# Alexion Pharmaceuticals and Minoryx Therapeutics to Present at SMi's Orphan Drugs & Rare Diseases Event

*SMi Reports: Alexion Pharmaceuticals and Minoryx Therapeutics to deliver presentations at the 9th Annual Orphan Drugs and Rare Diseases Conference*

Orphan Drugs & Rare Diseases 2019

LONDON, UNITED KINGDOM, June 27, 2019 /EINPresswire.com/ -- The orphan drugs market recently saw an explosion of interest from pharmaceutical companies and biotechs, as a need for effective, long-term treatments for rare disease patients continues to be a priority.

This year's Orphan Drugs and Rare Diseases Conference, returning for its 9th year to London this October, will tackle the main challenges facing the industry, and look at how commercialisation and patient access can be advanced.

Recent success stories demonstrate the impressive progress made by the industry to tackle the difficult mission of commercialising drugs that rare disease patients desperately need. Here are a few recent achievements made by some of the companies speaking at the 2019 conference:

This year's top 10 drug launches featured many rare disease treatments, with Alexion Pharmaceuticals' Soliris follow-up, Ultomiris, topping the list at number one, which received an early FDA approval ahead of its scheduled decision date (source: Fierce Pharma).

Executive Director, Portfolio Products of Alexion Pharmaceuticals, Michael Page, will be chairing day one of the conference as well as presenting on an "Overview of Current Orphan Drug Regulation", focusing on:
- Discussion on current state regulation of orphan drugs, and the level of scientific rigour needed for orphan drug approval (accepting small patient group studies and short studies)
- Assessing the importance of clinical evidence during the launch of an orphan drug, considering the difficulty in planning and executing clinical trials for rare/ultra-rare diseases
- Synopsis of the evidence requirements before and after the registration and open access to a new drugs

Meanwhile, Minoryx Therapeutics, a clinical stage biotech company leading the development of new therapies for rare diseases, announced its first patient dosed in the FRAMES phase 2 trial in Friedreich's Ataxia (Source: Biospace).

Based on preclinical studies, the drug showed impressive efficacy, increasing neuron survival, improving mitochondrial function and biogenesis, and restoring energy production.

Chief Regulatory Office of Minoryx, Maria Pascual, will deliver a high-level presentation on "Technology and Orphan Drug Development" at this year's conference, discussing:
•Experiences and perspective on the various challenges in the development of orphan drugs and the available facilitating and accelerating toolbox
•Analysing the challenges associated with the data sharing and analysis for rare disease research
•Changes in market prospects for rare diseases following the improvement in efficiency of drug development

Alexion and Minoryx are paving the way to success by repurposing existing drugs in order to treat rare diseases, which means fewer complex trials, less expenditure, and a quicker timeline.

Hear from an unparalleled expert speaker line-up at this year's Orphan Drugs and Rare Diseases Conference. The brochure with full speaker line up is available to download online at http://www.orphandrugs.co.uk/einpr3

Plus, attendees will have the benefit of being able to take advantage of 5+ hours of dedicated networking with solution providers, biotechnology companies, clinical researchers, regulatory professionals and charity leaders.

There is an early bird saving of £300 for conference bookings made before Friday 28th June. Register online at http://www.orphandrugs.co.uk/einpr3

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Orphan Drugs and Rare Diseases 2019
Conference: 15th – 16th October 2019
Workshop: 17th October 2019
London, UK
http://www.orphandrugs.co.uk/einpr3
#SMiOrphanDrugs

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