

AARDA Board approves policy statement on biologics

With many questions surrounding the new biologic medicines and with requests concerning AARDA's view of biosimilars, the AARDA Board of Directors has written and approved in recent action a "Statement of Principles on Biologic Medicines." We are pleased to share this statement with our newsletter readers.

American Autoimmune Related Diseases Association Statement of Principles on Biologic Medicines

The past decade has brought about new treatments for many autoimmune diseases for the first time in over 50 years. These new therapies are biologic medicines which have revolutionized the treatment of many serious diseases providing drugs that treat the underlying cause of a disease rather than just the symptoms. As many patients are aware biologic medicines, like all treatments, can come with both benefit and risk; they also can be expensive. Biologics are large molecules made from living cells, so there is increased need to assure quality in the manufacturing process. Because they are usually injected or infused, there is greater need for vigilance to deal with potential adverse effects. And because of their complexity, they often come at a higher financial cost. Not surprisingly, as many of the first-generation biologic medicines come off patent, there is great interest in the reproductions of these originals as an opportunity to reduce costs. These reproductions are referred to internationally as biosimilars. In the United States, the Affordable Care Act (ACA) in 2010 provided the U.S. Food and Drug Administration (FDA) with authority to create a pathway for approving biosimilar biologic products for U.S. use. Under that pathway, FDA is poised to begin issuing U.S. approvals for biosimilars, perhaps as early as this year [2015].

The advent of biosimilars potentially may offer the prospect for lower-cost alternatives to original biologic medicines. Recognizing that biosimilar biologic products are not the same as generic copies of small-molecule chemical drugs, and consistent with the ACA, FDA has been working to establish regulatory guidelines for the development and approval of biosimilars. As companies work on research and development efforts on biosimilar products, and as FDA considers the criteria under which it will evaluate and regulate these products, we believe it is important for policymakers and other stakeholders to ensure that these efforts include guidelines for ongoing monitoring and other standards that will protect patients and facilitate the safe, efficacious, and cost-effective use of all biologics, including original innovator medicines as well as any biosimilars that receive approval from FDA. To those ends, and particularly in light of the current lack of long-term experience with biosimilars in the United States, we believe the following principles are essential to guide the introduction of biosimilars to our healthcare system:

1. Approval standards for a biosimilar product must meet the same standards of rigor and accountability as those for the innovator biologic.

Biologic medicines are made from living cells; and subsequent "copies" are—as the term "biosimilars" makes clear—*similar but not identical* to the innovator product. While a generic chemical drug is made from the same active ingredients and has the same structure as the original drug, for a biologic drug, it is not only the chemical structure of the protein but also the way this structure is folded that determines how it works. The process by which a biologic is manufactured has as much influence on the final product as does the starting ingredients.

Because the manufacturer of the original, innovator biologic does not need to share its manufacturing process, the company making the replication needs to develop its own process. The standards set by the FDA for the approval of a biosimilar must be as

rigorous and accountable as those for the innovator biologic to assure the same level of safety and effectiveness.

2. All users (including patients and their healthcare providers) must have access to information that distinguishes the biosimilars from the innovator biologic for appropriate prescribing.

Even small differences between biologic products can have implications for autoimmune patients. In order for clinicians to prescribe appropriately, it is important that they have access to all product information. Moreover, we believe it is critically important that each biosimilar product must have a unique and distinguishable nonproprietary names and a distinct name under the International Non-proprietary Names (INN) Program of the World Health Organization (WHO). Unique names are essential for accurate prescribing, dispensing, and tracing of adverse events back to the source product.

3. Accurate tracking and tracing of biologics must be assured for purposes of monitoring safety and effectiveness.

Biologics are large, complex molecules, and the immune systems of people differ. Therefore, the same biologic drug may have different immunological effects in individual patients. Likewise, a patient may respond well to one biologic but have serious reactions to another version of that drug. Because small differences in the manufacturing process or the type of stabilizer can lead to significant differences in the final product, it is imperative that the FDA monitor all biologics, including innovators and biosimilars, once available to patients. A complete and accurate tracking system is required to make certain that a concern about efficacy or adverse events can be attributed to the specific product, manufacturer, and product lot. This will enable authorities to identify product-specific problems that develop after approval and to minimize patient risk.

4. Patients and their physicians must have the final choice on what product a patient receives.

Biologics treat serious and life-threatening conditions, and autoimmune patients often have multiple health challenges as well as a heightened immune response. Treatment decisions take into consideration the patient's unique health circumstances, history of treatment responses and sensitivities, economic circumstances, and other relevant factors. Because different versions of the same biologic, including the biosimilars, are not identical, the physician should be the one to decide which product best meets the patient's needs. Similarly, no patient should be switched from one medicine to another biologic or biosimilar without the treating physician's and the patient's advance notification and agreement with the switch.

In summary, all public policies, approval processes, and reimbursement practices involving biologics should be guided by the foregoing essential principles. The availability of biosimilars, once approved by FDA, has the potential to contribute meaningfully to overall public health if patient safety remains the utmost priority. Lower cost should not mean lower quality or additional risk to patients and the public health. Regulatory authorities play an essential role in realizing this potential. We urge these authorities to collaborate with relevant stakeholders to achieve these critical objectives of protecting patient safety, facilitating positive clinical outcomes, and promoting the public health.