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.