October 26, 2017

Alexion Reports Third Quarter 2017 Results

- 3Q17 Total Revenues of $859 Million, an 8 Percent Increase Year-Over-Year
- 3Q17 GAAP EPS of $0.35 Per Share, a 17 Percent Decrease Year-Over-Year, and Non-GAAP EPS of $1.44 Per Share, a 17 Percent Increase Year-Over-Year
- Received FDA Approval for Soliris for the Treatment of Patients with Generalized Myasthenia Gravis (gMG) and European Commission Approval for Soliris for the Treatment of Patients with Refractory gMG
- Completed Enrollment in the Phase 3 Trial of Eculizumab in Patients with Neuromyelitis Optica Spectrum Disorder
- Completed Enrollment in the ALXN1210 Phase 3 PNH Naive and Switch Studies
- Announces Plans to Initiate a Single, PK-based Phase 3 Study of ALXN1210 Delivered Subcutaneously Once Per Week to Support Registration in PNH and aHUS
- Provides Updated 2017 Guidance

NEW HAVEN, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ: ALXN) today announced financial results for the third quarter of 2017. Total revenues in the quarter were $859 million, an 8 percent increase compared to the same period in 2016. Second quarter revenue benefited from approximately $35 million in favorable timing of orders, which directly impacted quarter-over-quarter and year-over-year growth in the third quarter of 2017. The negative impact of foreign currency on total revenue year-over-year was less than 1 percent, or 5 million, net of hedging activities. On a GAAP basis, diluted earnings per share (EPS) in the quarter was $0.35 per share, inclusive of $164 million of expenses related to the previously announced restructuring, compared to $0.42 per share in the third quarter of 2016. Non-GAAP diluted EPS for the third quarter of 2017 was $1.44 per share, compared to $1.23 per share in the third quarter of 2016.

"Alexion delivered strong commercial, R&D, and financial performance in the third quarter of 2017. We received regulatory approvals for the third indication for Soliris in the U.S. and European Union, strengthened our patent portfolio with three new U.S. patents for Soliris that extend protection into 2027, reached new funding agreements in key European countries for Strensiq and Kanuma, and advanced the ALXN1210 clinical development programs," said Ludwig Hantson, Chief Executive Officer of Alexion. "We also announced a restructuring that aligns the global organization with our strategy and we expect to deliver significant savings. We enter the fourth quarter of 2017 in a position of strength and are confident in our strategic plan to build long-term sustainable value for shareholders."

Third Quarter 2017 Financial Highlights

- Soliris® (eculizumab) net product sales were $756 million, compared to $728 million in the third quarter of 2016, representing a 4 percent increase. Soliris volume increased 3 percent year-over-year.
- Strensiq® (asfotase alfa) net product sales were $87 million, compared to $61 million in the third quarter of 2016, representing a 44 percent increase.
- Kanuma® (sebelipase alfa) net product sales were $16 million, compared to $9 million in the third quarter of 2016, representing a 79 percent increase.
- GAAP cost of sales was $157 million, inclusive of restructuring related expenses of $83 million, compared to $71 million in the same quarter last year. Non-GAAP cost of sales was $71 million, compared to $62 million in the same quarter last year.
- GAAP R&D expense was $195 million, inclusive of restructuring related expenses of $1 million, compared to $195 million in the same quarter last year. Non-GAAP R&D expense was $175 million, compared to $179 million in the same quarter last year.
- GAAP SG&A expense was $271 million, inclusive of restructuring related expenses of $6 million, compared to $230 million in the same quarter last year. Non-GAAP SG&A expense was $230 million, compared to $201 million in the same quarter last year.
- GAAP income tax benefit was $20 million, compared to income tax expense of $65 million in the same quarter last

year. Non-GAAP income tax expense was $35 million, compared to $56 million in the same quarter last year. Both GAAP and non-GAAP income tax expense for the third quarter of 2017 includes a benefit from the conclusion of a routine IRS audit for the 2013-2014 years.

- GAAP diluted EPS was $0.35 per share, inclusive of restructuring and related expenses of $164 million, compared to $0.42 per share in the same quarter last year. Non-GAAP diluted EPS was $1.44 per share, compared to $1.23 per share in the third quarter of 2016.

Research and Development

Complement Portfolio Updates

- **Soliris® (eculizumab)- Generalized Myasthenia Gravis (gMG):** The U.S. Food and Drug Administration (FDA) approved Soliris as a treatment for adult patients with generalized myasthenia gravis (gMG) who are anti-acetylcholine receptor (AchR) antibody-positive. The European Commission approved the extension of the indication for Soliris to include the treatment of refractory gMG in adults who are AchR antibody-positive. Alexion has submitted an application in Japan to extend the indication for Soliris as a potential treatment for patients with refractory gMG who are AchR antibody-positive.
- **Eculizumab- Relapsing Neuromyelitis Optica Spectrum Disorder (NMOSD):** Alexion completed enrollment in the PREVENT study, a single, multinational, placebo-controlled Phase 3 trial of eculizumab in patients with relapsing neuromyelitis optica spectrum disorder (NMOSD), and expects to report data in mid-2018.
- **ALXN1210- PNH:** Enrollment is complete in a Phase 3 trial comparing ALXN1210 administered intravenously every eight weeks to Soliris in complement inhibitor treatment-naive patients with PNH and in a Phase 3 PNH Switch study of ALXN1210 administered intravenously every eight weeks compared to patients currently treated with Soliris. Alexion expects to report data from these studies in the second quarter of 2018.
- **ALXN1210- aHUS:** Enrollment and dosing are ongoing 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 early 2018. Alexion also initiated enrollment in a Phase 3 trial of ALXN1210 in pediatric patients with aHUS.
- **ALXN1210- Subcutaneous:** Initial pharmacokinetic and tolerability data from the Phase I study in healthy volunteers support progressing the development of a subcutaneous formulation of ALXN1210. Based on discussions with regulators, Alexion plans to initiate a single, PK-based Phase 3 study of ALXN1210 delivered subcutaneously once per week to support registration in PNH and aHUS in late 2018.

Manufacturing

Alexion received a notification from the FDA that the Warning Letter received in March 2013 regarding compliance with Good Manufacturing Practices at the Company's Rhode Island manufacturing facility has been resolved. As previously announced, Alexion plans to close the Rhode Island manufacturing facility to align its manufacturing facilities with its ongoing multi-product network manufacturing strategy, which utilizes Alexion's manufacturing operations in the U.S. and Ireland, and manufacturing capacity through its manufacturing partners.

2017 Financial Guidance

Alexion is increasing the low-end of its revenue guidance, lowering its GAAP EPS guidance and increasing its non-GAAP EPS guidance. Full guidance updates are outlined below.

	Previous	**Updated**
Total revenues	$3,450 to $3,525 million	$3,475 to $3,525 million
Soliris revenues	$3,075 to $3,125 million	$3,090 to $3,125 million
Metabolic revenues	$375 to $400 million	$385 to $400 million
R&D (% total revenues)		
GAAP	23% to 25%	23% to 25%
Non-GAAP	21% to 22%	21% to 22%
SG&A (% total revenues)		
GAAP	29% to 30%	30% to 32%
Non-GAAP	25% to 26%	26% to 27%
Operating margin		
GAAP	23% to 26%	16% to 19%

Non-GAAP	43% to 44%	43% to 44%
Earnings per share		
GAAP	$2.82 to $3.12	$2.00 to $2.35
Non-GAAP	$5.40 to $5.55	$5.50 to $5.65

Updated 2017 financial guidance assumes the following:

- Foreign currency headwinds of $30 to $40 million versus prior assumption of $40 to $50 million
- Soliris revenue impact of $80 to $90 million from ALXN1210 and other clinical trial recruitments versus prior assumption of $70 to $100 million

Alexion's 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 and related activity outside of the previously announced plan 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 third quarter 2017 results, at 10:00 a.m. Eastern Time. To participate in the call, dial 877-723-9522 (USA) or 719-457-2615 (International), passcode 3433245 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 3433245. The audio webcast can be accessed on the Investor page of Alexion's website at: http://ir.alexion.com.

About Alexion

Alexion is a global biopharmaceutical company focused on serving patients and families affected by rare diseases through the innovation, development and commercialization of life-changing therapies. 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), atypical hemolytic uremic syndrome (aHUS), and anti-acetylcholine receptor (AchR) antibody-positive generalized myasthenia gravis (gMG). In addition, Alexion has two highly innovative enzyme replacement therapies for patients with life-threatening and ultra-rare metabolic disorders, hypophosphatasia (HPP) and lysosomal acid lipase deficiency (LAL-D). As the leader in complement biology for over 20 years, Alexion focuses its research efforts on novel molecules and targets in the complement cascade, and its development efforts on the core therapeutic areas of hematology, nephrology, neurology, and metabolic disorders. 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, anticipated savings, costs and expenses resulting from the restructuring, 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, Kanuma and Soliris for the treatment of gMG, and plans for regulatory filings and clinical programs for our product candidates, and anticipated changes to the Company's R&D strategy. 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, uncertainties surrounding legal proceedings, company investigations and government investigations, including investigations of Alexion by the U.S. Securities and Exchange Commission (SEC) and U.S. Department of Justice, the risk that anticipated regulatory filings are delayed, the risk that estimates regarding the number of patients with PNH, aHUS, gMG, HPP and LAL-D are inaccurate, the risks of changing foreign exchange rates, risks relating to the potential effects of the Company's restructuring and relocation of its corporate headquarters, and a variety of other risks set forth from time to time in Alexion's filings with the SEC, including but not limited to the risks discussed in Alexion's Quarterly Report on Form 10-Q for the period ended June 30, 2017 and in our other filings with the SEC. 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 and related expenses, upfront and milestone payments related to licenses and collaborations, impairment of intangible assets and certain 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 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 nine month periods ended September 30, 2017 and 2016 and projected twelve months ended December 31, 2017.

Reported values for the three and nine months ended 2016 have been rounded to millions to match 2017 presentation.

(Tables Follow)

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

	Three months ended September 30		Nine months ended September 30	
	2017	2016[1]	2017	2016[1]
Net product sales	$ 859	$ 798	$ 2,640	$ 2,251
Other revenue	—	1	1	2
Total revenues	859	799	2,641	2,253
Cost of sales	157	71	310	190
Operating expenses:				
Research and development	195	195	613	551
Selling, general and administrative	271	230	798	695
Amortization of purchased intangible assets	80	82	240	242
Change in fair value of contingent consideration	4	41	32	31
Acquisition-related costs	—	—	—	2
Restructuring expenses	72	1	99	2
Impairment of intangible assets	—	—	31	—
Total operating expenses	622	549	1,813	1,523
Operating income	80	179	518	540
Other income and expense:				
Investment income	5	5	13	8
Interest expense	(25)	(24)	(73)	(72)
Other expense	(2)	(1)	—	(4)
Income before income taxes	58	159	458	472
Income tax (benefit) expense	(20)	65	45	166
Net income	$ 78	$ 94	$ 413	$ 306
Earnings per common share				
Basic	$ 0.35	$ 0.42	$ 1.84	$ 1.37
Diluted	$ 0.35	$ 0.42	$ 1.83	$ 1.35

Shares used in computing earnings per common share				
Basic	223	224	224	224
Diluted	225	226	226	226

(1) Reported values for the three and nine months ended 2016 have been rounded to millions to match 2017 presentation.

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

	Three months ended September 30		Nine months ended September 30	
	2017	2016[4]	2017	2016[4]
GAAP net income	$ 78	$ 94	$ 413	$ 306
Before tax adjustments:				
Cost of sales:				
Share-based compensation	3	3	8	8
Fair value adjustment in inventory acquired	—	6	5	8
Restructuring related expenses (1)	83	—	83	—
Research and development expense:				
Share-based compensation	19	14	55	44
Upfront and milestone payments related to licenses and collaborations	—	2	9	5
Restructuring related expenses (1)	1	—	1	—
Selling, general and administrative expense:				
Share-based compensation	35	29	109	99
Restructuring related expenses (1)	6	—	6	—
Amortization of purchased intangible assets	80	82	240	242
Change in fair value of contingent consideration	4	41	32	31
Acquisition-related costs	—	—	—	2
Restructuring expenses (1)	72	1	99	2
Impairment of intangible assets (2)	—	—	31	—
Other income and expense:				
Restructuring related expenses (1)	2	—	2	—
Adjustments to income tax expense (3)	(55)	9	(94)	19
Non-GAAP net income	$ 328	$ 281	$ 999	$ 766
GAAP earnings per common share - diluted	$ 0.35	$ 0.42	$ 1.83	$ 1.35
Non-GAAP earnings per common share - diluted	$ 1.44	$ 1.23	$ 4.38	$ 3.36
Shares used in computing diluted earnings per common share (GAAP)	225	226	226	226
Shares used in computing diluted earnings per common share (non-GAAP)	228	228	228	228

(1) The following table summarizes the total restructuring and related expenses recorded by type of activity and the classification within the Reconciliation of GAAP to non-GAAP Financial Results:

	Three months ended September 30, 2017				Nine months ended September 30, 2017			
	Employee Separation Costs	Asset-Related Charges	Other	Total	Employee Separation Costs	Asset-Related Charges	Other	Total
Cost of sales	$ —	$ 83	$ —	$ 83	$ —	$ 83	$ —	$ 83

Research and development	—	1	—	1	—	1	—	1
Selling, general and administrative	—	6	—	6	—	6	—	6
Restructuring expenses	66	—	6	72	87	—	12	99
Other expense	—	—	2	2	—	—	2	2
	$ 66	$ 90	$ 8	$164	$ 87	$ 90	$ 14	$191

(2) In the second quarter 2017, we recognized an impairment charge of $31 million, which fully impaired SBC-103, the acquired in-process research and development asset, due to clinical trial results.

(3) 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.

(4) Reported values for the three and nine months ended 2016 have been rounded to millions to match 2017 presentation.

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	$ 451	$ 530
Before tax adjustments:		
Share-based compensation	241	218
Fair value adjustment in inventory acquired	5	5
Upfront and milestone payments related to licenses and collaborations	10	9
Amortization of purchased intangible assets	320	320
Change in fair value of contingent consideration	36	36
Restructuring and related expenses	286	256
Impairment of intangible assets	31	31
Adjustments to income tax expense	(126)	(117)
Non-GAAP net income	$ 1,254	$ 1,288
Diluted GAAP earnings per common share	$ 2.00	$ 2.35
Diluted non-GAAP earnings per common share	$ 5.50	$ 5.65
Operating expense and margin (% total revenues)		
GAAP research and development expense	25%	23%
Share-based compensation	(2)%	(2)%
Upfront and milestone payments related to licenses and collaborations	(1)%	0%
Restructuring related expenses	0%	0%
Non-GAAP research and development expense	22%	21%
GAAP selling, general and administrative expense	32%	30%
Share-based compensation	(4)%	(4)%
Restructuring related expenses	(1)%	0%
Non-GAAP selling, general and administrative expense	27%	26%
GAAP operating margin	16%	19%
Share-based compensation	7%	6%

Fair value adjustment in inventory acquired	0%	0%
Upfront and milestone payments related to licenses and collaborations	1%	1%
Amortization of purchased intangible assets	9%	9%
Change in fair value of contingent consideration	1%	1%
Restructuring and related expenses	8%	7%
Impairment of intangible assets	1%	1%
Non-GAAP operating margin	43%	44%

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

	Three months ended September 30		Nine months ended September 30	
	2017	2016[1]	2017	2016[1]
Soliris				
United States	$ 308	$ 275	$ 914	$ 773
Europe	248	240	738	703
Asia Pacific	82	80	241	223
Rest of World	118	133	459	395
Total Soliris	$ 756	$ 728	$ 2,352	$ 2,094
Strensiq				
United States	$ 71	$ 51	$ 204	$ 118
Europe	9	6	23	10
Asia Pacific	5	3	13	9
Rest of World	2	1	4	2
Total Strensiq	$ 87	$ 61	$ 244	$ 139
Kanuma				
United States	$ 11	$ 6	$ 31	$ 12
Europe	4	2	9	5
Asia Pacific	1	1	2	1
Rest of World	—	—	2	—
Total Kanuma	$ 16	$ 9	$ 44	$ 18
Net Product Sales				
United States	$ 390	$ 332	$ 1,149	$ 903
Europe	261	248	770	718
Asia Pacific	88	84	256	233
Rest of World	120	134	465	397
Total Net Product Sales	$ 859	$ 798	$ 2,640	$ 2,251

[1] Reported values for the three and nine months ended 2016 have been rounded to millions to match 2017 presentation.

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

	September 30 2017	December 31 2016
Cash and cash equivalents	$ 550	$ 966
Marketable securities	979	327
Trade accounts receivable, net	705	650
Inventories	441	375

Prepaid expenses and other current assets (1)	207	260
Property, plant and equipment, net	1,354	1,036
Intangible assets, net	4,032	4,303
Goodwill	5,037	5,037
Other assets	338	299
Total assets	$ 13,643	$ 13,253
Accounts payable and accrued expenses	$ 738	$ 572
Deferred revenue	18	37
Current portion of long-term debt	167	167
Current portion of contingent consideration	—	24
Other current liabilities	49	23
Long-term debt, less current portion	2,763	2,888
Contingent consideration	160	129
Facility lease obligation	333	233
Deferred tax liabilities	353	396
Other liabilities	118	90
Total liabilities	4,699	4,559
Total stockholders' equity (1)	8,944	8,694
Total liabilities and stockholders' equity	$ 13,643	$ 13,253

(1) In October 2016, the FASB issued a new income tax standard that eliminates the exception for an intra-entity asset transfer other than inventory. We elected to early adopt this standard in the first quarter 2017. As a result of the adoption, we recorded a $19 million decrease in retained earnings, primarily resulting from the elimination of previously recorded prepaid tax assets.

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