GAIN THERAPEUTICS

Factsheet | Q4 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 idiopathic and GBA1 Parkinson's disease™

Borne of Magellan™, our proprietary drug discovery 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. Preclinical data in models of both GBA1-PD and idiopathic PD, demonstrating a disease-modifying effect after administration of GT-02287, suggests that GT-02287 may have the potential to **slow or stop the progression of Parkinson's disease**.

Phase 1 Study of GT-02287™

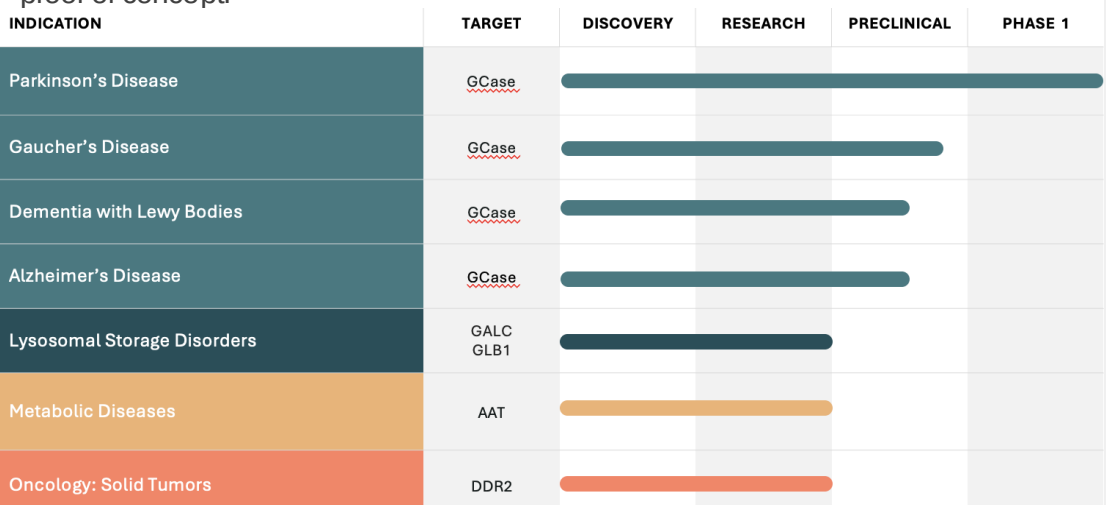
The Phase 1 first-in-human study of GT-02287 enrolled 72 healthy volunteers, males and females, up to the age of 64 years (approximately 15% of participants were age 50 or older). The single and multiple dose levels tested were safe and generally well tolerated, with no serious adverse events or Grade 3 (severe) adverse events observed, and no other safety signals detected. The PK profile of GT-02287 was linear across the tested dose ranges, and plasma exposures at daily doses of 7.7 mg/kg and above were within the projected therapeutic range. GT-02287 was **measurable in cerebrospinal fluid (CSF)** at levels in line with rodent levels at effective doses, demonstrating CNS exposure. Notably, **GCase activity** in dried blood spots **increased approximately 53%** in subjects who received GT-02287 but not in those who received placebo, demonstrating **target engagement and modulation of GCase enzyme.**

Initiation of a Phase 1b POC clinical trial in Parkinson's disease patients

- **3 month treatment**
- **Open label**
- **Primary Endpoint:** Safety and tolerability in Parkinson's disease patients

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.



EQUITY OVERVIEW

Ticker (Exchange)	GANX (NASDAQ-GM)
Stock Price (USD)	$2.92
Market Cap	~$75M
Cash[1]	~$16.9M
Outstanding Shares[2]	25.1M
52-Week Range	$0.89 - $5.33
Avg. Daily Volume	~366,000
Analyst Coverage	BTIG – Thomas Schrader Oppenheimer & Co. – Jay Olson H.C. Wainwright - Raghuram Selvaraju Chardan – Keay Nakae Maxim – Jason McCarthy

All figures as of 10/16/2024 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 moving into Phase 1b clinical trial in Parkinson's disease patients

Positive results from Phase 1 Study of GT-02287 including safety and tolerability, presence in cerebrospinal fluid, and target engagement and modulation of GCase enzyme

GT-02287 demonstrates disease-modifying capacity in GBA1 and idiopathic Parkinson's disease models

UPCOMING MILESTONES

2H 2024

- Start of Phase 1b POC clinical trial in Parkinson's disease patients
- Pre-IND meeting

1H 2025

- Potential clinical proof of concept based on biomarkers of GBA1-PD
- Interim data from Phase 1b clinical trial expected midpoint 2025

CONTACT

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