

February 27, 2025

Support for the Rare Disease Advisory Council (RDAC) Bill H.46 in Vermont

Good morning. My name is Sarah Elliott, and I am a constituent from Glover, Vermont. I am here to ask for your support in establishing a rare disease advisory council (RDAC) in Vermont. A rare disease is defined by federal law (the Orphan Drug Act) as a condition affecting fewer than 200,000 people in the US. I am a patient with a rare disorder and have a variation of skeletal dysplasia- also known as dwarfism.

Dwarfism is not something to be cured or fixed, but it is important to understand there are medical needs and extensive management that goes into this diagnosis. Babies with dwarfism are often born to parents of average height, which was the situation for my parents. There was no history of dwarfism in my family, and in the late 1980's my mom had a healthy pregnancy and did not learn of my condition until after I was born. I was misdiagnosed- still with dwarfism, just a different type, and 25 years later began to work with a genetics team to find out my actual diagnosis.

I had not met any other individuals with dwarfism until after I was an adult and learned there are few medical experts in the U.S. and limited information where it is available. One expert is in New York City, which is challenging to access, but something I was fortunate to be able to do last fall. It is important to note there are upwards of 700 different variations of skeletal dysplasia, and everyone's individual needs are going to be unique, which is why dwarfism organizations would be able to work with RDAC's to support improvements on healthcare policies that concern dwarfism.

I have seen improvements to Vermont health care policy- not specifically aimed towards individuals with dwarfism per say, but 14 years ago I had back to back hip replacements and during the second one my pelvis was broken by accident. This changed the course of rehab for the hip, and it took well over a year to recover. At the time, most patients on Medicaid were given 30 physical therapy visits per year. I struggled with my hip and ended up needing more than 30 visits. I remember being frustrated I could not possibly get more, and was told there were exceptions, but only for patients with traumatic brain injuries. I had to pay out of pocket for visits- but was also unemployed as I had just graduated college 2 months prior to having the joint replacements, and had not had time or ability to find employment, and was ineligible for disability benefits at that time. For the last 14 years I have struggled with that same hip, often ending up in physical therapy again and again; always mindful that I only had 30 visits per year. In early 2023 I learned I had been moved to a different plan- and now was given unlimited physical therapy visits. It was such a relief to hear because physical therapy is one of the most important tools of managing chronic pain, joint contractures and stiffness that results from the dwarfism I have.

Vermont RDAC bill H.46 will create the RDAC- an advisory body providing a platform for the rare community to have a stronger voice in their state government- within the Vermont Department of Health, and advise lawmakers and agencies on rare disease issues, which ensures patients, caregivers, healthcare providers, researchers and policy experts can help shape better care and support. Through annual reports, public hearings, research and policy recommendations, there would be improvements to diagnosis and treatment for patients with rare disorders. There are currently thirty states with RDACs, and ten states considering RDAC bills in 2025.

I respectfully ask for your support in the creation of H.46 ensuring the voices of Vermonter's with rare diseases are able to be heard in state health policy. Thank you for your time and consideration.