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Cambridge biotech raises \$150 million and rehires founder of Vertex

By **Jonathan Saltzman** Globe Staff, Updated June 5, 2023, 6:00 a.m.



Leonide Saad, cofounder and chief executive of Alkeus Pharmaceuticals.

Buoyed by clinical trial results for its experimental drug to treat a rare inherited cause of blindness, a small Cambridge biotech said Monday it has raised \$150 million in venture capital to expand operations and plans to seek approval of the medicine next year.

Alkeus Pharmaceuticals also said it has rehired one of the industry's most prominent figures to help advance the drug for Stargardt disease: Joshua Boger, the founder of Vertex Pharmaceuticals.

Boger, who retired in 2009 as chief executive of Vertex, the homegrown Boston-based biotech best known for its cystic fibrosis drugs, had served as executive chairman of Alkeus from 2012 to 2016. During that period, Alkeus tested its drug on laboratory animals and began clinical trials on patients with Stargardt. There are no approved treatments for the disease.

Now, after Alkeus reported encouraging mid-stage trial data at scientific conferences and the Food and Drug Administration designated the medicine a potential breakthrough in 2021, Boger has returned to the firm as executive chairman. He will head the privately held company's board of directors and serve as an executive.

Boger said the once-a-day pill for Stargardt was the most exciting drug candidate he ever encountered when he learned about it shortly after Alkeus was founded in 2010 with technology licensed from Columbia University. His enthusiasm remains undimmed.

"It's as close to a perfect drug as I've ever seen in 40 years in the business," Boger, 72, said in an interview.

Stargardt is a leading cause of blindness in children and young adults, affecting at least 30,000 people in the United States and more than 150,000 worldwide. Patients with the disorder are typically born with normal vision, but mutations in a gene called ABCA4 lead to clumping of vitamin A in the eye. Although vitamin A is usually good for the eyes, too much causes damage to the retina and progressive vision loss, starting as early as 5 years of age.

The median age for when Stargardt patients are considered legally blind is 16 years old, according to Leonide Saad, cofounder and chief executive of Alkeus. "The earlier you have symptoms, the faster you have progression," he added.

The experimental medication, whose generic name is gildeuretinol, was invented by Ilyas Washington, an assistant professor at Columbia University Medical Center. He designed a chemically modified form of vitamin A that doesn't clump and cofounded the biotech.

In a placebo-controlled mid-stage clinical trial of 50 patients with Stargardt, the daily pill slowed retinal damage and was well tolerated over two years, according to the company, which began reporting results in 2021. In children and young adults with earlier cases of the disease, the pill appeared to halt retinal damage and vision loss.

Additional clinical trials are ongoing, but Saad said the firm already plans to expand from seven to at least 50 employees by the end of the year.

Boger said the decision by the FDA to designate gildeuretinol a "breakthrough therapy" is "not just a pat on the back."

"It means you enter into a much closer relationship with the FDA in order to get the breakthrough to patients as fast as possible," he said. About half of drugs that receive that status are approved on mid-stage trial data instead of having to use late-stage results, he added.

The medicine is also being tested on an advanced form of dry age-related macular degeneration that leads to progressive and irreversible vision loss. There are 1.5 to 2 million people in the United States living with that form of the disease, according to Alkeus.

The \$150 million fund-raising round was led by Bain Capital Life Sciences, with additional participation by TCGX, Wellington Management, and Sofinnova Investments. Alkeus had previously received about \$10 million in private and federal funding, Saad said.

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