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FOUNDATION FOR ANGELMAN SYNDROME THERAPEUTICS UK

ANGELMAN SYNDROME THERAPEUTICS

Issue 4. January 2024

Ionis publishes an update

The HALOS trial, now with 51 patients across 11 global sites, reports ION582 as generally well-tolerated. Side effects align with patients' medical histories, Angelman diagnosis, or intrathecal administration. One month post-final Part 1 dose, key findings include:

- Significant EEG improvement in most patients, with around 70% showing reduced slow-wave delta activity and over 80% experiencing faster frequency rhythms—surpassing natural history study results.
- Most patients improved in overall functioning per the Angelman Syndrome Clinical Global Improvement Change scale and on the total Bayley score, again exceeding natural history study observations.

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

January 2024: Current phase enrollment is now completed. Announced publication of data for 20 patients who have been on therapy for 6+ months in first half of 2024. Promising messages about Phase 3 coming up.

Rugonersen by Roche HALTED
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

November 2023: The trial remains **halted** but Roche are potentially closing a deal with a different company who **might** continue the program beyond February 2024. No updates were issued since Nov.

Alogabat by Roche
aka ALDEBARAN trial

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

November 2023: First patient was recruited in July 2023.
January 2024: 13 sites across 6 countries (USA, Germany, Italy, Spain, Australia, France) are in the state of active recruitment

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

November 2023: 51 patients globally found ION582 generally well-tolerated. Most patients showed significant EEG improvements and better overall functioning as per the AS Clinical Global Improvement Change scale and Bayley score, exceeding results from natural history studies.

NNZ-2591 by Neuren

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

December 2023: the trial has entered the last phase of screening for its phase two, with all subjects now enrolled. Expecting updates later in 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, Poland, and here in the UK, FAST has become the largest non-governmental funder of Angelman Syndrome research.

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