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ANGELMAN SYNDROME THERAPEUTICS

Issue 3. July 2023



Natural History Study: Recruitment Complete

We're thrilled to announce the **completion of recruitment** for the Natural History Study at Oxford. This significant milestone, involving 40 participants, was made possible by the unwavering dedication of our community and the tireless efforts of our incredible team. The study, a £1m, four-year project funded by FAST UK, is pivotal for drug development, with each participant's involvement contributing to valuable data collection and future trials.

Our journey continues despite this milestone. The study's complexity requires substantial funding. **We need your support** - every contribution, whether a donation or a fundraising challenge, advances our research and makes a significant difference.

Active human clinical trials updates

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GTX-102 by Ultragenyx
aka KIK-AS trial

Phase: 1/2
Sites: USA, CAN, UK, AUS, FRA, ESP, ISR
Strategy: 'Unsilencing' of the paternal allele
Class: Antisense oligonucleotides (ASO)
Included genotypes: deletion

May 2023: FDA approval to expand the Phase 1/2 trial in the U.S., harmonizing dosage ranges with international cohorts and **accelerating** the study's completion.

Rugonersen by Roche CLOSED
aka TANGELO trial

Phase: 1
Sites: US, Italy, The Netherlands, Spain
Strategy: 'Unsilencing' of the paternal allele
Class: Antisense oligonucleotides (ASO)
Included genotypes: deletion and mutation

June 2023: The trial is **halted** due to insufficient clinical improvements despite an acceptable safety profile. Roche is seeking an external partner to continue the drug's development.

Alogabat by Roche
aka ALDEBARAN trial

Phase: 2
Sites: US, Spain
Strategy: Downstream Treatments
Class: Receptor function enhancement
Included genotypes: deletion only

June 2023: Recruitment is kicking off in 2 sites in Spain and United States.

ION582 by Ionis
aka HALOS trial

Phase: 1/2
Sites: USA, UK, AUS, FRA, ISR, ITA
Strategy: 'Unsilencing' of the paternal allele
Class: Antisense oligonucleotides (ASO)
Included genotypes: deletion and mutation

July 2023: No adverse events observed so far in the trial. No safety concerns as of now. The company says their recruitment is proceeding per their plan. And the long-term extension program continues. Announced plans to extend the age range and genotypes, but no dates.

NNZ-2591 by Neuren

Phase: 2
Sites: Australia
Strategy: Downstream Treatments
Class: Synaptic function
Included genotypes: all except mosaicism

July 2023: Enrolment continues, the Phase 2 results were pushed to 2024.

What is Angelman syndrome?

Angelman syndrome is a rare neurogenetic disorder that affects approximately one in 15,000 people. It is estimated that ~500,000 individuals live with Angelman syndrome worldwide. Children and adults with Angelman Syndrome typically have severe cognitive and motor impairments, and can have debilitating seizures. Some individuals never walk. Most do not speak. Disruptive sleep also can be a serious challenge. All require 24/7 care throughout their lives.

FAST Search & Rescue. How can I help?

Only about 2% of AS patients are identified globally. Every person living with Angelman syndrome in the UK should be registered with the **Global Angelman Syndrome Registry**. Otherwise we are not giving pharmaceutical companies the opportunity to identify and treat the population affected by this condition.

We need to show a strong presence of AS patients to advance research and treatments. Don't wait for someone else to step up - take action now and encourage others to do the same. Help us create a comprehensive database of individuals living with Angelman syndrome by registering and spreading awareness amongst your colleagues and professionals in the field.

Act now by registering here: https://angelmansearchandrescue.org/join-us/

Fundraising

Angelman Syndrome research in the UK does not receive government funding. Therefore, all current research only has happened due to the fundraising efforts from parents and wider community. If you would like to support us, please donate or start your own Cure Angelman Now campaign - more details are on our website.

Angelman Syndrome Library

We have launched a Library where we publish AI-generated quick overviews and summary pages for hundreds of conference talks, webinars and interviews on all aspects of Angelman Syndrome - therapies, scientific research, communication, behaviours, family support and planning. We welcome your feedback: https://cureangelman.org.uk/library/

About us

FAST, the Foundation for Angelman Syndrome Therapeutics, was founded in 2008 in the USA with an urgent mission: cure Angelman Syndrome. Today, with operations also in Australia, Canada, France, Italy, Spain, LatAm and here in the UK, FAST has become the largest non-governmental funder of Angelman Syndrome research.

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