April 25, 2013

Alexion Reports First Quarter 2013 Results

Soliris® (eculizumab) Net Product Sales Increased 38 Percent vs. Year-Ago Quarter to $338.9 Million

Continued Steady Growth in PNH; aHUS Launch Progressing

Guidance Revised Upward for Revenues and Non-GAAP EPS

Deceased-Donor Kidney Transplant Trial Enrollment Completed

Hypophosphatasia Infant Natural History Study Enrollment Completed

Single Myasthenia Gravis Registration Trial Now Planned

Retrospective cPMP Clinical Study in MoCD Commenced; First Study With Synthetic cPMP Planned

First Quarter 2013 Financial Highlights:

- Q1 2013 net product sales increased 38 percent to $338.9 million, compared to $244.7 million in Q1 2012.
- Q1 2013 GAAP net income increased 81 percent to $82.2 million, or $0.41 per share, compared to Q1 2012 GAAP net income of $45.4 million, or $0.23 per share.
- Q1 2013 non-GAAP net income increased 49 percent to $131.3 million, or $0.65 per share, compared to Q1 2012 non-GAAP net income of $88.1 million, or $0.45 per share.

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ: ALXN) today announced financial results and a development pipeline update for the three months ended March 31, 2013. The Company reported net product sales of Soliris® (eculizumab) of $338.9 million, an increase of 38 percent from the same period in 2012. Revenue performance for the quarter reflects steady additions of new patients with paroxysmal nocturnal hemoglobinuria (PNH) globally, and an increasing number of new patients with atypical Hemolytic Uremic Syndrome (aHUS) commencing Soliris treatment in the US and Western Europe.

"The first quarter was particularly noteworthy for the significant progress across our lead development programs. This included completing enrollment in the deceased-donor kidney transplant trial and the hypophosphatasia infant natural history study, initiating plans for a single myasthenia gravis registration trial, and accelerating our cPMP clinical programs," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "During this past quarter we continued to serve more patients with PNH around the world, as well as a growing number of aHUS patients in the US, with initial aHUS patients commencing treatment in countries of Western Europe. Further, in line with our longer term growth objectives, we have also announced earlier today that we are broadening and strengthening our executive leadership team."

First Quarter 2013 Financial Results:

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

First Quarter 2013 Non-GAAP Financial Results:

The Company reported non-GAAP net income of $131.3 million, or $0.65 per share, in the first quarter of 2013, compared to non-GAAP net income of $88.1 million, or $0.45 per share, in the first quarter of 2012.

Alexion's non-GAAP operating expenses for Q1 2013 were $164.4 million, compared to $119.9 million for Q1 2012. Non-GAAP research and development (R&D) expenses for Q1 2013 were $66.4 million, compared to $42.1 million for Q1 2012. Non-GAAP selling, general and administrative (SG&A) expenses for Q1 2013 were $97.9 million, compared to $77.9 million for Q1 2012.

First Quarter 2013 GAAP Financial Results:

Alexion reported GAAP net income of $82.2 million, or $0.41 per share, in the first quarter of 2013, compared to GAAP net income of $45.4 million, or $0.23 per share, in the first quarter of 2012.

On a GAAP basis, operating expenses for Q1 2013 were $186.7 million, compared to $146.4 million for Q1 2012. GAAP R&D expenses for Q1 2013 were $74.5 million, compared to $45.4 million for Q1 2012. GAAP SG&A expenses were $108.8 million for Q1 2013, compared to $87.2 million for Q1 2012.

Balance Sheet:

As of March 31, 2013, the Company had $1.02 billion in cash and cash equivalents compared to $989.5 million at December 31, 2012.

Research and Development Progress:

Alexion currently has development programs underway with its five highly innovative therapeutic candidates: eculizumab (Soliris) and four additional novel therapeutic candidates beyond eculizumab that have the potential to become first-in-class therapies for patients with other severe and ultra-rare disorders.

Ultra-Rare Disease Programs With Eculizumab

- **Neurology: NMO** - Alexion is preparing to commence what it expects to be a single Company-sponsored, multi-national, placebo-controlled, registration trial in relapsing neuromyelitis optica (NMO).
- **Neurology: MG —** Alexion is also preparing to commence what it expects to be a single Company-sponsored, multi-national, placebo-controlled, registration trial in severe refractory myasthenia gravis (MG).
- **Nephrology: Kidney Transplant** — Enrollment has now been completed in the Company-sponsored, multi-national deceased-donor kidney transplant trial in patients at elevated risk of antibody mediated rejection (AMR). Enrollment in the Company-sponsored, multi-national living-donor kidney transplant trial in patients at elevated risk of AMR is on-going. Alexion is also expanding its kidney transplant program to include a delayed-graft function (DGF) clinical trial.
- **Nephrology: STEC-HUS** — The Company is obtaining and analyzing additional longer-term control clinical outcome data from an epidemiologic study in approximately 400 STEC-HUS patients who received only best supportive care during the 2011 German epidemic.

Ultra-Rare Disease Programs with Highly Innovative Therapeutics Beyond Eculizumab

- **Asfotase Alfa:** Alexion completed enrollment in a retrospective natural history study in infants with hypophosphatasia (HPP), an ultra-rare, inherited and life-threatening metabolic disease.
- **cPMP Replacement Therapy:** Alexion is developing a cPMP replacement therapy for the treatment of patients with Molybdenum Cofactor Deficiency Type A (MoCD), a severe, ultra-rare and genetic metabolic disorder that is fatal in newborns. Alexion has commenced a retrospective cPMP study in MoCD patients and a study with the Company's synthetic cPMP is planned.
- **ALXN1102/1103:** Enrollment continues in a Phase I study to characterize the mechanism of action and develop initial safety data for ALXN1102 and ALXN1103, intravenous and subcutaneous versions, respectively, of one of Alexion's novel complement inhibitors.
- **ALXN1007:** Alexion has completed dosing in a single-dose Phase I study of ALXN1007, a novel anti-inflammatory antibody, to evaluate the safety, tolerability, pharmacokinetics and pharmacodynamics of this therapeutic candidate in healthy volunteers. The Company expects to meet with regulators to discuss a multi-dose Phase I study.

2013 Financial Guidance:

Alexion today announced that it is raising its 2013 revenue guidance from the previous range of $1.490 to $1.505 billion, now to the higher range of $1.505 to $1.520 billion. The upward revision reflects continued global growth of Soliris in PNH and growth from the ongoing launch of Soliris in aHUS. Guidance for 2013 non-GAAP earnings per share is also being raised, from the previous range of $2.82 to $2.92, now to the higher range of $2.87 to $2.97 per share. Alexion is reiterating all other items of the 2013 financial guidance provided in the Company's press release of February 14, 2013.

Conference Call/Web Cast Information:

Alexion will host a conference call/audio web cast to discuss matters mentioned in this release. The call is scheduled for today, April 25, at 10:00 a.m., Eastern Time. To participate in this call, dial 888-312-3051 (USA) or 719-325-2168 (International), passcode 7274940, shortly before 10:00 a.m., Eastern Time. A replay of the call will be available for a limited period following

the call, beginning at 1:00 PM, Eastern Time. The replay number is 888-203-1112 (USA) or 719-457-0820 (International), passcode 7274940. The audio webcast can be accessed 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 US, European Union, Japan 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 US and the European Union 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 the effects on TMA and renal function. Prospective clinical trials in additional patients 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 prescribing information on Soliris is available at www.soliris.net.

About Alexion:

Alexion Pharmaceuticals, Inc. is a biopharmaceutical company focused on serving patients with severe and ultra-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 more than 40 countries for the treatment of PNH, and in the United States and European Union for the treatment of aHUS. Alexion is evaluating other potential indications for Soliris and is developing four other highly innovative biotechnology product candidates, which are being investigated across nine severe and ultra-rare disorders beyond PNH and aHUS. This press release and further information about Alexion Pharmaceuticals, Inc. 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 2013, 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, expansion of clinical and commercial operations to additional countries, medical and commercial potential of Alexion's complement-inhibition technology and other technologies, plans for clinical programs for each of our product candidates and progress in developing commercial infrastructure. 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 in arranging satisfactory manufacturing capabilities and establishing commercial infrastructure, failure to satisfactorily address the issues raised by the FDA in the Warning Letter recently disclosed by Alexion, 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 risk that third parties will not agree to license any necessary intellectual property to Alexion on reasonable terms or at all, 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 disorders are inaccurate, and a variety of other risks set forth from time to time in Alexion's filings with the US Securities and Exchange Commission, including but not limited to the risks discussed in Alexion's Annual Report on Form 10-K for the period ended December 31, 2012 and in our other filings with the US 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: the impact of share-based compensation expense, acquisition-related costs, taxes related to acquisition structuring, intangible asset impairments, upfront and milestone payments related to licensing and collaboration agreements, and non-cash taxes. 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 month periods ended March 31, 2013 and 2012.

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

	Three months ended March 31	
	2013	**2012**
Net product sales	$ 338,941	$ 244,733
Cost of sales	35,269	28,268
Research and development	74,536	45,408
Selling, general and administrative	108,826	87,242
Acquisition-related costs	3,234	13,673
Amortization of purchased intangible assets	104	104
Total operating expenses	186,700	146,427
Operating income	116,972	70,038
Interest and other expense	(231)	(2,229)
Income before income taxes	116,741	67,809
Income tax provision	34,524	22,396
Net income	$ 82,217	$ 45,413
Earnings per common share		
Basic	$ 0.42	$ 0.24
Diluted	$ 0.41	$ 0.23
Shares used in computing earnings per common share		
Basic	194,771	185,682
Diluted	199,057	194,560

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

	Three months ended March 31	
	2013	**2012**
GAAP net income	$ 82,217	$ 45,413
Share-based compensation expense (1)	16,855	13,318
Acquisition-related costs (2)	3,234	13,673
Amortization of purchased intangible assets	104	104
Upfront and milestone payments related to license and collaboration agreements (3)	3,000	-
Non-cash taxes (4)	25,904	15,553

Non-GAAP net income	$ 131,314	$ 88,061
Shares used in computing diluted earnings per share (GAAP)	199,057	194,560
Shares used in computing diluted earnings per share (non-GAAP)	202,225	195,895
GAAP earnings per share - diluted	$ 0.41	$ 0.23
Non-GAAP earnings per share - diluted	$ 0.65	$ 0.45

(1) The following table summarizes the share-based compensation expense for each expense category in our condensed consolidated statements of operations:

	Three months ended March 31	
	2013	2012
Share-based compensation expense:		
Cost of sales	$ 875	$ 603
Research and development	5,090	3,349
Selling, general and administrative	10,890	9,366
	$ 16,855	$ 13,318

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

	Three months ended March 31	
	2013	2012
Acquisition-related costs:		
Separately-identifiable employee costs	$ 248	$ 2,296
Professional fees	775	8,469
Changes in fair value of contingent consideration	2,211	2,908
	$ 3,234	$ 13,673

(3) In January 2013, the Company entered into a license agreement providing Alexion with an exclusive research license and an option for an exclusive commercial license for specific targets and products to be developed from a platform technology. Under the terms of the agreement, the Company recorded research and development expense for an upfront payment of $3,000.

(4) Non-cash taxes represents the adjustment from GAAP tax expense to the amount of taxes that are payable in cash. 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.

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

	March 31 2013	December 31, 2012
Cash and cash equivalents	$ 1,022,960	$ 989,501
Trade accounts receivable, net	323,907	295,598
Inventories, net	101,856	94,521
Deferred tax assets, current	19,995	26,086
Other current assets	81,323	89,894
Property, plant and equipment, net	164,966	165,629
Deferred tax assets, noncurrent	11,980	13,954

Intangible assets, net	644,965	646,678
Goodwill	254,073	253,645
Other noncurrent assets	59,856	38,054
Total assets	$ 2,685,881	$ 2,613,560
Accounts payable and accrued expenses	$ 212,545	$ 271,275
Current portion of long-term debt	48,000	48,000
Other current liabilities	35,202	40,814
Long-term debt	101,000	101,000
Contingent consideration	141,181	139,002
Other noncurrent liabilities	57,900	42,619
Total liabilities	595,828	642,710
Total stockholders' equity	2,090,053	1,970,850
Total liabilities and stockholders' equity	$ 2,685,881	$ 2,613,560

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Source: Alexion Pharmaceuticals, Inc.