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## Altay Therapeutics Closes \$37 Million Series A Financing to Advance First Oral DUX4 Inhibitor for FSHD into the Clinic

Share



*DX5057 is an orally administered small molecule designed to inhibit DUX4 transcriptional activity and expression of its targeted proteins*

*Clinical program will explore DUX4-related biomarkers and muscle function, with a Phase 1 safety study expected to initiate in Q1 2027*

SAN CARLOS, Calif.--([BUSINESS WIRE](#))--Altay Therapeutics, Inc., a biotechnology company developing novel small molecule inhibitors of transcription factors, today announced the completion of a \$37 million Series A financing. The round was co-led by Sound Bioventures and HealthCap, with participation from new investors including Fountain Healthcare Partners, Biogen New Ventures, Pappas Capital, and Global BioAccess Fund.

Proceeds from the financing will be used to accelerate the development of Altay's lead therapeutic candidate, DX5057, through Phase 1 and Phase 2 proof-of-concept clinical trials for patients with FSHD (facioscapulohumeral muscular dystrophy).

"There are no disease-modifying therapies approved for FSHD, and the patient community is in need of a durable, practical solution. DX5057 is an oral medicine that acts systemically to shut down DUX4 at its source," said James Breitmeyer, M.D., Ph.D., Chief Executive Officer of Altay. "With this financing backed by top-tier

life sciences investors as well as past support from SOLVE FSHD, we are on track to initiate clinical development in the first quarter of 2027.”

DX5057 is an orally administered small molecule inhibitor of DUX4 (double homeobox 4), a transcription factor whose aberrant expression in skeletal muscle drives FSHD. DX5057 is expected to distribute freely to all affected muscle and inhibit DUX4 deeply.

“Altay has done the difficult lead discovery and preclinical work and has delivered an oral small molecule candidate on its way into clinical development,” said Ester Sklarsky, M.Sc., of Sound Bioventures. “We co-led this round based on the novel and differentiated approach in FSHD, the strength of the preclinical package, and the team’s strong track record as drug developers.”

“A small molecule-based, disease-modifying therapy could not only make long-term treatment for FSHD practical, but also suppress DUX4 deeply enough to preserve muscle before loss becomes irreversible,” said Carl Kilander, M.D., Ph.D., of HealthCap. “We are excited to support Altay in their mission and during this next phase of growth.”

Ali Ozes, Ph.D., Founder and Chief Scientific Officer of Altay added: “We are especially grateful for the early and essential support from Friends of FSH Research, FSHD Global Research Foundation and SOLVE FSHD that helped us reach this inflection point.”

Since 2022, SOLVE FSHD has invested nearly \$7 million in seed financing and provided sustained strategic and Board-level leadership.

In connection with the financing, four new directors will join Altay’s board of directors: Ester Sklarsky, M.Sc., of Sound Bioventures; Carl Kilander, M.D., Ph.D., of HealthCap; Aidan King, MBA, of Fountain Healthcare Partners; and Chelsea Johnson, Ph.D., of Biogen New Ventures. They join Mike Grey, Executive Chairman; Eric Bjerkholt; and James Breitmeyer, M.D., Ph.D., Chief Executive Officer.

### **About DX5057**

DX5057 is an orally available small-molecule investigational product designed to bind double homeobox 4 (DUX4) and inhibit its function, thereby reducing downstream DUX4-regulated gene and protein expression. DUX4 is an embryonic transcription factor that is aberrantly expressed in skeletal muscle of patients with

FSHD, where the downstream gene-expression programs it activates are associated with muscle degeneration, impaired regeneration, progressive injury, atrophy, and muscle weakness. In nonclinical pharmacology studies including a transgenic mouse model expressing human DUX4, DX5057 reduced DUX4-associated activity and downstream gene-expression programs, reduced markers of muscle injury, and preserved disease-relevant muscle function. DX5057 has received orphan drug designation from the U.S. Food and Drug Administration.

## About FSHD

Facioscapulohumeral muscular dystrophy (FSHD) is one of the most common inherited muscular dystrophies characterized by progressive skeletal muscle weakness and atrophy, typically involving the facial, scapular, and humeral muscles before extending to the trunk and lower extremities. Disease progression occurs over decades and is associated with relentless, irreversible loss of muscle function and increasing disability requiring wheelchair assistance in some cases. With autosomal dominant inheritance, FSHD is associated with increased morbidity and reduced survival. There are currently no approved disease-modifying therapies for FSHD.

## About Altay Therapeutics

Altay Therapeutics is a biotechnology company developing novel small-molecule inhibitors of transcription factors. The company's lead candidate, DX5057, is an orally administered small molecule designed to block DUX4 transcription and degrade the DUX4 protein for the treatment of FSHD. Altay is also developing a STAT3 inhibitor and an ETS family inhibitor with potential across several cancers. Altay's proprietary discovery platform identifies allosteric binding pockets on transcription factors previously believed to be undruggable and utilizes AI modeling and in silico docking models to identify small molecule antagonists. Backed by marquee investors and led by a team of seasoned drug developers, Altay is driven to turn its discoveries into disease-modifying therapies for patients. For more information, please visit <https://www.altaytherapeutics.com>.

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## ALTAY THERAPEUTICS, INC.

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### RELEASE SUMMARY

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### RELEASE VERSIONS

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