
SPUR THERAPEUTICS
Toward More™
May 2026

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Imagine a world where a single dose of genetic medicine could truly alter the course of a disease—and the course of people's lives.

This is the vision that spurs us forward. That drives us on our mission to redefine what gene therapy can do, so we can bring its transformative impact to more people.

Moving toward life-changing therapies, and brighter futures.

Toward more.

# Toward tailored gene therapies

**Where many first-generation therapies fall short**

- Safety
- No improvement on standard of care
- Commercial uptake

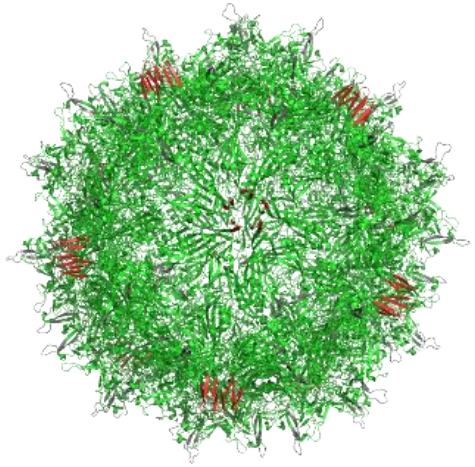
**Tailored gene therapies offer more**

- Optimized for specific diseases to drive higher efficacy at lower doses
- Improved outcomes, including changing the course of the disease
- Potential to impact more prevalent conditions

# Optimizing every component of our product candidates to realize outsized clinical results at lower doses

**Selective capsids**

Improved tissue targeting, more efficient transfers of genetic material, and reduced immunogenicity



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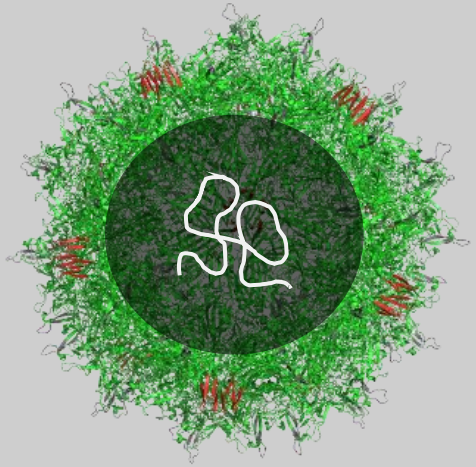
**Optimized genomes**

Promoter design and codon optimization improve tissue selectivity and advance protein synthesis



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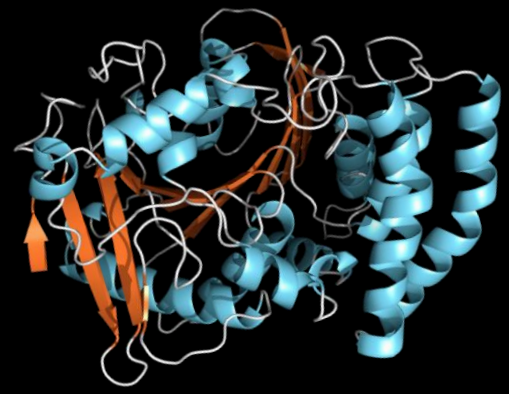
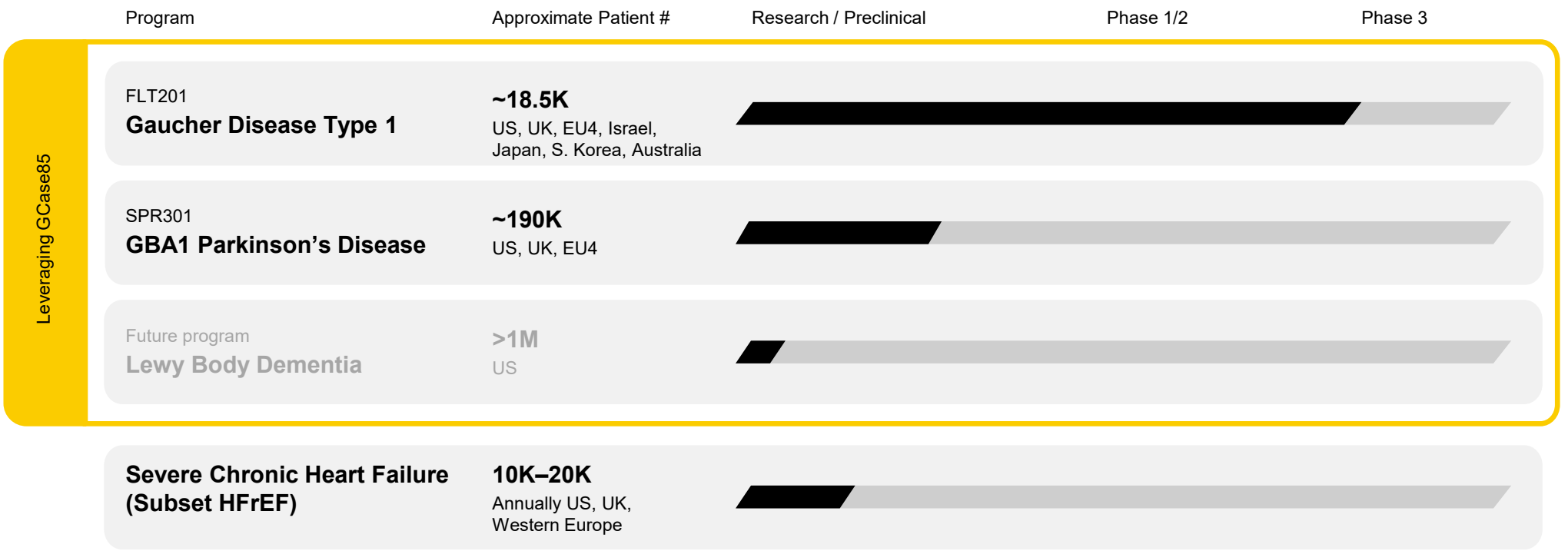
**Engineered therapeutic gene**

Increased half-life, stability, and activity, and more precise targeting of the therapeutic protein



**Come together to create our product candidates**

# Moving from rare to more prevalent conditions

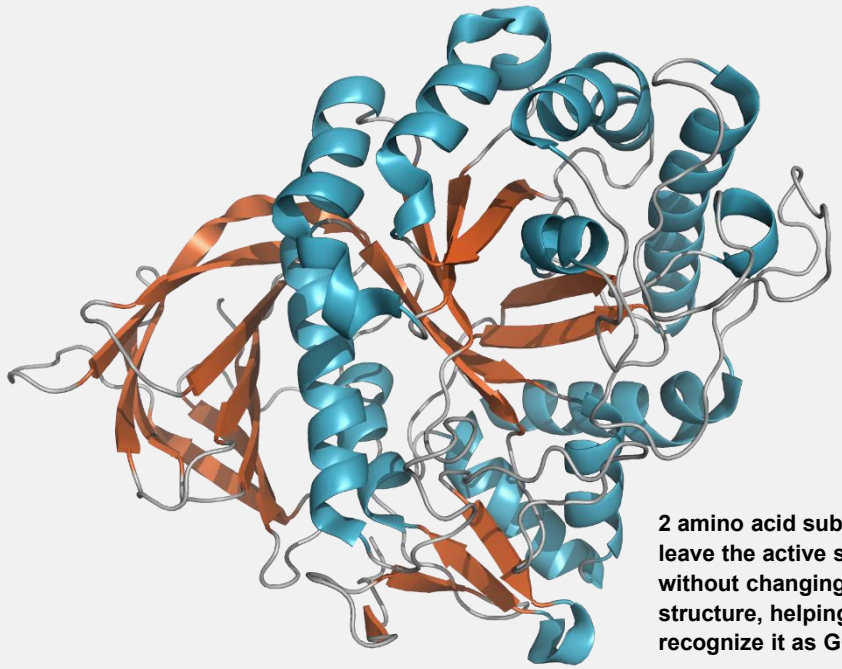



HFrEF = heart failure with reduced ejection fraction

Estimated patient numbers for Gaucher disease type 1 represent the total theoretical genetic prevalence of the indication. The seroprevalence of antibodies against the AAVS3 capsid renders some patients ineligible for AAVS3 gene therapy. Estimated GBA1-PD population is based on 5%-15% of diagnosed PD patients, representing the approximate number of patients with *GBA1* mutations. Lewy body dementia patient number from the Lewy Body Dementia Association. Estimated annual incidence of HFrEF based on company analysis.

# **GCase85:** An enzyme with transformative potential

Our rationally engineered GCase85 offers a longer half-life and increased stability, supporting greater activity levels at lower doses.



**2 amino acid substitutions leave the active site clear without changing the outside structure, helping the body recognize it as GCase**

**6X**

longer half-life in serum than the wildtype

**21X**

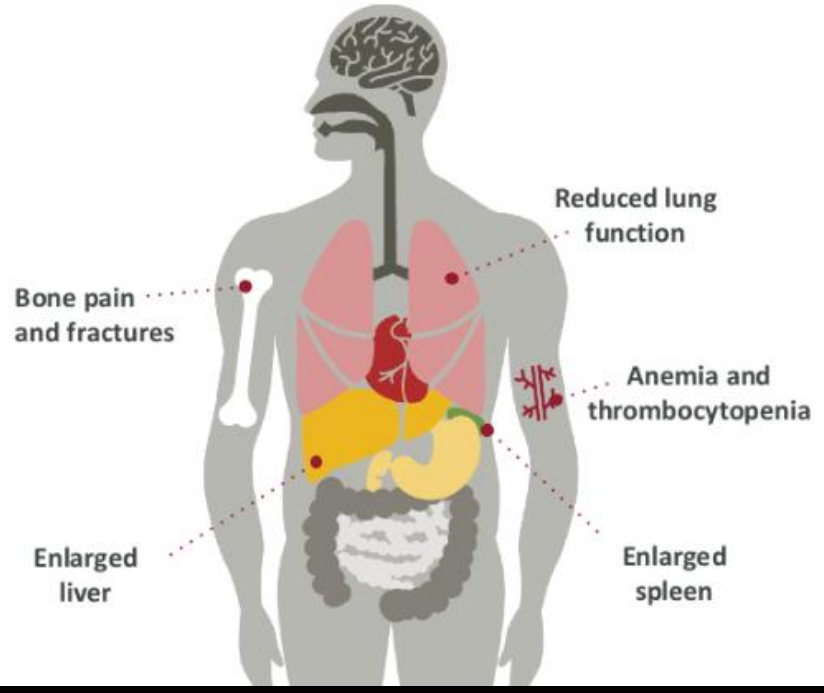
longer half-life in lysosomal pH— **6 days** instead of 6 hours

Gaucher disease can be debilitating, even with current treatments.

**Our new therapy candidate could change that—and change lives.**

# Targeting a chronic, progressive, and life-altering condition

**Gaucher disease type 1**
GCase deficiency leads to a buildup of toxic substrates, Gb-1 and lyso-Gb1, impacting multiple organ systems.




Many people experience debilitating symptoms despite lifelong treatment on ERT (current standard of care).

**95%** of people with Gaucher disease have type 1[1]

**~18.5K patients**
in US, UK, EU4, Israel, Japan, South Korea & Australia

[1]Charrow 2000; US, EU4, UK, Israel, Australia

# A need for a new treatment

The enzyme used in ERT has a short half-life, leaving patients without enzyme coverage between doses and with lingering symptoms.

Up to

**60%**

of people with Gaucher still experience symptoms after 10+ years on ERT[1]

[1] Weinreb et al. 2013

**80%**

of people with severe bone marrow burden showed no meaningful improvement after 8 years on ERT[2]

[2] De Fost 2006; low ERT dose cohort

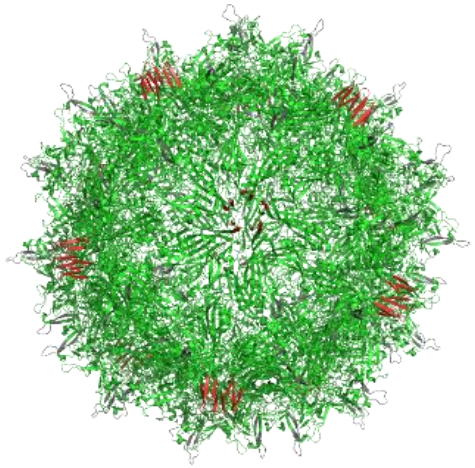
**65%**

report fatigue despite treatment with ERT[3]

[3] Wagner 2018

# **FLT201:** A first-in-class gene therapy candidate designed to address the limitations of ERT

**AAVS3 capsid** has much higher transduction efficiency than other AAVs



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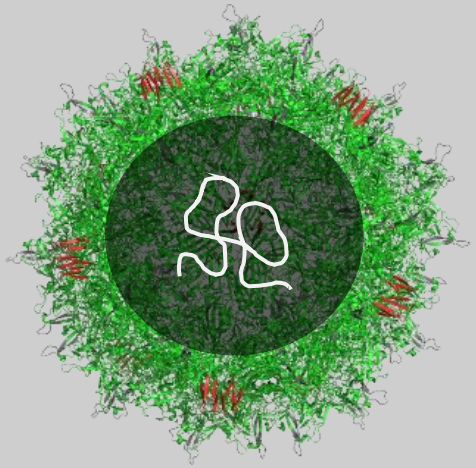
**Optimized genome** focuses expression in the liver



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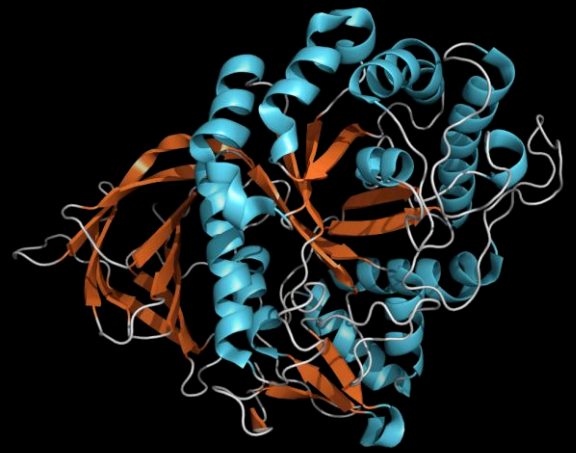
**Engineered GBA** transgene encodes more stable GCase85



**Demonstrating compelling efficacy and safety profile**

Data from completed Phase 1/2 trial

# Completed Phase 1/2 dose-finding study

GALILEO-1: A first-in-human, open-label, multicenter study of FLT201


- Patients come off prior therapy
- IM regimen begins 3 weeks post-infusion; 4 weeks of tacrolimus and 4 weeks of prednisone, followed by slow taper off prednisone

**Endpoints**

- Safety and tolerability
- Lyso-Gb1 levels
- Hemoglobin levels
- Platelet counts
- Spleen and liver volume
- Bone involvement
- PROs (pain, fatigue, QoL)

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= Dosed

**GALILEO-1 trial results:**

# Demonstrated safety and efficacy

Data support selection of low dose of 4.5e11 vg/kg for planned Phase 3 trial

## Clean safety

Favorable safety and tolerability in **all** dosed patients

## Compelling efficacy[1]

Dramatic improvements in **lyso-Gb1** in patients with persistently high levels despite prior therapy

Maintenance or improvement in **hemoglobin, platelets, bone disease** and **organ volume**

Significant reduction in **pain** and **fatigue** in the one patient who entered trial with debilitating pain and fatigue

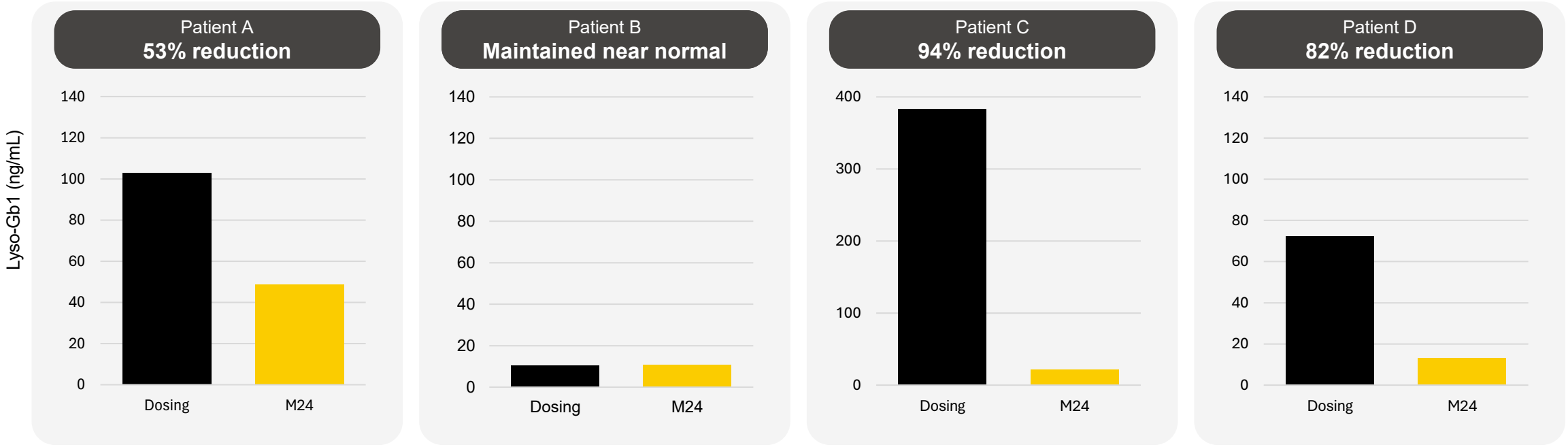
**~50% of Gaucher disease type 1 patients** are AAVS3 NAb-negative and available for treatment with FLT201

[1]One patient with detectable neutralizing antibodies (NAbs) to the AAVS3 capsid below protocol cut-off was excluded from efficacy analysis; only patients with no detectable NAbs will be eligible for Phase 3 trial. Efficacy data for another patient not taken off background therapy is not shown.

**GALILEO-1 trial results:**

# Dramatic reductions in lyso-Gb1 levels sustained up to two years to date

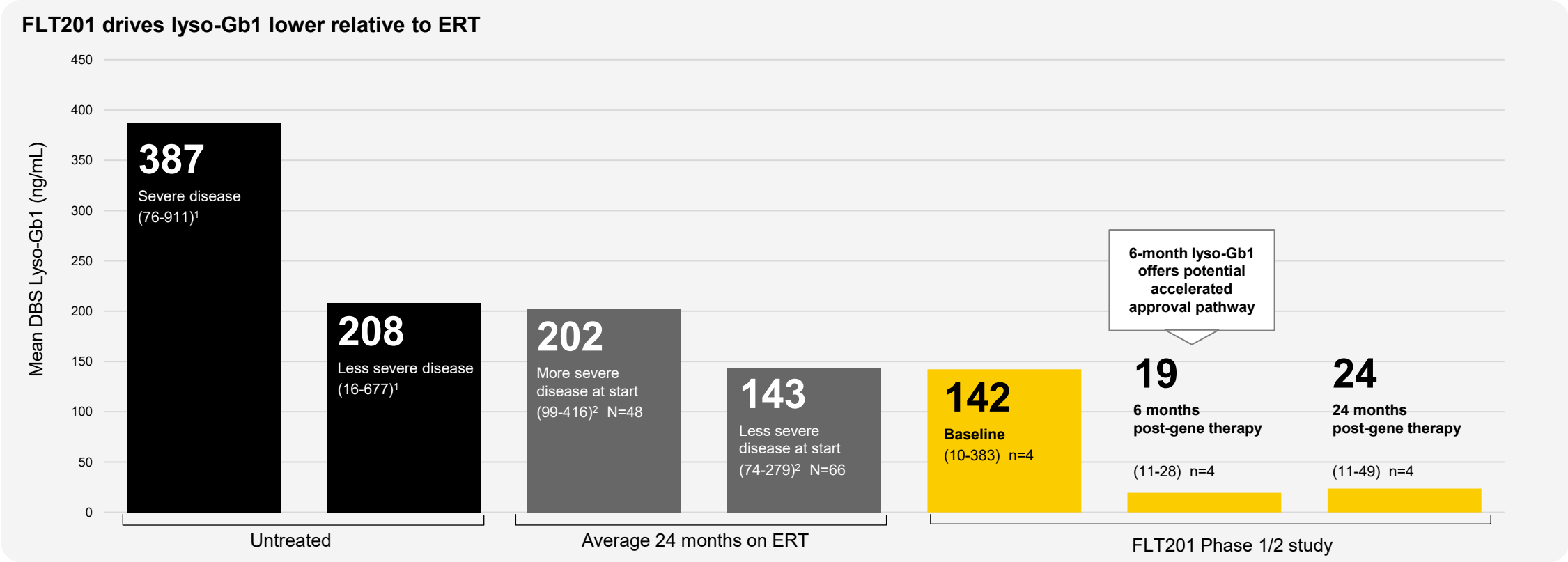
**One of the best predictors of disease severity and clinical response, lyso-Gb1 is a potential endpoint for 6-month approval pathway**




Dried blood spot lyso-Gb1 concentration over time.
Data cut off November 20, 2025
Patients A-D have been off their background therapies ~22-26 months as of data cut

**GALILEO-1 trial results:**

# FLT201 rapidly reduces lyso-Gb1 to near-normal levels




[1]Curado 2023 (mean value and range); [2]Dinur 2021 (model-based marginal means and 95% CI for males)

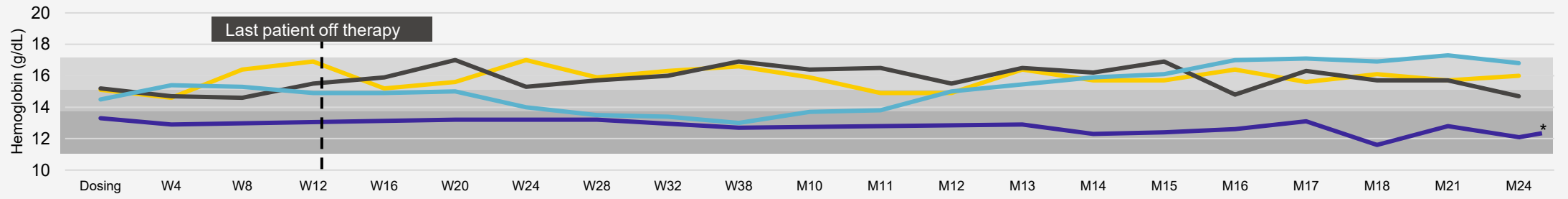
Mean DBS lyso-Gb1 concentration (range); mean concentration in healthy population is 5.4 (1.5-16) ng/mL (Dinur 2022); Data cut off November 20, 2025

**GALILEO-1 trial results:**

# Sustained improvement or maintenance of hemoglobin and platelets observed in all patients, including after withdrawal of ERT or SRT

**Reductions are seen quickly in heme and platelets when patients come off ERT/SRT[1]**

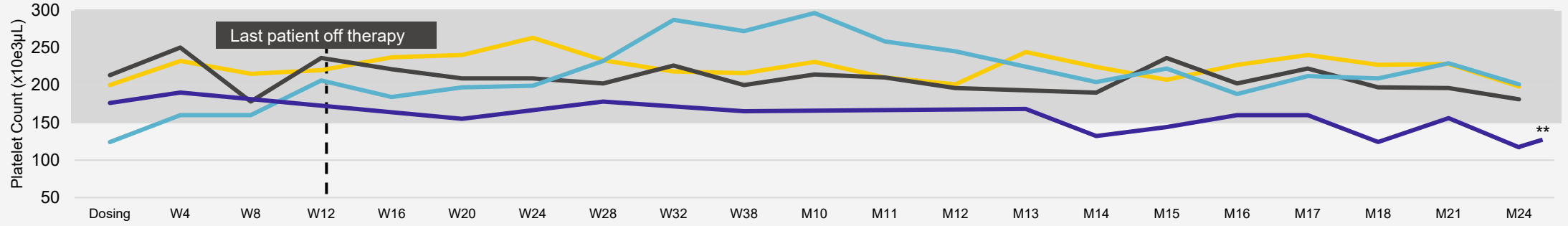
Hemoglobin concentration over time in individual study patients




Data cut off November 20, 2025

***Central lab value from an unscheduled M24.5 visit.**

Platelet count over time in individual study patients




Data cut off November 20, 2025

****Central lab value from an unscheduled M24.5 visit. A local lab platelet count was also collected at the same timepoint and was within the normal range (154 × $10^3$/µL).**

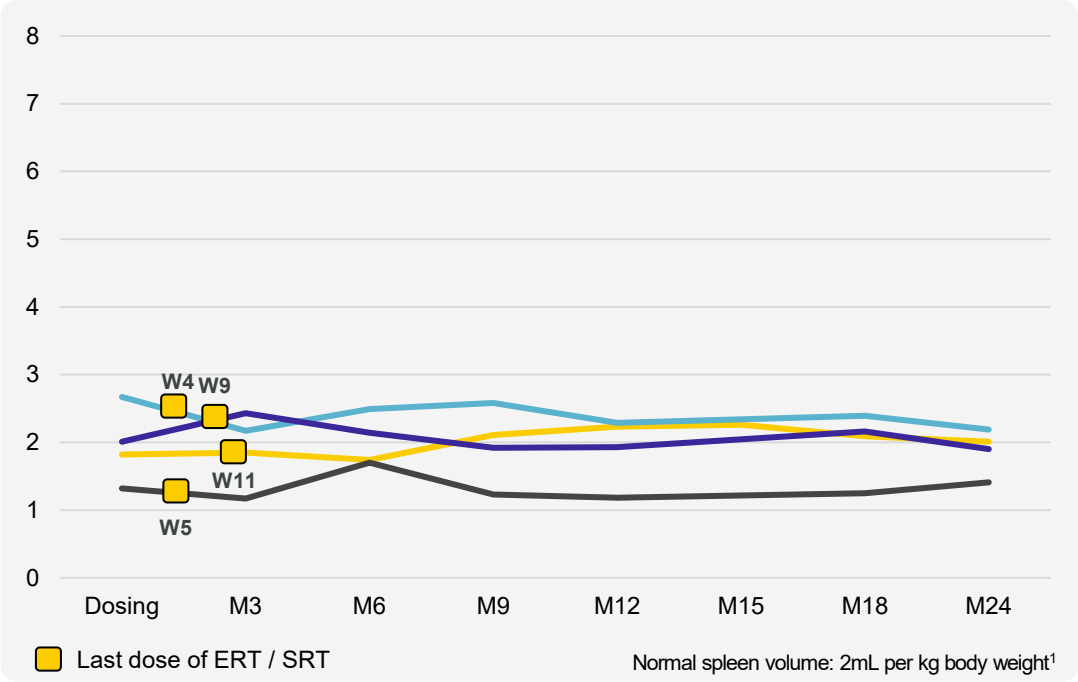
Patient A Patient B Patient C Patient D

[1]Zimran 2011

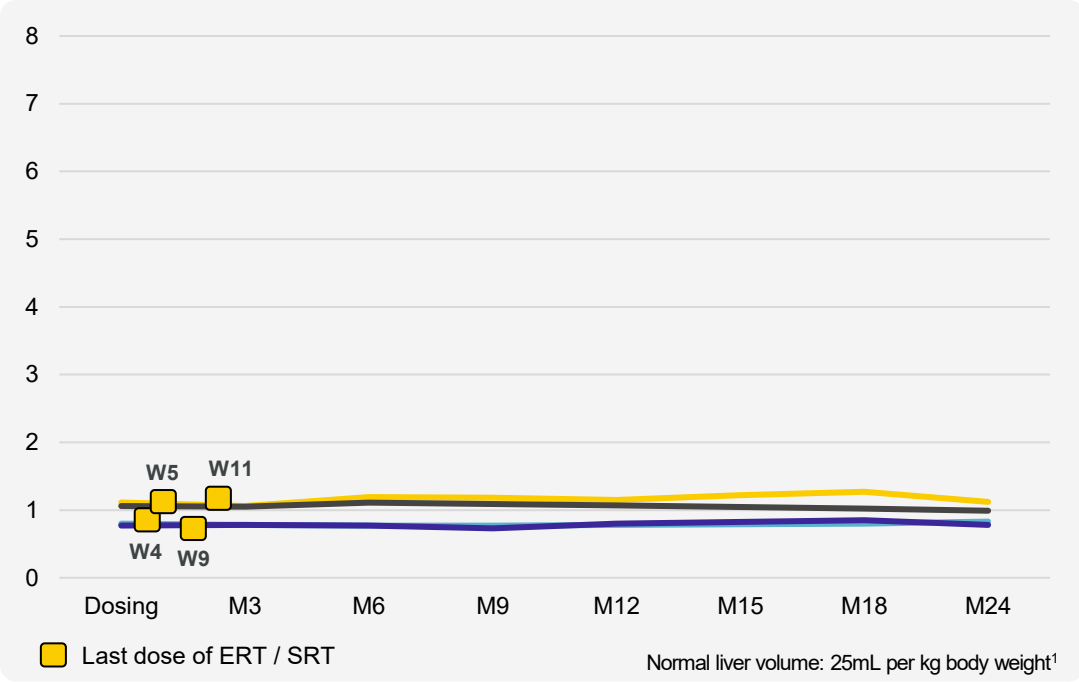
**GALILEO-1 trial results:**

# Spleen and liver volume maintenance or improvement observed after withdrawal of ERT and SRT

Spleen volume by MRI as a multiple of normal in individual study patients




Liver volume by MRI as a multiple of normal in individual study patients





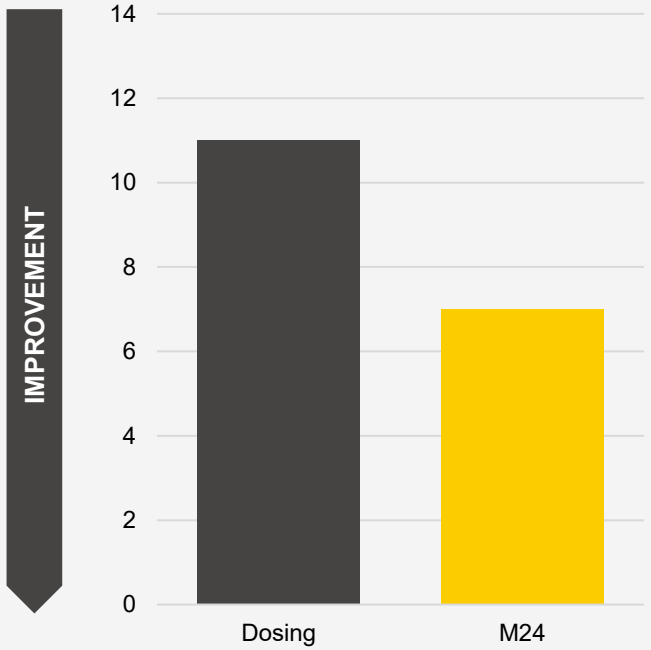
Data cut off November 20, 2025

[1]Pastores et al. *Blood Cells, Molecules and Diseases*. 2014;53: 253–260

**GALILEO-1 trial results:**

# Clinically Meaningful Improvement in Patient with Significant Bone Infiltration

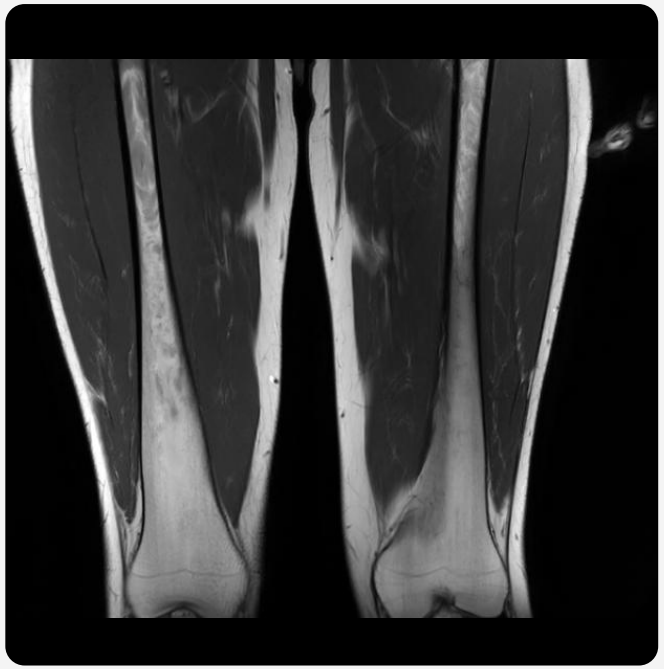
**Improvement in bone marrow burden (BMB)**



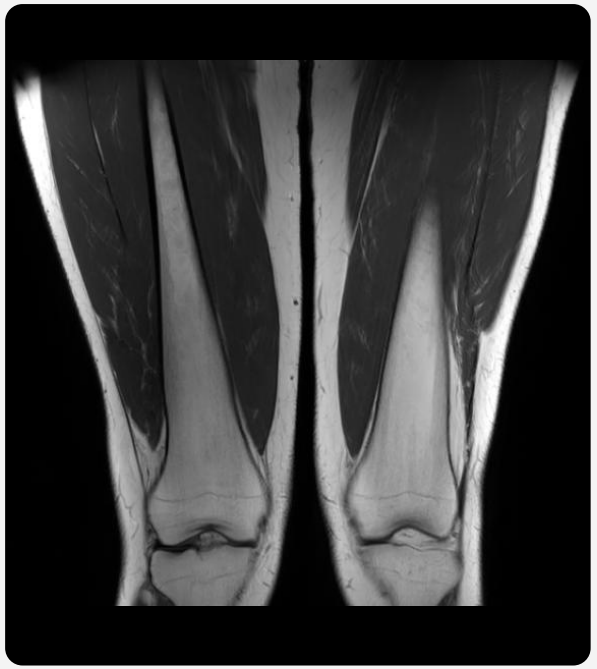

BMB correlates with bone necrosis, fractures, bone pain and joint replacements and remains one of the greatest unmet needs in Gaucher disease

**MRI shows clearance of diseased cells and reappearance of healthy fatty marrow**

**Baseline: SOC for 9 years**



**FLT201 for 2 years**





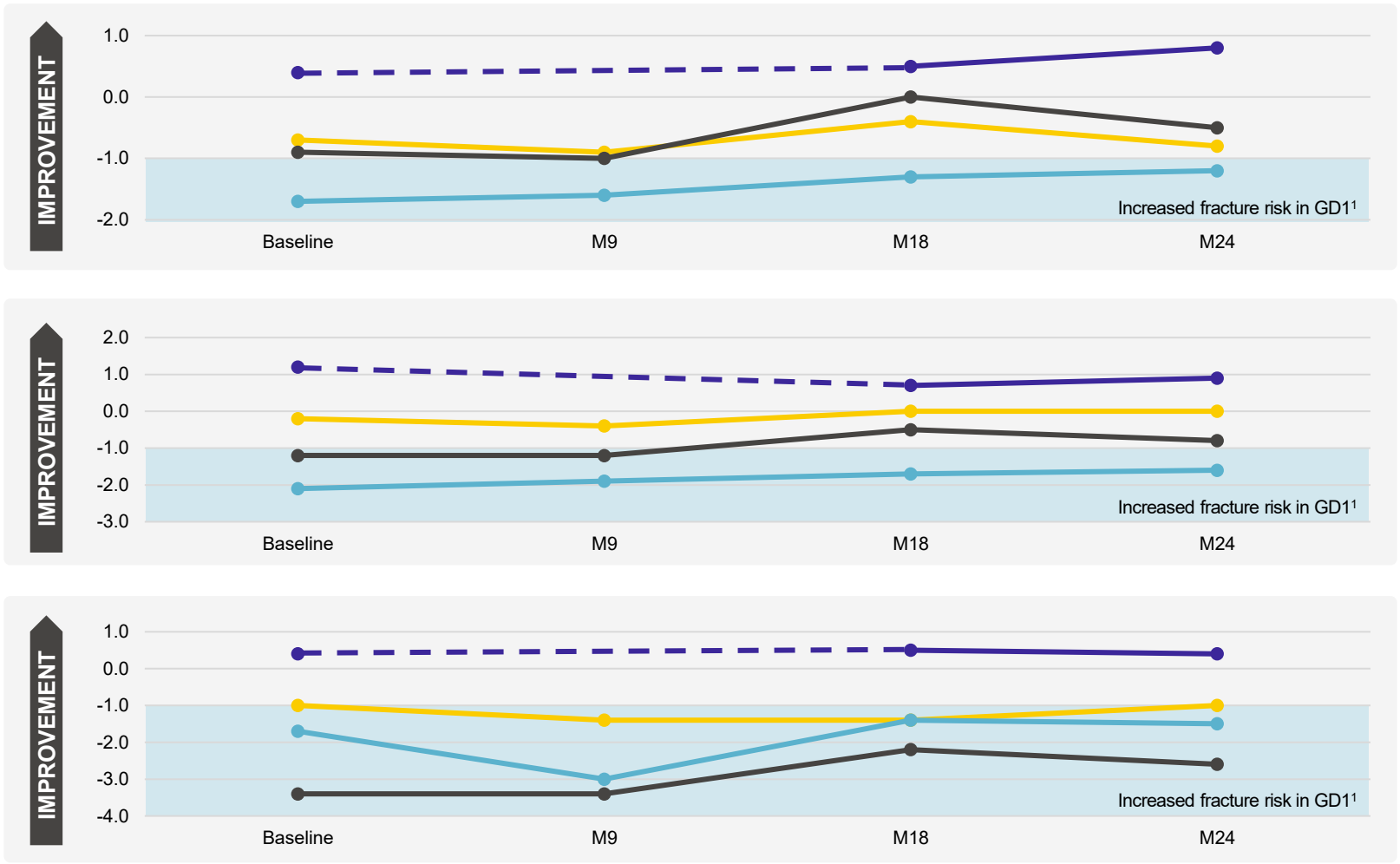
Data cut off November 20, 2025 Patient C BMB scores and MRIs presented

**GALILEO-1 trial results:**

# Clinically meaningful improvement in bone density Z-score

- Z-scores ≤ -1.0 are associated with a significantly increased risk of fracture in GD1 patients[1]
- At study entry, significant bone pathology was seen even among patents with hematologic and organ volumes managed on SoC
- Z-scores improved significantly in the 2 patients with the greatest pathology

**DEXA Z Scores Over Time**




Data cut off November 20, 2025

Patient D is missing data for Week 38. The line connecting the surrounding points is dotted to indicate the missing measurement.

1. Khan A, et al. *Journal of Bone and Mineral Research*, 27 (8); 2012

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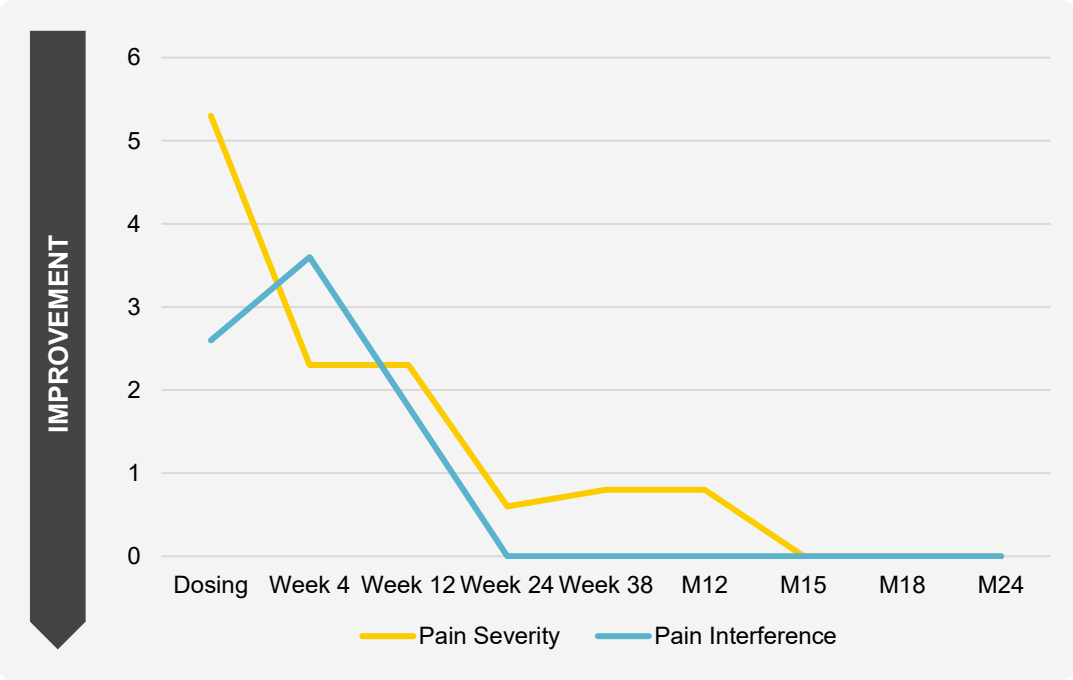
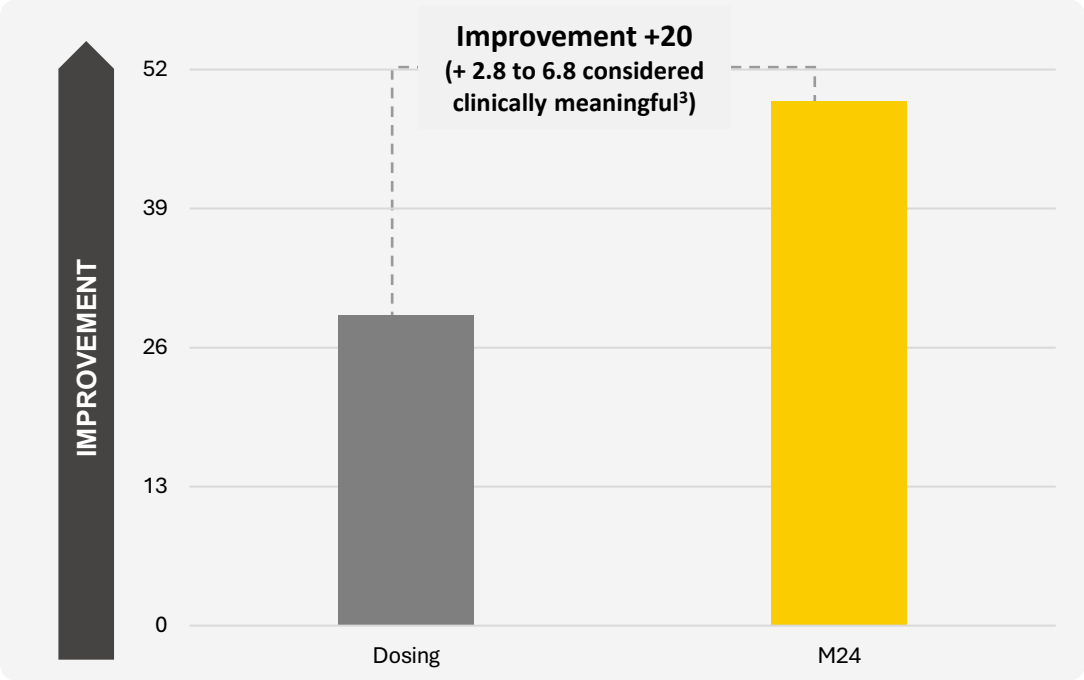
**GALILEO-1 trial results:**

# Substantial improvement in fatigue and pain leading to improved functioning

**Pain severity and interference (0-10)[1]**




**FACIT fatigue scale (0–52)[2]**




Patient A data presented

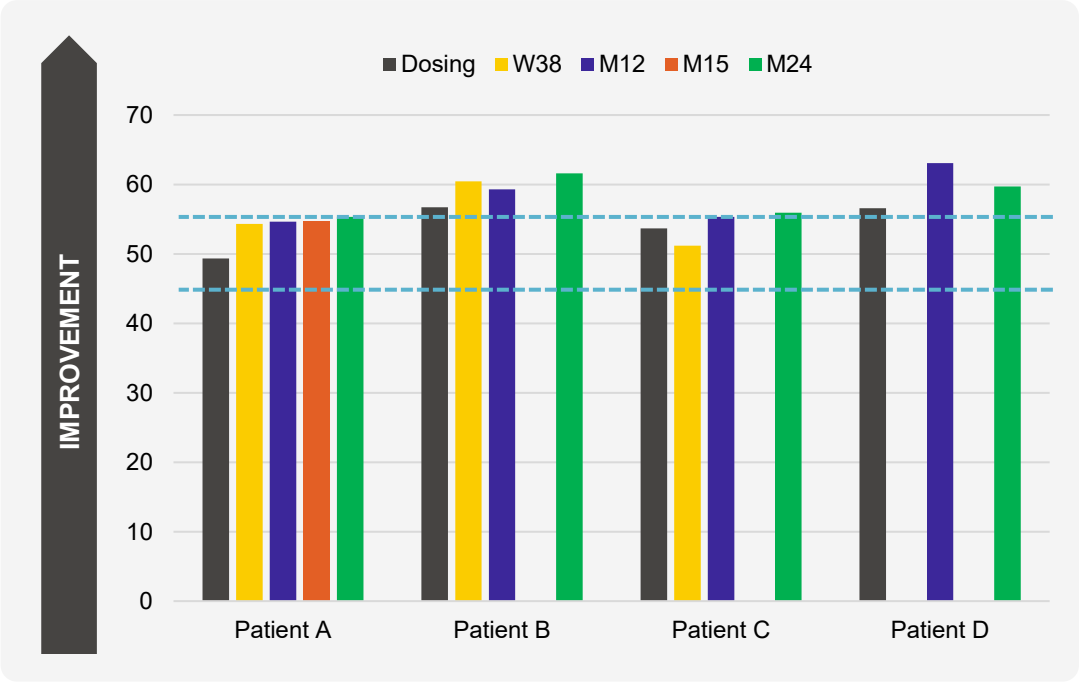
Data cut off November 20, 2025

[1]Measured by Brief Pain Inventory Short Form; [2]FACIT = Functional Assessment of Chronic Illness Therapy; [3]Greenbaum 2020; clinically meaningful in cancer, lupus, HUS, RA

**GALILEO-1 trial results:**

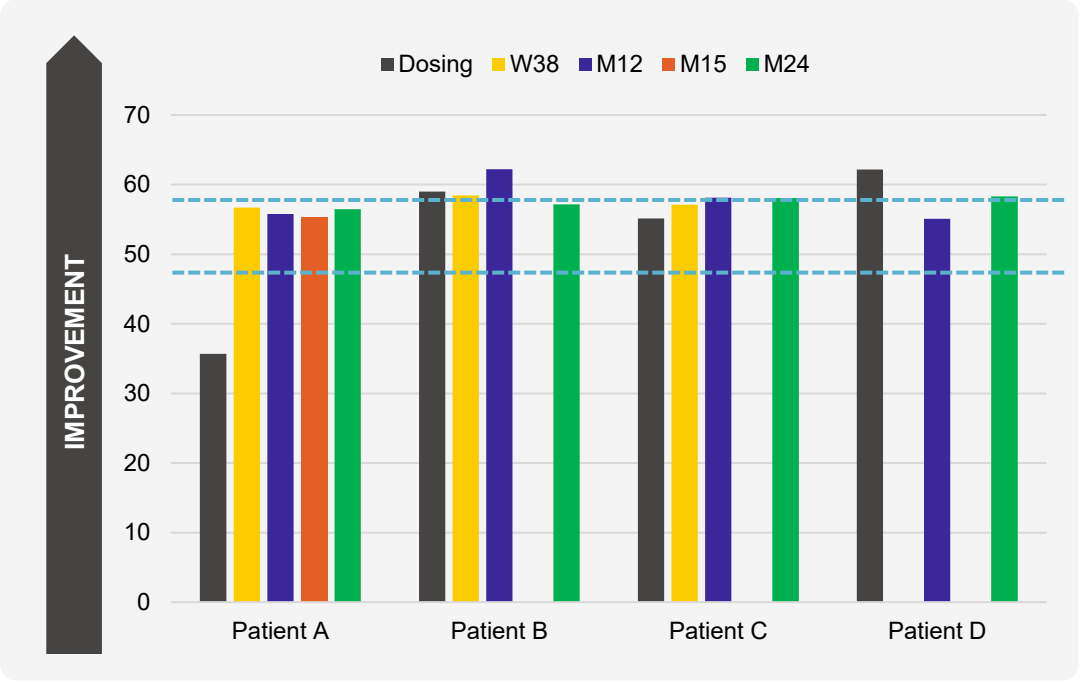
# Overall patient-reported improvements in physical and mental health

**Overall physical score change over time as measured by SF-36**




**Minimally Important difference in physical health score: 2-point change**

**Overall mental score change over time as measured by SF-36**




**Minimally Important difference in mental health score: 3-point change**

Normal upper and lower range bounds based on a healthy US population (n=4024)

Data cut off November 20, 2025

SF-36 = 36-Item Short Form Health Survey

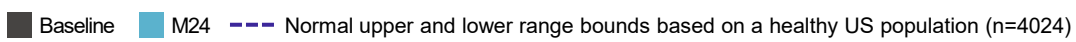
Maruish, M. E. (Ed.). User's manual for the SF-36v2 Health Survey (3rd ed.). Lincoln, RI: QualityMetric Incorporated.

**GALILEO-1 trial results:**

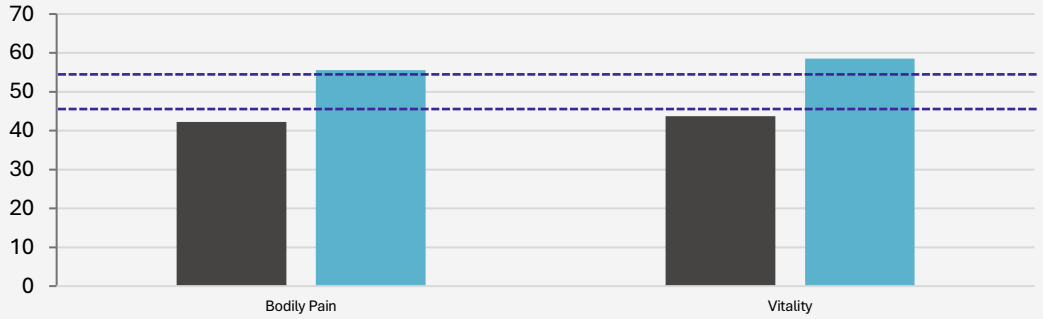
# Patient-reported improvements in pain and vitality/fatigue

**Addressing key area of unmet need in the market**

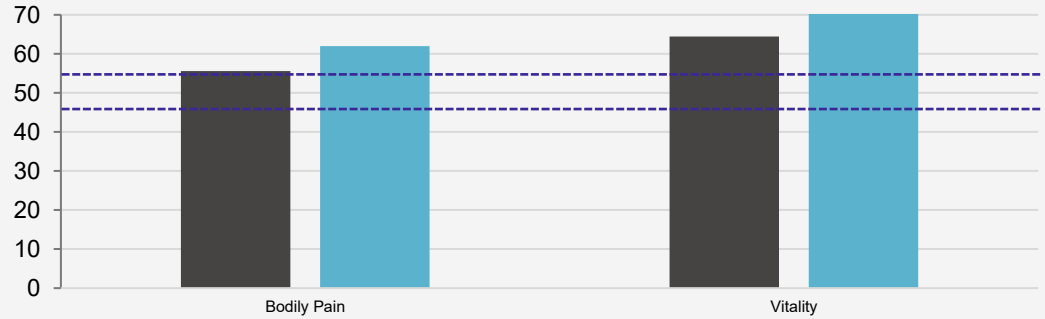
SF-36 Scores at Baseline and Month 24



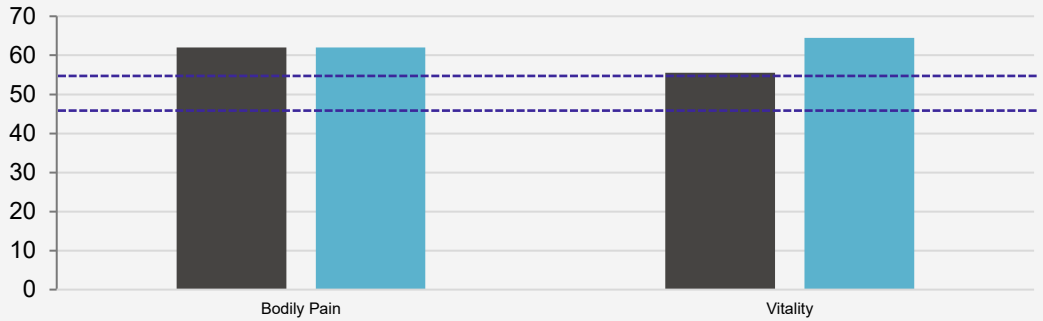

**Patient A**



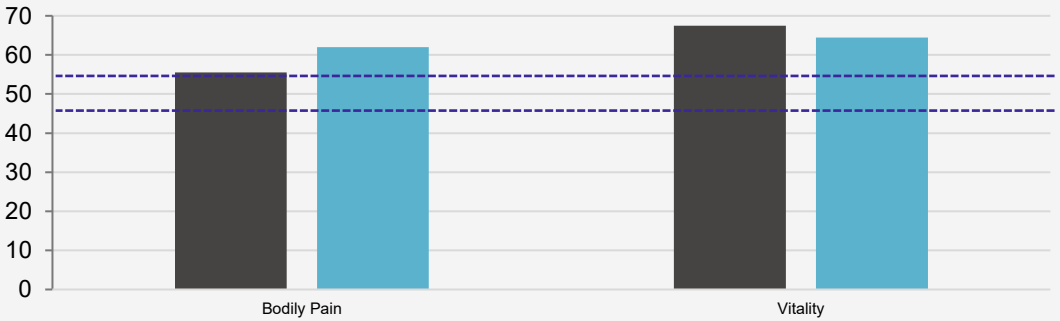

**Patient B**




**Patient C**




**Patient D**




Data cut off November 20, 2025

SF-36 = 36-Item Short Form Health Survey; 2 of the sub-score measurements are represented.

Maruish, M. E. (Ed.). User's manual for the SF-36v2 Health Survey (3rd ed.). Lincoln, RI: QualityMetric Incorporated.

**Minimally Important Difference:**
The smallest change in score that a person perceives as meaningful.

| | |
|---|---|
| Bodily Pain | 3 |
| Vitality | 2 |

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GALILEO-1 trial results:

# Well tolerated, with a favorable safety profile

- No dose-limiting toxicities
- Two cases of ALT elevations above normal range deemed related to therapy
  - Spontaneously resolved or managed with immune therapy
  - Transient anti-GCase antibodies in two patients
- ADRs related to immune management consistent with known profile

| Summary of Adverse Drug Reactions for FLT201 and Immune Management (n≥2) | |
|---|---|
| **Adverse Drug Reactions (ADR)** | # events (# patients) |
| **FLT201** | |
| Elevated alanine aminotransferase (ALT) | 7 (6) |
| Fatigue | 4 (3) |
| Activated partial thromboplastin time prolonged | 2 (2) |
| Anti-GCase neutralizing antibodies | 2 (2) |
| **Prednisone** | |
| Hyperglycemia | 3 (3) |
| Weight increase | 2 (2) |
| Discomfort | 2 (1) |
| Panic attack | 2 (1) |
| **Tacrolimus** | |
| Diarrhea | 4 (4) |

Data cut off November 20, 2025

# Regulator-aligned Phase 3 design

~35 trial sites selected across US, UK, EU, Latin America and Israel

**Patient population: ~45 adults with Gaucher type 1 on ERT or SRT**

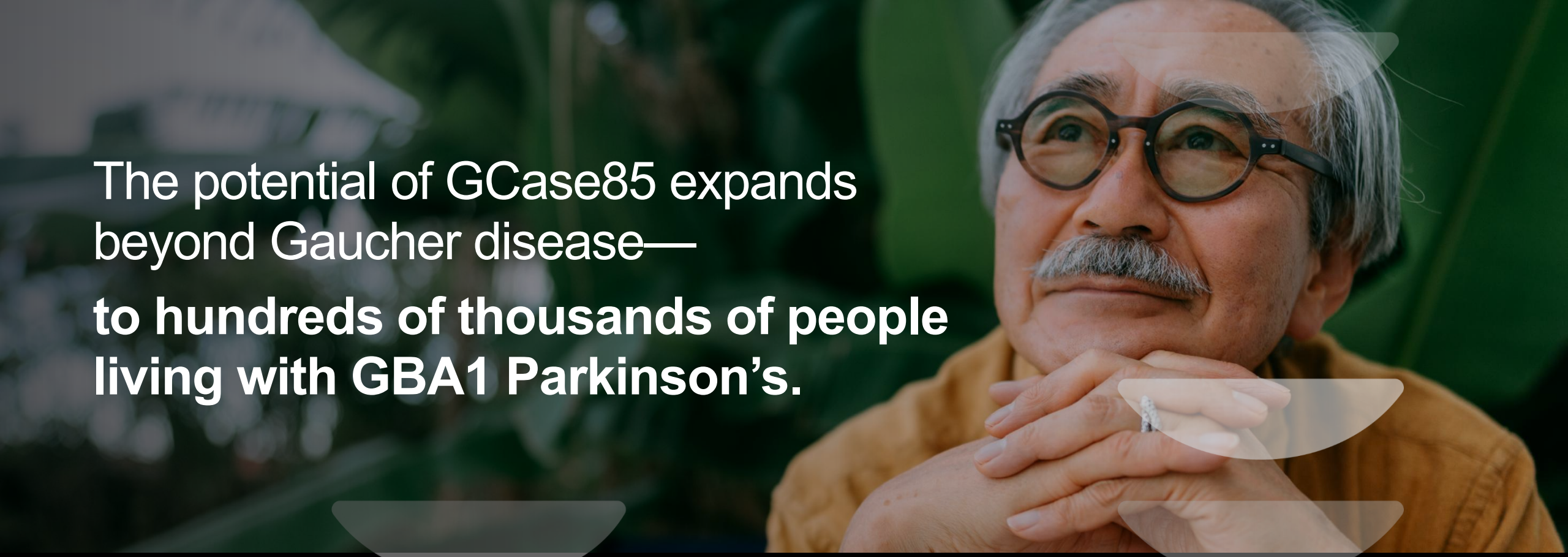
Patient population comparable to Phase 1/2 study


FLT201 administration (4.5e11 vg/kg dose)

Potential accelerated approval endpoint: Change in lyso-Gb1 levels at 6 months vs. baseline

Endpoints: Maintenance or improvement of hemoglobin levels, platelet count, spleen and liver volume at 12 months vs. baseline

[1]Standard of care (SoC) continues until week 4 post-dose

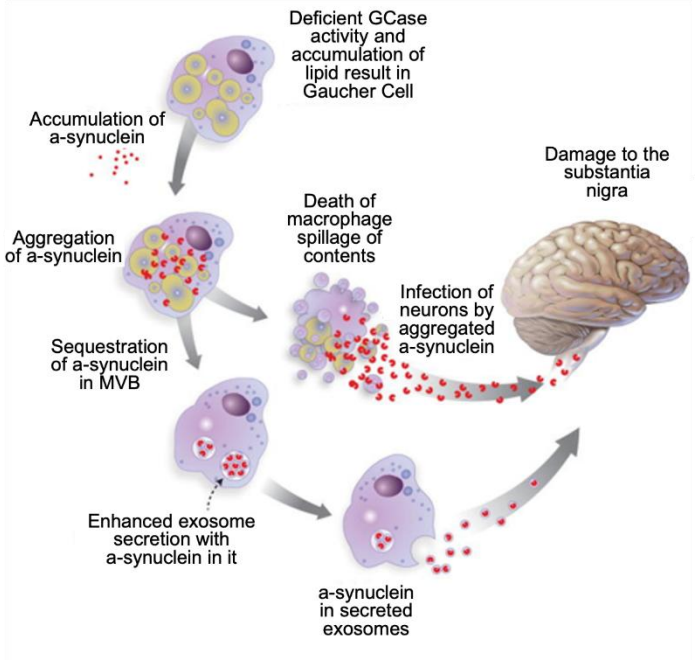


The potential of GCase85 expands beyond Gaucher disease—

**to hundreds of thousands of people living with GBA1 Parkinson's.**

# A debilitating disease with a clear, unmet need

**GBA1 Parkinson's disease**
GCase deficiency leads to accumulation of α-synuclein, a hallmark of Parkinson's




Progressive, neurodegenerative condition with no disease-modifying therapy

**5-15%**

of people with Parkinson's disease have *GBA1* mutations[1]

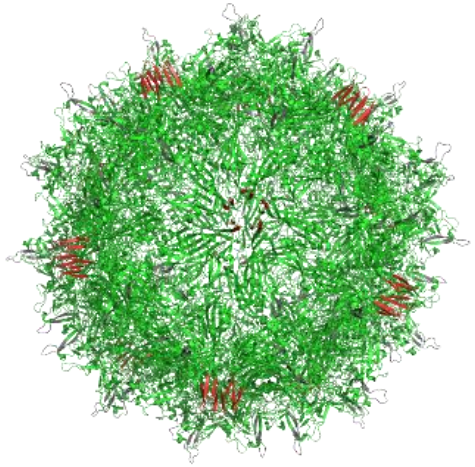
**~190K people**

have GBA1 Parkinson's in the U.S., U.K., and EU4

[1]*Cells* 2022, 11(8), 1261; https://doi.org/10.3390/cells11081261

# **SPR301:** A potentially disease-modifying treatment for GBA1 Parkinson's with a highly differentiated profile

**AAV9 capsid** is known for effective transduction of brain cells at low doses



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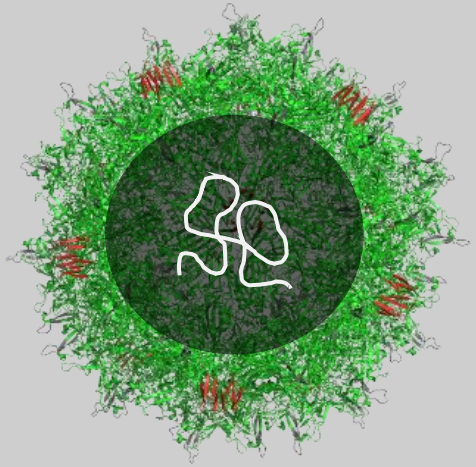
**Optimized genome** boosts neuronal cell expression while minimizing harmful astrocyte and microglia activation to increase therapeutic window



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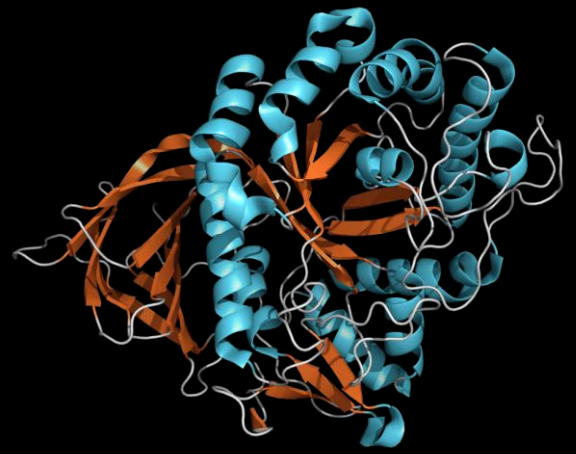
**Engineered *GBA1* transgene** encodes engineered GCase85, which offers dramatically longer half-life and more stability in the brain



**Achieving broad distribution at low doses**

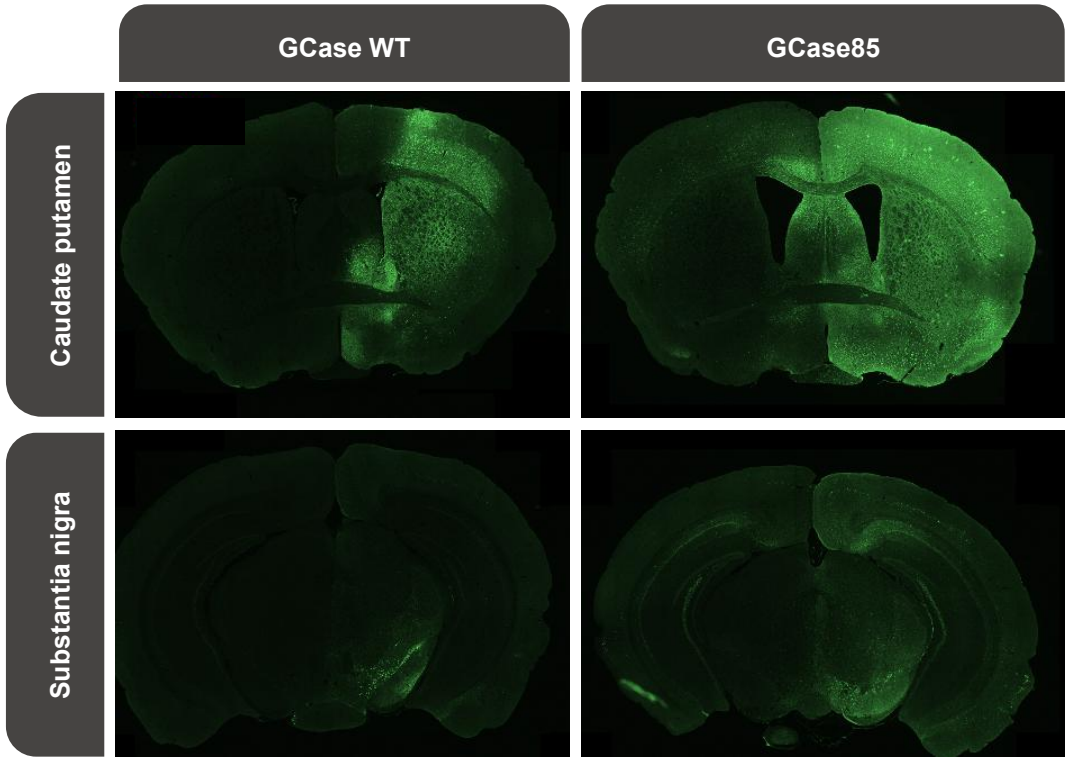
Data from preclinical studies

**SPR301 preclinical study results:**

# Superior distribution throughout the brain compared to wildtype

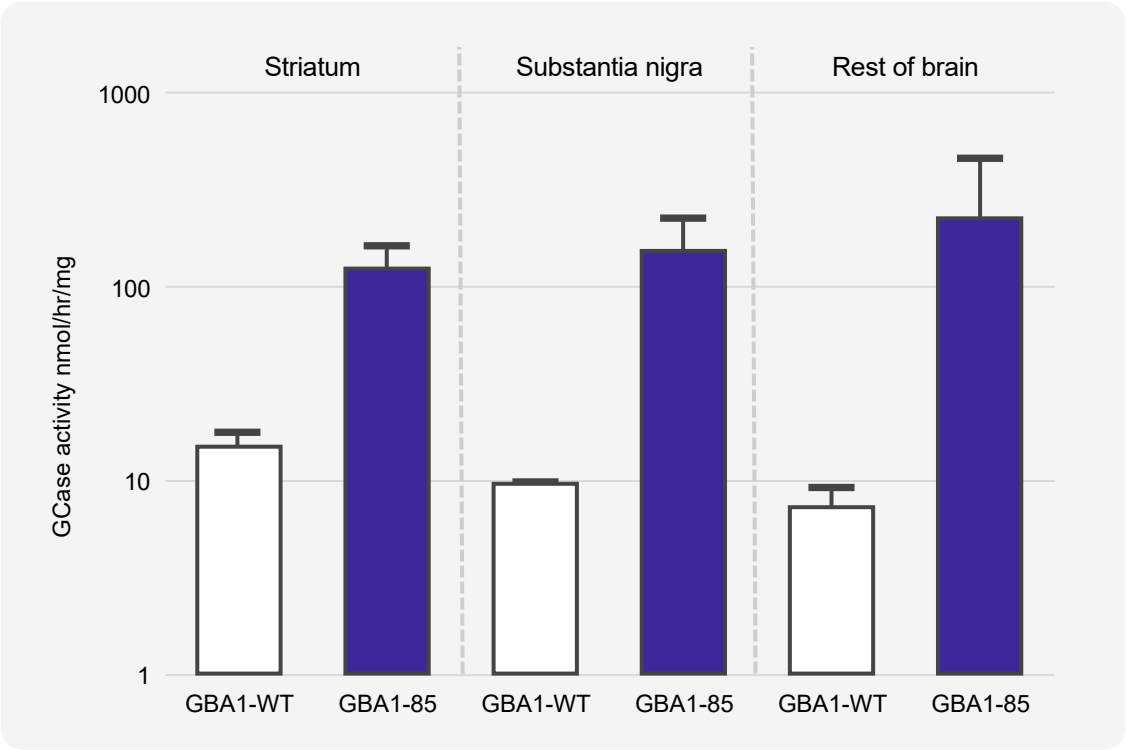
## GCase85 distributes broadly and cross-corrects non-transduced cells

Distribution in the brain




Representative coronal sections of animals injected with either AAV9-GBA1-WT or AAV9-GBA1-85 labeled for GCase, n=4. Dosed AAV9 at 1.3e10 vg per mouse by unilateral injection of the right hemisphere striatum.

Activity in brain regions

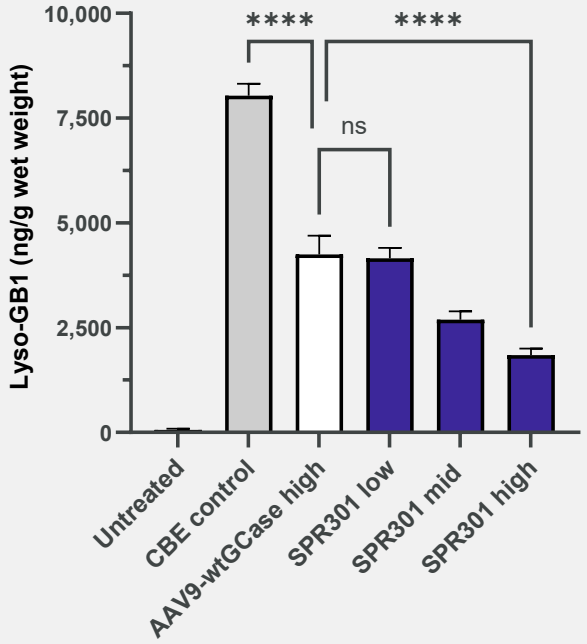



Injected with indicated AAVs, samples dissected from striatum, substantia nigra, or the rest of the brain. The GCase activity is normalized for VG, n=3, data denoted as mean ± SD.

**SPR301 preclinical study results:**

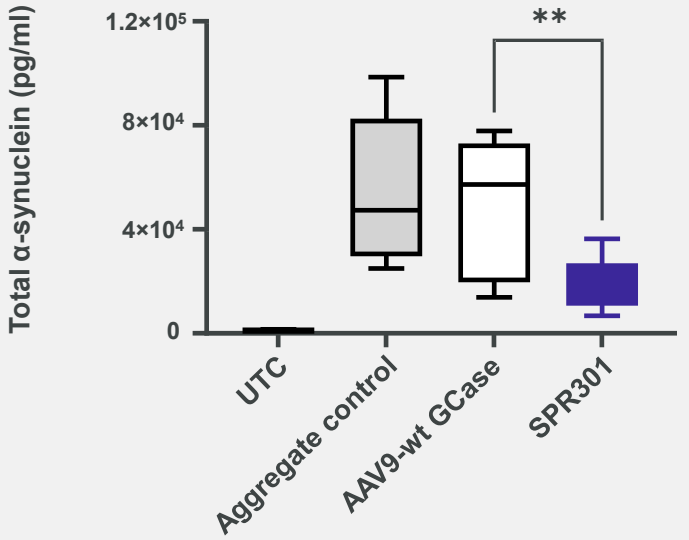
# Potential for greater efficacy with a favorable safety profile

**Wider therapeutic window, with 25-fold lower dose showing equivalent lyso-Gb1 reduction as high-dose AAV9-wtGCase**




CB57BL/6J (n=8) was treated with CBE 100 mg/kg i.p. daily for 15 days either alone or in combination with a single dose of AAV9 control (1.3e10 vg), AAV9-wt GCase (1.3e10 vg) or a series of increasing doses of SPR301 in 5-fold steps from low (5.2e8 vg) to medium (2.6e9 vg) to high (1.3e10 vg). All vector doses were administered directly to the putamen as a single injection. Ordinary one-way ANOVA; ***p=0.0002 and ****p<0.0001

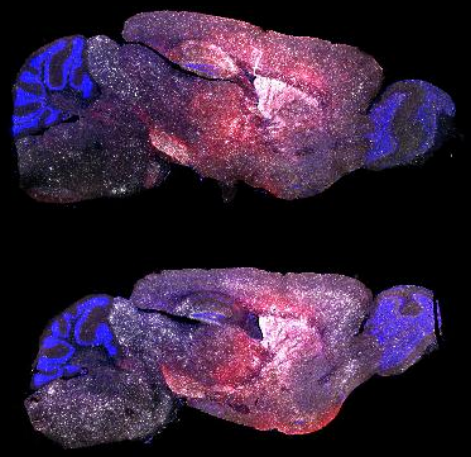
**Higher, sustained activity levels in the brain more effectively reduce α-synuclein in neuronal cells compared to wildtype**

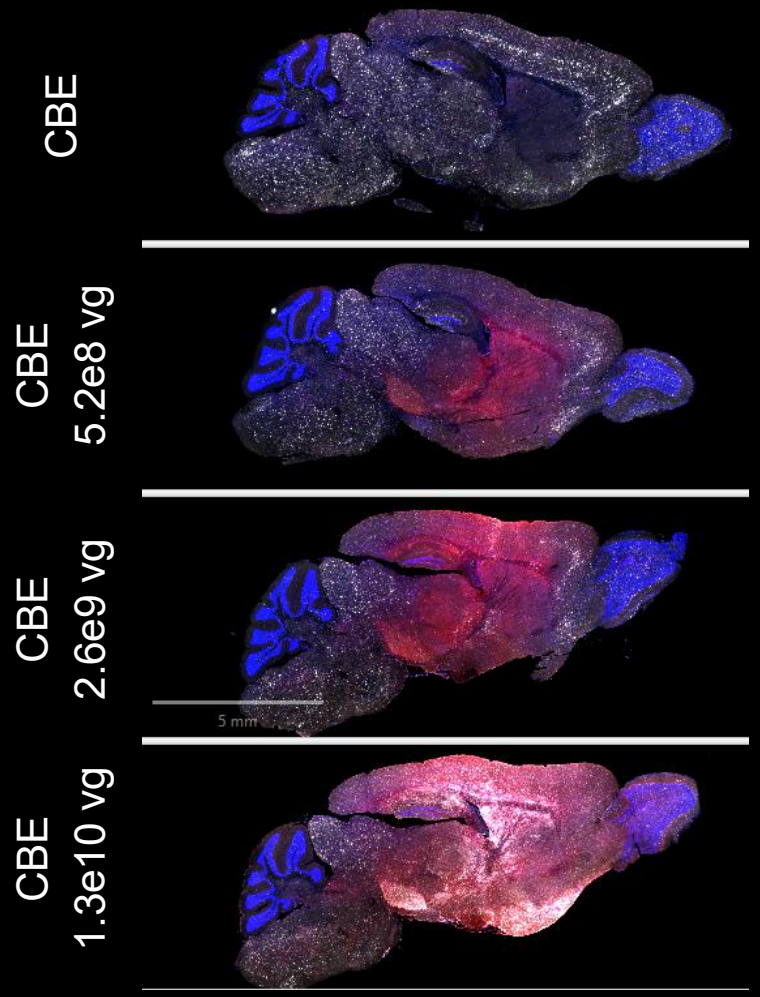



Tested in the surrogate disease model, SH-SY5Y plus α-synuclein (4µg/ml), with vectors at MOI 2.5x10$^5$; SH-SY5Y cells were pre-treated with *GBA* gene therapy for 24h before challenging them for 24h with recombinant α-synuclein aggregate; N=3 (n=6-10), data denoted as mean ± SEM. T-test analysis vs. AAV9-wtGCase; **p<0.01.

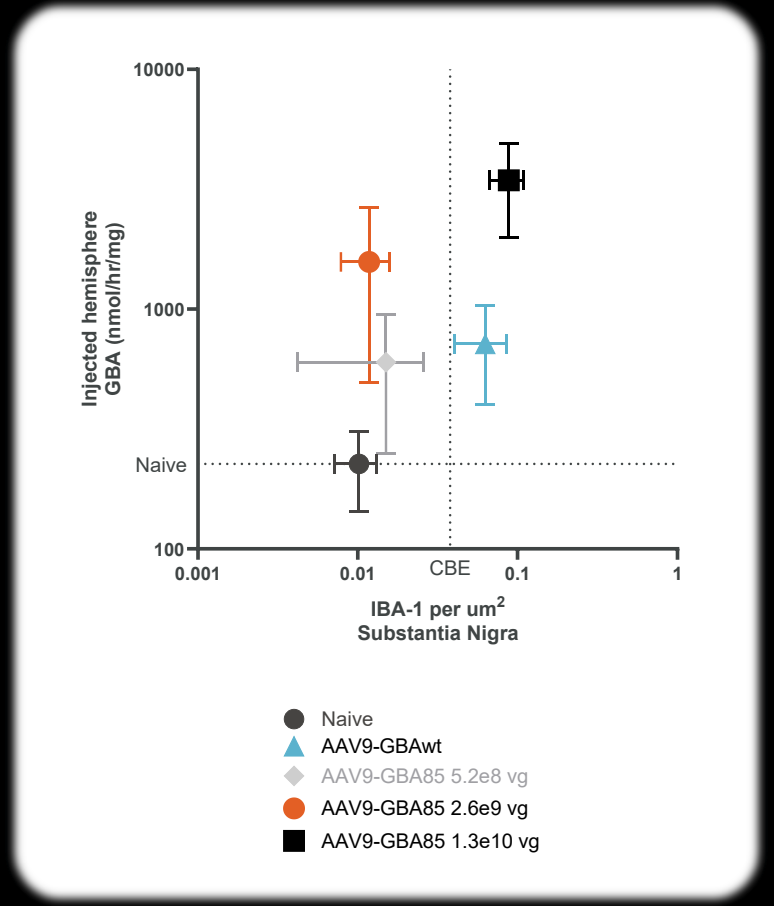
# SPR301 provides superior GCase exposure while minimizing microglia activation

CBE
AAV9-GBAwt
1.3e10 vg






DAPI
GBA
IBA-1




GBA glucocerebrosidase; IBA-1 marker for activated microglia

# Moving toward more

- Optimizing every component of our product candidates to realize outsized clinical results at lower doses
- Advancing Phase 3 gene therapy candidate with potential to set new standard of care for Gaucher disease
- Leveraging same rationally engineered transgene in preclinical gene therapy program for subset of Parkinson's disease patients with GBA1 mutations

**Creating more impact for more people.**

# A team known for making an impact

## Michael Parini

Chief Executive Officer and Director

20+ years as a senior executive in leading biopharmaceutical companies

VERTEX · Pfizer

## Pam Foulds, MD

Chief Medical Officer

25+ years of medical and clinical leadership

Auregen · Aegerion Pharmaceuticals · Biogen · genzyme

## Henning Stennicke, PhD

Chief Scientific Officer

25+ years of scientific leadership

novo nordisk · Sanford Burnham Prebys

## Paul Schneider

Chief Financial Officer

25+ years of global financial, commercial, and operational experience

EXO THERAPEUTICS · CASEBIA THERAPEUTICS · Aegerion Pharmaceuticals · Shire

## Jay Bircher

Chief Technical Operations Officer

30 years of quality and technical operations experience

Abeona Therapeutics · BD · gsk · AMGEN

## Nicole Jones

Chief People Officer

25+ years of global human resources experience

ALEXION · MERCK · Fidelity

## Chip McCorkle

VP, GC & Corporate Secretary

10 years of experience advising leading biopharmaceutical companies

Help us spur gene therapy **forward.**