December 14, 2021

The Honorable Patty Murray
Chair
Senate HELP Committee
154 Russell Senate Office Building
Washington, DC 20510

The Honorable Richard Burr
Ranking Member
Senate HELP Committee
455 Dirksen Senate Office Building
Washington, DC 20510

Dear Chair Murray and Ranking Member Burr,

We, the undersigned rare disease patient organizations, are writing to enthusiastically endorse President Biden's nomination of Dr. Robert Califf as Commissioner of the Food and Drug Administration (FDA). We ask that the Senate take immediate action to confirm Dr. Califf.

As you know, Dr. Califf joined the FDA in 2015 as the Deputy Commissioner for Medical Products and Tobacco before serving as Commissioner for 11 months starting in February 2016. Prior to joining the FDA, Dr. Califf's career focused on issues that directly impact rare disease patients' lives. As Vice Chancellor of Clinical and Translational Research at Duke University, Dr. Califf led numerous landmark clinical studies in cardiovascular medicine, health outcomes research, health care quality, and clinical research. In addition, he led initiatives to improve clinical research, including the Clinical Trials Transformation Initiative (CTTI), a public-private partnership that included the FDA and Duke.

While interest in rare disease therapy development has increased since the passage of the historic Orphan Drug Act of 1983, the regulatory systems we have in place struggle to meet the unique challenges and complexities inherent in rare disease such as how to design, conduct and analyze clinical trials for small populations. Dr. Califf's previous time as Commissioner and his leadership in the area of innovative clinical trial designs will bring much needed experience to address the unmet needs in the rare disease community.

Most important to us, the undersigned, is that the FDA must continue to expand upon the patient focused drug development momentum, including patient communities and clinical experts as key stakeholders within development and regulatory review. Patients are key partners in all aspects of health, and in all phases of the continuum in therapy and intervention development. Dr. Califf's professional experiences and work with large data initiatives, will enhance the FDA's efforts to identify how best to incorporate patient reported outcomes into the FDA review process.

Dr. Califf's leadership will continue to greatly benefit the more than 30 million Americans with one of the more than 7,000 rare diseases. At an estimated economic cost of $976 billion in

2019[1], we cannot afford to delay the implementation of policy that will help to provide approved therapies for the 93 percent of rare diseases that do not have an approved therapy.

In his work, Dr. Califf continues to advocate for innovative trial design while not sacrificing the use of data to guide the FDA's decision-making process. Dr. Califf's exemplary knowledge of clinical and translational medicine ensures that he will continue to improve the FDA's drug approval process so that patients receive safe and effective treatments at the earliest moment possible.

Sincerely,

American Behcet's Disease Association
Amyloidosis Foundation
Amyloidosis Research Consortium
Angelman Syndrome Foundation
APBD Research Foundation
Autoinflammatory Alliance
Avery's Hope
AXYS
Born a Hero, Research Foundation
CARES Foundation, Inc.
Choroideremia Research Foundation
Cure Sanfilippo Foundation
CureDuchenne
Dana's Angels Research Trust
EB Research Partnership
EveryLife Foundation for Rare Diseases
Foundation for Sarcoidosis Research
Friedreich's Ataxia Research Alliance
Gaucher Community Alliance
Gene Giraffe Project
HCU Network America
Hide and Seek Foundation
Histiocytosis Association
International Foundation for CDKL5 Research
Maryland Rare
Mission: Cure
MitoAction
MLD Foundation
MTS Sickle Cell Foundation, Inc.
Myositis Support and Understanding
National Fragile X Foundation

[1] The National Economic Burden of Rare Disease Study, EveryLife Foundation for Rare Diseases

National Leiomyosarcoma Foundation
National PKU Alliance
NTM Info & Research
Organic Acidemia Association
Parent Project Muscular Dystrophy
People With Empathy
Project Alive
PWSA | USA
Rare New England
SCAD Alliance
Sick Cells
SSADH Association
Stronger Than Sarcoidosis
Syngap Research Fund
Texas Rare Alliance
The Akari Foundation
The Firefly Fund
The Oxalosis and Hyperoxaluria Foundation
The Sudden Arrhythmia Death Syndromes (SADS) Foundation
Undiagnosed Diseases Network Foundation (UDNF)
United Mitochondrial Disease Foundation
USTMA