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## Alexion Joins Patient Organizations Worldwide in Support of International Rare Disease Day 2015

*Company will participate in events across the globe to raise awareness of the need for improved diagnosis and treatment for patients with rare diseases*

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (Nasdaq:ALXN) joins the European Organization for Rare Diseases (EURORDIS), the National Organization for Rare Disorders (NORD) and rare disease patient and healthcare advocates around the world in recognizing and supporting Rare Disease Day 2015. Rare Disease Day, celebrated annually on the last day of February, is dedicated to elevating public understanding of rare diseases and calling attention to the special challenges faced by patients and the community, including the need for improved diagnosis and treatment.

The theme of this year's Rare Disease Day, "Living with a Rare Disease," focuses on the daily lives of the patients, families and caregivers who are impacted by rare diseases and pays tribute to the millions of family members and friends who are living "day-by-day, hand-in-hand" with rare disease patients. Alexion supports these key objectives of EURORDIS and NORD every day through its mission to develop and deliver life-transforming therapies for patients worldwide who live with severe and life-threatening rare diseases.

"On this eighth annual Rare Disease Day, and every day, we pay tribute to patients and families living with rare diseases, as we recognize the challenges they continue to face, including receiving an accurate and timely diagnosis and accessing effective treatment options," said Leonard Bell, M.D., Chairman and Chief Executive Officer of Alexion. "At Alexion, our deep commitment to patients, families and caregivers drives us to work harder every day to overcome these obstacles through disease education efforts and by partnering with governments worldwide to ensure patients have access to life-transforming therapies."

As a part of its support for Rare Disease Day, Alexion is engaging in a range of initiatives spanning across North America, Europe, Latin America, Asia and Australia to raise awareness of rare diseases, elevate rare diseases as an important public health issue and highlight the impact of rare diseases on patients and their loved ones' lives. These activities include educational efforts involving policymakers and medical professionals, partnerships with local organizations and participation in local Rare Disease Day-related events.

### North America

- Alexion is a sponsor and participant in the Rare Disease Legislative Advocates (RDLA) 4<sup>th</sup> Annual Rare Disease Week on Capitol Hill in Washington, D.C. and will also participate in briefing legislators on rare diseases in multiple states.
- Alexion employees will address the various aspects of improving patient outcomes and orphan drug development through participation in several symposia and panel forums taking place in settings that include the Massachusetts Biotechnology Council, the Canadian Rare Disease Day Conference, the Massachusetts State House, the University of Connecticut School of Pharmacy, the University of Utah, and a Google "Hang Out."
- Alexion is a Silver Sponsor of the Rare Disease United Foundation's "Beyond the Diagnosis" Art Exhibit at the Brown University Alpert Medical School in Providence, RI (through the end of February).
- Connecticut-based employees will host a session for students about rare diseases at the Celentano Biotech, Health and Medical Magnet School (K-8) in New Haven.
- Alexion is a corporate sponsor and the 2015 Rare Disease Leadership Award recipient at the Canadian Organization for Rare Disorders (CORD) Rarity Awards Dinner Gala (March 5).
- Alexion is a Gold Sponsor of the Utah World Rare Disease Day Statehouse Event at the Utah State Capitol Building Rotunda (Feb. 27, 3-5 p.m.).

### Europe

- Alexion is participating in parliamentary events from Belgium to Sweden that include a EURORDIS policy briefing on rare disease for EU policymakers.
- Alexion is a sponsor of the EURORDIS Annual Fund Raising Gala Dinner in Brussels, Belgium
- In France, Alexion is supporting a local walk to raise funds for local patient organizations.

- In Finland, Alexion is supporting the production of a Rare Disease Day supplement in Helsingin Sanomat, the largest subscription newspaper in Finland and the Nordic countries, in order to increase awareness of rare diseases.
- In Russia, Alexion is supporting the Congress on Rare Diseases, led by rare disease patient organizations, the National Association of Patients with Rare Diseases "GENETICA," and the Russian Association of Rare Diseases. This is the first rare disease congress with participation of almost all of Russia's patient organizations. Additionally, Alexion is supporting the Annual National Award "Blue Bird," which recognizes individuals who have made a significant contribution to the care of patients with rare diseases.

## Latin America

- In Argentina, Alexion is sponsoring the Federación Argentina de Enfermedades Poco Frecuentes (FADEPOF) local events and social "Connectivity and Union" campaign to address the needs of patients with rare diseases.
- Alexion is sponsoring the Federación Colombiana de Enfermedades Raras (FECOER) legislative events in Colombia to commemorate the fifth anniversary of the approval of the Colombian Rare Disease Law and empower advocates to take action for those affected by rare diseases.
- Alexion is sponsoring the Organización Mexicana de Enfermedades Raras (OMER) Rare Disease Day Summit in Guadalajara, Mexico which will feature a series of workshops, exhibits and informative sessions about rare diseases.

## Australia

- Alexion is supporting Rare Voices Australia as a sponsor of the Rare Disease Summit in Melbourne, which aims to drive collaboration among stakeholders to address the common needs and concerns of people living with a rare disease.

In addition to these global initiatives to support the rare disease community, scientists at Alexion are working to understand the underlying causes of rare diseases and to discover and develop breakthrough medicines to treat them. Alexion's development programs include several highly innovative therapeutic candidates, which are being investigated in severe and rare disorders across the areas of hematology, nephrology, transplant, neurology, metabolic disorders and inflammatory disorders. To learn more about Alexion's Research & Development programs, visit [www.alexion.com/pipeline](http://www.alexion.com/pipeline).

For more information about Rare Disease Day in the U.S., go to [www.rarediseaseday.us](http://www.rarediseaseday.us), and for information about global Rare Disease Day activities, go to [www.rarediseaseday.org](http://www.rarediseaseday.org).

## 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.<sup>1</sup> The European Union definition of a rare disease is one that affects fewer than five patients per 10,000 of population.<sup>2</sup> In contrast, a disease is generally considered to be ultra-rare if it affects fewer than 20 patients per million of population<sup>3</sup> (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 paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (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.alexion.com](http://www.alexion.com).

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## References:

1. U.S. Food and Drug Administration. Definition of Disease Prevalence for Therapies Qualifying Under Orphan Drug Act: <http://www.fda.gov/ForConsumers/ConsumerUpdates/ucm135130.htm>

2. REGULATION (EC) No 141/2000 OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL of 16 December 1999 on

orphan medicinal products. <http://eur-lex.europa.eu/legal-content/EN/TXT/PDF/?uri=CELEX:32000R0141&qid=1421232987002&from=EN>

3. REGULATION (EU) No 536/2014 OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL of 16 April 2014 on clinical trials on medicinal products for human use, and repealing Directive 2001/20/EC. <http://eur-lex.europa.eu/legal-content/EN/TXT/PDF/?uri=CELEX:32014R0536&qid=1421232837997&from=EN>

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