



Dedicated to Rare Diseases

FOR IMMEDIATE RELEASE

SYNAGEVA BIOPHARMA FILES FOR ORPHAN DRUG DESIGNATION FOR SBC-102, BEING DEVELOPED TO BE THE FIRST ENZYME REPLACEMENT THERAPY TO TREAT LYSOSOMAL ACID LIPASE DEFICIENCY – A PROGRESSIVE, OFTEN FATAL DISEASE

WALTHAM, Mass, May 4, 2010 -- [Synageva BioPharma Corp.](#), a privately held biopharmaceutical company, today announced its filing with the U.S. Food and Drug Administration (FDA) requesting orphan drug designation for its program, SBC-102. This program is an enzyme replacement therapy in preclinical development to treat lysosomal acid lipase (LAL) deficiency, also known as Wolman disease or cholesteryl ester storage disease (CESD), a condition for which there is currently no effective treatment.

"Synageva BioPharma is built on the guiding philosophy that every patient deserves a treatment for their disease, no matter how rare their condition," said Sanj K. Patel, president & CEO of Synageva BioPharma. "SBC-102 for LAL deficiency is the lead program among an exciting pipeline of novel therapeutics we are developing for a variety of rare diseases. These include lysosomal storage diseases and other progressive, serious and life-threatening genetic conditions."

The profound clinical effects of LAL deficiency include cirrhosis, liver failure, severe malabsorption, and aggressive, early onset atherosclerosis. These effects are due to a failure to breakdown cholesteryl esters and triglycerides, which leads to massive accumulation of lipid in a number of tissues and a marked disturbance of lipid homeostasis. Many of these patients die from complications of the disease, with the most severely affected patients dying in the first year of life.

"The strength of the data we have generated and the information we will obtain from our natural history study brings us closer to evaluating SBC-102 in clinical trials to help patients with this devastating disease," said Anthony Quinn, MD, PhD, chief medical officer and head of research & development at Synageva BioPharma.

"We are always pleased when companies focus on bringing new treatments to the rare disease community," said Peter L. Saltonstall, president and CEO of NORD (National Organization for Rare Disorders). "LAL deficiency is a devastating rare disorder with no treatment at this time. NORD would be very happy for this patient community to have a safe, effective treatment."

About Synageva BioPharma Corp.

Synageva BioPharma Corp. is a biopharmaceutical company dedicated to discovering, developing and commercializing therapies for patients with rare conditions and high unmet medical need. The company has developed a pipeline of novel therapeutics for underserved populations and has assembled a team with a proven track record of bringing orphan therapies to patients. Due to its flexibility, scalability, and consistency of end product, the company's proprietary protein expression technology, SEP™, is uniquely

suited to develop and commercialize personalized medicines for patients with rare diseases. Further information regarding Synageva BioPharma Corp. is available at www.synageva.com.

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