CureDuchenne FUTURES National Conference

Presenter: Frank Gleeson, Co-founder & CEO

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satellos

REGENERATING MUSCLE FROM WITHIN™

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Did you know?

In Duchenne, muscles are damaged faster than the body can repair and regenerate them as intended.

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About Satellos

Grounded in science. Inspired by need.

Our mission is to restore muscle in Duchenne muscular dystrophy and other devastating muscle diseases.

A New Way to Think About Treatment

- We're focused on helping muscle heal itself

How Our Approach Is Different

- It is designed to improve muscle strength and function in Duchenne
- It works by resetting the body's in-built cycle of repair and regeneration
- It's a simple oral medication — no needles or infusions

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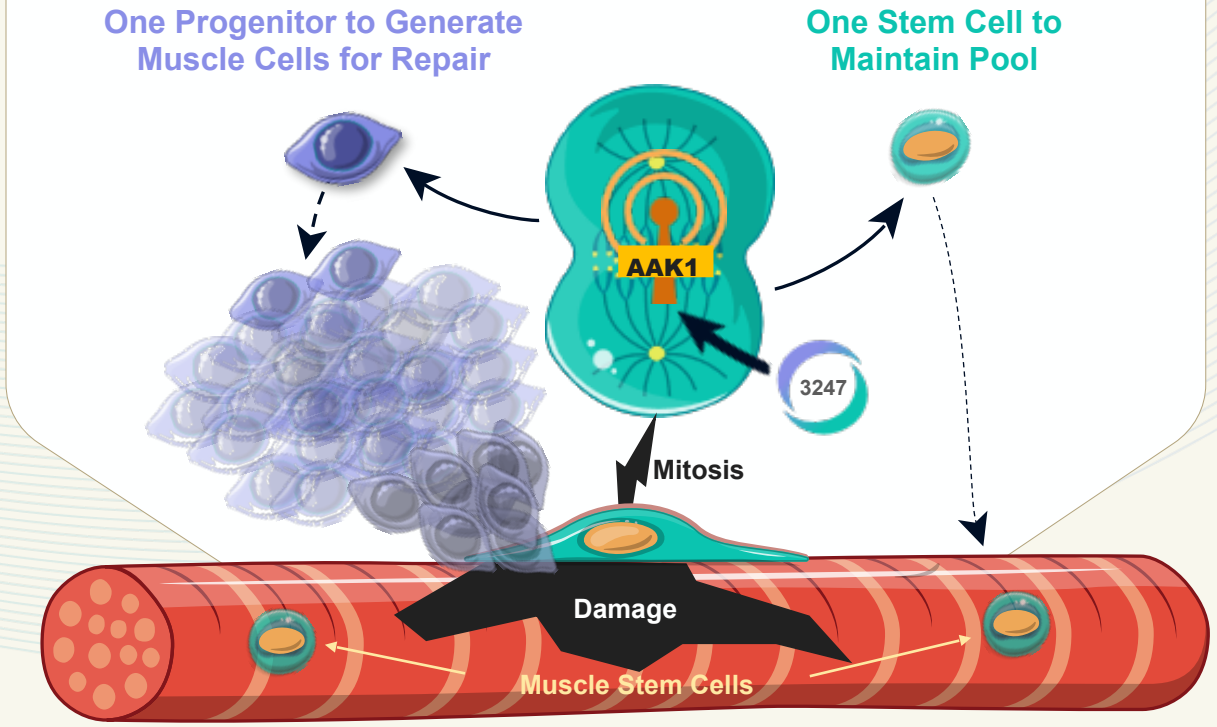
What We're Working On

At Satellos, we're developing SAT-3247, a daily pill intended to restore repair and regeneration.



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

SAT-3247 Designed to Inhibit AAK1 and Replace a Missing Signal in Duchenne



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Phase 1 Study Overview

Phase 1a Summary

We tested SAT-3247 in 72 healthy people — from low to higher doses. Reported at MDA in March 2025.



Bottom Line:
SAT-3247 appears safe and well tolerated — even at higher doses.

Phase 1b Study Design

- Open-label study:
  - Up to 10 patients over 18 years of age
  - Genetically confirmed with DMD
- Dosing:
  - 60/mg per day as oral tablets
  - Weekdays for 28 days (i.e., 20 doses)

Primary Endpoints	Secondary Endpoints
▪ Safety and tolerability	▪ Pharmacokinetics
Exploratory Measures	
▪ Grip and Pinch Strength ▪ 99th maximum effort	▪ % predicted FVC ▪ Serum markers

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SAT-3247 Displayed Encouraging Results in Phase 1b Study

Overview

- Open-label study
- Partnered with MDA Australia for recruitment
- 5 patients enrolled
- **5 patients completed**
- Age range 20 - 27
- All patients remained on daily steroids
- We are grateful to the patients and caregivers

Thank you!

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Data highlights

- ☑ Safe and well tolerated
- ☑ **SAT-3247** met desired PK profile regardless of concomitant steroid
- ☑ Potential trend to improved grip strength
  - ☑ **Average strength doubled** from ~2kg to ~4kg
- ☑ Patients appeared stable on remaining measurements

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Next steps in Ph 1

- 11-month follow-up study has received ethics approval (AUS)
  - Up to 10 DMD adults that completed the phase 1b study
- 1st dosing expected in Q3 2025, pending qualification of the clinical site
- Will continue to measure grip strength and additional dynamometry measures to:
  - determine whether improvement trend continues
  - determine usefulness of these efficacy measures for future trials

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SAT-3247 Phase 2 Proposed Study Design

Design

- Global, randomized, placebo-controlled, two-dose comparison
- 3 months of treatment in pediatric DMD patients

Objectives

- Safety and tolerability
- Determine **SAT-3247** proof of concept by exploring effects on:
  - muscle and serum biomarkers
  - muscle strength and function

Current Status

- Finalizing standard preclinical studies required to initiate Ph 2 treatment in children
- Multiple clinical sites being selected in USA and internationally
- Study design has not yet been submitted to or approved by any health authorities

Next Steps

- Country regulatory submissions including USA planned for Q3, 2025
- Initiation of Ph 2 following regulatory and ethics committee approvals

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Reimagine

how muscle degeneration is treated.

Regenerate

with small molecule medicines.

Realize

the next horizon to improve lives.