ADVOCACY: BIOSIMILARS

The Patient Protection and Affordable Care Act (ACA) established a new regulatory pathway for a class of drugs known as biosimilars. Biosimilars, also known as "follow on biologics," are medicines that are similar to, but not exact copies of, biologically derived drugs. Biologically derived drugs are called "biologics." A biologic[1] is a drug or vaccine made from a living organism. Biologics are very complex and extremely difficult to manufacture. Unlike chemically derived drugs that can have generic versions, biosimilars are not generic versions of the original biologic they seek to mimic. Biosimilars are only similar to the original biologic and not an exact replication. The upside for biosimilars is that they may be less expensive than the biologic they mimic and thus more patients could have access to these less expensive drugs. On March 6, 2015 the FDA approved the first US biosimilar, Zarxio, which treats various cancer conditions. The new abbreviated regulatory pathway established by the ACA may enable patients to have access to more affordable biosimilar products. Easier access to biosimilars also comes with risks to patient safety that requires evaluation. An abbreviated regulatory pathway that allows for shorter drug evaluation time and quicker approval of biosimilars has been at the forefront of cost-savings discussions; however, past lapses in blood product safety have led to tragic consequences for patients with bleeding disorders, thus it is extremely vital that quicker access does not come at the price of safe products.

Individuals with bleeding disorders depend on Factor Replacement Therapy (either plasma based or recombinant) for the treatment of their disorder. Factor is a biologic used to replace the missing or deficient protein needed for blood to clot. This dependence on biologics makes the safe development, approval, and use of biosimilars of factor products an extremely vital issue for individuals with bleeding disorders. As it relates to product safety, three issues surrounding biosimilars are critical to the bleeding disorders community: the approval process, regulation of names, and rules regarding substitution.

APPROVAL PROCESS

The Hemophilia Federation of America (HFA) supports an approval process that meets or exceeds the progress already made in developing and producing safe therapies. HFA strongly urges government accountability and oversight in the following areas before any biosimilar for clotting factor is approved:

- ✓ Assessment of Immunogenicity,
- ✓ Robust Clinical Trials, and
- ✓ Post-Marketing Surveillance.

ASSESSMENT OF IMMUNOGENICITY

Patients using biologics may face risk of an inhibitor, an immune response to a biologic that can have critical adverse health impacts and limit the effectiveness of the product. Research must prove that patients will not suffer from adverse effects of immunogenicity from biosimilars products. Treating an individual who has an inhibitor is extremely complex and demanding and is very costly – often millions of dollars per year – and the negative health consequences, including disability, are significantly greater. The FDA must ensure that an approved biosimilar has "no clinically meaningful differences" between it and the reference product and is thus "interchangeable."[2] HFA expects the FDA to release draft guidance on interchangeability standards in 2016.

[1]Biologics also are offered referred to as "reference products" or "innovator drugs."
[2]According to the Biologics Price Competition and Innovation Act of 2009. 5 November 2014. <http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/UCM216146.pdf>

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CLINICAL TRIALS AND POST MARKETING SURVEILLANCE

The U. S. Food and Drug Administration (FDA) must conduct robust human clinical trials before approving any biosimilar. The approval process of biosimilars must have the same standards of safety and efficacy as that of other FDA approved products. There is the potential for adverse reactions whenever an individual uses a new factor product for the first time or is switched to a new treatment. The inclusion of additional post-marketing surveillance is essential to monitor potential risks. The FDA should require high standards to determine whether a biosimilar is "interchangeable" and can be expected to produce the same clinical results as the reference product in any given patient.

NAMING AND LABELING

It is extremely important that biosimilars have unique nonproprietary names that are distinct from their reference products. A biosimilar is not a generic version of a biologic. Generic drugs are exact chemical copies of the branded products and can be interchanged without causing harm to patients. Biologics are so complex and sensitive to their environment that identical copies are impossible to produce.[3] The use of distinct names will eliminate confusion among prescribers and patients and allow prescribers to track product usage and quickly report adverse events. In August of 2015, the FDA released draft guidance requiring biosimilars to have unique nonproprietary names. HFA expects the final guidance to be released in 2016.

In conjunction with nonproprietary names, biosimilars should include clear and transparent labeling information that identifies the product as a biosimilar for both physicians and patients. Labeling should include information that the product is a biosimilar, information about interchangeability, indications of approved use, indications of approved use based on extrapolation, as well as adverse event information. HFA expects the FDA to release draft guidance on labeling in 2016.

SUBSTITUTION

The regulatory framework must prohibit automatic substitution by providers and pharmacies of the original biologic with a biosimilar. Because biosimilars cannot be exact copies of biologics, they should not be automatically substituted without the fully informed permission of both patients and their providers. Each patient's immune response and associated health risks are unique, substitution cannot be made safely without input from both groups. If the practice of automatic substitution is adopted through either legislative or regulatory pathways, both physician and patients should be notified of the intent to switch products. Providers must be able to observe how the medicines interact with and affect their patients.

For more information, contact HFA at advocacy@hemophiliafed.org

We are here to support your Advocacy!

[3]"Biosimilars." Amgen Inc., 1 Jan 2014. Web. 3 November 2014. <http://www.amgen.com/pdfs/misc/Biologics_and_Biosimilars_Overview.pdf>