HOPE IN ACTION

PROGRESS THROUGH ADVOCACY

angelman SYNDROME FOUNDATION fast

2026 ANGELMAN SYNDROME CONGRESSIONAL ADVOCACY DAY

MARCH 4

ABOUT ANGELMAN SYNDROME

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Facts about Angelman Syndrome

1. Angelman syndrome (AS) is a rare disease affecting 1 in 15,000 people
2. At present, there are no treatments approved for individuals living with AS.
3. AS is caused by a lack or dysfunction of the UBE3A protein in the brain.
4. Individuals with AS suffer a nearly universal absence of verbal speech, motor impairment, seizures, and other severely debilitating symptoms.
5. Individuals with AS suffer from balance and coordination disorders, some are unable to walk.
6. Some individuals with AS exhibit severe anxiety due to their inability to communicate effectively.
7. Many individuals with AS have a hard time falling asleep or staying asleep. This is very challenging for the individual and the caregivers.
8. Symptoms of AS manifest in the earliest months of life, with many individuals diagnosed by the age of 2.
9. Despite the impact to the brain, AS is not degenerative, meaning that brain tissue is not deteriorating due to the disease.
10. Scientists believe AS has the potential to be truly transformed by a treatment compared to other similar neurological disorders.

There is an urgent need for more research funding in AS

Research is our best chance to unlock the mysteries of Angelman syndrome and develop targeted therapies that can improve the quality of life for those living with this condition. It fuels the work of dedicated scientists, researchers, and clinicians who are tirelessly working to unravel the complexities of Angelman syndrome. It supports clinical trials, innovative therapies, and the development of much-needed tools and resources. However, without adequate funding, our ability to accelerate the pace of discovery and deliver meaningful solutions is severely limited. Families affected by Angelman syndrome face daily challenges that demand immediate attention. Every dollar raised for research brings us one step closer to a breakthrough, one step closer to improved treatments, and one step closer to fulfilling our ultimate goal: a world where Angelman syndrome is no longer a source of hardship. As of March 2024, the therapeutic pipeline includes antisense oligonucleotide therapies (ASO), small molecule treatments, gene editing (CRISPR), gene therapies, and enzyme replacement therapy (ERT).

WHO ARE WE?

The **Angelman Syndrome Foundation (ASF)** and the **Foundation for Angelman Syndrome Therapeutics (FAST)** are two organizations united in their commitment to making a significant impact in the Angelman syndrome community.

ASF's mission is to advance awareness and treatment of Angelman syndrome through education, research, clinical care and unwavering support for individuals with AS, their families, and all concerned parties. They are dedicated to improving lives and fostering understanding. (angelman.org)

FAST, as the leading patient advocacy organization, shares a resolute goal: to cure Angelman syndrome. As the world's largest funder of Angelman syndrome research, they focus on expediting safe and effective treatments into current medical practice. They work tirelessly to transform the future for individuals affected by Angelman syndrome. (cureangelman.org)

Together, ASF and FAST combine their strengths and expertise, creating a powerful force that drives progress, offers support, and seeks cures for Angelman syndrome. Our collaborative efforts epitomize our shared dedication to making a lasting difference in the lives of those we serve.

To better understand Angelman syndrome, watch the "Day in the Life" video of three families living with AS.

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Angelman Syndrome Therapeutic Pipeline

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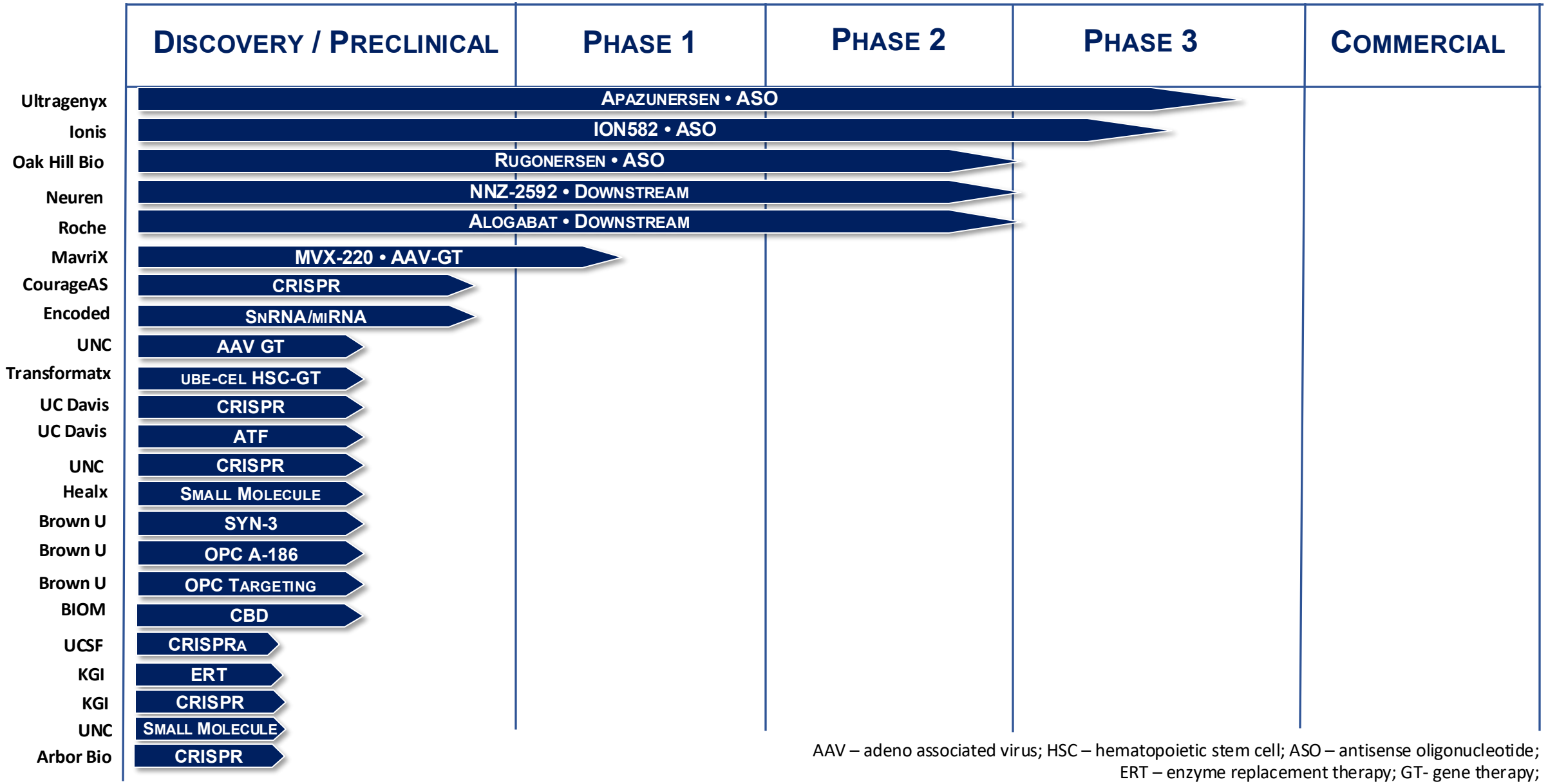

AAV – adeno associated virus; HSC – hematopoietic stem cell; ASO – antisense oligonucleotide;
ERT – enzyme replacement therapy; GT- gene therapy;

ANGELMAN SYNDROME

Voice of the Patient Report

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The Angelman syndrome (AS) community had the unique opportunity to share our experiences on living with the condition and perspectives on treatments for our loved ones directly with the Food and Drug Administration (FDA). On April 7, 2025, the Foundation for Angelman Syndrome Therapeutics and the Angelman Syndrome Foundation cohosted a virtual externally-led patient-focused drug development (EL-PFDD) meeting including nearly 400 participants:

227 AS caregivers and family members	**41** FDA staff
67 drug developer company representatives	**27** researchers and health care providers

This "**Hope in Action: an EL-PFDD Meeting on Angelman Syndrome**" and the resulting Voice of the Patient Report, submitted to FDA in November 2025, created a platform for our community to share insights and priorities directly with the FDA and other stakeholders, ultimately shaping the future of therapy development.

Angelman syndrome is a serious neurogenetic condition that affects the brain and causes profound developmental and cognitive challenges across the typical lifespan. Current treatments only address symptoms and have minimal effects on how individuals with AS feel or function. The EL-PFDD Meeting highlighted the need for increased awareness of the impact of AS, on both the person living with the condition and their caregivers, as well as the need for improved treatments.

Key Takeaways:

- The symptoms of AS are wide-ranging and complex, with devastating impacts on an individual's activities of daily living that significantly limit his or her independence.
- **Communication challenges, cognitive limitations, challenging behaviors, and sleep disturbances** are among the symptoms that place the greatest strain on people living with AS and their caregivers.
- Treatment of AS is limited to symptom management through a dizzying array of medications, assistive technologies, and other interventions deployed across a normal lifespan with minimal improvements in quality of life at best.
- AS has significant physical and mental impacts on both the individuals affected and their caregivers. **Caregivers must provide constant care for the entirety of their child's life.**
- There is an urgent need for a disease-modifying treatment for AS. Families are eager for a treatment that could provide **even small improvements** in cognition, communication, and the ability of their loved ones to carry out activities of daily living.
- The need for FDA concurrence on the utility of existing outcome measures and possible biomarkers intended to measure potential effects of new disease-modifying treatments will help advance late-stage trials. Such advances could accelerate innovation to address AS unmet needs.

> "When it comes to those challenges, I would say nonverbal communication is number one. I feel like the behaviors are a result of him not being able to communicate quickly and verbally is something that, if he had five words, I would give my life for him to have five words."
>
> *– Mother of nine-year-old son*

> "Our son requires care in everything he does, helping him with dressing, toileting, and getting to school. Independence is non-existent, which is unusual for an 11-year-old. As he gets older and bigger, so do his needs."
>
> *– Mother of 11-year-old son*

> "As an adult, he's had the experience that I have most feared. He's big, he looks like an adult when he doesn't feel like getting shaved. And he had a caregiver in his group home who wasn't familiar with him, who panicked because of his behaviors and called the police who then came into the home, and wanted to take him to the emergency room…"
>
> *– Mother of 23-year-old son*

> "[Our son] never slept through the night, which means that he must be awake or one of us must be awake with him 24/7. So my husband and I do take turns caring for him try and prevent caregiver burnout."
>
> *– Mother of nine-year-old son*

Scan the QR code to read the Angelman Syndrome Voice of the Patient report

Angelman Syndrome FY27 Appropriations Requests

Department of Defense (DOD)

Angelman syndrome — Continue to include Angelman syndrome as an eligible condition for the Peer-Reviewed Medical Research Program.

Food & Drug Administration (FDA)

Angelman syndrome — Rare diseases are often thought of as severe when they are deadly or progressive. However, non-degenerative conditions such as Angelman syndrome are just as severe due to the life-long and debilitating symptoms and need impactful drug development and regulatory flexibility. The Committee recognizes the importance of the Externally-Led Patient-Focused Drug Development (EL-PFDD) meeting on Angelman syndrome (AS) that was held in April 2025. The Voice of the Patient report generated from the meeting demonstrates that seemingly small or subtle clinical gains for people with AS have a profound impact. Available clinical endpoints and biomarkers are not always sensitive enough to measure small but meaningful changes and innovative analysis strategies may need to be employed. The Committee urges the FDA to ensure clinically meaningful improvements that matter to patients and families are recognized by utilizing this patient experience data to inform regulatory decision-making and the further development of therapies for Angelman syndrome.

National Institute of Health – National Institute of Neurological Disorders and Stroke (NINDS)

Angelman syndrome — The Committee recognizes the importance of advancing research in Angelman syndrome (AS), a rare neurogenetic disorder with significant unmet medical need. The Committee is aware that ongoing gene therapy and gene-targeted approaches, including gene editing and CRISPR technologies, require robust natural history studies with long-term follow-up to inform clinical development and regulatory decision making. With appropriate clinician education on data collection during follow up, real-world evidence and observational data collection can significantly enhance natural history study data. Natural history databases could collect post-market data with or in addition to sponsor data collection to ensure that data adds to the general understanding of the condition and the long-term impact of potential therapies. The Committee urges NINDS to prioritize and support funding for natural history studies and real-world data collection in AS to establish critical benchmarks and facilitate the evaluation of emerging therapeutics.

National Institute of Health- National Center for Advancing Translational Sciences (NCATS)

Angelman syndrome – As gene therapy and gene targeted approaches continue to develop with significant potential for changing outcomes for patients with Angelman syndrome and other rare conditions, the Committee acknowledges the critical leadership of NCATS, particularly through the Somatic Cell Genome Editing (SCGE) program. The SCGE initiative has made meaningful progress in advancing gene-editing technologies and has demonstrated how development strategies for one disease area can create a platform for other indications. The Committee understands that scientific endeavors don't always follow a pre-ordained pathway, and that manufacturing and other challenges can arise that result in changes in project plans and timelines. Especially given promising initial results in animal models, the Committee urges NCATS to continue this initiative and to expand this type of work to benefit all gene-targeted modalities and to continue to apply learnings across conditions.

The Honorable Robert Aderholt
Chair
Labor HHS, Education, and Related
Agencies Subcommittee
Committee on Appropriations
Washington, DC 20515

The Honorable Rosa DeLauro
Ranking Member
Labor, HHS, Education and Related
Agencies Subcommittee
Committee on Appropriations
Washington, DC 20515

The Honorable Andy Harris
Chair
Agriculture, Rural Development, FDA,
& Related Agencies Subcommittee
Committee on Appropriations
Washington, DC 20515

The Honorable Sanford Bishop, Jr.
Ranking Member
Agriculture, Rural Development, FDA, &
Related Agencies Subcommittee
Committee on Appropriations
Washington, DC 20515

The Honorable Ken Calvert
Chair
Defense
Committee on Appropriations
Washington, DC 20515

The Honorable Betty McCollum
Ranking Member
Defense
Committee on Appropriations
Washington, DC 20515

Dear Chairs Aderholt, Harris, Calvert and Ranking Members DeLauro, Bishop and McCollum:

Thank you for supporting and including Angelman syndrome (AS) priorities in Fiscal Year 2026 appropriations reports. We have seen the impact of federal investment in research for other conditions and believe that there is real potential to make a meaningful difference in the lives of those living with Angelman syndrome.

Angelman syndrome is a rare disease that affects approximately 1 in 15,000 individuals or an estimated 20,000 to 25,000 people in the United States. Despite its genetic rarity the condition impacts constituents in every state. Angelman syndrome is caused by a lack of or dysfunction in the UBE3A protein in the brain. As a result, individuals with AS experience a near universal absence of speech, significant sleep challenges, motor impairments, seizures, intellectual disability and other debilitating symptoms. Although AS primarily affects the brain it is not degenerative and does not impact other body systems. Individuals with the condition typically live a full lifespan though without a full quality of life. At present there are no approved treatments for Angelman syndrome. However, the AS community has been exceptionally active in self-funding research and clinical trials.

There is growing hope for individuals with Angelman syndrome as new gene therapy and gene targeted treatments continue to move forward. These approaches have the potential to improve daily functioning and quality of life, but progress depends on having a clear understanding of how the condition changes over time. Long term follow up, natural history studies, and information collected during routine care are important to help researchers understand the disease and measure whether new treatments are making a difference.

Decisions about new treatments should reflect what matters most to people living with Angelman syndrome and their caregivers. Families have documented that even small improvements such as better sleep, communication, or movement can make a real difference in daily life.

As you work on the Fiscal Year 2027 Appropriations bill, we respectfully request the inclusion of language that continues support for efforts at the National Institutes of Health's National Institute of Neurological Disorders and Stroke (NINDS) and National Center for Advancing Translational Sciences (NCATS), as well as the Food and Drug Administration (FDA), to incorporate patient experience, long-term data, and flexible regulatory approaches when reviewing potential treatments for Angelman syndrome.

In addition, while the list of eligible conditions under the Department of Defense (DOD) Peer-Reviewed Medical Research Program (PRMRP) is typically only listed in the Senate appropriations report, we wish to underscore that continued support for DOD-led research efforts remains a priority for the Angelman syndrome community and is an important component of our broader, coordinated request.

Below is the specific language we are requesting:

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Sincerely,

TROY BALDERSON
Member of Congress

ANGIE CRAIG
Member of Congress