

The Honorable Alex Azar
Secretary
Health and Human Services
200 Independence Avenue, SW
Washington, DC 20201

The Honorable Seema Verma
Administrator
Centers for Medicare & Medicaid Services
7500 Security Boulevard
Baltimore, MD 21244

May 1, 2020

Secretary Azar and Administrator Verma,

On behalf of the undersigned patient organizations representing the estimated 30 million Americans living with rare diseases, we write to thank you for your ongoing leadership during the coronavirus (COVID-19) public health emergency. As the Department of Health and Human Services (HHS) and other agencies across the federal government prepare the country for the next stage—including an eventual national reopening—we urge you to continue to keep the unique needs of rare disease patients and complex health conditions in mind. Specifically, we ask that you work with the patient community to consider guidelines and other policies to improve access to therapies and supportive services, the continuation of clinical trials, and the conduct of newborn screening programs.

Patients with rare diseases and disorders, as well as others living with complex or chronic conditions, are at higher risk of complications from COVID-19. In recent months, social distancing and added precautions have been essential to protecting the health and safety of our community members. However, at the same time, because of their health status, members of our community continue to require regular medical care, much of which cannot be provided remotely or deferred indefinitely. While stay-home orders and the national shutdown of sectors of our economy have been necessary to protect rare disease patients, these actions have resulted in several unintended consequences and challenges.

As HHS and the Administration work to establish parameters and guidelines for resuming healthcare and other operations, we urge you to consider fully the needs of the rare disease community as part of this process. Any disruption to care and treatment, such as a delay or denial due to authorization requirements, delay in annual or new referral appointments with the rare disease specialist or postponement of clinical trial places patients at risk for worse health outcomes, particularly those patients with progressive, life-threatening diseases who face irreversible loss of function or abilities. Of great concern during this pandemic, treatment disruptions pose the risk of acute health crises requiring hospitalization at a time when healthcare systems are already at serious risk of exceeding capacity.

Specifically, we ask that you support policies that incorporate the following:

- Recognize the needs of rare disease patients and others with complex and chronic conditions who require regular access to medical care—including surgical, other in-person procedures and physician-administered therapies such as infusions and injections in the clinic or home setting—receive uninterrupted treatment. Moreover, as regions of the country begin to “reopen”, rare disease patients, particularly those at high risk for COVID, may continue to practice maximum caution through social distancing measures until a treatment and/or vaccine is available. Such caution may include a patient’s unwillingness to travel to the clinic for risk of being exposed to COVID. As such, we request public and private payers be flexible with prior (re)authorization policies for vulnerable patient populations.
- Stratify or more clearly define the term “elective” particularly as the pandemic and shutdowns continue. The term “elective” may unintentionally connote care that is entirely optional and can be postponed indefinitely. This is not the case with our patients for whom postponing seemingly nonurgent care could have dire effects. As healthcare providers, state governments and others look to Washington for further guidance on the reopening, it is essential that this term be more fully defined and/or other terms be used to address these important nuances. Elective does not mean optional for our patients.
- Offer guidance regarding populations and/or types of care that should be prioritized when healthcare systems are able to resume regular or near-regular operations. We know providers across the country will be faced with a significant and growing backlog, and we believe guidance from HHS will be essential, particularly to ensure patients with the most emergent needs – those that if not met will soon fall into the category of emergency and non-elective – are taken care of.
- Issue standards or guidelines to address the needs of rare disease and other communities whose members have underlying health challenges yet absolutely need near-term access to in-person care. The federal government has a longstanding and important role in setting quality and safety standards and must continue this work by putting forward the most evidence-based standards to protect vulnerable patients while ensuring access to needed care.
- Build upon administrative efforts to ensure patients have access to in-home services including home infusion and injection services as that may continue to be the safest setting during the first phases of re-opening. We applaud CMS for its prompt action on multiple emergency measures, including broadening the definition of “homebound” for the purposes of being eligible for homecare during the public health emergency and other temporary changes. However, we ask for consideration of the following:

- Additional guidance and actions to ensure Medicaid beneficiaries would have similar access to in-home infusion during the PHE.
- Additional actions to address barriers to home infusion that still remain in practice, including sizeable out-of-pocket costs and delivery challenges.
- Issue guidance to aggregate best practices and encourage contingency plans for the continuation of clinical trials including site initiation, screening visits, remote monitoring, and assessments now and in the future, should there be a second wave of COVID-19 transmissions after re-opening.
- Examine how the nation’s newborn screening system has been impacted by this health crisis to ensure that families receiving a new diagnosis during this time have access to timely and supportive intervention and care services, as intended.

To help inform these considerations, we urge you to consider convening a group of rare disease advocates and other stakeholders – including healthcare providers as well as patients and their caregivers—to hear directly from the community. We believe the patient community perspective during this unprecedented time is invaluable and would help ensure an informed and appropriate process moving forward. Few actions are as important to our community today than the ability to access largely shuttered healthcare services in a way that is safe, and we wish to serve as a resource to inform these actions.

We thank you for your prompt attention to this matter. Please do not hesitate to contact Annie Kennedy, Chief of Policy and Advocacy (akennedy@everylifefoundation.org) at the EveryLife Foundation for Rare Diseases with any questions.

Sincerely,

Aidan Jack Seeger Foundation

Alport Syndrome Foundation

American Kidney Fund

Amyloidosis Foundation

Amyloidosis Research Consortium

AnCan

Association for Creatine Deficiencies

BORN A HERO

Bridge the Gap – SYNGAP Education and Research Foundation

Caregiver Action Network

Choroideremia Research Foundation

CureDuchenne

debra of America

Dreamsickle Kids Foundation

EveryLife Foundation for Rare Diseases

Fabry Support & Information Group

Friedreich's Ataxia Research Alliance
Gaucher Community Alliance
Global Foundation for Peroxisomal Disorders
Global Genes
HCU Network America
Hope For Dante'
Hunter Syndrome Foundation
IGA Nephropathy Foundation of America
International Fibrodysplasia Ossificans Progressiva Association
International Pemphigus and Pemphigoid Foundation
Krabbe Connect
LGS Foundation
Little Hercules Foundation
Lymphangiomatosis & Gorham's Disease
MLD Foundation
MTM-CNM Family Connection
Narcolepsy Network
National Ataxia Foundation
National Fabry Disease Foundation
National Fragile X Foundation
National MPS Society
National PKU Alliance
NephCure Kidney International
Neuromuscular Disease Foundation
NGLY1.org
One Rare
Parent Project Muscular Dystrophy
Piper's Kidney Beans Foundation
Pompe Alliance
Project Alive
Pulmonary Fibrosis Foundation
Rare and Undiagnosed Network (RUN)
Rare New England
RASopathies Network
Sarcoidosis of Long Island
SATB2 Gene Foundation
Spina Bifida Association
Steering Committee of the Pennsylvania Rare Disease Advisory Council
TEAM 4 Travis
Texas Rare Alliance
The Myositis Association
Tuberous Sclerosis Alliance
United Mitochondrial Disease Foundation

Cc: FDA Commissioner Stephen Hahn, HRSA Administrator Thomas Engels, CDC Director Robert Redfield; NIH Director Francis Collins