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## **Retrophin Recognizes Rare Disease Day<sup>®</sup> 2018 and Joins Others Worldwide to Raise Awareness About Rare Diseases**

**SAN DIEGO (February 28, 2018)** – Retrophin, Inc. (NASDAQ: RTRX) today joins the National Organization for Rare Disorders (NORD), the European Organisation for Rare Diseases (EURORDIS), and rare disease advocates and patients worldwide to recognize Rare Disease Day<sup>®</sup> 2018. This year's theme, "Research," recognizes the critical role medical research plays in the identification, diagnosis, and development of treatments and cures for rare diseases. Retrophin is honored to support the researchers whose expertise and perseverance help give hope to the estimated 350 million<sup>i</sup> people worldwide living with a rare disease.

"We believe that the approach to rare disease research, diagnosis and treatment has to be comprehensive," said Stephen Aselage, chief executive officer of Retrophin. "On this Rare Disease Day, we are grateful to the patients and their families who generously share their experiences with us so we can better understand their unmet needs, and to our partners – physicians, scientists, advocates and many others – for working with us to find real solutions."

As part of its dedication to the rare disease community, Retrophin made a philanthropic commitment to support the work of the Children's National Rare Disease Institute (CNARDI) through the creation of the [Retrophin Rare Disease Network](#) at Children's National Health System in Washington, DC. The innovative, global "hub and spoke" model utilized by CNARDI aims to improve patient access, standardize care models, and establish worldwide best practices in the diagnosis and treatment of rare diseases.

Rare diseases are defined as those that affect fewer than 200,000 people in the United States<sup>ii</sup>, and fewer than 1 in 2,000 people in the EU.<sup>iii</sup> Roughly 80 percent of rare diseases are genetic, with approximately half<sup>iv</sup> affecting children. Rare Disease Day, which is held on the last day of February every year, seeks to build public awareness about rare diseases and their impact on people's lives. It is led internationally by EURORDIS and in the U.S. by NORD. For information on Rare Disease Day participation, visit [rarediseaseday.us](http://rarediseaseday.us).

### **About EURORDIS**

EURORDIS is a non-governmental patient-driven alliance of 606 rare disease patient organizations in 56 countries. EURORDIS strives to build a strong pan-European community of patient organizations and people living with rare diseases, to be their voice at the European level, and - directly or indirectly - to fight against the impact of rare diseases on their lives. EURORDIS leads global efforts for Rare Disease Day, which it established in 2008. The observance is celebrated around the world on the last day of February each year with the goal of raising awareness of rare diseases as a public health issue. For more information about EURORDIS, visit [eurordis.org](http://eurordis.org).

## About NORD

Established in 1983, the National Organization for Rare Disorders (NORD) is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. A nonprofit organization, NORD represents the 30 million Americans with rare diseases and is committed to the identification, treatment and cure of rare disorders through patient assistance, education, advocacy, research and patient/family services. For more information about NORD, visit [rarediseases.org](http://rarediseases.org).

## About Retrophin

Retrophin is a biopharmaceutical company specializing in identifying, developing and delivering life-changing therapies to people living with rare diseases. The Company's approach centers on its pipeline featuring late-stage assets targeting rare diseases with significant unmet medical needs, including fosmetpantotenate for pantothenate kinase-associated neurodegeneration (PKAN), a life-threatening neurological disorder that typically begins in early childhood, and sparsentan for focal segmental glomerulosclerosis (FSGS) and IgA nephropathy (IgAN), disorders characterized by progressive scarring of the kidney often leading to end-stage renal disease and glomerulonephritis, respectively. Research in additional rare diseases is also underway, including a joint development arrangement evaluating the potential of CNSA-001 in phenylketonuria (PKU), a rare genetic metabolic condition that can lead to neurological and behavioral impairment. Retrophin's R&D efforts are supported by revenues from the Company's commercial products Chenodal<sup>®</sup>, Cholbam<sup>®</sup> and Thiola<sup>®</sup>.

[Retrophin.com](http://Retrophin.com)

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<sup>i</sup> Global Genes Web Site. <https://globalgenes.org/rare-diseases-facts-statistics/>. Last accessed February 20, 2018.

<sup>ii</sup> National Institutes of Health Web Site. <https://rarediseases.info.nih.gov/diseases>. Last accessed February 20, 2018

<sup>iii</sup> Rare Disease Day Web Site. <https://www.rarediseaseday.org/article/what-is-a-rare-disease>. Last accessed February 20, 2018

<sup>iv</sup> Global Genes Web Site <https://globalgenes.org/rare-diseases-facts-statistics/>. Last accessed February 20, 2018