May 19, 2021

The Honorable Gus Bilirakis
United States House of Representatives
2354 Rayburn House Office Building
Washington, DC 20515

The Honorable Amy Klobuchar
United States Senate
425 Dirksen Senate Building
Washington, DC 20510

The Honorable G.K. Butterfield
United States House of Representatives
2080 Rayburn House Office Building
Washington, DC 20515

The Honorable Roger Wicker
United States Senate
555 Dirksen Senate Office Building
Washington, DC 20510

Dear Senators Klobuchar and Wicker and Representatives Bilirakis and Butterfield,

We, the undersigned patient organizations and related stakeholders, are writing to express our enthusiastic support and appreciation for your leadership on the Speeding Therapy Access Today (STAT) Act, H.R. 1730 and S. 670. The STAT Act will accelerate development of therapies across the spectrum of rare diseases and disorders and facilitate access to such therapies. Now more than ever, as Americans have seen the benefits of strong public health policy and targeted federal investments in medical and scientific innovation, we must come together to apply these lessons to addressing the unmet need in the rare disease community.

More than 30 million Americans live with one or more rare diseases yet between 93-95% of the over 7,000 known rare diseases have no approved treatments. When a product does successfully navigate the average 15+ year process that it takes to develop a rare disease treatment, patients often face unnecessary delays and barriers to access them. These delays result in avoidable deterioration in health and quality of life for members of the rare disease community.

The STAT Act builds on the passage of the 21st Century Cures Act and the successes of the first FDA Center of Excellence established for oncology products by adapting the model to address the unique challenges of developing treatments for rare diseases. The legislation proposes the creation of a Center of Excellence (Intercenter Institute) for rare diseases and conditions that will serve as a cross-cutting, capacity-building, collaborative hub for rare disease activity at the FDA, supplementing but not supplanting any authorities of the existing Centers.

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. The STAT Act's proposed Center of Excellence will ensure rare disease expertise is coordinated across the many FDA divisions reviewing rare disease products, and it will

establish an effort to address the additional challenges in therapeutic development for communities affected by ultra-rare diseases.

The STAT Act also creates a Rare Disease Drug and Condition Advisory Committee to provide the FDA with community input and expert guidance on a wide range of rare disease issues, supporting the efforts of the Center of Excellence and providing guidance, when requested, to review divisions. This policy will expand FDA's existing community and patient engagement efforts, creating the first-ever federal advisory committee focused on rare diseases, something 16 states have instituted in recent years.

Finally, the STAT Act creates a voluntary third-party payor program to facilitate better communication and information sharing between the FDA, payors and the companies developing rare disease treatments. So many in the rare disease community face long coverage delays after a treatment is approved, and in some cases, patient access never materializes. The STAT Act seeks to ensure coverage policies reflect the totality of information used by the FDA to determine a drug's indicated use. The third-party payor program will leverage the experiences of similar efforts in the medical device space.

Rare disease is not just a health crisis, it is an economic one, too. The recently released National Economic Burden of Rare Disease Study found that the annual economic burden of rare disease reached nearly $1 trillion in the U.S. in 2019, including $418 billion in direct medical costs, $437 billion in productivity losses and $111 billion absorbed by families in uncovered medical and non-medical costs. Only 10% of these costs are attributed to treatments like prescription drugs and outpatient therapies, in part because so many rare disease patients have no approved treatments. This lack of condition-specific treatments instead leads to increased costs associated with hospitalization, outpatient care management, and productivity losses. Most of these expenses could be avoided with the development and access to treatments and cures.

Time is the most precious commodity for rare disease community. Each time a promising therapeutic target faces delays or demise due to the complexities in rare disease and strain on the existing regulatory infrastructure, lives are lost, investment is lost, and future scientific promise is unfulfilled.

Thank you for your continued commitment to the rare disease community as co-chairs of the Rare Disease Caucus. We look forward to continuing to work with you and your staff to ensure this targeted, impactful and attainable policy is enacted this year.

Sincerely,

ADNP Kids Research Foundation
Alexion Pharmaceuticals
Alliance for Patient Access
Alport Syndrome Foundation
American Behcet's Disease Association (ABDA)
Amicus Therapeutics
Amyloidosis Foundation
AnCan and Ann's Place/UsTOO Danbury PCa support

Angelman Syndrome Foundation
Argenx
Association for Creatine Deficiencies
Autoinflammatory Alliance
Avery's Hope
Barth Syndrome Foundation
Biogen
Born a Hero, Research Foundation
BRBN Alliance
CA Action Link for Rare Diseases (Cal Rare)
CARES Foundation
CFC International
Chiesi Farmaceutical, Global Rare Diseases
Children's PKU Network/ NPKUA
Choroideremia Research Foundation
Cure CMD
Cure GM1 Foundation
Cure HHT
Cure VCP Disease, Inc.
CureDuchenne
CureSHANK
Cyclic Vomiting Syndrome Association
Cystic Fibrosis Research Institute (CFRI)
Daphne's Lamp
EB Research Partnership
Endosalpingiosis Foundation Inc
EveryLife Foundation for Rare Diseases
Gaucher Community Alliance
Gene Giraffe Project
Genetic Alliance
Genetic Alliance
Global Blood Therapeutics
Global Genes
Global Liver Institute
Harmony Biosciences
Haystack Project
HCU Network America
Hermansky-Pudlak Syndrome Network
Histiocytosis Association
Homology Medicines
Hunter Syndrome Foundation
International Pemphigus Pemphigoid Foundation
International Waldenstrom's Macroglobulinemia Foundation

Jack McGovern Coats Disease Foundation
KPM Group DC
Little Hercules Foundation
Little Miss Hannah Foundation
Littlest Tumor Foundation
Lowe Syndrome Association
Lymphangiomatosis & Gorham's Disease Alliance (LGDA)
MEPAN Foundation
Mission: Cure
MLD Foundation
MTM-CNM Family Connection
Myasthenia Gravis Foundation of America
Myositis Support and Understanding (MSU)
Narcolepsy Network
National Fragile X Foundation
National Leiomyosarcoma Foundation
National MPS Society
National Tay-Sachs & Allied Diseases Association (NTSAD)
NBIA Disorders Association
NTM Info & Research
Organic Acidemia Association
Orphazyme, US, Inc
Otsuka America Pharmaceutical
Ovid Therapeutics
Parent Project Muscular Dystrophy
People With Empathy
Phelan-McDermid Syndrome Foundation
Phoenix Nest
Pompe Alliance
Prader-Willi Syndrome Association | USA
PXE International
Rare and Undiagnosed Network (RUN)
Rare Disease Innovations Institute, Inc.
Rare New England
RASopathies Network
Remember the Girls [a rare disease organization]
RSD Foundation
Sanofi Genzyme
SCID Angels for Life Foundation
Sick Cells
Siegel Rare Neuroimmune Association
Stronger Than Sarcoidosis
STXBP1 Foundation
SynGAP Research Fund, 501(c)(3)

The Assistance Fund
The E.WE Foundation
The Ehlers-Danlos Society
The Global Foundation for Peroxisomal Disorders
The Mast Cell Disease Society
The Oxalosis & Hyperoxaluria Foundation
The VHL Alliance
Travere Therapeutics
Treating CBS
UDN PEER
United Mitochondrial Disease Foundation
Usher Syndrome Coalition
VHL Alliance
Williams Syndrome Association
WISKOTT ALDRICH FOUNDATION
Wylder Nation Foundation
Zambon USA Ltd.