September 21, 2018

Division of Dockets Management (HFA-305)
U.S. Food and Drug Administration
5630 Fishers Lane, Room 1061
Rockville, MD 20852

Re: Docket No. FDA-2018-N-2689: Facilitating Competition and Innovation in the Biological Products Marketplace; Public Hearing; Request for Comments

Dear Sir or Madam:

On behalf of the 30 million Americans with one of the 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks the Food and Drug Administration (FDA) for the opportunity to provide comments in response to the Agency's "Facilitating Competition and Innovation in the Biological Products Marketplace; Public Hearing; Request for Comments."

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

NORD has long supported a robust biological products marketplace. Innovative biologics offer hope of longer, healthier lives for rare disease patients, and consistent biosimilar development allows for greater access to affordable innovative therapies. Consequently, NORD lauds FDA for making the wellbeing of the biological marketplace a top priority.

In many ways, FDA has a unique ability to gather stakeholders in deliberation. In doing so on September 4, FDA gave renewed emphasis to crucially important aspects of the further development and sustainment of biological products. The following comments detail those aspects that NORD finds of particular importance.

Education

NORD wholeheartedly agrees that FDA must increase its efforts to educate stakeholders on biological products. To do that, NORD recommends that FDA work closely with the advocacy groups that represent said stakeholders. Specifically, NORD encourages FDA to reach out to patient organizations for consultation. There is still much about biological products that is not widely understood in the patient community. NORD would be happy to work with FDA to improve understanding of the complex yet enormously beneficial role of biologics and biosimilars within the rare disease community. For example, NORD could partner with FDA to create focused webinars or incorporate education on biological products into one of NORD's educational programs for medical professionals.

The Purple Book

Another way FDA could improve stakeholder education is through improvements made to the Purple Book. The Purple Book is often cited and recognized as the primary source of guidance when prescribing, dispensing, or evaluating a biological product, and yet it is severely lacking in instruction. The lists that compose the Purple Book do not contain much information, and the information that is included is incomplete. Dates for exclusivity expiration and first licensure, which are crucially relevant dates in the development of biosimilars, are missing for the majority of the products listed.

Further, particularly when compared to the Orange Book, the Purple Book is missing a significant amount of important information. The Purple Book could include information on dosage form, route of administration, and patents to name a few. The Purple Book could also include vitally important information on the relationship between biological products beyond whether a biosimilar is a "B" for biosimilar or an "I" for interchangeable. Given the precarious and unique nature of biologics and biosimilars, FDA should take advantage of every opportunity to enhance clarity, increase confidence, and assuage concerns. All stakeholders should be able to refer to the Purple Book and clearly understand what it means for a product to be a biosimilar to a reference biologic.

Perhaps even more importantly, all stakeholders should be able to refer to the Purple Book and understand that, unlike with small-molecule drugs, biosimilars with the same reference product are not necessarily interchangeable. NORD hopes that FDA will acknowledge the important role that the Purple Book plays in setting the stage for practitioners and patients alike and, consequently, will increase its functionality.

Interchangeability

NORD is disconcerted by the amount of uncertainty surrounding interchangeability standards and strongly recommends that FDA offer clarity through finalized guidance. NORD is of the opinion that in order for the biological product marketplace to flourish, there must be clear guidelines and commonly understood foundational boundaries when considering switching between biological products.

As mentioned above, this does not just apply to a biosimilar and its reference product but also between two biosimilars with the same reference product. NORD is deeply concerned that until FDA can provide additional structure surrounding interchangeability, patient safety and the prosperity of the biological product marketplace will remain at risk.

Exclusivity

NORD adamantly supports the protection of exclusivity granted by orphan designation. For those orphan indications of biologics, exclusivity must remain intact for seven years. Once this exclusivity does lapse, however, NORD believes that FDA should do what it can to assist manufacturers of biosimilars in adding previously protected indications to a biosimilar's label. In doing so, however, NORD urges FDA to be careful to not endanger the value of exclusivity for protected indications prior to expiration, particularly for orphan exclusivity, and to keep patient safety the top priority. At no point

prior to the expiration of the exclusivity should FDA publicly consider the addition of the relevant indication to a biosimilar's label.

Furthermore, within the realm of exclusivity, NORD believes that it would be appropriate for FDA to explore implementing umbrella exclusivity for biological products similar to that of provisions under Hatch-Waxman. So long as companies would be unable to exploit and extend the exclusivity beyond the intended twelve years, NORD is of the opinion that it would be beneficial to all stakeholders for FDA to remove a disincentive to improve an innovator product.

Naming

While labeling was not an official topic of discussion at the hearing on September 4, NORD wishes to reiterate its long-standing belief that biosimilars ought to have distinct non-proprietary names. A potentially negative impact on competition does not justify the threat to patient safety that would result from removing the unique identifiers. Confidence in prescribing and using biosimilars will not stem from ignoring the differences between biosimilars. Rather, confidence will emanate from focusing on the differences, learning the differences, and understanding the differences.

We thank FDA for the opportunity to comment, and we look forward to working with FDA to ensure the success of the biological products marketplace. For questions regarding NORD or the above comments, please contact me at pmelmeyer@rarediseases.org, or 202-545-3828.

Thank you in advance for your consideration.

Sincerely,

Paul Melmeyer
Director of Federal Policy