Breakthrough Medical Innovation for Patients with Severe and Rare Disorders

2013 Annual Report

"For nine months, Justice was a growing and healthy boy with overflowing amounts of energy. But then one day I went to pick him up and he was limp ... the days that followed in the hospital were intense, and we were so afraid for our son. I vividly recall the doctor sitting us down and telling us Justice has atypical hemolytic uremic syndrome. We were overwhelmed and panicked. But then we learned there is a treatment called Soliris, which gave us hope."

— Danielle W., mother of a child with aHUS receiving Soliris

2013 Accomplishments

April

Alexion broadens and strengthens its executive leadership team with the addition of Martin Mackay, PhD, as Executive Vice President, Global Head of R&D, and of Saqib Islam, JD, as Senior Vice President, Chief Strategy and Portfolio Officer; Company Co-founder Stephen Squinto, PhD, assumes the newly created position of Executive Vice President, Chief Global Operations Officer

May

The U.S. Food and Drug Administration (FDA) grants Breakthrough Therapy designation to asfotase alfa for the treatment of perinatal-, infantile- and juvenile-onset hypophosphatasia (HPP)

June

The *New England Journal of Medicine* publishes data from two pivotal studies demonstrating that chronic Soliris® (eculizumab) therapy significantly improved clinical outcomes in patients with aHUS

Alexion breaks ground on its new global headquarters in New Haven, Connecticut, to support the company's continued growth

The U.S. FDA grants orphan drug designation to eculizumab (Soliris) for the treatment of neuromyelitis optica (NMO)

Alexion commences dosing in a study of the company's cyclic pyranopterin monophosphate (cPMP) replacement therapy in healthy volunteers; cPMP is an investigational therapy for patients with molybdenum cofactor deficiency (MoCD) type A, a severe, ultra-rare and genetic metabolic disorder that causes catastrophic neurologic damage in newborns in the first weeks of life

July

The Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMA) issues a positive opinion for orphan medicinal product designation for eculizumab for the treatment of NMO

Alexion establishes global supply chain and quality operations in Ireland

Alexion commences a multi-dose Phase 1 study of ALXN1007, the company's novel anti-inflammatory antibody, in healthy volunteers

August

Alexion initiates a natural history study of its cPMP replacement therapy in patients with MoCD type A

September

The Ministry of Health, Labour and Welfare (MHLW) in Japan approves Soliris for the treatment of all patients with aHUS

Investigators present preliminary data from a single-arm Phase 2 study demonstrating the potential efficacy of eculizumab in preventing early acute antibody-mediated rejection (AMR) in sensitized deceased-donor kidney transplant recipients at the European Society for Organ Transplantation (ESOT) congress

Forbes ranks Alexion #2 on its annual list of "The World's Most Innovative Companies" for the second year in a row

Investigators present new data from an ongoing multinational Phase 2 study in which asfotase alfa was associated with early and continued improvement in skeletal mineralization in infants and young children with HPP at the Joint Meeting of Paediatric Endocrinology congress

October

The U.S. FDA grants Breakthrough Therapy designation to Alexion's cPMP replacement therapy for the treatment of MoCD type A

Alexion expands enrollment in its study evaluating eculizumab in preventing AMR in deceased-donor kidney transplant recipients

November

Researchers present new data from the largest prospective trial of adult patients with aHUS and the first prospective trial in pediatric patients with aHUS at the American Society of Nephrology (ASN) meeting

The ASN meeting also features the presentation of three-year update data from two pivotal Phase 2 extension studies that highlight the long-term benefits of Soliris therapy in patients with aHUS

December

Researchers present data from multiple studies at the American Society of Hematology (ASH) meeting that enhance understanding of aHUS and paroxysmal nocturnal hemoglobinuria (PNH) to provide optimal care for patients, including new biomarker data that support the need for chronic terminal complement blockade with Soliris in patients with aHUS

Early 2014

Alexion announces a significant expansion of its drug discovery capabilities with a broad and long-term strategic agreement with Moderna Therapeutics for Alexion to develop messenger RNA (mRNA) Therapeutics™ for rare diseases

The U.S. FDA grants orphan drug designation to eculizumab for prevention of delayed graft function (DGF) in renal transplant patients and, separately, the European Commission grants orphan drug designation to eculizumab for the prevention of DGF after solid organ transplantation

Alexion begins screening patients with two different severe neurologic disorders, NMO and myasthenia gravis (MG), for enrollment in separate single multinational registration studies with eculizumab. Additionally, Alexion plans to initiate enrollment in a single registration study with eculizumab in patients with DGF

Alexion's consistent strong focus is on developing therapeutic candidates with life-transforming potential for patients with severe and rare disorders.

To Our Shareholders:

2013 was another year of significant accomplishments for Alexion in our mission to develop and deliver breakthrough medical innovation for patients suffering from severe and life-threatening rare disorders. During the year, we:

- Continued to serve new patients with paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS) worldwide with Soliris® (eculizumab).
- Continued to optimize our global organization to support the further expansion of our commercial and clinical operations, including the establishment of global supply chain operations in Ireland.
- Achieved a series of significant milestones as we progressed our R&D programs toward as many as seven potential approvals, between 2014 and 2018, for new, life-transforming products or indications for patients with severe and devastating diseases — starting with our second product, asfotase alfa, as a treatment for patients with hypophosphatasia (HPP).

Serving More Patients with PNH and aHUS

Soliris, our breakthrough medical innovation in terminal complement inhibition, is the first and only treatment approved for patients with PNH and aHUS, two severe and life-threatening, ultra-rare disorders for which Soliris is a life-transforming therapy.

PNH: Continued Steady Uptake Worldwide

In 2013 — more than six years following PNH approval in the initial countries — we observed the continued steady uptake of Soliris across the nearly 50 countries in which we serve patients with this devastating genetic disease. Throughout 2013, our disease awareness and diagnostic programs continued to support optimal patient care — even in countries where we have operated the longest — as physicians identified new patients with PNH and then rapidly initiated Soliris therapy. While we continued our steady performance in our core territories of the U.S., Western Europe and Japan, we increasingly saw important growth in Turkey, Brazil and Russia, and continued with early initiatives to serve new patients in Korea and various countries in Latin America. In 2014, our plans are driven by the urgency of delivering Soliris to patients with PNH in both established and newer markets, as most patients with PNH have yet to receive an accurate diagnosis, let alone begin appropriate therapy.

aHUS: Strong Performance in the Initial Global Launch

In aHUS, we were pleased with our strong progress in 2013 as we move forward with our global launch in the second indication for Soliris. Our performance in 2013 was augmented by initial funding approvals in Western Europe, and our aHUS disease education and diagnostic initiatives resulted in a steady increase in the number of new patients in the U.S. and Western Europe commencing Soliris therapy during the year. Near year-end, we were pleased by the aHUS regulatory approval and initial commercial launch in Japan.

We remain committed to bringing Soliris therapy to more patients who are living with PNH and aHUS in both new and existing markets worldwide.

"My name is Margarita and I have PNH. When I was diagnosed with PNH 23 years ago, I was experiencing extreme fatigue and debilitating pain. I was terrified that I would not be able to work or even take care of my kids. Fortunately, I was able to enroll in a clinical trial because at the time there were no treatments available. Today I am still taking Soliris, and 2013 was a great year – I got married and proudly watched my son graduate from high school. I am grateful that I am able to build many happy memories with my family."

– Margarita S., patient with PNH receiving Soliris

Working Toward Future Breakthroughs and Product Launches

In 2013, we made significant progress across all of our lead development programs. As we advance our programs and strengthen our early-stage research, we remain focused exclusively on delivering life-transforming therapies to patients with devastating and rare disorders that do not have effective treatment options. In these disease settings, we seek to move forward only if we can provide transformative clinical benefits, as we are doing in PNH and aHUS.

Eculizumab: Growing Our Portfolio with New Indications

As we build on the strong, long-term safety and efficacy profile of Soliris in PNH and aHUS, we are investigating eculizumab in other severe and rare complement-mediated disorders. Two of these programs are in the kidney transplant setting: antibody-mediated rejection (AMR) and delayed graft function (DGF). Acute AMR can lead to severe damage to the transplanted kidney, resulting in rapid loss of function and possible loss of the organ, which makes the risk of AMR a significant clinical barrier to transplantation in many patients. Research suggests that uncontrolled activation of complement, triggered by the binding of donor-specific antibodies (DSAs) to the target proteins (antigens) of the donor kidney, may be the primary reason for acute AMR in kidney transplant recipients who are sensitized, or have DSAs, to their donors. Prophylaxis with eculizumab is a potential strategy to prevent acute AMR, for which there is no approved treatment.

At the 2013 Annual Congress of the European Society for Organ Transplantation, researchers presented preliminary interim data from an open-label, single-arm, multicenter Phase 2 trial of eculizumab in sensitized deceased-donor kidney transplant recipients, in which the rates of AMR and related manifestations, such as graft loss, were substantially lower than expected. In AMR, we also significantly progressed with enrollment of at-risk patients in our living-donor study during the year. Additionally, we continue to enroll patients in our expanded deceased-donor AMR study.

DGF is an early and serious complication of organ transplantation that is characterized by the failure of a transplanted organ to function normally immediately following transplantation. In patients undergoing a kidney transplant, DGF leads to the patient requiring dialysis in order to survive. There is currently no approved therapy to prevent DGF in kidney transplant recipients. In addition, a significant number of donor kidneys are reportedly discarded each year due to the risk of DGF and its associated poor clinical outcomes. Following positive discussions with regulatory authorities in 2013, we are preparing to conduct a single registration trial in DGF, and in January 2014, the FDA granted an orphan drug designation to eculizumab for prevention of DGF.

Following meetings with regulators in 2013, we now expect to conduct single registration studies in our lead neurology programs with eculizumab in two settings: neuromyelitis optica (NMO) and myasthenia gravis (MG). In early 2014, we began screening patients for each of these double-blind, placebo-controlled studies.

"Willem would literally cry for two to three hours a night, screaming that his hands and feet hurt. I felt completely helpless because there was nothing I could do to alleviate the pain, and I wondered what was around the corner for us if it was already this bad. Hearing the diagnosis of hypophosphatasia was devastating because at first we were told that the doctors could only try to manage his symptoms. Willem is now participating in a clinical trial, and we are grateful that progress is being made to treat this devastating condition."

— Linda T., mother of a child with HPP

NMO is a life-threatening ultra-rare neurological disorder in which uncontrolled complement activation leads to severe damage to the central nervous system, including the spinal cord and optic nerve, and our study is focused on patients who continue to experience relapses despite supportive treatment. The primary endpoint of this study is time to first relapse.

MG is a debilitating and potentially life-threatening disorder in which uncontrolled complement activation results in destruction and inflammation at the neuromuscular junction, leaving muscles severely weakened. Our study in MG is focused on patients with severe disease who are refractory to other treatment options. The primary endpoint of the study is change in a measure of physical functioning in patients with MG.

Asfotase Alfa: Preparing to Launch Our Next Product

These guiding principles are clearly illustrated in our asfotase alfa development program for patients with HPP, a chronic, life-threatening, genetic and ultra-rare metabolic disease that leads to progressive damage to multiple vital organs, destruction and deformity of bones and, in too many cases, premature death. Asfotase alfa is designed to address the underlying cause of HPP, which affects people of all ages, by normalizing the genetically defective metabolic process and preventing or reversing the severe and potentially life-threatening complications of lifelong, dysregulated mineral metabolism.

In mid-2013, asfotase alfa received Breakthrough Therapy designation from the U.S. FDA. The Breakthrough Therapy designation is designed to expedite the development of a drug to treat a serious or life-threatening disease when preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. In this case, the U.S. FDA recognized asfotase alfa as a Breakthrough Therapy for patients with HPP whose first signs or symptoms occurred prior to 18 years of age, including patients with perinatal-, infantile- and juvenile-onset forms of the disease.

Other Highly Innovative Therapeutic Candidates

Beyond eculizumab and asfotase alfa, our research and development programs include additional compounds with breakthrough potential. In the metabolic disease area, we continue to accelerate development of our cyclic pyranopterin monophosphate (cPMP) replacement therapy for the treatment of molybdenum cofactor deficiency (MoCD) type A, a severe and life-threatening, ultra-rare, genetic metabolic disorder that causes catastrophic and irreversible neurologic damage within the first weeks of life. We were pleased to report that in 2013 the U.S. FDA granted Breakthrough Therapy designation to cPMP for this indication.

ALXN1007 is a novel anti-inflammatory antibody. In 2013 we completed dosing in a single-dose study and initiated and completed a multi-dose study in healthy volunteers. We will begin screening in our first proof-of-concept study with ALXN1007 in early 2014.

Research Pipeline

Preclinical	Clinical Studies	Registration Studies	Market

Soliris® (eculizumab) – Franchise

Approved Indications

Paroxysmal Nocturnal Hemoglobinuria (PNH)

Atypical Hemolytic Uremic Syndrome (aHUS)

Investigational Indications – Kidney Transplant, Neurology and Nephrology

Antibody-Mediated Rejection (AMR) – Living and Deceased Donor

Delayed Graft Function (DGF)

Relapsing Neuromyelitis Optica (NMO)

Severe and Refractory Myasthenia Gravis (MG)

Shiga-toxin *E. coli*-Related (STEC) HUS

Soliris Lifecycle Management

"Next-Gens"

Investigational Candidates – Metabolic Disorders

Hypophosphatasia (HPP)

Asfotase Alfa

Molybdenum Cofactor Deficiency (MoCD) Type A

Cyclic Pyranopterin Monophosphate (cPMP) Replacement Therapy (ALXN1101)

Investigational Candidates – Inflammatory Disorders

ALXN1007 – Anti-inflammatory Antibody

ALXN1102/1103 – Anti-Complement

Additionally, enrollment continues in our Phase 1 clinical trial of ALXN1102/1103, the intravenous and subcutaneous versions, respectively, of Alexion's novel alternative pathway complement inhibitor, which we are investigating for the treatment of severe, ultra-rare inflammatory disorders.

Expanding Early-Stage Research

Beyond our current clinical development programs, we initiated a collaboration with Ensemble Therapeutics in July 2013 to discover macrocycle drug candidates targeting patients with severe and ultra-rare disorders. The collaboration will utilize Ensemble's proprietary drug discovery platforms to address several undisclosed drug targets identified by Alexion with the objective of creating highly innovative small molecule therapeutic candidates. We will have the exclusive worldwide rights to develop and commercialize candidates arising from the collaboration.

In January 2014 we completed a broad, long-term, exclusive strategic agreement with Moderna Therapeutics for the discovery and development of messenger RNA (mRNA) Therapeutics™ to treat rare diseases. The agreement marks a significant expansion of our drug discovery capabilities and an exciting drug discovery platform for Alexion. We believe that the technology is optimally positioned to address the broadest number of severe and rare disorders, as these conditions are typically caused by single protein deficiencies, and Moderna's highly innovative technology is designed to directly utilize the body's natural processes to produce targeted proteins. This has the potential to speed the development and manufacture of treatments for many rare diseases that are currently untreatable with existing technologies.

Continued Strong Financial Discipline and Performance

2013 was another year of sustained growth and profitability for Alexion as we worked to provide Soliris to an increasing number of patients with PNH and aHUS worldwide. For the full year, we recorded sales of $1.55 billion, an increase of 37% compared to 2012. By maintaining fiscal discipline, we achieved a 47% increase in non-GAAP net income to $624.2 million. Our year-on-year revenue growth was robust across all territories we serve.

During 2013, we further aligned our global structure and made additional investments needed to improve operational efficiency so that we can serve even more patients with PNH and aHUS around the world.

Strengthening Our Organization

In 2013 we grew to more than 1,800 employees working in company facilities in 25 countries. We significantly broadened and deepened our executive leadership team with the hiring of Martin Mackay, PhD, as Executive Vice President, Global Head of R&D, and of Saqib Islam, JD, as Senior Vice President, Chief Strategy and Portfolio Officer. Also during 2013, Alexion Co-founder Stephen Squinto, PhD, assumed the newly created position of

Back (from left): David L. Hallal, *Executive Vice President and Chief Commercial Officer;* Saqib Islam, JD, *Senior Vice President and Chief Strategy and Portfolio Officer;* Stephen P. Squinto, PhD, *Executive Vice President and Chief Global Operations Officer;* Clare Carmichael, *Senior Vice President and Chief Human Resources Officer.* Front (from left): John B. Moriarty, Jr., JD, *Senior Vice President and General Counsel;* Leonard Bell, MD, *Chief Executive Officer;* Martin Mackay, PhD, *Executive Vice President and Global Head of Research & Development;* Vikas Sinha, MBA, CA, CPA, *Executive Vice President and Chief Financial Officer.*

"2013 was a year of strong performance for Alexion across a growing number of commercial and clinical objectives. In 2014, we will build on our recent accomplishments as we aggressively push forward the frontiers of medical innovation for the benefit of patients with severe and rare disorders."

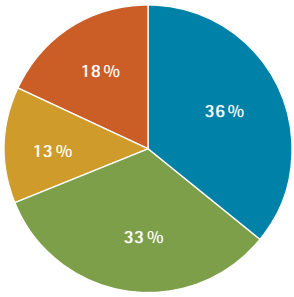
— Leonard Bell, MD, Chief Executive Officer

Executive Vice President, Chief Global Operations Officer, further strengthening our capabilities in the manufacturing, quality and supply chain functions.

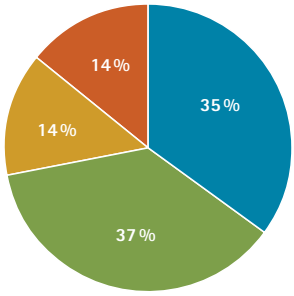
During 2013 we began to distribute Soliris from our newly approved contract manufacturing facility in Singapore. We also reached a key milestone in the expansion of our technical operations by establishing global supply chain operations in Ireland. In January 2014, we agreed to purchase what will become Alexion's first company-owned vialing facility for Soliris and other clinical and commercial products in Ireland. Finally, we are pleased with the continued improvements in our Rhode Island manufacturing and quality operations and look forward to the U.S. FDA reinspection of this facility in 2014.

To enable our continued global expansion, in 2013 we broke ground on our new global headquarters at 100 College Street in New Haven, Connecticut. Alexion will be the anchor tenant in the state-of-the-art laboratory and office building. The ground-breaking is a milestone in the growth of our research and business operations, and our return to New Haven will be a homecoming for Alexion, where the company was first established in 1992. The building, which is expected to open for occupancy in 2015, is located in the Downtown Crossing section of New Haven, and we are excited to take part in the redevelopment of the city's downtown.

Soliris Net Product Sales



2013 $1,551,346



2012 $1,134,114

- United States
- Europe
- Asia-Pacific (primarily Japan)
- Other

Looking Ahead

Our track record of accomplishments across key growth objectives in 2013 has provided a foundation for continued innovation and progress in 2014 and beyond. As always, we are grateful to everyone who supports our mission and makes our work possible: our employees, our board members and shareholders, physicians and other health professionals, healthcare authorities around the world and especially the patients, families and caregivers whose needs we strive to meet. Together we will work tirelessly to attain additional breakthroughs in 2014 and beyond.

Leonard Bell, MD
Chief Executive Officer

April 2014

Selected GAAP Financial Results (In thousands, except per share data)

Year Ended December 31,	2013	2012	2011
Consolidated Statements of Operations Data:			
Net product sales	$ 1,551,346	$ 1,134,114	$ 783,431
Cost of sales	177,556	72,837	93,140
Operating expenses:			
Research and development	317,093	222,732	137,421
Selling, general and administrative	489,720	384,678	308,176
Acquisition-related costs	5,029	22,812	13,486
Impairment of intangible assets	33,521	26,300	—
Amortization of purchased intangible assets	417	417	382
Total operating expenses	**$ 845,780**	**$ 656,939**	**$ 459,465**
Operating income	528,010	404,338	230,826
Other expense	1,741	6,772	1,158
Income before income taxes	526,269	397,566	229,668
Income tax provision	273,374	142,744	54,353
Net income	**$ 252,895**	**$ 254,822**	**$ 175,315**
Earnings per common share — diluted	$ 1.27	$ 1.28	$ 0.91
Shares used in computing earnings per common share — diluted	199,712	198,501	191,806

As of December 31,	2013	2012	2011
Consolidated Balance Sheet Data:			
Cash, cash equivalents and marketable securities	$ 1,514,851	$ 989,501	$ 540,865
Trade accounts receivable, net	421,752	295,598	244,288
Inventories	102,602	94,521	81,386
Property, plant and equipment, net	201,109	165,629	165,852
Goodwill and intangible assets, net	863,792	900,323	171,243
Deferred tax assets	44,826	40,040	123,000
Other assets	168,764	127,948	68,117
Total assets	**$ 3,317,696**	**$ 2,613,560**	**$ 1,394,751**
Accounts payable and accrued expenses	$ 423,940	$ 271,275	$ 199,653
Deferred revenue	53,801	31,266	17,905
Contingent consideration	142,676	141,670	18,120
Long-term debt	113,000	149,000	—
Deferred tax liabilities	101,265	20,994	862
Other liabilities	100,935	28,505	23,719
Total liabilities	**935,617**	**642,710**	**260,259**
Total stockholders' equity	**2,382,079**	**1,970,850**	**1,134,492**
Total liabilities and stockholders' equity	**$ 3,317,696**	**$ 2,613,560**	**$ 1,394,751**

Shareholder Information

Directors

Max Link, PhD[1,4]
Chairman of the Board

Former Chairman of the Board and CEO, Centerpulse AG

Former CEO, Corange

Former Chairman of the Board and CEO, Sandoz Pharma, Ltd.

Leonard Bell, MD
Chief Executive Officer

William R. Keller[2,3]
Vice Chairman of Shanghai Association of Foreign Investment Enterprises

Senior Consultant of Shanghai Foreign Investment Development Board

Former General Manager, Roche China Ltd.

Joseph A. Madri, PhD, MD[2,4,5]
Professor of Pathology,
Yale University School of Medicine

Larry L. Mathis[1,3,5]
Former President and CEO,
The Methodist Hospital System

R. Douglas Norby[1,3]
Former Senior Vice President,
Chief Financial Officer,
Tessera Technologies, Inc.

Alvin S. Parven[2,3]
President, ASP Associates

Former Vice President, Aetna Health Plans

Andreas Rummelt, PhD[1,4]
CEO, InterPharmaLink AG

Former Group Head, Quality Assurance and Technical Operations, Novartis

Former Member of Executive Committee, Novartis

Former CEO, Sandoz AG

Ann M. Veneman[2,3]
Former Executive Director of UNICEF

Former Secretary of US Department of Agriculture

Executive Management

Leonard Bell, MD
Chief Executive Officer

Stephen P. Squinto, PhD
Executive Vice President,
Chief Global Operations Officer

David L. Hallal
Executive Vice President,
Chief Commercial Officer

Martin Mackay, PhD
Executive Vice President,
Global Head of Research & Development

Vikas Sinha, MBA, CA, CPA
Executive Vice President,
Chief Financial Officer

Clare Carmichael
Senior Vice President,
Chief Human Resources Officer

Saqib Islam, JD
Senior Vice President,
Chief Strategy & Portfolio Officer

John B. Moriarty, Jr., JD
Senior Vice President, General Counsel

Annual Shareholders Meeting

To be held on May 5, 2014
5:00 p.m.

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Trading Symbol
Listing for Alexion Pharmaceuticals, Inc. is found on the NASDAQ stock market under the symbol ALXN.

alexionpharma.com

[1] Member of the Audit Committee

[2] Member of the Compensation Committee

[3] Member of the Nominating and Corporate Governance Committee

[4] Member of the Pharmaceutical Compliance and Quality Committee

[5] Retiring May 2014

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