



April 7, 2021

The Honorable Ned Claxton
Chair
Joint Health and Human Services Committee
72 Danbury Drive
Auburn, ME 04210

The Honorable Michele Meyer
Chair
Joint Health and Human Services Committee
2 State House Station
Augusta, ME 04333

Re: Support for Legislative Document 972

Dear Chairman Claxton, Chairwoman Meyer and Members of the Joint Health and Human Services Committee:

On behalf of the 1-in-10 individuals in Maine with one of the approximately 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks you for putting Legislative Document 972 (LD 972) on the Joint Health and Human Services Committee's agenda. LD 972 establishes a Rare Disease Advisory Council (RDAC), which if passed, would help to give a voice to the rare disease community within Maine's state government.

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. We are committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

Rare diseases are present across a broad spectrum of medical conditions. For example, there are more than 500 types of rare cancers and all forms of pediatric cancer are rare. For patients suffering from these and numerous other rare conditions, it can take several years to receive an accurate diagnosis and effective treatment. Further, only a handful of rare diseases are well-understood, with most not receiving sufficient attention or funding for research.

A Rare Disease Advisory Council in Maine would provide a forum for stakeholders across the state to analyze the needs of the rare disease community and make recommendations on how to improve public policy.

We are grateful to Representative Craven for her introduction of LD 972. LD 972 will accomplish critical goals of an RDAC, including increasing awareness of the burden caused by rare diseases and requiring the issuance of an annual report that includes a summary of the activities of the council and any legislative or policy recommendations of the council. However, we would recommend the following modifications to the legislation:



1. First, increase the diversity of membership within the council. We recommend adding a representative from an academic research institution, a representative from MaineCare, a representative from the Bureau of Insurance, a geneticist practicing in the state, a representative from biopharma, and a representative of a health plan operating in the state. Including broad participation from the different health care sectors operating in Maine will help the RDAC serve as an educational resource to all stakeholders about the ways rare disease patients interact with our health care system.

2. Second, NORD recommends removing some of the duties that focus on state-based research and instead focus on other duties that can benefit the Maine rare disease community at large. Some state RDACs that emphasized research from the outset have struggled to get comprehensive data, which resulted in delays in the development and delivery of recommendations that could help rare disease patients. Additionally, data around incidence, causes and economic burden of rare diseases can already be found through the National Institutes of Health and other rare or disease specific research and advocacy organizations, including NORD. Therefore, we'd encourage the Legislature to instead require the Council to convene public hearings and solicit comments from the general public to help set the course for issues a newly implemented RDAC can work on.

3. Third, we would also recommend the Council be tasked with making recommendations on key public policy issues and distributing educational resources for patients and health care providers. This can be accomplished by publishing a list of publicly accessible resources for health care providers to assist them with recognizing and optimizing the treatment of rare diseases, as well as resources for the public on research, diagnosis, treatment, and education in Maine on the Council's website. Additionally, we'd recommend that the Council be clearly tasked with making recommendations to state agencies and insurers on issues that impact Maine's rare disease community to ensure that their concerns are consistently being heard and addressed by decision makers.

With these modifications, the RDAC established through LD 972 will provide decision-makers in Maine with a forum to better understand and meet the needs of their constituents.

In creating this council, Maine will be joining seventeen other states (Alabama, Connecticut, Illinois, Kentucky, Massachusetts, Minnesota, Missouri, New Hampshire, New York, Nevada, North Carolina, Ohio, Pennsylvania, Tennessee, Utah, Virginia and West Virginia) who have already enacted similar legislation in support of their rare disease community.

Establishing an RDAC in Maine will give rare disease patients a unified voice in Maine's state government, helping to provide educational awareness and make recommendations to elected officials and other state leaders on how rare diseases are handled and treated in the state. From providing information on the diagnostic journey, to making recommendations on state programs such as newborn screening, the council will serve as a tremendous opportunity for important decision-makers in Maine to better understand and meet the needs of their constituents.



Once again, thank you for adding LD 972 to the Joint Health and Human Services Committee's agenda. We urge its swift passage as amended to reflect NORD's suggested modifications.

Sincerely,

Heidi Ross
Director of Policy
National Organization for Rare Disorders

Annissa Reed
State Policy Manager, Eastern Region
National Organization for Rare Disorders

CC: Members of the Joint Health and Human Services Committee



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Dear Chairman Claxton, Chairwoman Meyer and Members of the Joint Health and Human Services Committee:

My name is Kevin Dickinson, and I am the Maine Rare Action Network Volunteer State Ambassador for the National Organization for Rare Disorders. Thank you for the opportunity to testify before your committee in support of creating a Rare Disease Advisory Council in Maine.

In 2014, I was blessed with the arrival of my youngest daughter, Kenzie. At first, everything seemed normal, but as the months went on, it became evident that Kenzie was falling behind and not meeting her appropriate developmental milestones. First, we were told that she is not out of the ordinary, she just may need more time to develop. Until she was eventually referred to a neurologist who started genetic testing. Everything was getting ruled out and checked off the list. Until finally this neurologist, ordered an MRI reluctantly. She immediately pulled us aside after seeing the imaging. The doctor told us the good news, Kenzie did not have cancer and the bad news, has a mitochondrial disease known as Leigh's Disease, and she will not make it out of her single years most likely. This was our first misdiagnosis.

Our story is not that unique amongst the rare disease world. Many families and patients go through numerous misdiagnoses and testing before finding their answer. Our family started doing our own research and found a team in Boston that takes care of Kenzie to this day. They found that she has an ultra-rare genetic disorder of the VPS13D gene. So, it is an unnamed disorder, initially one of six known cases in the world, which today Kenzie is one of twenty known cases.

Insurance will cover most claims, but this disorder is unnamed and does not exist under any claim codes as a diagnosis. This makes covering some equipment and medications extremely difficult.

A Rare Disease Advisory Council in Maine is so important to help remove barriers that exist in not only getting treatment, therapies, medications, and most importantly help for those dealing with Rare Disease, but also identifying and diagnosing those with rare disease. This can be extremely challenging in our state, from the northern rural areas of Aroostook County, where I was born and raised, down our rugged coastline and forested interior, to the more populous southern counties where I live today.



Today, Kenzie is 6 years old, and still, we encounter a lot of barriers in her care. An RDAC in Maine, will be fundamental in starting the process of change for the better, to recognize that the rare disease community exists, and tackle the barriers so many of us have faced.

As Maine's Volunteer State Ambassador, I have been able to help families firsthand, to welcome and ease them into the Rare Disease world. An RDAC will bring the needs of the rare disease community in Maine front and center, to help address and overcome the barriers we face as a community.

Sincerely,

Kevin Dickinson
Maine Volunteer State Ambassador
Maine Rare Action Network

CC: Members of the Joint Health and Human Services Committee