Public Meeting - Reauthorization of the Medical Device User Fee Program

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Paul Melmeyer
Associate Director of Public Policy
Introduction to NORD and Rare Diseases
Facts About Rare Diseases

• There are an estimated 7,000 rare diseases
• 1 in 10 or nearly 30 million Americans have one or more rare diseases
• A rare or “orphan” disease in the US affects less then 200,000 people in a year
• Two-thirds of people with rare diseases are children
• 80% of rare diseases have a genetic component
• Orphan drugs are drugs used to treat rare diseases
• Only about 350 diseases have an FDA approved treatment
Experience of Patients

- It can take years for patients to obtain an accurate diagnosis
- There are limited treatment options for most rare diseases
- Lack of knowledge among many medical professionals
- Treatments are generally more expensive than for regular diseases
- Reimbursement issues related to private insurance, Medicare and Medicaid
In the decade before 1983, only 10 new treatments were brought to market by industry for diseases that today would be defined as rare.

Leaders of rare-disease patient organizations began to realize that there were certain problems their patients and families shared...problems that were common to all people with rare diseases.

A small story in the LA Times led to an episode on a popular TV show, Quincy ME. Then letters began to arrive from people all over the nation who had rare diseases and thought they were alone in their struggles.

The Orphan Drug Act passed in 1983 and the patient leaders who had worked to bring national recognition to the problem founded NORD as an umbrella organization to represent the rare disease community.

Today, NORD provides information, advocacy, research, and patient services to help all patients and families affected by rare diseases.
Major NORD Programs and Initiatives

- Policy and regulatory advocacy
- State advocacy and alliance partnerships
- Patient representation (FDA, NIH, SSA)
- Education (patients, professionals, public)
- Mentoring (patient organizations)
- Patient assistance programs
- Patient Networking (disease specific meetings, online communities, creation of new patient organizations)
- Increase disease understanding (Research grants, patient registries)
- International Partnerships
- US Sponsor of International Rare Disease Day
Goals for MDUFA
Goals for MDUFA IV

• Fund the FDA and CDRH appropriately in order to ensure safe and expedient review of medical devices
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• Strengthen the patient voice in the medical device development and review process
Medical Device Innovation Consortium (MDIC)

• Patient Centered Benefit-Risk Project (PCBR)
  – Catalog of Methods for Assessing Patient Preferences for Benefits and Harms of Medical Technologies (April 22\textsuperscript{nd}, 2015)
  – Framework for Incorporating Information on Patient Preferences Regarding Benefit and Risk into Regulatory Assessments of New Medical Technology (April 22\textsuperscript{nd}, 2015)

• Incorporates the patient perspective throughout the device development process
Goals for MDUFA IV

• Fund the FDA and CDRH appropriately in order to ensure safe and expedient review of medical devices
• Strengthen the patient voice in the medical device development and review process
• Ensure the Humanitarian Use Device program remains strong
Humanitarian Use Devices (HUDs)

• Treats or diagnoses a disease that affects 4,000 or fewer individuals

• Approved for marketing through a Humanitarian Device Exemption (HDE)
  – Required to be safe with a favorable risk/benefit assessment
  – Data on efficacy is NOT required for approval
  – Must show there is no other way to bring this device to market
  – No other comparable device in existence
Humanitarian Use Devices (HUDs)

• Approved for marketing through a Humanitarian Device Exemption (HDE) (continued)
  – Cannot be sold for more than R&D, manufacturing, and distribution costs.
    • Profit exemption for pediatric devices
  – Used in IRB-supervised facilities
  – Exempt from MDUFA user-fees
The Titanium Rib Development
1987-2005

-IOM Report, Safe Medical Devices for children, 2005
Before...
After...
“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.”

Devin Today
Thank You!

pmelmeyer@rarediseases.org