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:

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.

Sincerely,

TROY BALDERSON
Member of Congress

ANGIE CRAIG
Member of Congress