



February 9, 2021

The Honorable Joseph F. Vitale
Chair
Senate Health, Human Services, and Senior Citizens Committee
New Jersey Senate
569 Rahway Ave
Woodbridge, NJ 07095

Re: Support for S2682

Dear Chairman Vitale and Members of the Senate Health, Human Services, and Senior Citizens Committee:

On behalf of the 1-in-10 individuals in New Jersey with one of the approximately 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks you for putting Senate Bill 2682 on the Senate Health, Human Services, and Senior Citizen Committee's agenda. S2682 establishes a Rare Disease Advisory Council (RDAC), which if passed, would help to give a voice to the rare disease community within New Jersey's state government. NORD urges you to vote in support of S2682 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 New Jersey will give rare disease patients a unified voice in New Jersey'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

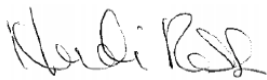
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programs such as newborn screening, the council will serve as a tremendous opportunity for important decision-makers in New Jersey to better understand and meet the needs of their constituents. Since this council would include broad participation from the different health care sectors present in New Jersey, it will also serve as an education resource to all stakeholders about the ways rare disease patients interact with our health care system.

In creating this council, New Jersey 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 New Jersey rare disease community, we thank you for putting S2682 on the Senate, Health, Human Services, and Senior Citizens 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,

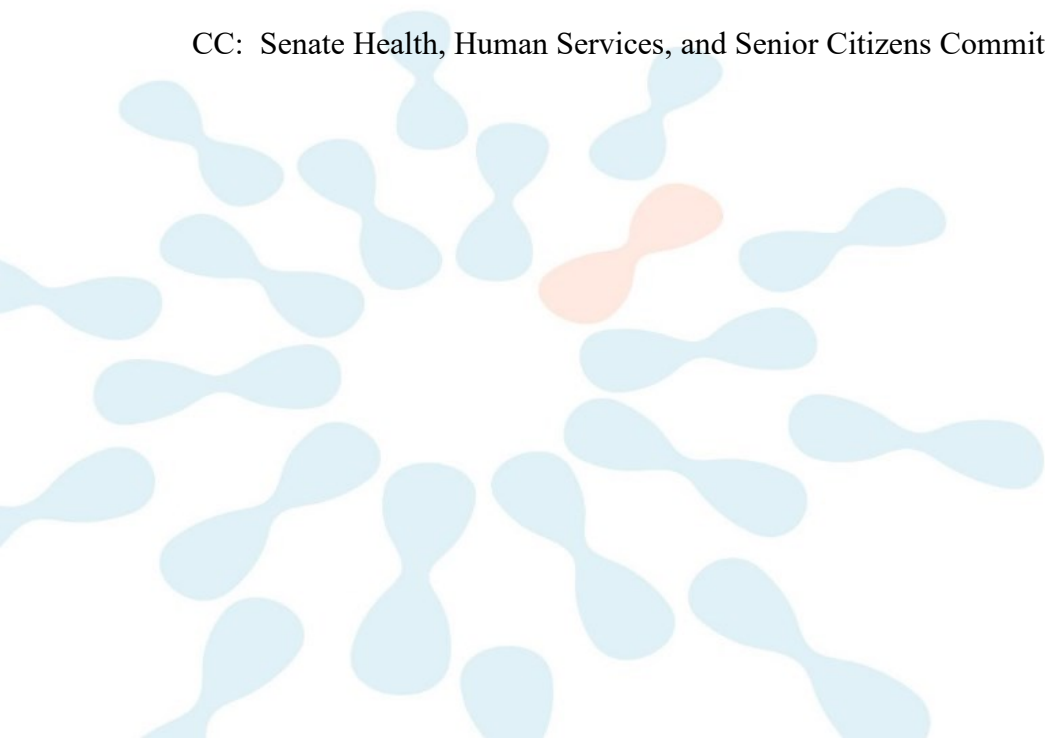


Heidi Ross
Director of Policy
National Organization for Rare Disorders



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

CC: Senate Health, Human Services, and Senior Citizens Committee





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Re: Support for S2682

Dear Chairman Vitale and Members of the Senate Health, Human Services, and Senior Citizens Committee:

My name is Julie Raskin of Glen Ridge, NJ and I am the New Jersey Rare Action Volunteer State Ambassador for 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 honored to be here today on behalf of the one in ten New Jersey residents with a rare disease to share my support for S2682 which would establish a rare disease advisory council in New Jersey.

Rare disease patients in New Jersey face unique challenges every day, from obtaining an accurate diagnosis and accessing medical specialists with knowledge of their condition, to battling for fair insurance coverage of their treatment and care.

My son was born with the rare disease, congenital hyperinsulinism, twenty-four years ago. This condition, often considered the opposite of diabetes, can cause brain damage and death if not diagnosed and treated in a timely manner. For those with severe disease who survive, health management remains complicated for life.

There is no medication to treat the condition my son was born with and he had his pancreas removed, which causes other lifelong illnesses, in order to keep him alive. He is not alone; it is estimated that 90% of people with rare diseases also do not have an FDA-approved treatment for their condition. He also lives with irreversible disabilities because there was not a newborn screening test for his rare disease, which is the case for millions of others with rare diseases. His condition led me to join with others in New Jersey, across the US, and around the world to found Congenital Hyperinsulinism International, to foster research for better treatments and a cure, to raise awareness of the condition to prevent death and brain damage, and to support families every step of the way.



The New Jersey Rare Disease Advisory Council would help address the challenges faced by rare disease patients, their families and their caregivers by providing 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.

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 S2682 and help New Jersey become the 17th state to enact an RDAC.

Sincerely,

A handwritten signature in blue ink that reads "Julie Raskin".

Julie Raskin
New Jersey Volunteer State Ambassador
New Jersey Rare Action Network

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Re: Support for S2682

Dear Chairman Vitale and Members of the Senate Health, Human Services, and Senior Citizens Committee:

My name is Pat Geurds. I am the New Jersey Volunteer Community Engagement Liaison with the National Organization for Rare Disorders' Rare Action Network.

I am also the mother of 5, an author, and a former second grade teacher. My youngest daughter, Kinsley, has been very ill since she was two months old. Her disease is so rare that she has still not been diagnosed. She has had over 50 surgeries and procedures and has spent a third of her life in the hospital.

Sadly, Kinsley's experience with a rare disease, and our experience as her family caring for a loved one with a rare disease, is not unusual. A recent survey conducted by NORD found for 28% of respondents, it took seven or more years for them or their loved one to get an accurate diagnosis and 50% of patients and caregivers attributed diagnostic delays to a lack of disease awareness.

When we first received the news, resources were limited and most services and support we discovered was through friends or doctors. As a result, over the past 7 years, I have connected with an incredible group of doctors, nurses, teachers, pharmacists, therapists, and equipment devices in order to give Kinsley all the best.

It took many hours and research to achieve this. Originally, I was isolated and scared not knowing how to help her. Still, so many affected by rare diseases feel like I once did. They don't know where to turn to get the help they need, let alone advocate for the awareness and policies they need to help themselves for their loved ones thrive.



The Rare Disease Advisory Council will provide rare disease patients and their families an avenue to share their experiences and work with elected officials, health care providers and other experts to improve the outcomes for rare disease patients living in New Jersey, like my daughter Kinsley.

Thank you for the opportunity to testify before your committee today and urge your support for S2682 when it comes before the Committee for a vote.

Sincerely,

Pat Geurds

Pat Geurds
New Jersey Volunteer Community Engagement Liaison
New Jersey Rare Action Network

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