



November 29, 2016

The Honorable Mitch McConnell, Majority Leader
United States Senate
Washington, D.C. 20510

The Honorable Harry Reid, Minority Leader
United States Senate
Washington, D.C. 20510

The Honorable Paul Ryan, Speaker
United States House of Representatives
Washington, D.C. 20515

The Honorable Nancy Pelosi, Minority Leader
United States House of Representatives
Washington, D.C. 20515

Dear Majority Leader McConnell, Speaker Ryan, and Minority Leaders Reid and Pelosi,

On behalf of the 30 million men, women, and children in the U.S. living with a rare disease, the National Organization for Rare Disorders (NORD) is pleased to support the *21st Century Cures Act*, and we urge its swift enactment.

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and the organizations that serve them. NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

There are many provisions within the *21st Century Cures Act* that will improve the discovery, development, and delivery of orphan therapies for rare disease patients, including:

- Extending the Rare Pediatric Disease Priority Review Voucher (PRV) Program through September 2020, with an additional two years extension for products that received an application designation
- Streamlining of FDA review of genetically targeted and protein variant therapies for rare diseases
- Funding in the form of \$4.8 billion over ten years for the NIH to fund the Precision Medicine Initiative, BRAIN Initiative, the Cancer Moonshot, and more
- Requiring companies to publicly post their expanded access policies on their website
- Further expanding of the Patient-Focused Drug Development Initiative and requirements for the FDA to report on how patient experience data was used in regulatory review
- Creating the National Neurological Conditions Surveillance System to better quantify the incidence and prevalence of neurological diseases, including rare neurological diseases
- Expanding of the Humanitarian Use Device program to include devices used by up to 8,000 individuals

NORD will continue to advocate for a strong Rare Pediatric Disease PRV program, additional FDA funding, the enactment of the *OPEN Act*, and the replenishment of funds aimed at prevention.

Thank you again for your commitment to our nation's most vulnerable citizens. We appreciate your time and attention on this important matter.

Sincerely,

Peter L. Saltonstall
President and CEO