

## 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 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**.

### Ongoing 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 through a biomarker-based Phase 1b study to evaluate efficacy in Parkinson's disease patients and demonstrate proof of concept.

INDICATION	TARGET	DISCOVERY	RESEARCH	PRECLINICAL	PHASE 1
Parkinson's Disease	GCase				
Gaucher's Disease	GCase				
Dementia with Lewy Bodies	GCase				
Alzheimer's Disease	GCase				
Lysosomal Storage Disorders	GALC GLB1				
Metabolic Diseases	AAT				
Oncology: Solid Tumors	DDR2				

## EQUITY OVERVIEW

Ticker (Exchange)	GANX (NASDAQ-GM)
Stock Price (USD)	\$1.95
Market Cap	~\$57M
Cash¹	~\$10.4M
Outstanding Shares²	26.5M
52-Week Range	\$0.89 - \$3.19
Avg. Daily Volume	~278,000

Analyst Coverage	BTIG – Thomas Schrader
	Oppenheimer & Co. – Jay Olson
	H.C. Wainwright - Raghuram Selvaraju
	Chardan – Keay Nakae
	Maxim – Jason McCarthy
	ROTH - Boobalan Pachaiyappan
	Scotiabank – Louise Chen

All figures as of 05/07/2025 unless otherwise noted (Source: NASDAQ)

¹ Cash, cash equivalents and marketable securities as of December 31, 2024

² Form 10-K filed on 03/27/2025

## INVESTMENT HIGHLIGHTS

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

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

## UPCOMING MILESTONES

### 1H 2025

- First biomarker analysis from Phase 1b study in people with Parkinson's disease

### 2H 2025

- IND submission
- Start of Phase 2 planning
- Complete analysis of Phase 1b study

## CONTACT

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