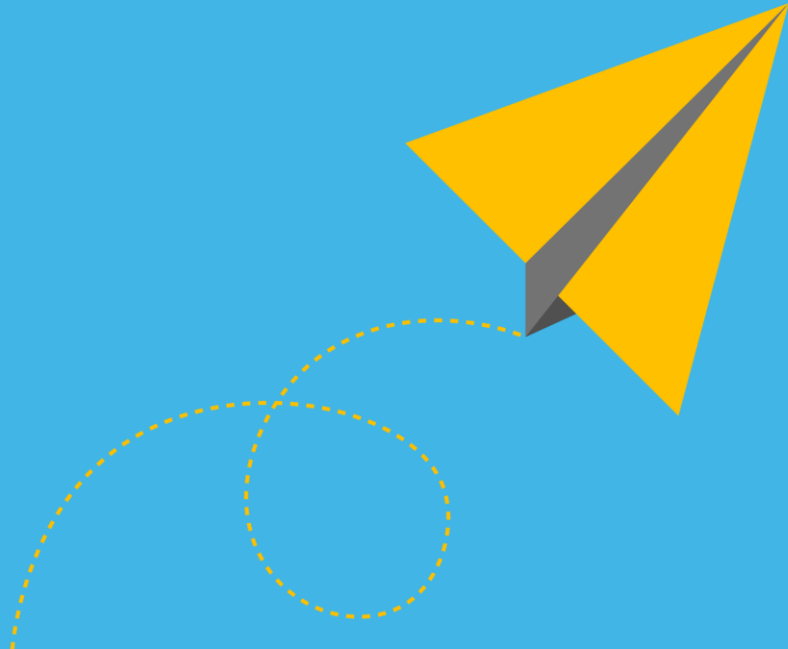
of conditions have FDA approved drug⁴

1. Haendel, M., et al (2020) How many rare diseases are there?, Nature reviews. Drug discovery. U.S. National Library of Medicine. Available at: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC7771654/> (Accessed: 4 January 2024).

2. Introducing the Rare Diseases Genomics and Precision Health Knowledge Base (no date) Centers for Disease Control and Prevention. Centers for Disease Control and Prevention. Available at: <https://blogs.cdc.gov/genomics/2019/04/04/introducing-the-rare-diseases/> (Accessed: 4 January 2024).

3. About GARD (no date) Genetic and Rare Diseases Information Center. U.S. Department of Health and Human Services. Available at: <https://rarediseases.info.nih.gov/about> (Accessed: 4 January 2024).

4. Willmer, G. (2022) The building blocks to make rare disease treatments more common, Horizon Magazine. Available at: <https://projects.research-and-innovation.ec.europa.eu/en/horizon-magazine/building-blocks-make-rare-disease-treatments-more-common> (Accessed: 4 January 2024).

A yellow paper airplane flying upwards and to the right, leaving a dotted yellow trail behind it.

... however, that 5%
is generating enormous,
and growing revenues

- Market size of \$119bn in 2022¹
- Current CAGR of 12.8%²
- Fastest growing pharma niche

1. Rare Disease Treatment Market Size: Share Report, 2023-2032 (no date) Global Market Insights Inc. Available at: <https://www.gminsights.com/industry-analysis/rare-disease-treatment-market> (Accessed: 4 January 2024).

2. (No date) *Rare Diseases Treatment Market Size, Share Report, 2030*. Available at: <https://www.grandviewresearch.com/industry-analysis/rare-diseases-treatment-market-report#:~:text=The%2520global%2520rare%2520disease%2520treatment,USD%2520335.84%2520billion%2520in%25202030> (Accessed: 11 January 2024).

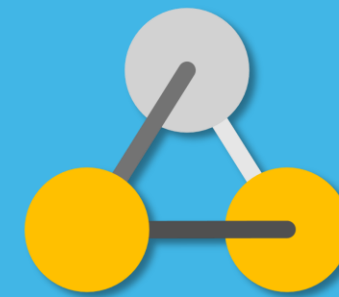
Existing business models aren't working for the other 95%



\$2.3bn average cost of developing a new drug



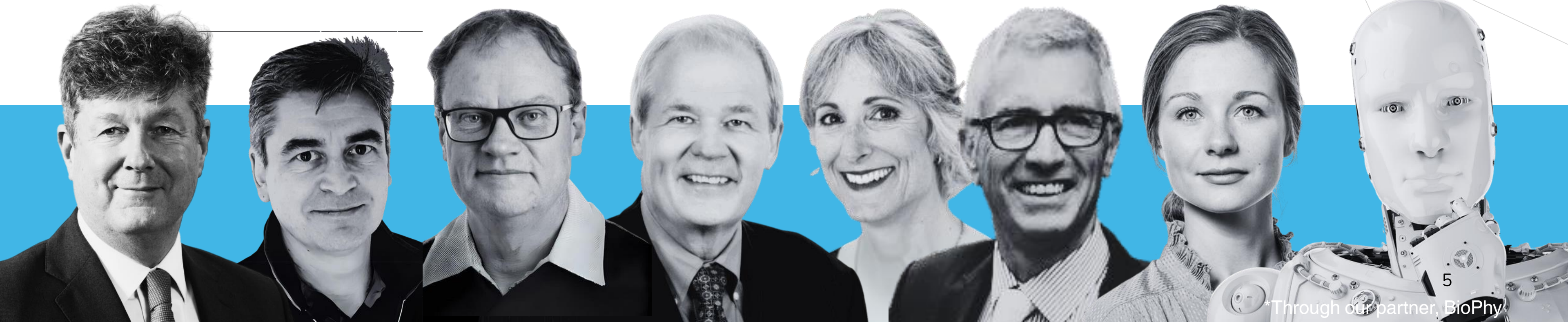
And many promising drugs are stuck



So new business models are needed for rare disease drug development

Meet Ambrose Healthcare

- An award-winning pharmaceutical start-up with a highly-experienced team.
- We are rethinking rare by combining deep human expertise with resourceful approaches, including AI*.



We are building a broad, late-stage, lower-risk portfolio



Drug discovery



Phase 1



Phase 2a



Phase 2b



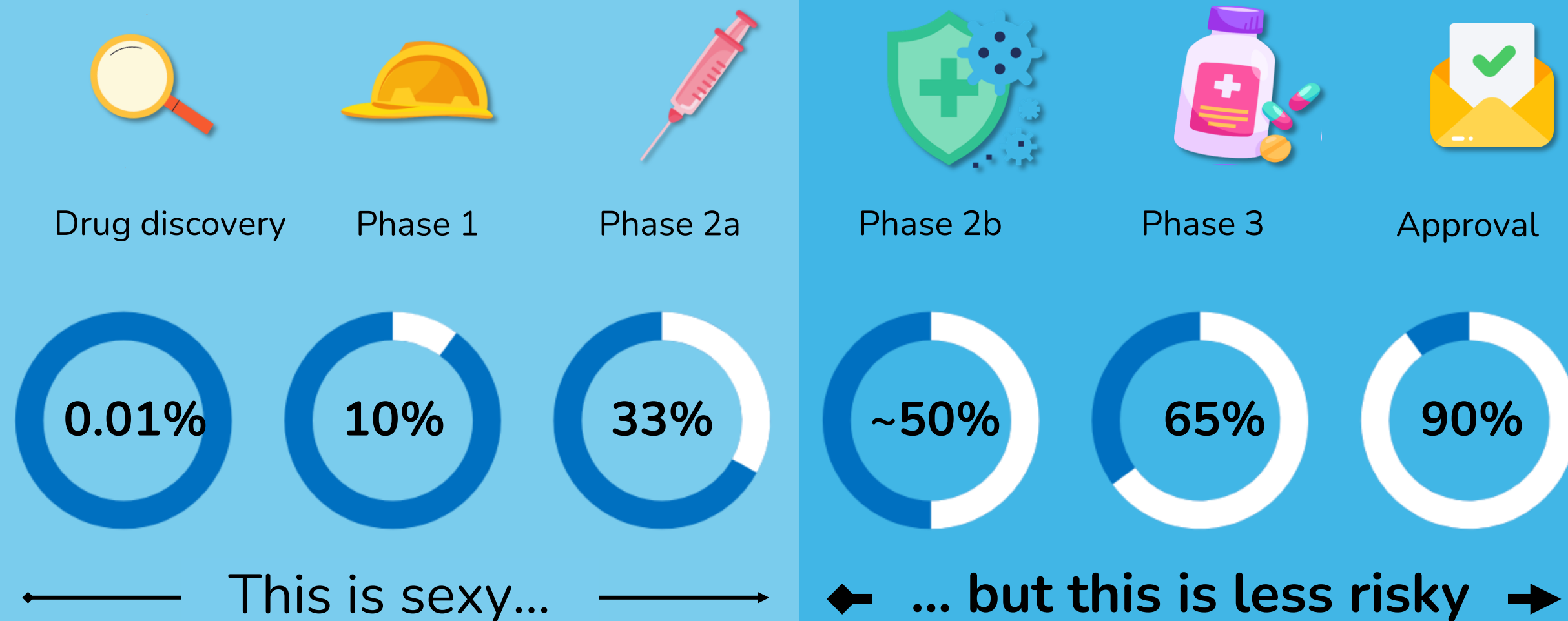
Phase 3



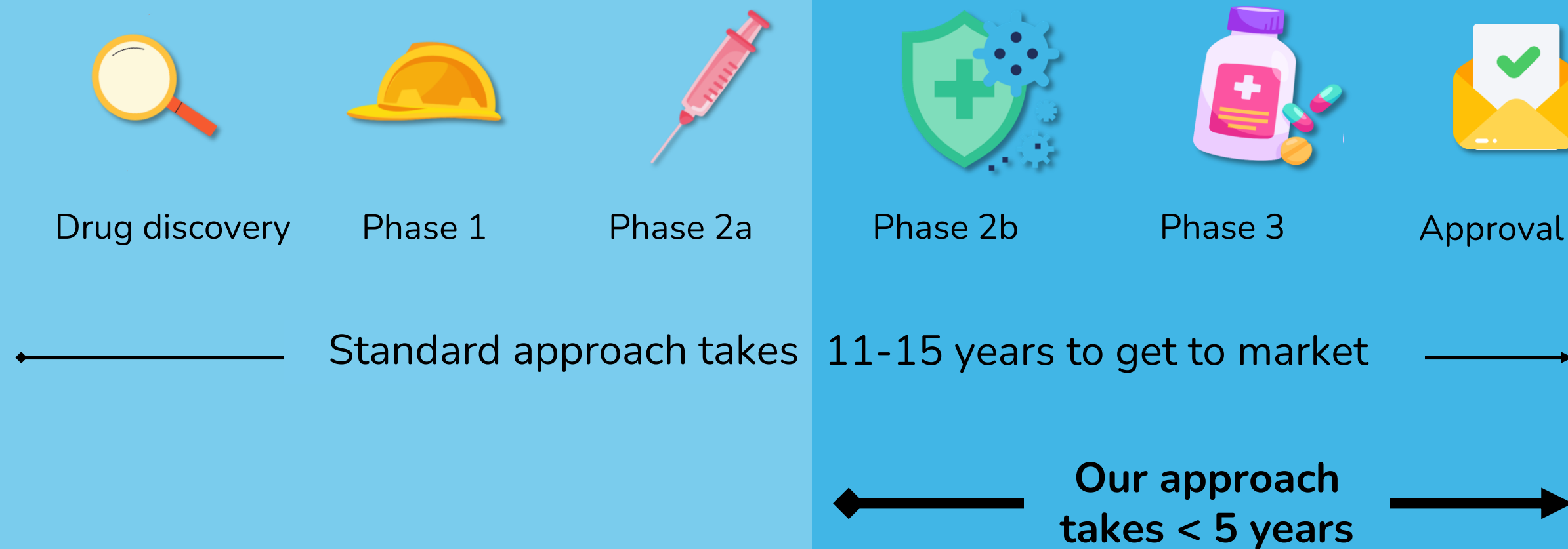
Approval

- **With our RxEvaluator**
- **Identify & acquire drugs**
- **Manage trials & contract manufacturing**
- **Navigate accelerated regulatory pathways**
- **Supply across the US, Europe & Middle East**

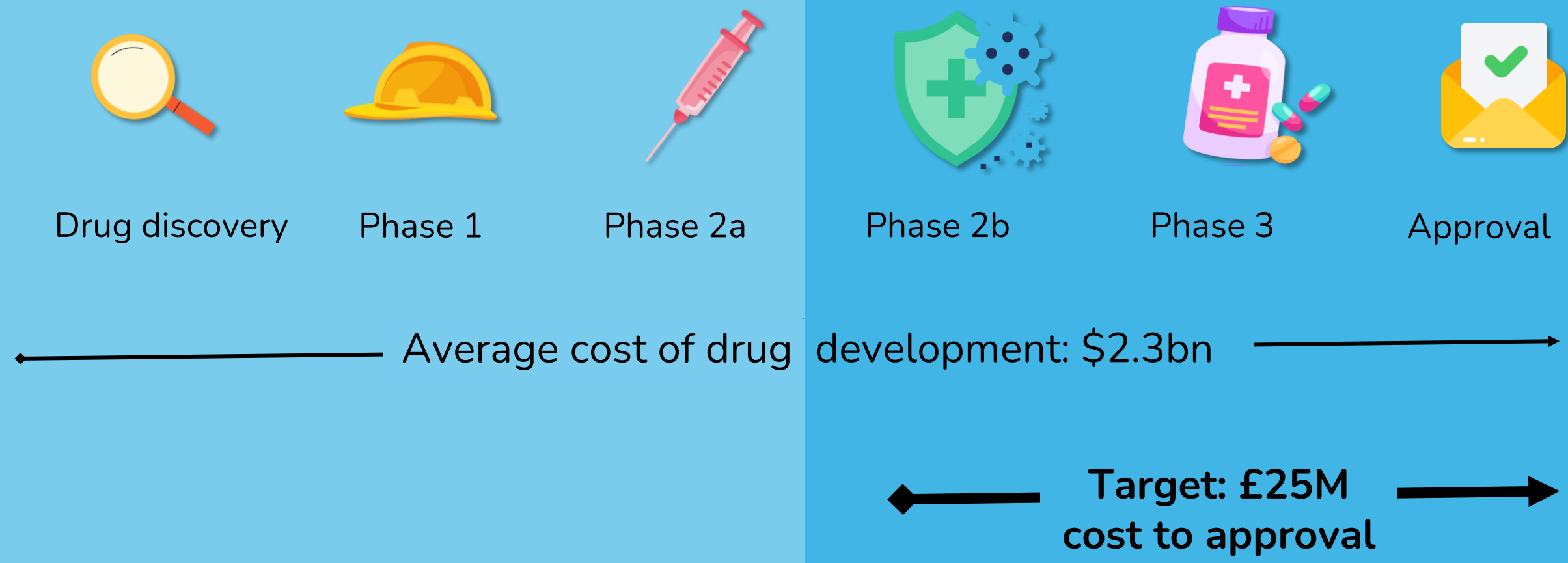
... targeting higher success rates in drug development



... with shorter times to revenues and market



... at a lower cost



Hitting milestones early

2023



Business founded &
team established



Acquired rights to 2
assets, using share
equity

Ahead of schedule

2024



2023 Won National
Start-up Award 2023

H1 CLOSE OF
£1.5M

Growing pipeline ahead of plan

Rights over 2 products; estimated combined NPV of £110m

Val401

- **Indication:**
 - Patients with Stage 3-4 Pancreatic Cancer
 - Treatment for palliative care/quality of life extension
- **£70m /\$87m current NPV estimate**
- **Peak revenues: £170m**
- **Approval ~ 4 year plan**

Product 2

- **Indication:**
 - Undisclosed
- **£40m /\$50m current NPV estimate**
- **Peak revenues: £150m**
- **Approval ~ 5 year plan**

Targeting:

£320m

Peak revenues pa

Targeting a
5 year exit at a
~£800m / \$1bn
valuation

- **Run dual IPO/trade sale process**
- **Mid-cap pharma consolidators seeking products for sales pipelines**
- **Growing number of rare disease company acquisitions**

- **Ipsen acquires Albireo for \$952m**
 - 2 recent approvals
- **Chiesi Farmaceutici acquires Amryt Pharma for \$1.25bn**
 - 3 recent approvals
- **Amgen acquires Horizon Therapeutics for \$28.7bn**
 - Larger portfolio, 3 recent approvals

Why invest now?

- **Building value ahead of plan**
- **£1.5m upscaled round:**
 - £455k closed
 - Institutional investor for £500k
 - Final £500k available, to match
- **Last opportunity for ground floor investment**
 - EIS tax approval
- **5-year exit**
 - 30x ROI potential



Seeking investors

... to close round 1

... discussions with VCs and institutions for future rounds

In Summary

- **Award-winning rare disease pharma start-up**
 - Massive void in rare diseases, experienced team and international network
 - Building a late-stage drug portfolio for patient supply and commercialization, selected using our Rx Evaluator, paid for with share equity
- **Ahead of plan**
 - Rights to 2 products acquired, estimated combined NPV of £110m
 - £1.5m upscaled raise, at £4m pre-money
- **Last £500k available for ground-floor investors**
- **Clear route to 5-year exit ... x30 ROI potential**

“New treatments for rare diseases are urgently needed – and I am excited to see that Ambrose is taking a different approach to the provision of new rare disease treatments. It’s exactly what is needed to support patients.”

NHS Wales

Toby Wilson Waterworth, CEO

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