



GAIN THERAPEUTICS

Corporate Deck

March 2026

NASDAQ: GANX

Forward-Looking Statements

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.

These statements are based on the Company's historical performance and on its current plans, estimates and projections in light of information currently available to the Company, and therefore, you should not place undue reliance on them. The inclusion of forward-looking information should not be regarded as a representation by the Company or any other person that the future plans, estimates or expectations contemplated by us will be achieved. Forward-looking statements made in this presentation speak only as of the date of this presentation, and the Company undertakes no obligation to update them in light of new information or future events, except as required by law.

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Industry Information Market data and industry information used throughout this presentation are based on management's knowledge of the industry and the good faith estimates of management. We also relied, to the extent available, upon management's review of independent industry surveys and publications and other publicly available information prepared by a number of third-party sources. All of the market data and industry information used in this presentation involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. Although we believe that these sources are reliable, we cannot guarantee the accuracy or completeness of this information, and we have not independently verified this information. While we believe the estimated market position, market opportunity and market size information included in this presentation are generally reliable, such information, which is derived in part from management's estimates and beliefs, is inherently uncertain and imprecise. No representations or warranties are made by the Company or any of its affiliates as to the accuracy of any such statements or projections. Projections, assumptions and estimates of our future performance and the future performance of the industry in which we operate are necessarily subject to a high degree of uncertainty and risk due to a variety of factors, including those described above. These and other factors could cause results to differ materially from those expressed in our estimates and beliefs and in the estimates prepared by independent parties.

Gain Therapeutics At – a - Glance



Established in 2017, IPO March 2021



Headquartered in Bethesda, MD with offices in Lugano, Switzerland, Barcelona, Spain



25 Employees



Founder and Executive Chairman:
Dr. Khalid Islam



Partnered with several leading medical & scientific organizations

Proprietary Platform enables novel therapeutic candidates

Our proprietary drug discovery platform, **Magellan™**, integrates AI-supported structural biology with proprietary algorithms and physics-based models to identify novel allosteric binding sites on disease-implicated proteins across all therapeutic areas.

The allosteric MOA enables us to generate the first-in-class / best-in-class product candidates in our developmental pipeline.

GANX Investment Summary

Clinical PoC for GT-02287; Disease-Modifying Treatment for Parkinson's Disease



- Completed **Phase 1a in healthy volunteers** and **Phase 1b open-label trial in PD** (90-day) with 84% of patients electing to join 9-month extension study
- Biomarker evidence from Phase 1b supports **disease-modifying potential** for GT-02287 in both GBA1 and idiopathic Parkinson's disease

Multiple Near-Term Catalysts



- **Q2 2026** - FDA IND review clearance
- **Q3 2026** - Commencement of Phase 2 in people with Parkinson's disease
- **Q4 2026** - Additional data from GT-02287 Phase 1b study extension analysis

Global Rights with Strong IP



- Full worldwide rights to GT-02287 with composition of matter **patent protection through 2038** (additional Hatch-Waxman extension for R&D)
- Patent applications for **5 NCE families** under review

Strong Financial and Developmental Support from Scientific / Medical Community



Grant Support from:

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

Leadership: Extensive Biotech and Pharma Experience



Gene Mack, MBA
Chief Executive Officer




Jonas Hannestad, MD, PhD
Chief Medical Officer




Joanne Taylor, PhD
SVP Research




Khalid Islam
Chairman, Co-Founder




Terenzio Ignoni, PharmD
SVP Technical Operations




Gianluca Fuggetta
Senior Vice President, Finance



Gain Therapeutics Pipeline

ASSET	INDICATION	TARGET	DISCOVERY	RESEARCH	PRECLINICAL	PHASE 1
GT-02287	Parkinson's Disease	GCase	Progress bar spanning Discovery, Research, and Preclinical stages.			
	Gaucher's Disease	GCase	Progress bar spanning Discovery and Research stages.			
	Dementia with Lewy Bodies	GCase	Progress bar spanning Discovery and Research stages.			
	Alzheimer's Disease	GCase	Progress bar spanning Discovery and Research stages.			
Multiple Undisclosed	Lysosomal Storage Disorders	GALC GLB1	Progress bar spanning Discovery and Research stages.			

Parkinson's Disease: High Need for Disease-Modifying Therapies

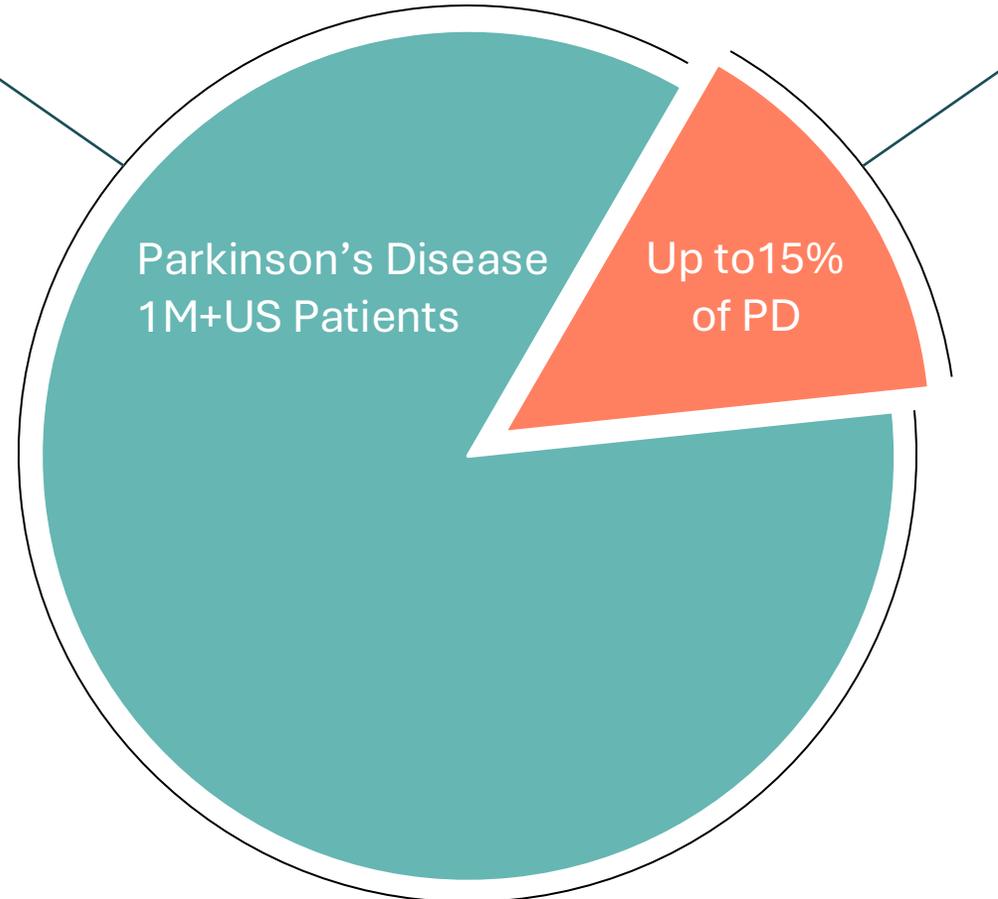
Existing therapies address symptoms without slowing progression

Parkinson's Disease

US Market Potential:

\$4B

- Second most common neurodegenerative disease after Alzheimer's
- People with PD inevitably get worse over time



GBA1-PD

US Market Potential:

\$3B

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

GT-02287: A Disease-Modifying Therapy in Development for Parkinson's Disease

Novel Mechanism

GT-02287 binds at allosteric site, chaperones and modulates glucocerebrosidase enzyme (GCase)

Disease Modification

Restores GCase function and improves disease cascade and neuronal survival

Efficacy

Phase 1b: Statistically significant biomarker evidence of disease-modifying effect with clinical improvement measured via MDS-UPDRS scores

Pre-clinical: Demonstrates a disease-modifying effect in both GBA-1 and iPD models

Safety/Tolerability

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

Next Steps

- Anticipate launch of Phase 2a trial in Q3 2026
- Analysis from Phase 1b 9-month extension study anticipated in Q4 2026

GT-02287: Clinical PoC as a Disease-Modifying Treatment for PD

Phase 1b 90-day data support biological rationale and validate disease-modifying MOA identified in pre-clinical studies

Patients with baseline elevation in CSF GluSph had an **average 81% reduction** to normal levels ($p < 0.05$) after 90 days of treatment.

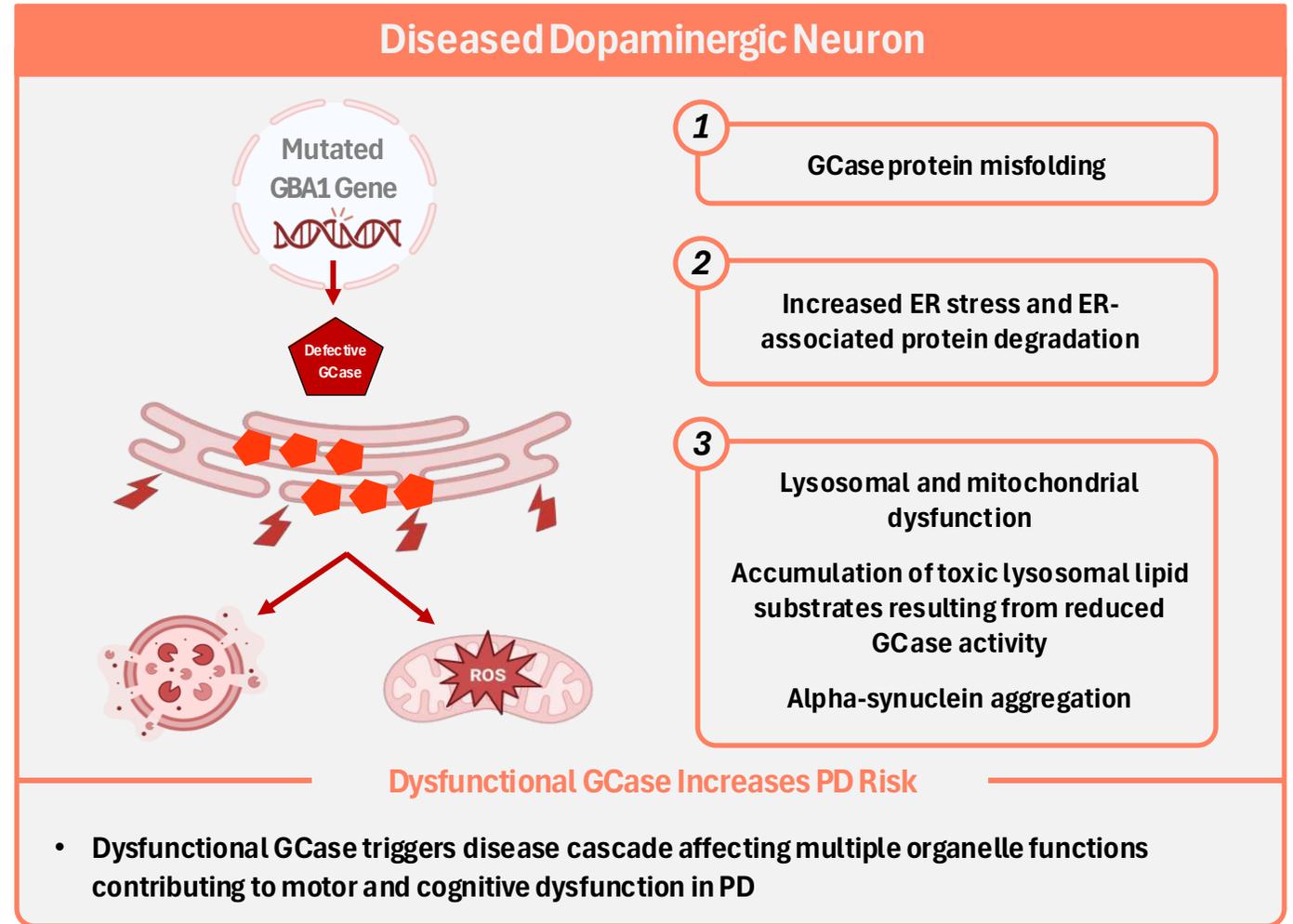
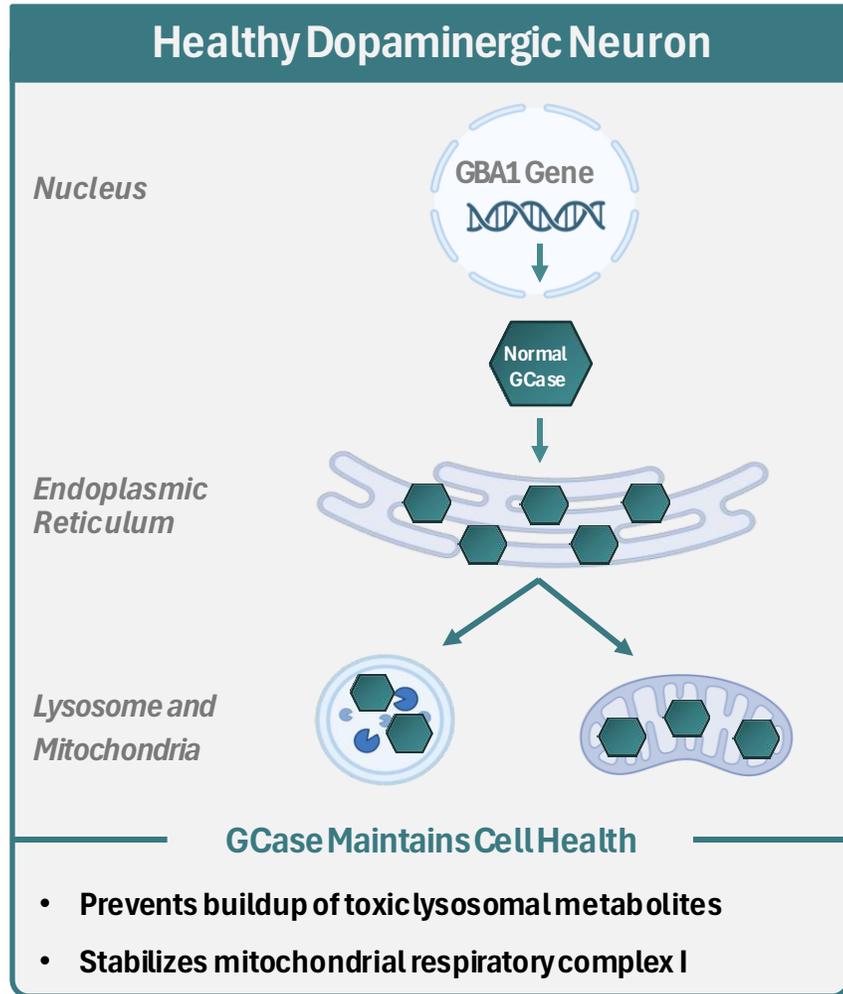
These patients **also** demonstrated a significant improvement ($p < 0.05$) in the sum of MDS-UPDRS Parts II + III scores of 6.17 points

GT-02287 was well-tolerated: 16/19 patients chose to remain on treatment for 9-month OLE which is expected to run through Q3 2026.

Additional readouts expected: Correlative biomarker analysis
Updates on MDS-UPDRS scores, etc. expected at additional medical conferences throughout 2026.

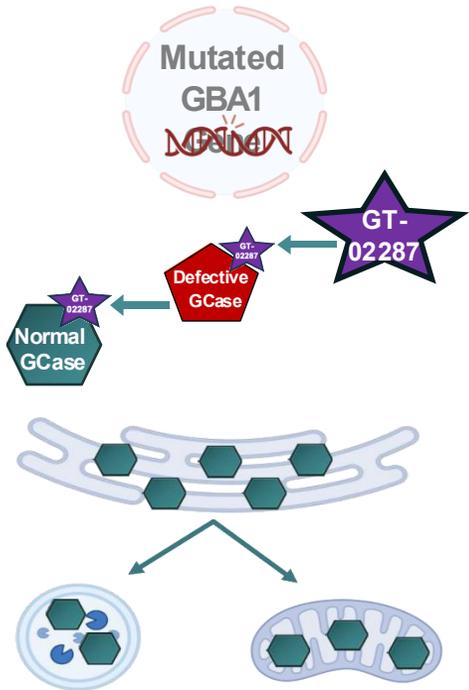
Phase 2a safety and efficacy trial planned for Q3 2026:
Goal to further evaluate tolerability, biological evidence of substrate reduction, and clinical outcomes on MDS-UPDRS scores.

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



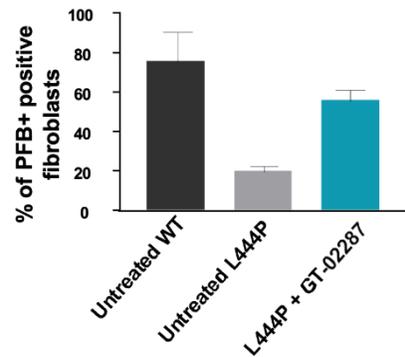
Allosteric Modulator GT-02287 Restores GCase Function: Improves Disease Cascade to Promote Neuronal Survival (i.e., Disease-Modifying)

Dopaminergic Neuron with Restored GCase Function

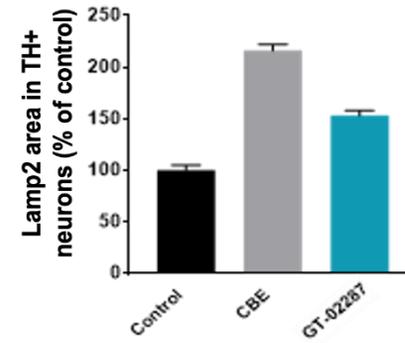


- Corrects protein misfolding
- Restores enzymatic activity

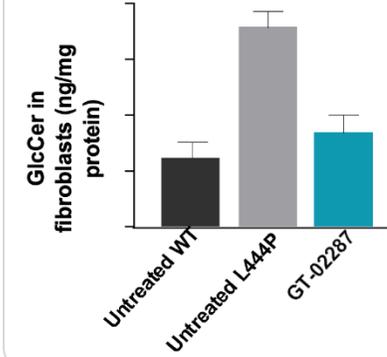
GCase Activity Restoration



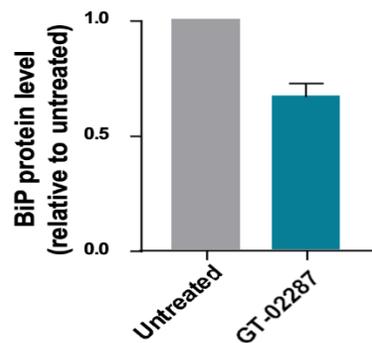
Restored Lysosomal Function



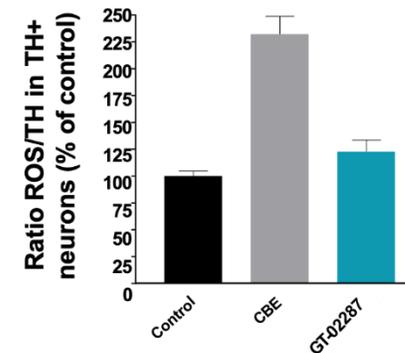
Toxic Substrate Depletion



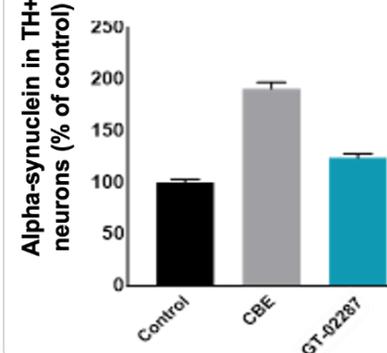
Reduced ER Stress



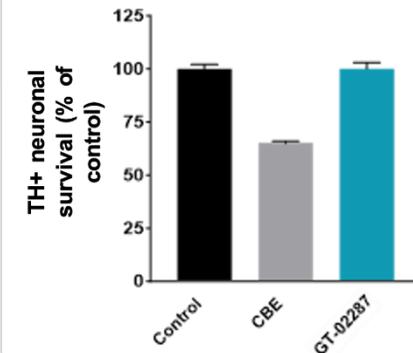
Restored Mitochondrial Function



Alpha-Synuclein Depletion

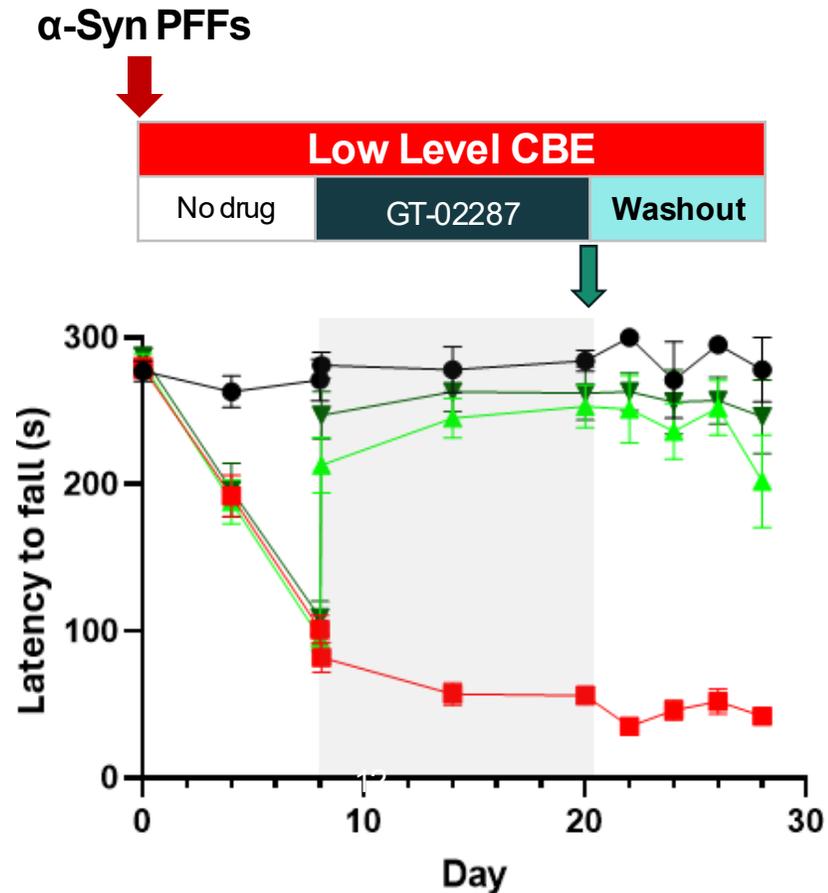


Neuronal Survival

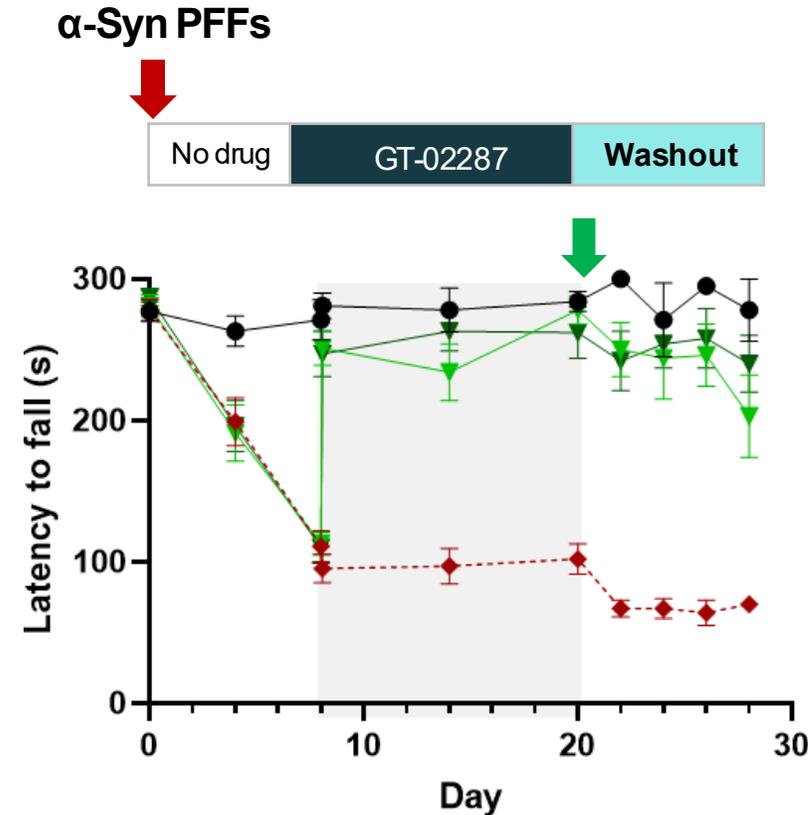


GT-02287 Demonstrates a Disease-Modifying Effect in Animal Models of GBA1 and iPD

GBA1- PD Model (CBE+PFFs)



Idiopathic PD Model (PFFs only)



Mouse Wire Hang

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

GT-02287 is Well-Tolerated in SAD/MAD and Bioavailability Studies

Demonstrated CNS Exposure and GCase Engagement

79 Healthy Volunteers

- Single and multiple dose levels tested were **generally well-tolerated, with no SAEs** or Grade 3 (severe) adverse events observed; no other safety signals detected
- Most common treatment-related AEs (TEAEs) in MAD were nausea (32%), abdominal pain (8%), diarrhea (8%) and headache (8%)
- **Therapeutic exposure levels achieved** in line with results from preclinical models
- **CNS exposure** comparable to that observed in rodent studies

GCase Activity in Dried Blood Spots (DBS)

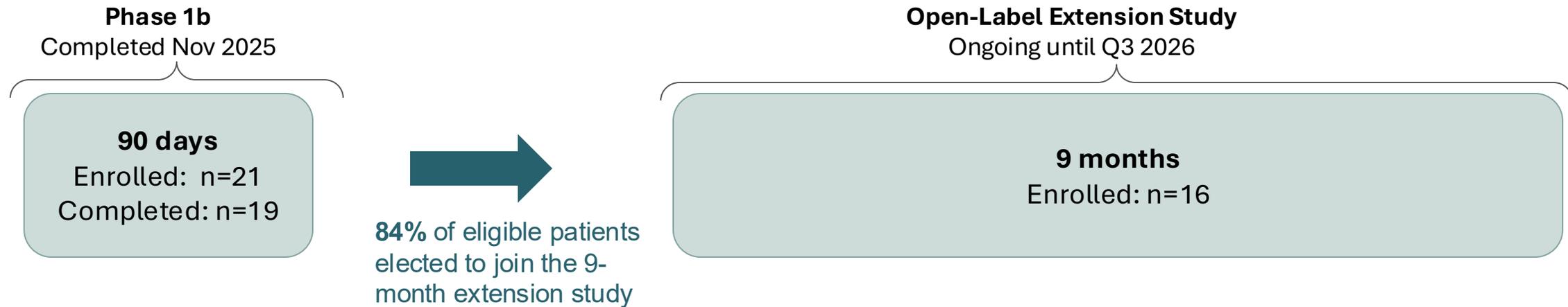
GCase activity in dried blood spots was measured in MAD Cohort 4

5 out of 6 subjects had increased GCase activity. No increase was observed in PBO subjects

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

**One-way, paired, repeated measures ANOVA*

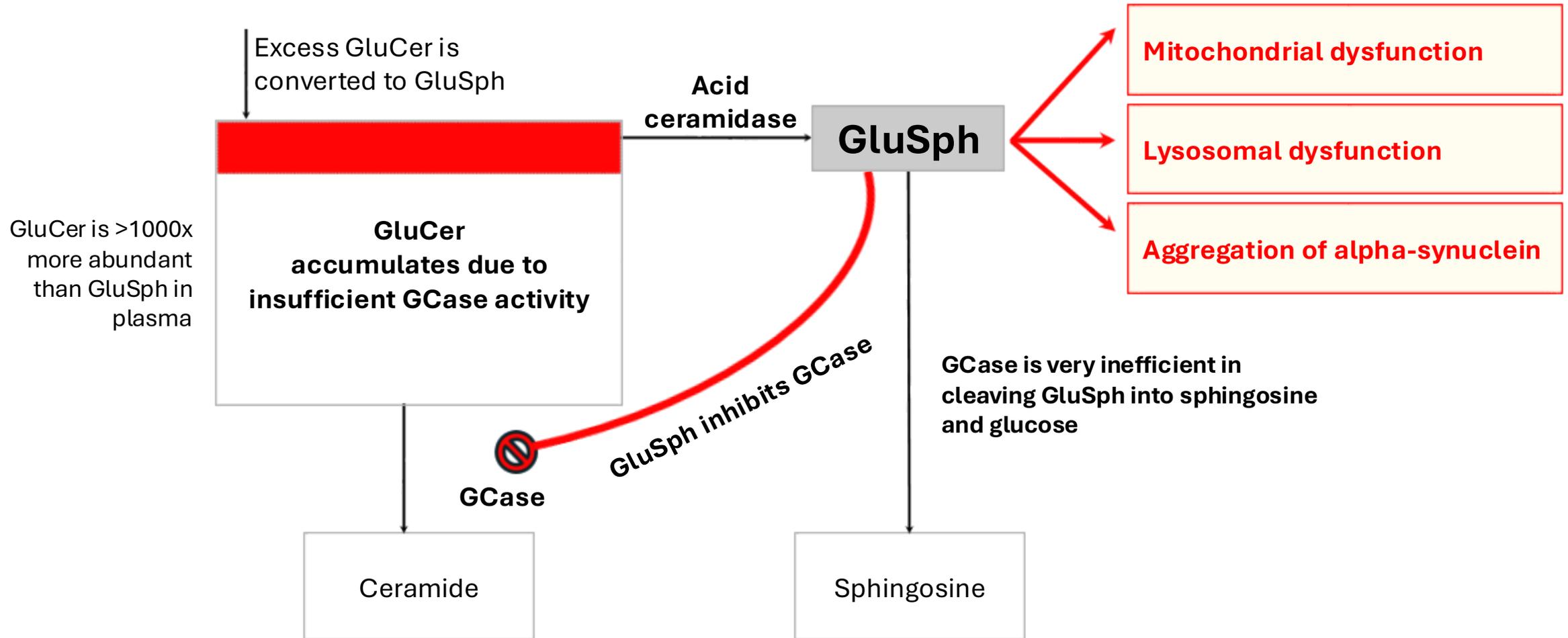
Phase 1b Study Overview + 90 day and Open-Label Extension



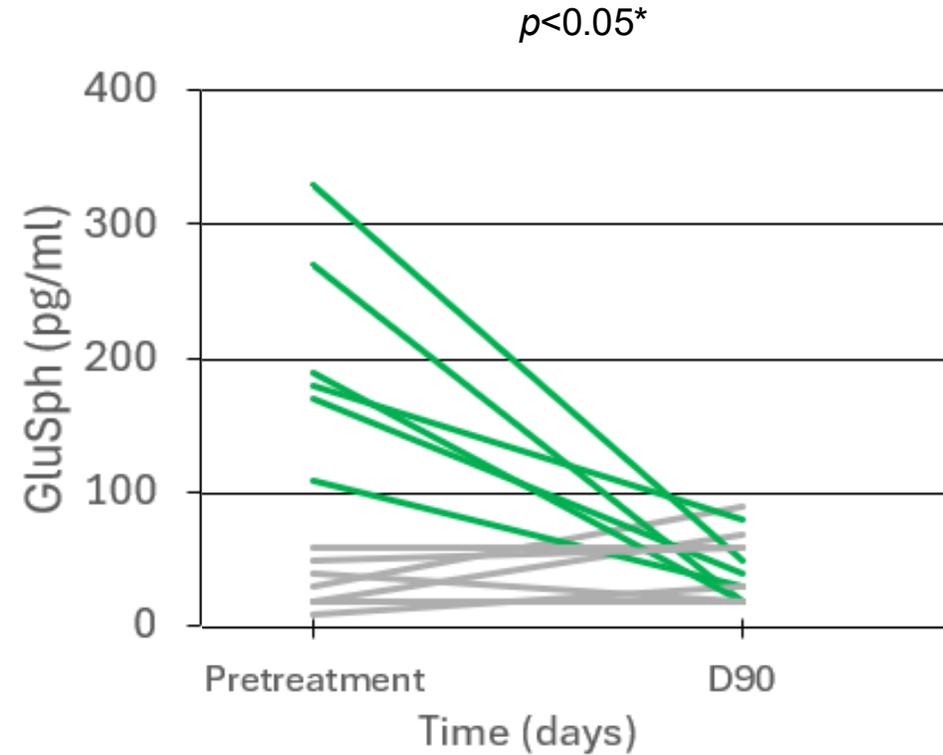
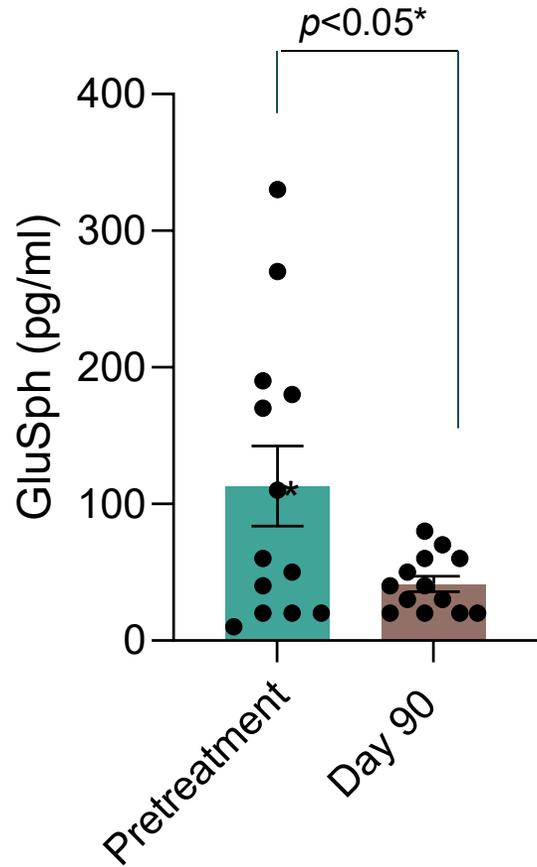
Overview:

- 3-month open label multi-center study across 7 sites (AU)
- 1x daily dose for 90 days
- **Objective:** Assess the Safety, Tolerability, PK/PD (blood & CSF) of GT-02287 in Parkinson's Disease patients with or without a pathogenic GBA1 mutation, along with mechanistic biomarkers, and clinical progression measured via MDS-UPDRS
- Phase 1 SAD/MAD First-in-human study demonstrated GCase target engagement in healthy volunteers

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

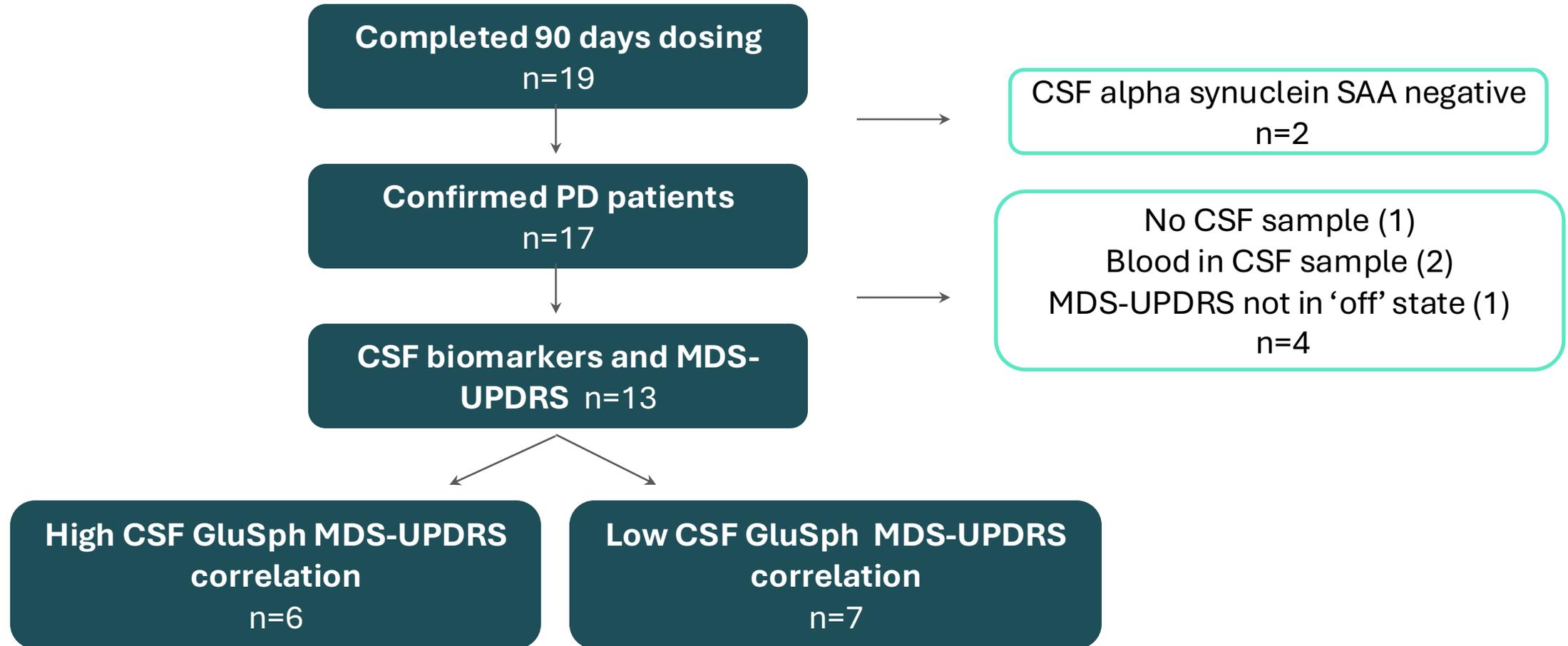


GT-02287 Treatment Leads to a Decrease in CSF GluSph, a Prespecified Endpoint, Demonstrating CNS Target Engagement



* Significant difference compared to pretreatment Two-way ANOVA $P < 0.05$
Data are shown as mean + s.e.m. $n = 13$

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



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

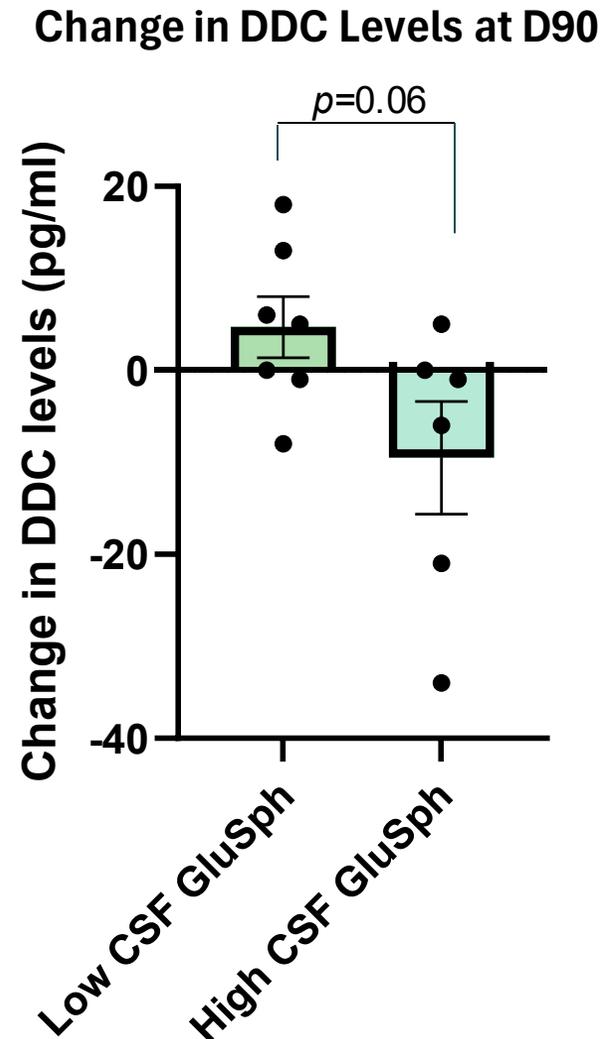
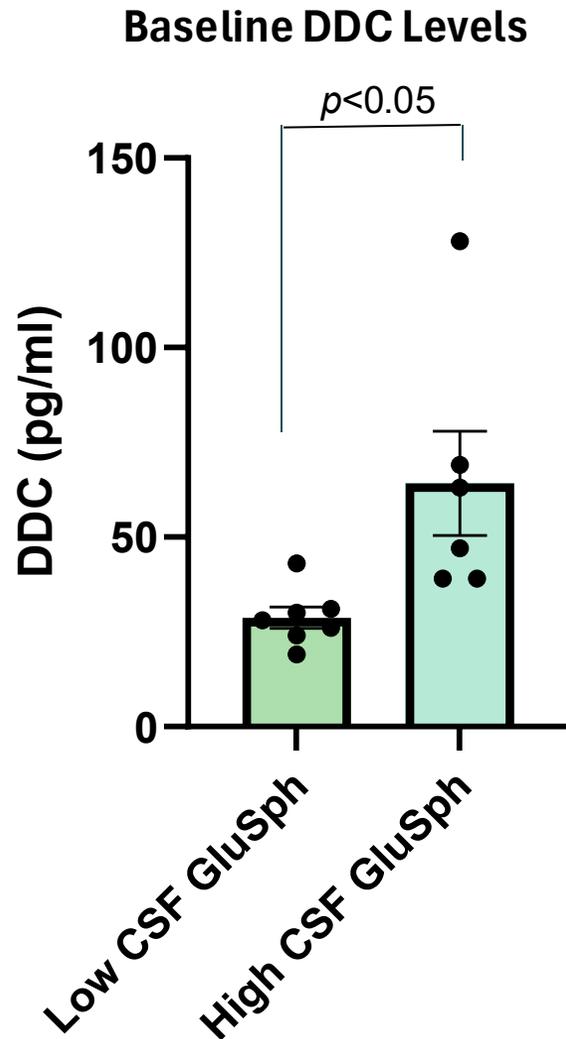
Patients with **high** CSF GluSph (n=6)

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

Data shown as mean (n=13). Statistically significant difference as compared to lowGluSph group. One-sample t-test. *P < 0.05.

Higher CSF DDC Levels in Participants Who Had High CSF GluSph at Baseline

DDC Decreases Following GT-02287 Treatment in High GluSph Group



DDC is elevated in CSF in people with Parkinson's, likely due to dopaminergic neuron dysfunction

DOPA decarboxylase (DDC) is an enzyme responsible for converting L-DOPA (levodopa) into dopamine in the brain and peripheral nervous system.

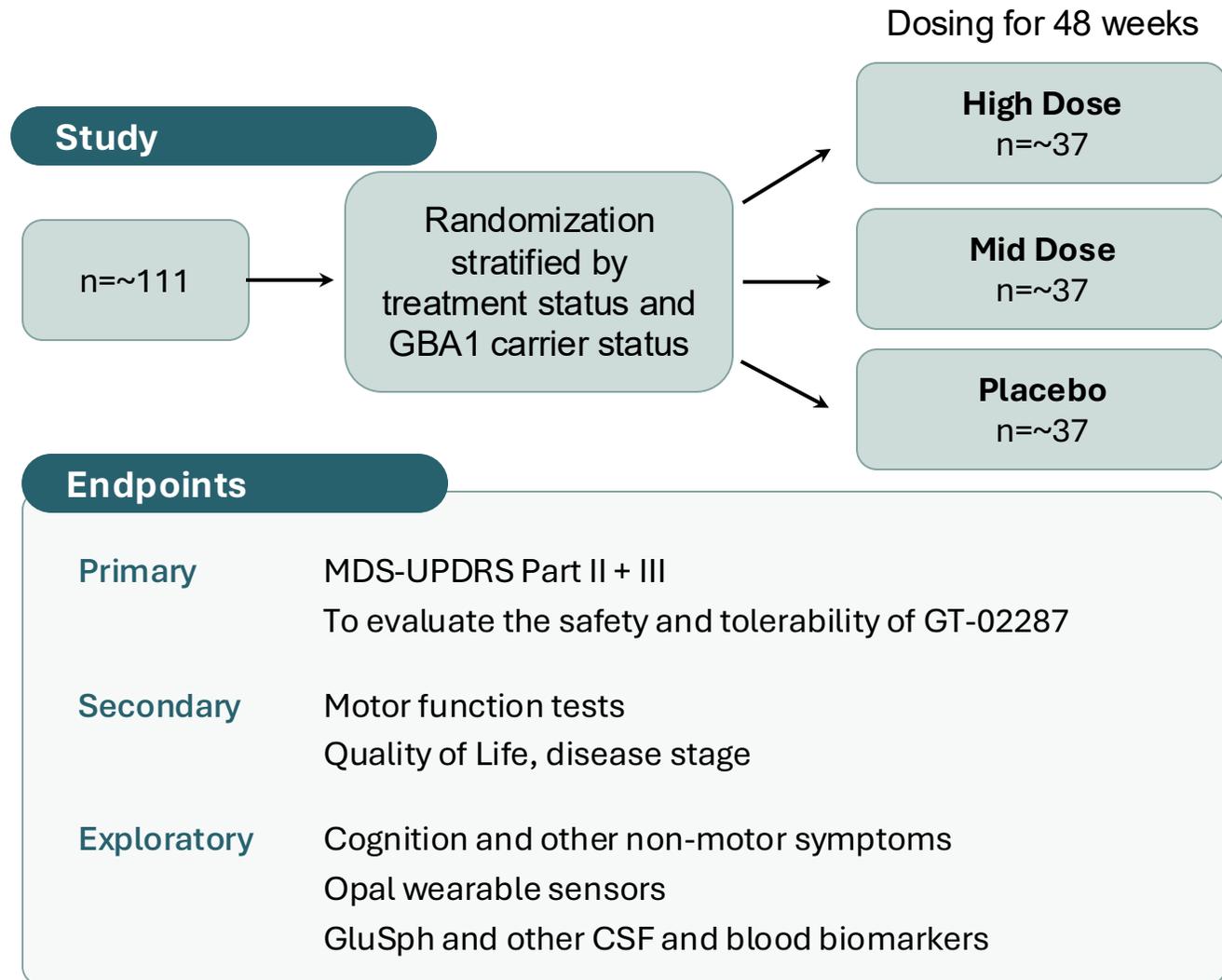
Data are + s.e.m. Analysed by unpaired t-test for difference between Low and High CSF GluSph groups.

Phase 2a Study

48-week, double blind, randomized, placebo-controlled Phase 2a study of 2 dose levels of oral GT-02287 in treated and untreated participants with early PD

Participants: Clinical diagnosis of PD according to Movement Disorder Society Criteria; time since diagnosis of no more than 5 years; positive SAA in CSF; Modified Hoehn and Yahr Stages 1 to 2.5; Idiopathic and GBA1, on stable dopaminergic treatment

Primary endpoint: Change from Baseline to Week 48 in the sum of the MDS-UPDRS Part II and Part III score assessed in the practically defined OFF state



GT-02287: A Disease-Modifying Therapeutic in Development for 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

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

KOL Events and Industry Support

JANUARY 2026

UNDERSTANDING GCASE SUBSTRATES IN PARKINSON'S DISEASE – PERSPECTIVES ON BIOMARKERS AND DISEASE MODIFICATION

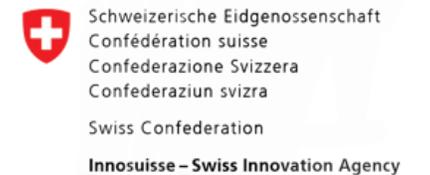
Featured Key Opinion Leaders Roy Alcalay, M.D., M.Sc., Chief of Movement Disorders Division, Tel Aviv Sourasky Medical Center, Professor of Neurology, Tel Aviv University, Associate Professor of Clinical Neurology, Columbia University, and Peter Lansbury, Ph.D., Professor of Neurology, Harvard



OCTOBER 2025

BIOMARKERS, CLINICAL ENDPOINTS, AND THE PATH TO DISEASE MODIFICATION: CONTEXTUALIZING THE EMERGING DATA FROM GT-02287

Featured Key Opinion Leaders Karl Kieburtz, M.D., M.P.H., Professor of Neurology, University of Rochester and Kenneth Marek, M.D., President and Senior Scientist, Institute for Neurodegenerative Disorders



Financials



Established in 2017, IPO March 2021



Headquartered in Bethesda, MD with offices in Lugano, Switzerland, Barcelona, Spain



25 Employees



Founder and Executive Chairman:
Dr. Khalid Islam



Partnered with several leading medical & scientific organizations

Financials

- **Nasdaq: GANX**
- Cash: \$20.8 million as of December 31, 2025
- Cash Runway: through 1Q27
- No debt
- 42.1 million shares outstanding

GANX Investment Summary

Clinical PoC for GT-02287, a Disease-Modifying Treatment for Parkinson's Disease

Multiple Near-Term Catalysts

Global Rights with Strong IP

Strong Financial and Developmental Support from Scientific / Medical Community

Thank you