



## **SYNAGEVA BIOPHARMA ANNOUNCES INTERIM DATA AT 8<sup>th</sup> ANNUAL LYSOSOMAL DISEASE NETWORK (LDN) WORLD SYMPOSIUM**

**- SBC-102 was well tolerated and demonstrated predicted pharmacodynamic effects in a Phase I/II trial in late onset LAL Deficiency -**

LEXINGTON, Mass., February 9, 2012 -- [Synageva BioPharma Corp.](#) ("Synageva") (NASDAQ:GEVA), a clinical stage biopharmaceutical company developing therapeutic products for rare disorders, today announced encouraging safety and tolerability data as well as effects consistent with preclinical findings and known mechanism of action. Patients from this 4-week Phase I/II trial of SBC-102 in adults with late onset LAL Deficiency continue to transition into an open-label extension study.

"Patients with LAL Deficiency have significant unmet medical need. The data from this trial, the first human study of an enzyme replacement therapy for subjects with late onset LAL Deficiency, are important, and combined with the initial safety profile of SBC-102, warrants further investigation in longer-term trials," said Dr. Gregory Enns, MBChB, Assistant Professor of Pediatrics and the Director of the Biochemical Genetics Program at Stanford University and principal investigator in the trial.

### **About the Phase I/II trial of SBC-102 in late onset LAL Deficiency**

The trial fully enrolled 9 patients from multiple sites in the United States and Europe. The primary objective of the study was to evaluate the safety and tolerability of SBC-102 administered weekly in adult patients with liver dysfunction due to late onset LAL Deficiency. Additional assessments included evaluating pharmacokinetics and biomarkers of SBC-102 activity including liver transaminases and serum lipids. Patients enrolled in the trial were diagnosed with LAL Deficiency and demonstrated evidence of liver involvement as assessed by the presence of hepatomegaly and/or elevated transaminases. Patients received four once-weekly infusions of SBC-102 (0.35 mg/kg, 1.0 mg/kg, or 3.0 mg/kg).

SBC-102 was well-tolerated with no serious adverse events or infusion-related reactions, and all subjects completed their scheduled infusions. The most common adverse events included headache, nausea and diarrhea. The majority of adverse events were mild and unrelated to SBC-102.

SBC-102 resulted in rapid and significant decreases in serum transaminases, with evidence of mobilization of lipids out of the liver and other tissues and into the blood, consistent with its mechanism of action.

“We are pleased with the results of SBC-102 from this trial. We believe these insights combined with additional data collected throughout the year will support, following discussions with the regulatory authorities, initiation of a randomized, double-blind, placebo-controlled trial,” said Dr. Anthony Quinn, MBChB, PhD, FRCP, Senior Vice President and Chief Medical Officer of Synageva BioPharma.

### **About Synageva’s Lead Program**

SBC-102 is being developed as an enzyme replacement for Lysosomal Acid Lipase (LAL) Deficiency, a lysosomal storage disorder (LSD), and is a recombinant form of the human LAL enzyme. SBC-102 is currently being evaluated in global clinical trials and has been granted orphan designations by the U.S. Food and Drug Administration (“FDA”) and the European Medicines Agency. Additionally, SBC-102 received fast track designation by the FDA.

### **About LAL Deficiency**

Lysosomal Acid Lipase Deficiency is a rare, autosomal recessive lysosomal storage disorder (LSD) that is caused by a marked decrease in lysosomal acid lipase activity. Late onset LAL Deficiency, sometimes called Cholesteryl Ester Storage Disease (CESD), affects both children and adults. In these patients, the buildup of fatty material in the liver, spleen and blood vessel walls leads to complications resulting in significant morbidity and mortality. Early onset LAL Deficiency, sometimes called Wolman Disease, affects infants in the first year of life and is characterized by growth failure, malabsorption, steatorrhea and hepatomegaly and is rapidly fatal, usually within the first year of life.

### **About the Lysosomal Disease Network WORLD Symposium**

The Lysosomal Disease Network (LDN) WORLD Symposium is an ACCME-accredited annual symposium which includes lectures and poster presentations on basic, translational and clinical research for lysosomal storage disorders. The goal of the meeting is to provide an interdisciplinary forum to explore and discuss specific areas of interest, research and clinical applicability related to lysosomal diseases.

In addition to the interim Phase I/II data for SBC-102, preclinical data for SBC-103 for Sanfilippo B syndrome were presented earlier at this meeting, as were data describing a method for the diagnosis of LAL Deficiency using dried blood spots.

### **About Synageva BioPharma Corp.**

Synageva is a clinical stage biopharmaceutical company focused on the discovery, development, and commercialization of therapeutic products for patients with life-threatening rare diseases and unmet medical need. Synageva has several protein therapeutics in its pipeline. The company has assembled a team with a proven record of bringing orphan therapies to patients.

Further information regarding Synageva BioPharma Corp. is available at [www.synageva.com](http://www.synageva.com).

### **Forward-Looking Statements**

This news release and oral statements made from time to time by Synageva representatives in respect of the same subject matter may contain “forward-looking statements” under the provisions of the Private Securities Litigation Reform Act of 1995. Such statements can be identified by introductory words such as “expects,” “plans,” “intends,” “believes,” “will,” “estimates,” “forecasts,” “projects,” or words of similar meaning, and by the fact that they do not relate strictly to historical or current facts. Many factors may cause actual results to differ materially from forward-looking statements, including

inaccurate assumptions and a broad variety of risks and uncertainties, some of which are known, including those identified under the heading “Risk Factors” in the Company’s Registration Statement on Form S-3 filed with the Securities and Exchange Commission (the “SEC”) on December 21, 2011 and other filings the Company periodically makes with the SEC, and others of which are not. Synageva cannot be sure when or if it will be permitted by regulatory agencies to undertake additional clinical trials or to commence any particular phase of clinical trials or how quickly patient enrollment in clinical trials will occur. In addition, early clinical results are not necessary predictive of results that may be achieved from subsequent clinical trials. Because of this, statements regarding the expected timing of clinical trials or ultimate regulatory approval cannot be regarded as actual predictions of when Synageva will obtain regulatory approval for any phase of clinical trials or when it will obtain ultimate regulatory approval by a particular regulatory agency. No forward-looking statement is a guarantee of future results or events, and one should avoid placing undue reliance on such statements. Synageva undertakes no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

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