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BASECAMP Clinical and Regulatory Update

NOVEMBER 6 · INVESTOR CALL

REGENERATING MUSCLE FROM WITHIN™

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TODAY'S CALL

Agenda

1	Welcome		**Caitlin Lowie** Vice President of Investor Relations & Communications
2	Opening remarks		**Frank Gleeson** Co-founder & CEO
3	Clinical & regulatory update		**Wildon Farwell, MD** Chief Medical Officer
4	Closing remarks		**Frank Gleeson** Co-founder & CEO
5	Q&A		**Leadership team** with Elizabeth Williams, Chief Financial Officer

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Strategic Overview and Mission

OUR MISSION

To improve the lives of people living with devastating muscle diseases by restoring the body's natural ability to regenerate muscle.

Grounded in science. Inspired by need.

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BASECAMP Endpoint Refinement and Regulatory Strategy

Following recent FDA interactions, BASECAMP's primary efficacy endpoint is change in vastus lateralis fat fraction measured by MRI

WHAT CHANGED

MRI fat fraction

Primary efficacy endpoint now aligned with novel mechanism of muscle preservation and regeneration

FDA

Type C Interaction

Feedback supports MRI as a potential surrogate endpoint reasonably likely to predict benefit; continued engagement planned

WHAT IT MEANS

Potential accelerated approval

The totality of the MRI and clinical data may support accelerated approval based on FDA feedback

KEY TAKEAWAY: BASECAMP now prioritizes an objective 12-week placebo-controlled efficacy measure while retaining functional and other clinical endpoints.

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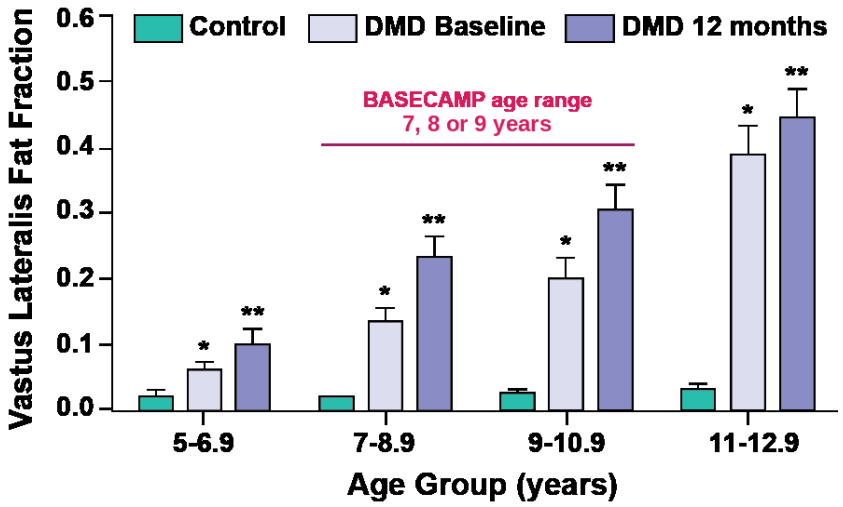
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A Clinical Measure Aligned with Forazapadin's Novel Mechanism

Objective measure with the potential to reflect DMD pathophysiology and forazapadin's expected biological effect

1. Fat Fraction consistently increases with age and DMD progression
2. Changes in fat fraction most often occur before changes in function
3. Fat fraction may serve as a surrogate endpoint reasonably likely to predict clinical benefit

Fat fraction increases with age, including at BASECAMP ages



Healthy controls vs. boys with DMD at baseline and 12 months[1]

KEY TAKEAWAY: Slowing fat accumulation or improving fat fraction may result in clinical benefit over time

1. Willcocks RJ, et al. Ann Neurol 2016;79:535–547.

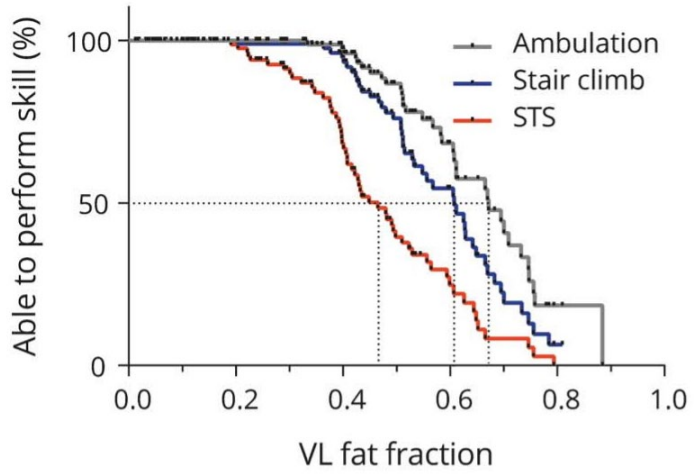
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Increasing Fat Fraction Is Associated with Functional Decline

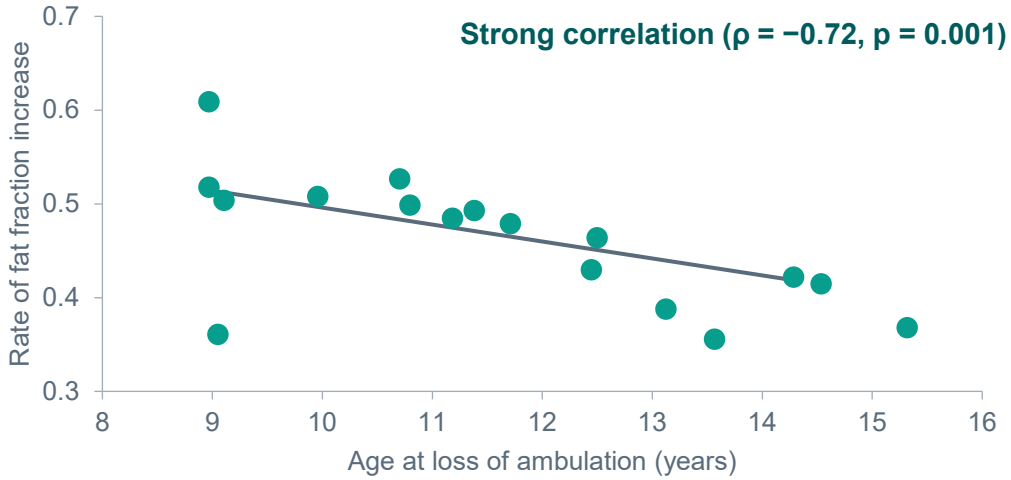
As vastus lateralis fat fraction increases, boys with DMD lose function, and a faster increase is associated with earlier loss of ambulation.

As fat fraction increases, function is lost



The percent of boys able to rise from the floor (STS), climb stairs and walk by vastus lateralis (VL) fat fraction

Faster increase, earlier loss of ambulation



Boys with a faster increase in vastus lateralis fat fraction lose ambulation earlier²

KEY TAKEAWAY: Forazapadin's novel mechanism supports the use of MRI, which is aligned with muscle preservation and regeneration rather than dystrophin expression

1. Barnard AM, et al. Neurology 2020. 2. Naarding KJ, et al. Neurology 2020.

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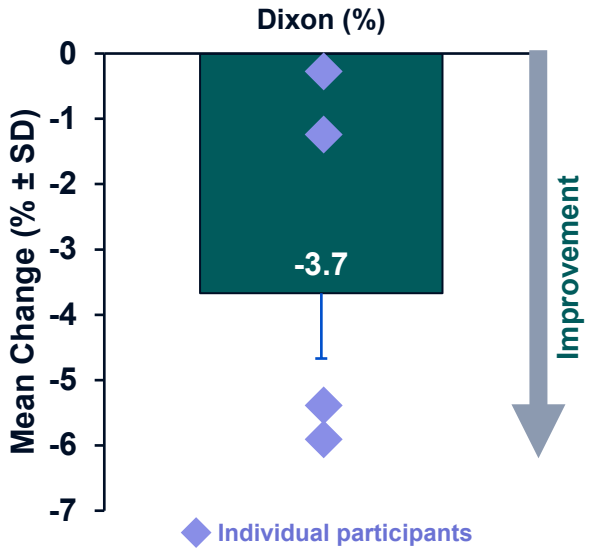
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TRAILHEAD Showed Decrease in Biceps Brachii Fat Fraction at 5 Months

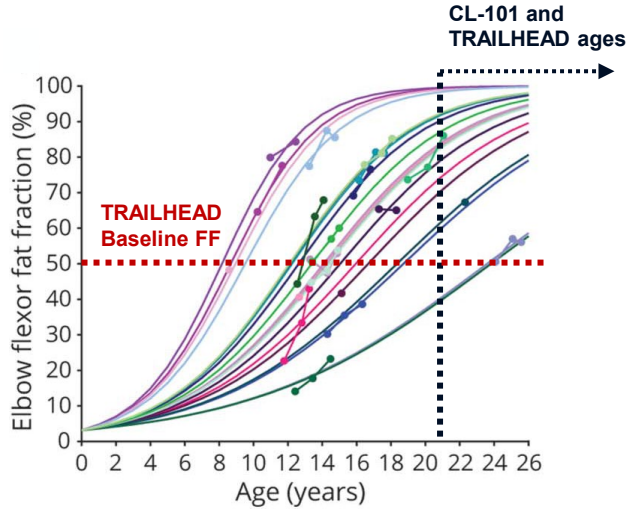
Individual Values and Changes

	Biceps Brachii MRI Fat Fraction (%)		
	Baseline	Month 5	Change
Participant 1	81.4%	75.3%	-6.2%
Participant 2	35.3%	34.4%	-0.9%
Participant 3	27.9%	22.1%	-5.8%
Participant 4	54.1%	52.2%	-1.9%
Mean ± SD	49.7 ± 23.9%	46.0 ± 23.1%	-3.7 ± 2.7%

Mean Change



Natural History of Elbow Flexor Fat-Fraction[1]



[1]Naarding KJ, et al. Neurology 2021. 97:e1737-e1742
Elbow flexor muscles included biceps and brachialis muscles

KEY TAKEAWAY

We believe there may be an even greater opportunity to observe a meaningful treatment effect in BASECAMP participants

TRAILHEAD data presented at the 2026 International Congress on Neuromuscular Diseases (ICNMD).

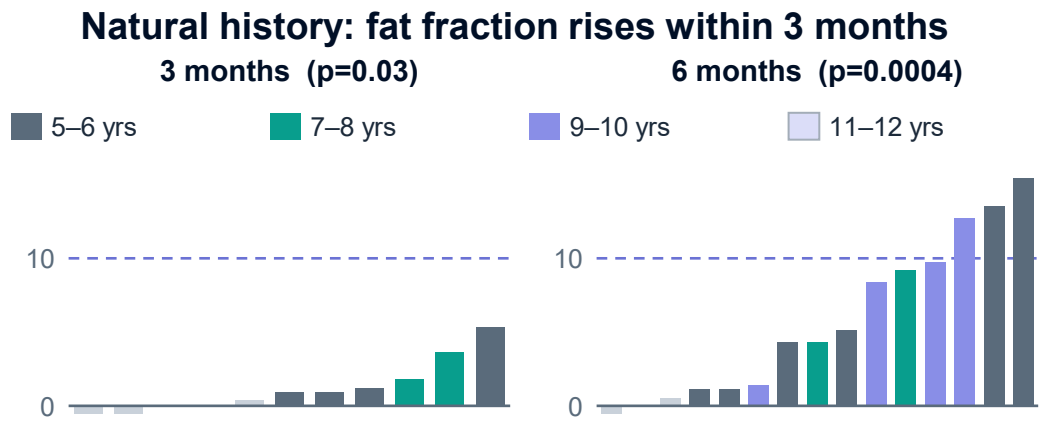
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Rationale for Assessing Muscle Composition at 12 Weeks

Biology, preclinical work and early human data support a measurable signal within the controlled period.

1. Muscle regeneration can begin within about one month of injury
2. Increases in fat fraction detectable over 3-months in boys with Duchenne
3. Biological responses in muscle observed over short periods in forazapadin-treated mdx mice and DMD dogs
4. Increases in grip strength observed within 28 days for treatment with forazapadin in Phase 1b study (CL-101)



Change in vastus lateralis fat fraction (percentage points), individual untreated boys. Adapted from Willcocks RJ, et al. Ann Neurol 2016.

Untreated, fat fraction rises within months in boys with DMD — so, **12 weeks may be enough** to see a difference.

~10% annual rise in vastus lateralis fat fraction in boys who already have 10–40% fat fraction[1]

KEY TAKEAWAY: Meeting the primary endpoint means a favorable fat fraction difference vs. placebo with acceptable safety; supportive trends in clinical endpoints would strengthen the evidence for approval.

1. Barnard AM, et al. Neurology 2020.

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MRI Fat Fraction May Support Potential Accelerated Approval Pathway

Type C interaction

- FDA interactions informed the clinical and regulatory strategy
- Amended BASECAMP protocol submitted to change primary endpoint
- Continued engagement planned to define path to potential approval

Totality of the evidence

- MRI fat fraction alongside clinical endpoints, biomarkers and other measures
- BASECAMP together with supporting TRAILHEAD data
- Longer-term follow-up in all participants
- Favorable safety profile

Treatment objective

- Evaluate stabilization or reduction in fat fraction versus placebo
- Assess supportive changes in clinical endpoints
- Interpret the totality, magnitude and consistency of the findings

KEY TAKEAWAY: Expect MRI fat fraction, clinical endpoints and totality of evidence to inform future regulatory discussions.

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BASECAMP Protocol, Readout and Longer-Term Follow-up

Global, randomized, double-blind, placebo-controlled Phase 2 study in 53 ambulatory boys aged 7, 8 and 9.

53 boys
17 sites · 7 countries

12 weeks · forazapadin (2 doses) vs. placebo
randomized, double-blind · topline analysis

36 weeks · all on forazapadin
longer-term follow-up

PRIMARY ENDPOINTS (AMENDED)

- **Safety and tolerability**
- **MRI fat fraction at 12 weeks** (primary efficacy)
- **Secondary:** dynamometry, physical activity, function and biomarkers
- **All participants** continue into longer-term evaluation

EXPECTED Q1 2027 TOPLINE

- **12 weeks:** fat fraction vs. placebo in all participants
- **Safety, tolerability** and available secondary measures
- **6 months:** supportive data from a subset at data cut

KEY TAKEAWAY: Anticipated Q1 2027 readout expected to center on the 12-week, placebo-controlled fat fraction comparison.

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Key Takeaways and Upcoming Milestones

An objective primary efficacy endpoint, a defined readout and continued regulatory engagement.

Key takeaways

1. MRI fat fraction is now BASECAMP's primary efficacy endpoint.
2. TRAILHEAD showed reductions in fat fraction among the four participants included in the interim analysis.
3. Functional measures remain, to assess benefit over time

Upcoming anticipated milestones

Q4 2026	TRAILHEAD 12-month data (first four participants)
Ongoing	TRAILHEAD enrollment updates
Q1 2027	BASECAMP topline data
Q1 2027	TRAVERSE enrollment begins (Phase 2, FSHD)

KEY TAKEAWAY: BASECAMP's anticipated Q1 2027 readout expected to inform the program's clinical and regulatory path.

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THANK YOU

Q&A

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THANK YOU

Reimagine

how muscle diseases are treated

Regenerate

muscle with an oral, once-daily medicine

Realize

a new future for patients and families