Using Transgenic Chickens To Treat Rare Human Diseases

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Lysosomal acid lipase deficiency (LAL Deficiency) is a rare disease affecting an enzyme, lysosomal acid lipase, which is responsible for breaking down fat, including cholesterol, within the body. LAL Deficiency can be devastating considering it is life-threatening, associated with early mortality and significant morbidity, as fatty materials build up within the body. Diagnosing LAL Deficiency is a relatively simple procedure requiring only a blood test, yet treatment was lacking – until Kanuma®.

Robert Ivarie, head of the genetics department at the University of Georgia (UGA), began researching how to create artificial proteins – biomedical proteins – to assist in treating disorders that involve mutated proteins, like LAL Deficiency. At UGA, Ivarie patented technologies involving genetically engineered, "transgenic", chickens to serve as "bioreactors" for proteins. These modified chickens provide a controlled environment to support and maintain biological functions, including protein production.

Ivarie created an entire portfolio dedicated to this technology and, with the assistance of the UGA's Innovation Gateway tech-transfer office, patented the technology and founded Avigencis, which later became Synageva Biomedical. Synageva served to advance and advertise the protein manufacturing technology, covered by more than 40 patents worldwide.

Synageva manufactured therapeutic protein by extracting it from the white of eggs produced by the transgenic chickens. In 2008, it began researching treatment of LAL Deficiency. Using the protein-manufacturing technology, it

created Kanuma, a recombinant form of the natural human LAL enzyme. Through enzyme replacement therapy, Kanuma is the first FDA-approved drug to be produced in transgenic chickens and the first viable treatment for LAL Deficiency.

Considering the medical need and lack of treatment for LAL Deficiency patients, Kanuma received Fast Track and Breakthrough Therapy designations by the U.S. Food and Drug Administration (FDA) for LAL Deficiency presenting in infants. The FDA designated Kanuma as an orphan drug, a classification reserved for drugs that serve a small population, followed by the European Medicines Agency (EMA) and the Japanese Ministry of Health, Labour and Welfare.

In 2014, Synageva completed Phase 3 of their clinical trials for Kanuma; that same year, they filed for drug approval in the U.S., Europe and Japan. Kanuma, a company now part of AstraZeneca after it acquired Synageva, is approved in the U.S., Europe and Japan for long-term enzyme replacement therapy in patients of all ages with LAL Deficiency, being the only approved treatment for the disease.

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