

Testimony

H. 573 - An act relating to the Rare Disease Advisory Council

Hello all,

My name is Danelle Lello Birong and I am here today to provide testimony in regards to **H. 573 - an act relating to the Rare Disease Advisory Council (RDAC)** as I am a proponent of Vermont moving forward with this bill.

As you already know, 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), also known as Lou Gehrigs disease, and this is why I am here today.

ALS is a fatal progressive neurodegenerative disease that slowly robs a person's ability to walk, talk, eat, and eventually breathe.

Unfortunately, I witnessed my father lose every single one of those faculties over the course of 11 months.

My father, a Pennsylvania resident, was diagnosed with Bulbar-onset ALS in June 2022 at the age of 74 after 6 months of every possible test, scan, etc, meeting with a variety of medical specialists.

If you are not aware, please note that there is no specific test to determine if one has ALS. Instead, it is a progression of ruling out every other possibility which may lead to this final, terminal diagnosis. According to the ALS Association, it takes an average of 9 to 12 months to be diagnosed with ALS after the first symptoms appear. The average life expectancy is 2-5 years.

For my dad, the first symptom that my mom noticed was his slurring of words on Christmas in 2021. I have never prayed more for a stroke, hoping that's what it was, than I did in those following months. In June, when he was issued his diagnosis, he was still golfing regularly, playing with his grandchildren, and dancing every chance he had. By December of that year, he was using a walker, received a feeding tube as he could no longer swallow, and could barely speak. Fortunately, he still had mobility in his fingers, as we began to only communicate via Text to Speech apps on his phone. April 2022 was his first, last, and only hospitalization due to aspiration pneumonia. 7 weeks later, he passed away at the local VA hospital after spending 4 weeks in hospice.

Luckily, which is a weird thing to say when discussing a terminal illness, my father was a veteran of the Vietnam War, and since ALS is deemed a service-related illness, and the VA hospital being less than 10 miles from his home, it allowed him to take advantage of nearby medical

professionals at no cost, but the process was still quite difficult due to the lack of knowledge and research surrounding ALS.

For example, the morning when I called 911 as my father's oxygen level was 72 and he was struggling to breathe, the EMTs immediately gave him oxygen as well as the first ER doctor we met, not understanding that he was not able to expel the CO2 on his own due to respiratory muscle weakness. After advocating for him multiple times and an eventual visit from the pulmonologist, they finally put him on a non-invasive ventilation system as this an essential therapeutic component in ALS.

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, necessary equipment, and care.

I understand that 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 this Council in Vermont will raise awareness and give rare disease patients a unified voice in VT's state government. Additionally, the Council 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.

The RDAC represents enormous value to the ALS organization and the community they serve by allowing them 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.

Twenty-seven other states have already enacted similar legislation in support of their rare disease community and proven that the RDAC can be an invaluable resource.

I hope that VT chooses to moves forward with the creation of a **Rare Disease Advisory Council**.

Thank you for your time.

Danelle Lello Birong
Vergennes, Vermont