Congress of the United States

Washington, DC 20515

May 23, 2025

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

Dear Chairs Aderholt and Harris and Ranking Members DeLauro and Bishop:

Thank you for supporting and including Angelman syndrome (AS) priorities in Fiscal Year 2025 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 difference in the lives of those living with Angelman syndrome.

Angelman syndrome is a rare disease that impacts 1 in 15,000, or approximately 20,000-25,000 individuals in the United States of America. Despite the genetic rarity, the disease impacts constituents in every state. The disease is caused by a lack of or dysfunction in the UBE3A protein in the brain. As a result of the protein deficiencies, individuals with AS suffer nearly universal absence of speech, significant sleep challenges, motor impairments, seizures, intellectual disability, and other debilitating symptoms. Despite the issues with the brain, AS is not degenerative, does not impact other body systems, and individuals with the disease live full lifespans, just not a full quality of life. At present, there are no approved treatments for AS. However, the community has been incredibly active in self-funding research and trials.

Most individuals with Angelman syndrome are dependent on caregivers to complete daily tasks including eating, toileting, dressing, grooming and navigating their daily environment. A seemingly small improvement in one of those tasks can have significant impact on a patient's ability to be independent, but current tools do not always measure progress in that way or account for its importance. To advance research and drug development for rare conditions such as Angelman syndrome, we must encourage federal agencies to prioritize coordination and information sharing, assessment of novel approaches, and inclusion of the patient and caregiver experience in regulatory decision-making.

Research and guidance spurred under Congress' direction could dramatically better the lives of individuals with Angelman syndrome and their families. For example, medical research funded through the Department of Defense peer-reviewed medical research program (PRMRP) has had

demonstrable results. As you work to prepare the Fiscal Year 2026 Appropriations bill, we urge you to continue your support through report language directed at the Food and Drug Administration (FDA) and the National Institutes of Health (NIH).

Below is the specific language we are requesting:

National Institute of Health – Neurological Disorders and Stroke

Angelman syndrome.— The Committee understands that NINDS is convening research leaders, patient organizations, and other stakeholders to prepare a roadmap for clinical outcomes measures and biomarkers for Angelman syndrome, a rare neurogenetic disorder. As a part of and following this convening, NINDS should support funding for clinical outcome measure and biomarker development, determine more efficient pathways for developing and manufacturing novel gene therapies for neurodevelopmental diseases, and inform the next generation of clinical studies that should be pursued based on approved biomarkers. NIH should ensure timely and comprehensive data-sharing across investigators and industry in order to advance these goals.

Food & Drug Administration

Angelman syndrome. – The Committee recognizes the importance of patient-focused drug development for Angelman syndrome, a rare and devastating monogenic neurodevelopmental disorder. Patient and family preferences and broader patient experience data should have an impact on drug development. For this population, seemingly small gains in self-care are critical for patients and their caregivers in building toward independence. Without treatments individuals are completely dependent on a caregiver to perform the fundamental activities of daily living such as dressing, eating, grooming, and navigating their environment. The Committee encourages FDA to utilize patient experience data to inform regulatory decision-making, as well as the further development of Angelman syndrome clinical endpoints and biomarkers. FDA should ensure clinically meaningful improvements that matter to patients and families are recognized in regulatory decision-making.

Sincerely,

Troy Balderson
Member of Congress

Angie Craig
Member of Congress

Congress of the United States

Washington, DC 20515

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