BIOPHARMADIVE

Actio, a precision medicine startup, launches with $55M and a mouse lab partnership

The two-year-old San Diego biotech is allied with The Jackson Laboratory to engineer better mouse models of the rare diseases it plans to target.

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Actio Bio CEO David Goldstein *Permission granted by Actio Bio*

A new biotechnology startup is getting help from an unusual partner in its attempt to develop rare disease drugs that could also treat more common conditions.

Actio Biosciences, which launched Tuesday with $55 million in venture funding, is working with The Jackson Laboratory to engineer mouse models that better recapitulate diseases, potentially allowing Actio scientists to more accurately vet would-be drugs.

The Bar Harbor, Maine-based Jackson, or Jax — a top supplier of lab mice — helped develop an animal model Actio is using to study a target called TRPV4. The San Diego startup believes TRPV4, an ion channel protein, could be important for treating a degenerative nerve disease called Charcot-Marie-Tooth.

“We want to be able to really derisk the biology for the target before we ever get into the clinic,” said David Goldstein, Actio’s co-founder and CEO, in an interview. “And that means that we need

to be able to really understand how mutations cause disease, which is often very complicated."

Goldstein's vision is to pick rare disease drug targets that are also relevant to conditions affecting larger groups of people. Jax's models could give Actio the tools to make that jump, which many drugmakers aspire to, more reliably.

"The theme of starting with a more homogeneous disease setting and then moving out into a broader population is certainly something that's been around for decades," said Goldstein. "But our particular approach to doing that is to develop a framework that really can effectively start with 4,000 different Mendelian disease genes, and pull out the ones that really represent the best opportunities."

According to Goldstein, the pace of research into Mendelian diseases — single gene disorders like cystic fibrosis and sickle cell — has moderated after a period of rapid discoveries enabled by genetic sequencing. His view was shared by Lon Cardon, who moved from BioMarin Pharmaceutical to become Jax's CEO at the same time Goldstein left Columbia University to found Actio. A phone call between them helped start Jax and Actio's partnership, Goldstein said.

"For us at Jax, we believe this collaboration with Actio will help to serve the rare disease community in transformative ways, to build a bridge of preclinical work to therapies that are desperately needed," said Cat Lutz, head of Jax's rare disease translational center, in an emailed statement.

Charcot-Marie-Tooth, Actio's first target, was already familiar to Jax. The center created a mouse model of another form of the disease called Type 4J that was recently selected for a gene therapy

research partnership led by The Foundation for the National Institutes of Health.

Actio is aiming at another form, Type 2C, that causes severe muscle weakness and respiratory complications. The company believes its choice of target, a gene called TRPV4, could also play a rose in serious bone diseases like metatropic dysplasia.

The Series A funding Actio announced Tuesday is meant to get the TRPV4 program into the first stages of clinical testing, and make "meaningful progress" on at least one other program, Goldstein said. The round was led by Canaan and Droia Ventures, and also involved existing investors Deerfield Management and EcoR1.

The company is being advised by a board that includes Droia's George Golumbeski, who led business development at Celgene for many years, as well as John McHutchinson, formerly the CEO of Assembly Bio and, before that, Gilead's top scientist.