NORD
National Organization for Rare Disorders

February 2016

The Honorable Paul Ryan, Speaker
1233 Longworth House Office Building
Washington, DC 20515

Dear Speaker Ryan,

On February 29, millions of Americans will observe Rare Disease Day, an awareness event that takes place on the last day of February each year. At this special time, and on this special day, we ask that you join us in recognizing the successes the rare disease community has achieved, as well as the challenges the rare disease patient continues to face in accessing safe, effective and affordable treatments.

The rare disease community celebrated many achievements in 2015, and 2016 is looking just as exciting. Twenty one new orphan drugs (47 percent of all new drugs) and five new orphan biologics (42 percent of all new biologics) were approved by the FDA in 2015, a record number and percentage. Congress also passed several important measures including the Ensuring Access to Clinical Trials Act (S.139/H.R.209), and the repeal of the SGR and reauthorization of CHIP. There were also critically important pieces of the omnibus, including the additional $2 billion for the NIH, the short-term reauthorization of the rare pediatric disease priority review voucher program, and the additional $2.5 million for the orphan product grants program.

As we enter 2016, the rare disease community is particularly excited about the 21st Century Cures Act (H.R.6). Passed with broad bipartisan support in the House, we are hopeful the Senate HELP Committee will succeed in its parallel Senate Innovation for Healthier Americans Initiative. The 21st Century Cures Act and the accompanying Senate initiative promise a number of reforms that will accelerate the discovery, development, and delivery of therapies for rare diseases.

The 21st Century Cures Act is especially important when considering that 95% of the thirty million Americans with a rare disease have no treatment. While the Orphan Drug Act of 1983 has succeeded in bringing approximately 500 novel orphan drugs to rare disease patients, there is still more work to be done.

We hope that you join us this Rare Disease Day to recognize the importance of rare disease research and orphan therapy development, while recognizing the many challenges the rare disease community continues to face. Please also consider showing your support for the rare disease community this Rare Disease Day on social media, on your website, or on any platform you see fit. We have enclosed sample posts to assist. Approximately one in ten of your constituents has a rare disease, and they and their families will be heartened to know their Representative is standing up for them.

If you desire additional information, please contact Martha Rinker, Vice President of Public Policy, at mrinker@rarediseases.org or at 202-588-5700, ext. 102.

Sincerely,

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