



GAIN THERAPEUTICS

Corporate Deck

February 2026

NASDAQ: GANX

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Certain statements set forth in this presentation are forward-looking and reflect the Company's plans, beliefs, expectations and current views with respect to, among other things, future events and financial performance (collectively referred to herein as "forward-looking statements"). Forward-looking statements can be identified by the fact that they do not relate strictly to historical or current facts and are often characterized by the use of words such as "believe," "can," "could," "potential," "plan," "predict," "goals," "seek," "should," "may," "may have," "would," "estimate," "continue," "anticipate," "intend," "expect" or by discussions of strategy, plans or intentions. Such forward-looking statements involve known and unknown risks, uncertainties, assumptions and other important factors that could cause our actual results, performance or achievements or industry results to differ materially from historical results or any future results, performance or achievements expressed, suggested or implied by such forward-looking statements.

These include, but are not limited to, statements about the Company's ability to develop, obtain regulatory approval for and commercialize its product candidates; the timing of future IND submissions, initiation of preclinical studies and clinical trials, and timing of expected clinical results for our product candidates; the Company's success in early preclinical studies, which may not be indicative of results obtained in later studies or clinical trials; the outbreak of the novel strain of coronavirus disease, COVID-19, which could adversely impact our business, including our preclinical studies and any future clinical trials; the potential benefits of our product candidates; the Company's ability to obtain regulatory approval to commercialize our existing or any future product candidates; the Company's ability to identify patients with the diseases treated by our product candidates, and to enroll patients in clinical trials; the success of our efforts to expand our pipeline of product candidates and develop marketable products through the use of our Magellan platform; the Company's expectations regarding collaborations and other agreements with third parties and their potential benefits; the Company's ability to obtain, maintain and protect our intellectual property; the Company's reliance upon intellectual property licensed from third parties, including the license to use the Company's Magellan platform; the Company's ability to identify, recruit and retain key personnel; the Company's financial performance; developments or projections relating to the Company's competitors or industry; the impact of laws and regulations; the Company's expectations regarding the time during which it will be an emerging growth company under the JOBS Act; and other factors and assumptions described in the Company's public filings.

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GANX Corporate Highlights

Lead Program GT-02287 Moving to Phase 2 Clinical Evaluation in Parkinson's Disease



- Studies completed in both **Phase 1a healthy volunteers** and **Phase 1b open-label trial in PD** (90-day) with 9-month open-label extension ongoing
- Biomarker evidence from Phase 1b supports **disease modifying hypothesis** for GT-02287

Multiple Assets in Discovery and Preclinical Development



- Assets discovered and developed with our **proprietary Magellan AI platform**
- Initial disease targets include neurodegenerative diseases, lysosomal storage disorders including Gaucher disease as well as metabolic disease and solid tumors

Strong Intellectual Property Estate



- Gain retains full WW rites to GT-02287 with composition of matter **patent protection through 2038** not including Hatch Waxman extension for R&D
- Patent applications for 5 NCE families under review

Upcoming Milestones



- Complete FDA IND review – **1H 2026**
- GT-02287 Phase 1b study extension analysis – **2H 2026**
- Commencement of Phase 2 in people with Parkinson's disease – **2H 2026**

Leadership: Extensive Biotech and Pharma Experience



Gene Mack, MBA
Chief Executive Officer



Jonas Hannestad, MD, PhD
Chief Medical Officer



Gianluca Fuggetta
Senior Vice President,
Finance



Joanne Taylor, PhD
SVP Research



Terenzio Ignoni, PharmD
SVP Technical
Operations



Gain Therapeutics Pipeline

ASSET	INDICATION	TARGET	DISCOVERY	RESEARCH	PRECLINICAL	PHASE 1
GT-02287	<i>Parkinson's Disease</i>	GCase	[Progress bar spanning Discovery, Research, Preclinical, and Phase 1]			
	<i>Gaucher's Disease</i>	GCase	[Progress bar spanning Discovery, Research, and Preclinical]			
	<i>Dementia with Lewy Bodies</i>	GCase	[Progress bar spanning Discovery and Research]			
	<i>Alzheimer's Disease</i>	GCase	[Progress bar spanning Discovery and Research]			
Multiple Undisclosed	<i>Lysosomal Storage Disorders</i>	GALC GLB1	[Progress bar spanning Discovery and Research]			
Undisclosed	<i>Metabolic Diseases</i>	AAT	[Progress bar spanning Discovery and Research]			
Multiple Undisclosed	<i>Oncology: Solid Tumors</i>	DDR2	[Progress bar spanning Discovery and Research]			

GT-02287 – Parkinson’s Disease

Mechanism

Binds at allosteric site, chaperones and modulates glucocerebrosidase enzyme (GCase)

Disease Modification

Restores GCase function and improves disease cascade and neuronal survival

Efficacy

Pre-clinical: Demonstrates a disease modifying effect in models of GBA-1 and iPD
Phase 1b: Demonstrates statistically significant biomarker evidence of disease modifying effect with improvement in clinical presentation based on MDS-UPDRS scores

Safety/Tolerability

Well-tolerated in both healthy volunteers and PD patients with therapeutic CNS exposures achieved

Next Steps

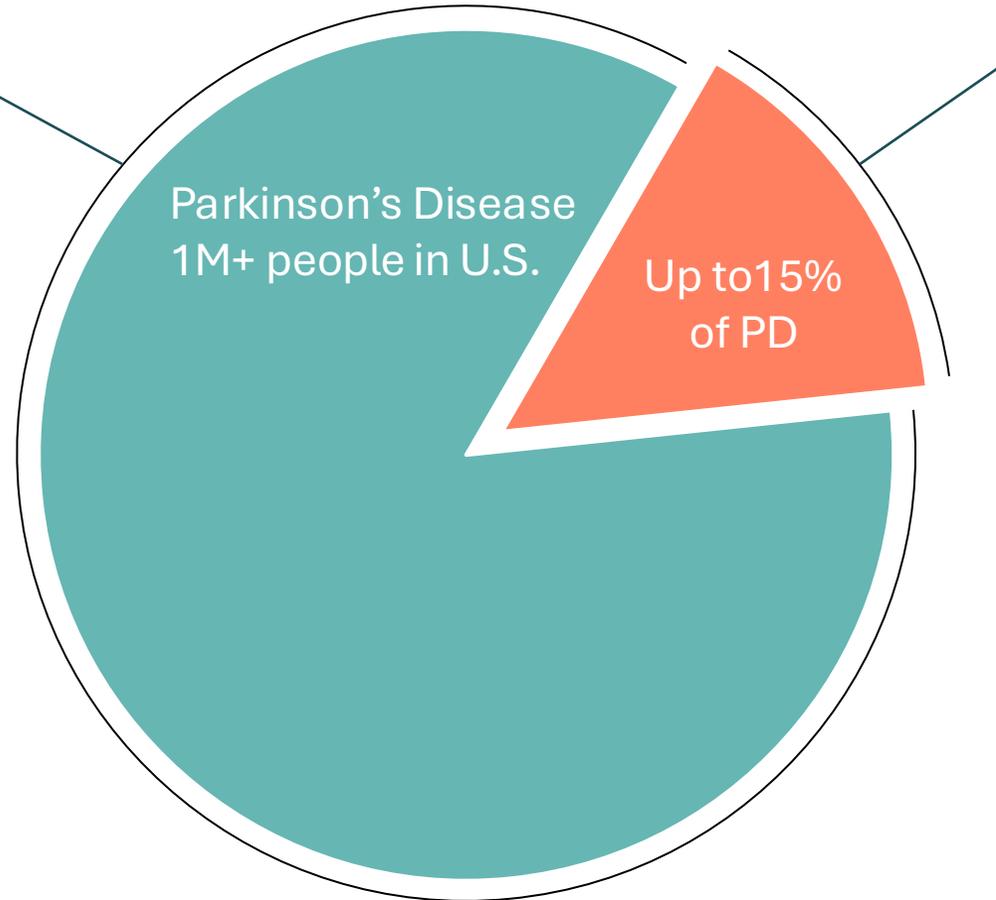
- Phase 1b Initial 90-day dosing (Part 1) complete 4Q25; Open-label extension ongoing
- Phase 2 trial initiation planned for 3Q26

Parkinson's Disease – Unmet Medical Need for Disease Modifying Therapy

Parkinson's Disease

U.S. Market Potential:
\$4B

- Second most common neurodegenerative disease after Alzheimer's
- Existing therapies address symptoms without slowing progression
- People with PD inevitably get worse over time
- High need for therapy that slows disease progression

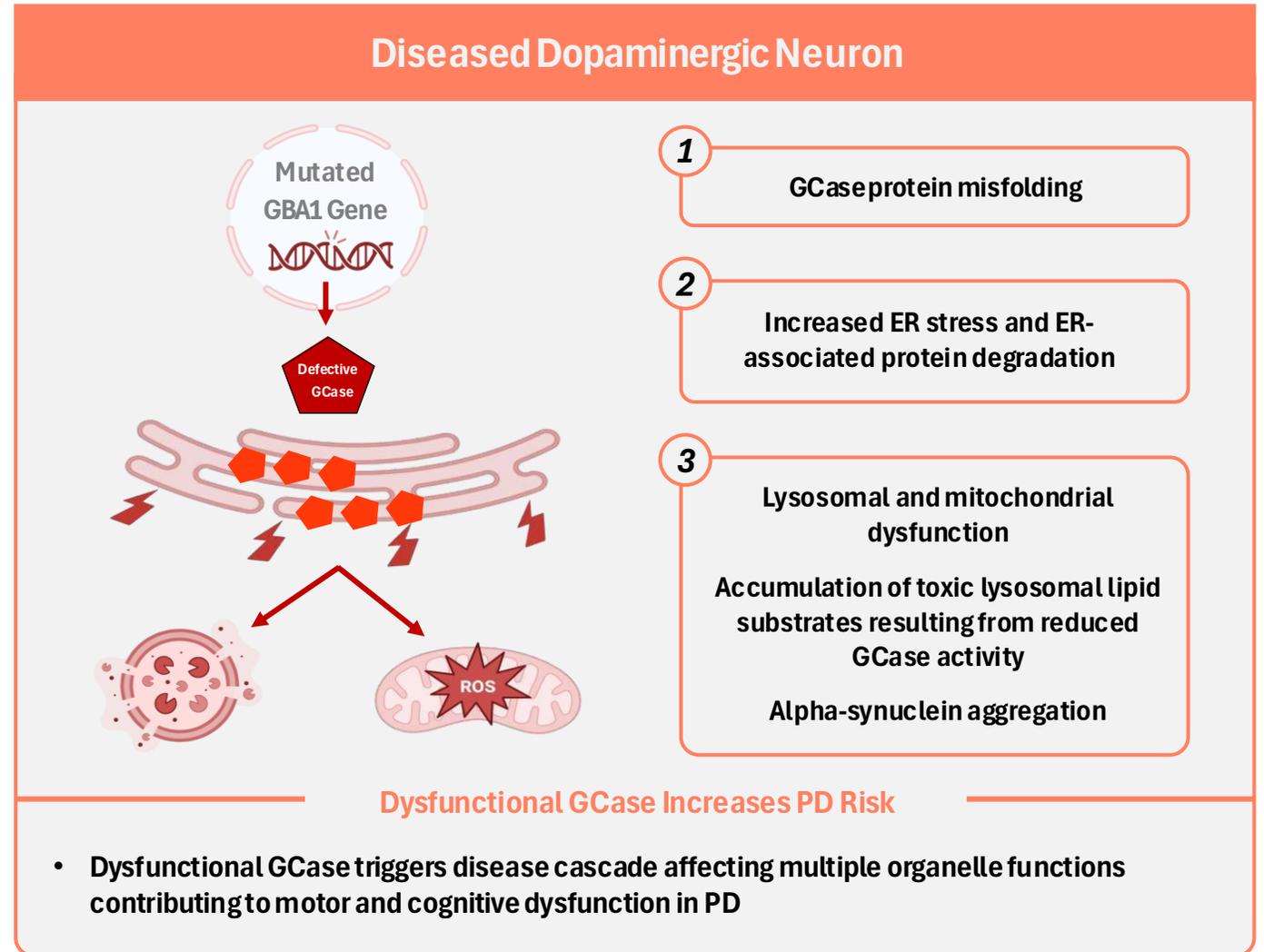
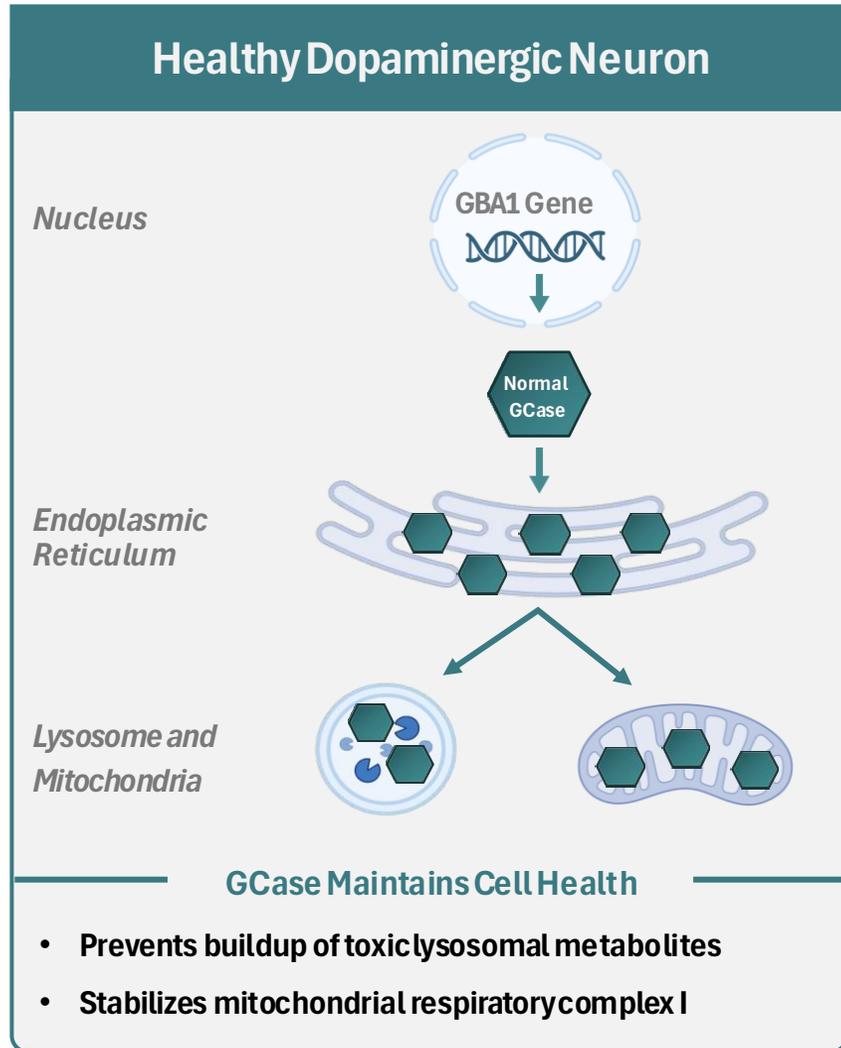


GBA1-PD

U.S. Market Potential:
\$3B

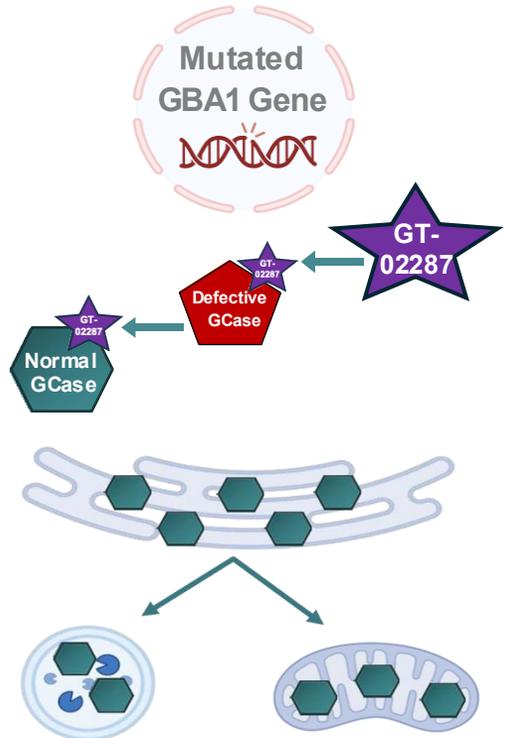
- Most common genetically-defined subpopulation of PD
- GBA1 variants cause misfolding of glucocerebrosidase (GCCase)
- Considerable overlap in pathobiology between GBA-PD and idiopathic PD
- A therapy targeting GCCase-related pathway abnormalities could slow disease progression in both GBA-PD and idiopathic PD

GCCase is Essential to Cell Pathways Involved in Both Maintenance and Energy Production



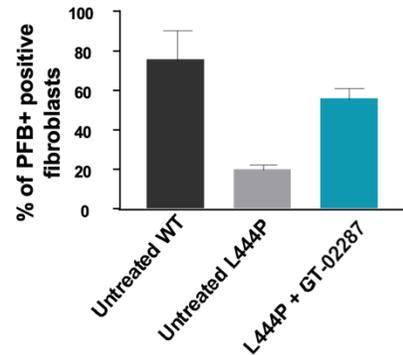
Allosteric Modulator GT-02287 Restores GCase Function: Improves Disease Cascade to Promote Neuronal Survival (i.e., Disease Modifying)

Dopaminergic Neuron with Restored GCase Function

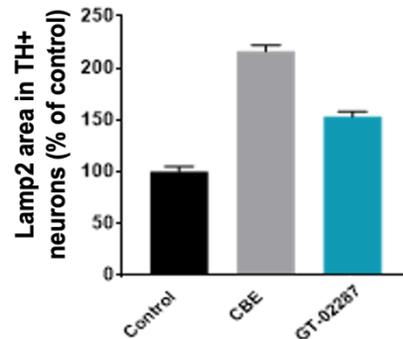


- Corrects protein misfolding
- Restores enzymatic activity

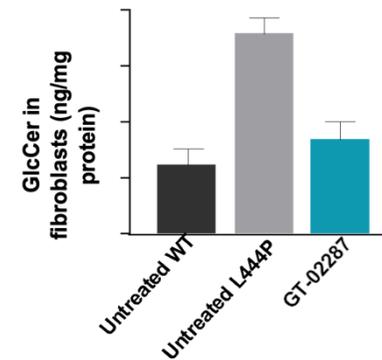
GCase Activity Restoration



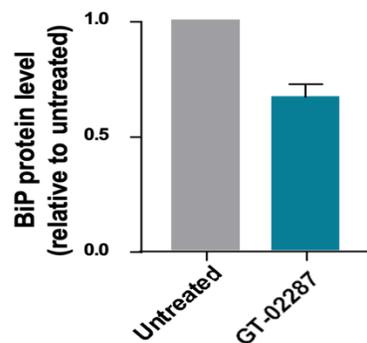
Restored Lysosomal Function



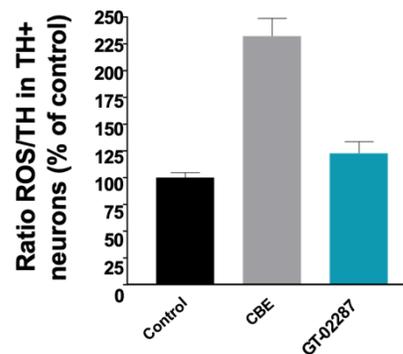
Toxic Substrate Depletion



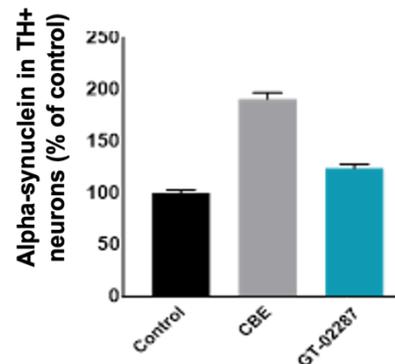
Reduced ER Stress



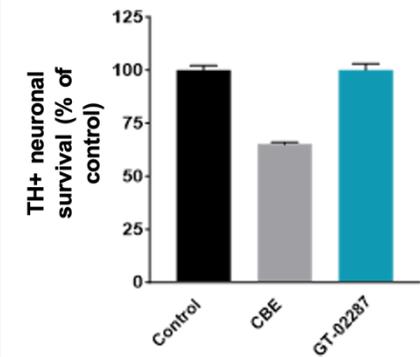
Restored Mitochondrial Function



Alpha-Synuclein Depletion



Neuronal Survival



GT-02287 Displays a Disease Modifying Effect in Animal Models of GBA1 and iPD

GBA1- PD Model (CBE+PFFs)

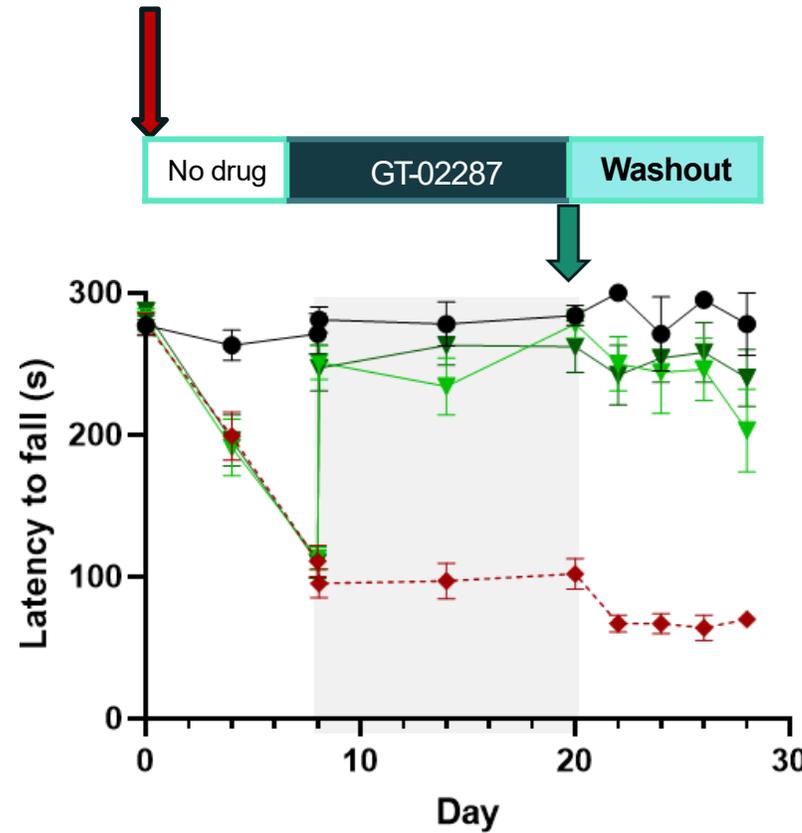
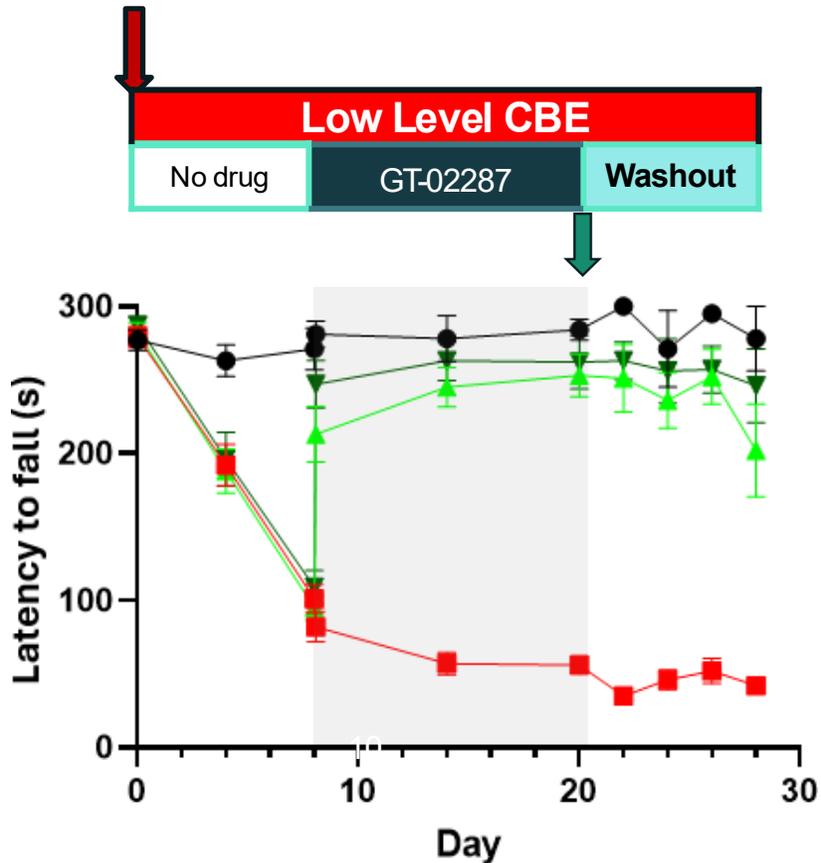
Idiopathic PD Model (PFFs only)



Mouse Wire Hang Washout

α -Syn PFFs

α -Syn PFFs



- Control
- CBE/PFFs
- ◆ PFFs
- ▲ GT-02287 60mg/Kg
- ▼ GT-02287 90mg/Kg

GT-02287 is Well-Tolerated in SAD/MAD and Bioavailability Study and Shows GCase Engagement

96 Healthy Volunteers in SAD/MAD and Bioavailability Studies

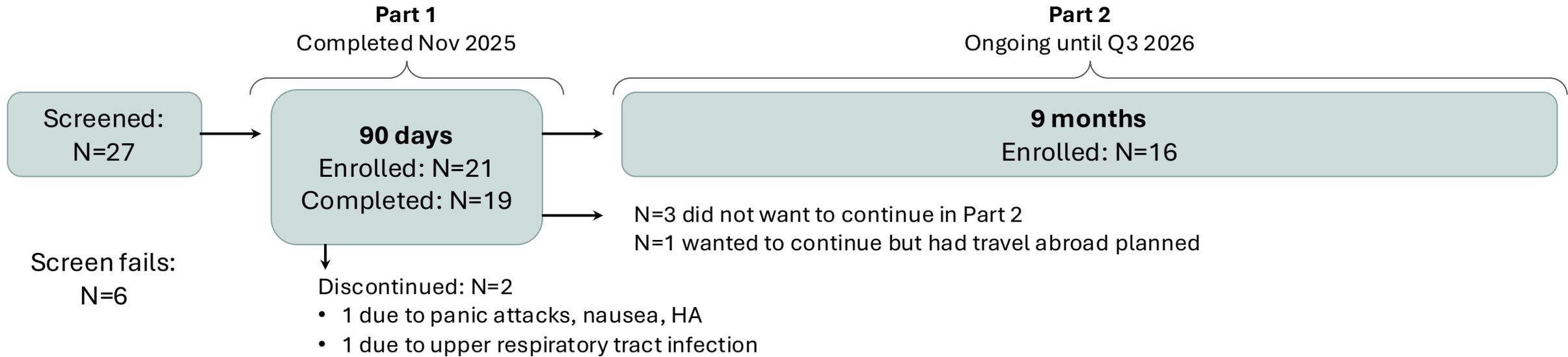
- Single and multiple dose levels tested were **generally well tolerated, with no serious adverse events** or Grade 3 (severe) adverse events observed, and no other safety signals detected
- Most common TEAEs in MAD were nausea (32%), abdominal pain (8%), diarrhea (8%), headache (8%)
- **Therapeutic exposure levels achieved** *vis-à-vis* preclinical models
- **CNS exposure** comparable to that observed in rodents

GCase Activity in Dried Blood Spots (DBS)

- GCase activity in dry blood spots was measured in MAD Cohort 4
- In GT-02287 subjects, 5 out of 6 had increased GCase activity. No increase was observed in placebo subjects.

**53% increase in GCase activity
observed by Day 14 ($p < 0.001$)**

Phase 1b Study Overview: 90 day (Part 1) and Open-Label Extension (Part 2)



- GANX-001-V102 is a two-part, open-label, Phase 1b study in people with Parkinson’s disease
- Objectives: Safety and tolerability, plasma PK, mechanistic biomarkers, and clinical progression
- Phase 1 SAD/MAD First-in-human study demonstrated GCase target engagement in healthy volunteers

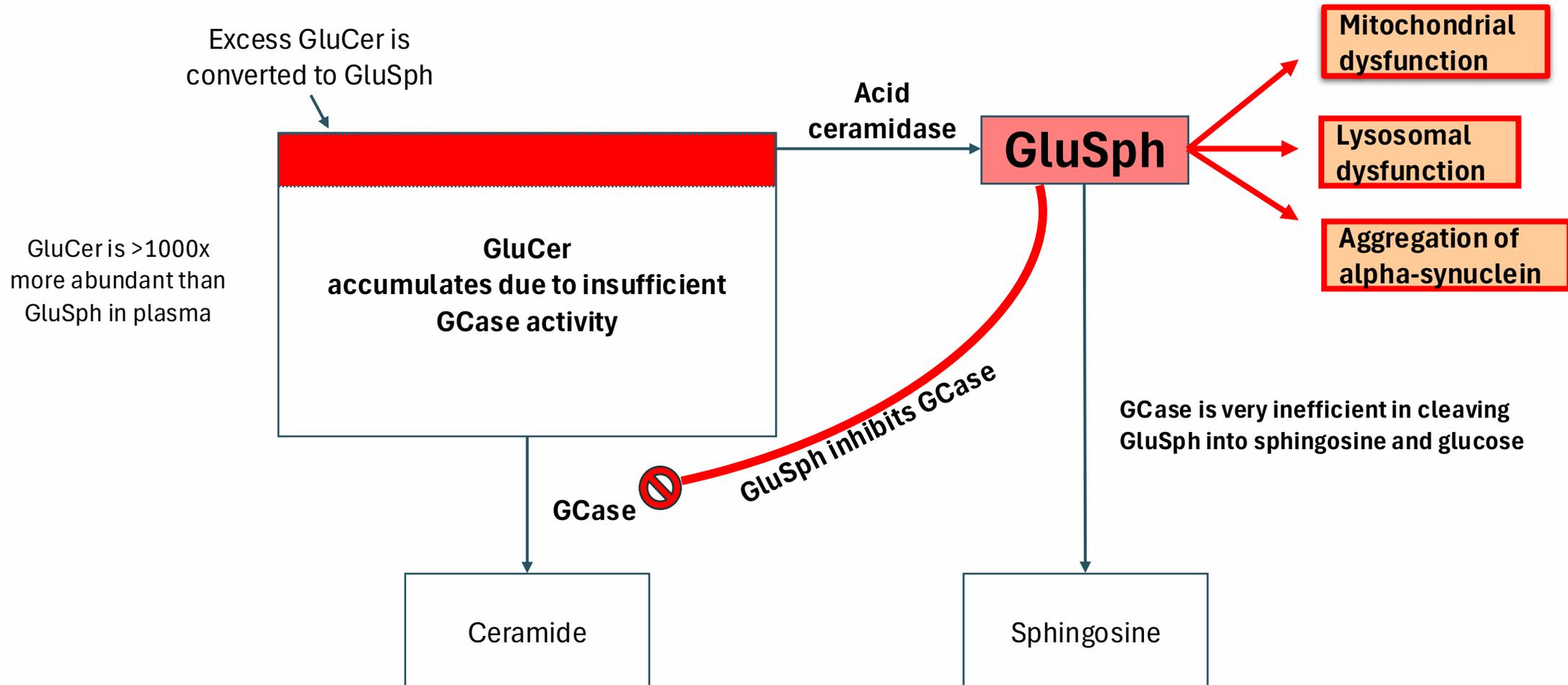
Endpoints Objectives

Primary To evaluate the safety and tolerability of GT-02287

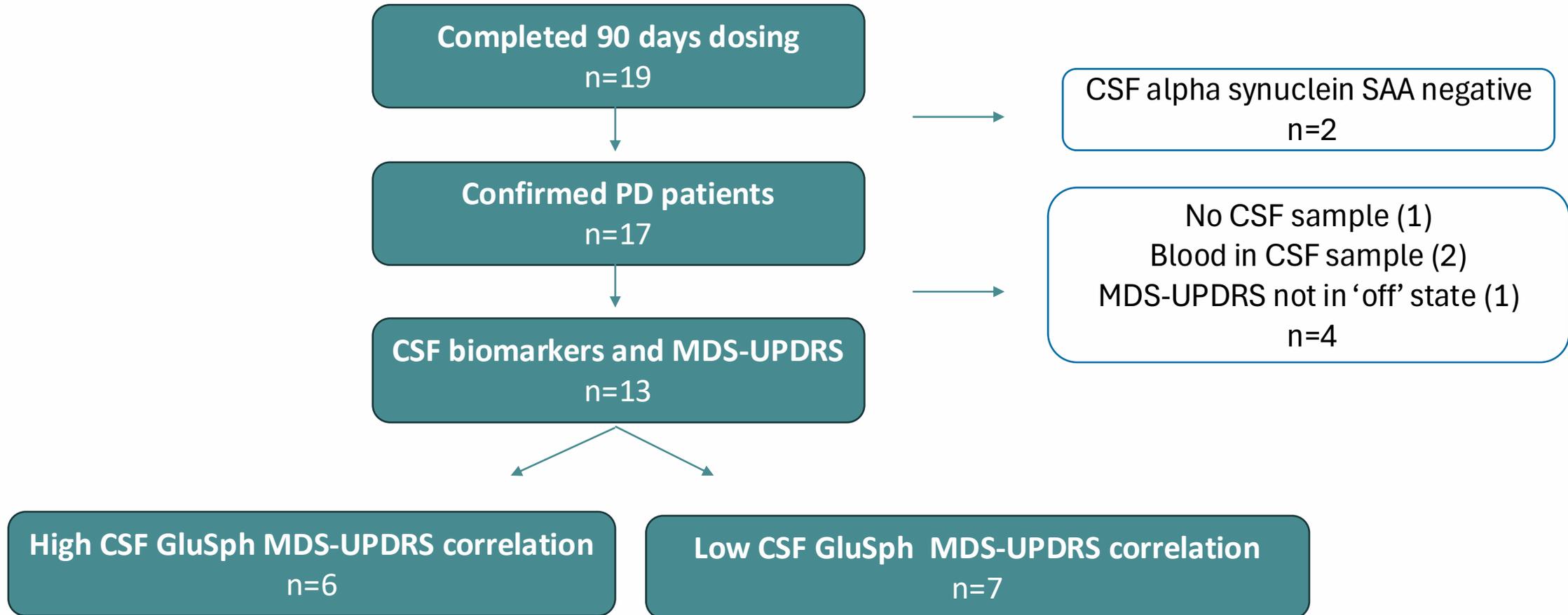
Secondary To characterize the single-dose and steady state plasma PK profile of GT-02287
To assess levels of GT-02287 in CSF after at least 12 weeks of daily administration in participants with PD

Exploratory Pharmacodynamic response to GT-02287 via biomarkers analysis of plasma, whole blood, blood cells, and CSF samples
To explore the effect of GT-02287 on scores from selected clinical scales and questionnaires over a 90-day treatment

Decreased GCCase Leads to Excess GluCer and Consequently Increased GluSph in Parkinson's

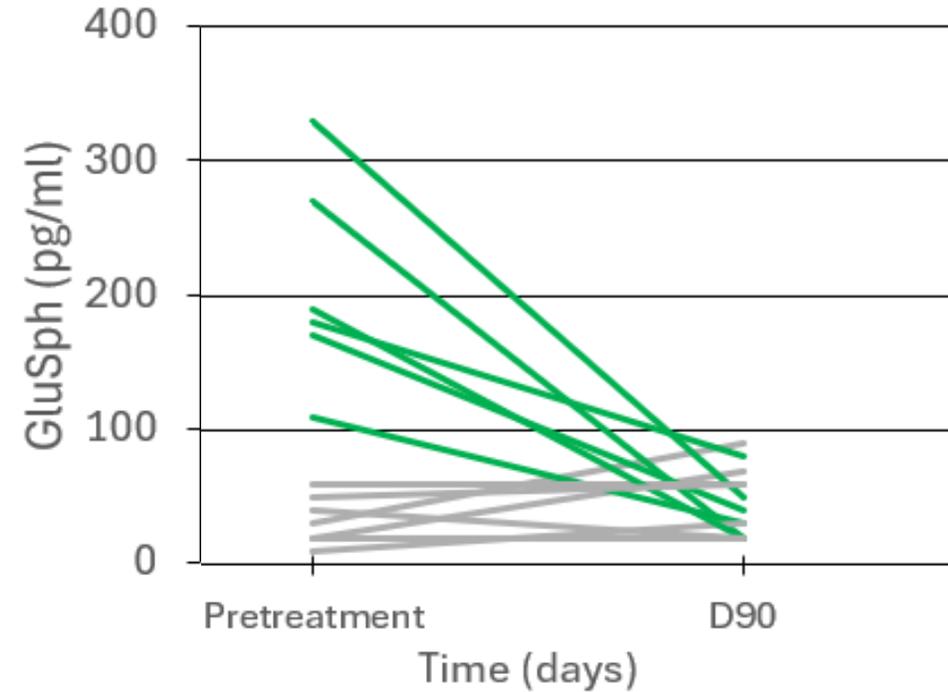
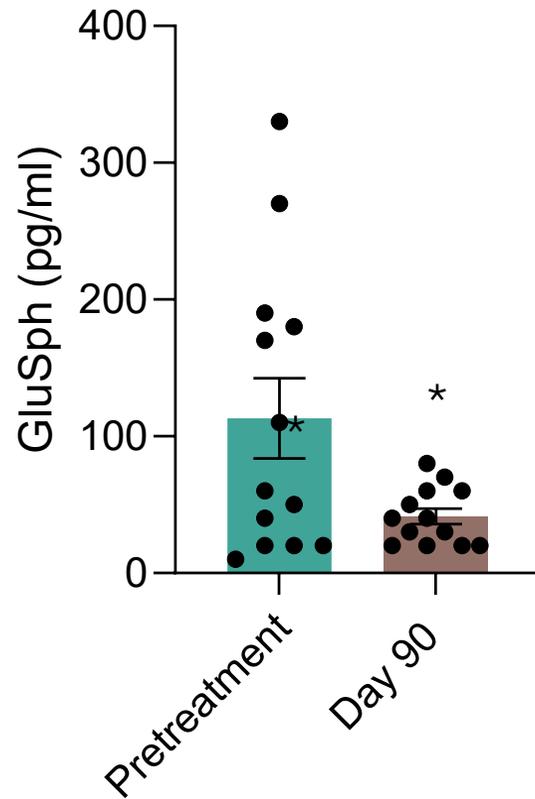


Phase 1b Evaluable Population for CSF Biomarker Analysis and MDS-UPDRS Scores



GT-02287 Treatment Leads to a Decrease in CSF GluSph, a Prespecified Endpoint, Demonstrating CNS Target Engagement

CNS GluSph N=13



PD Patients With High Levels of CSF GluSph at Baseline Show a Markedly Greater Improvement in MDS-UPDRS Motor Scores

Negative score indicates improvement of symptoms

All patients for which CSF GluSph and UPDRS scores available (n=13)

Part II	Part III	Part II+III
-0.54	-2.00	-2.54

Patients with low CSF GluSph (n=7)

Part II	Part III	Part II+III
0.13	0.50	0.63

Patients with high CSF GluSph (n=6)

Part II	Part III	Part II+III*
-1.5	-4.67	-6.17

GT-02287 – Parkinson’s Disease

Mechanism

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Efficacy

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Phase 1b: Demonstrates statistically significant biomarker evidence of disease modifying effect with improvement in clinical presentation based on MDS-UPDRS scores

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Next Steps

- Phase 1b Initial 90-day dosing (Part 1) complete 4Q25; Open label extension ongoing
- Phase 2 trial initiation planned for 3Q26

**GT-02287 acts on brain Gcase,
reduces toxic substrate GluSph, and leads to
improvements seen in clinical MDS-UPDRS scores**

GT-02287 – Translation From Animal Models to Clinic

- **Disease modifying properties GT-02287 demonstrated in earlier studies and preclinical animal models are now reflected in our Phase 1b clinical biomarker and functional readouts, supporting successful translation into individuals with Parkinson’s disease.**
 - 100% of individuals with baseline elevation in CSF GluSph benefitted from an average 81% reduction in GluSph to normal levels ($p<0.05$) after 90 days of treatment with GT-02287.
 - Those individuals with elevated baseline CSF GluSph also benefitted from a statistically significant improvement in MDS-UPDRS Parts II and III scores of 6.17 points ($p<0.05$).
- **GT-02287 is well tolerated** with 16 of 19 patients choosing to remain on treatment for the 9-month open-label extension to the Phase 1b completed in November. The **open-label extension will complete in September 2026.**
- **Correlative biomarker analysis remains ongoing** from the initial 90-day treatment duration with additional details to be presented at upcoming conferences including **AD/PD 2026 in March 2026.** Additional data including MDS-UPDRS updates from the open-label extension will also be presented at upcoming conferences.
- **A Phase 2 safety and efficacy trial is planned** to initiate in early 2H26 that will further evaluate tolerability, biological evidence of substrate reduction, and clinical outcomes on MDS-UPDRS scores.

Company Background

Corporate Background

- Established in 2017
- 25 employees in three locations: HQ in Bethesda, Maryland, Lugano, Switzerland, Barcelona, Spain
- Founder and Executive Chairman: Dr. Khalid Islam

Analyst Coverage

BTIG	<i>Tom Shrader, Ph.D., CFA</i>
Oppenheimer & Co	<i>Jay Olson, CFA</i>
H.C. Wainwright	<i>Ram Selvaraju, Ph.D.</i>
Maxim	<i>Jason McCarthy, Ph.D.</i>
ROTH	<i>Boobalan Pachaiyappan, Ph.D.</i>

Financial and Stock Data

IPO (NASDAQ: GANX)

- March 2021
- Led by BTIG and Oppenheimer & Co.

CAPITAL STRUCTURE

- 36.0 million shares outstanding
- No debt*

CASH POSITION

- \$8.8 million as of September 30, 2025

GRANT SUPPORT

- Michael J. Fox Foundation for Parkinson's Research
- The Silverstein Foundation for Parkinson's with GBA
- Innosuisse (Swiss Innovation Agency)

