ENDPOINTS NEWS

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The 20(+1) under 40: Your guide to the next generation of biotech leaders

ENDPOINTS

For a second year in a row, we've launched a 20 under 40 project and arrived, months later, with a list of 21 names.

The reason for that is simple: No matter how much we valorize the lone genius, science is ultimately and always a team sport. Like last year, we set out to honor individuals and found, when we looked, that it was really a team — or in this case a duo — that deserved the recognition.

There's a long history there: Watson and Crick; Marie and Pierre Curie; Doudna and Charpentier; heck, even Lennon and McCartney.

NAME
Sharif Tabebordbar

COMPANY
Kate Therapeutics

POSITION
Co-Founder and CSO

AGE
36

A bioengineer with a personal history in rare disease looks to tackle one of the biggest hurdles in gene therapy

Not everyone gets a Gina Kolata profile in the *New York Times* and a blog post from former NIH director Francis Collins highlighting his research.

But Sharif Tabebordbar, who's recently founded the AAV-based gene therapy-focused Kate Therapeutics, has had both.

While straddling the worlds of academic research and the biotech industry, Tabebordar made clear in a recent interview that it's his love for the work that drives his work ethic.

"I really enjoy the work I do, so that's what drives me," he said. "I dream a lot about the day we inject our first patients and change their lives. These are kids that are going to live a much happier life."

Born in Shiraz, Iran in 1986, Tabebordbar said his mother's focus on education, and his father's struggles with a rare genetic muscular disease (explained more in the Kolata profile), pushed Tabebordbar to plug into science early on.

"This is unfair that people are born with these genetic diseases that they have no control over, and that fueled my passion for biology and genetics," he said.

So after studying biotechnology in undergrad and for a master's, he said he applied to the 10 schools in the US that have muscular dystrophy PhD programs, and he got into Harvard.

That's where he said he worked on *in vivo* gene editing, immune responses of CRISPR in muscle, as well as the nitty-gritty of muscle biology and different therapeutic strategies in different models of disease.

After his PhD he said he wanted to go into academic research, but instead jumped at an opportunity to lead a gene therapy program at Editas Medicine, which focuses on developing CRISPR-based therapeutics.

Only about a year later, Editas decided to reorganize and told Tabebordbar that they had to scrap his program because there wasn't yet a good gene delivery modality to get these components into muscle.

"And that was a fair point," Tabebordbar said. "And this wasn't going to be a problem for only this company, but for the whole field.

"So I decided to tackle that," he said, delving into a discussion of the unresolved challenges that the field now faces.

Among the challenges: identifying the underlying genetic issue (which in the case of his father and others with rare dystrophies can be very difficult without the appropriate tests); manufacturing that much virus; figuring out whether re-dosing might be necessary; and the elephant in the room: safety.

"We know that if you get the missing gene into the body, it's going to work, it's going to have a therapeutic effect. It's all about can you make it safe enough at this point," he said, adding:

Can we deliver drugs that are potent enough that we can go to a low enough dose that doesn't result in the toxicity issues that we're seeing? There have been multiple holds in the last year and a lot of them are associated with injecting an extremely high dose of virus because these naturally occurring viruses are mainly delivered to the liver.

Two gene therapies have won FDA approval so far (Roche subsidiary Spark's Luxturna and Novartis' Zolgensma) but Tabebordbar is optimistic and said he expects others for genetic muscle diseases will be approved in the next 5-10 years.

"The first ones will be gene replacement therapies for recessive diseases that we just need to replenish the missing protein at a certain level. Gene editing as therapeutics is going to be more challenging, particularly for the muscle, because of its structure," he noted. — *Zachary Brennan*