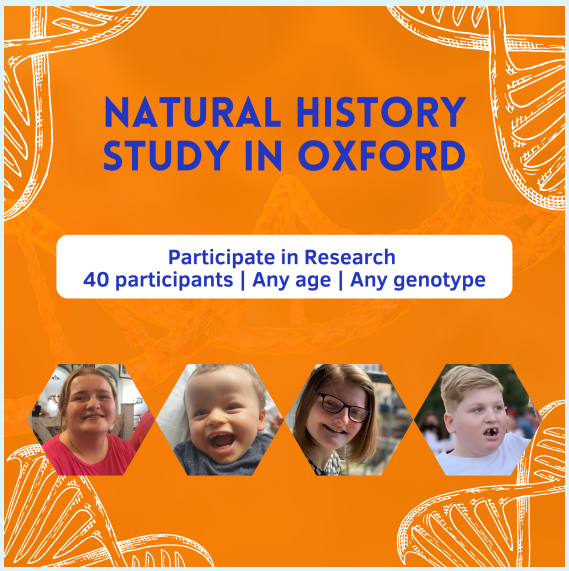
fast
cureangelman.org.uk

ANGELMAN SYNDROME THERAPEUTICS

Issue 2. January 2023



Join the Natural History Study now

The Study is to assess and observe participants affected by Angelman syndrome (AS) over time with the aim to identify and design specific assessment tools tailored to the needs of Angelman syndrome patients.

We need to recruit **6 more participants** to join in the **next 2 months**. Join now and tell everyone you know who is affected by AS in the UK.

Email theodora.markati@paediatrics.ox.ac.uk

The Study is invaluable for the drug development, clinical trials, regulatory approval and NHS adoption of any future disease-modifying therapeutics for Angelman syndrome.

Active human clinical trials updates

Scan to Subscribe and Share

GTX-102 by Ultragenyx
aka KIK-AS trial

Phase: 1/2
Sites: US, Canada, UK
Strategy: 'Unsilencing' of the paternal allele
Class: Antisense oligonucleotides (ASO)
Included genotypes: deletion

July 2022: Ultragenyx acquires GeneTx. The proceeds from the sale will be reinvested into AS research by FAST.

Rugonersen by Roche
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

December 2022: Long-term extension program is in progress, 61 patients recruited. Completion expected in 2026.

Alogabat by Roche NEW
aka ALDEBARAN trial

Phase: 2
Sites: 6 countries. TBC in February 2023
Strategy: Downstream Treatments
Class: Receptor function enhancement
Included genotypes: deletion only

December 2022: This new trial is announced to specifically address AS deletions that have GABA genes impacted. Alogabat is already known to have acceptable safety, hence Phase 2.

ION582 by Ionis
aka HALOS trial

Phase: 1/2
Sites: US, Israel, UK, more to open
Strategy: 'Unsilencing' of the paternal allele
Class: Antisense oligonucleotides (ASO)
Included genotypes: deletion and mutation

December 2022: Recruitment will be extended to 13 sites across 9 countries. US sites are all active, other sites are expected to launch within a month. No adverse events observed so far in the trial. 12 months long-term extension program has been started

NNZ-2591 by Neuren

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

December 2022: Phase 2 trial is continuing. First subject has completed the full treatment with good tolerability and safety. Results from the trial are now anticipated in H2 2023. In parallel with the trial, Neuren is also executing the foundational work to prepare for Phase 3 development.

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.

Upcoming events

International Angelman Day is observed on the **15th February** to raise awareness worldwide for Angelman Syndrome. We celebrate each year on the 15th February in recognition of the 15th chromosome affected, and with February being International Rare Disease month.

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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