



## Startup's vision to succeed

### MassChallenge winner Alkeus developing eye ailment treatments

By Donna Goodison | Sunday, October 30, 2011 | <http://www.bostonherald.com> | [Healthcare](#)

A Boston biotech startup is working on treatments for some of the biggest unmet medical needs in the field of ophthalmology.

Alkeus Pharmaceuticals is targeting dry age-related macular degeneration, the No. 1 cause of blindness in the United States that generally affects people over 50, and Stargardt's disease, a genetic, juvenile form of macular degeneration that affects children as young as 6.

There are currently no treatments for either condition, according to CEO Leonide Saad.

Some 3.5 million Americans have experienced loss of sight due to dry-AMD, which affects 75 percent of those patients who suffer from AMD. Stargardt's disease is rare, meanwhile, affecting 1 in 10,000 people or about 35,000 to 40,000 in the United States.

"But if you are a parent with kids who are 6 to 10 years old and losing their vision and you just found out its Stargardt's disease — in almost all cases, it leads to blindness — it's very devastating to learn about because there's no treatment," Saad said. "That pushes me even more because I know sight in the most cherished sense."

Alkeus, which licensed the compounds for its treatments from Columbia University, has received U.S. Food and Drug Administration clearance to start clinical trials next year for its lead Stargardt's drug, ALK-001.

"We've completed very nice efficacy studies in animals to help vision loss," Saad said. "We did it with mice who have Stargardt's disease, and we showed we could prevent them from losing vision."

Saad decided to target dry-AMD and Stargardt's disease because he knew that treatment breakthroughs would have huge implications.

"I was doing my Ph.D. in the scientific field of medicine at MIT, so I've always been very interested in strong impact drugs," he said. "And when you're a startup, you have very limited resources, so you need to focus on ideas with the biggest impact."

A \$100,000 winner in MassChallenge's \$1 million startup competition last week, Alkeus raised undisclosed funding from other private sources and has sufficient funds to complete first-phase clinical trials, according to Saad.

The pharmaceutical landscape has changed, and startups can develop drugs with fewer resources if they operate in a very lean, focused manner, he said.

"We have a semi-virtual business model," Saad said. "We don't need too many people because we outsource a lot of work. Twenty years ago, people were not that specialized. Today it's like making a car. Car manufacturers most times do the assembly, they don't make each part."

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