February 16, 2017

Alexion Reports Fourth Quarter and Full Year 2016 Results and Provides Financial Guidance for 2017

- Total Revenues of $3.084 Billion, an 18% Increase Over 2015 and 22% Volume Increase
- Global Soliris® Revenue Growth Driven by Steady Number of New Patients with PNH and aHUS
- Strensiq® and Kanuma® Global Launches Progress With New Patients Starting on Treatment
- Filed Regulatory Submissions for Soliris in Patients with Refractory gMG in the U.S. and Europe
- ALXN1210 Phase 3 Studies Underway in Patients with PNH and aHUS
- Alexion Board Increases Authorized Share Repurchase to a Total of $1 Billion

NEW HAVEN, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ: ALXN) today announced financial results for the fourth quarter and full year of 2016. Total revenues for the full year of 2016 were $3.084 billion, an 18 percent increase compared to 2015. The negative impact of foreign currency on total revenue year over year was 3 percent or $74 million, net of hedging activities. On a GAAP basis, diluted earnings per share (EPS) for the full year of 2016 was $1.76 per share, compared to $0.67 per share in 2015. Non-GAAP diluted EPS for the full year of 2016 was $4.62 per share. Non-GAAP diluted EPS was $4.65 per share for the full year of 2015, reflecting a reduction of $0.34 per share to conform to the current non-GAAP income tax expense definition.

Total revenues in the fourth quarter grew to $831 million, a 19 percent increase compared to the same period in 2015. The negative impact of foreign currency on total revenue in the fourth quarter was 2 percent or $12 million, net of hedging activities. On a GAAP basis, diluted EPS for the fourth quarter of 2016 was $0.41 per share, compared to $0.29 per share in the fourth quarter of 2015. Non-GAAP diluted EPS for the fourth quarter of 2016 was $1.26 per share. Non-GAAP diluted EPS was $1.04 per share in the fourth quarter of 2015, reflecting a reduction of $0.09 per share to conform to the current non-GAAP income tax expense definition. Both GAAP and non-GAAP results are inclusive of legal, accounting, and other costs associated with the Audit and Finance Committee's completed investigation.

"In 2016 the global Alexion team delivered on our patient-centered objectives as we grew our leadership in complement by serving more patients with PNH and aHUS, and continued to build our metabolic franchise with the global launches of Strensiq and Kanuma. We also achieved important regulatory milestones towards new indications for Soliris and initiated two registration studies for ALXN1210 to drive our future growth," said David Brennan, Interim Chief Executive Officer of Alexion. "Our 2017 guidance reflects double-digit revenue and EPS growth as we continue to grow our complement and metabolic franchises, prepare for the potential launches of Soliris in refractory gMG, and focus on our highest priority R&D programs."

Full Year 2016 Financial Highlights

- Soliris® (eculizumab) net product sales were $2,843 million, compared to $2,591 million in 2015.
- Strensiq® (asfotase alfa) net product sales were $210 million, compared to $12 million in 2015.
- Kanuma® (sebelipase alfa) net product sales were $29 million.
- GAAP R&D expense was $757 million, compared to $709 million in 2015. Non-GAAP R&D expense was $690 million, compared to $515 million in 2015.
- GAAP SG&A expense was $954 million, compared to $863 million in 2015. Non-GAAP SG&A expense was $830 million, compared to $707 million in 2015.
- GAAP diluted EPS was $1.76 per share, compared to $0.67 per share in 2015. Non-GAAP diluted EPS was $4.62 per share. Non-GAAP diluted EPS was $4.65 per share in 2015, reflecting a reduction of $0.34 per share to conform to the current non-GAAP income tax expense definition.

Fourth Quarter 2016 Financial Highlights

- Soliris® net product sales were $749 million, compared to $689 million in the fourth quarter of 2015.

- Strensiq® net product sales were $71 million, compared to $12 million in the fourth quarter of 2015.
- Kanuma® net product sales were $11 million.
- GAAP R&D expense was $206 million, compared to $191 million in the same quarter last year. Non-GAAP R&D expense was $186 million, compared to $155 million in the same quarter last year.
- GAAP SG&A expense was $259 million, compared to $242 million in the same quarter last year. Non-GAAP SG&A expense was $234 million, compared to $198 million in the same quarter last year.
- GAAP diluted EPS was $0.41 per share, compared to $0.29 per share in the same quarter last year. Non-GAAP diluted EPS was $1.26 per share. Non-GAAP diluted EPS was $1.04 per share in the fourth quarter of 2015, reflecting a reduction of $0.09 per share to conform to the current non-GAAP income tax expense definition.
- During the fourth quarter, the Company recognized an impairment charge of $85 million related to SBC-103, an early stage, clinical indefinite-lived intangible asset from the Synageva acquisition. This charge was taken as a result of a strategic evaluation of the asset, increases in the development and commercial timelines, and updated cash flows. In February 2017, Alexion decided to reduce its investment in SBC-103. Patients currently enrolled in the Phase 1/2 trial will continue to receive SBC-103, and no additional Alexion studies are planned. Alexion will reassess the value of this asset on a go forward basis.

Share Repurchase Authorization

The Company also announced that its Board of Directors has increased the size of the Company's share repurchase authorization to a total of $1 billion. The Board's authorization is open-ended.

Product and Pipeline Updates

Complement Portfolio

- **Eculizumab- Refractory Generalized Myasthenia Gravis (gMG):** Alexion has filed regulatory submissions for eculizumab for the treatment of patients with refractory gMG in both the United States and Europe.
- **Eculizumab- Relapsing Neuromyelitis Optica Spectrum Disorder (NMOSD):** Alexion expects to complete enrollment in the PREVENT study, a single, multinational, placebo-controlled Phase 3 trial of eculizumab in patients with relapsing NMOSD, in 2017.
- **Eculizumab- Delayed Graft Function (DGF):** In December 2016, Alexion announced that the PROTECT study, a single, multinational, placebo-controlled trial of eculizumab in the prevention of DGF, did not meet its primary endpoint.
- **ALXN1210- PNH:** Patients are being dosed in a Phase 3 trial comparing ALXN1210 administered intravenously every eight weeks to Soliris in complement inhibitor treatment-naive patients with PNH. To broaden the PNH program, Alexion is also initiating a Phase 3 PNH switch study of ALXN1210 administered intravenously every eight weeks compared to patients currently treated with Soliris. Alexion expects to complete enrollment in both studies in 2017.
- **ALXN1210- aHUS:** Recruitment is underway in a Phase 3 trial with ALXN1210 administered intravenously every eight weeks in complement inhibitor treatment-naive adolescent and adult patients with aHUS. Enrollment is expected to be complete in 2017. Alexion expects to initiate a Phase 3 trial of ALXN1210 in pediatric patients with aHUS in the second quarter of 2017.
- **ALXN1210- Subcutaneous:** Alexion has completed enrollment in a Phase 1 study of a new formulation of ALXN1210 administered subcutaneously in healthy volunteers.

Metabolic Portfolio

- **SBC-103:** In February 2017, Alexion decided to reduce its investment in SBC-103, a recombinant form of the NAGLU enzyme being evaluated in patients with mucopolysaccharidosis IIIB, or MPS IIIB. Patients currently enrolled in the Phase 1/2 study will continue to receive SBC-103, and no additional Alexion studies are planned.
- **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. Alexion has initiated a Phase 1 study of samalizumab in patients with advanced solid tumors. Patients are also being dosed in The Leukemia and Lymphoma Society's BEAT AML Master Trial, a multi-arm clinical trial, which is evaluating samalizumab as well as other potential therapies for the treatment of acute myeloid leukemia (AML).

2017 Financial Guidance

	GAAP Guidance	Non-GAAP Guidance
Total revenues	$3,400 to $3,500 million	$3,400 to $3,500 million
Soliris revenues	$3,025 to $3,100 million	$3,025 to $3,100 million
Metabolic revenues	$375 to $400 million	$375 to $400 million
Research and development expense (% total revenues)	24% to 27%	22% to 23%
Selling, general and administrative expense (% total revenues)	29% to 30%	25% to 26%
Operating margin	25% to 28%	43% to 44%
Earnings per share	$2.55 to $3.05	$5.00 to $5.25

Alexion's 2017 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 the fourth quarter and full year 2016 results, at 10:00 a.m. Eastern Time. To participate in the call, dial 877-681-3372 (USA) or 719-325-4794 (International), passcode 5877612 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 5877612. 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 is the global leader in complement inhibition and has developed and commercializes 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. In addition, Alexion's metabolic franchise includes two highly innovative enzyme replacement therapies for patients with life-threatening and ultra-rare disorders, hypophosphatasia (HPP) and lysosomal acid lipase deficiency (LAL-D). Alexion is advancing its rare disease pipeline 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 2017, assessment of the Company's commercialization efforts 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 the adequacy of our research, 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, 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, the possibility that current rates of adoption of Soliris in PNH, aHUS or other diseases are not sustained, the possibility that clinical trials of our product candidates could be delayed, 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, 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 September 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, impairment of intangible assets 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 2016 Financial Results and GAAP to non-GAAP 2017 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 twelve month periods ended December 31, 2016 and 2015 and projected twelve months ended December 31, 2017.

(Tables Follow)

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

	Three months ended December 31		Twelve months ended December 31	
	2016	2015	2016	2015
Net product sales	$ 831	$ 701	$ 3,082	$ 2,603
Other revenue	-	-	2	1
Total revenues	831	701	3,084	2,604
Cost of sales	68	58	258	233
Operating expenses:				
Research and development	206	191	757	709
Selling, general and administrative	259	242	954	863
Amortization of purchased intangible assets	80	80	322	117
Change in fair value of contingent consideration	5	19	36	64
Acquisition-related costs	-	3	2	39
Restructuring expenses	1	11	3	42
Impairment of intangible assets	85	-	85	-
Total operating expenses	636	546	2,159	1,834
Operating income	127	97	667	537
Other income and expense:				
Investment income	3	1	11	8
Interest expense	(25)	(23)	(97)	(48)
Foreign currency (loss) gain	(1)	(1)	(5)	1
Income before income taxes	104	74	576	498
Income tax expense	11	7	177	354
Net income	$ 93	$ 67	$ 399	$ 144
Earnings per common share				
Basic	$ 0.41	$ 0.30	$ 1.78	$ 0.68
Diluted	$ 0.41	$ 0.29	$ 1.76	$ 0.67

Shares used in computing earnings per common share				
Basic	225	225	224	213
Diluted	226	228	227	216

ALEXION PHARMACEUTICALS, INC.
TABLE 2: RECONCILIATION OF GAAP TO NON-GAAP FINANCIAL RESULTS
(in millions, except per share amounts)
(unaudited)

	Three months ended December 31		Twelve months ended December 31	
	2016	**2015**	**2016**	**2015**
GAAP net income	$ 93	$ 67	$ 399	$ 144
Before tax adjustments:				
Cost of sales:				
Share-based compensation	3	3	11	7
Fair value adjustment of inventory acquired (1)	2	-	11	-
Research and development expense:				
Share-based compensation	14	21	57	64
Upfront and milestone payments related to license and collaboration agreements	6	15	10	130
Selling, general and administrative expense:				
Share-based compensation	25	44	124	156
Amortization of purchased intangible assets (2)	80	80	322	117
Change in fair value of contingent consideration	5	18	36	64
Acquisition-related costs (3)	-	3	2	39
Restructuring expenses	1	12	3	42
Impairment of intangible assets (4)	85	-	85	-
Adjustments to income tax expense (5) (6)	(27)	(23)	(6)	251
Non-GAAP net income	$ 287	$ 240	$ 1,054	$ 1,014
GAAP earnings per share - diluted	$ 0.41	$ 0.29	$ 1.76	$ 0.67
Non-GAAP earnings per share - diluted (6)	$ 1.26	$ 1.04	$ 4.62	$ 4.65
Shares used in computing diluted earnings per share (GAAP)	226	228	227	216
Shares used in computing diluted earnings per share (non-GAAP)	228	230	228	218

(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 and Kanuma.

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

	Three months ended December 31		Twelve months ended December 31	
	2016	**2015**	**2016**	**2015**
Acquisition-related costs:				
Transaction costs	$ -	$ -	$ -	$ 27
Integration costs	-	3	2	12
	$ -	$ 3	$ 2	$ 39

(4) During the fourth quarter of 2016, the Company recognized an impairment charge related to SBC-103, an early stage, clinical indefinite-lived intangible asset related to the Synageva acquisition.

(5) 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.

(6) 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 $1.13 and $4.99 for the three and twelve months ended December 31, 2015, respectively.

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

	Twelve months ended December 31, 2017	
	Low	High
GAAP net income	$ 578	$ 692
Before tax adjustments:		
Share-based compensation	231	207
Fair value adjustment in inventory acquired	5	5
Upfront and milestone payments related to licenses and collaborations	51	—
Amortization of purchased intangible assets	320	320
Change in fair value of contingent consideration	14	14
Adjustments to income tax expense	(54)	(36)
Non-GAAP net income	$ 1,145	$ 1,202
Diluted GAAP earnings per share	$ 2.55	$ 3.05
Diluted Non-GAAP earnings per share	$ 5.00	$ 5.25
Operating expense and margin (% total revenues)		
GAAP research and development expense	27%	24%
Share-based compensation	(3)%	(2)%
Upfront and milestone payments related to licenses and collaborations	(1)%	0%
Non-GAAP research and development expense	23%	22%
GAAP selling, general and administrative expense	30%	29%
Share-based compensation	(4)%	(4)%
Non-GAAP selling, general and administrative expense	26%	25%
GAAP operating margin	25%	28%
Share-based compensation	7%	6%
Fair value adjustment in inventory acquired	0%	0%
Upfront and milestone payments related to licenses and collaborations	2%	0%
Amortization of purchased intangible assets	9%	9%
Change in fair value of contingent consideration	0%	1%
Non-GAAP operating margin	43%	44%

ALEXION PHARMACEUTICALS, INC.
TABLE 4: NET PRODUCT SALES
(in millions)
(unaudited)

	Three months ended	Twelve months ended

	December 31		December 31	
	2016	2015	2016	2015
Soliris	$ 749	$ 689	$ 2,843	$ 2,591
Strensiq	71	12	210	12
Kanuma	11	—	29	—
Total net product sales	$ 831	$ 701	$ 3,082	$ 2,603

ALEXION PHARMACEUTICALS, INC.
TABLE 5: NET PRODUCT SALES BY GEOGRAPHY
(in millions)
(unaudited)

	Three months ended December 31		Twelve months ended December 31	
	2016	2015	2016	2015
United States	$ 354	$ 273	$ 1,257	$ 951
Europe	244	222	961	841
Asia-Pacific	85	73	318	276
Rest of World	148	133	546	535
Total net product sales	$ 831	$ 701	$ 3,082	$ 2,603

ALEXION PHARMACEUTICALS, INC.
TABLE 6: CONDENSED CONSOLIDATED BALANCE SHEETS
(in millions)
(unaudited)

	December 31 2016	December 31 2015
Cash and cash equivalents	$ 966	$ 1,010
Marketable securities	327	375
Trade accounts receivable, net	650	533
Inventories	375	290
Prepaid expenses and other current assets	260	208
Property, plant and equipment, net	1,036	697
Intangible assets, net	4,303	4,708
Goodwill	5,037	5,048
Other assets	299	228
Total assets	$ 13,253	$ 13,097
Accounts payable and accrued expenses	$ 572	$ 460
Deferred revenue	37	21
Current portion of long-term debt	167	166
Other current liabilities	23	6
Current portion of contingent consideration	24	56
Long-term debt, less current portion	2,888	3,254
Facility lease obligation	233	151
Contingent consideration	129	121
Deferred tax liabilities (1)	396	529
Other liabilities	90	74
Total liabilities	4,559	4,838
Total stockholders' equity (1)	8,694	8,259
Total liabilities and stockholders' equity	$ 13,253	$ 13,097

(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, $238 million 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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