



Alone we are rare. Together we are strong.®

Written Testimony Submitted to the New York State Legislature

Joint Legislative Public Hearing on the FY25
Executive Budget Proposal
Health/Medicaid and Higher Education

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Presented by:

National Organization for Rare Disorders®

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Senate Finance Chair Krueger, Assembly Ways & Means Chair Weinstein, Senate Health Chair Rivera, Assembly Health Chair Paulin, Senate Higher Education Chair Stavisky, Assembly Higher Education Chair Fahy & distinguished Members of the New York State Senate and Assembly,

Thank you for the opportunity to submit testimony for this year's Joint Legislative Budget Hearing on FY25 Executive Proposals related to Health and Medicaid.

The National Organization for Rare Disorders® (NORD) is a federation of non-profits and health organizations dedicated to improving the health and wellbeing of people with a rare disease. We have over 330 member organizations which represent patients and caregivers living with one of the over 7,000 known rare diseases. For forty years, we have been at the forefront of advocacy for policies and programs that improve the health and well-being of people in the rare community and work to ensure that the voice of the rare disease patient is front and center when important policy and regulatory decisions are made at both the federal and state levels.

On behalf of the 1-in-10 New Yorkers living with a rare disease and their families, NORD® respectfully submits the following requests:

- Make New York State's Rare Disease Working Group a Permanent Rare Disease Advisory Council (RDAC)
- Support Proposals for New York State to Join Interstate Compacts (HMH Part R)
- Support the \$25M in Proposed Investments into Rare Disease Research

The Orphan Drug Act defines a rare disease as a disease or condition that affects less than 200,000 people in the United States.

Make New York State's Rare Disease Working Group a Permanent Rare Disease Advisory Council (RDAC)

Through [Project RDAC](#), NORD® is working to establish an effective RDAC in every state to help address the complex needs of the rare community at the state level. These councils, enacted in 26 other states across the country, are advisory bodies that give a platform for the rare community to have a stronger voice in state government.

NORD® asks that you ensure that the Executive follows through on the intent to establish "a permanent Rare Disease Advisory Council to develop a statewide approach to combating the effects of these diseases."¹

¹ State of the State 2024, Our New York, Our Future, Governor Kathy Hochul ([p. 137](#))

New York State first established a Rare Disease Advisory Council in 2019, when S4497 (Rivera)/A5762 (Paulin) was signed into law. Pursuant to a Chapter Amendment, the Council was enacted as a temporary working group with a sunset two years following its signing. Legislative actions during the FY24 budget extended the lifecycle for an additional two years thus, the working group has sunset effective December 20, 2023.

The work of New York's Rare Disease working group had only just begun. Due to the State's unprecedented and critical response to the COVID-19 Pandemic, the group did not meet for the first time until March of 2023. New York's rare community has not had the opportunity to see their Council reach its full potential.

In the 2024 State of the State Book, the Executive announced the intent to establish a permanent Rare Disease Advisory Council. However, there was no corresponding Article VII language within the budget bills released on January 16th. We anxiously await the release of the Executive's 30-Day Amendments, with the hope that the proposal to be inserted at that time. If it is included, we ask for your support for the proposal and if it is not, we ask you please hold the Executive accountable to promises made and include language in your One House Resolution making New York's working group permanent.

Support HMH Part R – Enters New York State into Interstate Compacts

Many rare disease patients must travel significant distances, including across state lines, to obtain appropriate specialized medical care for their rare condition.

To increase patient access to physicians with expertise in their rare disease, NORD® supports New York joining the Interstate Medical Licensure Compact.

Currently, New York's licensure laws create barriers to care for rare disease patients by limiting providers ability to see patients located either temporarily or permanently in a different state. Flexibilities enacted during the COVID-19 Public Health Emergency allowed many patients to see providers across state lines through telehealth visits even if the provider was not licensed in the state where the patient was physically located. As pre-pandemic licensure and telehealth restrictions return, however, many rare patients have lost access to these providers due to fragmented medical licensure laws.

The Interstate Medical Licensure Compact (IMLC) is an agreement among participating U.S. states to work together to significantly streamline the licensing process for physicians who want to practice in multiple states. Joining the IMLC will allow New York-based rare disease



specialists, like those working at any one of the four NORD Rare Disease Centers of Excellence², to virtually open their doors to rare patients nationwide who otherwise may not have access to health care providers operating at the top of their licensure. This could potentially cut down the often-extensive “diagnostic journey” patients go through to obtain an accurate diagnosis and find a treatment that works. Currently, 37 other U.S. states, the District of Columbia, and the Territory of Guam are participants in the IMLC. While joining the IMLC will not eliminate fragmented licensure requirements, it would make it much easier for providers to be licensed in states where they seek to treat patients via telehealth.

Support \$25M in Proposed Investments into Rare Disease Research

There are over 7,000 known rare diseases, an estimated 95% of which do not have an FDA-approved treatment. The tremendous unmet needs that exist in the rare disease community has significant economic impacts on rare patients, their families, and the broader health care system so investment in rare research broadly is critical to the rare disease community at large.

As the leading independent advocacy organization working at the intersection of care, research, policy, and community for all rare diseases, NORD® supports broad language for investments into research and treatment of rare disorders.

Research and development into therapies to treat rare diseases is particularly challenging. Rare diseases impact small patient populations that are often geographically dispersed. Limited natural history data and knowledge of disease progression combined with heterogeneous disease presentation mean that clinical trials to determine the safety and effectiveness of potential treatments can be very difficult and expensive to conduct. Investments into research across the entire rare disease community benefits all and helps to foster a successful bench to bedside pathway for rare disease treatments.

NORD® thanks you for providing us with the opportunity to submit testimony for your consideration. We hope you all consider the National Organization for Rare Disorders® a resource for you in the development of policies and programs that will improve the lives of rare disease patients and their families living in New York.

For additional information on any of the issues raised here, please contact Heidi Ross, Vice President of Policy & Regulatory Affairs (hross@rarediseases.org) or Lindsey Viscarra, State Policy Manager (lviscarra@rarediseases.org).

² Centers include: (1) [Columbia University Irving Medical Center](#), (2) [Department of Genetics & Genomic Sciences, Icahn School of Medicine at Mt. Sinai](#), (3) [New York Center for Rare Diseases](#), and (4) [New York-Presbyterian/Weill Cornell Rare Disease Center](#).