



**Public Meeting – Reauthorization of the Medical Device User Fee  
Program  
Patient Panel  
July 13, 2015**

The National Organization for Rare Disorders (NORD) thanks the Food and Drug Administration (FDA) for inviting us to present today on our views on the Medical Device User Fee (MDUFA) program.

NORD represents the over thirty million Americans with a rare disease, which is defined as a disease that affects 200,000 individuals or fewer in the United States in a given year. Two thirds of rare disease patients are children, and over 80% of the approximately 7,000 known rare diseases are genetic. Of these 7,000 rare diseases, only 350 have an FDA approved treatment. Clearly there is much work still to be done.

The symptoms and experience of rare disease patients can differ dramatically, yet there are common struggles faced by rare disease patients. The average time to diagnosis for a rare disease patient is seven years, and there are still millions of undiagnosed patients searching for an answer. There are very little treatment options for rare disease patients, and when treatment exists, it is usually very expensive due to the small disease population, leading to various reimbursement problems.

Following the passage of the Orphan Drug Act in 1983, NORD was founded by a coalition of patient advocates in the attempt to tackle these very problems. Over the past thirty years, we have provided policy and regulatory advocacy, educational programs, patient assistance programs, patient networking meetings, organizational counseling to our patient organization members, and more for the entire rare disease stakeholder population.

**FDA FUNDING:**

First and foremost, we must ensure that the user fee agreement funds the FDA and the Center for Device and Radiological Health (CDRH) appropriately, as there must be a proper balance between user fees and congressionally appropriated funds in order to ensure the safe and expedient review of medical devices. We are proud to be founding members of the Alliance for a Stronger FDA where we have advocated successfully for increased appropriations for the FDA. We look forward to advocating for increased funding for the FDA through both user fees and appropriations.

**PATIENT INVOLVEMENT IN THE DEVICE DEVELOPMENT PROCESS:**

Second, we must strengthen and incorporate the patient voice throughout the device development process. The Medical Device Innovation Consortium (MDIC), of which NORD is proud to be a

founding member, has been making great strides in developing proposals for including patient preference data in the device review process. In April, MDIC released two documents; the “Catalog of Methods for Assessing Patient Preferences for Benefits and Harms of Medical Technologies”, and the “Framework for Incorporating Information on Patient Preferences Regarding Benefit and Risk into Regulatory Assessments of New Medical Technology”. These proposals include provisions to better incorporate the patient’s perspectives in the device development process.

NORD also believes there should be greater coordination across centers on patient involvement. There are various patient engagement initiatives happening across the Agency, but there is little coordination between centers on these programs.

Finally, we want to ensure the patient voice is included throughout the development process, not just in the FDA review stage. Many patient involvement proposals focus on the review of the drug or device when the patients’ benefit/risk viewpoint is considered. However, the patient’s perspective must be included throughout the development of the device so the final result reflects the patient population’s needs.

#### HUMANITARIAN USE DEVICES:

Third, NORD aims to ensure the Humanitarian Use device (HUD) program remains strong. Humanitarian Use devices treat or diagnose a disease that affects 4,000 or fewer individuals in any given year. HUDs are approved for marketing through a Humanitarian Device Exemption (HDE).

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 in 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 wants to ensure that the HUD and HDE programs remain strong within the FDA, and the proper incentives for the development of humanitarian use devices are in existence in order to ensure patients receive the proper care they need.

#### CLOSING:

I would like to close with the story of a very brave patient, Devin Alvarez. The Titanium Rib was first developed in 1987 for children with certain spinal malformations, and over the course of the next 18 years has been improved upon several times.

Devin, diagnosed with Sprengel’s Deformity at birth, was not expected to live more than a week, and then no more than a few months, and then again no more than a few years. Thanks to the Titanium Rib, Devin was able to play baseball growing up, and has recently graduated high school and would like to pursue a career in music.

Devin's words show the power that innovative medical devices can have on one's life. Honored at NORD's gala in May of this year, Devin recently said,

"I want to make music that inspires others to never lose hope, to never stop seeing the light, and to keep going. Trust me, I did not know if I was going to live or going to die; I did not see myself reaching the age of 13, and then I did not see myself graduating high school, and I'm doing it. My story is for everyone."

We again thank the FDA for allowing us to present our views on the reauthorization of the Medical Device User Fee program, and we look forward to continuing the conversation as the MDUFA reauthorization process moves forward.

Again, thank you.

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