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Alexion Joins Patient Organizations Worldwide in Recognizing Rare Disease Day 2014

- Global Effort Reinforces Need for Education, Improved Diagnosis and Treatment of Rare Diseases -

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals (Nasdaq:ALXN) joins the European Organization for Rare Diseases (EURORDIS), the National Organization for Rare Disorders (NORD) and patient organizations worldwide in recognizing Rare Disease Day 2014, a global effort to increase awareness of rare diseases, their profound impact on patients and the need for improved diagnosis and treatment.

Alexion supports key objectives of EURORDIS, NORD and other patient organizations through its mission to develop and deliver life-transforming therapies for patients worldwide who suffer from severe and life-threatening rare diseases.

Diagnosis and Treatment Challenges for Rare and Ultra-Rare Diseases

Many rare and ultra-rare diseases are chronic, progressive and marked by continuing pain, severe disability and high mortality. Few physicians are familiar with diagnosing and treating these illnesses, which frequently leads to missed, delayed or inaccurate diagnoses even when an approved, effective therapy is available.¹ According to global survey results published in the current issue of *The Journal of Rare Disorders (JRD)*, conducted on behalf of Global Genes, patients living with rare diseases visited an average of 7.3 physicians before receiving an accurate diagnosis.² The survey also reveals that 44 percent of patients surveyed believed that a slow diagnosis resulted in delayed treatment with a negative impact on their condition.²

"Patients with rare and ultra-rare life-threatening disorders often face long delays in receiving an accurate diagnosis. Without an accurate diagnosis, or access to effective treatment options, patients too often are at risk for catastrophic medical consequences," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "On this Rare Disease Day and every day, through our continued commitment to breakthrough medical research, each of us at Alexion are focused on improving the knowledge of rare diseases and developing and delivering therapies to transform the lives of these patients worldwide."

Delivering Life-Transforming Therapies Across the Globe

Alexion's research and development programs are focused on highly innovative therapies with the potential to transform the lives of patients with severe and life-threatening ultra-rare disorders and for which there are no effective treatment options. The company's development programs are solely focused on:

- Severe disorders with devastating and life-threatening medical consequences
- Disorders with ineffective, or no treatment options
- Disorders that are ultra-rare and affect very small numbers of patients

Scientists at Alexion are working relentlessly to understand the underlying causes of these diseases and to discover and develop breakthrough medicines to treat them. To learn more about Alexion's Research & Development programs, visit www.alexionpharma.com/pipeline.

To learn more about Rare Disease Day, visit www.rarediseaseday.us for U.S. activities and www.rarediseaseday.org for global activities.

About Rare and Ultra-Rare Disorders

In the United States, a disease is defined as rare if it affects fewer than 650 patients per million of population.³ The European Union definition of a rare disease is one that affects fewer than five patients per 10,000 of population.⁴ In contrast, a disease is generally considered to be ultra-rare if it affects fewer than 20 patients per million of population⁵ (one patient per 50,000) - and most ultra-rare diseases affect far fewer people than this.

Despite the very small numbers of patients they affect, the impact of these rare and ultra-rare diseases on patients, their families, and society is profound, as many of these conditions are severe, chronic and progressive, with significant premature

mortality. Patients with severe and life-threatening ultra-rare diseases often live without hope, have no effective treatment options and may face premature death.

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 a treatment for patients with PNH and aHUS, two debilitating, ultra-rare and life-threatening disorders caused by chronic uncontrolled complement activation. This press release and further information about Alexion can be found at: www.alexionpharma.com.

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