

SLC1A4 Gene Therapy Programme

One discovery. **Countless possibilities.**

Mission & Model

Rare Alliance Foundation accelerates treatments for rare genetic conditions by funding shared scientific infrastructure and milestone-based programmes. Instead of building each disease programme from scratch, we invest in reusable platforms — models, datasets, screening tools, and translational pathways — that enable faster progress across multiple disorders in parallel.

Impact: Building Infrastructure That Scales

This programme is designed to deliver impact twice:

- 1 accelerating a gene therapy path for SLC1A4, and
- 2 building reusable infrastructure that reduces time, cost, and risk for additional rare genetic disorders. One programme strengthens many future programmes.

Governance & Reporting

Rare Alliance Foundation provides milestone-based oversight with transparent use of funds and structured reporting. Founding partners receive defined deliverables, timelines, and quarterly progress updates.

Flagship Programme: SLC1A4

Our flagship programme focuses on SLC1A4 disorder, a severe neurological condition affecting development and brain function. The programme is structured to deliver a credible gene therapy path while also establishing a blueprint that can be reused across future rare-disease programmes affecting the central nervous system.

Programme Framework (Two Parallel Workstreams)

1 AAV Gene Therapy Development + Toxicology (Human Readiness) Dr Wendy Chung

The programme progresses into AAV gene therapy development and the preclinical packages required for human readiness, including toxicology planning aligned with regulatory expectations.

PROGRAMME LEAD:

Dr Wendy Chung *Current Chair of Paediatrics at Boston Children's Hospital*

FUNDING REQUIREMENT:

\$2,000,000

2 iPSC Human Disease Modelling & Validation

Parallel Workstream

In parallel, we will develop iPSC-based human disease models to validate disease biology in human cellular systems, strengthen translation, and support treatment optimisation and screening.

FUNDING REQUIREMENT:

\$1,500,000

Milestone Funding Summary

- **\$2.0m**
AAV Development + Toxicology / Human Readiness
Boston Children's Hospital
- **\$1.5m**
iPSC Human Model Research
Parallel Workstream


TOTAL PROGRAMME MILESTONES:

\$3.5m

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