February 16, 2021

Dear Government Officials:

As the leading advocacy groups, healthcare providers and biotech companies working to bring treatments to patients living with rare diseases, we thank you for swift and urgent distribution efforts of COVID-19 vaccinations, and for ensuring equitable and effective rollouts. The lives of our patients are at risk, and we need your help.

As you know, President Biden announced a strategy in late January committed to vaccine distribution for high-risk individuals, specifically those with intellectual and developmental disorders, and those with underlying conditions, including rare diseases. Our loved ones and their caregivers must be included in your **current** and high-risk vaccination priority phase.

On behalf of patients and families affected by rare diseases, we ask that you include all high-risk patients and caregivers living with rare diseases as a priority population in your immediate rollout of FDA-approved COVID-19 vaccines.

Rare diseases have extensive, and often severe comorbidities. Additionally, many rare diseases are progressive and cause physical, developmental, and cognitive disabilities. Early prevention and intervention are paramount, and this vaccine is critical for our high-risk community and public health. Families affected by rare diseases live daily with conditions including: cardiopulmonary dysfunction; neurological deficits; debilitating seizures; chronic kidney disease; immunosuppression; failure to thrive; loss of muscle tone and coordination; muscle atrophy; chronic pain; vision and/or hearing loss; gastrointestinal issues; intellectual and learning disabilities; autism spectrum disorder; and organ failure.[1] It is estimated 25-30 million individuals in the United States have a rare disease, of which many are life-threatening with limited or no treatment options.[2]

As leaders and caregivers in this community, we strongly believe individuals living with a rare disease who require daily hands-on care, and their caregivers should be included in the high-risk population for COVID-19 vaccination administration.[3] Due to the complexity of such diseases, patients are at increased risk for catastrophic outcomes due to infection of COVID-19. The effects of COVID-19 could be devastating for individuals and family members, of all ages, who are constantly at high-risk for neurological and organ damage caused by potential infection. During the pandemic, these families have lost vital resources (such as physical, occupational and speech therapies) and myriad interventions otherwise afforded to them. Compounded by COVID-related delays in routine public and private care management due to risk of exposure, many patients have experienced disease regression, and the only way to safely resume these crucial interventions is through immediate access to COVID-19 vaccination.[4]

We call on you to urgently categorize all patients living with rare disease and their caregivers as a high-risk population included in your state's COVID-19 vaccination plan. These caregivers are essential to public health, safety, and care of individuals living with rare diseases. If essential caregivers are exposed to COVID-19, isolation from those they care for could cause severe, irreversible damage.[5]

We welcome the opportunity to discuss our request further. For questions, please contact Patroski Lawson at patroski@kpmgroupdc.com or (202) 812-3546 or and/or Ashley Pounders, MSN, FNP-C, Director of Medical Affairs at the Tuberous Sclerosis Alliance, apounders@tsalliance.org or (301) 562-9890.

Respectfully submitted in alphabetical order,

Alagille Syndrome Alliance
Allergy & Asthma Network
Alport Syndrome Foundation
Angelman Syndrome Foundation
Autism Science Foundation
Batten Disease Support and Research Association
Neil S. Belloff, Esq. - Chief Operating Officer, and General Counsel, Eloxx Pharmaceuticals, Inc.
BPAN Warriors
Brain Donor Project
Bridge the Gap – SYNGAP Education and Research Foundation
Jeffrey R. Buchhalter, MD
CACNA1A Foundation
Child Neurology Foundation
Wendy Chung, MD, PhD - Kennedy Family Professor of Pediatrics and Medicine, Chief, Clinical Genetics, Columbia University
Coalition to Cure CHD2
COMBINEDBrain
Peter B. Crino, MD, PhD – Chair, Department of Neurology, University of Maryland Medical System, Chair, Tuberous Sclerosis Alliance Board of Directors
CURE Epilepsy
CureSHANK
Cute Syndrome Foundation
Cystic Fibrosis Research, Inc.
Danny Did Foundation
Dravet Syndrome Foundation
Dup15q Alliance
Epilepsy Foundation
FamiliesSCN2A Foundation
Kyle Fink, PhD - Institute for Regenerative Cures, UC Davis School of Medicine
FOXG1 Research Foundation
Global Genes
Global Liver Institute
Glut1 Deficiency Foundation
GRIN2B Foundation
HCU Network America
Hermansky-Pudlak Syndrome Network
Hope for Hypothalamic Hamartomas
Hope for ULD
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LGS (Lennox-Gastaut Syndrome) Foundation
National Alliance for Caregiving
NORSE Institute
PACS1 Syndrome Research Foundation
PCDH19 Alliance
phaware global association
Phelan-McDermid Syndrome Foundation
Primary Ciliary Dyskinesia Foundation
Project 8p
Pulmonary Fibrosis Foundation
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SLC6A1 Connect
SNAP25 Foundation
STXBP1 Foundation
SynGAP Research Fund (SRF)
Tbc1d24 Foundation
TESS Research Foundation for SLC13A5 Epilepsy
The Brain Recovery Project: Childhood Epilepsy Surgery Foundation
The Global Foundation for Peroxisomal Disorders
The LAM Foundation
The Schinzel-Giedion Syndrome Foundation
Tuberous Sclerosis Alliance
Wishes for Elliott/DEE-P Connections

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[1] "FAQs About Rare Diseases." *National Center for Advancing Translational Sciences (NCATS)*. Updated 11/30/2017. Accessed via web: https://rarediseases.info.nih.gov/diseases/pages/31/faqs-about-rare-diseases#:~:text=How%20many%20rare%20diseases%20are,at%20between%2025-30%20million.
[2] Ibid.
[3] Landers, Scott D. et.al. "COVID-19 outcomes among people with intellectual and developmental disability living in residential group homes in New York State" https://doi.org/10.1016/j.dhjo.2020.100969
[4] Hotez, E. et. al. (2021). Prioritizing COVID - 19 Vaccinations for Individuals with Intellectual and Developmental Disabilities. EClinical Medicine.
[5] Wong, Allen, Carl, Tyler, and Emily Johnson. "Joint Position Statement on Equity for People with Intellectual and Developmental Disabilities Regarding COVID-19 Vaccine Allocation and Safety." *American Academy of Developmental Medicine & Dentistry.* Updated 12/9/2020. Accessed via web:

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