

February 27, 2025

The Honorable Theresa Wood, Chair
Vermont House of Representatives
Human Services Committee
115 State Street, Drawer 33
Montpelier, VT 05602

Re: H.46 - An act relating to the Rare Disease Advisory Council

Greetings Chair Wood and Representatives Garafano, Donahue, Bishop, Cole, Eastes, McGill, Nielsen, Noyes and Steady,

On behalf of The ALS Association and the roughly 50 families we serve in Vermont, we urge you to swiftly move H.46 through your committee. H.46 establishes a rare disease advisory council (RDAC) within the state, which if passed, would help to give a voice to the estimated 1-in-10 individuals living with a rare disease in the state of Vermont. Almost half the people we serve are under 65 and face high costs with fewest resources. 83% are in rural areas and only 8% are veterans with the greatest resources as ALS is a 100% service-related disability for those who have served in the military.

Any conditions that affect fewer than 200,000 Americans are considered rare. Rare diseases are present across a broad spectrum of medical conditions, including amyotrophic lateral sclerosis (ALS). ALS is a fatal progressive neurodegenerative disease that slowly robs a person's ability to walk, talk, eat, and eventually breathe. There is currently no cure or significantly effective treatment options for ALS. But, like other rare diseases, ALS patients face many unique challenges every day, from obtaining an accurate diagnosis and accessing medical specialists with knowledge of their condition to battling for fair insurance coverage for their treatment and care.

However, due to small patient populations and the large variety of rare diseases, it can be difficult for state governments to have an in-depth understanding of the rare disease community's needs. This lack of awareness often contributes to the obstacles faced by rare disease patients and their loved ones. Creating an RDAC in Vermont will raise awareness and give rare disease patients a unified voice in Vermont state government. Additionally, the RDAC will be a valuable advisory body to elected officials and other state leaders on rare disease research, beneficiary access to treatments, and best practices for the care of those with rare diseases. Consultation and coordination can prevent disruptions such as took place temporarily last summer when prior authorization for Non-Emergency Medical Transportation (NEMT) was implemented on short notice, causing some wheelchair using patients to miss multidisciplinary care appointments that have been proven to extend life span and prevent harms of ALS. It has also slowed adopting efficiencies and made finding solutions cumbersome.

The RDAC represents enormous value by allowing people with ALS to hear directly from a diverse group of stakeholders interested in identifying and solving pressing challenges. In addition, the RDAC would help relieve some of the burden from the state by expeditiously delivering direct feedback, solutions, and resources with one community voice.



OUR VISION Create a world without ALS
OUR MISSION To discover treatments and a cure for ALS, and to serve, advocate for, and empower people affected by ALS to live their lives to the fullest.

Member National Health Council

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In creating this council, Vermont would join twenty-nine other states that have already enacted similar legislation in support of their rare disease community and proven that the RDAC can be an invaluable resource. Those states include Alabama, California, Colorado, Connecticut, Delaware, Florida, Georgia, Illinois, Indiana, Kentucky, Louisiana, Maine, Maryland, Massachusetts, Michigan, Minnesota, Missouri, Nevada, New Hampshire, New Jersey, New York, North Carolina, Ohio, Pennsylvania, South Carolina, Tennessee, Utah, Virginia, and West Virginia. A Rare Disease advisory would create comparable treatment to consultation with other high cost and high risk groups – notably people with Alzheimer’s, developmental disabilities seeking autonomy, and persons needing attendant care, among others.

Once again, on behalf of The ALS Association and people living with ALS and their families in the state of Vermont, we thank you for considering H.46 that would enable the creation of a Rare Disease Advisory Council. Please support this legislation to give a voice to Vermont residents living with rare diseases. For any questions, please feel free to contact Danielle Spadafora with The ALS Association via email at danielle.spadafora@als.org Thank you for your consideration.

Sincerely,

Danielle Spadafora, MPH
Managing Director, Advocacy | The ALS Association

Karin Hammer-Williamson, BASW
Care Services Manager - Vermont

**Additional Statement of Nancy Luke of Bristol, VT
ALS Ambassador – Vermont**

My name is Nancy Luke. ALS, also known as Lou Gehrig’s Disease is a rare progressive degenerative motor neuron disease. As it progresses, the body becomes paralyzed resulting in an inability to walk, talk, swallow, and eventually to breathe. The average life span from diagnosis is generally 2-5 years. There currently is no cure. I was diagnosed with ALS in 2022. It took 4 years to reach a diagnoses, which in turn makes me ineligible for clinical trials. When you are diagnosed with an unusual disease in a small state like ours, it can be difficult to get coordinated information, and find all the resources that might be available to you. With ALS for example; everyone has heard of the Ice Bucket Challenge, but they don’t necessarily understand the disease it supports. I didn’t. I often have to explain whatALS is.

Living with ALS, or any rare terminal disease is scary. You are always wondering what part of your body is going to stop working next. Right now, my speech is deteriorating and I need the assistance of a walker and sometimes a wheelchair to get around. Not fun in snowy Vermont! Just a couple of years ago I was skiing, snowshoeing, hiking, long distance cycling and kayaking. I can no longer do any of those things. Caregivers carry a heavy load that most people cannot comprehend, both physically, mentally, and financially. Many are balancing work as well as caregiving, some have to leave their jobs as they cannot afford in home care. More understanding of what people living with rare diseases face would go a long way in making life a bit easier for families and employers, and enable the general public to be more informed. A Rare Disease Advisory Council would help bring attention to diseases like ALS and give patients and caregivers a voice.

Thank you for considering this legislation and for allowing me to speak today. Most states in the Northeast already have an RDA Council in place, do we want to be left behind? I don’t think so! Let’s show Vermonters that we care and prioritize people living with rare diseases and get this done.



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