

## **Press release**

Crucible Therapeutics and University of Sheffield secure £2.3 million Innovate UK Biomedical Catalyst award to develop breakthrough siRNA therapies that address underlying causes of ALS

**Sheffield, UK, 10 June 2025.** Crucible Therapeutics, a biotechnology company developing novel gene therapies for neurodegenerative diseases, is pleased to announce that it has won a substantial £2.3M award from Innovate UK's Biomedical Catalyst program. In partnership with the Sheffield Institute for Translational Neuroscience (SITraN) at the University of Sheffield, the funding will support the advancement of Crucible's differentiated siRNA program aimed at treating amyotrophic lateral sclerosis (ALS) and other devastating neurodegenerative conditions.

The Innovate UK grant will enable Crucible to scale up manufacturing and advance its lead clinical candidate into pivotal non-clinical safety studies – an essential step toward initiating first-in-human clinical trials for ALS.

Crucible's unique therapeutic approach targets rogue RNA molecules and toxic proteins that contribute to neurodegeneration, aiming to protect motor neurons from progressive damage. This innovative strategy holds promise as a first-in-class, disease-modifying treatment for patients affected by ALS.

ALS is a progressive, fatal neurodegenerative disorder characterized by muscle weakness, paralysis, and ultimately respiratory failure. Toxic protein accumulation, seen in the vast majority of ALS cases, directly damages motor neurons, disrupting nerve signaling and leading to severe muscle atrophy and loss of function that defines the disease.

Jonathan Foley, Executive Director & CDO of Crucible Therapeutics said: "We are thrilled to receive Innovate UK funding as part of the Biomedical Catalyst, which will accelerate our research into a potential new treatment for ALS. This funding will support crucial translational research that brings us a step closer to delivering a therapy that could make a real difference for people living with this devastating disease."

**Professor Guillaume Hautbergue, Professor of Translational RNA Biology at SITraN and Chief Scientific Officer at Crucible said:** "This grant strengthens the partnership between Crucible and SITraN, combining world-leading science with translational expertise to accelerate



the development of new therapies for ALS. It exemplifies how academic–industry partnerships can transform cutting-edge research into meaningful advances for patients."

**Duncan Johnson, CEO of Northern Gritstone said:** "Crucible's foundations are built on the world leading expertise of Professors Guillaume Hautbergue, Mimoun Azzouz and Pamela Shaw. With support from Northern Gritstone and Argobio, Crucible is committed to developing therapies that halt the progression of neurological diseases. Winning this Innovate UK funding is testimony to the exciting potential of the science developed at the University of Sheffield"

**Neill Mackenzie, Executive Chair of Crucible and EIR & Partner at Argobio says:** "This is a tremendous achievement for Crucible and the founders and scientists at the University of Sheffield. This grant puts Crucible in a strong position to develop potentially life-changing treatments for patients with devastating neurodegenerative diseases. We are very grateful to the Innovate UK Biomedical Catalyst for this recognition."

## **About Crucible Therapeutics**

Crucible Therapeutics is a UK-based biotechnology company developing RNA-based medicines that target the root causes of neurodegenerative diseases. Founded in 2023, Crucible builds on discoveries from the Sheffield Institute for Translational Neuroscience (SITraN) and the NIHR Sheffield Biomedical Research Centre – global leaders in neuroscience research – to advance transformative therapies that halt disease progression and improve patients' lives. For more information, please visit <u>www.crucible-tx.com</u>

## About SITraN

The Sheffield Institute for Translational Neuroscience (SITraN), based at the University of Sheffield, is a world-leading centre for research into neurodegenerative diseases. Opened by Queen Elizabeth II in 2010, SITraN drives translational research from lab to clinic, pioneering therapies for conditions including ALS, SMA, and Parkinson's disease. With cutting-edge facilities and global partnerships, SITraN accelerates the development of innovative treatments that bring real hope to patients worldwide. For more information, please visit https://www.sheffield.ac.uk/sitran

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