

WinQure

MEDICINE

WHO ARE WE?

We are a **biotech** company headquartered in Copenhagen, with scientific operations in Oxford and Istanbul.



Dr Stephanie Manton
CHIEF EXECUTIVE OFFICER & CO-FOUNDER



Dr Henrik Ørum
CHIEF SCIENTIFIC OFFICER & CO-FOUNDER



Dr Andrew Roddam
CHIEF DEVELOPMENT OFFICER



Prof Udo Oppermann
HEAD OF BIOLOGY & CO-FOUNDER



WHAT DO WE DO?

We develop precision **ASO** therapies for ultra-rare genetic diseases that have no treatment.

HOW DO WE DO IT?

We use our **RAREplatform** to:

- give patients a voice in how therapies are developed
- remove the obstacles that stall rare disease programs

What is precision ASO therapy?

PRECISION ASO THERAPY

Precision

targets the root cause of disease

ASO

antisense oligonucleotide

HOW IT WORKS

EXAMPLE

THE PATIENT

A patient has HSAN1A, caused by the C133W mutation in the *SPTLC1* gene.

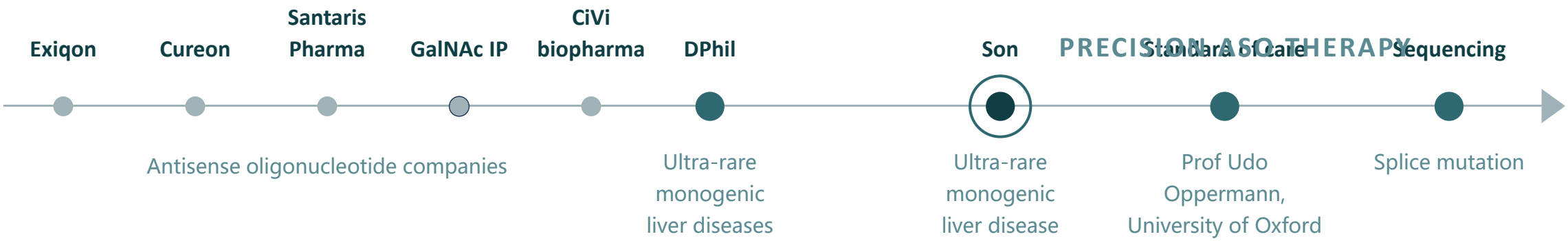
THE ASO

The ASO binds only to that mutated site and silences the faulty copy.

THE RESULT

That is a precision ASO therapy for that patient.

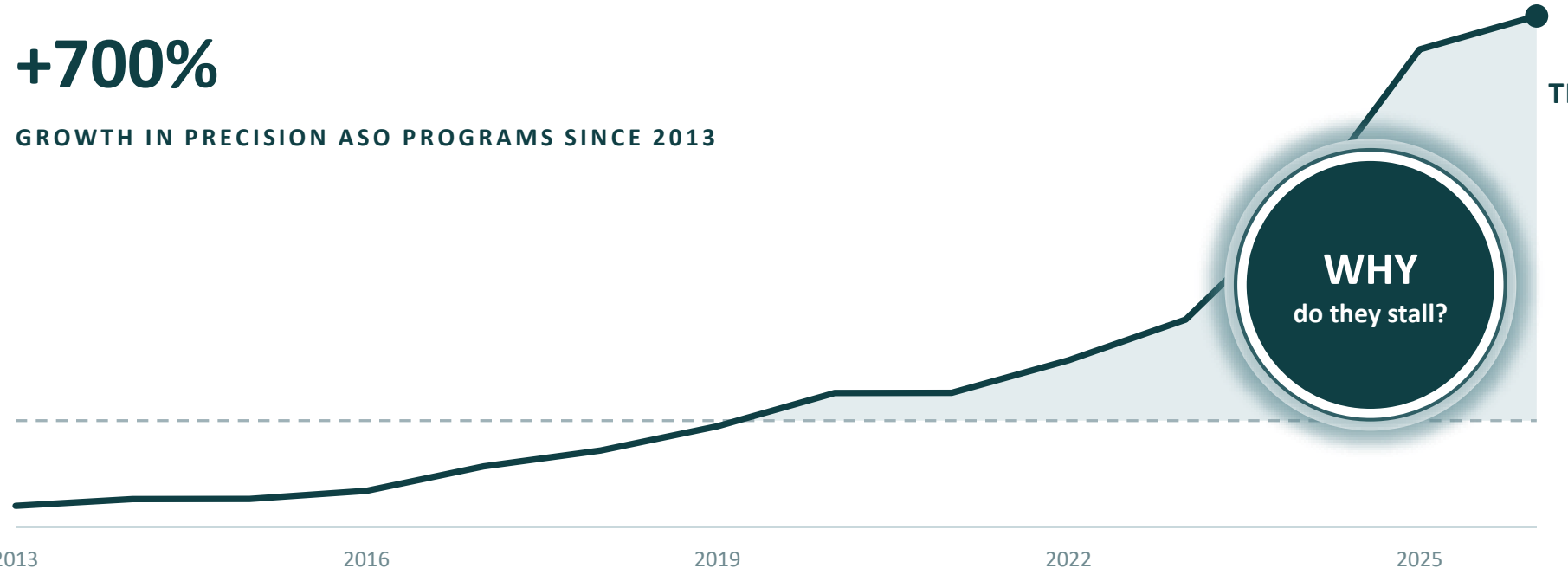
Taking a step back…



The first challenge – development platform

+700%

GROWTH IN PRECISION ASO PROGRAMS SINCE 2013

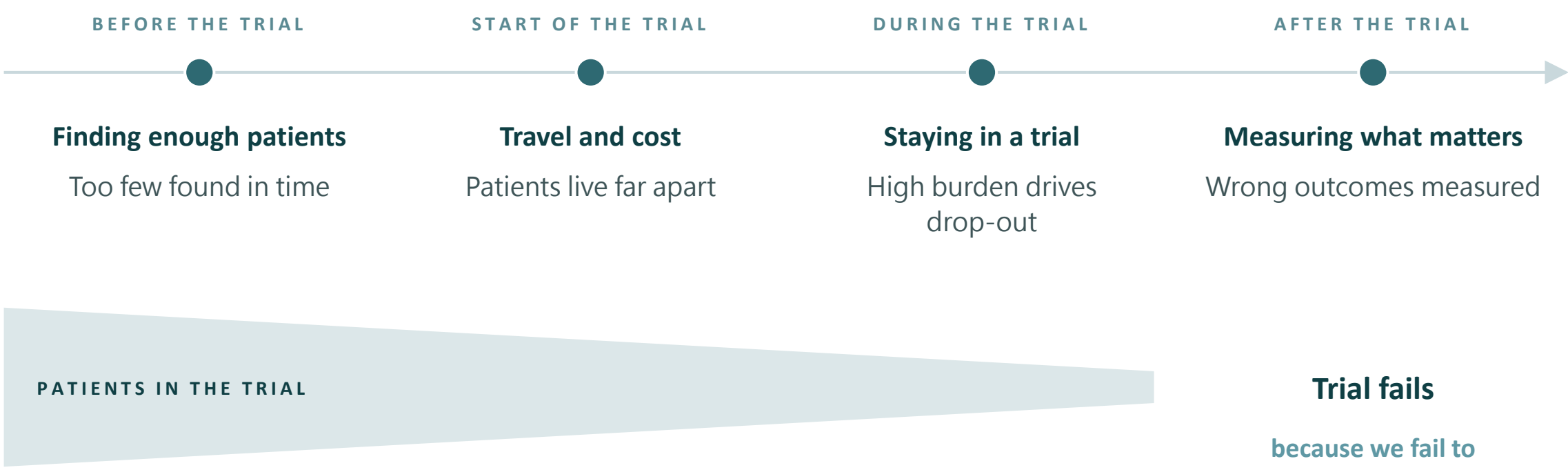


Their market is too small for industry to to take these programs forward → **Academics have to go it alone** →

Lack infrastructure
Lack capital
Lack expertise

* WinQure market tracker, September 2026.

The second challenge – clinical trials



Mishra S, Venkatesh MP. Orphanet J Rare Dis 2024;19:285.

Two challenges. What is the solution?

1 **Academic programs stall in discovery**

→ **Give academic programs a development development platform**

2 Trials are not designed for success

→ Involve patients in trial planning so it it works for them

SOLUTION

A patient-driven drug development platform that that brings stalled academic academic therapies to patients

The RAREplatform

STEP 1 OF 4 | STEP 2 OF 4 | STEP 3 OF 4 | STEP 4 OF 4

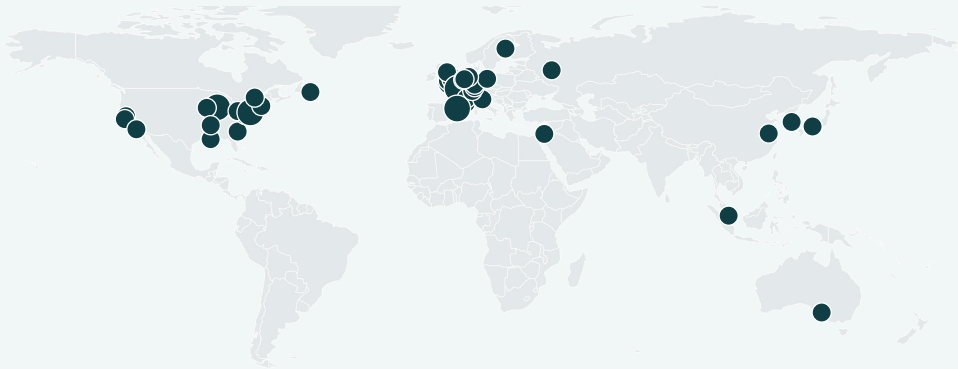
Stalled program data → **RAREkey** → **RAREscore** → **WinQure program**

Program data inputted in any format

- Papers
- Patents
- Grant applications
- Lab data
- Slide decks

→ **Converted to one structured profile per program**

Labs in RAREplatform today



The RAREplatform

STEP 1 OF 4 | STEP 2 OF 4 | STEP 3 OF 4 | STEP 4 OF 4

Stalled program data → **RAREkey** → RAREscore → WinQure program

A different kind of patient registry

Upload genetic report

Age

Location

Contact details

Register

Free, easy and secure

winqure.com/rarekey

RAREkey

RAREkey directly connects developers to patients:

- **Who will participate in the clinical trial**
- **Who are in the treatment window**
- **Who we can reach today**
- **And informs us where to place the trial site**

The RAREplatform

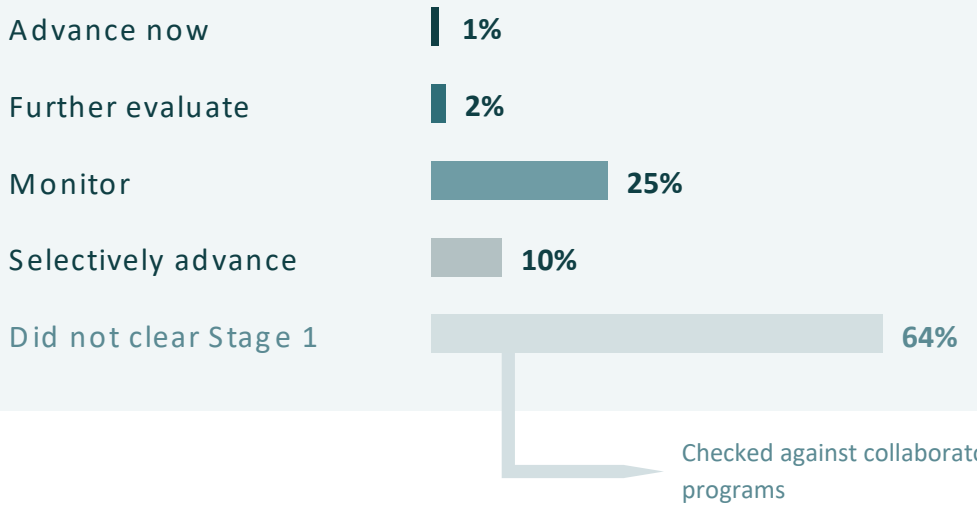
STEP 1 OF 4 | STEP 2 OF 4 | **STEP 3 OF 4** | STEP 4 OF 4

Stalled program data → RAREkey → **RAREscore** → WinQure program

What does RAREscore test?

- **Is the ASO design ready for clinic?**

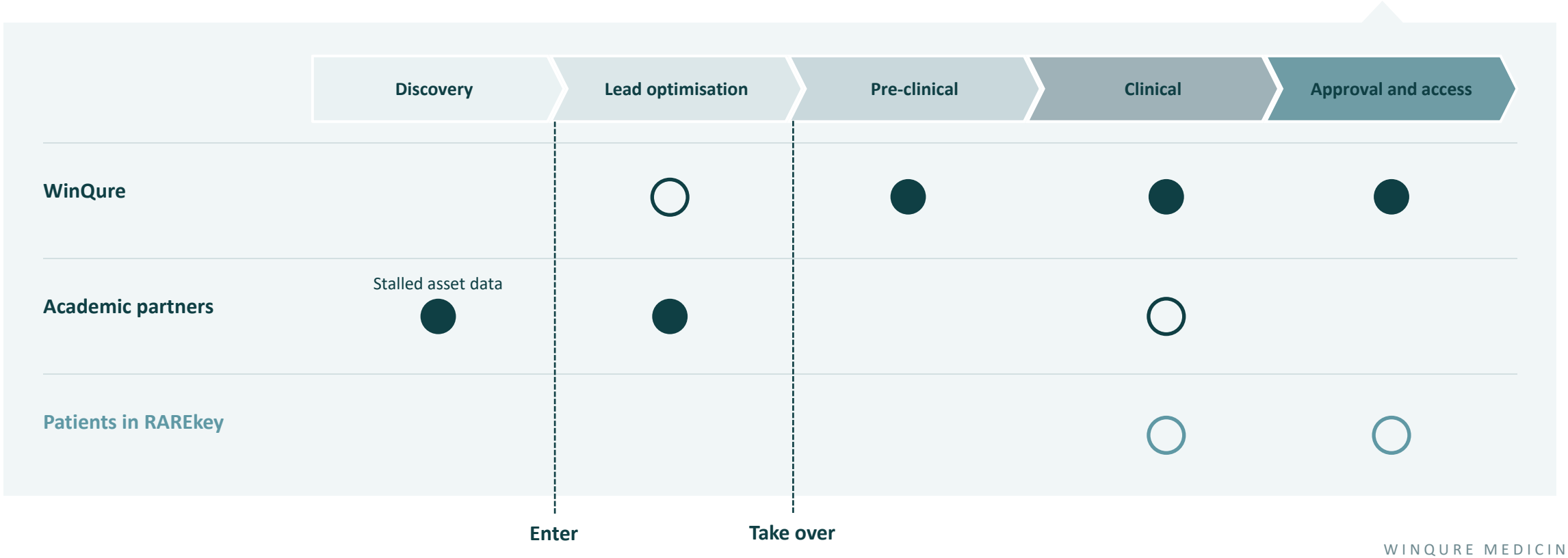
Where stalled programs stand



The RAREplatform

STEP 1 OF 4 | STEP 2 OF 4 | STEP 3 OF 4 | STEP 4 OF 4

Stalled program data → **RAREkey** → **RAREscore** → **WinQure program**



WinQure programs to date

Sensory neuropathy 1A

81/100

RAREscore

SPTLC1 C133W Our first in-licensed program

OUTPUT

Can we make an ASO?	96
Can we deliver it?	94
High patient impact?	55
Limited treatment options?	80

Alagille syndrome

75/100

RAREscore

JAG1 intron 3 Our in-house program

OUTPUT

Can we make an ASO?	93
Can we deliver it?	94
High patient impact?	69
Limited treatment options?	20

WinQure is redefining drug development

HOW PHARMA DOES IT TODAY

Find a disease with a commercial market → Develop a therapy based on expected need and demand → Find patients to treat

HOW WE DO IT

Find patients who need treatment → Develop a therapy based on what they need → Treat the patients waiting

We start with the patient, not the market.

WinQure MEDICINE

Register in RAREkey

winqure.com/rarekey