



February 4, 2021

The Honorable Manny Diaz Jr.
Chair, Senate Health Policy Committee
Florida Senate
10001 Northwest 87th Avenue
Hialeah Gardens, FL 33016

Re: Support for Senate Bill 272

Dear Chairman Diaz and Members of the Senate Health Policy Committee:

On behalf of the 1-in-10 individuals in Florida with one of the approximately 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks you for putting Senate Bill 272(SB272) on the Senate Health Policy Committee's agenda. SB272 establishes a Rare Disease Advisory Council (RDAC), which if passed, would help to give a voice to the rare disease community within Florida's state government. NORD urges you to vote in support of SB272 and swiftly move it out of your committee.

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.

Creating an RDAC in Florida will give rare disease patients a unified voice in Florida'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 Florida to better understand and meet the needs of their constituents. Since

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this council would include broad participation from the different health care sectors present in Florida, it will also serve as an educational resource to all stakeholders about the ways rare disease patients interact with our health care system.

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

Once again, on behalf of the Florida rare disease community, we thank you for putting SB272 on the Senate Health Policy Committee's agenda and urge its swift passage. For any questions, please feel free to contact Annissa Reed via email at areed@rarediseases.org. Thank you for your consideration.

Sincerely,

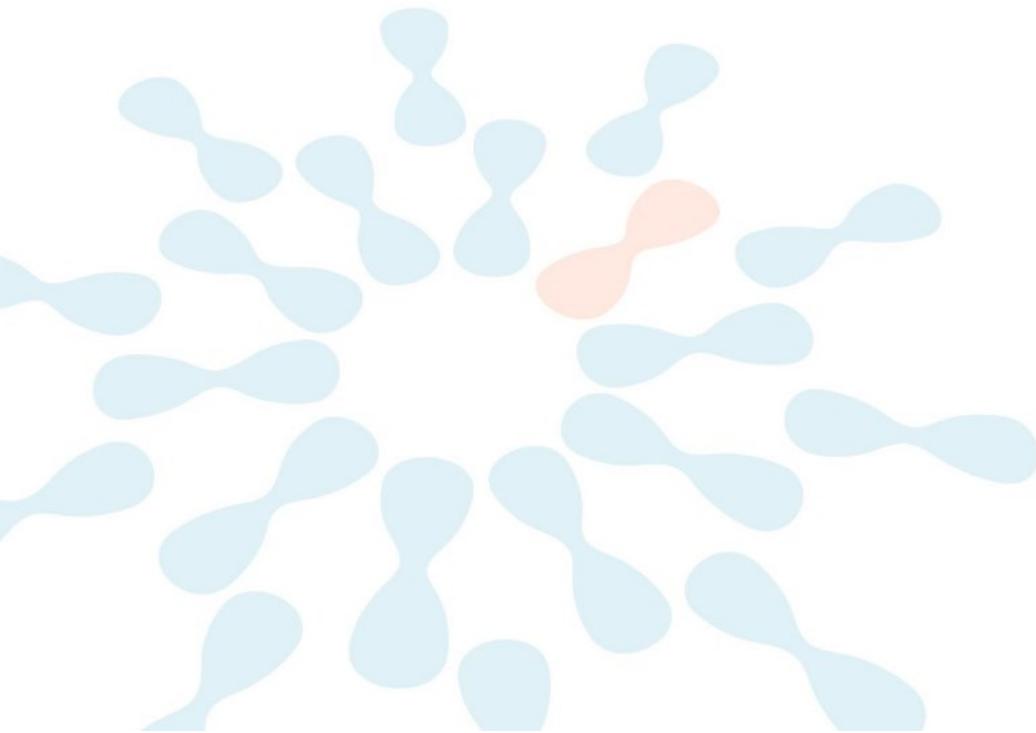


Heidi Ross
Director of Policy
National Organization for Rare Disorders



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

CC: Members of the Senate Health Policy Committee





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Dear Chairman Diaz and Senate Health Policy Committee Members:

My name is Anne Rugari, and I am the Florida Rare Action Network Volunteer State Co-Ambassador for the National Organization for Rare Disorders. NORD is a unique federation of voluntary health organizations dedicated to helping the 25-30 million Americans living with a rare disease.

As a rare disease advocate, parent, and caregiver, I have had many opportunities to not only work with the rare disease community, but to also hear the voices of many people who are affected with a rare disorder or disease. It is estimated that One in 10 Floridians is affected by a rare disorder.

A Rare Disease Advisory Council (RDAC) in the State of Florida would address various issues that the rare disease community in Florida experiences, and will work with the Florida Legislature to help improve policies would support the rare disease population in having accessibility to better health care.

My first experience with rare diseases came from having two children born with a rare, genetic, neurologic disorder called Krabbe disease. Krabbe is a demyelinating disease that affects the central and peripheral nervous systems. Children born with this disorder typically die within the first two years of life. My son, Nicholas lived to be a year old. My daughter, Gina, was tested at birth for Krabbe and was able to receive a treatment that helped her to live a much longer and better quality of life than her brother. The many healthcare issues associated with Krabbe, has given me firsthand knowledge of the challenges faced by those in the rare disease community and has prepared me to be a voice and advocate for all of the rare disease community.

Patients, caregivers, family members, physicians, medical support teams, and others have their hands tied and are frustrated in getting the help that they need. It has become increasingly complicated for the rare disease community to receive the support services, prescription drugs, and other medical treatments, even more so during the COVID-19

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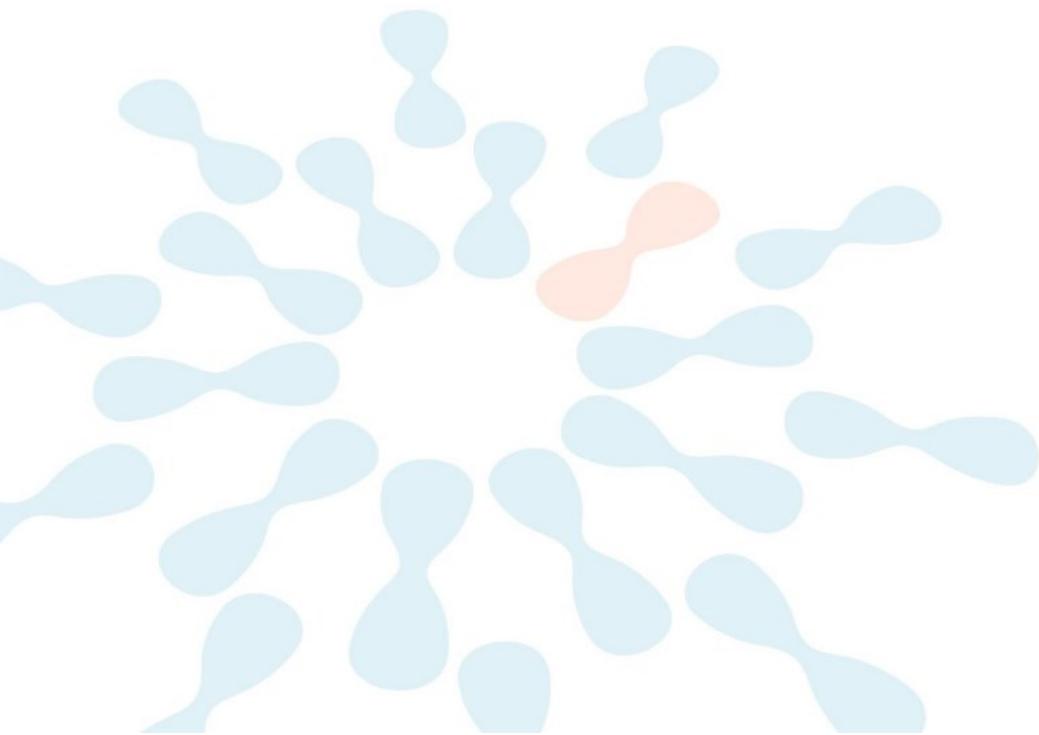
pandemic. The RDAC would work to alleviate some of these healthcare burdens for this vulnerable population.

To date, sixteen states (Alabama, Connecticut, Illinois, Kentucky, Massachusetts, Minnesota, Missouri, New Hampshire, New York, Nevada, North Carolina, Ohio, Pennsylvania, Tennessee, Utah, and West Virginia) have created a Rare Disease Advisory Council and proven that it is an effective way to ensure better government engagement and action on issues of importance to the rare disease community. I urge you to vote in support of Senate Bill 272 and help Florida become the 17th state to enact an RDAC.

Sincerely,

Anne Rugari
Florida Volunteer State Ambassador
Florida Rare Action Network

CC: Members of the Senate Health Policy Committee





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Re: Support for Senate Bill 272

Dear Chairman Diaz and Senate Health Policy Committee Members:

My name is Frances Gingell Hokkanen, and I am the Florida Rare Action Network Volunteer State Community Engagement Liaison for the National Organization for Rare Disorders. NORD is a unique federation of voluntary health organizations dedicated to helping the 25-30 million Americans living with a rare disease. I am also the mother of a daughter with a rare disorder and a cancer survivor.

As someone who lived 30 years in a medically simple life, there is nothing more life altering than hearing hushed whispers in the delivery room that something is wrong with your child. No amount of reading, diligent pre-natal research, or universally shared knowledge about parenthood can prepare you. We skipped the newborn bliss phase when we were told that our daughter needed lifelong, daily medications to survive. Not to mention emergency protocols, medical specialists, therapies, surgery, and regular blood draws. There are the nearly 7,000 different rare diseases, each of which affect only a fraction of the overall population, but this medically complex life is one shared by the estimated one in ten Floridians living with a rare disorder, and their families; we are the rare community. Sadly, 90% of rare disease patients do not have an FDA approved treatment for their disease.

It is my experience, as a Rare Action Network volunteer over the last 6 years, the rare community is often negatively affected by the shortcomings of systems designed to serve the average Floridian. As members of the Committee on Health Policy, it is your duty to make decisions on proposed legislative solutions to meet the health needs of Floridians. It requires insight into issues affecting vulnerable communities who are often disenfranchised simply for being labeled 'rare.' This is where a Rare Disease Advisory Council can be helpful. This legislation requires broad participation from the different health care sectors present in Florida – patients, caregivers, doctors, hospitals, insurers, manufacturers, and researchers are all represented on the council – it will also serve as an educational resource to all stakeholders about the ways in which rare disease patients interact with our health care system.

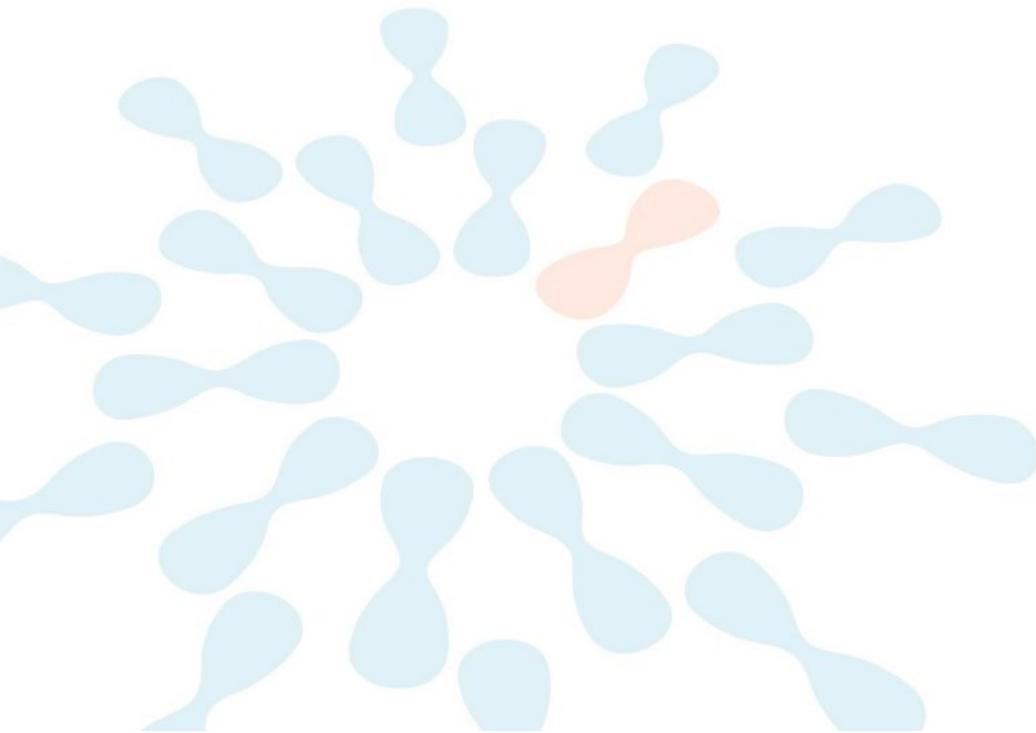
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That is why I urge you to vote in favor of SB 272 to establish a Rare Disease Advisory Council to represent and support the rare community.

Sincerely,

Frances Gingell Hokkanen
Florida Volunteer State Ambassador
Florida Rare Action Network

CC: Members of the Senate Health Policy Committee





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Dear Chairman Diaz and Senate Health Policy Committee Members:

My name is Jhoanny Cardenas, and I am the Florida Rare Action Network Volunteer State Co-Ambassador for the National Organization for Rare Disorders. NORD is a unique federation of voluntary health organizations dedicated to helping 25-30 million American living with a rare disease.

As my younger son, Alexander, entered the first grade, his teachers brought up some behavior changes that they had observed and concerned them.

I began taking my son Alex to the pediatrician to discuss their concerns, which resulted in getting referrals to see other specialists. I was frequently told by these specialists that “there was nothing wrong with your son, he is being a regular six-year-old,” but I was not satisfied with this response. I made additional appointments with a child neurologist and endocrinologist. I was discouraged from doing this because I was told my insurance may not pay as there was no family history, even though I had previously expressed some concerns. It was after several tests ordered by the endocrinologist that on March of 2015 Alexander was diagnosed with Adrenoleukodystrophy (X-ALD). This was a new and scary diagnosis. We understood that time was not on our side. We quickly traveled to Minnesota where Alexander received treatment.

Sadly, Alexander’s diagnosis was not my first connection to the rare disease community, which began at a young age when my younger sister, Luzel, was paralyzed in what we have now been told was due to Gillian-Barre Syndrome. She made a full recovery and lived a “normal” life until seven years ago, when she began to experience debilitating headaches and muscle weakness which at times resembled a stroke. I have supported my sister through research and visiting various specialists throughout the state. Two years ago, she was diagnosed with Dystonia and most recently, Stiff Person Syndrome. I think about the challenges that my sister faces each day with her conditions, and how it impacts her life. I also think about the difference it would have made for our family as we navigated the new diagnosis and all that came along with Alexander’s care. Alexander went from being a healthy and active six-year-old, to very medically complex and fragile in a matter of months. Had he been diagnosed earlier his story may have had a different ending. Just a short seventeen months after diagnosis, Alexander experienced

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complications after two transplants which he was not able to overcome and lost his battle to Adrenoleukodystrophy in October of 2016, at just eight years old.

Navigating any medical diagnosis can be challenging and complicated and a rare disorder add even more complexity. Many of us find ourselves not knowing where to turn and feel alone and without a voice. A RDAC can help guide and encourage all stakeholders to collaborate and help provide recommendations to improve the diagnosis, care and treatment of rare disease patients. I urge you to vote in support of Senate Bill 272 and help Florida become the 17th state to enact an RDAC.

Sincerely,

Jhoanny Cardenas
Florida Volunteer Community Engagement Liaison
Florida Rare Action Network

CC: Members of the Senate Health Policy Committee

