

## Retrophin Gift to Establish Rare Disease Network at Children's National

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**The gift will support operations at the Children's National Rare Disease Institute and promote the further research and study of rare diseases.**

WASHINGTON, D.C. & SAN DIEGO — Children's National Health System and Retrophin, Inc. (NASDAQ: RTRX) are pleased to announce the creation of the Retrophin Rare Disease Network at Children's National. Retrophin, a biopharmaceutical company specializing in identifying, developing and delivering life-changing therapies to people living with rare diseases, has committed \$3 million over the next six years to support the work of the Children's National Rare Disease Institute (CNRDI).

"One of the chief challenges of 21st century pediatric medicine is our continued inability to provide more help to those born with rare genetic diseases," said Marshall Summar, M.D., director of the CNRDI. "We are grateful for this gift from Retrophin that will help us accelerate progress for our patients and families and pursue work that will have a far-reaching impact on both children and adults across the country and around the world. This is an exciting first step toward a new era of rare disease care and innovation."

As a dedicated source of funding, the Retrophin Rare Disease Network will advance the CNRDI's efforts to create a global "hub and spoke" model for disseminating and streamlining patient access to optimal care methods among national and international peer institutions. The network is designed to enhance the field of rare disease medicine by standardizing care models and establishing world-wide best practices in diagnosis and treatment.

"Retrophin is proud to support the important work that Children's National Rare Disease Institute is doing to advance the care and treatment of rare diseases," said Stephen Aselage, chief executive officer of Retrophin. "The tremendous difficulty that patients with rare diseases have in getting an accurate diagnosis and early treatment is well documented. The creation of this network is critical to broadening access to the Institute's innovative resources and programs, and will ultimately lead to expanded specialized care for patients and families in the rare disease community."

The Retrophin Rare Disease Network will provide funding for new dedicated positions at the CNRDI and build on the Institute's existing digital and telemedicine programs, to extend the reach of its researchers and caregivers in areas where there is currently limited care available for children and adults living with rare diseases.

The CNRDI is a first-of-its-kind center focused exclusively on advancing the care and treatment of children and adults with rare genetic diseases. It is the first National Organization for Rare Disorders (NORD) Center of Excellence and aims to provide a medical home for patients and families seeking the most advanced care and expertise for rare genetic conditions that remain largely unknown to the general medical community.

According to the National Institutes of Health (NIH), any disease, illness or disorder that affects fewer than 200,000 people in the United States is considered to be rare. Currently, it is estimated that one in 10 Americans has a rare disease – approximately 80 percent of which are genetically based. Additionally, the NIH reports that more than 80 percent are childhood diseases, and more than 25 percent of children admitted to pediatric hospitals have a rare disease. Yet, of the more than 7,000 rare diseases known to exist, fewer than 500 (roughly five percent) have a known treatment approved by the U.S. Food and Drug Administration (FDA).

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### About Children's National Health System

Children's National Health System, based in Washington, D.C., has been serving the nation's children since 1870. Children's National is [#1 for babies](#) and ranked in every specialty evaluated by *U.S. News & World Report* including placement in the top 10 for: [Cancer](#) (#7), [Neurology](#) and [Neurosurgery](#) (#9), [Orthopaedics](#) (#9) and [Nephrology](#) (#10). Children's National has been designated two times as a Magnet® hospital, a designation given to hospitals that demonstrate the highest standards of nursing and patient care delivery. This pediatric academic health system offers expert care through a convenient, community-based primary care network and specialty outpatient centers. Home to the Children's Research Institute and the Sheikh Zayed Institute for Pediatric Surgical Innovation, Children's National is one of the nation's top NIH-funded pediatric institutions. Children's National is recognized for its expertise and innovation in pediatric care and as a strong voice for children through advocacy at the local, regional and national levels. For more information, visit [ChildrensNational.org](http://ChildrensNational.org), or follow us on [Facebook](#) and [Twitter](#).

### 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. Learn more at [Retrophin.com](http://Retrophin.com).

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