EMBARGOED UNTIL 6:30 P.M. ET ON MONDAY, OCTOBER 12, 2015

Significant Improvements in Skeletal Mineralization and Physical Function Observed in Children with Hypophosphatasia (HPP) Treated With Strensiq™ (asfotase alfa) Sustained for at least Five Years

– Researchers also Report Significant Improvements in Functional Mobility in Children with HPP who were Treated with Strensiq –

– Data Presented at ASBMR 2015 –

CHESHIRE, Conn.—October 12, 2015—Alexion Pharmaceuticals, Inc. (NASDAQ: ALXN) today announced that researchers presented new data from the extension phase of an ongoing, open-label Phase 2 study in children with hypophosphatasia (HPP) who were treated with Strensiq™ (asfotase alfa) for at least five years (n=12, ages five to 12 years at study entry). In this study, children treated with Strensiq experienced significant healing of HPP-related skeletal manifestations and improvements in physical function within three to six months from treatment onset, which were sustained through five years of treatment.[1] These data were presented in an oral session at the 2015 American Society for Bone and Mineral Research (ASBMR) Annual Meeting in Seattle. In a separate analysis presented at ASBMR, clinically significant improvements in functional mobility compared to historical controls were observed in patients with juvenile-onset HPP who were treated with Strensiq in the ongoing, open-label Phase 2 study.[2]

HPP is a genetic and progressive metabolic disease in which patients experience devastating effects on multiple systems of the body, leading to debilitating or life-threatening complications.[3] It is an ultra-rare disease, which is defined as a disease that affects fewer than 20 patients per one million in the general population.[4] HPP is characterized by low alkaline phosphatase (ALP) activity and defective bone mineralization that can lead to deformity of bones and other skeletal abnormalities, as well as systemic complications such as profound muscle weakness, seizures, pain, and respiratory failure leading to premature death in infants.[3,5-8]

“The data presented at ASBMR 2015 build upon the growing body of clinical knowledge on the use of Strensiq in patients with infantile- and juvenile-onset HPP and further enhance our understanding of this ultra-rare, life-threatening metabolic disease so that we can improve diagnosis and care for patients,” said Martin Mackay, Ph.D., Executive Vice President and Global Head of R&D at Alexion. “These new longer-term results from the ongoing Phase 2 studies with Strensiq provide additional critical information on the long-term safety and efficacy of this highly innovative therapy for patients with HPP.”

Asfotase Alfa: Sustained Efficacy and Tolerability in Children with Hypophosphatasia Treated for 5 Years (Oral 1071)[1]

In an oral session, Cheryl Rockman-Greenberg, M.D., Distinguished Professor, Department of Pediatrics and Child Health, University of Manitoba and Clinician Scientist at the Children's Hospital Research Institute of Manitoba, Winnipeg, Canada, presented new results from the extension phase of a

multinational, open-label, Phase 2 study in children with HPP (ages 5-12 years at study entry) who were treated with Strensiq for at least five years (n=12).[1]

Dr. Rockman-Greenberg reported that:

- Patients treated with Strensiq demonstrated significant improvement of HPP-related skeletal manifestations, including the primary endpoint at 6 months, that were sustained through five years as measured by a median improvement in RGI-C score of +2.2 ($p<0.01$ for all time points).[1]
- Median Six Minute Walking distance improved from 61 percent of that predicted for healthy peers at baseline to 85 percent at 6 months and 83 percent at five years of treatment ($p\leq0.0005$ mean change from baseline at each time point), indicating sustained improvements in walking over five years.[1]
- Strength and agility, measured as a composite of Running Speed/Agility and Strength sub-tests of Bruininks-Oseretsky Test of Motor Proficiency, Second Edition (BOT-2), improved from below normal at baseline to within the normal range at one year of Strensiq treatment, which was sustained through five years of treatment ($p<0.005$ mean change from baseline at each time point).[1]
- In patients treated with Strensiq, a significant and clinically meaningful reduction in disability was observed, as measured by the Child Health Assessment Questionnaire (CHAQ) Disability Index, with scores decreasing from a median of 1.0 at baseline to 0.25 at six months to 0.0 at two years, which was sustained through five years of treatment ($p<0.05$ mean change from baseline at each time point).[1]
- Median change in height Z-score (a measurement of patient growth) from baseline to five years improved by 0.65 ($p=0.0017$).[1]

“The findings presented today continue to indicate that treatment with Strensiq can lead to significant and clinically meaningful improvements in skeletal manifestations and physical function, with many of the treated patients achieving outcomes within the normal range of healthy peers. Importantly, these improvements were sustained through five years of treatment,” said Dr. Rockman-Greenberg.

The most common treatment-related adverse events (AEs) were mild-to-moderate injection site reactions (one severe) in all patients. There were no AEs leading to withdrawal, serious AEs, or deaths.

Improved Functional Mobility with Asfotase Alfa Treatment in Childhood Hypophosphatasia (Abstract MO0382)[2]

Katherine L. Madson, M.D., Ph.D., from the Center for Metabolic Bone Disease and Molecular Research at Shriners Hospitals for Children in St. Louis, presented results from a functional mobility analysis that assessed gait, or walking ability, in patients aged 5 to 15 years with juvenile-onset HPP who were treated with Strensiq compared with historical control patients using videos of basic mobility. The analysis included eight patients treated with Strensiq in an ongoing, Phase 2, open-label study and six historical control patients enrolled in the non-interventional natural history study.[2] At baseline, 100 percent of both historical control and treated patients had gait disturbance.[2]

Gait was assessed from patient videos using the 12-point modified Performance-Oriented Mobility Assessment – Gait score (mPOMA-G) (12 = no impairments; lower scores indicate greater impairment). The primary endpoint was rate of change in mPOMA-G in treated patients compared to historical control patients. In the analysis, children with HPP treated with Strensiq had a statistically significant improvement in the rate of change per year in mPOMA-G scores compared with historical controls (2.51

vs. 0.33; p=0.0303). The improvements in mPOMA-G scores of patients with HPP treated with Strensiq were mainly due to improvements in step length and stance.[2]

“At baseline, all of the patients had difficulty walking, underscoring the severe impact HPP can have on children’s day-to-day lives,” said Dr. Madson. “In this analysis, we were pleased to see improved functional mobility for patients treated with Strensiq compared with untreated historical control patients, as reflected by significant improvements in key measures of walking ability.”

Additional Studies Presented at ASBMR

Researchers also presented the following data at ASBMR 2015:

- A dose/exposure-response simulation supporting a weekly dose of 6 mg/kg of Strensiq administered three or six times a week for patients with HPP.[9]
- Data supporting the clinical validity and reproducibility of the seven point Radiographic Global Impression of Change (RGI-C) scale in evaluating the bone health of infants and children with HPP.[10]
- A case literature review of HPP manifestations in adults with pediatric-onset HPP, which found that the most frequently reported systemic manifestations of HPP in adult patients were fractures, pain, and early tooth loss.[11] The case literature review also revealed that missed diagnosis of pediatric-onset HPP is common and highlights the need for increased recognition of HPP symptoms in childhood.[11]
- An overview of a new multinational, multicenter, observational, prospective HPP Registry Study.[12]

About Hypophosphatasia (HPP)

HPP is a genetic, chronic, progressive, and life-threatening ultra-rare metabolic disease characterized by low alkaline phosphatase (ALP) activity and defective bone mineralization that can lead to destruction and deformity of bones and other skeletal abnormalities, as well as systemic complications such as profound muscle weakness, seizures, pain, and respiratory failure leading to premature death in infants.[3,5-8]

HPP is caused by mutations in the gene encoding an enzyme known as tissue non-specific alkaline phosphatase (TNSALP).[3,5] The genetic deficiency in HPP can affect people of all ages.[3] HPP is traditionally classified by the age of the patient at the onset of symptoms of the disease, with infantile- and juvenile-onset HPP defined as manifestation of the first symptom prior to 18 years of age.

HPP can have devastating consequences for patients at any stage of life.[3] In a natural history study, infants who had their first symptom of HPP within the first 6 months of life had high mortality, with an overall mortality rate of 73% at 5 years.[13] In these patients, mortality is primarily due to respiratory failure.[3,8,14] In patients surviving to adolescence and adulthood, long-term clinical sequelae include recurrent and non-healing fractures, profound muscle weakness, debilitating pain, and the requirement for ambulatory assistive devices such as wheelchairs, wheeled walkers, and canes.[3,7]

About Strensiq™ (asfotase alfa)

Strensiq™ (asfotase alfa) is an innovative enzyme replacement therapy designed to address the underlying cause of HPP—a deficiency of TNSALP activity. By replacing the defective enzyme, treatment with Strensiq aims to prevent or reverse the mineralization defects of the skeleton, thereby preventing serious skeletal and systemic morbidity and premature death.

Strensiq is approved in Japan as a treatment for patients with HPP and in the European Union and Canada as a treatment for patients with pediatric-onset HPP. The U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy designation for Strensiq and accepted Alexion's Biologics License Application (BLA) for Priority Review.

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. Alexion is also establishing a premier global metabolic rare disease franchise, which includes Kanuma™ (sebelipase alfa) for patients with lysosomal acid lipase deficiency (LAL-D), and Strensiq™ (asfotase alfa) for patients with hypophosphatasia (HPP). In addition, Alexion is advancing the most robust rare disease pipeline in the biotech industry, with highly innovative product candidates in multiple therapeutic areas. As the global leader in complement inhibition, the Company is strengthening and broadening its portfolio of complement inhibitors across diverse platforms, including evaluating potential indications for Soliris in additional severe and ultra-rare disorders. This press release and further information about Alexion can be found at: www.alexion.com.

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Forward-Looking Statements

This news release contains forward-looking statements, including statements related to potential medical benefits of Strensiq™ (asfotase alfa) for hypophosphatasia (HPP). 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 Strensiq for HPP, delays in arranging satisfactory manufacturing capabilities and establishing commercial infrastructure for Strensiq for HPP, the possibility that results of clinical trials are not predictive of safety and efficacy results of Strensiq in broader or different patient populations, the risk that third party payors (including governmental agencies) will not reimburse for the use of Strensiq at acceptable rates or at all, the risk that estimates regarding the number of patients with Strensiq and observations regarding the natural history of patients with Strensiq are inaccurate, and a variety of other risks set forth from time to time in Alexion's filings with the 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 June 30, 2015. 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.

References

1. Rockman-Greenberg C, Madson KL, Reeves A, et al. Asfotase Alfa: Sustained Efficacy and Tolerability in Children with Hypophosphatasia Treated For 5 Years. Oral presented at the American Society for Bone and Mineral Research, Seattle, October 10. Abstract 1071.

2. Madson KL, Phillips D, Rockman-Greenberg C, et al. Improved Functional Mobility with Asfotase Alfa Treatment in Children with Hypophosphatasia. Poster presented at the American Society for Bone and Mineral Research, Seattle, October 12. Abstract MO0382.

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9. Pradhan RS, Gastonguay MR, Gao X, et al. Exposure-Response Modeling and Simulation to Support Evaluation of Efficacious and Safe Exposure and Dose Range for Asfotase Alfa in Patients with Hypophosphatasia. Poster presented at the American Society for Bone and Mineral Research, Seattle, October 11. Abstract SU0380.

10. Whyte MP, Fujita KP, Moseley S, et al. Validation of a Novel Scoring System, The Radiographic Global Impression of Change (RGI-C) Scale, For Assessing Skeletal Manifestations of Hypophosphatasia in Infants and Children. Poster presented at the American Society for Bone and Mineral Research, Seattle, October 12. Abstract MO059.

11. Sawyer EK, Andersen K. Manifestations of Hypophosphatasia in Adults with Pediatric-Onset of Symptoms: A Review of the Case Literature. Poster presented at the American Society for Bone and Mineral Research, Seattle, October 11. Abstract SU0381.

12. Seefried L, Hogler W, Langman C, et al. A Longitudinal, Prospective, Long-Term Registry of Patients with Hypophosphatasia. Poster presented at the American Society for Bone and Mineral Research, Seattle, October 10. Abstract SA0376.

13. Whyte MP, Leung E, Wilcox W, et al. Hypophosphatasia: a retrospective natural history study of the severe perinatal and infantile forms. Poster presented at the 2014 Pediatric Academic Societies and Asian Society for Pediatric Research Joint Meeting, Vancouver, B.C., Canada, May 5, 2014. Abstract 752416.

14. Whyte MP, Rockman-Greenberg C, Hofmann C, et al. Improved survival with asfotase alfa treatment in pediatric patients with hypophosphatasia at high risk of death. Poster presented at the American Society for Bone and Mineral Research (ASBMR) 2014 Annual Meeting, Houston, September 14, 2014. Abstract 1097.

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