April 27, 2017

Alexion Reports First Quarter 2017 Results

- 1Q17 Total Revenues of $870 Million, a 24% Increase and 26% Volume Increase Year-Over-Year
- Revenue Growth Benefited from Continued Strong Volume Growth of Soliris® In Core Markets
- Strensiq® Revenue Driven by Steady Addition of New Patients Starting Treatment
- Applications Filed in U.S., Europe and Japan for Soliris in Patients with Refractory gMG
- Enrollment in ALXN1210 Phase 3 Studies Advanced in Patients with PNH and aHUS
- New Phase 1 Data Support Advancing Development of ALXN1210 Subcutaneous Formulation
- Reiterating 2017 Revenue Guidance of $3.4 to $3.5 Billion and Increasing GAAP EPS Guidance to $2.80 to $3.20 per share and Non-GAAP EPS Guidance to $5.10 to $5.30 per share

NEW HAVEN, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ: ALXN) today announced financial results for the first quarter of 2017. Total revenues in the quarter were $870 million, a 24 percent increase compared to the same period in 2016. First quarter revenues included a benefit of $29 million from a change in revenue recognition in 2017 for certain non-U.S. markets; excluding the benefit of this accounting change, revenues increased to $841 million, a 20 percent increase compared to the same period in 2016. The negative impact of foreign currency on total revenue year-over-year was 2 percent or $12 million, net of hedging activities. On a GAAP basis, diluted earnings per share (EPS) in the quarter was $0.75 per share, compared to $0.41 per share in the first quarter of 2016. Non-GAAP diluted EPS for the first quarter of 2017 was $1.38 per share. Non-GAAP diluted EPS was $0.99 per share in the first quarter of 2016, including a reduction of $0.12 per share to conform to the current non-GAAP income tax expense definition.

"We delivered continued double-digit revenue growth in the quarter from our complement and metabolic portfolios and achieved important regulatory milestones towards the potential approval of Soliris as a treatment for patients with refractory gMG in the U.S., Europe and Japan. We also demonstrated strong commercial execution for Soliris while simultaneously enrolling patients with PNH and aHUS into the ALXN1210 Phase 3 trials," said Ludwig Hantson, Chief Executive Officer of Alexion. "Alexion's strong performance in the first quarter positions us well for continued success in 2017 and beyond. As we continue to grow our business, we will be anchored by a culture of compliance and driven by passion and dedication to patients. I am excited to work with our team to capitalize on Alexion's fundamentals to drive continued momentum and long-term growth by enhancing our commercial, R&D and capital allocation priorities to drive superior financial performance and shareholder returns."

First Quarter 2017 Financial Highlights

- Soliris® (eculizumab) net product sales were $783 million, including a benefit of $29 million from a change in revenue recognition in 2017 for certain non-U.S. markets, compared to $665 million in the first quarter of 2016. Starting in the first quarter of 2017, Alexion is recording revenue shipped to certain distributors earlier as the Company has sufficient sales data to estimate returns. The $29 million benefit is composed of approximately $21 million from bulk orders in Latin America and the remaining $8 million from deferred sales in other non-U.S. markets.
- Strensiq® (asfotase alfa) net product sales were $74 million, compared to $33 million in the first quarter of 2016.
- Kanuma® (sebelipase alfa) net product sales were $12 million, compared to $2 million in the first quarter of 2016.
- GAAP R&D expense was $219 million, compared to $176 million in the same quarter last year. Non-GAAP R&D expense was $194 million, compared to $158 million in the same quarter last year.
- GAAP SG&A expense was $262 million, compared to $233 million in the same quarter last year. Non-GAAP SG&A expense was $226 million, compared to $194 million in the same quarter last year.
- GAAP diluted EPS was $0.75 per share, compared to $0.41 per share in the same quarter last year. Non-GAAP diluted EPS was $1.38 per share. Non-GAAP diluted EPS was $0.99 per share in the first quarter of 2016, reflecting a reduction of $0.12 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 has submitted applications in the U.S., EU and Japan to extend the indication for eculizumab as a potential treatment for patients with refractory gMG who are AChR-positive. The applications have been accepted for review by the U.S. Food and Drug Administration (FDA) and validated by the European Medicines Agency (EMA).
- **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.
- **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. In the second quarter of 2017, Alexion plans to initiate a Phase 3 PNH Switch study of ALXN1210 administered intravenously every eight weeks compared to patients currently treated with Soliris. The Company expects to complete enrollment in both studies in 2017.
- **ALXN1210- aHUS:** Patients are being dosed 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. Initial pharmacokinetic and tolerability data from the Phase I study support progressing the development of this subcutaneous formulation of ALXN1210.

Metabolic Portfolio

- **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):** Alexion has initiated a Phase 1 study of samalizumab, a first-in-class immunomodulatory humanized monoclonal antibody that blocks the key immune checkpoint protein, CD200, 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

Alexion is reiterating its 2017 revenue and operating margin guidance provided on the fourth quarter and full year 2016 earnings call and increasing its GAAP and non-GAAP EPS guidance.

	Updated GAAP Guidance	Prior GAAP Guidance	**Updated Non-GAAP Guidance**	Prior Non-GAAP Guidance
Total revenues	$3,400 to $3,500 million	$3,400 to $3,500 million	$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	$3,025 to $3,100 million	$3,025 to $3,100 million
Metabolic revenues	$375 to $400 million	$375 to $400 million	$375 to $400 million	$375 to $400 million
R&D (% total revenues)	24% to 26%	24% to 27%	22% to 23%	22% to 23%
SG&A (% total revenues)	28% to 30%	29% to 30%	25% to 26%	25% to 26%
Operating margin	25% to 28%	25% to 28%	43% to 44%	43% to 44%
Earnings per share	$2.80 to $3.20	$2.55 to $3.05	$5.10 to $5.30	$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 first quarter 2017 results, at 10:00 a.m. Eastern Time. To participate in the call, dial 888-329-8877 (USA) or 719-457-2648 (International), passcode 6536169 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 6536169. 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 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, HPP and LAL-D are inaccurate, the risks of changing foreign exchange rates, 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 Annual Report on Form 10-K for the period ended December 31, 2016 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 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 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 month periods ended March 31, 2017 and 2016 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 March 31

	2017	2016
Net product sales	$ 869	$ 700
Other revenue	1	1
Total revenues	870	701
Cost of sales	69	59
Operating expenses:		
Research and development	219	176
Selling, general and administrative	262	233
Amortization of purchased intangible assets	80	80
Change in fair value of contingent consideration	4	(15)
Acquisition-related costs	—	1
Restructuring expenses	24	1
Total operating expenses	589	476
Operating income	212	166
Other income and expense:		
Investment income	4	1
Interest expense	(24)	(24)
Other income	2	—
Income before income taxes	194	143
Income tax expense	24	51
Net income	$ 170	$ 92
Earnings per common share		
Basic	$ 0.76	$ 0.41
Diluted	$ 0.75	$ 0.41
Shares used in computing earnings per common share		
Basic	224	225
Diluted	226	227

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

	Three months ended March 31	
	2017	2016
GAAP net income	$ 170	$ 92
Before tax adjustments:		
Cost of sales:		
Share-based compensation	2	3
Fair value adjustment in inventory acquired (1)	2	1
Research and development expense:		
Share-based compensation	16	15
Upfront and milestone payments related to licenses and collaborations	9	3
Selling, general and administrative expense:		
Share-based compensation	36	38
Amortization of purchased intangible assets	80	80
Change in fair value of contingent consideration	4	(15)
Acquisition-related costs	—	1
Restructuring expenses (2)	24	1

Adjustments to income tax expense (3) (4)	(27)	8
Non-GAAP net income	$ 316	$ 227
GAAP earnings per common share - diluted	$ 0.75	$ 0.41
Non-GAAP earnings per common share - diluted (4)	$ 1.38	$ 0.99
Shares used in computing diluted earnings per common share (GAAP)	226	227
Shares used in computing diluted earnings per common share (non-GAAP)	229	229

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

(2) Restructuring expenses of $24 million are related to the company-wide restructuring initiated in the first quarter 2017.

(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) Previously reported non-GAAP tax expense and diluted EPS have been modified to conform to the current non-GAAP income tax expense definition adopted in Q2 2016. Previously reported non-GAAP EPS was $1.11 for the three months ended March 31, 2016.

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	$ 635	$ 726
Before tax adjustments:		
Share-based compensation	230	197
Fair value adjustment in inventory acquired	5	5
Upfront and milestone payments related to licenses and collaborations	16	9
Amortization of purchased intangible assets	320	320
Change in fair value of contingent consideration	14	14
Restructuring expenses	34	24
Adjustments to income tax expense	(86)	(81)
Non-GAAP net income	$ 1,168	$ 1,214
Diluted GAAP earnings per share	$ 2.80	$ 3.20
Diluted Non-GAAP earnings per share	$ 5.10	$ 5.30
Operating expense and margin (% total revenues)		
GAAP research and development expense	26%	24%
Share-based compensation	(2)%	(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%	28%
Share-based compensation	(4)%	(3)%
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	1%	0%
Amortization of purchased intangible assets	9%	9%
Change in fair value of contingent consideration	0%	0%
Restructuring expenses	1%	1%
Non-GAAP operating margin	43%	44%

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

	Three months ended March 31	
	2017	**2016**
Soliris	$ 783	$ 665
Strensiq	74	33
Kanuma	12	2
Total net product sales	$ 869	$ 700

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

	Three months ended March 31	
	2017	**2016**
United States	$ 360	$ 265
Europe	248	227
Asia-Pacific	83	72
Rest of World	178	136
Total net product sales	$ 869	$ 700

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

	March 31 2017	**December 31 2016**
Cash and cash equivalents	$ 713	$ 966
Marketable securities	749	327
Trade accounts receivable, net	660	650
Inventories	396	375
Prepaid expenses and other current assets (1)	215	260
Property, plant and equipment, net	1,138	1,036
Intangible assets, net	4,223	4,303
Goodwill	5,037	5,037
Other assets	304	299
Total assets	$ 13,435	$ 13,253
Accounts payable and accrued expenses	$ 607	$ 572
Deferred revenue	16	37
Current portion of long-term debt	167	167
Current portion of contingent consideration	25	24

Other current liabilities	22	23
Long-term debt, less current portion	2,846	2,888
Contingent consideration	132	129
Facility lease obligation	270	233
Deferred tax liabilities	384	396
Other liabilities	110	90
Total liabilities	4,579	4,559
Total stockholders' equity (1)	8,856	8,694
Total liabilities and stockholders' equity	$ 13,435	$ 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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