GAIN THERAPEUTICS

FACTSHEET | Q3 2024

Leading the Discovery of New Therapies with Allosteric Modulators

Gain Therapeutics, Inc. is a clinical-stage biotechnology company accelerating drug discovery and unlocking novel disease-modifying treatments. Deploying our highly advanced platform, we are identifying and targeting never-before-seen allosteric binding sites on disease-implicated proteins and proprietary small molecules with **first-in-class** or **best-in-class** profiles.

Lead Program with Disease-Modifying Potential in GBA1-Parkinson's disease

Borne of our proprietary platform, our lead program GT-02287 is in clinical development for the treatment of Parkinson's disease with or without a GBA1 mutation. GT-02287 is a an oral, brain penetrant small molecule that restores the function of the lysosomal enzyme glucocerebrosidase (GCase) which becomes misfolded and impaired due to mutations of the GBA1 gene, the most common genetic abnormality associated with PD. In preclinical models of PD, GT-02287 restored GCase enzymatic function, reduced aggregated α-synuclein, neuroinflammation and neuronal death, increased dopamine levels, completely restored motor function and reduced neurofilament light chain (NfL), an emerging biomarker for neurodegeneration, to normal levels. Based on this best-in-class preclinical data package, GT-02287 has the potential to **stop progression of Parkinson's** and other neurodegenerative diseases.

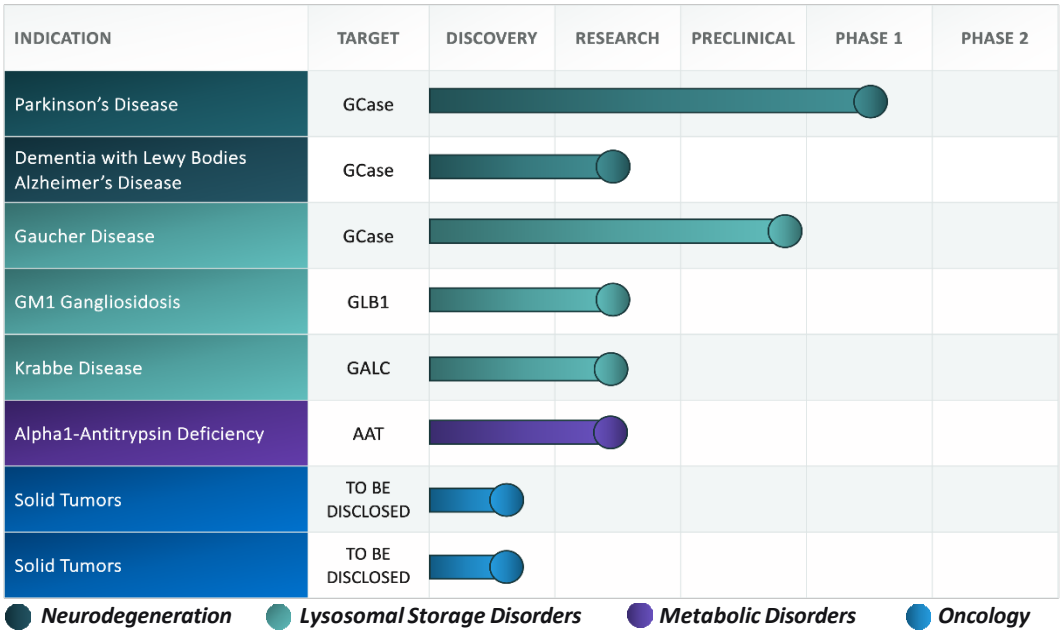
Our Magellan™ Drug Discovery Platform

Leveraging AI-supported structural biology, proprietary algorithms, and supercomputer-powered physics-based models, the company's Magellan™ drug discovery platform can identify novel allosteric binding sites on disease-implicated proteins, pinpointing pockets that cannot be found or drugged with current technologies. Its AI and machine-learning tools and virtual screening capabilities leverage the emerging on-demand compound libraries covering vast chemical spaces of over five trillion compounds to identify and select suitable small molecule hits for experimental validation.

Our Strategy for Value Creation

Pipeline Progression – Our lead program GT-02287 is advancing to a biomarker-based Phase 1b study to evaluate efficacy in Parkinson's disease patients and demonstrate proof of concept.

Grant Funding – We anticipate building on our track record of securing non-dilutive grant funding to advance our innovative science and product pipeline.



EQUITY OVERVIEW

Ticker (Exchange)	GANX (NASDAQ-GM)
Stock Price (USD)	$1.35
Market Cap	~$34.5M
Cash[1]	~$16.9M
Outstanding Shares[2]	25.1M
52-Week Range	$0.89 - $5.33
Avg. Daily Volume	~341,400
Analyst Coverage	BTIG – Thomas Schrader Oppenheimer & Co. – Jay Olson H.C. Wainwright - Raghuram Selvaraju Chardan – Keay Nakae Maxim – Jason McCarthy

All figures as of 8/21/2024 unless otherwise noted (Source: NASDAQ)
[1] Cash, cash equivalents and marketable securities as of June 2024
[2] Form 10-Q filed on 8/08/2024

INVESTMENT HIGHLIGHTS

Clinical-stage lead program GT-02287 for Parkinson's disease with multiple value inflection points in upcoming 12 months

Clinically validated AI-drug discovery platform now accesses chemical space in excess of five trillion compounds

Favorable safety profile observed in Phase 1 Study of GT-02287

UPCOMING MILESTONES

2H 2024

- Start of Phase 1b POC clinical trial in Parkinson's disease patients
- Type B FDA meeting
- Presentation at International Congress of Parkinson's Disease and Movements Disorders
- Presentation at Neuroscience 2024

1H 2025

- Potential clinical proof of concept based on biomarkers of GBA1-PD (interim data from Phase 1b clinical trial)
- Pre-IND meeting with FDA in preparation of Phase 2 clinical trial

CONTACT

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