



## **Ultragenyx Announces Emil D. Kakkis, M.D., Ph.D. as Recipient of BIO's 2019 Henri A. Termeer Biotechnology Visionary Award**

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NOVATO, Calif., May 29, 2019 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development of novel products for serious rare and ultra-rare genetic diseases, today announced that the Biotechnology Innovation Organization (BIO) has awarded this year's Henri A. Termeer Biotechnology Visionary Award to Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer, President and founder of Ultragenyx.

The award, which will be presented to Dr. Kakkis on June 5 during the BIO 2019 International Convention keynote session, recognizes Dr. Kakkis' transformative work to accelerate research and development of novel treatments for rare diseases.

"Henri Termeer was a pioneer who worked relentlessly to do what many thought was impossible and bring therapies to patients with rare diseases," said Dr. Kakkis. "I am deeply honored to receive this award from BIO."

"Dr. Kakkis' innovative work has pushed the boundaries of our knowledge in rare diseases and through his efforts our industry better prioritizes treatments for rare and ultra-rare diseases and understands the unique needs of clinical trials in this category," said Jim Greenwood, President and CEO of BIO. "His contributions to the study of rare diseases have been monumental for patients and families, and we're looking forward to presenting him with this well-deserved honor at BIO 2019."

Over the last 25 years, Dr. Kakkis has spearheaded the development of many rare disease treatments, including an enzyme replacement therapy for the rare and debilitating disorder, mucopolysaccharidosis type I (MPS I), which was approved by the FDA in 2003. Dr. Kakkis has also guided the development and approval of two other treatments for rare disorders affecting infants and children with MPS VI and PKU, and has contributed to the initiation or development multiple other approved products for rare diseases. In 2009, Dr. Kakkis also founded the EveryLife Foundation for Rare Diseases to help drive the scientifically sound changes in policy, regulation, law and drug development strategies to accelerate the diagnosis and treatment of patients with rare diseases. The Foundation is celebrating its 10<sup>th</sup> year of advocating for rare disease patients.

In 2010, Dr. Kakkis founded Ultragenyx, to focus on rare metabolic, bone, muscle and neurologic diseases with limited treatment options. Since its founding, Ultragenyx has worked on developing treatments for twenty different genetic diseases and has now received commercial approvals for its first two products, Crysvida® for x-linked hypophosphatemia XLH and Mepsevii® for mucopolysaccharidosis VII (MPS VII). The company is being developed as a next-generation rare disease company with novel approaches to study design, statistical analysis, patient diagnosis, patient access, and long-term follow-up programs intended to advance the way rare disease treatments are developed and commercialized.

### **About BIO's Henri A. Termeer Biotechnology Visionary Award**

The Henri A. Termeer Biotechnology Visionary Award was established in 2018 in memory of the late Genzyme CEO. Mr. Termeer's contributions to the biotechnology industry were powerful and extensive, from the creation of Genzyme to his leadership in the orphan rare disease community. His impact originates from more than his business strategy; it was born of his perseverance, generosity, intellect and deep commitment to heal the world. The award is designed to recognize one individual annually who reflects Mr. Termeer's contributions and indelible legacy.

### **About BIO**

BIO is the world's largest trade association representing biotechnology companies, academic institutions, state biotechnology centers and related organizations across the United States and in more than 30 other nations. BIO members are involved in the research and development of innovative healthcare, agricultural, industrial and environmental biotechnology products. BIO also produces the [BIO International Convention](#), the world's largest gathering of the biotechnology industry, along with industry-leading investor and partnering meetings held around the world.

### **About Ultragenyx Pharmaceutical Inc.**

Ultragenyx is a biopharmaceutical company committed to bringing to patients novel products for the treatment of serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved therapies and product candidates aimed at addressing diseases with high unmet medical need and clear biology for treatment, for which there are typically no approved therapies treating the underlying disease.

The company is led by a management team experienced in the development and commercialization of rare disease therapeutics. Ultragenyx's strategy is predicated upon time and cost-efficient drug development, with the goal of delivering safe and effective therapies to patients with the utmost urgency.

For more information on Ultragenyx, please visit the Company's website at [www.ultragenyx.com](http://www.ultragenyx.com).

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