

Angelman Syndrome FY26 Appropriations Requests

DOD

Continue to include Angelman syndrome as an eligible condition for the Department of Defense (DOD) Peer Reviewed Medical Research Program (PRMRP).

NIH, NINDS

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.

FDA Office of Commissioner

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.