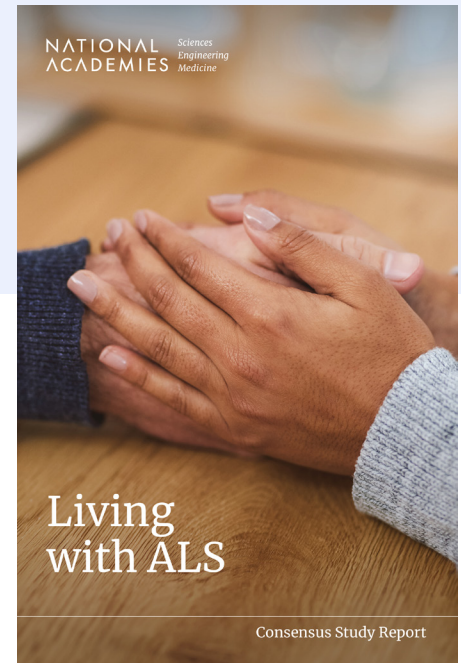


Living with ALS

At any one time, at least 30,000 people in the United States are living with amyotrophic lateral sclerosis (ALS), a progressive and inevitably fatal neurological disease with no treatments that stop disease progression or provide a cure. In addition, there are a number of structural challenges that make navigating the system of ALS care and research incredibly difficult for people living with ALS. Despite heroic efforts undertaken at ALS clinics across the country, many people living with ALS experience significant delays in diagnosis and never receive the multidisciplinary care they need. ALS clinical trials often struggle to identify participants that meet eligibility criteria and therefore are insufficiently enrolled and incapable of providing meaningful data. There is no comprehensive national registry of ALS, precluding meaningful assessment of overall trends in the numbers and health of people with ALS. Progress in therapeutics has been inconsistent and halting, and there is little to offer people who are at-risk ALS genetic carriers.

There are bright spots in the national picture of ALS, particularly the type of care and support provided by the U.S. Department of Veterans Affairs (VA). Everyone living with a progressive disease such as ALS deserves, at a minimum, the type of care VA provides—care that frees an individual and their families from financial devastation because of the need to pay for ventilators, accessible vans, and home modifications; care that proactively delivers the interventions and equipment one needs in a timely manner; and care planning and services landscape navigation that includes partnering with caregivers and family members.

In response, Congress directed the National Institutes of Health (NIH) to commission a committee of the National Academies of Sciences,



Engineering, and Medicine to recommend key actions for the public, private, and nonprofit sectors that would make ALS a livable disease within the next 10 years. The study committee comprised experts in neurology, rehabilitation, pulmonary, and primary care; translational ALS and frontotemporal dementia research; health law and policy; ethics; public health; health care financing; nursing and long-term care; therapeutic development and regulatory pathways, and individuals with ALS lived experience. The resulting report, *Living with ALS*, presents an analysis of the difficulties people with ALS and their caregivers face and provides recommendations to address these challenges.

MAKING ALS LIVABLE WITH SHORT- AND LONG-TERM ACTIONS

The committee defined making ALS livable around two dimensions: survivability and quality of life. This means that ALS is livable when an individual diagnosed with ALS or at genetic risk of developing ALS can survive, thrive, and live a long, meaningful life while meeting the medical, psychosocial, and economic challenges of the disease. Based on its review of the literature, discussions with individuals with lived ALS experience, and committee deliberations, the committee identified several issues that make life harder for people with ALS. These include:

- difficulty obtaining the necessary multidisciplinary care and services, including home services and respiratory care,
- gaps in support for the emotional and mental health toll of having a progressively disabling and invariably fatal disease for individuals with ALS and their caregivers,
- the monetary burden arising from high out-of-pocket expenses that can devastate a family's finances, and
- insurance barriers that create challenges in acquiring medically indicated equipment, technology, and therapeutics.

To address these challenges, the committee provided recommendations in four areas that, if implemented, would make ALS a more livable disease within a decade:

1. Short-term actions that Congress, the Centers for Medicare & Medicaid Services (CMS), private insurers, and ALS nonprofits should take to remove barriers for people with ALS and their caregivers to receiving care and services that improve quality of life (Recommendations 3-1 to 3-5 in the report).
2. Longer-term actions that Congress, CMS, private insurers, NIH, ALS multidisciplinary care leaders, and community-based providers should take to build a sustainable, integrated, and coordinated system of ALS care and research (Recommendations 4-1 to 4-4 in the report).
3. Actions that NIH, the Centers for Disease Control and Prevention (CDC), the Agency for Healthcare Research and Quality (AHRQ), and public-private partnerships funded under the Accelerating Access to Critical Therapies Act should take to improve epidemiological data, accelerate research and therapeutic development, and advance understanding of what works best in ALS care (Recommendations 5-1 to 5-4 in the report).
4. Actions that CMS, private insurers, state legislatures, research funders, and the ALS community should take to advance ALS prevention research and ultimately stop the disease from developing in at-risk populations (Recommendations 6-1 and 6-2 in the report).

THE IMPERATIVE TO PROVIDE EQUITABLE, HIGH-QUALITY MULTIDISCIPLINARY CARE

Because ALS is a multisystem disease requiring proactive treatments and interventions that are useful early in the disease but may be different from what is useful later in the disease, people with ALS require care from multiple medical disciplines over the full course of the illness. Research has shown that high-quality, multidisciplinary care can extend life expectancy, reduce hospitalizations and cost of care, improve quality-of-life outcomes,

and increase patient and caregiver satisfaction. There is no definitive count of the number of people living with ALS today who do receive evidence-based multidisciplinary care, but one estimate suggests that it is at best about half of the population. The committee concluded that ensuring equitable access to high-quality, multidisciplinary care for all individuals, regardless of socioeconomic status or geographical location, is of paramount importance.

One of the committee’s key recommendations (Recommendation 4-1) is that CMS and NIH, in partnership with ALS multidisciplinary care programs and community-based providers, build a re-envisioned, highly inclusive and integrated three-level ALS care and research network comprising Community-Based ALS Care Centers, Regional ALS Centers, and Comprehensive ALS Care and Research Centers (see Figure 1). Every person with ALS and their family will be able to use all three

care settings to meet their needs for specialized care and research services at a geographically convenient location. The resulting highly integrated system of care would help reach underrepresented and underserved individuals and those living in rural or remote areas.

ACCELERATING ALS RESEARCH AND DRUG DEVELOPMENT

Efforts to develop effective therapeutics for ALS have largely failed, even though research advances over the past decade identified potential drug targets and genes associated with ALS. One impediment to these efforts is the limited number of individuals available to participate in clinical trials—many people with ALS are diagnosed too late in the disease process to participate in a clinical trial, while others are not aware of the opportunities to participate in research. Other obstacles to progress include a lack of biomarkers and an overall limited understanding of the disease and how it progresses.

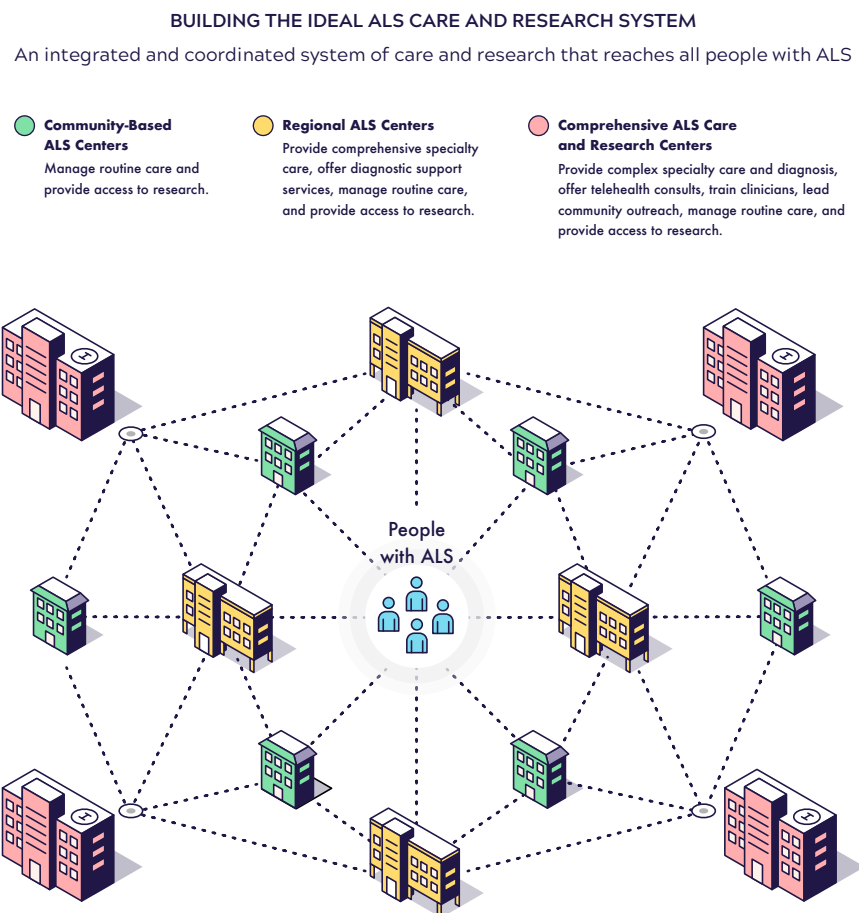


FIGURE 1 A reimagined ALS hub-and-spoke care system to increase access to multidisciplinary care.

To accelerate ALS research, the committee recommended the following actions:

1. NIH should ensure the existence of a dedicated ALS clinical trials network distributed across diverse geographic regions and integrated with the proposed ALS care delivery network to enable every individual with ALS to participate in clinical trials if they desire. A widespread clinical trials network would also reach more diverse ethnic, racial, and socioeconomic populations (Recommendation 5-1 in the report).
2. NIH, AHRQ, and other ALS research funders should prioritize research to learn what works best in ALS care and increase support for other critical areas of ALS research that are currently neglected but would yield near-term gains in quality of life for persons with ALS (Recommendation 5-4 in the report).
3. CDC and NIH should build a comprehensive ALS registry as part of a larger ALS data platform. CDC should work with local epidemiology leaders to ensure ALS is added to the National Notifiable Diseases Surveillance System, and states should require that clinicians report all cases of ALS (Recommendation 5-3 in the report).
4. CMS and private insurers should pay for genetic testing for all people living with ALS and their families. State legislatures should examine possible measures to prohibit genetic discrimination in life insurance, long-term care insurance, and disability insurance based on genetic risk of ALS (Recommendation 6-1 in the report).
5. Research funders should partner with drug developers and the ALS community to accelerate research focused on populations at risk of developing ALS, including at-risk genetic carriers (Recommendation 6-2 in the report).

LOOKING FORWARD

In the short term, the recommendations outlined in the report aim to ensure all individuals with ALS have expedited access to and coverage of essential ALS medical and support services. In the longer term, implementing the committee's recommendations will help make ALS a livable disease in 10 years by increasing survivability and quality of life for everyone affected, including people living with ALS, people at genetic risk of developing ALS, and their families and caregivers.

COMMITTEE ON AMYOTROPHIC LATERAL SCLEROSIS: ACCELERATING TREATMENTS AND IMPROVING QUALITY OF LIFE Alan I.

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FOR MORE INFORMATION

This Consensus Study Report Highlights was prepared by National Academies' staff based on the Consensus Study Report *Living with ALS* (2024).

The study was sponsored by the National Institute of Neurological Disorders and Stroke, National Institutes of Health. Any opinions, findings, conclusions, or recommendations expressed in this publication do not necessarily reflect the views of any organization or agency that provided support for the project. Copies of the Consensus Study Report are available from the National Academies Press, (800) 624-6242 or <https://nationalacademies.org/Living-with-ALS>.

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