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Alexion to Highlight Innovative Rare Disease Portfolio at Investor Day Meeting

- Interim Phase 1/2 Data of SBC-103 in Patients with MPS-IIIB to be Presented -

- Interim Phase 2 Data of ALXN1007 in Patients with GI-GVHD to be Presented -

- First Patients with PNH Dosed in ALXN1210 Study -

- Completed Enrollment in Registration Trial of Eculizumab in DGF -

- First mRNA Product Candidate to Target Crigler-Najjar Syndrome (CN-1) -

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) announced today that the Company will highlight significant progress in its early- and late-stage rare disease portfolio and provide updates on key aspects of its long-term growth strategy at an Investor Day meeting being held in New York. Alexion's leadership team will discuss the corporate strategy to grow the Company's complement franchise, build a leading metabolic franchise, advance its broad and robust rare disease pipeline, and expand innovation to new franchises.

Alexion has achieved several key developmental milestones across its complement and metabolic franchises as it further strengthens its leadership position in rare diseases. The Company's presentations today will include:

- Interim Phase 1/2 data of SBC-103, an enzyme replacement therapy in patients with mucopolysaccharidosis IIIB (MPS-IIIB);
- Interim Phase 2 data of ALXN1007, a complement inhibitor that targets C5a, in patients with acute graft-versus-host disease involving the lower gastrointestinal tract (GI-GVHD);
- Dosing of the first patients with paroxysmal nocturnal hemoglobinuria (PNH) in a Phase 1/2 trial of ALXN1210;
- Completion of enrollment in the registration trial of eculizumab in delayed graft function (DGF); and
- Additional data supporting the advancement of four pre-clinical programs into the clinic in 2016.

Metabolic Franchise

SBC-103

SBC-103 is an enzyme replacement therapy being investigated in a Phase 1/2 trial for patients with mucopolysaccharidosis IIIB, or MPS IIIB (also known as Sanfilippo B syndrome). MPS IIIB is a rare, devastating and progressive autosomal recessive lysosomal storage disease caused by a genetic deficiency of the enzyme known as NAGLU. This enzyme deficiency leads to the buildup of abnormal amounts of heparan sulfate in the brain and other organs, resulting in profound neurocognitive abnormalities including severe cognitive decline, behavioral problems, speech loss, increasing loss of mobility, and premature death.[1] Patients with MPS IIIB have a greater than 50 percent mortality rate by 17 years of age.[2]

The primary endpoint of the Phase 1/2 trial is safety and tolerability. Key secondary efficacy endpoints include changes from baseline in heparan sulfate levels in cerebral spinal fluid (CSF), serum and urine as well as measurements of change in brain structures as assessed by magnetic resonance imaging and the effects of neurocognitive and developmental function. An interim analysis from the study will be presented at today's event.

Complement Franchise

ALXN 1210

ALXN 1210 is a next-generation complement inhibitor in development for once-monthly dosing. Phase I data from the first-in-human single-ascending dose study of ALXN1210 were published online in the journal *Blood* at the American Society of Hematology congress last week.[3] In the study, healthy volunteers received a single intravenous (IV) dose of ALXN1210 in sequential ascending doses (starting dose was 200 mg) or placebo, and were then followed for 150 days. Results showed

that ALXN1210 was well-tolerated in healthy volunteers. Mean chicken red blood cell (cRBC) hemolysis was completely inhibited after a single IV dose of 200 mg of ALXN1210, and levels of free C5 were reduced by > 99 percent from baseline in these healthy volunteers. In addition, serum concentration data of ALXN1210 exhibited a terminal half-life of 32 days, which is longer than that of Soliris® (eculizumab), which has a terminal half-life of 9 days. A multiple-ascending dose study of ALXN1210 is ongoing to further evaluate the safety and efficacy of ALXN1210.

More information on the ALXN1210 clinical development program in patients with PNH will be shared at the event later today.

ALXN 1007

ALXN1007 is a novel anti-inflammatory antibody targeting complement protein C5a, which is being evaluated in a Phase 2 study in patients with graft-versus-host disease involving the lower gastrointestinal tract, or GI-GVHD, a severe and life-threatening rare auto-immune disease. The primary endpoint of the trial is overall acute GI-GVHD response rate at day 28. Interim data will be presented today.

Acute GI-GVHD is an immune-mediated disease and a complication of stem cell transplantation occurring in 10 to 12 percent of allogenic hematopoietic stem cell transplants.[4,5] Patients with severe acute GI-GVHD have a 30 to 40 percent mortality rate within the first six months post-transplant.[6] There are no approved treatments for GI-GVHD.

Pre-Clinical Programs

Alexion is progressing its preclinical pipeline of more than 30 diverse programs across a range of therapeutic modalities. The company expects four of these programs to enter the clinic in 2016. These include:

- Asfotase alfa as a potential treatment for the bone complications of neurofibromatosis Type 1, or NF1. NF1 is a severe, multi-systemic genetic disorder caused by mutations in the NF-1 gene that develops in childhood and disturbs cell growth, resulting in widespread lesions throughout the body including the skin, eyes, nervous system (including brain, spinal cord, and nerves) and bone. Lesions occurring in long bones can progress to non-healing fractures (pseudo-arthroses), which are thought to be due in part to calcification being impaired by excess pyrophosphate production (PPI).[7] It is hypothesized that asfotase alfa could restore the balance in bone metabolism, thereby healing the lesions and preventing progression to fracture.
- ENPP1, an enzyme replacement therapy being developed for generalized arterial calcification of infancy, or GACI, and other rare disorders of calcification. GACI is an ultra-rare genetic disorder affecting infants in which excess of calcification in the medium and large arteries results in heart failure and respiratory distress. Patients with GACI have a 35 percent survival at 6 months of age.[8]
- ALXN 1540, one of the mRNA rare disease programs from the Company's collaboration with Moderna, in patients with Crigler-Najjar Syndrome (CN-1). CN-1 is a chronic congenital condition of excessive serum bilirubin caused by mutations in the UGT1A1 gene that presents in newborns. CN-1 is associated with irreversible brain damage and premature mortality;[9] and
- One additional candidate from Alexion's growing complement inhibitor portfolio.

Webcast/Conference Call Information

Today's presentation will be broadcast via a live webcast which can be accessed on the Investor page of Alexion's website at: http://ir.alexionpharm.com. A replay of the webcast will be archived on the website following the presentation. To listen to the conference call, dial 866-546-3377 (USA) or 719-234-7872 (International), passcode 737 644 3057 shortly before 12:00 p.m. ET.

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®Â (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.

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Forward-Looking Statements

This press release contains forward-looking statements, including statements related to the potential medical benefits of the company's product candidates, including ALXN 1210, ALXN 1007, and SBC-103, 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 our products, delays, interruptions or failures in the manufacture and supply of our products and our product candidates, failure to satisfactorily address issues raised by the FDA in regulatory correspondence, 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 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 payers (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, the risk that estimates regarding the number of patients with PNH, GI-GVHD, MPS III 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 Quarterly Report on Form 10-Q for the period ended September 30, 2015 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.

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