June 3, 2014

The Honorable Margaret Hamburg, Commissioner
The Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, MD 20993

Dear Commissioner Hamburg,

The National Organization for Rare Disorders (NORD) urges you to adopt distinguishable names\(^1\) for biologics, including biosimilars. Our position stems from the existing lack of consistent naming protocols for biologics and the potential for significant physician and patient confusion once biosimilars become available.\(^2\) The need for distinguishable names is independent of FDA’s latest guidance on levels of evidence of biosimilarity.

With nearly 7000 rare diseases identified and 30 million Americans affected, the population represented by NORD is extraordinarily heterogeneous. We believe strongly that every patient deserves the medical care that is best suited for their medical situation and that is most likely to give them the best results. Based on the reports we receive from member organizations, as well as individuals, it is increasingly difficult for rare disease patients to receive optimum care if any degree of customization to individual patients is required.

In light of this problem of access to optimum care, distinguishable names for biologics, including biosimilars, addresses three important concerns to the rare disease community.

**Distinguishable names for biologics support the medical community’s vital post-approval learning curve about which medicines are best for their rare disease patients.** Health care providers need to know that a prescribed medicine was actually given to the patient and whether a substitution was made and to what alternative product. This can’t be achieved unless biologic products—especially ones with similar therapeutic purposes—cannot be distinguished, tracked and studied.

This is a critical issue for a large segment of the rare disease community: patients who are treated—on or off-label—with medicines that have a primary indication that is not for their specific rare disease. Which drug will work for what patients in what therapeutic regimen is, for these patients, almost entirely based on a post-approval trial and error process conducted by clinicians and researchers. Similar medications might work equally well, but such a judgment can only be made based on tracking of results that relies on distinguishable biologic names.

\(^1\) We consider the specific format to be a technical issue and have no position at this time on how FDA should assure that biological products be distinguishable, e.g. prefixes, suffixes, etc.

\(^2\) Closely related to the naming issue, we urge FDA to require the labeling of biosimilars to specify the reference product so that biosimilars cannot inadvertently be confused within a therapeutic category of similar, but not identical originator products.
Distinguishable names for biologics support surveillance and tracking of adverse events. Rare disease patients often do not respond to medications the same way other individuals might. Idiosyncratic reactions to medications occur frequently.

As a group, rare disease patients are more dependent than others on “medical necessity” determinations. Quite often, this is based on changing medications because of adverse events. For an individual, this might be an unpleasant, expensive and perhaps life-threatening event, but the record is then established for that individual that alternative therapy is needed. What rare disease patients need is accurate surveillance and clinical tracking data so that subsequent patients don’t have to fail therapy or experience an adverse event before justifying a medical necessity determination to receive a different medication.

Distinguishable names for biologics reinforce a critical distinction in the biosimilars law between biosimilars (similar not identical) and interchangeable biosimilars (similar but demonstrated to have comparable clinical results). NORD welcomes biosimilars because many rare disease patients will benefit from having additional therapies that might be available at a substantial discount to current products. However, there is no assurance—legal, clinical or scientific—that biosimilar products will work the same as the reference product in rare disease patients. Even when interchangeable biosimilars are approved by FDA—likely well into the future—it still will not always be the case that rare disease patients will respond to a biosimilar in the same way as they do to the reference product.

Having distinguishable names would promote the needed learning curve and assist with tracking of adverse events. Critically, distinguishable names for biologics would serve as a reminder to payers, states and health care facilities that market access has been granted only on the basis of similarity and not on the basis of substitution or interchangeability. This is very different from the situation with generic drugs, where common names are used and FDA’s approval findings include whether interchange or substitutions is supported by evidence.

Commissioner, thank you for your attention to this issue, which is of the utmost importance to rare disease patients. If we can supply additional information, please do not hesitate to let us know. For questions regarding NORD or the above comments, please contact Diane Dorman, Vice President of Public Policy, at ddorman@rarediseases.org or (202) 588-5700 ext. 102.

Respectfully,

Peter L. Saltonstall
NORD President and CEO

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