ALEXION PHARMACEUTICALS INC
Form 10-K
February 19, 2013

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

FORM 10-K
x 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
For the transition period from to
Commission file number: 0-27756

ALEXION PHARMACEUTICALS, INC.
(Exact Name of Registrant as Specified in Its Charter)

Delaware	13-3648318
(State or Other Jurisdiction of Incorporation or Organization)	(I.R.S. Employer Identification No.)

352 Knotter Drive, Cheshire Connecticut 06410
(Address of Principal Executive Offices) (Zip Code)
203-272-2596
(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Act:	Common Stock, par value $0.0001
	Rights to Purchase Junior Participating
	Cumulative Preferred Stock, par value $0.0001
Name of each exchange on which registered:	The Nasdaq Stock Market LLC
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 x 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 x
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 x No ¨
Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Website, 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 x 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 x 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 Act). Yes ¨ No x

The aggregate market value of the Common Stock held by non-affiliates of the registrant, based upon the last sale price of the Common Stock reported on The Nasdaq Stock Market LLC on June 30, 2012, was $18,920,399,568.(1)

The number of shares of Common Stock outstanding as of February 11, 2013 was 195,209,249.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's Definitive Proxy Statement to be used in connection with its Annual Meeting of Stockholders to be held on May 06, 2013, are incorporated by reference into Part III of this report.

(1) Excludes 2,231,014 shares of common stock held by directors and executive officers at June 30, 2012. Exclusion of shares held by any person should not be construed to indicate that such person possesses the power, directly or indirectly, to direct or cause the direction of the management or policies of the registrant, or that such person is controlled by or under common control with the registrant.

PART I

Unless the context requires otherwise, references in this report to "Alexion", the “Company”, "we", "our" or "us" refer to Alexion Pharmaceuticals, Inc. and its subsidiaries.

Note Regarding Forward-Looking Statements

This Annual Report on Form 10-K contains forward-looking statements that have been made pursuant to the provisions of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements are based on our management's current expectations, estimates and projections about our industry and certain assumptions made by our management, and may include, but are not limited to, statements regarding the potential benefits and commercial potential of Soliris® (eculizumab) for its approved indications and any expanded uses, timing and effect of sales of Soliris in various markets worldwide, pricing for Soliris, level of insurance coverage and reimbursement for Soliris, level of future Soliris sales and collections, timing regarding development and regulatory approvals for additional indications or in additional territories for Soliris, the medical and commercial potential of additional indications for Soliris, costs, expenses and capital requirements, cash outflows, cash from operations, status of reimbursement, price approval and funding processes in various countries worldwide, progress in developing commercial infrastructure and interest about Soliris and our drug candidates in the patient, physician and payer communities, the safety and efficacy of Soliris and our product candidates, estimates of the potential markets and estimated commercialization dates for Soliris and our drug candidates around the world, sales and marketing plans, any changes in the current or anticipated market demand or medical need for Soliris or our drug candidates, status of our ongoing clinical trials for eculizumab, asfotase alfa and our other product candidates, commencement dates for new clinical trials, clinical trial results, evaluation of our clinical trial results by regulatory agencies, prospects for regulatory approval, need for additional research and testing, the uncertainties involved in the drug development process and manufacturing, performance and reliance on third party service providers, our future research and development activities, plans for acquired companies and programs, assessment of competitors and potential competitors, the outcome of challenges and opposition proceedings to our intellectual property, assertion or potential assertion by third parties that the manufacture, use or sale of Soliris infringes their intellectual property, estimates of the capacity of manufacturing and other facilities to support Soliris and our product candidates, potential costs resulting from product liability or other third party claims, the sufficiency of our existing capital resources and projected cash needs, assessment of impact of recent accounting pronouncements, declines in sovereign credit ratings or sovereign defaults in countries where we sell Soliris, delay of collection or reduction in reimbursement due to adverse economic conditions or changes in government and private insurer regulations and approaches to reimbursement, the short and long term effects of other government healthcare measures, and the effect of shifting foreign exchange rates. Words such as “anticipates”, “expects”, “intends”, “plans”, “believes”, “seeks”, “estimates”, variations of such words and similar expressions are intended to identify such forward-looking statements, although not all forward-looking statements contain these identifying words. These statements are not guarantees of future performance and are subject to certain risks, uncertainties, and assumptions that are difficult to predict; therefore, actual results may differ materially from those expressed or forecasted in any such forward-looking statements. Such risks and uncertainties include, but are not limited to, those discussed later in this report under the section entitled “Risk Factors”. Unless required by law, we undertake no obligation to update publicly any forward-looking statements, whether because of new information, future events or otherwise. However, readers should carefully review the risk factors set forth in this and other reports or documents we file from time to time with the Securities and Exchange Commission.

Item 1. BUSINESS.

(dollars and shares in thousands)

Overview

We are a biopharmaceutical company focused on serving patients with severe and ultra-rare disorders through the innovation, development and commercialization of life-transforming therapeutic products. Our marketed product Soliris® (eculizumab) is the first and only therapeutic approved for patients with two severe and ultra-rare disorders resulting from chronic uncontrolled activation of the complement component of the immune system: paroxysmal

nocturnal hemoglobinuria (PNH), a life-threatening and ultra-rare genetic blood disorder, and atypical hemolytic uremic syndrome (aHUS), a life-threatening and ultra-rare genetic disease. We are also evaluating additional potential indications for Soliris in severe and ultra-rare diseases in which chronic uncontrolled complement activation is the underlying mechanism, and we are progressing in various stages of development with additional biotechnology product candidates as treatments for patients with severe and ultra-rare disorders. We were incorporated in 1992 and began commercial sale of Soliris in 2007.

Soliris is designed to inhibit a specific aspect of the complement component of the immune system and thereby treat inflammation associated with chronic disorders in the therapeutic areas of hematology, nephrology, transplant rejection and neurology. Soliris is a humanized monoclonal antibody that effectively blocks terminal complement activity at the doses

currently prescribed. The initial indication for which we received approval for Soliris is PNH. PNH is an ultra-rare, debilitating and life-threatening, genetic deficiency blood disorder defined by chronic uncontrolled complement activation leading to the destruction of red blood cells (hemolysis). The chronic hemolysis in patients with PNH may be associated with life-threatening thromboses, recurrent pain, kidney disease, disabling fatigue, impaired quality of life, severe anemia, pulmonary hypertension, shortness of breath and intermittent episodes of dark-colored urine (hemoglobinuria).

Soliris was approved for the treatment of PNH by the U.S. Food and Drug Administration (FDA) and the European Commission (EC) in 2007 and by Japan's Ministry of Health, Labour and Welfare (MHLW) in 2010, and has been approved in several other territories. Additionally, Soliris has been granted orphan drug designation for the treatment of PNH in the United States, Europe, Japan and several other territories.

In September 2011, Soliris was approved by the FDA for the treatment of pediatric and adult patients with aHUS. aHUS is a genetic ultra-rare disease characterized by chronic uncontrolled complement activation and thrombotic microangiopathy, the formation of blood clots in small blood vessels throughout the body, causing a reduction in platelet count (thrombocytopenia) and life-threatening damage to the kidney, brain, heart and other vital organs. Also, in November 2011, the EC granted marketing authorization for Soliris to treat pediatric and adult patients with aHUS in Europe. The FDA and EC have granted Soliris orphan drug designation for the treatment of patients with aHUS.

Significant Developments

Enobia Acquisition

On February 7, 2012, we acquired Enobia Pharma Corp. (Enobia), a privately held clinical-stage biotechnology company based in Montreal, Canada and Cambridge, Massachusetts, in a transaction accounted for under the acquisition method of accounting for business combinations. Enobia's lead product candidate, asfotase alfa, is a human recombinant targeted alkaline phosphatase enzyme-replacement therapy for patients suffering with hypophosphatasia (HPP), an ultra-rare, life-threatening, genetic metabolic disease for which there are no approved treatments. We agreed to make an upfront payment of $610,000 subject to purchase price adjustments, which resulted in us making a cash payment of $623,876 for 100% of Enobia's capital stock. Additional contingent payments of up to an aggregate of $470,000 may be due upon reaching various regulatory and sales milestones. We financed the acquisition with a combination of existing cash and proceeds from our credit facility.

Credit Facilities

On February 7, 2012, we and our wholly-owned Swiss subsidiary, Alexion Pharma International Sàrl, entered into a Credit Agreement (Credit Agreement) with the lenders party thereto, Bank of America, N.A., as administrative agent, Merrill Lynch, Pierce, Fenner & Smith Incorporated and J.P. Morgan Securities LLC, as joint lead arrangers and joint book managers, JPMorgan Chase Bank, N.A., as syndication agent and RBS Citizens, National Association and Suntrust Bank as co-documentation agents. The Credit Agreement provides for a $240,000 senior secured term loan facility and a $200,000 senior secured revolving credit facility, which includes up to a $60,000 sublimit for letters of credit and a $10,000 sublimit for swingline loans. Alexion used the facilities to pay a portion of the consideration for the acquisition of Enobia. The facilities can also be used for working capital requirements, acquisitions and other general corporate purposes. Simultaneously with entering into the Credit Agreement, we terminated our Second Amended and Restated Credit Agreement, dated March 7, 2011.

Common Stock Offering

In May 2012, in conjunction with our addition into the S&P 500 Index, we completed the sale of 5,000 shares of our common stock in a public offering. The net proceeds from the sale of shares in the offering were $462,212.

Products and Development Programs

The human immune system defends the body from attack or invasion by infectious agents or pathogens. This is accomplished through a complex system of proteins and cells, primarily complement proteins, antibodies and white blood cells, each with a specialized function. Under normal circumstances, complement proteins, together with antibodies and white blood cells, act to protect the body by removing:

- harmful micro-organisms;
- cells containing foreign proteins known as antigens; and
- potential disease-causing combinations of antigens and antibodies known as immune complexes.

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When activated by certain stimuli, the immune system triggers a series of enzymatic and biochemical reactions called the complement cascade that results in an inflammatory response. This inflammatory response is one of the immune system's weapons against foreign pathogens or otherwise diseased tissue. However, under certain circumstances, the complement cascade may cause excessive or inappropriate activation, and/or an individual may be deficient in naturally occurring complement inhibitors, all of which may result in acute and chronic inflammatory conditions and damage to healthy tissues.

We focus our product development programs on life transforming therapeutics for diseases for which we believe current treatments are either non-existent or inadequate. Eculizumab is a humanized antibody known as a C5 terminal complement inhibitor (C5 Inhibitor), which is designed to selectively block the production of inflammation-causing proteins of the complement cascade. We believe that selective suppression of this immune response may provide a significant therapeutic advantage relative to existing therapies. In addition to PNH and aHUS, for which the use of eculizumab has been approved in the United States and Europe, we believe that C5 Inhibitors may be useful in the treatment of a variety of other serious diseases and conditions resulting from uncontrolled complement activation. Our programs, including investigator sponsored clinical programs, include the following:

Product	Development Area	Indication	Development Stage
Soliris (eculizumab)	Hematology	Paroxysmal Nocturnal Hemoglobinuria (PNH)	Commercial
		PNH Registry	Phase IV
		PNH Pediatric Trial	Phase II
		Cold Agglutinin Disease (CAD)*	Phase II
	Hematology/Nephrology	Atypical Hemolytic Uremic Syndrome (aHUS)	Commercial
		aHUS Trials	Phase IV
		aHUS Registry	Phase IV
	Nephrology	STEC-HUS (Shiga-toxin producing E. Coli Hemolytic Uremic Syndrome)	Phase II
		MPGN II/C3 Nephropathy*	Phase II
		Presensitized Renal Transplant - Living Donor	Phase II
		Presensitized Renal Transplant - Deceased Donor	Phase II
		Delayed Kidney Transplant Graft Function*	Phase II
		ABO Incompatible Renal Transplant*	Phase II
	Neurology	Neuromyelitis Optica (NMO)*	Phase II
		Myasthenia Gravis (MG)	Phase II
Asfotase alfa	Metabolic Disorders	Hypophosphatasia (HPP)	Phase II
cPMP	Metabolic Disorders	MoCD Type A	Preclinical
ALXN 1102/1103	Hematology	PNH	Phase I
ALXN 1007	Inflammatory Disorders		Phase I

*Investigator Initiated Trial

Our most advanced programs focus on two therapeutic areas: hematology and nephrology. We are also advancing our pipeline programs with a focus primarily on neurology and metabolic disorders.

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Soliris (eculizumab)

Soliris is designed to inhibit a specific aspect of the complement component of the immune system and thereby treat inflammation associated with chronic disorders in the therapeutic areas of hematology, nephrology including transplant rejection, and neurology. Soliris is a humanized antibody which, administered at the doses currently prescribed, generally blocks complement activity for one to two weeks after a single dose.

Soliris was approved for the treatment of PNH by the FDA and the EC in 2007, by Japan's MHLW in 2010 and has been approved in several other territories. Additionally, Soliris was granted orphan drug designation for the treatment of PNH in the United States, Europe, Japan and several other territories.

Soliris was approved for the treatment of aHUS by the FDA and the EC in 2011. Soliris was granted orphan drug designation for the treatment of aHUS in the United States and Europe.

Orphan drug designation generally entitles us to exclusivity for certain periods of time, subject to limited circumstances. However, if a competitive product that is the same as Soliris, as defined under the applicable regulations, is shown to be clinically superior to our product in the treatment of PNH, or if a competitive product is different from Soliris, as defined under the applicable regulations, the orphan drug exclusivity we have obtained may not restrict the approval of such competitive product.

Hematology

Paroxysmal Nocturnal Hemoglobinuria (PNH)

PNH is an ultra-rare, debilitating and life-threatening blood disorder in which an acquired genetic deficiency causes uncontrolled complement activation which leads to life-threatening complications. Patients with PNH have an acquired genetic deficiency in certain protective proteins on the surface of their blood cells, allowing their own complement system to attack and destroy these blood cells. Patients with PNH suffer from chronic complement activation of some of their blood cells and also hemolysis, or destruction of red blood cells caused by the C5 cleavage product C5b-9. This hemolysis is believed to lead to further clinical complications including thromboses, kidney disease, liver dysfunction, disabling fatigue, impaired quality of life, recurrent pain, shortness of breath, pulmonary hypertension, intermittent episodes of dark colored urine (hemoglobinuria), and anemia. Approximately one-half of the patients with PNH die from the disease within 10 to 15 years of diagnosis.

Our marketed product Soliris is the first and only therapy approved for the treatment of patients with PNH. We continue to work with researchers to expand the base of knowledge in PNH and the utility of Soliris to treat patients with PNH. Additionally, we are sponsoring multinational registries to gather information regarding the natural history of patients with PNH and the longer term outcomes during Soliris treatment.

Cold Agglutinin Disease (CAD)

We are aware that dosing is ongoing in an investigator-initiated Phase II study of eculizumab in patients for the treatment of CAD. CAD is a severe, ultra-rare complement-mediated autoimmune disease characterized by the presence of high concentrations of circulating complement-activating antibodies directed against red blood cells. As observed with PNH patients, CAD patients also suffer from the clinical consequences of severe hemolysis.

As blood is cooled during circulation through the distal parts of the arms and legs, specific antibodies bind to the red blood cells resulting in activation of the complement cascade and red blood cell lysis. Clinical manifestations of CAD include symptoms of chronic hemolysis such as fatigue, dyspnea, weakness, hemoglobinuria, kidney damage, pallor and jaundice. In the most severe cases, complications of progressive hemolysis or anemia may result in death. Current therapies, including cold avoidance, corticosteroids, immunosuppressive drugs, intravenous immunoglobulin G and chemotherapy agents are largely ineffective in controlling hemolysis in patients with CAD.

Hematology/Nephrology

Atypical Hemolytic Uremic Syndrome (aHUS)

aHUS is a chronic and life-threatening ultra-rare genetic disease in which uncontrolled complement activation causes blood clots in small blood vessels throughout the body (thrombotic microangiopathy, or TMA) leading to kidney failure, stroke, heart attack and death. Our marketed product Soliris is the first and only therapy approved for the treatment of patients with aHUS.

In patients with aHUS, deficiency of naturally occurring complement inhibitors causes uncontrolled complement activation which leads to systemic TMA, the formation of blood clots in small blood vessels throughout the body causing a reduction in platelet count (thrombocytopenia) and life-threatening damage to the kidney, brain, heart and other vital organs. The prognosis for patients with aHUS is generally poor. Approximately 70% of patients with the most common mutation experience chronic renal insufficiency, chronic dialysis, or death within one year after the first clinical manifestation of TMA. aHUS commonly recurs in patients who undergo renal transplantation and, depending upon the mutation, the disease can lead to loss of the transplanted kidney in up to approximately 90% of aHUS patients who undergo kidney transplantation.

Approximately 50% of patients diagnosed with aHUS have been identified to have genetic mutations in at least one of the complement control proteins or neutralizing autoantibodies to complement regulatory factors, which can lead to uncontrolled complement activation. Excessive complement activation may contribute to the blood vessel inflammation and clotting by stimulating activation of white blood cells, platelets and the endothelial lining of blood vessels.

As a post marketing requirement, we have now completed enrollment in a prospective open-label trial in adult aHUS and, separately, enrollment has been completed in a prospective pediatric aHUS study.

Nephrology

Shiga-toxin producing E. Coli Hemolytic Uremic Syndrome (STEC-HUS)

STEC-HUS is a life-threatening, complement-mediated ultra-rare disorder that results from exposure to Enterohemorrhagic E.Coli, (EHEC). Our STEC-HUS development program was initiated in connection with the widespread outbreak of EHEC in Germany in May and June 2011. Many EHEC patients rapidly progressed to STEC-HUS during this outbreak. As in several other conditions with severe and uncontrolled complement activation, including aHUS, complement activation in STEC-HUS results in TMA. Although aHUS and STEC-HUS exhibit similar life-threatening TMA manifestations, aHUS and STEC-HUS are different disorders. aHUS is a chronic genetic disease of uncontrolled complement activation, while STEC-HUS is not genetic and follows an isolated episode of infection. STEC-HUS is an ultra-rare disorder, comprising only a small sub-set of the already rare population of patients with EHEC. Following an authorization by the Paul-Ehrlich-Institut, Germany's health care regulatory body for biologics, and an access program for patients initiated in May 2011, we initiated an open-label clinical trial to investigate eculizumab as a treatment for patients with STEC-HUS. Enrollment in this trial has been completed. The FDA and the EC have each granted orphan designation for eculizumab as a treatment for patients with STEC-HUS.

MPGN II/C3 Nephropathy

We are aware that independent investigators have completed enrollment in studies aimed at evaluating eculizumab in patients with membrano-proliferative glomerulonephritis (MPGN II or dense deposit disease) as well as patients with a similar disease referred to as C3 nephropathy. MGPN II and C3 nephropathy are ultra-rare forms of glomerulonephritis, associated with genetic mutations in complement inhibitor genes leading to sustained uncontrolled complement activation and inflammation. Clinically, this disease is characterized by the onset of severe proteinuria (excess protein in the urine), often accompanied by nephrotic syndrome which is refractory to immunosuppressant therapy. In most cases, the disease progresses to chronic renal failure, requiring dialysis and renal transplantation.

Acute Humoral Rejection (AHR) in Presensitized Kidney Transplant Patients

Patients undergoing solid organ transplantation may experience severe AHR in the early post-transplant period. For example, in a patient undergoing a kidney transplant this may be characterized by the acute onset of renal dysfunction and rapid progression to destruction of the transplanted kidney.

AHR results when antibodies in the transplant recipient vigorously attack the blood vessels of the donor kidney. During severe AHR, these donor specific antibodies bind to the blood vessel lining of the donor organ and initiate activation of the complement cascade, resulting in severe blood vessel inflammation and clotting. Administration of a C5 inhibitor in animal models of AHR inhibits complement activation, tissue damage and transplant rejection.

We initiated enrollment in a multi-national, multi-site controlled clinical trial of eculizumab in presensitized renal transplant patients at elevated risk for AHR who will receive living donor grafts, and we have initiated enrollment in a multi-national, multi-site controlled clinical trial of eculizumab in presensitized renal transplant patients at elevated risk for AHR who will receive deceased donor grafts. We are also aware that an independent investigator has started enrolling patients in a clinical trial to evaluate eculizumab in kidney transplant patients sensitized to their donor kidney due to an ABO blood group mismatch between donor and recipient.

Delayed Kidney Transplant Graft Function

We are aware that dosing is ongoing in an investigator-initiated Phase II study of eculizumab in patients at elevated risk for delayed graft function (DGF) following kidney transplant.

DGF is the term used to describe the failure of a kidney or other organs to function immediately after transplantation due to ischemia-reperfusion and immunological injury. After kidney transplantation, DGF can be considered a form of acute kidney injury post-transplantation and is an important complication of kidney transplantation. The frequency of DGF can be as high as 50% in some kidney transplant settings. DGF complicates post-transplant management, increases morbidity and prolongs patient hospitalization. In addition to the acute kidney injury, DGF predisposes the transplanted kidney to both acute and chronic rejection and increases the risk of chronic allograft nephropathy and premature graft loss. Studies have indicated that activation of the complement cascade may be a key early event required for the development of DGF following kidney transplant. There are currently no accepted or approved therapies for prevention or treatment of DGF following kidney transplantation.

Neurology

Neuromyelitis Optica (NMO)

NMO is a severe and ultra-rare autoimmune disease of the central nervous system (CNS) that primarily affects the optic nerves and spinal cord. Individuals with NMO develop optic neuritis, which causes pain in the eye and vision loss, and transverse myelitis, which causes weakness, numbness, and sometimes paralysis of the arms and legs, weakness or paralysis of respiratory muscles sometimes leading to respiratory failure, along with sensory disturbances and loss of bladder and bowel control.

Preliminary data from the investigator-initiated Phase II clinical trial of eculizumab in severe and relapsing NMO patients was presented to the American Neurological Association (ANA) meeting in October 2012. The study was reported to have achieved its primary efficacy endpoint with a high degree of clinical and statistical significance and several key secondary endpoints were also achieved.

Myasthenia Gravis (MG)

MG is an ultra-rare autoimmune syndrome characterized by complement activation leading to the failure of neuromuscular transmission. Patients with MG initially experience weakness in their ocular, or eye muscles, and the disease typically progresses to head, spinal, limb and respiratory muscles. Symptoms can include drooping eyelids, blurred vision, slurred speech, difficulty chewing or swallowing, weakness in the arms and legs and difficulty breathing. In an experimental animal model of MG, administration of a C5 Inhibitor was found to prevent experimentally acquired MG and to inhibit disease progression.

Preliminary data from a Phase II trial evaluating the safety and efficacy of eculizumab in patients with severe, refractory MG demonstrated an encouraging disease improvement signal and was presented at the Myasthenia Gravis Foundation Annual Meeting in September 2011. We continue to work with investigators to design the next clinical trial to evaluate eculizumab as a treatment for patients with severe and refractory MG.

Asfotase Alfa

Hypophosphatasia (HPP)

HPP is an ultra-rare, genetic, and life-threatening metabolic disease characterized by impaired phosphate and calcium regulation, leading to progressive damage to multiple vital organs including destruction and deformity of bones, profound muscle weakness, seizures, impaired renal function, and respiratory failure. The severe manifestations of the genetic deficiency in HPP affect people of all ages, and approximately 50% of infants with the disease do not survive past one year of age. HPP is caused by mutations in the gene encoding the enzyme Tissue Nonspecific Alkaline Phosphatase. This enzyme normally breaks

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down metabolic substrates such as inorganic pyrophosphate and pyridoxal phosphate.
Asfotase alfa, a targeted enzyme replacement therapy in Phase II clinical trials for patients with HPP, is designed to directly address the morbidities and mortality of HPP by targeting alkaline phosphatase directly to the deficient tissue. In this way, asfotase alfa is designed to normalize the genetically defective metabolic process and prevent or reverse the severe, crippling and life-threatening complications of dysregulated mineral metabolism in patients with HPP. Initial studies with asfotase alfa in HPP patients indicate that the treatment significantly decreases the levels of targeted metabolic substrates. We have initiated a natural history study in infants with HPP and are currently dosing patients in a separate global trial of severe infant HPP patients. We acquired asfotase alfa in February 2012 in connection with our acquisition of Enobia.

cPMP
Molybdenum Cofactor Deficiency (MoCD) Disease Type A (MoCD Type A)
MoCD Type A is a rare metabolic disorder characterized by severe and rapidly progressive neurologic damage and death in newborns. MoCD Type A results from a genetic deficiency in cyclic Pyranopterin Monophosphate (cPMP), a molecule that enables production of certain enzymes, the absence of which allows neurotoxic sulfite to accumulate in the brain. To date, there is no approved therapy available for MoCD Type A. There has been some early clinical experience with the cPMP replacement therapy in a small number of children with MoCD Type A. We are currently conducting pre-Investigational New Drug (IND) toxicology studies with cPMP replacement therapy.

ALXN 1102/1103
ALXN 1102/1103 is a novel alternative pathway complement inhibitor with a mechanism of action unique from Soliris. ALXN 1102 is currently being investigated in a Phase I single dose, dose escalating safety and pharmacology study. ALXN 1103 is being dosed in the same Phase I trial as a subcutaneous formulation.

ALXN 1007
ALXN 1007 is a novel humanized antibody designed to target rare and severe inflammatory disorders and is a product of our proprietary antibody discovery technologies. ALXN 1007 is currently being investigated in a Phase I single dose, dose escalating safety and pharmacology study in healthy volunteers.
Manufacturing
We currently rely on two manufacturing facilities, Alexion's Rhode Island manufacturing facility (ARIMF) and Lonza Group AG and its affiliates (Lonza), to produce commercial and clinical bulk quantities of Soliris, and we rely on Lonza for clinical quantities of asfotase alfa. We produce our clinical and preclinical quantities of our other product candidates at ARIMF. We also depend on a limited number of third party providers for other services with respect to our clinical and commercial requirements, including product finishing, packaging, vialing and labeling.
We have various agreements with Lonza, with remaining total commitments of approximately $169,000 through 2018. Such commitments may be canceled only in limited circumstances. If we terminate certain supply agreements with Lonza without cause, we will be required to pay for product scheduled for manufacture under our arrangement. Under an existing arrangement with Lonza, we also pay Lonza a royalty on sales of Soliris manufactured at ARIMF.
Sales and Marketing
We have established a commercial organization to support current and future sales of Soliris in the United States, in the major markets in European Union, Japan, Asia Pacific countries, and other territories. Our sales force for Soliris is small compared to that of other drugs with similar gross revenues; however, we believe that a relatively smaller sales force is appropriate to effectively market Soliris due to the limited PNH and aHUS patient populations. If we receive regulatory approval in new territories, we may expand our own commercial organizations in such territories and market and sell Soliris through our own sales force in these territories. However, we will evaluate each jurisdiction on a country-by-country basis, and it is possible that we will promote Soliris in collaboration with marketing partners or rely on relationships with one or more companies with established distribution systems and direct sales forces in certain countries.

Customers

In the United States, our customers are primarily specialty distributors and specialty pharmacies which supply physician office clinics, hospital outpatient clinics, infusion clinics or home health care providers. We also sell Soliris to government agencies. Outside the United States, our customers are primarily hospitals, hospital buying groups, pharmacies, other health care providers and distributors.

During 2012, sales to our two largest customers accounted for 21% and 12% of our Soliris net product sales. During 2011, sales to our two largest customers accounted for 19% and 12% of our Soliris net product sales.

Because of factors such as the pricing of Soliris, the limited number of patients, the short period from product sale to patient infusion and the lack of contractual return rights, Soliris customers generally carry limited inventory. We also monitor inventory within our sales channels to determine whether deferrals are appropriate based on factors such as inventory levels, contractual terms and financial strength of distributors.

Please also see "Management’s Discussion and Analysis – Net Product Sales," and Note 15 of the Consolidated Financial Statements included in this Annual Report on Form 10-K, for financial information about geographic areas.

Patents and Proprietary Rights

Patents and other proprietary rights are important to our business. Our practice is to file patent applications to protect technology, inventions and improvements to our technologies that are considered important to the development of our business. We also rely upon our trade secrets, know-how, and continuing technological innovations, as well as patents that we have licensed or may license from other parties, to develop and maintain our competitive position.

We have filed several U.S. patent applications and international counterparts of certain of these applications. In addition, we have in-licensed several additional U.S. and international patents and patent applications. As of December 31, 2012, we owned or in-licensed 89 U.S. patents and 45 U.S. patent applications. These patents and patent applications relate to technologies or products in the C5 Inhibitor program, high throughput screening, vectors, cancer, recombinant antibodies, bone delivery conjugates, natriuretic peptides, human molybdenum cofactor deficiency, targeted complement inhibitors, and other technologies. As of December 31, 2012, we owned or in-licensed 93 foreign patents and 370 pending foreign patent applications.

With respect to Soliris, we have an issued U.S. patent that will expire in 2021, taking into account patent term extension. In Europe, a corresponding issued patent covering Soliris expires in 2015 and, taking into account the Supplementary Protection Certificates (SPC) that we have filed for in various European countries, exclusivity for Soliris will extend into 2020 in those countries in which an SPC is granted. Patents covering Soliris in Japan and other countries expire between 2015 and 2020. We owe royalties and other fees to owners of one or more patents in connection with the manufacture and sale of Soliris for PNH and aHUS, and we may owe royalties and fees to other third parties with respect to any previous or future manufacture and sale of Soliris and our product candidates.

We also own U.S. and foreign patents and patent applications for our product candidates other than Soliris. At present, each such product candidate is in early stage development, and it is not known whether any such product candidate will ever be approved for human use and sale.

Our success will depend in part on our ability to obtain and maintain U.S. and international patent protection for our products and development programs, to preserve our trade secrets and proprietary rights, and to operate without infringing on the proprietary rights of third parties or having third parties circumvent our rights. Because of the length of time and expense associated with bringing new products through development and regulatory approval to the marketplace, the health care industry has traditionally placed considerable importance on obtaining patent and trade secret protection for significant new technologies, products and processes. Significant legal issues remain to be resolved as to the extent and scope of patent protection for biotechnology products and processes in the United States and other important markets outside of the United States. Accordingly, there can be no assurance that patent applications owned or licensed by us will issue as patents, or that any issued patents will afford meaningful protection against competitors. Moreover, once issued, patents are subject to challenge through both administrative and judicial proceedings in the United States and in foreign jurisdictions. Such proceedings include interference proceedings before the U.S. Patent and Trademark Office and opposition proceedings before the European Patent Office. Litigation may be required to enforce our intellectual property rights. Any litigation or administrative proceeding may result in a significant commitment of our resources and, depending on outcome, may adversely affect the validity and scope of certain of our patent or other proprietary rights.

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We are aware of broad patents owned by others relating to the manufacture, use and sale of recombinant humanized antibodies, recombinant human antibodies, and recombinant human single chain antibodies. Soliris and many of our product candidates are either genetically engineered antibodies, including recombinant humanized antibodies, recombinant human antibodies, or recombinant human single chain antibodies. We have received notices from the owners of patents claiming that their patents may be infringed by the development, manufacture or sale of Soliris or some of our drug candidates. For example, in January 2011, Novartis Vaccines & Diagnostics, Inc. (Novartis) filed a civil action in the U.S. District Court for the District of Delaware alleging that the manufacture of Soliris infringes their U.S. patent number 5,688,688. We are also aware of other patents owned by third parties that might be claimed by such third parties to be infringed by the development and commercialization of Soliris or some of our product candidates. In respect to some of these patents, we have obtained licenses, or expect to obtain licenses. However, with regard to such other patents, we have determined in our judgment that:

- our products do not infringe the patents;
- the patents are not valid; or
- we have identified and are testing various modifications that we believe should not infringe the patents and which should permit commercialization of our product candidates.

If any patent holder successfully challenges our judgment that our products do not infringe their patents or that their patents are invalid, we could be required to pay costly damages or to obtain a license to sell or develop our drugs. A required license may be costly or may not be available on acceptable terms, if at all. A costly license, or inability to obtain a necessary license, could materially and adversely affect our ability to commercialize our products, including Soliris.

On a quarterly basis, we review the status of each significant claim or legal proceeding and assess its potential financial exposure. If the potential loss from any claim, asserted or unasserted, or legal proceeding is considered probable and the amount can be reasonably estimated, we accrue a liability for the estimated loss. Because of uncertainties related to claims and litigation, accruals are based on our best estimates based on available information. On a periodic basis, as additional information becomes available, or based on specific events such as the outcome of litigation or settlement of claims, we may reassess the potential liability related to these matters and may revise these estimates, which could result in a material adverse adjustment to our operating results.

It is our policy to require our employees, consultants and parties to collaborative agreements to execute confidentiality agreements upon the commencement of employment or consulting relationships or collaborations with us. These agreements generally provide that all confidential information developed or made known during the course of the relationship with us is to be kept confidential and not to be disclosed to third parties except in specific circumstances. In the case of employees, the agreements also provide that all inventions resulting from work performed for us, utilizing our property or relating to our business and conceived or completed by the individual during employment shall be our exclusive property to the extent permitted by applicable law.

License Agreements

In March 1996, we entered into a license agreement with the Medical Research Council (MRC) whereby MRC granted to us worldwide non-exclusive rights to certain patents related to the humanization and production of monoclonal antibodies. We pay MRC royalties on a quarterly basis with respect to sales of Soliris. The royalty is payable until the expiration of the last patent covered by the license agreement, which is expected to be in 2015, except that royalties for sales in Canada will continue until January of 2017. MRC may terminate the license if we file for bankruptcy or become insolvent, or if we fail to perform our obligations under the agreement and such failure is not remedied within three months after delivery of notice. Under the agreement, we agreed to (a) make royalty payments with respect to sales of licensed products, (b) promote the sale of Soliris of good marketable quality, and (c) use reasonable endeavors to meet market demand for licensed products.

We are party to other license agreements related to the manufacture and sale of Soliris. Under an existing arrangement with Lonza, Lonza produces commercial and clinical bulk quantities of Soliris. We pay Lonza royalties on a quarterly basis with respect to sales of Soliris manufactured at ARIMF. We have various agreements with Lonza, with commitments through 2018. Such commitments may be canceled only in limited circumstances. If we terminate certain supply agreements with Lonza without cause, we will be required to pay for product scheduled for manufacture under our arrangement.

In October 2012, we entered into a settlement and non-exclusive license agreement with a third party. Under the terms of the agreement, we made an upfront payment of approximately $38,000 in the fourth quarter of 2012 and will pay royalties on sales of Soliris in accordance with the terms of the agreement.

Government Regulation

The preclinical studies and clinical testing, manufacture, labeling, storage, record keeping, advertising, promotion, export, and marketing, among other things, of our products and product candidates, including Soliris, are subject to extensive regulation by governmental authorities in the United States, the European Union and other territories. In the United States, 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. Soliris is regulated by the FDA as a biologic. Biologics require the submission of a Biologics License Application (BLA) and approval by the FDA prior to being marketed in the United States. 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 United States generally include:

(1) preclinical laboratory tests and animal tests;
(2) submission to the FDA of an IND for human clinical testing, which must become effective before human clinical trials may commence;
(3) adequate and well-controlled human clinical trials to establish the safety and efficacy of the product;
(4) submission to the FDA of a BLA or supplemental BLA;
(5) FDA pre-approval inspection of product manufacturers; and
(6) 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. We cannot assure you that submission of an IND will result in FDA authorization to commence clinical trials or that once commenced, other concerns will not arise.

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, sponsor or institutional review board 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 $1,500, 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 60 days following submission of the application. If found sufficiently complete, the FDA will “file” 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 and original efficacy supplements within six months and 90% of Standard applications and original efficacy supplements in ten 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 because the review process is often significantly extended by FDA requests for additional information or clarification. As part of its review, the FDA may refer the BLA to an advisory committee for evaluation and a recommendation as to whether the application should be approved. Although the FDA is not bound by the recommendation of an advisory committee, the agency usually has followed such recommendations.

Further, the outcome of the review, even if generally favorable, may not be an actual approval but instead a “complete response letter” communicating the FDA's decision not to approve the application, outlining the deficiencies in the BLA, and identifying what information and/or data (including additional pre-clinical or clinical data) is required before the application can be approved. Even if such additional information and data are submitted, the FDA may decide that the BLA still does not meet the standards for approval. Data from clinical trials are not always conclusive and the FDA may interpret data differently than us.

Before approving a BLA, the FDA may inspect the facilities at which the product is manufactured and will not approve the product unless current Good Manufacturing Practices (cGMP) compliance 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, and may require that additional studies be conducted following approval as a condition of the approval, may impose restrictions and conditions on product distribution, prescribing or dispensing in the form of a Risk Evaluation Mitigation Strategies (REMS), or otherwise limit the scope of any approval. To market a product for other indicated uses, or to make 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 may be 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.

In 2010, the Biologics Price Competition and Innovation Act (BPCI) was enacted, creating a statutory pathway for licensure, or approval, of biological products that are biosimilar to, and possibly interchangeable with, reference 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 United States. This means that the FDA may not approve a biosimilars application until 12 years after the date of approval of the original reference biological product date (with a potential six-month extension of exclusivity if certain pediatric studies are conducted and the results reported to FDA), although a biosimilar application may be submitted four years after the date of approval of the original reference biological product.

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 small molecule 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 February of 2012, the FDA published draft guidance documents on biosimilar product development. The guidance defines a biosimilar as a biological product that is highly similar to an already approved biological product, notwithstanding minor differences in clinically inactive components, and for which there are no clinically meaningful differences between the biosimilar and the approved biological product in terms of the safety, purity, and potency. Under this proposed approval pathway, biological products are approved based on demonstrating they are biosimilar to, or interchangeable with, a biological product that is already approved by the FDA, which is called a reference product. The approval of a biologic product biosimilar to one of our products could have a material impact on our business because 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. If ongoing regulatory requirements are not satisfied or if safety problems occur after the product reaches the market, the FDA may at any time withdraw its product approval or take actions that would suspend marketing. 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. Failure to comply with applicable cGMP requirements and conditions of product approval may lead the FDA to seek sanctions, including fines,

civil penalties, injunctions, suspension of manufacturing operations, operating restrictions, withdrawal of FDA approval, seizure or recall of products, and criminal prosecution. Accordingly, manufacturers must continue to spend time, money and effort to maintain cGMP compliance.

The FDA and other federal regulatory agencies also closely regulate the promotion of drugs through, among other things, standards and regulations for direct-to-consumer advertising, communications regarding unapproved uses, industry-sponsored scientific and educational activities, and promotional activities involving the Internet. A product cannot be commercially promoted before it is approved. After approval, product promotion can include only those claims relating to safety and effectiveness that are consistent with the labeling approved by the FDA. Healthcare providers are permitted to prescribe drugs for “off-label” uses - that is, uses not approved by the FDA and therefore not described in the drug's labeling - because the FDA does not regulate the practice of medicine. However, FDA regulations impose stringent restrictions on manufacturers' communications regarding off-label uses. Broadly speaking, a manufacturer may not promote a drug for off-label use, but may engage in non-promotional, balanced communication regarding off-label use under certain conditions. Failure to comply with applicable FDA requirements and restrictions in this area may subject a company to adverse publicity and enforcement action by the FDA, the Department of Justice, or the Office of the Inspector General of the Department of Health and Human Services, as well as state authorities. This could subject a company to a range of penalties that could have a significant commercial impact, including civil and criminal fines and agreements that materially restrict the manner in which a company promotes or distributes drug products.

Orphan Drug Designation in the United States, the European Union and other foreign jurisdictions

Under the Orphan Drug Act, the FDA may grant orphan drug designation to drugs intended to treat a “rare disease or condition,” which generally is a disease or condition that affects fewer than 200,000 individuals in the United States. Orphan drug designation must be requested before submitting a BLA or supplemental BLA. After the FDA grants orphan drug designation, the generic identity of the therapeutic agent and its potential orphan use are publicly disclosed by the FDA. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. If a product which has an orphan drug designation subsequently receives the first FDA approval for the indication for which it has such designation, the product is entitled to an orphan exclusivity period, in which the FDA may not approve any other applications to market the same drug for the same indication for seven years, except in limited circumstances.

Medicinal products used to treat life-threatening or chronically debilitating conditions that affect no more than five in 10,000 people in the European Union and medicinal products which, for economic reasons, would be unlikely to be developed without incentives may be granted an orphan designation in the European Union. The application for orphan designation is submitted to the European Medicines Agency (EMA) before an application is made for marketing authorization. Once authorized, orphan medicinal products are entitled to ten years of market exclusivity. During this ten year period, with a limited number of exceptions, neither the competent authorities of the European Union member states nor the EMA are permitted to accept applications or grant marketing authorization for other similar medicinal products with the same orphan indication. However, marketing authorization may be granted to a similar medicinal product with the same orphan indication during the ten year period with the consent of the marketing authorization holder for the original orphan medicinal product or if the manufacturer of the original orphan medicinal product is unable to supply sufficient quantities. Marketing authorization may also be granted to a similar medicinal product with the same orphan indication if this product is safer, more effective or otherwise clinically superior to the original orphan medicinal product.

Soliris has received orphan drug designation for the treatment of PNH in the United States and in several other territories, including the European Union, Australia and South Korea, which provides certain regulatory and filing fee advantages, including market exclusivity, except in limited circumstances, for several years after approval. In 2009, Soliris also received orphan drug designation for the treatment of patients with aHUS in the United States and the European Union. Soliris received marketing authorization for the treatment of PNH in 2007 and for aHUS in 2011. In 2008, asfotase alfa received orphan drug designation for the treatment of patients with hypophosphatasia in the United States and the European Union.

Foreign Regulation

In addition to regulations in the United States, 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.

For example, in the European Union, applications for marketing authorization are based on the ICH Common Technical Document and must include a demonstration that the applicant has conducted studies with the medicinal product in the pediatric population as provided by a Pediatric Investigation Plan (PIP) approved by the Pediatric Committee of the EMA. Alternatively, confirmation that the applicant has been granted a waiver or deferral for the conduct of these studies must be provided.

Under the European Union regulatory system, we may submit applications for marketing authorizations either under a centralized or decentralized marketing authorization procedure. The centralized procedure provides for the grant of a single marketing authorization for a medicinal product by the EC on the basis of an opinion by the EMA. A centralized marketing authorization is valid for all European Union member states and three of the four EFTA States (Iceland, Liechtenstein and Norway). The decentralized procedure and the mutual recognition procedure apply between European Union member states. The decentralized marketing authorization procedure involves the submission of an application for marketing authorization to the approval authority of all European Union member states in which the product is to be marketed. One national approval authority, selected by the applicant, assesses the application for marketing authorization. The authorities of the other European Union member states subsequently decide whether to grant or refuse marketing authorization for their territory on the basis of this assessment. The mutual recognition procedure provides for mutual recognition of marketing authorizations delivered by the national approval authorities of European Union member states by the approval authorities of other European Union member states. The holder of a national marketing authorization may submit an application to the approval authority of a European Union member state requesting that this authority recognize the marketing authorization delivered by the approval authority of another European Union member state.

Similarly to the U.S., both marketing authorization holders and manufacturers of medicinal products are subject to comprehensive regulatory oversight by the EMA and the competent authorities of the individual European Union member states both before and after grant of the manufacturing and marketing authorizations. This includes control of compliance by these entities with European Union cGMP rules.

Failure to comply with European Union laws and the related national laws of individual European Union member states governing the conduct of clinical trials, marketing authorization of medicinal products and marketing of such products, both before and after grant of marketing authorization, may result in administrative, civil or criminal penalties. These penalties could include refusal to authorize the conduct of clinical trials, refusal to grant marketing authorization, product withdrawals and recalls, product seizures, suspension or withdrawal of the marketing authorization, fines and criminal penalties.

We submitted our Marketing Authorization Application for Soliris for the treatment of PNH and aHUS to the EMA using the centralized marketing authorization procedure.

The European Union has had an established regulatory pathway for biosimilars since 2005 and has approved several biosimilar products. The approval of a biologic product biosimilar to one of our products marketed in the European Union could have a material impact on our business. The biologic product biosimilar may be significantly less costly to bring to market and may be priced significantly lower than our products.

Reimbursement

Sales of pharmaceutical products depend in significant part on the coverage and reimbursement policies of government programs, including Medicare and Medicaid in the United States, and other third party payers. All third party payers are sensitive to the cost of drugs and have taken efforts to control those costs and will continue to do so in the future. Private health insurance plans may restrict coverage of some products by using payer formularies under which only selected drugs are covered, variable co-payments that make drugs that are not preferred by the payer more expensive for patients, and by using utilization management controls, such as requirements for prior authorization or prior failure on another type of treatment. Payers may especially impose these obstacles to coverage for higher priced drugs, and consequently Soliris may be subject to payer-driven restrictions.

Medicare is a government insurance program for U.S. citizens age 65 and older and younger individuals with disabilities. For Medicare beneficiaries, Soliris may be covered under Part B, which reimburses physicians and

hospital outpatient departments for furnishing drugs, or Part A, which is the inpatient hospital benefit. Under Part B, reimbursement is based on average sales price (ASP). Manufacturers must report ASP information to the Centers for Medicare and Medicaid Services (CMS), on a quarterly basis. In the physician office setting, the reimbursement rate for drugs and biologics is ASP + 6%. For 2013, in the hospital outpatient department setting, the reimbursement rate for drugs and biologics is ASP + 6%. This reimbursement rate may decrease in the future. In both settings, the reimbursement rate is updated quarterly based on the

submission of new ASP information. Hospital inpatient services are covered under Medicare Part A. Hospitals typically receive a single payment for an inpatient stay depending on the Medicare Severity Diagnosis Related Group (MS-DRG) to which the inpatient stay is assigned. The MS-DRG for a hospital inpatient stay varies based on the patient's condition. In general, hospitals do not receive separate payment for drugs and biologicals administered to patients during a hospital stay.

Medicaid is a government insurance program for certain low-income individuals, including children. It is jointly funded by the federal and state governments and it is administered by the states within parameters established by the federal government. Coverage and reimbursement for drugs and biologics thus varies by state. Drugs and biologics may be covered under the medical or pharmacy benefit. State Medicaid programs may impose utilization management controls, such as prior authorization, step therapy, or quantity limits on drugs and biologics. As a result of the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability of 2010 (collectively, the PPACA), many states are expanding their Medicaid programs. The manner in which this expansion occurs may affect beneficiary access to prescription drugs and the types of utilization management controls that apply.

In addition, in some foreign countries, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country. For example, in the European Union the sole legal instrument at the European Union level governing the pricing and reimbursement of medicinal products is Council Directive 89/105/EEC (the Price Transparency Directive). The aim of the Price Transparency Directive is to ensure that pricing and reimbursement mechanisms established in European Union member states are transparent and objective, do not hinder the free movement and trade of medicinal products in the European Union and do not hinder, prevent or distort competition on the market. The Price Transparency Directive does not, however, provide any guidance concerning the specific criteria on the basis of which pricing and reimbursement decisions are to be made in individual European Union member states. Neither does it have any direct consequence for pricing or levels of reimbursement in individual European Union member states. The national authorities of the individual European Union member states are free to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices and/or reimbursement of medicinal products for human use. Individual European Union member states adopt policies according to which a specific price or level of reimbursement is approved for the medicinal product. Other European Union member states adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market, including volume-based arrangements and reference pricing mechanisms.

Health Technology Assessment (HTA) of medicinal products is becoming an increasingly common part of the pricing and reimbursement procedures in some European Union member states. These countries include the United Kingdom, France, Germany and Sweden. The HTA process in the European Union member states is governed by the national laws of these countries. HTA is the procedure according to which the assessment of the public health impact, therapeutic impact and the economic and societal impact of the use of a given medicinal product in the national healthcare systems of the individual country is conducted. HTA generally focuses on the clinical efficacy and effectiveness, safety, cost, and cost-effectiveness of individual medicinal products as well as their potential implications for the healthcare system. Those elements of medicinal products are compared with other treatment options available on the market.

The outcome of HTA regarding specific medicinal products will often influence the pricing and reimbursement status granted to these medicinal products by the competent authorities of individual European Union member states. The extent to which pricing and reimbursement decisions are influenced by the HTA of a specific medicinal product vary between the European Union member states.

In 2011, Directive 2011/24/EU was adopted at the European Union level. This Directive concerns the application of patients' rights in cross-border healthcare. The Directive is intended to establish rules for facilitating access to safe and high-quality cross-border healthcare in the European Union. It also provides for the establishment of a voluntary network of national authorities or bodies responsible for HTA in the individual European Union member states. The purpose of the network is to facilitate and support the exchange of scientific information concerning HTAs. This could lead to harmonization of the criteria taken into account in the conduct of HTA and their impact on pricing and reimbursement decisions between European Union member states.

On a continuous basis, we engage with appropriate authorities on the operational, reimbursement, price approval and funding processes that are separately required in each country.

Competition

There are currently no approved drugs other than Soliris for the treatment of PNH and aHUS. However, many companies, including major pharmaceutical and chemical companies as well as specialized biotechnology companies, are engaged in activities similar to our activities. Universities, governmental agencies and other public and private research organizations also conduct research and may market commercial products on their own or through joint ventures. Many of these entities may have:

- substantially greater financial and other resources;
- larger research and development staffs;
- lower labor costs; and/or
- more extensive marketing and manufacturing organizations.

Many of these companies and organizations have significant experience in preclinical testing, human clinical trials, product manufacturing, marketing, sales and distribution and other regulatory approval and commercial procedures. They may also have a greater number of significant patents and greater legal resources to seek remedies for cases of alleged infringement of their patents by us to block, delay or compromise our own drug development process.

We compete with large pharmaceutical companies that produce and market synthetic compounds and with specialized biotechnology firms in the United States, Europe and in other countries and regions, as well as a growing number of large pharmaceutical companies that are applying biotechnology to their operations. A number of biotechnology and pharmaceutical companies are developing new products for the treatment of the same diseases being targeted by us; in some instances, these products have already entered clinical trials or are already being marketed. Other companies are engaged in research and development based on complement proteins.

Several companies have either publicly announced their intentions to develop drugs which target the inflammatory effects of complement in the immune system or have had programs to develop complement inhibitor therapies. We believe that our potential C5 Inhibitors differ substantially from those of our potential competitors due to our compounds' demonstrated ability to specifically intervene in the complement cascade, for potentially prolonged periods of time. We believe this action to be the optimal point so that the disease-causing actions of complement proteins are inhibited, while the normal disease-preventing functions of complement proteins and other aspects of immune function remain intact.

Employees

As of December 31, 2012, we had 1,373 full-time, world-wide employees, of which 574 were engaged in research, product development, manufacturing, and clinical development, 552 in sales and marketing, and 247 in administration, human resources, information technology and finance. Our U.S. employees are not represented by any collective bargaining unit, and we regard the relationships with all our employees as satisfactory.

EXECUTIVE OFFICERS OF THE COMPANY

The executive officers of the Company and their respective ages and positions as of February 11, 2013 are as follows:

Name	Age	Position with Alexion
Leonard Bell, M.D.	54	Chief Executive Officer, Treasurer and Director
Stephen P. Squinto, Ph.D.	56	Executive Vice President and Head of Research and Development
David L. Hallal	46	Executive Vice President and Chief Commercial Officer
Vikas Sinha, M.B.A., C.A., C.P.A.	49	Executive Vice President and Chief Financial Officer
Clare Carmichael	53	Senior Vice President and Chief Human Resources Officer
John B. Moriarty, J.D.	45	Senior Vice President and General Counsel
Frank J. Wright	65	Senior Vice President and President of Alexion Pharma International Sàrl

Leonard Bell, M.D. is the principal founder of Alexion and has been a director of Alexion since February 1992 and the Company's Chief Executive Officer since January 1992. From 1991 to 1992, Dr. Bell was an Assistant Professor of Medicine and Pathology and co-Director of the program in Vascular Biology at the Yale University School of Medicine. From 1990 to 1992, Dr. Bell was an attending physician at the Yale-New Haven Hospital and an Assistant Professor in the Department of

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Internal Medicine at the Yale University School of Medicine. Dr. Bell was a recipient of the Physician Scientist Award from the National Institutes of Health and Grant-in-Aid from the American Heart Association as well as various honors and awards from academic and professional organizations. His work has resulted in more than 20 scientific publications and 9 patent applications. Dr. Bell received his A.B. from Brown University and M.D. from Yale University School of Medicine. Dr. Bell is currently an Adjunct Assistant Professor of Medicine and Pathology at the Yale University School of Medicine.

Stephen P. Squinto, Ph.D. is a founder of Alexion and has been Executive Vice President and Head of Research and Development since June 2007. He held the position of Executive Vice President and Head of Research between August 2000 and June 2007. He also held the positions of Senior Vice President and Chief Technical Officer from March 1998 to July 2000, Vice President of Research, Molecular Sciences, from August 1994 to March 1998, Senior Director of Molecular Sciences from July 1993 to July 1994, and Director of Molecular Development from 1992 to July 1993. From 1989 to 1992, Dr. Squinto held various positions at Regeneron Pharmaceuticals, Inc. including as Senior Scientist and Assistant Head of the Discovery Group. From 1986 to 1989, Dr. Squinto was an Assistant Professor of Biochemistry and Molecular Biology at Louisiana State University Medical Center and an Adjunct Professor of Neuroscience at the Tulane University Medical School. Dr. Squinto's work has led to over 70 scientific papers in the fields of gene regulation, growth factor biology and gene transfer. Dr. Squinto's work is primarily in the fields of molecular and cellular biology. Dr. Squinto served as a Director of the Biotechnology Research and Development Corporation, a biotechnology consortium, from 1997 to 2003. Dr. Squinto received his B.A. in Chemistry and Ph.D. in Biochemistry and Biophysics from Loyola University of Chicago.

David L. Hallal has been Executive Vice President and Chief Commercial Officer since October 2012. From May 2010 and October 2012, he was Senior Vice President , Global Commercial Operations. From November 2008 to May 2010, he was Senior Vice President, Commercial Operations Americas, and from June 2006 until November 2008 he served as Senior Vice President, US Commercial Operations. Before joining Alexion, Mr. Hallal served as Vice President, Sales at OSI Eyetech from April 2004 until June 2006, where he led the U.S. launch of a first-in-class anti-VEGF therapy for age-related macular degeneration. Prior to OSI Eyetech, from 1992 until 2004, Mr. Hallal held various sales and marketing leadership positions at Amgen and Biogen Idec, where he was involved in multiple product launches in the areas of hematology, oncology, nephrology and immunology. Mr. Hallal received a B.A. in Psychology from the University of New Hampshire.

Vikas Sinha, M.B.A., C.A., C.P.A. has been Executive Vice President and Chief Financial Officer since October 2012. From September 2005 to October 2012, he was Senior Vice President and Chief Financial Officer. From June 1994 to August 2005, Mr. Sinha held various positions with Bayer AG in the United States, Japan, Germany, and Canada, most recently serving as Vice President and Chief Financial Officer of Bayer Pharmaceuticals Corporation, USA from February 2001 until August 2005. At Bayer, Mr. Sinha has been responsible for financial and business risk management, strategic planning, contracting, customer services, information systems, and supply chain and site administration in North America. Mr. Sinha was also a member of the Pharmaceutical Management Committee for North America. Prior to his appointment in the United States, Mr. Sinha was Vice President and Chief Financial Officer of Bayer Yakuhin Ltd., in Japan and Manager, Mergers and Acquisitions with Bayer AG in Germany. He began his career at Bayer in Toronto as part of an executive development program in the healthcare division. Prior to Bayer, Mr. Sinha held several positions of increasing responsibilities with ANZ Bank and Citibank in South Asia. Mr. Sinha holds a Masters of Business Administration from the Asian Institute of Management which included an exchange program with the University of Western Ontario (Richard Ivey School of Business). He is also a qualified Chartered Accountant from the Institute of Chartered Accountants of India and a Certified Public Accountant in the United States.

Clare Carmichael has been Senior Vice President and Chief Human Resources Officer, since August 2011. From August 2008 to March 2011, Ms. Carmichael served as Senior Vice President, Global Human Resources at Watson Pharmaceuticals, Inc., where she established and executed global HR strategies. From December 2005 to August 2008, Ms. Carmichael held various human resources positions of increasing responsibility at Schering-Plough Corporation, including Vice President of Global Human Resources at the Schering-Plough Research Institute. From December 2003 to December 2005, Ms. Carmichael was Vice President of Human Resources at Eyetech Pharmaceuticals, Inc. Prior to Eyetech, she held various positions of increasing responsibility in human resources at

Pharmacia Corporation. Ms. Carmichael received a B.A. in Psychology from Rider University.

John B. Moriarty, J.D. has been Senior Vice President and General Counsel since December 2012. From December 2010 to December 2012, Mr. Moriarty served as General Counsel and Chief Legal Officer at Elan Corporation plc, an Irish public limited company traded on the New York and Irish Stock Exchanges, and also served as a member of Elan's Executive Management team. Prior to assuming the role of General Counsel, Mr. Moriarty served as Senior Vice President of Law, Litigation and Commercial Operations at Elan from December 2008 to December 2010. From 2002 to 2008, Mr. Moriarty held various positions with Amgen, Inc., including Executive Director and Associate General Counsel, Global Commercial Operations - Amgen Oncology and Senior Counsel, Complex Litigation, Products Liability and Government Investigations. Between 1994 and 2002, Mr. Moriarty served in various capacities in private practice focused on healthcare and as a healthcare

fraud prosecutor in the U.S. Attorney's Office and the Virginia Attorney General's Office. Mr. Moriarty received his J.D., cum laude, from the University of Georgia School of Law and his B.A., with distinction, from the University of Virginia.

Frank J. Wright has been Senior Vice President and President of Alexion Pharma International Sàrl since January 2013. Before joining Alexion, from August 2011 to February 2012, Mr. Wright served as Interim President of the Clinical Trials Distribution Unit at Marken Limited, a global clinical supply chain solutions provider. Mr. Wright co-founded Aptuit LLC in 2004, a provider of integrated drug discovery and development services, and from 2004 to 2010 served as its Vice Chairman and Chief Operating Officer. From 2000 to 2004, he was an independent consultant advising pharmaceutical and biotechnology companies and private equity firms with respect to acquisitions, asset valuation and emerging markets. From 1994 to 2000, Mr. Wright held various positions of increasing responsibility at ChiRex, Inc., a Nasdaq-listed services company providing process research and development and contract manufacturing services, acquired by Rhodia Pharma in 2000, including as Co-Chief Executive Officer and Chief Operating Officer. Prior to joining ChiRex, Mr. Wright served at Glaxo for 15 years in various operational management, outsourcing and procurement positions. Mr. Wright studied Mechanical Engineering at the University of Strathclyde, Glasgow.

Available Information

Our internet website address is http://www.alxn.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 Securities and Exchange Commission (SEC). These SEC reports can be accessed through the “Investors” section of our website. 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, Alexion Pharmaceuticals, Inc., 352 Knotter Drive, Cheshire, CT 06410.

Item 1A. Risk Factors.

(amounts in thousands, except percentages)

You should carefully consider the following risk factors before you decide to invest in Alexion and our business because these risk factors may have a significant impact on our business, operating results, financial condition, and cash flows. The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties not presently known to us or that we currently deem immaterial may also impair our business operations. If any of the following risks actually occurs, our business, financial condition and results of operations could be materially and adversely affected.

Risks Related to Our Lead Product Soliris

We depend heavily on the success of our lead product, Soliris. If we are unable to increase sales of Soliris, or obtain approval or commercialize Soliris in new territories for the treatment of PNH, aHUS or for additional indications, or if we are significantly delayed or limited in doing so, our business may be materially harmed.

Our ability to generate revenues will continue to depend on commercial success of Soliris in the United States, Europe, Japan and in a number of key markets in the rest of the world and whether physicians, patients and health care payers view Soliris as therapeutically effective and safe relative to cost. Since we launched Soliris in the United States in April 2007, essentially all of our revenue has been attributed to sales of Soliris, and we expect that Soliris product sales will continue to contribute to a significant percentage or almost all of our total revenue over the next several years.

In September and November 2011 we obtained marketing approval in the United States and the European Union, respectively, for Soliris for the treatment of a second indication, aHUS.

We dedicate significant resources to the worldwide commercialization of Soliris. We have established sales and marketing capabilities in the United States and in many countries throughout the world. We cannot guarantee that any marketing application for Soliris for the treatment of PNH, aHUS or any other indication, will be approved or maintained in any country where we seek marketing authorization to sell Soliris. In certain countries, we continue discussions with authorities to finalize operational, reimbursement, price approval and funding processes so that we may, upon conclusion of such discussions, commence commercial sales of Soliris for the treatment of PNH in those countries. We have and will continue to commence similar discussions with authorities to facilitate the commercialization of Soliris for the treatment of aHUS in certain countries in the European Union. We cannot guarantee that we will be able to obtain reimbursement for Soliris or successfully commercialize Soliris in any additional countries, or that we will be able to maintain coverage or reimbursement at anticipated levels in any country in which we have already received marketing approval. As a result, sales in certain countries may be delayed or never occur, or may be subsequently reduced.

The commercial success of Soliris and our ability to generate and increase revenues will depend on several factors, including the following:

- receipt of marketing approvals for Soliris for the treatment of PNH in new territories and the maintenance of marketing approvals for the treatment of PNH in the United States, the European Union, Japan and other territories;
- receipt and maintenance of marketing approvals for Soliris for the treatment of aHUS in Japan and other territories and the maintenance of the marketing approval in the United States and the European Union;
- the number of patients with PNH and aHUS, and the number of those patients who are diagnosed with PNH and aHUS and identified to us;
- the number of patients with PNH and aHUS that may be treated with Soliris;
- successful continuation of commercial sales in the United States, Japan and in European countries where we are already selling Soliris for the treatment of PNH, and successful launch in countries where we have not yet obtained, or only recently obtained, marketing approval or commenced sales;
- successfully launching commercial sales of Soliris for the treatment of aHUS in the United States and Europe, and in countries where we have not yet obtained marketing approval;
- our ability to obtain sufficient coverage or reimbursement by third-party payers and our ability to maintain coverage or reimbursement at anticipated levels;

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- acceptance of Soliris and maintenance of safety and efficacy in the medical community;
- establishment and maintenance of commercial manufacturing capabilities ourselves or through third-party manufacturers; and
- our ability to develop, register and commercialize Soliris for indications other than PNH, including aHUS.

If we are not successful in increasing sales of Soliris in the United States, Europe and Japan and commercializing in the rest of the world, or are significantly delayed or limited in doing so, we may experience surplus inventory, our business may be materially harmed and we may need to significantly curtail operations.

Because the target patient populations of Soliris for the treatment of PNH and aHUS are small and have not been definitively determined, we must be able to successfully identify patients and achieve a significant market share in order to maintain profitability and growth.

PNH and aHUS are each ultra-rare diseases with small patient populations that have not been definitively determined. There can be no guarantee that any of our programs will be effective at identifying patients and the number of patients in the United States, Europe and elsewhere may turn out to be lower than expected, may not be otherwise amenable to treatment with Soliris, or new patients may become increasingly difficult to identify, all of which would adversely affect our results of operations and our business.

If we are unable to obtain, or maintain at anticipated levels, reimbursement for Soliris from government health administration authorities, private health insurers and other organizations, our pricing may be affected or our product sales, results of operations or financial condition could be harmed.

We may not be able to sell Soliris on a profitable basis or our profitability may be reduced if we are required to sell our product at lower than anticipated prices or reimbursement is unavailable or limited in scope or amount. Soliris is significantly more expensive than traditional drug treatments and almost all patients require some form of third party coverage to afford its cost. Our future revenues and profitability will be adversely affected if we cannot depend on governmental payers, such as Medicare and Medicaid in the United States or country specific governmental organizations, and private third-party payers to defray the cost of Soliris to patients. These entities may refuse to provide coverage and reimbursement with respect to Soliris, determine to provide a lower level of coverage and reimbursement than anticipated, or reduce previously approved levels of coverage and reimbursement, including in the form of higher mandatory rebates or modified pricing terms. In any such case, our pricing or reimbursement for Soliris may be affected and our product sales, results of operations or financial condition could be harmed.

In certain countries where we sell or are seeking or may seek to commercialize Soliris, including certain countries where we both sell Soliris for the treatment of PNH and sell or seek to commercialize Soliris for the treatment of aHUS, if approved by the appropriate regulatory authority, pricing, coverage and level of reimbursement of prescription drugs are subject to governmental control. We may be unable to negotiate coverage, pricing, and reimbursement on terms that are favorable to us, or such coverage, pricing, and reimbursement may differ in separate regions in the same country. In some foreign countries, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country, and we cannot guarantee that we will have the capabilities or resources to successfully conclude the necessary processes and commercialize Soliris in every or even most countries in which we seek to sell Soliris. Reimbursement sources are different in each country and in each country may include a combination of distinct potential payers, including private insurance and governmental payers.

For example, the European Union member states' authorities may restrict the range of medicinal products for which their national health insurance systems provide reimbursement and adopt additional measures to control the prices of medicinal products for human use. This includes the use of reference pricing and HTA. HTA is the procedure according to which the assessment of the public health impact, therapeutic impact and the economic and societal impact of the use of a given medicinal product in the national healthcare systems of the individual country is conducted. HTA generally focuses on the clinical efficacy and effectiveness, safety, cost, and cost-effectiveness of individual medicinal products as well as their potential implications for the healthcare system. These elements of medicinal products are compared with other treatment options available on the market. The national authorities of some European Union member states may from time to time approve a specific price for the medicinal product. Others may adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the national market. Some countries have or may seek to impose limits on the aggregate reimbursement for Soliris or

for the use of Soliris for certain indications. In such cases, our commercial operations in such countries and our results of operations and our business are and may be adversely affected. Our results of operations may suffer if we are unable to successfully and timely conclude reimbursement, price approval or funding processes and market Soliris in such foreign countries or if coverage and reimbursement for Soliris is limited or reduced. If we are not able to obtain coverage, pricing or reimbursement on terms acceptable to us or at all, or if such terms should change in any foreign

countries, we may not be able to or we may determine not to sell Soliris for one or more indications in such countries, or we could decide to sell Soliris at a lower than anticipated price in such countries, and our revenues may be adversely affected as a result.

The potential increase in the number of patients receiving Soliris may cause third-party payers to modify or limit coverage or reimbursement for Soliris for the treatment of PNH, aHUS, or both indications.

Changes in pricing or the amount of reimbursement in countries where we currently commercialize Soliris may also reduce our profitability and worsen our financial condition. In the United States, the European Union member states, and elsewhere, there have been, and we expect there will continue to be, efforts to control and reduce health care costs. Government and other third-party payers in the United States and the European Union member states are challenging the prices charged for health care products and increasingly limiting and attempting to limit both coverage and level of reimbursement for prescription drugs. For example, during 2010 the German government adopted legislation to increase mandatory discounts on pharmaceutical products and impose a temporary freeze on pharmaceutical pricing, including Soliris. A significant reduction in the amount of reimbursement or pricing for Soliris in one or more countries may have a material adverse effect on our business. See additional discussion below under the headings “Government initiatives that affect coverage and reimbursement of drug products could adversely affect our business” and “The credit and financial market conditions may aggravate certain risks affecting our business.” In addition, certain countries establish pricing and reimbursement amounts by reference to the price of the same or similar products in other countries. If coverage or the level of reimbursement is limited in one or more countries, we may be unable to obtain or maintain anticipated pricing or reimbursement in current or new territories.

Many third-party payers cover only selected drugs, making drugs that are not preferred by such payer more expensive for patients, and require prior authorization or failure on another type of treatment before covering a particular drug. Third-party payers may be especially likely to impose these obstacles to coverage for higher-priced drugs such as Soliris.

Even in countries where patients have access to insurance, their insurance co-payment amounts or annual or lifetime caps on reimbursements may represent a barrier to obtaining or continuing Soliris. We have financially supported non-profit organizations which assist patients in accessing treatment for PNH and aHUS, including Soliris. Such organizations assist patients whose insurance coverage leaves them with prohibitive co-payment amounts or other expensive financial obligations. Such organizations' ability to provide assistance to patients is dependent on funding from external sources, and we cannot guarantee that such funding will be provided at adequate levels, if at all. We have also provided Soliris without charge to patients who have no insurance coverage for drugs for related charitable purposes. We are not able to predict the financial impact of the support we may provide for these and other charitable purposes; however, substantial support could have a material adverse effect on our profitability in the future.

We are also focusing development efforts on the use of eculizumab for the treatment of additional diseases. The success of these programs depends on many factors, including those described under the heading "Risks Related to Development, Clinical Testing and Regulatory Approval of Our Product Candidates." As eculizumab is approved by regulatory agencies for indications other than PNH, the potential increase in the number of patients receiving Soliris may cause third-party payers to refuse coverage or reimbursement for Soliris for the treatment of PNH or for any other approved indication, or provide a lower level of coverage or reimbursement than anticipated or currently in effect.

We may not be able to gain or maintain market acceptance among the medical community or patients, which would prevent us from maintaining profitability or growth in the future.

We cannot be certain that Soliris will gain or maintain market acceptance in a particular country among physicians, patients, health care payers, and others. Although we have received regulatory approval for Soliris in certain territories, including the United States, Japan and the European Union, such approvals do not guarantee future revenue. We cannot predict whether physicians, other health care providers, government agencies or private insurers will determine or continue to accept that Soliris is safe and therapeutically effective relative to its cost. Medical doctors' willingness to prescribe, and patients' willingness to accept, Soliris depends on many factors, including prevalence and severity of adverse side effects in both clinical trials and commercial use, effectiveness of our marketing strategy and the pricing of Soliris, publicity concerning Soliris, our other product candidates or competing products, our ability to obtain and maintain third-party coverage or reimbursement, and availability of alternative treatments, including bone marrow transplant as an alternative treatment for PNH. The likelihood of medical doctors

to prescribe Soliris for patients with aHUS may also depend on how quickly Soliris can be delivered to the hospital or clinic and our distribution methods may not be sufficient to satisfy this need. If Soliris fails to achieve or maintain market acceptance among the medical community or patients in a particular country, we may not be able to market and sell it successfully in such country, which would limit our ability to generate revenue and could harm our overall business.

If we or our contract manufacturers fail to comply with continuing United States and foreign regulations, we could lose our approvals to market Soliris or our manufacturers could lose their approvals to manufacture Soliris, and our business would be seriously harmed.

We cannot guarantee that we will be able to maintain our regulatory approvals for Soliris. If we do not maintain our regulatory approvals for Soliris, the value of our company and our results of operations will be materially harmed. We and our current and future partners, contract manufacturers and suppliers are subject to rigorous and extensive regulation by the FDA, other federal and state agencies, and governmental authorities in other territories. These regulations continue to apply after product approval, and cover, among other things, testing, manufacturing, quality control, finishing, vialing, labeling, advertising, promotion, risk mitigation, adverse event reporting requirements, and export of biologics. For example, the risk management program established in 2007 upon the FDA's approval of Soliris for the treatment of PNH was replaced with a REMS program, approved by the FDA in 2010. The REMS program requires mandatory physician certification in the United States. Each physician must certify that the physician is aware of the potential risks associated with the administration of Soliris and that the physician will inform each patient of these risks using educational material approved by the FDA.

As a condition of approval for marketing Soliris, governmental authorities may require us to conduct additional studies. For example, in connection with the approval of Soliris in the United States, European Union and Japan, for the treatment of PNH, we agreed to establish a PNH Registry, monitor immunogenicity, monitor compliance with vaccination requirements, and determine the effects of anticoagulant withdrawal among PNH patients receiving eculizumab, and, specifically in Japan, we agreed to conduct a trial in a limited number of Japanese PNH patients to evaluate the safety of a meningococcal vaccine. Further, in connection with the approval of Soliris in the United States for the treatment of aHUS, we agreed to establish an aHUS Registry and complete additional human clinical studies in adult and pediatric patients. In the United States, for example, the FDA can propose to withdraw approval for a product if it determines that such additional studies are inadequate or if new clinical data or information shows that a product is not safe for use in an approved indication. We are required to report any serious and unexpected adverse experiences and certain quality problems with Soliris to the FDA, the EMA, the competent authorities of the European Union member states, MHLW, and certain other health agencies. We or any health agency may have to notify health care providers of any such developments.

The discovery of any previously unknown problems with Soliris, a manufacturer or a facility may result in restrictions on Soliris, a manufacturer or a facility, including withdrawal of Soliris from the market, batch failures, or interruption of production. Certain changes to an approved product, including the way it is manufactured or promoted, often require prior regulatory approval before the product as modified may be marketed. Our manufacturing and other facilities and those of any third parties manufacturing Soliris will be subject to inspection prior to grant of marketing approval by each regulatory authority where we seek marketing approval and subject to continued review and periodic inspections by the regulatory authorities. We and any third party we would use to manufacture Soliris for sale, including Lonza, must also be licensed by applicable regulatory authorities.

Failure to comply with the laws and requirements, including statutes and regulations, administered by the FDA, the EMA, the competent authorities of the European Union member states, the MHLW or other agencies, could result in:

- a product recall or withdrawal;
- significant administrative and judicial sanctions, including, warning letters or untitled letters;
- significant fines and other civil penalties;
- suspension or withdrawal of a previously granted approval for Soliris;
- interruption of production;
- operating restrictions, such as a shutdown of production facilities or production lines;
- suspension of ongoing clinical trials;
- delays in approving or refusal to approve Soliris or a facility that manufactures Soliris;
- seizing or detaining product;
- injunctions; and/or
- criminal prosecution.

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If the use of Soliris harms people, or is perceived to harm patients even when such harm is unrelated to Soliris, our regulatory approvals could be revoked or otherwise negatively impacted and we could be subject to costly and damaging product liability claims.

The testing, manufacturing, marketing and sale of drugs for use in humans exposes us to product liability risks. Side effects and other problems from using Soliris could (1) lessen the frequency with which physicians decide to prescribe Soliris, (2) encourage physicians to stop prescribing Soliris to their patients who previously had been prescribed Soliris, (3) cause serious adverse events and give rise to product liability claims against us, and (4) result in our need to withdraw or recall Soliris from the marketplace. Some of these risks are unknown at this time.

We tested Soliris in only a small number of patients. The FDA marketing approval for the treatment of patients with aHUS was based on two prospective studies in a total of 37 adult and adolescent patients, together with a retrospective study that included 19 pediatric patients. PNH and aHUS are ultra-rare diseases. As more patients use Soliris, including more children and adolescents, new risks and side effects may be discovered, the rate of known risks or side effects may increase, and risks previously viewed as less significant could be determined to be significant. Previously unknown risks and adverse effects of Soliris may also be discovered in connection with unapproved uses of Soliris, which may include administration of Soliris under acute emergency conditions, such as the Enterohemorrhagic E. coli health crisis in Europe, primarily Germany, that began in May 2011. We do not promote, or in any way support or encourage the promotion of Soliris for unapproved uses in violation of applicable law, but physicians are permitted to use products for unapproved purposes and we are aware of such uses of Soliris. In addition, we are studying and expect to continue to study Soliris in diseases other than PNH and aHUS in controlled clinical settings, and independent investigators are doing so as well. In the event of any new risks or adverse effects discovered as new patients are treated for approved indications and as Soliris is studied in or used by patients for other indications, regulatory authorities may delay or revoke their approvals, we may be required to conduct additional clinical trials and safety studies, make changes in labeling of Soliris, reformulate Soliris or make changes and obtain new approvals for our and our suppliers' manufacturing facilities. We may also experience a significant drop in the potential sales of Soliris, experience harm to our reputation and the reputation of Soliris in the marketplace or become subject to lawsuits, including class actions. Any of these results could decrease or prevent any sales of Soliris or substantially increase the costs and expenses of commercializing and marketing Soliris.

We may be sued by people who use Soliris, whether as a prescribed therapy, during a clinical trial, during an investigator initiated study, or otherwise. Many patients who use Soliris are already very ill. Any informed consents or waivers obtained from people who enroll in our trials or use Soliris may not protect us from liability or litigation. Our product liability insurance may not cover all potential types of liabilities or may not cover certain liabilities completely. Moreover, we may not be able to maintain our insurance on acceptable terms. In addition, negative publicity relating to the use of Soliris or a product candidate, or to a product liability claim, may make it more difficult, or impossible, for us to market and sell Soliris. As a result of these factors, a product liability claim, even if successfully defended, could have a material adverse effect on our business, financial condition or results of operations.

Patients who use Soliris already often have severe and advanced stages of disease and known as well as unknown significant pre-existing and potentially life-threatening health risks, including, for example, bone marrow failure, kidney failure and thrombosis. During the course of treatment, patients may suffer adverse events, including death, for reasons that may or may not be related to Soliris. Such events could subject us to costly litigation, require us to pay substantial amounts of money to injured patients, delay, negatively impact or end our opportunity to receive or maintain regulatory approval to market Soliris, or require us to suspend or abandon our commercialization efforts. Even in a circumstance in which we do not believe that an adverse event is related to Soliris, the investigation into the circumstance may be time consuming or inconclusive. These investigations may interrupt our sales efforts, delay our regulatory approval process in other countries, or impact and limit the type of regulatory approvals Soliris receives or maintains.

Some patients treated with Soliris for PNH and other diseases, including patients who have participated in our clinical trials, have died or suffered potentially life-threatening diseases either during or after ending their Soliris treatments. In particular, use of C5 Inhibitors, such as Soliris, is associated with an increased risk for certain types of infection, including meningococcal infection. Serious cases of meningococcal infection can result in severe illness, including but

not limited to brain damage, loss of limbs or parts of limbs, kidney failure, or death. Under controlled settings, patients in our eculizumab trials all receive vaccination against meningococcal infection prior to first administration of Soliris and patients who are prescribed Soliris in most countries are required by prescribing guidelines to be vaccinated prior to receiving their first dose. A physician may not have the opportunity to timely vaccinate a patient in the event of an acute emergency episode, such as in a patient presenting with aHUS or during the health crisis that began in May 2011 in Europe, principally in Germany, due to the epidemic of infections from Enterohemorrhagic E.coli. Vaccination does not, however, eliminate all risk of meningococcal infection. Additionally, in some countries there may not be any vaccine approved for general use or approved for use in infants and children. Some patients treated with Soliris who had been vaccinated have nonetheless experienced meningococcal infection, including patients who have suffered serious illness or death. Each such incident is required to be reported to

appropriate regulatory agencies in accordance with relevant regulations.

We are also aware of a potential risk for PNH patients who delay a dose of Soliris or discontinue their treatment of Soliris. Treatment with Soliris blocks complement and allows complement-sensitive PNH red blood cells to increase in number. If treatment with Soliris is thereafter delayed or discontinued, a greater number of red blood cells therefore would become susceptible to destruction when the patient's complement system is no longer blocked. The rapid destruction of a larger number of a patient's red blood cells may lead to numerous complications, including death. Several PNH patients in our studies of Soliris have received delayed doses or discontinued their treatment. In none of those circumstances were significant complications shown to be due to rapid destruction of a larger number of PNH red blood cells; however, we have not studied the delay or termination of treatment in enough patients to determine that such complications in the future are unlikely to occur. Additionally, such delays or discontinuations may be associated with significant complications without evidence of such rapid cell destruction.

We are aware of a risk for aHUS patients who delay or miss a dose of Soliris or discontinue their treatment of Soliris. Treatment with Soliris blocks complement and inhibits complement-mediated TMA. After missing a dose or discontinuing Soliris, blood clots may form in small blood vessels throughout the body, causing a reduction in platelet count. The reduction in platelet count may lead to numerous complications, including changes in mental status, seizures, angina, thrombosis, renal failure or even death. In our aHUS clinical studies, such TMA complications were observed in some patients who missed a dose.

Clinical evaluations of outcomes in the post-marketing setting are required to be reported to appropriate regulatory agencies in accordance with relevant regulations. Determination of significant complications associated with the delay or discontinuation of Soliris could have a material adverse effect on our ability to sell Soliris.

Although we obtained regulatory approval to market and sell Soliris for PNH and aHUS in the United States and European Union, and Soliris for PNH in Japan and other territories, we cannot guarantee that we will obtain the regulatory approval or reimbursement approval for Soliris for the treatment of PNH, aHUS or other diseases in each territory where we seek approvals.

Governments in countries where we seek to commercialize Soliris regulate the distribution of drugs and the facilities where such drugs are manufactured, and obtaining their approvals can be lengthy, expensive and highly uncertain. The approval process varies from country to country, and the requirements governing the conduct of clinical trials, product manufacturing, product licensing, pricing and reimbursement vary greatly from country to country. In certain jurisdictions, we are required to finalize operational, reimbursement, price approval and funding processes prior to marketing our products, even in countries where marketing approval has been obtained. We have received regulatory approval for Soliris for treatment of patients with PNH in the United States, the European Union, Japan and other territories. In September and November 2011 we received regulatory approval for Soliris for the treatment of patients with aHUS in the United States and the European Union, respectively. We may not receive regulatory approval for Soliris for the treatment of PNH, aHUS or any other disease in any other territories on a timely basis, if ever.

Regulatory agencies may require additional information or data with respect to our submissions for Soliris, including the marketing authorization applications submitted to the EMA for the treatment of patients with aHUS. We may have to conduct additional lengthy clinical testing and other costly and time-consuming procedures to satisfy foreign regulatory agencies. Even with approval of Soliris in certain countries, the regulatory agencies in other countries may not agree with our interpretations of our clinical trial data for Soliris and may decide that our results are not adequate to support approval for marketing of Soliris. In those circumstances, we would not be able to obtain regulatory approval in such country on a timely basis, if ever. Even if approval is granted in such country, the approval may require limitations on the indicated uses for which the drug may be marketed. The foreign regulatory approval process includes all of the risks associated with FDA approval as well as country-specific regulations. We must obtain approval of a product by the comparable regulatory authorities and ethics committees of foreign countries before we can commence clinical trials or marketing of the product in those countries. We were required to conduct clinical studies with Soliris in patients with PNH in Japan prior to obtaining marketing approval in that country and Japanese authorities could require additional studies in Japan for Soliris for the treatment of patients with aHUS. We are also conducting prospective clinical trials in adult and pediatric patients to confirm the benefit of Soliris for the treatment of aHUS. Commercialization of Soliris for the treatment of PNH, aHUS or any other indication could be delayed, limited or may not occur in territories where we seek marketing approval if the applicable regulatory agency requires

additional information or data.

Our commercialization of Soliris may be stopped, delayed or made less profitable if we or any other third party provider fails to provide sufficient quantities of Soliris. Commercial quantities of Soliris can only be manufactured at two facilities, including our own facility in Rhode Island. Vial filling can only be performed at two third party facilities.

Commercial quantities of Soliris are manufactured by us at ARIMF and by Lonza. Manufacturing processes must comply with applicable regulations and manufacturing practices, as well as our own quality standards. In particular, the manufacture of Soliris is heavily regulated by governmental authorities around the world, including the FDA, EMA, the competent authorities of the European Union member states, and MHLW. If we or our third-party providers, including our product vialers, packagers and labelers, fail to comply fully with such regulations then we may be required to initiate a recall or withdrawal of our products or regulatory agencies could take action that leads to product shortages. Such action may include:

- issuing warning or untitled letters;
- requiring corrective action or restrictions on operations, including costly new manufacturing requirements;
- ordering shutdown of production facilities or production lines;
- seizing or detaining product;
- suspending or withdrawing the approval of Soliris;
- imposing significant civil penalties and criminal fines;
- suspending ongoing clinical studies for Soliris; and
- refusing to approve pending BLAs or BLA supplements for Soliris.

The manufacture of Soliris is difficult. Manufacture of a biologic requires a multi-step controlled process and even minor problems or deviations could result in defects or failures. We cannot be certain that we, Lonza or our other third party providers will be able to perform uninterrupted supply chain services. The failure to manufacture appropriate supplies of Soliris, on a timely basis, or at all, may prevent or interrupt the commercialization of Soliris. If we, Lonza or our other third party providers were unable to manufacture Soliris for any period, or if we, Lonza or our other third party providers do not obtain approval for the manufacturing of Soliris in the respective facility by the applicable regulatory agencies, we may incur substantial loss of sales. If we are forced to find an alternative supplier or other third party providers for Soliris, in addition to loss of sales, we may also incur significant costs and experience significant delay in establishing a new arrangement.

We are authorized to sell product that is manufactured at ARIMF in the United States, the European Union, Japan and certain other territories. However, we will not be capable of manufacturing Soliris at ARIMF for commercial sale in certain other territories until such time as we have received the required regulatory approval for our facility, if ever. We will continue to depend entirely on one company, Lonza, to manufacture Soliris for commercial sale in such other territories until that time.

In September and November 2011 we received marketing approval for Soliris for the treatment of patients with aHUS in the United States and European Union, respectively. If Soliris is approved in other territories for the treatment of patients with aHUS, or for additional indications, we expect that the demand for Soliris will increase. We may underestimate demand, or experience product interruptions at ARIMF, Lonza or a facility of a third party provider, including as a result of risks and uncertainties described in this report. If we, Lonza or our other third party providers do not manufacture sufficient quantities of Soliris to satisfy demand, our business will be materially harmed.

We depend on a very limited number of third party providers for other services with respect to our clinical and commercial requirements, including product finishing, packaging, vialing and labeling. We have changed or added third party vialers in the past in order to support uninterrupted supply, and may do so in the future. We currently rely on two third party vialers to support our commercial requirements in the United States and the European Union, and a single third party vialer to support requirements in Japan. No guarantee can be made that any third party vialer will be able to perform such services for sufficient product volumes for any country or territory. We do not have control over any third party provider's compliance with our internal or external specifications or the rules and regulations of the FDA, EMA, competent authorities of the European Union member states, MHLW or any other applicable regulations or standards. In the past, we have had to write off and incur other charges and expenses for production that failed to meet requirements. Any difficulties or delays in our third party manufacturing of Soliris, or any failure of our third party providers to comply with our internal and external specifications or any applicable rules, regulations and

standards could increase our costs, constrain our ability to satisfy demand for Soliris from customers, cause us to lose revenue or incur penalties for failure to deliver product, make us postpone or cancel clinical trials, or cause our products to be recalled or withdrawn.

Many additional factors could cause production interruptions at ARIMF or at the facilities of Lonza or our third party providers, including natural disasters, labor disputes, acts of terrorism or war, human error, equipment malfunctions, contamination, or raw material shortages. The occurrence of any such event could adversely affect our ability to satisfy demand for Soliris, which could materially and adversely affect our operating results.

We are dependent upon a small number of customers for a significant portion of our revenue, and the loss of, or significant reduction or cancellation in sales to, any one of these customers could adversely affect our operations and financial condition.

For the year ended December 31, 2012, our largest customer accounted for 21% of our global Soliris net product sales, and our three largest customers accounted for approximately 41% of our global net product sales. As of December 31, 2012, our single largest customer accounted for 18% of the global accounts receivable balance. We expect such customer concentration to continue for the foreseeable future. We typically sell Soliris to third party distributors, such as specialty pharmacies, who in turn sell to patient health care providers. We do not promote Soliris to these distributors, and they do not set or determine demand for Soliris. Our ability to successfully commercialize Soliris will depend, in part, on the extent to which we are able to provide adequate distribution of Soliris to patients. Although a number of specialty distributors and specialty pharmacies, which supply physician office clinics, hospital outpatient clinics, infusion clinics, home health care providers, and governmental organizations, distribute Soliris, they generally carry a very limited inventory and may be reluctant to distribute Soliris in the future if demand for the product does not increase. Further, it is possible that our distributors could decide to change their policies or fees, or both, at some time in the future. This could result in their refusal to distribute smaller volume products such as Soliris, or cause higher product distribution costs, lower margins or the need to find alternative methods of distributing our product. Although we believe we can find alternative distributors on a relatively short notice, our revenue during that period of time may suffer and we may incur additional costs to replace a distributor. The loss of any large customer, a significant reduction in sales we make to them, any cancellation of orders they have made with us or any failure to pay for the products we have shipped to them could materially and adversely affect our results of operations and financial condition.

If we are unable to establish and maintain effective sales, marketing and distribution capabilities, or to enter into agreements with third parties to do so, we will be unable to successfully commercialize Soliris.

We are marketing and selling Soliris ourselves in the United States, Europe, Japan and several other territories. If we are unable to establish and/or expand our capabilities to sell, market and distribute Soliris for the treatment of PNH, aHUS or, if approved by the necessary regulatory agencies, other future indications, either through our own capabilities or by entering into agreements with others, or to maintain such capabilities in countries where we have already commenced commercial sales, we will not be able to successfully sell Soliris. In that event, we will not be able to generate significant revenues. We cannot guarantee that we will be able to establish and maintain our own capabilities or enter into and maintain any marketing or distribution agreements with third-party providers on acceptable terms, if at all. Even if we hire the qualified sales and marketing personnel we need to support our objectives, or enter into marketing and distribution agreements with third parties on acceptable terms, we may not do so in an efficient manner or on a timely basis. We may not be able to correctly judge the size and experience of the sales and marketing force and the scale of distribution capabilities necessary to successfully market and sell Soliris. Establishing and maintaining sales, marketing and distribution capabilities are expensive and time-consuming. Our expenses associated with building up and maintaining the sales force and distribution capabilities around the world may be disproportionate compared to the revenues we may be able to generate on sales of Soliris. We cannot guarantee that we will be successful in commercializing Soliris.

If we market Soliris in a manner that violates health care fraud and abuse laws and other laws regulating marketing and promotion, we may be subject to investigations and civil or criminal penalties.

In addition to FDA and related regulatory requirements, we are subject to health care “fraud and abuse” laws, such as the Federal False Claims Act, the anti-kickback provisions of the federal Social Security Act, and other state and federal laws and regulations. Federal and state anti-kickback laws prohibit, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration to induce, or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any health care item or service reimbursable under Medicare, Medicaid, or other federally or state financed health care programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, patients, purchasers and formulary managers on the other. Although there are a number of statutory exemptions and regulatory safe harbors protecting certain common activities from prosecution, the exemptions and safe harbors are drawn narrowly, and practices that involve remuneration intended to induce prescribing, purchasing, or recommending may be subject to scrutiny or penalty if

they do not qualify for an exemption or safe harbor. We seek to comply with the anti-kickback laws and with the available statutory exemptions and safe harbors whenever possible. However, our practices may not in all cases fit within the safe harbors, and our practices may therefore be subject to case-by-case scrutiny.

The Federal False Claims Act prohibits any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to get a false claim paid. Pharmaceutical companies have been investigated and have reached substantial financial settlements with the Federal government under this Act for a variety of alleged promotional and marketing activities, such as allegedly providing free

product to customers with the expectation that the customers would bill federal programs for the product; providing consulting fees and other benefits to physicians to induce them to prescribe products; reporting inflated prices to private publications that were then used by federal programs to set reimbursement rates; engaging in promotion for uses that the FDA has not approved, or "off-label" uses, that caused claims to be submitted to Federal programs for non-covered off-label uses; and submitting inflated best price information to the Medicaid Rebate Program.

Although physicians are permitted to, based on their medical judgment, prescribe products for indications other than those cleared or approved by the FDA, manufacturers are prohibited from promoting their products for such off-label uses. In the United States, we market Soliris for PNH and aHUS and provide promotional materials and training programs to physicians regarding the use of Soliris for PNH and aHUS. Although we believe our marketing materials and training programs for physicians do not constitute off-label promotion of Soliris, the FDA may disagree. If the FDA determines that our promotional materials, training or other activities constitute off-label promotion of Soliris, it could request that we modify our training or promotional materials or other activities or subject us to regulatory enforcement actions, including the issuance of a warning letter, injunction, seizure, civil fine and criminal penalties. It is also possible that other federal, state or foreign enforcement authorities might take action if they believe that the alleged improper promotion led to the submission and payment of claims for an unapproved use, which could result in significant fines or penalties under other statutory authorities, such as laws prohibiting false claims for reimbursement. Even if it is later determined we are not in violation of these laws, we may be faced with negative publicity, incur significant expenses defending our position and have to divert significant management resources from other matters.

The majority of states also have statutes or regulations similar to the federal anti-kickback law and false claims laws, which apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payer. Sanctions under these federal and state laws may include civil monetary penalties, exclusion of a manufacturer's products from reimbursement under government programs, criminal fines, and imprisonment. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which would also harm our financial condition. Because of the breadth of these laws and the narrowness of the safe harbors and because government scrutiny in this area is high, it is possible that some of our business activities could come under that scrutiny.

In recent years, several states and localities, including California, the District of Columbia, Minnesota, Nevada, New Mexico, Vermont, and West Virginia, have enacted legislation requiring pharmaceutical companies to establish marketing compliance programs, file periodic reports with the state or make periodic public disclosures on sales, marketing, pricing, clinical trials, and other activities. Similar legislation is being considered in other states. Additionally, as part of the Patient Protection and Affordable Care Act, the federal government has enacted the Physician Payment Sunshine provisions, which when implemented will require manufacturers to publicly report gifts and payments made to physicians and teaching hospitals. Many of these requirements are new and uncertain, and the penalties for failure to comply with these requirements are unclear. Nonetheless, if we are found not to be in full compliance with these laws, we could face enforcement action and fines and other penalties, and could receive adverse publicity.

Similar strict restrictions are imposed on the promotion and marketing of drug products in the European Union and other countries. Laws in the European Union, including in the individual European Union member states, require promotional materials and advertising for drug products to comply with the product's Summary of Product Characteristics (SmPC), which is approved by the competent authorities. Promotion of a drug product which does not comply with the SmPC is considered to constitute off-label promotion. The off-label promotion of drug products is prohibited in the European Union. Laws in the European Union, including in the individual European Union member states, also prohibit the direct-to-consumer advertising of prescription-only drug products. Violations of the rules governing the promotion of drug products in the European Union could be penalized by administrative measures, fines and imprisonment.

Interactions between pharmaceutical companies and physicians are also governed by strict laws, regulations, industry self-regulation codes of conduct and physicians' codes of professional conduct in the individual European Union member states. The provision of any inducements to physicians to prescribe, recommend, endorse, order, purchase, supply, use or administer a drug product is prohibited. A number of European Union member states have introduced additional rules requiring pharmaceutical companies to publicly disclose their interactions with physicians and to

obtain approval from employers, professional organizations and/or competent authorities before entering into agreements with physicians. Violations of these rules could lead to the imposition of fines or imprisonment. Laws, including those governing promotion, marketing and anti-kickback provisions, industry regulations and professional codes of conduct are often strictly enforced. Increasing regulatory scrutiny of the promotional activities of pharmaceutical companies has been observed in a number of European Union member states. We are also subject to the United States Foreign Corrupt Practices Act (FCPA), the U.K. Bribery Act, and other anti-corruption laws and regulations pertaining to our financial relationships with government officials. Worldwide regulators are increasing their regulatory and enforcement

efforts in this area. For example, the Bribery Act in the United Kingdom entered into force in July 2011 applies to any company incorporated in or "carrying on business" in the United Kingdom, regardless of the country in which the alleged bribery activity occurs and even if the inappropriate activity is undertaken by our international distribution partners.

Risks Related to Development, Clinical Testing and Regulatory Approval of Our Product Candidates

None of our product candidates except for Soliris has received regulatory approvals. Soliris has not been approved for any indication other than for the treatment of patients with PNH and aHUS. If we are unable to obtain regulatory approvals to market one or more of our product candidates, including asfotase alfa and Soliris for other indications, our business may be adversely affected.

All of our product candidates except Soliris are in early stages of development, and we do not expect our other product candidates to be commercially available for several years, if at all. Similarly, Soliris has not been approved for any indication other than for the treatment of patients with PNH in the United States, the European Union, Japan and other territories, and for patients with aHUS in the United States and the European Union. We do not know when or if our product candidates, including asfotase alfa and Soliris for other indications, will be approved. Our product candidates are subject to strict regulation by regulatory authorities in the United States, in the European Union and in other territories. We cannot market any product candidate until we have completed all necessary preclinical studies and clinical trials and have obtained the necessary regulatory approvals. We do not know whether regulatory agencies will grant approval for any of our product candidates. Even if we complete preclinical studies and clinical trials successfully, we may not be able to obtain regulatory approvals or we may not receive approvals to make claims about our products that we believe to be necessary to effectively market our products. Data obtained from preclinical studies and clinical trials are subject to varying interpretations that could delay, limit or prevent regulatory approval, and failure to comply with regulatory requirements or inadequate manufacturing processes are examples of other problems that could prevent approval. In addition, we may encounter delays or rejections due to additional government regulation from future legislation, administrative action or changes in the FDA policy. Even if the FDA approves a product, the approval will be limited to those indications covered in the approval.

Outside the United States, our ability to market any of our potential products is dependent upon receiving marketing approvals from the appropriate regulatory authorities. These foreign regulatory approval processes include all of the risks associated with the FDA approval process described above. If we are unable to receive regulatory approvals, we will be unable to commercialize our product candidates, and our business may be adversely affected.

Completion of preclinical studies or clinical trials does not guarantee advancement to the next phase of development.

Completion of preclinical studies or clinical trials does not guarantee that we will initiate additional studies or trials for our product candidates, including asfotase alfa, that if further studies or trials are initiated what the scope and phase of the trial will be or that they will be completed, or that if these further studies or trials are completed, that the design or results will provide a sufficient basis to apply for or receive regulatory approvals or to commercialize products. Results of clinical trials could be inconclusive, requiring additional or repeat trials. If the design or results achieved in our clinical trials are insufficient to proceed to further trials or to regulatory approval of our product candidates, including asfotase alfa, our company could be materially adversely affected. Failure of a clinical trial to achieve its pre-specified primary endpoint generally increases the likelihood that additional studies or trials will be required if we determine to continue development of the product candidate, reduces the likelihood of timely development of and regulatory approval to market the product candidate, and may decrease the chances for successfully achieving the primary endpoint in scientifically similar indications.

There are many reasons why drug testing could be delayed or terminated.

For human trials, patients must be recruited and each product candidate must be tested at various doses and formulations for each clinical indication. In addition, to ensure safety and effectiveness, the effect of drugs often must be studied over a long period of time, especially for the chronic diseases that we are studying. Many of our programs focus on diseases with small patient populations and insufficient patient enrollment in our clinical trials could delay or cause us to abandon a product development program. We may decide to abandon development of a product candidate, including asfotase alfa, at any time due to unfavorable results or other reasons, or we may have to spend considerable resources repeating clinical trials or conducting additional trials, either of which would increase costs and delay any

revenue from those product candidates, if any.

Additional factors that can cause delay, impairment or termination of our clinical trials or our product development efforts include:

- delay or failure in obtaining institutional review board (IRB), approval or the approval of other reviewing entities to conduct a clinical trial at each site;

- delay or failure in reaching agreement on acceptable terms with prospective contract research organizations(CROs), and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- withdrawal of clinical trial sites from our clinical trials as a result of changing standards of care or the ineligibility of a site to participate in our clinical trials;
- clinical sites and investigators deviating from trial protocol, failing to conduct the trial in accordance with regulatory requirements, or dropping out of a trial;
- slow patient enrollment, including, for example, due to the rarity of the disease being studied;
- delay or failure in having patients complete a trial or return for post-treatment follow-up;
- long treatment time required to demonstrate effectiveness;
- lack of sufficient supplies of the product candidate;
- disruption of operations at the clinical trial sites;
- adverse medical events or side effects in treated patients, and the threat of legal claims and litigation alleging injuries;
- failure of patients taking the placebo to continue to participate in our clinical trials;
- insufficient clinical trial data to support effectiveness of the product candidates;
- lack of effectiveness or safety of the product candidate being tested;
- lack of sufficient funds;
- inability to meet required specifications or to manufacture sufficient quantities of the product candidate for development or commercialization activities in a timely and cost-efficient manner;
- decisions by regulatory authorities, the IRB, ethics committee, or us, or recommendation by a data safety monitoring board, to suspend or terminate clinical trials at any time for safety issues or for any other reason;
- failure to obtain the necessary regulatory approvals for the product candidate or the approvals for the facilities in which such product candidate is manufactured; and
- decisions by competent authorities, IRBs or ethics committees to demand variations in protocols or conduct of clinical trials.

The regulatory approval process is costly and lengthy and we may not be able to successfully obtain all required regulatory approvals.

The preclinical development, clinical trials, manufacturing, marketing and labeling of pharmaceuticals are all subject to extensive regulation by numerous governmental authorities and agencies in the United States, the European Union and other territories. We must obtain regulatory approval for each of our product candidates, including asfotase alfa, before marketing or selling any of them. It is not possible to predict how long the approval processes of the FDA or any other applicable federal or foreign regulatory authority or agency for any of our product candidates will take or whether any such approvals ultimately will be granted. The FDA and foreign regulatory agencies have substantial discretion in the drug approval process, and positive results in preclinical testing or early phases of clinical studies offer no assurance of success in later phases of the approval process. The approval process varies from country to country and the requirements governing the conduct of clinical trials, product manufacturing, product licensing, pricing and reimbursement vary greatly from country to country. Generally, preclinical and clinical testing of product candidates can take many years and require the expenditure of substantial resources, and the data obtained from these tests and trials can be susceptible to varying interpretations that could delay, limit or prevent regulatory approval. If we encounter significant delays in the regulatory process that result in excessive costs, this may prevent us from continuing to develop our product candidates, including asfotase alfa. Any delay in obtaining, or failure to obtain, approvals could adversely affect the marketing of our products and our ability to generate product revenue. The risks associated with the approval process include:

- failure of our product candidates to meet a regulatory agency's requirements for safety, efficacy and quality;
- disagreement over interpretation of data from preclinical studies or clinical trials;
- restricted distribution or limitation on the indicated uses for which a product may be marketed;
- unforeseen safety issues or side effects and potential requirements to establish REMS;

- disapproval of the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- governmental or regulatory delays and changes in regulatory requirements and guidelines.

Even if asfotase alfa and our other product candidates obtain regulatory approval, they may not gain market acceptance among physicians, patients and health care payers.

Physicians may elect not to recommend our drugs even if they receive marketing approval for a variety of reasons, including the timing of the market introduction of competitive drugs; lower demonstrated clinical safety and efficacy compared to other drugs; perceived lack of cost-effectiveness; lack of availability of reimbursement from third-party payers; convenience and ease of administration; prevalence and severity of adverse side effects; other potential advantages of alternative treatment methods; and ineffective marketing and distribution support. Sales of pharmaceutical products depend in significant part on the coverage and reimbursement policies of government programs, including Medicare and Medicaid in the United States and similar programs in other countries, and other third-party payers. These health insurance programs may restrict coverage of some products by using payer formularies under which only selected drugs are covered, variable co-payments that make drugs that are not preferred by the payer more expensive for patients, and by using utilization management controls, such as requirements for prior authorization or failure on another type of treatment. Payers may especially impose these obstacles to coverage for higher-priced drugs, and consequently our drug candidates may be subject to payer-driven restrictions. In addition, in some foreign countries, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country. For example, European Union member states may restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices and/or reimbursement of medicinal products for human use. A European Union member state may approve a specific price or level of reimbursement for the medicinal product, or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. The reimbursement or budget identified by a government or non-government payer for Soliris in a new indication, if obtained, may be adversely affected by the reimbursement or budget for Soliris in previously approved indications and/or adversely affect the reimbursement or budget for Soliris in such previously approved indication by that payer.

Inability to contract with third party manufacturers and other third party providers on commercially reasonable terms, or failure or delay by us or our third party manufacturers or other third party providers to provide services with respect to our drug products, including asfotase alfa if approved, in the volumes and quality required, would have a material adverse effect on our business.

Clinical quantities of eculizumab are manufactured by us at ARIMF and by Lonza. Clinical quantities of our other product candidates are manufactured by us at ARIMF or by a third party. We also depend on a very limited number of third party providers for other services with respect to our clinical and commercial requirements, including product finishing, packaging, vialing and labeling. We have changed or added third party vialers in the past in order to support uninterrupted supply, and may do so in the future. No guarantee can be made that regulators will approve additional third party vialers in a timely manner or at all, or that any third party vialer will be able to perform such services for sufficient product volumes for any country or territory. Manufacture of our drug products, including asfotase alfa, is highly technical, and only a small number of companies have the ability and capacity to manufacture our drug products for our development and commercialization needs. Due to the highly technical requirements of manufacturing our drug products and the strict quality and control specifications, we and our third party providers may be unable to manufacture or supply our drug products despite our and their efforts. In addition, we cannot be certain that any third party will be able or willing to honor the terms of its agreement, including any obligations to manufacture the drug products in accordance with regulatory requirements and to our quality specifications and volume requirements. Further, we have limited experience manufacturing the drug candidates that we acquired from Enobia, Taligen Therapeutics, Inc. (Taligen) and Orphatec Pharmaceuticals GmbH (Orphatec), such as asfotase alfa, ALXN1102 and cPMP. We cannot guarantee that we or any third party provider will be able to manufacture or supply such drug candidates, or that we or a third party provider will be able to manufacture or supply sufficient quantities to satisfy our requirements.

Manufacture of drug products, including the need to develop and utilize manufacturing processes that consistently produce our drug products to their required quality specifications, is highly regulated by the FDA, EMA and other domestic and foreign authorities. Regulatory authorities must approve the facilities in which our products are manufactured vialed, packaged and labeled prior to granting marketing approval for any product candidate. Such facilities are also subject to ongoing inspections, and minor changes in manufacturing or other related processes may require additional regulatory approvals. We cannot assure you that we or our third party providers will successfully comply with all requirements and regulations, which failure could have a material adverse effect on our business.

We currently have limited experience in manufacturing drug products in volumes that would be necessary to support commercial sales, and we can provide no assurance that we will be able to do so successfully. We acquired ARIMF in July 2006. The EC, the FDA and MHLW have approved the use of ARIMF for the production of Soliris, and we are authorized to sell Soliris manufactured in ARIMF in the United States, the European Union, Japan and certain other territories. We are entirely dependent on only one third party provider for commercial vialing in certain territories, including Japan. We have limited experience in developing commercial-scale manufacturing. We can provide no assurance that we will be able to manufacture our drug products at ARIMF under conditions required by the FDA or foreign regulatory agencies on a timely basis, if at all. ARIMF is subject to approval by other national and regional regulatory agencies before we can begin sales of Soliris or other drug products manufactured in this facility in the applicable countries or regions, and we will continue to be subject to ongoing regulatory inspections thereafter.

We, and our third party providers, may experience higher failure rates than in the past if and when we attempt to increase production volume. If we experience interruptions in the manufacture or supply of our products, our drug development and commercialization efforts will be delayed. If any of our outside third party providers stops manufacturing or supplying our products or reduces the amount manufactured or supplied, or is otherwise unable to provide our required amounts at our required quality, we may need to find other alternatives, which is likely to be expensive and time consuming, and also may result in reduced revenue during this period. Even if we are able to find alternatives they may ultimately be insufficient for our needs. As a result, our ability to conduct testing and drug trials and our plans for commercialization could be materially adversely affected. Submission of products and new development programs for regulatory approval, as well as our plans for commercialization, would be delayed or suspended. Our competitive position and our prospects for achieving or maintaining profitability could be materially and adversely affected.

Due to the nature of the current market for third-party commercial manufacturing, many arrangements require substantial penalty payments by the customer for failure to use the manufacturing capacity for which it contracted. Penalty payments under these agreements typically decrease over the life of the agreement, and may be substantial initially and de minimis or non-existent in the final period. The payment of a substantial penalty could harm our financial condition.

Risks Related to Intellectual Property

If we cannot obtain new patents, maintain our existing patents and protect the confidentiality and proprietary nature of our trade secrets and other intellectual property, our business and competitive position will be harmed.

In order to protect our drugs and technology more effectively, we need to obtain and maintain patents covering the drugs and technologies we develop. We have and may in the future obtain patents or the right to practice patents through ownership or license. Our patent applications may not result in the issue of patents in the United States or other countries. Our patents may not afford adequate protection for our products. Third parties may challenge our patents. If any of our patents are narrowed, invalidated or become unenforceable, competitors may develop and market products similar to ours that do not conflict with or infringe our patents rights, which could have a material adverse effect on our financial condition. We may also finance and collaborate in research conducted by government organizations, hospitals, universities or other educational or research institutions. Such research partners may be unwilling to grant us exclusive rights to technology or products developed through such collaborations. There is also a risk that disputes may arise as to the rights to technology or products developed in collaboration with other parties. Soliris and our drug candidates are expensive and time-consuming to test and develop. Even if we obtain and maintain patents, our business may be significantly harmed if the patents are not broad enough to protect our drugs from copycat products.

In addition, our business requires using sensitive technology, techniques and proprietary compounds that we protect as trade secrets. However, we may also rely heavily on collaboration with, or discuss the potential for collaboration with, suppliers, outside scientists and other drug companies. Collaboration and discussion of potential collaboration present a strong risk of exposing our trade secrets. If our trade secrets were exposed, it would help our competitors and adversely affect our business prospects.

If we are found to be infringing on patents owned by others, we may be forced to pay damages to the patent owner and/or obtain a license to continue the manufacture, sale or development of our drugs. If we cannot obtain a license, we may be prevented from the manufacture, sale or development of our drugs, including Soliris, which would

adversely affect our business.

Parts of our technology, techniques and proprietary compounds and potential drug candidates, including those which are or may be in-licensed, may be found to infringe patents owned by or granted to others. On January 26, 2011, Novartis filed a civil action against us and other biopharmaceuticals companies claiming willful infringement by us of its patent. If it is finally determined that we infringe the Novartis patent, we may be required to pay royalties to Novartis on sales of Soliris regarding certain manufacturing technology. Although we do not believe that the manufacture of Soliris infringes a valid patent claim

owned by Novartis, we cannot guarantee that we will be successful in defending against such action. In addition to Novartis, other third parties may claim that the manufacture, use or sale of Soliris or other drugs under development infringes patents owned or granted to such third parties. We are aware of broad patents owned by others relating to the manufacture, use and sale of recombinant humanized antibodies, recombinant human antibodies, and recombinant human single chain antibodies. Soliris and many of our product candidates are genetically engineered antibodies, including recombinant humanized antibodies, recombinant human antibodies, or recombinant human single chain antibodies. In addition to the actions described above, we have received and may receive notices from the owners of some of these broad patents claiming that their patents may be infringed by the development, manufacture or sale of Soliris or some of our drug candidates. We are also aware of other patents owned by third parties that might be claimed by such third parties to be infringed by the development and commercialization of Soliris and some of our drug candidates. In respect to some of these patents, we have obtained licenses, or expect to obtain licenses. However, with regard to such other patents, we have determined in our judgment that:

- Soliris and our product candidates do not infringe the patents;
- the patents are not valid; or
- we have identified and tested or are testing various modifications that we believe should not infringe the patents and which should permit commercialization of our product candidates.

Any holder of these patents or other patents covering similar technology could sue us for damages and seek to prevent us from manufacturing, selling or developing our drugs. Legal disputes can be costly and time consuming to defend. If we cannot successfully defend against any future actions or conflicts, if they arise, we may incur substantial legal costs and may be liable for damages, be required to obtain costly licenses or need to stop manufacturing, using or selling Soliris, which would adversely affect our business. We may seek to obtain a license prior to or during legal actions in order to reduce further costs and the risk of a court determination that our product infringes the third party's patents. A required license may be costly or may not be available on acceptable terms, if at all. A costly license, or inability to obtain a necessary license, could have a material adverse effect on our business.

There can be no assurance that we would prevail in a patent infringement action or that we would be able to obtain a license to any third-party patent on commercially reasonable terms or any terms at all; successfully develop non-infringing alternatives on a timely basis; or license alternative non-infringing technology, if any exists, on commercially reasonable terms. Any impediment to our ability to manufacture, use or sell approved forms of Soliris or our product candidates could have a material adverse effect on our business and prospects.

Risks Related to Our Operations

We cannot guarantee that we will achieve our financial goals, including our ability to maintain profitability on a quarterly or annual basis in the future.

Until the quarter ended June 30, 2008, we had never been profitable since we were incorporated in January 1992. We have maintained profitability on a quarterly basis since the quarter ended June 30, 2008 and on an annual basis beginning with the year ended December 31, 2008. We believe that we formulate our annual operating budgets with reasonable assumptions and targets, however we cannot guarantee that we will be able to generate sufficient revenues or control expenses to achieve our financial goals, including continued profitability. Even if we do achieve profitability in any subsequent quarters, we may not be able to sustain or increase profitability on a quarterly or annual basis. You should not consider our revenue growth in recent periods as indicative of our future performance. Our revenue in future periods could decline. We may make errors in predicting and reacting to relevant business trends or our business may be subject to factors beyond our control, which could harm our operations. Since we began our business, we have focused on research and development of product candidates. We launched Soliris for sale for the treatment of patients with PNH in the United States and Europe during 2007. We obtained marketing approval from the FDA and the EC for Soliris for the treatment of patients with aHUS in September and November 2011, respectively, and have not obtained marketing approval for aHUS in any other country or territory. We cannot guarantee that we will be successful in marketing and selling Soliris on a continued basis in countries or regions where we have obtained marketing approval, including the United States, Europe and Japan, and we do not know when we will have Soliris available for sale in territories where we have applied or will apply for marketing approval, if ever. We incurred significant debt to finance the acquisition of Enobia and we will have substantial expenses as we continue our research and development efforts, continue to conduct clinical trials and continue to develop

manufacturing, sales, marketing and distribution capabilities in the United States and abroad. The achievement of our financial goals, including the extent of our future profitability, depends on many factors, including our ability to successfully market Soliris in the United States, the European Union and Japan and other territories, our ability to obtain regulatory, pricing, coverage, and reimbursement approvals of Soliris in additional countries and regions and for aHUS and other indications, our ability to successfully market Soliris in additional countries and regions, our ability to successfully manufacture and commercialize our drug candidates and our ability to successfully bring our other product candidates, including product candidates we acquired from Enobia, Taligen and Orphatec, to the major commercial

markets throughout the world.

If our competitors get to the marketplace before we do, or with better or less expensive drugs, it may not be profitable to continue to produce Soliris and our product candidates.

The FDA, EC and the MHLW granted orphan drug designation for Soliris in the treatment of PNH and the FDA and EC granted orphan drug designation for aHUS. Orphan drug status which entitles Soliris to market exclusivity for a total of seven years in the United States and for ten years in the European Union and Japan. However, if a competitive product that is the same as or similar to Soliris, as defined under the applicable regulations, is shown to be clinically superior to Soliris in the treatment of PNH or aHUS, or if a competitive product is different from Soliris, as defined under the applicable regulations, the orphan drug exclusivity we have obtained may not block the approval of such competitive product. Several biotechnology and pharmaceutical companies throughout the world have programs to develop complement inhibitor therapies or have publicly announced their intentions to develop drugs which target the inflammatory effects of complement in the immune system. These and other companies, many of which have significantly greater resources than us, may develop, manufacture, and market better or cheaper drugs than Soliris or our product candidates. They may establish themselves in the marketplace before us for Soliris for other indications or for any of our other product candidates. Other pharmaceutical companies also compete with us to attract academic research institutions as drug development partners, including for licensing these institutions' proprietary technology. If our competitors successfully enter into such arrangements with academic institutions, we will be precluded from pursuing those unique opportunities and may not be able to find equivalent opportunities elsewhere.

If we fail to satisfy our debt service obligations or obtain the capital necessary to fund our operations, we will be unable to continue the commercialization of Soliris or continue or complete our product development.

In February 2012, we acquired Enobia and made an upfront payment of $623,876. We used a substantial portion of our cash on hand and incurred $320,000 of debt under the terms of a senior secured credit facility to finance the acquisition. In addition, the definitive agreements for the Enobia, Taligen and Orphatec acquisitions include contingent payments totaling $470,000, $367,000 and $42,000, respectively, if and when certain development and commercial milestones are achieved. In May 2012, Alexion issued 5,000 shares of its common stock in a public offering resulting in net proceeds of approximately $462,000. We believe that revenues and collections from sales of Soliris along with our existing cash and cash equivalents will provide sufficient capital to satisfy our debt service obligations and the contingent consideration required by the acquisitions, and to fund our operations and product development for at least 12 months. We may need to raise additional capital before or after that time to complete or continue the development or commercialization of our products and product candidates or for other purposes. We are currently selling or preparing for the commercialization of Soliris in the United States, the European Union, Japan, and several other territories, evaluating and preparing regulatory submissions for Soliris in several countries, and conducting, preparing or evaluating several clinical trials. Funding needs may shift between projects and potentially accelerate and increase as we continue launch and commercialization activities throughout the world and as we initiate or continue clinical trials for our product candidates.

Additional financing could take the form of public or private debt or equity offerings, equity line facilities, bank loans, including additional borrowing under our existing credit facility, collaborative research and development arrangements with corporate partners and/or the sale or licensing of some of our property. The amount of capital we may need depends on many factors, including:

- the cost necessary to sell, market and distribute Soliris;
- the rate of new patient sales and drug utilization by treated patients;
- the time and cost necessary to obtain and maintain regulatory approvals for Soliris in multiple countries;
- the ability to obtain and maintain reimbursement approvals and funding for Soliris and the time necessary to obtain such approvals and funding;
- the time and cost necessary to develop sales, marketing and distribution capabilities outside the United States;
- the time and cost necessary to purchase or to further develop manufacturing processes, arrange for contract manufacturing or build manufacturing facilities and obtain and maintain the necessary regulatory approvals for those facilities;
- changes in applicable governmental regulatory policies or requests by regulatory agencies for additional information or data;

- the progress, timing and scope of our research and development programs;
- the progress, timing and scope of our preclinical studies and clinical trials;

- the integration of the Enobia, Taligen and Orphatec businesses;
- any new collaborative, licensing or other commercial relationships that we may establish; and
- the cost of any acquisition.

We may not receive additional funding when we need it or funding may only be available on unfavorable terms.

Financial markets in the United States, Europe and the rest of the world have been experiencing significant volatility in security prices, substantially diminished liquidity and credit availability, rating downgrades of certain investments and declining valuations of others. There can be no assurance that we will be able to access additional credit or the equity markets in order to finance our operations, grow our operations in any territory, or expand development programs for our product candidates, or that there will not be a further deterioration in financial markets and confidence in economies. If we cannot raise adequate funds to satisfy our capital requirements, we may have to delay, scale-back or eliminate our research and development activities or future operations. We might have to license our technology to others or relinquish commercialization rights. This could result in sharing revenues that we might otherwise retain for ourselves. Any of these actions would harm our business.

If we fail to recruit and retain personnel, we may not be able to implement our business strategy.

We are highly dependent upon the efforts of our senior management and scientific personnel, particularly Dr. Leonard Bell, M.D., our Chief Executive Officer and a member of our Board of Directors, and Dr. Stephen P. Squinto, Ph.D., our Executive Vice President and Head of Research and Development. There is intense competition in the biopharmaceutical industry for qualified scientific and technical personnel. Since our business is science-oriented and specialized, we need to continue to attract and retain such people. We may not be able to continue to attract and retain the qualified personnel necessary for developing our business. We have employment agreements with Dr. Bell and Dr. Squinto. None of our key personnel is nearing retirement age or to our knowledge, planning to retire. To our knowledge, there is no tension between any of our key personnel and the Board of Directors. If we are unable to retain and recruit highly qualified personnel, our ability to execute our business plan will be materially and adversely affected.

In particular, we highly value the services of Dr. Bell, our Chief Executive Officer. The loss of his services could materially and adversely affect our ability to achieve our objectives.

We are significantly leveraged.

In February 2012 we and our wholly-owned Swiss subsidiary, Alexion Pharma International Sàrl, entered into the Credit Agreement with a syndicate of banks. The Credit Agreement provides for a $240,000 senior secured term loan facility and a $200,000 senior secured revolving credit facility, which includes up to a $60,000 sublimit for letters of credit and a $10,000 sublimit for swingline loans. Our obligations under the credit facilities are unconditionally guaranteed, jointly and severally, by certain of our existing domestic subsidiaries and are required to be guaranteed by certain of our future domestic subsidiaries. The obligations of Alexion Pharma International Sàrl under the credit facilities are unconditionally guaranteed, jointly and severally, by us, certain of our existing domestic subsidiaries, and certain of our foreign subsidiaries, and are required to be guaranteed by certain of our future subsidiaries. All obligations of each borrower under the credit facilities, and the guarantees of those obligations, are secured, subject to certain exceptions, by substantially all of each borrower's assets and the assets of certain guarantors, including the pledge of the equity interests of certain of our subsidiaries and real estate located in Smithfield, Rhode Island, but excluding intellectual property and assets of certain foreign subsidiaries. We may elect that the loans under the credit facilities bear interest at a rate per annum equal to (i) LIBOR plus 1.25% to 2.00% depending on our consolidated leverage ratio (as calculated in accordance with the Credit Agreement), or (ii) in the case of borrowings in U.S. dollars, a Base Rate equal to the higher of the (A) Prime Rate then in effect, (B) Federal Funds Rate then in effect plus 0.50%, and (C) Eurodollar Rate then in effect plus 1.00%, plus in each case of (A), (B) or (C), 0.25% to 1.00% depending on our consolidated leverage ratio (as calculated in accordance with the Credit Agreement).

The credit facilities, and the contingent consideration payable in connection with our acquisitions remain outstanding or available, and the degree to which we are leveraged could, among other things:

- make it difficult for us to make payments on the credit facilities;
- make it difficult for us to obtain financing for additional acquisitions or in-licensing opportunities or other purposes on favorable terms, if at all;
-

- make us more vulnerable to industry downturns and competitive pressures; and
- limit our flexibility in planning for, or reacting to changes in, our business.

Our ability to meet our debt service obligations will depend upon our future performance, which will be subject to financial, business and other factors affecting our operations, many of which are beyond our control.

We are subject to environmental laws and potential exposure to environmental liabilities.

We are subject to various federal, state and local environmental laws and regulations that govern our operations, including our manufacturing operations at ARIMF, the handling and disposal of non-hazardous and hazardous wastes, such as medical and biological wastes, and emissions and discharges into the environment, such as air, soils and water sources. Failure to comply with such laws and regulations could result in costs for corrective action, penalties or the imposition of other liabilities. We also are subject to laws and regulations that impose liability and clean-up responsibility for releases of hazardous substances into the environment. Under certain of these laws and regulations, a current or previous owner or operator of property may be liable for the costs of remediating its property or locations to which wastes were sent from its facilities, without regard to whether the owner or operator knew of, or necessarily caused, the contamination. Such obligations and liabilities, which to date have not been material, could have a material impact on our business and financial condition.

We may expand our business through acquisitions or in-licensing opportunities that could disrupt our business and harm our financial condition.

Our business strategy includes expanding our products and capabilities. In 2011, we acquired Taligen and certain assets of Orphatec. In February 2012 we acquired Enobia. We may seek additional acquisitions or in-licensing of businesses or products to expand our products and capabilities. Acquisitions of new businesses or products, including the Enobia, Taligen and Orphatec acquisitions, and in-licensing of new products may involve numerous risks, including:

- substantial cash expenditures;
- potentially dilutive issuance of equity securities;
- incurrence of debt and contingent liabilities, some of which may be difficult or impossible to identify at the time of acquisition;
- difficulties in assimilating the operations of the acquired companies;
- diverting our management's attention away from other business concerns;
- risks of entering markets in which we have limited or no direct experience;
- the potential loss of our key employees or key employees of the acquired companies; and
- failure of any acquired businesses or products or in-licensed products to achieve the scientific, medical, commercial or other results anticipated.

We have limited experience in the acquisition and integration of other companies. We cannot assure you that the Enobia, Taligen and Orphatec acquisitions, or any other acquisition or in-licensing of new products, will result in short-term or long-term benefits to us. We may incorrectly judge the value or worth of an acquired company or business, such as Enobia, Taligen or Orphatec, or an acquired or in-licensed product. In addition, the future success of such transactions would depend in part on our ability to manage the rapid growth associated with any such acquisitions or in-licensing. We cannot assure you that we will be able to make the combination of our business with that of Enobia, Taligen or Orphatec, or any other acquired businesses or companies work or be successful.

We compete with pharmaceutical companies that have significantly greater resources than we for many of the same acquisition and in-licensing opportunities. Such pharmaceutical companies may be less leveraged and have better access to capital resources that may preclude us from completing any acquisition or in-licensing. For this and other reasons, we may not be able to acquire the rights to additional product candidates and approved products on terms that we find acceptable, or at all. Furthermore, the development or expansion of our business, any acquired business or any acquired or in-licensed products may require a substantial capital investment by us. We may not have these necessary funds or they might not be available to us on acceptable terms or at all. We may also seek to raise funds by selling shares of our capital stock, which could dilute current stockholders' ownership interest in our company, or securities convertible into our capital stock, which could dilute current stockholders' ownership interest in our company upon conversion.

Our ability to use net operating loss carry forwards to reduce future tax payments may be limited if there is a change in ownership of Alexion, or if taxable income does not reach sufficient levels.

As of December 31, 2012, we had $453,617 of U.S. federal net operating loss carryforwards (NOL's), available to reduce taxable income in future years. Included in our U.S. federal net operating losses is $10,337 associated with the acquisition of Enobia. A portion of these NOL's are currently subject to an annual limitation under section 382 of the

Internal Revenue Code of 1986, as amended (section 382). We believe it is more likely than not that we will use the majority of net operating losses. However, the ability to use net operating loss carryforwards will be dependent on our ability to generate taxable income. The net operating loss carryforwards may expire before we generate sufficient taxable income.

Our ability to utilize the NOL's may be further limited if we undergo an ownership change, as defined in section 382. This ownership change could be triggered by substantial changes in the ownership of our outstanding stock, which are generally outside of our control. An ownership change would exist if the stockholders, or group of stockholders, who own or have owned, directly or indirectly, 5% or more of the value of our stock, or are otherwise treated as 5% stockholders under section 382 and the regulations promulgated there under, increase their aggregate percentage ownership of our stock by more than 50 percentage points over the lowest percentage of our stock owned by these stockholders at any time during the testing period, which is generally the three-year period preceding the potential ownership change. In the event of an ownership change, section 382 imposes an annual limitation on the amount of post-ownership change taxable income a corporation may offset with pre-ownership change NOL's. The limitation imposed by section 382 for any post-change year would be determined by multiplying the value of our stock immediately before the ownership change (subject to certain adjustments) by the applicable long-term tax-exempt rate. Any unused annual limitation may be carried over to later years, and the limitation may under certain circumstances be increased by built-in gains which may be present with respect to assets held by us at the time of the ownership change that are recognized in the five-year period after the ownership change. Our use of NOL's arising after the date of an ownership change would not be affected.

We may have exposure to additional tax liabilities which could have a material impact on our results of operations and financial position.

As a company with international operations, we are subject to income taxes, as well as non-income based taxes, in both the United States and various foreign jurisdictions. Significant judgment is required in determining our worldwide tax liabilities. Although we believe our estimates are reasonable, the ultimate outcome with respect to the taxes we owe may differ from the amounts recorded in our financial statements. If the Internal Revenue Service, or other taxing authority, disagrees with the positions we take, we could have additional tax liability, and this could have a material impact on our results of operations and financial position. In addition, the United States government and other governments are considering and may adopt tax reform measures that significantly increase our worldwide tax liabilities which could materially harm our business, financial condition and results of operations.

Our sales and 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 sales and operations to be limited or disrupted.

Since 2007, we have significantly expanded our operations and expect to continue to do so in the future. Our operations in foreign countries subject us to the following additional risks:

- fluctuations in currency exchange rates;
- political or economic determinations that adversely impact pricing or reimbursement policies;
- economic problems or political instability that disrupt health care payment systems;
- difficulties or inability to obtain financing in markets;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- difficulties enforcing contractual and intellectual property rights;
- changes in laws, regulations or enforcement practices with respect to our business, including without limitation laws relating to reimbursement, competition, pricing and sales and marketing of our products;
- trade restrictions and restrictions on direct investments by foreign entities;
- 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;
- costs and difficulties in managing and monitoring international operations; and
- longer payment cycles.

Our business and marketing methods are also subject to regulation by the governments of the countries in which we operate. The FCPA and similar anti-bribery laws in other countries prohibit companies and their representatives from offering, promising, authorizing or making payments to foreign officials for the purpose of obtaining or retaining business. We have policies and procedures designed to help ensure that we and our representatives, including our employees, comply with such laws, however we cannot guarantee that these policies and procedures will protect us against liability under the FCPA or other anti-bribery laws for actions taken by our representatives. Failure to comply

with the laws and regulations of the countries in

which we operate could materially harm our business.

We conduct, or anticipate that we will conduct, a substantial portion of our business in currencies other than the U.S. dollar. We are exposed to fluctuations in foreign currency exchange rates in the normal course of our business. The exposures result from portions of our revenues, as well as the related receivables, and expenses that are denominated in currencies other than the US dollar, primarily the Euro, Japanese Yen and Swiss Franc. We manage our foreign currency transaction risk within specified guidelines through the use of derivatives. All of our derivative instruments are utilized for risk management purposes, and we do not use derivatives for speculative trading purposes. We enter into foreign exchange forward contracts, with durations of up to 36 months, to hedge exposures resulting from portions of our forecasted intercompany revenues that are denominated in currencies other than the U.S. dollar. The purpose of the hedges of intercompany revenue is to reduce the volatility of exchange rate fluctuations on our operating results and to increase the visibility of the foreign exchange impact on forecasted revenues. Further, we enter into foreign exchange forward contracts, with durations of approximately 30 days, designed to limit the balance sheet exposure of monetary assets and liabilities. We enter into these hedges to reduce the impact of fluctuating exchange rates on our operating results. Gains and losses on these hedge transactions are designed to offset gains and losses on underlying balance sheet exposures. While we attempt to hedge certain currency risks, currency fluctuations between the U.S. dollar and the currencies in which we do business have, in the past, caused foreign currency transaction gains and losses and have also impacted the amounts of revenues and expenses calculated in U.S. dollars and will likely do so in the future. Likewise, past currency fluctuations have at times resulted in foreign currency transaction gains, and there can be no assurance that these gains can be reproduced. See also Footnote 6, Derivative Instruments and Hedging Activities, in the Consolidated Financial Statements included in this Annual Report on Form 10-K.

The credit and financial market conditions may aggravate certain risks affecting our business.

Sales of Soliris are dependent, in large part, on reimbursement from government health administration organizations and private and governmental third-party payers, and also co-payments from individual patients in certain situations. As a result of adverse credit and financial market conditions, and the overall financial climate, these governmental organizations and payers, and/or individuals, may reduce or delay initiation of treatment, may be unable to satisfy their reimbursement obligations, may delay payment or may seek to reduce reimbursement for Soliris in the future, which could have a material adverse effect on our business and results of operations. For example, in July 2011, we received non-interest bearing bonds issued by the Greek government that mature in 2012 and 2013 for payment on receivables from 2008 and 2009 as part of the Greek government's plan repayment of its debt to international pharmaceutical companies. We sold the associated bonds in July 2011 and recorded expense of approximately $4,100 through December 31, 2011 related to the reduction of value of the Greek bonds and other delays impacting the book value of our accounts receivable in other countries. Soliris is approved for the treatment of patients with PNH and aHUS in the United States and the European Union and for the treatment of PNH in several other territories. If Soliris is approved in additional territories for PNH, aHUS, or for additional indications that are under clinical development, the reimbursement risks and uncertainties associated with adverse credit and financial market conditions may be exacerbated due to increases in the number of patients receiving Soliris that require reimbursement. Payment defaults by a government payer could require us to expense previously recorded revenue as uncollectible, and might cause us to end or restrict sales to patients in that country. Further, the risk of payment default by a government payer could require us to revise our revenue recognition policies in regard to that payer, causing revenue to be recorded only on a cash basis, and we may be required to end or restrict sales to patients in that country.

We continue to monitor economic conditions, including volatility associated with international economies, associated impacts on the financial markets and our business, and the sovereign debt crisis in Europe. The credit and economic conditions in Greece, Italy and Spain, among other members of the European Union deteriorated in 2011 and 2012. These conditions have resulted in, and may continue to result in, an increase in the average length of time it takes to collect our outstanding accounts receivable in these countries. We have recorded an allowance related to all or a portion of receivables in each of Greece, Italy and Spain that have been outstanding for greater than one year as of December 31, 2012.

We may not be able to successfully mitigate or prevent our exposures to volatile economic and financial conditions and our failure to operate successfully or adapt to changes in these conditions could cause our sales and operations to

be limited or disrupted or otherwise harm our business.

Additionally, we rely upon third-parties for certain parts of our business, including Lonza, licensees, wholesale distributors of Soliris, contract clinical trial providers, contract manufacturers and other third-party suppliers and financial institutions. Because of the volatility in the financial markets, there may be a disruption or delay in the performance or satisfaction of commitments to us by these third parties which could have a material adverse effect on our business and results of operations.

Government initiatives that affect coverage and reimbursement of drug products could adversely affect our business. Governments in countries where we operate have adopted or have shown significant interest in pursuing legislative initiatives to reduce costs of health care. Any such government-adopted health care measures could adversely impact the pricing of Soliris or the amount of coverage and reimbursement available for Soliris from governmental agencies or other third-party payers. For example, in March 2010, the President signed the PPACA, which substantially changes the way healthcare is financed by both governmental and private insurers, and significantly impacts the pharmaceutical industry. PPACA contains a number of provisions that are expected to impact our business and operations, in some cases in ways we cannot currently predict. Changes that may affect our business include those governing enrollment in federal healthcare programs, reimbursement changes, rules regarding prescription drug benefits under health insurance exchanges, and fraud and abuse enforcement. While the constitutionality of key provisions of PPACA have been upheld by the Supreme Court, legislative changes remain possible. In addition, our industry may be affected by broader legislation addressing federal spending, including, for example, a sequester that is scheduled to take effect in March 2013 and cuts to most Medicare spending by 2%. As another example, the governments of Germany and Spain each approved increases to mandatory rebates on the sales of pharmaceutical products. Further in January 2013, Alexion was informed by the Advisory Group for National Specialised Services (AGNSS) within the U.K. National Health Service that although Soliris would help save lives and improve the quality of life for children and adults with aHUS, the U.K. Health Ministers decided not to recommend national commissioning of Soliris for the treatment of aHUS at this stage. Rather, Soliris will be referred to NICE for further review as part of its new Highly Specialised Technologies programme.

We expect that the implementation of current laws and policies, the amendment of those laws and policies in the future, as well as the adoption of new laws and policies, could have a material adverse effect on our industry generally and on our ability to maintain or increase our product sales or successfully commercialize our product candidates, or could limit or eliminate our future spending on development projects. In many cases, these government initiatives, even if enacted into law, are subject to future rulemaking by regulatory agencies. Although we have evaluated these government initiatives and the impact on our business, we cannot know with certainty whether any such law, rule or regulation will adversely affect coverage and reimbursement of Soliris, or to what extent, until such laws, rules and regulations are promulgated, implemented and enforced. The announcement or adoption of regulatory or legislative proposals could delay or prevent our entry into new markets, affect our reimbursement or sales in the markets where we are already selling Soliris and materially harm our business, financial condition and results of operations.

Security breaches and other disruptions could compromise our information and expose us to liability, which would cause our business and reputation to suffer.

In the ordinary course of our business, we collect and store sensitive data, including intellectual property, our proprietary business information and that of our suppliers, customers and business partners, and personally identifiable information. The secure maintenance of this information is critical to our operations and business strategy. Despite our security measures, our information technology and infrastructure may be vulnerable to attacks by hackers or breached due to employee error, malfeasance or other disruptions. Any such breach could compromise our networks and the information stored there could be accessed, publicly disclosed, lost or stolen. Any such access, disclosure or other loss of information could result in legal claims or proceedings, liability under laws that protect the privacy of personal information, disrupt our operations, and damage our reputation which could adversely affect our business.

Natural disasters, acts of war or terrorism, shipping embargoes, labor unrest or political instability, could adversely affect our operations, including our ability to supply and commercialize Soliris.

Natural disasters such as earthquakes, hurricanes, tsunamis or other adverse weather and climate conditions, whether occurring in the U.S. or abroad, and the effects of these natural disasters, as well as acts of war or terrorism, shipping embargoes, labor unrest or political instability could disrupt our operations, or the operations of our vendors and other suppliers. Such events could adversely impact our facilities, or interfere with the manufacture or distribution of Soliris and our product candidates.

Risks Related to Our Common Stock

If the trading price of our common stock continues to fluctuate in a wide range, our stockholders will suffer considerable uncertainty with respect to an investment in our common stock.

The trading price of our common stock has been volatile and may continue to be volatile in the future. Factors such as announcements of fluctuations in our or our competitors' operating results or clinical or scientific results, fluctuations in the trading prices or business prospects of our competitors and collaborators, changes in our prospects, particularly with respect to sales of Soliris, and market conditions for biopharmaceutical stocks in general could have a significant impact on the future trading prices of our common stock. In particular, the trading price of the common stock of many biopharmaceutical companies, including ours, has experienced extreme price and volume fluctuations, which have at times been unrelated to the operating performance of the companies whose stocks were affected. This is due to several factors, including general market conditions, sales of Soliris, the announcement of the results of our clinical trials or product development and the results of our efforts to obtain regulatory approval for our products. While we cannot predict our future performance, if our stock price continues to fluctuate in a wide range, an investment in our common stock may result in considerable uncertainty for an investor.

Anti-takeover provisions of Delaware law, provisions in our charter and bylaws and our stockholders' rights plan, or poison pill, could make a third-party acquisition of us difficult and may frustrate any attempt to remove or replace our current management.

Because we are a Delaware corporation, the anti-takeover provisions of Delaware law could make it more difficult for a third party to acquire control of us, even if the change in control would be beneficial to stockholders. We are subject to the provisions of Section 203 of the Delaware General Laws, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

Our corporate charter and by-law provisions and stockholder rights plan may discourage certain types of transactions involving an actual or potential change of control that might be beneficial to us or our stockholders. Our bylaws provide that special meetings of our stockholders may be called only by the Chairman of the Board, the President, the Secretary, or a majority of the Board of Directors, or upon the written request of stockholders who together own of record 50% of the outstanding stock of all classes entitled to vote at such meeting. Our bylaws also specify that the authorized number of directors may be changed only by resolution of the board of directors. Our charter does not include a provision for cumulative voting for directors, which may have enabled a minority stockholder holding a sufficient percentage of a class of shares to elect one or more directors. Under our charter, our board of directors has the authority, without further action by stockholders, to designate up to 5,000 shares of preferred stock in one or more series. The rights of the holders of common stock will be subject to, and may be adversely affected by, the rights of the holders of any class or series of preferred stock that may be issued in the future.

Pursuant to our stockholder rights plan, each share of common stock has an associated preferred stock purchase right. The rights will not trade separately from the common stock until, and are exercisable only upon, the acquisition or the potential acquisition through tender offer by a person or group of 20% or more of the outstanding common stock. The rights are designed to make it more likely that all of our stockholders receive fair and equal treatment in the event of any proposed takeover of us and to guard against the use of partial tender offers or other coercive tactics to gain control of us. These provisions could delay or discourage transactions involving an actual or potential change in control of us or our management, including transactions in which stockholders might otherwise receive a premium for their shares over then current prices. These provisions could also limit the ability of stockholders to remove current management or approve transactions that stockholders may deem to be in their best interests and could adversely affect the price of our common stock.

Item 1B. UNRESOLVED STAFF COMMENTS.

None.

Item 2. PROPERTIES.

We conduct our primary operations at the owned and leased facilities described below.

Location	Operations Conducted	Approximate Square Feet	Lease Expiration Dates
Cheshire, Connecticut	Executive, sales, research and development offices	235,300	2016 and 2020
Smithfield, Rhode Island	Commercial, research and development manufacturing	67,000	N/A
Lausanne, Switzerland	Regional executive and sales office	40,000	2014

We believe that our administrative office space is adequate to meet our needs for the foreseeable future. We also believe that our research and development facilities and our manufacturing facility, together with third party manufacturing facilities, will be adequate for our on-going activities. In addition to the locations above, we also lease space in other U.S. states and foreign countries to support our operations as a global organization.

In November 2012, we entered into a new lease agreement for approximately 283,000 square feet of office and laboratory space to be constructed in New Haven, Connecticut. The construction of the facility is expected to begin in 2013 and be completed in 2015. Upon completion of the new facility, we will relocate our headquarters and Cheshire operations to New Haven.

Item 3. LEGAL PROCEEDINGS.

On January 26, 2011, Novartis Vaccines & Diagnostics, Inc. (Novartis) filed a civil action against Alexion and other biopharmaceutical companies in the U.S. District Court for the District of Delaware. Novartis claims willful infringement by Alexion of a Novartis patent and seeks, among other things, monetary damages.

Item 4. MINE SAFETY DISCLOSURES.

None.

PART II

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

Our common stock is quoted on The Nasdaq Stock Market, LLC under the symbol "ALXN." The following table sets forth the range of high and low sales prices for our common stock on The Nasdaq Stock Market, LLC for the periods indicated since January 1, 2011.

Fiscal 2011	High	Low
First Quarter (January 1, 2011 to March 31, 2011)	$49.87	$40.67
Second Quarter (April 1, 2011 to June 30, 2011)	$52.19	$44.61
Third Quarter (July 1, 2011 to September 30, 2011)	$66.99	$47.51
Fourth Quarter (October 1, 2011 to December 31, 2011)	$71.55	$60.87
Fiscal 2012		
First Quarter (January 1, 2012 to March 31, 2012)	$95.01	$69.82
Second Quarter (April 1, 2012 to June 30, 2012)	$99.70	$81.28
Third Quarter (July 1, 2012 to September 30, 2012)	$116.43	$94.80
Fourth Quarter (October 1, 2012 to December 31, 2012)	$119.54	$86.20

As of February 1, 2013, we had approximately 391 stockholders of record of our common stock and an estimated 122,035 beneficial owners. The closing sale price of our common stock on February 1, 2013 was $97.87 per share.

DIVIDEND POLICY

We have never paid cash dividends. We do not expect to declare or pay any cash dividends on our common stock in the near future. We intend to retain all earnings, if any, to invest in our operations. The payment of future dividends is within the discretion of our board of directors and will depend upon our future earnings, if any, our capital requirements, financial condition and other relevant factors.

ISSUER PURCHASES OF EQUITY SECURITIES (amounts in thousands except per share amounts)

The following table summarizes our common stock repurchase activity during the fourth quarter of 2012:

Period	Total Number of Shares Purchased	Average Price Paid per Share	Total Number of Shares Purchased as Part of Publicly Announced Programs	Maximum Dollar Value of Shares that May Yet Be Purchased Under the Program
October 2012	—	$—	—	$—
November 2012	127	$88.60	127	$388,703
December 2012	3	$90.50	3	$388,447
Total	130	$88.64	130	

On November 8, 2012, we announced that our Board of Directors authorized the repurchase of up to $400,000 of our common stock. This repurchase program does not have an expiration date.

EQUITY COMPENSATION PLAN INFORMATION (amounts in thousands except per share amounts)

Plan Category	Number of shares of common stock to be issued upon exercise of outstanding options (2)	Weighted-average exercise price of outstanding options	Weighted-average term to expiration of options outstanding	Number of shares of common stock remaining available for future issuance under equity compensation plans
Equity compensation plans approved by stockholders (1)	9,557	$35.92	6.77	5,132
Equity compensation plans not approved by stockholders	—	$—	—	—

(1) Reflects number of shares of common stock to be issued upon exercise of outstanding options under all our equity compensation plans, including our 2004 Incentive Plan. All 5,132 shares of common stock remaining available for future issuance are available under the 2004 Incentive Plan.

(2) Does not include 1,761 restricted shares outstanding that were issued under the 2004 Incentive Plan.

The outstanding options and restricted shares are not transferable for consideration and do not have dividend equivalent rights attached.

THE COMPANY'S STOCK PERFORMANCE

The following graph compares cumulative total return of the Company's Common Stock with the cumulative total return of (i) the NASDAQ Stock Market-United States, and (ii) the NASDAQ Biotechnology Index. The graph assumes (a) $100 was invested on December 31, 2007 in each of the Company's Common Stock, the stocks comprising the NASDAQ Stock Market-United States 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.

CUMULATIVE TOTAL RETURN

	12/07	12/08	12/09	12/10	12/11	12/12
Alexion Pharmaceuticals, Inc.	100.00	96.47	130.13	214.71	381.18	499.75
NASDAQ Composite	100.00	59.03	82.25	97.32	98.63	110.78
NASDAQ Biotechnology	100.00	93.40	103.19	113.89	129.12	163.33

Item 6. SELECTED FINANCIAL DATA.

The following selected financial data is derived from, and should be read in conjunction with, the financial statements, including the notes thereto, and "Management's Discussion and Analysis of Financial Condition and Results of Operations" included elsewhere in this Annual Report on Form 10-K.

(amounts in thousands, except per share amounts)

Condensed Consolidated Statements of Operations:

	Year Ended December 31,				
	2012	2011	2010	2009	2008
Revenues:					
Net product sales	$1,134,114	$783,431	$540,957	$386,800	$259,004
Contract research revenue	—	—	—	—	95
Total revenues	1,134,114	783,431	540,957	386,800	259,099
Cost of sales:					
Cost of sales	126,214	93,140	64,437	45,059	28,366
Gain on intellectual property settlement	(53,377)	—	—	—	—
Total cost of sales	72,837	93,140	64,437	45,059	28,366
Operating expenses:					
Research and development	222,732	137,421	98,394	81,915	62,581
Selling, general and administrative	384,678	308,176	226,766	172,767	133,543
Acquisition-related costs	22,812	13,486	722	—	—
Impairment of intangible asset	26,300	—	—	—	—
Amortization of purchased intangible assets	417	382	—	—	—
Total operating expenses	656,939	459,465	325,882	254,682	196,124
Operating income	404,338	230,826	150,638	87,059	34,609
Interest and other income (expense)	(6,772)	(1,158)	(1,627)	(3,745)	121
Income before income taxes	397,566	229,668	149,011	83,314	34,730
Income tax provision (benefit)	142,744	54,353	51,981	(211,852)	1,581
Net income	$254,822	$175,315	$97,030	$295,166	$33,149
Earnings per common share					
Basic	$1.34	$0.96	$0.54	$1.73	$0.22
Diluted	$1.28	$0.91	$0.52	$1.63	$0.20
Shares used in computing earnings per common share					
Basic	190,461	183,220	178,542	170,652	155,360
Diluted	198,501	191,806	186,074	181,164	179,934

Condensed Consolidated Balance Sheet Data:

	As of December 31,				
	2012	2011	2010	2009	2008
Cash, cash equivalents and marketable securities	$989,501	$540,865	$361,605	$176,220	$138,012
Trade accounts receivable, net	295,598	244,288	168,732	113,731	74,476
Inventories	94,521	81,386	62,165	40,885	49,821
Deferred tax assets, current	26,086	19,132	19,643	16,726	972
Other current assets	89,894	55,599	34,411	25,894	13,820
Property, plant and equipment, net	165,629	165,852	162,240	164,691	139,885
Intangible assets, net	646,678	91,604	24,146	28,589	32,325
Goodwill	253,645	79,639	19,954	19,954	19,954
Deferred tax assets, noncurrent	13,954	103,868	154,569	194,308	3,397
Other noncurrent assets	38,054	12,518	4,572	5,403	4,889
Total assets	$2,613,560	$1,394,751	$1,012,037	$786,401	$477,551
Accounts payable and accrued expenses	271,275	199,653	123,056	78,445	54,855
Notes payable	—	—	—	—	27,500
Current portion of long-term debt	48,000	—	—	—	—
Other current liabilities	40,814	28,132	15,459	6,817	2,063
Mortgage loan	—	—	—	—	44,000
Convertible notes	—	—	3,718	9,918	97,222
Long-term debt	101,000	—	—	—	—
Contingent consideration	139,002	18,120	—	—	—
Other noncurrent liabilities	42,619	14,354	10,068	2,865	4,910
Total liabilities	$642,710	$260,259	$152,301	$98,045	$230,550
Total stockholders’ equity	1,970,850	1,134,492	859,736	688,356	247,001
Total liabilities and stockholders' equity	$2,613,560	$1,394,751	$1,012,037	$786,401	$477,551

Item 7. MANAGEMENT’S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS. (amounts in thousands, except percentages and per share data)

In addition to historical information, this report contains forward-looking statements that involve risks and uncertainties which may cause our actual results to differ materially from plans and results discussed in forward-looking statements. We encourage you to review the risks and uncertainties, discussed in the section entitled item 1A “Risk Factors”, and the “Note Regarding Forward-Looking Statements”, included at the beginning of this Annual Report on Form 10-K. The risks and uncertainties can cause actual results to differ significantly from those forecast in forward-looking statements or implied in historical results and trends.

The following discussion should be read in conjunction with our consolidated financial statements and related notes appearing elsewhere in this Annual Report on Form 10-K.

Overview

We are a biopharmaceutical company focused on serving patients with severe and ultra-rare disorders through the innovation, development and commercialization of life-transforming therapeutic products. Our marketed product Soliris is the first and only therapeutic approved for patients with two severe and ultra-rare disorders resulting from chronic uncontrolled activation of the complement component of the immune system: PNH, a life-threatening and ultra-rare blood disorder, and aHUS, a life-threatening and ultra-rare genetic disease. We are also evaluating

additional potential indications for Soliris in severe and ultra-rare diseases in which chronic uncontrolled complement activation is the underlying mechanism, and we are progressing in various stages of development with additional biotechnology product candidates as treatments for patients with

severe and ultra-rare disorders. We were incorporated in 1992 and began commercial sale of Soliris in 2007.

Soliris is designed to inhibit a specific aspect of the complement component of the immune system and thereby treat inflammation associated with chronic disorders in the therapeutic areas of hematology, nephrology, transplant rejection and neurology. Soliris is a humanized monoclonal antibody that effectively blocks terminal complement activity at the doses currently prescribed. The initial indication for which we received approval for Soliris is PNH. PNH is an ultra-rare, debilitating and life-threatening, genetic deficiency blood disorder defined by chronic uncontrolled complement activation leading to the destruction of red blood cells, or hemolysis. The chronic hemolysis in patients with PNH may be associated with life-threatening thromboses, recurrent pain, kidney disease, disabling fatigue, impaired quality of life, severe anemia, pulmonary hypertension, shortness of breath and intermittent episodes of dark-colored urine (hemoglobinuria).

Soliris was approved for the treatment of PNH by the FDA and the EC in 2007 and by MHLW in 2010, and has been approved in several other territories. Additionally, Soliris has been granted orphan drug designation for the treatment of PNH in the United States, Europe, Japan and several other territories.

In September 2011, Soliris was approved by the FDA for the treatment of pediatric and adult patients with aHUS. aHUS is a genetic ultra-rare disease characterized by chronic uncontrolled complement activation and thrombotic microangiopathy, the formation of blood clots in small blood vessels throughout the body, causing a reduction in platelet count (thrombocytopenia) and life-threatening damage to the kidney, brain, heart and other vital organs. Also, in November 2011, the EC granted marketing authorization for Soliris to treat pediatric and adult patients with aHUS in Europe. The FDA and EC granted Soliris orphan drug designation for the treatment of patients with aHUS.

On February 7, 2012, we acquired Enobia, a privately held clinical-stage biotechnology company based in Montreal, Canada and Cambridge, Massachusetts, in a transaction accounted for under the acquisition method of accounting for business combinations. The acquisition was intended to further our objective to develop and commercialize therapies for patients with severe, ultra-rare and life-threatening disorders. Enobia's lead product candidate, asfotase alfa, is a human recombinant targeted alkaline phosphatase enzyme-replacement therapy for patients suffering with hypophosphatasia (HPP), an ultra-rare, life-threatening, genetic metabolic disease for which there are no approved treatments. We made a cash payment of $610,000, subject to purchase price adjustments, for 100% of Enobia's capital stock. Additional contingent payments of up to an aggregate of $470,000 may be due upon reaching various regulatory and sales milestones. We financed the acquisition with a combination of existing cash and proceeds from our new credit facility.

On February 8, 2011, we acquired patents and assets from Orphatec related to an investigational therapy for patients with MoCD Type A, an ultra-rare genetic disorder characterized by severe brain damage and rapid death in newborns. We made initial payments of $3,050 in cash and may make additional future payments of up to $42,000 in contingent milestone payments upon various development, regulatory and commercial milestones.

On January 28, 2011, we acquired Taligen , a privately held development stage biotechnology company based in Cambridge, Massachusetts, in a transaction accounted for under the acquisition method of accounting for business combinations. The acquisition was intended to broaden our portfolio of preclinical compounds and to expand our capabilities in translational medicine. We acquired preclinical compounds and novel antibody and protein regulators of the complement inflammatory pathways. We made an upfront cash payment of $111,773 for 100% of Taligen’s equity interests. Additional contingent payments of up to an aggregate of $367,000 may be due upon the achievement of various development and commercial milestones in both the United States and European Union for up to six product candidates.

Critical Accounting Policies and the Use of Estimates

The significant accounting policies and basis of preparation of our consolidated financial statements are described in Note 1, “Business Overview and 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;
- Contingent liabilities;
- Inventories;

- Research and development expenses;
- Share-based compensation;
- Valuation of goodwill, acquired intangible assets and in-process research and development (IPR&D);
- Valuation of contingent consideration; and
- Income taxes.

Revenue Recognition

Net Product Sales

Our principal source of revenue is product sales. We recognize revenue from product sales when persuasive evidence of an arrangement exists, title to product and associated risk of loss has passed to the customer, the price is fixed or determinable, collection from the customer is reasonably assured, and we have no further performance obligations. Revenue is recorded upon receipt of the product by the end customer, which is typically a hospital, physician's office, private or government pharmacy or other health care facility. Amounts collected from customers and remitted to governmental authorities, such as value-added taxes (VAT) in foreign jurisdictions, are presented on a net basis in our statements of operations and do not impact net product sales.

In the United States, our customers are primarily specialty distributors and specialty pharmacies which supply physician office clinics, hospital outpatient clinics, infusion clinics or home health care providers. We also sell Soliris to government agencies. Outside the United States, our customers are primarily hospitals, hospital buying groups, pharmacies, other health care providers and distributors.

Because of factors such as the pricing of Soliris, the limited number of patients, the short period from product sale to patient infusion and the lack of contractual return rights, Soliris customers often carry limited inventory. We also monitor inventory within our sales channels to determine whether deferrals are appropriate based on factors such as inventory levels, contractual terms and financial strength of distributors.

In addition to sales in countries where Soliris is commercially available, we have also recorded revenue on sales for patients receiving Soliris treatment through named-patient programs. The relevant authorities or institutions in those countries have agreed to reimburse for product sold on a named-patient basis where Soliris has not received final approval for commercial sale.

We record estimated rebates payable under governmental programs, including Medicaid in the United States and other programs outside the United States, as a reduction of revenue at the time of product sale. Our calculations related to these rebate accruals require analysis of historical claim patterns and estimates of customer mix to determine which sales will be subject to rebates and the amount of such rebates. We update our estimates and assumptions each period and record any necessary adjustments, which may have an impact on revenue in the period in which the adjustment is made. Generally, the length of time between product sale and the processing and reporting of the rebates is three to six months.

We have entered into volume-based arrangements with governments in certain countries in which reimbursement is limited to a contractual amount. Under this type of arrangement, amounts billed in excess of the contractual limitation are repaid to these governments as a rebate. We estimate incremental discounts resulting from these contractual limitations, based on estimated sales during the limited period, and we apply the discount percentage to product shipments as a reduction of revenue. Our calculations related to these arrangements require estimation of sales during the limitation period, and adjustments in these estimates may have a material impact in the period in which these estimates change.

We have provided balances and activity in the rebates payable account for the years ended December 31, 2012, 2011 and 2010 as follows:

	Rebates Payable
Balance at December 31, 2009	$(4,068)
Current provisions relating to sales in current year	(11,314)
Payments/credits relating to sales in current year	6,488
Payments/credits relating to sales in prior years	4,234
Balance at December 31, 2010	$(4,660)
Current provisions relating to sales in current year	(36,045)
Payments/credits relating to sales in current year	15,226
Payments/credits relating to sales in prior years	3,733
Balance at December 31, 2011	$(21,746)
Current provisions relating to sales in current year	(81,132)
Payments/credits relating to sales in current year	22,634
Payments/credits relating to sales in prior years	17,910
Balance at December 31, 2012	$(62,334)

We record distribution and other fees paid to our customers as a reduction of revenue, unless we receive an identifiable and separate benefit for the consideration, and we can reasonably estimate the fair value of the benefit received. If both conditions are met, we record the consideration paid to the customer as an operating expense. These costs are typically known at the time of sale, resulting in minimal adjustments subsequent to the period of sale.

We enter into foreign exchange forward contracts to hedge exposures resulting from portions of our forecasted intercompany revenues that are denominated in currencies other than the U.S. dollar. These hedges are designated as cash flow hedges upon inception. We record the effective portion of these cash flow hedges to revenue in the period in which the sale is made to an unrelated third party and the derivative contract is settled.

We sell Soliris to a limited number of customers, and we evaluate the creditworthiness of each customer on a regular basis. In certain European countries, sales by us are subject to payment terms that are statutorily determined. This is primarily the case in countries where the payer is government-owned or government-funded, which we consider to be creditworthy. The length of time from sale to receipt of payment in certain countries typically exceeds our credit terms. In countries in which collections from customers extend beyond normal payment terms, we seek to collect interest. We record interest on customer receivables as interest income when collected. For non-interest bearing receivables with an estimated payment beyond one year, we discount the accounts receivable to present value at the date of sale, with a corresponding adjustment to revenue. If creditworthiness declines further, subsequent adjustments for further declines in credit rating are recorded as bad debt expense as a component of selling, general and administrative expense. We assess on an ongoing basis whether collectibility is reasonably assured at the time of sale and we also use judgments as to our ability to collect outstanding receivables and provide allowances for the portion of receivables if and when collection becomes doubtful.

We continue to monitor economic conditions, including volatility associated with international economies and the sovereign debt crisis in Europe, and the associated impacts on the financial markets and our business. For additional information related to our concentration of credit risk associated with certain international accounts receivable balances, refer to the "Liquidity and Capital Resources" section below.

Contingent liabilities

We are currently involved in various claims and legal proceedings. On a quarterly basis, we review the status of each significant matter and assess our potential financial exposure. If the potential loss from any claim, asserted or unasserted, or legal proceeding is considered probable and the amount can be reasonably estimated, we accrue a liability for the estimated loss. Because of uncertainties related to claims and litigation, accruals are based on our best estimates based on available information. On a periodic basis, as additional information becomes available, or based on specific events such as the outcome of litigation or settlement of claims, we may reassess the potential liability related to these matters and may revise these estimates, which could result in a material adverse adjustment to our

operating results.

Inventories

Inventories are stated at the lower of cost or estimated realizable value. We determine the cost of inventory using the weighted-average cost method.

We capitalize inventory produced for commercial sale, including costs incurred prior to regulatory approval but subsequent to the filing of a Biologics License Application (BLA) when the Company has determined that the inventory has probable future economic benefit.

Products that have been approved by the FDA or other regulatory authorities, such as Soliris, are also used in clinical programs to assess the safety and efficacy of the products for usage in diseases that have not been approved by the FDA or other regulatory authorities. The form of Soliris utilized for both commercial and clinical programs is identical and, as a result, the inventory has an "alternative future use" as defined in authoritative guidance. Raw materials and purchased drug product associated with clinical development programs are included in inventory and charged to research and development expense when the product enters the research and development process and no longer can be used for commercial purposes and, therefore, does not have an "alternative future use".

For products which are under development and have not yet been approved by regulatory authorities, purchased drug product is charged to research and development expense upon delivery. Delivery occurs when the inventory passes quality inspection and ownership transfers to us. Nonrefundable advance payments for research and development activities, including production of purchased drug product, are deferred and capitalized until the goods are delivered. We also recognize expense for raw materials purchased when the raw materials pass quality inspection and we have an obligation to pay for the materials.

We also capitalize the cost of inventory manufactured at ARIMF in property, plant and equipment prior to the approval of the facility by regulatory authorities.

We analyze our inventory levels to identify inventory that may expire prior to sale, inventory that has a cost basis in excess of its estimated realizable value, or inventory in excess of expected sales requirements. Although the manufacturing of our product is subject to strict quality control, certain batches or units of product may no longer meet quality specifications or may expire, which would require adjustments to our inventory values. Soliris currently has a maximum estimated life of 48 months and, based on our sales forecasts, we expect to realize the carrying value of the Soliris inventory. In the future, reduced demand, quality issues or excess supply beyond those anticipated by management may result in an adjustment to inventory levels, which would be recorded as an increase to cost of sales. The determination of whether or not inventory costs will be realizable requires estimates by our management. A critical input in this determination is future expected inventory requirements based on internal sales forecasts. We then compare these requirements to the expiry dates of inventory on hand. To the extent that inventory is expected to expire prior to being sold, we will write down the value of inventory. If actual results differ from those estimates, additional inventory write-offs may be required.

Research and Development Expenses

We accrue costs for clinical trial activities based upon estimates of the services received and related expenses incurred that have yet to be invoiced by the contract research organizations (CRO’s), clinical study sites, laboratories, consultants, or other clinical trial vendors that perform the activities. Related contracts vary significantly in length, and may be for a fixed amount, a variable amount based on actual costs incurred, capped at a certain limit, or for a combination of these elements. Activity levels are monitored through close communication with the CRO’s and other clinical trial vendors, including detailed invoice and task completion review, analysis of expenses against budgeted amounts, analysis of work performed against approved contract budgets and payment schedules, and recognition of any changes in scope of the services to be performed. Certain CRO and significant clinical trial vendors provide an estimate of costs incurred but not invoiced at the end of each quarter for each individual trial. The estimates are reviewed and discussed with the CRO or vendor as necessary, and are included in research and development expenses for the related period. For clinical study sites, which are paid periodically on a per-subject basis to the institutions performing the clinical study, we accrue an estimated amount based on subject screening and enrollment in each quarter. The estimates may differ from the actual amount subsequently invoiced, which may result in adjustment to research and development expense several months after the related services were performed.

Share-Based Compensation

We grant equity awards under one share-based compensation plan known as the Amended and Restated Incentive 2004 Plan. Under this plan, restricted stock, restricted stock units, stock options and other stock-related awards may be granted to our directors, officers, employees and consultants or advisors of the Company or any subsidiary. Stock-related awards are also outstanding under other share-based compensation plans, but we have not granted awards under these plans since 2004.

Our estimates of employee stock option values rely on estimates of factors we input into the Black-Scholes model. The key factors involve an estimate of future uncertain events. Significant assumptions include the use of historical volatility to determine the expected stock price volatility. We also estimate expected term until exercise, forfeiture or cancellation, as well as the reduction in the expense from expected forfeitures. We currently use historical exercise and cancellation patterns as our best estimate of future estimated life. Actual volatility and lives of options may be significantly different from our estimates. If factors change or we employ different assumptions, the share-based compensation expense that we record in future periods may differ significantly from our prior recorded amounts.

Valuation of Goodwill, Acquired Intangible Assets and In-Process Research and Development (IPR&D)

We have recorded goodwill, acquired intangible assets and IPR&D related to our acquisitions. When identifiable intangible assets, including IPR&D, are acquired, we determine the fair values of the assets as of the acquisition date. Discounted cash flow models are typically used in these valuations if quoted market prices are not available, and the models require the use of significant estimates and assumptions including but not limited to:

- timing and costs to complete the in-process projects;
- timing and probability of success of clinical events or regulatory approvals;
- estimated future cash flows from product sales resulting from completed products and in-process projects; and
- discount rates.

We may also utilize a cost approach, which estimates the costs that would be incurred to replace the assets being purchased. Significant inputs into the cost approach include estimated rates of return on historical costs that a market participant would expect to pay for these assets.

Intangible assets with definite useful lives are amortized to their estimated residual values over their estimated useful lives and reviewed for impairment if certain events occur.

Intangible assets related to IPR&D are treated as indefinite-lived intangible assets and not amortized until the product is approved for sale by regulatory authorities in specified markets. At that time, we will determine the useful life of the asset, reclassify the asset out of IPR&D and begin amortization. Impairment testing is also performed at least annually or when a triggering event occurs that could indicate a potential impairment. In the third quarter 2012, we recognized an impairment charge of $26,300 to write-down an IPR&D asset to fair value, which was determined to be de minimis. As of December 31, 2012, the carrying value of our IPR&D was not impaired.

If these projects are not successfully developed, our sales and profitability may be adversely affected in future periods. Additionally, the value of the acquired intangible assets, including IPR&D, may become impaired if the underlying projects do not progress as we initially estimated. We believe that the assumptions used in developing our estimates of intangible asset values were reasonable at the time of the respective acquisitions. No assurance can be given, however, that the underlying assumptions used to estimate expected project sales, development costs, profitability, or the events associated with such projects, such as clinical results, will occur as estimated.

Goodwill represents the excess of purchase price over fair value of net assets acquired in a business combination and is not amortized. Goodwill is subject to impairment testing at least annually or when a triggering event occurs that could indicate a potential impairment. We are organized as a single reporting unit and therefore the goodwill impairment test is done using our overall market value, as determined by our traded share price, as compared to our book value of net assets. We completed our annual impairment test as of December 31, 2012 and determined the carrying value of goodwill was not impaired.

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Valuation of Contingent Consideration

We record contingent consideration resulting from a business combination at its fair value on the acquisition date. We determine the fair value of the contingent consideration based primarily on the following factors:

•timing and probability of success of clinical events or regulatory approvals;
•timing and probability of success of meeting commercial milestones, such as sales levels of a specific compound; and
•discount rates.

Our contingent consideration liabilities arose in connection with our acquisitions. On a quarterly basis, we revalue these obligations and record increases or decreases in their fair value as an adjustment to operating earnings. Changes to contingent consideration obligations can result from adjustments to discount rates, accretion of the discount rates due to the passage of time, changes in our estimates of the likelihood of or timing of achieving any development or commercial milestones, changes in the probability of certain clinical events or changes in the assumed probability associated with regulatory approval.

The assumptions related to determining the value of contingent consideration include a significant amount of judgment, and any changes in the underlying estimates could have a material impact on the amount of contingent consideration expense recorded in any given period.

Income Taxes

We utilize the asset and liability method of accounting for income taxes. Under this method, deferred tax assets and liabilities are determined based on the difference between the financial statement carrying amounts and tax basis of assets and liabilities using enacted tax rates in effect for years in which the temporary differences are expected to reverse. We provide a valuation allowance when it is more likely than not that deferred tax assets will not be realized. We recognize the benefit of an uncertain tax position that has been taken or we expect to take on income tax returns if such tax position is more likely than not to be sustained.

We follow the authoritative guidance regarding accounting for uncertainty in income taxes, which prescribes a recognition threshold and measurement attribute for the financial statement recognition and measurement of a tax position taken or expected to be taken in a tax return. These unrecognized tax benefits relate primarily to issues common among multinational corporations in our industry. We apply a variety of methodologies in making these estimates which include studies performed by independent economists, advice from industry and subject experts, evaluation of public actions taken by the Internal Revenue Service and other taxing authorities, as well as our own industry experience. We provide estimates for unrecognized tax benefits which may be subject to material adjustments until matters are resolved with taxing authorities or statutes expire. If our estimates are not representative of actual outcomes, our results of operations could be materially impacted.

We continue to maintain a valuation allowance against certain deferred tax assets where realization is not certain. We periodically evaluate the likelihood of the realization of deferred tax assets and reduce the carrying amount of these deferred tax assets by a valuation allowance to the extent we believe a portion will not be realized. We consider many factors when assessing the likelihood of future realization of deferred tax assets, including our recent cumulative earnings experience by taxing jurisdiction, expectations of future taxable income, carryforward periods available to us for tax reporting purposes, various income tax strategies and other relevant factors. Significant judgment is required in making this assessment and, to the extent future expectations change, we would assess the recoverability of our deferred tax assets at that time. If we determine that the deferred tax assets are not realizable in a future period, we would record material adjustments to income tax expense in that period.

New Accounting Pronouncements

In June 2011, the Financial Accounting Standards Board (FASB) issued a new standard on the presentation of comprehensive income. The new standard eliminated the current 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. We adopted the provisions of this standard during the first quarter of 2012.

In July 2012, the FASB issued a new standard which amends the guidance on testing indefinite-lived intangible assets, other than goodwill. The new standard allows companies an option to first assess qualitative factors to determine whether it is

more likely than not that an indefinite-lived intangible asset is impaired as a basis for determining if it is necessary to perform a quantitative assessment and calculate the fair value of the asset. Under the new standard, a company is no longer required to calculate the fair value of an indefinite-lived intangible asset unless the company determines, based on the qualitative assessment, that it is more likely than not impaired. The new standard is effective for annual and interim impairment tests performed in fiscal years beginning after September 15, 2012, with early adoption permitted. We adopted the provisions of this guidance for our 2012 annual impairment test. The adoption did not have a material effect on our consolidated financial statements.

Results of Operations

The following table sets forth consolidated statements of operations data for the periods indicated. This information has been derived from the consolidated financial statements included elsewhere in this Annual Report on Form 10-K.

	Year Ended December 31,		
	2012	2011	2010
Net product sales	$1,134,114	$783,431	$540,957
Cost of sales:			
Cost of sales	126,214	93,140	64,437
Gain on intellectual property settlement	(53,377)	—	—
Total cost of sales	72,837	93,140	64,437
Operating expenses:			
Research and development	222,732	137,421	98,394
Selling, general and administrative	384,678	308,176	226,766
Acquisition-related costs	22,812	13,486	722
Impairment of intangible asset	26,300	—	—
Amortization of purchased intangible assets	417	382	—
Total operating expenses	656,939	459,465	325,882
Operating income	404,338	230,826	150,638
Interest and other expense	(6,772)	(1,158)	(1,627)
Income before income taxes	397,566	229,668	149,011
Income tax provision	142,744	54,353	51,981
Net income	$254,822	$175,315	$97,030
Earnings per common share:			
Basic	$1.34	$0.96	$0.54
Diluted	$1.28	$0.91	$0.52

Comparison of the Year Ended December 31, 2012 to the Year Ended December 31, 2011

Net Product Sales

Net product sales by significant geographic region are as follows:

	Year Ended December 31,			
	2012	2011	% Variance	
Net product sales:				
United States	$400,483	$263,387	52	%
Europe	418,321	340,812	23	%
Asia Pacific (primarily Japan)	161,480	115,377	40	%
Other	153,830	63,855	141	%
	$1,134,114	$783,431	45	%

The increase in revenue for fiscal year 2012 versus 2011 was primarily due to an increased number of patients treated with Soliris globally. The increase in treated patients was due to physicians requesting Soliris therapy for additional patients, as well as reimbursement and price approvals in additional territories and reimbursement for aHUS in the United States. We also recognized $3,300 related to an agreement reached with a payer in the second quarter of 2012

related to product shipped during

2011.

The increase in revenues was offset by the negative impact of approximately $16,566 for the year ended December 31, 2012 due to changes in foreign currency exchange rates (inclusive of hedging activity) versus the U.S. dollar for the year ended December 31, 2011. The negative impact was primarily due to the Euro, offset by a positive impact of the Japanese Yen. We recorded a gain (loss) in revenue of $12,869 and $(6,558) related to our foreign currency cash flow hedging program, which is included in revenue from outside the United States, for the years ended December 31, 2012 and 2011, respectively.

Cost of Sales

In October 2012, we entered into a settlement and non-exclusive license agreement with a third party. Under the terms of the agreement, we made an upfront payment of approximately $38,000 in the fourth quarter of 2012 and will pay royalties on sales of Soliris in accordance with the terms of the agreement. As a result of this settlement and non-exclusive license agreement, we reduced our estimate for probable contingent liabilities and recorded a gain in cost of sales of $53,377 in the third quarter 2012.

Exclusive of the settlement noted above, cost of sales were $126,214 and $93,140, or 11% and 12% of product revenue, respectively, for the years ended December 31, 2012 and 2011. Cost of sales includes manufacturing costs as well as actual and estimated royalty expenses associated with sales of Soliris.

Research and Development Expense

Our research and development expense includes personnel, facility and external costs associated with the research and development of our product candidates, as well as product development costs. We group our research and development expenses into two major categories: external direct expenses and all other R&D expenses.

External direct expenses are comprised of costs paid to outside parties for clinical development, product development and discovery research. Clinical development costs are comprised of costs to conduct and manage clinical trials related to eculizumab and other product candidates. Product development costs are those incurred in performing duties related to manufacturing development, medical affairs and regulatory functions, including manufacturing of material for clinical and research activities. Discovery research costs are incurred in conducting laboratory studies and performing preclinical research for other uses of eculizumab and other product candidates. Clinical development costs have been accumulated and allocated to each of our programs, while product development and discovery research costs have not been allocated.

All other R&D expenses consist of costs to compensate personnel, to maintain our facility, equipment and overhead and similar costs of our research and development efforts. These costs relate to efforts on our clinical and preclinical products, our product development and our discovery research efforts. These costs have not been allocated directly to each program.

The following table provides information regarding research and development expenses:

	Year Ended December 31, 2012	Year Ended December 31, 2011	$ Variance	% Variance	
Clinical development	$46,711	$33,417	$13,294	40	%
Product development	57,028	23,133	33,895	147	