April 28, 2016

Alexion Reports First Quarter 2016 Results

- Total Revenues of $701 Million; Increased 17 Percent Year-on-Year; 22 Percent Increase Year-on-Year on a Constant Currency Basis -

- Soliris® (eculizumab) Revenues Driven by Steady Number of New Patients Treated in the U.S., Europe and Japan -

- Strensiq® (asfotase alfa) Launch Off to a Strong Start in Initial Countries -

- Kanuma® (sebelipase alfa) U.S. Launch Under Way -

- Exceeded Target Enrollment for Two Initial Studies of ALXN1210 in Patients with PNH -

- Commenced Dose Escalation for SBC-103 in Phase 1/2 Study in Patients with MPS IIIB -

- Progressed Three Registration Studies of Eculizumab: Data from gMG Expected Mid-Year; Data from DGF Expected Second Half 2016; On Track to Complete Enrollment in NMOSD This Year -

NEW HAVEN, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ: ALXN) today announced financial results for the first quarter of 2016. Total revenues grew to $701 million, a 17 percent increase, compared to $600 million for the same period in 2015. In the first quarter, the negative impact of currency on total revenue was 5 percent or $30 million, net of hedging activities, compared to the same quarter last year. First quarter revenue growth was further negatively impacted by increased macroeconomic weakness in Latin American countries, primarily Brazil and Argentina. Non-GAAP diluted earnings per share (EPS) for the first quarter of 2016 was $1.11 per share, compared to $1.28 per share in the first quarter of 2015. On a GAAP basis, diluted EPS for the first quarter of 2016 was $0.41 per share, compared to $0.45 per share in the first quarter of 2015.

"In Q1 2016, we grew our core Soliris business by serving a steady number of new patients with PNH and aHUS in the U.S., Europe and Japan, partially offset by the increased impact of macroeconomic weakness in Latin America. We are very pleased with the strong start to the Strensiq launches in initial countries and have now commenced the U.S. launch of Kanuma," said David Hallal, Chief Executive Officer of Alexion. "We look forward to 2016 being another transformative year for Alexion as we serve an increasing number of patients with four devastating, ultra-rare diseases and progress multiple milestones in our robust rare disease pipeline."

First Quarter 2016 Financial Highlights

- Soliris® (eculizumab) net product sales were $665 million compared to $600 million in Q1 2015. Net product sales increased 11 percent year-on-year, despite continued currency headwinds as well as increased macroeconomic weakness in Latin American countries, primarily Brazil and Argentina. Soliris volume increased 18 percent year-on-year.
- Strensiq® (asfotase alfa) net product sales were $33 million.
- Kanuma® (sebelipase alfa) net product sales were $2.5 million.
- Non-GAAP R&D expense was $158 million, compared to $97 million in the same quarter last year. GAAP R&D expense was $176 million, compared to $221 million in the same quarter last year.
- Non-GAAP SG&A expense was $194 million, compared to $157 million in the same quarter last year. GAAP SG&A expense was $233 million, compared to $187 million in the same quarter last year.
- Non-GAAP diluted EPS was $1.11 per share, compared to $1.28 per share in the same quarter last year. On a GAAP basis, diluted EPS was $0.41 per share, compared to $0.45 per share in the same quarter last year.

Product and Pipeline Updates

Complement Portfolio

- **Eculizumab—Generalized Myasthenia Gravis (gMG):** Enrollment is complete in the REGAIN study, a single, multinational, placebo-controlled registration trial of eculizumab in refractory gMG, and data are expected in mid-2016.
- **Eculizumab—Neuromyelitis Optica Spectrum Disorder (NMOSD):** Alexion expects to complete enrollment this year in the PREVENT study, a single, multinational, placebo-controlled registration trial of eculizumab in patients with relapsing NMOSD.
- **Eculizumab—Delayed Graft Function (DGF):** Enrollment is complete in the PROTECT study, a single, multinational, placebo-controlled registration trial of eculizumab in the prevention of DGF, and data are expected in the second half of 2016.
- **ALXN1210:** Alexion exceeded target enrollment in both a Phase 1/2 study and a Phase 2 study of ALXN1210, our highly innovative longer-acting C5 antibody, in patients with paroxysmal nocturnal hemoglobinuria (PNH), and we expect data from the Phase 1/2 study to be presented in mid-2016. Alexion also expects to initiate a clinical program in patients with atypical hemolytic uremic syndrome (aHUS) later this year.
- **ALXN1007:** Alexion is continuing to advance the development of ALXN1007, a complement inhibitor that targets C5a, in patients with graft-versus-host disease involving the lower gastrointestinal tract (GI-GVHD). Interim Phase 2 data reported in the fourth quarter of 2015 support the evaluation of higher doses of ALXN1007 in additional patients with acute GI-GVHD.

Metabolic Portfolio

- **Strensiq:** New long-term data presented at the Endocrine Society's 98th Annual Meeting and Expo (ENDO) in April showed sustained improvements in survival rates, bone healing, respiratory support, and growth and mobility in children with HPP treated with Strensiq. In addition, the data presented at ENDO showed that adolescent and adult patients treated with Strensiq reduced or eliminated their need of ambulatory assistive devices and had improvements in physical function as measured by the Six Minute Walk Test.
- **Kanuma:** Kanuma received marketing approval from Japan's Ministry of Health, Labour and Welfare on March 28, 2016. Additionally, new data presented by researchers at the WORLD*Symposium* meeting in March showed a substantial survival benefit beyond 2 years of age in infants with LAL-D treated with Kanuma.
- **SBC-103:** Alexion has commenced the planned dose escalation in the Phase 1/2 trial of SBC-103, a recombinant form of the NAGLU enzyme, in patients with mucopolysaccharidosis IIIB, or MPS IIIB. Patients are now being randomized to either a 5 mg/kg or 10 mg/kg dose. Six-month data presented at the WORLD*Symposium* meeting in March showed continued reductions in heparan sulfate cerebrospinal fluid with a mean reduction of 26% in the highest dose studied, 3 mg/kg.
- **cPMP Replacement Therapy (ALXN1101):** Alexion is progressing a pivotal study to evaluate ALXN1101 in neonates with Molybdenum Cofactor Deficiency (MoCD) Type A. Alexion received Breakthrough Therapy designation for its cPMP replacement therapy.

Preclinical Portfolio

- Alexion has more than 30 diverse preclinical programs across a range of therapeutic modalities, with four of these programs expected to enter the clinic in 2016.

2016 Financial Guidance

Alexion expects 2016 total revenues to be at the low end of our previously guided range of $3,050 million to $3,100 million, primarily due to increased macroeconomic weakness in Latin America, partially offset by an increase in Strensiq revenues and the strengthening of foreign currencies.

R&D and SG&A expenses are expected to be at the high end of guidance primarily due to continued investment in key programs in our R&D pipeline and the commercial launches of Strensiq and Kanuma, as well as the strengthening of foreign currencies.

Alexion expects 2016 non-GAAP EPS to be at the low end of the previously guided range of $5.00 to $5.20 per share.

Updated 2016 non-GAAP financial guidance is as follows:

	Updated Guidance (1)	Prior Guidance (1)
Total revenues	Low end of $3,050 to $3,100 million	$3,050 to $3,100 million
Soliris revenues	$2,835 to $2,875 million	$2,900 to $2,925 million
Metabolic revenues	$180 to $200 million	$150 to $175 million
Cost of sales	8% to 9%	8% to 9%
Research and development expense	High end of $650 to $680 million	$650 to $680 million
Selling, general and administrative expense	High end of $760 to $790 million	$760 to $790 million
Interest expense	$100 million	$100 million
Effective tax rate	7% to 8%	7% to 8%
Earnings per share	Low end of $5.00 to $5.20	$5.00 to $5.20
Diluted shares outstanding	230 million	230 million

(1) Financial guidance is based on forecasted results at current spot rates net of hedging activities.

Conference Call/Webcast Information:

Alexion will host a conference call/audio webcast to discuss matters mentioned in this release. The call is scheduled for today, April 28, at 10:00 a.m., Eastern Time. To participate in this call, dial 888-576-4397 (USA) or 719-325-2301 (International), passcode 4785731 shortly before 10:00 a.m., Eastern Time. A replay of the call will be available for a limited period following the call, beginning at 1:00 p.m., Eastern Time. The replay number is 888-203-1112 (USA) or 719-457-0820 (International), passcode 4785731. 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.

[ALXN-E]

This news 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 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, 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 the SEC and DOJ investigations, 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 Annual Report on Form 10-K for the period ended December 31, 2015 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 news 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 license and collaboration agreements, and non-cash taxes. 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 Reconciliation of GAAP to non-GAAP Financial Results 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, 2016 and 2015.

(Tables Follow)

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

	Three months ended March 31	
	2016	**2015**
Net product sales	$ 700,425	$ 600,333
Other revenue	613	-
Total revenues	701,038	600,333
Cost of sales	58,986	69,399
Operating expenses:		
Research and development	176,290	221,080
Selling, general and administrative	232,561	187,116
Amortization of purchased intangible assets	80,094	-
Change in fair value of contingent consideration	(14,800)	11,979
Acquisition-related costs	1,339	-
Restructuring expenses	722	7,052
Total operating expenses	476,206	427,227
Operating income	165,846	103,707
Other income and expense:		
Investment income	1,551	2,884
Interest expense	(23,890)	(651)
Foreign currency gain	91	1,005
Income before income taxes	143,598	106,945
Income tax provision	51,432	15,622
Net income	$ 92,166	$ 91,323
Earnings per common share		
Basic	$ 0.41	$ 0.46
Diluted	$ 0.41	$ 0.45

Shares used in computing earnings per common share		
Basic	225,060	199,361
Diluted	226,873	202,034

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

	Three months ended March 31	
	2016	**2015**
Net income reconciliation:		
GAAP net income	$ 92,166	$ 91,323
Share-based compensation expense	56,889	42,797
Fair value adjustment of inventory acquired (1)	531	-
Amortization of purchased intangible assets (2)	80,094	-
Change in fair value of contingent consideration (3)	(14,800)	11,979
Acquisition-related costs (4)	1,339	-
Restructuring expenses (5)	722	7,052
Upfront and milestone payments related to license and collaboration agreements	3,050	112,500
Non-cash taxes (6)	33,784	(3,672)
Non-GAAP net income	$ 253,775	$ 261,979
GAAP earnings per share - diluted	$ 0.41	$ 0.45
Non-GAAP earnings per share - diluted	$ 1.11	$ 1.28
Shares used in computing diluted earnings per share (GAAP)	226,873	202,034
Shares used in computing diluted earnings per share (non-GAAP)	229,174	204,383
Cost of sales reconciliation:		
GAAP cost of sales	$ 58,986	$ 69,399
Share-based compensation expense	(3,403)	(1,409)
Fair value adjustment of inventory acquired	(531)	-
Non-GAAP cost of sales	$ 55,052	$ 67,990
Research and development expense reconciliation:		
GAAP research and development	$ 176,290	$ 221,080
Share-based compensation expense	(15,185)	(11,084)
Upfront and milestone payments related to license and collaboration agreements	(3,050)	(112,500)
Non-GAAP research and development expense	$ 158,055	$ 97,496
Selling, general and administrative expense reconciliation:		
GAAP selling, general and administrative expense	$ 232,561	$ 187,116
Share-based compensation expense	(38,301)	(30,304)
Non-GAAP selling, general and administrative expense	$ 194,260	$ 156,812
Income tax provision reconciliation:		
GAAP income tax provision	$ 51,432	$ 15,622
Non-cash taxes	(33,784)	3,672
Non-GAAP income tax provision	$ 17,648	$ 19,294

(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) In the first quarter of 2016, the Company realized a change in fair value of contingent consideration due to decreases in the likelihood of payments for contingent consideration associated with our prior business combinations.

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

	Three months ended March 31	
	2016	2015
Acquisition-related costs:		
Transaction costs	$ 375	$ -
Integration costs	964	-
	$ 1,339	$ -

(5) First quarter 2016 restructuring expenses of $0.7 million includes $1.6 million related to the European headquarters relocation, offset by a $0.9 million benefit resulting from the acquisition of Synageva due to changes in estimates associated with employee costs.

(6) Non-cash taxes represents the adjustment from GAAP tax expense to the taxes payable in cash on current period operations.

ALEXION PHARMACEUTICALS, INC.
REVENUES
(in thousands)
(unaudited)

	Three months ended March 31	
	2016	2015
Soliris	$ 664,656	$ 600,333
Strensiq	33,242	-
Kanuma	2,527	-
Total net product sales	700,425	600,333
Royalty revenue	613	-
Total other revenue	613	-
Total revenues	$ 701,038	$ 600,333

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

	March 31, 2016	December 31, 2015
Cash and cash equivalents	$ 710,198	$ 1,010,111
Marketable securities	317,354	374,904
Trade accounts receivable, net	586,249	532,832
Inventories	293,962	289,874
Prepaid expenses and other current assets	219,746	208,993
Property, plant and equipment, net	749,295	697,025
Intangible assets, net	4,627,817	4,707,914

Goodwill	5,049,321	5,047,885
Other assets	248,503	228,343
Total assets	$12,802,445	$ 13,097,881
Accounts payable and accrued expenses	$ 401,974	$ 460,708
Deferred revenue	78,416	20,504
Current portion of long-term debt	35,358	166,365
Other current liabilities	87,865	62,038
Long-term debt, less current portion	3,212,772	3,254,536
Contingent consideration	107,085	121,424
Facility lease obligation	172,970	151,307
Deferred tax liabilities	535,910	528,990
Other liabilities	107,818	73,393
Total liabilities	4,740,168	4,839,265
Total stockholders' equity	8,062,277	8,258,616
Total liabilities and stockholders' equity	$12,802,445	$ 13,097,881

View source version on businesswire.com: http://www.businesswire.com/news/home/20160428005436/en/

Alexion Pharmaceuticals, Inc.
Media
Stephanie Fagan, 203-271-8223
Senior Vice President, Corporate Communications
or
Investors
Elena Ridloff, CFA, 203-699-7722
Vice President, Investor Relations

Source: Alexion Pharmaceuticals, Inc.