February 18, 2022

The Honorable Roger Wicker U.S. Senate 555 Dirksen Senate Office Building Washington, DC 20510

The Honorable Doris Matsui House of Representatives 2311 Rayburn House Office Building Washington, DC 20515 The Honorable Amy Klobuchar U.S. Senate 425 Dirksen Senate Building Washington, DC 20510

The Honorable Brad Wenstrup House of Representatives 2419 Rayburn House Office Building Washington, DC 20515

## RE: Support for the BENEFIT Act of 2021 (S. 373 and H.R. 4472)

Dear Senators Wicker and Klobuchar and Representatives Matsui and Wenstrup:

Thank you for your tireless efforts to encourage development of and expand access to treatments and cures for patients, including those with rare diseases. On behalf of the undersigned patient advocacy organizations, we write in strong support of your legislation, the Better Empowerment Now to Enhance Framework and Improve Treatments (BENEFIT) Act of 2021, S. 373 and H.R. 4472.

As you know, the 21<sup>st</sup> Century Cures Act (P.L. 114-255) includes sections 3001 and 3002, the Patient-Focused Impact Assessment (PFIA), which has accelerated the field of patient-focused drug development (PFDD). FDA now has a number of programs and policies in place to gather and assess patient perspectives within the regulatory review process, and patient advocacy organizations have been deeply engaged with the FDA over the past several years to develop PFDD tools that produce scientifically valid patient experience information. Tremendous progress has been made over the past decade since the fifth Prescription Drug User Fee Act (PDUFA) was authorized, including with PFIA and other provisions of 21<sup>st</sup> Century Cures. Now is the time to take the next step in moving patient perspectives and experience forward by enacting the BENEFIT Act.

The BENEFIT Act would require FDA to include in the benefit-risk assessment framework of a new drug application how patient experience data was considered in the review process. Currently, FDA includes patient experience data in reviews, but does not indicate how such data impacted the drug approval. Providing this information to the public, and patient communities making significant investments in developing PFDD, builds on transparency from PFIA and will accelerate PFDD strategies more broadly.

The field of patient engagement in drug development continues to flourish thanks to the continued interest and focus by Congress. The BENEFIT Act will build upon this foundation and fill a gap by appropriately disclosing how this data is considered as part of FDA review of new therapies. The BENEFIT Act initially passed the Senate in 2017 but further action was deferred as the 21<sup>st</sup> Century Cures was being implemented.

Now is the time to take this critical step in building the PFDD environment by passing the BENEFIT Act. The Cures 2.0 Act recognizes this as well by including a parallel provision to the BENEFIT Act. Thank you again for your leadership and we look forward to working with you to enact this legislation this Congress.

Sincerely,

**Alport Syndrome Foundation** 

**ALS Association** 

**Alstrom Syndrome International** 

Ara Parseghian Medical Research Foundation

**Barth Syndrome Foundation** 

**Best Day Ever Foundation** 

**Beyond Celiac** 

Casimir LLC

Coalition Duchenne

CSNK2A1 Foundation

Cure CMD

Cure HHT

**Cure Sanfilippo Foundation** 

Cure SMA

CureDuchenne

**Dravet Syndrome Foundation** 

Dup15q Alliance

Emily's Entourage

**EveryLife Foundation** 

**FND Hope** 

FORCE: Facing Our Risk of Cancer Empowered

Foundation for Prader-Willi Research

Foundation to Eradicate Duchenne

Genetic Alliance

**Global Liver Institute** 

Hannah's Hope Fund

Hemophilia Federation of America

Hope For Marian

Immune Deficiency Foundation

International Pemphigus and Pemphigoid Foundation

Jett Foundation

Kindness Over Muscular Dystrophy

Little Hercules Foundation

Little Miss Hannah Foundation

Lupus Foundation of America

M-CM Network

MLD Foundation

**National Ataxia Foundation** 

**National Multiple Sclerosis Society** 

National Niemann Pick Disease Foundation

**NBIA Disorders Association** 

Organic Acidemia Association

Parent Project Muscular Dystrophy

Phelan-McDermid Syndrome Foundation

**PXE International** 

**RASopathies Network** 

Rivkin Center for Ovarian Cancer

**RUNX1** Research Program

Ryan's Quest
Samantha Search for the Cure
Siegel Rare Neuroimmune Association
SYNGAP1 Foundation
The Firefly Fund
The Life Raft group
The Sudden Arrhythmia Death Syndromes (SADS) Foundation
Team Joseph
Tuberous Sclerosis Alliance
Usher 1F Collaborative
Wiskott-Aldrich Foundation
Zack Heger Foundation