



Corporate Presentation



Forward-Looking Statements

These materials and oral statements made from time to time by Synageva representatives in respect of the same subject matter may contain “forward-looking statements.” These 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. Forward-looking statements frequently are used in discussing potential product applications, potential collaborations, product development activities, clinical studies, regulatory submissions and approvals, and similar operating matters. Many factors may cause actual results to differ from forward-looking statements, including inaccurate assumptions and a broad variety of risks and uncertainties, some of which are known and others of which are not. 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. 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 patent enrollment in clinical trials will occur. 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.

Synageva Investment Highlights

Team

Rare Disease Experience

Rare Disease

Unmet Medical Need

Lead Product

ERT for LSD – Model is Predictive

Commercial

Pull-through Expertise

Manufacturing

Robust, Efficient and Scalable

Proven Management Team

Sanj K. Patel

Chief Executive Officer

genzyme



Carsten Boess

Chief Financial Officer

ALEXION



Eric Grinstead

Commercial Operations

ALEXION

genzyme

Anthony Quinn

Chief Medical Officer



AstraZeneca

Mark Goldberg

Product Development

genzyme



Donna Mackey

Clinical Operations

genzyme



Joe DeCoursey

Manufacturing



IMMUNOGEN

Synageva
BioPharma

Dedicated to Rare Diseases®

Lead Program

SBC-102 (rhLAL)

ERT for Lysosomal Acid Lipase Deficiency

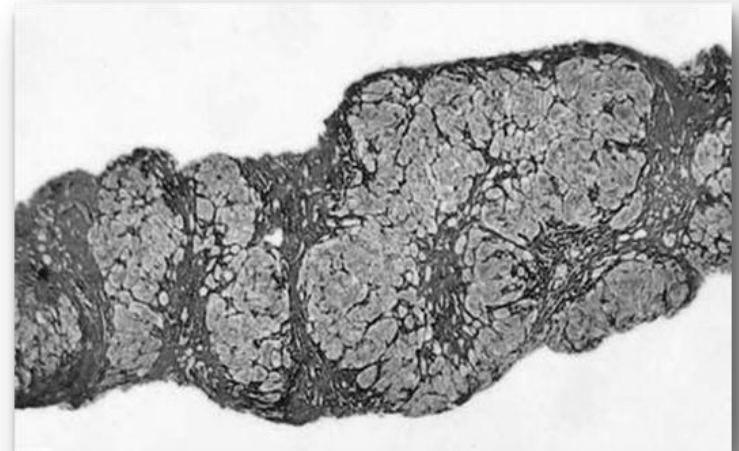


SBC-102 (rhLAL)

- SBC-102 is an enzyme replacement therapy being developed for Lysosomal Acid Lipase (LAL) Deficiency
 - LAL Deficiency is a rare lysosomal storage disorder characterized by gastrointestinal, liver and cardiovascular complications due to massive accumulation of lipid material in a number of organs
 - LAL Deficiency presents as a clinical continuum with two major phenotypes, early onset/Wolman Disease and late onset/Cholesteryl Ester Storage Disease (CESD)
 - SBC-102 replaces the missing LAL enzyme, allowing clearance of the accumulated lipids
- SBC-102 has received Orphan designation in both the US and EU, and Fast Track designation from the FDA
- Synageva is currently dosing patients as part of the global clinical trials for SBC-102

Late Onset LAL Deficiency (CESD)

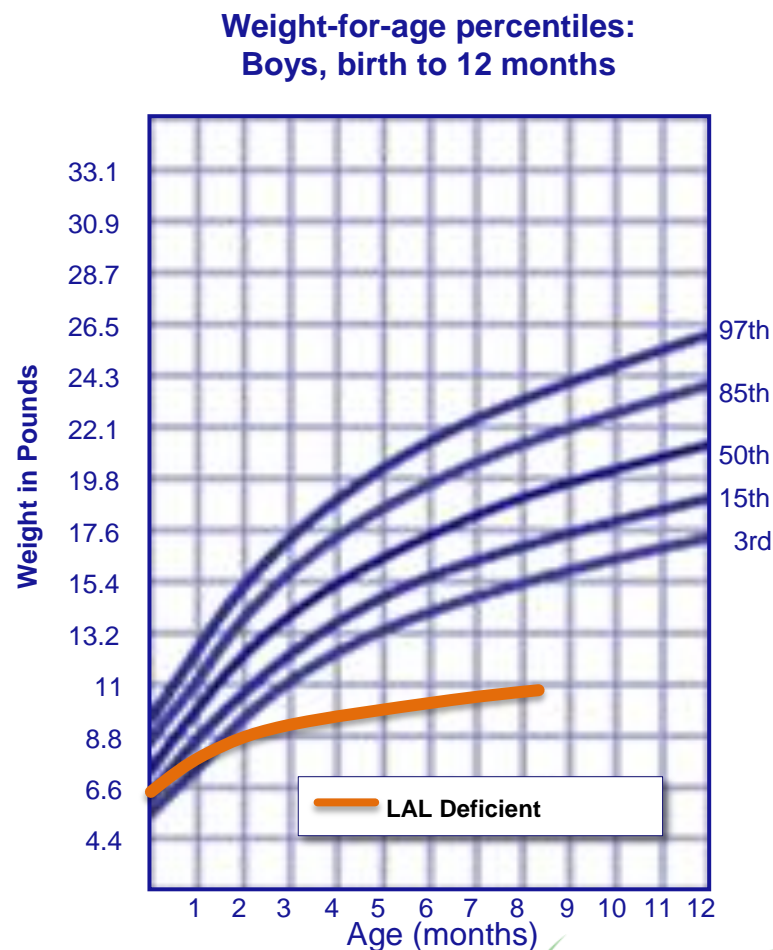
- Prominent hepatic manifestations
 - Fatty liver
 - Elevated transaminases
 - Fibrosis, Cirrhosis, Liver transplant, Liver failure
- Other manifestations: splenomegaly, type II hyperlipidemia
- Shortened lifespan and increased morbidity



Liver biopsy showing cirrhosis in CESD

Early Onset LAL Deficiency (Wolman)

- Rapidly progressive and fatal
- Prominent hepatic and GI manifestations
 - Hepatomegaly and liver failure
 - Splenomegaly
 - Persistent vomiting
 - Abdominal distension
 - Profound growth failure
- Adrenal calcification



Source: Assmann, G, Seedorf U. Acid lipase deficiency: Wolman disease and cholesteryl ester storage disease. In: Scriver CR, Beaudet AL, Sly WS, Valle, D, eds. The Metabolic and Molecular Bases of Inherited Disease. New York: McGraw-Hill, 2011: 3551-71.

Synageva Pipeline

Program	▶	SBC-102 (rhLAL)	SBC-103 (rhNAGLU)	SBC-104	SBC-105	SBC-106
Therapeutic	▶	Recombinant Lysosomal Acid Lipase	Recombinant α -N-acetyl- glucosaminidase	Extra Cellular Protein	Enzyme Replacement Therapy	Enzyme Replacement Therapy
Disease	▶	LAL Deficiency (LSD)	MPS IIIB/ Sanfilippo B (LSD)	Severe Genetic Condition	Severe Metabolic Disorder	Severe Genetic Condition
Development Status	▶	Clinical	Preclinical	Preclinical	Preclinical	Preclinical
Regulatory Opportunity	▶	Orphan Designation • Granted US • Granted EU Fast Track Designation	Potential for Orphan & Fast Track Designation	Potential for Orphan & Fast Track Designation	Potential for Orphan & Fast Track Designation	Potential for Orphan & Fast Track Designation

Synageva Summary

- Management team with extensive rare/ ultra-rare disease expertise
 - Previous leadership roles with almost every ultra-rare protein marketed to date
- Pipeline of protein therapeutics for devastating rare diseases
 - Lead program for LAL Deficiency in clinical trials
 - Advancing additional product development programs for other diseases with high unmet need
- Design, development and production capabilities enabling internal creation and manufacture of products at commercial scale
- Publicly traded company (NASDAQ:GEVA)
- Headquarters in Lexington, Massachusetts



Synageva Opportunity

Team

Proven Record with Rare Diseases

Lead Program

Significant Opportunity - ERT for LAL Deficiency

Technology

Robust Platform, Product Pipeline

Multiple Value Driving Events

Every Second Counts!



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