

## HEALTH NEWS: AFRICA AND THE UNITED STATES

### FDA, Advisory Committees & Recombinant Antithrombin.

Year after year, scientists and health professionals work hard to cure patients of diseases or improve their quality of life, or by developing new treatments or developing new uses for treatments already on the market. These treatments usually are chemically produced substances used to treat or prevent disease. But they may also be biologics, treatments such as vaccines or recombinant proteins that are made from living organisms. The United States Food and Drug Administration (FDA) defines a biologic as any therapeutic serum, toxin, antitoxin, vaccine, virus, blood, blood component or derivative, allergenic product, or analogous product, or derivatives applicable to the prevention, treatment, or cure of injuries or disease of humans.

The FDA is the division of the United States Department of Health and Human Services with the legal power to make sure that drugs and biologics are safe and effective before they go on the market. The official FDA site mission statement says: "The FDA is responsible for protecting the public health by assuring the safety, efficacy, and security of human and veterinary drugs, biological products, medical devices, our nation's food supply, cosmetics, and products that emit radiation. The FDA is also responsible for advancing the public health by helping to speed innovations that make medicines and foods more effective, safer, and more affordable; and helping the public gets the accurate, science-based information they need to use medicines and foods to improve their health."

FDA regulators have 2 main challenges and objectives during the drug or biologic approval process:

- 1) First, they must make sure the treatment is safe and effective.
- 2) Second, to make promising treatments available as quickly as possible to the patients in need. This complex job involves many areas of expertise, and often FDA turns to outside experts for counsel.

The FDA's Advisory Committees give independent, expert advice to the agency on a range of complex scientific, technical, and policy issues. This includes questions related to the development and evaluation of products regulated by FDA. They are a valuable resource and make an important contribution to the agency's decision-making processes. An Advisory Committee includes a chairperson, several members, a consumer representative, an industry representative, and often a patient representative.

Committee membership typically includes ethnic, gender, and geographic diversity. Members have recognized expertise and judgment in a specific field.

While the FDA will make the final decision, advisory committee meetings assure that we're getting the best available expert advice on important decisions that affect the health and safety of the public.

On 09 January 2009, the FDA's Blood Products Advisory Committee voted that recombinant antithrombin is safe and efficacious for the prevention and treatment of venous thromboembolism in hereditary antithrombin deficient (HD) patients undergoing surgery or childbirth procedures. If approved, this recombinant antithrombin will be the first recombinant human antithrombin available in the United States. It is the first recombinant antithrombin product approved in the world and the first antithrombin product that has been approved through the centralized European Medicines Agency (EMA) procedure in the European Union.

GTC Biotherapeutics develops, produces, and commercializes therapeutic proteins through transgenic animal technology. In mid 2008, GTC entered into a collaboration agreement with Ovation Pharmaceuticals to develop and market recombinant antithrombin in the United States for patients with hereditary antithrombin deficiency. Recombinant antithrombin is a recombinant form of human antithrombin, the first transgenically produced protein to be approved anywhere in the world, having recently been approved by the European Commission for the prophylactic treatment of deep vein thrombosis in patients with hereditary antithrombin deficiencies that are undergoing surgical procedures.

Recombinant antithrombin is produced in the milk of goats developed using micro-injection technology to incorporate a human antithrombin transgene. In addition to recombinant antithrombin GTC is developing additional recombinant forms of therapeutic proteins normally found in human blood plasma as well as monoclonal antibodies. These products have potential applications in hematology, oncology, and autoimmune diseases.

#### **By Ana Maria Rodriguez-Rojas MS**

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