April 14, 2023

The Honorable Chiquita Brooks-LaSure
Administrator
Centers for Medicare and Medicaid Services
U.S. Department of Health and Human Services
200 Independence Avenue SW
Washington, DC 20201

Meena Seshamani, M.D., Ph.D.
Deputy Administrator and Director of the Center for Medicare
Centers for Medicare & Medicaid Services
7500 Security Boulevard
Baltimore, Maryland 21244-1850

Dear Administrator Ms. Brooks-LaSure and Dr. Seshamani,

The 101 undersigned organizations, representing patients living with rare diseases and their families, thank you for the guidance released on March 15, 2023, specific to the Medicare Drug Price Negotiation

Program as required by the Inflation Reduction Act of 2022 (IRA). As the Centers for Medicare and Medicaid Services (CMS) continues to implement the provisions of this law, we urge you to further consider the unique perspective and challenges faced by rare disease patients and provide the following recommendations to ensure this law best serves the needs of the rare disease community.

As part of the IRA, and for the first time, CMS will negotiate the price of some prescription drugs available through the Medicare program. This will have significant impacts for some rare disease patients on their ability to afford needed treatments but could also impact broader rare disease drug development. For many of the more than 25 million Americans living with a rare disease, out of pocket prescription drug costs create significant financial barriers and hinder access to needed therapies. Key provisions in the IRA, including the $2,000 annual and amortized monthly caps on out-of-pocket costs for Medicare Part D beneficiaries, ensure that more rare disease patients will be able to afford the life-altering therapies they need.

At the same time, the vast majority of the more than 7,000 known rare diseases do not have an FDA approved treatment. This makes continued research and innovation especially important to the rare disease community. Unfortunately, the small patient populations and medical complexity associated with rare diseases creates unique challenges to rare disease drug development. These same factors result in a scarcity of the data necessary to determine a fair negotiated price for products that treat rare diseases.

While CMS' most recent guidance includes several elements that positively impact the rare disease community, our organizations urge CMS to incorporate several changes into future guidance and program implementation to ensure the rare disease community fully benefits from the IRA.

Patient Engagement

We appreciate CMS' effort to incorporate the patient perspective into the negotiation program. Patients and caregivers have key insights on issues such as determining the value of a therapy and how it compares to potential alternate treatment options. For instance, rare disease patients are often uniquely positioned to share the challenges associated with unmet medical needs - when there are no or very few options available to treat their condition - and the benefits to themselves, their families and the community from a safe and effective therapy. Patient experience data will be particularly important given CMS' desire to evaluate price on an indication-specific level. We commend the agency for recognizing the unique value that drugs can bring to specific parts of the treated patient population, including patients that have few or no therapeutic alternatives.

In the rare disease community, published data to assess these specific uses remain scarce and patients and providers are often the best experts from which to elicit such information. While we are grateful CMS recognizes the value of patient experience data in the guidance we strongly encourage the agency to expand the opportunities available to patients to provide such input. We worry that the short timelines and limited proposed mechanisms for providing this input essentially make it impossible for patients to provide meaningful data. We urge CMS to 1) simplify and streamline the data submission process for patients and caregivers; 2) to clarify ahead of time what information the agency is seeking from patients and in what format to allow data standardization and aggregation, 3) to organize patient listening sessions specific to selected drugs to collect representative data while CMS is preparing the initial offer for a negotiated drug; and 4) include consistent and granular summaries of the data and assumptions on which each negotiation was based, including patient experience data.

Patient Access to Negotiated Drugs

We are supportive of the provision that requires negotiated products within the Medicare Part D program to be included on Part D plan formularies. However, we encourage CMS to take additional steps to ensure rare disease patients benefit from associated reduced out-of-pocket expenses and have timely access to negotiated products. Often, therapies that treat rare diseases are placed on the specialty tier of plan formularies, resulting in significant out-of-pocket costs and access delays for Medicare beneficiaries. Once a drug is negotiated it has been shown to be appropriately priced from CMS' perspective and should be placed on a higher formulary tier to reduce patient out-of-pocket costs.

Another common source of treatment delays or denials for our community are utilization management tools, such as prior authorization and/or step therapy. Health care providers, in partnership with their patients, are best positioned to choose the right therapy to treat the often-complex health care challenges faced by those with a rare disease. Given negotiated drugs will have been appropriately valued from CMS' perspective, we encourage CMS to require Medicare Part B and Medicare Part D plans to reduce or eliminate utilization management tools, including step therapy and/or prior authorization barriers to ensure patients are able to quickly access a negotiated drug.

Orphan Drug Exclusion

We acknowledge that the IRA includes a limited exclusion for orphan drugs that only treat one rare disease from drug price negotiation. However, we are concerned CMS' current interpretation of this rare disease exclusion, which makes products eligible for negotiation if they have been designated for two or more orphan diseases – even if the drug is not actually FDA approved to treat the second orphan disease - will disincentivize drug companies from conducting even the basic research necessary to develop a drug for additional rare diseases. Designating a drug for a rare disease is done very early on in the drug development process and does not allow the company to market the drug because it has not been proven to be safe and effective to treat that specific disease. We urge CMS to clarify that obtaining additional designations for a small molecule or biologic will not make a drug negotiation eligible until the drug has been approved by FDA to treat a second disease or condition.

From the rare disease patient community's perspective, successful IRA implementation hinges on a careful balance between greater affordability and maintaining appropriate incentives for continued investment in rare disease specific drug development. We thank CMS for the opportunity to comment on this latest IRA guidance and look forward to working with CMS to ensure rare disease patients and patient advocacy organizations can fully participate within this important effort and benefit from the law.

For questions related to this comment letter, please contact Heidi Ross, Vice President of Policy and Regulatory Affairs at the National Organization for Rare Disorders at HRoss@rarediseases.org.

Thank you for your consideration,

National Organization for Rare Disorders
A Cure in Sight
Alpha-1 Foundation
ALS Association
American Behcet's Disease Association (ABDA)
American Partnership for Eosinophilic Disorders
Angelman Syndrome Foundation
Autoimmune Encephalitis Alliance, Inc.
Avery's Hope
Bladder Cancer Advocacy Network (BCAN)
Cancer*Care*
Children's PKU Network
Chondrosarcoma CS Foundation
Choroideremia Research Foundation

Chronic Disease Coalition
Coalition to Cure Calpain 3
Congenital Hyperinsulinism International
Consortium of MS Centers
Cure CMD
Cure HHT
Cure SMA
Cutaneous Lymphoma Foundation
Cystic Fibrosis Research Institute
DADA2 Foundation
Desmoid Tumor Research Foundation
Diann Shaddox Foundation for Essential Tremor
Epilepsy Foundation
FACES: The National Craniofacial Association
FD/MAS Alliance
Fibromuscular Dysplasia Society of America
FOD (Fatty Oxidation Disorders) Family Support Group
Foundation for Sarcoidosis Research
Friedreich's Ataxia Research Alliance (FARA)
Global Liver Institute
Gaucher Community Alliance
Global Healthy Living Foundation
Glut1 Deficiency Foundation
GRIN2B Foundation
Hepatitis B Foundation
Hydrocephalus Association
Hypertrophic Olivary Degeneration Association
IgA Nephropathy Foundation
International Autoimmune Encephalitis Society
International Foundation for Autoimmune & Autoinflammatory Arthritis
International Pemphigus Pemphigoid Foundation
International Waldenstrom's Macroglobulinemia Foundation
KBG Foundation
Leukodystrophy Newborn Screening Action Network
LGMD Awareness Foundation, Inc
Li-Fraumeni Syndrome Association (LFS Association / LFSA)
LUNGevity Foundation
Lymphangiomatosis & Gorham's Disease Alliance
MdDS Balance Disorder Foundation
MLD Foundation
Muscular Dystrophy Association
Myasthenia Gravis Foundation of America
Myocarditis Foundation
National Ataxia Foundation
National Bone Marrow Transplant Link
National MALS Foundation
National Oncology State Network
National PKU News
National Scleroderma Foundation
NBIA Disorders Association
NR2F1 Foundation
NTM Info & Research
Organic Acidemia Association
Phaware Global Association
Phelan-McDermid Syndrome Foundation
Project Alive
PWSA | USA
Reflex Sympathetic Dystrophy Syndrome Association
RETpositive
SATB2 Gene Foundation
Spastic Paraplegia Foundation
SSADH Association
Super T's Mast Cell Foundation
Superficial Siderosis Research Alliance
Syngap 1 Foundation
TargetCancer Foundation
Tatton Brown Rahman Syndrome Community
TEAM TELOMERE
Texas Prader Willi Association
The Akari Foundation
The Avalon Foundation
The Global Foundation for Peroxisomal Disorders
The Jansen's Foundation
The Life Raft Group
The Mast Cell Disease Society
The Multiple System Atrophy Coalition
The RYR-1 Foundation
Thrive with Pyruvate Kinase Deficiency Organization

United MSD Foundation
United Porphyrias Association
Usher 1F Collaborative
Usher Syndrome Coalition
Usher Syndrome Society
Vasculitis Foundation
Vestibular Disorders Associations
wAIHA Warriors
Xia -Gibbs Society