

Spark CCO maps gene therapy vision

Speaking with Dr. Rogerio Vivaldi, it's easy to forget that he's in the middle of one of the most intense periods of activity in a distinguished career that has spanned three decades. He dryly characterizes himself as "an old guy who feels old," talks up his newfound appreciation for American sports since moving to the Boston area ("but I'm not that strong on baseball") and self-deprecates about his straight-arrow personality ("my wife jokes that I'll be in a dangerous position if I do anything wrong because I don't know how to lie").

And then the conversation turns back to business, and then you remember: Vivaldi is the chief commercial officer of Spark Therapeutics, which is as good a bet as any to become the first company to have a gene therapy approved in the United States. In early October Spark announced that SPK-RPE65, its product to treat inherited retinal dystrophy, had succeeded in a late-stage trial—that, basically, it helped restore sight.

Vivaldi has spent much of his career on the cusp of scientific breakthrough. In 1992 he became the first doctor to treat Gaucher disease in Brazil, courtesy of a complex enzyme-replacement therapy. His success led him to Genzyme, where he spent 20 years building the company's Brazil and Latin America operations and establishing himself as one of the world's foremost commercial leaders in the rare-disease space. After a stint as CEO and president of Minerva Neurosciences, during which he oversaw the company's IPO and the development of a portfolio of products for patients with neuropsychiatric conditions, he joined Spark last December.

"I'd been on Spark's board for a few months but I wanted to partner with them even [earlier]," he says. "It's impossible not to be excited by what we do here."

Vivaldi issues the usual caveats about drawing premature conclusions from the data and news flow to date, stressing that he's "not claiming anything" about the product's potential

"Gene therapy is a collection of small details. It's not a secret sauce."

Rogerio Vivaldi

Dec. 2014–present
Chief commercial officer,
Spark Therapeutics

Nov. 2013–Dec. 2014
President and CEO,
Minerva Neurosciences

Jan. 2012–Oct. 2013
SVP, head of rare diseases,
Genzyme/Sanofi

to restore visual function. At the same time, he doesn't downplay his belief that SPK-RPE65 could have great life-changing potential for patients.

"There are still lots of questions, but what we demonstrated in Phase III is that it works," Vivaldi says cautiously. "We were trying to address the concerns many people have about durability—and, well, it's durable. Patients who were followed for three years are keeping the same efficacy they gained during year one."

Vivaldi dismisses further questions about the product. He's more eager to address the groundwork Spark has done. It has focused a great deal of attention on broader education efforts—not just about inherited retinal dystrophies, but about gene therapy itself.

Spark, to its great credit, has gone out of its way to make sure that gene therapy isn't perceived as a magic bullet. "As [company president and chief scientific officer] Kathy High has put it, gene therapy is a collection of small details. It's not a secret sauce," Vivaldi explains. Similarly, he notes the challenges associated with educating patients about "the novel

end point" of vision restoration. "For that to happen, it assumes a flawless operation. The entire episode of care has to be flawless. It is extremely, extremely complex."

To that end Spark has endeavored to keep the patient community very much in the loop, working closely with the Foundation Fighting Blindness and other advocacy organizations. The goals? To identify ideal candidates for the therapy—those with the correctable gene defect—in advance and to prepare physicians and their institutions for the challenges and demands that come with the procedure.

Vivaldi seems to be taking it all in stride. He's the rare pharma commercial exec who doesn't have an answer at the ready when asked about what comes next. "When I started out I wanted to help someone, even if it was just one person," he says after a pause. "That's still the goal. The other day I heard from one of my first patients, who was 14 years old and severely ill. Now he's 35 and has a daughter and is working in biotech. There's an aha moment almost every day when you're doing something you love." — *Larry Dobrow*

