



May 13, 2021

The Honorable Bronna Kahle
Chair
House Health Policy Committee
Anderson House Office Building
N-991 House Office Building
Lansing, MI 48933

RE: Support for House Bill 4654-Michigan Rare Disease Advisory Council

Dear Chair Kahle and Members of the House Health Policy Committee:

On behalf of the 1-in-10 individuals in Michigan with one of the approximately 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks you for putting House Bill 4654 (HB 4654) on the House Health Policy Committee's agenda for consideration. HB 4654 establishes clear membership and duties for a Rare Disease Advisory Council (RDAC), which if passed, would help to give a voice to the rare disease community within Michigan's state government. NORD urges you to vote in support of HB 4654 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.

Last year, the Michigan Legislature approved, as part of the FY 2021 General Omnibus budget, a \$70,000 appropriation for the establishment of a rare disease review committee in the state. Rep. Clemente's legislation, HB 4654, is an opportunity for the Legislature to provide additional direction for how the Council should be implemented and the funds should be ultimately utilized.

As outlined in HB 4654, the RDAC will act as the advisory body on rare diseases to the Michigan Legislature and state departments. Additionally, the RDAC will be tasked with: 1) convening public hearings, making inquiries, and soliciting comments from the general public; 2) consulting with experts to develop policy recommendations; 3) researching and identifying policy priorities related to treatment and services; 4) identifying best practices that



could improve the education, care, and treatment of adults and minors with rare diseases in the state; and 5) coordinating with other rare disease advisory bodies, community-based organizations, and other public and private organizations.

In creating this council, Michigan 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 Michigan rare disease community, we thank you for putting HB 4654 on the House Health Policy Committee's agenda and urge its swift passage. For any questions, please feel free to contact Annessa Reed via email at areed@rarediseases.org. Thank you for your consideration.

Sincerely,

Heidi Ross
Director of Policy
National Organization for Rare Disorders

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

CC: Members of the House Health Policy Committee



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RE: Support for House Bill 4654-Michigan Rare Disease Advisory Council

Dear Chair Kahle and Members of the House Health Policy Committee:

My name is Laura Bonnell and I am the Michigan Rare Action Network Volunteer State Ambassador for the National Organization for Rare Disorders (NORD). Thank you for the opportunity to testify before your Committee in support of creating a Rare Disease Advisory Council in Michigan.

I am also the mother of two college age daughters with cystic fibrosis, a genetic disease that causes chronic and fatal lung infections and interferes with digestion. The average life expectancy for someone born today with cystic fibrosis is 47 years old. Although there is no cure, medications are being developed that can correct the underlying condition of the disease. This gives patients a chance at a longer life with a chronic illness. In 2010, I founded The Bonnell Foundation. Our organization supports patients living with cystic fibrosis and provides financial assistance for lung transplant grants, college scholarships, and medical assistance.

Cystic fibrosis is just one of more than 7,000 currently known rare diseases impacting the lives of more than 25 million Americans and their families. We are not just one voice, but we combine our voices and are stronger together. Patients with rare diseases face challenges daily, including obstacles around diagnosis, a lack of treatment options, and high out-of-pocket costs. The thousands of complex disorders also make it difficult for state policymakers and government officials to have an in-depth understanding of the rare disease community.

My daughters, Molly and Emily, have been personally impacted by the misunderstanding of rare diseases and the lack of patient protections. My daughters struggled to access coverage for the lifesaving treatment they needed to live. When the FDA approved a cystic fibrosis modulator drug, we couldn't get it until we educated the insurance company and made sure it was added to the formulary. There is a situation right now where an insurance company has decided to only pay for one brand of enzymes. This would cause my daughters extensive pain each time they eat because we spent years finding the enzymes that work best for them and now the insurance company has decided that it will no longer cover it without jumping through many hoops to try and get it covered.



In 2015, the first state advisory council on rare diseases was created in North Carolina by patients, caregivers, families and providers. A “Rare Disease Advisory Council” or “RDAC” is an advisory body providing a platform for the rare community to have a stronger voice in state government. RDACs address the needs of rare patients and families by giving stakeholders an opportunity to make recommendations to state leaders on critical issues that the rare disease community faces including the need for increased awareness, diagnostic tools and access to affordable treatments and cures.

We hope Michigan will join the seventeen other states that have passed similar legislation to help patients with rare diseases.

Sincerely,

Laura Bonnell
Michigan Volunteer State Ambassador
Michigan Rare Action Network

CC: Members of the House Health Policy Committee