

**DATE**

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

The Honorable Amy Klobuchar  
U.S. Senate  
425 Dirksen Senate Building  
Washington, DC 20510

The Honorable Doris Matsui  
House of Representatives  
2311 Rayburn House Office Building  
Washington, DC 20515

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  
CSNK2A1 Foundation  
Cure CMD  
Cure HHT  
Cure Sanfilippo Foundation  
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  
Genetic Alliance  
Global Liver Institute  
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 Dytrophy  
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  
The Firefly Fund  
The Life Raft group

The Sudden Arrhythmia Death Syndromes (SADS) Foundation  
Tuberous Sclerosis Alliance  
WISKOTT ALDRICH FOUNDATION  
Zack Heger Foundation