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Rebuilding muscle from within™

SAT-3247: An Oral Small Molecule Inhibitor Targeting AAK1, a Critical Effector Of Skeletal Muscle Regeneration

Dr. Phil Lambert, CSO

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Corporate Mission and Highlights

We **challenge** conventions**, break** scientific ground, and **urgently** advance drug development, **because patients are waiting.**

Our mission is to develop **a new class of drugs** that treat degenerative muscle conditions by **resetting muscle repair and regeneration, thereby restoring muscle function.**

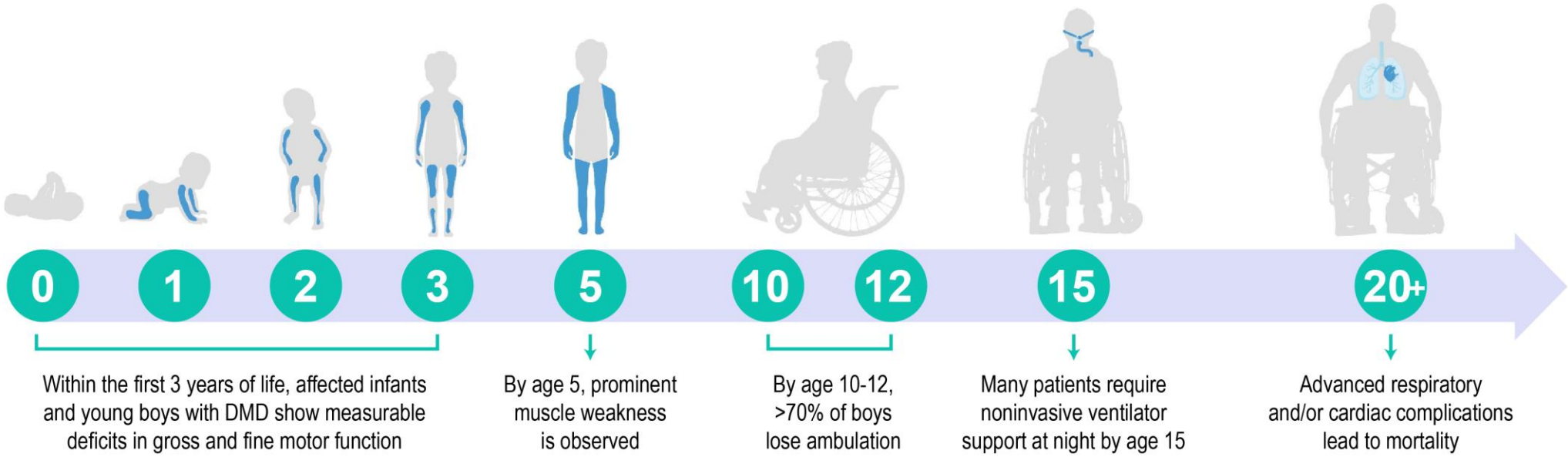
Presentation Highlights

New dystrophin independent approach to treating Duchenne	**Small molecule based regenerative medicine with disease modifying potential**	**Large breadth of preclinical proof of concept across multiple indications**	**Lead drug completing IND enabling studies in 1st half 2024**	**Phase 1 HV PK and safety study in 2nd half 2024**

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Duchenne muscular dystrophy: *progressive muscle loss*



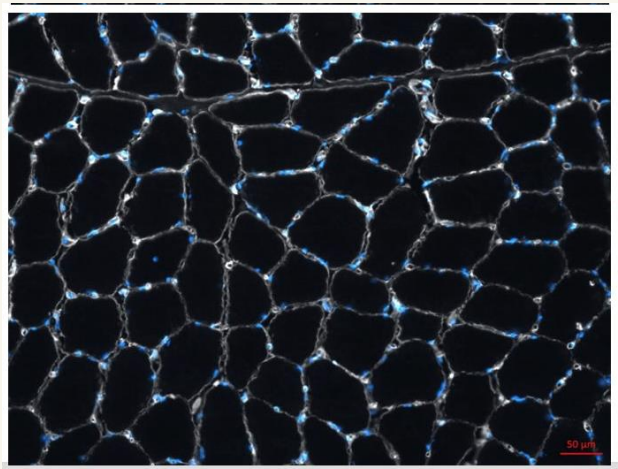
- Muscle regeneration in Duchenne fails to repair the constantly accruing damage
- Muscle regeneration fails because the stem cell population is not functioning properly

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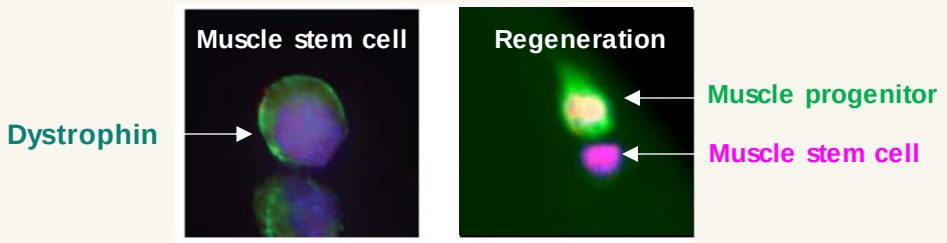
Regeneration is severely compromised in Duchenne:

due to the absence of dystrophin in muscle stem cells

*Healthy muscle fibers

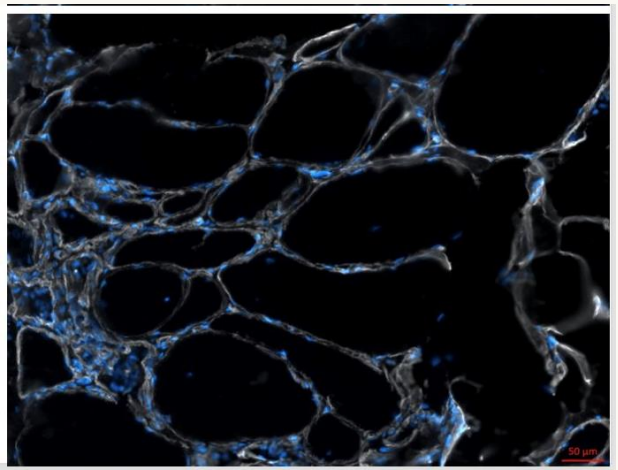


Dystrophin in muscle stem cells enables regeneration

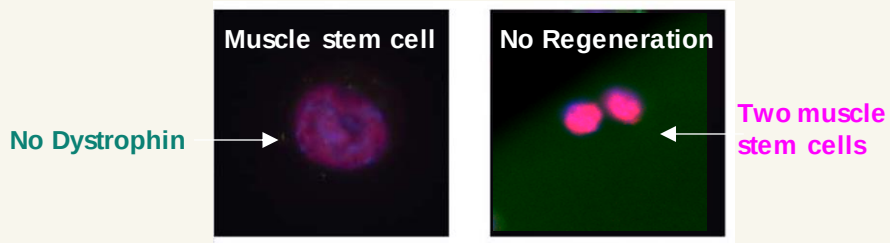


Asymmetric stem cell division

*Duchenne muscle fibers



Without dystrophin in muscle stem cells, regeneration is blunted



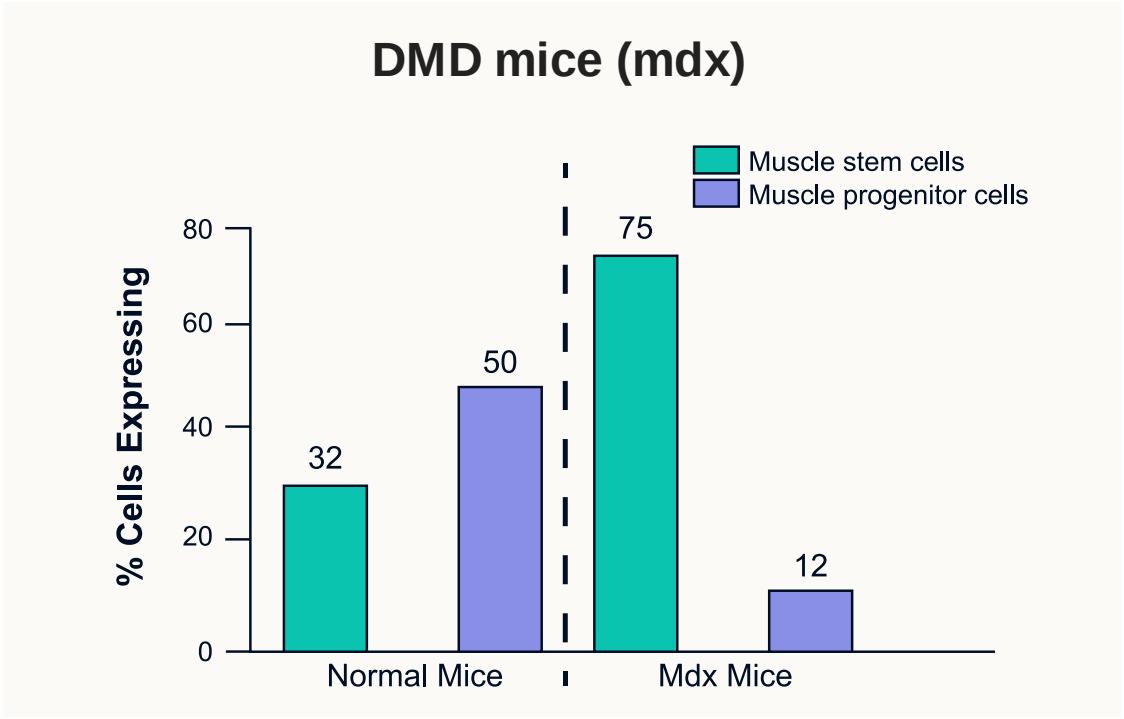
Symmetric stem cell division

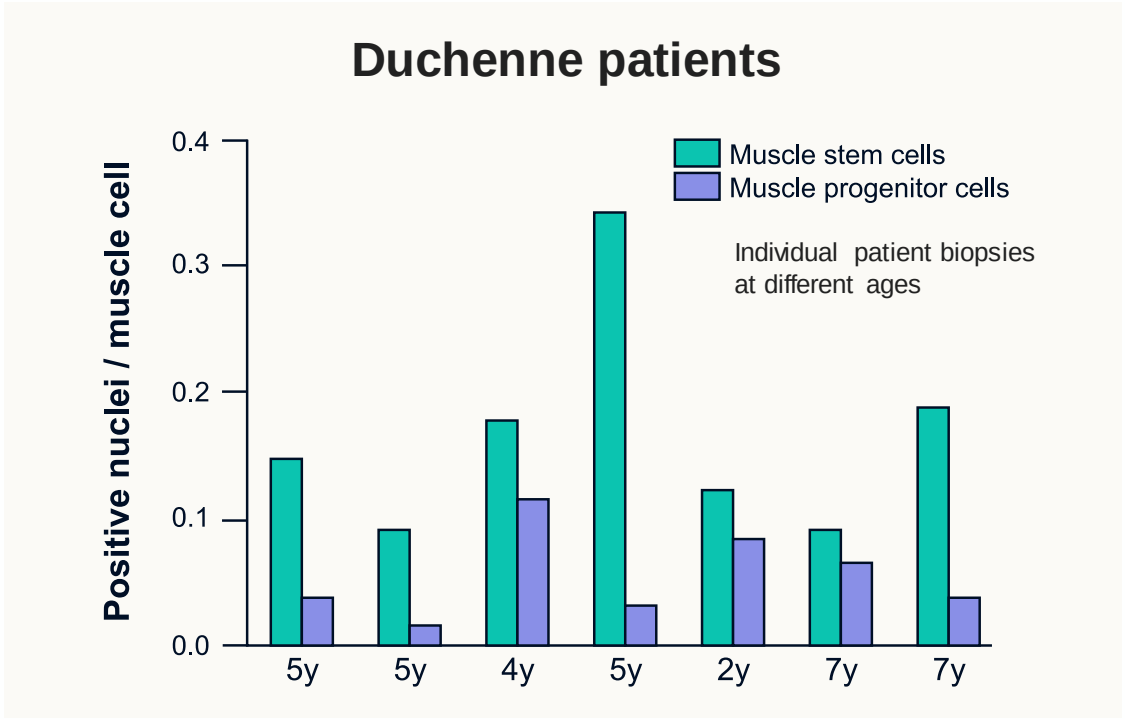
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* Human bioposy material

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This defect in muscle progenitor formation is clearly present in Duchenne preclinical models and patients



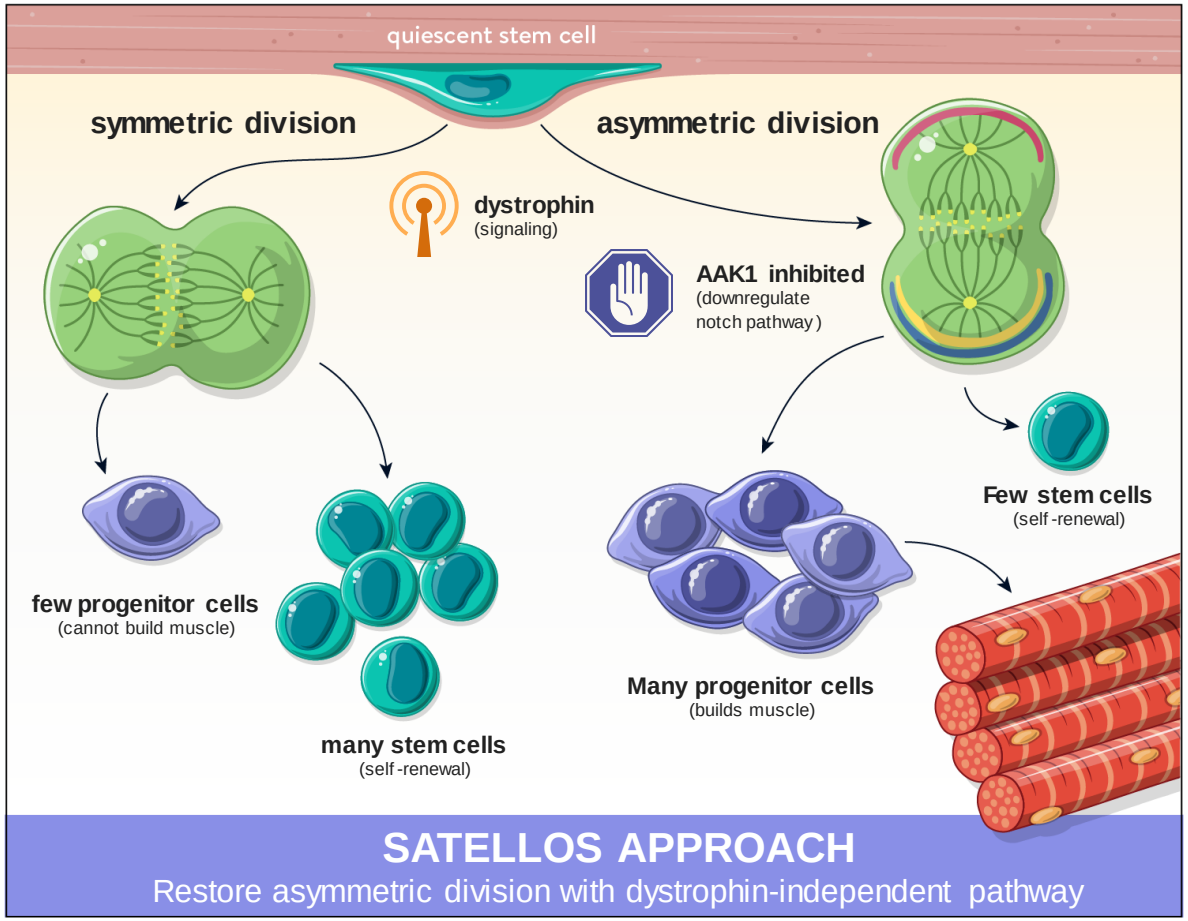


In DMD patients and the mdx mouse model have **abundant muscle stem cells**, but they **lack muscle progenitor cells** necessary for **formation** of **mature muscle fibers.**

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The role of dystrophin in muscle stem cells can be replaced to restore muscle regeneration



- Dystrophin promotes asymmetric stem cell divisions[1]
- Asymmetric division of stem cells produces **progenitor cells**
- Progenitors are required for repair and regeneration
- Satellos discovered an alternate regulator of asymmetric divisions : **AAK1 a regulator of Notch signaling**
- Inhibition of AAK1 promotes asymmetric division and progenitor formation

- **Satellos has developed SAT-3247 as an inhibitor of AAK1 to promote repair and regeneration of injured skeletal muscle**

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[1] Dumont et al., *Nat Med* 2015, [2] Wang et al., *Cell Stem Cell* 2019

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Why regenerate muscle without dystrophin?

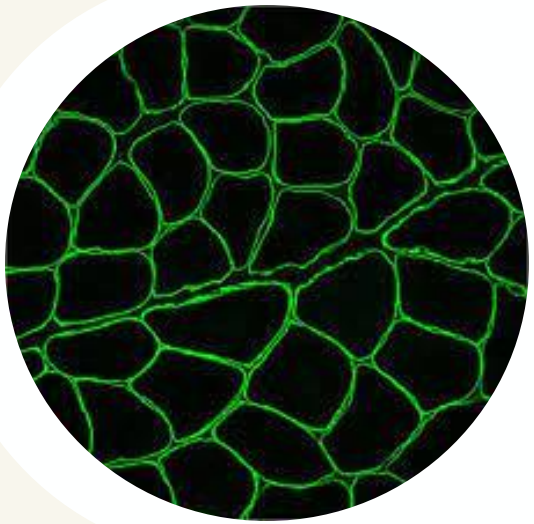
Duchenne patients walk without any muscle dystrophin

Milder course in Duchenne patients with nonsense mutations and no muscle dystrophin

M. Zatz*, R.C.M. Pavanello, M. Lazar, G.L. Yamamoto, N.C.V. Lourenço, A. Cerqueira, L. Nogueira, M. Vainzof

The discovery that Duchenne patients were ambulatory, despite having no muscle dystrophin, provides genetic proof of concept that it is possible to have functional muscle in the absence of dystrophin.

Also, this report, as well as additional reports of ambulatory Duchenne patients lacking dystrophin[1], provide strong support for Satellos' therapeutic strategy to restore regeneration as a new approach in treating Duchenne.



Dystrophin in Fibers

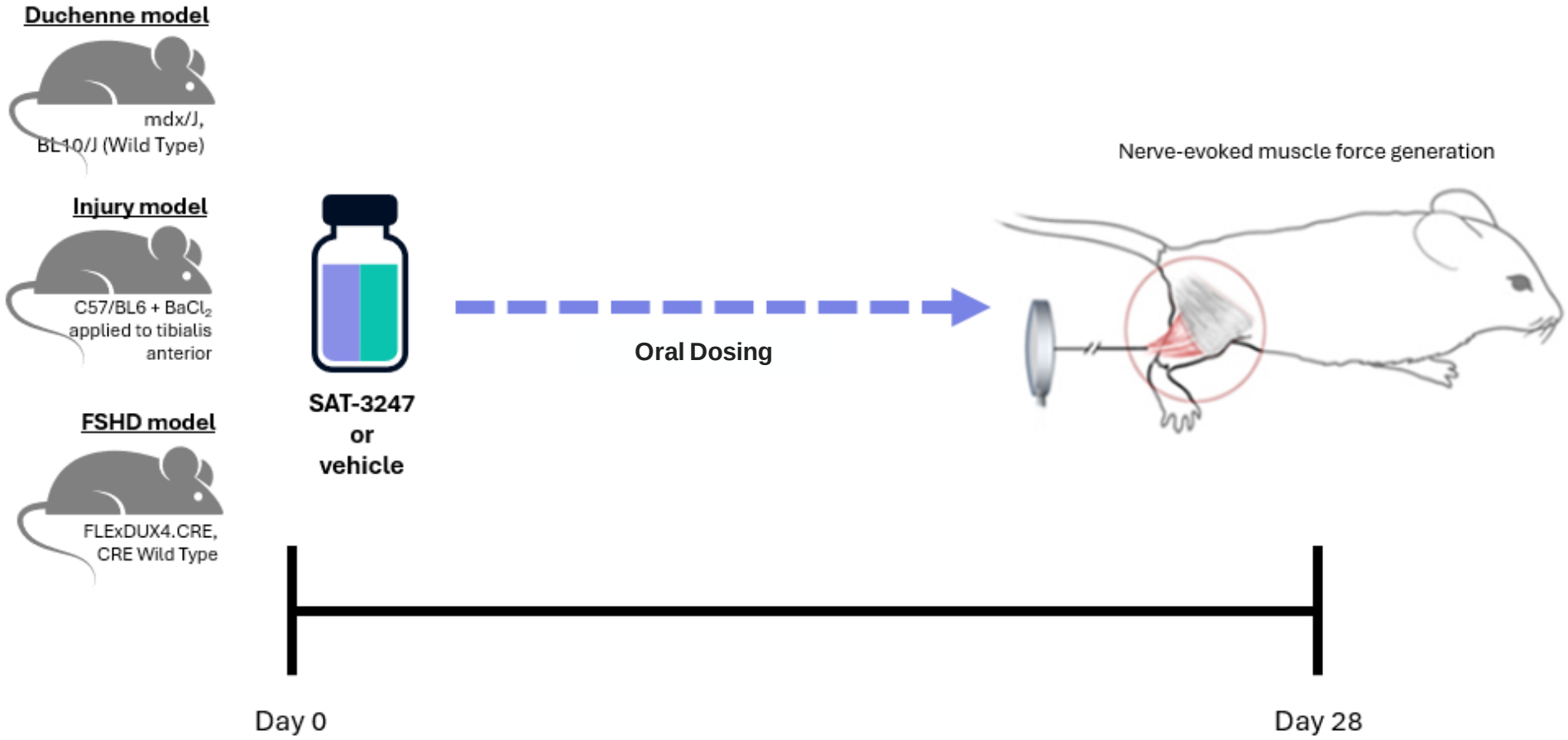
Kunkel and Zatz (unpublished) see: https://www.youtube.com/watch?v=RfobvyolEzU&t=2501s)

1. Flanigan et al. 2023 *ESHG*

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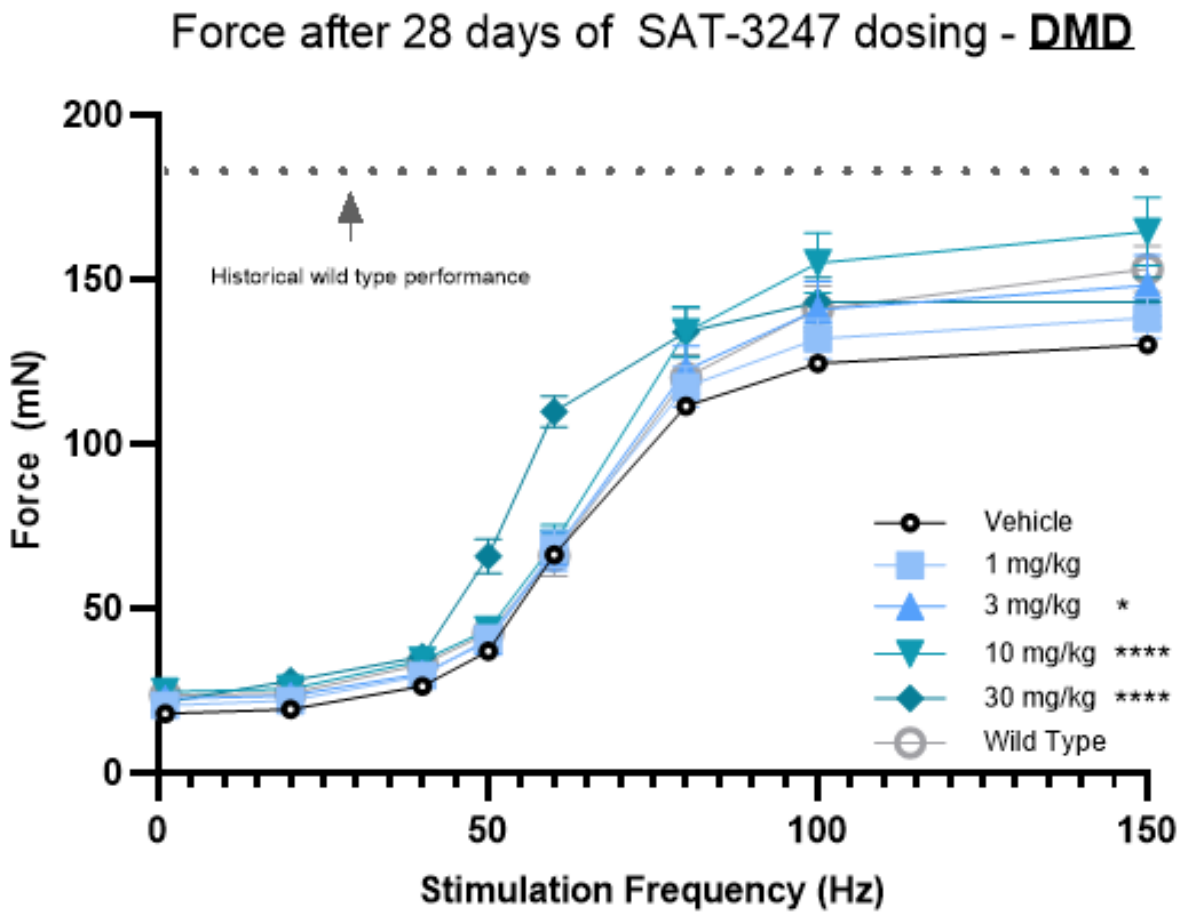
Multiple preclinical proof of concept models

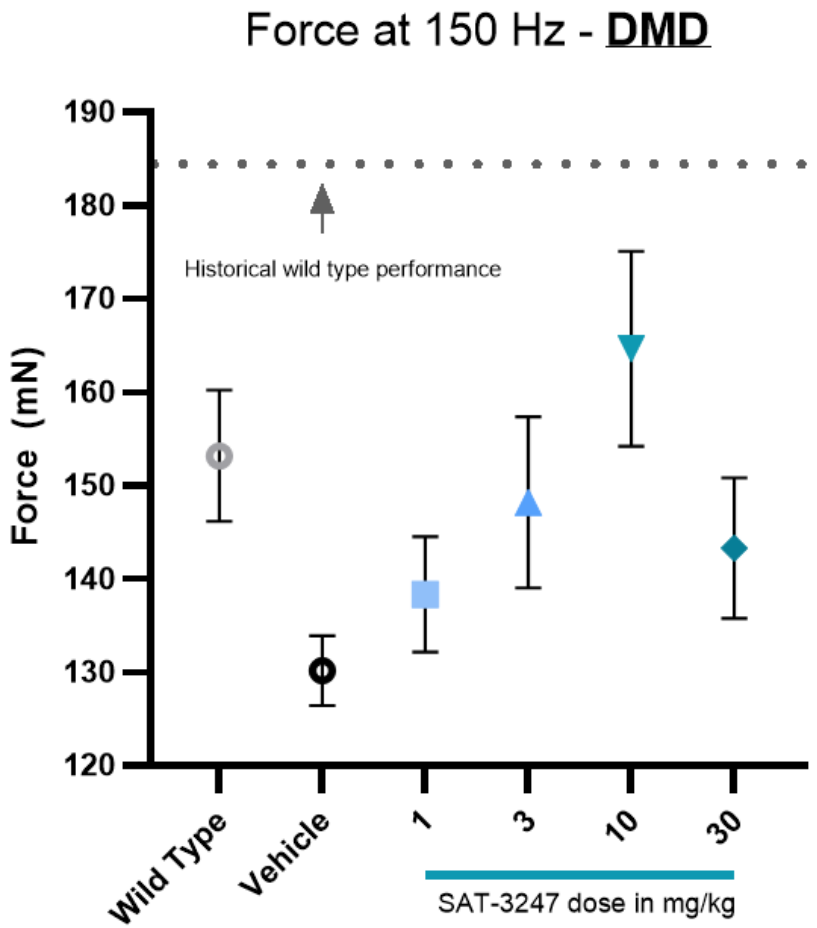
Live animal limb function assessment



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SAT-3247 improves muscle strength in a mouse model of DMD

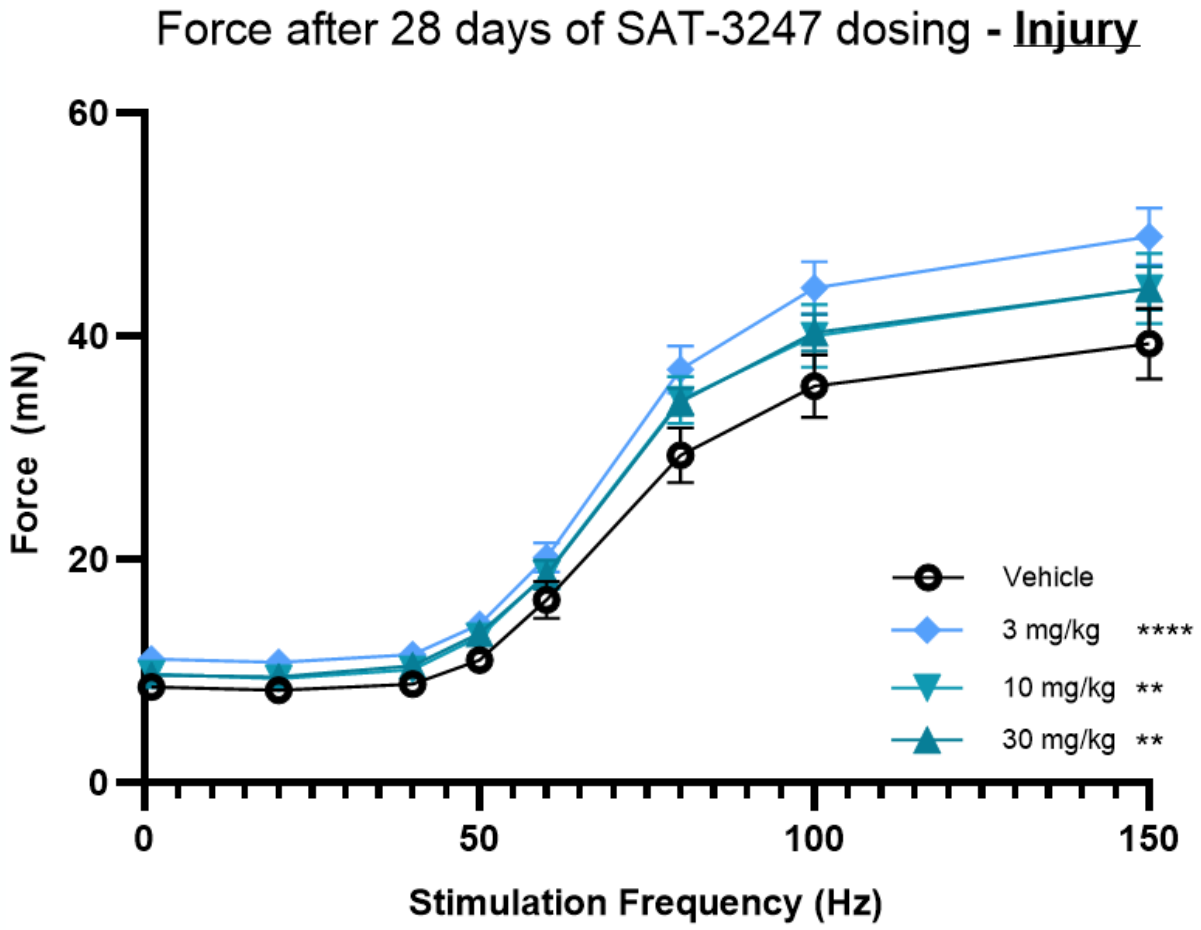


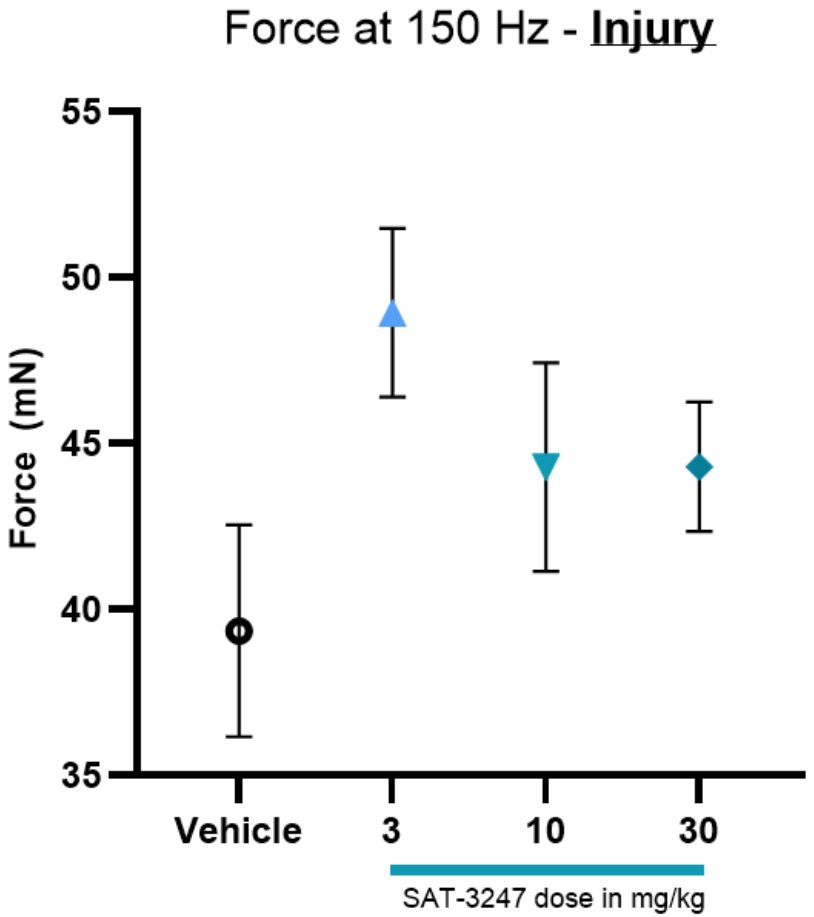


* p<0.05, **** p<0.00001

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SAT-3247 improves muscle strength in a WT muscle injury model

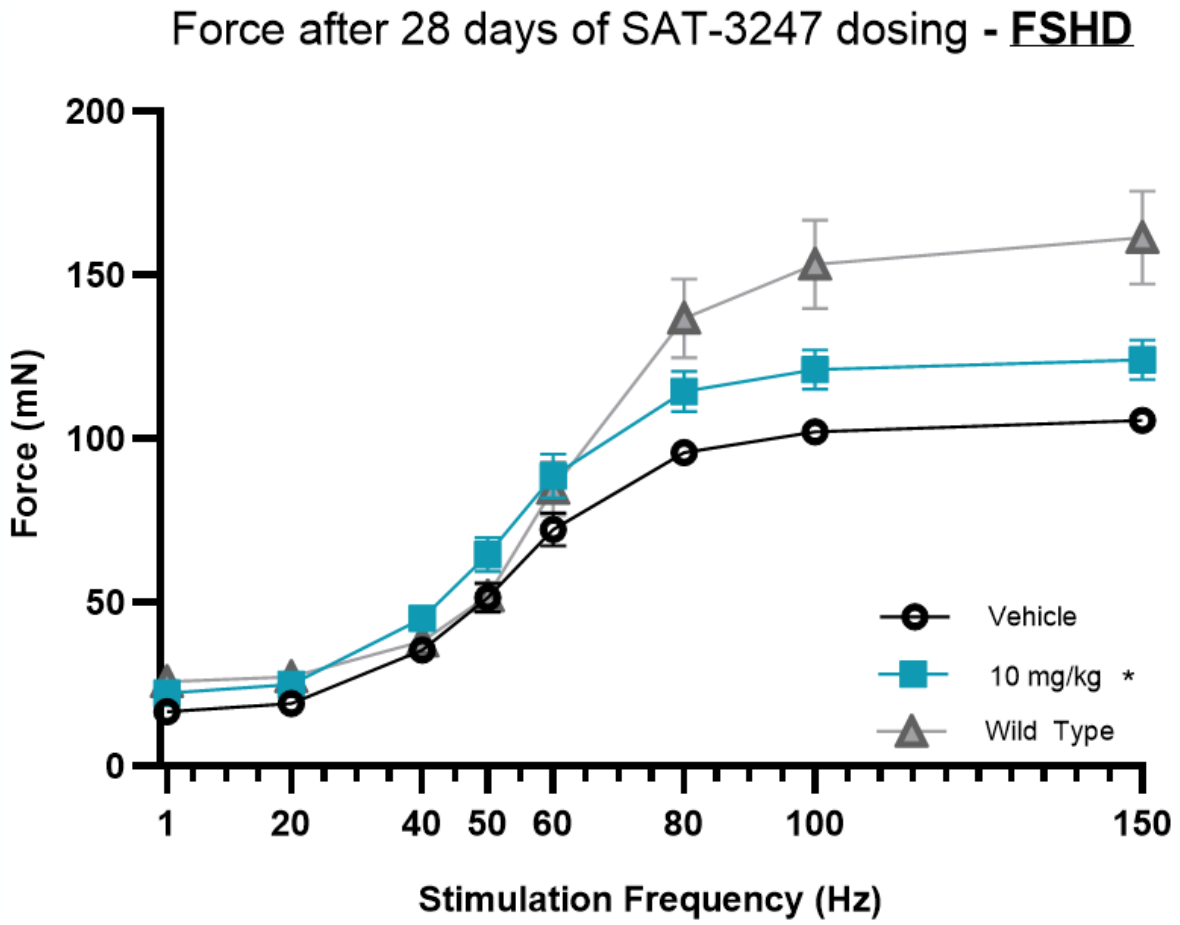


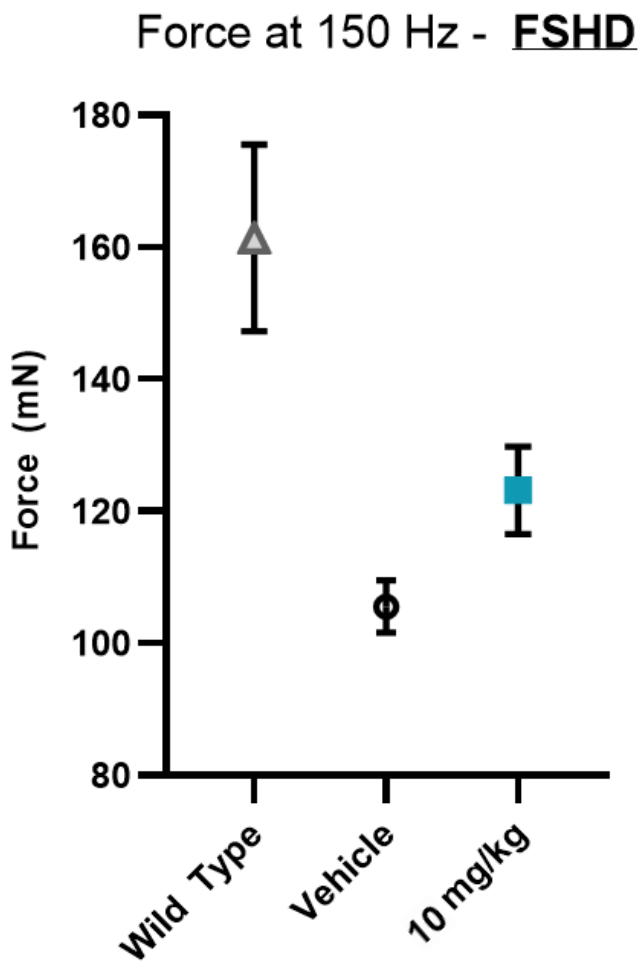


** p<0.01, **** p<0.00001

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SAT-3247 improves muscle strength in a mouse model of FSHD





* p<0.05,

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On target for FIH studies for 2024

Lead drug candidate:
SAT-3247



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REIMAGINE how degenerative muscle diseases are treated.

REGENERATE with novel small molecule medicines.

REALIZE the potential to improve lives.

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