NORD® National Organization for Rare Disorders
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Apfed American Partnership for Eosinophilic Disorders
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EPILEPSY FOUNDATION NORTHEASTERN NEW YORK
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NEW YORK STATE BLEEDING DISORDERS COALITION
Sarcoidosis OF LONG ISLAND
THE Sumaira FOUNDATION
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May 31, 2024

The Honorable Andrea Stewart-Cousins
Majority Leader, New York State Senate
New York State Capitol
Albany, NY 12247

The Honorable Carl Heastie
Speaker of the Assembly, New York State Assembly
New York State Capitol
Albany, NY 12247

RE: Support for S9724 (Persaud)/A10292-A (Paulin) – Establishes a Permanent Rare Disease Advisory Council in the State of New York

Dear Majority Leader Stewart-Cousins and Speaker Heastie,

The 17 undersigned organizations representing individuals with rare diseases in New York, write in **support of S9724 (Persaud)/A10292-A (Paulin)** which would establish a permanent and robust Rare Disease Advisory Council (RDAC) in the state, preserving the forum first established in 2019 for the estimated 1-in-10 New Yorkers living with a rare disease.

As defined by the Orphan Drug Act, any disease or condition that affects fewer than 200,000 Americans is considered rare.[1] Overall, there are more than 10,000 known rare diseases, affecting an estimated 30 million Americans across a broad spectrum of medical conditions. Rare disease patients face many 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. However, due to small patient populations and the variety of rare diseases, it can be difficult for state government officials to have an in-depth understanding of the rare disease community's

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[1] United States Food & Drug Administration, *Rare Diseases at FDA*

1779 MASSACHUSETTS AVENUE NW, SUITE 500
WASHINGTON, DC 20036
T 202-588-5700 ▪ F 202-588-5701

7 KENOSIA AVENUE
DANBURY, CT 06810
T 203-744-0100 ▪ F 203-263-9938

1900 CROWN COLONY DRIVE, SUITE 310
QUINCY, MA 02169
T 617-249-7300 ▪ F 617-249-7301

rarediseases.org ▪ orphan@rarediseases.org

complex needs. This lack of awareness often contributes to the obstacles faced by rare disease patients and their loved ones.

New York State first established a Rare Disease Advisory Council in 2019, when A5762 (Paulin)/S4497 (Rivera) 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 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. As a result, New York's rare community has not had the opportunity to see their Council reach its full potential.

While New Yorkers with rare diseases benefit from services like newborn screening, mandated coverage of medical nutrition, and prescription cost-sharing protections, there remain many unmet needs within our community. The creation of a permanent New York State Rare Disease Advisory Council is the next logical step to help bring greater care and support for those living with rare diseases and their families.

The undersigned organizations are particularly encouraged to see language included in these bills that will enhance what the working group has already accomplished like making the Council subject to Open Meetings Law, adding a duty that empowers the Council or the Department to identify priorities which will provide the group the flexibility needed to adapt to the ever-changing healthcare landscape, and finally a provision that allows the Council to tap into public, private and other available funding sources to support them in their work. We were thrilled and encouraged to see the $25M of investments made into the community as part of the FY25 Enacted Budget and the potential value of a New York RDAC having access to those funds cannot be understated.

With diverse membership and broad community support, a New York RDAC will serve as a valuable resource as our health care system evolves. A strong RDAC can help relieve burdens on the state by expeditiously delivering direct feedback, solutions, and resources to New York government decision makers with one community voice. Without an RDAC, New York laws and regulations that affect the rare community will be developed without the consultation of rare disease stakeholders, leaving this vulnerable population at greater risk for poor health and economic outcomes.

On behalf of the undersigned organizations, **we ask you to see the bills through your respective Chambers, preserving the voice of the rare disease community in New York.** For any questions, please feel free to contact Lindsey Viscarra with the National Organization for Rare Disorders via email at lviscarra@raredisases.org. Thank you for your consideration.

Sincerely,

National Organization for Rare Disorders®
The ALS Association
American Partnership for Eosinophilic Disorders
Cystic Fibrosis Research Institute
Epilepsy Foundation of Northeastern New York
Epilepsy Foundation of Long Island
The Hemophilia Consortium, Inc
Leukemia & Lymphoma Society
Lymphedema Advocacy Group

Mellie J Foundation
MSUD Family Support Group
The Myositis Association (TMA)
NephCure
New York State Bleeding Disorders Coalition
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