January 30, 2014

Alexion Reports Fourth Quarter and Full Year 2013 Results and Provides Financial Guidance for 2014

- Soliris® (eculizumab) Net Product Sales Increased 37 Percent to $1.551 Billion in 2013 -

- 2013 Non-GAAP EPS Increased 47 Percent to $3.08; GAAP EPS of $1.27 -

- Steady Soliris Growth in PNH Worldwide -

- aHUS Launch Progresses in U.S. and Europe; Japan Launch Commences in Q4 -

- Strong Progress Across Lead Development Programs -

- Establishment of mRNA Research Capabilities Through Strategic Agreement With Moderna Therapeutics -

- 2014 Guidance: Revenue $2.00 to $2.02 Billion; Non-GAAP EPS $3.70 to $3.80 -

Full Year 2013 Financial Highlights

- 2013 net product sales increased 37 percent to $1.551 billion, compared to $1.134 billion in 2012.
- 2013 GAAP net income was $252.9 million, or $1.27 per share, which was impacted by $0.48 per share related to a non-cash tax expense associated with centralizing certain business operations and $0.17 per share related to impairment of intangible assets. 2012 GAAP net income was $254.8 million, or $1.28 per share.
- 2013 non-GAAP net income increased 47 percent to $624.2 million, or $3.08 per share, compared to 2012 non-GAAP net income of $425.2 million, or $2.13 per share.

Fourth Quarter 2013 Financial Highlights

- Q4 2013 net product sales increased 38 percent to $441.9 million, compared to $320.5 million in Q4 2012.
- Q4 2013 GAAP net loss was $19.0 million, or $0.10 per share, which was impacted by $0.48 per share related to a non-cash tax expense associated with centralizing certain business operations and $0.17 per share related to impairment of intangible assets. Q4 2012 GAAP net income was $81.0 million, or $0.40 per share.
- Q4 2013 non-GAAP net income increased 45 percent to $177.7 million, or $0.87 per share, compared to Q4 2012 non-GAAP net income of $122.3 million, or $0.60 per share.

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced financial results for the quarter and year ended December 31, 2013. For the three months ended December 31, 2013, Alexion Pharmaceuticals, Inc. ("Alexion" or the "Company") reported net product sales of Soliris® (eculizumab) of $441.9 million, compared to $320.5 million for the same period in 2012. The year-on-year increase in Q4 net product sales of 38 percent reflected steady additions of new patients with paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS) commencing Soliris treatment.

Soliris is approved in nearly 50 countries for the treatment of patients with PNH, including the United States, European Union and Japan. Soliris is also approved in the United States, European Union, Japan and other countries as the first and only treatment for pediatric and adult patients with aHUS, a genetic, chronic, ultra-rare disease associated with vital organ failure and premature death.

Alexion's non-GAAP operating results are GAAP operating results adjusted for the impact of certain items described below. A full reconciliation of GAAP to non-GAAP financial results is included later in this press release.

Full Year 2013 Non-GAAP Financial Results
The Company reported non-GAAP net income of $624.2 million in 2013, or $3.08 per share, compared to non-GAAP net income of $425.2 million, or $2.13 per share, in 2012.

Alexion's non-GAAP operating expenses for the full year 2013 were $719.3 million, compared to $556.2 million for 2012. Non-GAAP research and development (R&D) expenses for 2013 were $278.7 million, compared to $208.9 million for the prior year. Non-GAAP selling, general and administrative (SG&A) expenses for 2013 were $440.6 million, compared to $347.3 million in 2012.

Full Year 2013 GAAP Financial Results
Alexion reported GAAP net income of $252.9 million, or $1.27 per share, in 2013 compared to 2012 GAAP net income of $254.8 million, or $1.28 per share. Full year 2013 GAAP results were impacted by $153.0 million, or $0.77 per share, related to non-cash tax expense associated with centralizing certain business operations, impairment of intangible assets, expenses from license agreements, and an intellectual property settlement. Full year 2012 GAAP results included an increase of $27.1 million, or $0.13 per share, related to the net effect of an intellectual property settlement and an impairment of an intangible asset.

Alexion's GAAP operating expenses for the full year 2013 were $845.8 million, compared to $656.9 million for the prior year. GAAP R&D expenses for 2013 were $317.1 million, compared to $222.7 million in 2012. GAAP SG&A expenses for 2013 were $489.7 million, compared to $384.7 million for the prior year.

Fourth Quarter Non-GAAP Financial Results
The Company reported non-GAAP net income of $177.7 million, or $0.87 per share in Q4 2013, compared to non-GAAP net income of $122.3 million, or $0.60 per share, in Q4 2012.

Alexion's non-GAAP operating expenses for Q4 2013 were $201.7 million, compared to $163.2 million for Q4 2012. Non-GAAP R&D expenses for Q4 2013 were $79.8 million, compared to $59.9 million for Q4 2012. Non-GAAP SG&A expenses for Q4 2013 were $121.8 million, compared to $103.3 million for Q4 2012.

Fourth Quarter GAAP Financial Results
Alexion reported a GAAP net loss of $19.0 million, or $0.10 per share in Q4 2013, compared to Q4 2012 GAAP net income of $81.0 million, or $0.40 per share. Q4 2013 GAAP results were impacted by $95.8 million, or $0.48 per share, related to a non-cash tax expense associated with centralizing certain business operations, and $33.5 million, or $0.17 per share, related to impairment of intangible assets.

On a GAAP basis, operating expenses for Q4 2013 were $252.3 million, compared to $179.5 million for Q4 2012. GAAP R&D expenses for Q4 2013 were $85.8 million, compared to $63.4 million for Q4 2012. GAAP SG&A expenses for Q4 2013 were $134.8 million, compared to $112.6 million for Q4 2012.

Balance Sheet
As of December 31, 2013, the Company had $1.515 billion in cash, cash equivalents and marketable securities compared to $989.5 million at December 31, 2012.

"In 2013, we provided Soliris to an increasing number of patients with PNH and aHUS worldwide. We demonstrated steady growth in PNH, grew steadily the number of new patients with aHUS receiving Soliris in the U.S. and the first countries of Western Europe, and began serving initial patients with aHUS in Japan," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "Throughout 2014, we will focus on serving more patients with PNH and aHUS globally. At the same time, we will advance our lead pipeline initiatives toward achieving ten or more development milestones as we drive toward our anticipated series of as many as seven potential product approvals between 2014 and 2018."

Research and Development Progress
Alexion currently has development programs underway with eculizumab (Soliris) and additional highly innovative therapeutic candidates that have the potential to become first-in-class therapies for patients with severe and ultra-rare disorders.

Ultra-Rare Disease Programs With Eculizumab

- **Transplant: Antibody-Mediated Rejection (AMR)** - Enrollment is ongoing in the Company-sponsored, multinational living-donor kidney transplant trial in patients at elevated risk of AMR and in the expanded Company-sponsored, multinational deceased-donor kidney transplant trial in patients at elevated risk of AMR.
- **Transplant: Delayed Graft Function (DGF)** - Alexion is planning to commence a single, multinational registration trial for the prevention of delayed graft function (DGF) in renal transplant patients. Earlier this month, eculizumab received an orphan drug designation from the U.S. Food and Drug Administration (FDA) for the prevention of delayed graft function (DGF) in renal transplant patients.
- **Neurology: Neuromyelitis Optica (NMO)** - Alexion is planning to commence a single, multinational, placebo-controlled, registration trial in relapsing NMO.
- **Neurology: Myasthenia Gravis (MG) -** Alexion is planning to commence a single, multinational, placebo-controlled,

registration trial in severe, refractory MG.

Ultra-Rare Disease Programs with Additional Highly Innovative Therapeutics

- **Asfotase Alfa:** Alexion is developing asfotase alfa as a treatment for patients with pediatric-onset hypophosphatasia (HPP), an ultra-rare, inherited and life-threatening metabolic disease. The Company received Breakthrough Therapy designation for asfotase alfa in pediatric-onset HPP in Q2 2013. Alexion completed the initial analysis of its natural history study in infants with HPP and has now initiated a natural history study in juveniles with HPP.
- **cPMP Replacement Therapy (ALXN 1101):** Alexion is developing cPMP as a treatment for patients with Molybdenum Cofactor Deficiency (MoCD) Type A, a severe, ultra-rare and genetic metabolic disorder that causes catastrophic and irreversible neurologic damage within the first few weeks of life. The Company received Breakthrough Therapy designation for cPMP replacement therapy for patients with MoCD Type A in Q3 2013. A natural history study in MoCD patients is ongoing and Alexion plans to initiate a synthetic cPMP bridging study.
- **ALXN1007:** Alexion is preparing to commence two Phase 2 proof-of-concept studies of ALXN1007, a novel anti-inflammatory antibody, in severe and life-threatening ultra-rare disorders.

Establishment of mRNA Research Capabilities

Beyond its current development programs, the Company announced on January 13, 2014 that it is establishing messenger RNA research capabilities through an exclusive strategic agreement with Moderna Therapeutics. Products based on messenger RNA are expected to have significant potential for Alexion, as they are well-suited to address the large number of severe and rare disorders caused by protein deficiencies. Under the agreement, Alexion will purchase 10 product options to develop and commercialize treatments for rare diseases with Moderna. Alexion will lead the discovery, development and commercialization of the treatments produced through this broad, long-term strategic agreement, while Moderna will retain responsibility for the design and manufacture of the messenger RNA product candidates.

2014 Financial Guidance

In 2014, worldwide net product sales are expected to be within a range of $2.00 to $2.02 billion. On a non-GAAP basis, R&D expenses are expected to be in the range of $360 to $380 million, and SG&A expenses in the range of $560 to $580 million. Cost of goods sold is expected to be approximately 9 percent of net product sales. Non-GAAP earnings per share for the year are expected to be $3.70 to $3.80, based on a forecast of approximately 205 million diluted shares outstanding. The non-GAAP tax rate, reported on a cash tax liability basis, is expected to be approximately 10 to 11 percent; the GAAP tax rate is expected to be approximately 20 to 22 percent.

Conference Call/Web Cast Information

Alexion will host a conference call/webcast to discuss matters mentioned in this release. The call is scheduled for today, January 30 at 10:00 a.m., ET. To participate in this conference call, dial 888-487-0361 (USA) or 719-325-2249 (International), passcode 9926357 shortly before 10:00 a.m. ET. A replay of the call will be available from 1:00 p.m. ET through a limited time thereafter. The replay number is 888-203-1112 (USA) or 719-457-0820 (International), passcode 9926357. The audio webcast can be accessed on the Investor page at www.alexionpharma.com.

About Soliris

Soliris is a first-in-class terminal complement inhibitor developed from the laboratory through regulatory approval and commercialization by Alexion. Soliris is approved in the U.S. (2007), European Union (2007), Japan (2010) and other countries as the first and only treatment for patients with paroxysmal nocturnal hemoglobinuria (PNH), a debilitating, ultra-rare and life-threatening blood disorder, characterized by complement-mediated hemolysis (destruction of red blood cells). Soliris is indicated to reduce hemolysis. Soliris is also approved in the U.S. (2011), the European Union (2011), Japan (2013) and other countries as the first and only treatment for patients with atypical hemolytic uremic syndrome (aHUS), a debilitating, ultra-rare and life-threatening genetic disorder characterized by complement-mediated thrombotic microangiopathy, or TMA (blood clots in small vessels). Soliris is indicated to inhibit complement-mediated TMA. The effectiveness of Soliris in aHUS is based on its effects on TMA and renal function. Prospective clinical trials in additional patients, the preliminary results of which were reported at international nephrology and hematology conferences in 2013, are ongoing to confirm the benefit of Soliris in patients with aHUS. Soliris is not indicated for the treatment of patients with Shiga-toxin E. coli-related hemolytic uremic syndrome (STEC-HUS). For the breakthrough innovation in complement inhibition, Alexion and Soliris have received the pharmaceutical industry's highest honors: the 2008 Prix Galien USA Award for Best Biotechnology Product with broad implications for future biomedical research and the 2009 Prix Galien France Award in the category of Drugs for Rare Diseases.

More information including the full U.S. prescribing information on Soliris is available at www.soliris.net.

About Alexion

Alexion is a biopharmaceutical company focused on serving patients with severe and rare disorders through the innovation, development and commercialization of life-transforming therapeutic products. Alexion is the global leader in complement inhibition and has developed and markets Soliris® (eculizumab) as a treatment for patients with PNH and aHUS, two debilitating, ultra-rare and life-threatening disorders caused by chronic uncontrolled complement activation. Soliris is currently approved in

nearly 50 countries for the treatment of PNH, and in the United States, European Union, Japan and other countries for the treatment of aHUS. Alexion is evaluating other potential indications for Soliris in additional severe and ultra-rare disorders beyond PNH and aHUS, and is developing other highly innovative biotechnology product candidates across multiple therapeutic areas. This press release and further information about Alexion Pharmaceuticals can be found at: www.alexionpharma.com.

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This news release contains forward-looking statements, including statements related to guidance regarding anticipated financial results for 2014, assessment of the Company's financial position and commercialization efforts, medical benefits and commercial potential for Soliris for PNH and aHUS and other potential indications, medical and commercial potential of Alexion's complement-inhibition technology and other technologies, and plans for clinical programs for each of our product candidates. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including for example, decisions of regulatory authorities regarding marketing approval or material limitations on the marketing of Soliris for PNH and aHUS and other potential indications, delays, interruptions or failures in the manufacture and supply of Soliris and our product candidates, progress in establishing and developing commercial infrastructure, failure to satisfactorily address the issues raised by the FDA in the Warning Letter received by Alexion in March 2013, the possibility that results of clinical trials are not predictive of safety and efficacy results of Soliris in broader patient populations in the disease studied or other diseases, the risk that acquisitions will not result in short-term or long-term benefits, the possibility that current results of commercialization are not predictive of future rates of adoption of Soliris in PNH, aHUS or other diseases, the possibility that clinical trials of our product candidates could be delayed or that additional research and testing is required by regulatory agencies, the risk that third party payors (including governmental agencies) will not reimburse or continue to reimburse for the use of Soliris at acceptable rates or at all, the risk that estimates regarding the number of patients with PNH, aHUS or other diseases are inaccurate, and a variety of other risks set forth from time to time in Alexion's filings with the U.S. Securities and Exchange Commission, including but not limited to the risks discussed in Alexion's Quarterly Report on Form 10-Q for the period ended September 30, 2013 and in our other filings with the U.S. Securities and Exchange Commission. Alexion does not intend to update any of these forward-looking statements to reflect events or circumstances after the date hereof, except when a duty arises under law.

In addition to financial information prepared in accordance with GAAP, this news release also contains non-GAAP financial measures that Alexion believes, when considered together with the GAAP information, provide investors and management with supplemental information relating to performance, trends and prospects that promote a more complete understanding of our operating results and financial position during different periods. The non-GAAP results exclude the impact of the following GAAP items: share-based compensation expense, acquisition-related costs, amortization of purchased intangible assets, intellectual property settlements, upfront and milestone payments related to license and collaboration agreements, intangible asset impairments, non-cash taxes, and taxes related to acquisition structuring. These non-GAAP financial measures are not intended to be considered in isolation or as a substitute for, or superior to, the financial measures prepared and presented in accordance with GAAP and should be reviewed in conjunction with the relevant GAAP financial measures. Please refer to the attached Reconciliation of GAAP to Non-GAAP Net Income for explanations of the amounts adjusted to arrive at non-GAAP net income and non-GAAP earnings per share amounts for the three and twelve month periods ended December 31, 2013 and 2012.

ALEXION PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(in thousands, except per share amounts)
(unaudited)

	Three months ended December 31		Twelve months ended December 31	
	2013	**2012**	**2013**	**2012**
Net product sales	$ 441,909	$ 320,526	$1,551,346	$1,134,114
Cost of sales:				
Cost of sales	51,552	33,147	168,375	126,214
Change in contingent liability from intellectual property settlements	-	-	9,181	(53,377)
Total cost of sales	51,552	33,147	177,556	72,837
Operating expenses:				
Research and development	85,785	63,409	317,093	222,732
Selling, general and administrative	134,819	112,624	489,720	384,678

Acquisition-related costs	(1,945)	3,365	5,029	22,812
Impairment of intangible assets	33,521	-	33,521	26,300
Amortization of purchased intangible assets	105	105	417	417
Total operating expenses	252,285	179,503	845,780	656,939
Operating income	138,072	107,876	528,010	404,338
Other expense	95	606	1,741	6,772
Income before income taxes	137,977	107,270	526,269	397,566
Income tax provision	156,969	26,298	273,374	142,744
Net income (loss)	$ (18,992)	$ 80,972	$ 252,895	$ 254,822
Earnings (loss) per common share				
Basic	$ (0.10)	$ 0.42	$ 1.29	$ 1.34
Diluted	$ (0.10)	$ 0.40	$ 1.27	$ 1.28
Shares used in computing earnings (loss) per common share				
Basic	196,430	194,141	195,532	190,461
Diluted	196,430	201,061	199,712	198,501

ALEXION PHARMACEUTICALS, INC.
RECONCILIATION OF GAAP TO NON-GAAP FINANCIAL RESULTS
(in thousands, except per share amounts)
(unaudited)

	Three months ended December 31		Twelve months ended December 31	
	2013	**2012**	**2013**	**2012**
Net income (loss) reconciliation:				
GAAP net income (loss)	$ (18,992)	$ 80,972	$ 252,895	$ 254,822
Share-based compensation expense	19,794	13,691	76,203	54,013
Acquisition-related costs (1)	(1,945)	3,365	5,029	22,812
Amortization of purchased intangible assets	105	105	417	417
Change in contingent liability from intellectual property settlements (2)	-	-	9,181	(53,377)
Upfront and milestone payments related to license and collaboration agreements (3)	-	-	14,500	-
Impairment of intangible assets (4)	33,521	-	33,521	26,300
Non-cash taxes (5)	145,266	24,158	232,460	98,364
Taxes related to acquisition structuring (6)	-	-	-	21,812
Non-GAAP net income	$ 177,749	$122,291	$ 624,206	$ 425,163
GAAP earnings (loss) per share - diluted	$ (0.10)	$ 0.40	$ 1.27	$ 1.28
Non-GAAP earnings per share - diluted	$ 0.87	$ 0.60	$ 3.08	$ 2.13
Shares used in computing diluted earnings (loss) per share (GAAP)	196,430	201,061	199,712	198,501
Shares used in computing diluted earnings per share (non-GAAP)	203,586	202,249	202,943	199,787

Cost of sales reconciliation:				
GAAP cost of sales	$ 51,552	$ 33,147	$ 177,556	$ 72,837
Share-based compensation expense	(865)	(876)	(3,214)	(2,815)
Change in contingent liability from intellectual property settlements (2)	-	-	(9,181)	53,377
Non-GAAP cost of sales	$ 50,687	$ 32,271	$ 165,161	$ 123,399
Research and development reconciliation:				
GAAP research and development	$ 85,785	$ 63,409	$ 317,093	$ 222,732
Share-based compensation expense	(5,944)	(3,466)	(23,905)	(13,839)
Upfront and milestone payments related to license and collaboration agreements (3)	-	-	(14,500)	-
Non-GAAP research and development	$ 79,841	$ 59,943	$ 278,688	$ 208,893
Selling, general and administrative reconciliation:				
GAAP selling, general and administrative	$ 134,819	$112,624	$ 489,720	$ 384,678
Share-based compensation expense	(12,985)	(9,349)	(49,084)	(37,359)
Non-GAAP selling, general and administrative	$ 121,834	$103,275	$ 440,636	$ 347,319
Income tax provision reconciliation:				
GAAP income tax provision	$ 156,969	$ 26,298	$ 273,374	$ 142,744
Non-cash taxes (5)	(145,266)	(24,158)	(232,460)	(98,364)
Taxes related to acquisition structuring (6)	-	-	-	(21,812)
Non-GAAP income tax provision	$ 11,703	$ 2,140	$ 40,914	$ 22,568

(1) The following table summarizes acquisition-related costs:

	Three months ended December 31		Twelve months ended December 31	
	2013	2012	2013	2012
Acquisition-related costs:				
Separately-identifiable employee costs	$ -	$ 117	$ 248	$ 3,669
Professional fees	-	1,031	775	12,593
Changes in fair value of contingent consideration	(1,945)	2,217	4,006	6,550
	$ (1,945)	$ 3,365	$ 5,029	$ 22,812

(2) In October 2013, the Company entered into a litigation settlement and license agreement, which resulted in an increase of $9.2 million in cost of sales in the third quarter 2013.

In October 2012, the Company entered into an intellectual property settlement and license agreement, which resulted in a decrease of $53.4 million in cost of sales in the third quarter 2012.

(3) In July 2013, the Company entered into a license and collaboration agreement for the identification, development, and commercialization of therapeutic candidates based on specific drug targets. The Company recorded research and development expense for an upfront payment of $11.5 million.

In January 2013, the Company entered into a license agreement for specific targets and products to be developed. The Company recorded research and development expense for an upfront payment of $3.0 million.

(4) During the three and twelve months ended December 31, 2013, the Company recorded an impairment of intangible assets of $33.5 million related to early stage development assets.

During the twelve months ended December 31, 2012, the Company recorded an impairment of an intangible asset of $26.3 million related to an early stage development asset.

(5) Non-cash taxes represents the adjustment from GAAP tax expense to the amount of taxes that are payable in cash on our operating profits.

The adjustment includes tax amounts that are not currently payable in cash due to the continued utilization of our US net

operating losses and credits. In addition, during the three and twelve months ended December 31, 2013, we also recorded non-cash tax expense in connection with our centralization of certain business operations of $95.8 million. This tax expense was attributable to the recording of a deferred tax liability on basis differences related to our foreign subsidiaries.

(6) The tax provision for the twelve months ended December 31, 2012 includes tax expense of $21.8 million related to the structuring of the Enobia acquisition.

ALEXION PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(in thousands)
(unaudited)

	December 31, 2013	December 31, 2012
Cash and cash equivalents	$ 529,857	$ 989,501
Marketable securities	984,994	-
Trade accounts receivable, net	421,752	295,598
Inventories	102,602	94,521
Deferred tax assets, current	41,432	26,086
Other current assets	106,220	89,894
Property, plant and equipment, net	201,109	165,629
Deferred tax assets, noncurrent	3,394	13,954
Intangible assets, net	609,719	646,678
Goodwill	254,073	253,645
Other noncurrent assets	62,544	38,054
Total assets	$ 3,317,696	$ 2,613,560
Accounts payable and accrued expenses	$ 423,940	$ 271,275
Current portion of long-term debt	48,000	48,000
Other current liabilities	110,489	40,814
Long-term debt, less current portion	65,000	101,000
Contingent consideration, noncurrent	106,744	139,002
Deferred tax liabilities, noncurrent	101,241	19,827
Other noncurrent liabilities	80,203	22,792
Total liabilities	935,617	642,710
Total stockholders' equity	2,382,079	1,970,850
Total liabilities and stockholders' equity	$ 3,317,696	$ 2,613,560

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Source: Alexion Pharmaceuticals, Inc.

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