Synageva BioPharma

Dedicated to Rare Diseases®

2012 ANNUAL REPORT

Dear Fellow Shareholders,

2012 was a transformational year for Synageva. After becoming a public company over a year ago, we have made significant progress towards our goal of becoming a leading global organization focused on discovering, developing and commercializing therapeutics for patients suffering from rare diseases. 2013 promises to be another landmark year as we execute towards that goal.

Before highlighting what makes me the most excited about the future for Synageva, I would like to reflect upon the team's accomplishments during 2012.

First, we advanced our lead program, sebelipase alfa, an enzyme replacement for patients suffering from a rare and devastating condition called lysosomal acid lipase deficiency (LAL Deficiency). We recently commenced enrollment and started dosing patients in the ARISE (Acid Lipase Replacement Investigating Safety and Efficacy) clinical trial, a global Phase 3 double-blind, placebo-controlled study in children and adults with LAL Deficiency. We also continue to treat adult patients in a long-term, open-label Phase 1/2 extension study. Data from this extension study, along with discussions held with the U.S., European and other global regulatory authorities, helped to inform the design of the global ARISE Phase 3 clinical trial.

Most recently at the Lysosomal Disease Network (LDN) WORLD meeting in February 2013, investigators presented nine months of data from the Phase 1/2 extension study. These data continue to demonstrate sebelipase alfa's ability to correct a broad range of abnormalities associated with LAL Deficiency. These include evidence of reduction in liver damage as measured by sustained reductions of transaminases in conjunction with improvements in patients' abnormal lipid profiles. These improvements are typically accompanied by reduced liver volume towards the normal range and improvements in abnormal fat accumulations in the liver. Sebelipase alfa was generally well-tolerated at nine months with mostly mild adverse events that were considered unrelated to the drug.

We continue to make solid progress in the ongoing Phase 2/3 trial in early onset LAL Deficiency. As a result of our efforts to raise awareness in this most severe and devastating form of LAL Deficiency, we continue to enroll infants in this trial. The first of these infants received sebelipase alfa over two years ago. Natural history data from infants with early onset LAL Deficiency (sometimes referred to as Wolman disease) indicate that they typically die before six months of age. I am thrilled to report that this infant–now a toddler!–continues on treatment, exhibits excellent development progress, and celebrated his second birthday in late 2012. His remarkable progress not only reinforces our conviction but also strengthens our determination and drive to make sure we find and treat as many other infants as quickly as possible. It is quite frankly, one of the biggest reasons why it is a privilege to work at Synageva.

Disease awareness for LAL Deficiency is increasing around the world. Finding patients is the greatest challenge when working in ultra-rare diseases. Identifying more patients with LAL Deficiency remains a key priority for the company and we have taken several important steps to ensure that our outreach has a global impact. During 2012, we hired regional country managers in the U.S., Japan, Turkey, Germany, U.K., France and Latin America with specific rare disease expertise. These are rare disease experts initially focused on finding more LAL Deficiency patients but longer term will support the broader portfolio of Synageva's programs. We will continue to hire additional staff in other regions with this specific expertise to help raise awareness around the world and identify more patients that are in need of therapy.

Synageva continues to advance its pipeline programs, all of which are focused on improving the lives of patients with rare and devastating conditions. We are pleased with the progress we have made in the development of our next program, SBC-103. This is an enzyme replacement for Mucopolysaccharidosis Type IIIB (MPS IIIB, also known as Sanfilippo B), a devastating condition with marked central nervous system involvement including cognitive decline, behavioral problems, speech loss and loss of mobility. We recently presented pre-clinical data on SBC-103 at the LDN World meeting in February 2013. Using various dosing approaches, these data demonstrated reduced lysosomal substrate storage in the brains, liver, and kidney tissues of a MPS IIIB animal model. Our efforts on this program are now focused on completing the necessary preclinical toxicology and formulation testing to allow us to enter the clinic with this program during the first half of 2014. In addition to SBC-103, our other preclinical programs for patients with rare and devastating conditions continue to progress.

This year we plan to continue to execute on a range of activities to support Synageva's development with the following key areas of focus:

- Progress enrollment in the ARISE Phase 3 trial with sebelipase alfa in children and adults with late onset LAL Deficiency
- Progress enrollment in the Phase 2/3 trial with sebelipase alfa in infants with early onset LAL Deficiency
- Report data from the ongoing Phase 1/2 extension study with sebelipase alfa in adults with LAL Deficiency including one full year of treatment data
- Continue global disease awareness programs to support identification of more infants, children and adults with LAL Deficiency
- Initiate first-in-human clinical trials with SBC-103 for MPS IIIB in the first half of 2014
- Advance additional pipeline programs
- Progress towards completion of a redundant, commercial-scale manufacturing facility to further supply protein therapeutics for our lead programs

The Synageva team is highly focused on achieving these goals. Our team is made up of brilliant, energetic, passionate, and dedicated individuals who are determined to continue to make a meaningful impact on the lives of patients suffering from rare diseases. We have a culture that encourages speed, excellence, good science and good medicine (which will help ensure good business). We believe in a mantra of thoughtful, innovative and strategic planning together with rigorous and responsible execution. We have a drive that is rarely matched in the industry.

While the team is determined to successfully execute on our 2013 goals, we continue to operate with a much longer-term time horizon in mind. We are building a global company with the intent of making a multi-generational impact on the lives of patients and their families, and we will continue to take deliberate steps to help us achieve this goal.

Thank you for joining us on this journey.

Sincerely,

Sanj K. Patel
President and Chief Executive Officer
Synageva BioPharma Corp.
April 2013

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-K

(Mark One)

☑ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended December 31, 2012

or

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

SYNAGEVA BIOPHARMA CORP.

(Exact Name of Registrant as Specified in Charter)

Delaware	**0-23155**	**56-1808663**
(State or Other Jurisdiction of Incorporation)	**(Commission File Number)**	**(I.R.S. Employer Identification No.)**

128 Spring Street, Suite 520,
Lexington, Massachusetts 02421
(Address of Principal Executive Offices) (Zip Code)

Registrant's telephone number, including area code: (781) 357-9900

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Name of each exchange on which registered**
Common Stock, $0.001 par value	**Nasdaq Global Select Market**

Securities registered pursuant to Section 12(g) of the Act:
None.

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes ☑ No ☐

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes ☐ No ☑

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☑ No ☐

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes ☑ No ☐

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§ 229.405 of this chapter) is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. ☑

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definitions of "large accelerated filer," "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act. (Check one):

Large accelerated filer ☐ Accelerated filer ☑ Non-accelerated filer ☐ (Do not check if a smaller reporting company) Smaller reporting company ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☑

The aggregate market value of the voting stock held by non-affiliates of the registrant (based on the last sale price of such stock as reported by The NASDAQ Stock Market, LLC, on its NASDAQ Global Select Market on June 29, 2012) was $450.8 million. The number of shares of the registrant's common stock outstanding as of March 1, 2013 was 27,227,788.

Documents Incorporated by Reference

Portions of the registrant's definitive proxy statement to be used in connection with its 2012 annual meeting of stockholders are incorporated by reference into Part III.

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Explanatory Note

On November 2, 2011, Trimeris, Inc., a Delaware corporation ("Trimeris"), closed a merger transaction (the "Reverse Merger") with Synageva BioPharma Corp., a privately held Delaware corporation ("Private Synageva"), pursuant to an Agreement and Plan of Merger and Reorganization, dated as of June 13, 2011 (the "Merger Agreement"), by and among Trimeris, Private Synageva and Tesla Merger Sub, Inc., a wholly owned subsidiary of Trimeris ("Merger Sub"). Pursuant to the Merger Agreement, Private Synageva became a wholly owned subsidiary of Trimeris through a merger of Merger Sub with and into Private Synageva, and the former stockholders of Private Synageva received shares of Trimeris that constituted a majority of the outstanding shares of Trimeris. In connection with the Reverse Merger, Trimeris changed its name to Synageva BioPharma Corp.

The Reverse Merger was accounted for as a reverse acquisition under which Private Synageva was considered the acquirer of Trimeris. As such, the financial statements of Private Synageva are treated as the historical financial statements of the combined company, with the results of Trimeris included from November 2, 2011.

All references in this Annual Report on Form 10-K to "we," "us" and "our" refer to Synageva BioPharma Corp. (f/k/a Trimeris, Inc.) and its consolidated subsidiaries for periods after the closing of the Reverse Merger, and to Private Synageva and its consolidated subsidiaries for periods prior to the closing of the Reverse Merger unless the context requires otherwise. References to "Pre-Merger Trimeris" mean Trimeris prior to the closing of the Reverse Merger.

Certain portions of this Annual Report on Form 10-K may contain information that relates to Pre-Merger Trimeris' operations and may no longer be material to our business. Any comparison of Pre-Merger Trimeris' revenues and operations with ours may not be helpful to an understanding of our results for the fiscal year ended December 31, 2011, 2012, or future periods.

FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, or the "Securities Act," and Section 21E of the Securities and Exchange Act of 1934, as amended, or the "Exchange Act." These statements include, but are not limited to, statements regarding our development programs, our capabilities, our goals, the expected timeline for achievement of our clinical milestones, the expected properties and benefits of our product candidates, the results of clinical and other studies, the size of the market for our products and our financial results. Any statements about our expectations, beliefs, plans, objectives, assumptions or future events or performance are not historical facts and may be forward-looking. These statements often, but not always, include the use of words or phrases such as "anticipate," "estimate," "plan," "project," "continuing," "ongoing," "expect," "management believes," "we believe," "we intend" and similar words or phrases. Accordingly, these statements involve estimates, assumptions and uncertainties that could cause actual results to differ materially from those expressed or implied in them. Any forward-looking statements are qualified in their entirety by reference to the factors discussed in this report or incorporated by reference.

Because the risk factors discussed in this Annual Report on Form 10-K, and other risk factors of which we are not aware, could cause actual results or outcomes to differ materially from those expressed in any forward-looking statements made by or on behalf of us, you should not place undue reliance on any such forward-looking statements. These statements are subject to risks and uncertainties, known and unknown, which could cause actual results and developments to differ materially from those expressed or implied in such statements. We have included important factors in the cautionary statements included in this report, particularly under Item 1A Risk Factors that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. These and other risks may also be detailed and modified or updated in our reports and other documents filed with the Securities and Exchange Commission ("SEC") from time to time under the Securities Act and/or the Exchange Act. You are encouraged to read these filings as they are made.

Further, any forward-looking statement speaks only as of the date on which it is made, and we undertake no obligation to update any forward-looking statement or statements to reflect events or circumstances after the date on which such statement is made or to reflect the occurrence of unanticipated events. New factors emerge from time to time, and it is not possible for us to predict which factors will arise. In addition, we cannot assess the impact of each factor on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements.

PART I

ITEM 1. OUR BUSINESS

Overview

We are 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 needs. We have several protein therapeutics in our pipeline, including two enzyme replacement therapies for lysosomal storage disorders (LSDs) and additional programs for other life-threatening genetic conditions for which there are currently no approved treatments.

Our business focus on products for rare diseases was established in 2008 with the appointment of Sanj K. Patel as President and Chief Executive Officer and his redirection of the company. This change represented a substantial shift in the business strategy of the company, formerly known as AviGenics, Inc., which was focused on the development of a novel protein production technology for biosimilars. Today, our protein therapeutics are generally produced using this proprietary expression system, which is based on over 15 years of research and clinical development. Our expression system relies upon proprietary vectors that express proteins into egg white. We believe our expression system allows consistent, scalable, sustainable and capital efficient production of protein therapeutics with the further potential to leverage this technology to develop improved biologic therapies for unmet medical needs.

Our Strategy

We have retained the worldwide rights to our pipeline programs, including our lead program, sebelipase alfa (formerly referred to as SBC-102) for the treatment of an ultra-rare LSD known as lysosomal acid lipase deficiency (LAL Deficiency). We are focused on maximizing long-term stockholder value and intend to build our business to independently develop and globally market therapies for patients with life-threatening rare diseases.

Key elements of our strategy include:

- *Advancement of sebelipase alfa toward regulatory approval and commercialization for the treatment of LAL Deficiency*. We plan to seek marketing approvals for sebelipase alfa globally. No treatments are currently approved for this severe and life-threatening LSD, and a near-term objective is to advance sebelipase alfa toward regulatory approval and global commercialization. We have established a global medical affairs effort to assist in identifying and enrolling patients and to build upon the existing connections we have within the physician community. This group will take on medical education support upon sebelipase alfa marketing approval, as well as education of the caregiver community during its development. We also established an initial commercial team that, in addition to supporting the efforts of medical affairs, is focused on laying the groundwork necessary for the launch of an ultra-orphan product.
- *Advancement of our pipeline programs, all of which are focused on rare and life-threatening conditions with unmet medical need*. We believe that it is important to maintain a diverse pipeline of product candidates to sustain future growth. Our most advanced pipeline program, other than sebelipase alfa, is an enzyme replacement therapy for mucopolysaccharidosis type IIIB (MPS IIIB), or Sanfilippo B, which is a life-threatening rare disease in children. A key near-term objective is to advance SBC-103 through pre-clinical testing and into the clinic. We plan to advance our other pipeline programs and may have the ability to efficiently add new research programs targeting other rare life-threatening conditions. In addition, we assess, on an ongoing basis, the availability and desirability of third party businesses and assets to determine whether they represent complementary acquisition or licensing opportunities for us.

We have a team with a proven track record of successfully developing and delivering products that provide meaningful medical value. In order to achieve these strategic objectives, we have, and will remain, focused on hiring and retaining a highly skilled management team that has extensive experience and specific skill sets relating to the selection, development and commercialization of therapies for life-threatening rare diseases. We intend to continue our efforts to build and expand this team as we aggressively grow our business.

Disease Overview

Late onset LAL Deficiency

Our lead program, sebelipase alfa is in late stage clinical development for LAL Deficiency. LAL Deficiency is an autosomal recessive LSD caused by a marked decrease in the activity of the native LAL enzyme, which plays a key role in the degradation of cholesteryl esters and triglycerides. The significant reduction of LAL activity in patients leads to the accumulation of these lipids in various tissues and cell types, and this accumulation of lipids can lead to the clinical manifestations associated with the disease. LAL Deficiency span all ages with a wide spectrum of severity thought to relate to a patient's residual levels of LAL activity. The more common form of LAL Deficiency with an estimated prevalence of 1:40,000 to 1:300,000, is frequently referred to as late onset LAL Deficiency, or Cholesteryl Ester Storage Disease (CESD), and manifests post infancy. This phenotype is associated with predominant liver involvement and type II hyperlipidemia. The liver is most severely affected with marked hepatomegaly and elevation of transaminases with the potential progression to cirrhosis and liver failure. The cardiovascular involvement is characterized by dyslipidemia (high cholesterol, high triglyceride and low high density lipoprotein cholesterol, or low HDL) and accelerated atherosclerosis. An accumulation of fatty deposits on the artery walls (atherosclerosis) is sometimes described early in life. We have initiated natural history studies in approximately 20 countries for LAL Deficiency. These studies will be used to investigate and characterize key aspects of the clinical course of the disease to inform the evaluation and care of affected patients. Although the natural history of late onset LAL Deficiency is not well described, there is evidence that life expectancy is reduced with premature death due to liver complications and early onset of cardiovascular disease.

Late onset LAL Deficiency presents with predominant liver involvement and type II hyperlipidemia (high cholesterol and triglycerides), and is the more common form of LAL Deficiency . The liver is the most severely affected organs with marked organ enlargement, elevation of transaminases and severe liver fibrosis progressing to cirrhosis. Cardiovascular involvement is characterized by dyslipidemia (high cholesterol, high triglycerides and low HDL) with early onset vascular disease due to accumulation of lipid deposits in arterial walls. The presentation of late onset LAL Deficiency is highly variable with some patients going undiagnosed until complications manifest in adulthood, while others can present with liver dysfunction in early childhood. Late onset LAL Deficiency is associated with significant ill health, and while the natural history is not well described, there is evidence that life expectancy is reduced with premature death due to liver, cardiovascular and vascular complications, including strokes. This evidence includes case reports described by Beaudet, A, et al., in *The Journal of Pediatrics* in 1977, Cagel, et al., in *American Journal of Medical Genetics* in 1986, Elleder, et al., in *Journal of Hepatology* in 2000, and Riva, et al., in *Digestive and Liver Disease* in 2008.

Although no approved therapies are available for treatment of LAL Deficiency, palliative care is sometimes used to try to mitigate some of the effects of the disease. These treatments are mainly focused on control of plasma lipid levels through diets that exclude foods rich in cholesterol and triglycerides and suppression of cholesterol synthesis and apolipoprotein B production through administration of statins and other lipid lowering therapies. As described in the medical literature, including in the case study by Di Bisceglie, A., et al., published in *Hepatology*, Volume 11, Issue 5, 1990, although some improvement may be seen in serum lipid levels, the underlying disease manifestations persist and disease progression still occurs. As the disease progresses, this can lead to the need for liver transplantation or may result in death.

In October 2012, an academic group presented its analysis of 135 cases of late onset LAL Deficiency found in the published scientific literature for which clinical information, liver pathology, and serum lipid levels were reported. The analysis revealed CESD-associated liver disease in all patients, including hepatomegaly in 99% of patients and elevated transaminases in all 52 patients with alanine aminotransferase (ALT) and aspartate transaminase (AST) results. The academics' review of all 112 biopsy samples demonstrated characteristic histopathology including massive lysosomal lipid accumulation (microvesicular steatosis), cholesteryl ester crystals, fibrosis, and/or micronodular cirrhosis. Liver failure resulted in esophageal varices in 12 patients, nine liver transplants, and 8 of 11 reported deaths.

An additional poster from the same group of cases provided further details of the liver biopsy results. In CESD patients who had liver biopsies, fibrosis and/or cirrhosis due to abnormal fat accumulation affected more than 50% of patients, and importantly these abnormalities occurred in some patients within the first year of life.

Early onset LAL Deficiency

Early onset LAL Deficiency presents shortly after birth with predominant gastrointestinal and liver involvement. According to an article by Meikle, PJ, et al., in the *Journal of the American Medical Association* from 1999, this disorder has an estimated incidence of approximately two individuals per million lives. This form of LAL Deficiency is the most rapidly fatal and is characterized by growth failure, malabsorption, steatorrhea, and liver enlargement. In early onset LAL Deficiency, infants have little to no residual LAL enzyme activity. This results in the same buildup of fatty material as in late onset LAL Deficiency but the effects are more profound and accelerated, with severe malabsorption of nutrients and profound growth failure, leading to early mortality. Hepatic involvement, as evidenced by liver enlargement and elevation of transaminases, is common in all LAL Deficiency patients, although it appears to be more aggressive in early onset patients than in other patients across the LAL Deficiency continuum.

In the absence of approved therapies for LAL Deficiency, supportive therapies are used in an attempt to mitigate some of the effects of this rapidly fatal disease. Although some stabilization of the clinical condition has been described with nutritional support, these interventions are not believed to substantially modify the outcome in affected patients. As there is presently no effective treatment for LAL Deficiency (including enzyme replacement therapy), patients with early onset LAL Deficiency are sometimes offered experimental therapy with hematopoietic stem cell transplantation. Based on information presented in a chapter by Assmann, G. and Seedorf, U. in the The Metabolic and Molecular Bases of Inherited Disease edited by A. Beaudet et al., early onset LAL Deficiency remains almost universally fatal.

Data from a natural history study of infants with early onset LAL Deficiency were presented in April 2012 at the 3rd International Congress for Lysosomal Diseases. An investigator of the study, Dr. Christian J. Hendriksz presented data demonstrating that infants diagnosed with the early onset form of LAL Deficiency suffer from rapid disease progression and often die prior to, or very shortly after, diagnosis. The early onset natural history study represents a retrospective, observational study based on the compilation of patient records from 19 infants diagnosed with LAL Deficiency and growth failure by 6 months of age. In these patients, the median age at first symptom, diagnosis and death were 1.0 month, 2.2 months and 3.4 months, respectively. Of the six of 19 infants who underwent liver, bone marrow or umbilical cord transplants, five died before 9 months of age.

About Sebelipase Alfa

Sebelipase alfa is a recombinant form of the human LAL enzyme under development as an enzyme replacement therapy for LAL Deficiency. We are currently evaluating sebelipase alfa in a Phase III, global clinical trial known as the ARISE (Acid Lipase Replacement Investigating Safety and Efficacy) trial, a randomized, double-blind, placebo-controlled study of sebelipase alfa in children and adults with late onset LAL Deficiency. Sebelipase alfa has been granted orphan drug designations by the U.S. Food and Drug Administration (FDA), the European Medicines Agency (EMA), and the Japanese Ministry of Health, Labour and Welfare. Additionally, sebelipase alfa received *Fast Track* Designation by the FDA. Sebelipase alfa produced a positive impact on a broad range of abnormalities associated with LAL Deficiency in Phase I/II clinical trials for early and late onset LAL Deficiency as well as in a highly relevant disease model.

In February 2011, we initiated a Phase I/II trial of sebelipase alfa in adults with late onset LAL Deficiency and completed enrollment in December 2011. Patients were predominantly male with a mean age of 32 years (range 19-45). Eight of the nine patients had a history of hepatomegaly, and two of the nine patients had evidence of more advanced liver disease, including cirrhosis and portal hypertension. Seven patients had a history of other cardiovascular conditions. Seven of the nine patients were receiving treatment with lipid modifying therapies including ezetimibe, statins, and other medications. After completing four weeks of treatment in the initial trial and at least four weeks of a post-treatment observation period, patients were allowed to receive continued treatment with sebelipase alfa by enrolling in a long-term and still on-going open-label extension study.

Eight of the nine patients have enrolled in the extension study. These patients received four once-weekly infusions of sebelipase alfa (0.35 mg/kg, 1.0 mg/kg, or 3.0 mg/kg) and then transitioned to every other week infusions of sebelipase alfa (1.0 mg/kg or 3.0 mg/kg). In February 2013, at the Lysosomal Disease Network (LDN) WORLD Symposium, we announced 9-month (38-week) results from the extension study. Data at the LDN WORLD Symposium were derived from the seven patients who completed six months of dosing in the extension study.

For these seven patients, longer term dosing with sebelipase alfa produced mean decreases for ALT and AST from the initial baseline to week 38 of the extension study of 54% and 40%, respectively ($p < 0.016$ for both comparisons). In addition, sebelipase alfa resulted in mean percent decreases from the initial baseline to week 38 of the extension study for low density lipoprotein cholesterol (LDL-C) of 47% (p=0.016), total cholesterol of 33% (p=0.016), triglycerides of 29% (p=0.047), as well as a mean increase in high density lipoprotein cholesterol HDL of 15% (p=0.313).

Sebelipase alfa was generally well tolerated throughout the initial 38 weeks of the extension study. The majority of adverse events were mild and unrelated to sebelipase alfa. Infusion-related reactions were uncommon and the majority were gastrointestinal (diarrhea, abdominal cramping) events of mild severity. One patient with a moderate (Grade 2) allergic type infusion-related reaction has paused treatment with sebelipase alfa pending further testing. No anti-drug antibodies have been detected in any subjects in either the initial portion or extension portion of the Phase I/II study. A single patient during the extension study developed acute cholecystitis and cholelithiasis (two serious adverse events) which were later treated with elective cholecystectomy. This patient has continued treatment with sebelipase alfa without changes in dosing and administration. The investigator considered it unlikely that the two serious adverse events related to sebelipase alfa.

On February 11, 2013 we announced the first patient initiated treatment in the ARISE trial. Additionally, we are conducting ongoing natural history studies to investigate and characterize key aspects of the clinical course of LAL Deficiency to inform the evaluation and care of affected patients.

We are currently enrolling and dosing infants with early onset LAL Deficiency in a Phase II/III open-label trial with sebelipase alfa. One infant, as previously disclosed, began receiving sebelipase alfa on expanded access in April 2011 at the age of four months. This child continues to tolerate treatment well and demonstrates improvement in growth rate and liver function tests. This patient has been enrolled into an extension study as part of the clinical trial program.

Sebelipase alfa Pharmacology

Sebelipase alfa is a recombinant form of the human LAL enzyme. This enzyme is responsible for the metabolism of cholesteryl esters and triglycerides that are delivered to lysosomes by a variety of routes, including low-density lipoprotein receptor mediated endocytosis. Sebelipase alfa is produced by recombinant DNA technology in egg white using our proprietary protein manufacturing platform. The protein contains glycan structures which are specifically recognized and internalized via receptors into key target cells. LAL Deficiency has parallels with Gaucher Disease, as both require effective macrophage targeting. Unlike certain other approved enzyme replacement therapies, however, sebelipase alfa does not require additional processes during manufacturing to either modify glycan synthesis or remove terminal glycans to allow for the correct glycan ligands for macrophage uptake. In addition, levels of mannose-6-phosphate that enable efficient uptake into other cell types are higher in sebelipase alfa than described in other forms of recombinant LAL produced using cell culture based manufacturing platforms. Data from preclinical studies of sebelipase alfa have demonstrated uptake and localization to lysosomes within key cell types, including macrophages and fibroblasts.

Preclinical Development

We reported data from preclinical studies of sebelipase alfa at the American Society of Human Genetics meeting in November 2010, which demonstrated sebelipase alfa's efficacy in a disease model of LAL Deficiency. Sebelipase alfa reduced lipid substrate levels in diseased tissues and corrected disease-related abnormalities associated with LAL enzyme deficiency, including growth failure and liver pathology. The results from the disease model of LAL Deficiency showed marked disruption of liver structure due to the abnormal accumulation of lipids in the hepatocytes and especially in the Kupffer cells. For animals treated with sebelipase alfa, the pathological abnormalities were corrected and normal liver architecture was restored.

Additional data was reported at the European Society for the Study of Liver Disease meeting in March 2011 and the European Society for Pediatric Gastroenterology, Hepatology and Nutrition meeting in May 2011. These studies reported that weekly ("qw") and every other week ("qow") administration of sebelipase alfa to the animals with LAL

Deficiency improved growth, decreased LAL substrate content in affected organs and normalized liver pathology in association with decreases in liver size and in serum transaminases and reversal of liver histopathologic findings. Detailed dose response analysis in a LAL deficient animal model has established a range of effective qw and qow doses supporting dose selection in clinical studies. These data demonstrate that doses equal to or greater than 1 mg/kg qow are highly effective in reducing lipid accumulation in the liver, which is the organ predominantly affected in late onset LAL Deficiency. To reverse growth failure, which is characteristic of patients with early onset LAL Deficiency, maximum efficacy is seen at doses equal to or greater than 3 mg/kg qw.

These studies established proof of concept for sebelipase alfa as an enzyme replacement therapy for LAL Deficiency. In contrast to preclinical testing of most other experimental therapies, it is generally accepted that efficacy for this class of therapies in LSD disease models is highly predictive of clinical effectiveness. In these storage diseases, where animal models have been developed, the missing protein performs a similar function in the animal as it does in humans and replacement of the missing enzyme corrects the disease-related abnormalities. Published examples of preclinical efficacy data in an animal model of a LSD predicting clinical efficacy for products that have been subsequently approved include Schull, et al., in Medical Sciences in 1994 regarding Aldurazyme (the "Schull Article"), Byers, et al., in Pediatrics Research in 2000 regarding Naglazyme (the "Byers Article"), Kikuchi, et al., in The Journal of Clinical Investigation in 1998 regarding Myozyme (the "Kikuchi Article"), and Ioannou, et al., in The American Journal of Human Genetics in 2001 regarding Fabrazyme (the "Ioannou Article").

We have also conducted toxicology studies to support initiation of human dosing. There were no meaningful toxicological findings in four-week repeat dose toxicology studies in rats and monkeys administered intravenous ("IV") infusions of sebelipase alfa at doses up to 50 mg/kg once weekly (i.e., 10-fold greater than anticipated human doses). In a six-month repeated dose toxicity study in monkeys administered once-weekly IV infusions of sebelipase alfa at doses of 3 mg/kg, 10 mg/kg or 30 mg/kg (five males and five females per dose group), or placebo infusions (five males, five females), sebelipase alfa was well tolerated at dose levels up to 30mg/kg per dose, which supports long-term dosing with sebelipase alfa in humans.

Clinical Development

We are pursuing a development strategy for sebelipase alfa that includes clinical trials in patients with both early and late onset LAL Deficiency. The overall goals of the program are to assess safety and tolerability in a broad population of patients, including infants, children, and adults, and to demonstrate clinically meaningful effects on the medical complications of LAL Deficiency. Consistent with study protocols for marketed products for other rare diseases such as Fabrazyme and Myozyme, we anticipate that the patient numbers required for the sebelipase alfa clinical program will be small. We have gained *Fast Track* designation for sebelipase alfa and we intend to file a Biologic License Application ("BLA") with the FDA and marketing applications in other regions if clinical trials are successful.

In December 2010, we filed an investigational new drug ("IND") application with the FDA and submitted clinical trial applications with the UK Medicines and Healthcare Products Regulatory Agency in January 2011. Subsequent clinical trial applications have also been filed in other countries in support of our clinical studies.

Unlike most common diseases where clinical familiarity exists, many aspects of the clinical presentation, disease progression (including mortality and key morbidities) and response to treatment are poorly understood for rare diseases. Two key factors are responsible for these differences. First, the rarity of the disease often limits a physician's clinical experience to a single case. Secondly, the historical absence of any effective therapy reduces the interest and research funding available to support coordinated investigation of the disease. The development of a new potential therapy requires accurate knowledge of the natural course of the disease to support patient diagnosis, endpoint selection and to provide historical data on mortality and morbidity. These are required to inform the evaluation and care of affected patients and to provide a reference for efficacy studies of enzyme replacement or other novel therapies. In order to generate this historical context for LAL Deficiency in support of the sebelipase alfa development program, we are currently conducting two clinical study protocols in approximately 20 countries requiring case record review of patients with early and late onset LAL Deficiency. These natural history studies will be used to investigate and characterize key aspects of the clinical course of the disease, to inform the evaluation and care of affected patients.

In addition to the patients with late-onset LAL Deficiency enrolled in clinical trials, infants with the early onset form of the disease are receiving treatment with sebelipase alfa. One infant, as previously disclosed, began receiving sebelipase alfa on expanded access in April 2011 at the age of four months. This child continues to tolerate treatment well and demonstrates improvement in growth rate and liver function tests. This patient has been enrolled into an extension study as part of the clinical trial program.

The current and planned clinical trials for sebelipase alfa are:

- *Phase I/II Open Label Dose Escalation Study in Adult Patients with Liver Dysfunction Due to LAL Deficiency*. In December 2011, we completed a four week, multi-center U.S. and European study in nine patients to evaluate the safety and tolerability of sebelipase alfa administered weekly in patients with liver dysfunction due to late onset LAL Deficiency. This study was intended to characterize the pharmacokinetics of sebelipase alfa delivered by IV infusion. Additional assessments included evaluating pharmacokinetics and biomarkers of sebelipase alfa 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 sebelipase alfa (0.35 mg/kg, 1.0 mg/kg, or 3.0 mg/kg). Sebelipase alfa 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, however the majority of adverse events were mild and unrelated to sebelipase alfa. The data from the study indicates that treatment with sebelipase alfa 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.
- *Phase II/III Open Label Dose Escalation Study in Children with Growth Failure Due to LAL Deficiency*. In 2011, we also initiated a study to evaluate the safety and tolerability of sebelipase alfa in children with growth failure due to early onset LAL Deficiency. This study will also determine the effect of sebelipase alfa on growth and explore pharmacokinetics of sebelipase alfa and change in pharmacodynamics markers in this population. This study is expected to enroll a total of approximately eight patients at multiple centers in the U.S. and Europe. As early onset LAL Deficiency is rare and progresses rapidly to death within the first year of life, enrollment in this study is contingent on timely identification of newly diagnosed cases.
- *Phase I/II Extension Study to Evaluate the Long-Term Safety, Tolerability, and Efficacy of Sebelipase Alfa in Adult Subjects With LAL Deficiency.* In 2011, we initiated an extension study to assess the long-term safety, tolerability, and efficacy of sebelipase alfa. Eight of the nine patients from the Phase I/II Open Label Dose Escalation Study in Children with Growth Failure Due to LAL Deficiency have enrolled in the extension study. These patients received four once-weekly infusions of sebelipase alfa (0.35 mg/kg, 1.0 mg/kg, or 3.0 mg/kg) and then transitioned to every other week infusions of sebelipase alfa (1.0 mg/kg or 3.0 mg/kg). In February 2013, at LDN WORLD Symposium meeting, we announced 38 week results from the extension study. Data at the LDN WORLD Symposium meeting were derived from seven of the eight patients who completed nine months of dosing in the extension study. For these seven patients, longer term dosing with sebelipase alfa produced mean decreases for ALT and AST from the initial baseline to week 38 of the extension study of 54% and 40%, respectively ($p < 0.05$ for both comparisons). In addition, sebelipase alfa resulted in mean percent decreases from the initial baseline to week 38 of the extension study for LDL-C of 47% (p=0.016), total cholesterol of 33% (p=0.016), triglycerides of 29% (p=0.047), as well as a mean increase in HDL of 15% (p=0.313). In the extension study, liver fat fraction is measured by multi-echo magnetic resonance imaging (MRI) or magnetic resonance spectroscopy and liver volume is measured by MRI. At the end of 24 weeks, treatment with sebelipase alfa in the extension study resulted in a mean decrease in liver fat fraction of 34% and a mean decrease in liver volume of 8%. At the LDN World Symposium in February 2013, investigators presented nine month data from the extension study that demonstrate comparable safety and efficacy to the six month data presented at the November 2012 AASLD meeting. The clinical relevance of these various endpoints remains subject to regulatory assessment and, while these preliminary data are encouraging, they do not guarantee eventual regulatory approval, which will be based on the totality of clinical evidence when studies are complete. Sebelipase alfa was well tolerated throughout the initial 38 weeks of the extension study. The majority of adverse events were mild and unrelated to sebelipase alfa. Related or possibly related adverse events included headache, diarrhea, mild abdominal pain and cholesterol elevation. The majority of infusion-related reactions were gastrointestinal (diarrhea, abdominal cramping) and

of mild severity. No antidrug antibodies were detected in any of the nine patients in the four week portion of the trial or in the seven patients tested in the extension study at nine months. A single patient during the extension study developed acute cholecystitis and cholelithiasis later treated with cholecystectomy. The investigator considered it unlikely that the two serious adverse events related to sebelipase alfa.

- *Phase III Trial of Sebelipase Alfa in Children and Adults with Late Onset LAL Deficiency.* In February 2013, we announced the treatment initiation in the ARISE trial, a global, Phase III, randomized, double-blind, placebo-controlled study of sebelipase alfa in children and adults with late onset LAL Deficiency. The ARISE trial will enroll 50 patients (children and adults) with late onset LAL Deficiency. Patients enrolled in the trial are randomized on a one-to-one basis to every other week infusions of sebelipase alfa (1 mg/kg), or placebo for the double-blind treatment period of 20 weeks. Results from the double-blind period will be used to demonstrate efficacy and safety in support of global submissions for product registration. Patients who participate in the trial will qualify to enter a long-term, open-label extension period. The primary endpoint of the trial is the proportion of patients relative to placebo who achieve normalization of ALT, a marker of liver damage, at the completion of the double-blind treatment period (week 20). Key secondary endpoints include the relative reduction from baseline to week 20 in LDL-C, non-HDL-C, triglycerides, the proportion of patients who achieve AST normalization, and the relative increase in HDL-C. Additional secondary endpoints, including reductions in liver fat content and liver volume and improvements in liver pathology, will be examined in a proportion of patients who undergo these assessments. Deficiency of LAL enzyme activity will be confirmed during patient screening with a dried blood spot biochemical enzyme activity assay performed by Laboratory Corporation of America® Holdings, the central diagnostic testing laboratory performing the tests.

Additional studies may be initiated in order to support requirements for long-term safety and to provide patients with ongoing access to the drug until BLA approval of sebelipase alfa, if received.

Regulatory

Sebelipase alfa has been granted Orphan Drug Designation by the FDA, and we are conducting clinical trials in the U.S. under an active investigational new drug application (IND). The FDA may grant Orphan Drug Designation to a product that treats a rare disease, a condition that affects fewer than 200,000 Americans. As a result of the Orphan Drug Designation, we are eligible to receive a number of benefits, including access to grant funding for clinical trials, tax credits, waiver of the FDA filing and registration fees, and seven years of market exclusivity if approval is received. Additionally, due to the severity of LAL Deficiency, the FDA granted sebelipase alfa *Fast Track* Designation which allows for an expedited regulatory review for the product.

Sebelipase alfa has also been granted Orphan Drug Designation by the EMA, and we have received regulatory clearance in key countries in the European Union (EU) to conduct clinical trials in patients with late and early onset LAL Deficiency. The EMA's Orphan Drug Designation is given to therapies that treat rare diseases, defined as conditions that affect no more than five in 10,000 persons in the EU. As a result of the EMA Orphan Drug Designation, we are eligible to receive access to protocol assistance, direct access to centralized marketing authorization, up to 10 years of marketing exclusivity if approval is received, fee reductions or exemptions, and other national incentives. Under the EU Pediatric Regulations established in 2007, sebelipase alfa may be eligible for an additional two years of market exclusivity.

Sebelipase alfa has also been granted Orphan Drug Designation by the Japanese Ministry of Health, Labour and Welfare, and we have received regulatory clearance in Japan to conduct clinical trials in patients with late and early onset LAL Deficiency. As stipulated in Article 77-2 of the Pharmaceutical Affairs Law of Japan, a drug must meet the following conditions in order to be considered for orphan drug designation in Japan: the drug should be used to treat a disease that affects less than 50,000 people in Japan; the drug treats a disease or condition for which there are no other treatments available in Japan, or the proposed drug is clinically superior to drugs already available on the Japanese market; and the applicant should have a clear product development plan and scientific rationale to support the necessity of the drug in Japan. Specific measures to support the development of orphan drugs include prioritized consultation regarding clinical development and priority review of applications, reduced application fees, extended registration validity period, financial assistance to help cover research and development expenditures, and tax incentives.

Commercialization

LAL Deficiency is an LSD, and like other lysosomal storage diseases such as Gaucher Disease, Fabry Disease, and Pompe Disease, affects a very small patient population hallmarked by unmet medical need, substantial morbidity and increased risk of mortality. As indicated in the table below, Cerezyme®, Fabrazyme®, Myozyme® and Soliris® are ultra-orphan drugs that provide precedent for the ability to commercialize a breakthrough treatment for an ultra-rare medical condition. Members of our management team have previously held leadership roles in the development and commercialization for all four of these precedent products.

Drug	sebelipase alfa	Cerezyme	Fabrazyme	Myozyme	Soliris
Indication	LAL Deficiency	Type I Gaucher Disease	Fabry Disease	Pompe Disease	PNH and aHUS
Estimated Prevalence	1 in 40,000 to 300,000	1 in 59,000 to 86,000	1 in 40,000 to 476,000	1 in 40,000 to 146,000	1 in ~77,000

Table 1. A sampling of ultra-orphan products and their associated incidence

LAL Deficiency is an ultra-rare disorder that falls within the scope of metabolic specialists, hepatologists and lipidologists. Liver complications such as fibrosis, cirrhosis and liver failure dominate the late onset form, and patients may resemble those with other, more common diseases such as non-alcoholic fatty liver disease or non-alcoholic steatohepatitis. Similar to other LSDs, increased disease awareness and improvements in diagnosis supported by the patient and physician communities are critical for identifying patients and facilitating treatment. We are in the process of engaging the physician and patient communities to establish a disease registry that will encourage involvement of all parties to raise awareness of and interest in LAL Deficiency. These include metabolic, hepatic, and lipid physician specialists and patient groups such as LAL Solace, National Organization for Rare Disorders, Eurordis and CLIMB.

The diagnosis of LAL Deficiency patients is anticipated to begin with the specialists' clinical diagnosis aided by the use of differentiating biochemical markers, including abnormal lipid profile and confirmed by a simple blood test for the LAL enzyme.

Our commercial strategy for sebelipase alfa focuses on : (i) raising disease awareness, (ii) facilitating diagnosis, (iii) supporting treatment, and (iv) facilitating third party reimbursement. By aligning resources against these imperatives, we believe that our commercial footprint will be efficient and scaled to a highly specialized market niche. Similar to other companies with such specialized call points, our current plans for resourcing the commercialization effort for sebelipase alfa include a small number of highly specialized field-based representatives supported by a specialized organization in-house. The past experience of members of our management team in leading commercial efforts for ultra-rare products highlights the value of recruiting professionals who have experience with these specialized and focused physician and patient communities. The planned commercial organization is being developed to initially address the needs and opportunities for the North American, European, Latin American, and Asia Pacific regions, as well as other emerging regions.

Pipeline Programs

In addition to sebelipase alfa, we are progressing protein therapeutic programs for other rare diseases, which are currently at different stages of preclinical development. These include two enzyme replacement therapies for other LSDs and additional programs for other rare life-threatening conditions. These protein therapeutic programs are selected based on scientific rationale, unmet medical need within the patient population, potential to substantially impact disease course, and strategic alignment with our corporate and commercial efforts, including a potentially significant commercial opportunity.

The following table describes our product candidate pipeline:

Program	Sebelipase alfa (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- Phase III	Preclinical	Preclinical	Preclinical	Preclinical
Regulatory Opportunity	Orphan Drug Designation • Granted U.S. • 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

We believe our other programs, SBC-103 through -106, also have the potential to present patients and health care practitioners with effective therapies to treat the rare and life-threatening diseases targeted by these programs, which, like LAL Deficiency, are characterized by significant morbidity and mortality. The most advanced of these additional programs is SBC-103, an enzyme replacement therapy for MPS IIIB, or Sanfilippo B. This enzyme is a recombinant form of human N-acetyl-alpha-glucosaminidase (NAGLU). There is currently no approved therapy available for MPS IIIB. Similar to LAL Deficiency, MPS IIIB is an autosomal recessive LSD. While initially appearing unaffected, children born with MPS IIIB usually present with a slowing of development and/or behavioral problems around two years of age, followed by progressive intellectual decline and immobility with complete dependency on care providers. The life-span of most affected individuals does not usually extend beyond late teens to early twenties. Preliminary characterization of the enzyme produced using our production platform demonstrates favorable uptake properties compared to previously published attempts to produce this enzyme using standard cell culture based approaches. At the LDN World Symposium in February 2013, we presented key preclinical data from our SBC-103 program that shows that dosing with SBC-103 reduces the accumulation of substrate in the brain of a MPS IIIB animal model with various dosing approaches.

SBC-104 is an extracellular protein that targets a severe, rare genetic condition. There are no approved therapies for this disease, which is often fully debilitating and frequently results in early death. SBC-104 is being developed as a protein replacement therapy.

SBC-105 is an enzyme replacement therapy being developed to treat a severe, rare metabolic disorder. We believe this program has opportunity in a number of related rare diseases with similar underlying biology.

SBC-106 is a protein therapy that targets a severe and rare genetic condition.

Our Expression Platform

Overview

Our business focus on products for rare diseases was established in 2008 with the appointment of Sanj K. Patel as President and Chief Executive Officer and his redirection of the company. This change represented a substantial shift in the business strategy of the company, formerly known as AviGenics, Inc., which was focused on the development of a technology for expressing protein therapeutics in egg white (EW) with the intention of creating follow-on biologics. With the change in business strategy, the expression platform was switched to a focus on the manufacture of protein therapeutics for rare diseases with unmet medical need.

Our proprietary expression platform remains a key element of our business, and is an important contributor to our current pipeline of rare disease therapeutics. Our expression platform is an integrated approach using recombinant

DNA technology for the creation, optimization and commercial production of protein therapeutics. This mature platform, encompassing over 15 years of research and clinical development, is distinct from cell culture based approaches for protein therapeutic manufacturing and has a number of potential advantages which include:

- Reduced capital investment;
- Competitive protein expression levels;
- Flexibility and consistency during scale-up; and
- Human-like glycosylation patterns that can be tailored for the application.

We believe that this system may reduce the scale-up risks and inconsistency issues often associated with cell culture-based manufacturing of many types of therapeutic proteins. The ability to manufacture our products using our manufacturing platform is a key element of our business. This capability may decrease the capital requirements for progressing a protein therapeutic into clinical development.

Biology

The foundation of our platform is an integrated system of proprietary vectors and methods that allow the generation of transgenic lines of hens which produce high levels of therapeutic protein in EW, a protein friendly matrix. Expression is achieved using our proprietary vectors which allow targeted expression in EW-producing cells, and results in high expression levels of therapeutic proteins. The EW matrix facilitates bulk storage of unpurified EW prior to purification for prolonged periods and is one of a number of manufacturing advantages of our platform over traditional protein manufacturing technologies. In contrast to mammalian cell culture based approaches which utilize immortalized cell lines with high genetic and epigenetic instability, our proprietary vectors allow incorporation of the gene of interest into the genome of normal cells of an avian (*Gallus*) with selective expression of the resulting protein in the oviduct tissues and secretion into EW. The importance of this cellular environment for therapeutic protein expression is highlighted by the tight consistency of post-translational modification, including glycosylation, seen in proteins manufactured using our platform compared to cell culture produced material. Furthermore, our expression system yields consistent expression levels and quality of protein within production lines and through multiple generations. We believe that our platform is the only approach for creating transgenic hens that produce a therapeutic protein which has been used successfully in clinical trials and which can enable commercial scale manufacturing.

Our proprietary technology allows for the production of proteins with glycosylation patterns that are suitable for many different diseases without a requirement for additional processes either during manufacturing (inhibition of specific glycosylation enzymes) or post purification (enzymatic removal of terminal sugars) to modify terminal glycan structures impacting biodistribution. Furthermore, unlike some alternative expression platforms, our expression system produces proteins with ‘human like’ glycan structures and does not incorporate non-human sugars into glycans.

Manufacturing and Supply

We have demonstrated the platform’s ability to successfully produce a wide array of therapeutic proteins, including therapeutic enzymes, cytokines, monoclonal antibodies and fusion proteins. Furthermore, from 2004 to 2008, we manufactured investigational products using our expression system to supply Phase I and Phase II multinational clinical studies run in the U.S., EU, and India, which included more than 250 patients. In addition, this expression system has been used in the manufacturing of clinical phase I/II and III materials to support the development of sebelipase alfa. Within this clinical development program greater than 200 infusions or doses have been provided to pediatric and adult patients in global trials using clinical materials manufactured from this platform. The regulatory clearance for these studies followed substantial review by global regulatory authorities of detailed information related to the manufacturing platform and the expression system. This platform is covered by a comprehensive intellectual property portfolio owned or exclusively licensed by us and, we believe, provides us with expanded freedom to operate compared to other systems.

We currently rely on our manufacturing facilities, as discussed in further detail in “Item 2. Properties” for the production of products utilized in our clinical and preclinical activities. We also depend on a limited number of third

party providers for other services in the manufacturing process, including product purification, product finishing, packaging, vialing and labeling.

FUZEON

As part of the Reverse Merger, we acquired the rights to a royalty stream related to FUZEON®, an HIV fusion inhibitor, developed in collaboration with F. Hoffmann-La Roche Ltd. ("Roche"). The FDA approved the use of FUZEON in combination with other anti-HIV drugs for the treatment of HIV-1 infection in treatment-experienced patients with evidence of HIV-1 replication despite ongoing anti-HIV therapy. After the FDA granted accelerated approval, commercial sales of FUZEON began in March 2003. Full approval was granted by the FDA in October 2004. Roche also filed an application for European marketing approval of FUZEON in September 2002 and was granted marketing approval under exceptional circumstances by the European Agency for the Evaluation of Medicinal Products in May 2003.

We granted Roche an exclusive license to manufacture and sell FUZEON worldwide, and we receive royalties from Roche on net sales of FUZEON. Although royalties from sales of FUZEON, which have declined every year from 2007 through 2012, is a significant source of revenue, we do not consider the sales of FUZEON to be material to our business strategy.

Patents and Proprietary Rights

We seek to aggressively protect the proprietary technology that is important to our business, including pursuing patents that cover our product candidates and compositions, their methods of use and the processes for their manufacture, as well as any other relevant inventions and improvements that are commercially important to the development of our business. We also rely on trade secrets and other know-how that may be important to the development of our business.

Our patent portfolio is currently composed of over 200 issued patents and 150 patent applications in major territories, including the U.S., Europe, Brazil, Japan, China, Canada, India, and Australia, and includes patents and patent applications that we own as well as license from other parties. These patents and patent applications cover various aspects of our manufacturing expression platform, product candidate pipeline and other product candidates that we are no longer developing. Patents covering aspects of our manufacturing expression platform will expire between 2016 and 2026. If they were to issue, current patent applications covering the expression platform will expire between 2018 and 2032 and current patent applications covering sebelipase alfa will expire between 2031 and 2032.

In addition, our own pending applications contain claims directed to compositions and improved methods for expression of therapeutic proteins, with expirations between 2018 and 2032. We continue to develop new intellectual property based on ongoing research to improve and enhance our expression platform.

While there can be no assurance that patent applications relating to our product candidates will ultimately issue or what the scope of the claims of such patent applications will cover if they were to issue, we expect to rely heavily on orphan drug exclusivity for our product candidates, including sebelipase alfa, which generally grants seven years of marketing exclusivity under the Federal Food, Drug, and Cosmetic Act, and up to 10 years of marketing exclusivity in Europe. In addition, we continue to aggressively pursue intellectual property protection for our product candidates in the form of patent applications that have been and will continue to be filed in the U.S. and internationally.

The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries in which we file, the patent term is 20 years from the date of filing the non-provisional application. In the U.S., a patent's term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the U.S. Patent and Trademark Office ("USPTO") in granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier-filed patent. Additional patent extension can be granted for time spent in development and regulatory review in certain jurisdictions as well.

University of Georgia Research Foundation

Our patent rights include an exclusive, worldwide, sublicensable license to the patents owned by University of Georgia Research Foundation ("UGARF") to develop, make, use and commercialize the avian transgenesis technology. Under the license, which was initially executed in 1996 and amended in 2007, we paid UGARF an upfront license fee and issued shares of our common stock in return for worldwide exclusive rights under the license. Additionally, UGARF is eligible to receive low single digit royalties on net sales of products covered under the license. This license agreement covers patents and patent applications pending worldwide that are the basis of our expression platform. The UGARF license is effective until the last to expire of the licensed patents. Patents exclusively licensed from UGARF covering key aspects of our expression platform will expire between 2018 and 2021. UGARF can terminate the license or, at UGARF's discretion, convert the license into a non-exclusive license, if we materially breach the agreement, make any materially false reports to UGARF, or fail to pay any required consideration under the agreement. We have the right to terminate the agreement upon 60 days' prior written notice to UGARF.

University of Minnesota

In 2009, we entered into an exclusive license agreement, with the right to grant sublicenses, with the University of Minnesota that relates to compositions and methods useful for generating transgenic *Gallus*. In exchange for the license, which is effective until the last to expire of the licensed patents, we paid the University of Minnesota an upfront license fee. In addition, University of Minnesota is entitled to minimal annual royalties which are creditable against low single digit royalties on net sales of products covered under the license. The 18 patents included in this license agreement expire by 2016. The University of Minnesota may terminate the agreement for our failure to timely cure any material breach or failure to perform any obligations under the agreement. We may terminate the agreement at any time after the third anniversary of the agreement upon 90 days' prior written notice.

Pangenix

In 2000, we entered into a non-exclusive, sublicensable license agreement with Pangenix that relates to patents covering compositions and methods useful for generating transgenic *Gallus*. In exchange for the license granted by Pangenix, which is effective until the last to expire of the licensed patents, Pangenix received an upfront license fee and is entitled to receive minimum annual royalties creditable against low single digit royalties on net sales of products covered under the license. The patents included in this license agreement are set to expire in 2015. Pangenix may terminate the agreement for our failure to timely cure any material breach or failure to perform our obligations under the agreement. We may terminate upon 60 days' notice to Pangenix.

Roche

On May 25, 2011, Pre-Merger Trimeris and Roche entered into the Amended and Restated License Agreement (the "Roche License Agreement"), effective as of January 1, 2011, pursuant to which Roche has an exclusive license to manufacture and sell FUZEON worldwide and we receives royalty payments equal to 16% of worldwide net sales of FUZEON occurring from and after January 1, 2011. Under the Roche License Agreement, Roche may deduct from its royalty payments to us 50% of any royalties paid to third parties which are reasonably required to allow Roche to sell FUZEON in a given country, including royalties paid to Novartis Vaccines and Diagnostics, Inc. (Novartis) pursuant to a settlement agreement between Roche and Novartis related to the manufacture, sale and offer of FUZEON. To calculate the royalty revenue, a 5.5% distribution charge is deducted from Roche's reported net sales, and we receive a 16% royalty on the adjusted net sales amount.

Sales and Marketing

We are currently developing marketing, sales and distribution capabilities to support commercialization for sebelipase alfa. Similar to other companies with such specialized call points, our current plans for commercialization resources include limited field-based representatives supported by a specialized in-house organization to address the needs and opportunities for the North American, European, Latin American, and Asia Pacific regions.

With respect to pipeline products and other product candidates, we may elect to utilize our own commercial capabilities to market and sell a product for which we obtain regulatory approval.

Competition

Our industry is highly competitive and subject to rapid and significant technological change. Our potential competitors include large pharmaceutical and biotechnology companies and specialty pharmaceutical companies, academic institutions, government agencies, and research institutions. The market for enzyme replacement therapies is becoming increasingly competitive. However, our products, upon approval, will be focused, at least initially, on specific orphan markets characterized by high unmet medical need. Key competitive factors affecting the commercial success of our product candidates are likely to be efficacy, safety and tolerability profile, reliability and durability of response, convenience of dosing, and price and reimbursement.

Government Regulation

The preclinical studies and clinical testing, manufacture, labeling, storage, record keeping, advertising, promotion, export, and marketing, among other things, of our product candidates and future products, are subject to extensive regulation by governmental authorities in the U.S. and other countries. In the U.S., pharmaceutical products are regulated by the FDA under the Federal Food, Drug, and Cosmetic Act and other laws, including, in the case of biologics, the Public Health Service Act. We expect sebelipase alfa to be regulated by the FDA as a biologic. Biologics require the submission of a BLA and approval by the FDA prior to being marketed in the U.S. Manufacturers of biologics may also be subject to state regulation. Failure to comply with FDA requirements, both before and after product approval, may subject us and/or our partners, contract manufacturers, and suppliers to administrative or judicial sanctions, including FDA refusal to approve applications, warning letters, product recalls, product seizures, total or partial suspension of production or distribution, fines and/or criminal prosecution.

The steps required before a biologic may be approved for marketing of an indication in the U.S. generally include:

- preclinical laboratory tests and animal tests;
- submission to the FDA of an IND for human clinical testing, which must become effective before human clinical trials may commence;
- adequate and well-controlled human clinical trials to establish the safety and efficacy of the product;
- submission to the FDA of a BLA or supplemental BLA;
- FDA pre-approval inspection of product manufacturers; and
- FDA review and approval of the BLA or supplemental BLA.

Preclinical studies include laboratory evaluation, as well as animal studies to assess the potential safety and efficacy of the product candidate. Preclinical safety tests must be conducted in compliance with FDA regulations regarding good laboratory practices. The results of the preclinical tests, together with manufacturing information and analytical data, are submitted to the FDA as part of an IND, which must become effective before human clinical trials may be commenced. The IND will automatically become effective 30 days after receipt by the FDA, unless the FDA before that time raises concerns about the drug candidate or the conduct of the trials as outlined in the IND. The IND sponsor and the FDA must resolve any outstanding concerns before clinical trials can proceed. Submission of an IND does not guarantee FDA authorization to commence clinical trials.

Clinical trials involve the administration of the investigational product to healthy volunteers or to patients, under the supervision of qualified principal investigators. Each clinical study at each clinical site must be reviewed and approved by an independent institutional review board prior to the recruitment of subjects.

Clinical trials are typically conducted in three sequential phases, but the phases may overlap and different trials may be initiated with the same drug candidate within the same phase of development in similar or differing patient populations. Phase I studies may be conducted in a limited number of patients, but are usually conducted in healthy volunteer subjects. The drug is usually tested for safety and, as appropriate, for absorption, metabolism, distribution, excretion, pharmaco-dynamics and pharmaco-kinetics.

Phase II usually involves studies in a larger, but still limited, patient population to evaluate preliminarily the efficacy of the drug candidate for specific, targeted indications to determine dosage tolerance and optimal dosage and to identify possible short-term adverse effects and safety risks.

Phase III trials are undertaken to further evaluate clinical efficacy of a specific endpoint and to test further for safety within an expanded patient population at geographically dispersed clinical study sites. Phase I, Phase II, or Phase III testing might not be completed successfully within any specific time period, if at all, with respect to any of our product candidates. Results from one trial are not necessarily predictive of results from later trials. Furthermore, the FDA may suspend clinical trials at any time on various grounds, including a finding that the subjects or patients are being exposed to an unacceptable health risk.

The results of the preclinical studies and clinical trials, together with other detailed information, including information on the manufacture and composition of the product, are submitted to the FDA as part of a BLA requesting approval to market the product candidate for a proposed indication. Under the Prescription Drug User Fee Act, as amended, the fees payable to the FDA for reviewing a BLA, as well as annual fees for commercial manufacturing establishments and for approved products, can be substantial. The BLA review fee alone can exceed $500,000, subject to certain limited deferrals, waivers, and reductions that may be available. Each BLA submitted to the FDA for approval is typically reviewed for administrative completeness and reviewability within 45 to 60 days following submission of the application. If found complete, the FDA will "accept" the BLA, thus triggering a full review of the application. The FDA may refuse to file any BLA that it deems incomplete or not properly reviewable at the time of submission. The FDA's established goal is to review 90% of priority BLA applications in six months and 90% of standard BLA applications in 10 months, whereupon a review decision is to be made. The FDA, however, may not approve a drug within these established goals and its review goals are subject to change from time to time. Further, the outcome of the review, even if generally favorable, may not be an actual approval but an "action letter" that describes additional work that must be done before the application can be approved. Before approving a BLA, the FDA may inspect the facilities at which the product is manufactured and will not approve the product unless compliance with cGMP is satisfactory. The FDA may deny approval of a BLA if applicable statutory or regulatory criteria are not satisfied, or may require additional testing or information, which can delay the approval process. FDA approval of any application may include many delays or never be granted. If a product is approved, the approval will impose limitations on the indicated uses for which the product may be marketed, may require that warning statements be included in the product labeling, may require that additional studies be conducted following approval as a condition of the approval, and may impose restrictions and conditions on product distribution, prescribing, or dispensing in the form of a risk management plan, or otherwise limit the scope of any approval. Marketing a product for other indicated uses or making certain manufacturing or other changes requires FDA review and approval of a BLA supplement or new BLA. Further post-marketing testing and surveillance to monitor the safety or efficacy of a product is required. Also, product approvals may be withdrawn if compliance with regulatory standards is not maintained or if safety or manufacturing problems occur following initial marketing. In addition, new government requirements may be established that could delay or prevent regulatory approval of our product candidates under development.

As part of the Patient Protection and Affordable Care Act of 2010, Public Law No. 111-148, under the subtitle of Biologics Price Competition and Innovation Act of 2009 ("BPCI"), a statutory pathway has been created for licensure, or approval, of biological products that are biosimilar to, and possibly interchangeable with, earlier biological products licensed under the Public Health Service Act. Also under the BPCI, innovator manufacturers of original reference biological products are granted 12 years of exclusive use before biosimilars can be approved for marketing in the U.S. The objectives of the BPCI are conceptually similar to those of the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the "Hatch-Waxman Act," which established abbreviated pathways for the approval of drug products. The implementation of an abbreviated approval pathway for biological products is under the direction of the FDA and is currently being developed. In late 2010, the FDA held a hearing to receive comments from a broad group of stakeholders regarding the implementation of the BCPI. The approval of a biologic product biosimilar to one of our products could have a material adverse impact on our business as it may be significantly less costly to bring to market and may be priced significantly lower than our products.

Both before and after the FDA approves a product, the manufacturer and the holder or holders of the BLA for the product are subject to comprehensive regulatory oversight. For example, quality control and manufacturing procedures

must conform, on an ongoing basis, to cGMP requirements, and the FDA periodically inspects manufacturing facilities to assess compliance with cGMP. Accordingly, manufacturers must continue to spend time, money and effort to maintain cGMP compliance.

Orphan Drug Act

The Orphan Drug Act provides incentives to manufacturers to develop and market drugs for rare diseases and conditions affecting fewer than 200,000 persons in the U.S. at the time of application for Orphan Drug Designation. The first developer to receive FDA marketing approval for an orphan drug is entitled to a seven year exclusive marketing period in the U.S. for that product. However, a drug that the FDA considers to be clinically superior to, or different from, another approved orphan drug, even though for the same indication, may also obtain approval in the U.S. during the seven year exclusive marketing period. In addition, holders of exclusivity for orphan drugs are expected to assure the availability of sufficient quantities of their orphan drugs to meet the needs of patients. Failure to do so could result in the withdrawal of marketing exclusivity for the drug.

Legislation similar to the Orphan Drug Act has been enacted in other countries outside the U.S., including in the EU. The orphan legislation in the EU is available for therapies addressing chronic debilitating or life-threatening conditions that affect five or fewer out of 10,000 persons or are financially not viable to develop. The market exclusivity period is for 10 years, although that period can be reduced to six years if, at the end of the fifth year, available evidence establishes that the product is sufficiently profitable not to justify maintenance of market exclusivity. The market exclusivity may be extended to 12 years if sponsors complete a pediatric investigation plan agreed upon with the relevant committee of the EMA.

Foreign Regulation

In addition to regulations in the U.S., we are subject to a variety of foreign regulatory requirements governing human clinical trials and marketing approval for drugs. The foreign regulatory approval process includes all of the risks associated with FDA approval set forth above, as well as additional country-specific regulations. Whether or not we obtain FDA approval for a product, we must obtain approval of a product by the comparable regulatory authorities of foreign countries before we can commence clinical trials or marketing of the product in those countries. The approval process varies from country to country, and the time may be longer or shorter than that required for FDA approval. The requirements governing the conduct of clinical trials, product licensing, pricing, and reimbursement vary greatly from country to country.

Employees

As of December 31, 2012, we had 115 full-time employees. Approximately 81 were primarily engaged in research and development activities and 34 were primarily engaged in general, administrative, and commercial activities. None of our employees are subject to a collective bargaining agreement, and we believe our employee relations to be good.

Available Information

Our internet website address is http://www.synageva.com. Through our website, we make available, free of charge, our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K, any amendments to those reports, proxy and registration statements, and all of our insider Section 16 reports, as soon as reasonably practicable after such material is electronically filed with, or furnished to, the SEC. These SEC reports can be accessed through the "Investor Relations" section of our website. Additionally, our Board of Directors adopted a Code of Business Conduct and Ethics applicable to the Board of Directors, our officers and all other employees. The Code of Business Conduct and Ethics is available on our web site. We intend to disclose on our website any amendments or waivers to our Code of Business Conduct and Ethics that are required to be disclosed pursuant to SEC rules.

The information found on our website is not part of this or any other report we file with, or furnish to, the SEC. Paper copies of our SEC reports are available free of charge upon request in writing to Investor Relations, Synageva BioPharma Corp., 128 Spring Street, Suite 520, Lexington MA 02421.

ITEM 1A. RISK FACTORS

RISK FACTORS

Investing in our securities involves risk. Prior to making a decision about investing in our securities, you should carefully consider the specific risk factors discussed below and all of the other information contained or incorporated by reference in this Annual Report on Form 10-K. The risks and uncertainties we have described are not the only ones we face. Additional risks and uncertainties not presently known to us or that we currently deem immaterial may also affect our operations. If any of these risks were to occur, our business, financial condition or results of operations would likely suffer. In that event, the trading price of our common stock could decline, and you could lose all or part of your investment.

Risks Related to Our Business

We are largely dependent on the success of our leading product candidate, sebelipase alfa. All of our product candidates, including sebelipase alfa, are still in either preclinical or clinical development. Clinical trials of our product candidates may not be successful. If we are unable to commercialize sebelipase alfa, or experience significant delays in doing so, our business will be materially harmed.

Our business prospects are largely dependent upon the successful development and commercialization of sebelipase alfa. We are currently initiating global clinical trial sites and recently dosed the first patient in the ARISE trial, a randomized, double-blind, placebo-controlled Phase III trial of sebelipase alfa in children and adults with late onset LAL Deficiency. We are currently enrolling infants with early onset LAL Deficiency in a Phase II/III open-label trial with sebelipase alfa. Before we can commercialize product candidates, including sebelipase alfa, we need to:

- conduct substantial research and development;
- undertake preclinical and clinical testing, sampling activity and other costly and time consuming measures;
- scale-up manufacturing processes; and
- pursue and obtain marketing and manufacturing approvals and, in some jurisdictions, pricing and reimbursement approvals.

This process involves a high degree of risk and takes many years. Our product development efforts with respect to a product candidate may fail for many reasons, including:

- failure of the product candidate in preclinical studies;
- failure of later trials to confirm positive results from earlier preclinical studies or clinical trials;
- delays or difficulty enrolling patients in clinical trials, particularly for disease indications with small patient populations;
- failure to identify a sufficient number of patients who meet the clinical trial enrollment criteria and/or who would support commercial launch;
- patients exhibiting adverse reactions to the product candidate or indications of other safety concerns;
- insufficient clinical trial data to support the effectiveness or superiority of the product candidate;
- inability to manufacture sufficient quantities of the product candidate for development or commercialization activities in a timely and cost-efficient manner, if at all;
- inability to produce or sufficiently test the comparability of drug materials derived from our manufacturing processes or the processes run by our third party manufacturing partners which could impact our ability or timing with respect to receiving regulatory approval for our product candidates;
- failure to obtain, or delays in obtaining, the required regulatory approvals for the product candidate, the facilities or the processes used to manufacture the product candidate; or
- changes in the regulatory environment, including pricing and reimbursement, that make development of a new product or of an existing product for a new indication no longer desirable.

Few research and development projects result in commercial products, and success in preclinical studies or early clinical trials often is not replicated in later studies.

We may decide to abandon development of a product candidate or service at any time, or we may be required to expend considerable resources repeating clinical trials or conducting additional trials, either of which would increase costs of development and delay any revenue from those programs.

In addition, a regulatory authority may deny or delay an approval because it is not satisfied with the structure or conduct of clinical trials or due to its assessment of the data we supply. A regulatory authority, for instance, may not believe that we have adequately addressed negative safety signals. Clinical data is subject to varied interpretations, and regulatory authorities may disagree with our assessments of data. In any such case, a regulatory authority could insist that we provide additional data, which could substantially delay or even prevent commercialization efforts, particularly if we are required to conduct additional pre-approval clinical studies.

The results of our clinical trials may not prove sufficient to obtain regulatory approval of our product candidates, and subsequent trials may fail to replicate promising data seen in earlier preclinical studies and clinical trials.

Our Phase I/II study in adults with late onset LAL Deficiency completed enrollment in December 2011. Interim data from this study provided encouraging indications regarding safety and tolerability as well as effects consistent with preclinical findings and the known mechanism action for LAL. We are currently initiating global clinical trial sites to continue to enroll the ARISE trial, a randomized, double-blind, placebo-controlled Phase III trial of sebelipase alfa in children and adults with CESD, the late onset form of LAL Deficiency. Our Phase II/III study in infants with early onset LAL Deficiency is currently on-going. We also are developing other product candidates for other rare diseases, including SBC-103 targeting a disease known as MPS IIIB. In February 2013, we presented preclinical data from the SBC-103 program that shows that dosing with SBC-103 reduces the accumulation of substrate in the brain of an MPS IIIB animal model.

Promising results in our preclinical studies or clinical trials may not be replicated in ongoing and future studies or trials, and final data analysis may differ from interim data analysis. Even if our additional trials of sebelipase alfa are conducted and completed as planned, the results may not prove sufficient to obtain regulatory approval. Success in preclinical testing does not ensure success in clinical trials, and success in early stage clinical trials does not ensure success in later clinical trials. Phase III clinical trials often fail to replicate encouraging results seen in preclinical studies and early clinical trials. We will incur substantial expenses moving our product candidates through stages of development, with no assurance that any of our product candidates will ultimately be commercialized. Future studies may, for example, indicate safety concerns that regulatory authorities view as unacceptable. Final data analysis of our completed, ongoing and future clinical trials may fail to demonstrate that our product candidates are sufficiently safe and effective for pursued indications. Any such failure could cause us to abandon a product candidate, substantially delay development of other product candidates, or require substantial expenditures to conduct additional trials. Both preclinical and clinical data are often susceptible to varying interpretations that may delay, limit or prevent regulatory approvals or commercialization. Any delay in, or termination of, our clinical trials would delay our obtaining regulatory approval of the affected product candidate and, consequently, our ability to commercialize that product candidate and potentially our other product candidates. Development and commercialization of therapies for rare diseases requires expenditure of significant funds with no assurance of success.

We may find it difficult to enroll patients in our clinical trials.

Sebelipase alfa is being developed to treat LAL Deficiency, which is very rare. Studies by investigators who screened various populations for a common mutation that causes LAL Deficiency indicate a prevalence range of 1:40,000 to 1:300,000 for late onset LAL Deficiency. There is no (or very little) prevalent population for early onset LAL Deficiency, since these infants rarely survive beyond the first year of life. Potential patients for our product candidates, including sebelipase alfa, may not be adequately diagnosed or identified with the diseases being targeted by our product candidates. We may not be able to initiate or continue clinical trials if we are unable to locate a sufficient number of eligible patients to participate in the clinical trials required by the FDA or other non-U.S. regulatory

agencies. In addition, the process of finding and diagnosing patients may prove costly. We are currently initiating global clinical trial sites to continue patient enrollment in the ARISE trial, a randomized, double-blind, placebo-controlled Phase III trial of sebelipase alfa in children and adults with CESD, the late onset form of LAL Deficiency. We are currently enrolling infants with early onset LAL Deficiency, or Wolman disease, in a Phase II/III open-label trial with sebelipase alfa. Our inability to enroll a sufficient number of patients for any of our current or future clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether.

If our preclinical studies do not produce positive results, if our clinical trials are delayed or if serious side effects are identified during drug development, we may experience delays, incur additional costs and ultimately be unable to commercialize our product candidates.

Before obtaining regulatory approval for the sale of our product candidates, we must conduct, at our own expense, extensive preclinical tests to demonstrate the safety of our product candidates in animals, and clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Preclinical and clinical testing is expensive, difficult to design and implement, and can take many years to complete. A failure of one or more preclinical studies or clinical trials can occur at any stage of testing. We may experience numerous unforeseen events during, or as a result of, preclinical testing and the clinical trial process, which could delay or prevent our receipt of regulatory approval for, or the commercialization of, our product candidates, including:

- our preclinical tests or clinical trials may produce negative or inconclusive results, and we may decide to conduct, or regulators may require, additional preclinical testing or clinical trials, or we may abandon projects that we expect to be promising;
- a regulatory authority or institutional review board may not authorize us to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- conditions imposed on us by the FDA or any non-U.S. regulatory authority regarding the scope or design of our clinical trials may require us to resubmit our clinical trial protocols to institutional review boards for re-inspection due to changes in the regulatory environment;
- the number of patients required for clinical trials may be larger than we anticipate or participants may drop out of clinical trials at a higher rate than we anticipate;
- our third-party contractors or clinical investigators may fail to comply with regulatory requirements or fail to meet their contractual obligations to us in a timely manner or at all;
- we might have to suspend or terminate one or more of our clinical trials if we, a regulatory authority or an institutional review board determine that the participants are being exposed to unacceptable health risks;
- a regulatory authority or institutional review board may require that we hold, suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements;
- the cost of our clinical trials may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct our clinical trials may be insufficient or inadequate or we may not be able to reach agreements on acceptable terms with prospective contract manufacturing organizations;
- we may not be able to reach agreements on acceptable terms with prospective clinical research organizations; or
- the effects of our product candidates may not be the desired effects, may include undesirable side effects, or the product candidates may have other unexpected characteristics.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate or are unable to successfully complete our clinical trials or other testing or if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- be delayed in obtaining, or may not be able to obtain, marketing approval for one or more of our product candidates;

- obtain approval for indications that are not as broad as intended or entirely different than those indications for which we sought approval; or
- have the product removed from the market after obtaining marketing approval.

Our product development costs will also increase if we experience delays in testing or approvals. We do not know whether any preclinical tests or clinical trials will be initiated as planned, will need to be restructured or will be completed on schedule, if at all. Significant preclinical or clinical trial delays could also shorten the patent protection period during which we may have the exclusive right to commercialize our product candidates. Such delays could allow our competitors to bring products to market before we do and impair our ability to commercialize our products or product candidates.

We have neither obtained marketing approval, nor commercialized any of our current rare disease product candidates.

We have neither obtained marketing approval nor commercialized any of our current product candidates and do not expect to receive marketing approval or generate revenue from the direct sale of our products, including sebelipase alfa, for several years, if ever. We still have only limited experience in conducting clinical trials for sebelipase alfa even though we are currently initiating global clinical trial sites to prepare for initial patient enrollment in the first half of 2013 for a randomized, double-blind, placebo-controlled Phase III trial of sebelipase alfa in children and adults with CESD, the late onset form of LAL Deficiency. We are also currently enrolling infants with early onset LAL Deficiency, or Wolman disease, in a Phase II/III open-label trial with sebelipase alfa. Additionally, we are conducting preclinical studies with other product candidates for various other indications. Our limited experience might prevent us from successfully designing or implementing a clinical trial for any of these diseases. We may not be able to demonstrate that our product candidates meet the appropriate standards for regulatory approval. If we are not successful in conducting and managing our preclinical development activities or clinical trials or obtaining regulatory approvals, we might not be able to commercialize our lead product candidates, or might be significantly delayed in doing so, which will materially harm our business.

If we infringe the rights of third parties we might have to forgo selling our future products, pay damages, or defend litigation.

If our product candidates, methods, processes, or other technologies infringe the proprietary rights of other parties, we could incur substantial costs and might have to:

- obtain rights or licenses from such third parties, which might not be available on commercially reasonable terms, if at all;
- abandon an infringing product candidate;
- redesign products or processes to avoid infringement;
- stop using the subject matter claimed in the patents held by others;
- pay damages; and/or
- defend litigation or administrative proceedings which might be costly whether we win or lose, and which could result in a substantial diversion of financial and management resources.

Any of these events could substantially harm our earnings, financial condition, and operations.

Although no infringement, misappropriation, or similar claim or action is pending or threatened, we are aware of a patent issued by the European Patent Office, and subsequently validated in Great Britain, France, Germany, Italy and Spain, that relates generally to the use of LAL and may therefore be relevant to sebelipase alfa. On November 23, 2012, at an initial opposition hearing at the European Patent Office, a three member panel upheld claims that relate generally to LAL for the treatment of LAL Deficiency. This decision is subject to appeal. We disagree with the ruling at the initial hearing, and continue to believe that the patent claims are invalid due to a substantial body of prior art. Our lead development program currently evaluates our LAL product candidate, sebelipase alfa, as a treatment for

patients with early onset and late onset LAL Deficiency and therefore the upheld patent claims may be relevant to sebelipase alfa. We continue to believe that such patent does not currently affect our freedom to conduct clinical trials for sebelipase alfa. We also believe that the patent is invalid due to a substantial body of prior art. We have recently taken additional proactive steps, including instituting proceedings in the UK Patents Court and a French court seeking a declaration that the issued patent is invalid and that the current anticipated activities related to our development and commercialization of sebelipase alfa do not infringe the patent. We also may take further steps to accelerate resolution of this matter, which further steps may include contacting the patentee, bringing additional prior art documents to the attention of the European Patent Office or other actions. The UK Patents Court has scheduled a trial for May 2013. If the patent is ultimately maintained in unamended form by the European Patent Office, then our ability to commercialize sebelipase alfa in the countries where the patent is validated could be adversely affected and/or halted until the patent expires, unless the patent is invalidated by the relevant national courts prior to expiration. In addition, the same patentee has been granted a U.S. patent on the use of LAL to treat atherosclerosis, and has two pending patent applications in the U.S. related to LAL, whose claims have currently been rejected by the U.S. patent office. Equivalent patent applications are also pending in Canada and Brazil. We believe that we will not be infringing this issued U.S. patent on atherosclerosis in commercializing sebelipase alfa for the indications for which we are seeking approval and that the issued U.S. patent is invalid due to the prior art described above. We further believe that, due to the substantial body of prior art, the pending applications will not grant with claims that would encompass our contemplated activities or, if they did, they would be invalid due to the prior art. If, however, these pending applications are granted in a form which covers sebelipase alfa, then our ability to commercialize sebelipase alfa in these countries and the United States could be adversely affected and/or halted until the patent expires in these countries, unless a license is obtained to the patents or the patents are otherwise revoked.

If the market opportunities for our product candidates are smaller than we believe they are, our revenues may be adversely affected and our business may suffer.

We focus our research and product development on treatments for rare diseases. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on estimates. Currently, most reported estimates of the prevalence of these diseases are based on studies of small subsets of the population of specific geographic areas, which are then extrapolated to estimate the prevalence of the diseases in the broader world population. For example, studies estimate the prevalence of late onset LAL Deficiency between 1:40,000 and 1:300,000. These estimates may prove to be incorrect and new studies may change the estimated prevalence of these diseases.

The commercial success of any product candidate that we may develop, including sebelipase alfa, will depend upon the degree of market acceptance by physicians, patients, third party payors and others in the medical community.

Any future product that we may bring to the market, including sebelipase alfa, may not gain market acceptance by physicians, patients, third party payors and others in the medical community. If these rare disease products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of these product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the prevalence and severity of any side effects, including any limitations or warnings contained in a product's approved labeling;
- the efficacy and potential advantages over alternative treatments;
- relative convenience and ease of administration;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support and timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third party insurance coverage or reimbursement.

Even if a potential product displays a favorable efficacy and safety profile in preclinical and clinical trials, market acceptance of the product will not be known until after it is launched. Our efforts to educate the medical community and third party payors on the benefits of the product candidates may require significant resources and may never be successful. Such efforts to educate the marketplace may require more resources than are required by the conventional technologies marketed by our competitors.

Uncertainties relating to third-party reimbursement and health care reform measures could limit payments or reimbursements for future products that we may develop could materially adversely affect our business.

In the U.S. and elsewhere, sales of prescription drugs depend in part on the consumer's ability to obtain reimbursement for the cost of the drugs from third-party payors, such as private and government insurance programs. Third-party payors are increasingly challenging the prices charged for medical products and services in an effort to promote cost containment measures and alternative health care delivery systems. Our prospects for achieving profitability will depend heavily upon the availability of adequate reimbursement for the use of our approved product candidates from governmental and other third party payors, both in the U.S. and in other markets. Reimbursement by a third party payor may depend upon a number of factors, including the third party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

Obtaining reimbursement approval for a product from each governmental or other third party payor is a time consuming and costly process that could require us to provide supporting scientific, clinical and cost effectiveness data for the use of our products to each payor. We may not be able to provide data sufficient to gain acceptance with respect to reimbursement or might need to conduct post-marketing studies in order to demonstrate the cost-effectiveness of any future products to such payors' satisfaction. Such studies might require us to commit a significant amount of management time and financial and other resources. Even when a payor determines that a product is eligible for reimbursement, the payor may impose coverage limitations that preclude payment for some uses that are approved by the FDA or non-U.S. regulatory authorities. In addition, there is a risk that full reimbursement may not be available for high priced products. Moreover, eligibility for coverage does not imply that any product will be reimbursed in all cases or at a rate that allows us to make a profit or even cover our costs. Interim payments for new products, if applicable, may also not be sufficient to cover costs and may not be made permanent.

We are exposed to product liability and preclinical and clinical liability risks which could place a substantial financial burden upon us, should we be sued, if we do not have adequate liability insurance or general insurance coverage for such a claim.

Our business exposes us to potential product liability and other liability risks that are inherent in the testing, manufacturing and marketing of pharmaceutical formulations and products. In addition, the use in our clinical trials of pharmaceutical formulations and products that our potential collaborators may develop and the subsequent sale of these formulations or products by us or our potential collaborators may cause us to bear a portion or all of the product liability risks. As is common for companies sponsoring such clinical testing, we carry product liability insurance. The limit of this insurance may in some instances may be insufficient to offset a negative judgment or settlement payment. As a result, a successful liability claim or series of claims brought against us could have a material adverse effect on our business, financial condition and results of operations.

We are subject to regulations regarding the manufacturing of therapeutic proteins.

We are subject to ongoing periodic unannounced inspections by the FDA, corresponding state agencies or non-U.S. regulatory authorities to ensure strict compliance with current good manufacturing practice ("cGMP") and other government regulations and corresponding foreign standards. The cGMP requirements govern quality control and

documentation policies and procedures. Complying with cGMP and non-U.S. regulatory requirements will require that we expend time, money, and effort in production, recordkeeping, and quality control to assure that the product meets applicable specifications and other requirements. We must also pass a pre-approval inspection prior to regulatory approval. Failure to pass a pre-approval inspection might significantly delay FDA approval of our products. If we fail to comply with these requirements, we would be subject to possible regulatory action and might be limited in the jurisdictions in which we are permitted to sell our products. As a result, our business, financial condition, and results of operations might be materially harmed.

We currently manufacture the therapeutic protein products used in the production of sebelipase alfa; however, we have limited experience in manufacturing or procuring products in commercial quantities and our manufacturing system has never produced a product approved by regulatory authorities for commercial use. We may not be able to manufacture enough product to conduct clinical trials or for later commercialization at an acceptable cost or at all. We may not be able to produce or sufficiently test the comparability of drug materials derived from our manufacturing processes or the processes run by our third party manufacturing partners which could impact our ability or timing with respect to receiving regulatory approval for our product candidates.

Even if we receive regulatory approval of our rare disease product candidates, we may have to rely on third parties to manufacture the product and/or complete the manufacturing process, including to purify, finish and fill any product for commercial sale.

We may have to rely on third parties to complete the manufacturing process. Our anticipated future reliance on a limited number of third-party manufacturers exposes us to the following risks:

- We might be unable to identify manufacturers on acceptable terms or at all because the number of potential manufacturers is limited and the FDA and other regulatory authorities must approve any replacement contractor. This approval would generally require new testing and compliance inspections. In addition, a new manufacturer would have to be educated in, or develop substantially equivalent processes for, production of our products after receipt of FDA approval, if any.
- Our third-party manufacturers might be unable to formulate and manufacture the relevant drugs in the volume and of the quality required to meet our clinical and commercial needs, if any.
- Our third-party contract manufacturers might not perform as agreed or might not remain in the contract manufacturing business for the time required to supply possible clinical trials or to successfully produce, store and distribute our products.
- Drug manufacturers are subject to ongoing periodic unannounced inspections by the regulatory, corresponding state agencies and non-U.S. regulatory authorities to ensure strict compliance with cGMP, and other government regulations and corresponding foreign standards. We do not have control over third-party manufacturers' compliance with these regulations and standards.
- If any third-party manufacturer makes improvements in the manufacturing process for the relevant products, we might not own, or might have to share, the intellectual property rights to the innovation with our licensors.
- We might compete with other companies for access to these manufacturers' facilities and might be subject to manufacturing delays if the manufacturers give other clients higher priority than us.

Each of these risks could delay our clinical trials or the approval, if any, of our product candidates by the FDA or the commercialization of our product candidates and could result in higher costs or deprive us of potential product revenues. As a result, our business, financial condition, and results of operations might be materially harmed.

We face significant competition from other pharmaceutical and biotechnology companies. Our operating results will suffer if we fail to compete effectively.

The pharmaceutical and biotechnology industries are intensely competitive and subject to rapid and significant technological change. Our competitors include organizations such as major multinational pharmaceutical companies, established biotechnology companies and specialty pharmaceutical and generic drug companies. Many competitors

have greater financial and other resources than we have, such as larger research and development staff, more extensive marketing, distribution, sales and manufacturing organizations and experience, more extensive clinical trial and regulatory experience, expertise in prosecution of intellectual property rights and access to development resources like personnel and technology. As a result, these companies may obtain regulatory approval more rapidly than we are able to and may be more effective in selling and marketing their products.

Our operations are subject to the economic, political, legal and business conditions in the countries in which we do business, and our failure to operate successfully or adapt to changes in these conditions could cause our operations to be limited or disrupted.

We have expanded our operations outside of the United States and expect to continue to do so in the future. Our current operations in foreign countries subject us to certain risks that could cause our operations to be limited or disrupted, including volatility in international economies, political instability, difficulties enforcing contractual and intellectual property rights, changes in laws, regulations or enforcement practices with respect to our business, compliance with tax, employment and labor laws, costs and difficulties in recruiting and retaining qualified managers and employees to manage and operate the business in local jurisdictions and costs and difficulties in managing and monitoring international operations.

Our business depends on protecting our intellectual property.

We are pursuing intellectual property protection for sebelipase alfa and other product candidates in the form of patent applications that have been and will continue to be filed in the U.S. and in other countries; however, there can be no assurance that patents will issue with the scope for which they are originally filed, if at all.

If we and our licensors do not obtain protection for our respective intellectual property rights and our products are not, or are no longer, protected by regulatory exclusivity protection, such as orphan drug protection, our competitors might be able to take advantage of our research, development, and manufacturing efforts to develop and commercialize competing drugs.

Our success, competitive position, and future revenues, if any, depend in part on our ability and the abilities of our licensors to obtain and maintain patent protection for our products, methods, processes, and other technologies, to preserve our trade secrets, to prevent third parties from infringing on our proprietary rights, and to operate without infringing on the proprietary rights of third parties. We currently hold various issued patents and exclusive rights to issued patents and own and have licenses to various patent applications, in each case in the U.S. as well as rights under foreign patents and patent applications. We anticipate filing additional patent applications both in the U.S. and in other countries, as appropriate. However, the patent process is subject to numerous risks and uncertainties, and there can be no assurance that we will be successful in protecting our products by obtaining and defending patents. These risks and uncertainties include the following:

- our patent rights might be challenged, invalidated, or circumvented, or otherwise might not provide any competitive advantage;
- our competitors, many of which have substantially greater resources than we do and many of which might make significant investments in competing technologies, might seek, or might already have obtained, patents that will limit, interfere with, or eliminate our ability to make, use, and sell our potential products either in the U.S. or in international markets;
- as a matter of public policy regarding worldwide health concerns, there might be significant pressure on the U.S. government and other international governmental bodies to limit the scope of patent protection both inside and outside the U.S. for disease treatments that prove successful; and
- countries other than the U.S. might have less restrictive patent laws than the U.S., giving foreign competitors the ability to exploit these laws to create, develop, and market competing products.

In addition, the USPTO and patent offices in other jurisdictions have often required that patent applications concerning pharmaceutical and/or biotechnology-related inventions be limited or narrowed substantially to cover only the specific innovations exemplified in the patent application, thereby limiting the scope of protection against competitive challenges. Thus, even if we or our licensors are able to obtain patents, the patents might be substantially narrower than anticipated.

Patent and other intellectual property protection is crucial to the success of our business and prospects, and there is a risk that such protections will prove inadequate. Our business and prospects might be materially harmed if these protections prove insufficient.

On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to United States patent law, including provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. The USPTO has issued regulations and procedures to govern administration of the Leahy-Smith Act, but many of the substantive changes to patent law associated with the Leahy-Smith Act have only recently become effective and other changes will not become effective until early 2013. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

We rely on trade secret protections through confidentiality agreements with our employees and third parties, and the breach of these agreements could adversely affect our business and prospects.

We rely on trade secrets, which we seek to protect, in part, through confidentiality and non-disclosure agreements with our employees, collaborators, suppliers, and other parties. There can be no assurance that these agreements will not be breached, that we would have adequate remedies for any such breach, or that our trade secrets will not otherwise become known to or independently developed by our competitors. We might be involved from time to time in litigation to determine the enforceability, scope, and validity of our proprietary rights. Any such litigation could result in substantial cost and divert management's attention from operations. If any of these events occurs, or we otherwise lose protection for our trade secrets or proprietary know-how, the value of this information may be greatly reduced.

We are dependent on certain license relationships.

We have licensed technology that is related to our proprietary expression technology from the University of Georgia, University of Minnesota and Pangenix and we might enter into additional licenses in the future. Licenses to which we are a party contain, and we expect that any future licenses will contain, provisions requiring up-front, milestone, and royalty payments to licensors and other conditions to maintaining the license rights. If we fail to comply with our obligations under any such license, the applicable licensor may have the right to terminate the license on relatively short notice and as a result, we would not be able to commercialize drug candidates or technologies that were covered by the applicable license. Also, the milestone and other payments associated with these licenses will make it less profitable for us to develop our drug candidates.

We will be dependent on orphan drug status to commercialize sebelipase alfa, and a competitor may receive orphan drug marketing authorization prior to us for the same indication for which we are seeking approval.

We expect to rely heavily on the orphan drug exclusivity for sebelipase alfa, which grants seven years of marketing exclusivity under the Federal Food, Drug, and Cosmetic Act, and up to 10 years of marketing exclusivity in Europe. While the orphan drug exclusivity for sebelipase alfa will provide market exclusivity in the U.S., Europe, and other countries, we will not be able to exclude other companies from manufacturing and/or selling drugs using the same active ingredient for the same indication beyond that timeframe. Furthermore, the marketing exclusivity in Europe can be reduced from 10 years to six years if the initial designation criteria have significantly changed since the market authorization of the orphan drug. Even if we have orphan drug exclusivity for a particular drug indication, we cannot guarantee that another company also with orphan drug exclusivity will not receive marketing authorization for the same indication before we do. If that were to happen, our applications for that indication may not be approved until the competing company's period of exclusivity has expired. Even if we are the first to obtain marketing authorization for an orphan drug indication, there are circumstances under which a competing product may be approved for the same indication during the seven-year period of marketing exclusivity in the U.S., such as if the later product is shown to be clinically superior to our product, or if the later product is a different drug than sebelipase alfa. Further, the seven-year marketing exclusivity in the U.S. would not prevent competitors from obtaining approval of the same compound for other indications or the use of other types of drugs for the same use as the orphan drug.

If we are unable to retain and recruit qualified scientists and advisors, or if any of our key executives, key employees or key consultants discontinues his or her employment or consulting relationship with us, it may delay our development efforts or otherwise harm our business.

The loss of any of our key executives, employees or key consultants could impede the achievement of our research and development objectives. Furthermore, recruiting and retaining qualified scientific personnel to perform research and development work in the future is critical to our success. We may be unable to attract and retain personnel on acceptable terms given the competition among biotechnology, biopharmaceutical, and health care companies, universities, and non-profit research institutions for experienced scientists and other disciplines. Competition for employees may impact our ability to recruit and retain qualified personnel in the future. Certain of our officers, directors, scientific advisors, and/or consultants or certain of the officers, directors, scientific advisors, and/or consultants hereafter appointed may from time to time serve as officers, directors, scientific advisors, and/or consultants of other biopharmaceutical or biotechnology companies. We do not maintain "key man" insurance policies on any of our officers or employees. We currently have employment contracts with our Chief Executive Officer, Sanj K. Patel, and other executive officers which provide for certain severance benefits. Consistent with our current employment policies, all of our employees are employed "at will" and, therefore, each employee may leave our employment at any time. If we are unable to retain our existing employees, including qualified scientific personnel, and attract additional qualified candidates, our business and results of operations could be adversely affected. We are not aware of any key personnel who intend to retire or otherwise leave us in the near future.

We derive a significant portion of our income from royalties on sales of FUZEON. If FUZEON sales continue to decline, our business could suffer.

Royalties on sales of FUZEON are currently a significant source of revenue for us. FUZEON competes with numerous existing therapies for the treatment of HIV. From 2007 through 2012, overall FUZEON net sales reported by our commercialization partner Roche have declined, totaling $112.2 million, $88.4 million, $50.7 million and $41.3 million for fiscal 2009, 2010, 2011 and 2012, respectively. We cannot predict if or when sales levels for FUZEON will stabilize.

Uncertainties relating to third-party reimbursement and health care reform measures could limit payments or reimbursements for FUZEON, which could adversely affect our business.

Currently, because of the high cost of the treatment of HIV, many state legislatures are reassessing reimbursement policies for this therapy. If third-party payor reimbursements for FUZEON are limited or reduced, our results of operations will be adversely affected. In addition, emphasis in the U.S. on the reduction of the overall costs of health care through managed care has increased and will continue to increase the pressure to reduce the prices of pharmaceutical products.

The wholesale acquisition cost of a one-year supply of FUZEON in the U.S. is approximately $32,500. A high drug price could also negatively affect patients' ability to receive reimbursement coverage for FUZEON from third-party payors, such as private or government insurance programs. If Roche is unable to obtain and maintain reimbursement from a significant number of third-party payors, it would have a material adverse effect on our business, financial condition and results of operations.

Currently, FUZEON is covered by Medicaid in all 50 states in the U.S. In addition, the AIDS Drug Assistance Programs in all 50 states and a majority of private insurers provide some amount of access to FUZEON. However, there are reimbursement challenges remaining. Some of the payors require patients to meet minimum medical requirements, such as maintaining certain cell levels associated with HIV, to receive reimbursement. Other payors limit the number of patients to which they will provide reimbursement for FUZEON, and other payors may require co-payments by the patient in order to receive reimbursement for FUZEON that are significantly higher than those required for other anti-HIV drugs.

Several major pharmaceutical companies have offered to sell their anti-HIV drugs at or below cost to certain countries in Africa and Least Developed Countries (as defined by the United Nations), which could adversely affect the reimbursement climate of, and the prices that may be charged for, HIV medications in the U.S. and the rest of the

world. Third-party payors could exert pressure for price reductions in the U.S. and the rest of the world based on these lower costs offered in Africa and Least Developed Countries. This price pressure could limit the price that Roche would be able to charge for FUZEON, thereby adversely affecting our results of operations

If the sale of FUZEON infringes the proprietary rights of third parties, we may need to obtain licenses, pay damages or defend litigation.

If the sale of FUZEON infringes the proprietary rights of third parties, we could incur substantial costs and may have to:

- obtain licenses, which might not be available on commercially reasonable terms, if at all;
- pay damages; and/or
- defend litigation or administrative proceedings which might be costly whether we win or lose, and which could result in a substantial diversion of financial and management resources.

Any of these events could substantially harm our earnings, financial condition, and operations.

On November 20, 2007, Novartis filed a lawsuit against us and Roche and certain of its affiliated entities, alleging infringement of the '271 Patent, related to the manufacture, sale and offer for sale of FUZEON. On September 23, 2010, we entered into a settlement agreement (the "Settlement Agreement") with Roche and Novartis settling the lawsuit and the lawsuit was dismissed with prejudice from the Eastern District of North Carolina on September 28, 2010. Under the terms of the Settlement Agreement, we, in collaboration with Roche, have the right to continue to sell FUZEON under a license to Novartis' '271 Patent in exchange for the payment of royalties to Novartis on net sales of FUZEON. We will share responsibility for payment of these royalties equally with Roche.

We may pursue rapid expansion of our workforce or diversify our business strategy through mergers, acquisitions, licensing arrangement or other contractual arrangements with third parties which may require substantial resources and substantial amounts of time from members of our senior management and involve numerous risks.

We may spend substantial resources to hire additional employees or pursue acquisitions of new technologies or businesses that are complementary to our current technologies or business focus through mergers, acquisitions, licensing arrangement or other contractual arrangements with third parties. Acquisitions involve numerous risks, including potential difficulties in the integration of acquired operations such as retaining key employees of the acquired business, integrating research and development programs, not meeting financial objectives, increased costs, undisclosed liabilities not covered by insurance or terms of acquisition, and diversion of management's attention and resources in connection with an acquisition. No assurance can be given as to our success in identifying, executing, and integrating acquisitions in the future.

Our success will depend in part on relationships with third parties. Any adverse changes in these relationships could adversely affect our business, financial condition, or results of operations.

Our success will be dependent on our ability to maintain and renew business relationships with third parties and to establish new business relationships. There can be no assurance that our management will be able to maintain such business relationships, or enter into or maintain new business contracts and other business relationships, on acceptable terms, if at all. The failure to maintain important business relationships could have a material adverse effect on our business, financial condition, or results of operations.

Our charter documents and indemnification agreements require us to indemnify our directors and officers to the fullest extent permitted by law, which may obligate us to make substantial payments and to incur significant insurance-related expenses.

Our charter documents require us to indemnify our directors and officers to the fullest extent permitted by law. This could require us, with some legally prescribed exceptions, to indemnify our directors and officers against any and all expenses, judgments, penalties, fines, and amounts reasonably paid in defense or settlement of an action, suit, or

proceeding brought against any of them by reason of the fact that he or she is or was our director or officer. In addition, expenses incurred by a director or officer in defending any such action, suit, or proceeding must be paid by us in advance of the final disposition of that action, suit or proceeding if we receive an undertaking by the director or officer to repay us if it is ultimately determined that he or she is not entitled to be indemnified. We have also entered into indemnification agreements with each of our directors and officers. In furtherance of these indemnification obligations, we maintain directors' and officers' insurance in the amount of $30,000,000. For future renewals, if we are able to retain coverage, we may be required to pay a higher premium for our directors' and officers' insurance than in the past and/or the amount of its insurance coverage may be decreased.

Risks Relating Our Financial Position and Capital Requirements

We may be unable to raise the substantial additional capital that we will need to further develop and commercialize our products.

As is typical of biotechnology companies at our stage of development, our operations consume substantial amounts of cash and we will need substantial additional funds to further develop and commercialize our products.

While we will need to seek additional funding, we may not be able to obtain financing on acceptable terms, or at all. In addition, the terms of our financings may be dilutive to, or otherwise adversely affect, holders of our common stock. We may also seek additional funds through arrangements with collaborators or other third parties. These arrangements would generally require us to relinquish rights to some of our technologies, product candidates or products, and we may not be able to enter into such agreements, on acceptable terms, if at all. If we are unable to obtain additional funding on a timely basis, we may be required to curtail or terminate some or all of our development programs, including some or all of our product candidates.

We have incurred significant losses since our inception and anticipate that we will continue to incur losses for the foreseeable future. We are a clinical-stage company with limited historical revenues, which makes it difficult to assess our future viability.

We are a clinical-stage biopharmaceutical company. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. We expect our expenses to increase in connection with our efforts to seek approval for and commercialize sebelipase alfa and our research and development of our other product candidates, including but not limited to, SBC-103. As a result, we expect to continue to incur significant research and development and other expenses related to our ongoing operations for the foreseeable future. If any of our product candidates fail in clinical trials or do not gain regulatory approval, or if any of our product candidates, if approved, fail to achieve market acceptance, we may never become profitable. Even if we achieve profitability in the future, we may not be able to sustain profitability in subsequent periods. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' equity and working capital.

Our ability to utilize Trimeris' net operating loss and tax credit carryforwards in the future is subject to substantial limitations and may be further limited as a result of the Reverse Merger.

Federal and state income tax laws impose restrictions on the utilization of net operating loss ("NOL") and tax credit carryforwards in the event that an "ownership change" occurs for tax purposes, as defined by Section 382 of the Internal Revenue Code of 1986, as amended (the "Code"). In general, an ownership change occurs when stockholders owning 5% or more of a "loss corporation" (a corporation entitled to use NOL or other loss carryforwards) have increased their aggregate ownership of stock in such corporation by more than 50 percentage points during any three-year period. If an "ownership change" occurs, Section 382 of the Code imposes an annual limitation on the amount of post-ownership change taxable income that may be offset with pre-ownership change NOLs of the loss corporation experiencing the ownership change. The annual limitation is calculated by multiplying the loss corporation's value immediately before the ownership change by the greater of the long-term tax-exempt rate determined by the IRS in the month of the ownership change or the two preceding months. This annual limitation may be adjusted to reflect any unused annual limitation for prior years and certain recognized built-in gains and losses for the year. Section 383 of the Code also imposes a limitation on the amount of tax liability in any post-ownership change year that can be reduced by the loss corporation's pre-ownership change tax credit carryforwards.

On November 2, 2011, we completed the Reverse Merger which resulted in an "ownership change" of Trimeris. Trimeris previously experienced an "ownership change" in 2008. Accordingly, our ability to utilize Trimeris' NOL and tax credit carryforwards may be substantially limited. These limitations could, in turn, result in increased future tax payments for us, which could have a material adverse effect on our business, financial condition.

Our management is required to devote substantial time to comply with public company regulations.

As a public company, we incur significant legal, accounting and other expenses. The Sarbanes-Oxley Act of 2002 ("Sarbanes-Oxley"), as well as rules implemented by the SEC and the NASDAQ Global Select Market, impose various requirements on public companies, including those related to corporate governance practices. Our management and other personnel will need to devote substantial time to these requirements. Certain members of our management do not have experience in addressing these requirements.

Sarbanes-Oxley requires, among other things, that we maintain effective internal controls for financial reporting and disclosure controls and procedures. In particular, we will be required to perform system and process evaluation and testing of our internal controls over financial reporting to allow management and our independent registered public accounting firm to report on the effectiveness of our internal controls over financial reporting, as required by Section 404 of Sarbanes-Oxley ("Section 404"). We will incur substantial accounting and related expenses to comply with Section 404. We may need to hire additional accounting and financial staff to satisfy the ongoing requirements of Section 404. Moreover, if we are not able to comply with the requirements of Section 404, or if we or our independent registered public accounting firm identifies deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses, the market price of our stock could decline and we could be subject to sanctions or investigations by the NASDAQ Global Select Market, the SEC, or other regulatory authorities.

We rely on Roche to timely deliver important financial information relating to sales of FUZEON. In the event that this information is inaccurate, incomplete, or not timely, we will not be able to meet our financial reporting obligations as required by the SEC.

Under the Roche License Agreement, Roche has exclusive control over the flow of information relating to sales of FUZEON that we require to meet our SEC reporting obligations. Roche is required under the Roche License Agreement to provide us with timely and accurate financial data related to sales of FUZEON so that we may meet our reporting requirements under federal securities laws. In the event that Roche fails to provide us with timely and accurate information, we may incur significant liability with respect to the federal securities laws, our disclosure controls and procedures under Sarbanes-Oxley may be inadequate, and we may be forced to restate our financial statements, any of which could adversely affect the market price of our common stock.

Changes in the estimated performance periods of our collaboration arrangements may negatively impact current period revenue.

We account for our collaboration arrangements under the proportional performance method, whereby the amount of revenue recognized in the current period is based on our performance compared to the total estimate to complete the project. If our estimates to complete change in future periods, our collaboration revenue may be reduced and could potentially be negative.

Risks Related to Ownership of Our Common Stock

The market price and trading volume of our common stock may be volatile.

The market price of our common stock could fluctuate significantly for many reasons, including the following factors:

- announcements of clinical or regulatory developments or technological innovations by us or our competitors,
- changes in our relationship with our licensors and other strategic partners,
- our quarterly operating results,

- declines in sales of FUZEON,
- developments in patent or other technology ownership rights,
- public concern regarding the safety of our products,
- additional funds may not be available on terms that are favorable to us and, in the case of equity financings, may result in dilution to our stock holders,
- government regulation of drug pricing, and
- general changes in the economy, the financial markets or the pharmaceutical or biotechnology industries.

Additional factors beyond our control may also have an impact on the price of our stock. For example, to the extent that other large companies within our industry experience declines in their stock price, our stock price may decline as well. In addition, when the market price of a company's common stock drops significantly, stockholders often institute securities class action lawsuits against the company. A lawsuit against us could cause us to incur substantial costs and could divert the time and attention of our management and other resources.

Future sales of substantial amounts of our common stock, or the perception that such sales could occur, could adversely affect the market price of our common stock.

Future sales into the public market of substantial amounts of our common stock, or securities convertible or exchangeable into shares of our common stock, including shares of our common stock issued upon exercise of options and warrants, or perceptions that such sales could occur, could adversely affect the market price of our common stock and our ability to raise capital in the future.

Ownership of our common stock is highly concentrated, and it may prevent you and other stockholders from influencing significant corporate decisions and may result in conflicts of interest that could cause our stock price to decline.

Our executive officers and directors, together with their respective affiliates, beneficially own or control approximately 40.0% of our issued and outstanding common stock. Accordingly, these executive officers, directors and their affiliates, acting individually or as a group, have substantial influence over the outcome of a corporate action requiring stockholder approval, including the election of directors, any merger, consolidation or sale of all or substantially all of our assets or any other significant corporate transaction. These stockholders may also exert influence in delaying or preventing a change in control, even if such change in control would benefit our other stockholders. In addition, the significant concentration of stock ownership may adversely affect the market value of our common stock due to investors' perception that conflicts of interest may exist or arise.

Anti-takeover provisions in our charter and bylaws may prevent or frustrate attempts by stockholders to change the board of directors or current management and could make a third-party acquisition of us difficult.

Our certificate of incorporation and bylaws contain provisions that may discourage, delay or prevent a merger, acquisition or other change in control that stockholders may consider favorable, including transactions in which stockholders might otherwise receive a premium for their shares. These provisions could limit the price that investors might be willing to pay in the future for shares of our common stock.

We have never declared or paid dividends on our common stock and do not anticipate paying dividends in the foreseeable future.

Our business requires significant funding, and we do not anticipate paying any cash dividends on our common stock in the foreseeable future.

ITEM 1B. UNRESOLVED STAFF COMMENTS

None.

ITEM 2. PROPERTY AND FACILITIES

Our corporate headquarters and research laboratories are located in Lexington, Massachusetts, where we lease and occupy approximately 19,000 square feet of office and laboratory space. The lease expires on August 31, 2013. On January 15, 2013, we entered into a new lease agreement under which we will lease approximately 52,000 square feet of office, research and laboratory space in Lexington, Massachusetts. This will be our corporate headquarters after we move into the facility. We will occupy the facility in stages as building modifications are completed. We currently anticipate that we will begin occupying a portion of the facility in May 2013. The initial term of the lease is 77-months after we begin occupying the entire facility, which is currently expected in August 2013. We have an option to extend the lease for two separate but successive periods of three years each.

We have technical and manufacturing operations located in and around Athens, Georgia, where we lease and occupy approximately 64,000 square feet of office, research manufacturing and laboratory space in four different buildings pursuant to three leases, and in central Massachusetts where we lease 39,000 square feet of research manufacturing and laboratory space. Our leases in Athens, Georgia, expire on June 2013, with a rental option of up to an additional five years, and February 2017. Our lease in central Massachusetts expires in April 2021with two separate but successive option periods of seven years each. We believe that our facilities are suitable and adequate for our current needs.

ITEM 3. LEGAL PROCEEDINGS

Although no infringement, misappropriation, or similar claim or action is pending or threatened, we are aware of a patent issued by the European Patent Office, and subsequently validated in Great Britain, France, Germany, Italy and Spain, that relates generally to the use of LAL and may therefore be relevant to sebelipase alfa. On November 23, 2012, at an initial opposition hearing at the European Patent Office, a three member panel upheld claims that relate generally to LAL for the treatment of LAL Deficiency. This decision is subject to appeal. We disagree with the ruling at the initial hearing, and continue to believe that the patent claims are invalid due to a substantial body of prior art. Our lead development program currently evaluates our LAL product candidate, sebelipase alfa, as a treatment for patients with early onset and late onset LAL Deficiency and therefore the upheld patent claims may be relevant to sebelipase alfa. We continue to believe that such patent does not currently affect our freedom to conduct clinical trials for sebelipase alfa. We also believe that the patent is invalid due to a substantial body of prior art. We have recently taken additional proactive steps, including instituting proceedings in the UK Patents Court and a French court seeking a declaration that the issued patent is invalid and that the current anticipated activities related to our development and commercialization of sebelipase alfa do not infringe the patent. We also may take further steps to accelerate resolution of this matter, which further steps may include contacting the patentee, bringing additional prior art documents to the attention of the European Patent Office or other actions. The UK Patents Court has scheduled a trial for May 2013. If the patent is ultimately maintained in unamended form by the European Patent Office, then our ability to commercialize sebelipase alfa in the countries where the patent is validated could be adversely affected and/or halted until the patent expires, unless the patent is invalidated by the relevant national courts prior to expiration. In addition, the same patentee has been granted a U.S. patent on the use of LAL to treat atherosclerosis, and has two pending patent applications in the U.S. related to LAL, whose claims have currently been rejected by the U.S. patent office. Equivalent patent applications are also pending in Canada and Brazil. We believe that we will not be infringing this issued U.S. patent on atherosclerosis in commercializing sebelipase alfa for the indications for which we are seeking approval and that the issued U.S. patent is invalid due to the prior art described above. We further believe that, due to the substantial body of prior art, the pending applications will not grant with claims that would encompass our contemplated activities or, if they did, they would be invalid due to the prior art. If, however, these pending applications are granted in a form which covers sebelipase alfa, then our ability to commercialize sebelipase alfa in these countries and the U.S. could be adversely affected and/or halted until the patent expires in these countries, unless a license is obtained to the patents or the patents are otherwise revoked.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

PART II

ITEM 5. MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES.

Information About Our Common Stock

Our common stock trades on the Nasdaq Global Select Market under the symbol "GEVA." Set forth below are the high and low sales prices for our common stock for each full quarterly period within the most two recent fiscal years.

	High	Low
Year Ended December 31, 2012		
First Quarter	$39.33	$24.50
Second Quarter	$42.38	$32.70
Third Quarter	$56.88	$39.89
Fourth Quarter	$58.00	$41.49
Year Ended December 31, 2011		
First Quarter	$13.15	$11.80
Second Quarter	$14.80	$11.41
Third Quarter	$13.25	$ 8.30
Fourth Quarter[1]	$27.42	$10.04

(1) Prior to completing the Reverse Merger with Private Synageva on November 2, 2011, our stock traded under the symbol "TRMS" on the Nasdaq Global Market.

The closing price as of March 1, 2013 was $51.70.

Holders

The number of record holders of our common stock as of March 1, 2013 was 87.

Dividend Policy

We have never declared or paid any cash dividends on our common stock. We currently intend to retain earnings, if any, to support our business strategy and do not anticipate paying cash dividends in the foreseeable future. Payment of future dividends, if any, will be at the sole discretion of our board of directors after taking into account various factors, including our financial condition, operating results, capital requirements and any plans for expansion.

Repurchases

We did not repurchase any shares of our equity securities during the year ended December 31, 2012.

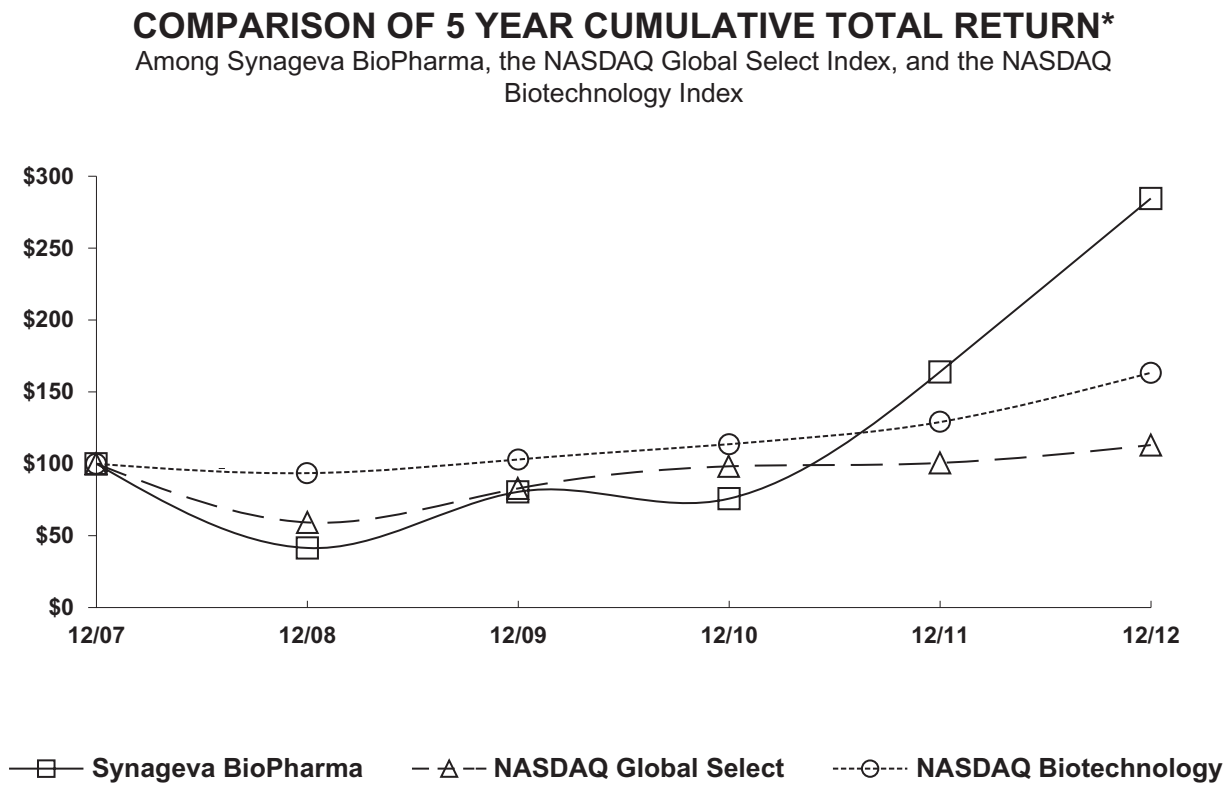
Securities Authorized for Issuance Under Equity Compensation Plans

The following table sets forth information about our equity compensation plans as of December 31, 2012, including the number of shares of our common stock issuable upon exercise of all outstanding options, the weighted-average exercise price of all outstanding options and the number of shares available for future issuance under our equity compensation plans (share amounts in thousands).

Plan Category	(a) Number of securities to be issued upon exercise of outstanding options, warrants and rights	(b) Weighted-average exercise price of outstanding options, warrants and rights	(c) Number of securities remaining available for future issuance under equity compensation plans (excluding securities reflected in column (a))
Equity Compensation Plans Approved by Stockholders	2,529	$25.06	471
Equity Compensation Plans Not Approved by Stockholders	—	—	—

Synageva's Stock Performance

The following graph compares cumulative total return of our Common Stock with the cumulative total return of (i) the NASDAQ Global Select Index, and (ii) the NASDAQ Biotechnology Index. The graph assumes (a) $100 was invested on December 31, 2007 in each of our Common Stock, the stocks comprising the NASDAQ Global Select Index and the stocks comprising the NASDAQ Biotechnology Index, and (b) the reinvestment of dividends. The comparisons shown in the graph are based on historical data and the stock price performance shown in the graph is not necessarily indicative of, or intended to forecast, future performance of our stock. Prior to the Reverse Merger on November 2, 2011, the stock of Pre-Merger Trimeris traded under the symbol "TRMS" on the Nasdaq Global Market and any comparison with any comparison with Pre-Merger Trimeris' historical stock prices may not be meaningful.



Cumulative Total Return

	12/07	12/08	12/09	12/10	12/11	12/12
Synageva BioPharma Corp.	100.00	41.21	80.57	75.65	163.79	284.70
NASDAQ Global Select	100.00	59.35	82.89	98.17	100.47	113.11
NASDAQ Biotechnology	100.00	93.40	103.19	113.89	129.12	163.33

ITEM 6. SELECTED FINANCIAL DATA.

The following Selected Financial Data should be read in conjunction with "Management's Discussion and Analysis of Financial Condition and Results of Operations" included in Item 7 beginning on page 35 and the consolidated financial statements and related notes thereto beginning on page 51 of this Annual Report on Form 10-K.

Basis of Presentation (2011 and future periods)

The Reverse Merger was accounted for as a reverse acquisition in fiscal 2011. As such, the financial statements of Private Synageva are treated as our historical financial statements, with the results of the combined company being included from November 2, 2011. Immediately following the closing of the Reverse Merger, Private Synageva designees to our Board of Directors represented a majority of our directors, Private Synageva's senior management represented our entire senior management and the operations formerly conducted by Private Synageva were our only continuing development efforts. For periods prior to the closing of the Reverse Merger, therefore, our discussion below relates to the historical business and operations of Private Synageva. Certain portions of this Annual Report on Form 10-K may contain information that relates to Pre-Merger Trimeris' previous operations, which are no longer material to our business. Any comparison of Pre-Merger Trimeris' revenues and operations with ours may not be helpful to an understanding of our results for the year ended December 31, 2011 or future periods.

	Years Ended December 31,				
	2012	2011	2010	2009	2008
	(in thousands, except per share amounts)				
Consolidated Statement of Operations Data:					
Revenues:					
Royalty revenue	$ 7,023	$ 1,083	$ —	$ —	$ —
Collaboration and license revenue	7,875	640	280	80	—
Other revenue	56	376	315	82	64
Total revenue	14,954	2,099	595	162	64
Costs and expenses:					
Research and development	37,347	17,346	9,866	6,583	17,309
General and administrative	17,396	9,268	3,852	3,843	4,073
Amortization of developed technology	3,232	504	—	—	—
Total costs and expenses	57,975	27,118	13,718	10,426	21,382
Loss from operations	(43,021)	(25,019)	(13,123)	(10,264)	(21,318)
Other (expense) income, net	—	(259)	2,295	82	—
Interest income (expense), net	72	(28)	4	(1,238)	(519)
Net loss	$(42,949)	$(25,306)	$(10,824)	$(11,420)	$ (21,837)
Basic and diluted net income per share(1)	$ (1.90)	$ (8.58)	$(338.25)	$(516.13)	$(1,180.44)
Weighted average shares used in basic and diluted per share computations(1)	22,579	2,950	32	22	18

(1) Per share computations for fiscal 2011 are based on (i) Private Synageva's historic common stock balances (excluding preferred stock) up to the Merger date and (ii) post-Merger common stock from the Merger date to year end. For fiscal 2010 through 2007, per share computations are based on Private Synageva's historic common stock balances, which exclude preferred stock.

	As of December 31,				
	2012	2011	2010	2009	2008
	(in thousands)				
Consolidated Balance Sheet Data:					
Cash, cash equivalents, and short-term investments	$ 218,953	$ 60,232	$ 14,715	$ 25,851	$ 2,693
Working capital	212,028	56,393	14,285	25,135	(3,733)
Total assets	243,256	83,298	16,982	27,019	4,101
Long-term debt	—	—	—	—	4,979
Total convertible preferred stock	—	—	95,581	95,556	59,040
Accumulated deficit	(158,789)	(115,840)	(90,534)	(79,710)	(68,290)
Total convertible preferred stock and stockholders' equity (deficit)	230,177	74,048	15,403	26,023	(7,545)

ITEM 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS.

This discussion of our financial condition and results of operations should be read together with the consolidated financial statements and notes contained elsewhere in this report. Certain statements in this section are forward-looking. While we believe these statements are accurate, our business is dependent on many factors, some of which are discussed in the sections entitled "Risk Factors" and "Business". Many of these factors are beyond our control and any of these and other factors could cause actual results to differ materially from the forward-looking statements made in this report. We undertake no obligation to release publicly the results of any revisions to the statements contained in this report to reflect events or circumstances that occur subsequent to the date of this report.

Overview

We are 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 needs. Our management team is experienced in the development and commercialization of drugs for diseases with small patient populations, including clinical and translational research, working with payors to establish reimbursement, and designing and building commercial organizations to reach highly specialized physicians to facilitate patient identification. We have several protein therapeutics in our pipeline, including two enzyme replacement therapies for lysosomal storage disorders and additional programs for life-threatening genetic conditions for which there are currently no approved treatments. Our lead program, sebelipase alfa, is a recombinant human lysosomal acid lipase ("LAL") currently under global clinical investigation for the treatment of patients with early and late onset LAL deficiency ("LAL Deficiency"), which is a rare, devastating genetic disease that causes significant morbidity and mortality. We currently evaluate sebelipase alfa in global clinical trials and sebelipase alfa has been granted orphan designations by the U.S. Food and Drug Administration ("FDA"), the European Medicines Agency, and the Japanese Ministry of Health, Labour and Welfare. Additionally, sebelipase alfa received *Fast Track D*esignation by the FDA. We have not yet received approval to market this product and we are not currently commercializing any other products.

Basis of Presentation (Fiscal 2011 and prior periods)

As described in Item 6, on November 2, 2011, Synageva BioPharma Corp., a privately held Delaware corporation ("Private Synageva") closed the a merger transaction (the "Reverse Merger") with Trimeris, Inc. ("Trimeris") As of November 2, 2011, after giving effect to the Reverse Merger the former stockholders of Private Synageva collectively owned approximately 75% and the stockholders of Trimeris prior to the Reverse Merger ("Pre-Merger Trimeris") owned approximately 25% of our outstanding common stock.

The Reverse Merger was accounted for as a reverse acquisition. As such, the financial statements of Private Synageva are treated as our historical financial statements, with the results of Trimeris being included from November 2, 2011. Immediately following the closing of the Reverse Merger, Private Synageva designees to our Board of Directors represented a majority of our directors, Private Synageva's senior management represented our entire senior management and the operations formerly conducted by Private Synageva were our only continuing development efforts. For periods prior to the closing of the Reverse Merger, therefore, our discussion below relates to the historical business and operations of Private Synageva. Certain portions of this Annual Report on Form 10-K may contain information that relates to Pre-Merger Trimeris' previous operations, which are no longer material to our business. Any comparison of Pre-Merger Trimeris' revenues and operations with ours may not be helpful to an understanding of our results for the years ended December 31, 2011, 2012 or future periods.

Financial Operations Overview

General

Our future operating results will depend on the progress of drug candidates currently in our research and development pipeline. The results of our operations will vary significantly from year to year and quarter to quarter and will depend largely on, among other factors, the cost and outcome of any preclinical development or clinical trials then being conducted.

A discussion of certain risks and uncertainties that could affect our liquidity, capital requirements and ability to raise additional funds is set forth under the section entitled "Risk Factors" in this report.

Revenue

Royalty Revenue

Royalty revenue relates to amounts earned from the sale of FUZEON by F. Hoffman-La Roche Ltd. ("Roche"). The FUZEON royalty stream was acquired in the Reverse Merger in the fourth quarter of fiscal 2011.

Collaboration and License Revenue

Collaboration and license revenue primarily relates to our collaboration agreements with Mitsubishi Tanabe Pharma Corporation ("Mitsubishi Tanabe") whereby we utilize our proprietary expression technology in two development programs, in exchange for upfront license payments, funded development, and the potential for additional payments upon the successful completion of the development programs. Under the first program, which was entered into in August 2011, we received an upfront license payment of $3.0 million, on-going funding of development costs, and the potential for an additional payment of $3.0 million due upon the successful completion of the initial development. Additionally, we entered into a second agreement in March 2012, where we received an upfront license payment of $9.0 million, on-going funding of development costs, and the potential for an additional payment of $3.0 million due upon the successful completion of the initial development stage of the second program. Under both agreements, Mitsubishi Tanabe has an option to obtain an exclusive royalty-bearing license, with the right to grant sublicenses, to further develop and commercialize the licensed compound. If Mitsubishi Tanabe exercises its option, the parties intend to negotiate a follow-on collaboration and license agreement that may include potential future development and commercial sales based milestone payments, and potential royalty payments.

Other Revenue

Other revenues relate to a National Institutes of Health ("NIH") grant, which was completed in the first quarter of fiscal 2012.

Research and Development

We expense research and development costs as incurred. Research and development expense consists of costs incurred to discover, research and develop drug candidates, including personnel and facility-related expenses, outside contracted services including clinical trial costs, manufacturing and process development costs, research costs, outside consulting services and other external costs. Research and development expense includes any costs associated with generating collaboration or grant revenue.

General and Administrative

General and administrative expense consists primarily of salaries, stock-based compensation expense and other related costs for personnel in executive, business development, commercial, finance, human resource, legal, information technology, and support personnel functions. We also expense patent costs and expenses associated with maintaining our intellectual property as incurred. Other costs include facility costs not otherwise included in research and development expense, insurance, and professional fees for legal, accounting and commercial services.

Amortization of Developed Technology

We provide for the cost of amortization of developed technology, computed using an accelerated method based on the undiscounted cash flows received from the FUZEON royalty stream, in proportion to the estimated total undiscounted cash flows.

Interest Income (expense), net

Interest income relates to interest earned on our cash equivalent and short-term investment balances.

Results of Operations

Years Ended December 31, 2012 and 2011

Revenues

The following table presents total revenue for the years ended December 31, 2012 and 2011, respectively (in thousands):

	Year Ended December,			
	2012	2011	$ Change	% Change
Royalty revenue	$ 7,023	$1,083	$ 5,940	548%
Collaboration and license revenue	7,875	640	7,235	1,131
Other revenue	56	376	(320)	(85)
Total revenue	$14,954	$2,099	$12,855	612%

Total revenues increased by approximately $12.9 million for the year ended December 31, 2012, as compared to the comparable period of the prior year. The increase in revenues was primarily the result of higher FUZEON royalty revenue and higher collaboration revenue.

Royalty revenues of $7.0 million relate to the royalty payment earned from Roche, based on total worldwide net sales of FUZEON. We did not have any royalty revenue from FUZEON until after the Reverse Merger, which occurred in the fourth quarter of fiscal 2011. Collaboration and license revenue for the year ended December 31, 2012 relates to revenue recognized from development programs with Mitsubishi Tanabe. For the year ended December 31, 2012, we recognized $1.9 million and $6.0 million related to the first and second Mitsubishi Tanabe programs, respectively. For the year ended December 31, 2011, collaboration and license revenue totaled $0.6 million primarily related to revenue recognized related to the first Mitsubishi Tanabe program. Other revenues relate to an NIH Grant, and totaled approximately $0.1 million and $0.4 million for the years ended December 31, 2012 and 2011, respectively.

We expect FUZEON royalty revenues to decrease over time, from the levels experienced in fiscal 2012. Royalty revenue increased in 2012 as compared to 2011, due to the inclusion of a full year of FUZEON royalty revenue in our results of operations. Additionally, as of December 31, 2012, our deferred revenue balance related to both of the Mitsubishi Tanabe collaboration agreements, totaled $5.4 million. We expect to recognize both the upfront development payments, which are included in deferred revenue, and the additional funded development payments related to both arrangements over the next year, in proportion to our performance under the arrangements.

Research and Development Expenses

Research and development expenses for the years ended December 31, 2012 and 2011 are summarized as follows (in thousands):

	Year Ended December 31,			
	2012	2011	$ Change	% Change
Compensation and benefits-related	$10,339	$ 5,947	$ 4,392	74%
Clinical trials and manufacturing	17,383	6,126	11,257	184
Other development related external services	4,384	2,992	1,392	47
Facilities and related	3,465	2,050	1,415	69
Stock-based compensation expense	1,432	231	1,201	520
Acquired in-process research and development	344	—	344	—
Total research and development expense	$37,347	$17,346	$20,001	115%

Research and development expense increased by approximately $20.0 million, or 115%, to $37.3 million for the year ended December 31, 2012 as compared to $17.3 million for the comparable prior year period. The increase in total research and development expense is due to increased clinical trial costs and manufacturing fees, as well as other

development related external services, associated with on-going development of sebelipase alfa and our pipeline programs and higher compensation expense from hiring additional staff to move the sebelipase alfa and pipeline programs forward. Additionally, in fiscal 2012, research and development expense includes $1.4 million of stock-based compensation expense and $0.3 million related to acquired in-process research and development. We expect research and development expense to continue to increase as development activities for sebelipase alfa and our other pipeline programs continue.

General and Administrative Expenses

General and administrative expenses for the year ended December 31, 2012 and 2011 are summarized as follows (in thousands):

	Year Ended December 31,			
	2012	2011	$ Change	% Change
Compensation and benefits-related	$ 7,537	$4,187	$3,350	80%
External and professional services	5,545	4,167	1,378	33
Facilities related and other	775	512	263	51
Stock-based compensation expense	3,539	402	3,137	780
Total general and administrative expense	$17,396	$9,268	$8,128	88%

General and administrative expense increased by approximately $8.1 million, or 88%, to $17.4 million for the year ended December 31, 2012 as compared to $9.3 million for the comparable prior year period. The increase was primarily due to higher compensation-related expenses of $3.4 million and increased stock-based compensation expense of $3.1 million, resulting from hiring additional staff to support expanded operations and the increasing financial and legal costs associated with public company requirements and commercial preparations. Additionally, external fees and professional service costs increased $1.4 million period over period, primarily a result of higher commercial related spending, higher public company costs, including insurance expense and higher professional service fees. We expect general and administrative expense to continue to increase in the future as our business grows.

Amortization of Developed Technology

Costs recognized for the amortization of developed technology for the year ended December 31, 2012 and 2011 are summarized as follows (in thousands):

	Year Ended December 31,			
	2012	2011	$ Change	% Change
Amortization of developed technology	3,232	504	2,728	541%

In fiscal 2012, we recognized $3.2 million of amortization related to the developed technology acquired in the Reverse Merger, compared to $0.5 million in the prior year. The increase was due to the inclusion of a full year of FUZEON royalty revenue, and therefore, the related amortization, in our results of operations.

Other (Expense) Income, Net

In 2011, we recognized $0.3 million of other expense associated with the revaluation of preferred stock warrants that were exercised in fiscal 2011. All preferred stock warrants have been exercised. We did not have any activity in other expense (income) in fiscal 2012.

Years Ended December 31, 2011 and 2010

Revenues

Total revenues increased by approximately $1.5 million, or 252%, to $2.1 million, for the year ended December 31, 2011, as compared to $0.6 million for the year ended December 31, 2010. The increase in revenues was

the result of the addition of FUZEON royalty revenue following the completion of the Reverse Merger, higher collaboration revenues and higher grant revenues as compared to the prior year. Royalty revenues of $1.1 million represent the royalty payment earned from Roche based on total worldwide net sales of FUZEON since the closing of the Reverse Merger in November 2011. Collaboration and license revenue totaled approximately $0.6 million and $0.3 million for the years ended December 31, 2011 and 2010, respectively. Grant revenues relate to an NIH Grant, and totaled approximately $0.4 million and $0.3 million for the years ended December 31, 2011 and 2010, respectively.

Research and Development Expenses

Research and development expenses are summarized as follows:

	Year Ended December 31,			
	2011	2010	$ Change	% Change
Compensation and benefits-related	$ 5,947	$3,674	$2,273	62%
Clinical trials and manufacturing	6,126	1,378	4,748	345
Other development related external services	2,992	3,286	(294)	(9)
Facilities related	2,050	1,483	567	38
Non-cash stock-based compensation	231	45	186	413
Total research and development expense	$17,346	$9,866	$7,480	76%

Research and development expenses increased by approximately $7.5 million, or 76%, to $17.3 million for the year ended December 31, 2011 as compared to $9.9 million for the year ended December 31, 2010. The increase is primarily attributable to increased spending related to our lead program sebelipase alfa. The increase included clinical trial costs and manufacturing fees associated with our on-going sebelipase alfa development as well as higher compensation expense from the hiring of additional staff to move the sebelipase alfa program forward. As part of these activities, we also incurred additional facilities expense resulting from the opening of our corporate headquarters and laboratory facilities in Lexington, Massachusetts in September 2010. Increases were partially off-set by lower pre-clinical expenses for 2011 as compared to the prior year.

General and Administrative Expenses

General and administrative expenses increased by approximately $5.4 million to $9.3 million for the year ended December 31, 2011 as compared to $3.9 million for the year ended December 31, 2010. This increase was primarily due to higher external services costs of $3.3 million, including legal and accounting fees as well as commercial activities, and higher compensation expense of $2.0 million, resulting from hiring additional staff to support public company requirements as well as commercial preparations. In 2011, we incurred approximately $1.1 million in transaction related fees related to the Reverse Merger with Trimeris. As part of these activities, we also incurred additional facilities expense of $0.2 million resulting from the opening of our corporate headquarters and laboratory facilities in Lexington, Massachusetts in September 2010.

Amortization of Developed Technology

We recognized $0.5 million of costs related to the amortization of developed technology in fiscal 2011. Amortization of developed technology is computed using an accelerated method based on the undiscounted cash flows received from the FUZEON royalty stream (post Reverse Merger), in proportion to the estimated total undiscounted cash flows.

Other (Expense) Income, Net

Other (expense) income, net decreased by approximately $2.6 million to ($0.3) million for the year ended December 31, 2011 as compared to $2.3 million for the year ended December 31, 2010. The decrease was due to the receipt of a tax grant related to the approval of our applications for the Qualifying Therapeutic Discovery Project program during 2010. For 2011, this grant program was not available. In 2011, we recognized other expense associated with the revaluation of preferred stock warrants.

Liquidity and Capital Resources

Sources of Liquidity

We have financed our operations to date primarily through the placement of our equity and debt securities and debt financings, and, to a lesser extent, license and royalty fees, upfront cash payments and research and development funding from collaborators, government grants and licensors. In November 2011, we acquired $50.1 million in cash as a result of the Reverse Merger with Trimeris. On January 10, 2012, we announced the closing of a $90.0 million underwritten public offering of approximately 3.6 million shares of our common stock at a price of $25.18. We received net proceeds of approximately $84.6 million from this offering. In addition, on July 13, 2012, we announced the closing of a $115.0 million, second underwritten public offering of approximately 2.8 million shares of common stock at a price of $41.20. We received net proceeds of approximately $108.1 million. On January 9, 2013, we announced the closing of a $117.5 million underwritten public offering of approximately 2.5 million shares of common stock at a price of $47.53. We received net proceeds of approximately $111.1 million from this offering. We intend to use the net proceeds from these offerings for general corporate purposes, which may include working capital, capital expenditures, research and development expenditures, preclinical and clinical trial expenditures, commercial expenditures, acquisitions of new technologies or businesses that are complementary to our current technologies and business focus and investments.

We do not expect to generate any revenue from the direct sale of products currently in development for several years, if ever. As a result of our acquisition of Trimeris, we now receive royalties from the sale of FUZEON by Roche, which we expect to decrease over time. A significant portion of our revenues for the foreseeable future will be quarterly royalty payments from Roche based on sales of FUZEON, up-front license payments and funded research and development that we may receive under existing or new collaboration agreements, if any.

As of December 31, 2012, our principal sources of liquidity consisted of cash and cash equivalents and short-term investments of approximately $219.0 million. Our cash equivalents are highly liquid investments with a maturity of three months or less at date of purchase and consist of investments in money market funds.

Cash Flows

The use of our cash flows for operations has primarily consisted of salaries and wages for our employees, facility and facility-related costs for our office, laboratory, and manufacturing facilities, fees paid in connection with preclinical studies, clinical studies, outsourced manufacturing, laboratory supplies, consulting fees, commercial fees and legal fees. We expect that costs associated with clinical studies and manufacturing costs as well as commercial planning costs will increase in future periods as sebelipase alfa advances into further stages of clinical testing and our other preclinical candidates move forward in development.

Net cash used in operating activities was $30.9 million, $16.6 million and $10.6 million for the years ended December 31, 2012, 2011 and 2010, respectively.

Net cash used in operating activities was $30.9 million for the year ended December 31, 2012 and was primarily the result of a net loss of $43.4 million, which is discussed in further detail in “Results of Operations.” In addition, non-cash items and changes in certain operating assets and liabilities affected operating cash during fiscal 2012. Non-cash items partially offsetting net loss include depreciation of fixed assets of $1.0 million, amortization of developed technology of $3.2 million, stock-based compensation of $5.0 million, amortization of discount on available for sale investments of $0.3 million and in-process research and development of $0.3 million. Changes in operating assets and liabilities resulted in a net source of cash of $2.9 million, which was primarily the result of increased deferred revenue of $2.7 million from December 31, 2011. The increase in deferred revenue in the period was primarily the result of the upfront license fees related to the second Mitsubishi Tanabe development program. Other significant sources of cash included increased accounts payable and accrued expenses of $1.2 million from December 31, 2011, which was offset by a use of cash of $1.3 million related to prepaid expenses and other current assets and $0.4 million related to increased accounts receivable.

Net cash used in operating activities for the year ended December 31, 2011 was primarily the result of a net loss of $25.3 million, as discussed in further detail in “Results of Operations.” In addition, changes in certain operating assets and liabilities and non-cash items affected operating cash during the year ended December 31, 2011. Changes in

operating assets and liabilities that partially offset the cash impact of our net loss include $1.1 million from a net decrease in accounts receivable, prepaid expenses and other current assets, primarily due to receipt of royalty receivables that were recorded as part of the Reverse Merger in fiscal 2011; increased accounts payable and accrued expenses of $2.9 million as a result of an increased level of research and development spending related to our lead program and increased compensation and related costs; and, an increase in deferred revenue of approximately $2.7 million from the receipt of an upfront license payment related to the collaboration arrangement with Mitsubishi Tanabe. Non-cash items partially offsetting our net loss include depreciation and amortization of fixed assets of $1.0 million, stock-based compensation of $0.6 million, and $0.3 million from the revaluation of preferred stock warrants. Amortization related to the developed technology intangible asset contributed $0.5 million to depreciation and amortization expense for fiscal 2011, accounting for the majority of the year-over-year increase from fiscal 2010.

We expect to continue to use cash in operations as we continue to seek to advance our orphan drug programs through clinical development and preclinical testing. In addition, in the future, we may owe royalties and other contingent payments to our licensors based on the achievement of developmental milestones, product sales, and other specified objectives.

Net cash used in investing activities totaled $199.5 million in fiscal 2012, and was primarily a result of the purchase of available-for-sale investments of $195.4 million. Our investment portfolio includes short-term treasury securities, and was funded from our cash and cash equivalents balance in the fourth quarter of fiscal 2012. We had not received proceeds from either the sale of maturity of investments as of December 31, 2012. Other uses of cash for investing activities included $3.7 million for capital expenditures and $0.4 million for the purchase of certain IP rights and other long-term assets. We anticipate cash spent on capital expenditures will continue to increase as we expand our internal production and development capabilities.

Investing activities provided $49.4 million of cash in fiscal 2011, primarily as a result of $50.1 million of cash received as part of the Reverse Merger with Trimeris, partially offset by $0.7 million of cash paid for capital expenditures.

Financing activities provided cash of approximately $194.1 million in fiscal 2012, resulting from net cash received in secondary offerings of $192.7 million and proceeds from the exercise of stock options of $1.4 million.

Financing activities provided cash of approximately $12.7 million for fiscal 2011, primarily resulting from $12.5 million of proceeds from the issuance of convertible notes, and $0.2 million of proceeds from the exercises of stock options. In March 2011, we issued $12.5 million of convertible notes. In conjunction with the Reverse Merger, these notes were converted to common stock, and were no longer outstanding at December 31, 2011. This conversion did not have a cash impact.

Supplemental Disclosure Regarding Non-Cash Investing and Financing Activities

In fiscal 2011, our capitalization structure was impacted by the Reverse Merger with Trimeris. In conjunction with the Reverse Merger, $95.6 million of convertible preferred stock and $12.5 million of convertible notes was converted into common stock. We also extinguished a merger-related liability of $0.5 million through the issuance of common stock and issued common stock to the holders of warrants in a net settlement transaction. Although each of these transactions did not directly impact our cash balance at December 31, 2011, they are disclosed on our statement of cash flows.

Funding Requirements

We have incurred significant losses since our inception. As of December 31, 2012, we had an accumulated deficit of approximately $158.8 million. Our cash and cash equivalents and investments balance at December 31, 2012 totaled $219.0 million and we raised an additional $111.1 million in January of 2013, as discussed above. We expect to use our existing cash and cash equivalents and investments to continue our research and development programs and commercialization activities and to fulfill our planned operating goals. In particular, our currently planned operating and capital requirements include the need for working capital to support our research and development activities for

sebelipase alfa and other preclinical candidates that we are seeking to develop, and to fund our general and administrative and commercial costs and expenses. We expect that we will have sufficient cash and cash equivalents to sustain operations for more than the next two years.

Our ability to finance our operations into the future and to generate revenues will depend heavily on our ability to obtain favorable results in the ongoing clinical trials of sebelipase alfa and to successfully develop and commercialize sebelipase alfa. We expect that our significant sources of cash flows from operations for the foreseeable future will be quarterly royalty payments from Roche based on sales of FUZEON and additional collaboration revenues, if any. Net sales of FUZEON by Roche have decreased in each of the last three years and are expected to continue to decline.

We may not be able to successfully enter into any new corporate collaborations and the timing, amount, and likelihood of us receiving additional payments under our current collaborations is highly uncertain. As a result, we cannot assure that we will attain any further funding from collaborations or licensing arrangements.

There are a number of factors that may adversely affect our planned future capital requirements and accelerate our need for additional financing, many of which are beyond our control, including the following:

- unanticipated costs in our research and development programs;
- the timing, receipt and amount of payments, if any, from current and potential future collaborators;
- the timing and amount of payments due to licensors of patent rights and technology used in our drug candidates; and
- unplanned costs to prepare, file, prosecute, maintain and enforce patent claims and other patent-related costs, including litigation costs and technology license fees.

We may seek additional funding through debt or equity financings. The fundraising environment for life science companies, in general, is highly volatile. Due to this and various other factors, including currently adverse general market conditions and the early-stage status of our development pipeline, additional funding may not be available on acceptable terms, if at all. In addition, the terms of any financing may be dilutive or otherwise adversely affect other rights of our stockholders. We also expect to seek additional funds through arrangements with collaborators, licensees or other third parties. These arrangements would generally require us to relinquish or encumber rights to some of our technologies or drug candidates, and we may not be able to enter into such arrangements on acceptable terms, if at all. If we are unable to obtain additional funding on a timely basis, whether through sales of debt or equity or through third-party collaboration or license arrangements, we may be required to curtail or terminate some or all of our development programs, including some or all of our drug candidates.

Contractual Obligations and Requirements

As of December 31, 2012, our contractual obligations consisted primarily of operating leases for our headquarters and other facilities, contractual purchase obligations and to a lesser extent, royalty payments on licensed technology.

Our contractual obligations were as follows at December 31, 2012 (in thousands):

	Total	Less than 1 year	1-3 years	3-5 years	More than 5 years
Operating leases(1)	$ 1,493	$ 365	$ 332	$362	$434
Capital expenditures(2)	6,382	6,382	—	—	—
Purchase obligations(3)	23,529	13,372	10,126	31	—
Royalty payments(4)	80	20	40	20	—
	$31,484	$20,139	$10,498	$413	$434

(1) Represents minimum lease payments for operating leases, including leases with remaining lease terms of under one year. Our new lease agreement for our headquarters is not included in the table above, however, the estimated minimum lease payments are disclosed in Note 12. See Item 2.—Properties and Facilities and Note 12 for additional information about our operating leases.

(2) Represents our estimate of amounts related to leasehold improvements that will be paid under contractual arrangements existing at December 31, 2012. For the avoidance of doubt, the contractual obligations herein are dependent upon the third party providing the good or services under the contract and are cancelable.
(3) Purchase obligations primarily represent our estimate of amounts that will be paid to third parties, assuming continued development of sebelipase alfa and our pipeline, relating to contractual arrangements existing as of December 31, 2012. For the avoidance of doubt, the purchase obligations herein are dependent upon the third party providing goods or services under the contract and are cancelable.
(4) The royalty payments listing above represent amounts expected to be owed through December 31, 2012 to Pangenix and the University of Minnesota.

Off-Balance Sheet Arrangements

As of December 31, 2012, we did not have any off-balance sheet arrangements as defined in Item 303(a)(4)(ii) of SEC Regulation S-K.

Critical Accounting Estimates and Judgments

The significant accounting policies and basis of preparation of our consolidated financial statements are described in Note 2, "Summary of Significant Accounting Policies" of the Consolidated Financial Statements included in this Annual Report on Form 10-K. Under accounting principles generally accepted in the United States, we are required to make estimates and assumptions that affect the reported amounts of assets, liabilities, revenues, expenses and disclosure of contingent assets and liabilities in our financial statements. Actual results could differ from those estimates.

We believe the judgments, estimates and assumptions associated with the following critical accounting policies have the greatest potential impact on our consolidated financial statements:

- Revenue recognition;
- Research and development expenses;
- Amortization of developed technology; and,
- Income taxes

The methods, estimates, and judgments we use in applying our most critical accounting policies have a significant impact on the results reported in our consolidated financial statements. We evaluate our estimates and judgments on an ongoing basis. We base our estimates on historical experience and on assumptions that we believe to be reasonable under the circumstances. Our experience and assumptions form the basis for our judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may vary from what we anticipated, and different assumptions or estimates about the future could materially change our reported results.

Revenue Recognition

Our business strategy includes entering into collaborative agreements with biotechnology and pharmaceutical companies. Revenue under collaborations may include the receipt of non-refundable license fees, payments based on achievement of development objectives, reimbursement of research and development costs and royalties on product sales.

We recognize revenue when all of the following criteria are met: persuasive evidence of an arrangement exists, services are performed or products are delivered, the fee is fixed and determinable, and collection is reasonably assured. Determination of whether persuasive evidence exists and whether delivery has occurred or services have been rendered are based on our judgment regarding the fixed nature of the fee charged for deliverables and the collectability of those fees. Should changes in conditions cause us to determine these criteria are not met for future transactions, revenue recognized could be adversely affected.

Royalty Revenue

Royalty revenues are recognized in the period earned, based on contract terms when reported sales are reliably measurable and collectability is reasonably assured. Following the Reverse Merger, we received royalties due to the Roche License Agreement. As part of the Roche License Agreement, Roche has an exclusive license to manufacture and sell FUZEON worldwide and we receive royalty payments equal to 16% of worldwide net sales of FUZEON occurring from and after January 1, 2011. Under the Roche License Agreement, Roche may deduct from its royalty payments to us 50% of any royalties paid to third parties which are reasonably required to allow Roche to sell FUZEON in a given country, including royalties paid to Novartis Vaccines and Diagnostics, Inc. ("Novartis")." To calculate the royalty revenue paid to us, a 5.5% distribution charge is deducted from Roche's reported net sales, and we receive a 16% royalty on the adjusted net sales amount.

Collaboration and License Revenue

We recognize revenue related to collaboration and license agreements in accordance with the provisions of ASC Topics 605-25 "Revenue Recognition—Multiple Element Arrangements" ("ASC Topic 605-25"). In January 2011, we adopted Accounting Standards Update ("ASU") No. 2009-13, "Multiple Deliverable Revenue Arrangements" for contracts entered into or materially modified after that date. ASU 2009-13 updates the previous multiple-element revenue arrangements guidance. The revised guidance primarily provides three significant changes: 1) it eliminates the need for objective and reliable evidence of the fair value of the undelivered element in order for a delivered item to be treated as a separate unit of accounting; 2) it eliminates the residual method to allocate the arrangement consideration; and 3) it modifies the fair value requirements of EITF Issue 00-21 by providing best estimate of selling price ("BESP") in addition to vendor specific objective evidence and vendor objective evidence ("VSOE"), for determining the selling price of a deliverable. In addition, the guidance also expands the disclosure requirements for revenue recognition. We determine the selling price of a deliverable using the hierarchy as prescribed in ASC Topic 605-25 based on VSOE, third party evidence ("TPE"), or BESP. VSOE is based on the price charged when the element sold separately and is the price actually charged for that deliverable. TPE is determined based on third party evidence for a similar deliverable when sold separately and BESP is the price which we would transact a sale if the elements of the collaboration and license agreements were sold on a stand-alone basis. We evaluate the above noted hierarchy when determining the fair value of a deliverable. The process for determining VSOE, TPE, or BESP involves significant judgment on our part and can include considerations of multiple factors such as estimated direct expenses and other costs and available data.

We evaluate all deliverables within an arrangement to determine whether or not they provided value on a stand-alone basis. Based on this evaluation, the deliverables are separated into units of accounting. The arrangement consideration that is fixed and determinable at the inception of the arrangement is allocated to the separate units of accounting based on the estimated selling price. We may exercise significant judgment in determining whether a deliverable is a separate unit of accounting as well as in estimating the selling prices of such units of accounting.

For multiple element arrangements, including collaboration and license agreements, entered into prior to January 1, 2011, guidance required that the fair value of the undelivered item be the price of the item either sold in a separate transaction between unrelated third parties or the price charged for each item when the item is sold separately by the vendor. This was difficult to determine when the service or product was not individually sold because of its unique features. Under this guidance, if the fair value of all of the undelivered elements in the arrangement was not determinable, then revenue would be deferred until all of the items have been delivered or fair value was determined.

Whenever we determine that an arrangement should be accounted for as a single unit of accounting, we must determine the period over which the performance obligations will be performed and revenue will be recognized. Revenue will be recognized using either a proportional performance or straight-line method. We recognize revenue using the proportional performance method provided that we can reasonably estimate the level of effort required to complete the performance obligations under an arrangement and such performance obligations are provided on a best-efforts basis. Direct labor hours or full-time equivalents are typically used as the measure of performance. Revenue recognized under the relative performance method would be determined by multiplying the total payments under the contract, excluding royalties and payments contingent upon achievement of substantive milestones, by the ratio of level

of effort incurred to date to estimated total level of effort required to complete the performance obligations under the arrangement. Revenue is limited to the lesser of the cumulative amount of payments received or the cumulative amount of revenue earned, as determined using the relative performance method, as of each reporting period.

Significant management judgment is required in determining the level of effort required under an arrangement and the period over which we are expected to complete its performance obligations under an arrangement.

Effective January 1, 2011, we adopted ASU No. 2010-17, "Milestone Method of Revenue Recognition", which provides guidance on revenue recognition using the milestone method. Under the milestone method, a payment that is contingent upon the achievement of a substantive milestone is recognized in its entirety in the period in which the milestone is achieved. A milestone is an event (i) that can be achieved based in whole or in part on either our performance or on the occurrence of a specific outcome resulting from our performance, (ii) for which there is substantive uncertainty at the date the arrangement is entered into that the event will be achieved and (iii) that would result in additional payments being due. The determination that a milestone is substantive is subject to considerable judgment and is made at the inception of the arrangement. Milestones are considered substantive when the consideration earned from the achievement of the milestone is (i) commensurate with either our performance to achieve the milestone or the enhancement of value of the item delivered as a result of a specific outcome resulting from our performance to achieve the milestone, (ii) relates solely to past performance and (iii) is reasonable relative to all deliverables and payment terms in the arrangement. The adoption of this standard did not impact our financial position or our results of operations for any prior period.

Research and Development

Research and development expenses primarily consist of internal labor, clinical and non-clinical studies, materials and supplies, facilities, depreciation, third-party costs for contracted services, manufacturing process improvement and testing costs, and other research and development related costs. Clinical development and manufacturing costs are a significant component of our research and development expenses. We contract with third parties that perform various clinical trial activities and outsourced manufacturing activities on our behalf in the ongoing development of our product candidates. The financial terms of these contracts are subject to negotiations and may vary from contract to contract and may result in uneven payment flow. Research and development costs are expensed as incurred if no planned alternative future use exists for the technology and if the payment is not payment for future services. We defer and capitalize our nonrefundable advance payments that are for research and development activities until the related goods are delivered or the related services are performed.

Amortization of Developed Technology

We provide for amortization of developed technology, computed using an accelerated method according to the undiscounted expected cash flows to be received from the underlying assets, over an estimated useful life of 10 years.

Income Taxes

Deferred income taxes are provided for the net tax effects of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes and operating loss carryforwards and credits. Valuation allowances are recorded to reduce the net deferred tax assets to amounts we believe are more-likely-than-not to be realized.

New Accounting Pronouncements

In February 2013, the FASB issued Accounting Standard Update No. 2013-02, Other Comprehensive Income. The amendments require an entity to provide information about the amounts reclassified out of accumulated other comprehensive income by component. In addition, an entity is required to present, either on the face of the statement where net income is presented or in the notes, significant amounts reclassified out of accumulated other comprehensive income by the respective line items of net income but only if the amount reclassified is required under U.S. GAAP to be reclassified to net income in its entirety in the same reporting period. For other amounts that are not required under U.S. GAAP to be reclassified in their entirety to net income, an entity is required to cross-reference to other disclosures required under U.S. GAAP that provide additional detail about those amounts. We do not expect its adoption to have a material effect on our financial statements.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK.

Interest Rate Risk

The primary objective of our investment activities is to preserve our capital to fund operations, while at the same time maximizing the income we receive from our investments without significantly increasing risk. Our exposure to market risk for changes in interest rates relates primarily to the increase or decrease in the amount of interest income we can earn on our investment portfolio. Our risk associated with fluctuating interest income is limited to our investments in interest rate-sensitive financial instruments. Under our current policies, we do not use interest rate derivative instruments to manage this exposure to interest rate changes. We seek to ensure the safety and preservation of our invested principal by limiting default risk, market risk and reinvestment risk. We mitigate default risk by investing in treasury-backed money market funds and short-term treasury securities. In accordance with our investment policy, we do not invest in auction rate securities. We estimate that a hypothetical 50 and 100-basis point movement in market interest rates would impact the fair value of our December 31, 2012 investment portfolio by $0.5 million and $1.0 million, respectively. While changes in interest rates may affect the fair value of our investment portfolio, any gains or losses are not recognized in our statement of operations until the investment is sold prior to maturity or if a reduction in fair value is determined to be a permanent impairment. We do not use derivative financial instruments in our investment portfolio.

Foreign Exchange Market Risk

We currently have a subsidiary in the United Kingdom and as a result, face exposure to movements in the foreign currency exchange rates of the British Pound against the U.S. dollar. Currently, our international operations do not represent a significant portion of our business, and as a result, we believe our exposure to foreign exchange risk is insignificant.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA.

Our consolidated financial statements and supplementary data required in this Item 8 are set forth beginning on page 53 of this report.

ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE.

None.

ITEM 9A. CONTROLS AND PROCEDURES.

Conclusion Regarding the Effectiveness of Disclosure Controls and Procedures

Under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, we conducted an evaluation of our disclosure controls and procedures, as such term is defined under Rule 13a-15(e) promulgated under the Securities Exchange Act of 1934, as amended (the "Exchange Act"). Based on this evaluation, our principal executive officer and our principal financial officer concluded that our disclosure controls and procedures were effective as of December 31, 2012.

Management's Report on Internal Control over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Exchange Act Rule 13a-15(f). Under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting based on the framework in "Internal Control—Integrated Framework" issued by the Committee of Sponsoring Organizations of the Treadway Commission. Based on the evaluation, our management concluded that our internal control over financial reporting was effective as of December 31, 2012.

PricewaterhouseCoopers LLP, the independent registered public accounting firm, that audited our financial statements for the fiscal year ended December 31, 2012, has issued a report on the effectiveness of internal control over financial reporting, as stated in its report which is included herein.

ITEM 9B. OTHER INFORMATION.

None.

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE.

The information required by Item 10 is incorporated by reference from our definitive Proxy Statement to be filed by us with the SEC within 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K.

ITEM 11. EXECUTIVE COMPENSATION.

The information required by Item 11 is incorporated by reference from our definitive Proxy Statement to be filed by us with the SEC within 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K.

ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS.

The information required by Item 12 is incorporated by reference from our definitive Proxy Statement to be filed by us with the SEC within 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE.

The information required by Item 13 is incorporated by reference from our definitive Proxy Statement to be filed by us with the SEC within 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K.

ITEM 14. PRINCIPAL ACCOUNTANT FEES AND SERVICES.

The information required by Item 14 is incorporated by reference from our definitive Proxy Statement to be filed by us with the SEC within 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K.

PART IV

ITEM 15. EXHIBITS, FINANCIAL STATEMENT SCHEDULES.

The following documents are filed as part of this report:

2. Financial Statement Schedules

All financial statement schedules required under Regulation S-X are omitted, as the required information is not applicable.

3. Exhibits

The Exhibits filed as part of this Form 10-K are listed on the Exhibit Index immediately preceding such Exhibits and are incorporated by reference. We have identified in the Exhibit Index each management contract and compensation plan or arrangement filed as an exhibit to this Annual Report on Form 10-K in response to Item 15(b) of Form 10-K.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

Date: March 13, 2013

SYNAGEVA BIOPHARMA CORP.

By: /s/ Sanj K. Patel
Sanj K. Patel
President and Chief Executive Officer

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the Registrant in the capacities and on the dates indicated.

SIGNATURES	TITLE	DATE
/s/ Sanj K. Patel Sanj K. Patel	President and Chief Executive Officer (Principal Executive Officer) and Director	March 13, 2013
/s/ Carsten Boess Carsten Boess	Chief Financial Officer (Principal Financial Officer)	March 13, 2013
/s/ Chris Heberlig Chris Heberlig	Vice President, Finance (Principal Accounting Officer)	March 13, 2013
/s/ Felix J. Baker Felix J. Baker	Director	March 13, 2013
/s/ Stephen R. Biggar Stephen R. Biggar	Director	March 13, 2013
/s/ Stephen R. Davis Stephen R. Davis	Director	March 13, 2013
/s/ Thomas R. Malley Thomas R. Malley	Director	March 13, 2013
/s/ Barry Quart Barry Quart	Director	March 13, 2013
/s/ Thomas J. Tisch Thomas J. Tisch	Director	March 13, 2013
/s/ Peter Wirth Peter Wirth	Director	March 13, 2013

Report of Independent Registered Public Accounting Firm

To the Board of Directors and Stockholders of
Synageva BioPharma Corp.

In our opinion, the accompanying consolidated balance sheets and the related consolidated statements of operations, of comprehensive loss, of changes in convertible preferred stock and stockholders' equity (deficit), and of cash flows present fairly, in all material respects, the financial position of Synageva BioPharma Corp. and its subsidiaries at December 31, 2012 and 2011, and the results of their operations and their cash flows for each of the three years in the period ended December 31, 2012 in conformity with accounting principles generally accepted in the United States of America. Also in our opinion, the Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2012, based on criteria established in *Internal Control—Integrated Framework* issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). The Company's management is responsible for these financial statements, for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting, included in Management's Report on Internal Control over Financial Reporting appearing under Item 9A. Our responsibility is to express opinions on these financial statements and on the Company's internal control over financial reporting based on our audits (which was an integrated audit in 2012). We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audits to obtain reasonable assurance about whether the financial statements are free of material misstatement and whether effective internal control over financial reporting was maintained in all material respects. Our audits of the financial statements included examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. Our audit of internal control over financial reporting included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, and testing and evaluating the design and operating effectiveness of internal control based on the assessed risk. Our audits also included performing such other procedures as we considered necessary in the circumstances. We believe that our audits provide a reasonable basis for our opinions.

A company's internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company's internal control over financial reporting includes those policies and procedures that (i) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (ii) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (iii) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

/s/ PricewaterhouseCoopers LLP

Boston, Massachusetts
March 14, 2013

Synageva BioPharma Corp.

Consolidated Balance Sheets
December 31, 2012 and 2011
(In thousands, except per share amounts)

	2012	2011
Assets		
Current assets:		
Cash and cash equivalents	$ 23,883	$ 60,232
Short-term investments	195,070	—
Accounts receivable	2,599	2,211
Taxes receivable	—	1,240
Prepaid expenses and other current assets	3,555	968
Total current assets	225,107	64,651
Property and equipment, net	4,012	1,256
Developed technology, net	5,564	8,796
Goodwill	8,535	8,535
Other assets	38	60
Total assets	$ 243,256	$ 83,298
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 2,576	$ 1,507
Accrued expenses	5,112	5,003
Deferred revenue, short term	5,391	1,749
Total current liabilities	13,079	8,259
Deferred revenue, long term	—	991
Total liabilities	$ 13,079	$ 9,250
Commitments and contingencies (Note 12)		
Stockholders' equity:		
Common stock, par value $0.001; 60,000 shares authorized at December 31, 2012 and 2011, respectively; 24,467 and 17,582 shares issued and outstanding at December 31, 2012 and 2011, respectively	24	18
Additional paid-in capital	388,936	189,874
Accumulated other comprehensive loss	6	(4)
Accumulated deficit	(158,789)	(115,840)
Total stockholders' equity	$ 230,177	$ 74,048
Total liabilities and stockholders' equity	$ 243,256	$ 83,298

The accompanying notes are an integral part of these financial statements.

Synageva BioPharma Corp.

Statements of Operations
Years Ended December 31, 2012, 2011 and 2010
(In thousands, except per share amounts)

	2012	2011	2010
	Consolidated	Consolidated	
Revenues:			
Royalty revenue	$ 7,023	$ 1,083	$ —
Collaboration and license revenue	7,875	640	280
Other revenue	56	376	315
Total revenue	14,954	2,099	595
Costs and expenses:			
Research and development	37,347	17,346	9,866
General and administrative	17,396	9,268	3,852
Amortization of developed technology	3,232	504	—
Total costs and expenses	57,975	27,118	13,718
Loss from operations	(43,021)	(25,019)	(13,123)
Other (expense) income, net	—	(259)	2,295
Interest income (expense), net	72	(28)	4
Net loss	$(42,949)	$(25,306)	$(10,824)
Basic and diluted net loss per share	$ (1.90)	$ (8.58)	$(338.25)
Weighted average shares used in basic and diluted per share computations	22,579	2,950	32

The accompanying notes are an integral part of these financial statements.

Synageva BioPharma Corp.

Statements of Comprehensive Loss
Years Ended December 31, 2012, 2011 and 2010
(In thousands)

	2012	2011	2010
	Consolidated	Consolidated	
Net loss	$(42,949)	$(25,306)	$(10,824)
Other comprehensive loss:			
Fair market value adjustments of available for sale securities	15	—	—
Foreign currency translation adjustments	(5)	(4)	—
Total other comprehensive gain (loss)	10	(4)	—
Comprehensive loss	$(42,939)	$(25,310)	$(10,824)

The accompanying notes are an integral part of these financial statements.

Synageva BioPharma Corp.

Statements of Changes in Convertible Preferred Stock and Stockholders' Equity (Deficit)
Years Ended December 31, 2012, 2011 and 2010
(In thousands)

	Total Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Deficit	Accumulated Other Comprehensive Income (Loss)	Total Stockholder's Equity (Deficit)
	Shares	Amount	Shares	Amount				
Balances December 31, 2009	25,997	95,556	23	0	10,178	(79,710)	—	(69,532)
Accretion to redemption value of redeemable preferred stock	—	62	—	—	(62)	—	—	(62)
Issuance of Series D-2 preferred stock, net of issuance costs of $207	—	(37)	—	—	—	—	—	—
Exercise of stock options	—	—	38	0	36	—	—	36
Stock-based compensation expense	—	—	—	—	204	—	—	204
Net loss	—	—	—	—	—	(10,824)	—	(10,824)
Balances December 31, 2010	25,997	95,581	61	0	10,356	(90,534)	—	(80,178)
Conversion of notes payable to preferred stock	5,000	12,500	—	—	—	—	—	—
Exchange of preferred stock for common stock in connection with the Reverse Merger	(30,997)	(108,081)	12,789	13	108,067	—	—	108,080
Acquisition of Trimeris	—	—	4,480	5	69,860	—	—	69,865
Issuance of common stock to underwriter	—	—	33	0	500	—	—	500
Conversion of preferred stock warrants to common stock warrants	—	—	—	—	271	—	—	271
Exercise of common stock warrants	—	—	10	0	0	—	—	—
Exercise of stock options	—	—	209	0	187	—	—	187
Stock-based compensation expense	—	—	—	—	633	—	—	633
Cumulative translation adjustment	—	—	—	—	—	—	(4)	(4)
Net loss	—	—	—	—	—	(25,306)	—	(25,306)
Balances December 31, 2011	—	$ —	17,582	$18	$189,874	$(115,840)	$ (4)	$ 74,048
Issuance of common stock	—	—	6,366	6	192,730	—	—	192,736
Exercise of stock options	—	—	519	1	1,361	—	—	1,361
Stock-based compensation expense			—	—	4,971	—	—	4,971
Fair market value adjustments of available for sale investments	—	—	—	—	—	—	15	15
Cumulative translation adjustment	—	—	—	—	—	—	(5)	(5)
Net loss	—	—	—	—	—	(42,949)	—	(42,949)
Balances December 31, 2012	—	$ —	24,467	$24	$388,936	$(158,789)	$ 6	$230,177

The accompanying notes are an integral part of these financial statements. See Note 5 for information on the Reverse Merger.

Synageva BioPharma Corp.

Statements of Cash Flows
Years Ended December 31, 2012, 2011 and 2010
(In thousands)

	2012	2011	2010
	Consolidated	Consolidated	
Cash flows from operating activities			
Net Loss	$ (42,949)	$ (25,306)	$(10,824)
Adjustments:			
Depreciation and amortization	4,272	1,034	384
Stock compensation expense	4,971	633	204
Amortization of discount on investments	310	—	—
Acquired in-process research and development	344	—	—
Revaluation of preferred stock warrants	—	259	(17)
Changes in assets and liabilities, net of acquisition:			
Accounts receivable	(388)	1,586	(800)
Prepaid expenses, other current assets, and other assets	(1,324)	(464)	(97)
Accounts payable	1,069	1,079	230
Accrued expenses	109	1,850	370
Deferred revenue	2,651	2,740	—
Net cash used in operating activities	**(30,935)**	**(16,589)**	**(10,550)**
Cash flows from investing activities			
Purchase of available for sale investments	(195,365)	—	—
Capital expenditures	(3,746)	(683)	(585)
Purchase of in-process research and development and other assets	(394)	—	—
Cash received from merger	—	50,107	—
Net cash provided by (used in) investing activities	**(199,505)**	**49,424**	**(585)**
Cash flows from financing activities			
Proceeds from issuance of common stock, net of issuance costs	192,736	—	—
Proceeds from exercise of stock options	1,361	186	36
Proceeds from issuance of convertible preferred stock, net of issuance costs	—	—	(37)
Proceeds from issuance of convertible notes	—	12,500	—
Net cash provided by (used in) financing activities	**194,097**	**12,686**	**(1)**
Effect of exchange rate on cash	(6)	(4)	—
Net increase (decrease) in cash and equivalents	(36,349)	45,517	(11,136)
Cash and equivalents at the beginning of period	60,232	14,715	25,851
Cash and equivalents at the end of period	**$ 23,883**	**$ 60,232**	**$ 14,715**
Supplemental schedule of noncash investing and financing activities			
Conversion of convertible note into preferred stock	—	12,500	—
Conversion of preferred stock into common stock	—	108,081	—
Payments made in common stock to underwriter	—	500	—
Conversion of preferred stock warrants to common stock warrants	—	271	—
Accretion to redemption value of redeemable convertible preferred stock	—	—	62
Fair value of assets acquired in Merger	$ —	$ 72,379	$ —
Fair value of liabilities assumed in Merger	—	(2,510)	—
Fair value of net assets acquired in Merger	$ —	$ 69,869	$ —

The accompanying notes are an integral part of these financial statements.

1. Nature of the Business

Synageva BioPharma Corp. ("Synageva" or the "Company") 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, including two enzyme replacement therapies for lysosomal storage disorders and additional programs for other serious genetic conditions for which there are currently no approved treatments. Its lead program, sebelipase alfa, is a recombinant human lysosomal acid lipase ("LAL") currently under clinical investigation in North America and the European Union ("EU") for the treatment of patients with early onset and late onset LAL Deficiency, which is a rare, devastating disease that causes significant morbidity and mortality. Synageva currently evaluates sebelipase alfa in global clinical trials and sebelipase alfa has been granted orphan designations by the U.S. Food and Drug Administration ("FDA"), the European Medicines Agency, and the Japanese Ministry of Health, Labour and Welfare. Additionally, sebelipase alfa has received *Fast Track* Designation by the FDA. Synageva has not yet received approval to market this product and is not currently commercializing any other products.

The Company is subject to risks common to companies in the biopharmaceutical industry including, but not limited to, the successful development of products, clinical trial uncertainty, regulatory approval, fluctuations in operating results and financial risks, potential need for additional funding, protection of proprietary technology and patent risks, compliance with government regulations, dependence on key personnel and collaboration partners, competition, technological and medical risks and management of growth. See "Item 1A. Risk Factors" for additional discussion on the risks facing the Company.

The Company has incurred losses since inception and at December 31, 2012, had an accumulated deficit of $158.8 million. The Company expects to incur losses over the next several years as it continues to expand its drug discovery, development efforts and commercial activities. As a result of continuing losses, the Company may seek additional funding through a combination of public or private financing, collaborative relationships or other arrangements. The Company may not be able to obtain financing on acceptable terms, or at all, and the Company may not be able to enter into new collaboration or license agreements. If we are unable to obtain additional funding on a timely basis, whether through sales of debt or equity or through third-party collaboration or license arrangements, we may be required to curtail or terminate some or all of our development programs, including some or all of our drug candidates.

Through December 31, 2012, the Company has funded its operations primarily from proceeds of the sale of stock, issuance of convertible notes, royalty proceeds and proceeds from government grants and collaboration agreements. On November 2, 2011, the Company completed a merger transaction (the "Reverse Merger") with Trimeris, Inc., a Delaware corporation ("Trimeris") (see Note 5, "Merger"), which was accounted for as a business combination, through which it assumed certain assets and liabilities of the acquired entity, including $50.1 million in cash and cash equivalents and a royalty stream related to FUZEON, a product sold by F. Hoffman-La Roche Ltd. ("Roche"), which serves as further funding for the Company's operations.

On January 10, 2012, the Company announced the closing of a $90.0 million underwritten public offering of approximately 3.6 million shares of common stock at a price of $25.18. The Company received net proceeds of approximately $84.6 million from this offering. In addition, on July 13, 2012, the Company announced the closing of a $115.0 million underwritten public offering of approximately 2.8 million shares of common stock at a price of $41.20. The Company received net proceeds of approximately $108.1 million from this offering. On January 9, 2013, the Company announced the closing of a $117.5 million underwritten public offering of approximately 2.5 million shares of common stock at a price of $47.53. The Company received net proceeds of approximately $111.1 million from this offering.

The Company intends to use the proceeds from these offerings for general corporate purposes, which may include working capital, capital expenditures, research and development expenditures, preclinical and clinical trial

expenditures, commercial expenditures, acquisitions of new technologies or businesses that are complementary to our current technologies and business focus and investments. The Company expects that it will have sufficient cash and cash equivalents to sustain operations for more than two years.

2. Summary of Significant Accounting Policies

Basis of Presentation

Use of Estimates

The preparation of financial statements in conformity with accounting principles generally accepted in the United States of America ("GAAP") requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of revenues and expenses during the reporting period. Actual results could differ from those estimates.

Principles of Consolidation

Synageva's consolidated financial statements include the accounts of Synageva and its wholly owned subsidiaries. All intercompany accounts and transactions have been eliminated in consolidation.

Reclassification

The Company has separately presented costs associated with the amortization of developed technology in the consolidated statements of operations. In the prior year, amortization of developed technology was included within research and development expenses.

Reverse Merger

On November 2, 2011, Trimeris closed a merger transaction (the "Reverse Merger") with Synageva BioPharma Corp., a privately held Delaware corporation ("Private Synageva"), pursuant to an Agreement and Plan of Merger and Reorganization, dated as of June 13, 2011 (the "Merger Agreement"), by and among Trimeris, Private Synageva and Tesla Merger Sub, Inc., a wholly owned subsidiary of Trimeris ("Merger Sub"). Pursuant to the Merger Agreement, Private Synageva became a wholly owned subsidiary of Trimeris through a merger of Merger Sub with and into Private Synageva, and the former stockholders of Private Synageva received shares of Trimeris that constituted a majority of the outstanding shares of Trimeris. In connection with the Reverse Merger, Trimeris changed its name to Synageva BioPharma Corp.

The Reverse Merger was accounted for as a reverse acquisition under which Private Synageva was considered the acquirer of Trimeris. As such, the financial statements of Private Synageva are treated as the historical financial statements of the combined company, with the results of Trimeris being included from November 2, 2011.

See Note 5 for additional discussion of the Reverse Merger and the conversion ratio.

Cash and Cash Equivalents

The Company considers all highly liquid investments with a remaining maturity at the date of purchase of less than three months to be cash equivalents. At December 31, 2012 and 2011, substantially all cash equivalents were U.S. treasury bills and amounts held in money market accounts at commercial banks.

Investments

All investments were classified as available-for-sale at December 31, 2012. The principal amounts of short-term investments as of December 31, 2012, are summarized in the tables below:

	Less Than 12 Months to Maturity			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
	(in thousands)			
Balance at December 31, 2012:				
U.S. Treasury securities	$195,055	$15	$—	$195,070
Total	$195,055	$15	$—	$195,070

The Company completed an evaluation of its investments and determined that it did not have any other-than-temporary impairments as of December 31, 2012.

Fair Value Measurements

Under current accounting standards, fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs.

The current accounting guidance also establishes a hierarchy to categorize how fair value is measured and which is based on three levels of inputs, of which the first two are considered observable and the last unobservable, as follows:

Level 1 Quoted prices in active markets for identical assets or liabilities.

Level 2 Inputs other than Level 1 that are observable, either directly or indirectly, such as quoted prices for similar assets or liabilities, quoted prices in markets that are not active, or other inputs that are observable or can be corroborated by observable market data for substantially the full term of the assets or liabilities.

Level 3 Unobservable inputs that are supported by little or no market activity and that are significant to the fair value of the assets or liabilities.

The following table presents information about the Company's financial assets and liabilities measured at fair value on a recurring basis as of December 31, 2012:

	December 31, 2012	Quoted Price in Active Markets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Assets				
Cash equivalents				
Money market fund	$ 10,387	$ 10,387	$—	$—
US treasury securities	10,011	10,011	—	$—
Marketable securities				
US treasury securities	$195,070	$195,070	$—	$—
Total	$215,468	$215,468	$—	$—

The following table presents information about the Company's financial assets and liabilities measured at fair value on a recurring basis as of December 31, 2011:

	December 31, 2011	Quoted Price in Active Markets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Assets				
Cash equivalents—money market fund	$59,860	$59,860	$—	$—

The change in the valuation of preferred stock warrants for the year ended December 31, 2011 is summarized below.

	Year ended December 31, 2011
Fair value, beginning of year	$ 12
Change in fair value	259
Conversion of preferred stock warrants to common stock warrants	(271)
Fair value, end of year	$ —

Prior to the Reverse Merger, the Company accounted for warrants to purchase 31 shares of Series C-2 convertible preferred stock according to accounting standards regarding freestanding financial instruments with the characteristics of both liabilities and equities. Due to the redemption feature of the Series C-2 convertible preferred stock, these warrants were classified as liabilities. The warrants were revalued at each balance sheet date and any change in fair value was recorded as a component of other income or other expense. In connection with the Reverse Merger, the Series C-2 convertible preferred stock warrants were converted to common stock warrants, and a final mark to market calculation was performed. The common stock warrants were subsequently exercised in a net settlement transaction in the fourth quarter of fiscal 2011, resulting in the issuance of 10 shares of common stock.

Property and Equipment

Property and equipment are recorded at cost and are depreciated and amortized using the straight-line method over the assets' expected useful lives. Property and equipment held under capital leases and leasehold improvements are amortized over the shorter of the lease term or the estimated useful life of the related asset. Upon retirement or sale, the cost of the assets disposed of and the related accumulated depreciation are removed from the accounts and any resulting gain or loss is credited or charged to income. Repairs and maintenance costs are expensed as incurred.

Asset Classification	Estimated Useful Life
Computer hardware and software	3 years
Vehicles	5 years
Furniture and fixtures	7 years
Lab and facility equipment	5-7 years
Leasehold improvements	shorter of estimated useful life or lease term

Impairment of Other Long-Lived Tangible and Intangible Assets

The Company continually evaluates whether events or circumstances have occurred that indicate that the estimated remaining useful life of long-lived assets and intangible assets may warrant revision or if events or circumstances indicate that the carrying value of these assets may be impaired. To assess whether assets have been impaired, the estimated undiscounted future cash flows for the estimated remaining useful life of the assets are compared to the carrying value. To the extent that the future cash flows are less than the carrying value, the assets are written down to the estimated fair value, based on the discounted cash flows of the asset.

Amortization of Developed Technology

The Company provides for amortization of developed technology, computed using an accelerated method based on the undiscounted cash flows received from the FUZEON royalty stream, in proportion to the estimated total undiscounted cash flows, as discussed in further in Note 6 "Goodwill and Intangible Assets, net").

Goodwill

The difference between the purchase price and the fair value of assets acquired and liabilities assumed in a business combination is allocated to goodwill. Goodwill is not amortized; however, it is required to be tested for impairment annually. Furthermore, testing for impairment is required on an interim basis if an event or circumstance indicates that it is more likely than not an impairment loss has been incurred. An impairment loss would be recognized to the extent that the carrying amount of goodwill exceeds its implied fair value. Absent an event that indicates a specific impairment may exist, the Company has selected December 31 as the date for performing the annual goodwill impairment test.

The Company adopted ASU No. 2011-08, *Intangibles-Goodwill and Other (Topic 350): Testing Goodwill for Impairment,* during fiscal 2012, which allows companies to perform a simplified goodwill impairment test. Entities are no longer required to calculate the fair value of the reporting unit unless the qualitative factors indicate that it is more likely than not that a reporting unit's fair value is less than its carrying amount. We evaluated our goodwill using the simplified approach, and our goodwill was not impaired as of December 31, 2012. See Note 6, "Goodwill and Intangible Assets, Net," for additional information.

Revenue Recognition

The Company's business strategy includes entering into collaborative agreements with biotechnology and pharmaceutical companies. Revenue under collaborations may include the receipt of non-refundable license fees, payments based on achievement of development objectives, reimbursement of research and development costs and royalties on product sales.

The Company recognizes revenue when all of the following criteria are met: persuasive evidence of an arrangement exists, services are performed or products are delivered, the fee is fixed and determinable, and collection is reasonably assured. Determination of whether persuasive evidence exists and whether delivery has occurred or services have been rendered are based on management's judgment regarding the fixed nature of the fee charged for deliverables and the collectability of those fees. Should changes in conditions cause management to determine these criteria are not met for future transactions, revenue recognized could be adversely affected.

Collaboration and License Revenue

The Company recognizes revenue related to collaboration and license agreements in accordance with the provisions of ASC Topics 605-25 "Revenue Recognition—Multiple Element Arrangements" ("ASC Topic 605-25"). In January 2011, the Company adopted Accounting Standards Update ("ASU") No. 2009-13, "Multiple Deliverable Revenue Arrangements" for contracts entered into or materially modified after that date. ASU 2009-13 updates the previous multiple-element revenue arrangements guidance. The revised guidance primarily provides three significant changes: 1) it eliminates the need for objective and reliable evidence of the fair value of the undelivered element in order for a delivered item to be treated as a separate unit of accounting; 2) it eliminates the residual method to allocate the arrangement consideration; and 3) it modifies the fair value requirements of EITF Issue 00-21 by providing best estimate of selling price, or BESP, in addition to vendor specific objective evidence and vendor objective evidence, or VSOE, for determining the selling price of a deliverable. In addition, the guidance also expands the disclosure requirements for revenue recognition. The Company determines the selling price of a deliverable using the hierarchy as prescribed in ASC Topic 605-25 based on VSOE, third party evidence "TPE," or BESP. VSOE is based on the price charged when the element sold separately and is the price actually charged for that deliverable. TPE is determined based on third party evidence for a similar deliverable when sold separately and BESP is the price which the Company would transact a sale if the elements of the collaboration and license agreements were sold on a stand-alone basis. The Company evaluates the above noted hierarchy when determining the fair value of a deliverable. The

process for determining VSOE, TPE, or BESP involves significant judgment on the part of the Company and can include considerations of multiple factors such as estimated direct expenses and other costs and available data. ASC 605-25 is effective prospectively for new arrangements or upon material modification of existing arrangements.

The Company evaluates all deliverables within an arrangement to determine whether or not they provided value on a stand-alone basis. Based on this evaluation, the deliverables are separated into units of accounting. The arrangement consideration that is fixed and determinable at the inception of the arrangement is allocated to the separate units of accounting based on the estimated selling price. The Company may exercise significant judgment in determining whether a deliverable is a separate unit of accounting as well as in estimating the selling prices of such units of accounting.

For multiple element arrangements, including collaboration and license agreements, entered into prior to January 1, 2011, guidance required that the fair value of the undelivered item be the price of the item either sold in a separate transaction between unrelated third parties or the price charged for each item when the item is sold separately by the vendor. This was difficult to determine when the service or product was not individually sold because of its unique features. Under this guidance, if the fair value of all of the undelivered elements in the arrangement was not determinable, then revenue was deferred until all of the items were delivered or fair value was determined.

Whenever the Company determines that an arrangement should be accounted for as a single unit of accounting, it must determine the period over which the performance obligations will be performed and revenue will be recognized. Revenue will be recognized using either a proportional performance or straight-line method. The Company recognizes revenue using the proportional performance method provided that the Company can reasonably estimate the level of effort required to complete its performance obligations under an arrangement and such performance obligations are provided on a best-efforts basis. Direct labor hours or full-time equivalents are typically used as the measure of performance. Revenue recognized under the relative performance method would be determined by multiplying the total payments under the contract, excluding royalties and payments contingent upon achievement of substantive milestones, by the ratio of level of effort incurred to date to estimated total level of effort required to complete the Company's performance obligations under the arrangement. Revenue is limited to the lesser of the cumulative amount of payments received or the cumulative amount of revenue earned, as determined using the relative performance method, as of each reporting period.

Significant management judgment is required in determining the level of effort required under an arrangement and the period over which the Company is expected to complete its performance obligations under an arrangement

Effective January 1, 2011, the Company adopted ASU No. 2010-17, "Milestone Method of Revenue Recognition", which provides guidance on revenue recognition using the milestone method. Under the milestone method, a payment that is contingent upon the achievement of a substantive milestone is recognized in its entirety in the period in which the milestone is achieved. A milestone is an event (i) that can be achieved based in whole or in part on either our performance or on the occurrence of a specific outcome resulting from our performance, (ii) for which there is substantive uncertainty at the date the arrangement is entered into that the event will be achieved and (iii) that would result in additional payments being due. The determination that a milestone is substantive is subject to considerable judgment and is made at the inception of the arrangement. Milestones are considered substantive when the consideration earned from the achievement of the milestone is (i) commensurate with either the Company's performance to achieve the milestone or the enhancement of value of the item delivered as a result of a specific outcome resulting from our performance to achieve the milestone, (ii) relates solely to past performance and (iii) is reasonable relative to all deliverables and payment terms in the arrangement. The adoption of this standard in fiscal 2011 has not impacted our financial position or results of operations.

See Note 11, "License Agreements and Collaborations," for additional information on specific arrangements. Collaboration and license revenue totaled approximately $7.9 million, $0.6 million and $0.3 million for the years ended December 31, 2012, 2011 and 2010, respectively.

Royalty Revenue

Royalty revenues are recognized in the period earned, based on contract terms when reported sales are reliably measurable and collectability is reasonably assured. Following the merger with Trimeris, we received royalties

due to the Development and License Agreement with Roche (the "Roche License Agreement"). As part of the Roche License Agreement, Roche has an exclusive license to manufacture and sell FUZEON worldwide and the Company receives royalty payments equal to 16% of worldwide net sales of FUZEON occurring from and after January 1, 2011. Under the Roche License Agreement, Roche may deduct from its royalty payments to us 50% of any royalties paid to third parties which are reasonably required to allow Roche to sell FUZEON in a given country, including royalties paid to Novartis Vaccines and Diagnostics, Inc. ("Novartis")." To calculate the royalty revenue paid to Synageva, a 5.5% distribution charge is deducted from Roche's reported net sales, and Synageva receives a 16% royalty on the adjusted net sales amount. Revenue from royalties totaled $7.0 million and $1.1 million for the year ended December 31, 2012 and the approximate two month post- merger period ending December 31, 2011, respectively. These royalties represent the royalty payment earned from Roche based on total worldwide net sales of FUZEON since the closing of the Reverse Merger in November 2011.

Reimbursement of Costs

Reimbursement of research and development costs by third party collaborators is recognized as revenue provided the Company has determined that it is acting primarily as a principal in the transaction according to the provisions outlined in FASB Codification Topic 605-45, Revenue Recognition, Principal Agent Considerations, the amounts are determinable and collection of the related receivable is reasonably assured.

Grant Revenue

The Company recognizes revenues from grants in the period in which the Company has incurred the expenditures in compliance with the specific restrictions of the grant.

Revenue from grants was recognized in the period in which the related expenditures were incurred and totaled approximately $0.1 million, 0.4 million and $0.3 million for the years ended December 31, 2012, 2011 and 2010, respectively, and are reflected as other revenue in the statements of operations.

Deferred Revenue

Amounts received prior to satisfying the above revenue recognition criteria are recorded as deferred revenue in the accompanying consolidated balance sheets. Amounts not expected to be recognized within twelve months from the balance sheet date would be classified as long-term deferred revenue.

Research and Development

Research and development expenses primarily consist of internal labor, clinical and non-clinical studies, materials and supplies, facilities, depreciation, third-party costs for contracted services, manufacturing process improvement and testing costs, and other research and development related costs. Clinical development and manufacturing costs are a significant component of our research and development expenses. We contract with third parties that perform various clinical trial activities and outsourced manufacturing activities on our behalf in the ongoing development of our product candidates. The financial terms of these contracts are subject to negotiations and may vary from contract to contract and may result in uneven payment flow. Research and development costs are expensed as incurred if no planned alternative future use exists for the technology and if the payment is not payment for future services. The Company defers and capitalizes its nonrefundable advance payments that are for research and development activities until the related goods are delivered or the related services are performed.

Segment Reporting

The Company is managed and operated as one business, focused on the discovery, development, and commercialization of therapeutic products for patients with life-threatening rare diseases and unmet medical need. The entire business is managed by a single management team with reporting to the chief executive officer. We do not operate separate lines of business or separate business entities with respect to our products or product candidates. Accordingly, the Company does not prepare discrete financial information with respect to separate product areas or by location and only has one reportable segment.

Legal, Intellectual Property ("IP") and Patent Costs

The Company accrues estimated liabilities for loss contingencies when it is probable that a liability has been incurred and the amount of the claim assessment or damages can be reasonably estimated. Synageva expenses legal fees, IP-related and patent costs as they are incurred.

Income Taxes

Deferred income taxes are provided for the net tax effects of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes and operating loss carryforwards and credits. Valuation allowances are recorded to reduce the net deferred tax assets to amounts the Company believes are more-likely-than-not to be realized.

Stock-Based Compensation

The Company's share-based compensation awards to employees, including grants of employee stock options, are valued at fair value on the date of grant, and are expensed over the requisite service period. The requisite service period is the period during which an employee is required to provide service in exchange for an award, which generally is the vesting period.

Concentration of Credit Risk and Other Risks and Uncertainties

Financial instruments that potentially subject the Company to credit risk consist principally of cash, cash equivalents and U.S. treasury securities. The Company places its cash and cash equivalents in bank deposits, money market funds, and U.S. treasury bills which are maintained at several financial institutions. Deposits in these institutions may exceed the amount of insurance provided on such deposits. Management believes it has established guidelines relative to credit quality, diversification and maturities that maintain security and liquidity.

The Company is subject to risks and uncertainties common to the biotechnology industry. Such risks and uncertainties include, but are not limited to: (a) results from current and planned clinical trials, (b) scientific data collected on the Company's technologies currently in preclinical research and development, (c) decisions made by the FDA or other regulatory bodies with respect to the initiation of human clinical trials, (d) decisions made by the FDA or other regulatory bodies with respect to approval and commercial sale of any of the Company's proposed products, (e) the commercial acceptance of any products approved for sale and the ability of the Company to manufacture, distribute and sell for a profit any products approved for sale, (f) the Company's ability to obtain the necessary patents and proprietary rights to effectively protect its technologies, (g) the outcome of any collaborations or alliances entered into by the Company in the future with pharmaceutical or other biotechnology companies, (h) dependence on key personnel, (i) competition with better capitalized companies and (j) ability to raise additional funds.

Basic and Diluted Net Loss per Common Share

Basic net loss per common share has been computed by dividing net loss by the weighted average number of shares outstanding during the period. Diluted net income per share, if applicable, has been computed by dividing diluted net income by the diluted number of shares outstanding during the period. Except where the result would be antidilutive to loss from continuing operations, diluted net loss per share has been computed assuming the conversion of convertible obligations and the elimination of the related interest expense, the exercise of stock options and warrants, as well as their related income tax effects.

The following table sets forth the computation of basic and diluted net loss per common share:

	Years Ended December 31,		
	2012	2011	2010
Numerator:			
Net loss	$(42,949)	$(25,306)	$(10,824)
Denominator			
Weighted average common shares(1)			
Denominator for basic calculation	22,579	2,950	32
Denominator for diluted calculation	22,579	2,950	32
Net loss per share:(1)			
Basic	$ (1.90)	$ (8.58)	$(338.25)
Diluted	$ (1.90)	$ (8.58)	$(338.25)

(1) Per share computations for fiscal 2011 are based on (i) Private Synageva's historic common stock balances (excluding preferred stock) up to the Merger date and (ii) post-Merger common stock from the Merger date to year end. For fiscal 2010, per share computations are based on Private Synageva's historic common stock balances, which exclude preferred stock.

The Company's potential dilutive securities which include convertible debt, convertible preferred stock, stock options, and warrants have been excluded from the computation of diluted net loss per share as the effect would be to reduce the net loss per share. Therefore, the weighted-average common stock outstanding used to calculate both basic and diluted net loss per share are the same. The following shares of potentially dilutive securities have been excluded from the computations of diluted weighted average shares outstanding as the effect of including such securities would be antidilutive:

	As of December 31,		
	2012	2011	2010
Options to purchase common stock	2,529	2,371	1,244
Convertible preferred stock	—	—	25,997
Convertible preferred stock warrants	—	—	31
	2,529	2,371	27,272

Recently Issued and Proposed Accounting Pronouncements

In September 2011, the FASB issued ASU 2011-08, *Goodwill and Other (Topic 350): Testing Goodwill for Impairment*, which simplifies goodwill impairment tests. The revised standard is intended to reduce the cost and complexity of the annual goodwill impairment test by providing companies with the option of performing a qualitative assessment to determine whether future impairment testing is necessary. The Company adopted the new standard in fiscal 2012. Adoption of this new standard did not have a material effect on our financial statements.

In July 2012, the Financial Accounting Standards Board (FASB) issued ASU No. 2012-02, *Intangibles—Goodwill and Other (Topic 350): Testing Indefinite-Lived Intangible Assets for Impairment* (ASU 2012-02). This newly issued accounting standard allows an entity the option to first assess qualitative factors to determine whether it is necessary to perform a quantitative impairment test for indefinite-lived intangibles other than goodwill. Under that option, an entity would no longer be required to calculate the fair value of an indefinite-lived intangible asset unless the entity determines, based on that qualitative assessment, that it is more likely than not that the fair value of the indefinite-lived intangible asset is less than its carrying amount. The Company adopted this standard when performing its impairment evaluation as of December 31, 2012. Adoption of this new standard did not have a material effect on our financial statements.

In June 2011, the FASB issued a new standard on the presentation of comprehensive income. The new standard eliminated the alternative to report other comprehensive income and its components in the statement of changes in equity. Under the new standard, companies can elect to present items of net income and other comprehensive income in one continuous statement or in two separate, but consecutive statements. The Company adopted the provisions of this guidance during fiscal 2012.

In May 2011, the FASB issued a new standard on fair value measurement and disclosure requirements. The new standard changes fair value measurement principles and disclosure requirements including measuring the fair value of financial instruments that are managed within a portfolio, the application of applying premiums and discounts in a fair value measurement, and additional disclosure about fair value measurements. The adoption of this guidance in fiscal 2012 did not have a material effect on our financial statements.

In February 2013, the FASB issued Accounting Standard Update No. 2013-02, *Other Comprehensive Income*. The amendments require an entity to provide information about the amounts reclassified out of accumulated other comprehensive income by component. In addition, an entity is required to present, either on the face of the statement where net income is presented or in the notes, significant amounts reclassified out of accumulated other comprehensive income by the respective line items of net income but only if the amount reclassified is required under U.S. GAAP to be reclassified to net income in its entirety in the same reporting period. For other amounts that are not required under U.S. GAAP to be reclassified in their entirety to net income, an entity is required to cross-reference to other disclosures required under U.S. GAAP that provide additional detail about those amounts. We do not expect its adoption to have a material effect on our financial statements.

3. Property and Equipment

	December 31,	
	2012	2011
Laboratory equipment	$ 3,478	$ 2,438
Leasehold improvements	2,748	415
Computer, software and office equipment	1,004	699
Vehicles	111	94
	7,341	3,646
Less: Accumulated depreciation and amortization	(3,329)	(2,390)
	$ 4,012	$ 1,256

Depreciation and amortization expense was $1.0 million and $0.5 million for fiscal year 2012 and 2011, respectively.

4. Accrued Expenses

Accrued expenses consist of the following:

	December 31,	
	2012	2011
Accrued compensation and benefits	$1,862	$3,113
Clinical, manufacturing and scientific costs	1,978	819
Professional fees	685	619
Other	587	452
	$5,112	$5,003

5. Merger

On November 2, 2011, the Company completed the Reverse Merger, which was accounted for as a reverse acquisition under the acquisition method of accounting, with Private Synageva treated as the accounting acquiror and Trimeris treated as the "acquired" company for financial reporting purposes because, after the Merger, former stockholders of Private Synageva held a majority of the voting interest of the combined company. In addition, the former board of directors of Private Synageva possessed majority control of the board of directors of the combined company. Members of the management of Private Synageva are responsible for the management of the combined company and the majority of the combined company's activities are related to Synageva's current business. As such, the financial statements of Private Synageva are treated as the historical financial statements of the combined company, with the results of Trimeris being included from November 2, 2011.

Reverse Stock Split

On November 2, 2011, as contemplated by the Merger Agreement and as approved by Trimeris' stockholders, Trimeris filed a Certificate of Amendment to its Fifth Amended and Restated Certificate of Incorporation with the Secretary of State of the State of Delaware to effect a reverse stock split of Trimeris common stock at a ratio of 1:5 (the "Reverse Stock Split"). As a result of the Reverse Stock Split, each five shares of Trimeris common stock issued and outstanding immediately prior to the Reverse Stock Split were automatically combined into and became one share of Trimeris common stock. No fractional shares of Trimeris common stock were issued as a result of the Reverse Stock Split and any Trimeris stockholder who otherwise would have been entitled to receive fractional shares received cash in an amount, without interest, determined by multiplying such fraction of a share by $15.35, the closing price of a share of Trimeris common stock on the Nasdaq Global Market on November 2, 2011, after giving effect to the Reverse Stock Split. Also, as a result of the Reverse Stock Split, the per share exercise price of, and the number of shares of common stock underlying, Company stock options, warrants and other derivative securities outstanding immediately prior to the Reverse Stock Split were automatically proportionally adjusted based on the one-for-five split ratio in accordance with the terms of such options, warrants or other derivative securities, as the case may be. The Reverse Stock Split did not alter the par value of the Trimeris common stock or modify any voting rights or other terms of the common stock. Following the Reverse Stock Split, but prior to the Reverse Merger, there were 4.5 million shares of Trimeris stock outstanding.

Exchange Ratio

Based on the outstanding shares of Private Synageva's capital stock on November 2, 2011, each share of Private Synageva's preferred stock and common stock was exchanged for approximately 0.413 shares of Trimeris common stock (the "Exchange Ratio"). As part of the Reverse Merger, Private Synageva's convertible notes payable were converted into D-2 Preferred Stock, prior to the conversion of all of Private Synageva's preferred stock and common stock into shares of the combined company. The conversion of Private Synageva's preferred stock and common stock resulted in 13.0 million shares in the combined company.

In addition, (i) all outstanding options to purchase shares of Private Synageva common stock were assumed by Trimeris and converted into options to purchase shares of Trimeris common stock, in each case appropriately adjusted based on the Exchange Ratio; and (ii) all outstanding warrants to purchase shares of the capital stock of Private Synageva were assumed by Trimeris and converted into warrants to purchase shares of Trimeris common stock, in each case appropriately adjusted based on the Exchange Ratio. Immediately after the Reverse Merger, former stockholders of Private Synageva held approximately 75% of the combined company, calculated on a fully-diluted basis, and former stockholders of Trimeris held approximately 25% of the combined company, calculated on a fully-diluted basis, in each case excluding those shares of Trimeris held by the former Private Synageva stockholders immediately prior to the time of the Reverse Merger. After giving effect to the Reverse Stock Split and the Reverse Merger, the combined company had approximately 17.5 million shares of common stock outstanding.

Merger Purchase Price

The consolidated financial statements reflect the merger of Synageva with Trimeris as a reverse merger wherein Synageva is deemed to be the acquiring entity from an accounting perspective. Under the acquisition method of accounting, Trimeris' 4.5 million of outstanding shares of common stock (following the stock split but prior to the Merger) were valued using the closing price on the Nasdaq Global Market of $15.35 per share on November 2, 2011. Further, as a result of the merger, options to purchase an aggregate of 0.4 million shares of Trimeris common stock that were held by officers and directors of Trimeris immediately vested (see Note 9). The fair values of the Trimeris outstanding stock options were determined using the Black-Scholes option pricing model with the following assumptions: stock price of $15.35; volatility of 51%; risk-free interest rate of 1.02%; and a weighted average expected life of 2.8 years. In addition, Synageva incurred approximately $1.1 million of transaction costs related to the Merger. These costs were expensed as incurred, and classified as general and administrative expense.

The purchase price, based on the stock price as of the Reverse Merger date, was as follows:

Fair value of Trimeris shares outstanding	$68,767
Fair value of vested Trimeris stock options	1,102
Purchase price	$69,869

Reverse Merger Purchase Price Allocation

The fair value of acquired assets and liabilities were as follows (in thousands):

Cash and cash equivalents	$50,107
Accounts receivable, taxes refundable and other current assets	4,437
Developed technology—FUZEON	9,300
Goodwill(1)	8,535
Assumed liabilities	(2,510)
Total	$69,869

(1) The goodwill resulting from the Reverse Merger is not deductible for tax purposes.

Pro Forma Financial Information

The following table presents selected unaudited financial information, as if the Reverse Merger with Trimeris had occurred on January 1, 2010 (in thousands, except per share data).

	Year Ended	
	2011	2010
	(Unaudited)	
Pro forma net revenue(1)	$ 10,035	$27,312
Pro forma net (loss) income	(22,337)	695
Pro forma net (loss) income per common share—basic	$ (4.82)	$ 0.15
Pro forma net (loss) income per common share—diluted	$ (4.82)	$ 0.15

(1) Fiscal 2010 revenue includes $18.7 million of revenue resulting from entering into the Deferred Marketing Expenses Agreement with Roche, which relieved the obligation to repay certain deferred marketing expenses.

Fiscal 2011 pro forma net loss incorporates the elimination of $5.2 million of acquisition-related costs, which have been reflected in the 2010 pro forma net loss. Both 2011 and 2010 pro forma net loss includes $3.2 million of estimated amortization expense related to acquired developed technology.

6. Goodwill and Intangible Assets, net

In fiscal 2011, we acquired $8.5 million of goodwill in the Reverse Merger. We have not recognized any impairment charges, nor have we acquired additional goodwill in fiscal 2012 or 2011.

The Company performed its annual goodwill impairment test as of December 31, 2012, and notes that its goodwill does not appear to be impaired as of December 31, 2012.

Intangible assets, net of accumulated amortization is as follows (in thousands):

		As of December 31, 2012		
	Initial Estimated Life	Cost	Accumulated Amortization	Net
Developed Technology	10 years	$9,300	$(3,736)	$5,564

The developed technology asset represents the present value of the estimated future FUZEON royalty stream (Note 11). Amortization expense totaled $3.2 million and $0.5 million for fiscal 2012 and 2011, respectively. The developed technology asset acquired in the Reverse Merger with Trimeris is being amortized over the estimated life of the royalty stream, in proportion to the related royalty revenue. As a result, the estimated level of amortization expense is weighted toward the earlier years. Assuming no change to our estimates of the Roche royalty stream, the Company expects amortization expense to approximate the following:

Fiscal Year	Amortization (in thousands)
2013	$1,925
2014	1,347
2015	883
2016	574
2017 and beyond	835

7. Convertible Notes and Convertible Preferred Stock

Convertible Preferred Stock

As of December 31, 2010, of the 28,000 authorized shares of preferred stock, 246 shares were designated as Series A-2 convertible preferred stock ("Series A-2 preferred stock"), 4,169 shares were designated as Series B-2 convertible preferred stock ("Series B-2 preferred stock"), 3,659 shares were designated as Series C-2 convertible preferred stock ("Series C-2 preferred stock"), and 18,000 shares were designated as Series D-2 preferred stock. These shares converted to common stock of the combined company as part of the Reverse Merger at a ratio of approximately 0.413 for every share of previously issued stock. There were no shares of authorized preferred stock outstanding at December 31, 2012 or 2011.

2011 Convertible Notes

In March 2011, the Company issued Convertible Notes (the "2011 Convertible Notes") for proceeds of $12.5 million as the first tranche of a potential $25 million convertible note offering. The 2011 Convertible Notes were convertible into shares of Series D-2 preferred stock at the conversion price then applicable to the Series D-2 preferred stock or into a future series of Preferred Stock at its then applicable conversion price issued in conjunction with the next Qualified Offering (as defined in the 2011 Notes purchase agreement), at the election of the holders. The 2011 Convertible Notes had no stated interest rate. On November 2, 2011 as part of the completion of the merger with Trimeris, the 2011 Convertible Notes were converted into D-2 Preferred Stock, prior to the conversion of all Private Synageva preferred and common shares outstanding into shares of the combined entity.

8. Share Based Payments

The Company's stock-based compensation plans include the 1996 Stock Option Plan and the 2005 Stock Plan, as well as options outstanding under Trimeris, Inc.'s 1993 Plan and 2007 Stock Incentive Plan. As part of the Reverse Merger, options outstanding under Trimeris's previous option plans were adjusted by the conversion ratio, and remain in existence as options in the combined entity. The Board of Directors has the authority to determine to whom options will be granted, the number of shares, the term, and the exercise price.

At the Company's annual meeting of stockholders held June 27, 2012, stockholders approved amendments to the 2005 Stock Plan to increase the number of shares of common stock available for issuance by 1.5 million shares, increasing the maximum number of shares that may be issued under the 2005 Stock Plan to approximately 3.5 million shares. Shares subject to outstanding awards under the 2005 Stock Plan totaled 2.2 million as of December 31, 2012.

In addition, at the Company's annual meeting of stockholders held June 27, 2012, stockholders approved the Employee Stock Purchase Plan (the "ESPP"), which allows eligible employees to use payroll deductions to purchase shares of the Company's common stock at a discount of 15%. The plan is considered a compensatory

employee stock purchase plan, and will result in incremental stock-based compensation expense in future periods. Option periods under the ESPP will run from January 1 to June 30 and July 1 to December 31 of each year, with the first option period commencing in fiscal 2013.

The Company uses the Black-Scholes option pricing model to measure the fair value of its option awards. The fair value of each stock option grant was estimated on the date of grant using the Black-Scholes option-pricing model. The expected life assumption is based on the limited exercise historical experience at the Company, management's expectations based on the length of time that an employee will stay at the Company, the vesting period of four years and the contractual term of ten years. Volatility has been determined based on an analysis of reported data for a peer group of companies that granted options with substantially similar terms. The risk-free interest rate is based on the rate of U.S. Treasury zero coupon rate with a remaining term approximating the expected term used as the input to the Black-Scholes option pricing model.

The weighted average assumptions used in the option pricing model for stock option grants were as follows:

	Year Ended December 31,		
	2012	2011	2010
Expected dividend yield	None	None	None
Weighted average volatility in stock price	50%	51%	52%
Weighted average risk-free interest rate	.88%	1.51%	1.71%
Expected life of stock awards-years	6 years	6 years	5 years

A summary of stock option activity under all equity plans for the year ended December 31, 2012, 2011, and 2010 is as follows:

	Number of Shares	Weighted Average Exercise Price
Outstanding at December 31, 2009	1,395	$ 1.21
Options granted	34	1.09
Options exercised	(38)	0.97
Options canceled or expired	(147)	1.16
Outstanding at December 31, 2010	1,244	$ 1.27
Options granted	1,029	13.39
Options assumed through merger with Trimeris	373	57.23
Options exercised	(209)	0.89
Options canceled or expired	(66)	1.41
Outstanding at December 31, 2011	2,371	$15.34
Options granted	820	43.90
Options exercised	(519)	2.62
Options canceled or expired	(143)	53.83
Outstanding at December 31, 2012	2,529	$25.06
Exercisable at December 31, 2012	1,051	$21.24
Exercisable and expected to vest at December 31, 2012	2,369	$24.87

The weighted average grant date fair value of options granted in 2012, 2011 and 2010 was $20.38, $8.11, and $0.51, respectively. The total intrinsic value of stock options exercised during the years ended December 31, 2012, 2011 and 2010 was $21.2 million, $1.0 million, and less than $0.1 million, respectively.

Options outstanding and currently exercisable at December 31, 2012, under all equity plans, are as follows:

	Options Outstanding			Options Exercisable	
Range of Exercise Prices	Number Outstanding	Weighted Average Remaining Contractual Life (yrs)	Weighted Average Exercise Price	Number Exercisable	Weighted Average Exercise Price
$0.61-$0.95	414	5.6	$ 0.95	295	$ 0.95
$1.10-$1.70	328	8.2	1.67	97	1.65
$3.52-$8.73	234	7.0	5.78	158	4.56
$10.10-$20.60	79	6.7	14.88	79	14.88
$23.00-$40.70	1,177	8.4	32.28	301	26.85
$45.50-$69.50	262	7.3	57.30	85	59.07
$94.80-$194.00	13	0.6	140.58	13	140.58
$203.85-$231.65	22	0.4	225.23	23	225.23
	2,529	7.5	$ 25.06	1,051	$ 21.24

As of December 31, 2012, the unamortized compensation expense related to outstanding unvested options approximated $18.2 million and is expected to be recognized over a weighted average period of 3.2 years.

The aggregate intrinsic value of shares outstanding and shares exercisable at December 31, 2012 is approximately $61.8 million and $32.7 million, respectively, which represents the total intrinsic value (the excess of the fair value of the Company's stock on December 31, 2012 over the exercise price, multiplied by the number of in-the-money options) that would have been received by the option holders had all option holders exercised their options on December 31, 2012.

The Company recognized stock-based compensation expense on all stock option awards for the years ended December 31, 2012, 2011 and 2010 in the following categories:

	2012	2011	2010
Research and development	$1,432	$231	$ 45
General and administrative	3,539	402	159
	$4,971	$633	$204

9. Defined Contribution Plan

The Company has a defined contribution plan that is intended to qualify under Section 401(k) of the Internal Revenue Code of 1986, as amended (the "Code"). All employees, except part-time employees, are eligible to participate in the plan. Participants may contribute through payroll deductions, amounts not to exceed Internal Revenue Code limitations. During the years ended December 31, 2012, 2011 and 2010, the Company recognized expense for 401(k) matching contributions of $0.2 million, $0.2 million and $0.1 million, respectively.

10. Income Taxes

The Company has not recorded a benefit for income taxes related to its operating losses for the years ended December 31, 2012 and 2011. The Company's effective tax rate differs from that based on the federal statutory rate due to the following:

	Years Ended December 31,	
	2012	2011
Federal tax at statutory rate	$(14,603)	(8,571)
State income taxes	(1,149)	(1,278)
State rate changes	2,161	(1,443)
Federal and state credits	(15,784)	(1,611)
Trimeris net operating losses	—	(9,481)
Expiration of state NOLs	11,129	337
Stock-based compensation	335	(26)
Valuation allowance	17,887	22,081
Other	24	(8)
Benefit from income taxes	$ —	$ —

Significant components of the Company's net deferred tax asset as December 31, 2012 and 2011 are as follows:

	2012	2011
Deferred tax assets:		
Net operating losses	$ 37,451	$ 45,825
Capitalized research and development	10,286	10,428
Tax credit carryforwards	25,693	5,267
Deferred revenue	2,056	1,073
Accrued expenses	142	706
Depreciation and amortization	235	109
Stock-based compensation	1,289	6
Other	10	11
	77,162	63,425
Deferred tax liabilities:		
Acquired intangibles	(1,527)	(3,445)
Valuation allowance	(75,635)	(59,980)
Net deferred tax asset	$ —	$ —

At December 31, 2012, the Company had federal and state net operating loss carry forwards of $123.7 million and $134.3 million, respectively, which begin expiring in 2013. In conjunction with ceasing of Trimeris operations in 2012, approximately $11.1 million of tax effected state net operating loss carryforwards expired (or were forfeited). The Company has federal orphan drug credits and federal and state research tax credit carryforwards of $25.7 million, available to reduce future tax liabilities, which begin expiring in 2018 and 2023, respectively. Included in our December 31, 2012 deferred tax balance were orphan drug credits that were generated in earlier periods, resulting in a net increase in our gross deferred tax asset balance of approximately $4.1 million. Given the corresponding increase in the valuation allowance, this out-of-period adjustment had no impact on the Company's balance sheet, statement of operations, or statement of cash flows and is considered immaterial to the Company's current and prior period financial statements taken as a whole. Approximately $19.4 million of the federal and state net operating loss carryforwards relate to deductions from stock option compensation, which are tracked separately and not included in the Company's deferred tax asset in accordance with ASC 718. The future benefit from these deductions will be recorded as a credit to additional paid in capital to the extent they reduce taxes payable.

Utilization of the net operating loss ("NOL") and research and development ("R&D") credit carry forwards may be subject to a substantial annual limitation under Section 382 of the Code due to ownership change limitations

that have occurred previously or that could occur in the future. These ownership changes may limit the amount of NOL and R&D credit carry forwards that can be utilized annually to offset future taxable income and tax, respectively. As part of the Reverse Merger, the Company acquired federal tax attributes that are significantly limited under Section 382 of the Code. There also could be additional ownership changes in the future which may result in additional limitations on the utilization of NOL carryforwards and credits.

Management has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets, and has determined that it is more-likely-than-not that the Company will not recognize the benefits of its deferred tax asset. Accordingly, a valuation allowance of $75.7 million and $60.0 million has been established at December 31, 2012 and 2011, respectively.

The Company files tax returns as prescribed by the tax laws of the jurisdictions in which it operates. In the normal course of business the Company is subject to examination by federal and state jurisdictions, where applicable. There are currently no pending tax examinations.

Below is a table of the earliest tax years that remain subject to examination by jurisdictions:

Jurisdiction	Earliest Tax Year Subject to Examination
U.S Federal	2009
State of Georgia	2009
State of North Carolina	2009
Commonwealth of Massachusetts	2009

All years including and subsequent to the above years remain open to examination by the taxing authorities. Also, tax attributes carrying forward from years prior to 2009 are subject to adjustment by the Internal Revenue Service if they have been or will be used in a future period. The resolution of any tax matters is not expected to have a material effect on the Company's financial statements. The Company's policy is to record interest and penalties related to income taxes as part of the tax provision. There were no uncertain tax positions in 2012 and 2011.

The Company adopted the authoritative guidance on accounting for and disclosure of uncertainty in tax positions on January 1, 2009, which required the Company to determine whether a tax position of the Company is more likely than not to be sustained upon examination, including resolution of any related appeals of litigation processes, based on the technical merits of the position. The adoption of this authoritative guidance did not have a material effect on the financial statements. The Company has not recorded any amounts for unrecognized tax benefits as of December 31, 2012 and 2011.

A provision included in the Patient Protection and Affordable Care Act of 2010 created a temporary tax credit for businesses with less than 250 employees who engage in qualifying therapeutic discovery projects for the tax years 2009 and 2010. The program permitted applicants to elect to receive a cash grant in lieu of the tax credit. The amount of the tax credit or grant was equal to 50% of the qualified investment for the taxable year for any qualifying therapeutic discovery project. The total amount awarded by the U.S. Treasury Department to the Company was $2.3 million. The Company elected to receive the award as a cash grant and has recorded the award as other income within the accompanying statement of operations for the year ended December 31, 2010. Amounts received in cash totaled $1.5 million and $0.8 million in 2010 and 2011, respectively.

11. License Agreements and Collaborations

Collaborations

In August 2011, the Company entered into a collaboration agreement with Mitsubishi Tanabe Pharma Corporation ("Mitsubishi Tanabe") whereby the Company is utilizing its proprietary expression technology for the development of a certain targeted compound. The agreement included an upfront development payment to the Company of $3.0 million, on-going reimbursement or funding of development costs, estimated during the initial development period to be approximately $1.5 million, and the potential for an additional payment of $3.0 million due upon the successful completion of the initial development.

In March 2012, the Company entered into an agreement with Mitsubishi Tanabe to develop a second protein therapeutic. The agreement included an upfront license payment to the Company of $9.0 million and on-going reimbursement or funding of development costs, estimated during the initial development period to be approximately $0.8 million, as well as the potential for an additional payment of $3.0 million due upon successful completion of the initial development stage of the second program.

Under both agreements, Mitsubishi Tanabe has an option to obtain an exclusive royalty-bearing license, with the right to grant sublicenses, to further develop and commercialize the licensed compound (the "Option"). Additionally, upon exercise of the Option, the parties intend to negotiate a follow-on collaboration and license agreement that may include potential future development and commercial sales based milestone payments, and potential royalty payments. The Company determined that the Option is substantive as the decision to exercise is in the control of Mitsubishi Tanabe and is not essential to the functionality of the other deliverables in either agreement. Therefore, the Option was not considered to be a deliverable at the inception of either collaboration agreement. The Option terminates sixty days from date the Joint Steering Committee ("JSC") determines whether the initial development was successful.

The Company evaluated the collaboration agreements in order to determine whether the deliverables at the inception of the agreements: (i) the upfront license payments, (ii) research services during the development periods, and (iii) JSC participation should be accounted for as a single unit or multiple units of accounting. The Company concluded that the upfront license payments do not have standalone value to Mitsubishi Tanabe because (i) Mitsubishi Tanabe does not have the ability to transfer or sublicense and (ii) the activities to be conducted during the development period are highly dependent on the Company's unique knowledge and understanding of its proprietary technology which is critical to optimizing the compounds. The Company determined that the JSC is a deliverable through the development period. In both agreements, the Company concluded that there are two units of accounting which are being delivered over the same performance period.

Revenue recognized under the first and second Mitsubishi Tanabe development programs totaled $1.9 million and $6.0 million for the year ended December 31, 2012, respectively, and $0.5 million and $0 million, respectively as of December 31, 2011. Revenue is recognized using the proportional performance method. As of December 31, 2012, the deferred revenue balance includes $1.6 million and $3.8 million related to the first and second development programs, respectively.

Roche Collaboration

The Roche License Agreement

On May 25, 2011, Trimeris entered into the Roche License Agreement with Roche, pursuant to which Roche has an exclusive license to manufacture and sell FUZEON worldwide and the Company receives royalty payments equal to 16% of worldwide net sales of FUZEON occurring from and after January 1, 2011. The Roche License Agreement superseded and replaced the Prior Roche Agreements. Under the Roche License Agreement, Roche may deduct from its royalty payments to us 50% of any royalties paid to third parties which are reasonably required to allow Roche to sell FUZEON in a given country, including royalties paid to Novartis Vaccines and Diagnostics, Inc. ("Novartis")." To calculate the royalty revenue paid to Synageva, a 5.5% distribution charge is deducted from Roche's reported net sales, and Synageva receives a 16% royalty on the adjusted net sales amount. We recognized royalty revenue of $7.0 million and $1.1 million for the years ended December 31, 2012 and 2011, respectively.

Roche may terminate the Roche License Agreement as a whole or for a particular country or countries in its sole discretion with advance notice. The Roche License Agreement will effectively terminate upon expiration of the last relevant patent covering FUZEON, which is expected to occur in 2021.

FUZEON is manufactured and distributed by Roche through Roche's sales and distribution network throughout the world in countries where regulatory approval has been received. Roche has control over all aspects of the commercialization of FUZEON, including, but not limited to, pricing, sales force activities and promotional activities.

12. Commitments and Contingencies

Occupancy Arrangements

The Company leases office, laboratory and facility space under operating lease agreements expiring through 2020. Certain of the leases provide for options by the Company to extend the lease for multiple periods and also provide for annual minimum increases in rent, usually based on a consumer price index or annual minimum increases.

The following is a schedule by years of future minimum rental payments required under operating leases that have initial or remaining non-cancelable lease terms in excess of one year as of December 31, 2012:

Year Ending December 31,	
2013	$146
2014	167
2015	165
2016	179
2017	182
2018 and beyond	434

Rental expense for the years ended December 31, 2012, 2011 and 2010 approximated $1.0 million, $0.7 million and $0.5 million, respectively.

On January 15, 2013, the Company entered into a lease agreement to accommodate the Company's continued growth and to relocate its corporate headquarters in Lexington, Massachusetts. The Company will occupy the location in stages as building modifications are completed. The Company currently anticipates that it will begin occupying a portion of the facility in May 2013. The initial lease term is for 77 months after the Company begins occupying the entire location, with an option to extend the lease term for two separate three year renewal periods. Future minimum lease payments for the Lexington lease are as follows:

Year Ending December 31,	
2013	$ 167
2014-2019	1,336 (per year)
2020	334

UGARF License Agreement

On April 5, 2007, the Company amended and restated its technology license agreement with the University of Georgia Research Foundation ("UGARF"). In consideration for exclusive worldwide rights to the same patents, know-how and related technology under the original agreement, the Company provided 9 shares of its common stock to UGARF in addition to sublicense royalties, if applicable, and product royalties to be payable upon future commercialization and sale of any products subject to the license. No payments have been made in fiscal 2012 and 2011.

Other Licensing Agreements

The Company has licensing and sponsored research agreements with certain scientific and research institutions. The Company incurred expenses under these agreements of less than $0.1 million for the years ended December 31, 2012, 2011 and 2010. At December 31, 2012, the Company had approximately $0.1 million of potential milestone payments or other commitments payable over the next four years under agreements that are cancelable by either party under certain circumstances. These agreements also specify the payment of certain percentage royalties based on net sales of developed technologies.

13. Quarterly Financial Data (Unaudited)

The following tables present quarterly consolidated statement of operations data for fiscal 2012 and 2011. The below data is unaudited but, in our opinion, reflects all adjustments necessary for a fair presentation of this data in accordance with GAAP.

	Three Months Ended			
	March 31, 2012	June 30, 2012	September 30, 2012	December 31, 2012
	(unaudited)			
Revenue	$ 2,397	2,249	$ 5,428	$ 4,880
Loss from operations	(7,355)	(10,226)	(10,189)	(15,251)
Net loss	(7,353)	(10,223)	(10,182)	(15,191)
Loss per share, basic and diluted	$ (0.35)	$ (0.48)	$ (0.43)	$ (0.62)
Weighted average shares outstanding, basic and diluted	20,774	21,284	23,825	24,347

	Three Months Ended			
	March 31, 2011	June 30, 2011	September 30, 2011	December 31, 2011
	(unaudited)			
Revenue	$ 62	$ 238	$ 184	$ 1,614
Loss from operations	(5,412)	(5,387)	(6,819)	(7,401)
Net loss	(5,445)	(5,560)	(6,823)	(7,478)
Loss per share, basic and diluted	$(95.53)	$(75.14)	$(92.20)	$ (0.65)
Weighted average shares outstanding, basic and diluted	57	74	74	11,587

14. Subsequent Events

Underwritten Public Offering

On January 9, 2013, we announced the closing of a $117.5 million underwritten public offering of approximately 2.5 million shares of common stock at a price of $47.53. The Company received net proceeds of approximately $111.1 million from this offering.

Lease Agreement for Corporate Headquarters

On January 15, 2013, the Company entered into an operating lease agreement to accommodate the Company's continued growth and to relocate its corporate headquarters in Lexington, Massachusetts. The Company will occupy the location in stages as building modifications are completed. The Company currently anticipates that it will begin occupying a portion of the facility in May 2013. The initial lease term is for 77 months after the Company begins occupying the entire location, with an option to extend the lease term for two separate three year renewal periods (see Note 12 for additional information).

EXHIBIT LIST

Exhibit Number	Description
2.1	Agreement and Plan of Merger and Reorganization, dated as of June 13, 2011, by and among Trimeris, Inc., Tesla Merger Sub, Inc. and Synageva BioPharma Corp., incorporated by reference to the Company's Current Report on Form 8-K filed with the SEC on June 13, 2010.
3.1	Fifth Amended and Restated Certificate of Incorporation of the Registrant, as amended, incorporated by reference to the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2005 filed with the SEC on November 8, 2005, as amended by the Company's Current Report on Form 8-K filed with the SEC on November 3, 2011.
3.2	Second Amended and Restated Bylaws of the Registrant, incorporated by reference to the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2005 filed with the SEC on November 8, 2005.
4.1	Specimen certificate for shares of Common Stock, incorporated by reference to the Company Registration Statement on Form S-3 (File No. 333-178653) initially filed with the SEC on December 21, 2011.
4.2	Description of Capital Stock (contained in the Fifth Amended and Restated Certificate of Incorporation of Synageva BioPharma Corp., filed as Exhibit 3.1), incorporated by reference to the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2005 filed with the SEC on November 8, 2005, as amended by the Company's Current Report on Form 8-K filed with the SEC on November 3, 2011.
10.1	Trimeris, Inc. Amended and Restated Stock Incentive Plan, incorporated by reference to the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2006 filed with the SEC on November 13, 2006.*
10.2	Form of Indemnification Agreement between Synageva BioPharma Corp. and its directors, incorporated by reference to the Company's Quarterly Report on Form 10-Q filed with the SEC on November 14, 2011.
10.3	Form of Indemnification Agreement between Synageva BioPharma Corp. and its officers, incorporated by reference to the Company's Quarterly Report on Form 10-Q filed with the SEC on November 14, 2011.
10.4	Amended and Restated Registration Rights Agreement dated April 1, 2009, between the Company and the investors signatory to the agreement, as amended through January 4, 2012, incorporated by reference to the Company's Current Report on Form 8-K filed with the SEC on January 4, 2012.
10.5	Non-Exclusive Sub-License Agreement, between Abbey BioPharma Corp. (a wholly owned subsidiary of Synageva BioPharma Corp.) (f/k/a AviGenics, Inc.) and Pangenix, dated April 1, 2003, incorporated by reference to the Company's Quarterly Report on Form 10-Q filed with the SEC on November 14, 2011.†
10.6	Amended and Restated License Agreement, between Abbey BioPharma Corp. (a wholly owned subsidiary of Synageva BioPharma Corp.) (f/k/a AviGenics, Inc.) and the University of Georgia Research Foundation, dated April 5, 2007, incorporated by reference to the Company's Quarterly Report on Form 10-Q filed with the SEC on November 14, 2011.†
10.7	License Agreement between The Regents of the University of California and Hoffman-La Roche Inc. and Trimeris, Inc. for Method for Preventing and Treating a Viral Condition by Inhibiting Membrane Fusion, dated June 27, 2005, incorporated by reference to the Company's Current Report on Form 8-K filed with the SEC on June 27, 2005.†
10.8	Exclusive Patent License Agreement, between Abbey BioPharma Corp. (a wholly owned subsidiary of Synageva BioPharma Corp.) (f/k/a Synageva BioPharma Corp.) and the University of Minnesota, dated May 13, 2009, incorporated by reference to the Company's Quarterly Report on Form 10-Q filed with the SEC on November 14, 2011.†
10.9	Amended and Restated Agreement among Trimeris, Inc., F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc., dated May 25, 2011, incorporated by reference to the Company's Current Report on Form 8-K filed with the SEC on May 25, 2011.

Exhibit Number	Description
10.10	1996 Stock Option Plan of AviGenics, Inc, incorporated by reference to the Company's Registration Statement on Form S-8 filed with the SEC on November 8, 2011.*
10.11	Synageva BioPharma Corp. 2005 Stock Plan, as amended, incorporated by reference to the Company's Registration Statement on Form S-8 filed with the SEC on December 7, 2012.*
10.12	Synageva BioPharma Corp. 2005 Stock Plan-form of Option Agreement, incorporated by reference to the Company's Annual Report on Form 10-K filed with the SEC on March 22, 2013.*
10.13	Trimeris, Inc. 2007 Stock Incentive Plan, incorporated by reference to the Company's Definitive Proxy Statement on Schedule 14A filed with the SEC on March 16, 2010.*
10.14	Trimeris, Inc. 2007 Stock Incentive Plan—form of Option Agreement, incorporated by reference to the Company's Annual Report on Form 10-K, filed with the SEC on March 17, 2008.*
10.15	Employment Agreement between Synageva BioPharma Corp. and Sanj K. Patel, effective as of November 2, 2011, incorporated by reference to the Company's Current Report on Form 8-K filed with the SEC on November 10, 2011.*
10.16	Employment Agreement between Synageva BioPharma Corp. and Carsten Boess, effective as of November 2, 2011, incorporated by reference to the Company's Current Report on Form 8-K filed with the SEC on November 10, 2011.*
10.18	Employment Agreement between Synageva BioPharma Corp. and Anthony Quinn, effective as of November 2, 2011, incorporated by reference to the Company's Current Report on Form 8-K filed with the SEC on November 10, 2011.*
10.19	Employment Agreement between Synageva BioPharma Corp. and Glen Williams, effective as of September 24, 2012.*
10.20	Lease, between Abbey BioPharma Corp. (f/k/a Synageva BioPharma Corp.) and One Ledgemont LLC, dated April 8, 2010, incorporated by reference to the Company's Quarterly Report on Form 10-Q filed with the SEC on November 14, 2011.
10.21	First Amendment to Lease Agreement dated November 29, 2011 between Synageva BioPharma Corp. and One Ledgemont LLC, incorporated by reference to the Company's Annual Report on Form 10-K filed with the SEC on March 22, 2013.
10.22	Lease Agreement between Synageva BioPharma Corp. and Barrett Investment Properties, LLC, dated January 23, 2012, incorporated by reference to the Company's Annual Report on Form 10-K filed with the SEC on March 22, 2012.
10.23	Rental Agreement, between Abbey BioPharma Corp. (a wholly-owned subsidiary of Synageva BioPharma Corp.) (f/k/a Synageva BioPharma Corp.) and the Board of Regents of the University System of Georgia, dated July 1, 2010, incorporated by reference to the Company's Quarterly Report on Form 10-Q filed with the SEC on November 14, 2011.
10.24	Biopharmaceutical Services Agreement between Synageva BioPharma Corp. and Cytovance Biologics, Inc., effective February 28, 2012, incorporated by reference to the Company's Quarterly Report on Form 10-Q filed with the SEC on May 9, 2012. †
10.25	Amendment to the Biopharmaceutical Services Agreement between Synageva BioPharma Corp. and Cytovance Biologics, Inc., effective April 27, 2012, incorporated by reference to the Company's Quarterly Report on Form 10-Q filed with the SEC on May 9, 2012.
10.26	Synageva BioPharma Corp. Employee Stock Purchase Plan, as amended, incorporated by reference to the Company's Registration Statement on Form S-8 filed with the SEC on December 7, 2012.*
21.1	Subsidiaries of the Registrant.

Exhibit Number	Description
23.1	Consent of PricewaterhouseCoopers LLP.
31.1	Rule 13a-14(a) Certification by Sanj K. Patel as Chief Executive Officer.
31.2	Rule 13a-14(a) Certification by Carsten Boess as Chief Financial Officer.
32.1	Section 1350 Certification by Sanj K. Patel as Chief Executive Officer and Carsten Boess as Chief Financial Officer.
101.INS	XBRL Instance Document.
101.SCH	XBRL Taxonomy Extension Schema Document.
101.CAL	XBRL Taxonomy Calculation Linkbase Document.
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document.
101.LAB	XBRL Taxonomy Label Linkbase Document.
101.PRE	XBRL Taxonomy Presentation Linkbase Document.

* Management contract or compensatory plan or arrangement required to be filed as an exhibit to this form pursuant to Item 15(a) of this report.

† Confidential treatment has been granted by the Securities and Exchange Commission for portions of this exhibit.

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Management Team

Sanj K. Patel
President & CEO

Carsten Boess
Chief Financial Officer

Anthony Quinn, MBChB (MD), PhD, FRCP
Chief Medical Officer

Glen Williams
Global Technical Operations and Quality

Michael Glynn
Global Commercial Operations

Mark Goldberg, MD
Global Medical and Regulatory Affairs

Mark Hayes, PhD
Regulatory Affairs

Sandra Rojas, MD
Clinical Research and Exploratory Development

Stephen Mahoney, JD, MBA
General Counsel and Corporate Development

Board of Directors

Felix J. Baker, PhD
Managing Partner, Baker Brothers Investments

Stephen R. Biggar, MD, PhD
Partner, Baker Brothers Investments

Stephen R. Davis
Former Chief Operating Officer,
Ardea Biosciences, Inc.

Thomas R. Malley
Former Portfolio Manager,
Janus Global Life Sciences Fund

Sanj K. Patel
President & Chief Executive Officer, Synageva

Barry Quart, PharmD
President and Chief Executive Officer,
Ardea Biosciences, Inc.

Thomas J. Tisch
Managing Partner of Four Partners

Peter Wirth
Former Executive Vice President of Genzyme
Advanced Leadership Fellow at Harvard University

Corporate Headquarters

Synageva BioPharma Corp.
128 Spring Street, Suite 520
Lexington, Massachusetts 02421

Legal Counsel

Ropes & Gray LLP
Boston, MA

Independent Auditors

PricewaterhouseCoopers LLP
Boston, MA

Transfer Agent and Registrar

Computershare Trust Company, N.A.
Providence, RI

Annual Meeting

The Annual Meeting of Stockholders will be held on June 6th 2013 at 4:30 p.m., EDT
Ropes & Gray
1211 Avenue of the Americas
New York, NY 10036-8704

Stock Information

The common stock of the company is traded on the NASDAQ Global Select Market System under the symbol GEVA.

Synageva BioPharma Corp. | 128 Spring Street, Suite 520
Lexington, Massachusetts 02421