Dear Committee,

I submit this testimony for review and urge you to support H.46 and the crucial need here in Vermont for a Rare Disease Advisory Council.

For those Vermonters and caregivers navigating accessing medical professionals for rare disease diagnoses, treatment and care coordination it can be a matter of life or death.

As a caregiver to a spouse with Amyotrophic Lateral Sclerosis (ALS), I would like to share our experience and why for the most part we have sought care outside of Vermont as we navigate this disease because of ease of access to providers and clinical trials.

We began seeking a diagnosis for my husband in March of 2025 when symptoms of slurred speech and upper body weakness began, at our local hospital, by June of 2025 we were told to contact both UVM and Dartmouth to try and be seen by a neurologist specializing in ALS as soon as possible. After pulling some strings were able to been seen by UVM in early July of 2025 and had our first ALS diagnosis by the end of July. From there we received a confirmed diagnosis at Mass General Hospital (MGH) in Boston, MA in mid-August 2025. My husband was confirmed to have bulbar onset ALS which is the quickest progressing form of ALS.

UVM does have an ALS clinic and we had an appointment there at the end of August 2025 but also chose to be seen dually at MGH in order to have access to clinical trials, to give my husband any means possible to prolong his life expectancy of 2-4 years. And it is a good thing we made the decision to be seen dually due to access to care.

Due to scheduling conflicts by husband has not been able to be seen at the UVM ALS clinic since that appointment in August 2025, which I assume is due to the number of ALS cases here in VT and small number of providers givining care to these patients through UVM. Typically ALS patients are seen at "clinic" every 2-3 months.

I was shocked to hear at the UVM ALS clinic appointment in August of 2025 that Vermont per capita has the most ALS cases out of any state in the U.S. and yet anytime I had had to reschedule an appointment for him here in VT his appointment was pushed out another 2-3 months versus at MGH if you need to rescehdule an appointment you can be seen within 30 days if not sooner. Which makes me wonder why access to care for ALS is so difficult and not readily available in our state.

Since my husband has received his diagnosis, he is very close to no longer having the ability to speak clearly anymore, he no longer can use his arms/hands and now the ALS weakness has impacted his lower body so can no longer walk unassisted. All of this taking place in less than 12 months and yet he had not been able to be seen "locally" in Vermont since August of 2025. I'm not sure what our family would have done if we were not also being seen at MGH.

Because he has been seen at MGH, he has been able to participate in a clinical trial and is looking at possiblity participating in another or having access to "off label" medications being testing to slow ALS progression. He has been able to work with Boston Children's Hospital and receive adaptive communication equipment to allow him to communicate again and through an ALS organization outside of VT received a loaner power travel chair so he can attend appointments and events easily due to his continuing decline in mobility. As I writing this I am now trying to get him an appointment at a local wheel chair clinic where my husband can be evaluated for a power wheel chair because some chairs (depending on how complex) can take 2-6 months to arrive once approved by insurance. And I will be honest that by the end of the summer he may no longer be able to walk anymore with how his symptoms are progressing.

So why is an advisory council for rare diseases so needed here in Vermont?? Not all individuals impacted by ALS or any other disease here in Vermont are in the position my family is in to have the means and ability to travel to Boston. This disease, as I am sure all rare diseases are not all the same and impacts every individual differently....never in a million years did I think that when my husband received his ALS diagnosis that he would be 100% dependent on me to care for him and unable to speak clearly within 8 months. Vermonters need and deserve to be able to obtain medical care in this state. They deserve to have resources readily available because life change change in an instant.

Thank you,
Miranda Pudvah Cloutier