



July 25, 2013

Alexion Reports Second Quarter 2013 Results

Soliris® (eculizumab) Net Product Sales Increased 35 Percent to \$370.1 million

Continued Steady Soliris Growth Globally in PNH

aHUS Launch Progresses in US and Europe

Guidance Revised Upward for 2013 Revenues and Non-GAAP EPS

Strong Progress in HPP, NMO, Transplant and Other Pipeline Programs

Second Quarter 2013 Financial Highlights:

- Q2 2013 net product sales increased 35 percent to \$370.1 million, compared to \$274.7 million in Q2 2012.
- Q2 2013 GAAP net income increased 164 percent to \$95.9 million, or \$0.48 per share, compared to Q2 2012 GAAP net income of \$36.3 million, or \$0.18 per share. Q2 2012 GAAP net income included \$21.8 million of tax expense related to structuring of the Enobia acquisition.
- Q2 2013 non-GAAP net income increased 56 percent to \$147.2 million, or \$0.73 per share, compared to Q2 2012 non-GAAP net income of \$94.1 million, or \$0.47 per share.

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ: ALXN) today announced financial results for the three and six months ended June 30, 2013. The Company reported net product sales of Soliris® (eculizumab) of \$370.1 million in the second quarter of 2013, an increase of 35 percent from the same period in 2012.

Revenue performance for the quarter reflected 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 Europe.

"In the second quarter, we continued our strong and ongoing global performance with Soliris in PNH. In the early stages of our aHUS launch, we were especially pleased to provide Soliris to a steadily growing number of patients in the US and increasingly in Europe," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "Key pipeline initiatives, including our asfotase alfa program in HPP and our Soliris programs in NMO and transplant, reached new milestones. As we enter the second half of the year, we will continue to develop our product portfolio toward supporting an anticipated series of significant launches in new indications for Soliris and new products over the next several years."

Second 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.

Second Quarter 2013 Non-GAAP Financial Results:

The Company reported non-GAAP net income of \$147.2 million, or \$0.73 per share, in the second quarter of 2013, compared to non-GAAP net income of \$94.1 million, or \$0.47 per share, in the second quarter of 2012.

Alexion's non-GAAP operating expenses for Q2 2013 were \$174.5 million, compared to \$142.2 million for Q2 2012. Non-GAAP research and development (R&D) expenses for Q2 2013 were \$63.5 million, compared to \$56.3 million for Q2 2012. Non-GAAP selling, general and administrative (SG&A) expenses for Q2 2013 were \$111.0 million, compared to \$85.9 million for Q2 2012.

Second Quarter 2013 GAAP Financial Results:

Alexion reported GAAP net income of \$95.9 million, or \$0.48 per share, in the second quarter of 2013, compared to GAAP net income of \$36.3 million, or \$0.18 per share, in the second quarter of 2012. Q2 2012 GAAP net income included \$21.8 million of tax expense related to structuring of the Enobia acquisition.

On a GAAP basis, operating expenses for Q2 2013 were \$193.0 million, compared to \$159.4 million for Q2 2012. GAAP R&D

expenses for Q2 2013 were \$68.6 million, compared to \$59.6 million for Q2 2012. GAAP SG&A expenses were \$123.2 million for Q2 2013, compared to \$94.9 million for Q2 2012.

Balance Sheet:

As of June 30, 2013, the Company had \$1.119 billion in cash, cash equivalents and marketable securities 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 that have the potential to become first-in-class therapies for patients with severe and ultra-rare disorders.

Ultra-Rare Disease Programs With Eculizumab

- **Neurology: Neuromyelitis Optica (NMO)** - At the end of June, Soliris received an Orphan Drug designation from the US Food and Drug Administration (FDA) for the treatment of NMO. In mid-July, Soliris received a positive opinion for Orphan Drug designation from the European Committee for Orphan Medicinal Products. Upon regulatory approvals for this indication, the US and European Orphan Drug designations would entitle Soliris to seven years and ten years, respectively, of marketing exclusivity in NMO. The Company is preparing to commence what it expects to be a single Company-sponsored, multinational, placebo-controlled, registration trial in relapsing NMO.
- **Neurology: Myasthenia Gravis (MG)** - Alexion is also preparing to commence what it expects to be a single Company-sponsored, multinational, placebo-controlled, registration trial in severe, refractory MG.
- **Nephrology: Kidney Transplant** - During the quarter, the Company completed dosing in its multinational deceased-donor kidney transplant trial in patients at elevated risk of antibody mediated rejection (AMR). Enrollment in the Company-sponsored, multinational 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 has obtained and is analyzing longer-term control clinical outcome data from an epidemiologic study in approximately 400 STEC-HUS patients who received only best supportive care during the earlier German epidemic.

Ultra-Rare Disease Programs with Additional Highly Innovative Therapeutics

- **Asfotase Alfa:** During Q2, Alexion received Breakthrough Therapy designation status from the FDA for asfotase alfa for the treatment of patients with hypophosphatasia (HPP) whose first signs or symptoms occurred prior to 18 years of age. The Company also completed enrollment in a retrospective natural history study in infants with 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. Dosing with the Company's synthetic cPMP replacement has now commenced in a study in healthy volunteers. Additionally, enrollment continues in the retrospective cPMP study in MoCD patients.
- **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. Dosing in a multi-dose Phase I study in healthy volunteers has now commenced.
- **ALXN1102/1103:** Enrollment continues in a Phase I study to characterize the mechanism of action and develop initial safety data for ALXN1102 and ALXN1103, different formulations of one of Alexion's novel complement inhibitors.

2013 Financial Guidance:

Alexion today announced that it is raising its 2013 revenue guidance from the previous range of \$1.505 to \$1.520 billion, now to the higher range of \$1.520 to \$1.530 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 EPS is also being revised upward, from the previous range of \$2.87 to \$2.97, now to the higher range of \$2.97 to \$3.02, based on a forecast of 204 million diluted shares outstanding.

On a non-GAAP basis, 2013 guidance for R&D expenses is being revised downward, from the previous range of \$285 to \$295 million, now to the lower range of \$275 to \$285 million, and 2013 guidance for SG&A expenses is being revised upward, from the previous range of \$425 to \$435 million, now to the higher range of \$435 to \$445 million. The Company's non-GAAP effective tax rate, reported on a cash tax liability basis, is being revised downward, from the previous range of 7 to 9 percent, now to the lower range of 6 to 8 percent. The Company's share-based compensation expense for the year is being revised upward, from the previous range of \$63 to \$67 million, now to the higher range of \$76 to \$78 million.

Other items of 2013 guidance provided in the Company's press release of February 14, 2013 are being reiterated today: cost of sales is expected to be approximately 10 percent of net product sales, and the Company's GAAP effective tax rate is expected to be in the range of 29 to 31 percent.

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, July 25, at 10:00 a.m., Eastern Time. To participate in this call, dial 877-675-4749 (USA) or 719-325-4804 (International), passcode 3976928, 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 3976928. 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, 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 in arranging satisfactory manufacturing capabilities and establishing commercial infrastructure, failure to satisfactorily address the issues raised by the FDA in the Warning Letter disclosed 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 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 diseases 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 Quarterly Report on Form 10-Q for the period ended March 31, 2013 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: share-based compensation expense, acquisition-related costs, amortization of purchased intangible assets, 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 and six month periods ended June 30, 2013 and 2012.

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

	Three months ended		Six months ended	
	June 30		June 30	
	2013	2012	2013	2012
Net product sales	\$370,091	\$274,719	\$709,032	\$519,452
Cost of sales	39,377	31,613	74,646	59,881
Research and development	68,563	59,635	143,099	105,043
Selling, general and administrative	123,189	94,855	232,015	182,097
Acquisition-related costs	1,167	4,807	4,401	18,480
Amortization of purchased intangible assets	104	104	208	208
Total operating expenses	<u>193,023</u>	<u>159,401</u>	<u>379,723</u>	<u>305,828</u>
Operating income	137,691	83,705	254,663	153,743
Interest and other expense	<u>(428)</u>	<u>(1,983)</u>	<u>(659)</u>	<u>(4,212)</u>
Income before income taxes	137,263	81,722	254,004	149,531
Income tax provision	41,378	45,464	75,902	67,860
Net income	<u>\$ 95,885</u>	<u>\$ 36,258</u>	<u>\$178,102</u>	<u>\$ 81,671</u>
Earnings per common share				
Basic	<u>\$ 0.49</u>	<u>\$ 0.19</u>	<u>\$ 0.92</u>	<u>\$ 0.44</u>
Diluted	<u>\$ 0.48</u>	<u>\$ 0.18</u>	<u>\$ 0.90</u>	<u>\$ 0.42</u>
Shares used in computing earnings per common share				
Basic	<u>195,247</u>	<u>188,575</u>	<u>193,944</u>	<u>187,129</u>
Diluted	<u>199,299</u>	<u>197,051</u>	<u>198,096</u>	<u>195,832</u>

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

	Three months ended		Six months ended	
	June 30		June 30	
	2013	2012	2013	2012
Net income reconciliation:				

GAAP net income	\$ 95,885	\$ 36,258	\$178,102	\$ 81,671
Share-based compensation expense	17,957	12,989	34,812	26,306
Acquisition-related costs (1)	1,167	4,807	4,401	18,480
Amortization of purchased intangible assets	104	104	208	208
Upfront and milestone payments related to license and collaboration agreements	-	-	3,000	-
Non-cash taxes (2)	32,117	18,103	58,021	33,657
Tax related to acquisition structuring (3)	-	21,812	-	21,812
Non-GAAP net income	<u>\$147,230</u>	<u>\$ 94,073</u>	<u>\$278,544</u>	<u>\$182,134</u>
GAAP earnings per share - diluted	<u>\$ 0.48</u>	<u>\$ 0.18</u>	<u>\$ 0.90</u>	<u>\$ 0.42</u>
Non-GAAP earnings per share - diluted	<u>\$ 0.73</u>	<u>\$ 0.47</u>	<u>\$ 1.38</u>	<u>\$ 0.92</u>
Shares used in computing diluted earnings per share (GAAP)	<u>199,299</u>	<u>197,051</u>	<u>198,096</u>	<u>195,832</u>
Shares used in computing diluted earnings per share (non-GAAP)	<u>202,593</u>	<u>198,431</u>	<u>201,340</u>	<u>197,180</u>
Cost of sales reconciliation:				
GAAP cost of sales	\$ 39,377	\$ 31,613	\$ 74,646	\$ 59,881
Share-based compensation expense	(717)	(672)	(1,592)	(1,275)
Non-GAAP cost of sales	<u>\$ 38,660</u>	<u>\$ 30,941</u>	<u>\$ 73,054</u>	<u>\$ 58,606</u>
Research and development reconciliation:				
GAAP research and development	\$ 68,563	\$ 59,635	\$143,099	\$105,043
Share-based compensation expense	(5,068)	(3,381)	(10,158)	(6,730)
Upfront and milestone payments related to license and collaboration agreements	-	-	(3,000)	-
Non-GAAP research and development	<u>\$ 63,495</u>	<u>\$ 56,254</u>	<u>\$129,941</u>	<u>\$ 98,313</u>
Selling, general and administrative reconciliation:				
GAAP selling, general and administrative	\$123,189	\$ 94,855	\$232,015	\$182,097
Share-based compensation expense	(12,172)	(8,936)	(23,062)	(18,301)
Non-GAAP selling, general and administrative	<u>\$111,017</u>	<u>\$ 85,919</u>	<u>\$208,953</u>	<u>\$163,796</u>
Income tax provision reconciliation:				
GAAP income tax provision	\$ 41,378	\$ 45,464	\$ 75,902	\$ 67,860
Non-cash taxes (2)	(32,117)	(18,103)	(58,021)	(33,657)
Tax related to acquisition structuring (3)	-	(21,812)	-	(21,812)
Non-GAAP income tax provision	<u>\$ 9,261</u>	<u>\$ 5,549</u>	<u>\$ 17,881</u>	<u>\$ 12,391</u>

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

	Three months ended June 30		Six months ended June 30	
	2013	2012	2013	2012
Acquisition-related costs:				
Separately-identifiable employee costs	\$ -	\$ 799	\$ 248	\$ 3,095
Professional fees	-	2,041	775	10,510
Changes in fair value of contingent consideration	1,167	1,967	3,378	4,875
	<u>\$ 1,167</u>	<u>\$ 4,807</u>	<u>\$ 4,401</u>	<u>\$ 18,480</u>

(2) 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.

(3) The tax provision for the three and six months ended June 30, 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)

	June 30, 2013	December 31, 2012
Cash and cash equivalents	\$ 936,464	\$ 989,501
Marketable securities	182,926	-
Trade accounts receivable, net	355,284	295,598
Inventories, net	112,627	94,521
Deferred tax assets, current	19,892	26,086
Other current assets	71,440	89,894
Property, plant and equipment, net	173,721	165,629
Deferred tax assets, noncurrent	9,734	13,954
Intangible assets, net	643,252	646,678
Goodwill	254,073	253,645
Other noncurrent assets	55,825	38,054
Total assets	<u>\$2,815,238</u>	<u>\$ 2,613,560</u>
Accounts payable and accrued expenses	\$ 214,346	\$ 271,275
Current portion of long-term debt	48,000	48,000
Other current liabilities	40,632	40,814
Long-term debt	89,000	101,000
Contingent consideration	142,048	139,002
Other noncurrent liabilities	69,672	42,619
Total liabilities	<u>603,698</u>	<u>642,710</u>
Total stockholders' equity	2,211,540	1,970,850
Total liabilities and stockholders' equity	<u>\$2,815,238</u>	<u>\$ 2,613,560</u>

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Source: Alexion Pharmaceuticals, Inc.

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