NORD®

June 26, 2024

The Honorable Cathy McMorris Rodgers
Chair
House Committee on Energy and Commerce
2125 Rayburn House Office Building
Washington, D.C. 20515

The Honorable Frank Pallone, Jr.
Ranking Member
House Committee on Energy and Commerce
2322A Rayburn House Office Building
Washington, D.C. 20515

The Honorable Brett Guthrie
Chair, Health Subcommittee
House Committee on Energy and Commerce
2434 Rayburn House Office Building
Washington, D.C. 20515

The Honorable Anna Eshoo
Ranking Member, Health Subcommittee
House Committee on Energy and Commerce
272 Cannon House Office Building
Washington, D.C. 20515

Dear Chair McMorris Rodgers, Ranking Member Pallone, Chair Guthrie, Ranking Member Eshoo, and Members of the House Committee on Energy and Commerce,

On behalf of the more than 30 million Americans living with one of the over 10,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks the House Committee on Energy and Commerce for holding this week's markup to consider legislation that will benefit the rare disease community NORD so proudly represents. NORD is pleased to support H.R. 3433, the Give Kids a Chance Act and H.R 7623, the Telehealth Modernization Act and urges Committee members to vote yes in support of both of these bills in their current forms.

NORD is a unique federation of non-profit and health organizations dedicated to improving the health and well-being of people with rare diseases by driving advances in care, research, and policy. NORD was founded over 40 years ago, after the passage of the Orphan Drug Act (ODA), to formalize the coalition of patient advocacy groups that were instrumental in passing this landmark law. Since that time, NORD has been advancing rare disease research and funding to support the development of effective treatments and cures; raising awareness and addressing key knowledge gaps; and advocating for policies that support the availability of affordable, comprehensive health care, including access to safe and effective therapies.

NORD encourages members of the House Energy and Commerce Committee to vote yes to advance the following bills under consideration at this week's markup:

Amendment in the Nature of a Substitute to H.R. 3433, Give Kids a Chance Act, which includes key provisions of H.R.7384, Creating Hope Reauthorization Act, H.R. 7383, Retaining Access and Restoring Exclusivity (RARE) Act, and H.R. 6664, Innovation for Pediatric Drugs Act.

Of the more than 30 million individuals in the United States living with rare diseases,[1] as many as half are children, and they need – and deserve – access to therapies that have been proven to be safe and effective for them. At the same time, pediatric studies are particularly challenging for rare diseases and can be significantly more difficult to complete than for more common diseases. NORD believes this package of

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[1] U.S. Government Accountability. (2021, October 18). Rare diseases: Although Limited, available evidence suggests medical and other costs can be substantial. | U.S. GAO. https://www.gao.gov/products/gao-22-104235

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bills strikes the right balance of incentives and enforcement to help spur rare pediatric disease drug development and ensure more pediatric patients have safe and effective therapies to treat their conditions. NORD is supportive of the bipartisan agreement before the Committee today and grateful to the Members and their staffs who have worked so hard to negotiate this agreement.

Critical to NORD's support for this package are its provisions that would:

- Reauthorize the Rare Pediatric Disease Priority Review Voucher program in its current form through September 30, 2029. As of April 2024, 53 rare disease therapies across more than 39 different rare diseases have been awarded a PRV, including many diseases that are typically fatal before children reach adulthood. Additionally, more than half of the PRVs were awarded after 2019, the cut-off for the last Government Accountability Office (GAO) analysis into the effectiveness of the PRV program. The Creating Hope Reauthorization Act, which would reauthorize the Rare Pediatric Disease Priority Review Voucher program, is supported by 131 patient organizations.
- Updates funding to reflect how research is currently funded as part of the Best Pharmaceuticals for Children Act (BPCA) to help close data gaps around pediatric uses for approved drugs.
- Strengthens FDA's ability to enforce post-market commitments around pediatric studies.
- Clarifies the original intent of the ODA and codify the FDA's longstanding interpretation that orphan drug exclusivity is awarded based on FDA approved indications, not the much broader orphan designation. The RARE Act is supported by 78 patient organizations.

With the Rare Pediatric Disease Priority Review Voucher program authorization set to expire September 30, 2024, NORD urges Committee members to vote yes on this package without any additional amendments to the parameters of the program to maintain this important tool in ongoing efforts to address the significant unmet treatment needs that exist in the pediatric rare disease population.

H.R.7623, the Telehealth Modernization Act of 2024

For many rare disease patients, there are often only a handful providers in the country with the requisite knowledge to treat their condition. This results in significant geographic barriers to access, resulting in worse outcomes and longer wait times. A 2019 NORD survey of rare disease patients and caregivers found that nearly 40% of respondents traveled more than 60 miles to obtain medical care.[2]

Yet, expanded access to telehealth can help mitigate significant geographic barriers. A subsequent survey conducted by NORD in 2020 found that out of all patients who reported having had a telehealth visit, 92% described it as a positive experience. 70% of respondents also indicated that they would like the option of telehealth for future medical appointments.[3]

The Telehealth Modernization Act would extend flexibilities given to Medicare providers during the COVID-19 pandemic, which include waiving originating site requirements, permitting reimbursement for audio-only telehealth, expanding the types of providers eligible to provide care via telehealth, and permitting rural health centers and federally qualified health centers to receive reimbursement for telehealth for an additional two years. Without Congressional action, these flexibilities are set to expire at the end of

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[2] https://rarediseases.org/wp-content/uploads/2020/11/NRD-2088-Barriers-30-Yr-Survey-Report_FNL-2.pdf

[3] https://rarediseases.org/wp-content/uploads/2022/10/NRD-2098-RareInsights-Telehealth-Report-1.pdf

this year. NORD urges Committee members to advance this important legislation to ensure rare disease patients can continue receiving adequate, timely treatment via telehealth.

NORD is grateful for the Committee's support for the rare disease community and for their consideration of these critical bills at today's markup. NORD urges the Committee to advance these bills and push for their swift consideration on the House floor. Please do not hesitate to reach out to Heidi Ross at HRoss@rarediseases.org, Karin Hoelzer at KHoelzer@rarediseases.org, Hayley Mason at HMason@rarediseaess.org or Mason Barrett at MBarrett@rarediseases.org anytime NORD can be of assistance to the Committee's important work.

Sincerely,

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