RARE DISEASE DIVERSITY COALITION

November 4, 2022

The Honorable Chuck Schumer
Senate Majority Leader
U.S. Senate
Washington, D.C. 20510

The Honorable Mitch McConnell
Senate Minority Leader
U.S. Senate
Washington, DC 20510

The Honorable Nancy Pelosi
Speaker of the House
U.S. House of Representatives
Washington, DC 20515

The Honorable Kevin McCarthy
House Minority Leader
U.S. House of Representatives
Washington, DC 20515

RE: The Continuing Appropriations and Ukraine Supplemental Appropriations Act and End of Year Priorities

Dear Majority Leader Schumer, Minority Leader McConnell, Speaker Pelosi, and Minority Leader McCarthy,

The Rare Disease Diversity Coalition (RDDC) applauds Congress for working to ensure the fiscal 2023 continuing resolution (CR) that was signed into law on September 30, 2022 includes the reauthorization of the U.S. Food and Drug Administration (FDA) User Fee programs, which will allow the Agency to continue addressing the urgent treatment needs of the rare disease community.

RDDC is a coalition of the nation's leading rare disease and equity advocates, public health experts and industry leaders. We were launched in 2020 to address the extraordinary challenges faced by rare disease patients of color. RDDC and its partners are committed to being a catalyst for progress that guarantees equitable representation of people of color living with a rare disease by seeking to identify and advocate for evidenced-based solutions to alleviate the disproportionate burden of rare diseases on these communities.

RDDC was pleased by the incorporation of certain provisions into the Prescription Drug User Fee Authorization (PDUFA) VII, including the establishment of Rare Disease Endpoint Advancement (RARE) pilot, the establishment of the Split Real Time Application Review (STAR) pilot, advancement of patient-focused drug development within the Center for Biologics Evaluation and Research (CBER), and the improvement of policies to support use of Real-World Evidence (RWE). These critical programs will help to advance treatments for rare disease.

However, we were disappointed that many critical, bipartisan legislative initiatives that would benefit rare disease patients, particularly those from marginalized communities, have not advanced, including:

1) Diverse and Equitable Participation in Clinical Trials (DEPICT) Act (H.R. 6584)
2) Newborn Screening Saves Lives Reauthorization Act of 2021 (H.R. 582/S. 350)
3) Access to Genetic Counselor Services Act of 2021 (H.R. 2144/S. 1450)
4) Help Ensure Lower Patient (HELP) Copays Act (H.R. 5801)
5) Modernizing the Accelerated Approval Pathway Act of 2022 (S. 4446)
6) Advancing Telehealth Beyond COVID-19 Act of 2021 (H.R. 4040)
7) Telehealth Extension Act of 2021 (H.R. 6202)
8) Telehealth Extension and Evaluation Act (H.R. 7573/S. 3593)

RARE DISEASE DIVERSITY COALITION

We strongly encourage that these meaningful provisions be considered in the final omnibus spending bill before the end of the year so that all rare disease patients, particularly those from communities of color and other marginalized communities, are assured equal access to affordable, timely treatments.

Lack of diversity in clinical trials is a pressing issue that adversely affects our entire health care system.[1,2] One analysis of drug approvals by the U.S. Food and Drug Administration between 2014 and 2021 found that fewer than 20% of drugs had data regarding treatment benefits or side effects for Black patients.[3] This problem is particularly acute for patients of color with rare diseases who disproportionately face barriers to participation in clinical trials and access to timely, high-quality healthcare services. As the demographic diversity of America continues to increase, scientific imperative to ensure diverse clinical trial participation must follow. Otherwise, we risk developing treatments for diseases that may not be clinically effective, or potentially even dangerous, for large swaths of Americans. The DEPICT Act (H.R. 6584) would help to boost diversity in clinical trials by requiring enhanced data reporting on clinical trial demographics and providing resources to improve participation in clinical trials by underrepresented groups, including grants to Community Health Centers.

As genetic screening become increasingly important to disease diagnosis and as screening criteria for eligibility for participation in clinical trials, barriers to access for communities of color and other underserved groups needs to be addressed. The Access to Genetic Counselor Services Act of 2021 (H.R. 2144/S. 1450) would provide for Medicare coverage of genetic counseling services. The Newborn Screening Saves Lives Reauthorization Act (H.R. 582/S. 350) would reauthorize several critical newborn screening programs for certain conditions and genetic diseases and classify research on non-identified newborn blood spots as research on human subjects, which comes with additional protections.

Copay accumulator adjustment programs (CAPs), which prevent co-payment assistance for high-cost specialty drugs from counting toward a patient's deductible or maximum out-of-pocket expenses, are becoming increasingly more common and are disproportionately impacting communities of color and other marginalized groups, hindering their ability to afford and access treatment for their diseases. The HELP Copays Act (H.R. 5801) would require health insurance plans to apply certain payments made by or on behalf of a plan enrollee toward a plan's cost-sharing requirements.

The FDA Accelerated Approval Pathway is an important way for patients suffering from serious diseases to gain access to potentially lifesaving treatments faster with important implications for rare disease patients. However, patient safety should always be paramount. The Modernizing the Accelerated Approval Pathway Act of 2022 (S. 4446) would establish a pilot program and dedicated FDA council to support the development of drugs for rare diseases and ensure the appropriate use of the accelerated approval pathway while ensuring patient safety.

Continued access to telehealth services has important implications for rare disease patients who are managing complex, rare conditions that require more frequent check-ins and can make it infinitely more difficult to travel to in-person appointments that are often located out of state and adds an additional burden to patients living with rare disease, particularly those patients in diverse communities. Telehealth has helped

[1] Improving Representation in Clinical Trials and Research: Building Equity for Women and Underrepresented Groups. The National Academies of Sciences, Engineering, and Medicine. 2022.
[2] Clinical Trial Diversity. U.S. Food & Drug Administration.
[3] Despite The FDA's Five-Year Plan, Black Patients Remain Inadequately Represented In Clinical Trials For Drugs. Health Affairs. March 2022.

to break down barriers and equalize access to care, particularly for patients suffering from mobility issues or those that lack reliable transportation or an ability to take time off work, which disproportionately impacts communities of color and other diverse populations. Importantly for rare disease patients, telehealth access also enables patients to access care by world-renowned experts while eliminating the immense logistical and financial burden of travel, so that all rare disease patients, not only those with financial means, can have access to the highest quality medical care and can live their lives to the fullest extent. Telehealth services also help to reduce unnecessary risk of exposure, which is critically important to rare disease patients who are often immunocompromised.

Thanks to waivers and flexibilities created during the COVID-19 Public Health Emergency (PHE), we have witnessed a groundbreaking increase in telehealth access and usage. Through these waivers, home-based virtual care was made possible, the number of eligible practitioners able to render these services was expanded, and patients could receive these services from anywhere in the country, regardless of whether they lived in a rural or urban area. Unfortunately, nearly all of these newfound flexibilities are temporary. Thanks to the Consolidated Appropriations Act, many of these flexibilities will extend for five months beyond the end of the PHE, but a longer-term solution is needed.

Medicare patients have come to rely on telehealth services as a crucial component of their care. Many providers have redesigned the way they deliver care to incorporate telehealth. A recent Office of Inspector General (OIG) data brief helped to quell concerns about telehealth fraud, finding that only a small fraction of Medicare providers (0.2%) posed a "high risk" ultimately recommending "targeted oversight." An accompanying brief on utilization found that telehealth "improved access to telehealth for Medicare beneficiaries, particularly for those who are medically underserved" and recommended policymakers take appropriate steps to: enable a successful transition from current pandemic-related flexibilities to long-term policies for the use of telehealth in urban areas and from the beneficiaries' homes, extend and continue studying audio-only services, and use telehealth to advance health care equity. RDDC urges Congress to continue evaluating data on utilization, cost, and quality of telehealth services as it works to establish long-term telehealth policies, including access to audio-only services, which help to expand and equalize access for communities of color and other disenfranchised groups, particularly those suffering from rare disease.

RDDC urges Congress to take meaningful action to address these and other important healthcare priorities in an end-of-year omnibus package. Specifically, we urge lawmakers to pass policies that urge development of culturally and linguistically appropriate services, facilitate a modernized accelerated approval pathway for groundbreaking treatments, increase diverse representation in clinical trials, and promote timely, affordable access to treatment and medications for all. We stand ready to work with Congress towards common sense policies that would benefit all patients but will also help to close the disparity gap for those in greatest need.

Sincerely,

Jenifer Ngo Waldrop

Jenifer Ngo Waldrop
jwaldrop@bwhi.org
Executive Director
Rare Disease Diversity Coalition (RDDC)