Living with ALS

Role of the National Institutes of Health

Congress requested that the National Academies of Sciences, Engineering, and Medicine convene a committee of experts to recommend actions public, private, and nonprofit sectors should undertake to make amyotrophic lateral sclerosis (ALS) a livable disease within 10 years. This included considering pathways for developing more effective and meaningful treatments as well as a cure; identifying the type and range of care and services people with ALS and their families need; and ensuring equitable access to comprehensive care to improve quality of life.

The committee recognizes that the National Institute of Neurological Disorders and Stroke (NINDS) of the National Institutes of Health (NIH) is actively collaborating on and co-leading public–private partnerships created under the Accelerating Access to Critical Therapies for ALS (ACT for ALS). These partnerships hold enormous promise, and the committee believes realizing the full potential of current ACT for ALS initiatives is critical to achieving the goal of making ALS a livable disease in 10 years.

To complement existing efforts, the committee makes specific recommendations for NIH in order to (1) build an inclusive and integrated ALS multidisciplinary care and research system and align reimbursement to achieve the goals of the system; (2) create an ALS clinical trials network; (3) build a comprehensive ALS registry as part of a larger data platform; and (4) expand ALS translational research and prioritize neglected areas of research that would yield near-term gains in quality of life for people living with ALS.

RECOMMENDATIONS

The committee found there is notable variation in the quality and consistency of care across ALS clinics. A reimagined, inclusive, and integrated ALS care and research system, building on what already exists, is needed to ensure care quality, provide additional infrastructure to collect population health data, and coordinate care for individuals with ALS across care settings. As such, **the committee recommends that NIH partner with the Centers for Medicare & Medicaid Services (CMS), current ALS multidisciplinary care clinic leaders (e.g., U.S. Department of Veterans Affairs, ALS Association, Muscular Dystrophy Association), and community-based providers to build a care and research system comprised of Community-Based ALS Centers, Regional ALS Centers, and Comprehensive ALS Care and Research Centers. NIH should also work with CMS and private insurers to ensure that reimbursement is aligned with the goals of the new care and research system (see Recommendations 4-1 and 4-3 in the report).**

The committee concluded that while there are a variety of mechanisms for conducting clinical trials today, a

centralized, dedicated ALS clinical trials network that builds on and brings together existing ALS clinical trial consortia would provide a coherent approach to clinical trials and natural history studies. A centralized, NIH-led ALS clinical trials network would provide the best of each currently available network and harmonize approaches and support to see improvement in ALS trial success. As such, **the committee recommends that NIH should coordinate and fund an ALS clinical trials network distributed across diverse geographic regions in the United States (see Recommendation 5-1 in the report).**

The committee found that the current Centers for Disease Control and Prevention (CDC) National ALS Registry is inadequate in its ability to collect complete, representative, and timely data on the ALS population. A robust registry of people with ALS, as part of a larger ALS data platform, would help measure progress toward making ALS a more livable disease. As such, **the committee recommends that in partnership with CDC, NIH (e.g., via the new Access for All in ALS consortium) should integrate new and current data sources with CDC's National ALS Registry to create a comprehensive, interoperable data platform capable of collecting detailed, geocoded, longitudinal data on all individuals living with ALS, as well as people at increased genetic risk of developing ALS (see Recommendation 5-3 in the report).**

The committee recognizes the robust work of public–private partnerships under ACT for ALS and the fact that their initiatives were being designed in parallel with this report's development. As such, **the committee recommends that the ACT for ALS public–private partnerships consider additional translational research opportunities to accelerate therapeutic development. This could include research to understand the preclinical stage of disease, or prodromal state, to inform when to intervene with therapeutics (see Recommendation 5-2 in the report for full list).**

The committee also found that there are significant opportunities to prioritize research that would yield near-term gains in quality of life for people with ALS. As such, **the committee recommends that NIH and other ALS research funders prioritize research to learn what works best in ALS care. For example, health services research to evaluate nonpharmacologic interventions, services, and models that can provide a high quality of life for individuals with ALS, such as large, prospective studies of rehabilitative therapy interventions including physical therapy, speech and language supports, and respiratory therapy (see Recommendation 5-4 in the report).**

To access the full report and supporting materials, visit https://nationalacademies.org/Living-with-ALS.

Health and Medicine Division

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