NORD
National Organization for Rare Disorders

September 21, 2015

The Honorable Lamar Alexander, Chairman
U.S. Senate Committee on Health, Education, Labor and Pensions
428 Dirksen Senate Office Building
Washington, D.C. 20510

The Honorable Patty Murray, Ranking Member
U.S. Senate Committee on Health, Education, Labor and Pensions
428 Dirksen Senate Office Building
Washington, D.C. 20510

Dear Chairman Alexander and Ranking Member Murray:

On behalf of the 30 million men, women, and children affected by one of the 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks you for your continuing support of the rare disease community, and your dedication to accelerating the discovery, development, and delivery of innovative treatments and cures for the rare disease population through the Senate Innovation for Healthier Americans Initiative. Rare diseases present a public health issue in that the majority (95 percent) still has no treatment.

We are writing today to request that you include Section 2227 of the 21st Century Cures Act titled "Humanitarian Device Exemption Application" in the Innovation for Healthier Americans Initiative. This provision raises the Humanitarian Device Exemption (HDE) eligibility cap from 4,000 uses in a year to 8,000 uses in a year, and gives industry greater clarity on the FDA's perspective on the necessary "probable benefit" for an HDE approval.

We have long supported the Humanitarian Use Device (HUD) and HDE programs as we believe they have been valuable in delivering safe and effective medical devices to small and often forgotten populations. As you know, HUDs treat or diagnose a disease that affects 4,000 or fewer individuals in any given year.

For approval under an HDE, the device must meet certain qualifications. It must be proven safe, and it must carry "probable benefit" for the patient. There must not be any other way to bring this device to market outside of the HDE process, and there must not be any other comparable device in existence. A HUD approved under the HDE review process cannot be sold for more than the cost of research, development, manufacturing, and distribution except for pediatric devices and some devices indicated for adults. HDE applications are exempt from user-fees, and HUDs must be used in IRB-supervised facilities.

NORD supports Section 2227 for several reasons. First, the 4,000 patient limit is an arbitrary number having no scientific rationale, and while 8,000 patients is technically also an arbitrary number, we believe it aligns much more appropriately with the needs of the rare disease patient population.

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Second, there are various rare diseases in the 4,000 to 8,000 yearly incidence range that would greatly benefit from the lifting of the cap. For example, Amyotrophic Lateral Sclerosis (ALS), Cerebral Palsy, Hodgkin's Lymphoma, Mesothelioma, Tuberculosis, and Neuroleptic Malignant Syndrome are just a handful of the rare conditions that currently do not qualify for a humanitarian device exemption, but may see increased device development if this provision were to be passed.

The 4,000 use cap is particularly restrictive on diagnostics. In order for a diagnostic to qualify for an HDE, there must be a reasonable expectation that no more than 4,000 individuals will be given the diagnostic in a given year. Thus, it is likely that only diagnostics that diagnose diseases with an incidence of far fewer than 4,000 individuals, likely only a few hundred, would qualify for the HDE.

The FDA is also proposing using the 4,000 HDE limit to determine what lab-developed tests (LDTs) it will choose to regulate moving forward. LDTs that fall below the 4,000 use threshold will continue to enjoy enforcement discretion from the FDA, while LDTs that fall above this line will be subject to further regulatory requirements if the FDA's October 2014 Draft Guidance on LDT regulation moves forward. This additional regulatory hurdle makes the lifting of the cap from 4,000 uses to 8,000 uses all the more important.

Finally, we support the request for greater clarity from the FDA on the definition and criteria for establishing "probable benefit" in a medical device. This guidance will assist device manufacturers in the development of safe and effective HUDs.

Thank you again for your unwavering commitment to our nation's most vulnerable citizens, and we thank you for your time and attention to this important matter. For questions regarding NORD or the above comments, please contact Paul Melmeyer, Associate Director of Public Policy, at pmelmeyer@rarediseases.org or (202) 588-5700, ext. 104.

Sincerely,

Peter L. Saltonstall
President and CEO