October 24, 2013

Alexion Reports Third Quarter 2013 Results

Soliris® (eculizumab) Net Product Sales Increased 36 Percent to $400.4 million

Steady Soliris Growth in PNH Worldwide

aHUS Launch Progresses in US and Europe; Japan Launch Begins in Q4

Guidance Revised Upward for 2013 Revenues and Non-GAAP EPS

Strong Progress in Pipeline Programs, cPMP Replacement Therapy Receives FDA Breakthrough Therapy Designation

Third Quarter 2013 Financial Highlights:

- Q3 2013 net product sales increased 36 percent to $400.4 million, compared to $294.1 million in Q3 2012.
- Q3 2013 GAAP net income increased to $93.8 million, or $0.47 per share, compared to net income of $92.2 million, or $0.46 per share, in Q3 2012. Q3 2013 GAAP EPS included a decrease of $0.10 per share related to expenses from both a license agreement and a litigation settlement. Q3 2012 GAAP EPS included an increase of $0.13 per share related to the net effect of an intellectual property settlement and an impairment loss.
- Q3 2013 non-GAAP net income increased 39 percent to $167.9 million, or $0.83 per share, compared to Q3 2012 non-GAAP net income of $120.7 million, or $0.60 per share.

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced financial results for the three and nine months ended September 30, 2013. The Company reported net product sales of Soliris® (eculizumab) of $400.4 million in the third quarter of 2013, an increase of 36 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 third quarter, we continued our strong and ongoing global performance with Soliris in PNH and were pleased to provide Soliris to a steadily growing number of new patients with aHUS in the United States as well as an increasing number of patients with aHUS in Europe. In addition, we were pleased to receive our aHUS approval in Japan," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "Key pipeline initiatives, including our asfotase alfa program in HPP, our Soliris programs in kidney transplant and our cPMP replacement therapy program, reached new milestones in Q3. In Q4, we are further expanding our commercial and clinical initiatives as we prepare for further growth in 2014."

Third 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 to non-GAAP financial results is included later in this press release.

Third Quarter 2013 Non-GAAP Financial Results:
The Company reported non-GAAP net income of $167.9 million, or $0.83 per share, in the third quarter of 2013, compared to non-GAAP net income of $120.7 million, or $0.60 per share, in the third quarter of 2012.

Alexion's non-GAAP operating expenses for Q3 2013 were $178.8 million, compared to $130.9 million for Q3 2012. Non-GAAP research and development (R&D) expenses for Q3 2013 were $68.9 million, compared to $50.6 million for Q3 2012. Non-GAAP selling, general and administrative (SG&A) expenses for Q3 2013 were $109.9 million, compared to $80.3 million for Q3 2012.

Third Quarter 2013 GAAP Financial Results:
Alexion reported GAAP net income of $93.8 million, or $0.47 per share, in the third quarter of 2013, compared to GAAP net income of $92.2 million, or $0.46 per share, in the third quarter of 2012.

Q3 2013 GAAP results included a decrease of $20.7 million, or $0.10 per share, related to expenses from both a license agreement and a litigation settlement. Q3 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 loss.

On a GAAP basis, operating expenses for Q3 2013 were $213.8 million, compared to $171.6 million for Q3 2012. GAAP R&D expenses for Q3 2013 were $88.2 million, compared to $54.3 million for Q3 2012. GAAP SG&A expenses were $122.9 million for Q3 2013, compared to $90.0 million for Q3 2012.

Balance Sheet:
As of September 30, 2013, the Company had $1.30 billion in cash, cash equivalents and marketable securities compared to $1.12 billion at June 30, 2013.

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)** — Alexion will commence a single, multinational, placebo-controlled, registration trial in relapsing NMO.
- **Neurology: Myasthenia Gravis (MG)** — Alexion will commence a single, multinational, placebo-controlled, registration trial in severe, refractory MG.
- **Transplant: Antibody-Mediated Rejection (AMR)** — During the quarter, researchers presented preliminary data from the Company-sponsored, multinational deceased-donor kidney transplant trial in patients at elevated risk of AMR. Enrollment in the Company-sponsored, multinational living-donor kidney transplant trial in patients at elevated risk of AMR is ongoing.
- **Transplant: Delayed Graft Function (DGF)** — Based on recent regulatory discussions, Alexion now plans to conduct a single multinational, placeo-controlled, registration trial in patients at risk for DGF.
- **Nephrology: STEC-HUS** — The Company continues to analyze 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:** Alexion is developing asfotase alfa as a treatment for pediatric-onset hypophosphatasia (HPP), an ultra-rare, inherited and life-threatening metabolic disease. During the quarter, researchers presented data from the ongoing study of asfotase alfa in infants and young children with HPP. The Company completed its initial analysis of its natural history study in infants with HPP. The Company received Breakthrough Therapy designation for asfotase alfa in pediatric-onset HPP in Q2 2013.

- **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. Alexion has initiated a natural history study in patients with MoCD Type A and has also completed dosing with the synthetic cPMP in a study in healthy volunteers. The Company received Breakthrough Therapy designation for cPMP replacement therapy for patients with MoCD Type A, as announced earlier today.

- **ALXN1007:** Alexion has commenced a multi-dose Phase I clinical study of ALXN1007, a novel anti-inflammatory antibody, in healthy volunteers. The Company is preparing to commence a multi-dose Phase II proof-of-concept study of ALXN1007.
- **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 $1.520 to $1.530 billion, now to the higher and narrower range of $1.535 to $1.540 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 $2.97 to $3.02, now to the higher and narrower range of $3.02 to $3.04, based on a forecast of approximately 203 million diluted shares outstanding. Guidance for R&D is being narrowed from the previous range of $275 to

$285 million now to $280 to $285 million. SG&A is also being narrowed from the previous range of $435 to $445 million now to $440 to $445 million.

Other items of 2013 guidance provided in the Company's press release of July 25, 2013 are being reiterated today. The Company's non-GAAP effective tax rate, reported on a cash tax liability basis, is expected to be 6 to 8 percent. The Company's GAAP effective tax rate is expected to be in the range of 29 to 31 percent. The Company's share-based compensation expense for the year is expected to be $76 to $78 million. Cost of sales is expected to be approximately 10 percent of net product sales. The Company's GAAP effective tax rate is expected to be in the range of 29 to 31 percent.

Conference Call/Webcast Information:

Alexion will host a conference call/audio webcast to discuss matters mentioned in this release. The call is scheduled for today, October 24, at 10:00 a.m., Eastern Time. To participate in this call, dial 800-289-0438 (USA) or 913-312-0706 (International), passcode 3882932, 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 3882932. 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, European Union, and Japan 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 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, 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, 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 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 June 30, 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, 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 nine month periods ended September 30, 2013 and 2012.

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

	Three months ended September 30		Nine months ended September 30	
	2013	**2012**	**2013**	**2012**
Net product sales	$ 400,405	$ 294,136	$ 1,109,437	$ 813,588
Cost of sales:				
Cost of sales	42,177	33,186	116,823	93,067
Change in contingent liability from intellectual property settlements	9,181	(53,377)	9,181	(53,377)
Total cost of sales	51,358	(20,191)	126,004	39,690
Operating expenses:				
Research and development	88,209	54,280	231,308	159,323
Selling, general and administrative	122,886	89,957	354,901	272,054
Acquisition-related costs	2,573	967	6,974	19,447
Impairment of intangible asset	-	26,300	-	26,300
Amortization of purchased intangible assets	104	104	312	312
Total operating expenses	213,772	171,608	593,495	477,436
Operating income	135,275	142,719	389,938	296,462
Other income and expense	(987)	(1,954)	(1,646)	(6,165)
Income before income taxes	134,288	140,765	388,292	290,297
Income tax provision	40,503	48,586	116,405	116,446
Net Income	$ 93,785	$ 92,179	$ 271,887	$ 173,851
Earnings per common share				
Basic	$ 0.48	$ 0.48	$ 1.40	$ 0.92
Diluted	$ 0.47	$ 0.46	$ 1.37	$ 0.88

Shares used in computing earnings per common share				
Basic	195,662	193,353	194,520	189,219
Diluted	199,711	201,142	198,655	197,635

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

	Three months ended September 30		Nine months ended September 30	
	2013	**2012**	**2013**	**2012**
Net income reconciliation:				
GAAP net income	$ 93,785	$ 92,179	$271,887	$173,851
Share-based compensation expense	21,597	14,015	56,409	40,322
Acquisition-related costs (1)	2,573	967	6,974	19,447
Amortization of purchased intangible assets	104	104	312	312
Change in contingent liability from intellectual property settlements (2)	9,181	(53,377)	9,181	(53,377)
Upfront and milestone payments related to license and collaboration agreements (3)	11,500	-	14,500	-
Impairment of intangible asset (4)	-	26,300	-	26,300
Non-cash taxes (5)	29,173	40,550	87,194	74,207
Tax related to acquisition structuring (6)	-	-	-	21,812
Non-GAAP net income	$ 167,913	$ 120,738	$446,457	$302,874
GAAP earnings per share - diluted	$ 0.47	$ 0.46	$ 1.37	$ 0.88
Non-GAAP earnings per share - diluted	$ 0.83	$ 0.60	$ 2.21	$ 1.52
Shares used in computing diluted earnings per share (GAAP)	199,711	201,142	198,655	197,635
Shares used in computing diluted earnings per share (non-GAAP)	202,988	202,377	201,886	198,953
Cost of sales reconciliation:				
GAAP cost of sales	$ 51,358	$ (20,191)	$126,004	$ 39,690
Share-based compensation expense	(757)	(664)	(2,349)	(1,939)
Change in contingent liability from intellectual property settlements (2)	(9,181)	53,377	(9,181)	53,377
Non-GAAP cost of sales	$ 41,420	$ 32,522	$114,474	$ 91,128
Research and development reconciliation:				
GAAP research and development	$ 88,209	$ 54,280	$231,308	$159,323
Share-based compensation expense	(7,803)	(3,643)	(17,961)	(10,373)
Upfront and milestone payments related to license and collaboration agreements (3)	(11,500)	-	(14,500)	-
Non-GAAP research and development	$ 68,906	$ 50,637	$198,847	$148,950
Selling, general and administrative reconciliation:				
GAAP selling, general and administrative	$ 122,886	$ 89,957	$354,901	$272,054
Share-based compensation expense	(13,037)	(9,708)	(36,099)	(28,010)
Non-GAAP selling, general and administrative	$ 109,849	$ 80,249	$318,802	$244,044
Income tax provision reconciliation:				
GAAP income tax provision	$ 40,503	$ 48,586	$116,405	$116,446
Non-cash taxes (5)	(29,173)	(40,550)	(87,194)	(74,207)

Tax related to acquisition structuring (6)	-	-	-	(21,812)
Non-GAAP income tax provision	$ 11,330	$ 8,036	$ 29,211	$ 20,427

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

	Three months ended September 30		Nine months ended September 30	
	2013	2012	2013	2012
Acquisition-related costs:				
Separately-identifiable employee costs	$ -	$ 457	$ 248	$ 3,552
Professional fees	-	1,052	775	11,562
Changes in fair value of contingent consideration	2,573	(542)	5,951	4,333
	$ 2,573	$ 967	$ 6,974	$ 19,447

(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 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.

(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. Under the terms of the agreement, 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. Under the terms of the agreement, the Company recorded research and development expense for an upfront payment of $3.0 million.

(4) During the three months ended September 30, 2012, the Company recorded an impairment of an intangible asset of $26.3 million related to a preclinical program.

(5) 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.

(6) The tax provision for the nine months ended September 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)

	September 30, 2013	December 31, 2012
Cash and cash equivalents	$ 910,411	$ 989,501
Marketable securities	392,344	-
Trade accounts receivable, net	404,956	295,598
Inventories	105,196	94,521
Deferred tax assets, current	23,820	26,086
Other current assets	78,775	89,894
Property, plant and equipment, net	178,842	165,629
Deferred tax assets, noncurrent	9,743	13,954
Intangible assets, net	646,138	646,678
Goodwill	254,073	253,645
Other noncurrent assets	45,497	38,054
Total assets	$ 3,049,795	$ 2,613,560

Accounts payable and accrued expenses	$	280,200	$	271,275
Current portion of long-term debt		48,000		48,000
Other current liabilities		67,256		40,814
Long-term debt, less current portion		77,000		101,000
Contingent consideration		144,621		139,002
Other noncurrent liabilities		77,952		42,619
Total liabilities		695,029		642,710
Total stockholders' equity		2,354,766		1,970,850
Total liabilities and stockholders' equity	$	3,049,795	$	2,613,560

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Source: Alexion Pharmaceuticals, Inc.