

# Breakthrough Medical Innovation for Severe and Life-Threatening Disorders





"I got very sick when I was 19 years old and was told I had six months to a year to live. After nearly 16 years of blood transfusions, bone marrow biopsies, several misdiagnoses and life in and out of hospitals, I was finally diagnosed with PNH in 2007. Soon after I was told that I had PNH, I began to receive regular infusions of Soliris®, which I still get every two weeks. I am grateful for Soliris. Today, I am able to be a father, a husband and the man I always aspired to be."

# 2014 Accomplishments

#### **January**

Alexion Pharmaceuticals and Moderna Therapeutics announce exclusive strategic agreement to develop Messenger RNA Therapeutics™ for rare diseases

US Food and Drug Administration (FDA) grants orphan drug designation (ODD) to eculizumab for the prevention of delayed graft function (DGF) in renal transplant patients

#### **February**

European Commission (EC) grants ODD to eculizumab for the prevention of DGF after solid organ transplantation

#### March

The National Institute for Health and Clinical Excellence (NICE) Evaluation Committee acknowledges that Soliris® (eculizumab) is a very effective treatment for patients with atypical hemolytic uremic syndrome (aHUS) and produces substantial quality-adjusted life year gains of a magnitude rarely seen for a new drug

#### April

Alexion initiates the rolling submission of a Biologics License Application (BLA) for asfotase alfa as a treatment for patients with hypophosphatasia (HPP) with the FDA

Alexion initiates multinational registration trials of eculizumab as a potential treatment for patients with relapsing neuromyelitis optica (NMO) and refractory generalized myasthenia gravis (MG)

Alexion announces the appointment of John T. Mollen to its Board of Directors Alexion unveils plans for future expansion in Ireland with construction of a new global supply chain facility at College Park, Blanchardstown, Dublin

EC grants ODD to eculizumab for the prevention of graft rejection following solid organ transplantation

#### May

FDA approves conversion from accelerated to regular approval for Soliris in aHUS; revised label now specifies important longer-term clinical benefits associated with chronic and sustained Soliris treatment with inclusion of results from two years of ongoing treatment

Researchers present new data from the first large natural history study in infants with HPP, showing 73% mortality reported at five years in infants with severe perinatal and infantile HPP, at the joint meeting of the Pediatric Academic Societies (PAS) and the Asian Society for Pediatric Research

The PAS meeting also features data that show early and sustained improvements observed in infants, children and juveniles with HPP receiving asfotase alfa in the open-label extension phase of two on-going Phase 2 clinical studies

#### June

Investigators present new data demonstrating the efficacy of chronic Soliris treatment in a broad range of patients with aHUS, including patients with or without a history of renal transplant or dialysis, at the European Renal Association–European Dialysis and Transplant Association Congress

Alexion initiates proof-of-concept clinical study with ALXN1007 in antiphospholipid syndrome

FDA grants ODD to eculizumab for the treatment of patients with MG

Alexion and Cincinnati Children's announce collaboration and establish a fund for the advancement of research in rare disease

#### July

Alexion announces the appointments of David R. Brennan, M. Michele Burns and Christopher J. Coughlin to its Board of Directors

European Medicines Agency (EMA) accepts marketing authorization application for asfotase alfa as a treatment for patients with HPP

#### Augus

EC grants ODD to eculizumab for the treatment of patients with MG

Alexion initiates multinational registration trial of eculizumab for the prevention of DGF after kidney transplantation

#### September

For the third consecutive year, *Forbes* magazine names Alexion the #2 most innovative company in the world on its 2014 list of the World's 100 Most Innovative Companies

Fortune magazine ranks Alexion #20 on its annual list of the 100 Fastest-Growing Companies, making it the third fastest-growing company in the healthcare sector

Asfotase alfa granted ODD in Japan by the Ministry of Health, Labour and Welfare (MHLW)

Researchers present new data showing improved survival in pediatric patients with severe HPP who were treated with investigational asfotase alfa for up to five years at the American Society for Bone and Mineral Research annual meeting

Alexion announces the initiation of Phase 1 clinical studies with ALXN1210 and ALXN5500, two "next-generation" Soliris molecules

Alexion announces promotion of David Hallal to the newly created position of Chief Operating Officer and appoints Mr. Hallal to Alexion's Board of Directors

#### **October**

Alexion announces appointments of Leonard Bell, MD, CEO, to Chairman of the Board of Directors and R. Douglas Norby to the newly created Board position of Lead Independent Director

Alexion submits New Drug Application to Japan's MHLW for asfotase alfa as a treatment for patients with HPP

Alexion announces the initiation of Phase 1 clinical studies with ALXN1210 and ALXN5500, two "next-generation" Soliris molecules

#### November

Alexion announces plans to establish its new Europe, Middle East & Africa (EMEA) regional headquarters in Zurich, Switzerland

Investigators present longer-term outcome data from the largest prospective trial of Soliris in aHUS, which underscores the effectiveness of ongoing Soliris treatment at the American Society of Nephrology annual meeting

Eculizumab granted ODD by Japan's MHLW for the treatment of patients with NMO

NICE Highly Specialised Technologies Evaluation Committee issues final positive recommendation for national commissioning of Soliris for all patients with aHUS in England

Alexion initiates proof-of-concept clinical study with ALXN1007 in gastrointestinal graft-versus-host disease

#### December

Alexion completes rolling BLA submission to FDA for as fotase alfa as a treatment for patients with  $\ensuremath{\mathsf{HPP}}$ 

New data enhancing the clinical knowledge of aHUS and paroxysmal nocturnal hemoglobinuria (PNH) and underscoring the effectiveness of Soliris treatment presented at the American Society of Hematology annual meeting

Eculizumab granted ODD by Japan's MHLW for the treatment of patients with MG

#### **Early 2015**

Alexion appoints David Hallal as Chief Executive Officer, effective April 1, 2015, succeeding Leonard Bell, MD, principal founder of Alexion, who will retire as CEO and continue to serve as Chairman of the Board

FDA grants priority review for asfotase alfa as a treatment for patients with infantile- and juvenile-onset HPP

New data from first natural history study in juveniles with HPP, showing wide range of HPP-related complications and persistent nature of disease, presented as late-breaking data at the Endocrine Society's Annual Meeting and Expo

Forward-looking statements: This Annual Report contains forward-looking statements, all of which involve certain assumptions, risks and uncertainties that are beyond Alexion's control and could cause our actual results to differ materially from the statements described. Forward-looking statements involve significant risks and uncertainties, including those more fully described in our Form 10-K contained within this Annual Report and in the most recent periodic reports on Form 10-Q filed by Alexion with the U.S. Securities and Exchange Commission, and actual results may vary materially. Alexion does not undertake any duty to update any forward-looking statements contained in the Annual Report as a result of new information, future events or otherwise.

# Remembering Our Former Chairman



Max Link, PhD (b. 9.26.1940 – d. 10.6.2014)

Dr. Max Link was a globally respected leader of companies large and small in the pharmaceutical, biotechnology and medical device industries over the course of nearly four decades. Max was a tireless champion of medical innovation and entrepreneurship for the benefit of patients, and we were very fortunate to have had the benefit of his wisdom, experience and global perspective as a member of our Board of Directors since our inception in 1992, and as Chairman of the Board from 2002 until his untimely passing.

In our earlier years, Max provided invaluable guidance during the inevitable challenges we faced as a biotech startup. His leadership and fortitude were vital in these years. As Chairman, he was a key guide and mentor in our transition, starting in 2007, from a development-stage firm to a global development and commercial firm serving patients with devastating and life-threatening disorders worldwide. Through his work at Alexion and other companies, Max helped to bring a wide array of innovative therapies to patients.

Max was a colleague, friend, mentor, confidant and guide over the course of 22 years. Each one of us at Alexion – and all the patients we serve – has been positively touched by the invaluable guidance he provided. We miss Max dearly and will carry his memory with us every day. We join with his family and close friends in mourning our loss.

# To Our Shareholders:

In 2014, Alexion continued to expand its commercial and clinical operations as we advanced our mission to develop and deliver life-transforming therapies for patients with severe and life-threatening rare diseases. During the year, we reached a wide range of significant milestones on behalf of patients and their families while continuing to build a larger and highly efficient global enterprise to support a broad portfolio of transformative therapies, starting with our next product, asfotase alfa. Among our achievements in 2014, we:

- Continued to provide Soliris® (eculizumab) to an increasing number of patients worldwide with paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS).
- Completed the rolling submission of our US Biologics License Application (BLA) for asfotase alfa as a treatment for patients with infantile- and juvenile-onset hypophosphatasia (HPP), a severe metabolic disorder, which the US Food and Drug Administration (FDA) has accepted for priority review. In addition, we completed our submissions for marketing authorizations for this highly innovative therapy in the EU and Japan, and we have significantly expanded the body of knowledge about HPP and the clinical benefits of asfotase alfa to support optimal patient care.
- Advanced the first two of our next-generation Soliris molecules into the clinic, and — based on initial clinical data — now intend to advance at least one of these molecules into Phase 2 trials in patients with PNH in 2015.
- Progressed our other key clinical development programs while establishing a strong foundation for future growth with an additional 17 preclinical development programs spanning diverse modalities and therapeutic areas.

#### Reaching Patients with PNH and aHUS

We developed Soliris, the world's first and only approved terminal complement inhibitor, from the laboratory through regulatory approvals and commercialization in PNH and aHUS, two devastating and life-threatening ultra-rare disorders.

# PNH — Reaching More Patients Across our 50-Country Platform

The steady increase in the number of new patients starting on Soliris in 2014 affirms our view that, on a global basis, the majority of patients with PNH have yet to receive an accurate diagnosis, let alone commence appropriate therapy. Throughout 2014, as in prior years, we continued to identify a consistently high number of newly diagnosed patients with PNH in the US, Western Europe and Japan — the territories in which we have operated the longest — and we are also observing consistent additions of new patients commencing Soliris therapy across Turkey, Brazil and Russia. While we are pleased with the continued positive impact we are having on the lives of patients with PNH, we know that ongoing education is required to further enhance rapid and accurate diagnosis and effective treatment.

# aHUS — Strong Performance in the Ongoing Global Launch

We continued to observe a steady addition of new patients with aHUS commencing Soliris therapy in the US and Europe in 2014, while we made important progress in the early stages of serving patients with aHUS in Japan. The number of new patients with aHUS being identified by physicians — including children who have rapidly progressing, life-threatening complications - confirms our view that our opportunity to serve patients and families suffering with aHUS is at least as large as our opportunity to serve the PNH community, and perhaps larger. As one measure, in the US - more than three years following their respective FDA approvals — more patients are currently receiving Soliris for aHUS than there had been for PNH. In Europe, we are experiencing a similar trend among patients with aHUS. These observations are in line with our view that the incidence of aHUS is likely higher than PNH. Our educational initiatives in aHUS are supported by the strengthening of our Soliris labels in the US and Europe, which now specify the important longer-term clinical benefits associated with chronic and sustained Soliris treatment with inclusion of results from two years of ongoing treatment.



"I got sick when I was 14 and was in excruciating pain. It took nearly two years for the doctors to diagnose me with aHUS. As soon as I was diagnosed, I started receiving Soliris®. While I know that I will have aHUS for the rest of my life, I no longer think about it every day, and it is very reassuring to finally have a treatment that can help me. Before I got sick I was always interested in medicine, but my experience battling aHUS has motivated me to pursue a career in medicine."

# Advancing Our Relentless Mission to Provide Transformative Therapies

Beyond PNH and aHUS, we made significant progress across all of our lead development programs in 2014, all of which are focused on first-in-class therapeutic breakthroughs.

### Preparing for the Launch of Asfotase Alfa

As we prepare to provide our next product, asfotase alfa, to patients with HPP, we are applying key learnings from our experience in serving patients with PNH and aHUS for optimizing care with a highly innovative ultra-orphan therapy. We expect regulatory decisions in the US, EU and Japan this year, and are preparing to serve patients in the US and Germany during the first half of 2015 and in Japan by year-end. We are working with regulatory authorities to obtain these marketing authorizations as quickly as possible, given the high rates of mortality, severe debilitating effects and current lack of any approved therapy for patients with HPP.

In HPP, as in other ultra-rare disorders, education will be critical to helping patients, and our programs will employ the growing body of clinical data reflecting the potentially transformative impact of asfotase alfa. For example, researchers at the Endocrine Society Annual Meeting in March 2015 presented new data from a retrospective, multinational natural history study of children with HPP. Data from this study, which included 32 patients with juvenile-onset HPP, demonstrated that children with HPP have a substantial disease burden, particularly with regard to musculoskeletal abnormalities and growth deficiencies. These patients experienced HPP-related disease complications and morbidity that persisted despite standard efforts to control symptoms.

In parallel to the regulatory filings, we continue to build out our initial field-based medical teams and our in-country metabolic commercial teams, and they have begun educating physicians on the signs and symptoms of HPP and the appropriate pathways for a rapid and accurate diagnosis.

# Eculizumab: Expanding Our Portfolio with New Indications

As we seek to build on the strong, long-term safety and efficacy profile of Soliris® in PNH and aHUS, we are investigating eculizumab as a potential treatment for patients with other severe and rare complement-mediated disorders.

In neurology, we have development programs with eculizumab in patients with two severe disorders: neuromyelitis optica (NMO) and myasthenia gravis (MG). In 2014, we were pleased that eculizumab was granted orphan drug designations in Japan for NMO and in the US, EU and Japan for MG. NMO is a life-threatening ultra-rare neurological disorder in which uncontrolled complement activation leads to severe damage to the central nervous system in patients, including their spinal cord and optic nerve. Our study is focused on patients who continue to experience relapses despite supportive treatment. Enrollment and dosing are ongoing in the PREVENT study, our registration trial in relapsing NMO.

MG is a debilitating and potentially life-threatening disorder in which uncontrolled complement activation results in destruction and inflammation at the junction between nerves and muscles in patients, leaving their muscles severely weakened. The REGAIN study, our registration trial in MG, is focused on patients with severe disease who are refractory to other treatment options. We expect to complete enrollment in REGAIN in 2015.

In transplant, a multinational registration trial with eculizumab in kidney transplant patients at increased risk for delayed graft function (DGF) is ongoing, and we expect to complete enrollment by the end of the year. 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, potentially resulting in the loss of the organ. There is currently no approved therapy to prevent DGF in kidney transplant recipients. In addition, a significant number of

# Research Pipeline

Preclinical	Early Clinical Development	Advanced Clinical Development	Registration Filings	Market
Soliris® (eculizumab)				
Approved Indications				
Paroxysmal Nocturnal Hemo	globinuria (PNH)			
Atypical Hemolytic Uremic S	Syndrome (aHUS)			
Investigational Indications	– Transplant and Neuro	logy		
Refractory Myasthenia Grav	is (MG)			
Relapsing Neuromyelitis Opt	tica (NMO)			
Delayed Graft Function (DGF	=)			
Antibody-Mediated Rejectio	n (AMR)	_		
Next-Generation Portfo	lio			
ALXN1210				
ALXN5500				
Investigational Candidat	tes – Metabolic Disord	ers		
Asfotase Alfa				
Hypophosphatasia (HPP)				_
cPMP Replacement Therap	y (ALXN1101)			
Molybdenum Cofactor Defic	iency (MoCD) Type A			
Investigational Candidat	tes — Inflammatory Di	sorders		
ALXN1007 Anti-inflamma	tory Antibody			
Antiphospholipid Syndrome	(APS)			
Gastrointestinal Graft-versu	s-Host Disease (GI-GVHD)			
Preclinical Candidates				
Messenger RNA (mRNA) The	rapeutics			
Other Preclinical Candidates	;			

donor kidneys are reportedly discarded each year due to the risk of DGF and its associated poor clinical outcomes.

We are also evaluating eculizumab in antibody-mediated rejection (AMR), a severe and potentially life-threatening condition that can lead to rapid deterioration of function and possible loss of the transplanted organ. Later this year we expect data from our Phase 2 deceased-donor study for the prevention of AMR, as well as the initiation of our clinical study with eculizumab for the treatment of patients with AMR. We continue to evaluate the results of our clinical study with eculizumab for the prevention of AMR in patients receiving living-donor transplants, which missed its primary endpoint.

#### Other Highly Innovative Therapeutic Candidates

As the leaders in complement biology, we advanced the development of the first two next-generation Soliris® molecules in 2014. We initiated Phase I studies of ALXN1210 and ALXN5500, with additional molecules and programs in our expanding portfolio at earlier stages of development. The data already support advancing both of these molecules into further trials — and we intend to progress at least one of them into a Phase 2 PNH trial this year — with additional indications likely to follow. ALXN1210 is a longer-acting anti-C5 monoclonal antibody suitable for once-monthly dosing. We are targeting approval of at least one next-generation candidate in 2018.

We also have lead development programs with two additional highly innovative therapies, ALXN1007 and cyclic pyranopterin monophosphate (cPMP).

We have commenced dosing in two Phase 2 proof-of-concept studies to evaluate the safety and tolerability of ALXN1007 in patients with two severe and potentially life-threatening auto-immune diseases: graft-versus-host disease involving the gastrointestinal tract, or GI-GVHD, and antiphospholipid syndrome, or APS. We expect to have interim data in the GI-GVHD study later in 2015.

In our metabolic disease area, we continue to advance development of our cPMP replacement therapy for the

treatment of patients with 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. The synthetic cPMP bridging study in patients with MoCD is ongoing, and we expect to complete enrollment in 2015. We are also continuing our retrospective data collection and natural history study.

### **Expanding Early-Stage Research**

Within our portfolio of 17 preclinical programs, we have initiated preclinical development in our first seven messenger RNA (mRNA) programs with our collaborator Moderna Therapeutics, focused on the treatment of patients with severe and rare disorders. We are targeting our first candidate to enter the clinic in 2016. In addition, we entered into several other earlier-stage preclinical programs during 2014.

### **Strong Financial Performance**

2014 was another year of robust growth and profitability for Alexion as we provided Soliris to an increasing number of patients with PNH and aHUS worldwide. Net product sales for the year increased 44 percent to \$2.234 billion, compared to \$1.551 billion in 2013. Excluding the impact of \$88 million for reimbursement of prior year shipments, 2014 net product sales increased 38 percent to \$2.146 billion. By exceeding our revenue target, while maintaining strict financial discipline in our growing commercial and clinical activities, we reported 2014 non-GAAP EPS of \$5.21 per diluted share, an increase of 69% year-over-year. Excluding the impact of the \$88 million in pre-2014 sales, EPS would be \$4.84, an increase of 57% in 2014. Our year-on-year revenue growth was robust across all territories we serve.

During 2014, we further aligned our global structure and invested in improving operational efficiency as we provide more therapies to more patients around the world. We expanded our Global Supply Chain and Quality operations in Ireland, which will include our first company-owned fill/finish facility. We are also in the process of moving our EMEA headquarters to Zurich, a major center for the



Standing (from left): Julie O'Neill, Executive Vice President, Global Operations; Vikas Sinha, MBA, CA, CPA, Executive Vice President and Chief Financial Officer; Edward Miller, JD, Senior Vice President, Global Chief Compliance Officer; John B. Moriarty, Jr., JD, Executive Vice President, General Counsel; Saqib Islam, JD, Executive Vice President, Chief Strategy & Portfolio Officer; Martin Mackay, PhD, Executive Vice President and Global Head of Research & Development.

Seated (from left): David Hallal, Chief Operating Officer, CEO-elect; Leonard Bell, MD, Chairman of the Board, Chief Executive Officer; Clare Carmichael, Executive Vice President, Chief Human Resources Officer.

"Alexion is at the strongest and most promising point in our history as we move toward becoming a multi-product company with our most robust development pipeline ever.

Our significant accomplishments throughout 2014 have positioned our global organization to reach a new set of milestones on behalf of patients in 2015 and beyond as we maintain our relentless focus to bring life-transforming treatments to more patients with severe and life-threatening diseases worldwide."

pharmaceutical and biotechnology industries, to maximize our ability to attract talent and engage in commercial and academic collaborations. Finally, we have announced the establishment of the Alexion Research and Development Center in Paris, the first Alexion research facility outside of North America, which will focus on the discovery of innovative therapies for patients with severe, rare diseases.

### Recognizing Two Alexion Leaders

Along with our accomplishments in 2014, we were greatly saddened by the unexpected passing of Max Link, PhD, in October. Max had been a Director of Alexion since we were established in 1992, and served as Chairman of our Board of Directors since 2002. Please see page 2 herein to learn more about Max — an amazing person, a great friend and mentor, and one of our industry's leading champions of innovation for the benefit of patients.

Also in late 2014, Steve Squinto, PhD, my Alexion co-founder and friend, announced his planned retirement from Alexion effective January 1, 2015, following 22 years during which he helped to drive Alexion's evolution into a global leader in the development, manufacturing and delivery of biotechnology therapeutics. We are very pleased that Steve will continue to contribute to our mission as the Chair of our newly established Scientific Advisory Board, and likewise pleased that Julie O'Neill has now taken on Steve's most recent position as Executive Vice President, Global Operations.

#### Looking Ahead

In January 2015, we also announced my own retirement as CEO and the appointment of David Hallal, Alexion's Chief Operating Officer, as my successor, effective April 1, 2015. As Alexion is at a position of great strength with regard to commercial execution, financial discipline, and pipeline breadth and growth, the time is right for the Company, for David and for me personally to make this transition. In line with the thoughtful succession planning process undertaken by our Board, David will become the second CEO in our company's history. David has demonstrated outstanding leadership over the past decade at Alexion and has been

instrumental in driving our results and building the high-performing, patient-centered culture for which we are known, with accomplishments that include leadership of the highly successful launches of Soliris® for PNH and aHUS and a key role in the build-out of our 50-country operating platform. As we have worked closely together for nearly a decade and increasingly shared responsibilities over the past few years, I know that David is the right person to lead us into our next chapter of growth, and I look forward to continuing to guide the Board and advising the Company on strategic matters in my role as Chairman.

For all that the Alexion team has accomplished for patients to date, we are excited that the opportunities ahead of us are far greater. In 2015 and beyond, our goals are to focus our global skills and resources toward serving a continuously increasing number of patients with PNH and aHUS ... beginning to serve patients with HPP ... and significantly expanding our portfolio through both internally and externally developed therapeutic candidates to provide transformative outcomes to patients suffering with severe and rare disorders.

As always, we thank our growing number of employees and the many other people who make our work possible — including researchers, physicians, patients and families. We are united in our commitment to serve patients with severe and life-threatening disorders through breakthrough medical innovation and to transform their lives from illness and desperation ... into health and hope.

Leonard Bell, MD

Chairman and Chief Executive Officer

March 2015



"The medicines we develop need to be transformative because the diseases we strive to treat are so devastating. At the same time, the work we do must be global because these diseases know no boundaries and affect patients all around the world."

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

# Global Locations

# Global and Regional Headquarters

Cheshire, CT, USA

Global Headquarters North America Regional HQ

**Dubai, United Arab Emirates** 

Middle East Operations Country Operations

Dublin, Ireland

Global Supply Chain and Distribution

Lausanne, Switzerland

EMEA Regional Headquarters International Operations Center Country Operations

Miami, FL, USA

Latin America Regional Headquarters

Sydney, Australia

Asia-Pacific Regional Headquarters Country Operations

Tokyo, Japan

Japan Headquarters Country Operations

# **Specialized Facilities and Country Operations**

Barcelona, Spain

Country Operations

Bogotá, Colombia

Country Operations

Brussels, Belgium

Government Affairs, EMEA Country Operations

Buenos Aires, Argentina

Country Operations

Cambridge, MA, USA

Research and Development

Istanbul, Turkey

Country Operations

London, United Kingdom

Country Operations

Mexico City, Mexico

Country Operations

Milan, Italy

Country Operations

Moscow, Russia

Country Operations

Mumbai, India

Global Business Services

Munich, Germany

Country Operations

Osaka, Japan

**Country Operations** 

Paris, France

European Service Center Country Operations

São Paulo, Brazil

Country Operations

Shanghai, China

Country Operations

Smithfield, RI, USA

Manufacturing Operations

Stockholm, Sweden

Nordic Country Operations

Toronto, Canada

Country Operations

Washington, DC, USA

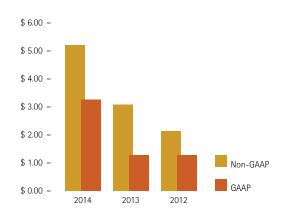
Global Government Affairs

# Financial Highlights



<sup>\*</sup> Included in Europe revenues for 2014 is a reimbursement of \$87.83 million for shipments made prior to 2014 as a result of an agreement with the French government.





Cash, Cash Equivalents and Marketable Securities (\$ in Millions)



# Reconciliation of GAAP to Non-GAAP Financial Results

(in thousands expect per share amounts)

Results for the years ended December 31,	2014	2013	2012
GAAP net income	\$ 656,912	\$ 252,895	\$ 254,822
Share-based compensation expense	114,461	76,203	54,013
Acquisition related costs	20,295	5,029	22,812
Upfront and milestone payments related to license and collaboration agreements	109,925	14,500	_
Impairment of intangible assets	11,514	33,521	26,300
Restructuring expenses	15,365	_	_
Amortization of purchased intangible assets	_	417	417
Change in contingent liability from intellectual property settlements	_	9,181	(53,377)
Tax related to acquisition structuring	_	_	21,812
Non-cash taxes	137,449	232,460	98,364
Non-GAAP net income	\$ 1,065,921	\$ 624,206	\$ 425,163
GAAP earnings per share - diluted	\$ 3.26	\$ 1.27	\$ 1.28
Non-GAAP earnings per share - diluted	\$ 5.21	\$ 3.08	\$ 2.13

# Shareholder Information

#### **Directors**

#### Leonard Bell, MD

Chairman of the Board, Chief Executive Officer

#### David R. Brennan<sup>4,5</sup>

Former Chief Executive Officer, Executive Director, AstraZeneca PLC

#### M. Michele Burns<sup>2,3,5</sup>

Center Fellow & Strategic Advisor, Stanford University Center on Longevity Chief Executive Officer, Retirement Policy Center, Marsh & McLennan Companies, Inc.

#### Christopher J. Coughlin<sup>1,4</sup>

Senior Advisor, McKinsey & Co.

Former Advisor to the Chairman and CEO of Tyco International Ltd.

Former Executive Vice President and Chief Financial Officer of Tyco

#### David Hallal

Chief Operating Officer, CEO-elect, effective April 1, 2015

#### William R. Keller<sup>2,3,5</sup>

Vice Chairman, Shanghai Association of Foreign Investment Enterprises

Senior Consultant, Shanghai Foreign Investment Development Board

Former General Manager, Roche China Ltd.

#### John T. Mollen<sup>1,2</sup>

Former Special Advisor to the Chairman, EMC Corporation

Former Executive Vice President, Human Resources, EMC Corporation

#### R. Douglas Norby<sup>1,3,4</sup>

Lead Independent Director Former Senior Vice President, Chief Financial Officer, Tessera Technologies, Inc.

#### Alvin S. Parven<sup>1,2,3</sup>

President, ASP Associates

Former Vice President, Aetna Health Plans

#### Andreas Rummelt, PhD4,5

CEO, InterPharmaLink AG

Former Group Head, Quality Assurance and Technical Operations, Novartis

Former Member of Executive Committee, Novartis Former CEO, Sandoz AG

#### Ann M. Veneman3,4,5

Former Executive Director of UNICEF Former Secretary of U.S. Department of Agriculture

#### **Senior Management**

#### Leonard Bell, MD,

Chairman of the Board, Chief Executive Officer

#### David Hallal

Chief Operating Officer, CEO-elect

#### Vikas Sinha, MBA, CA, CPA

Executive Vice President, Chief Financial Officer

#### Martin Mackay, PhD

Executive Vice President, Global Head of Research & Development

#### Clare Carmichael

Executive Vice President, Chief Human Resources Officer

#### Sagib Islam, JD

Executive Vice President, Chief Strategy & Portfolio Officer

#### John B. Moriarty, Jr., JD

Executive Vice President, General Counsel

#### Julie O'Neill

Executive Vice President, Global Operations

#### Edward Miller, JD

Senior Vice President, Global Chief Compliance Officer

#### Dominique Monnet

Senior Vice President, Chief Marketing Officer

#### Carsten Thiel, PhD

Senior Vice President, EMEA & Asia-Pacific

#### **Annual Shareholders Meeting**

To be held on Wed., May 6, 2015 5:30 p.m.

The Study at Yale 1157 Chapel Street New Haven, CT 06511 tel 203 503 3900

#### Other Information

#### Corporate Headquarters

Alexion Pharmaceuticals, Inc. 352 Knotter Drive Cheshire, CT 06410

tel 203.272.2596 fax 203.271.8190

#### Transfer Agent and Registrar

Computershare Trust Company, N.A. 250 Royall Street Canton, MA 02021

#### **Investor Relations**

Rx Communications 555 Madison Avenue 5th Floor New York, NY 10022

tel 917.322.2568 fax 917.322.2570

#### Legal Counsel

Ropes & Gray LLP Boston, MA

# Independent Auditors

PricewaterhouseCoopers LLP Hartford, CT

#### Trading Symbol

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

alexion.com

- <sup>1</sup> Member of the Audit and Finance Committee
- <sup>2</sup> Member of the Compensation Committee
- <sup>3</sup> Member of the Nominating and Corporate Governance Committee
- <sup>4</sup> Member of the Pharmaceutical Compliance and Quality Committee
- <sup>5</sup> Member of the Strategy and Risk Committee
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#### **About The Cover**

"Evie was diagnosed with hypophosphatasia when she was two weeks old, and we were so scared that she would not survive because she was having seizures, had difficulty breathing and was so fragile since she did not develop bones properly. A few months later we were fortunate to enroll Evie in a clinical trial for asfotase alfa. It has been a long road, but Evie is now five years old and is a true joy. She loves preschool and playing with her sisters — it is a blessing each day to watch who she is becoming."

- Lindsey E., mother of a child with HPP

# Alexion Pharmaceuticals, Inc.

352 Knotter Drive, Cheshire, CT 06410, USA

#### Alexion Pharma International Sàrl

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