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Health Care

Vertex founder Josh Boger is back in biotech. Here's why he 'un-retired.'



Image: Joshua Boger

Josh Boger is the founder of Vertex Pharmaceuticals and chairman of Alkeus Pharmaceuticals.

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By [Rowan Walrath](#) – Life Sciences Reporter, Boston Business Journal
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The founder of Vertex Pharmaceuticals Inc. has rejoined the biotech world, more than 30 years after he founded the company that would eventually change the course of disease for patients with cystic fibrosis.

Josh Boger has entered his second act as chairman of Alkeus Pharmaceuticals, a startup developing drugs for inherited eye diseases. What Vertex (Nasdaq: VRTX) did for cystic fibrosis, Boger thinks, Alkeus has the potential to do for Stargardt disease, an inherited disorder that causes around 30,000 American children and young adults to go blind.

"Blindness may not kill you, but it's a horrific thing for most people to contemplate," Boger said. "In this particular case, it's a monogenic recessive disease, which means that the parents are just carriers. They may not have any blindness in their family's history. So a child is actually born with normal sight, and the child begins to go blind as early as six."

The earlier the onset, the quicker the disease progresses. If a patient starts losing vision at age six, they may be completely blind by age seven, whereas if they start losing vision at 12, it could take another eight years or so to be fully blind, Boger said. Alkeus' drug, called gileuretinol or ALK-001, promises to slow that progression.

Alkeus has been around since 2010, when co-founder and CEO Leonide Saad licensed ALK-001 from the lab of Columbia University professor Ilyas Washington. Unbeknownst to Saad, Boger had read about the same molecule and filed it away as something to "find out and follow up on."

"What I didn't know was that Leonide was more action-oriented," Boger said. "He went to Columbia and licensed it. He came to me when I had just retired from Vertex, although I was still on the board, having retired in 2009. He came to me and said, 'Can you help me get this configured to go in the clinic?' And I said, 'That's great. Are you

going to start a company?' And he said, 'No, I want to do it myself.' I went, 'O.K., that can be done.'"

So from 2010 until 2018, Alkeus was a one-man show. Saad anchored the clinical development of ALK-001, with Boger serving as chairman from 2012 to 2017. Saad hired a second employee in 2018 to oversee clinical operations.

Around that time, Boger asked Saad if he planned to expand the company. When Saad said he wanted to await clinical results, Boger said, "O.K., nothing more for me to do right now. Get back to me when you have results."



Leonide Saad is the co-founder and CEO of Alkeus Pharmaceuticals.

COURTESY PHOTO

Those results finally came on New Year's Eve in 2020. ALK-001 had succeeded in slowing retinal damage in a placebo-controlled Phase 2 trial. A few months later, the U.S. Food and Drug Administration [awarded](#) the drug breakthrough therapy designation, which expedites the agency's review process.

Saad asked Boger to step back in. Saad wanted to use the good news to fundraise, so Boger got involved in conversations with Bain Capital, which eventually led the startup's \$150 million Series B round, [announced last week](#). He formally rejoined as chairman at the same time, writing on LinkedIn that he had "un-retired."

Now, their main task is company-building. Alkeus is slated to go from a two-person enterprise to 50 people by the end of the year, then 100 by the end of 2024. The startup is in the process of securing new real estate to make room for the explosion in headcount. By the end of 2024, Saad hopes, Alkeus will be able to file an application for FDA approval in Stargardt disease.

"We're very excited about it, because our vision now is to bring this treatment as fast as possible to market, self-commercialized," Saad said. "If we're able to do so, we could become the next Vertex or the next Genzyme, as we're extremely patient-centric."

For Alkeus, building the company goes beyond simply hiring employees and running clinical trials. Alkeus effectively has to create a commercial market. Because there's no treatment for Stargardt disease, there's no competition from other drugs, but there's also no real infrastructure for diagnostics or for provider education.

"This is the third-largest monogenic disease, and I've talked to doctors who've never heard of it," Boger said. "They haven't heard of it, because there's nothing for it, and they tend not to spend a lot of time in medical school on something where the doctor can't do anything."

Boger is hoping to do three major things: educate doctors on the basics of Stargardt disease, build a patient community that will in turn enable word-of-mouth marketing for ALK-001, and – perhaps the largest task of all – get hospitals to screen newborns for Stargardt disease along with other monogenic diseases they test for. That will be a state-by-state process, he said, and one that can't begin until ALK-001 is approved and on the market.

"There's a lot of work to do, but I think, ultimately, this is big," Boger said. "Building a company is great fun. I do feel like I have the experience in literally every aspect that needs to be done. Some of the experience is very similar [as building Vertex was]. I think it otherwise would be kind of wasting my potential. When Leonide called me originally

and said, 'Here are the results,' I didn't say, 'When could I come to work?' But when he asked me to help build a company, how could I say no?"

"My tennis game will suffer," Boger added. "But children won't go blind."

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