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**Foundation taps into JP Morgan Healthcare Conference Attendees to Accelerate Biotech Innovation for Rare Diseases**  
***FDA Regulatory Changes Are Needed to Spur Investment***

**January 10, 2010 (San Francisco, California)** — As thousands of investors, bankers, and biotech company executives pour into San Francisco for the Annual JP Morgan Healthcare Conference, the Kakkis EveryLife Foundation is hosting its inaugural Legends of Golf Tournament to bring attention to needed regulatory changes that will accelerate biotech innovation for rare diseases and spur investment in the industry.

The Golf Tournament is co-hosted by the Children’s Rare Disease Network, National MPS Society and the Ryan Foundation; and is sponsored by the Biotechnology Industry Organization, BioMarin, FoldRx, Michael Lee, RedMile Group and Adams Golf. Event sponsors along with golfers from BioMarin Pharmaceutical, Amicus Therapeutics, Davos Pharma, Collins Stewart, L.E.K Consulting, Visium Asset Management, BioMark Partners, Nile Therapeutics, Pioneer Path Capital and many others will enjoy 18 holes at the Presidio Golf Course. Following golf, there will be a presentation by Dr. Kakkis, wine tasting, dinner and some celebrity entertainment including Rodney Dangerfield and Tiger Woods (impersonators).

While the tournament will be entertaining, its goal will be to reach out to stakeholders in the investment and biotech community and show them a specific, near term solution to the current regulatory issues. Dr. Kakkis will highlight that there are currently more than 6,000 rare diseases with no approved therapy; and while almost 1,800 investigational therapies have entered the research pipeline since the Orphan Drug Act’s passage, only about 320 have been approved for marketing by the FDA in 25 years. Rare disorders together affect over 25 million Americans and their families; however few drug companies conduct research on rare diseases because it is difficult to recoup the costs of developing treatments for such small populations.

“When I started BioMarin with my co-founders, it was still possible to take good research information in animal models of a rare disease and get startup funding for a company based on the concept of a rapid development process,” says



John Klock, M.D., Co-founder of BioMarin and Board member of the Foundation. “Now it is very difficult to start a company on that information alone because the expectation for a predictable accelerated approval path does not exist any longer and so investors are instead focusing on lower risk startup companies making new versions of old drugs.”

However, the regulatory changes proposed by the Kakkis EveryLife Foundation’s campaign to CURETHEPROCESS would create a clear pathway for development, shortening timelines and reducing the financial risk associated with development of rare disease therapeutics. The result could be a surge in development activity for even the most rare disorders giving more patients with rare biochemical and genetic disorders earlier access to effective treatments. Additionally, increased investment in early stage biotech companies focused on rare diseases will have a positive impact in local communities and biotechnology jobs that are currently at risk.

The Campaign’s three goals are to:

1. **Establish a new Office of Drug Evaluation for Genetic and Biochemical Diseases** at the U.S. Food and Drug Administration (FDA), consolidating and expanding expertise to ensure safe, effective and timely patient access to needed treatment.
2. **Create a new standard to qualify biomarker or surrogate measures** of the effect for treatments of rare disorders, and allow these treatments full access to the accelerated approval pathway for life threatening diseases.
3. **Devise new clinical study designs and analyses for rare diseases** that account for disease complexity and patient variability to properly capture treatment effects on all aspects of the disease.

The Foundation has formed a Scientific Advisory Committee that is working on creating new science-driven regulatory policy. More than 70 patient organizations have endorsed the campaign, including the American Heart Association, National Organization for Rare Disorders (NORD), John F. & Aileen A. Crowley Foundation, Parent Project Muscular Dystrophy, United Pompe Foundation, National Niemann-Pick Disease Foundation, and National PKU Alliance. Find out more at [www.kakkis.org](http://www.kakkis.org) or about the campaign at [www.CURETHEPROCESS.org](http://www.CURETHEPROCESS.org).

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