October 27, 2016

Alexion Reports Third Quarter 2016 Results

- Total Revenues of $799 million; 20 Percent Increase Year-on-Year; 23 Percent Volume Increase Year-on-Year -

- Soliris® (eculizumab) Revenue Growth Driven by Steady Number of New Patients with PNH and aHUS Treated Globally -

- Robust Strensiq® (asfotase alfa) Launch Progresses with New Patients Starting on Treatment -

- Kanuma® (sebelipase alfa) Launch Continues with New Patients Treated in Initial Countries -

- Alexion to File Regulatory Submissions for Eculizumab for the Treatment of Patients with Refractory gMG in both the U.S. and Europe in Q1 2017 -

- ALXN1210 PNH Registration Trial Initiated with Once Every Eight Week Dosing; Enrollment to Begin in Q4 -

- ALXN1210 aHUS Registration Trial Initiated with Once Every Eight Week Dosing; Enrollment to Begin in Q4 -

- ALXN1210 Subcutaneous Clinical Program Commenced with Dosing Underway in Healthy Volunteers in Phase I Study -

NEW HAVEN, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ: ALXN) today announced financial results for the third quarter of 2016. Total revenues grew to $799 million, a 20 percent increase, compared to $667 million for the same period in 2015. In the third quarter, the negative impact of foreign currency on total revenue was 2.5 percent or $16 million, net of hedging activities, compared to the same quarter last year. On a GAAP basis, diluted earnings per share (EPS) for the third quarter of 2016 was $0.42 per share, compared to a loss of $0.81 per share in the third quarter of 2015. Non-GAAP diluted EPS for the third quarter of 2016 was $1.23 per share. Non-GAAP diluted EPS was $1.08 per share in the third quarter of 2015, reflecting a reduction of $0.08 per share to conform to the current non-GAAP income tax expense definition.

"In Q3 2016, we delivered strong financial performance and served an increasing number of patients with PNH, aHUS, HPP and LAL-D, while also achieving significant R&D milestones," said David Hallal, Chief Executive Officer of Alexion. "As we continue to grow our complement and metabolic businesses, we are working with urgency to file our regulatory submissions for eculizumab for the treatment of patients with refractory gMG in both the U.S. and Europe, and to enroll patients with PNH and aHUS into the global ALXN1210 registration trials."

Third Quarter 2016 Financial Highlights

- Soliris® (eculizumab) net product sales were $729 million, compared to $665 million in Q3 2015.
- Strensiq® (asfotase alfa) net product sales were $61 million.
- Kanuma® (sebelipase alfa) net product sales were $9 million.
- GAAP R&D expense was $196 million, compared to $166 million in the same quarter last year. Non-GAAP R&D expense was $180 million, compared to $147 million in the same quarter last year.
- GAAP SG&A expense was $230 million, compared to $213 million in the same quarter last year. Non-GAAP SG&A expense was $201 million, compared to $182 million in the same quarter last year.
- GAAP diluted EPS was $0.42 per share, compared to a loss of $0.81 per share in the same quarter last year. Non-GAAP diluted EPS was $1.23 per share. Non-GAAP diluted EPS was $1.08 per share in the third quarter of 2015, reflecting a reduction of $0.08 per share to conform to the current non-GAAP income tax expense definition.

Product and Pipeline Updates

Complement Portfolio

- **Eculizumab- Refractory Generalized Myasthenia Gravis (gMG):** Alexion plans to file regulatory submissions for eculizumab for the treatment of patients with refractory gMG in both the United States and Europe in the first quarter of 2017.
- **Eculizumab- Relapsing Neuromyelitis Optica Spectrum Disorder (NMOSD):** The PREVENT study, a single, multinational, placebo-controlled registration trial of eculizumab in patients with relapsing NMOSD is on-going, with data expected in 2017.
- **Eculizumab- Delayed Graft Function (DGF):** Data from the PROTECT study, a single, multinational, placebo-controlled registration trial of eculizumab in the prevention of DGF, are expected during the fourth quarter of 2016.
- **ALXN1210- PNH:** Alexion has initiated a PNH registration trial of ALXN1210 administered intravenously every eight weeks. Enrollment is expected to begin in the fourth quarter of 2016.
- **ALXN1210- aHUS:** Alexion has initiated an aHUS registration trial with ALXN1210 administered intravenously every eight weeks. Enrollment is expected to begin in the fourth quarter of 2016.
- **ALXN1210- Subcutaneous:** Alexion has commenced dosing of a new formulation of ALXN1210 administered subcutaneously in healthy volunteers in a Phase I study.
- **ALXN1007:** Alexion is evaluating higher doses of ALXN1007, a complement inhibitor that targets C5a, in a Phase 2 study of patients with graft-versus-host disease involving the lower gastrointestinal tract (GI-GVHD). The FDA and the European Commission granted orphan drug designation to ALXN1007 for the treatment of patients with GVHD.

Metabolic Portfolio

- **SBC-103:** A Phase 1/2 study of SBC-103, a recombinant form of the NAGLU enzyme, in patients with mucopolysaccharidosis IIIB, or MPS IIIB, is on-going. Alexion has completed the planned dose escalation, with all patients now randomized to either a 5 mg/kg or 10 mg/kg dose. A natural history study to characterize the course of disease progression in patients with MPS IIIB is also ongoing.
- **cPMP Replacement Therapy (ALXN1101):** Alexion is enrolling patients in a pivotal study to evaluate ALXN1101 in neonates with Molybdenum Cofactor Deficiency (MoCD) Type A.

Immuno-Oncology Program

- **Samalizumab (ALXN6000):** Samalizumab is a first-in-class immunomodulatory humanized monoclonal antibody that blocks the key immune checkpoint protein, CD200. The Leukemia and Lymphoma Society announced the BEAT AML Master Trial, a multi-arm clinical trial in acute myeloid leukemia (AML), which will evaluate samalizumab as well as other potential therapies for the treatment of AML.

Preclinical Portfolio

- Alexion has more than 30 diverse preclinical programs across a range of therapeutic modalities.

2016 Financial Guidance

Alexion expects 2016 total revenues to be at the upper end of our previously guided range of $3.05 to $3.10 billion. Alexion is reiterating its Soliris revenue guidance and based on the strength of the Strensiq launch is further increasing its Metabolic revenue guidance to $225 to $235 million.

R&D and SG&A expense guidance has been increased to reflect acceleration of the ALXN1210 programs and additional investment in the global infrastructure to support the launches of Strensiq and Kanuma, as well as an increase in legal expenses.

Alexion's updated 2016 GAAP EPS guidance is expected to be in the range of $1.79 to $2.09 and non-GAAP EPS guidance is now expected to be at the upper end of the previously guided range of $4.50 to $4.65 per share.

Updated 2016 financial guidance is as follows:

	Updated GAAP Guidance	Prior GAAP Guidance	**Updated Non-GAAP Guidance**	Prior Non-GAAP Guidance
Total revenues	Upper end of $3,050 to $3,100 million	$3,050 to $3,100 million	Upper end of $3,050 to $3,100 million	$3,050 to $3,100 million
	$2,835 to $2,875	$2,835 to $2,875	$2,835 to $2,875	

Soliris revenues	million	million	million	$2,835 to $2,875 million
Metabolic revenues	$225 to $235 million	$200 to $220 million	$225 to $235 million	$200 to $220 million
Cost of sales	8% to 9%	8% to 9%	8% to 9%	8% to 9%
Research and development expense	$740 to $781 million	$708 to $779 million	$680 to $690 million	High end of $650 to $680 million
Selling, general and administrative expense	$913 to $955 million	$883 to $935 million	$790 to $810 million	High end of $760 to $790 million
Interest expense	$100 million	$100 million	$100 million	$100 million
Effective tax rate	32% to 34%	32% to 34%	15.5% to 16.5%	15.5% to 16.5%
Earnings per share	$1.79 to $2.09	$1.91 to $2.26	Upper end of $4.50 to $4.65	$4.50 to $4.65
Diluted shares outstanding	228 million	228 million	230 million	230 million

Alexion's 2016 financial guidance is based on current foreign exchange rates net of hedging activities and does not include the effect of business combinations, license and collaboration agreements, asset acquisitions, intangible asset impairments, changes in fair value of contingent consideration or restructuring activity that may occur after the day prior to the date of this press release.

Conference Call/Webcast Information:

Alexion will host a conference call/audio webcast to discuss its third quarter 2016 results at 10:00 a.m. Eastern Time. To participate in this call, dial 888-487-0355 (USA) or 719-325-2123 (International), passcode 9676571 shortly before 10:00 a.m. Eastern Time. A replay of the call will be available for a limited period following the call. The replay number is 888-203-1112 (USA) or 719-457-0820 (International), passcode 9676571. The audio webcast can be accessed on the Investor page of Alexion's website at: http://ir.alexionpharm.com.

About Alexion

Alexion is a global biopharmaceutical company focused on developing and delivering life-transforming therapies for patients with devastating and rare disorders. Alexion developed and commercializes Soliris® (eculizumab), the first and only approved complement inhibitor to treat patients with paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS), two life-threatening ultra-rare disorders. As the global leader in complement inhibition, Alexion is strengthening and broadening its portfolio of complement inhibitors, including evaluating potential indications for eculizumab in additional severe and ultra-rare disorders. Alexion's metabolic franchise includes two highly innovative enzyme replacement therapies for patients with life-threatening and ultra-rare disorders, Strensiq® (asfotase alfa) to treat patients with hypophosphatasia (HPP) and Kanuma® (sebelipase alfa) to treat patients with lysosomal acid lipase deficiency (LAL-D). In addition, Alexion is advancing the most robust rare disease pipeline in the biotech industry with highly innovative product candidates in multiple therapeutic areas. This press release and further information about Alexion can be found at: www.alexion.com.

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This press release contains forward-looking statements, including statements related to guidance regarding anticipated financial results for 2016, assessment of the Company's financial position and commercialization efforts, medical benefits and commercial potential for Soliris, Strensiq and Kanuma, medical and commercial potential of each of Alexion's product candidates, launch expectations for Strensiq and Kanuma, and plans for regulatory filings and clinical programs for 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 our products, delays, interruptions or failures in the manufacture and supply of our products and our product candidates, progress in establishing and developing commercial infrastructure, failure to satisfactorily address matters raised by the FDA and other regulatory agencies, the possibility that results of clinical trials are not predictive of safety and efficacy results of our products in broader patient populations in the disease studied or other diseases, the risk that strategic transactions 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, including for ALXN1210, the adequacy of our pharmacovigilance and drug safety reporting processes, the risk that third party payors (including governmental agencies) will not reimburse or continue to reimburse for the use of our products at acceptable rates or at all, risks regarding government investigations, including investigations of Alexion by the SEC and DOJ investigations, the risk that anticipated regulatory filings are delayed, the risk that estimates regarding the number of patients with PNH, aHUS, HPP and LAL-D are inaccurate, the risks of shifting foreign exchange

rates, 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 June 30, 2016 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 press 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, fair value adjustment of inventory acquired, amortization of purchased intangible assets, changes in fair value of contingent consideration, acquisition-related costs, restructuring expenses, upfront and milestone payments related to licenses and collaborations and adjustments to income tax expense. 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 Reconciliations of GAAP to non-GAAP Financial Results and GAAP to non-GAAP 2016 Financial Guidance 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, 2016 and 2015 and projected twelve months ended December 31, 2016.

(Tables Follow)

ALEXION PHARMACEUTICALS, INC.
TABLE 1: CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(in thousands, except per share amounts)
(unaudited)

	Three months ended September 30		Nine months ended September 30	
	2016	**2015**	**2016**	**2015**
Net product sales	$798,524	$ 665,791	$2,251,495	$1,902,107
Other revenue	582	846	1,765	1,073
Total revenues	799,106	666,637	2,253,260	1,903,180
Cost of sales	71,095	54,057	190,708	175,463
Operating expenses:				
Research and development	195,687	165,664	551,288	518,437
Selling, general and administrative	230,128	212,520	694,491	621,019
Amortization of purchased intangible assets	82,036	36,608	242,185	36,608
Change in fair value of contingent consideration	40,290	29,684	30,676	45,707
Acquisition-related costs	—	6,075	2,313	35,852
Restructuring expenses	564	7,461	1,741	30,737
Total operating expenses	548,705	458,012	1,522,694	1,288,360
Operating income	179,306	154,568	539,858	439,357
Other income and expense:				
Investment income	4,626	1,967	8,049	7,077
Interest expense	(24,807)	(19,971)	(72,490)	(24,593)
Foreign currency (loss) gain	(1,011)	2,795	(3,740)	1,755
Income before income taxes	158,114	139,359	471,677	423,596
Income tax expense	63,776	323,116	165,113	345,815
Net income (loss)	$ 94,338	$(183,757)	$ 306,564	$ 77,781
Earnings (loss) per common share				
Basic	$ 0.42	$ (0.81)	$ 1.37	$ 0.37
Diluted	$ 0.42	$ (0.81)	$ 1.35	$ 0.37
Shares used in computing earnings per common share				
Basic	224,180	226,228	224,454	209,373
Diluted	226,088	226,228	226,560	211,808

ALEXION PHARMACEUTICALS, INC.
TABLE 2: 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	
	2016	2015	2016	2015
GAAP net income (loss)	$ 94,338	$ (183,757)	$ 306,564	$ 77,781
Before tax adjustments:				
Cost of sales:				
Share-based compensation	2,704	1,470	8,185	4,223
Fair value adjustment in inventory acquired (1)	6,585	-	8,442	-
Research and development expense:				
Share-based compensation	14,232	19,087	43,811	43,500
Upfront and milestone payments related to licenses and collaborations	1,489	-	4,539	114,250
Selling, general and administrative expense:				
Share-based compensation	29,405	30,499	99,213	113,130
Amortization of purchased intangible assets (2)	82,036	36,608	242,185	36,608
Change in fair value of contingent consideration	40,290	29,684	30,676	45,707
Acquisition-related costs (3)	-	6,075	2,313	35,852
Restructuring expenses	564	7,461	1,741	30,737
Adjustments to income tax expense (4) (5)	9,660	302,244	19,042	274,363
Non-GAAP net income	$ 281,303	$ 249,371	$ 766,711	$ 776,151
GAAP earnings (loss) per share - diluted	$ 0.42	$ (0.81)	$ 1.35	$ 0.37
Non-GAAP earnings per share - diluted (5)	$ 1.23	$ 1.08	$ 3.36	$ 3.62
Shares used in computing diluted earnings per share (GAAP)	226,088	226,228	226,560	211,808
Shares used in computing diluted earnings per share (non-GAAP)	228,008	230,875	228,464	214,146

(1) Inventory fair value adjustment associated with the amortization of Kanuma inventory step-up related to the purchase accounting for Synageva.

(2) In the third quarter of 2015, the Company initiated amortization of its purchased intangible assets due to the regulatory approvals for Strensiq & Kanuma.

(3) The following table summarizes acquisition related costs:

	Three months ended September 30		Nine Months Ended September 30	
	2016	2015	2016	2015
Acquisition-related costs:				
Transaction costs	$ —	$ —	$ 375	$ 26,799
Integration costs	—	6,075	1,938	9,053
	$ —	$ 6,075	$ 2,313	$ 35,852

(4) Alexion's non-GAAP income tax expense definition excludes the tax effect of pre-tax adjustments to GAAP net income

and intercompany transactions with our captive foreign partnership which would become due and payable only upon liquidation of a substantial portion of our non-US business interests.

(5) Previously reported non-GAAP tax expense and diluted EPS have been modified to conform to the current non-GAAP income tax definition adopted in Q2 2016. Previously reported non-GAAP EPS was $1.16 and $3.87 for the three and nine months ended September 30, 2015, respectively.

ALEXION PHARMACEUTICALS, INC.
TABLE 3: RECONCILIATION OF GAAP TO NON-GAAP FINANCIAL GUIDANCE
(in millions, except per share amounts)
(unaudited)

	Twelve months ended December 31 2016	
	Low	**High**
GAAP net income	$ 408	$ 477
Before tax adjustments:		
Cost of sales:		
Share-based compensation	12	10
Fair value adjustment in inventory acquired	12	10
Research and development expense:		
Share-based compensation	65	55
Upfront and milestone payments related to licenses and collaborations	26	5
Selling, general and administrative expense:		
Share-based compensation	145	123
Amortization of purchased intangible assets	322	322
Change in fair value of contingent consideration	36	36
Acquisition-related costs	2	2
Restructuring expenses	2	2
Adjustments to income tax expense	5	28
Non-GAAP net income	$ 1,035	$ 1,070
Diluted GAAP earnings per share	$ 1.79	$ 2.09
Diluted Non-GAAP earnings per share	$ 4.50	$ 4.65
Shares used in computing diluted earnings per share (GAAP)	228	228
Shares used in computing diluted earnings per share (non-GAAP)	230	230

	Twelve months ended December 31 2016	
	Low	**High**
GAAP income tax expense as a percentage of GAAP pre-tax income	34%	32%
Tax effect of pre-tax adjustments to GAAP net income	(6.5%)	(4.5%)
Tax effect of intercompany transactions	(11.0%)	(12.0%)
Non-GAAP income tax expenses as a percentage of non-GAAP pre-tax income	16.5%	15.5%

ALEXION PHARMACEUTICALS, INC.
TABLE 4: REVENUES
(in thousands)
(unaudited)

Three months ended September 30		Nine months ended September 30	
2016	**2015**	**2016**	**2015**

Soliris	$ 728,851	$ 665,404	$2,094,516	$1,901,720
Strensiq	60,531	357	138,914	357
Kanuma	9,142	30	18,065	30
Total net product sales	798,524	665,791	2,251,495	1,902,107
Royalty revenue	582	846	1,765	1,073
Total other revenue	582	846	1,765	1,073
Total revenues	$ 799,106	$ 666,637	$2,253,260	$1,903,180

ALEXION PHARMACEUTICALS, INC.
TABLE 5: CONDENSED CONSOLIDATED BALANCE SHEETS
(in thousands)
(unaudited)

	September 30, 2016	December 31, 2015
Cash and cash equivalents	$ 761,989	$ 1,010,111
Marketable securities	550,882	374,904
Trade accounts receivable, net	676,837	532,832
Inventories	363,058	289,874
Prepaid expenses and other current assets	241,768	208,993
Property, plant and equipment, net	931,060	697,025
Intangible assets, net	4,467,726	4,707,914
Goodwill	5,037,444	5,047,885
Other assets	262,698	228,343
Total assets	$ 13,293,462	$ 13,097,881
Accounts payable and accrued expenses	$ 530,083	$ 460,708
Deferred revenue	63,402	20,504
Current portion of long-term debt	122,942	166,365
Other current liabilities	36,066	6,234
Current portion of contingent consideration	81,848	55,804
Long-term debt, less current portion	3,129,384	3,254,536
Facility lease obligation	224,442	151,307
Contingent consideration	126,056	121,424
Deferred tax liabilities (1)	343,794	528,990
Other liabilities	131,342	73,393
Total liabilities	4,789,359	4,839,265
Total stockholders' equity (1)	8,504,103	8,258,616
Total liabilities and stockholders' equity	$ 13,293,462	$ 13,097,881

(1) In March 2016, the FASB issued a new standard intended to simplify certain aspects of the accounting for employee share-based payments. We elected to early adopt this standard during the third quarter of 2016. The adoption of the new standard requires recognition of excess tax benefits regardless of whether the benefit reduces taxes payable in the current period. As a result, $237,850 associated with previously unrecognized excess tax benefits was recorded as a deferred tax asset and an increase in retained earnings as of the beginning of 2016.

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