Public Meeting – Reauthorization of the Prescription Drug User Fee Program

Patient Panel

July 15, 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 Prescription Drug User Fee (PDUFA) 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 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 drugs and biologics. 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 DRUG DEVELOPMENT PROCESS:

Second, we must strengthen and incorporate the patient voice throughout the drug development process. We are supportive of the Patient-Focused Drug Development initiative that was enacted as part of the

Food and Drug Administration Safety and Innovation Act (FDASIA), and have been pleased to assist the FDA in facilitating the Patient-Focused Drug Development meetings, many focused on rare diseases.

However, we need to take the next step in Patient-Focused Drug Development and open up the program to patients whose conditions do not fall within the twenty mandated meetings. Patient organizations should have the opportunity to hold their own Patient-Focused Drug Development meetings with the FDA's attendance and participation. Outside of Patient-Focused Drug Development, patient organizations should also have the opportunity to submit draft guidances to the Agency on their disease much like the Duchenne Muscular Dystrophy community has recently done.

While patient participation has increased on FDA Advisory Committees, we still too often hear of Advisory Committees that are unable to find patients, family members, or medical experts to sit on Advisory Committees due to the overly stringent conflict of interest rules. This is a particularly pervasive problem for Advisory Committees reviewing orphan therapies as the rare disease stakeholder population is incredibly small. The entire community for a rare disease must come together to coordinate the fight against the disease, and this can often leave the Agency deeming almost the entire community as conflicted. These rules must be readdressed for the rare disease population.

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 when the patients' benefit/risk viewpoint is considered. However, the patient's perspective must be included throughout the development of the drug so the final result reflects the patient population's needs.

ORPHAN DRUG DEVELOPMENT:

We must also ensure that orphan drug incentives remain strong. The Rare Pediatric Disease Priority Review Voucher program, enacted as Section 908 of FDASIA, provides companies with a priority review voucher after they first develop a drug for a rare pediatric disease indication. This program is set to expire in March of 2016, and if not reauthorized as part of the 21st Century Cures Act, will need to be revisited when discussing legislation to pass the PDUFA reauthorization.

Second, the Orphan Products Grant Program should be strengthened and expanded. Section 906 of FDASIA included funding for this program that is set to expire in 2017. At least 45 orphan therapies have come to market that received funding from this program, and we want to ensure it continues to be a strong incentive for orphan product development.

OFF-LABEL USE OF MEDICATIONS:

The vast majority of rare disease patients are treated off-label, and we need to ensure that therapies that can treat a rare disease, yet don't have that disease on the label, can reach that patient population.

CONSISTENCY ACROSS REVIEW DIVISIONS:

Finally, we want to ensure there is consistency across review divisions in the use of expedited review pathways. Rare disease therapies often benefit from the use of expedited review pathways, but some review divisions are particularly hesitant to use these pathways for rare disease therapies. We need to encourage greater consistency across the Agency in the use of these tools.

CLOSING:

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

Again, thank you.

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