# Alexion Acquires Investigational Therapy for Infants Suffering From Catastrophic, Ultra-Rare Genetic Neurologic Disorder

### Metabolic Replacement Therapy Designed to Prevent Severe Brain Damage, Early Mortality in Infants with Molybdenum Cofactor Deficiency (MoCD) Type A

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) announced that it has purchased patents and assets from Germany-based Orphatec Pharmaceuticals GmbH related to an investigational therapy for patients with molybdenum cofactor deficiency (MoCD) Type A, a devastating ultra-rare genetic disorder characterized by severe brain damage and rapid death in newborns. Orphatec is a privately held development-stage biotechnology company with headquarters in Cologne, Germany. In addition, Alexion has established a research collaboration with key MoCD researchers from Orphatec to accelerate development of the investigational therapy.

**About MoCD Type A**

MoCD Type A is an ultra-rare metabolic disease affecting newborns in which a genetic deficiency of cPMP causes a deficiency of molybdenum cofactor which in turn leads to catastrophic brain damage, with survival generally measured in weeks or months. Deficiency of the cofactor leads to accumulation of neurotoxic sulfite, resulting in uncontrollable seizures, severe and rapid neurological damage, and death. There are currently no treatment options for patients with MoCD Type A.

**About the cPMP Replacement Therapy**

The investigational therapy is designed to replace the deficient cPMP, which enables MoCD production so that the infant's body can eliminate the toxic sulfite. Scientific discoveries underlying this highly innovative therapy were pioneered in Germany, and have led to encouraging early clinical experience with cPMP replacement therapy in several newborns. Investigators in Germany and Australia have reported clinically meaningful results in the first patient treated. (1)

"An essential part of Alexion's mission is to employ our skills in drug development and delivery on behalf of patients with ultra-rare and severe disorders who have no other hope. Our goal with this acquisition is to provide a first-in-class, life-transforming treatment for newborns and their families devastated by MoCD Type A," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "While development of a therapy for this type of very rare neonatal disorder involves significant commitment and risk, we are dedicated to driving forward expeditiously to investigate the potential of this innovative metabolic therapy."

"We appreciate Alexion's commitment to infants with MoCD Type A who currently have no chance of survival," said Guenter Schwarz, Ph.D., Professor and Chair in Biochemistry, Department of Chemistry & Center for Molecular Medicine Cologne, Germany, and a leader of the original Orphatec MoCD team. "Alexion's proven expertise in developing drugs for patients suffering from ultra-rare disorders now brings meaningful hope that the work begun by the Orphatec team can result in the first-ever approved treatment for these most helpless of patients and their families."

The assets were purchased with an upfront cash payment of approximately $3 million plus contingent payments which would be earned upon reaching various development, regulatory, and commercial milestones.

**About Alexion**

Alexion Pharmaceuticals, Inc. is a biopharmaceutical company working to develop and deliver life-changing drug therapies for patients with serious and life-threatening medical conditions. Alexion is engaged in the discovery, development and commercialization of therapeutic products aimed at treating patients with a wide array of severe disease states, including hematologic and kidney diseases, neurologic disorders, ophthalmic, transplant, other inflammatory disorders, and cancer. Soliris® (eculizumab), Alexion's first marketed product, is approved in more than 35 countries as a therapy for patients with PNH, a debilitating and ultra-rare life-threatening blood disorder. Alexion is evaluating other potential indications for Soliris and is pursuing development of other innovative biotechnology product candidates in early stages of development. This press release and further information about Alexion Pharmaceuticals, Inc. can be found at: www.alexionpharma.com.

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**Safe Harbor Statement**

*This news release contains forward-looking statements, including statements related to potential benefits from the acquired cPMP replacement therapy assets. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including for example those development, manufacturing, regulatory, commercialization and 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 September 30, 2010, and in Alexion's other filings with the 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.*

References:

1. Veldman A, et al. Successful treatment of molybdenum cofactor deficiency type A with cPMP *Pediatrics*. 2010 May;125 (5):e1249-54.

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