Assessing the Impact of SAT-3247 on Muscle Force in a Mouse Model of FSHD

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CSO

satellos

REGENERATING MUSCLE FROM WITHIN™

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SAT-3247: Making Regeneration Happen

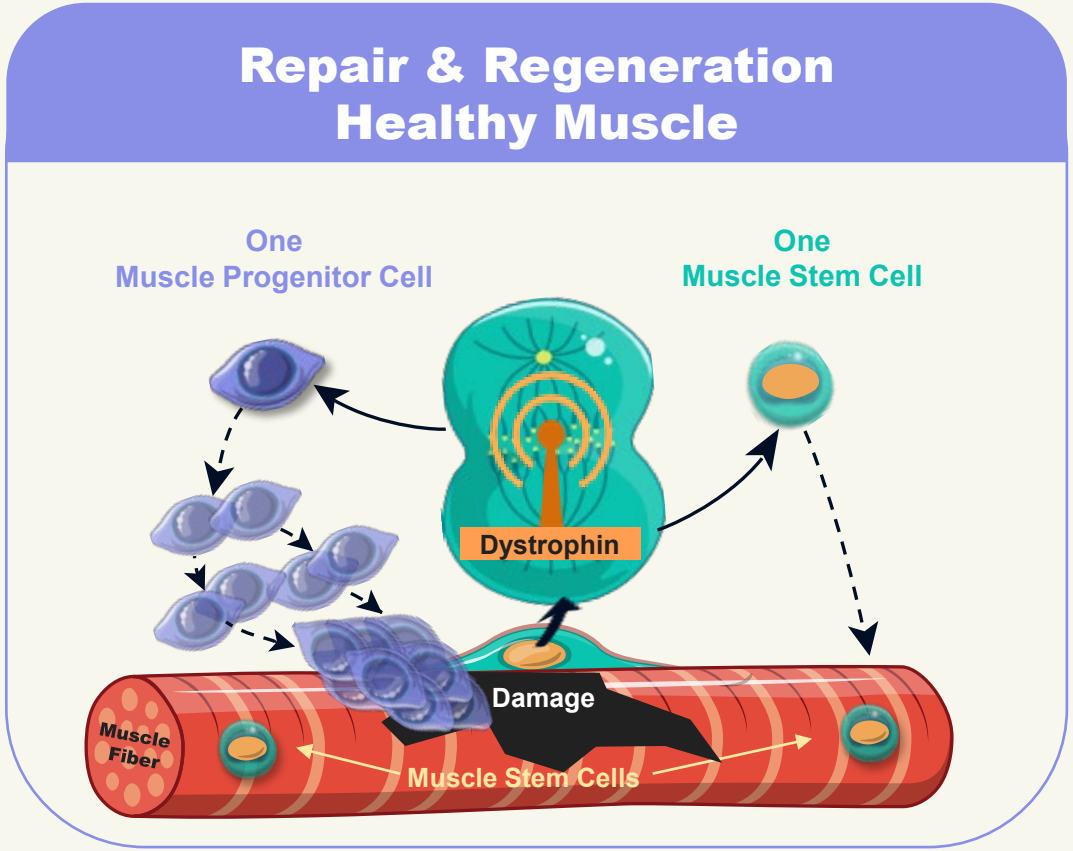
Why SAT-3247 is different

- Oral, small molecule, **disease modifying** therapy designed for **safety & tolerability**
- Harnessing the body's natural biology to **repair & regenerate muscle in response to injury**
- Potential applicability in multiple indications **beyond DMD**
- FSHD has **significant unmet medical need**
- Preclinical data supports that **impaired regeneration in FSHD** may be ameliorated by SAT-3247 leading to **an improvement in muscle function**
- Satellos plans to **extend its clinical program** with SAT-3247 into FSHD in 2026

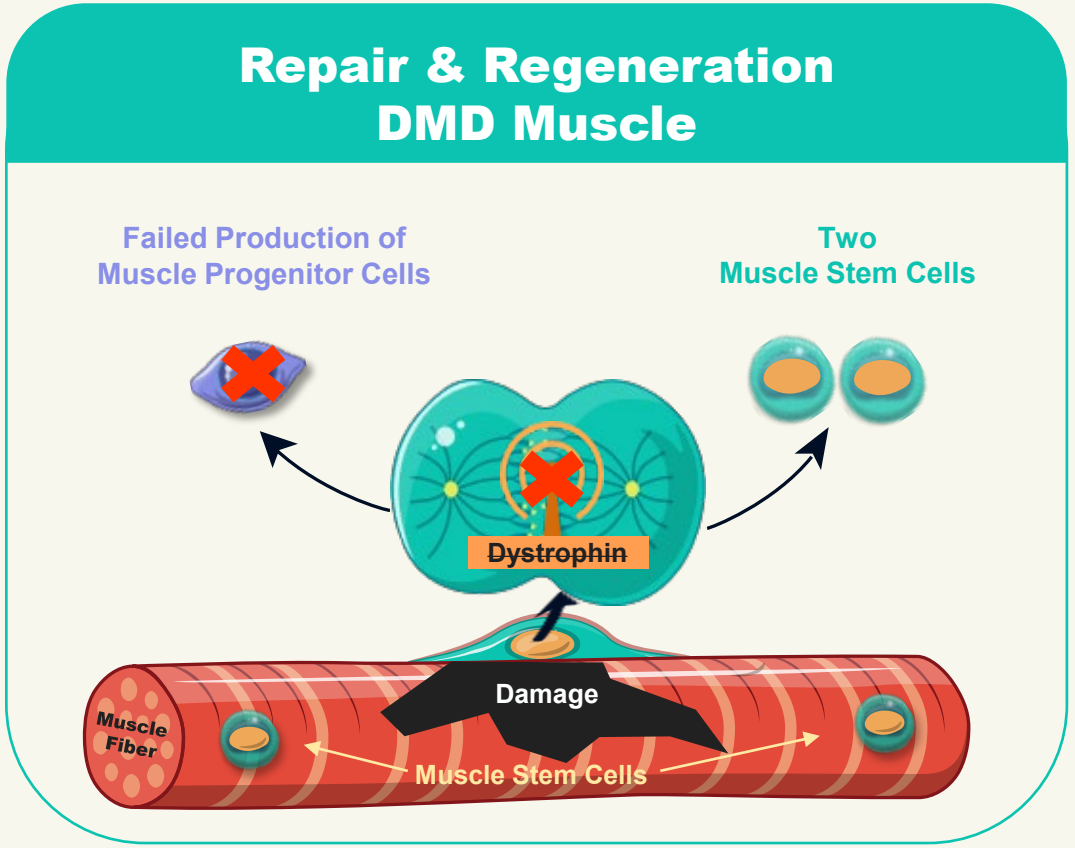
SAT-3247 Increases Asymmetric Division Improving Muscle Regeneration

Satellos Discovery: Why is regeneration impaired in Duchenne

Loss of dystrophin disrupts production of progenitors



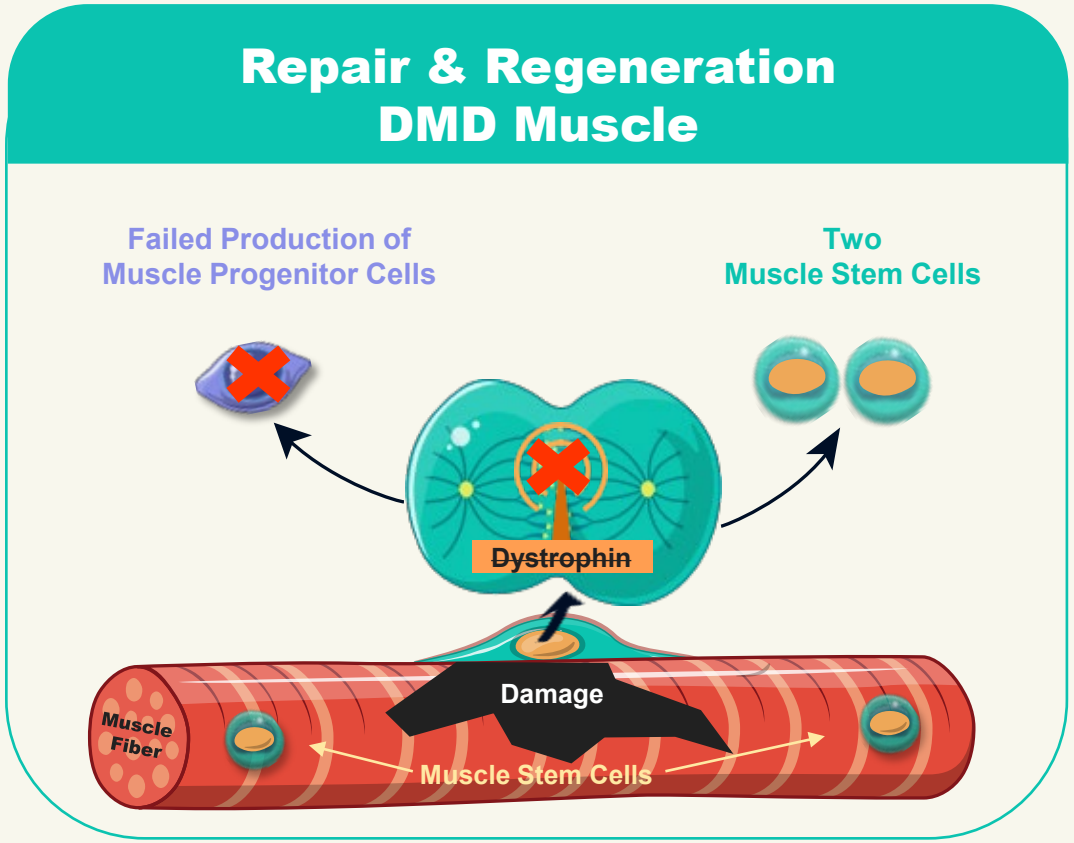
Asymmetric division with Dystrophin Signal
1 progenitor + 1 stem cell



Symmetric division without Dystrophin Signal
0 progenitors + 2 stem cells

Satellos Discovery: Progenitor Formation & Regeneration Can Be Restored

Inhibition of AAK1 with SAT-3247 Replaces Missing Dystrophin Signal



Symmetric division without Dystrophin Signal
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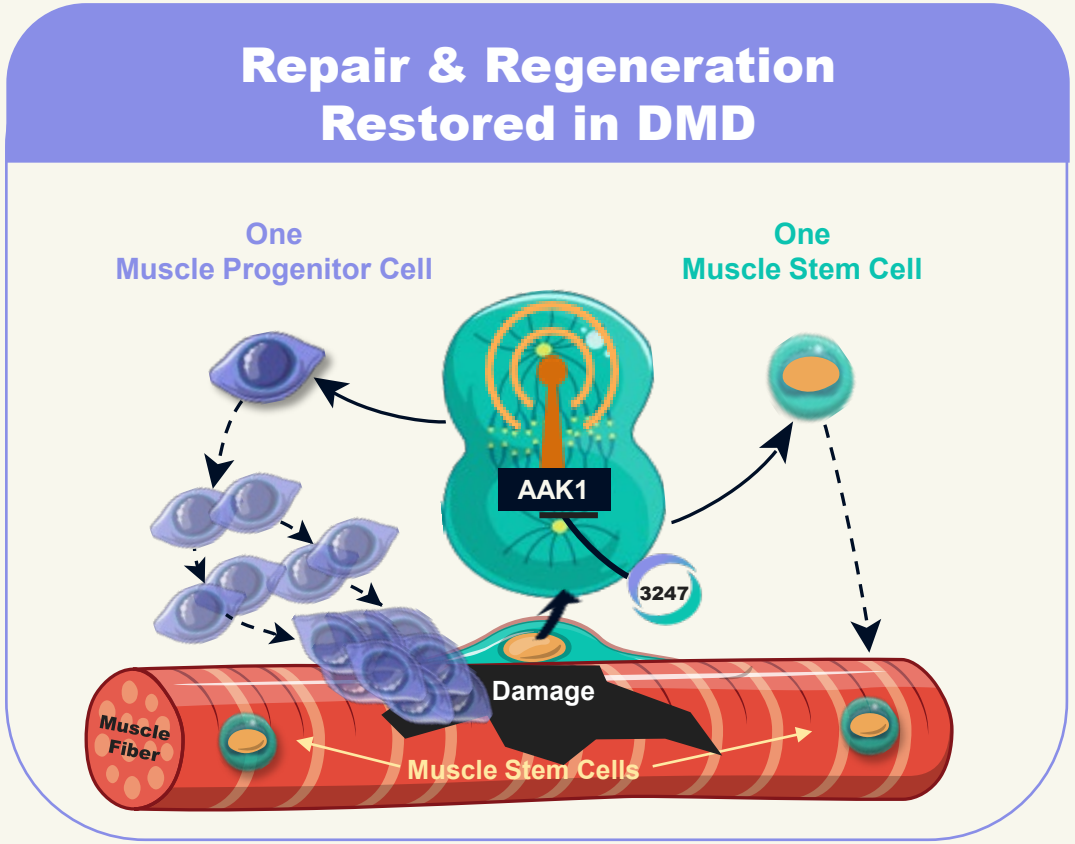
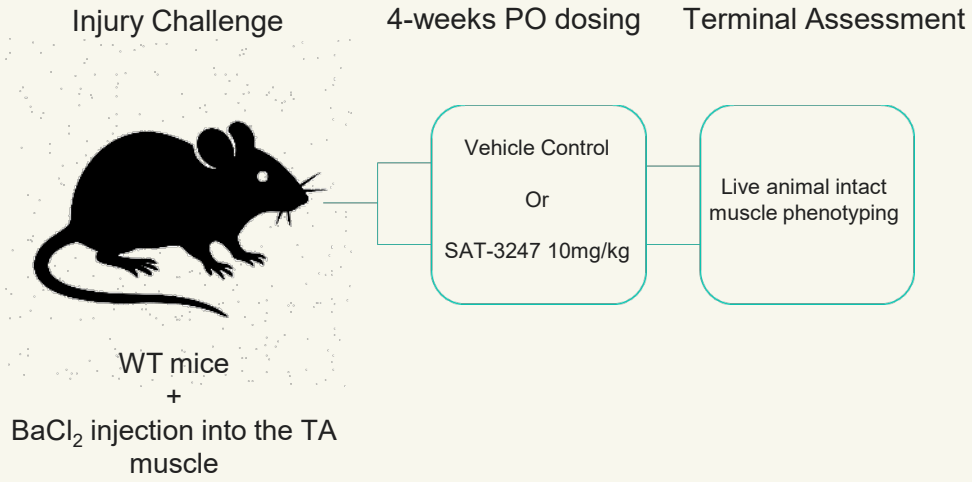
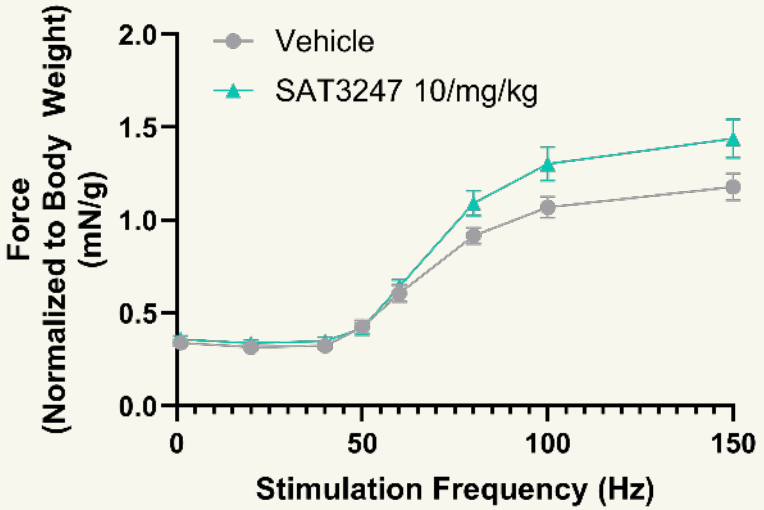


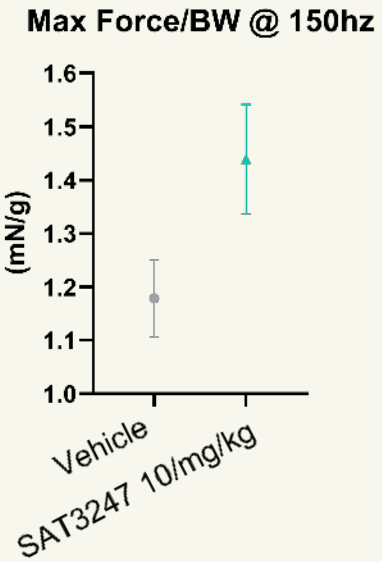
Asymmetric division with AAK1 inhibition
1 progenitor + 1 stem cell

Satellos Discovery: Healthy regeneration can be enhanced

Inhibition of AAK1 with SAT-3247 improves repair in non-diseased mice







- **Improvement with SAT-3247** beyond the natural level of repair – room to improve
- **Supports potential use of SAT-3247** in indications with impaired regeneration

SAT-3247 Works Independently of Dystrophin

Potential effectiveness in multiple severe muscle diseases

FSHD Has Significant Unmet Medical Need

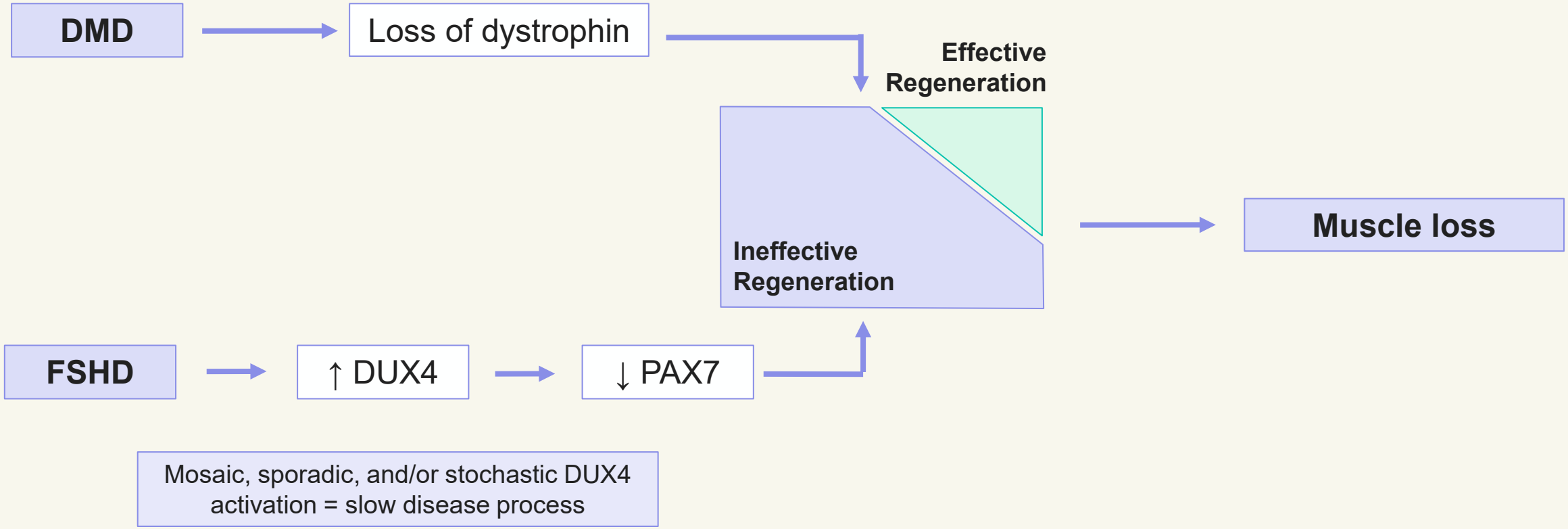
- FSHD is a genetically complex, progressive muscular dystrophy with variable severity and age of onset[1]
- One of the most common muscular dystrophies
  - 1 in 8000 to 1 in 20,000, depending on population
  - Affects >800,000 individuals, globally[2]

DMD	FSHD	Potential Opportunity for SAT-3247
✓ Impaired muscle regeneration ✓ Progressive loss of muscle strength and function ✓ Approved therapies but significant unmet need	✓ Impaired muscle regeneration ✓ Progressive loss of muscle strength and function ✗ No approved therapies	▪ Disease modifying potential as a stand-alone therapy or in combination ▪ Agnostic to genetics (FSHD1 vs FSHD2)

1. Preston MK, Wang LH. Facioscapulohumeral Muscular Dystrophy. 1999 Mar 8 [Updated 2025 Jul 10]. In: Adam MP, Feldman J, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2025. Available from: https://www.ncbi.nlm.nih.gov/books/NBK1443/
2. https://www.fshdsociety.org/living-with-fshd/understanding-fshd/

Why Use SAT-3247 in FSHD?

Two diseases, different triggers, same outcome: muscle loss



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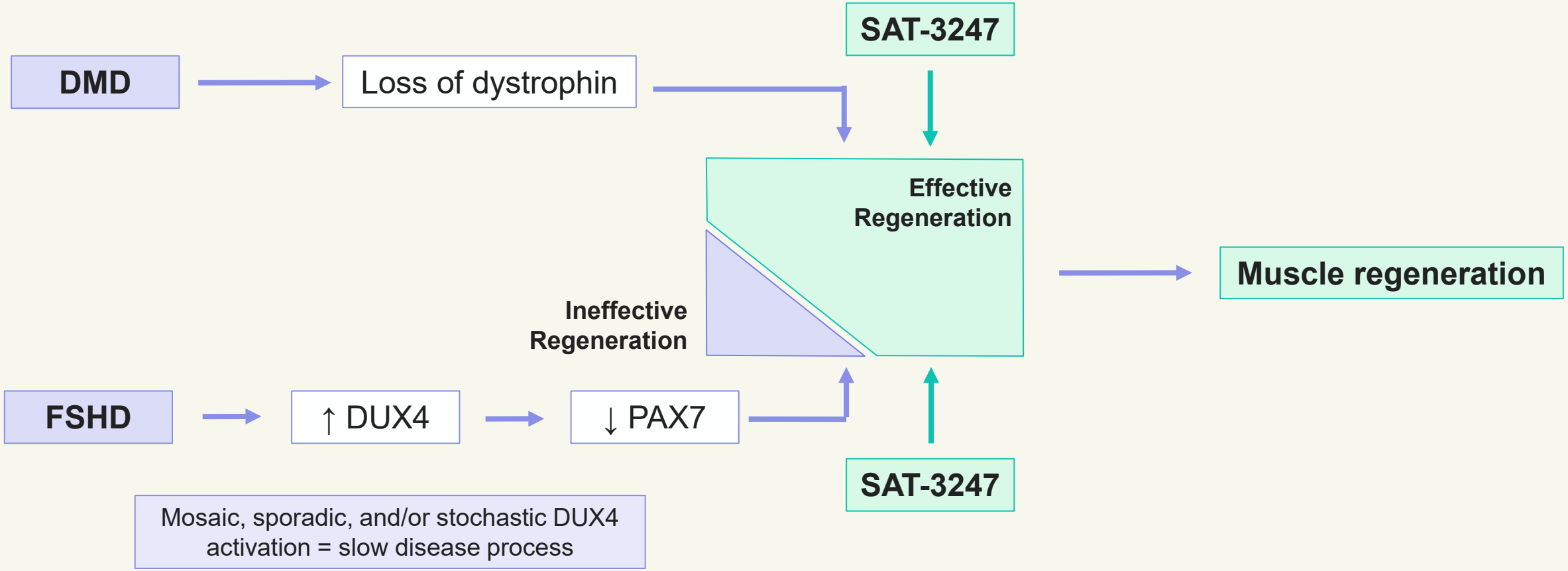


SAT-3247 enhances muscle strength in FLExDUX4 FSHD model

FLExDUX4 FSHD Model

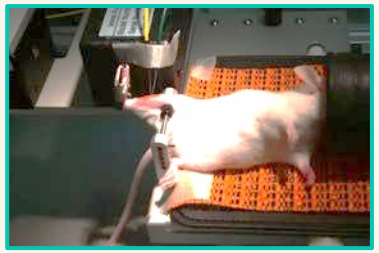
"leaky" DUX4 induction model

12-weeks PO dosing SAT-3247

1. WT Cre
2. FLExDUX4
3. 3mg/kg
4. 10mg/kg
5. 30mg/kg

Functional Assessment

Live animal in-tact muscle phenotyping



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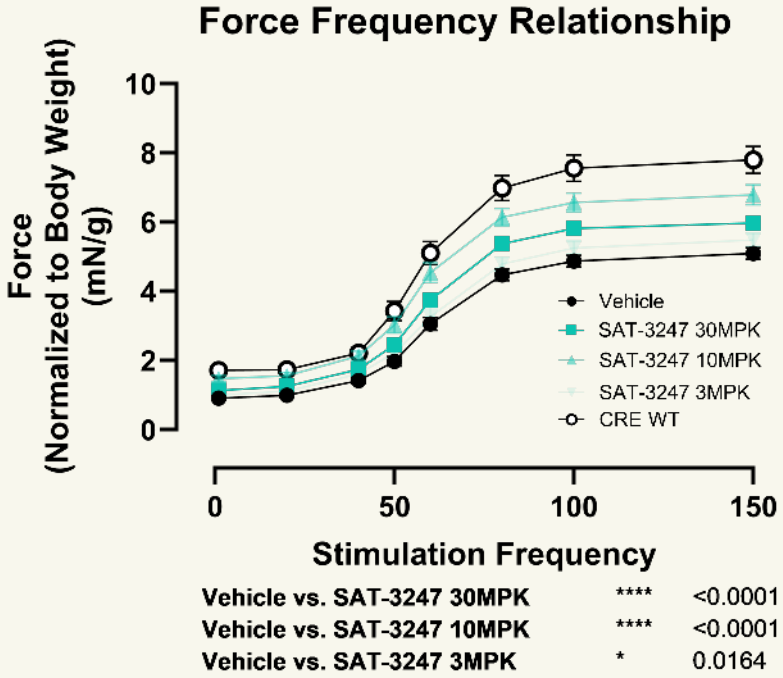

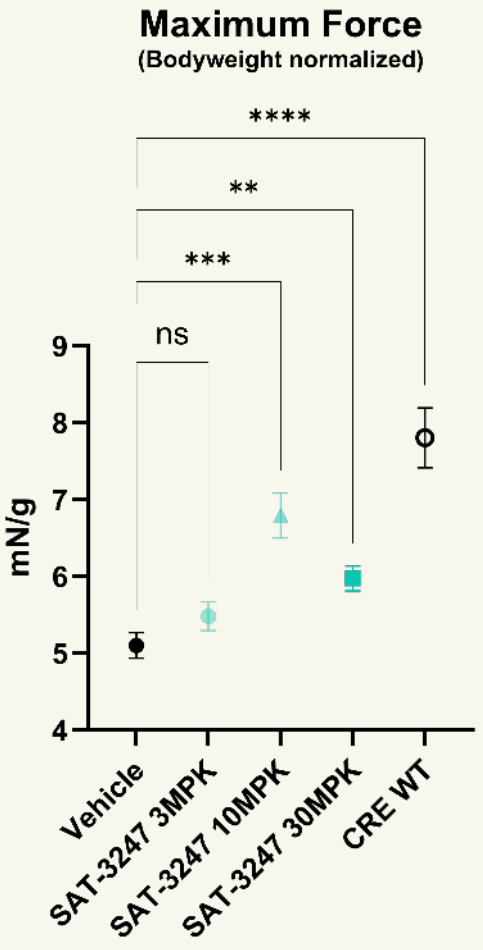

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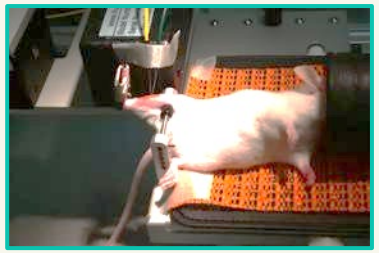

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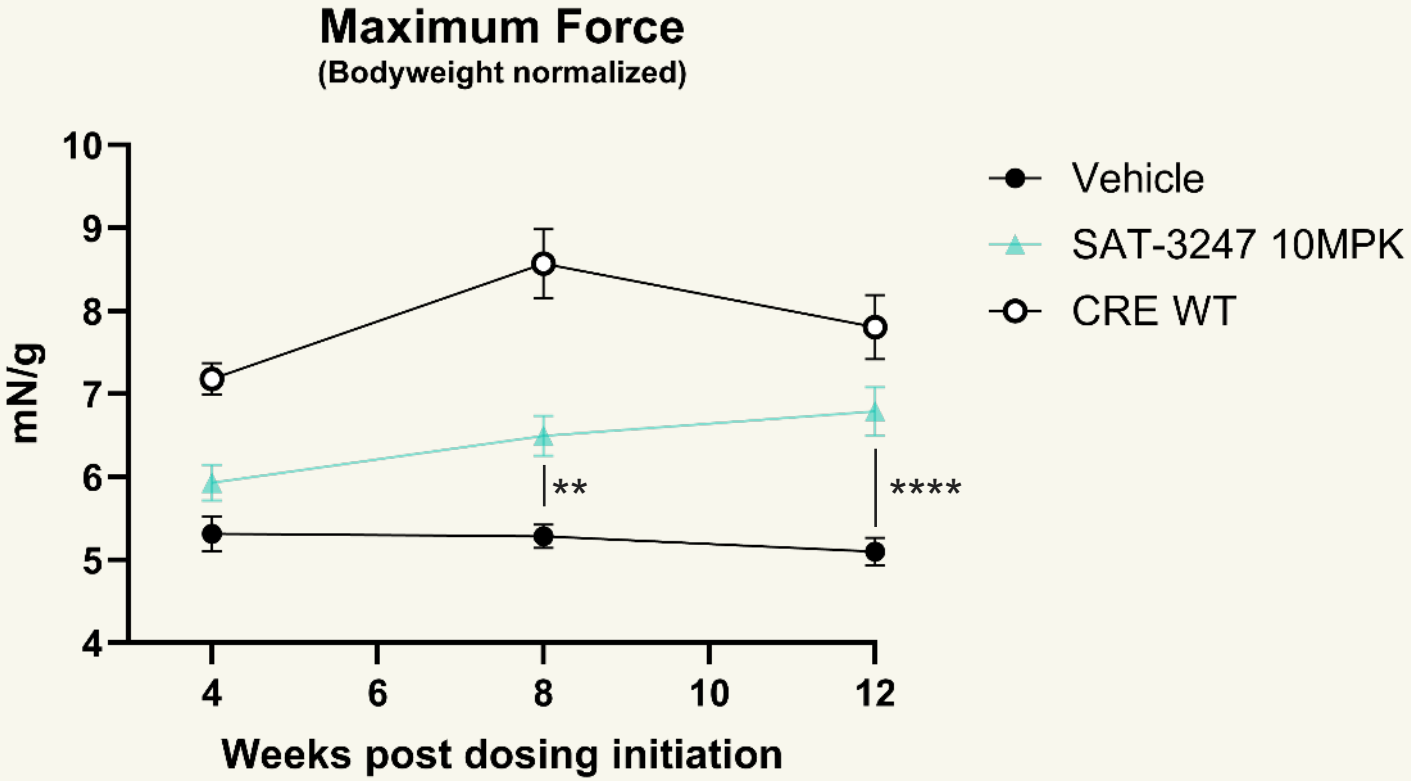

Summary

- SAT-3247 promotes asymmetric division of muscle satellite cells in response to injury.
- FSHD is one of the most common adult muscular dystrophies.
- There is correlation between regeneration markers and pathology severity in FSHD muscle which implies that regenerative capacity is limited or declines in more severely diseased tissue.
- Through an **alternative regenerative pathway,** SAT-3247 **increases the percentage** of activated satellite cells **undergoing asymmetric division** and therefore increases **effective muscle regeneration**.
- SAT-3247 may be an effective therapy in other clinical settings with significant muscle injury
- Satellos plans to initiate an FSHD clinical program with SAT-3247 in 2026.

Reimagine

how muscle degeneration is treated.

Regenerate

with small molecule medicines.

Realize

the next horizon to improve lives.