



May 20, 2021

The Honorable Craig J. Coughlin
Speaker of the General Assembly
New Jersey General Assembly
569 Rahway Ave.
Woodbridge, NJ 07095

RE: Support for Assembly Bill 4016-New Jersey Rare Disease Advisory Council

Dear Speaker Coughlin and Members of the New Jersey General Assembly:

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 Assembly Bill 4016 (A4016) on the General Assembly's agenda for consideration. A4016 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. In March, the New Jersey Senate unanimously passed Senate Bill 2682, which is identical to A4016. NORD urges you support A4016 and swiftly pass it out of the General Assembly so it can be sent to Governor Murphy for his signature.

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 will give rare disease patients a unified voice in New Jersey's state government, helping to make recommendations to elected officials and other state leaders on how rare diseases are handled and treated in the state. 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 educational resource to all stakeholders about the ways rare disease patients interact with our health care system.

In creating this council, New Jersey would join seventeen other states that have already enacted similar legislation in support of their rare disease community and proven that the RDAC can be



an invaluable resource. Those states are Alabama, Connecticut, Illinois, Kentucky, Massachusetts, Minnesota, Missouri, New Hampshire, New York, Nevada, North Carolina, Ohio, Pennsylvania, Tennessee, Utah, Virginia, and West Virginia.

Once again, on behalf of the New Jersey rare disease community, we thank you for putting A4016 on the General Assembly'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 New Jersey General Assembly



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RE: Support for Assembly Bill 4016-New Jersey Rare Disease Advisory Council

Dear Speaker Coughlin and Members of the New Jersey General Assembly:

My name is Julie Raskin of Glen Ridge, NJ and I am the New Jersey Rare Action Volunteer State 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.

On behalf of the one in ten New Jersey residents with a rare disease, I am honored to share my support for Assembly Bill 4016 (A4016) which would establish a Rare Disease Advisory Council (RDAC) 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, seventeen states (Alabama, Connecticut, Illinois, Kentucky, Massachusetts, Minnesota, Missouri, New Hampshire, New York, Nevada, North Carolina, Ohio, Pennsylvania, Tennessee, Utah, Virginia and West Virginia) have passed similar legislation 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 A4016, and help New Jersey become the 18th state to enact an RDAC.

Sincerely,

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

CC: Members of the New Jersey General Assembly