

February 25, 2014

The Honorable Edith Ramirez
Federal Trade Commission (FTC)
Chairperson
600 Pennsylvania Avenue, NW
Washington, DC 20580
Filed online at <https://ftcpublic.commentworks.com/ftc/biologicsworkshop>

RE: Workshop on Follow-On Biologics: Project No. P131208

Dear Commissioner Ramirez,

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 (PI) disease through advocacy, education and research.

Primary immunodeficiency disease is a constellation of disorders disrupting the immune system resulting in a spectrum of illnesses. In some cases, the body fails to produce any or enough antibodies to fight infection. In other cases, the cellular defenses against infection fail to work properly. There are more than 185 different primary immunodeficiency diseases currently recognized by the World Health Organization. The number of Americans now living with PI is estimated to be about 250,000.

As representatives of patients around the U.S. whose health could be impacted by the ability of biosimilars drugs, we applaud the FTC's decision to hold hearings to examine issues of market access and competition for products that will be approved by the Food and Drug Administration's (FDA) biosimilars pathway. Biosimilars can be of value to patients only if they are accessible and affordable.

However, as biosimilars are developed and approved in the United States, we urge the FTC to uphold patient safety above all else. Patient safety and consumer protection is particularly pertinent in an area that FTC has chosen to address: the naming of biosimilars products. Before approving the first biosimilars in the U.S., the FDA will first need to establish a naming policy for all biologic products, including biosimilars. Given the vast differences between chemical compounds and biologics/biosimilars, IDF supports the adoption of unique names for biosimilars and biologic products, rather than requiring biosimilars and their innovative reference product to share the same name.

All biosimilars should carry unique, non-proprietary names. IDF believes that patient safety across the nation will be best served if distinguishable names are required for all biologics. By

providing clarity of information dating back to the point of prescription, distinguishable names facilitate the process of determining the cause of an adverse event (AE) by creating a more expeditious route back to the origin of the problem. Some problems that may be particular to a specific biological product may even be entirely untraceable without distinguishable names or other distinct identifiers.

Accordingly, through the way it sets naming policy, the FDA can provide the best possible protection for other patients also using similar products, while simultaneously ensuring the highest credibility for biosimilars as they enter the marketplace. All products, including biosimilars, should carry unique nonproprietary names, brand and lot information to quickly trace a product to an adverse event. We encourage the FTC to give broad exposure to this viewpoint and to adopt it as their conclusion and recommendation to FDA.

The patients we represent have an enormous stake in the FDA's regulatory framework for biosimilars. IDF is keenly interested in the FDA guidelines for biosimilar manufacturers, and we believe the agency's foremost responsibility is to ensure that biosimilars are manufactured and prescribed safely. Therefore, IDF has also recommended that the FDA exempt immunoglobulin therapies from the biosimilars pathway at this time.

Exempt Immunoglobulin Therapies from the Biosimilars Pathway. Patients with a primary deficiency face additional risks from adverse reactions to biosimilars that have not been adequately tested for safety and efficacy. As states move forward to consider legislation related to dispensing biosimilars, it is important that the FDA exempt immunoglobulin therapies from the biosimilars pathway to adequately protect patient safety for these products.

Many patients diagnosed with PI require biologic medicines for long-term management. Specifically, antibody (immunoglobulin) replacement therapy is used to replace missing or improperly functioning antibodies needed to fight infection. Without lifelong immunoglobulin treatments, individuals with PI are unable to fight off even minor infections, including the common cold. PI is one of the indications approved by the FDA for Ig replacement and represents a significant use of the immunoglobulin therapy in the United States.

Therapeutic immunoglobulins are complex biologics, available in intravenous (IVIG) and subcutaneous (SCIG) modes of administration. These medicines are derived from human blood product, or plasma, sourced from tens of thousands of donors. The FDA requires manufacturers to maintain strict safety standards; however, the risk of infection from not having the proper antibodies can never be fully eliminated. Manufacturing changes, the composition of donor pools, and final formulations can impact patients' tolerability, the infusion rate, and potential efficacy and safety.

Concerning safety, the FDA recognizes each immunoglobulin brand as *unique* and requires each biologic to develop and complete an individual clinical trial protocol to receive licensure, even if it is from the same manufacturer. This reflects the many processing steps involved in plasma

fractionation, purification, stabilization and virus inactivation or removal that yields products that are distinct from one to another.

Unlike small-molecule drugs, plasma therapies such as Ig are natural proteins of the human body and can differ in terms of processing and end composition. The worldwide voluntary withdrawal of an Ig product in 2010 by a major manufacturer due to increased reports of thromboembolic events thought to be caused by a change in the manufacturing process approved by the FDA highlights the fragility of this class of medicines.

The FDA should exempt immunoglobulin therapies from the biosimilars pathway until the science advances significantly. This will be in keeping with the European Medicines Agency (EMA), which opted to exclude immunoglobulin from its regulatory pathway for biosimilars. It will also ensure that the FDA is appropriately focusing on international harmonization. Patients should be protected by similar safety standards, no matter where they receive the product.

In summary, all medicines must be thoroughly tested and meet the highest standards set by the FDA. Given the unique properties of biosimilars, and immunoglobulin therapies in particular, the focus should be on making sure that the biosimilars approval process meets the same strict criteria required for current manufacturers.

On behalf of patients with primary immunodeficiency diseases, I want to thank you for your consideration of our comments and concerns.

Sincerely,

Marcia Boyle
President and Founder
Primary Immune Foundation

Cc:

Margaret Hamburg, MD; Commission of Food and Drug, U.S. Food and Drug Administration

Janet Woodcock, MD; Director of the Center for Drug Evaluation and Research, U.S. Food and Drug Administration