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 Porphyrias Association
Vasculitis Foundation
Wilson Disease Association
Wiskott Aldrich Foundation