



February 29, 2016

## Alexion Employees Across the Globe Join Together to Support Rare Disease Day 2016

### *- New Global Headquarters Dedicated to Rare Disease Patient Community -*

NEW HAVEN, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (Nasdaq:ALXN) joins the worldwide rare disease community in honoring the ninth annual international Rare Disease Day. Rare Disease Day takes place the last day of February each year and is dedicated to raising awareness of rare diseases and their impact on patients and families. Alexion employees will participate in events around the globe to help raise awareness of rare diseases as a critical global health issue requiring ongoing research, public policy, disease education and new treatments.

This year's global theme, "Patient Voice," highlights the need for greater understanding of the experiences of rare disease patients and calls attention to the unique challenges faced by this community. Alexion supports the key objectives of Rare Disease Day 2016 through its mission to develop and deliver life-transforming therapies for patients around the world living with devastating and rare diseases.

"Today serves as a special milestone for Alexion as we celebrate Rare Disease Day by dedicating our new global headquarters in New Haven to the patients who inspire us every day to work with a sense of urgency to deliver medical breakthroughs where none currently exist," said David Hallal, Chief Executive Officer of Alexion. "Alexion's 3,000 employees around the world will participate in activities that demonstrate our commitment to transform lives and create lasting impact for the patients and families we serve today, and the generations that follow."

Alexion's Rare Disease Day 2016 activities include:

#### **North America**

- | Today, Alexion will dedicate its new global headquarters in New Haven, CT, to rare disease patients worldwide in an official ribbon-cutting ceremony with government officials, community partners, and patients and their families.
- | Alexion is a sponsor and participant in the Rare Disease Legislative Advocates (RDLA) 5<sup>th</sup> Annual Rare Disease Week on Capitol Hill in Washington, D.C., and will participate in briefing legislators on rare diseases in multiple states.
- | Alexion will attend MASSBIO Rare Disease Day at the State House in Boston, MA, coordinated by the National Organization for Rare Disorders (NORD), which includes patient organizations, professional societies, government agencies, medical researchers, life sciences companies, and patients.
- | Alexion is a sponsor of NORD's National Rare Disease Day Activities, which include local State House events, such as medical grand rounds, university and medical school events, and K-12 school events throughout Connecticut.
- | Alexion is a Platinum sponsor of the 2<sup>nd</sup> Annual Utah Rare Disease Day Symposium (February 27) and the Utah World Rare Disease Day State House Event, "Utah Rare Dinner" (February 29).
- | Alexion is a sponsor of the Canadian Organization for Rare Disorders (CORD)'s 2016 Rare Disease Day activities (March 9-10), which include the launch of the Rare Disease Strategy, updates on rare disease research, and training for rare disease patient groups. Alexion is the Action Hero Sponsor of CORD's Rare Disease Day Gala, the Leadership Sponsor of its Rare Disease Day Conference, and a Founding Partner and Premier Sponsor of "Rare Alliance Canada," which launched today in Ottawa.

#### **Europe**

- | Alexion will attend the Rare Disease UK (RDUK) Parliamentary reception at the House of Commons in London, England (March 3).
- | The Company is a corporate sponsor of the Genetic and Rare Disease Organization (GRDO)'s 2016 Rare Disease Day Conference, held today at Dublin Castle in Ireland. The event will bring together representatives from patient organizations, politicians, caretakers, medical professionals, researchers, and members of the industry.
- | In Spain, Alexion is supporting the Federación Española De Enfermedades Raras (FEDER)'s "Creating Networks of Hope," a national event held at the Superior Council for Scientific Research in Madrid (March 4). In addition, Alexion is supporting the Plataforma Malalties Minoritaries' "All Together for Rare Diseases" meeting at the Hospital Clínic de

Barcelona to support rare disease patient associations in Catalonia.

- | Alexion will support initiatives driven by Denmark's rare disease organization, Sjældne Diagnoser.
- | In Sweden, Alexion will join members of Parliament, county councils, and administrators in support of Sweden's rare disease organization, Sällsynta Diagnoser.

## Latin America

- | In Colombia, Alexion will participate in events organized by Asociación Colombiana de Pacientes con Enfermedades de Depósito Lisosomal (ACOPEL) and Fundación de Apoyo Solidario a Pacientes con Enfermedades Raras (FUNDAPER) that will bring together artists and government officials to support rare disease patients in the country.

## Australia

- | Alexion will participate in rare disease awareness events at local hospitals throughout Australia and distribute educational resources to further spread awareness about rare diseases.

## Asia

- | In China, Alexion is a sponsor of the Chinese Organization for Rare Disorder's Patient Care Video Release Ceremony being held in Beijing (February 29) as well as the Shanghai Rare Disease Medical Association's 2016 Rare Disease Day.

Today, Alexion has three innovative therapies to serve patients with four devastating ultra-rare diseases and we are partnering with governments worldwide to ensure patients have access to our life-transforming therapies. Throughout the year, Alexion's research and development teams dedicate themselves to understanding the underlying causes of rare diseases and discovering breakthrough therapies to treat them. Alexion is currently advancing the most robust rare disease pipeline in the biotech industry, which includes 10 clinical programs and 30 pre-clinical programs, with at least four moving into the clinic in 2016. To learn more about Alexion's R&D 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 more information about global Rare Disease Day activities, visit [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 the population.<sup>1</sup> The European Union definition of a rare disease is one that affects fewer than five patients per 10,000 of the population.<sup>2</sup> In contrast, a disease is generally considered ultra-rare if it affects fewer than 20 patients per million of the 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 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 and have no effective treatments.

## About Alexion

Alexion is a global biopharmaceutical company focused on developing and delivering life-transforming therapies for patients with devastating and rare disorders. 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 life-threatening ultra-rare disorders. In addition, Alexion's metabolic franchise includes two highly innovative enzyme replacement therapies for patients with life-threatening and ultra-rare disorders, hypophosphatasia (HPP) and lysosomal acid lipase deficiency (LAL-D). In addition, Alexion is advancing the most robust rare disease pipeline in the biotech industry with highly innovative product candidates in multiple therapeutic areas. 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/ucm387513.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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