Emil Kakkis, MD, PhD
President & Founder
EveryLife Foundation for Rare Diseases

Dr. Kakkis is best known for his work over the last 20 years to develop novel treatments for rare disorders. He began his work with minimal funding to develop an enzyme replacement therapy (Aldurazyme®) for the rare disorder MPS I. The struggle to get the therapy translated from a successful canine model to patients succeeded due to the critical financial support of the Ryan Foundation. Kakkis’ collaboration with the Ryan Foundation in the early development of Aldurazyme was highlighted in a 60 Minutes II segment in April 2001 (“Saving Ryan”), and a Reader’s Digest article in May 2001. Aldurazyme development was later supported by BioMarin™ and eventually their partner Genzyme™, leading to FDA approval in 2003.

Dr. Kakkis joined BioMarin in 1998 to develop more treatments for rare diseases. During his 11 year tenure at BioMarin, ultimately as Chief Medical Officer, Dr. Kakkis guided the development and approval of two more treatments for rare disorders, Naglazyme for MPS VI and Kuvan for PKU, and has contributed to the initiation of seven other treatment programs for rare disorders, three of which are now in clinical development. Two of these programs, Morquio (MPS IV A) and a new product for PKU, have recently announced positive clinical data.

In 2009, Dr. Kakkis left industry and started the EveryLife Foundation for Rare Diseases to accelerate biotech innovation for rare diseases. He initiated the CureTheProcess Campaign to improve the regulatory process for rare disease treatments. The Campaign has been endorsed by 181 patient organizations and physician societies and gained the attention of Congress and the Food and Drug Administration. His efforts also earned him recognition as a 2010 “Rock Star of Science” by the Geoffrey Beene Foundation.

After two years solely dedicated to the Foundation, Dr. Kakkis launched Ultragenyx Pharmaceutical to advance his efforts to treat patients with a rare disease. Dr. Kakkis remains the President of the Foundation as it continues to be a leader in improving the FDA’s regulatory process, build the rare disease community, and advocate for policy changes that will spur the development of treatments.

Dr. Kakkis graduated from Pomona College, magna cum laude and received the Vaile Prize in Biology for his thesis research in 1982. He received combined MD and PhD degrees from the UCLA Medical Scientist Program in 1989 and received the Bogen prize for his research on c-myc oncogene regulation. He completed a Pediatrics residency at Harbor-UCLA Medical Center in Torrance, CA and completed his fellowship training there in the UCLA Intercampus Medical Genetics Training Program in 1993. He became an assistant professor of Pediatrics at Harbor-UCLA Medical Center from 1993-1998 where he initiated the enzyme therapy program for MPS I. He is board certified in both Pediatrics and Medical Genetics. He received the Lifetime Achievement Award from the National MPS Society for his work on Aldurazyme. He has
authored numerous scientific articles on MPS I, immune tolerance during enzyme therapy, intrathecal enzyme therapy and studies on treatments for MPS VI and PKU.