

NEWS | 08 November 2024

## World-first stem-cell treatment restores vision in people

The treatment, given to four people with damaged corneas, seems safe but needs to be tested in larger trials.

By [Smriti Mallapaty](#)



# Stem Cells Restore Vision in World First

Nov 11, 2024 | Blog, Eye Conditions, News, Stem Cell News, Stem Cell Therapies, Uses for Cord Blood

Jun 23, 2023

## Vertex Presents Positive VX-880 Results From Ongoing Phase 1/2 Study in Type 1 Diabetes at the American Diabetes Association 83rd Scientific Sessions

- All six patients treated with VX-880 engrafted islet cells, produced endogenous insulin (C-peptide) and had improved glycemic control while reducing or eliminating insulin use

- The two patients with at least one year of follow-up met the criteria for the primary endpoint of elimination of severe hypoglycemic events (SHEs) and HbA1c <7.0 -

- Both of these patients also achieved insulin independence with HbA1c values of 5.3% and 6.0% -

- VX-880 generally well tolerated in all patients dosed to-date -



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### Press Release

**Neurona Therapeutics Presents Positive Clinical Update from NRTX-1001 Cell Therapy Trial in Adults with Drug-resistant Focal Epilepsy at American Academy of Neurology (AAN) 2024 Annual Meeting**

# Human vision restored by stem cell replacement in regenerative medicine breakthrough

## BlueRock Therapeutics' investigational cell therapy bemandaneprocel for Parkinson's disease shows positive data at 24-months

- Bemandaneprocel is the most clinically advanced investigational cell therapy in the U.S. for treating individuals living with Parkinson's disease
- At 24 months, data from the Phase 1 exPDite trial continue to show a favorable safety profile in all 12 participants in the trial's high and low dose cohorts

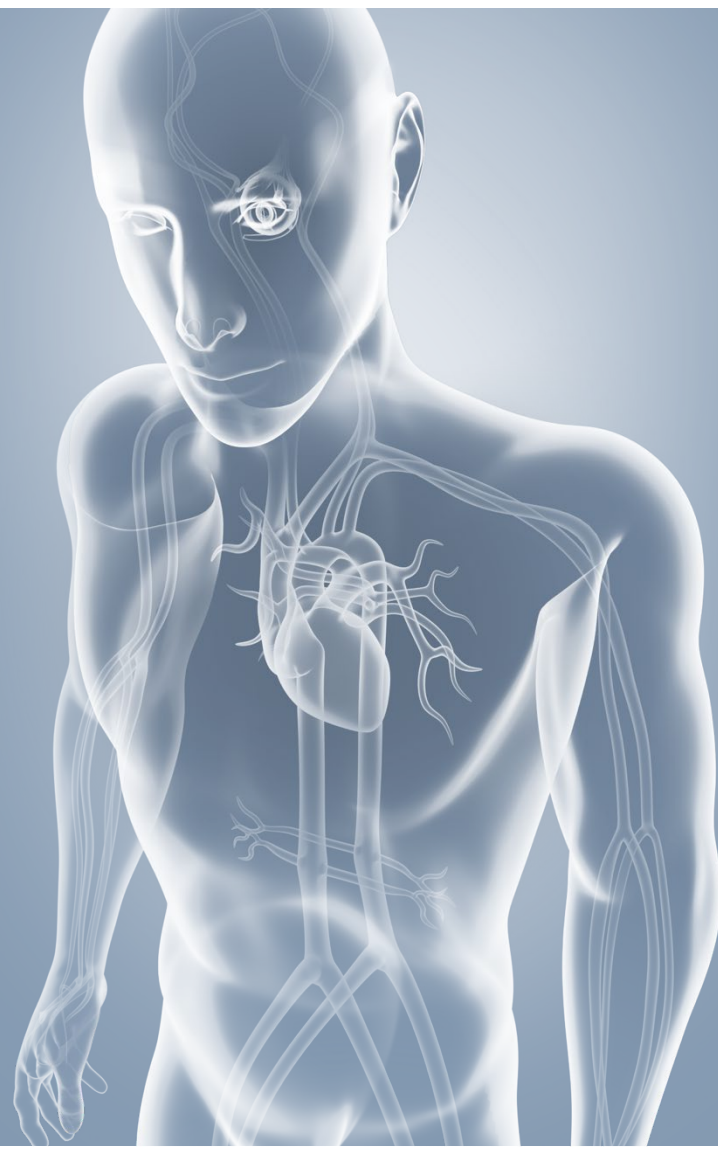
This material is intended for global media only.  
For journalistic assessment and preparation before publication.



FOR IMMEDIATE RELEASE

**Heartseed Announces a Positive Recommendation from the Safety Monitoring Committee for Continuing High-Dose Arm in its Phase 1/2 Clinical Trial using HS-001, an Investigational Stem Cell-Derived Therapy for the Treatment of Advanced Heart Failure**





# ALDER

THERAPEUTICS

## Mutation agnostic regenerative cell therapy for Retinitis Pigmentosa

DUTCH LIFE SCIENCE DAY 2024

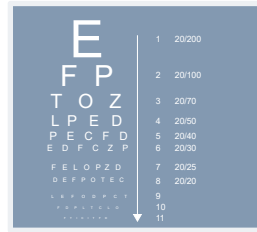
Stijn Heessen, co-founder & COO

# Retinitis Pigmentosa - an inherited retinal disorder with progressive vision impairment we aim to cure

## VISUAL DEFECTS IN RP PATIENTS:



Night blindness



Loss of visual acuity

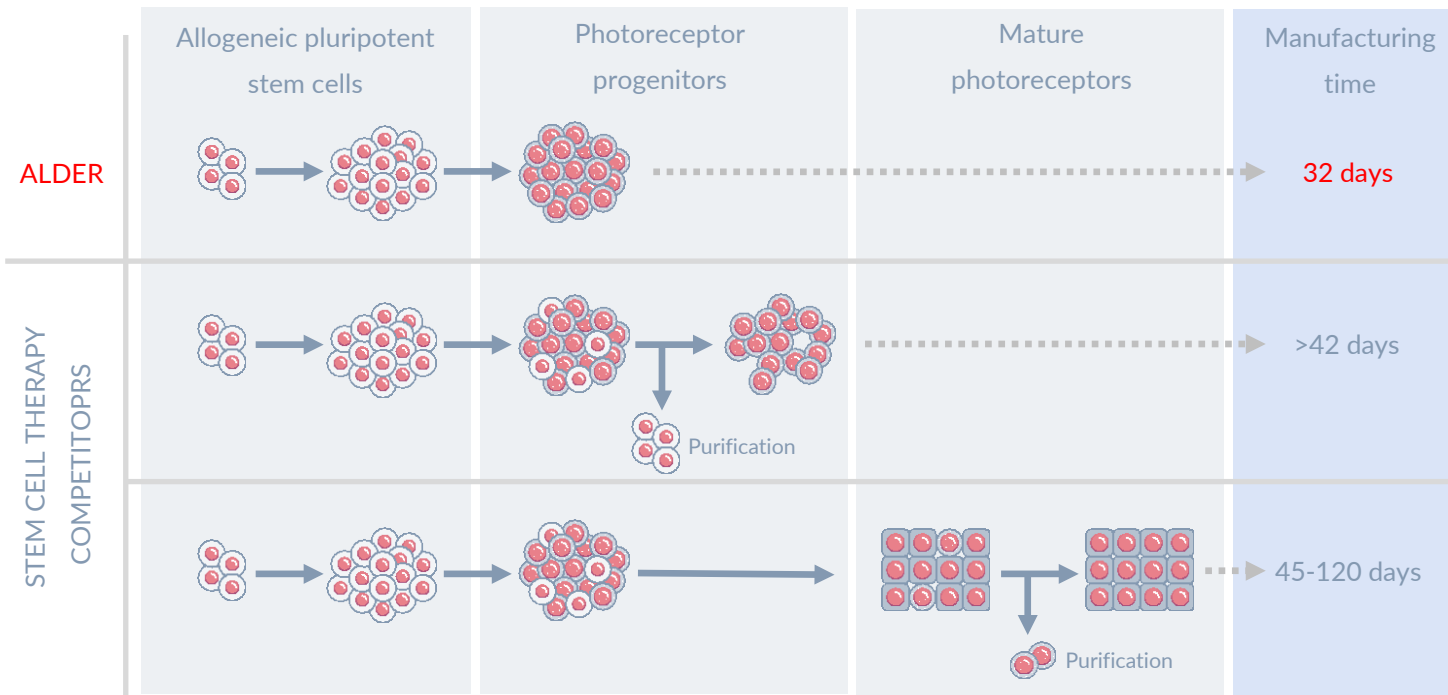


Progressive loss of visual field - Tunnel Vision

## KEY NUMBERS:

- Mutations in +100 genes
- ~1.5 million patients
- 98% w/o treatment options

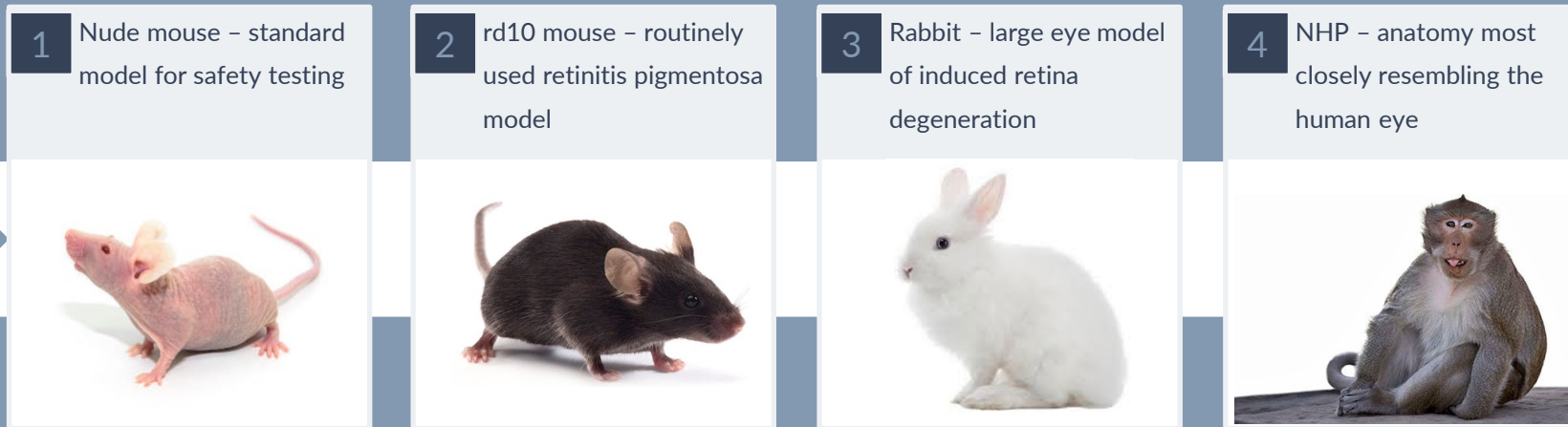
# Mutation agnostic cell therapy: from allogeneic stem cells to functional photoreceptor progenitors in a simple, robust and cheap 32-day process



## THE ALDER PROCESS

- Shortest, simplest and cheapest process
- Leveraging +10 years of development at DUKE-NUS
- Patented and exclusively licensed
- Estimated manufacturing cost at commercial scale <€1.000/dose

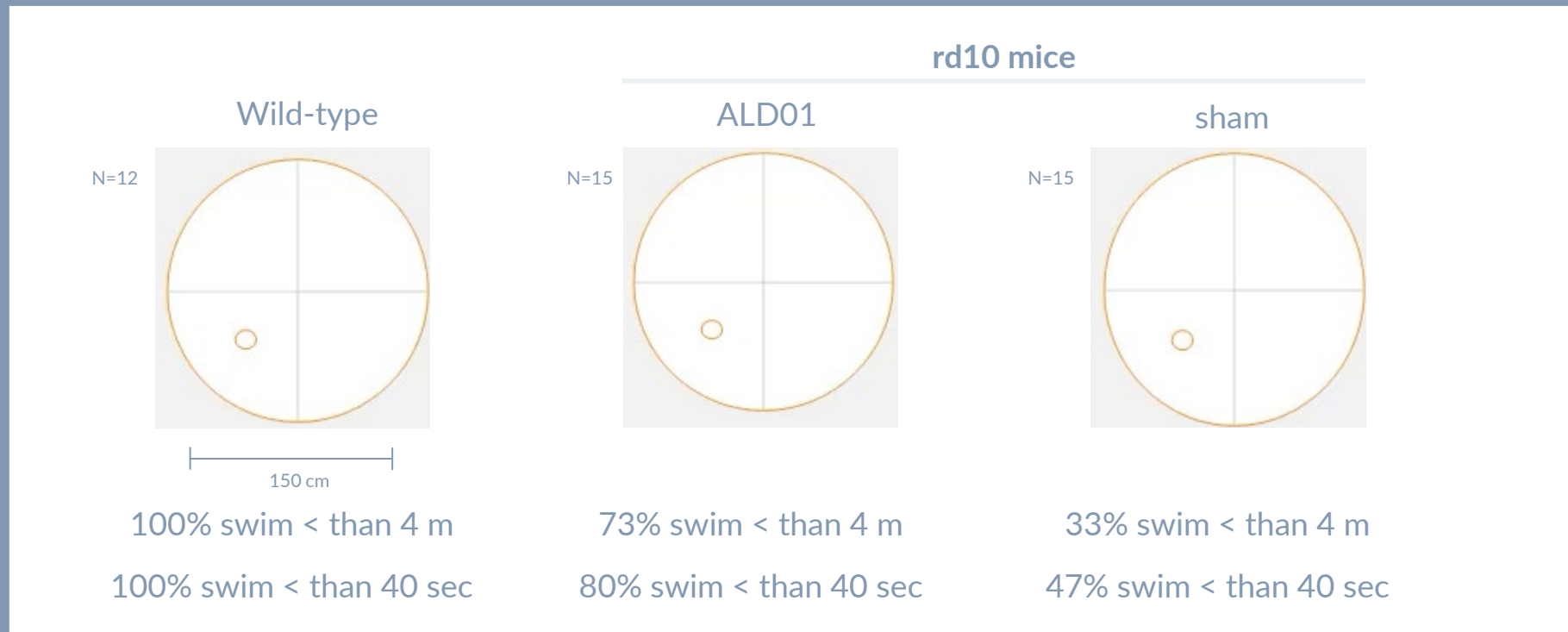
# 32-day old photoreceptor progenitors administered by subretinal injection are safe and provide long-term vision improvement in small and large animal models



## PRECLINICAL RESULTS\*:

- ✓ Safety (cells & administration)
- ✓ Engraftment & Maturation
- ✓ Functional improvement

# Long-term (>12 weeks) vision improvement in a genetic mouse retinitis pigmentosa model\*



# We are a diverse, agile and global team of experienced company builders, fundraisers and cell therapy development experts

## ALDER CORE TEAM



[CEO – Kristian Tryggvason](#)



[COO – Stijn Heessen](#)



[Hwee Goon Tay](#)



[Lynn Yap](#)



[Joanne Mountford](#)



[Nick Timmins](#)

Consulting CTO



[Amanda Mack](#)



## PRECLINICAL



[Michaela Sharpe](#)



## MARKET ACCESS



[David Aldersson](#)



## REGULATORY



[Sabine Rühle](#)



[Harriet Edwards](#)



## CLINICAL DEVELOPMENT & SCIENTIFIC ADVISORY BOARD



[Sonya Montgomery](#)

Interim CMO



[Dominik Fischer](#)



[Ander Kvanta](#)



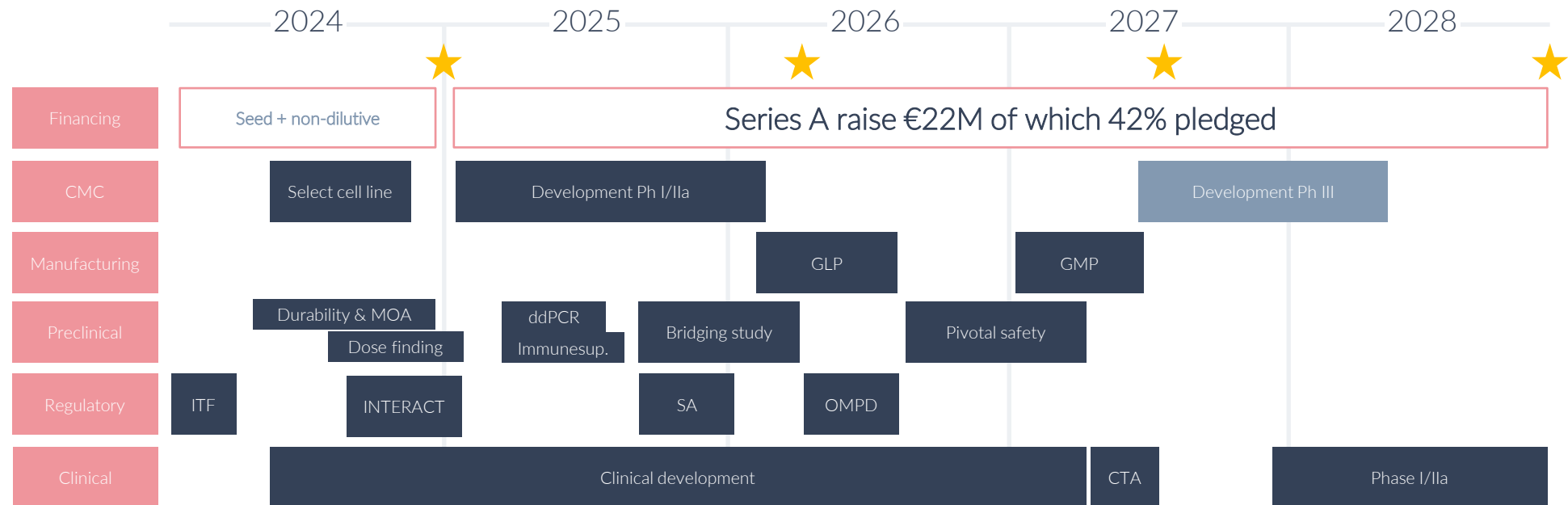
[Geert Boink](#)



# We have an actionable and achievable plan towards clinical proof-of-concept in Retinitis Pigmentosa

Total capital raised 2022-2024: €4.8M

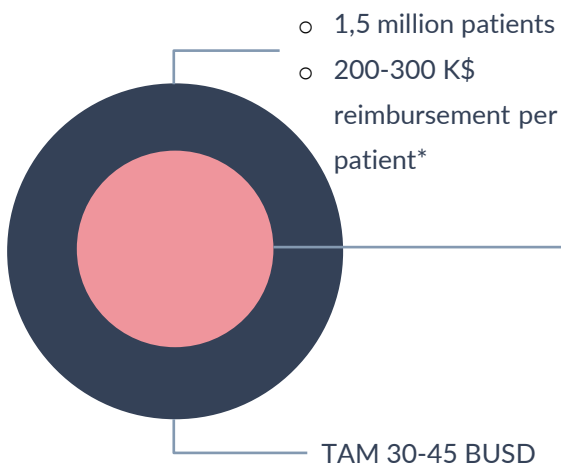
- €3.5M from Swedish VC investors Flerie and Linc
- €1.3M in non-dilutive funding





# Treating RP with mutation-agnostic cell therapy represents a multi-billion USD opportunity

## TOTAL ADDRESSABLE MARKET



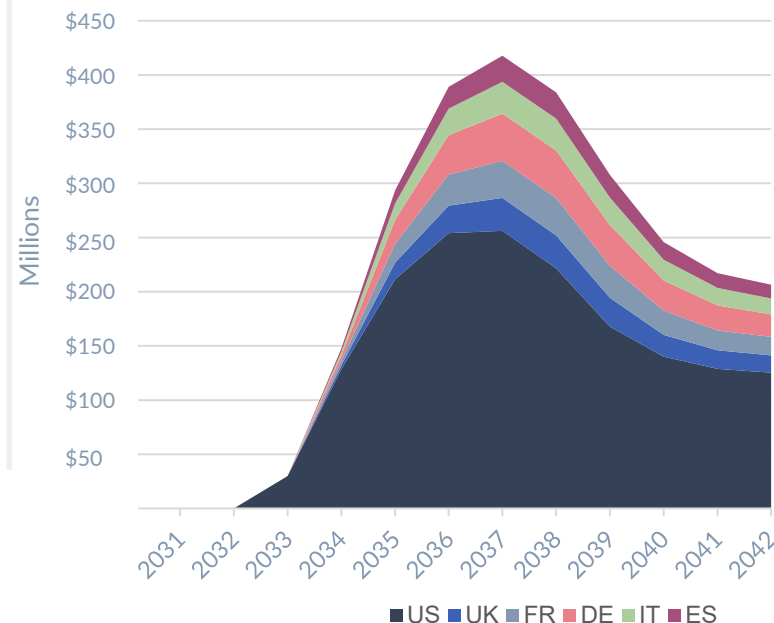
## TOTAL SERVICEABLE MARKET



- Exclude 20 % of the the least and most affected patients based on TPP\* and Early Value Strategy\*
- Include only US, EU, Japan, China (~25% of the patients)
- Reimbursement 200-300 K\$\*

**>6-9 BUSD**

## ESTIMATED REVENUE CASE US+EU5\* (DISCOUNTED BASE CASE)





## SHAPING THE FUTURE OF REGENERATIVE MEDICINE

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