
satellos
REGENERATING MUSCLE FROM WITHIN™

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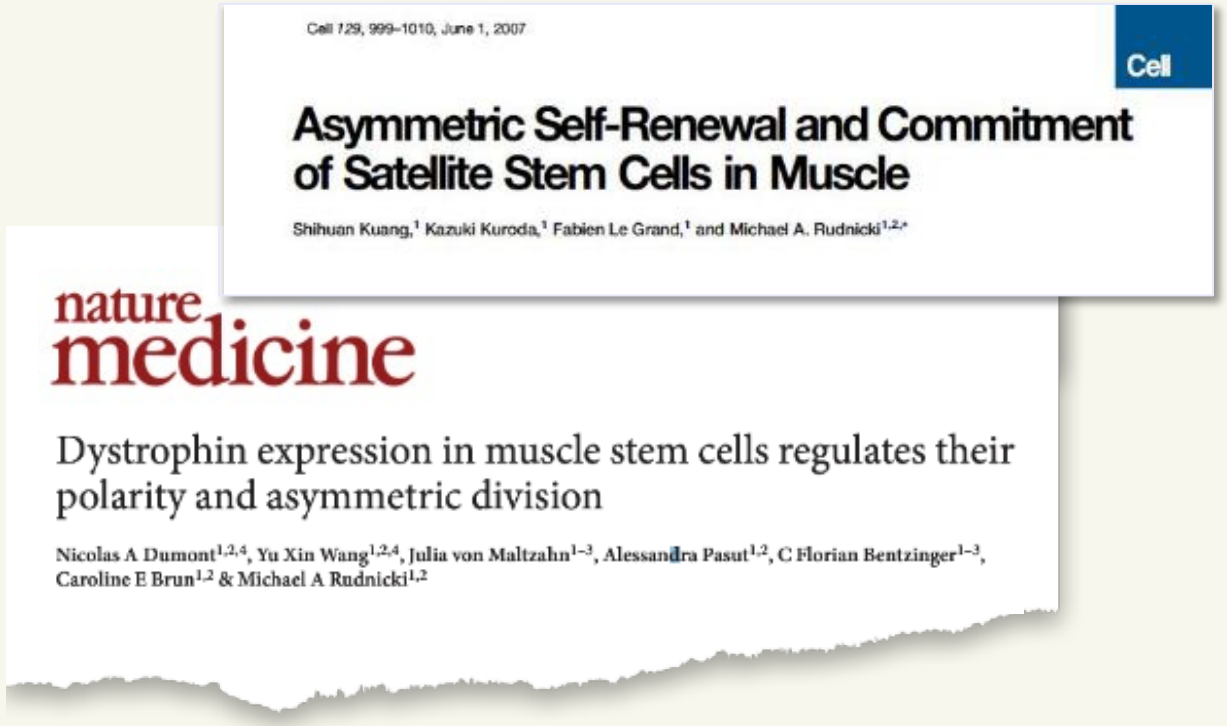
Regenerating Muscle and Restoring Function with SAT-3247

- Disruptive, differentiated approach
- Disease modifying potential in DMD
- Safe, well-tolerated, oral pill taken daily
- Intended to increase function vs delaying loss
- Applications in multiple degenerative conditions



Corporate Highlights

1.	2.	3.	4.	5.
Clinical stage company	Strong balance sheet	Upcoming flow of milestones	Accomplished leadership team	Elite institutional investors

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Grounded in science. Inspired by need.

Our mission is to improve the treatment of Duchenne muscular dystrophy and other devastating muscle diseases by restoring the body's ability to regenerate muscle.



CHARLIE, age 7,
living with Duchenne

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Rewriting the Duchenne Story



Infants	2 to 4 years	5 to 9 years	10 to 12 years	13 to 19 years	20+
Often no visible symptoms	Signs of early muscle weakness	Damage begins to outpace regeneration	≥70% of boys lose the ability to walk	Ventilator support needed; daily living activities limited	Increasing heart and respiratory challenges

Satellos is working to restore the body's ability to **regenerate and repair** skeletal muscle — throughout life.

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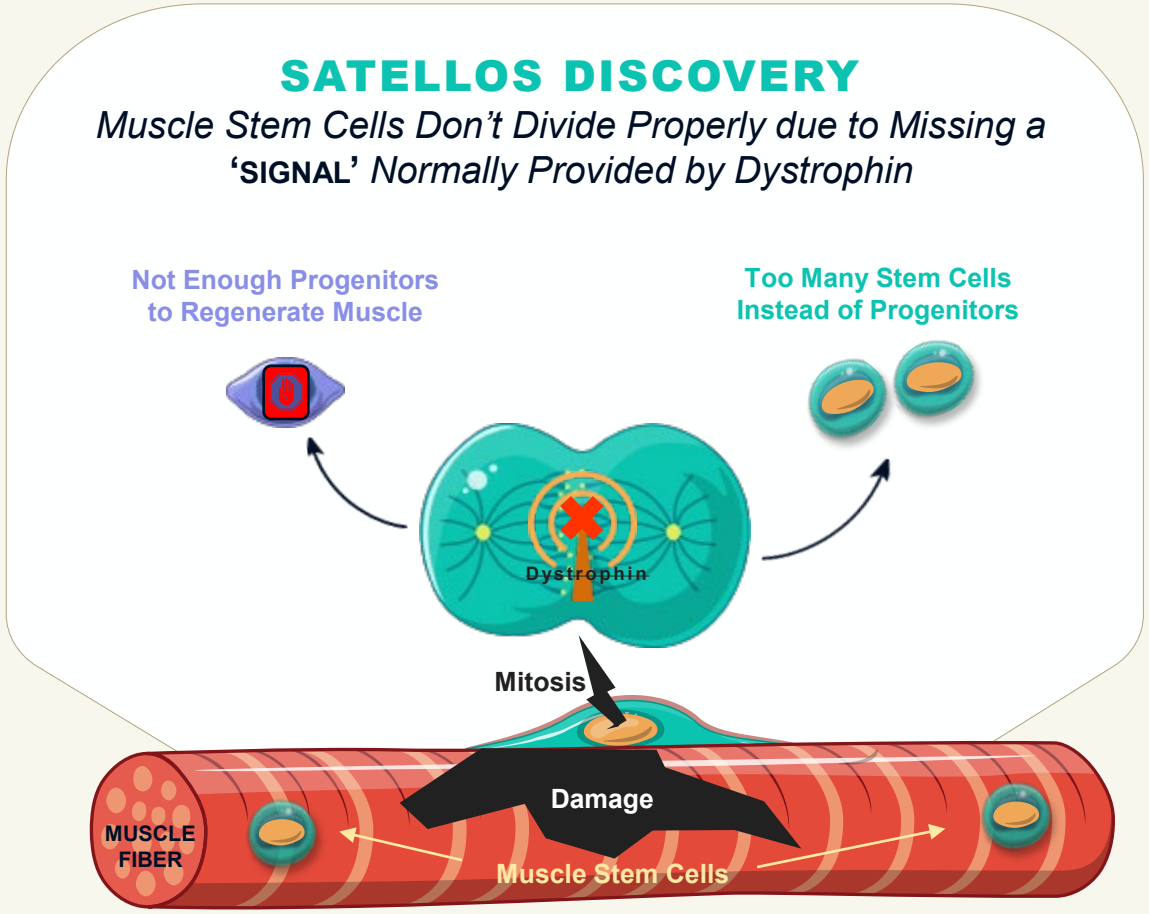
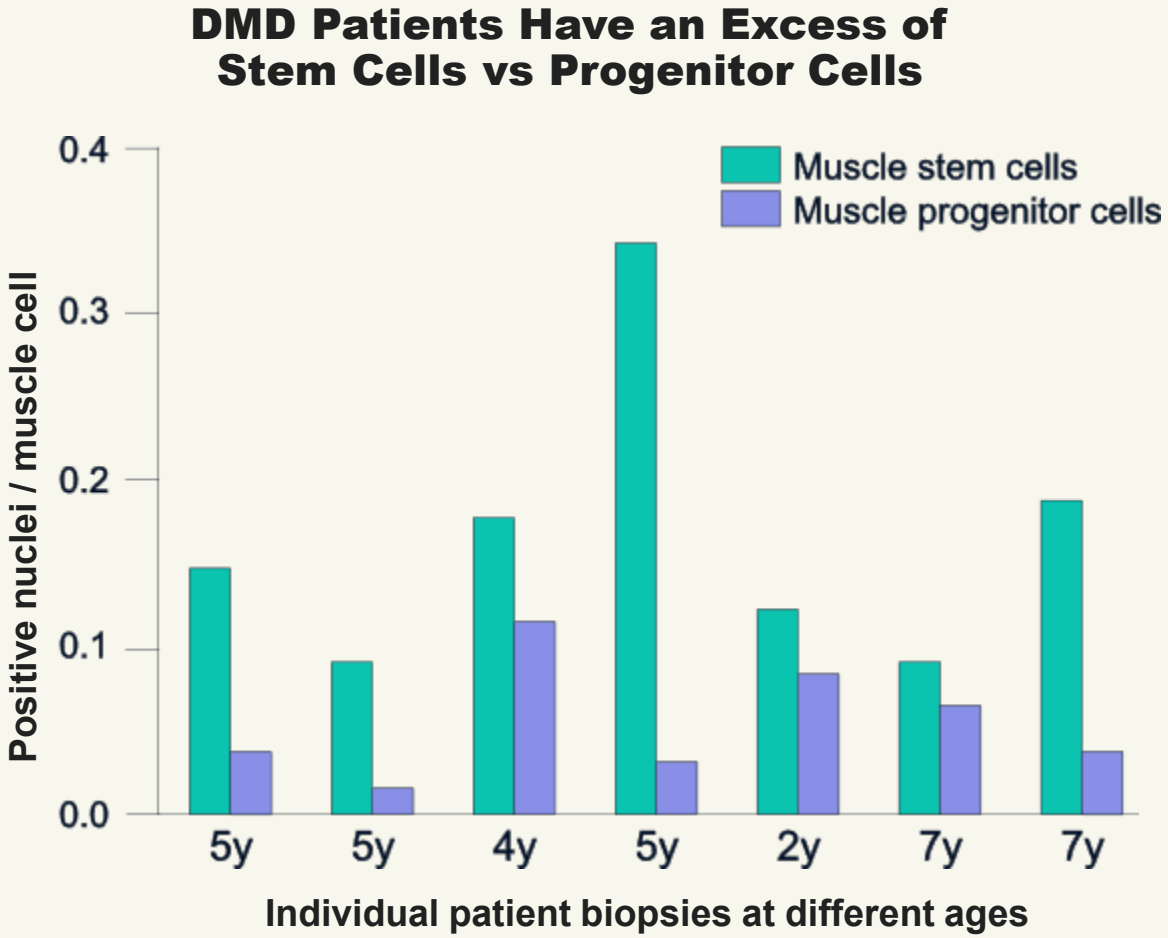
In DMD, Regeneration Fails as Muscle Stem Cells Don't Function as Intended



Nicolas A. Dumont et al., Nature Medicine. 2015



Michael Kottlors et al., Cell Tissue Research. 2010

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Satellos is Developing SAT-3247 to Address This Problem

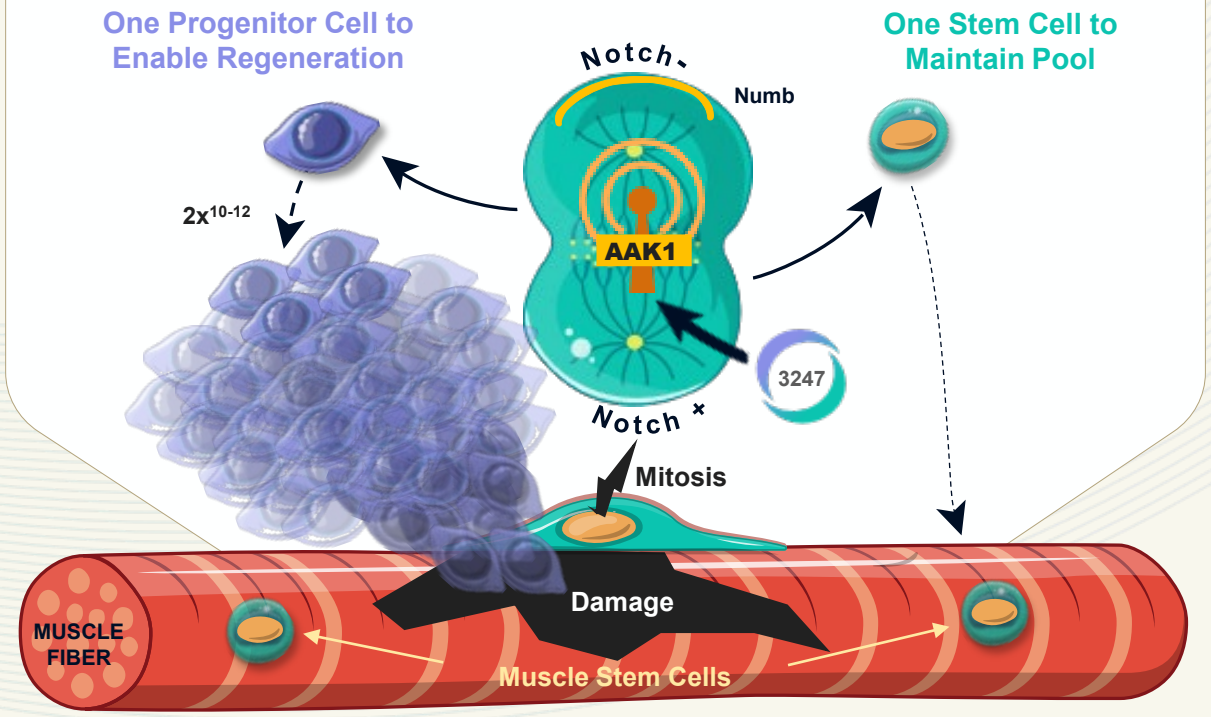
SAT-3247 has been designed as an oral pill intended to restore regeneration in DMD



SAT-3247 is currently in a Ph 1 clinical trial in AUS and not approved for use in USA or elsewhere.

RESTORING MUSCLE REGENERATION with SAT-3247

SAT-3247 inhibits AAK1 to replace the missing dystrophin **'SIGNAL'** *and reset proper muscle stem cell divisions*



1. Notch signaling is a conserved pathway that regulates cell proliferation, cell fate, and differentiation.
2. Inhibiting AAK1 establishes a notch gradient across the satellite cell, via Numb inhibition at the apical pole, restoring balanced stem cell division

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SAT-3247 is Totally Different: Not Just Stabilizing — Regenerating

Genetic approaches

- Administered by infusion or injection
- Gene therapy (DNA) or exon skipping (RNA)
- Aims to stabilize muscle fibers to slow disease progression
- Limited by exon status or potential immunogenicity concerns

WAVE LIFE SCIENCES
AVIDITY BIOSCIENCES
Dyne THERAPEUTICS
SAREPTA THERAPEUTICS
SOLID BIOSCIENCES

SAT-3247 Approach

- Oral, once-daily pill
- Designed to be safe and tolerable
- Aims to regenerate muscle to reverse disease progression
- Intended for all patients regardless of exon or immune status
- Potential as stand-alone or add-on therapy
- ***Increased strength or function expected as early sign of treatment effect***

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Preclinical Proof of Concept with SAT-3247

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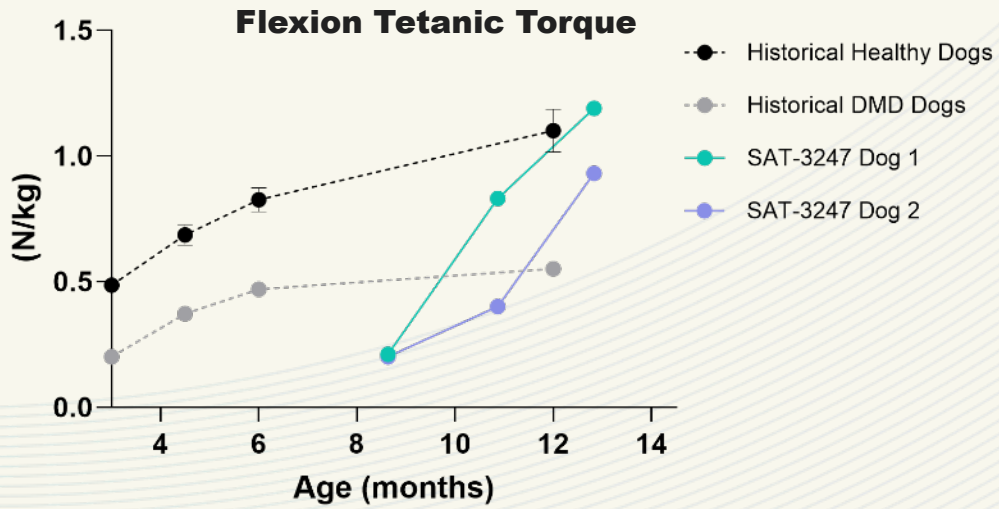
SAT-3247 Restores Muscle Strength to Normative Levels in Canine Model of DMD

Satellos Preclinical Study



- 4-month study
- 2 dogs: ~9 months of age to ~13 months
- Retriever cross with natural DMD mutation
- Oral daily dosing with **SAT-3247** (4x/week)
- Multiple serum measurements throughout
- Histology and strength at 2 and 4 months

SAT-3247 treated vs. natural history



Contraction force generated by tarsal joint flexion and extension in dogs with golden retriever muscular dystrophy

Joe N. Kornegay[a,b,*] Daniel J. Bogan[a,b], Janet R. Bogan[a,b], Martin K. Childers[b,c], Diane D. Cundiff[a,b], Gregory F. Petroski[d], Ronald O. Schueler[a,b]

Rapid, robust effect on strength offers promise for functional benefit and early signal in clinical trials

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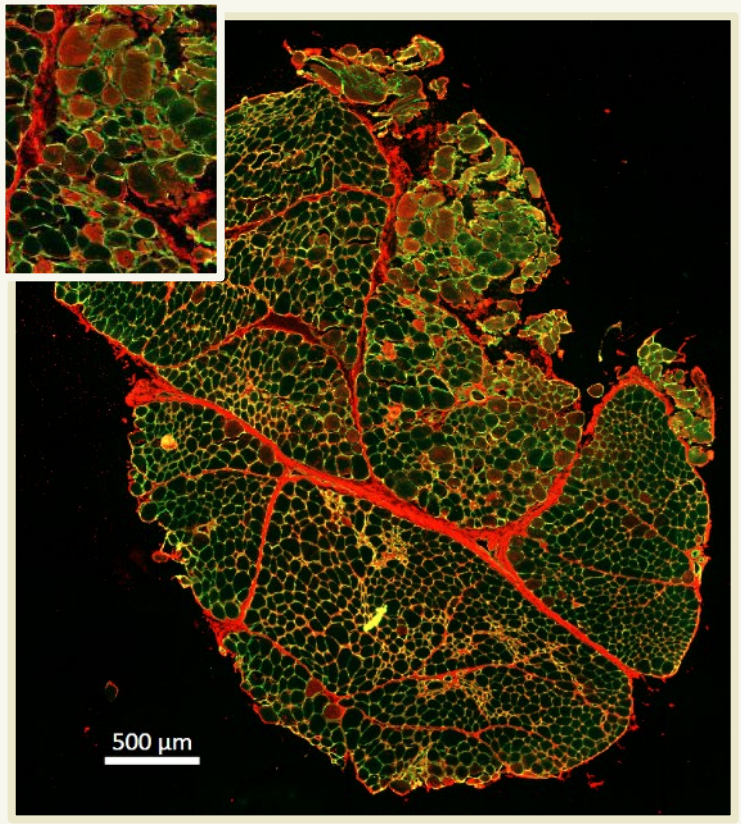
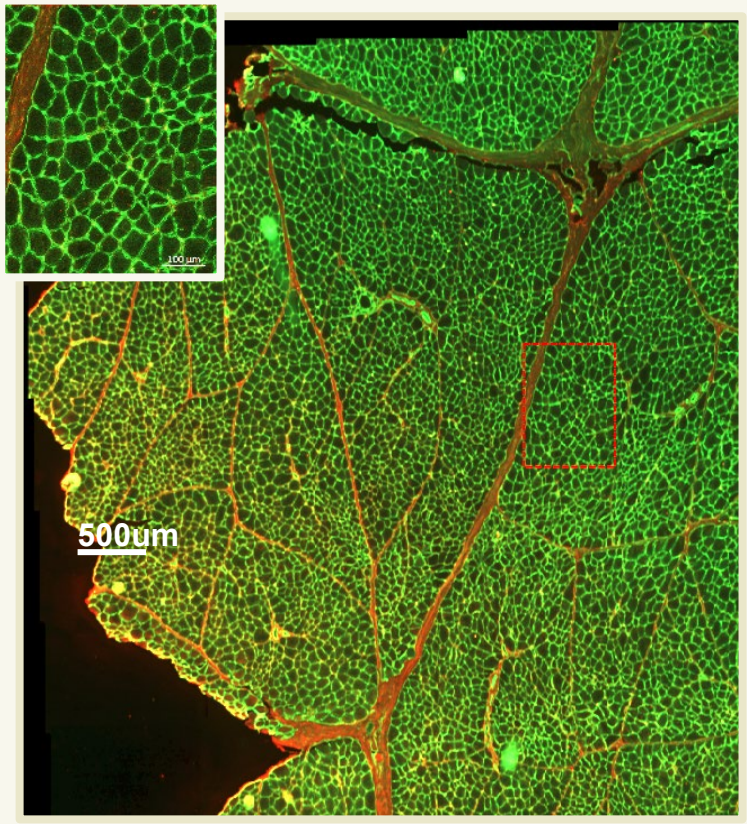
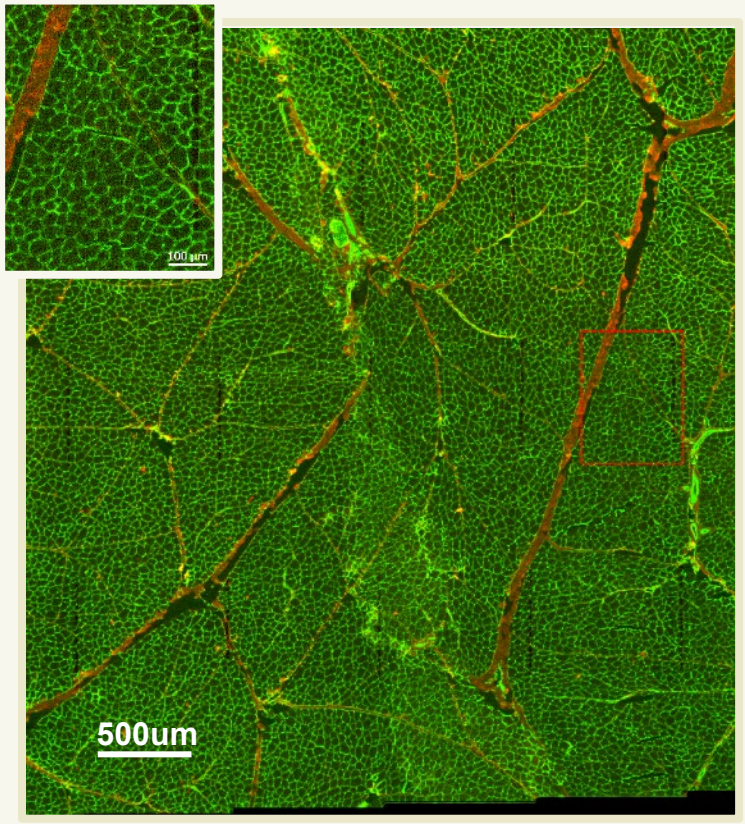
SAT-3247 Treatment Shows Evidence of Muscle Repair and Regeneration



SAT-3247 Study
DMD Animal / Pre-Treatment
Bicep Femoris



SAT-3247 Study
DMD Animal / Post-Treatment
Bicep Femoris



Healthy Age-Matched
Non-DMD Comparator
Bicep Femoris

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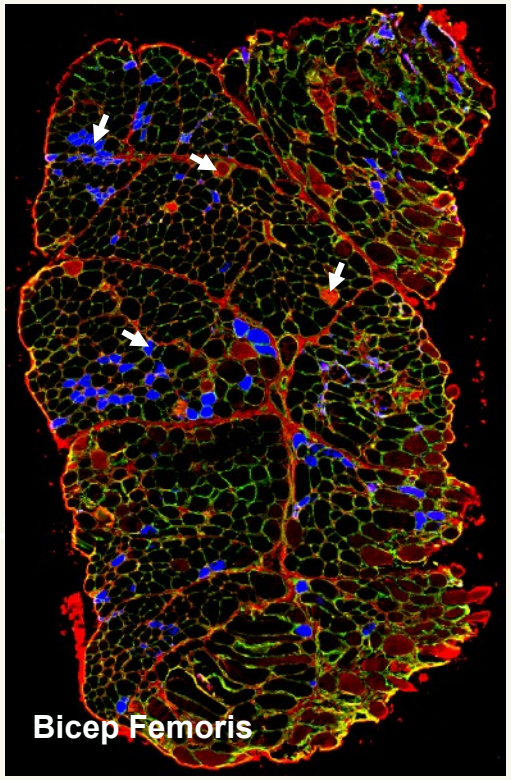
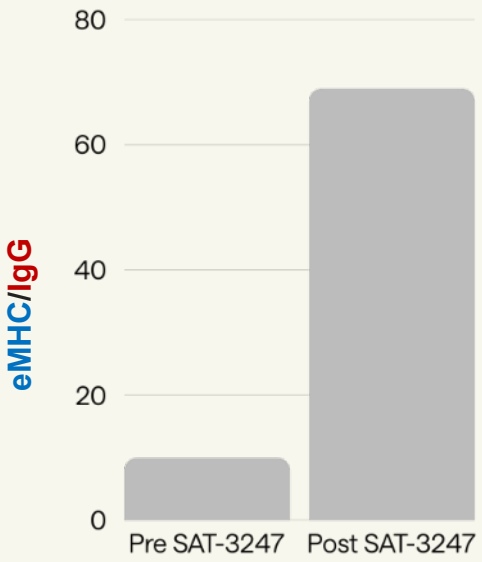
Regenerative Index and Inflammatory Markers Offer Potential for Use in Clinical Development

Regenerative Index (RI)

(from tissue biopsy)



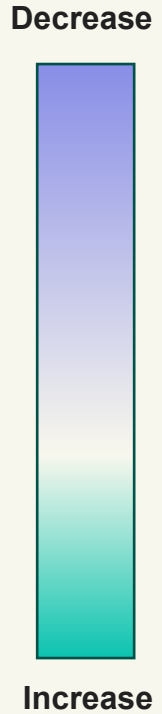


$$RI = \frac{\text{eMHC (forming fibers)}}{\text{IgG (dying fibers)}}$$

Inflammatory Markers

(from serum)

Cytokine	% Change
GM-CSF	-73
IL-7	-70
IL-18	-62
IL-2	-56
IL-6	-53
IL-15	-38
KC-Like	-36
MCP-1 (CCL2)	-24
IP-10	-13
IL-8	-0.3
IL-10	702
IFN-g	1229



eMHC = embryonic myosin heavy chain | IgG = immunoglobulin G

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Advancing SAT-3247 into Clinical Development

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SAT-3247 Phase 1 First-in-Human Clinical Trial

Ph 1a: Healthy Volunteers (n=72)			Ph 1b: DMD Adults
SAD* Study	MAD* Study	Food Effect Study	28-day, Open-Label Study
▪ 10 - 400 mg ▪ 5 cohorts of 8 ▪ Single dose / 1 day	▪ 60 - 240 mg ▪ 4 cohorts of 8 ▪ Single dose / 7 days	▪ 150 mg ▪ 1 cohort of 8 ▪ Single dose / 1 day	▪ 60 mg daily dose ▪ 1 cohort of up to 10 ▪ Single dose, weekdays only, for 4 weeks
Complete			Complete

Endpoints

Primary (Ph 1a & 1b): Safety & tolerability [labs, vitals, ECG, physical exam]

Secondary (Ph 1a and 1b): Pharmacokinetic exposure (PK)

Exploratory (Ph 1b only): Grip/pinch strength, upper body effort, lung function (FVC), serum markers

**SAD: single ascending dose; *MAD: multiple ascending dose.*

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SAT-3247 Appears Safe and Tolerable in Phase 1a

Single Ascending Dose[1]

No clinically significant findings observed in 10, 50, 150, 300 and 400 mg doses.

- ☑ Labs
- ☑ Vitals
- ☑ Physical Exam
- ☑ ECG

Drug-related adverse events, reversible:

- **400 mg:** mild nausea (1); mild abdominal pain (1)

Multiple Ascending Dose[1]

No clinically significant findings observed in 60, 120, 180 and 240 mg doses:

- ☑ Labs (mild ALT/AST elevations at 180 and 240 mg)[2]
- ☑ Vitals
- ☑ Physical Exam
- ☑ ECG

Drug-related adverse events, reversible:

- **120 mg:** mild lightheadedness (1)
- **180 mg:** intermittent mild epigastric pain (1); mild sleepiness (1); mild lethargy (1)

1. Based on 20 Feb 2025 data cut-off; analysis ongoing
2. Dose levels above expected therapeutic doses; no evidence of liver toxicity

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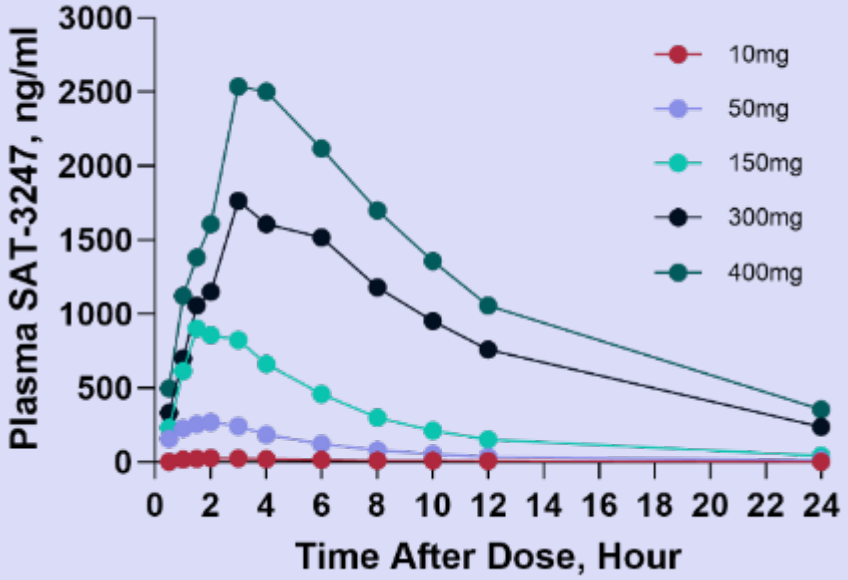
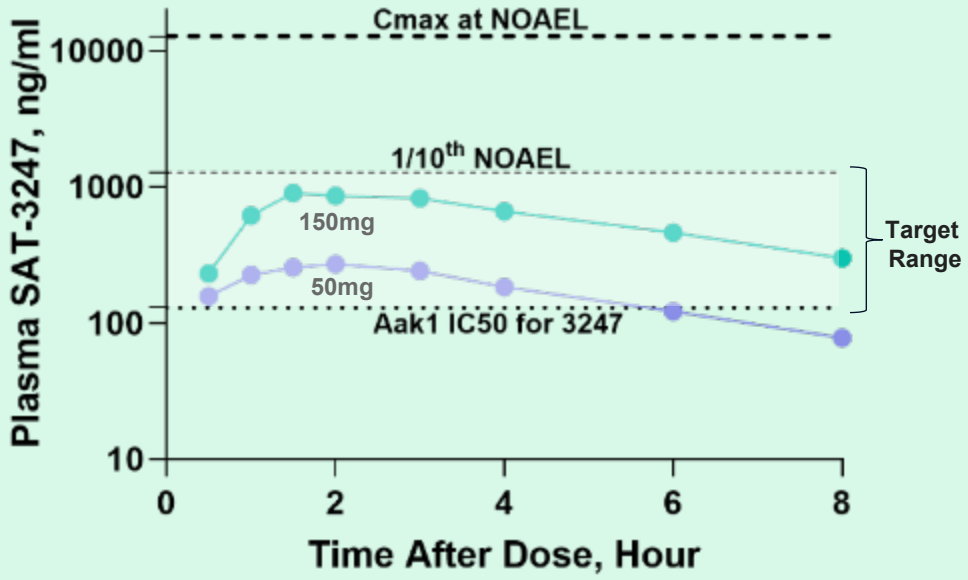
SAT-3247 Achieved Desired Profile Determined by Satellos

SAT-3247 Exposure (PK) Achieved



SAT-3247 Target Dose Achieved



Exposure (PK) Targets:

- ✓ Dose proportional exposure levels
- ✓ Drug clearance within 24 hours

Dose Targets:

- ✓ Exposure ≥IC50 for 6-8 hours
- ✓ Peak exposure 1/10th NOAEL or less

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SAT-3247 Treatment Displayed Encouraging Results and Possible Early Functional Signal in Phase 1b

Study Overview

- Safety and PK plus exploratory measures assessed
- 5 patients (ages 20-27), all on steroids
- 28-day treatment (weekdays only for 4 weeks)
- SAT-3247 dose: 60 mg daily
- **Primary endpoints met:**
  - ✓ SAT-3247 was safe and well tolerated
  - ✓ PK unaffected by steroid use

Exploratory Measures: Data Highlights

- **Grip strength** (days 1 and 28):
  - Avg. ⬆~2kg (108%)
  - 3 patients showed increases in both hands, 2 stable
- **Pinch strength**: Stable, no clear trends
- **Lung function (FVC)** (days 1 and 28):
  - 4 patients ⬆~5%
  - 1 patient unable to complete (non-drug related)
- **Serum markers** (days 1 and 15):
  - Baseline levels stable, analysis ongoing
- **Upper body effort** (wrist monitor):
  - No trends yet, analysis ongoing

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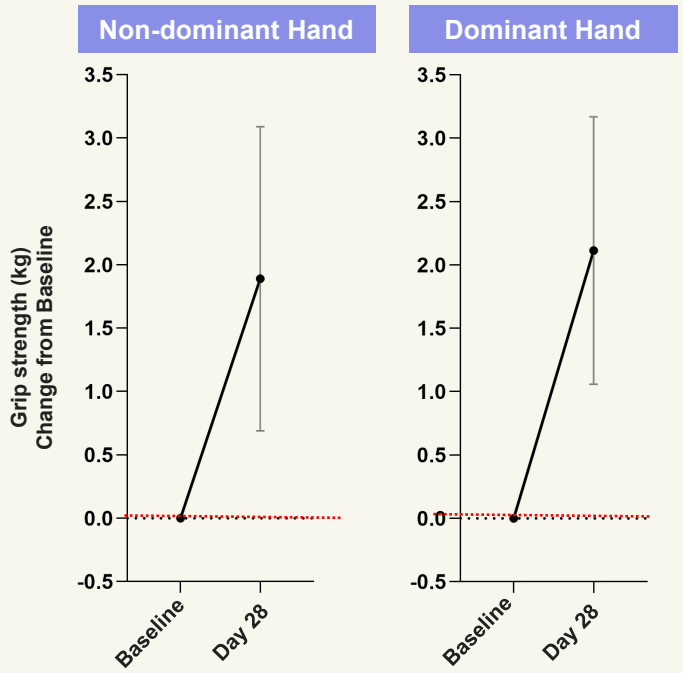
Comparison of Force Increase in Grip Strength

Satellos vs Competitor

Satellos Data

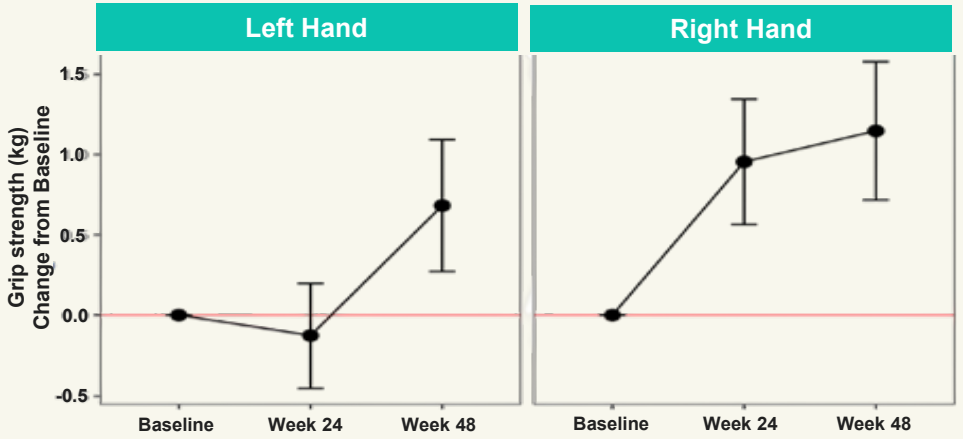
SAT-3247

- 5 patients: **20-27 years old**
- **28 days** of treatment
- ***Average increase in force***
  - **+ ~ 2 kg or** + 108%
  - 1.9 kg (baseline) to 3.9 kg (day 28)
- **3 of 5** patients showed increased grip strength in both hands
- **2 of 5** patients were stable



Competitor Data
(exon skipping)

- 11 patients: **~ 8 years old**
- **48 weeks** of treatment
- **+ ~ 1 kg** avg. increase in force



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Inside Satellos

Our company and the team leading it forward

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Overview of Ongoing and Planned Clinical Trials

2025				2026			
Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4

Ongoing Phase 1 Program (Underway: Australia)

Ph 1a
72 HVs
SAD/MAD/FE

Presented at MDA/2025

Ph 1b
Up to 10 Adult DMD Patients
Safety + PK | 28 day

Topline Data Reported at CureDuchenne/2025

Ph 1b
Long-Term Follow-up | 11 Months of Dosing
Safety | Pharmacodynamics

Planned DMD Phase 2 Program (Intended: USA, Canada, AUS, EU)

Global Reg. Filings

Ph 2 | Pediatric DMD patients | N=51
Randomized, Placebo Controlled Studies | 3 months of dosing | 2 dose groups
Endpoints: Safety, Muscle Function, Muscle Imaging, Serum Biomarkers & Biopsy (RI)

Ph 2 | Long-Term Follow-up
Up to 9 Months of Dosing, Cross Over
Safety, Muscle Function, Muscle Imaging, Serum Biomarkers & Biopsy (RI)

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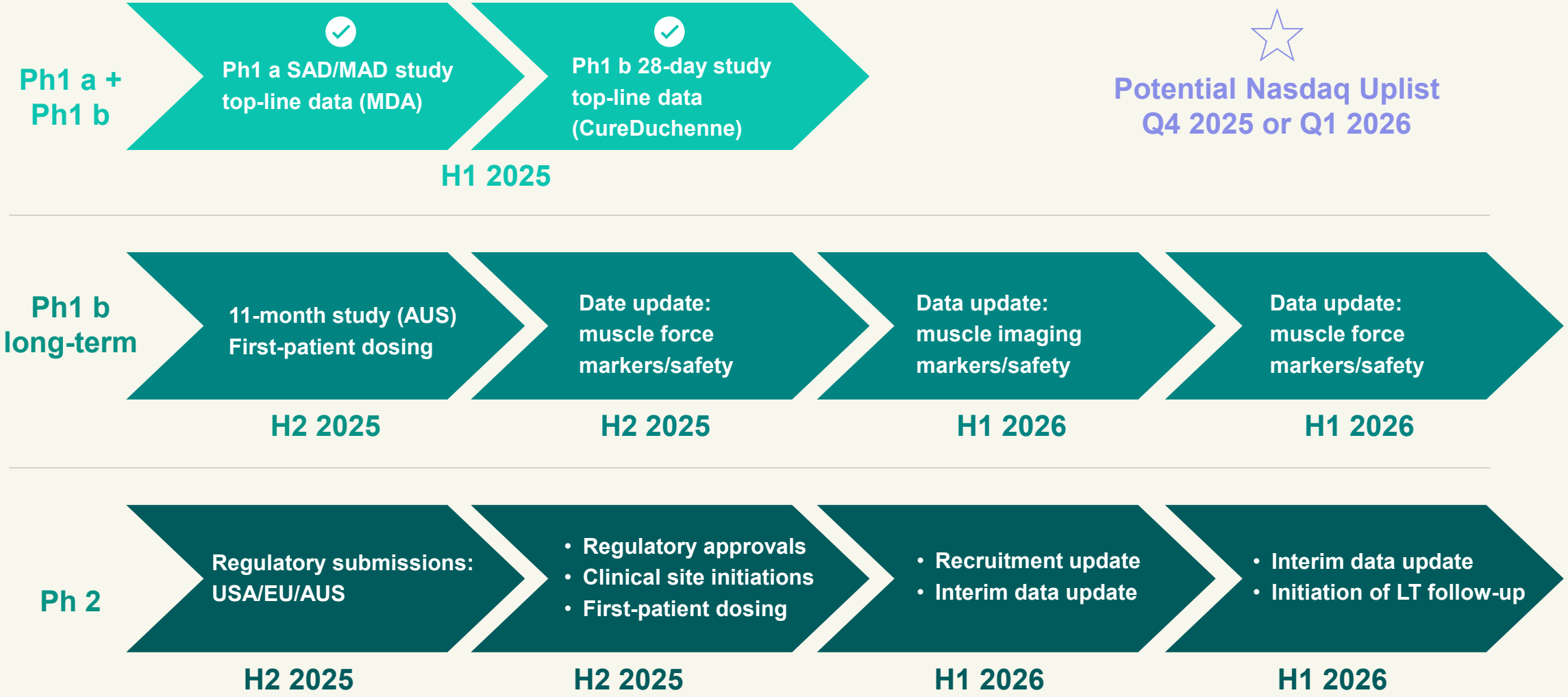
SAT-3247: Anticipated Program Milestones



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Reimagine

how muscle degeneration is treated.

Regenerate

with small molecule medicines.

Realize

the next horizon to improve lives.