MAHZI THERAPEUTICS

Advancing precision therapies for rare neurodevelopmental diseases

mahzi.com South San Francisco, CA

Highlights

1. $60M raised from leading VCs: Venrock, HealthCap, Droia Ventures & HBM Partners
2. $16M in non-dilutive grant awards from the California Institute for Regenerative Medicine
3. Leadership from Stanford, Cambridge, Yale, UC Berkeley & Weizmann Institute
4. Close collaborations with Pitt Hopkins Foundation & many other patient advocacy groups
5. CEO with 20+ years at Merck and rare disease pioneers Genzyme & Ultragenyx
6. Now enrolling patients in first-ever gene therapy clinical trial for Pitt Hopkins Syndrome
7. 800+ rare neurogenetic disorders addressable with our approach; targeting a $16B+ market
8. $200-$600M peak annual revenue projected per indication in pipeline (not guaranteed)

Featured Investor

Vimal Srivastava
Syndicate Lead

Follow

Invested $5,000

"Mahzi is on a great mission to develop precision genetic therapies for seizure and autism-based disorders in meaningful partnership with patient advocacy groups. Developing targeted genetic therapies for severe, under-served neurodevelopmental disorders like Pitt Hopkins syndrome, WOREE syndrome, and CHD2 deficiency is aligned with critical vision of this company. I'm excited to support Mahzi in moving to the next phase of growth and bringing potential disease-modifying treatments to this ve..."

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Team

Yael Weiss Founder & CEO

Biotech pioneer. 20+ years in executive leadership in rare disease and pharmaceutical roles at Merck, Ultragenyx & Genzyme. Contributed to 4 FDA approvals including Januvia & Gardasil. Hebrew University MD. Weizmann Institute Molecular Biology PhD.

Aaron Olsen CFO

Finance maven . 20+ years in rare disease biotech. SVP Corporate Strategy & VP FP&A at Ultragenyx (10+ yrs). Director FP&A, BioMarin. CFO, Mahzi Therapeutics. Dartmouth College BA.

Samuel Collins Chief Medical Officer

Clinical strategist. 10+ years advancing rare disease programs from preclinical to Phase 3. Gene therapy, biologics & small molecule dev. Ex Pfizer, Edgewise, Actio. Secured ODD & Fast Track designations. Cambridge Medical Sciences MA. UCL MD. Durham MBA.

Christina Theodore-Oklota VP, Clinical Outcomes Research

Endpoint architect. Key contributor to Crysvita FDA approval for XLH. 10+ years designing clinical outcome assessments for rare diseases. Ex Ultragenyx & Genentech. Ohio State University Psychology BS. Brown T32 and Stanford Pediatric Psychology Fellow.

Jennifer Pavillard Senior VP, Regulatory Affairs & Quality

Regulatory veteran. 27+ years experience at Pfizer, GSK, Shire, AstraZeneca, Sanofi & BridgeBio. Led global submissions for rare disease & gene therapy approvals. Managed FDA Advisory Committee meetings. University of Maryland Animal Sciences BS.

Emily Radomile VP, Program Management & Clinical Operations

Operations wizard . 22+ years in drug development. Clinical ops lead for 2 FDA/EMA approvals: Crysvita (XLH) & Kanuma (LAL Deficiency). Ex Ultragenyx, Synageva, Annexon. Boston University Psychology BA. UMass Nursing BSN, Summa Cum Laude.

Chris Lorenz Chief Technical Officer

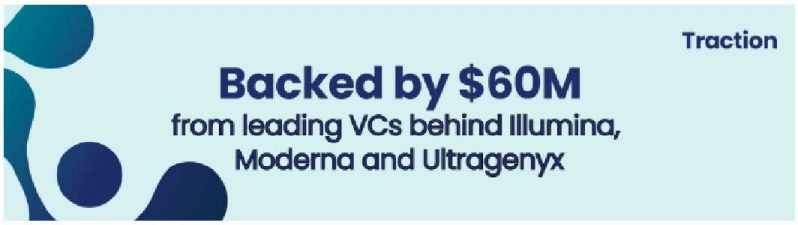
Manufacturing titan. Built Audentes' AAV GMP facility in 9 months. Led build-out of $100M commercial gene therapy plant in just 16 months. Key CMC role in $3B Astellas acquisition. Managed 250 FTEs, $180M budget. Stanford Chemical Engineering BS & MS.

Memo

Mahzi Therapeutics is a clinical-stage biotech developing precision genetic therapies for rare disorders that cause autism, seizures, and developmental disabilities in children. None of these disorders has an approved, disease-modifying treatment today.

Mahzi partners with patient advocacy groups to turn validated science into real therapies. Mahzi has now dosed the first patients in the first-ever gene therapy clinical trial for Pitt Hopkins Syndrome, marking the shift from preclinical promise to clinical execution – one of the most important value inflection points in biotech.

Led by a CEO with 20+ years at Merck, Genzyme, and Ultragenyx, Mahzi has advanced into clinical testing backed by $60M in funding from leading biotech venture firms Venrock, HealthCap, Droia Ventures, and HBM Partners, and $16M in non-dilutive funding from the California Institute for Regenerative Medicine (CIRM).

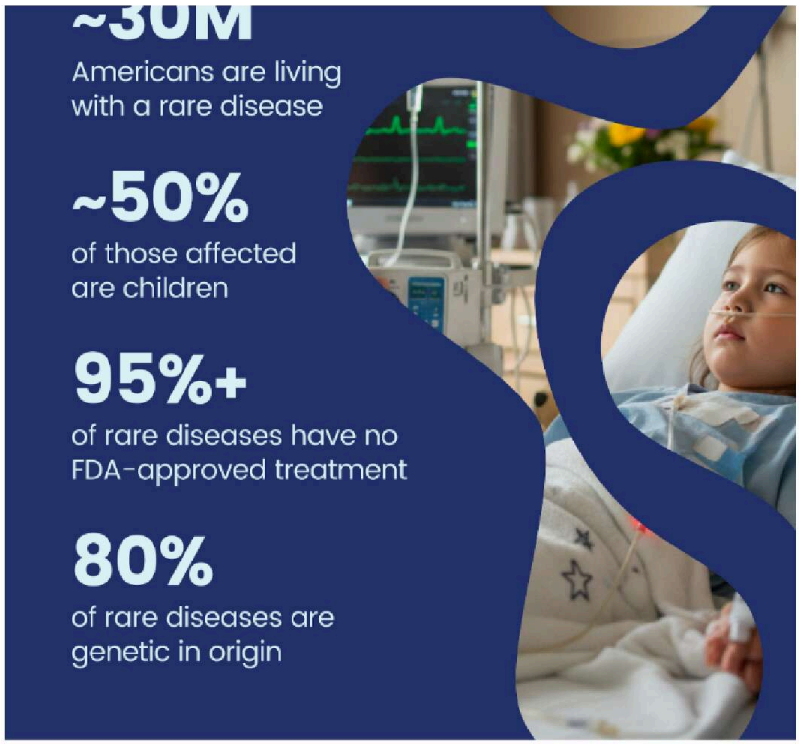


In January 2026, Mahzi became the first company ever to treat a patient with gene therapy for Pitt Hopkins Syndrome – a rare neurodevelopmental disorder with no approved treatments – and plans to treat all patients in the trial by the end of the year.

As the lead Pitt Hopkins program progresses in the clinic, Mazhi is simultaneously trailblazing to move a second gene therapy program into the clinic in 2026and advance several pre-clinical antisense oligonucleotide (ASO) programs supported by long-standing relationships with 10+ patient advocacy groups and academic collaborators.

With $76M in total funding, patients being treated in the clinic, and multiple programs advancing in parallel, Mahzi has rapidly moved beyond preclinical promise into real-world execution – a milestone few rare disease companies achieve.

30+ million Americans are living with rare diseases that impact their daily living and the lives of their families. While 10,000+ rare diseases are known, fewer than 500 have any approved treatment.



The barrier isn't science, it's economics. Many rare genetic disorders affect patient populations too small to meet the commercial thresholds of large pharmaceutical companies. As a result, even when science is well understood, these diseases are routinely deprioritized.

For decades, families living with rare genetic neurodevelopmental disorders were told their disorders were not treatable. A diagnosis often meant uncertainty, isolation, disruptive symptoms, and very few options beyond supportive care.

To fill this gap, families and patient advocacy foundations are funding academic research, partnering with leading universities, and validating therapeutic approaches. But foundations are not drug developers. They lack the clinical development and regulatory expertise, manufacturing infrastructure, and capital required to bring a therapy through FDA approval.

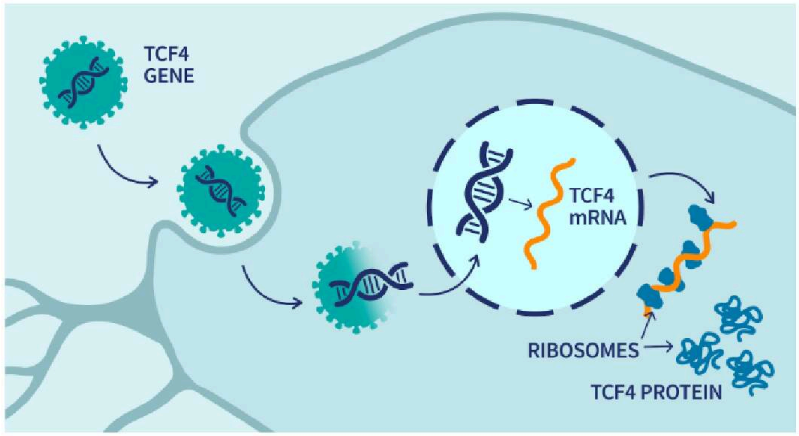
Rare neurological diseases represent a $16B+ market, and one of the fastest-growing segments within the broader $200B+ rare disease therapeutics space.

Advances in genetic testing are accelerating the diagnosis of rare diseases, while FDA orphan drug incentives — including seven years of market exclusivity, tax credits, and expedited review — have made the economics of development increasingly attractive. In 2023, more than half of all novel FDA drug approvals were for rare diseases.

Large biopharma companies increasingly acquire or partner with teams that already have clinical progress, rather than developing rare disease assets internally. This dynamic rewards companies with validated clinical assets, focused indications, and efficient execution.

Source: U.S. FDA, January 2024

Mahzi's lead programs are gene therapies for ultra-rare neurological disorders caused by single-gene mutations. Rather than managing symptoms, the approach targets the root cause by delivering a functional copy of the deficient gene, enabling cells to restore the levels of protein they lack.



Mahzi's lead program for Pitt Hopkins Syndrome is designed to restore the activity of TCF4, a protein essential for healthy brain development and function. Loss of TCF4 causes widespread neurological impairment and is modeled directly in animals.

In a well-established mouse model, Mahzi's gene therapy restored TCF4 expression after birth and drove measurable improvement, including better performance in learning, memory, spatial navigation, increased neuron connectivity, and behavior patterns closer to healthy littermates than untreated mice.

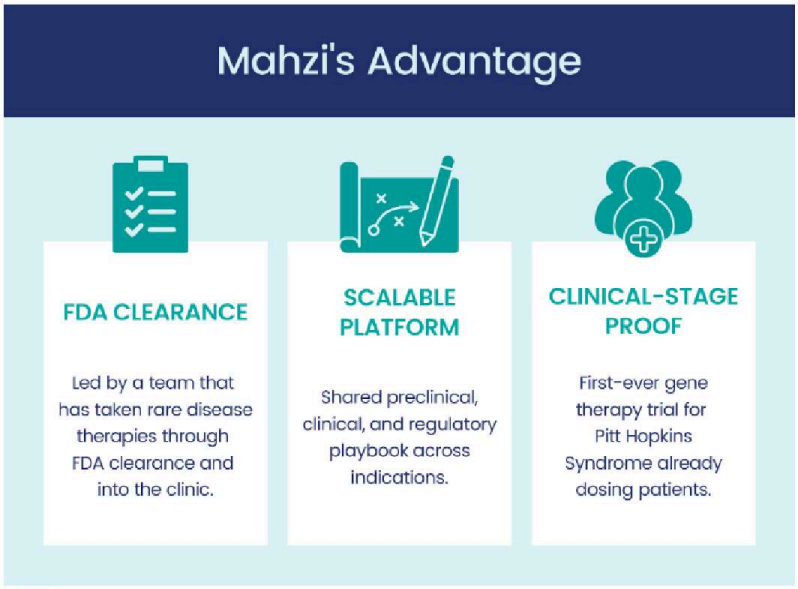
These results showed gene replacement could rescue both molecular function and higher-order behavior, a critical step in de-risking translation to humans.

In the first-ever gene therapy trial for Pitt Hopkins Syndrome, Mahzi is delivering its investigational gene therapy using AAV9 — a naturally occurring virus used in multiple FDA-approved gene therapies. The virus's genetic material was replaced with a functional human gene, creating a single-administration treatment designed for durable protein expression.

precision genetic therapies

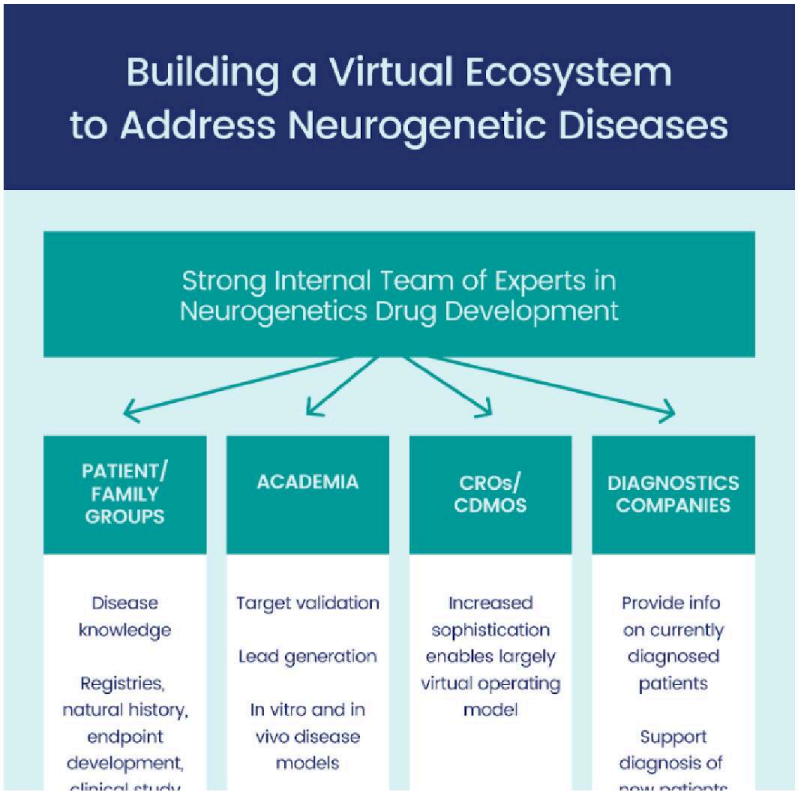
Mahzi takes validated biology and systematically translates it into regulated, clinical-stage investigational therapies. Each program leverages close collaboration with patient organizations, deep internal expertise in therapeutic development for neurodevelopmental disorders, and efficient clinical study design and execution, allowing new indications to move faster with lower incremental risk.

This repeatable approach to developing rare disease assets is why leading biotech investors and public funding agencies have backed Mahzi's team not as a research project, but as a scalable company.



Social Proof

Trusted by the people who know rare disease best



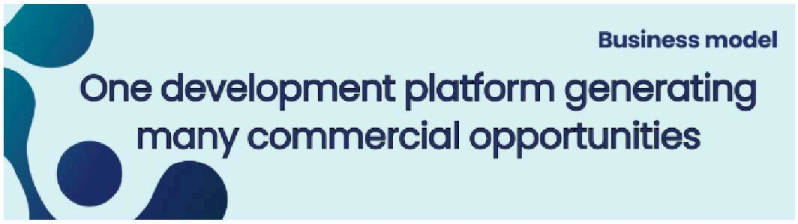
The Pitt Hopkins Research Foundation collaborated with Mahzi to advance its gene therapy into the clinic, resulting in the first-ever gene therapy trial for the disorder. Other organizations, including the WWOX Foundation and FamilieSCN2A Foundation, have also closely partnered with Mahzi in developing potential therapies.

Mahzi's CEO brings 20+ years at Merck, Genzyme, and Ultragenyx – companies that helped define the rare disease industry. A Termeer Fellow, Yael also led Mahzi to become the first recipient of the FamilieSCN2A Accelerator Award in 2025.

The leadership team brings comparable depth, with training from Stanford, Cambridge, Yale, UC Berkeley, and the Weizmann Institute, and hands-on experience leading clinical trials, regulatory submissions, and gene therapy manufacturing.

Mahzi's board includes senior biotech executives who built Alnylam (market cap $40

billion) and Zogenix (acquired by UCB for $1.9 billion) and investors with exits to Novo Nordisk, Merck, and Allergan - giants in the space.

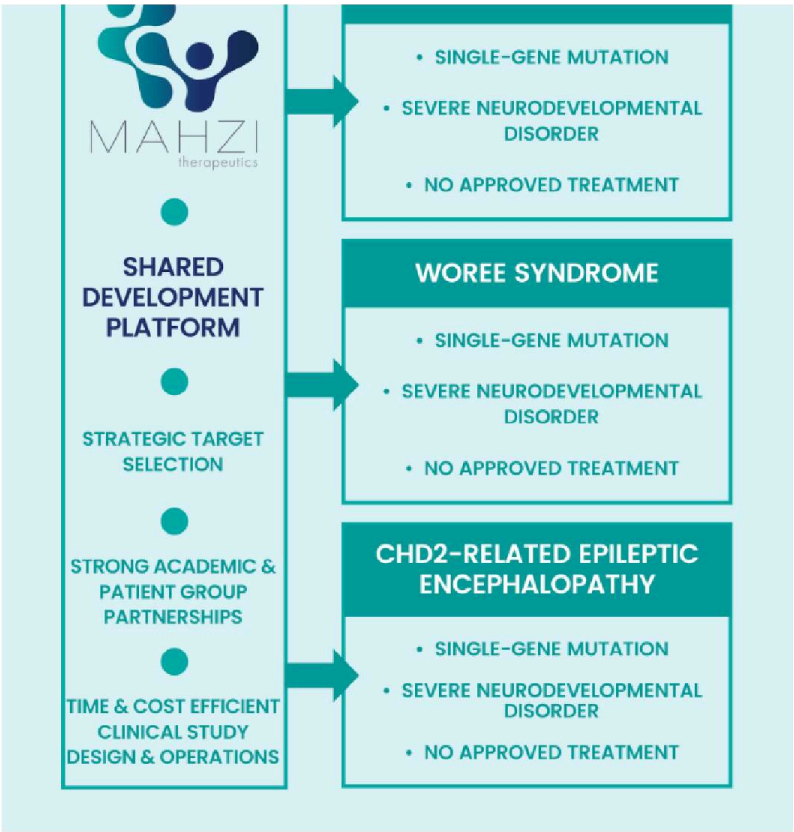


Each Mahzi indication represents a standalone commercial opportunity defined by clear genetic causality and limited competition, following a business model that has been validated in rare disease gene therapies.

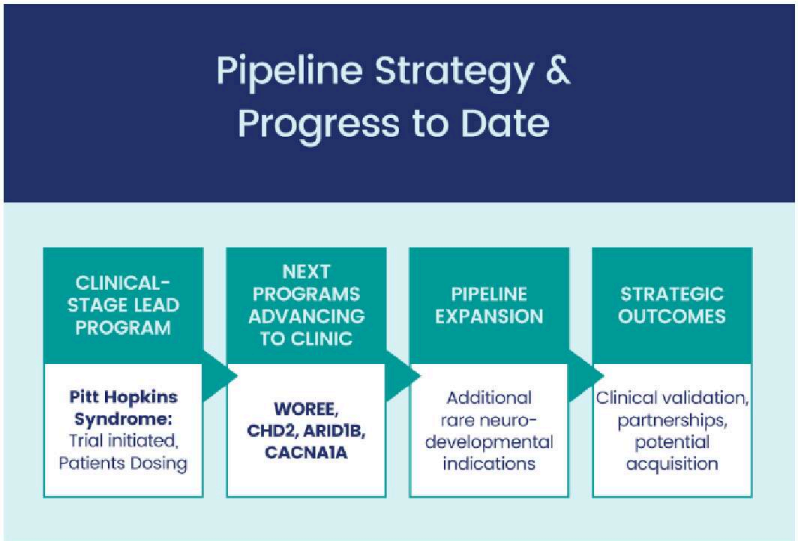
FDA-approved, one-time treatments such as Zolgensma and Luxturna have shown that severe genetic disorders with no alternatives can achieve treatment success and reimbursement that reflects both clinical value and durability.

In ultra-rare indications, pricing is driven by therapeutic impact rather than patient volume, allowing each program to function as an independent asset despite small populations. This dynamic enables meaningful per-indication economics without reliance on scale across a single disease.

In addition to direct commercialization, Mahzi may pursue selective regional partnerships after generating clinical data. These partnerships can provide non-dilutive capital and shared development risk, while allowing Mahzi to retain ownership of its core rare disease assets and platform.



With patients now being treated in the first-ever gene therapy trial for Pitt Hopkins Syndrome, Mahzi is focused on generating clinical data from its lead program while advancing additional therapies toward the clinic using the same development blueprint.



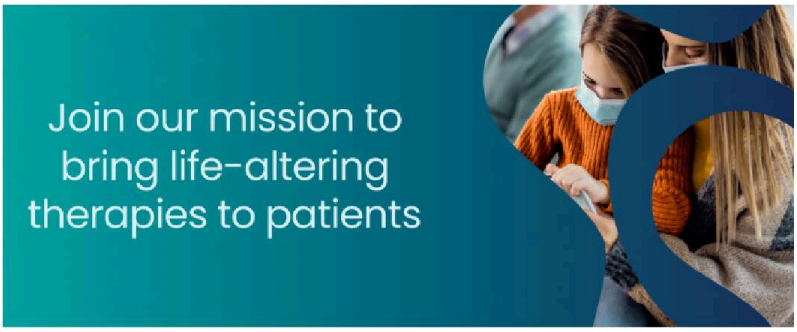
The most advanced follow-on program targets WOREE Syndrome (WWOX related epileptic encephalopathy), a severe neurodevelopmental and epileptic disorder caused by loss of a single gene and currently lacking any disease-modifying treatments. Mahzi is also progressing several additional antisense oligonucleotide (ASO) programs for neurogenetic disorders with clear genetic causality and limited or no competition.

Each milestone is designed to reduce development risk, demonstrate repeatable

execution, and position Mahzi for strategic partnerships or potential acquisition.

Rare disease therapies with clinical validation have driven major acquisitions.

Acquirers pay for clinical-stage assets plus teams that can repeat execution across multiple indications. Mahzi is building toward this established playbook with clinical progress, a reusable platform, and a team with multiple exits.



Mahzi is bringing long-awaited hope into the clinic. With patients being treated in an FDA-cleared trial, $76M in institutional and non-dilutive funding, and a robust pipeline of potentially transformative therapies, Mahzi is poised to lead in the rare neurodevelopmental disorder space.

Mahzi is seeking additional funding now to enable a complete data package for its lead indication for Pitt Hopkins syndrome and advance its next indication for WOREE syndrome to clinical trial readiness.

Invest in Mahzi today and help bring life-changing treatments to families who have waited too long for a solution.

wefunder.com/mahzitherapeutics