

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
- IGA Nephropathy Foundation
- International Cystinuria Foundation
- International Foundation for CDKL5 Research
- KCNQ2 Cure Alliance
- KIF1A.ORG
- Darcy A. Krueger, MD PhD – Clack Endowed Chair in Tuberous Sclerosis, Director, Tuberous Sclerosis Clinic, Professor of Clinical Pediatrics and Neurology,
- Cincinnati Children’s Hospital Medical Center, University of Cincinnati College of Medicine and Chair, Tuberous Sclerosis Alliance Professional Advisory Board
- 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:

- 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>
- Patroski Lawson, MSP – CEO, KPM Group DC
- Jeremy Levin, DPhil, MB BChir – CEO, Ovid Therapeutics, Chairman, The Biotechnology Innovation Organization
- 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
- Amit Rakhit, MD, MBA – President and Chief Medical Officer, Ovid Therapeutics
- RARE-X
- Rare Epilepsy Network (REN)
- RASopathies Network
- Ring14 USA
- Mustafa Sahin, MD, PhD – Director, Translational Neuroscience Center; Director, Translational Research Program; Rosamund Stone Zander Chair, Professor of Neurology, Harvard Medical School, Chair, Tuberous Sclerosis Alliance International Scientific Advisory Board
- Scleroderma Foundation
- Jill Silverman, PhD – MIND Institute, UC Davis School of Medicine
- 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
- <https://static1.squarespace.com/static/5cf7d27396d7760001307a44/t/5fd9690f9e3b1725e3d0d3e2/1608083731221/Covid19Vaccine-IDD-Statement.pdf>.