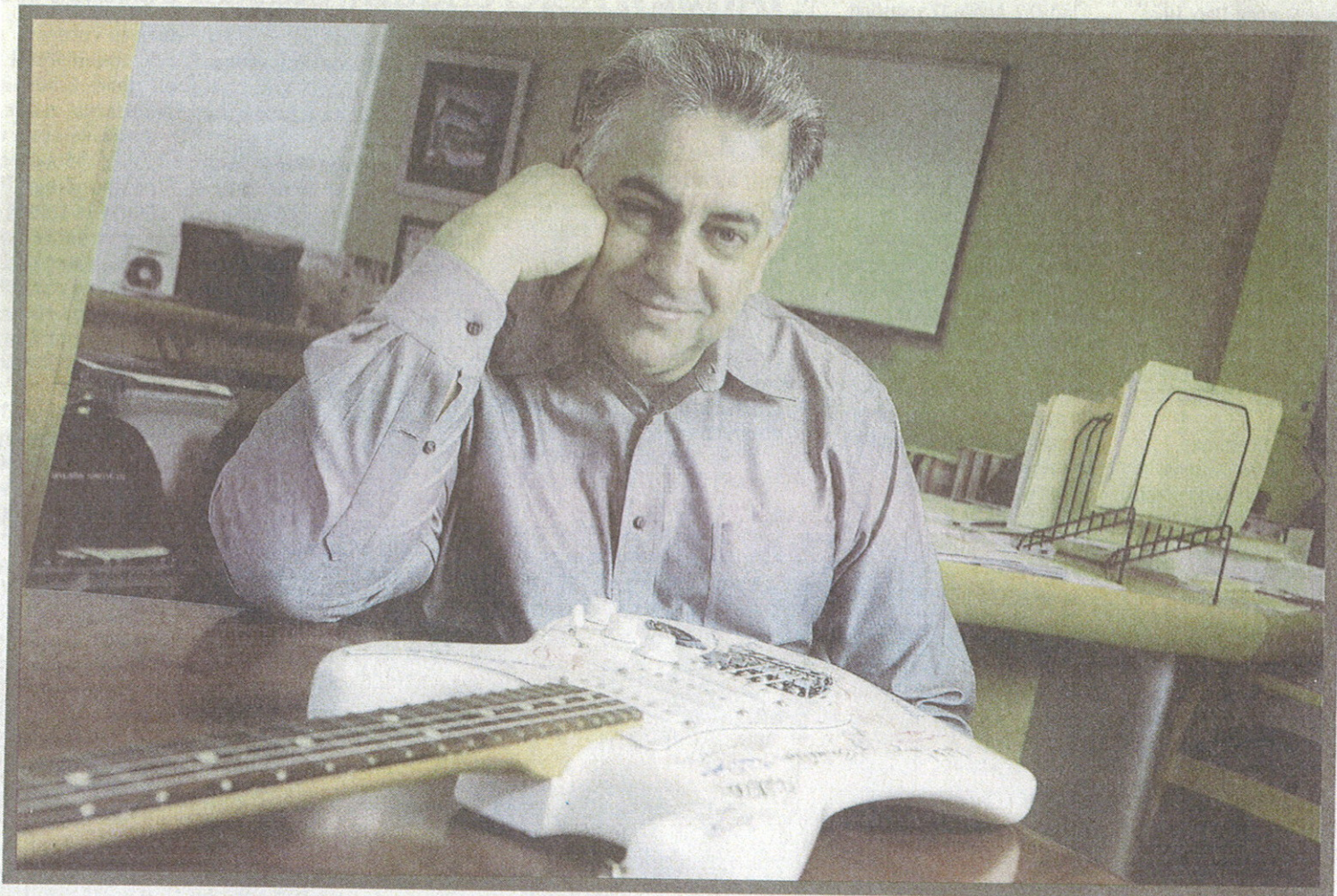


Doctor Emil Kakkis, called a 'Rock Star of Science' by GQ, was already a pioneer in his field when he launched the EveryLife Foundation. Though few directly benefit from his work, he's their savior — his life and career are devoted to treating and curing the rarest of diseases.



# RARE BREED

By Rob Rogers

Marin Independent Journal

**O**NLY ONE OUT OF EVERY 100,000 people on Earth is born with mucopolysaccharidosis I, or MPS-I, a genetic disorder that can cause organ damage in affected children.

It's a much rarer condition than birth disorders such as cystic fibrosis, which affects about one out of 2,000 children, or Down syndrome, which affects about one in 900. As a result, it's much more difficult for researchers to find funding for studies of drugs that could help or even cure children with these conditions — or to locate test

See **KAKKIS**, page 2



Dr. Emil Kakkis of Novato brought a Fender Strato-caster (top) with him to GQ's 'Men of the Year' issue shoot, getting it signed by rock stars and other scientists. At left, Kakkis stands over the shoulder of a rapper.

IJ photos/Frankie Frost

**HOW TO HELP** For more information on the EveryLife Foundation, or to make a donation, call 884-0562 or visit [www.kakkis.org](http://www.kakkis.org). For more information on MPS-I and other rare diseases, visit the National Organization for Rare Disorders at [www.rarediseases.org](http://www.rarediseases.org).

# KAKKIS: Devoted to treating, curing rarest of diseases

From page 1

subjects for those studies.

Novato's Dr. Emil Kakkis is trying to change that.

"About 25 million people have one of a few thousand different rare diseases," said Kakkis, 50, who helped develop a treatment for MPS-I, and whose EveryLife Foundation is dedicated to finding and funding cures for rare conditions. "A lot of those diseases affect less than 6,000 people. Only about 5 percent of those diseases have treatments."

Kakkis, who appears in the December issue of GQ as one of the magazine's "Rock Stars of Science," has devoted his career to understanding diseases most people have never heard of — including 11 years with the Novato biotech firm BioMarin, where he served as chief medical officer. Kakkis left BioMarin last year to lead the EveryLife Foundation, and is preparing to launch his own company, Ultragenics, in January.

"He was one of the true pioneers of the company who drove it to where it is today," said BioMarin spokesman Bob Purcell.

"His work led to the development of Aldurazyme and Naglazyme, treatments for the rare diseases generally known as MPS-I and MPS-VI."

Working on rare diseases "gives you the chance to

start from nowhere, and end up treating someone in a significant way in just a few years," Kakkis said. "There aren't many places in medicine where you can make the transition from bench to bedside in just a few years."

Kakkis began his work with MPS-I in 1993 while a researcher at the University of California at Los Angeles. There, he assisted lead researcher Elizabeth Neufeld in identifying the causes of the disease.

"I was on a fellowship for a project that cloned the gene that's responsible for the gene," Kakkis said. "It was the last bit of information required to be able to work on a treatment that became Aldurazyme."

Despite the project's success, Kakkis was disheartened by the difficulty it took to bring the treatment of MPS-I to market.

"Even after (Neufeld) had spent 30 years working on this treatment, no one wanted to invest in its development," Kakkis said. "How many scientists have a shot at developing a treatment? I had the feeling that I couldn't let this die."

A chance encounter at a medical conference put Kakkis in touch with Mark and Jeanne Dant, a Texas couple whose 9-year-old son, Ryan, was suffering from the disease. The couple's efforts helped

raise money toward the trials and studies needed to submit Aldurazyme for Food and Drug Administration approval.

Kakkis' EveryLife Foundation now works to coordinate the efforts of families like the Dants.

"My foundation has 155 organizations endorsing our campaign, and the majority are small organizations with the name of a particular patient, such as 'Hunter's Hope' or 'Save Cassie,'" Kakkis said. "They're all families taking the future into their own hands, and trying to drive the research through bake sales, writing grants and filing documents with the FDA."

The foundation has also worked with Congress and the FDA to change the way the FDA evaluates treatments for rare diseases. In 2009, Sen. Sam Brownback, R-Kansas, and Sen. Sherrod Brown, D-Ohio, sponsored an amendment to the 2010 and 2011 FDA appropriations bills that requires the agency to create a team to re-evaluate that regulatory process.

"He's been sort of a kindred spirit in many ways over the years, and there have been several issues related to advocacy on behalf of people with rare diseases where we have found ourselves on the same wave length," said Mary Dunkle, vice president for communications at the National Organiza-

tion for Rare Disorders, where Kakkis served as a member of the board of directors.

Kakkis grew up in the Los Angeles area and did his undergraduate work at Pomona College. His work with BioMarin brought him to Novato in 1998, together with his wife, Joyce, a fellow physician who works at the Kaiser Richmond Medical Center. The two met in medical school.

"We were in anatomy class, working on adjacent cadavers," Kakkis said.

Though his work in the lab and with the foundation keeps him busy, Kakkis continues to stay in touch with the Dant family. He is looking forward to seeing them at the EveryLife Foundation's Legends of Golf tournament, a fundraiser at San Francisco's Presidio Golf Course on Jan. 9.

Ryan Dant, a lifetime Texas Rangers fan, "went to the World Series and got to see the game the Rangers won" on Oct. 30, Kakkis said. "He's 22 now. He was 9 when I treated him. He would have died by his teenage years had he not been treated. The fact that he made it to that game is really the success that comes from working hard, developing treatments and not saying no when you need to get something done."

Contact Rob Rogers via e-mail at rogers@marinij.com

# TARGET: Referendum, suit threatened if store is approved

From page 1

"If the vote doesn't go correctly on Monday night, we're going to position ourselves with a referendum as well as a lawsuit," said Bill Daniels, owner of United Markets and a member of the opposition group Keep it Local San Rafael. "I don't feel they've done

member.

The letter called greenhouse gas impacts and traffic projections included the study flawed.

But City Attorney Rob Epstein said he doesn't believe there's any basis for a lawsuit at this point.

"At this juncture and pending a council decision

dum to voters.

"Conventional wisdom is we need 4,500 signatures in order to make absolutely positive that we hit the (2,144)," Moran said. "Will it be daunting? I'm sure."

Daniels noted that Target opponents have already collected around 2,000 signatures as part of a petition

Robyn Letters, Opinion Studies' principal.

The margin of error was about 5.3 percent for the larger sample size.

"We have all along felt tremendous community support for this project, and it's gratifying to see that the objective data reflects that support." Plante

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