Contact:

Christine Hanson

202-226-7040

cell 202-225-0434

Lawmakers call for safe, science-based regulation for follow-on biologics

WASHINGTON - U.S. Reps. Jay Inslee (D-Wash.), Gene Green (D-Texas) and Tammy Baldwin (D-Wis.) today filed legislation in the House that would provide clear guidance on a pathway for production of follow-on biologic, also known as bio-similar or bio-generic medicines.

His bill, the Patient Protection and Innovative Biologic Medicines Act, amends the Public Health Service Act to create an abbreviated process for the approval of potentially less expensive biotech drugs, while assuring patient safety and promoting innovation. These complex drugs, made from living cell lines, typically are injected and used in the treatment of serious conditions like cancer, diabetes and neurological disease.

"We can create a pathway to lower-cost copies of biotech drugs without sacrificing safety or eliminating incentives to create breakthrough medicines," said Inslee, who serves on the House Energy and Commerce Committee and represents a Seattle-area district with a strong biotech sector. "This is not an either-or situation. We can do both."

"An FDA pathway for the approval of bio-similar biologics is the critical next step to providing patients with increased access to lifesaving biologic therapies," added Green. "In creating that regulatory pathway, however, we must ensure that we strike the appropriate balance between bringing biosimilars to the market and ensuring that the market remains open to the development of the next generation biologics that offer so much hope to the millions of Americans suffering from incurable disease."

"As medical science develops new and better treatments for disease, we must ensure that they are safe and affordable. Emerging biologics companies need and deserve encouragement and incentives to succeed. In the pursuit of good health, patients and businesses should not and must not compete for our support," said Baldwin, who holds a seat on the House Energy and Commerce Subcommittee on Health.

The bill comes at a time when Congress is considering legislation to speed the approval of lower-cost versions of biologics by requiring fewer Food and Drug Administration (FDA) requirements. The unique qualities of biologics, however, pose a policy challenge. Biotech medicines can be determined to be the "same" as the original drug, but they never can be identical. And even small differences between a copy and an

original biologic can cause differences in effectiveness, and, in some cases, serious side effects.

A wide range of patient groups support Inslee's legislation as a means of protecting patient safety through clinical trials and preserving an environment that encourages the development of innovative and life-saving therapies. They include Candlelighter Childhood Cancer Foundation, the Interamerican College of Physicians and Surgeons and the Lupus Research Institute National Foundation, among others.

"At the critical moment when targeted therapies are finally bearing the fruit of decades of research and providing new hope for cancer patients and their families, the Patient Protection and Innovative Biologic Medicines Act would speed and expand access to life-saving biologics, while encouraging the development of future treatments," Executive Director of Candelighter Childhood Cancer Foundation Ruth Hoffman said in a statement.

The bill also wins high marks from physician groups as well. "The EU experience shows that a science-based, patient-focused pathway works," added Dr. Rene F. Rodriguez, president of the Interamerican College of Physicians and Surgeons. "The Patient Protection and Innovative Biologic Medicines Act of 2007 follows the example of the EMEA - the European Medicines Evaluation Agency - by testing both the structural and functional features of the follow-on biologic medicine, with an emphasis on identifying difference from the innovator."

Find below background on the issue and a summary of the bill.

Bill Background and Summary

I. Background

In the U.S., there is currently no approval pathway for "follow-on" versions of biologics. Unlike what we have come to know as traditional chemical drugs, biotech medicines are proteins made by splicing genetic material into living cell cultures. The natural variations introduced by reliance on living cells make biological medications impossible to replicate, a fact to which both innovator and follow-on companies have stipulated. Thus, "generic" versions (as we commonly understand them) of biologics are not truly feasible. As their name suggests, biosimilars are similar, rather than identical, to the original drugs they reference. The manufacturing process of a biosimilar version of a drug will inevitably yield a product that is different-often in clinically significant ways-from the original.

Biologics constitute the most promising and effective class of medications in the fight against cancer and other serious diseases. Unfortunately, many of these biotech wonder drugs are incredibly expensive and, as noted above, no generic versions currently exist. As patents on biotech medicines begin to expire, it is essential that we establish an FDA approval pathway for follow-on biologics to give patients more choices and ensure

affordable access to these ground-breaking therapeutic agents. Studies by both the generic manufacturers and innovators acknowledge that by creating a pathway for off-patent biologics, patients will be able to save as much as 30% on their biotech medicines. The Patient Protection and Innovative Biologic Medicines Act establishes an abbreviated and safe approval pathway for biotech drugs that are proven therapeutically "biosimilar" to existing biologics. By prudently balancing the interests of patient safety, affordable access, and innovative drug development, this bill will make important medicines available to patients in a safe and responsible manner while ensuring the continued development of new drugs.

Legislation creating an approval pathway for biosimilars must first safeguard patient safety while expanding and accelerating affordable access. The European Union, which experienced first-hand the unanticipated severe immunologic safety problems with biologics, has developed the first comprehensive system for the regulation of biosimilars. Under EU law, a biosimilar manufacturer must demonstrate that its product is highly similar to the original product through clinical and nonclinical testing to ensure that patients receive a product that is similar to the original. In addition, the law requires risk management planning and post-marketing surveillance to detect potential adverse patient reactions. It also recognizes that biosimilar products are not interchangeable with innovator products, which further influences post-market safety concerns in the naming of products. The Patient Protection and Innovative Biologic Medicines Act would incorporate these same safeguards while creating an FDA approval pathway that gives patients faster access to safety-tested biosimilars.

Finally, to preserve incentives for biotech innovation, this bill would include provisions for data exclusivity. By preserving the data exclusivity provisions that fostered today's cutting-edge biologics, we can help American biotechnology develop more life-saving innovations for ourselves and future generations.

II. Summary

Key features of the legislation include:

Access

- The bill amends the Public Health Service Act to create an abbreviated pathway exclusively for the approval of biological products that are claimed to be similar to currently approved drugs.

Clear Guidance

- Under the bill, the Secretary will issue guidance describing the data that will be required for approval of biosimilars in a particular product-class. This Guidance will involve stakeholder participation (from both sides) and expert advice from a Similar Biological Products Advisory Committee. A guidance

process will allow for the FDA to make a science-based determination about the data and information needed for approval accounting for factors like complexity and immunogenic reactions of the original product. Additionally, it will allow for a more public and transparent process by which the FDA can solicit comment from both sides, while avoiding many of the concerns raised about the "citizen petition" process currently in place for generic approval.

Safety

The bill will ensure safety of biosimilar drugs through:

- Documentation review of the manufacturing process for the similar biological product;

- Full characterization of the similar biological product in comparison with the reference medicine;

- FDA prescribed non-clinical studies and clinical trials at appropriate levels demonstrating that the similar biological product is safe, including with respect to unwanted immune reactions as well as effective for each clinical use proposed; and

- Post-marketing follow up studies on the similar biological product, including further immune reaction testing and additional clinical trials where appropriate.

- Assigning unique proper names to similar biological products to promote rapid identification if adverse reactions occur. This will allow for a quick and clear post-market reaction if necessary that can isolate a manufacturer and facility source- be it the innovator or the follow-on- more readily than the current system, which is vital in products where an immunogenic reaction of some degree is anticipated.

- Prohibiting a similar biological product to be characterized as interchangeable with (i.e., substitutable for) the reference product.

Innovation

- The bill provides fourteen years of data exclusivity for innovator drug manufacturer products, with an additional year available if the Secretary approves a new indication for the

reference product that offers a significant clinical benefit during the twelve years after its initial authorization.

- Innovator drug manufacturer trade secrets and nonpublic safety and effectiveness data would not be allowed to be publicly disclosed. (This mirrors the disclosure protocols for chemically synthesized drugs and the generics process under current law.) However, a summary of the innovator's safety and effectiveness data could be released after approval of the first similar biological product relying on the reference product.