

# BASECAMP: A Phase 2a Dose Comparison and Exploratory Efficacy Study of Forazapadin (SAT-3247) in Ambulatory Boys with Duchenne Muscular Dystrophy

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## INTRODUCTION

- In Duchenne muscular dystrophy (DMD), muscle damage outpaces repair and regeneration resulting in progressive muscle loss. It has been recently shown that net muscle regenerative capacity declines sharply in boys with DMD between 7-11 years (Reference 1 and **WMS Poster #2.68eP**), which is a critical age range associated with significant clinical decline.
- Seminal research established dystrophin as a key signaling protein in muscle stem cells<sup>2</sup>, in addition to its well-recognized structural role in muscle fibers (**Figure 1**). Variants in the *DMD* gene that result in the absence (or near absence) of dystrophin impair the ability of muscle stem cells to establish polarity in response to muscle damage, thereby reducing muscle progenitor formation and overall regenerative capacity (**Figure 2a**).
- Forazapadin (formerly SAT-3247) is an investigational, small molecule inhibitor of adaptor-associated protein kinase 1 (AAK1) designed to re-establish polarity and asymmetric division of muscle stem cells, enhance muscle progenitor formation, and promote muscle fiber regeneration in DMD and other serious muscle diseases (**Figure 2b**).
- Safety and efficacy data from the Phase 1a/b CL-101 study of forazapadin (NCT06565208), together with early findings from the ongoing Phase 2 TRAILHEAD study (NCT06867107) in older boys and young men who continued from CL-101, have been reported previously (References 5 and 6; **WMS Poster #2.75P**).
- Ongoing Phase 2 TRAILHEAD data continue to demonstrate a favorable safety profile of forazapadin, accompanied by stability or trends of improvement in assessments of muscle strength, function, and respiratory capacity.
- Here, we describe the rationale and design of BASECAMP, a Phase 2a study of forazapadin (NCT07287189) in ambulatory boys with DMD who are of an age associated with reduced muscle regeneration<sup>1</sup> and significant clinical decline.

Figure 2a

In Duchenne muscular dystrophy (DMD), absence of dystrophin in muscle stem cells impairs polarization and favors symmetric division, thereby reducing progenitor formation and muscle regeneration.

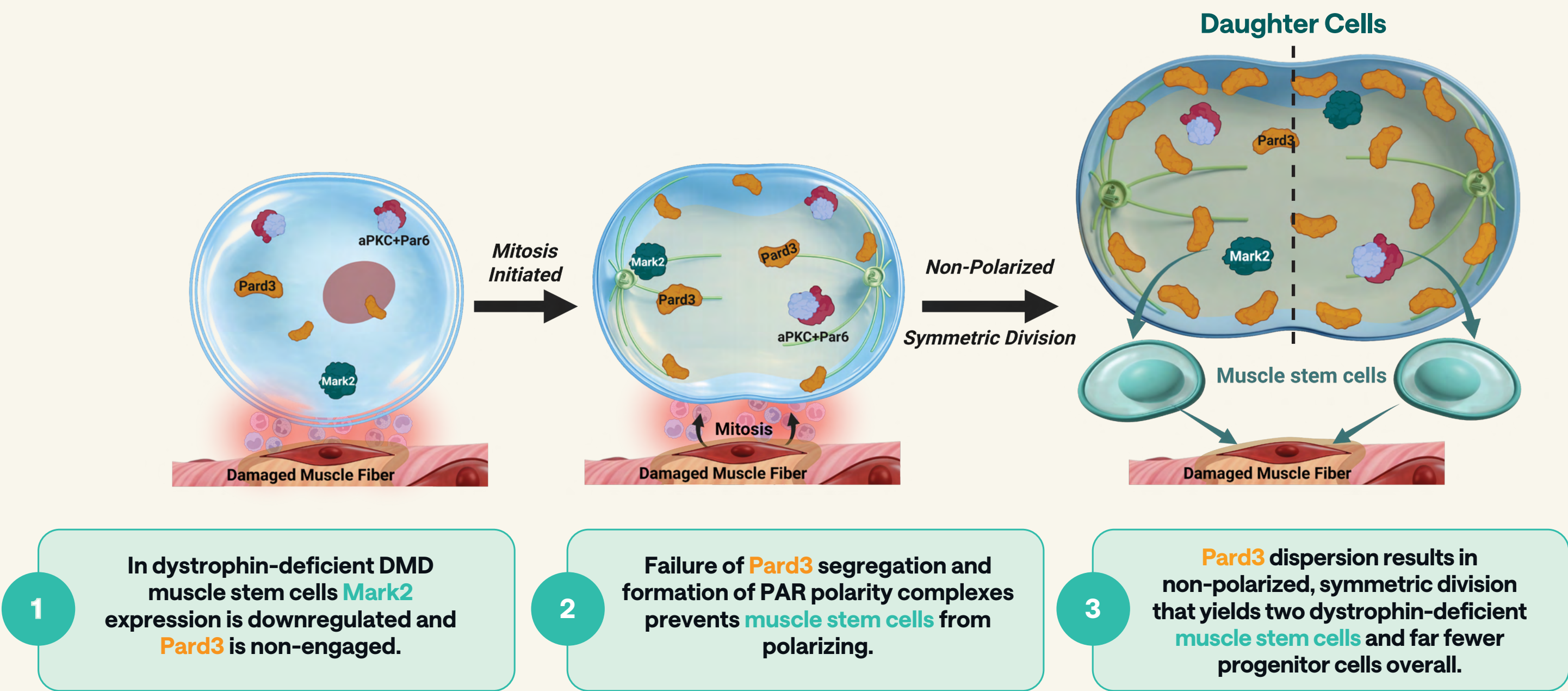


Figure 1

Dystrophin serves two critical functions in the skeletal muscle damage response: 1) as a cytoskeletal component to stabilize fibers, and 2) as a key regulator of stem cells enabling them to polarize, divide, and yield progenitor cells for muscle repair and regeneration.

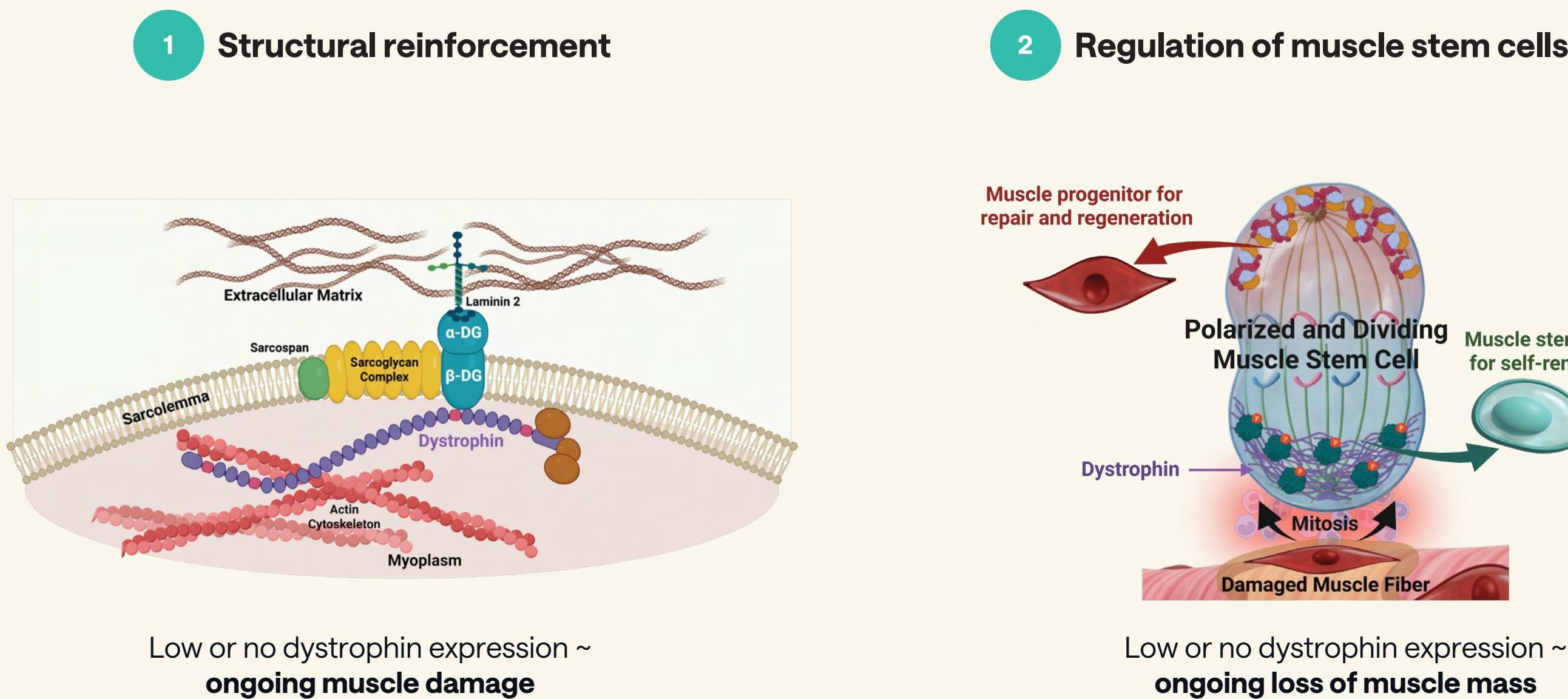
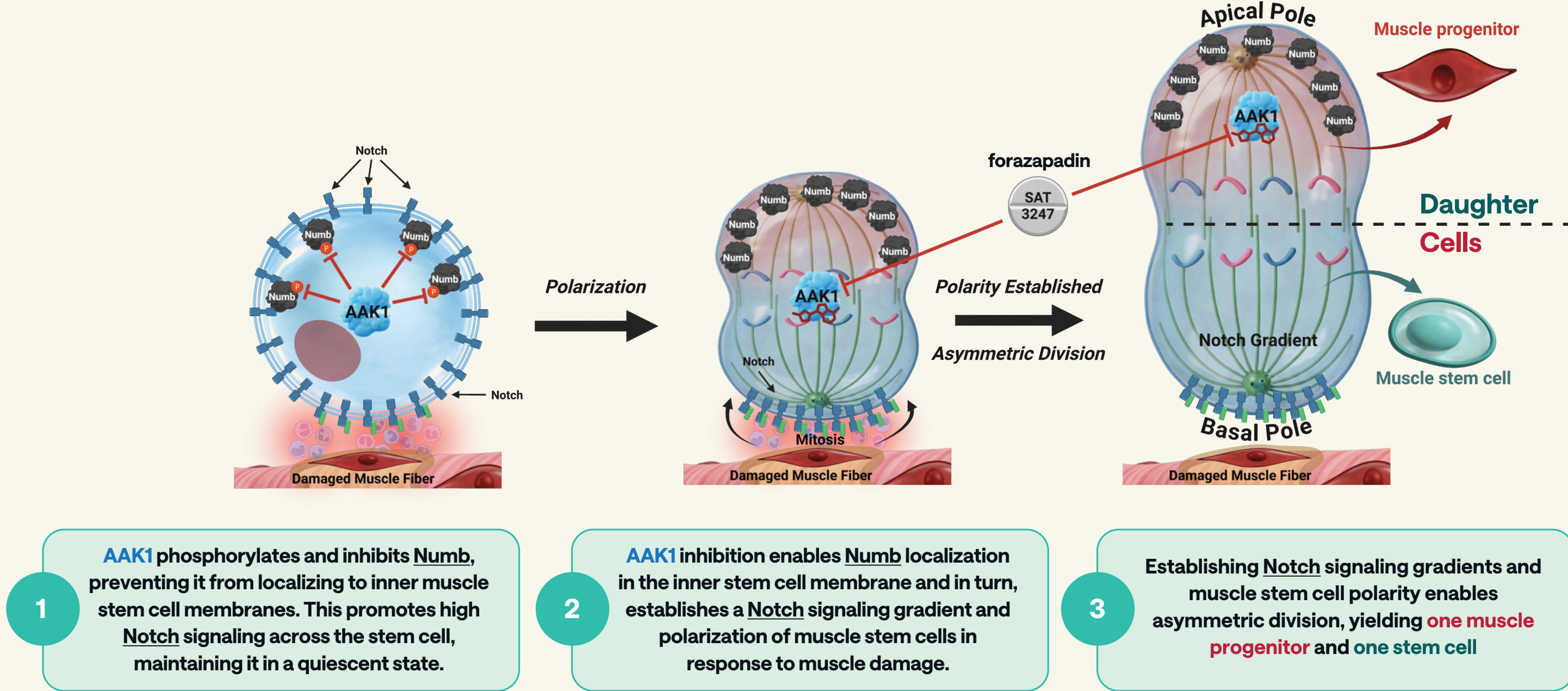


Figure 2b

Forazapadin (SAT-3247) is designed to inhibit adaptor-associated kinase 1 (AAK1), which enables a dystrophin/*Mark2* and *Pard3*-independent mechanism (*Numb*/*Notch*) to establish muscle stem cell polarity, which enhances muscle progenitor formation and regeneration in DMD muscle and other muscle diseases.



## METHODS

- BASECAMP is a Phase 2a, randomized, double-blind, placebo-controlled, dose comparison and exploratory efficacy study of orally administered forazapadin.
- BASECAMP will enroll up to 51 ambulatory participants with DMD  $\geq 7$  and  $< 10$  years of age in the United States, Canada, United Kingdom, Belgium, Spain, Poland, Serbia, and Australia with a definitive diagnosis of DMD and confirmed pathologic variant in the *DMD* gene.
- In Part 1 of the study, participants will be randomized to receive placebo vs. 60 mg or 120 mg forazapadin administered orally using a 5-days-on/2-days-off (weekday) dosing regimen for 12 weeks. Randomization will be stratified by baseline corticosteroid regimen and prior concomitant DMD medications (gene therapy, exon skippers, givinstat, or none).

Tables 1: Key inclusion and exclusion criteria

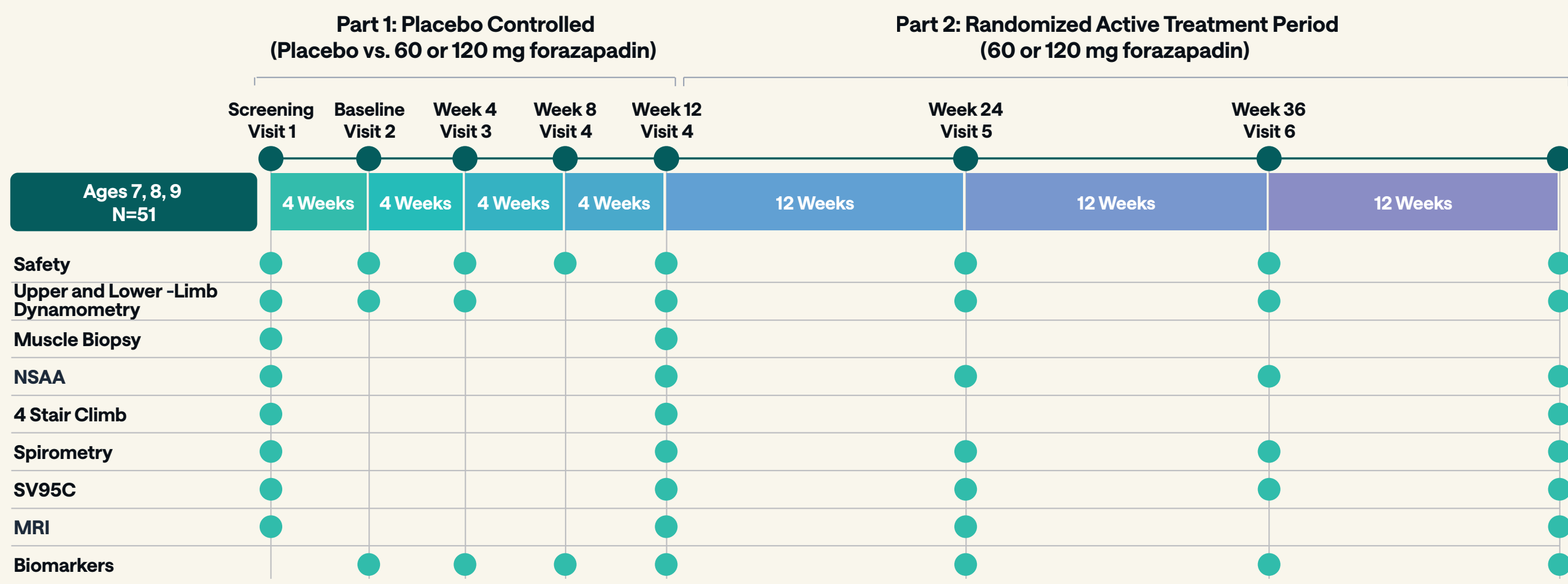
| Key* Inclusion Criteria                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                         | Key* Exclusion Criteria                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                       |
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| <ul style="list-style-type: none"><li>Definitive diagnosis of DMD, confirmed by clinical findings and pathogenic variant of the <i>DMD</i> gene</li><li>Age <math>\geq 7</math> to <math>&lt; 10</math> years</li><li>Two four-stair climb assessments at Visit 1 of <math>\leq 8</math> seconds</li><li>Time-to-rise between <math>\geq 3</math> and <math>&lt; 10</math> seconds</li><li>Stable dose of daily or weekend systemic glucocorticoids for <math>\geq 3</math> months prior to screening, and for the duration of the study</li><li>Stable doses of prescription or over-the-counter medications, and/or herbal supplements <math>\geq 1</math> month prior to screening, and for the duration of the study</li></ul> <p><b>Allowance for prior DMD treatments and modalities</b></p> <ul style="list-style-type: none"><li>Participants that have previously received <b>delandistrogene moxeparvovec</b> (Elevidys) either in a prior clinical trial or in the commercial setting <math>&gt; 18</math> months prior to screening, whose muscle function tests have stabilized or demonstrated decline <math>\geq 3</math> months prior to screening.</li><li>Participants that have received prior treatment with an <b>investigational gene therapy (other than delandistrogene moxeparvovec)</b> <math>\geq 24</math> months prior to screening</li><li>Participants that have previously received an <b>exon skipper</b> <math>&gt; 6</math> months prior to screening whose muscle function tests have stabilized or demonstrated decline <math>\geq 3</math> months prior to screening.</li><li>Participants receiving a stable dose of <b>givinostat (Duvyztat)</b> for <math>\geq 18</math> months prior to screening. Those who discontinued due to tolerability before 18 months are eligible if last dose is <math>\geq 30</math> days from screening.</li><li><b>Any other medication indicated for DMD (approved or investigational, other than corticosteroids including vanomolone, gene therapy, or givinstat):</b> participants whose last dose was <math>\geq 6</math> months prior to screening</li><li><b>Participation in physical therapy/strength training regimens:</b> must be stable for <math>\geq 2</math> months prior to the screening and for the duration of the trial.</li></ul> | <ul style="list-style-type: none"><li>Expected loss of ambulation within <math>\leq 12</math> months</li><li>Medical conditions (other than DMD) sufficient to invalidate participation or make participation unnecessarily hazardous</li><li>Medical conditions (other than DMD) sufficient to invalidate participation, adversely affect participant safety, reduce likelihood treatment course or follow-up would be completed, or impair study results</li><li>Evidence of significant hepatic dysfunction (GLDH <math>&gt; 2x</math> upper limit of normal)</li><li>Participants for whom MRI or open muscle biopsy is contraindicated</li><li>Surgeries or conditions that might affect absorption of medicines</li><li>Acute gastrointestinal symptoms, acute infection, or significant infection or known inflammatory process</li><li>Impaired cardiac function (LVEF <math>&lt; 50\%</math>) or evidence of symptomatic cardiomyopathy</li><li>FVC of <math>&lt; 60\%</math> predicted</li><li>Ongoing participation in any other clinical trial or follow-up study for a therapeutic intervention (studies involving no active intervention allowed)</li><li>Current or expected use of strong or moderate CYP3A4 inhibitors (last doses must be <math>\geq 5</math> elimination half lives prior to screening)</li><li>Received forazapadin (SAT-3247) in another study</li></ul> |

\*Non-exhaustive list of criteria; DMD=Duchenne muscular dystrophy, GLDH = glutamate dehydrogenase, LVEF = left ventricular ejection fraction, FVC = forced vital capacity, CYP3A4 = cytochrome P450 3A4

- Participants who have previously received a dystrophin-restoring gene therapy are eligible to enroll in BASECAMP. These participants will be capped at 4 (roughly 25%) per treatment arm. Key inclusion and exclusion criteria, including provision for DMD therapies prior to screening, are provided in **Table 1**.
- Following Part 1 of the study, participants will be allowed to continue into Part 2 which is a 36-week randomized, active treatment period receiving either 60 or 120 mg forazapadin on a 5 days on/2 days off (weekday) dosing regimen.
- The primary safety objective is to evaluate safety and tolerability of forazapadin.
- The primary efficacy objective is to evaluate forazapadin effects on muscle force (assessed by dynamometry) after 12 weeks of treatment.
- A study schematic and general schedule of assessments is provided in **Figure 3**.

Figure 3: BASECAMP study design and assessments

BASECAMP is a 12-week randomized, double-blind, placebo-controlled Phase 2a study of 60 or 120 mg forazapadin (Part 1) followed by a 36-week randomized active-treatment period in which participants will receive either 60 or 120 mg forazapadin administered on a 5-day on/2-day off (weekday) dosing regimen (Part 2). The primary objectives of BASECAMP are to evaluate safety and tolerability of forazapadin, and potential effects on muscle force and physical function, muscle regenerative capacity, and biomarkers of DMD progression.



NSAA = North Star Ambulatory Assessment, SV95C = stride velocity 95th centile; MRI = magnetic resonance imaging

## CONCLUSIONS & NEXT STEPS

- In DMD and other severe muscle diseases, muscle damage outpaces repair and regeneration, resulting in progressive muscle loss and replacement with fat and fibrosis.
- Significant clinical decline begins during early childhood in young boys with DMD. Recent studies have demonstrated that net regenerative capacity of muscle declines sharply between the ages of 7-11 years (reference 1 and **WMS Poster #2.68eP**). Therefore, mitigation during this critical period could potentially alter the progression of muscle and functional loss in the years that follow.
- Forazapadin (SAT-3247) is an investigational, orally administered small molecule inhibitor of adaptor-associated protein kinase (AAK1) designed to re-establish polarity and asymmetric division of muscle stem cells, muscle progenitor formation, and enhance muscle fiber regeneration in DMD and other muscle disorders.
  - Recent data from an ongoing Phase 2 TRAILHEAD study of forazapadin in older boys and adults with DMD demonstrate reduced muscle fat fraction, and stability or trends of improvement in assessments of muscle strength, function, and respiratory capacity (references 3 and 4, **WMS Poster #2.75P**).

- BASECAMP is a global Phase 2a, randomized, double-blind, placebo-controlled dose comparison and exploratory efficacy study of orally-administered forazapadin in ambulatory boys with DMD who are in this critical age range of muscular and functional decline.
  - Enrollment of 51 participants in BASECAMP is ongoing and enrollment is on track for completion in Q3 2026.
- Together, BASECAMP and TRAILHEAD are designed to evaluate forazapadin across the continuum of DMD disease progression, from early ambulatory disease through more advanced stages.

## REFERENCES

- Smid JK et al. *Skeletal Muscle* 2026. <https://doi.org/10.1186/s13395-026-00436-3>
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- Farwell W et al. A Phase 1a/b study of SAT-3247 in healthy volunteers and adult participants with Duchenne muscular dystrophy. AAN Annual Scientific Sessions (Chicago, IL), April 18-22. Presentation 19-003.
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