SAT-3247 Phase 1a Study Results Presented at MDA 2025

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Regeneration: the next horizon™

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SAT-3247 Phase 1 Study Conducted in Healthy Volunteers and Adults with DMD

Healthy Volunteers (n=72)

- **Single Ascending Doses:**
  - Up to 400 mg across 5 cohorts
  - Single group food effect crossover
- **Multiple Ascending Doses:**
  - Up to 240mg/day across 4 cohorts
  - Administered for 7 days
- **SAT-3247 administered as oral tablets**

Primary Endpoints	Secondary Endpoints
Safety and tolerability	Pharmacokinetics

Adult Duchenne Patients

- **Up to 10 adults:**
  - Over 18, genetically confirmed with DMD
- **Doses:**
  - 60/mg per day
  - Weekdays for 28 days (i.e., 20 doses)

Primary Endpoints

- Safety and tolerability

Secondary/Exploratory Endpoints

- Pharmacokinetics
- Upper extremity dynamometry
- 99th maximum effort
- % predicted forced vital capacity
- Serum markers

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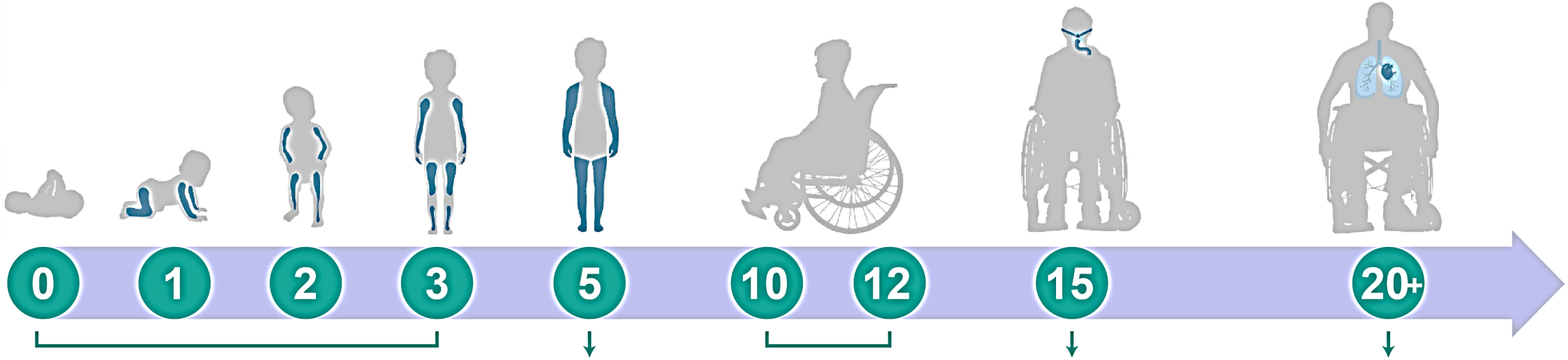
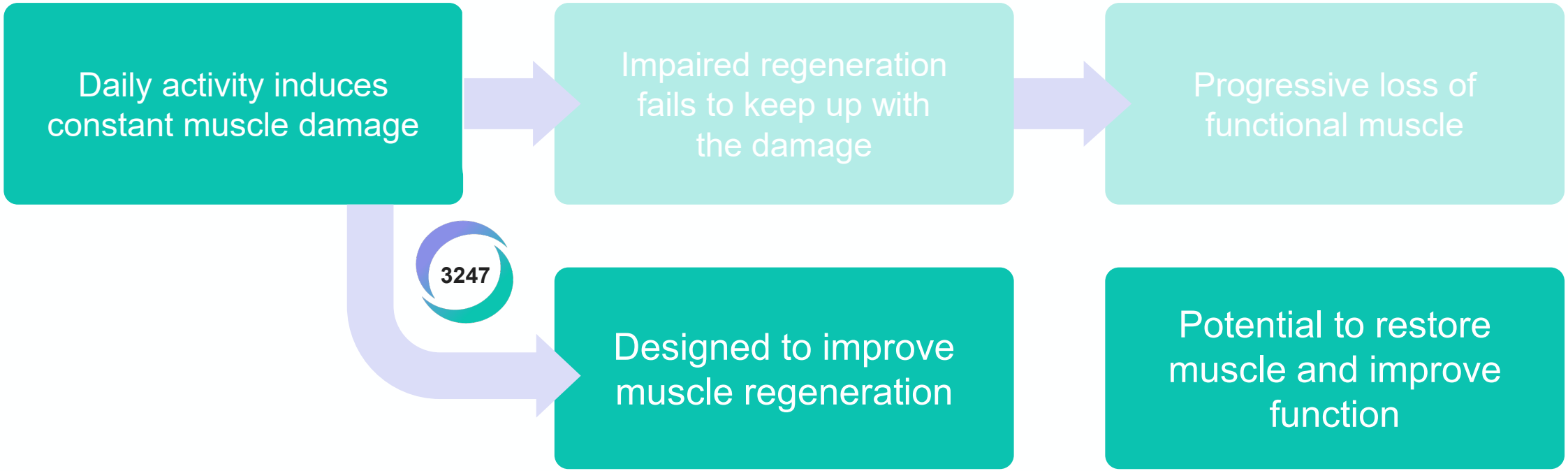
Progressive, Accumulating Muscle Loss Underlies Loss of Functional Capability in DMD



Impaired regeneration underlies muscle loss

SAT-3247 Designed to Correct Regeneration Deficit and Improve Functional Outcomes



SAT-3247 is an Orally Available Small Molecule Drug

Mechanism of Action

- Selective inhibitor of Aak1 (Adapter associated kinase 1)
- Replaces “the function of dystrophin” in stem cells
- Corrects ongoing muscle regeneration process

Proof of Concept

- Conducted multiple preclinical studies in DMD, FSHD and injury
- Consistently see correction of regeneration deficit
- Functional benefits in murine and canine models of DMD

Overview of Healthy Volunteers Phase 1a SAD/MAD with SAT-3247

SAT-3247 well tolerated with no adverse events reported at predicted efficacious dose

Plasma PK levels demonstrated desired coverage profile at predicted efficacious dose

Healthy Volunteer Safety Summary[1]

Cohort		Clinically Significant:				Drug-related AEs
		Labs	Vitals	Physical Exam	ECG	
SAD	10 mg	None	None	None	None	None
SAD	50 mg	None	None	None	None	None
SAD	150 mg	None	None	None	None	None
SAD	300 mg	None	None	None	None	None
SAD	400 mg	None	None	None	None	2 events: mild nausea; mild abdominal pain
MAD	60 mg	None	None	None	None	None
MAD	120 mg	None	None	None	None	1 event: mild lightheadedness
MAD	180 mg	Mild transaminase elevations	None	None	None	3 events: Intermittent mild epigastric pain; mild feeling sleepy; mild lethargy
MAD	240 mg	Mild transaminase elevations	None	None	None	

Predicted dose

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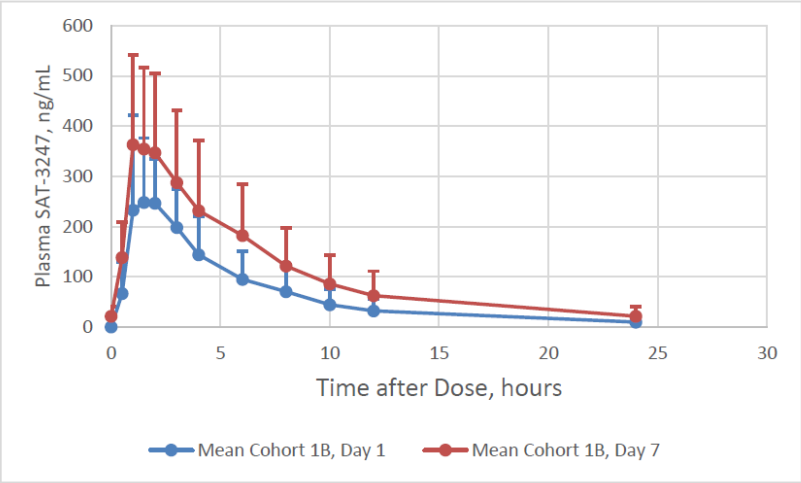
[1] Based on 20 Feb 2025 data cut-off; study is ongoing

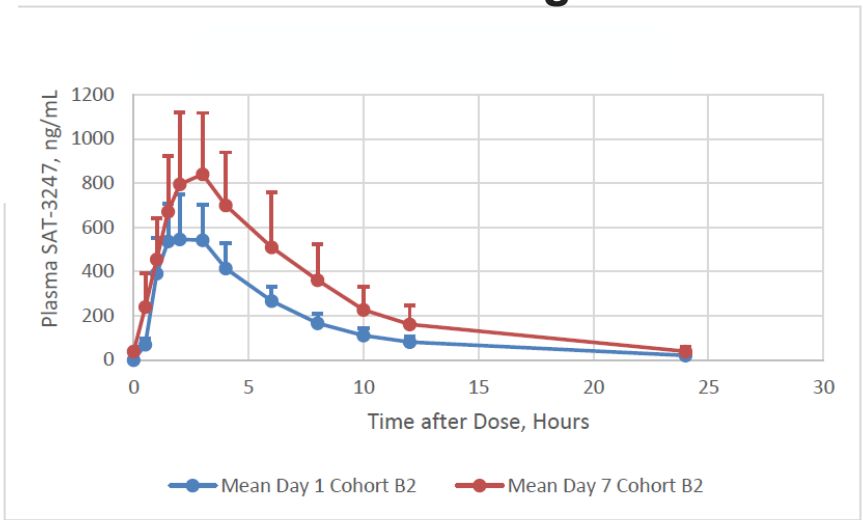
SAT-3247 Exhibits Dose Proportional PK Profile

MAD 60 mg

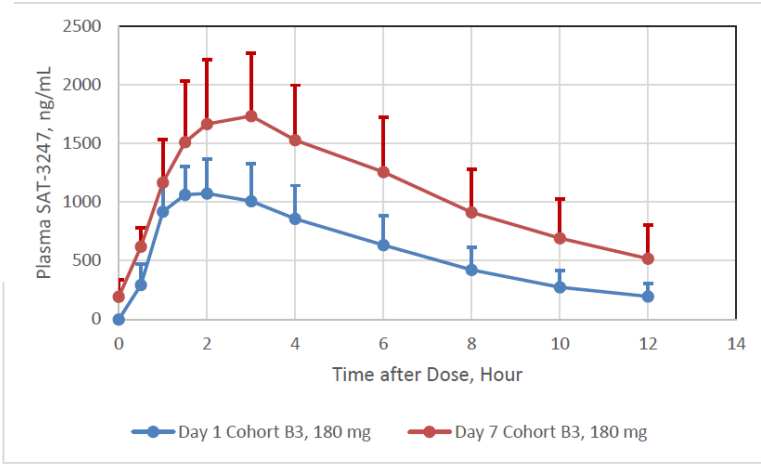


Mean Plasma Concentration-Time Day 1 and Day 7

MAD 120 mg

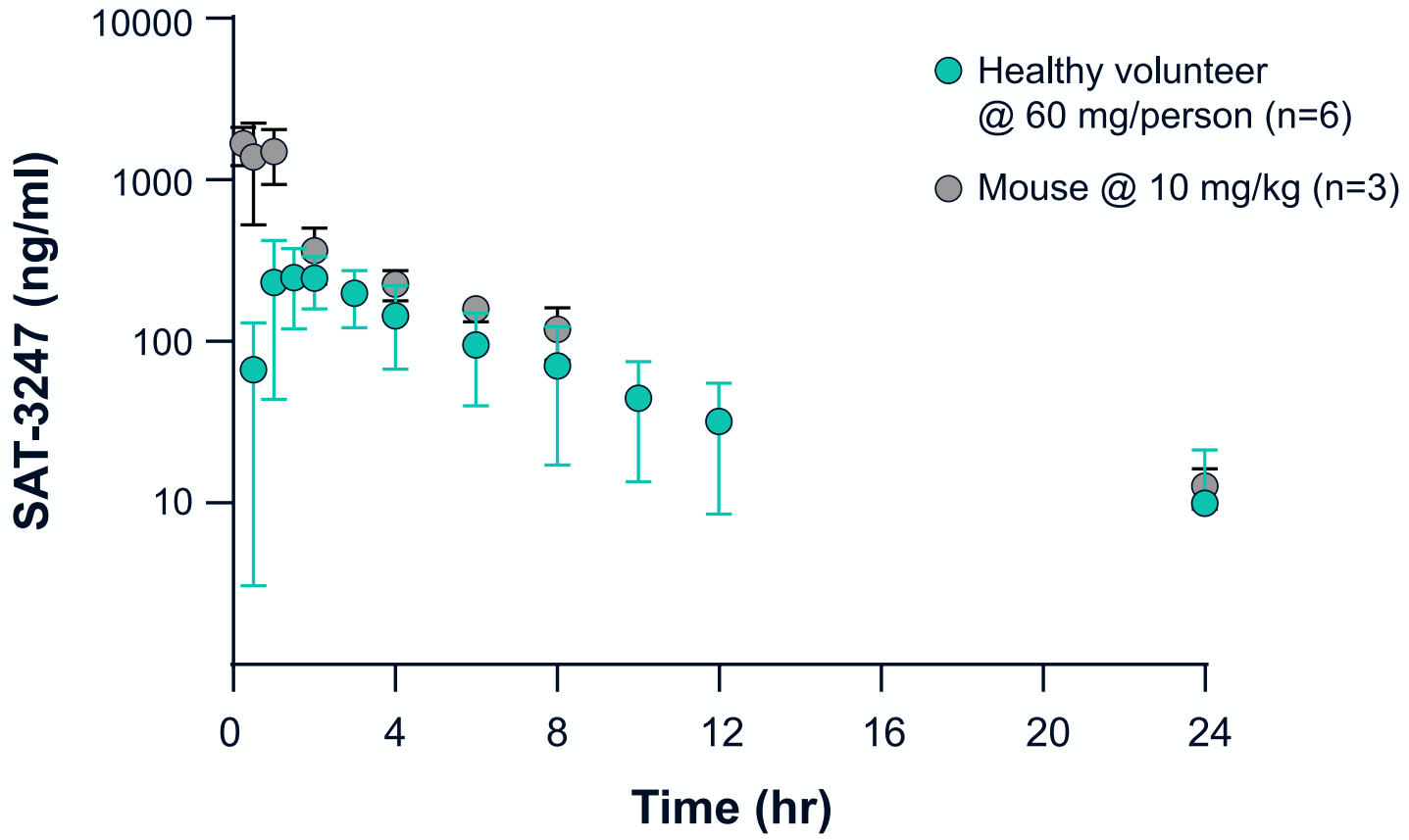


MAD 180 mg



SAT-3247 Exposure in Human Consistent with Preclinical Efficacious Dose

- Desired exposure: above Aak1 IC_{50} for 6-8hrs
- 60mg & 120mg dose align well
- PK profile translates



SAT-3247 Phase 1 Study: Next Steps

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Pharmacokinetics Upper extremity dynamometry 99th maximum effort	% predicted forced vital capacity Serum markers

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REIMAGINE how muscle degeneration is treated.

REGENERATE with molecule medicines.

REALIZE the next horizon to improve lives.

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