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## **FDA Grants Breakthrough Therapy Designation to cPMP Replacement Therapy for Patients with Molybdenum Cofactor Deficiency (MoCD) Type A**

LAUSANNE, Switzerland--(BUSINESS WIRE)-- Alexion Pharma International Sàrl, a subsidiary of Alexion Pharmaceuticals, Inc. (Nasdaq:ALXN), today announced that the U.S. Food and Drug Administration (FDA) has granted a Breakthrough Therapy designation to cyclic pyranopterin monophosphate (cPMP, or ALXN1101), an enzyme co-factor replacement therapy for the treatment of patients with molybdenum cofactor deficiency (MoCD) type A, a severe and life-threatening, ultra-rare, genetic metabolic disorder that causes catastrophic and irreversible neurologic damage within the first weeks of life.

According to the FDA, a Breakthrough Therapy designation is designed to expedite the development of a drug to treat a serious or life-threatening disease when preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. The Breakthrough Therapy designation is part of the FDA Safety and Innovation Act (FDASIA) of 2012.<sup>1</sup>

"In designating cPMP as a Breakthrough Therapy, the FDA recognizes the life-threatening nature of MoCD Type A, the positive early clinical results associated with cPMP, and the lack of any effective treatment options for infants born with this devastating disease," said Martin Mackay, Ph.D., Executive Vice President, Global Head of R&D at Alexion. "ALXN1101 is an innovative approach to the treatment of MoCD Type A, as it targets an essential step in the pathophysiology of the disease and the underlying cause of the disease by replacing the naturally occurring cPMP molecule, which is lacking in patients with MoCD Type A."

Alexion looks forward to working closely with the FDA and obtaining FDA guidance on the subsequent development of ALXN1101 for the treatment of patients with MoCD Type A, including obtaining advice on generating evidence needed to support approval of the drug in an efficient manner. Alexion has initiated a natural history study in patients with MoCD Type A and has also completed dosing with the synthetic cPMP in a study in healthy volunteers.

### **About Molybdenum Cofactor Deficiency (MoCD) Type A**

MoCD Type A is a severe, ultra-rare and genetic metabolic disease affecting newborns in which a genetic deficiency of cPMP results in the inability of the body to form an essential cofactor called molybdenum cofactor, resulting in its absence. This cofactor is essential for the appropriate functioning of several critical metabolic enzymes. A deficiency or absence of this cofactor leads to accumulation of toxic molecules, including the neurotoxin sulfite. Clinically, the absence of this cofactor and the resulting build-up of sulfite in the brain lead to damage and destruction of nerve cells, brain swelling, uncontrollable seizures, catastrophic and irreversible brain damage, and ultimately, death.<sup>2</sup> There are currently no treatment options for patients with MoCD Type A.

### **About cPMP/ALXN1101**

ALXN1101 is a synthetic version of cPMP and is designed to replace the naturally occurring cPMP lacking in infants with MoCD Type A. ALXN1101 restores the deficient enzyme activity which then causes clearance of the toxic metabolite sulfite thereby preventing the irreversible neurologic damage observed in untreated patients with MoCD Type A. Encouraging early clinical experience with an earlier form of cPMP replacement therapy has been reported by independent investigators in Germany and Australia.<sup>3</sup>

### **About Alexion**

Alexion Pharmaceuticals, Inc. is a biopharmaceutical company focused on serving patients with severe and ultra-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<sup>®</sup> (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 Pharmaceuticals, Inc. can be found at: [www.alexionpharma.com](http://www.alexionpharma.com).

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## Safe Harbor Statement

*This news release contains forward-looking statements, including statements related to potential medical benefits of cPMP/ALXN1101 for molybdenum cofactor deficiency (MoCD) Type A. 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 cPMP/ALXN1101 for MoCD Type A, delays in arranging satisfactory manufacturing capabilities and establishing commercial infrastructure for cPMP/ALXN1101 for MoCD Type A, the possibility that results of clinical trials are not predictive of safety and efficacy results of cPMP/ALXN1101 in broader or different patient populations, the risk that third party payors (including governmental agencies) will not reimburse for the use of cPMP/ALXN1101 (if approved) at acceptable rates or at all, the risk that estimates regarding the number of patients with cPMP/ALXN1101 and observations regarding the natural history of patients with cPMP/ALXN1101 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, 2013. 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. Public Law 112-144. U.S. Government Printing Office, July 9, 2012. <http://www.gpo.gov/fdsys/pkg/PLAW-112publ144/pdf/PLAW-112publ144.pdf>.
2. Vijayakumar\_Pediatric Neurology\_45\_2011; Per, Brain Dev.2005; Serrano, Pediatr Radiol, 2007
3. Veldman A, Santamaria-Araujo JA, Sollazzo S, et al. Successful treatment of molybdenum cofactor deficiency type A with cPMP. Pediatrics. 2010;125(5):e1249-54.

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