

June 23, 2025

The Honorable Marty Makary, M.D., M.P.H.  
Commissioner  
U.S. Food and Drug Administration  
10903 New Hampshire Avenue  
Silver Spring, MD 20993

Dear Commissioner Makary,

On behalf of the estimated 30 million Americans living with rare diseases served by the 101 undersigned leading patient advocacy organizations, and in alignment with the Food and Drug Administration's strong precedent of patient community engagement, **we respectfully request that you convene a timely and interactive town hall-style meeting series dedicated to engagement with patient advocacy organizations.**

We commend your commitment to strengthening the regulatory environment to ensure timely access to safe and effective treatments, and we appreciate your recognition of the unique needs of the rare disease community. The National Listening Tour with Pharmaceutical and Biotech CEOs is a valuable step—but it must be complemented by direct engagement with patient advocacy organizations, whose insights are also essential to this work.

In the last two decades, the FDA has instituted a structured approach to consider patient experience throughout product development in a meaningful way, demonstrating its commitment to regulatory science and to ensuring appropriate processes are in place to quantify the perspective of the patient or the caregiver.

Additionally, over the past six years, our rare disease community has led a formalized series of engagements with patients, as well as scientific, regulatory and clinical experts, and biopharmaceutical industry leaders, aimed at assessing and strengthening therapeutic development and regulatory review processes. The yield was a vision for optimizing rare disease expertise, processes, and engagement with stakeholders across all therapeutic areas, including drugs, cell and gene therapies, and medical devices. Our organizations enthusiastically welcomed the announcement of the Rare Disease Innovation Hub (RDIH) in 2024.

The launch of the RDIH comes as the patient-focused drug development movement continues to evolve and deepen in impact. Our organizations have witnessed improved engagement and understanding of the patient perspective through various approaches, including the Patient-Focused Drug Development meetings and the development of the FDA Benefit-Risk Framework. We've had increased opportunities for meaningful participation in formal service on advisory committees, seen the formation of rare disease-focused initiatives within CDER and CBER, and

have benefited from the establishment of patient engagement advisory committees, as well as reporting on the use of patient experience data within the regulatory review process.

Also transforming engagement and development, FDA's issuance of numerous guidance documents that are informing the conduct of patient-focused product development activities for drugs, cell- and gene-based therapies, diagnostics, and medical devices has been critical to our pipelines.

The milestones in rare disease regulatory infrastructure, patient engagement, and the impact of the patient experience on regulatory decisions should be celebrated; however, our optimism is tempered by the staggering extent of unmet needs that remain and the recognition that, in some cases, process and policy hurdles prevent scientific advances from reaching patients. We successfully collaborated with the Trump administration during the President's first term, advancing the use of patient experience data in research and therapy development together, and we hope to continue this successful partnership in support of the rare disease community.

**Our rare disease community is committed to continued collaboration with you and your teams and urges you to consider the establishment of a timely and interactive town hall-style meeting series dedicated to engagement with patient advocacy organizations.** Rare diseases affect the lives of every American, and we believe that prioritizing engagement with our organizations demonstrates your commitment to making America healthier. We encourage you to create opportunities for our organizations to share their perspectives directly in an interactive environment, which will propel progress in rare disease therapy development and lay the groundwork for meaningful collaborations in the months and years to come.

Sincerely,

EveryLife Foundation for Rare Diseases  
Acromegaly Community  
Akari Foundation  
ALD Alliance  
ALD Connect  
American Liver Foundation  
Amyloidosis Foundation  
Amyloidosis Research Consortium  
Angelman Syndrome Foundation  
Autoinflammatory Alliance  
Baby Ducks in a Row, LLC  
Barth Syndrome Foundation  
BDSRA Foundation  
Bleeding Disorders Council of California  
Bubba's Light, Inc.

CA Action Link for Rare Diseases (Cal Rare)  
Congenital Adrenal hyperplasia Research, Education & Support Foudation, Inc. DBA: CARES Foundation  
Congenital Hyperinsulinism International  
Cure CMD  
Cure GM1 Foundation  
Cure LGMD2i Foundation  
Cure Sanfilippo Foundation  
Cure SMA  
Cyclic Vomiting Syndrome Association  
Dana's Angels Research Trust  
Developmental and Epileptic Encephalopathies/DEE-P Connections  
Dravet Syndrome Foundation  
EB Research Partnership  
Eosinophilic & Rare Disease Cooperative  
Family Heart Foundation  
flok Health  
Foundation for Angelman Syndrome Therapeutics  
Foundation for Prader-Willi Research  
Foundations for Sarcoidosis Research  
Gastroparesis Patients Association for Cures and Treatments  
Global Liver Institute  
GRIN2B Foundation  
HCU Network America  
Hereditary Angioedema Association  
Hermansky-Pudlak Syndrome Network  
Huntington's Disease Society of America  
Hypoparathyroidism Association  
Immune Deficiency Foundation  
International Cystinuria Foundation  
International Foundation for CDKL5 Research  
International Myeloma Foundation  
International Pemphigus & Pemphigoid Foundation  
International SCN8A Alliance  
Krishnan Family Foundation  
Les Turner ALS Foundation  
Lipodystrophy United  
Little Hercules Foundation  
Mission MSA  
Mission: Cure

MLD Foundation  
MPN Research Foundation  
Muenzer MPS Research & Treatment Center  
Muscular Dystrophy Association  
Muscular Dystrophy Pakistan  
Myasthenia Gravis Association  
Myasthenia Gravis KY  
Myositis Support & Understanding  
National Ataxia Foundation  
National Health Council  
National MPS Society  
National PKU Alliance  
National Tay-Sachs and Allied Diseases Association  
National Urea Cycle Disorders Foundation  
Navigating Life with Genetic Mutations  
Niemann-Pick Disease Group  
NTM Info & Research, Inc.  
NW Rare Disease Coalition  
Oklahoma Rare  
Organic Acidemia Association  
Parent Project Muscular Dystrophy  
Partnership to Fight Chronic Disease  
Pathways for Rare and Orphan Solutions  
Phelan-McDermid Syndrome Foundation  
Pompe Consortium  
Project Alive  
PWSA | USA - Prader-Willi Syndrome Association  
Rare New England  
SCID Foundation  
Syngap Research Fund dba Cure Syngap1  
Taylor's Tale  
Team Joseph  
Team Telomere  
The Bluefield Project to Cure Frontotemporal Dementia  
The Global Foundation for Peroxisomal Disorders  
The Institute for Gene Therapies  
The Jansen's Foundation  
The LCC Foundation  
The Myositis Association

The Oxalosis and Hyperoxaluria Foundation

United Mitochondrial Disease Foundation

United MSD Foundation

United Porphyrrias Association

Vasculitis Foundation

Wilson Disease Association

Wiskott Aldrich Foundation