NIH-Supported Amyotrophic Lateral Sclerosis Research

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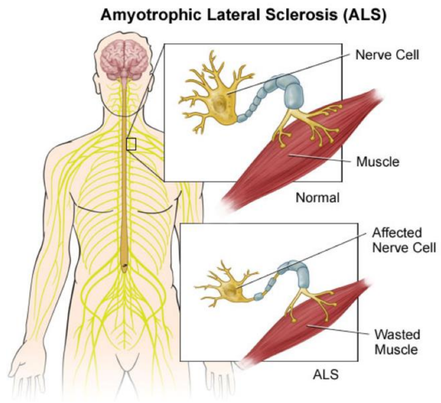
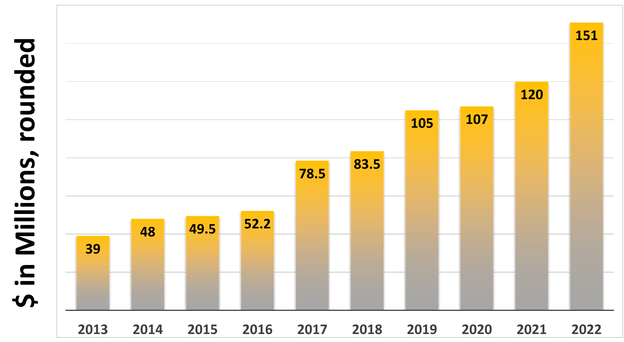
Amyotrophic Lateral Sclerosis (ALS) – at a Glance

- ALS is a neurodegenerative disorder that typically occurs in mid-life, causing weakness and wasting of most skeletal muscles including the diaphragm, as well as cognitive and behavioral changes in 35-45% of cases
- According to the National ALS Registry, approximately 32,000 people in the U.S. are living with ALS, and between 5,000 and 6,000 new cases are diagnosed every year
- About 15% of ALS cases have a family history
- FDA-approved, disease-modifying drugs for ALS include sodium phenylbutyrate-taurursodiol and several formulations of riluzole and edaravone for all forms of ALS, as well as tofersen for familial ALS associated with mutations in the *SOD1* gene; these drugs may have beneficial effects on the disease course and/or survival, but no treatments are known that stop or reverse ALS



NIH Funding of ALS Research per Fiscal Year



2023 NIH ALS Strategic Priorities

Working Group Topics

- Accelerating Research on the Biology behind ALS
- From Research to Therapy
- Optimizing ALS Clinical Research
- Optimizing ALS Quality of Life
- Improving Collaborative Partnerships

Five working groups led by a steering committee identified the highest priorities for research intended to result in the discovery of effective interventions for the diagnosis, treatment, management, prevention, or cure of ALS.

Strategic planners included people with lived experiences in ALS, scientists, clinicians, federal partners, and the broader ALS community.

Read more about the ALS Strategic Priorities:

NIH-Commissioned Study by the National Academy of Sciences, Engineering, and Medicine (NASEM) on Research, Care, and Services for ALS

Amyotrophic Lateral Sclerosis: Accelerating Treatments and Improving Quality of Life

- Study Task: Identify and recommend key actions for the public, private, and nonprofit sectors to undertake to make ALS a livable disease within a decade
- Process: An ad hoc committee convened by NASEM is currently reviewing the current state of ALS therapeutic development, care, services, and supports to recommend key actions by June 2024
- Register for public events on August 10, 23 & September 1, 2023

Provide feedback on this project

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Implementing the Accelerating Access to Critical Therapies for ALS Act: ACT for ALS (signed into law in December 2021)

Section 2: Grants for **scientific research utilizing data from expanded access** to investigational drugs for individuals who are not otherwise eligible for clinical trials in ALS.

- *Active, NIH-funded expanded access study:*

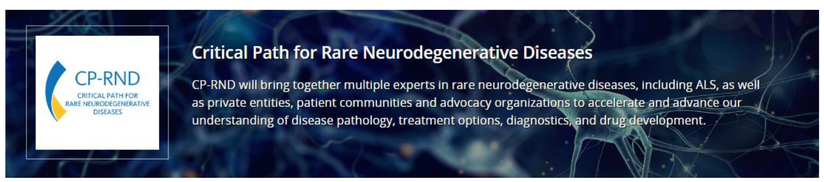
  An Expanded Access Protocol of Intravenous Trehalose Injection 90 mg/mL Treatment of Patients with Amyotrophic Lateral Sclerosis

- *Request for Applications, fiscal year 2023:*

  RFA-NS-23-012—Amyotrophic Lateral Sclerosis (ALS) Intermediate Patient Population Expanded Access

NIH RePORT

Section 3: Establish a **Public Private Partnership for Rare Neurodegenerative Diseases between the NIH, FDA, and other eligible partners**.



Goals:

- Ensure participation of people with lived experiences and non-profit organizations in the activities of the public private partnership
- Accelerate medical product development in ALS and other rare neurodegenerative diseases
- Harmonize, connect, and share large-scale clinical datasets of ALS
- Establish/expand a broadly accessible, large-scale repository of ALS clinical biospecimens and facilitate scientific analyses of these specimens
- Integrate clinical data with biologic measures to identify biomarkers for early diagnosis of ALS and monitoring disease progression and response to treatments