GAIN THERAPEUTICS

Corporate Presentation

October 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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Gain Therapeutics At – a - Glance

- Established in 2017, IPO March 2021
- Headquartered in Bethesda, MD with offices in Lugano, Switzerland, Barcelona, Spain
- 23 Employees
- Scientific Founder and 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.

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GANX Investment Summary

Clinical PoC for *rexaceract*; Disease-Modifying Treatment for Parkinson's Disease

- Completed **Phase 1b open-label extension** in PD**; MDS-UPDRS scores** in all 12 evaluable participants were **stable over 12-month study period** with *rexaceract***,** demonstrating **no clinically meaningful progression** of motor symptoms
- Biomarker evidence from Phase 1b supports **disease-modifying potential** for *rexaceract* in both GBA1and idiopathic Parkinson's disease

Multiple Near-Term Catalysts

- **Q4 2026 –** Selection of initial therapeutic indication for GT-04686
- **Q1 2027** – Start of Phase 2 in people with Parkinson's disease

Global Rights with Strong IP

- Full worldwide rights to *rexaceract* 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)

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Leadership: Extensive Biotech and Pharma Experience



Khalid Islam

Chairman, Co-Founder

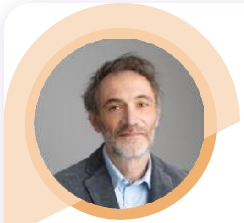
Gentium · Immunomedics · minoryx therapeutics · ARPIDA · FENNEC PHARMA



Gene Mack, MBA

Chief Executive Officer

Imcyse · OncoC4 · MERCK OncoImmune · nicox · biom'up · ΣELLAS LIFE SCIENCES GROUP · MIZUHO · HSBC · LAZARD



Terenzio Ignoni, PharmD

Chief Operating Officer

Jazz Pharmaceuticals · Vicuron Pharmaceuticals · Gentium · Zambon



Joanne Taylor, PhD

Chief Scientific Officer

Prescient · Eisai · ETH zürich



Jeanette Weidner

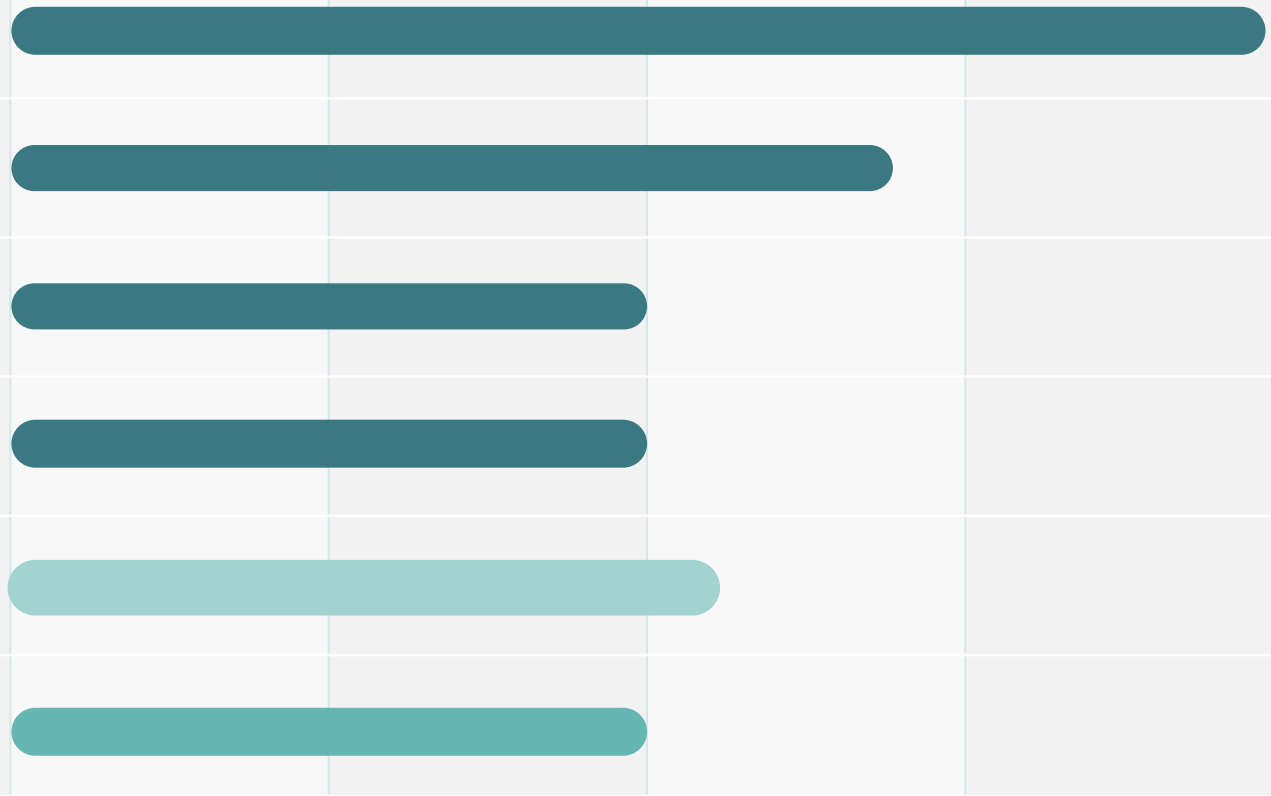
Senior Vice President, Finance

OncoC4

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Magellan™: One Platform, a Broadening Pipeline

ASSET	INDICATION	TARGET	DISCOVERY	RESEARCH	PRECLINICAL	PHASE 1
rexaceract	Parkinson's Disease	GCase				
	Gaucher's Disease	GCase				
	Dementia with Lewy Bodies	GCase				
	Alzheimer's Disease	GCase				
GT-04686	TBD	GCase				
Multiple Undisclosed	Lysosomal Storage Disorders	GALC GLB1				



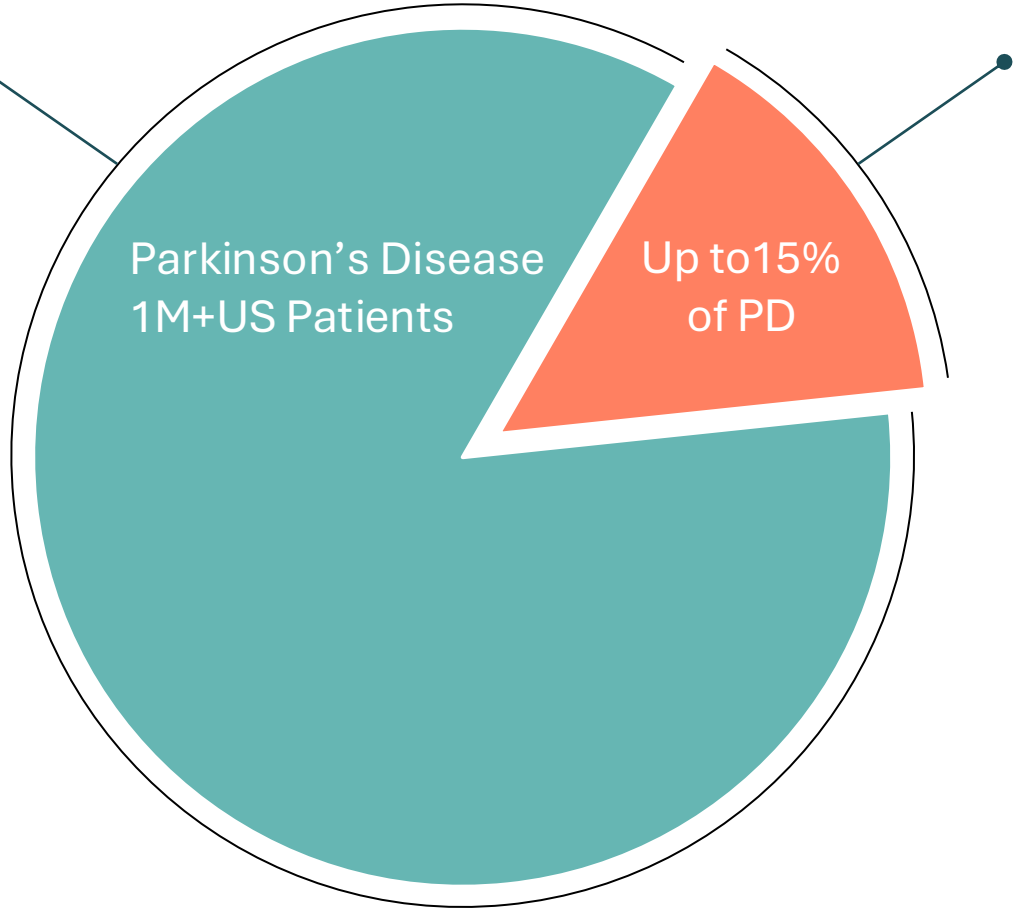
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Parkinson's Disease: High Need for Disease-Modifying Therapies

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 variantscause 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

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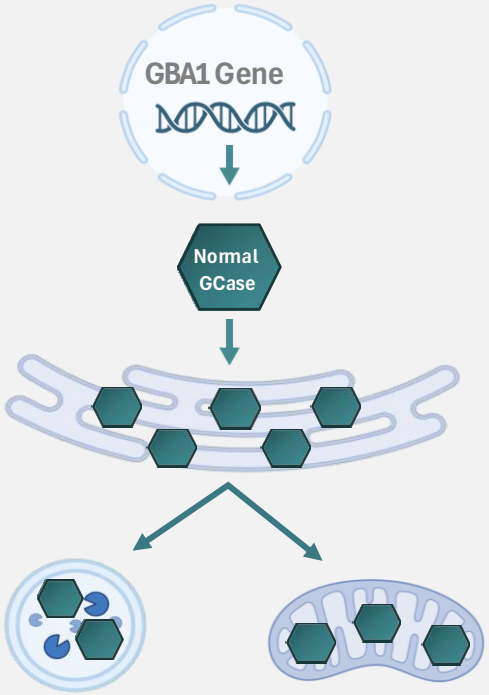
Rexaceract: A Disease-Modifying Therapy in Development for Parkinson's Disease

Novel Mechanism	*rexaceract* binds at allosteric site, and chaperones glucocerebrosidase (GCase) enzyme to lysosomes and mitochondria
Disease Modification	Restores GCase function and improves disease cascade and neuronal survival in both GBA1 and iPD models
Efficacy	Phase 1b shows statistically significant biomarker evidence of central target engagement with clinical improvement measured via MDS-UPDRS scores
Safety/Tolerability	Well-tolerated in both healthy volunteers and PD patients with therapeutic CNS exposures achieved
Next Steps	Start of Phase 2 trial in Q1 2027

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Dysfunctional GCase Affects Multiple Organelles

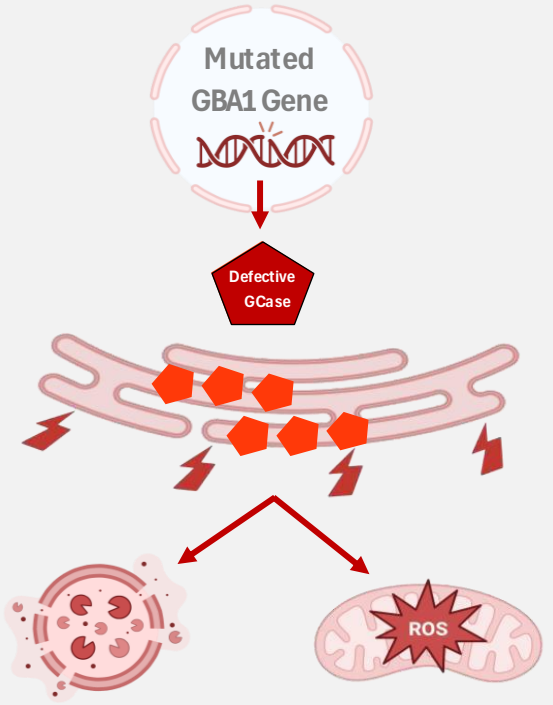
Healthy Dopaminergic Neuron



GCase Maintains Cell Health

- Prevents buildup of toxic lysosomal metabolites
- Stabilizes mitochondrial respiratory complex I

Diseased Dopaminergic Neuron



1. GCase protein misfolding
2. Increased ER stress and ER-associated protein degradation
3. Lysosomal and mitochondrial dysfunction
   - Accumulation of toxic lysosomal lipid substrates resulting from reduced GCase activity
   - Alpha-synuclein aggregation

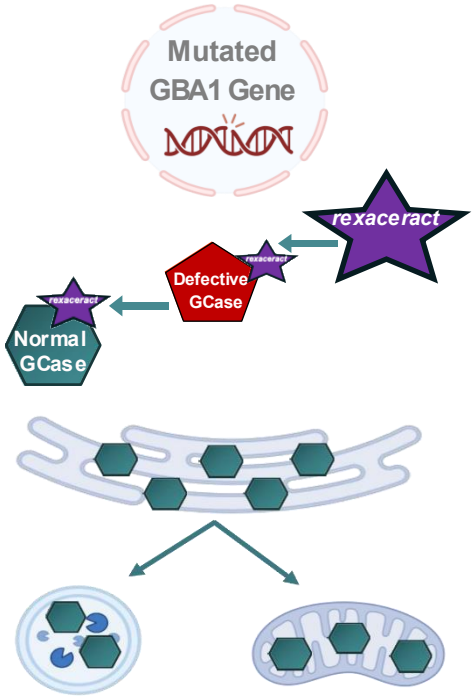
Dysfunctional GCase Increases PD Risk

- Dysfunctional GCase triggers disease cascade affecting multiple organelle functions contributing to motor and cognitive dysfunction in PD

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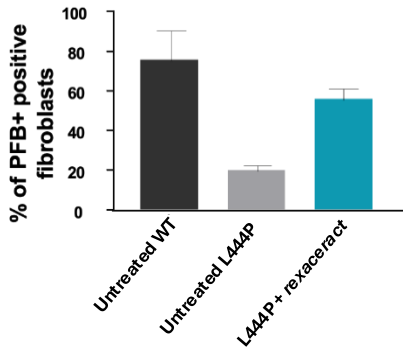
Allosteric Modulator *Rexaceract* Restores GCase Function: Improves Disease Cascade to Promote Neuronal Survival (i.e., Disease-Modifying)

Dopaminergic Neuron with Restored GCase Function

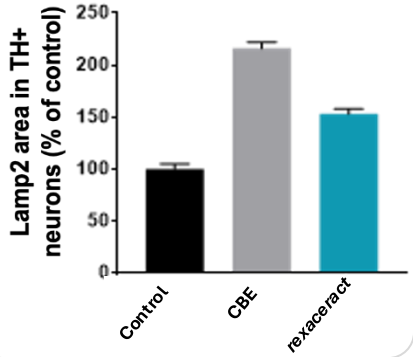


- Corrects protein misfolding
- Restores enzymatic activity

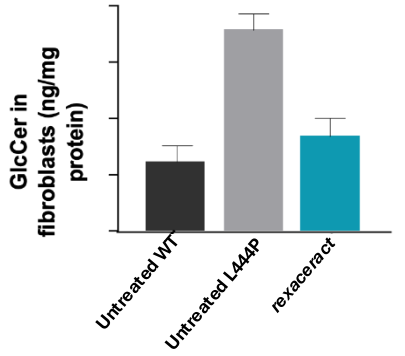
GCase Activity Restoration



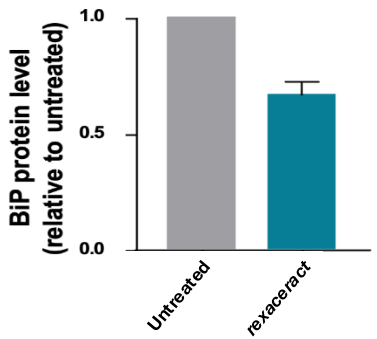
Restored Lysosomal Function



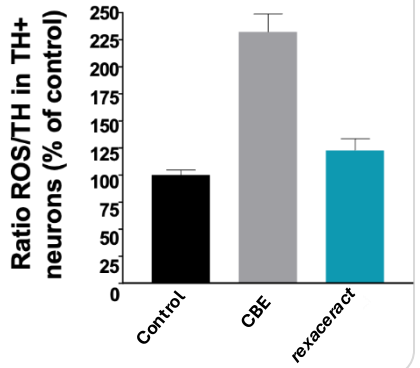
Toxic Substrate Depletion



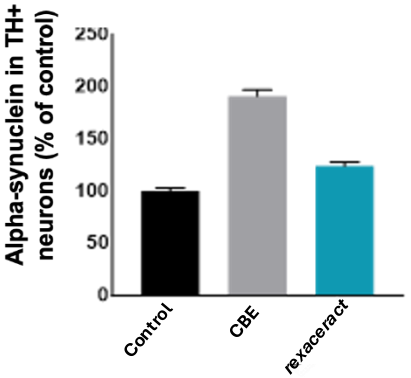
Reduced ER Stress



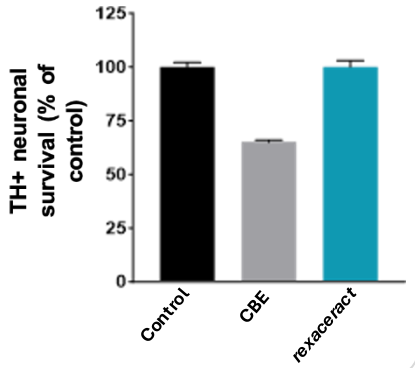
Restored Mitochondrial Function



Alpha-Synuclein Depletion



Neuronal Survival



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Rexaceract has Best-in-Class Profile for GBA1-Parkinson's Disease

	Effect on Disease Cascade	GAIN THERAPEUTICS *rexaceract*	Bial BIA 28-6156	VANQUABIO VQ-101
GCase Mechanism of Action	Increases Lysosomal GCase Activity	✓	?	✓
	Reduces Toxic Lipid Substrates	✓	✓	✓
	Reduces Aggregated α-Synuclein	✓	✗?	✓
	Improves Lysosomal Function	✓	✓	✓
	Improves Mitochondrial Function	✓	?	?
	Reduces Neuroinflammation	✓	?	?
	Provides Neuroprotection	✓	?	?
	Reduces ER Stress	✓	?	?
Disease-Modifying Effect	Increases Dopamine Levels	✓	?	?
	Restores Motor Function	✓	?	?
	Improves Cognitive Function	✓	?	?

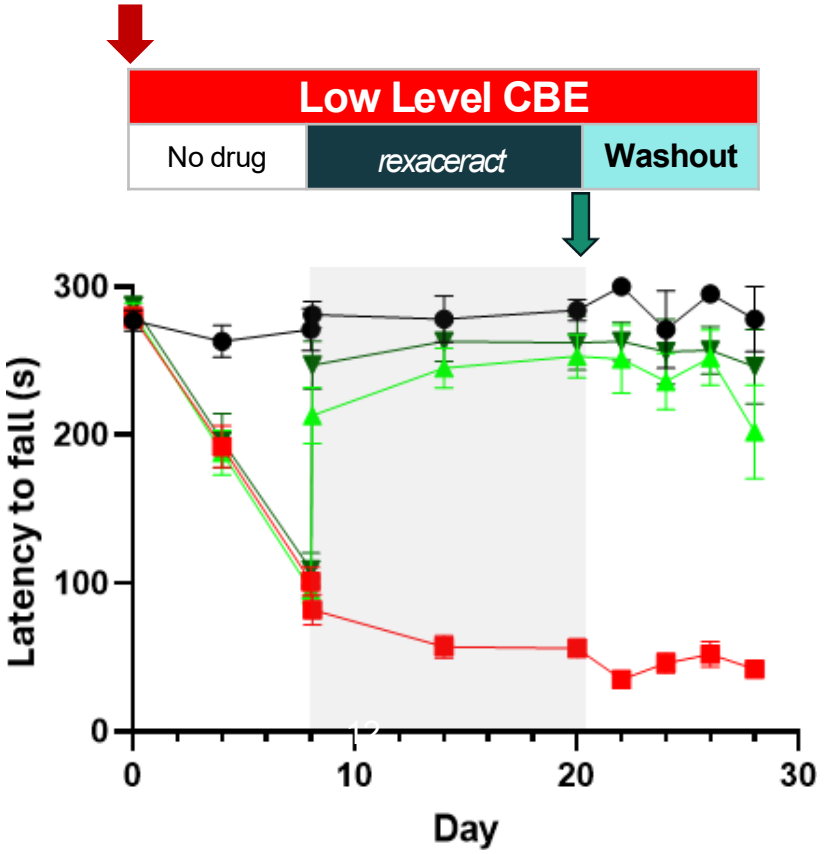
✓ Effect in Preclinical Studies ? No Data Shown ✗ No Clinical Effect Shown

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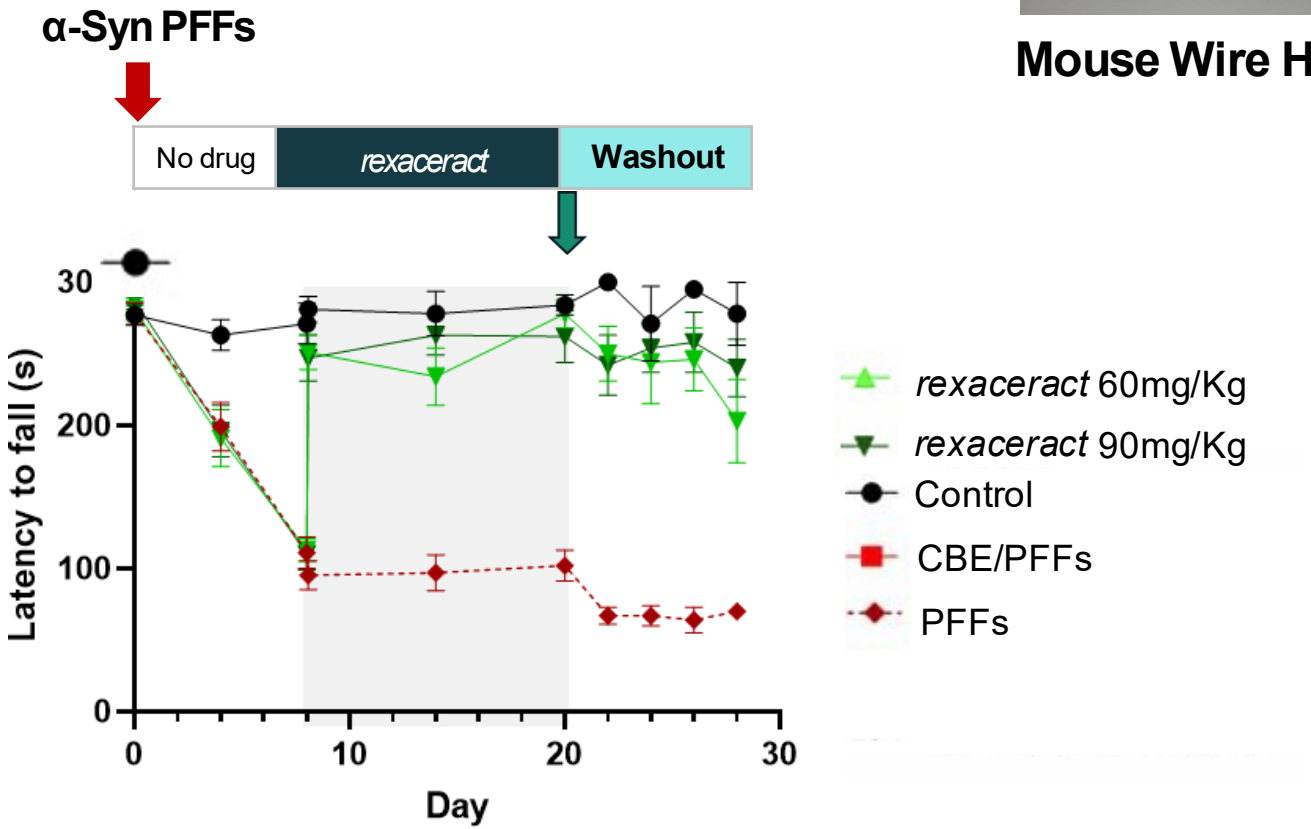
Rexaceract Demonstrates a Disease-Modifying Effect in Animal Models of GBA1 and iPD

GBA1- PD Model (CBE+PFFs)

α-Syn PFFs



Idiopathic PD Model (PFFs only)





Mouse Wire Hang

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Rexaceract 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*

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

Phase 1b
Completed Nov 2025

90 days
Enrolled: n=21
Completed: n=19

84% of eligible patients elected to join the 9-month extension study

Open-Label Extension Study
Ongoing until Q3 2026

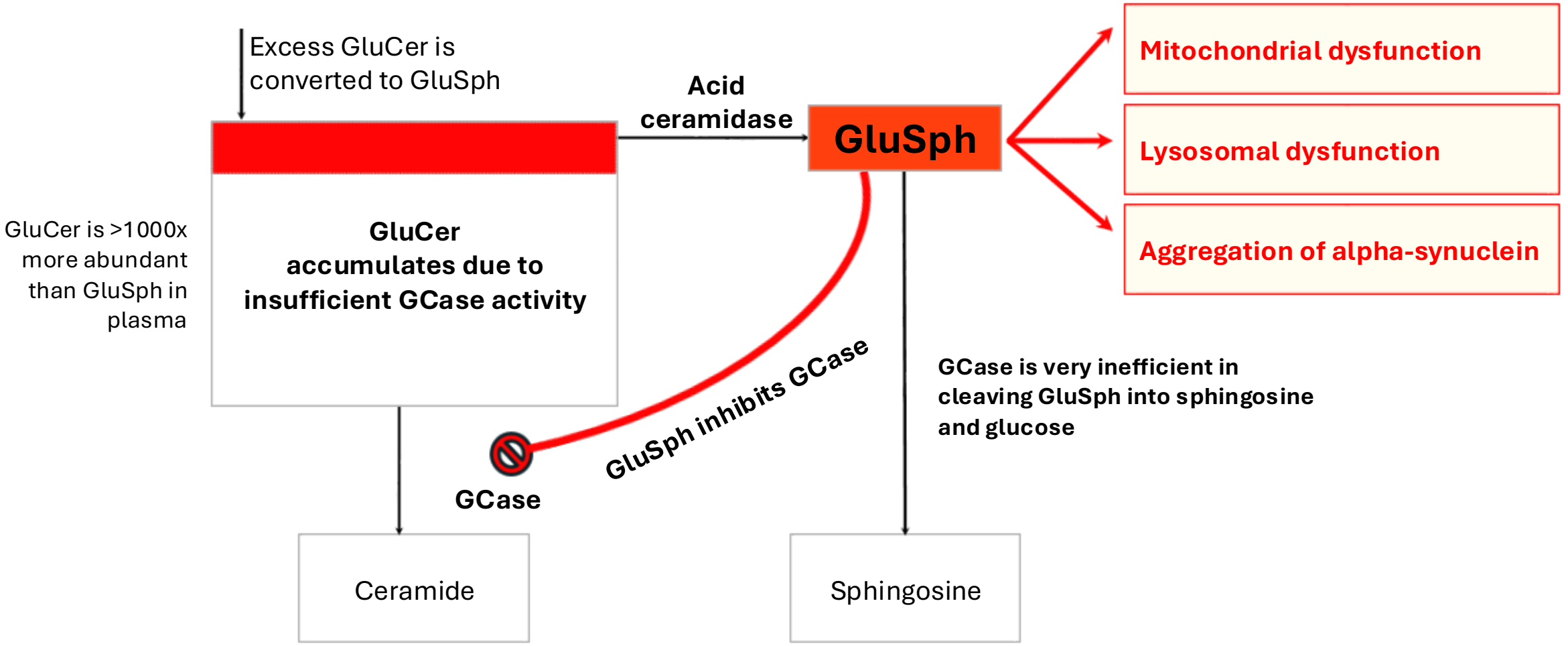
9 months
Enrolled: n=16

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 *rexaceract* 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

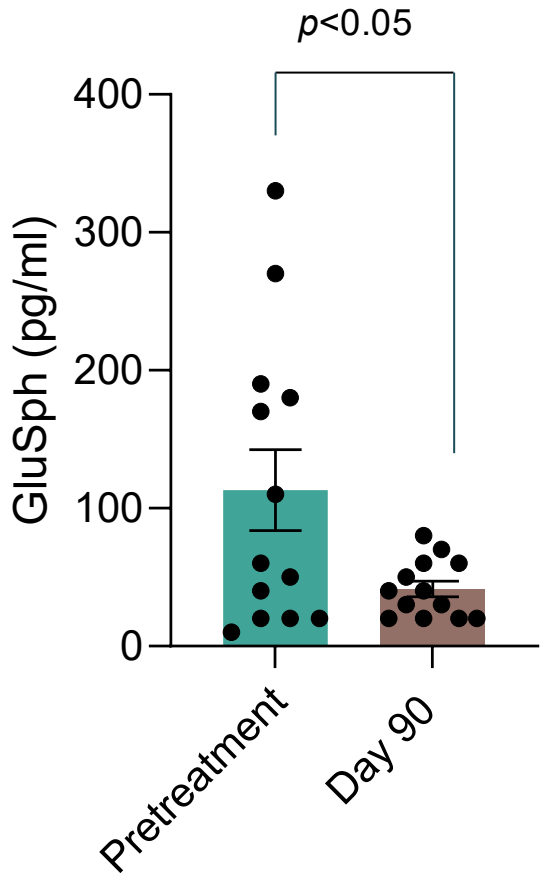
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Decreased GCase Leads to Excess GluCer and Consequently Increased GluSph in Parkinson's

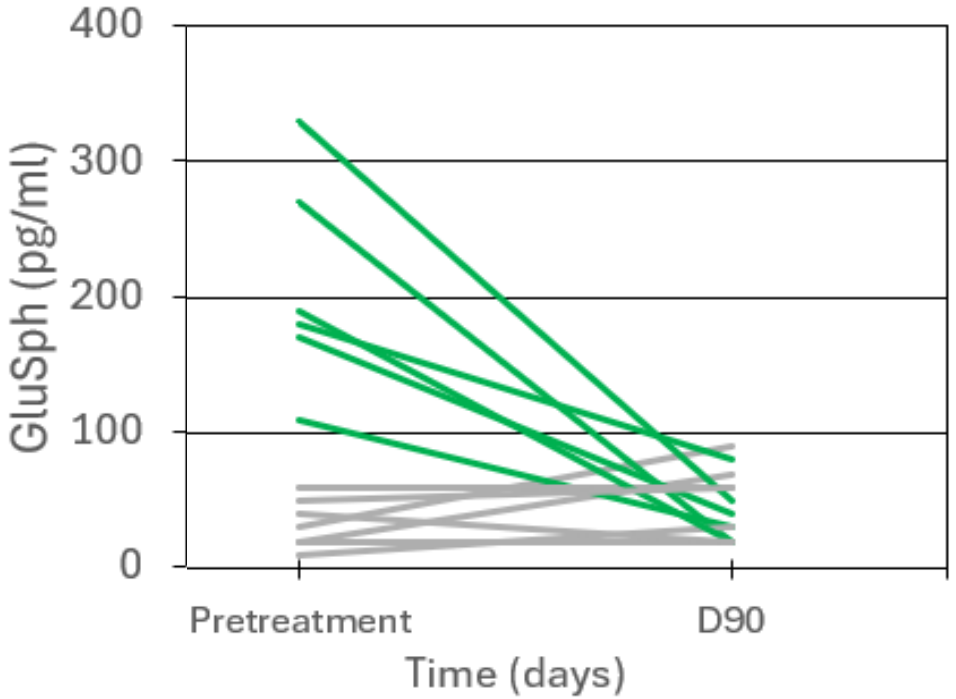


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Treatment with *Rexaceract* Leads to a Decrease in CSF GluSph, a Prespecified Endpoint, Demonstrating CNS Target Engagement



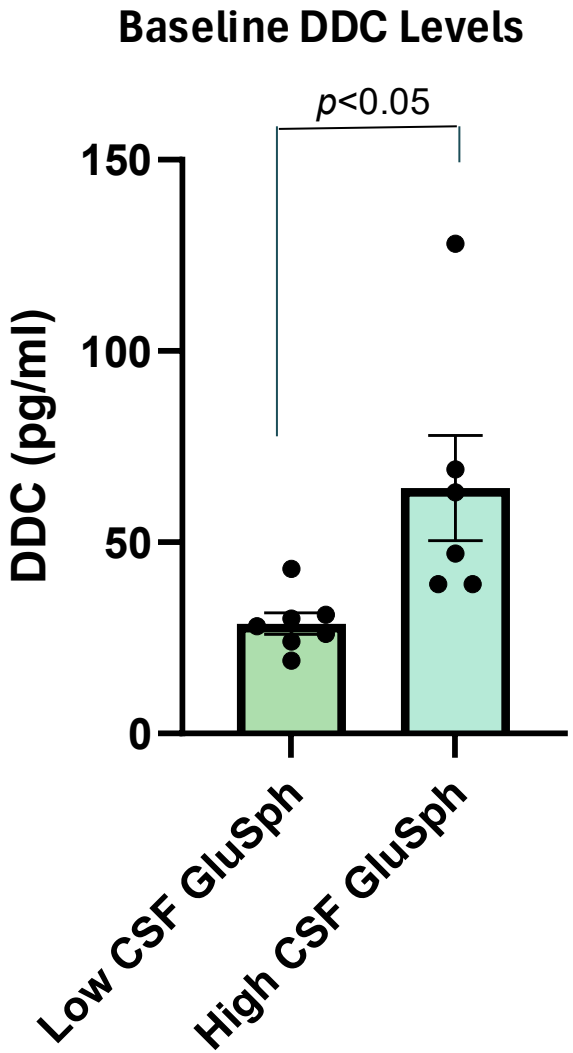
Data are shown as mean ± s.e.m. n=13. P<0.05 two-way ANOVA

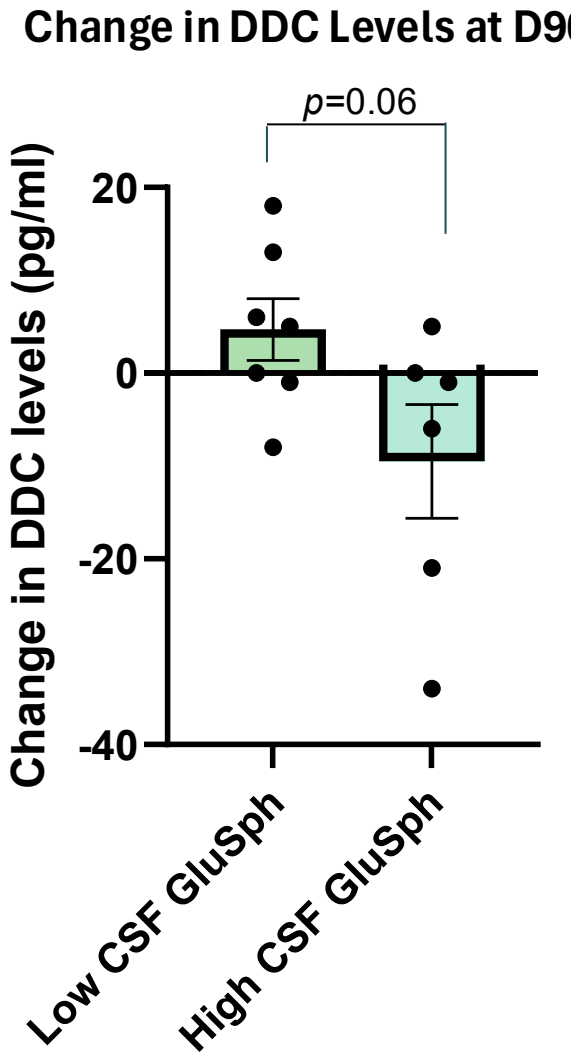


Data are shown as CSF GluSph levels for individual patients before and after treatment. Gray lines represent patients with low CSF GluSph and green lines represent patients with high CSF GluSph at baseline

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Higher CSF DDC Levels in Participants Who Had High CSF GluSph at Baseline
DDC Decreases Following *Rexaceract* Treatment in High GluSph Group





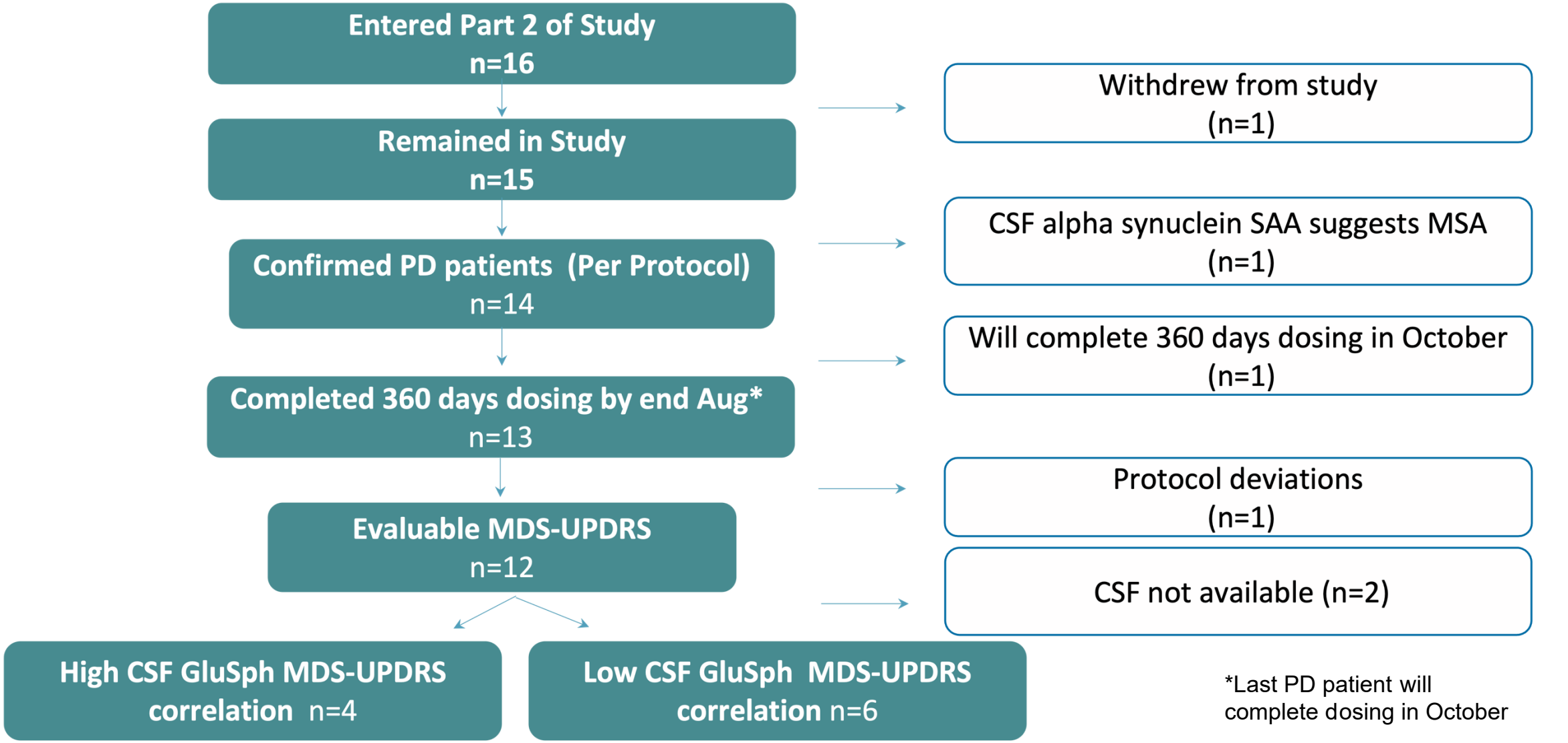
DDC is elevated in CSF in people with Parkinson's, likely due to dopaminergic neuron dysfunction

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

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

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Phase 1b Evaluable Population for CSF Biomarker Analysis and MDS-UPDRS Scores at 12 Months

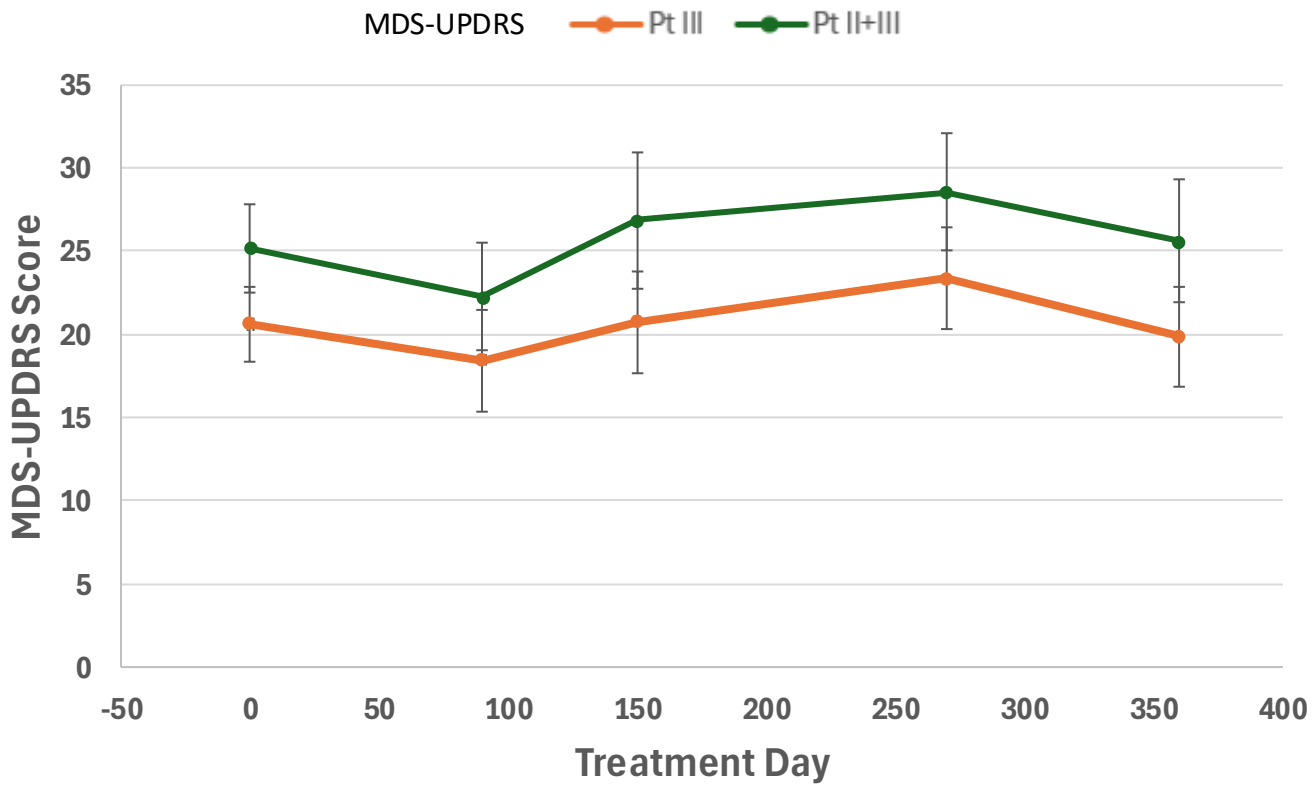


*Last PD patient will complete dosing in October

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12 Evaluable Patients Have Completed 12 Months of Dosing with *Rexaceract* and Show No Clinically Meaningful Progression of Parkinson's Disease on MDS-UPDRS Motor Scores

- *rexaceract* was associated with no meaningful progression on either the MDS-UPDRS Part 2 or Part 3 scale
- MDS-UPDRS Part II: Patient-reported motor function in daily life
- MDS-UPDRS Part III: Clinician-assessed motor function on examination

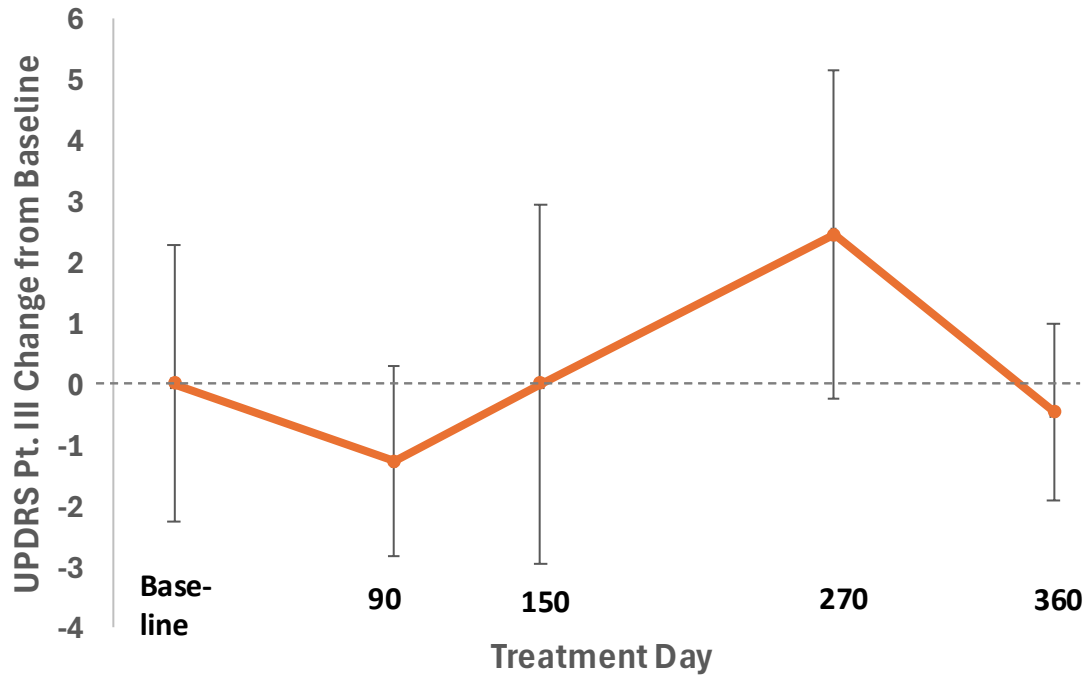


Data are mean ± s.e.m.

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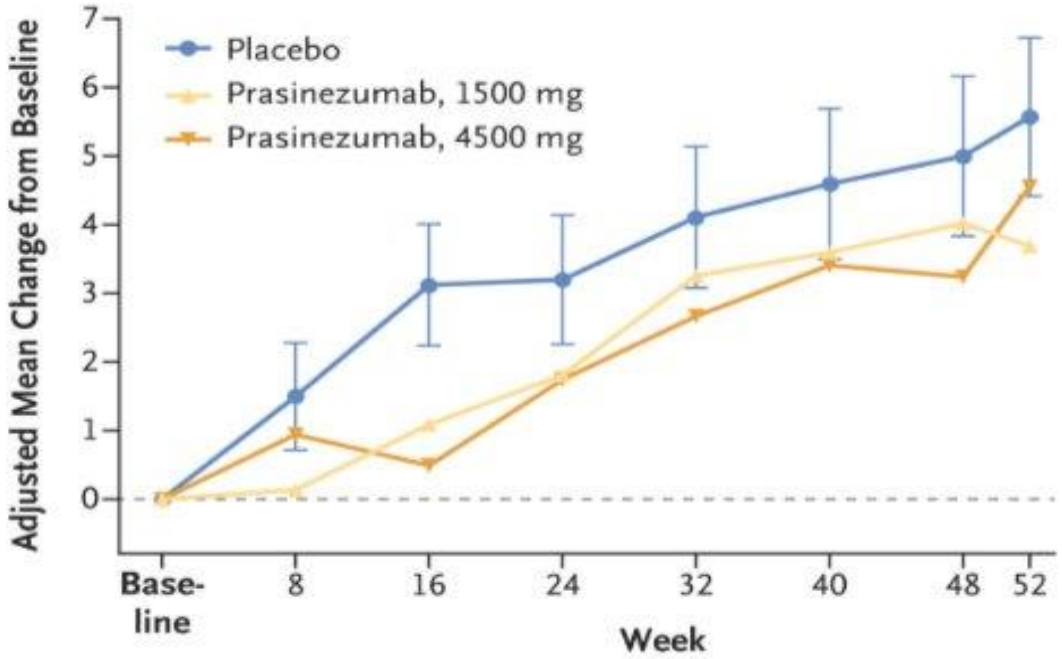
Patients Treated with *Rexaceract* for 12 Months Show No Meaningful Change in MDS-UPDRS Part III Compared to Placebo Arm from PASADENA Study

rexaceract change in UPDRS III score (n=12)



Data are mean ± s.e.m.

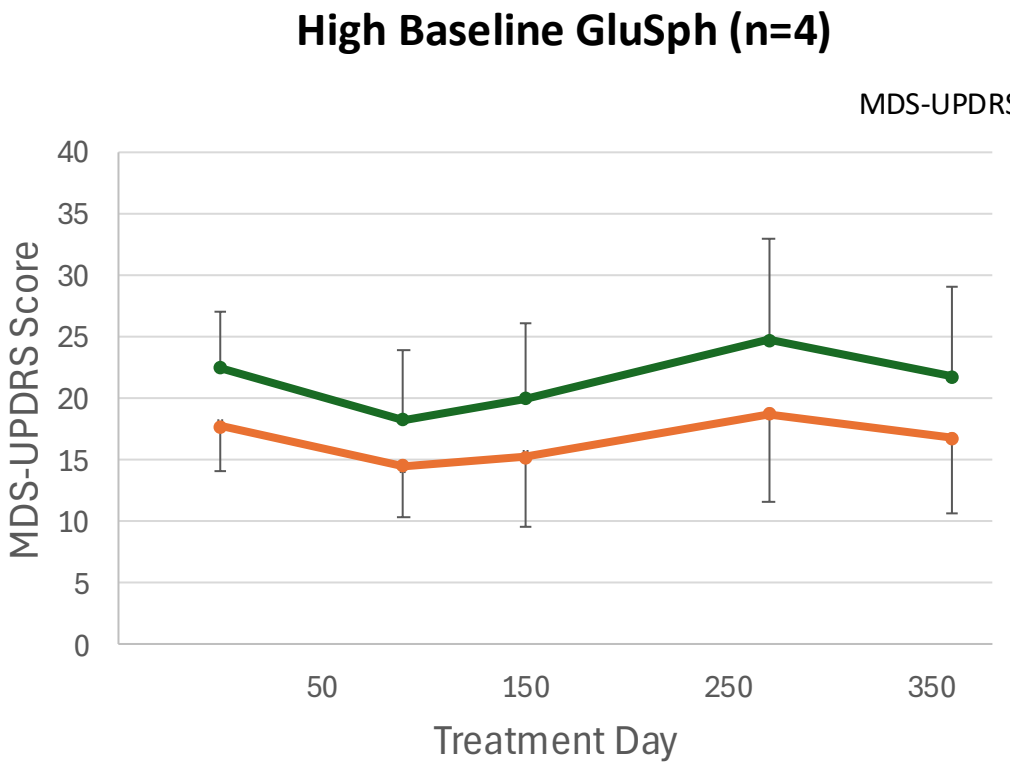
Prasinezumab change in UPDRS III score (n~105/group)

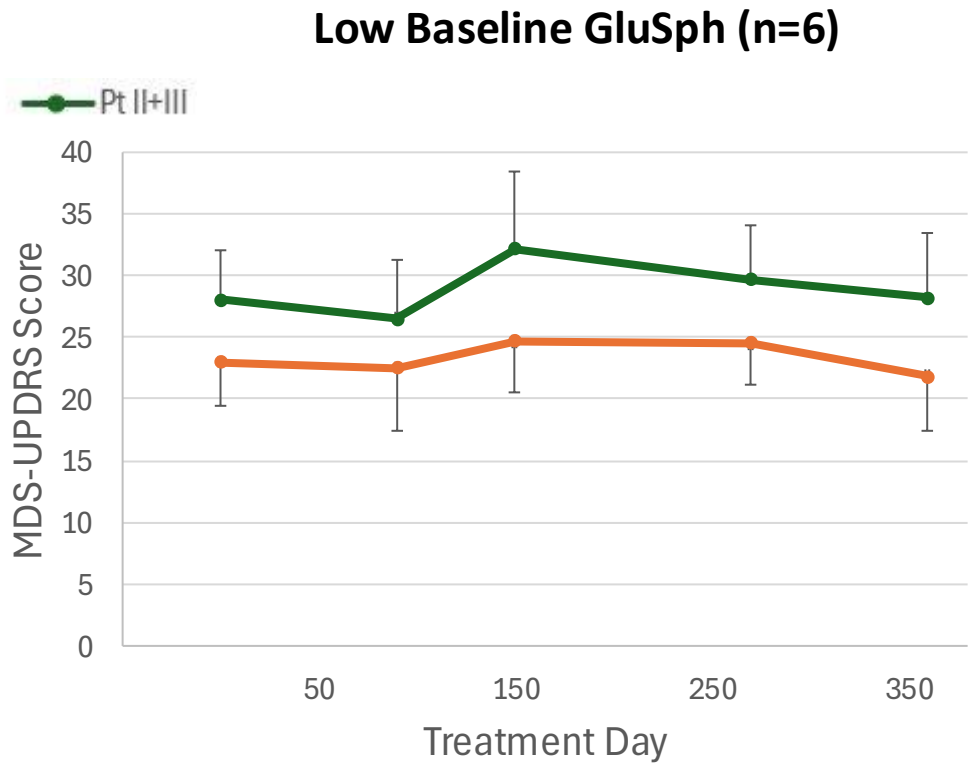


Modified From Pagano et al. NEJM 2022;387:421-432 DOI: 10.1056/NEJMoa2202867 *Data are mean ± s.e.m.*

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By 12 Months of *Rexaceract* Treatment, Patients with Both High and Low Baseline CSF GluSph Display Stabilization of MDS-UPDRS Motor Scores





Data are mean ± s.e.m.

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Rexaceract: A Disease-Modifying Therapeutic in Development for Parkinson's Disease

Mechanism	*Rexaceract* binds at allosteric site, and chaperones glucocerebrosidase (GCase) enzyme to lysosomes and mitochondria
Disease Modification	Restores GCase function and improves disease cascade and neuronal survival in both GBA1 and iPD models
Efficacy	Phase 1b shows statistically significant biomarker evidence of central target engagement with clinical improvement measured via MDS-UPDRS scores
Safety/Tolerability	Well-tolerated in both healthy volunteers and PD patients with therapeutic CNS exposures achieved
Next Steps	Start of Phase 2 trial in Q1 2027

Summary of *Rexaceract* Evidence:

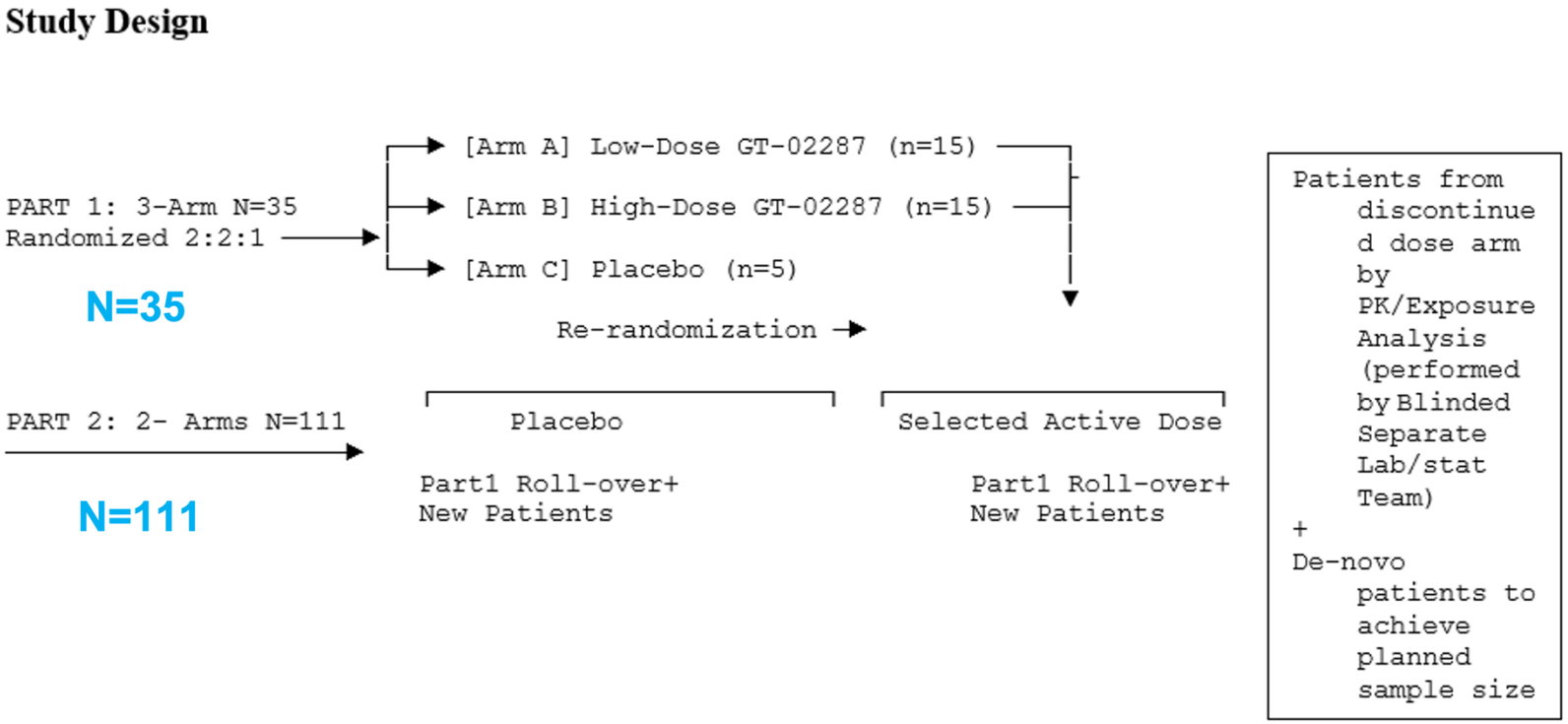
- Patients with elevated baseline **CSF GluSph** had an average 81% reduction after 90 days of treatment
- Elevated baseline **DDC** in **high CSF GluSph** group also reduced following 90 days of treatment
- 12-month results, including all participants expected by the end of November
- Preliminary Day 360 results from 12 (of 16) evaluable patients participating in Part 2 show no meaningful progression of motor symptoms
- Both baseline high and low CSF GluSph groups show stabilization of MDS-UPDRS motor scores by day 360
- The newly collected 9-month data show no new safety signals

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Proposed Phase 2 Study Design

New Design: An adaptive dose-optimization and proof-of-concept study designed to identify the effective and safe dose for Phase 3

- Small sample size in Part 1 AND expansion to 111 patients sample size in Part 2 (90 evaluable at endpoint)
- Primary Endpoint: Safety and Tolerability
- Key Secondary Endpoint: MDS-UPDRS Parts 3, 2, and 1



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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

REPLAY

OCTOBER 2025

BIOMARKERS, CLINICAL ENDPOINTS, AND THE PATH TO DISEASE MODIFICATION: CONTEXUALIZING THE EMERGING DATE 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

REPLAY

THE MICHAEL J. FOX FOUNDATION FOR PARKINSON'S RESEARCH

The Silverstein Foundation for Parkinson's with GBA

Schweizerische Eidgenossenschaft
Confédération suisse
Confederazione Svizzera
Confederaziun svizra
Swiss Confederation
Innosuisse – Swiss Innovation Agency

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Financials

- Established in 2017, IPO March 2021
- Headquartered in Bethesda, MD with offices in Lugano, Switzerland, Barcelona, Spain
- 23 Employees
- Scientific Founder andChairman: Dr. Khalid Islam
- Partnered with several leading medical & scientific organizations

Financials

- **Nasdaq: GANX**
- Cash: $13.1 million as of June 30, 2026
- Cash Runway: through 1Q27
- No debt
- 43.3 million shares outstanding

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GANX Investment Summary

Clinical PoC for *rexaceract*, 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

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Thank you

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