National Organization for Rare Disorders
Remarks at the FDA "Manufacturer Communications Regarding Unapproved Uses of Approved or Cleared Medical Products" Public Hearing
White Oak, Maryland
November 9, 2016

Good afternoon. My name is Paul Melmeyer, Associate Director of Public Policy at the National Organization for Rare Disorders (NORD). I have no disclosures.

I am here today on behalf of the men, women and children in the United States suffering with one of the 7,000 known rare diseases that, in the aggregate affect well over 30 million people.

NORD, a 501(c)(3) organization, is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. NORD's mission is to ensure that all people with rare diseases have access to diagnostics and therapies that extend and improve their lives, and that the United States maintains a regulatory environment that encourages the development and timely approval of safe and effective diagnostics and treatments for patients affected by rare diseases.

While we have made significant advances in the development and approval of rare disease therapies, the vast majority of individuals with rare diseases are left without an FDA-approved treatment indicated for their disease. Only approximately 375 of the 7,000 known rare diseases have an FDA-approved treatment, leaving between 80 and 90 percent of rare disease patients with no other option than to depend on therapies prescribed off-label.

There are two main barriers for individuals with rare diseases attempting to gain access to therapies prescribed off-label. First, due to restrictions in place on the communication of benefits of off-label use, many, if not most physicians who treat individuals with rare diseases are unaware of potential benefits therapies may have for their patients if prescribed off-label.

Primary care physicians and specialists alike see very few individuals with rare diseases, and thus are likely to be largely unfamiliar with treatment options. Dissemination of off-label information is quite limited, often only including reprints of peer-reviewed journal articles without abridgement.

Unfortunately rare diseases are rarely investigated within peer-reviewed journal articles, or included in compendia. Physicians may be totally unaware of potential therapies for patients with a rare disease. Even if they are aware, the information available is generally not tailored to the specific rare disease in question, leaving substantial ambiguity on how to care for the patient using the off-label treatment.

This needs to change. Individuals with rare diseases need a regulatory framework that permits the sharing of truthful and non-misleading information so their doctors, insurers and PBM's are aware of rapidly evolving science and the increased pace of new medicines.

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The second barrier to accessing therapies off-label occurs when insurers and PBM's will not reimburse for an FDA-approved drug or medical device that a physician has prescribed for an off-label use …notwithstanding that the use is specific to the patients' medical needs and backed by scientific evidence.

There are various disincentives for testing therapies in rare indications such as the cost of the requisite trials, the small size of the market, insufficient number of patients to conduct a trial, and potential ethical issues. One or more of these issues often disincentivize companies from studying the impact of a drug developed for a common disease, or another rare disease, in a small rare disease patient population that might benefit.

This results in a dearth of FDA-reviewed data for the use of therapies prescribed off-label. Still, these data can often be generated through real-world evidence collection using surveillance systems or registries.

This lack of FDA-reviewed data often leads both public and private insurers to reject coverage of therapies used off-label for rare diseases. With the rejection of coverage, patients are required to pay most, if not all, of the full cost of the drug outside of their out of pocket limit on their insurance plan.

Individuals with rare diseases are faced with having to pay thousands of dollars to access therapies prescribed off-label. This leads to an equity issue where only the wealthy can afford treatment prescribed off-label for their rare disease, while the poor cannot.

We have a few simple requests of the FDA. First, we ask that the FDA provide greater clarity on what constitutes allowable off-label communication. The rare disease community would benefit from drug and device manufacturers being able to, without fear of regulatory or legal action, share with physicians and with the patient community, medical information that would help enhance the use of their products in patients with rare diseases, even when that information has not been added to the FDA-approved labeling. Manufacturers are well positioned to provide that information. We believe that information from manufacturers is likely to be more reliable and accurate than information from many other sources.

We also ask that the FDA do everything it can to facilitate data collection from rare disease patient populations. This includes, but is not limited to, supporting efforts to facilitate the collection of real world evidence. We are very pleased that real-world evidence provisions are included within the PDUFA VI commitment goals letter, and we thank FDA for their inclusion.

Finally, we want the FDA to understand that the rare disease patient community is ready and willing to partner in the development of policies to address the critical need in the rare disease patient population for reliable information about off-label uses of approved drugs and devices. While we await the continued development of orphan therapies, NORD and its members recognize that drugs and devices used off-label are often the sole treatments for our patients. Facilitating the ability of companies to provide truthful and non-misleading information is a priority of ours, and we hope to partner with the FDA to address it collaboratively.

Thank you again for the opportunity to participate in today's hearing.

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