NORD National Organization for Rare Disorders
Axis Advocacy Sickle Cell Disease Community and Support
CFRI Cystic Fibrosis Research Institute
California Chronic Care Coalition
Dravet Syndrome Foundation
ALS Association
Chronic Care Policy Alliance
Family Voices of California
Gaucher Community Alliance
ippf International Pemphigus & Pemphigoid Foundation
Lymphedema Advocacy Group
Hemophilia Council of California
APS Foundation of America
Lupus Foundation of Southern California
National MPS Society
Project Alive
Neuropathy Action Foundation
LGS Foundation Lennox-Gastaut Syndrome
TSC Alliance
Epilepsy Foundation San Diego County
Liver Coalition of San Diego
NEC Society
RASopathies Network connect • collaborate • accelerate
nmdp Find cures. Save lives.
Parkinson's Association of San Diego
NephCure for Rare Kidney Disease
California Hepatitis C Task Force HCVTF
CAL RARE California Action Link for Rare Diseases
RARE science Accelerating Cures for RARE Kids

July 19, 2024

The Honorable Anna Caballero, Chair
The Honorable Brian Jones, Vice-Chair
California State Senate Committee on Appropriations
State Capitol, Room 412
Sacramento, CA 95814

Re: Support for Assembly Bill 2613

Dear Chair Caballero, Vice-Chair Jones, and Members of the Senate Committee on Appropriations:

On behalf of the 35 undersigned organizations representing individuals with rare diseases in California, thank you for your steadfast support of the rare disease community and your support for Assembly Bill 2613 (Zbur). AB 2613 establishes a Rare Disease Advisory Council (RDAC) within the state, which if passed, would help to give a voice to the estimated 1-in-10 individuals living with a rare disease in California. AB2613 has thus far passed with zero no votes from Assembly Health Committee, Assembly Appropriations Committee, the full California State Assembly, and Senate Health Committee.

Rare diseases are present across a broad spectrum of medical conditions. For patients living with one of the over 10,000 known 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. This lack of awareness often contributes to the obstacles to timely treatment and care faced by many rare disease patients.

Creating an RDAC in California will give rare disease patients a unified voice in state government to help address challenges that are faced by the rare disease community by serving as the advisory body on rare diseases to the Legislature and state departments. Additionally, the RDAC will be tasked with: 1) evaluating and making recommendations to improve Medi-Cal and state-regulated private insurance coverage of medications that treat rare disease, as well as engaging the Medi-Cal Drug Use Review Board; 2) preparing a survey of the rare disease landscape in California, and convene public hearings to assist the Council in fulfilling its duties; 3) consulting with experts in rare diseases to develop policy recommendations; 4) creating and maintaining a website for the Council; 5) identifying and publishing a list of rare disease resources on said website; and 6) identifying areas of unmet need for research and opportunities for collaboration with stakeholders and RDACs in other states that can inform future studies and work done by the Council.

The RDAC represents enormous value to our organizations and the communities we serve by allowing them to directly engage with a diverse group of stakeholders interested in identifying and solving pressing challenges that California's rare disease community faces. In addition, the RDAC would help relieve some of the burden on the state by expeditiously delivering direct feedback, solutions, and resources to California government decisionmakers with one community voice.

In creating this council, California will join twenty-eight other states that have already enacted similar legislation in support of their rare disease community and have proven that the RDAC can be an invaluable resource. Those states are Alabama, Colorado, Connecticut, Delaware, Florida, Georgia, Illinois, Indiana, Kentucky, Louisiana, Maine, Maryland, Massachusetts, Minnesota, Mississippi, Missouri, Nevada, New Hampshire, New Jersey, New York, North Carolina, Ohio, Pennsylvania, South Carolina, Tennessee, Utah, Virginia, and West Virginia.

Once again, on behalf of the undersigned organizations, we thank you for your support of AB 2613 and your commitment to the rare disease community. For any questions, please feel free to contact Lindsey Viscarra with the National Organization for Rare Disorders via email at lviscarra@rarediseases.org. Thank you for your consideration.

Sincerely,

National Organization for Rare Disorders
Cystic Fibrosis Research Institute
Axis Advocacy
AiArthritis: International Foundation for Autoimmune & Autoinflammatory Arthritis
ALS Association
APS Foundation of America, Inc.
CAL RARE
California Center for Rare Disease at UCLA
Center for Duchenne Muscular Dystrophy at UCLA
Chronic Care Policy Alliance
Dravet Syndrome Foundation
Epilepsy Foundation of San Diego County
Family Voices of California
Finding a Way
Gaucher Community Alliance
Hemophilia Council of California
International Association of Hepatitis Task Forces
International Pemphigus and Pemphigoid Foundation
LGS Foundation
Liver Coalition of San Diego
Lupus Foundation of Southern California
Lymphadema Advocacy Group
Mellie J Foundation
National MPS Society
Necrotizing Enterocolitis (NEC) Society
NephCure
Neuropathy Action Foundation
NMDP
Parkinson's Association of San Diego
Project Alive
RARE Science, Inc
RASopathies Network
Spastic Paraplegia Foundation
The California Chronic Care Coalition
TSC Alliance