



July 27, 2017

Alexion Reports Second Quarter 2017 Results

- | 2Q17 Total Revenues of \$912 Million, a 21 Percent Increase Over 2Q16
- | 2Q17 GAAP EPS of \$0.73 Per Share, a 38 Percent Increase Year-Over-Year and Non-GAAP EPS of \$1.56 Per Share, a 38 Percent Increase Year-Over-Year
- | Positive CHMP Opinion Received for Soliris in Patients with Refractory gMG in the EU
- | Completed Enrollment in the ALXN1210 Phase 3 PNH Study in Complement Inhibitor Treatment-Naive Patients
- | Increasing 2017 Revenue Guidance to \$3.450 to \$3.525 Billion; Narrowing GAAP EPS Guidance to \$2.82 to \$3.12 Per Share and Increasing Non-GAAP EPS Guidance to \$5.40 to \$5.55 Per Share
- | Alexion to Provide Strategy Update on Conference Call

NEW HAVEN, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ: ALXN) today announced financial results for the second quarter of 2017. Total revenues in the quarter were \$912 million, a 21 percent increase compared to the same period in 2016. Second quarter revenue reflected a benefit of approximately \$35 million due to favorable timing of orders compared to our prior forecast. 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 \$0.73 per share, compared to \$0.53 per share in the second quarter of 2016. Non-GAAP diluted EPS for the second quarter of 2017 was \$1.56 per share, compared to \$1.13 per share in the second quarter of 2016.

"Alexion delivered strong performance in the second quarter of 2017 while also executing on several initiatives to position the company for the future, including strengthening the Soliris patent portfolio, reaching a funding agreement for Strensiq in England, advancing the late-stage pipeline, enhancing compliance and culture and building a strong leadership team," said Ludwig Hantson, Chief Executive Officer of Alexion. "Our strategy for the next phase of growth will focus on our strengths to deliver sustainable long-term performance and increased value for shareholders. We will achieve this by growing our rare disease business, leveraging our expertise in complement, pursuing disciplined business development to expand the pipeline, and taking steps to optimize our infrastructure and operating model."

Second Quarter 2017 Financial Highlights

- | Soliris[®] (eculizumab) net product sales were \$814 million, compared to \$701 million in the second quarter of 2016, representing a 16 percent increase. Soliris volume increased 18 percent year-over-year.
- | Strensiq[®] (asfotase alfa) net product sales were \$83 million, compared to \$45 million in the second quarter of 2016, representing an 84 percent increase.
- | Kanuma[®] (sebelipase alfa) net product sales were \$15 million, compared to \$7 million in the second quarter of 2016, representing a 114 percent increase.
- | GAAP R&D expense was \$199 million, compared to \$180 million in the same quarter last year. Non-GAAP R&D expense was \$179 million, compared to \$165 million in the same quarter last year.
- | GAAP SG&A expense was \$265 million, compared to \$232 million in the same quarter last year. Non-GAAP SG&A expense was \$227 million, compared to \$200 million in the same quarter last year.
- | GAAP diluted EPS was \$0.73 per share, compared to \$0.53 per share in the same quarter last year. Non-GAAP diluted EPS was \$1.56 per share, compared to \$1.13 per share in the second quarter of 2016.

Research and Development

Alexion is redefining the Company's Research & Development (R&D) strategy to create greater efficiency and focus on its expertise in complement biology and core therapeutic areas of hematology, nephrology, neurology, and metabolic disorders. To optimize and align R&D investments and development efforts with the Company's redefined strategy, Alexion is de-prioritizing the ALXN1101 (cPMP replacement therapy) and ALXN6000 (somalizumab) clinical development programs and will seek to out-license these assets. Alexion is also discontinuing its preclinical programs with mRNA therapies as well as other preclinical programs that are outside of the complement franchise, and is therefore terminating its partnerships with

Moderna Therapeutics, Blueprint Medicines and Arbutus Biopharma.

Complement Portfolio Updates

- 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. Alexion received a positive opinion from the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) in the second quarter and a final decision from the European Commission (EC) is anticipated in the third quarter of 2017. Alexion's application in the U.S. has been accepted for review by the U.S. Food and Drug Administration (FDA) and the FDA has set a Prescription Drug User Fee Act (PDUFA) date of October 23, 2017.
- 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 and to report data in 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. Alexion expects to report data from this study in the second quarter of 2018. Alexion initiated a Phase 3 PNH Switch study of ALXN1210 administered intravenously every eight weeks compared to patients currently treated with Soliris in the second quarter of 2017. The Company expects to complete enrollment in this study in the third quarter of 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 early 2018. Alexion expects to initiate a Phase 3 trial of ALXN1210 in pediatric patients with aHUS in the third quarter of 2017.
- 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.

2017 Financial Guidance

Alexion is increasing its revenue guidance, narrowing its GAAP EPS guidance and increasing its non-GAAP EPS guidance. Further guidance updates are outlined below.

	Updated GAAP Guidance	Prior GAAP Guidance	Updated Non-GAAP Guidance	Prior Non-GAAP Guidance
Total revenues	\$3,450 to \$3,525 million	\$3,400 to \$3,500 million	\$3,450 to \$3,525 million	\$3,400 to \$3,500 million
Soliris revenues	\$3,075 to \$3,125 million	\$3,025 to \$3,100 million	\$3,075 to \$3,125 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)	23% to 25%	24% to 26%	21% to 22%	22% to 23%
SG&A (% total revenues)	29% to 30%	28% to 30%	25% to 26%	25% to 26%
Operating margin	23% to 26%	25% to 28%	43% to 44%	43% to 44%
Earnings per share	\$2.82 to \$3.12	\$2.80 to \$3.20	\$5.40 to \$5.55	\$5.10 to \$5.30

Updated 2017 financial guidance assumes the following:

- Foreign currency headwinds of \$40 to \$50 million versus prior assumption of \$50 to \$60 million
- Soliris revenue impact of \$70 to \$100 million from ALXN1210 and other clinical trial recruitments versus prior assumption of \$70 to \$110 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 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 second quarter 2017 results, at 10:00 a.m. Eastern Time.

To participate in the call, dial 877-852-6543 (USA) or 719-325-4789 (International), passcode 2566061 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 2566061. 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 commercialized 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.

[ALXN-E]

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 candidate, 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, 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 Quarterly Report on Form 10-Q for the period ended March 31, 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 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 and six month periods ended June 30, 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	Six months ended
	June 30	June 30

	<u>2017</u>	<u>2016</u>	<u>2017</u>	<u>2016</u>
Net product sales	\$ 912	\$ 753	\$ 1,781	\$ 1,453
Other revenue	—	—	1	1
Total revenues	<u>912</u>	<u>753</u>	<u>1,782</u>	<u>1,454</u>
Cost of sales	84	60	153	119
Operating expenses:				
Research and development	199	180	418	356
Selling, general and administrative	265	232	527	465
Amortization of purchased intangible assets	80	80	160	160
Change in fair value of contingent consideration	24	5	28	(10)
Acquisition-related costs	—	1	—	2
Restructuring expenses	3	—	27	1
Impairment of intangible assets	31	—	31	—
Total operating expenses	<u>602</u>	<u>498</u>	<u>1,191</u>	<u>974</u>
Operating income	226	195	438	361
Other income and expense:				
Investment income	4	2	8	3
Interest expense	(24)	(24)	(48)	(48)
Other income (expense)	—	(3)	2	(3)
Income before income taxes	206	170	400	313
Income tax expense (1)	41	50	65	101
Net income	<u>\$ 165</u>	<u>\$ 120</u>	<u>\$ 335</u>	<u>\$ 212</u>
Earnings per common share				
Basic	\$ 0.74	\$ 0.54	\$ 1.49	\$ 0.94
Diluted	\$ 0.73	\$ 0.53	\$ 1.49	\$ 0.94
Shares used in computing earnings per common share				
Basic	224	224	225	225
Diluted	225	226	225	226

(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 in the third quarter of 2016. The standard requires restatement of previously reported results in the year following adoption, as if the new standard was adopted effective January 1, 2016, and accordingly, we have reflected additional tax benefits of \$5 for the three and six months ended June 30, 2016 in our consolidated statement of operations.

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

	<u>Three months ended</u>		<u>Six months ended</u>	
	<u>June 30</u>		<u>June 30</u>	
	<u>2017</u>	<u>2016</u>	<u>2017</u>	<u>2016</u>
GAAP net income	\$ 165	\$ 120	\$ 335	\$ 212
Before tax adjustments:				
Cost of sales:				
Share-based compensation	3	2	5	5
Fair value adjustment in inventory acquired	3	1	5	2
Research and development expense:				
Share-based compensation	20	15	36	30

Upfront and milestone payments related to licenses and collaborations	—	—	9	3
Selling, general and administrative expense:				
Share-based compensation	38	32	74	70
Amortization of purchased intangible assets	80	80	160	160
Change in fair value of contingent consideration	24	5	28	(10)
Acquisition-related costs	—	1	—	2
Restructuring expenses (1)	3	—	27	1
Impairment of intangible assets (2)	31	—	31	—
Adjustments to income tax expense	(12)	2	(39)	10
Non-GAAP net income	<u>\$ 355</u>	<u>\$ 258</u>	<u>\$ 671</u>	<u>\$ 485</u>
GAAP earnings per common share - diluted	\$ 0.73	\$ 0.53	\$ 1.49	\$ 0.94
Non-GAAP earnings per common share - diluted	\$ 1.56	\$ 1.13	\$ 2.94	\$ 2.12
Shares used in computing diluted earnings per common share (GAAP)	225	226	225	226
Shares used in computing diluted earnings per common share (non-GAAP)	228	228	228	229

(1) Restructuring expenses were \$3 million and \$27 million for the three and six months ended June 30, 2017, respectively, related to the company-wide restructuring initiated in the first quarter 2017.

(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.

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	
	<u>Low</u>	<u>High</u>
GAAP net income	\$ 640	\$ 708
Before tax adjustments:		
Share-based compensation	246	219
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 expenses	37	27
Impairment of intangible assets	31	31
Adjustments to income tax expense	(88)	(84)
Non-GAAP net income	<u>\$ 1,237</u>	<u>\$ 1,271</u>
Diluted GAAP earnings per common share	\$ 2.82	\$ 3.12
Diluted non-GAAP earnings per common share	\$ 5.40	\$ 5.55
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%
Non-GAAP research and development expense	<u>22%</u>	<u>21%</u>

GAAP selling, general and administrative expense	30%	29%
Share-based compensation	(4)%	(4)%
Non-GAAP selling, general and administrative expense	<u>26%</u>	<u>25%</u>
GAAP operating margin	23%	26%
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	1%	1%
Restructuring expenses	1%	1%
Impairment of intangible assets	1%	1%
Non-GAAP operating margin	<u>43%</u>	<u>44%</u>

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

	Three months ended		Six months ended	
	June 30		June 30	
	2017	2016	2017	2016
Soliris				
United States	\$ 318	\$ 261	\$ 606	\$ 498
Europe	249	239	490	463
Asia Pacific	81	74	160	143
Rest of World	166	127	341	262
Total Soliris	<u>\$ 814</u>	<u>\$ 701</u>	<u>\$ 1,597</u>	<u>\$ 1,366</u>
Strensiq				
United States	\$ 70	\$ 40	\$ 133	\$ 67
Europe	8	2	14	4
Asia Pacific	4	3	8	6
Rest of World	1	—	2	1
Total Strensiq	<u>\$ 83</u>	<u>\$ 45</u>	<u>\$ 157</u>	<u>\$ 78</u>
Kanuma				
United States	\$ 11	\$ 5	\$ 20	\$ 6
Europe	3	2	5	3
Asia Pacific	1	—	1	—
Rest of World	—	—	1	—
Total Kanuma	<u>\$ 15</u>	<u>\$ 7</u>	<u>\$ 27</u>	<u>\$ 9</u>
Net Product Sales				
United States	\$ 399	\$ 306	\$ 759	\$ 571
Europe	260	243	509	470
Asia Pacific	86	77	169	149
Rest of World	167	127	344	263
Total Net Product Sales	<u>\$ 912</u>	<u>\$ 753</u>	<u>\$ 1,781</u>	<u>\$ 1,453</u>

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

	June 30	December 31
	2017	2016
Cash and cash equivalents	\$ 542	\$ 966

Marketable securities	902	327
Trade accounts receivable, net	710	650
Inventories	410	375
Prepaid expenses and other current assets (1)	225	260
Property, plant and equipment, net	1,233	1,036
Intangible assets, net	4,112	4,303
Goodwill	5,037	5,037
Other assets	318	299
Total assets	<u>\$13,489</u>	<u>\$ 13,253</u>

Accounts payable and accrued expenses	\$ 613	\$ 572
Deferred revenue	14	37
Current portion of long-term debt	167	167
Current portion of contingent consideration	25	24
Other current liabilities	37	23
Long-term debt, less current portion	2,804	2,888
Contingent consideration	156	129
Facility lease obligation	277	233
Deferred tax liabilities	387	396
Other liabilities	134	90
Total liabilities	<u>4,614</u>	<u>4,559</u>
Total stockholders' equity (1)	<u>8,875</u>	<u>8,694</u>
Total liabilities and stockholders' equity	<u>\$13,489</u>	<u>\$ 13,253</u>

(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.

View source version on [businesswire.com](http://www.businesswire.com/news/home/20170727005411/en/): <http://www.businesswire.com/news/home/20170727005411/en/>

Alexion Contacts:

Media

Stephanie Fagan, 475-230-3777
Senior Vice President, Corporate Communications
or

Kim Diamond, 475-230-3775
Executive Director, Corporate Communications
or

Investors

Elena Ridloff, CFA, 475-230-3601
Vice President, Investor Relations
or

Catherine Hu, 475-230-3599
Director, Investor Relations

Source: Alexion Pharmaceuticals, Inc.

News Provided by Acquire Media