Memorandum of SUPPORT

VT: H.46 (Stone)

Rare Disease Advisory Council (RDAC)

The National Organization for Rare Disorders (NORD) is proud to support H.46, which will establish Vermont's Rare Disease Advisory Council (RDAC) and thanks the sponsors for their commitment to addressing the unique challenges faced by Vermonters living with diseases, their families, and caregivers.

Background

A rare disease is defined federally in the Orphan Drug Act as any condition affecting 200,000 or fewer Americans. Individuals living with rare diseases often encounter delayed diagnoses, limited treatment options, and barriers to accessing specialized care. Due to small patient populations and the variety of rare diseases, it can be difficult for state government officials 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.

Rare Disease Advisory Councils currently serve 32 other states by connecting patients, caregivers, and other healthcare experts to focus on the exchange and development of knowledge and recommendations that improve the lives of those with rare diseases. This can range from helping to create databases on research, treatments, and treatment providers to helping develop public policies and support services which enable people with rare diseases to live healthier lives.

Currently, there are thirty-three states that have created legislation in support of their rare disease community and proven that the RDAC can be an invaluable resource. Council responsibilities vary by state and are tailored to meet state and local needs. Of the states with RDACs nationwide, 30 have established their councils through an act of the Legislature because statutory authority provides the strongest foundation for sustained impact and long-term stability.

NORD On the Bill

NORD would like to draw attention to and share our most ardent support for the following provisions of the bill;

1. **Reserved Seat for an Older Vermonter with a Rare Disease**
   The council includes two patient seats, on par with other councils nationwide. Unique to Vermont's RDAC-establishing legislation, is a seat reserved for an "older Vermonter." This is truly reflective of community priorities and ensures that the perspectives of aging patients facing rare diseases are represented in policy discussions.

2. **Member Seat for a Geneticist or Genetic Counselor**
   This is a growing membership seat trend in councils established in the last 2-3 years, especially as the cell and gene therapy space innovates. This ensures critical expertise is not missing, given almost 80% of rare diseases are genetic.

Get to Know the Facts About Rare Disease

1 in 10 Americans
have a rare disease.[1]

That is more than
30 million
of our loved ones, friends, and colleagues.[1]

There are more than
10,000
known rare diseases. Fewer than 5% have an FDA-approved treatment.[2]

Rare disease medical costs are
3–5x higher
than non-rare, totaling an estimated
$400B
annually—on par with cancer and Alzheimer's.[3]

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3. **The Council's Advisory Role to the State Newborn Screening Program**
   The Council will develop recommendations to help identify conditions to recommend to the Newborn Screening Advisory Committee as part of Vermont's Newborn Screening Program. This is especially salient given the dissolution of the federal Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC), leaving a real gap for state governments to fill with in-state expertise and cross-state collaborations.

4. **Empowering the Council with Flexibility to Identify Additional Priorities**
   The Council must meet quarterly as they work to address their prescribed duties. A notable provision of the bill is a duty that empowers the Council or the Department with the flexibility to identify additional priorities upon a majority vote of members. This will ensure the Council adapts alongside the ever-changing healthcare landscape.

5. **Providing Testimony on Pending Legislation and Rules**
   The Council is explicitly empowered to provide testimony and comments on pending legislation and rules that impact Vermont's rare disease community before the General Assembly and other State agencies. This explicit directive to engage in the legislative process will empower the Council to be a strong advisor to lawmakers and state government officials, ensuring the rare disease patient voice and experience informs policymaking.

By passing H.46, Vermont will join its New England neighbors—Connecticut, Maine, Massachusetts, New Hampshire, and Rhode Island—as well as 28 other states that have given their rare disease communities a stronger voice in state government. The bill's thoughtful membership structure, emphasis on equity through its older Vermonter seat, and integration with the Department of Health positions the Council to make meaningful, lasting impact.

A strong RDAC can help relieve burdens on the state by expeditiously delivering direct feedback, solutions, and resources to Vermont government decision makers with one community voice. Without an RDAC, Vermont laws and regulations that affect the rare community will be developed without the consultation of rare disease stakeholders, leaving this vulnerable population at greater risk for poor health and economic outcomes. NORD strongly urges the passage of H.46 to establish Vermont's Rare Disease Advisory Council.

With questions, please contact Carolyn Sheridan, Associate Director of State Policy at the National Organization for Rare Disorders (NORD) at csheridan@rarediseases.org.

About the National Organization for Rare Disorders

Founded in 1983, the National Organization for Rare Disorders (NORD®) is a leading independent, nonpartisan, nonprofit and patient advocacy organization dedicated to improving the health and lives of over 30 million Americans living with rare diseases. In partnership with more than 350 disease-specific member patient organizations, NORD drives progress in rare disease research, care, and policy.

Learn more at rarediseases.org

Sources:

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[2] ojrd.biomedcentral.com/articles/10.1186/s13023-023-02790-7
[3] ojrd.biomedcentral.com/articles/10.1186/s13023-021-02061-3