January 10, 2017

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 Greg Walden, Chairman
U.S. House Committee on Energy and Commerce
2125 Rayburn House Office Building
Washington, D.C. 20515

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

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

Re: Expiring Food and Drug Administration Safety and Innovation Act (FDASIA) Provisions

Dear Chairmen Alexander and Walden, and Ranking Members Murray and Pallone:

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.

We are writing today to alert you of several expiring provisions within FDASIA, and to request the reauthorization of these provisions, and their associated programs, within the upcoming user fee reauthorization package. They are as follows:

**TITLE V – PEDIATRIC DRUGS AND DEVICES**

**SEC. 507. REAUTHORIZATIONS**

(c) **HUMANITARIAN DEVICE EXEMPTION EXTENSION**: FDASIA extended the profit allowances for Humanitarian Use Devices (HUDs) from an expiration in 2012, until September 30, 2017. We support the permanent reauthorization of this provision by striking Section 520(m)(6)(A)(iv) of the Federal Food, Drug, and Cosmetic Act and thus removing the sunset date.

Profitability is critical to ensuring the sustained development of HUDs. HUDs, which are devices that treat diseases with an incidence of fewer than 8,000 cases per year, are often the sole medical device treatment option for rare disease patients. Since the inception of the HUD/HDE program in 1990, only 69 Humanitarian Device Exemptions (HDEs) have been granted. With the considerable dearth of treatment options for the vast majority of rare disease patients, removing incentives for development will only make the situation worse.

It is for these reasons that we support the permanent reauthorization of the profit allowance for HUDs.
TITLE VI—MEDICAL DEVICE REGULATORY IMPROVEMENTS

SEC. 620. PEDIATRIC DEVICE CONSORTIA: FDASIA reauthorized the Pediatric Device Consortia by authorizing “$5,250,000 for each of fiscal years 2013 through 2017”. This funding is $750,000 less per year compared to the prior funding awarded to this program. We support the reauthorization of this program, and support more robust funding moving forward.

NORD joined the American Academy of Pediatrics (AAP) in addition to several other physician and patient organizations in a March 17, 2016 letter to Congressional appropriators supporting the Pediatric Device Consortia. To quote from this letter:

“Since their inception in 2009, the Pediatric Device Consortia have been remarkably successful – the nine consortia have assisted in advancing the development of more than 570 proposed pediatric medical devices. Most of the devices supported by the consortia are in the early stages of development, including concept formation, prototyping, and preclinical (animal and bench testing) stages, however eight devices are now available to patients. Because of its innovative model for success Congress’s investment in the PDC Grant Program has enabled the consortia to leverage private sources of funding. In fact, the Pediatric Device Consortia has raised three times the amount of federal investment in private funding.

Examples of devices supported by the Consortia include the “Buzzy” device for relief from pain associated with needlesticks; the Rhinoguard to assist in nasotracheal intubation for children undergoing maxillofacial surgery or dental procedures; and an external compressor brace for pectus carinatum, a deformity of the chest.

Medical devices for children often lag 5-10 years behind those for adults. Pediatric populations pose significant challenges for device manufacturers as there are many factors that limit children’s access to safe and effective medical devices, including differences in size, weight, and metabolism rate.”

Given the continued success of the Pediatric Device Consortia, we support more substantial authorizations to be included within the user fee reauthorization package.

TITLE IX—DRUG APPROVAL AND PATIENT ACCESS

SEC. 906. GRANTS AND CONTRACTS FOR THE DEVELOPMENT OF ORPHAN DRUGS: FDASIA authorized $30,000,000 for each of fiscal years 2013 through 2017 for the Orphan Products Grants Program. This program was created by the Orphan Drug Act in 1983. According to the FDA, “since the program’s inception in 1983, OOPD has received over 2500 applications (generally, about 100 applications/year), reviewed over 2200, and funded over 590 studies. The Orphan Products Grants Program has been used to bring more than 55 products to marketing approval.”

This program has served as an invaluable incentive in encouraging orphan drug development. We request the continued authorization of funds for this program to be included within the user fee reauthorizations.

Thank you again for your unwavering commitment to our nation’s most vulnerable citizens. We appreciate your time and attention to this important matter. For questions regarding NORD or the
above comments, please contact Martha Rinker, Vice President of Public Policy, at mrinker@rarediseases.org or at 202-588-5700, ext. 102.

Sincerely,

Peter L. Saltonstall
President and CEO

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http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/WhomtoContactaboutOrphanProductDevelopment/ucm134580.htm