Immune Deficiency Foundation

The National Patient Organization Dedicated to Advocacy, Education and Research for Primary Immunodeficiency Diseases

Stephen Ostroff, M.D.
Acting Commissioner
Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, MD 20993

RE: Comment on Food and Drug Administration Draft Guidance "Nonproprietary Naming of Biological Products: Guidance for Industry"

Submitted electronically via www.regulations.gov

September 16, 2015

Dear Acting Commissioner Ostroff:

The Immune Deficiency Foundation (IDF) is the national patient organization, founded in 1980, dedicated to improving the diagnosis, treatment and quality of life of persons with primary immunodeficiency diseases through advocacy, education, and research. Primary Immunodeficiency Diseases, or PI, represent a group of more than 250 related, rare genetic diseases. The defining characteristic throughout each of these conditions is that the immune system is malfunctioning or non-existent, resulting in a decreased ability to fight off infection. IDF is a member of Patients for Biologics Safety and Access, a coalition representing over 20 national patient organizations.

Many patients diagnosed with PI require lifelong treatment with immunoglobulin (Ig) replacement therapy to replace missing or improperly functioning antibodies needed to fight infection. Since the treatment only replaces the missing antibodies and does not correct the defect in antibody production, immunoglobulin replacement is usually necessary for the patient's whole life. Depending on the route of administration, PI patients require infusions as often as every 2 or 3 days (subcutaneously) or once every 3 or 4 weeks (Intravenously).

Ig treatment is expensive: a single treatment can cost thousands of dollars. Perhaps better than anyone, our patients understand that the cost of biologics can be high, and that there is an urgent need for less expensive alternatives. This is why we welcome biosimilars to the U.S. market. Given the fragile health of the patients we represent,

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we've played an active role in the policy discussion of biosimilars, including in the debate about what nonproprietary name format biosimilars should bear.

We were very pleased to see the Food and Drug Administration (FDA) finally declare its policy on the naming of biosimilars and, more importantly, to prioritize patient safety in its proposed naming approach. Indeed, in "Nonproprietary Naming of Biological Products: Guidance for Industry" ("draft guidance"), the agency reiterates many of the concerns IDF has voiced since enactment of the biosimilar approval pathway in 2010. We believe it is a safety issue that patients should know what biologic is being put in their bodies, including the manufacturer and whether a therapy is a biosimilar. The same can be said for prescribers as well.

For example, FDA notes that biological products, because they are large and complex, "raise unique safety concerns related to immunogenicity." This accurately reflects the science and acknowledges the concerns voiced by patient and provider groups over the last few years. In addition, FDA notes its belief that any naming convention the agency decides to implement "should help prevent inadvertent substitution."

As we have stated before, we agree with the risk of inadvertent substitution, which is why we urge FDA to adopt a distinguishable suffix for interchangeable biosimilars as well. If and when the agency decides that a certain biosimilar is interchangeable with its reference product, that decision should be reflected on the label.. However, it is still within the treating physician's discretion to prescribe the reference product if (s)he so chooses, based on the patient's clinical history. If two products share an identical name and suffix, the risk of a physician inadvertently substituting one product for the other becomes much greater.

In addition, while we recognize that insurer practices are outside the scope of FDA's jurisdiction, we do urge you to keep in mind the effect of the agency's decision on coverage and access. When two products share the same suffix, it may be easier for an insurer to provide access to only one of the two, under the argument that these are two identical products and any formulary requirements are met by providing access to one of the two. This would create even more obstacles for our chronically ill, high cost patients to access the treatments their physicians believe are necessary – a challenge that is already difficult in a world without interchangeable biosimilars. In light of these concerns, we urge the agency to consistently require <u>all</u> biological products to bear a unique suffix, in acknowledgement of the fact that many of these products are large, complex, and difficult to replicate.

Finally, we believe that a robust adverse event reporting system requires unique names for all biological products, whether they are reference products, biosimilars, or interchangeable biosimilars. There are significant, documented obstacles to correct attribution in the existing FDA Adverse Event Reporting System (FAERS),[1] and we believe that a unique nonproprietary name will help reduce the existing difficulties with identifying the correct manufacturer of a product that is causing adverse events in patients.

In closing, we thank you for a guidance that we consider to be a major step in the right direction to protect patient safety and physician discretion, and we urge you to extend the proposed policy of a distinguishable suffix to all biologics. Please do not hesitate to contact me, should you require additional information. Thank you for your attention.

Sincerely,

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[1] See, e.g., "Biosimilar Naming: How Do Adverse Event Reporting Data Support the Need for Distinct Nonproprietary Names for Biosimilars?" by Erika Lietzan, Laura Sim, and Emily Alexander, The Food and Drug Law Institute's *Food and Drug Policy Forum*, Vol. 3, Issue 6 (March 27, 2013).