

Funding for Rare Neurodegenerative Disease Research and Access to ALS Investigational Drugs

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A report to congressional committees.

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What GAO Found

The National Institutes of Health (NIH) and Food and Drug Administration (FDA) awarded about \$276 million from fiscal years 2022 through 2025 to implement the Accelerating Access to Critical Therapies for ALS Act (ACT for ALS Act). Most of this funding was awarded by (1) NIH for grants that supported access to investigational drugs—drugs not yet approved for marketing by FDA—for individuals with amyotrophic lateral sclerosis (ALS) and related research (45 percent) and (2) NIH and FDA for a public-private partnership focused on rare neurodegenerative disease research (45 percent). FDA also awarded grants and contracts for research to further scientific knowledge on ALS and other rare neurodegenerative diseases and for the clinical development of therapies (10 percent). About 750 individuals with ALS are expected to receive access to investigational drugs through the NIH grants.

Summary of Funding Awarded by NIH and FDA to Implement the Accelerating Critical Therapies for ALS Act, Fiscal Years (FY) 2022 through 2025 (in millions)

	FY22	FY23	FY24	FY25	TOTAL
NIH grants for access to amyotrophic lateral sclerosis (ALS) investigational drugs and related research	\$18.1	\$32.5	\$39.2	\$35.4	\$125.3 (45%)
NIH and FDA funding for rare neurodegenerative disease public-private partnership	\$5.5	\$42.3	\$36.0	\$40.6	\$124.4 (45%)
FDA grants and contracts for ALS and other rare neurodegenerative disease research	\$5.8	\$5.1	\$5.5	\$10.1	\$26.5 (10%)
TOTAL	\$29.4	\$79.9	\$80.7	\$86.2	\$276.3

Source: GAO analysis of documentation from the National Institutes of Health (NIH) and Food and Drug Administration (FDA) and interviews with agency officials, as of December 2025. | GAO-26-107691

Note: The awarded funds include those appropriated by Congress specifically to implement the act, as well as funds identified by FDA from other sources. Totals may not add up due to rounding and do not include funds used to administer the funding awarded.

NIH and FDA officials identified challenges to implementing the ACT for ALS Act (particularly in fiscal year 2022) and took actions to address those within their control. For example, applicants had limited time to apply for NIH grants in fiscal year 2022 because appropriations were not available until midway through the fiscal year. As a result, in later years NIH posted requests for grant applications prior to appropriations being enacted. However, other challenges were outside the agencies' control—including no direct appropriations to FDA to support its priorities for other rare neurodegenerative diseases for the public-private partnership, according to agency officials.

Stakeholder interviews, available literature, and NIH data indicated benefits of NIH and FDA funding, such as increases in the number and geographic diversity of clinic sites providing access to ALS investigational drugs. However, most of the research funded by NIH and FDA is ongoing and the full effects are not yet known. Anticipated benefits include increased data on ALS and addressing research gaps for ALS and other rare neurodegenerative diseases. For example, stakeholders expect these data to be useful to ALS research because the data will meet high data quality standards and will be available for other researchers.

Why GAO Did This Study

ALS is a rare, progressive, and ultimately fatal neurological disorder. As with other rare diseases, diagnosis may be delayed, affecting the eligibility of individuals with ALS to participate in clinical trials. ALS has no cure and limited treatment options. Physicians or drug sponsors can request FDA authorization to make investigational drugs available outside of clinical trials for individuals with serious or life-threatening diseases through FDA's expanded access pathway.

The ACT for ALS Act includes a provision for GAO to report on the funding NIH and FDA awarded to implement the act. This report describes the funding NIH and FDA awarded to implement the act, challenges NIH and FDA identified in awarding that funding, and what is known about the effect of the funding on research and development of therapies for ALS and other rare neurodegenerative diseases.

GAO reviewed relevant laws, congressional reports, NIH and FDA documentation, and grant and contract applications and progress reports. GAO also reviewed relevant data in NIH databases as of November 2025 on the research studies funded to implement the act. GAO interviewed NIH and FDA officials and a nongeneralizable sample of 21 stakeholders—including national associations, NIH and FDA grant recipients, drug sponsors, clinic sites, public-private partnership entities, and a committee of individuals with ALS and caregivers. GAO selected stakeholders to attain variation in perspectives on ALS research implemented through the act and in organization type involved in clinical research. GAO also reviewed research relevant to the awarded funding published from January 2019 through September 2025.