April 24, 2014

# Alexion Reports First Quarter 2014 Results

- Soliris® (eculizumab) Net Product Sales Increased 67 Percent vs. Year-Ago Quarter to $567 Million -

- Soliris Net Product Sales Increased 41 Percent to $479 Million, Excluding $88 Million Related to Reimbursement of Prior Year Shipments -

- Rolling Submission of U.S. Biologics License Application for Asfotase Alfa Initiated -

- NMO and MG Registration Trials with Eculizumab Underway -

- Enrollment Complete in Living-Donor AMR Trial with Eculizumab -

- 2014 Guidance Increased for Non-GAAP EPS -

**First Quarter 2014 Financial Highlights:**

- Q1 2014 net product sales increased 67 percent to $566.6 million, compared to $338.9 million in Q1 2013. Excluding the impact of $87.8 million for reimbursement of prior year shipments, Q1 2014 net product sales increased 41 percent to $478.8 million.
- Q1 2014 GAAP EPS increased 93 percent to $0.79 per share from Q1 2013; Q1 2014 GAAP EPS included $0.31 per share related to reimbursement of prior year shipments.
- Q1 2014 non-GAAP EPS increased 135 percent to $1.53 per share compared to Q1 2013; Q1 2014 non-GAAP EPS increased 78 percent to $1.16 per share, compared to Q1 2013, excluding $0.37 per share in Q1 2014 related to reimbursement of prior year shipments.

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced financial results and a development pipeline update for the three months ended March 31, 2014. The Company reported net product sales of Soliris® (eculizumab) of $566.6 million, including reimbursement of prior year shipments related to an agreement with the French government of $87.8 million, an increase of 67 percent from the same period in 2013. Revenue performance for the quarter also reflected steady additions of new patients with paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS) commencing Soliris treatment.

Alexion is now serving patients with PNH and aHUS in nearly 50 countries. Both PNH and aHUS are severe and life-threatening ultra-rare disorders caused by chronic uncontrolled complement activation.

Alexion's non-GAAP operating results are GAAP operating results adjusted for the impact of certain items described below. A full reconciliation of GAAP to non-GAAP financial results is included later in this press release.

**First Quarter 2014 Financial Results:**

Alexion recorded increased revenue in the first quarter 2014 related to an agreement with the French government. The Company recorded $87.8 million of additional net product sales related to reimbursement for product shipments in prior years under this agreement.

**First Quarter 2014 Non-GAAP Financial Results:**

The Company reported non-GAAP net income of $312.6 million, or $1.53 per share, in the first quarter of 2014, compared to non-GAAP net income of $131.3 million, or $0.65 per share, in the first quarter of 2013. Q1 2014 non-GAAP EPS includes an increase of $0.37 per share related to the reimbursement of prior year shipments.

Alexion's non-GAAP operating expenses for Q1 2014 were $195.9 million, compared to $164.4 million for Q1 2013. Non-GAAP research and development (R&D) expenses for Q1 2014 were $81.5 million, compared to $66.4 million for Q1 2013. Non-GAAP selling, general and administrative (SG&A) expenses for Q1 2014 were $114.3 million, compared to $97.9 million for Q1 2013.

**First Quarter 2014 GAAP Financial Results:**

Alexion reported GAAP net income of $159.4 million, or $0.79 per share, in the first quarter of 2014, compared to GAAP net income of $82.2 million, or $0.41 per share, in the first quarter of 2013. Q1 2014 GAAP EPS includes an increase of $0.31 per share related to the reimbursement of prior year shipments.

On a GAAP basis, operating expenses for Q1 2014 were $324.2 million, compared to $186.7 million for Q1 2013. GAAP R&D expenses for Q1 2014 were $191.5 million, compared to $74.5 million for Q1 2013. Q1 2014 GAAP R&D expense included $100.0 million of expense for an upfront payment on the strategic agreement with Moderna Therapeutics. GAAP SG&A expenses were $129.3 million for Q1 2014, compared to $108.8 million for Q1 2013.

**Balance Sheet:**

As of March 31, 2014, the Company had $1.55 billion in cash and cash equivalents compared to $1.52 billion at December 31, 2013. During the quarter, cash flows were impacted by $125.0 million in upfront and equity payments under the Company's strategic agreement with Moderna Therapeutics.

"In the first quarter of 2014, we provided Soliris to an increasing number of patients with PNH worldwide, made important progress in our aHUS operations in Western Europe and other territories, and continued to execute on key initiatives to further improve operational and financial efficiencies across our global operations," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "As we performed strongly in our current therapeutic areas in the quarter, we also progressed toward our next levels of growth as we reached a number of significant milestones across our pipeline, including the initiation of the rolling submission for our BLA for our next product, asfotase alfa. Throughout 2014 we will remain focused on serving more patients with PNH and aHUS globally while simultaneously advancing our lead development programs as we drive toward as many as seven additional launches through 2018."

**Research and Development Progress**

Alexion has development programs underway with highly innovative therapeutic candidates that have the potential to become first-in-class therapies for patients with severe and ultra-rare disorders.

**Asfotase Alfa**

- Alexion has initiated the rolling submission of a Biologics License Application (BLA) for asfotase alfa, a targeted enzyme replacement therapy for the treatment of patients with hypophosphatasia (HPP) and anticipates completing the rolling submission in the fall of this year. The Company received Breakthrough Therapy designation for asfotase alfa in pediatric-onset HPP in 2013. Alexion has completed the analysis of its natural history study in infants with HPP and a natural history study in juveniles with HPP is ongoing.

**Ultra-Rare Disease Programs With Eculizumab**

- **Transplant: Antibody-Mediated Rejection (AMR) -** Enrollment is complete and dosing continues in the Company-sponsored, multinational living-donor kidney transplant trial in patients at elevated risk of AMR. Alexion continues to enroll patients in the expanded Company-sponsored, multinational deceased-donor kidney transplant trial in patients at elevated risk of AMR.
- **Transplant: Delayed Graft Function (DGF)** - This quarter, the U.S. Food and Drug Administration (FDA) granted orphan drug designation to eculizumab for the prevention of DGF in renal transplant patients, and the European Commission granted orphan drug designation to eculizumab for the prevention of DGF after solid organ transplantation. Alexion is planning to commence a single, multinational registration trial for the prevention of DGF in renal transplant patients.
- **Neurology: Neuromyelitis Optica (NMO)** - Alexion has commenced dosing in a single, multinational, placebo-controlled, registration trial in relapsing NMO.
- **Neurology: Myasthenia Gravis (MG) -** Alexion has commenced screening in a single, multinational, placebo-controlled, registration trial in severe, refractory MG.

**Ultra-Rare Disease Programs with Additional Highly Innovative Therapeutics**

- **cPMP Replacement Therapy (ALXN 1101):** A natural history study in patients with molybdenum cofactor deficiency (MoCD) is ongoing and Alexion has initiated a synthetic cPMP bridging study. Alexion received Breakthrough Therapy designation for its cPMP replacement therapy in 2013, which is being developed for patients with MoCD Type A.

- **ALXN1007:** Alexion is preparing to commence two Phase 2 proof-of-concept studies of ALXN1007, a novel anti-inflammatory antibody, in severe and life-threatening ultra-rare disorders.

**2014 Financial Guidance**

Alexion today announced that the Company is revising upward its guidance for 2014 non-GAAP earnings per share from the previous range of $4.37 to $4.47, now to the higher range of $4.75 to $4.85 per share. Guidance for cost of goods sold is being revised downward from approximately 9 percent of net product sales now to approximately 8 percent of sales. Guidance for 2014 SG&A expense is being revised downward from the previous range of $560 to $580 million now to the lower range of $550 to $570 million. Guidance for the 2014 non-GAAP tax rate, is being revised downward from 10 to 11 percent to the lower range of 8 to 9 percent. Guidance for the 2014 GAAP tax rate is being increased from the previous range of 20 to 22 percent now to the range of 23 to 25 percent. EPS guidance is based on a forecast of approximately 205 million diluted shares outstanding for the year.

Alexion is reiterating its 2014 revenue guidance of $2.15 to $2.17 billion as provided in the press release issued on March 10, 2014, and is also reiterating its non-GAAP R&D guidance of $360 to $380 million as provided in the Company's press release on January 30, 2014.

**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 24, at 10:00 a.m., Eastern Time. To participate in this call, dial 800-378-1475 (USA) or 719-785-9448 (International), passcode 6226785, 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 6226785. The audio webcast can be accessed at www.alexionpharma.com.

**About Soliris**

Soliris is a first-in-class terminal complement inhibitor developed from the laboratory through regulatory approval and commercialization by Alexion. Soliris is approved in the U.S. (2007), European Union (2007), Japan (2010) and other countries as the first and only treatment for patients with paroxysmal nocturnal hemoglobinuria (PNH), a debilitating, ultra-rare and life-threatening blood disorder, characterized by complement-mediated hemolysis (destruction of red blood cells). Soliris is indicated to reduce hemolysis. Soliris is also approved in the U.S. (2011), the European Union (2011), Japan (2013) and other countries as the first and only treatment for patients with atypical hemolytic uremic syndrome (aHUS), a debilitating, ultra-rare and life-threatening genetic disorder characterized by complement-mediated thrombotic microangiopathy, or TMA (blood clots in small vessels). Soliris is indicated to inhibit complement-mediated TMA. The effectiveness of Soliris in aHUS is based on its effects on TMA and renal function. Prospective clinical trials in additional patients, the preliminary results of which were reported at international nephrology and hematology conferences in 2013, are ongoing to confirm the benefit of Soliris in patients with aHUS. Soliris is not indicated for the treatment of patients with Shiga-toxin E. coli-related hemolytic uremic syndrome (STEC-HUS). For the breakthrough medical innovation in complement inhibition, Alexion and Soliris have received the pharmaceutical industry's highest honors: the 2008 Prix Galien USA Award for Best Biotechnology Product with broad implications for future biomedical research and the 2009 Prix Galien France Award in the category of Drugs for Rare Diseases.

More information, including the full U.S. prescribing information on Soliris, is available at www.soliris.net.

**About Alexion**

Alexion is a biopharmaceutical company focused on serving patients with severe and rare disorders through the innovation, development and commercialization of life-transforming therapeutic products. Alexion is the global leader in complement inhibition and has developed and markets Soliris® (eculizumab) as a treatment for patients with PNH and aHUS, two debilitating, ultra-rare and life-threatening disorders caused by chronic uncontrolled complement activation. Soliris is currently approved in nearly 50 countries for the treatment of PNH, and in the United States, European Union, Japan and other countries for the treatment of aHUS. Alexion is evaluating other potential indications for Soliris in additional severe and ultra-rare disorders beyond PNH and aHUS, and is developing other highly innovative biotechnology product candidates across multiple therapeutic areas. This press release and further information about Alexion can be found at www.alexionpharma.com.

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*This news release contains forward-looking statements, including statements related to guidance regarding anticipated financial results for 2014, assessment of the Company's financial position and commercialization efforts, medical benefits and commercial potential for Soliris for PNH and aHUS and other potential indications, medical and commercial potential of Alexion's complement-inhibition technology and other technologies, and plans for clinical programs for each of 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 Soliris for PNH and aHUS and other potential indications, delays, interruptions or failures in the manufacture and supply of Soliris and our product candidates, progress in establishing and developing commercial infrastructure, failure to satisfactorily address the issues raised by the FDA in the Warning Letter received by Alexion in March 2013, the possibility that results of clinical trials are not predictive of safety and efficacy results of Soliris in broader patient populations in the disease studied or other diseases, the risk that acquisitions 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 risk that third party payors (including governmental agencies) will not reimburse or continue to reimburse for the use of Soliris at acceptable rates or at all, the risk that estimates regarding the number of patients with PNH, aHUS or other diseases are inaccurate, 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, 2013 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, acquisition-related costs, amortization of purchased intangible assets, intellectual property settlements, upfront and milestone payments related to license and collaboration agreements, intangible asset impairments, 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 Net Income 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, 2014 and 2013.*

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

| | Three months ended March 31 | |
|---|---|---|
| | **2014** | **2013** |
| Net product sales | $566,616 | $338,941 |
| Cost of sales | 32,939 | 35,269 |
| Operating expenses: | | |
| Research and development | 191,457 | 74,536 |
| Selling, general and administrative | 129,291 | 108,826 |
| Impairment of intangible asset | 3,464 | - |
| Acquisition-related costs | (38) | 3,234 |
| Amortization of purchased intangible assets | - | 104 |
| Total operating expenses | 324,174 | 186,700 |
| Operating income | 209,503 | 116,972 |
| Other income (expense) | 2,408 | (231) |
| Income before income taxes | 211,911 | 116,741 |
| Income tax provision | 52,557 | 34,524 |
| Net income | $159,354 | $ 82,217 |

| | | |
|---|---|---|
| Earnings per common share | | |
| Basic | $ 0.81 | $ 0.42 |
| Diluted | $ 0.79 | $ 0.41 |
| Shares used in computing earnings per common share | | |
| Basic | 197,797 | 194,771 |
| Diluted | 201,804 | 199,057 |

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

| | Three months ended March 31 | |
|---|---|---|
| | **2014** | **2013** |
| **Net income reconciliation:** | | |
| GAAP net income | $ 159,354 | $ 82,217 |
| Share-based compensation expense | 23,840 | 16,855 |
| Acquisition-related costs (1) | (38) | 3,234 |
| Amortization of purchased intangible assets | - | 104 |
| Upfront and milestone payments related to license and collaboration agreements (2) | 101,925 | 3,000 |
| Impairment of intangible asset (3) | 3,464 | - |
| Non-cash taxes (4) | 24,054 | 25,904 |
| Non-GAAP net income | $ 312,599 | $131,314 |
| GAAP earnings per share - diluted | $ 0.79 | $ 0.41 |
| Non-GAAP earnings per share - diluted | $ 1.53 | $ 0.65 |
| Shares used in computing diluted earnings per share (GAAP) | 201,804 | 199,057 |
| Shares used in computing diluted earnings per share (non-GAAP) | 204,830 | 202,225 |
| **Cost of sales reconciliation:** | | |
| GAAP cost of sales | $ 32,939 | $ 35,269 |
| Share-based compensation expense | (883) | (875) |
| Non-GAAP cost of sales | $ 32,056 | $ 34,394 |
| **Research and development reconciliation:** | | |
| GAAP research and development | $ 191,457 | $ 74,536 |
| Share-based compensation expense | (7,984) | (5,090) |
| Upfront and milestone payments related to license and collaboration agreements (2) | (101,925) | (3,000) |
| Non-GAAP research and development | $ 81,548 | $ 66,446 |
| **Selling, general and administrative reconciliation:** | | |
| GAAP selling, general and administrative | $ 129,291 | $108,826 |
| Share-based compensation expense | (14,973) | (10,890) |
| Non-GAAP selling, general and administrative | $ 114,318 | $ 97,936 |
| **Income tax provision reconciliation:** | | |
| GAAP income tax provision | $ 52,557 | $ 34,524 |
| Non-cash taxes (4) | (24,054) | (25,904) |

| | | |
|---|---|---|
| Non-GAAP income tax provision | $ 28,503 | $ 8,620 |

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

| | Three months ended March 31 | |
|---|---|---|
| | 2014 | 2013 |
| **Acquisition-related costs:** | | |
| Separately-identifiable employee costs | $ - | $ 248 |
| Professional fees | - | 775 |
| Changes in fair value of contingent consideration | (38) | 2,211 |
| | $ (38) | $ 3,234 |

(2) In January 2014, the Company entered into an agreement that provides an option to purchase drug products for clinical development and commercialization. The Company recorded research and development expense for an upfront payment of $100.0 million.

(3) During the three months ended March 31, 2014, the Company recorded a $3.5 million impairment related to an early stage development asset.

(4) Non-cash taxes represents the adjustment from GAAP tax expense to the amount of taxes that are payable in cash on our operating profits.

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

| | March 31, 2014 | December 31, 2013 |
|---|---|---|
| Cash and cash equivalents | $ 528,285 | $ 529,857 |
| Marketable securities | 1,029,193 | 984,994 |
| Trade accounts receivable, net | 429,021 | 421,752 |
| Inventories | 126,154 | 102,602 |
| Deferred tax assets, current | 45,871 | 41,432 |
| Other current assets | 87,356 | 106,220 |
| Property, plant and equipment, net | 218,086 | 201,109 |
| Intangible assets, net | 603,021 | 609,719 |
| Goodwill | 254,073 | 254,073 |
| Deferred tax assets, noncurrent | 3,366 | 3,394 |
| Other noncurrent assets | 55,240 | 62,544 |
| Total assets | $3,379,666 | $ 3,317,696 |
| | | |
| Accounts payable and accrued expenses | $ 231,708 | $ 423,940 |
| Current portion of long-term debt | 48,000 | 48,000 |
| Other current liabilities | 117,114 | 110,489 |
| Long-term debt, less current portion | 45,500 | 65,000 |
| Contingent consideration, noncurrent | 106,241 | 106,744 |
| Deferred tax liabilties, noncurrent | 45,986 | 101,241 |
| Other noncurrent liabilities | 84,139 | 80,203 |
| Total liabilities | 678,688 | 935,617 |
| | | |
| Total stockholders' equity | 2,700,978 | 2,382,079 |
| Total liabilities and stockholders' equity | $3,379,666 | $ 3,317,696 |

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