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Former BioMarin exec targets orphan drugs

San Francisco Business Times - by [Ron Leuty](#)

Emil Kakkis loves orphans.

The former chief medical officer at **BioMarin Pharmaceutical Inc.** — the Novato-based poster child for developing treatments for so-called orphan drugs — is lining up cash and personnel for a new venture again aimed at rare diseases.

“When you see patients treated for the first time, you don’t forget that,” Kakkis said. “You want to do it over and over again.”

Whether Kakkis’ new company, **Ultragenyx** Pharmaceutical, can add to his string of successes depends largely on lining up financing. He hopes to have Series A funding of “substantially more” than \$10 million secured by April and 20 to 25 employees by June.

That may seem like a tough chore in a financing environment that has slowed venture investing in startup biotech companies. Orphan drug companies, however, are hot properties that some experts say can net relatively quick returns on investment for investors.

“VCs are always looking for the next big thing, and orphan drugs is really one of the next big things,” said Eric Nelson, business development lead for the **National Institutes of Health’s** Therapeutics for Rare and Neglected Diseases program. “Large pharma and specialty pharma are looking for big opportunities.”

Last year alone, 49 rare diseases were discovered, Nelson said. “There’s just a lot of opportunity for new drugs,” he said.

BioMarin, for one, has shown money can be made by focusing on enzyme replacement treatments for diseases with 200,000 or fewer patients that few people know about. Aldurazyme and Naglazyme, BioMarin’s therapies for the lysosomal storage disease MPS, are among the priciest drugs in the world at \$200,000 and \$375,000 annually, according to Forbes. BioMarin lost \$488,000 on revenue of more than \$324.6 million last year.

As an assistant professor in the genetics unit of the pediatrics department at **Harbor-UCLA Medical Center**, Kakkis and Elizabeth Neufeld discovered what would become Aldurazyme. After joining BioMarin in September 2008 — then a company less than 2 years old — he also oversaw development of Naglazyme and Kuvan, a treatment for the metabolic disease phenylketonuria.

Orphan drug development carries tax and intellectual property benefits, including tax credits and seven years of market exclusivity.

But as orphan drug development companies have grown, small-market products “don’t move the needle for them anymore,” said Kakkis, who retired from BioMarin in February 2009 and has since run the **Kakkis EveryLife Foundation** in Novato, an organization aimed at improving the regulatory system for rare diseases.

Ultragenyx already is working on its first small molecule drug — aimed at a biochemical disorder that affects skeletal muscle — with a small Japanese pharmaceutical company handling manufacturing and preclinical work. Ultragenyx also has a collaboration with an academic researcher on a second drug and is working on three other experimental treatments for diseases that Kakkis wouldn’t specify.

Among Kakkis’ heady goals for Ultragenyx is to take the lead drug into the clinic by July 2011 and complete the proof-of-concept study within two years.

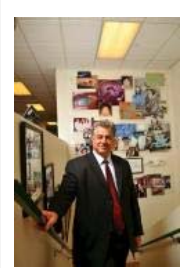
“We’re going to show how fast things can get done with planning and execution,” Kakkis said.

Kakkis has seen the process work quickly before. At BioMarin, for example, it took Kuvan just three years to go from its investigational new drug application to approval by the **Food and Drug Administration**, and it took Naglazyme, despite only 300 patients identified at launch, five years.

The average pharmaceutical drug takes 10 years to get from IND to approval.

To do that, Ultragenyx will create a boilerplate registry system to find patients over a variety of orphan diseases. That is important because orphan diseases can be so rare that there are no advocacy organizations or other way of finding out exactly how many people have the disease, much less who is eligible for the necessary clinical trials.

Such a system can knock months, if not years, off clinical development.



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“In rare diseases, you can go on forever, because there’s so little known,” says former BioMarin exec Emil Kakkis.

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"Rapid development requires insight and expertise. But you have to stay focused," Kakkis said. "In rare diseases, you can go on forever, because there's so little known."



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