BIOPHARMADIVE

DIVE BRIEF

Startup Airna emerges from stealth to bring RNA editing to a rare disease

The biotech company initially plans to develop a drug for alpha-1 antitrypsin deficiency.

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Reporter

Kris Elverum, the CEO of Airna, said the company is first focusing on developing a drug for alpha-1 antitrypsin deficiency Permission granted by AIRNA

Dive Brief:

- Airna, a biotechnology startup focused on RNA editing to make more precise medicines, emerged from stealth Tuesday with $30 million in hand.
- The company is based in Cambridge, Massachusetts, with research facilities in Tübingen, Germany. Its initial plan is to develop an experimental drug to treat the inherited disorder alpha-1 antitrypsin deficiency, or AATD.
- Airna's board is chaired by Rodger Novak, one of the co-founders of CRISPR Therapeutics. Kris Elverum, a former executive at Diagon Therapeutics, Rubius Therapeutics, Turnstone Biologics and SQZ Biotech, is the company's CEO.

Dive Insight:

Scientists have touted RNA editing as a safer derivation of early gene editing methods. Unlike CRISPR-based therapies, RNA editing can target specific sites in an RNA transcript without permanently changing a patient's genome.

"What's so wonderful about RNA editing is that it's using a modality and a medicine that can be accessible to millions of patients," Elverum said.

In it, Elverum sees a promise to develop a drug that resembles "a traditional biologic" that would appear more familiar to patients.

Airna, which was co-founded by a group of scientists including researchers at Stanford University and the University of Tübingen, looks at a protein known as ADAR, which stands for adenosine deaminase acting on RNA. The enzyme is already present in the body, helping to modify and regulate genes.

Alpha-1 antitrypsin is crucial to protect the body's tissues from being attacked by its own enzymes. When not produced or produced incorrectly, it can lead to liver or lung disease. The company estimates that approximately 100,000 people in the U.S. have the condition.

No cure exists for AATD; instead, doctors treat the symptoms of the disorder or patients can receive weekly injections of the alpha-1 antitrypsin protein. Airna is looking to develop a therapy that can correct the mutation that produces the errant protein.

RNA editing is "a much more accessible approach," Elverum said, "if you think through what a patient wants in terms of the risks they want to take, or even what a payer wants in terms of how medicines are paid for."

The company has not disclosed what other diseases it plans to work on outside of AATD, but said its medicines would target both

rare and common conditions.

“AIRNA has a uniquely powerful technology to maximize the therapeutic properties of RNA editing medicines and achieve the vision of restoring a patient’s health,” Novak said in a statement.

Its initial financing was led by Arch Venture Partners, and includes investors such as ND Capital, Fast Track Initiative, Novalis and Codon Capital.

Airna is part of a recent cohort of biotechnology companies trying to develop a treatment for AATD. One of its competitors, Wave Life Sciences, said in early September that it planned to dose the first person in its clinical trial for an RNA editing therapy for AATD by the end of the year. AlveoGene, another gene therapy company, launched Thursday with plans to develop a medicine to treat AATD lung disease.