BIOPHARMADIVE

Ultragenyx to spin out new company around Alzheimer's gene therapy

CEO Emil Kakkis says the company's findings are too exciting to ignore, but the "high-risk, high-return" venture needs to be pursued outside the organization.

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Amyloid protein clumps together to form plaques between neurons in the brains of people with Alzheimer's. National Institute on Aging. (2017). "Beta-Amyloid Plaques and Tau in the Brain" [Image]. Retrieved from Flickr.

Ultragenyx will spin out a new company to take advantage of research it claims could lead to a new way of treating Alzheimer's disease.

Researchers working for the company found that an enzyme called protective protein/cathepsin A, or PPCA, can break up a form of amyloid beta, the protein many consider responsible for Alzheimer's debilitating effects. Initial mouse studies were encouraging, Ultragenyx told investors Monday.

CEO Emil Kakkis called the finding too important not to pursue, but the work and funding needed to develop an Alzheimer's drug is well outside of Ultragenyx's rare disease focus. So, Ultragenyx is forming a new company called Amlogenyx to capitalize on the "high-risk, high return" discovery.

"I couldn't sit by and watch something this exciting and not actually put it into play," Kakkis told investors. "I can see this just being a leap ahead of how you might treat Alzheimer's."

Ultragenyx will maintain majority ownership in the new company and seek independent funding for it, with the goal of closing a Series A financing by the end of the year. The spinout will be cash neutral for Ultragenyx, Kakkis said.

Kakkis described the work until now as "low-key," with a small amount of money spent on research. Ultragenyx will grant the new company a license for the required intellectual property and "know-how," allowing it to use Ultragenyx's Pinnacle PCL manufacturing platform.

The announcement came as part of Ultragenyx's investor day program, though the company didn't flag the spinout in its press release. Kakkis laid out the details toward the end of a slideshow presentation.

Researchers have long focused on clearing amyloid plaques in the brain as a way of battling Alzheimer's, even though some experts have questioned the approach. Despite decades of work and billions of dollars, there is no cure for the disease.

The Food and Drug Administration has approved two drugs designed to slow Alzheimer's progression by targeting amyloid plaques. The first, Biogen's Aduhelm, was embroiled in controversy amid questions about how much it benefits patients. Biogen later stopped marketing it. The second, a drug called Leqembi developed by Eisai and Biogen, may prove more successful after initial pushback from insurers before its full approval in July. The data supporting it are clearer in showing a benefit to treatment.

A third, similarly acting drug, called donanemab and from Eli Lilly, could receive an FDA approval by the end of the year.

Still, the effects of these monoclonal antibody drugs are modest, Kakkis said. The Amlogenyx gene therapy approach, which would

use a viral vector to deliver the PPCA enzyme, may provide better potency, he said.