



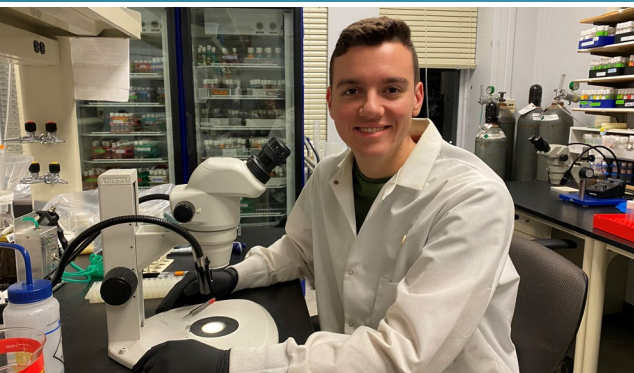
Investor & Analyst Event Series – Volume 9

# Transforming Facioscapulohumeral Muscular Dystrophy (FSHD)

## AOC 1020 FORTITUDE™ Phase 1/2 Initial Data

June 12, 2024

NASDAQ: RNA | [aviditybio.com](https://aviditybio.com)



# Forward-Looking Statements

We caution the reader that this presentation contains forward-looking statements that involve substantial risks and uncertainties. All statements other than statements of historical fact contained in this presentation are forward-looking statements. Forward-looking statements include, but are not limited to, statements regarding: our business strategy; the anticipated timing, design and conduct of our ongoing clinical trials; the timing of release of data from our ongoing clinical programs; the characterization of data and results from clinical trials, and conclusions drawn therefrom; research and development plans; plans and projected timelines for del-desiran (AOC 1001), del-brax (AOC 1020) and AOC 1044; plans and timing of adding cohorts for the FORTITUDE trial and such cohorts potentially serving as a basis of registration; safety and tolerability, and functional benefit, of our product candidates; the status and potential of del-brax as a first-in-class, best-in-class treatment for FSHD; our plans to develop programs that are currently pre-clinical, and the timing thereof; the potential of the AOC platform; the ability of our product candidates to treat rare diseases; timing and likelihood of success; prospective products; product approvals; plans and objectives of management for future operations; and future results of anticipated product development efforts. In some cases, the reader can identify forward-looking statements by terms such as “may,” “will,” “should,” “expect,” “plan,” “anticipate,” “could,” “intend,” “target,” “project,” “contemplates,” “believes,” “estimates,” “predicts,” “potential” or “continue” or the negative of these terms or other similar expressions. The inclusion of forward-looking statements should not be regarded as a representation by Avidity that any of our plans will be achieved. Actual results may differ from those set forth in this presentation due to the risks and uncertainties inherent in our business and beyond our control, including, without limitation: we may not be able to fully resolve the partial clinical hold related to del-desiran; additional requests for data in connection with the partial clinical hold or otherwise may result in significant additional expense and timing delays; data delivered to the FDA in connection with the partial clinical hold may not be satisfactory to the FDA; preliminary results of a clinical trial are not necessarily indicative of final results; additional participant data related to del-brax that continues to become available may be inconsistent with the data produced as of the most recent date cutoff, and further analysis of existing data and analysis of new data may lead to conclusions different from those established as of such date cutoff; unexpected adverse side effects or inadequate efficacy of our product candidates may delay or limit their development, regulatory approval and/or commercialization, or may result in additional clinical holds which may not be timely lifted, recalls or product liability claims; our planned additional cohorts in the FORTITUDE trial may not support the registration of del-brax; we are early in our development efforts; our approach to the discovery and development of product candidates based on our AOC platform is unproven, and we do not know whether we will be able to develop any products of commercial value; the success of our preclinical studies and clinical trials for our product candidates; the results of early clinical trials are not necessarily predictive of future results; potential delays in the commencement, enrollment, data readouts and completion of preclinical studies or clinical trials; our dependence on third parties in connection with preclinical and clinical testing and product manufacturing; we may not realize the expected benefits of our collaborations with third parties, our existing collaborations may terminate earlier than expected or we may not be able to form new collaborations; regulatory developments in the United States and foreign countries, including acceptance of INDs and similar foreign regulatory submissions and our proposed design of future clinical trials; Fast Track and Breakthrough Therapy designations by the FDA may not lead to a faster development or regulatory review or approval process; our ability to obtain and maintain intellectual property protection for our product candidates and proprietary technologies; we may exhaust our capital resources sooner than we expect and fail to raise additional needed funds; and other risks described in our filings with the SEC, including under the heading “Risk Factors” in our Form 10-K for the year ended December 31, 2023, filed with the SEC on February 28, 2024, and in subsequent filings with the SEC. The reader is cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise. All forward-looking statements are qualified in their entirety by this cautionary statement, which is made under the safe harbor provisions of the Private Securities Litigation Reform Act of 1995.

This presentation also contains estimates and other statistical data made by independent parties and by us relating to market size and growth and other data about our industry. This data involves a number of assumptions and limitations, and the reader is cautioned not to give undue weight to such estimates. In addition, projections, assumptions, and estimates of our future performance and the future performance of the markets in which we operate are necessarily subject to a high degree of uncertainty and risk. These and other factors could cause results to differ materially from those expressed in the estimates made by the independent parties and by us. This presentation shall not constitute an offer to sell or the solicitation of an offer to buy securities, nor shall there be any sale of securities in any state or jurisdiction in which such offer, solicitation or sale would be unlawful prior to registration or qualification under the securities laws of any such state or jurisdiction.



Amy  
Living with FSHD

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## OUR VISION

**To profoundly improve  
people's lives by  
revolutionizing  
the delivery of  
RNA therapeutics**

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# Revolutionizing New Class of Targeted RNA Therapeutics

AOC platform delivers reproducible, consistent data across muscle disease programs

Rare Muscle Disease Therapies in Development	Clinical Program	Safety & Tolerability	Delivery to Muscle	Target Engagement	Functional Improvement
<b><i>Del-desiran</i></b> Myotonic Dystrophy Type 1 (DM1)	 MARINA <sup>®</sup> MARINA <sup>OLE</sup> <sup>™</sup> HARBOR <sup>™</sup>	<input checked="" type="checkbox"/>	<input checked="" type="checkbox"/>	<input checked="" type="checkbox"/>	<input checked="" type="checkbox"/>
<b><i>Del-brax</i></b> Facioscapulohumeral Muscular Dystrophy (FSHD)	 FORTITUDE <sup>™</sup>	<input checked="" type="checkbox"/>	<input checked="" type="checkbox"/>	<input checked="" type="checkbox"/>	<input checked="" type="checkbox"/>
<b>AOC 1044</b> Duchenne Muscular Dystrophy (DMD44)	 explore 44 <sup>™</sup>	<input checked="" type="checkbox"/>	<input checked="" type="checkbox"/>	<input checked="" type="checkbox"/>	

# Purpose of Update

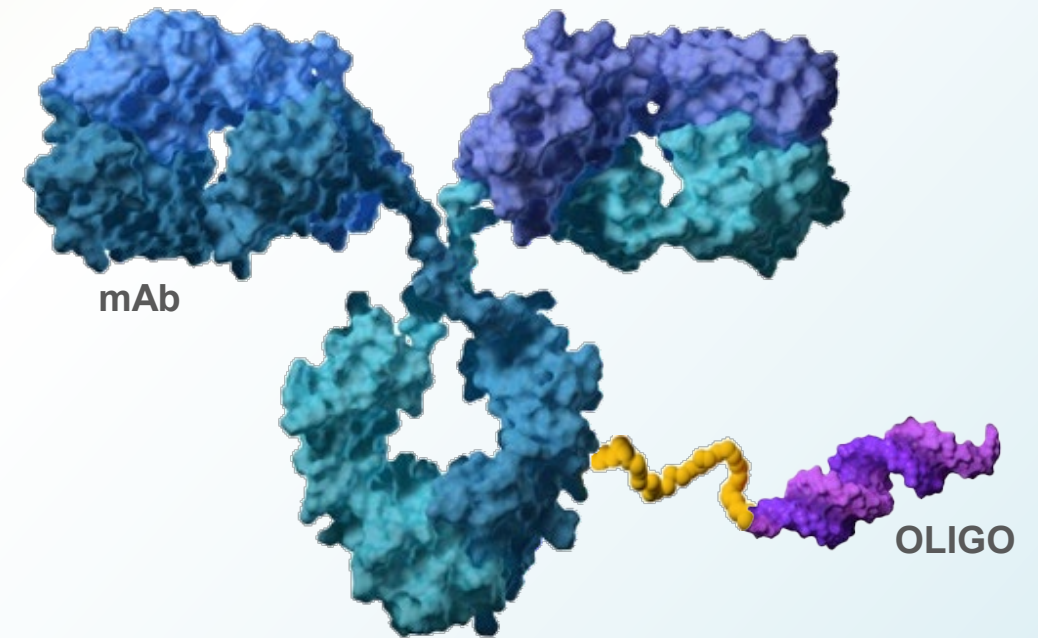
## Present Initial Data from Phase 1/2 FORTITUDE *Del-brax 2 mg/kg at 4 months*

- Unprecedented and consistent reduction in DUX4-regulated genes
- Signs of functional improvement: muscle strength; increased reachable workspace; positive patient and clinician reported outcomes
- Favorable safety and tolerability

## Accelerating *Del-brax* toward Approval *Positive data compels expedited program*

- Phase 1/2 fully enrolled
- Updated plans to initiate Phase 3 cohorts
  - Biomarker cohort in 2H 2024
  - Functional cohort in 1H 2025

*delpacibart braxlosiran*  
abbreviation: *del-brax*  
(formerly known as AOC 1020)



# Del-brax: Transforming the Treatment of FSHD

First-in-class and best-in-class: greater than 50% reduction in DUX4 regulated genes

Unprecedented & Consistent Reduction in DUX4 Regulated Genes	Signs of Functional Improvement and Reported Outcomes	Favorable Safety and Tolerability
<ul style="list-style-type: none"><li>• Greater than 50% reduction across multiple DUX4 gene panels</li><li>• All treated participants showed reductions greater than 20%</li><li>• Reduction of a newly-identified DUX4 circulating biomarker &amp; creatine kinase</li></ul>	<ul style="list-style-type: none"><li>• Improved muscle strength</li><li>• Increased reachable workspace compared to placebo and natural history study</li><li>• Positive patient and clinician reported outcomes</li></ul>	<ul style="list-style-type: none"><li>• All adverse events (AEs) were mild or moderate</li><li>• No serious AEs, No severe AEs</li><li>• No discontinuations</li></ul>

**Accelerating *Del-brax* Toward Approval**

## AVIDITY MANAGEMENT TEAM



**Sarah Boyce**  
President & CEO



**Steve Hughes, M.D.**  
Chief Medical Officer



**W. Michael Flanagan, Ph.D.**  
Chief Scientific & Technical Officer



**Geoff Grande, CFA**  
VP, Investor Relations & Corporate  
Communications

## GUEST SPEAKER



**Jeffrey M. Statland, M.D.**  
Professor of Neurology,  
University of Kansas Medical Center



# Transforming the Treatment of FSHD

# Agenda/Outline

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**Revolutionizing the Delivery of RNA**

**Sarah Boyce**, President & CEO

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**Delivering for People Living with FSHD**

**Steve Hughes, M.D., CMO**

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**Leading the Field: Directly Targeting DUX4**

**Michael Flanagan, Ph.D., CSO & CTO**

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**FORTITUDE™ Preliminary Data Assessment**

**Jeffrey M. Statland, M.D.**, Professor of Neurology, University of Kansas Medical Center

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**Closing Remarks**

**Sarah Boyce**, President & CEO

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**Q&A Session**

**Avidity Management & Dr. Statland**  
Moderator: **Geoff Grande, CFA**, VP of IR/CC

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# Facioscapulohumeral Muscular Dystrophy (FSHD)

Rare, hereditary disorder causing relentless loss of muscle function and progressive disability

~16,000 - 38,000

PEOPLE WITH FSHD IN THE US

0

APPROVED THERAPIES

- One of the most common forms of muscular dystrophy
- FSHD causes progressive muscle weakness, pain, fatigue and disability
- Onset typically occurs in teenage or early adult years
- Steady loss of independence and ability to care for oneself
  - 20% of patients become wheelchair dependent
- Autosomal dominant\* - multiple generations can be affected
  - 20-30% arise from spontaneous mutations
- **Del-brax**: designed to address root cause of FSHD by directly targeting double homeobox 4 (DUX4)



Russell  
Living with FSHD

# Phase 1/2 FORTITUDE™ Trial

Initial data presented today from 2 mg/kg cohort at 4 months

## Key Information

- Randomized, double blinded, placebo controlled
- Age 18-65
- 12-month multiple dose treatment/follow-up period
- Biopsies at Month 4

## Cohort

- Cohort A\*: First dose at 1 mg/kg; all subsequent doses at 2 mg/kg

## Primary & Secondary Objectives

- Safety and tolerability of ascending doses of *del-brax* in participants with FSHD
- Pharmacokinetics

## Key Exploratory Objectives

- Pharmacodynamics
  - Biomarkers
- Measures of clinical activity
  - Muscle strength
  - Muscle function
  - Muscle composition (MRI)
- Patient and Clinician reported outcomes

# Baseline Demographics Generally Well Matched Between Groups

	Cohort A Placebo N=4 % or mean (SD)	<i>Del-brax</i> 2 mg/kg* N=8 % or mean (SD)
Sex, % Male	75	62.5
Age, years	53.5 (10.15)	51.6 (11.62)
Genetic Diagnosis, % FSHD 1	100	100
FSHD Clinical Score	9.3 (1.71)	9.3 (2.31)
D4Z4 Repeat Number	5.0 (2.45)	5.8 (2.60)
Age at First Symptom Onset (y)	25.3 (13.5)	28.6 (17.75)
Reachable Workspace RSA with weight (Q1+Q3)	0.118 (0.0661)	0.088 (0.0598)
Reachable Workspace RSA without weight (Q1+Q3)**	0.156 (0.0810)	0.138 (0.0750)
Quantitative Muscle Testing - Percent Predicted Normal	33.97 (16.42)	30.14 (11.58)

\*Participants receive a first dose of 1mg/kg and then receive the 2mg/kg dose for the remainder of the study

\*\*Participants in FORTITUDE had >50% reduction in reachable workspace in Q1 & Q3 at baseline compared to normal controls (normal controls RWS (Q1+Q3) without weight: ~0.39, Han et al, 2015 Muscle Nerve)

Reachable Workspace (RWS) Relative Surface Area (RSA) (Q1+Q3) with or without weight was calculated using the average of both arms

# Del-brax: Favorable Safety and Tolerability

Subjects with $\geq 1$ AE n (%)	Placebo N=13	2 mg/kg* N=8	4 mg/kg N=18
Any AE	11 (84.6%)	8 (100%)	17 (94.4%)
Related to study drug	3 (23.1%)	4 (50%)	9 (50%)
Severe AE	0	0	0
Serious AE (SAE)	0	0	0
AE leading to study discontinuation	0	0	0
AE leading to death	0	0	0

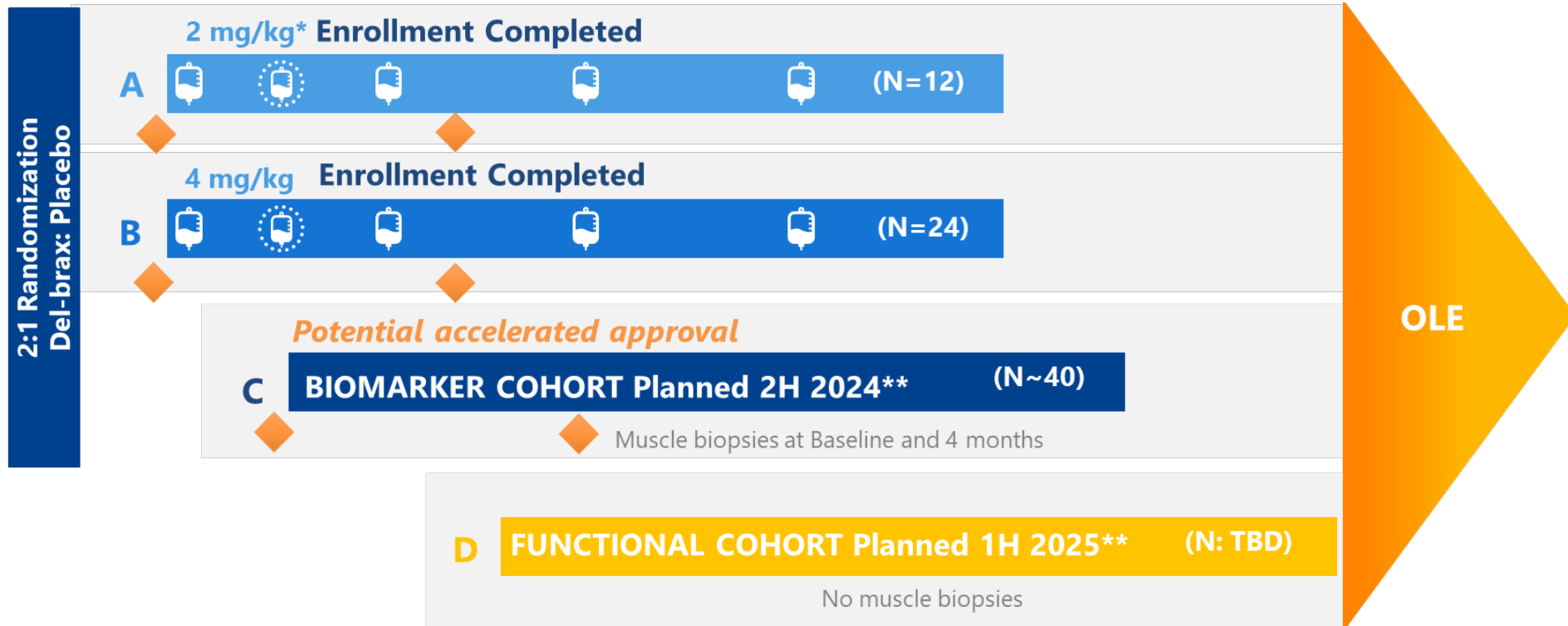
As of May 2024, data from FORTITUDE

## All 39 patients enrolled remain in study

- No serious adverse events (AE), no severe AE
- No discontinuations
- All AE were mild or moderate
- Most common related AE occurring in 2 or more participants:
  - Fatigue
  - Rash
  - Hemoglobin decreased/anemia
  - Chills

# Accelerating *Del-brax* Registrational Plan

## Pulling forward registrational cohorts



Dose Booster Multidose quarterly with 1 booster after first 6 weeks; Dose listed is siRNA Muscle biopsies to be performed at Baseline and 4 months

\* Participants receive a first dose of 1mg/kg and then receive the 2mg/kg dose for the remainder of the study

\*\*Dose and schedule to be determined in Q3 2024

# Agenda/Outline

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**Closing Remarks**

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**Q&A Session**

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# Del-brax: Transforming the Treatment of FSHD

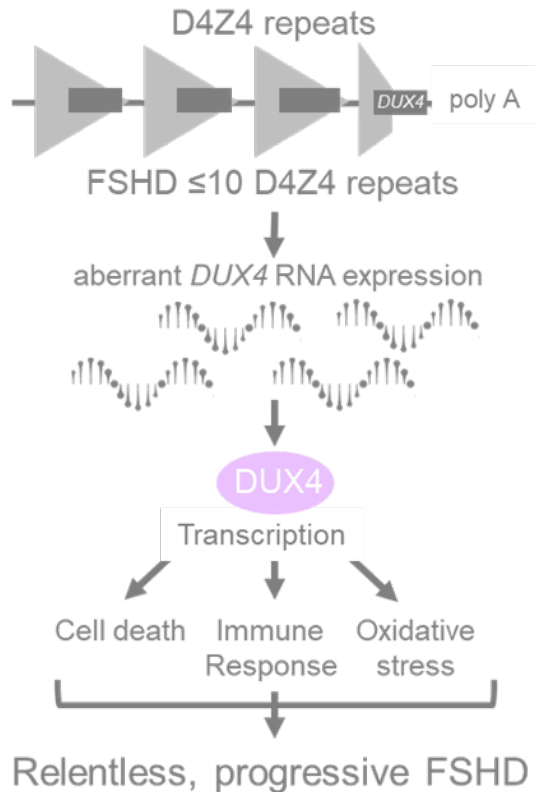
## Connecting the dots from delivery to function

 <b>Delivery to Muscle</b>	 <b>Reduction in DUX4 Gene Signature</b>	 <b>Impact on FSHD Disease Biology</b>	 <b>Decrease in Novel Circulating Biomarker</b>
<ul style="list-style-type: none"><li>• Consistent and effective delivery of siRNA to muscle</li><li>• Reinforcing the disruptive and broad potential of our AOC platform</li></ul>	<ul style="list-style-type: none"><li>• 53% reduction in Avidity selected DUX4-regulated gene panel</li><li>• Consistent &gt;50% decrease in DUX4-regulated genes across multiple gene panels</li></ul>	<ul style="list-style-type: none"><li>• Broad biological effects across hundreds of RNA transcripts</li></ul>	<ul style="list-style-type: none"><li>• Substantial 25% decrease in a novel DUX4-regulated protein discovered in patient plasma</li><li>• 30% reduction in creatine kinase, an indicator of muscle damage</li></ul>

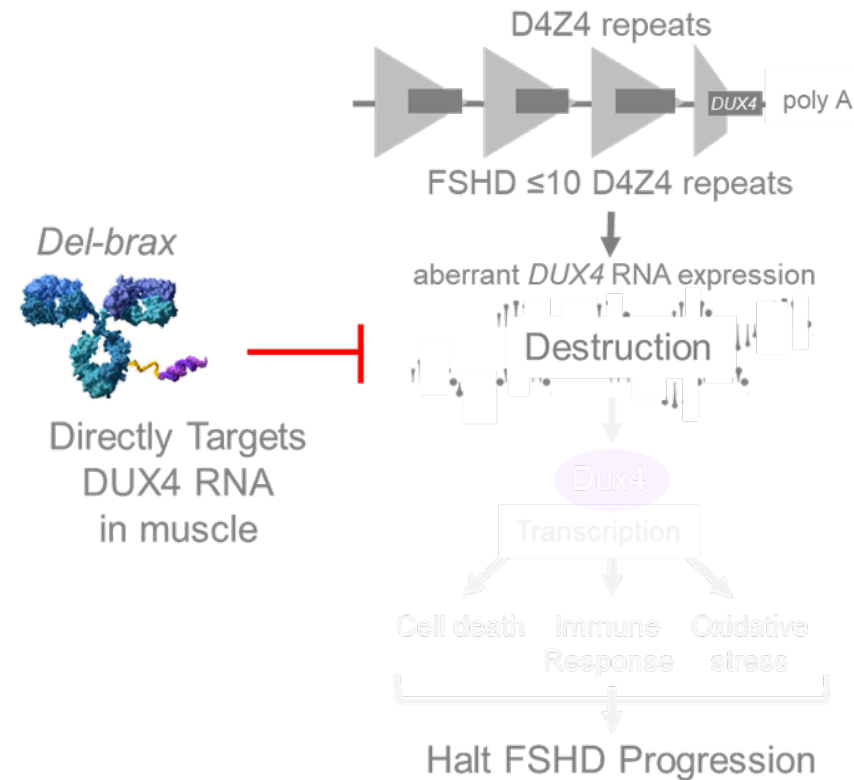
# Del-brax: Targets DUX4, the Root Cause of FSHD

Targets aberrant expression of DUX4 mRNA for destruction

## FSHD disease pathology<sup>1,2</sup>

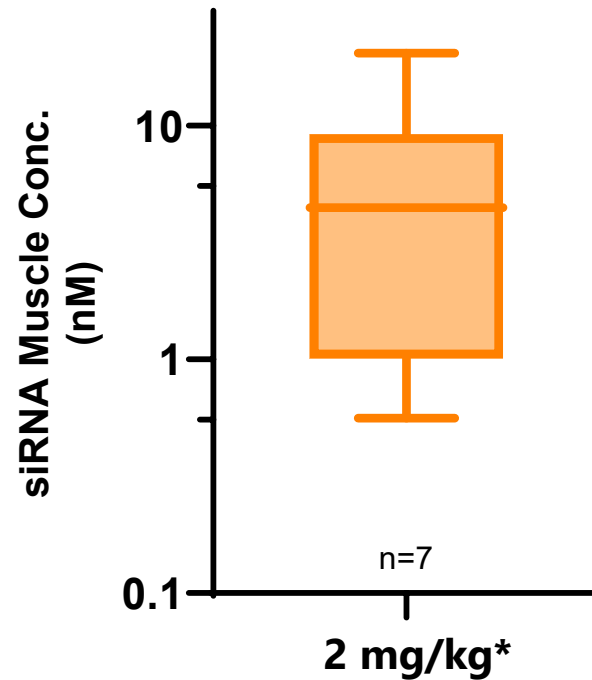


## Del-brax Therapeutic Hypothesis





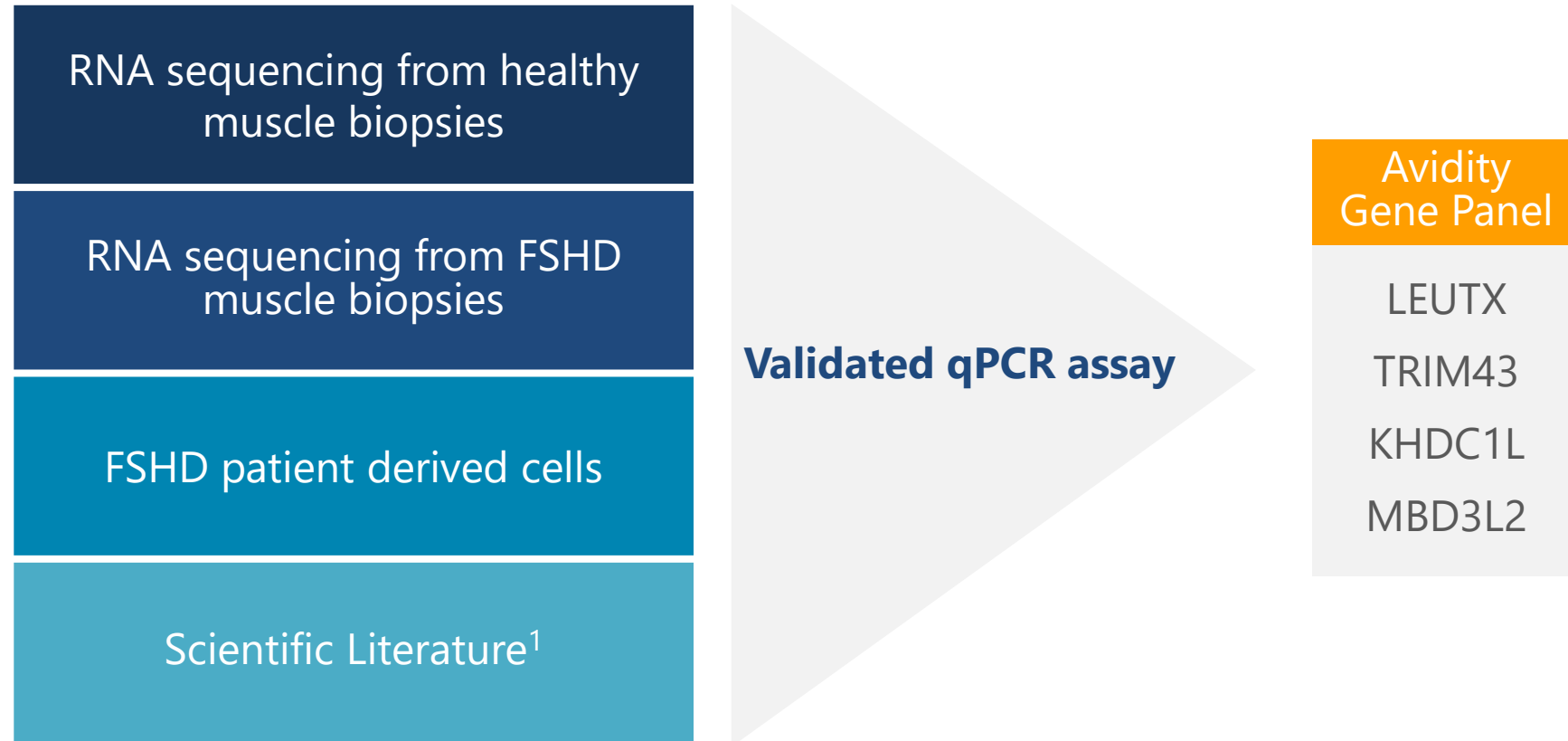
# Del-brax: Consistent and Effective Delivery of siRNA to Muscle



Muscle biopsies were collected in leg muscles (vastus lateralis, vastus medialis, tibialis anterior, gastrocnemius medialis or gastrocnemius lateralis) with fat fraction 15-40%, 4 weeks after 3rd dose. \*Doses were 1 mg/kg (D1), 2 mg/kg (D43 and D92). One participant in the del-brax treated group missed post-dose biopsy n=7

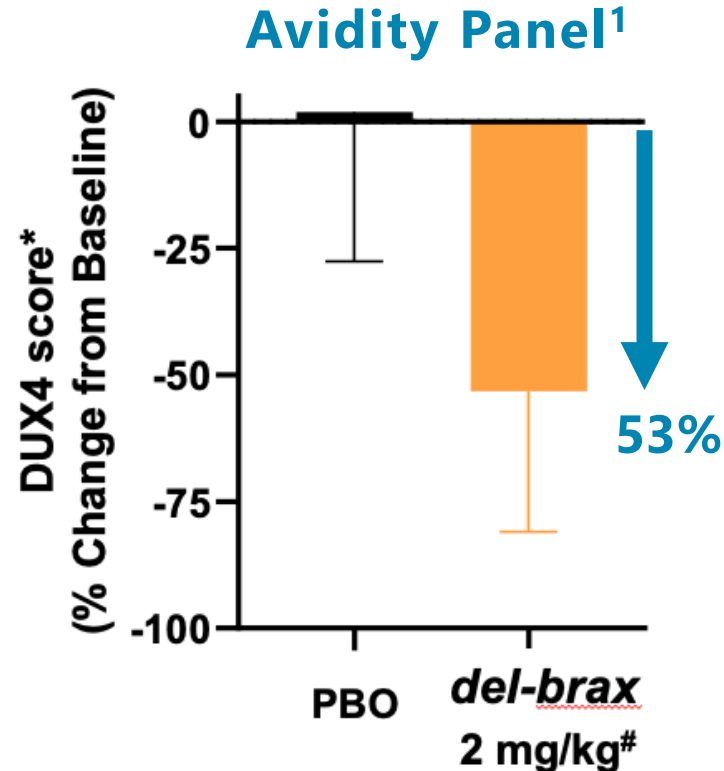
# DUX4-Regulated Genes Selected for Robustness and Reproducibility

Procured muscle biopsies, RNA sequencing, patient-derived cells informed the panel



# Del-brax Demonstrates Meaningful 53% Reduction in DUX4-regulated Genes

All *del-brax* treated participants showed reductions >20% in DUX4 regulated genes



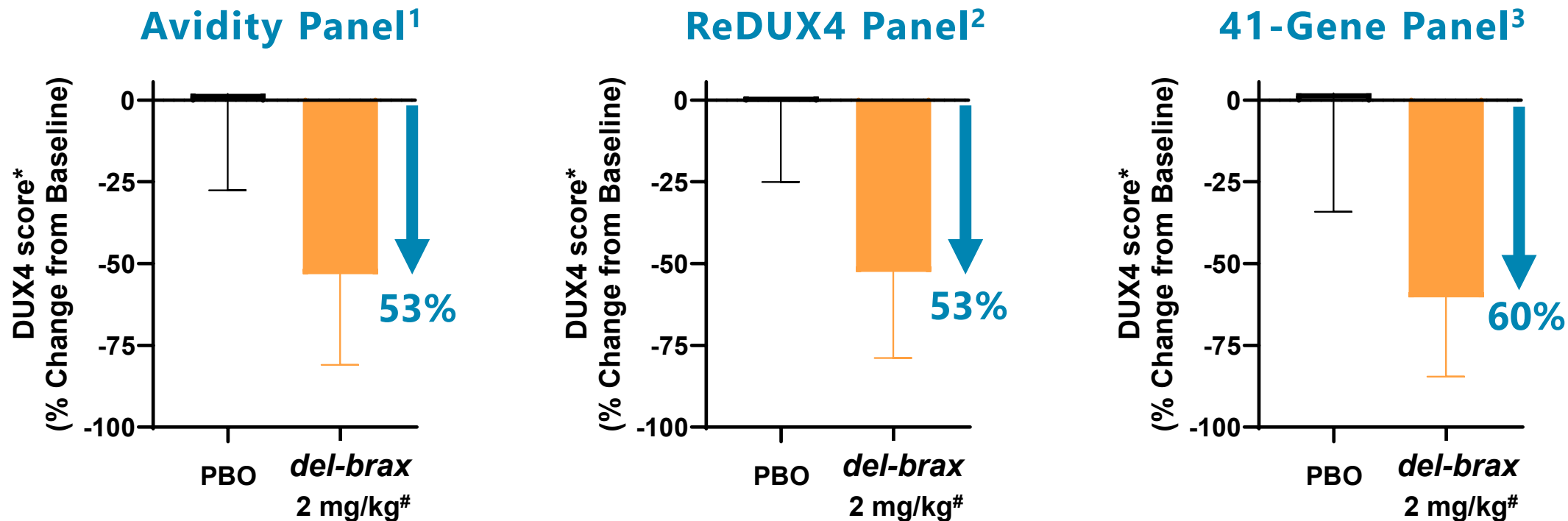
- All *del-brax* treated participants showed greater than 20% reduction in DUX4 regulated genes
- All muscle biopsies had evaluable DUX4 gene signatures at all timepoints
- MRI-informed muscle biopsies were successful

<sup>1</sup> Avidity 4-Gene panel (LEUTX, TRIM43, MBD3L2, KHDC1L, Reference genes: TBP, STATA5)

\* DUX4 score in MRI informed muscle biopsies were determined utilizing qPCR (Avidity panel). DUX4 score calculated as cumulative expression of each gene and data presented as change at 4M treatment relative to cohort normalized baseline. Mean +/- SEM, N=7 *del-brax*, N=4 PBO. One participant in treated group did not receive post-treatment biopsy.

# Doses were 1 mg/kg (D1), 2 mg/kg (D43 and D92) with biopsy 1 month after 3rd dose.

# Del-brax Shows Consistent >50% Reductions in DUX4-regulated Genes as Measured by Multiple Gene Panels



<sup>1</sup> Avidity 4-Gene panel (LEUTX, TRIM43, MBD3L2, KHDC1L; Reference genes: TBP, STATA5)

<sup>2</sup> ReDUX4 6-Gene panel (CCNA1, ZSCAN4, MBD3L2, KHDC1L, SLC34A2, PRAMEF6); Tawil, R. et al., *Lancet Neurol* **23**:477 (2024)

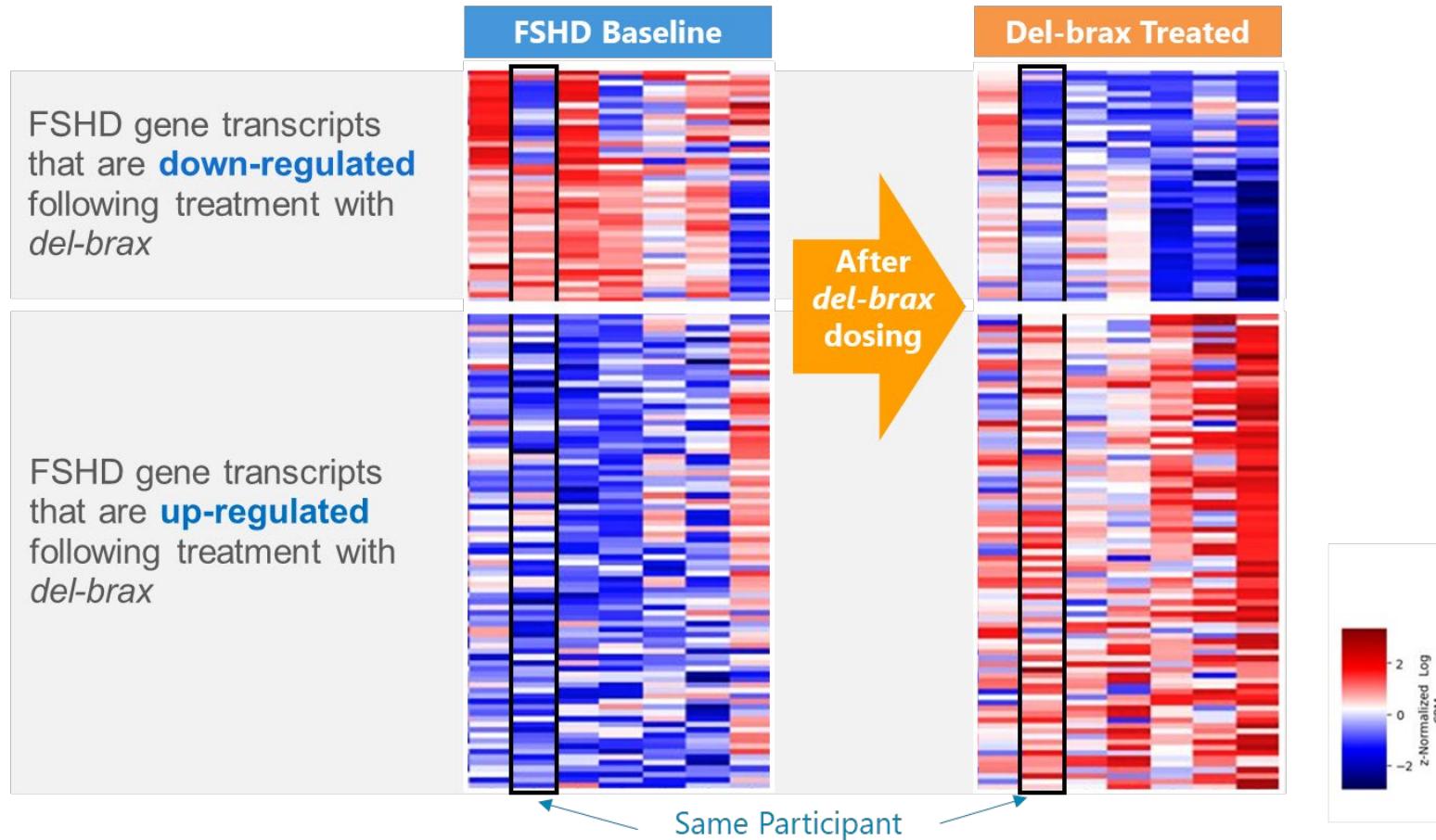
<sup>3</sup> Van den Heuvel, A. et al., *Scientific Reports* **12**:1426 (2022)

\* DUX4 score in MRI informed muscle biopsy were determined utilizing qPCR (Avidity panel) or RNASeq (ReDux and 41-Gene). DUX4 score calculated as cumulative expression of each gene and data presented as change at 4M treatment relative to cohort normalized baseline. Mean +/- SEM, N=7 del-brax, N=4 PBO. One participant in treated group did not receive post-treatment biopsy.

#Doses were 1 mg/kg (D1), 2 mg/kg (D43 and D92) with biopsy 1 month after 3rd dose.

# Del-brax Impacts Underlying FSHD Disease Biology

## Broad biological effects following *del-brax* treatment



Each column is a participant's disease signature at baseline compared to 1 month post 3<sup>rd</sup> dose

Differential gene expression (excluding DUX4 regulated genes) in muscle utilizing RNASeq.  
N=7 *del-brax* 1 mg/kg (D1), 2 mg/kg (D43 and D92). One participant missed post-dose biopsy.

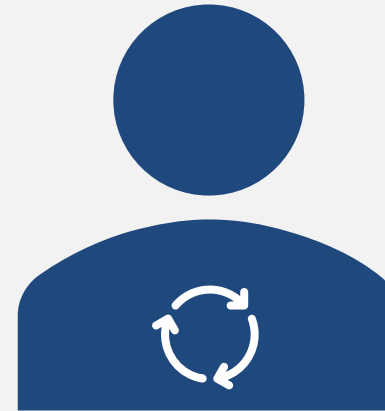
# Circulating Biomarkers Provide Early Detection of Whole-Body Response to *Del-brax* Treatment

## Muscle Biopsy



- Sampling of single muscle
- Limited timepoints
- Invasive

## Circulating Biomarker



- Comprehensive assessment throughout body
- Continuous monitoring
- Patient friendly

# Novel DUX4-Regulated Circulating Biomarker

## Potential accelerated approval endpoint

### Multi-year Discovery Process



FSHD & Healthy Biopsies



Plasma from FSHD & Healthy Volunteers



Advisors & Disease Expertise

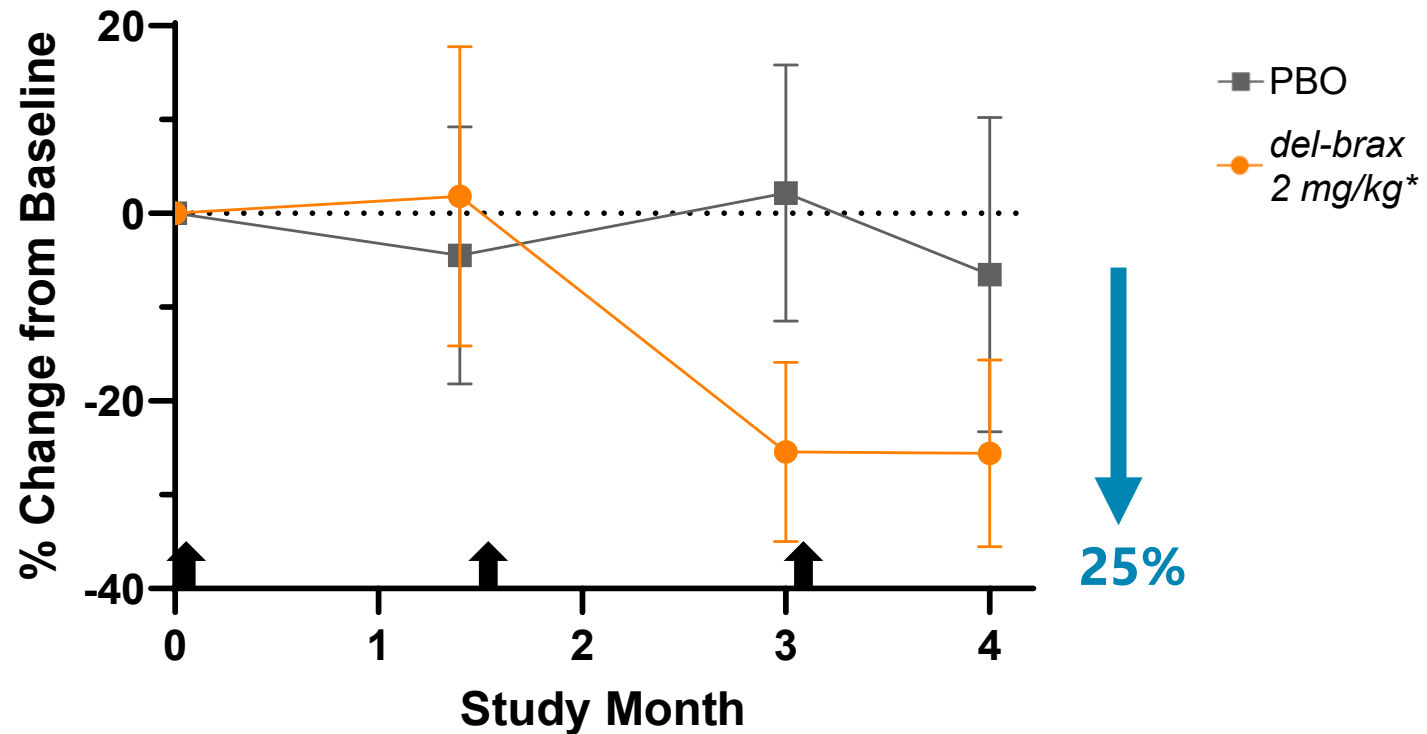
### Novel DUX4-Regulated Circulating Biomarker

#### *Potential Accelerated Approval Endpoint*

- Significantly elevated in patients with FSHD as compared to healthy individuals
- Allows rapid and continuous monitoring of how participants are responding to *del-brax*
- Non-invasive, patient-friendly
- Guides selection of dose regimen

# Del-brax Shows Early and Sustained Reduction of a Novel DUX4-Regulated Circulating Biomarker

**Del-brax treatment shows 25% reduction in circulating biomarker in participants with FSHD versus placebo**

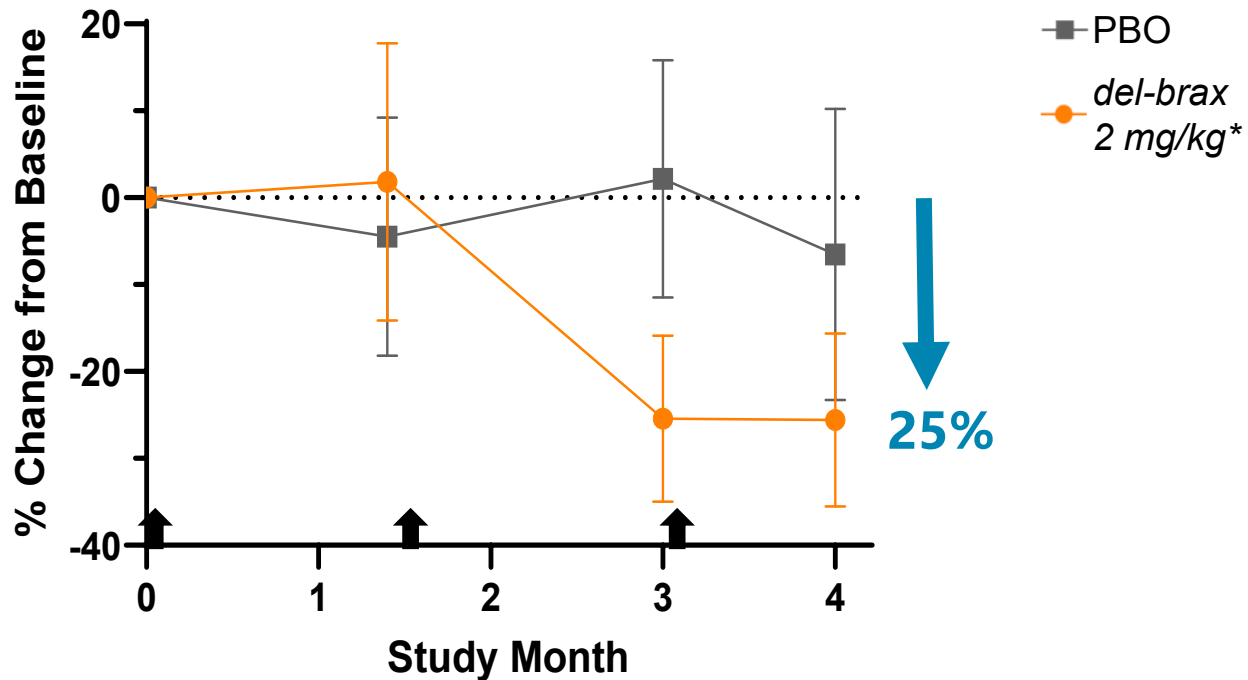




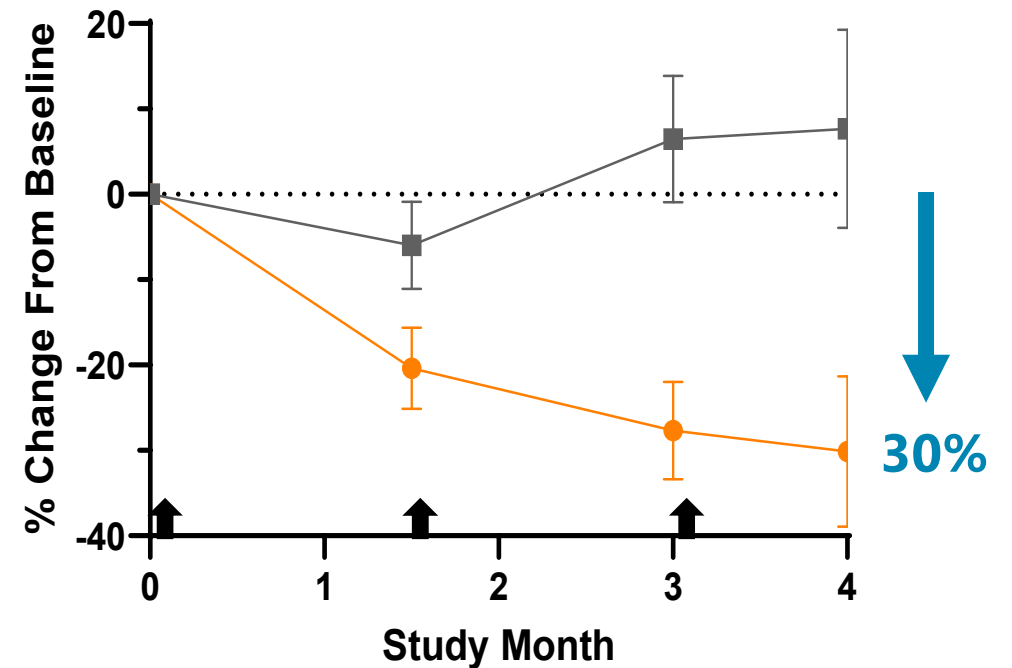
# Consistent and Confirmatory Decrease in Both Novel and Creatine Kinase Circulating Biomarkers

Decreases in creatine kinase, an indicator of muscle damage

## Novel DUX4-regulated biomarker







## Creatine kinase biomarker



# Del-brax: Transforming the Treatment of FSHD

## Connecting the dots from delivery to function

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# Jeffrey M. Statland, M.D.

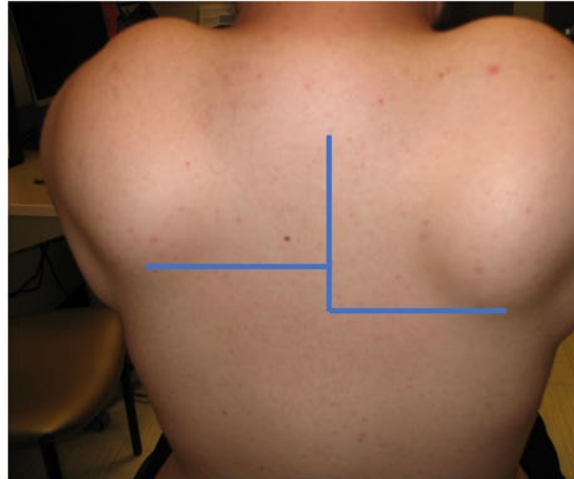
Professor of Neurology, University of Kansas Medical Center



Jeffrey M. Statland, M.D. is a Professor of Neurology at the University of Kansas Medical Center in Kansas City, Kansas. His research background has centered primarily on describing the natural history of and response to therapy for neuromuscular diseases. He completed a neuromuscular fellowship in Experimental Therapeutics of Neurological Diseases at the University of Rochester Medical Center and currently serves as principal investigator or co-investigator for research studies in Facioscapulohumeral Muscular Dystrophy (FSHD), Duchenne Muscular Dystrophy, Spinal Muscular Atrophy, and Myotonic Dystrophy. His specific research interest over the last 6 years has been preparing for clinical trials in FSHD. He has systematically analyzed the performance of strength and functional outcomes in prior FSHD clinical trials and compared to performance in a natural history study. He has worked with collaborators to develop new disease-relevant outcome measures to assess patient-reported disease burden, functional impairment, and physiological changes in muscle. He has obtained pilot data on the use of a number of novel outcomes for FSHD, including electrical impedance myography, a disease-specific functional rating scale, and a wireless motion analysis system in FSHD.

# Clinical Features of FSHD

- Inability to tightly close the eyes, flattened smile or inability to pucker
- Loss of scapular stabilizers leading to scapular asymmetry and inability to reach above the head
- Often asymmetric wasting in initially muscles of legs, TA and medial gastric, and later more proximal muscles
- Leads to instability of gait, steppage due to foot drop, throwing legs forward to lock knee, instability of the pelvis

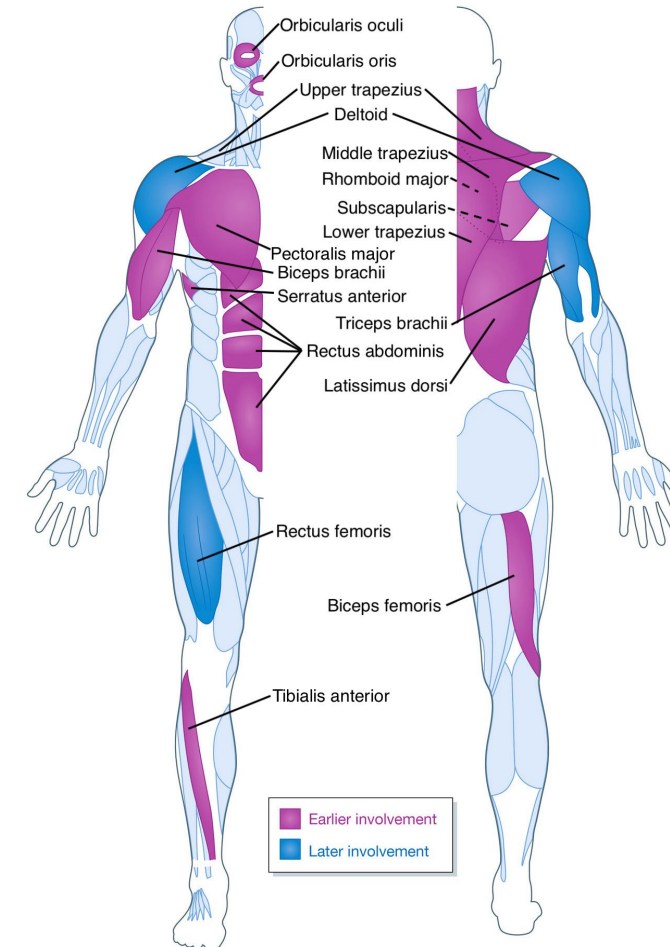


Rosenberg, R. N., & Pascual, J. M. (2020). Rosenberg's Molecular and Genetic Basis of Neurological and Psychiatric Disease: Volume 2 .

Huisinga J, et al. Muscle Nerve. 2018 Mar;57(3):503-506. doi: 10.1002/mus.25955. Epub 2017 Oct 6. PMID: 28877559; PMCID: PMC6631361.

# FSHD Causes Progressive Muscle Weakness

- Illustration shows typical early (pink) and later muscle involvement (blue)
- Approximately 60% of adults presenting symptom is inability to reach above the shoulder height
- The next most common presenting symptom in adults is foot drop
- About 20% of individuals will lose the ability to walk or maintain a job by the age of 50



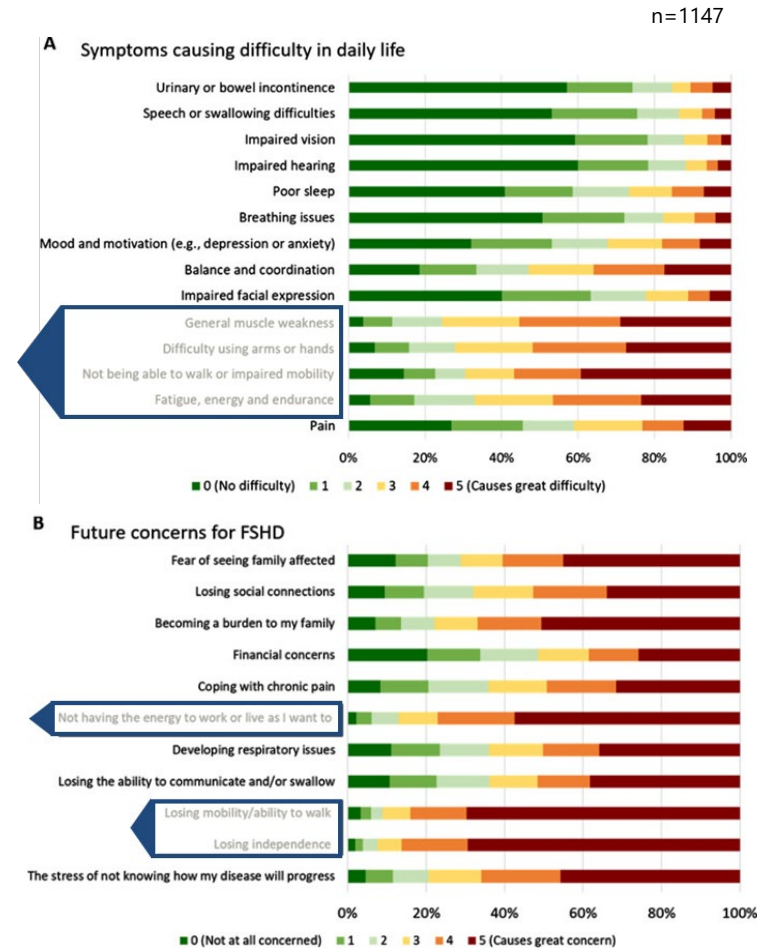
# Impact of Living with FSHD

- Large US and EU patient surveys have been consistent in results
- Prevalent and impactful symptom categories: motor strength or function and fatigue
- Future concerns: losing mobility and/or independence

**Therapies that improve muscle strength or function will be important for patients**

**General muscle weakness**  
**Difficulty using arms or hands**  
**Not being able to walk or impaired mobility**  
**Fatigue, energy and endurance**

**Not having the energy to work or live as I want to**  
**Losing mobility/ability to walk**  
**Losing independence**

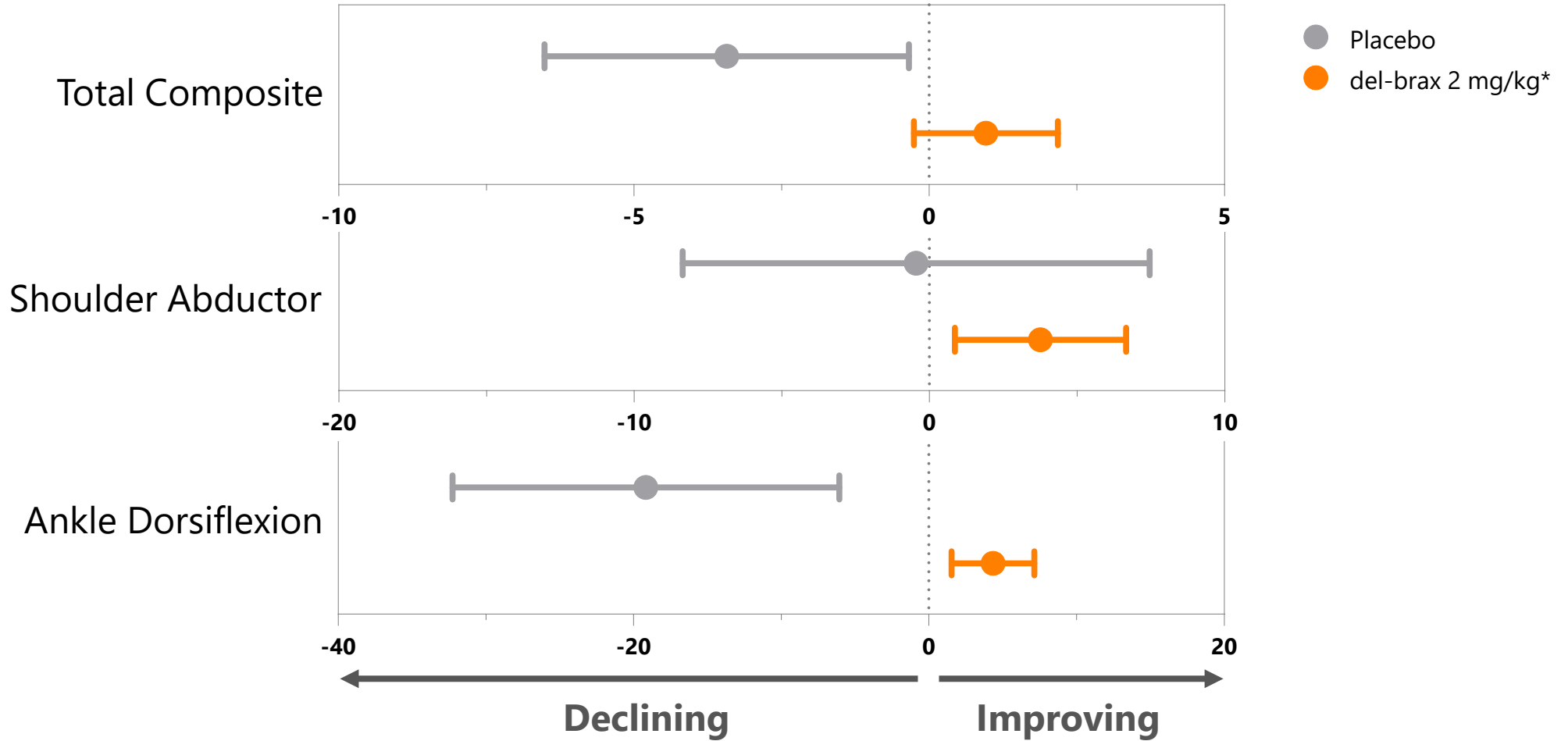




## **Exploratory Endpoints: Muscle Strength, Range of Motion and Function, Patient and Clinician Reported Outcomes**



# Del-brax Improved Muscle Strength in Both Upper and Lower Limb



# Reachable Workspace (RWS): Validated Measurement of Quantifying Improvement in Upper Extremity Range of Motion and Function

RWS correlates with ability to perform activities of daily living and maintain independence

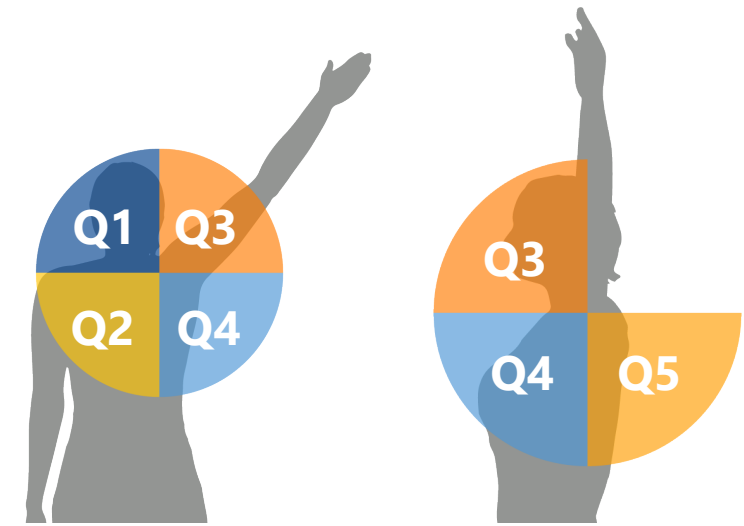
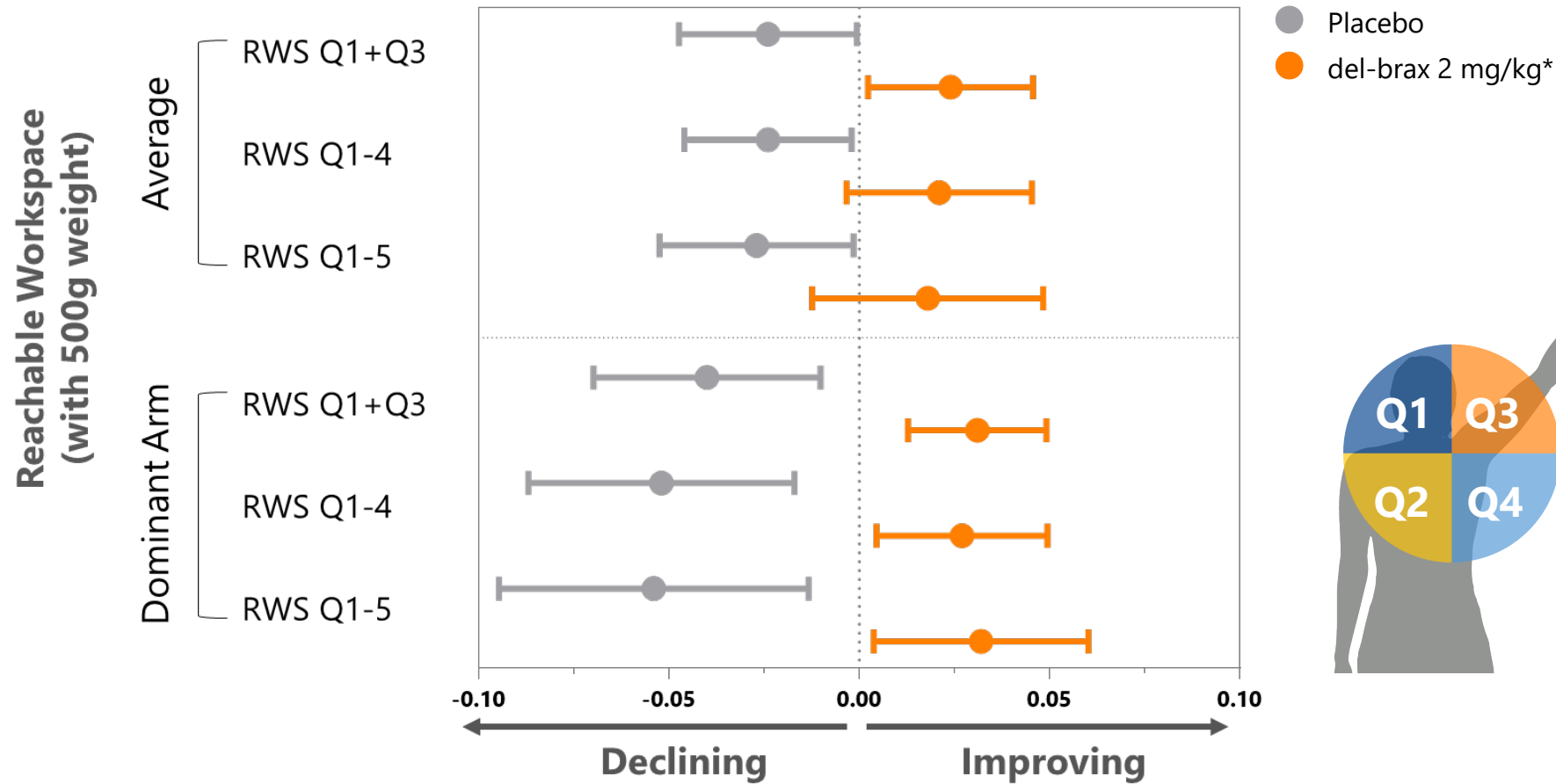


Video depicts healthy person demonstrating RWS



# Del-brax Improved Reachable Workspace Compared to Placebo

Improved range of motion and function; similar trends observed without weight



# ReSolve (NCT03458832): 24 Months Observational Study

- Clinical Trial Readiness to Solve Barriers to Drug Development in FSHD (ReSolve)
- FSHD Clinical Trial Research Network: at 11 centers in the US and EU
- Goal: to hasten drug development for FSHD by validating new clinical outcome assessments (COAs) and refining trial planning strategies
- COAs: FSHD Functional composite, electrical impedance myography, and **reachable workspace**
- Genetically defined, clinically affected, still able to walk independently: n=237 complete
- Visits: BL (day 1, day 2), 3, 12, 18, 24 months



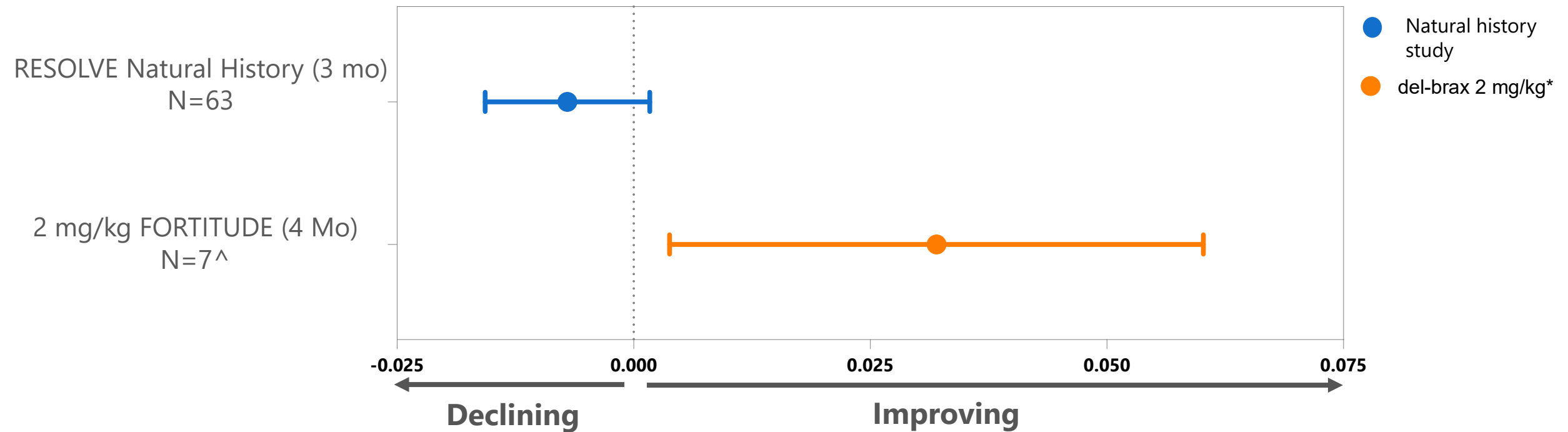
Current FSHD CTRN Members

FSHD CTRN Website



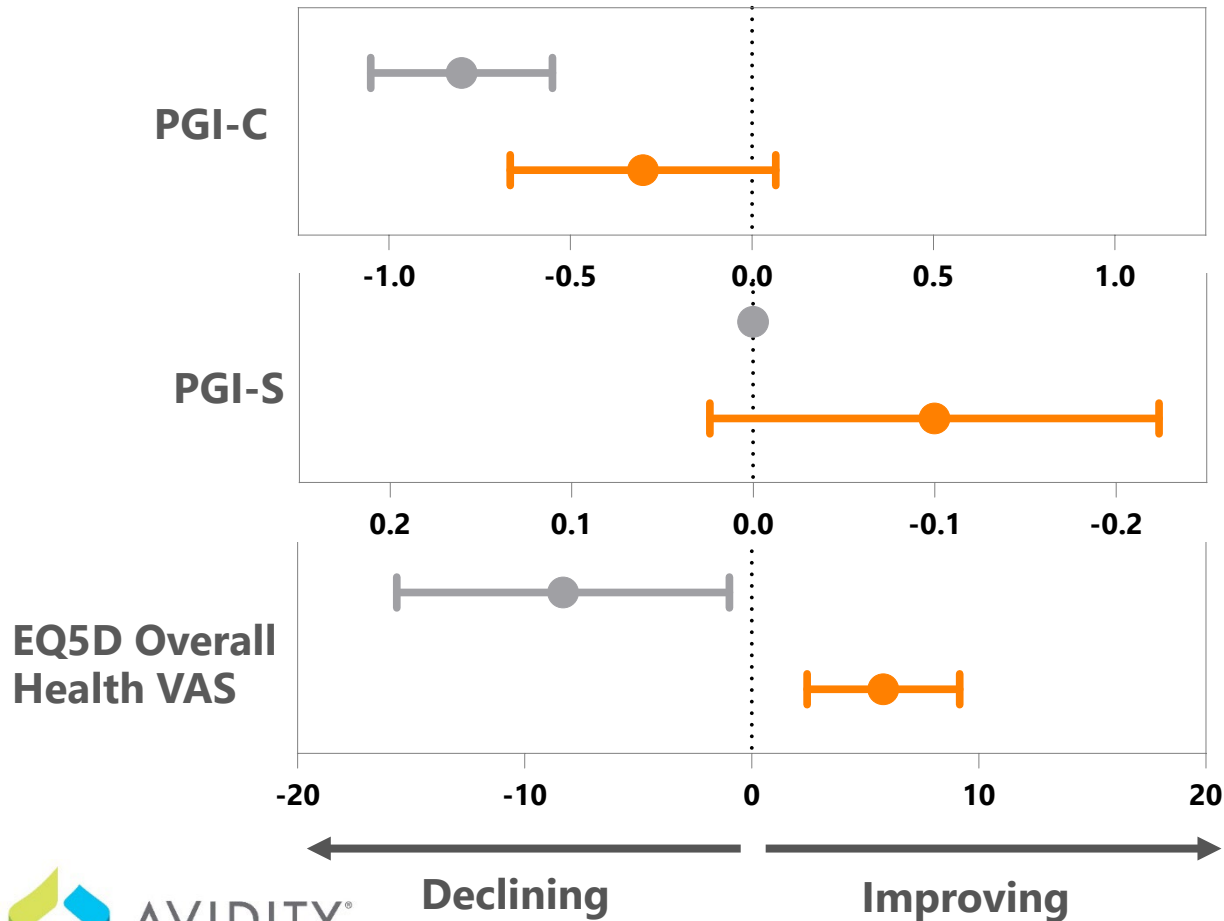
# Del-brax Improved Reachable Workspace Compared to Matched Natural History Data

## Reachable Workspace Q1-5; Dominant Arm; Weight: 500 g

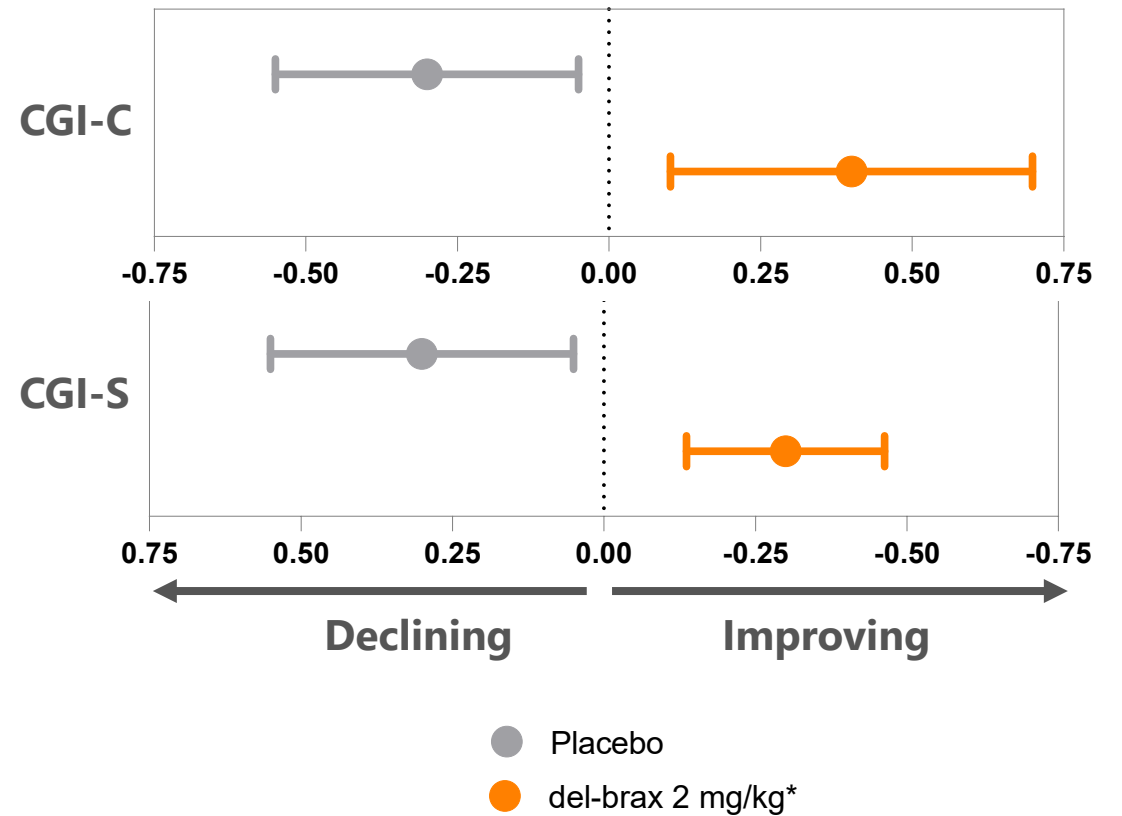


# Del-brax: Positive Trends Toward Improvement in Both Patient and Clinician Reported Outcome Measures

**Patient Reported Outcome Measures**  
Change from Baseline at Month 4 (SEM)



**Clinician Reported Outcome Measures**  
Change from Baseline at Month 4 (SEM)





# ***Del-brax*: Promising New Potential Treatment for Patients with FSHD**

## **First therapy to directly target DUX4 has potential to change course of disease**

- Effective muscle delivery with unprecedented and consistent >50% reduction in DUX4 regulated gene panels – impacting underlying FSHD disease biology
- Decrease in circulating biomarkers (novel and creatine kinase) indicate whole-body response
- Improvements in clinical measures of disease:
  - Muscle strength
  - Function: Reachable workspace compared to both placebo and natural history data
  - Patient and clinician reported outcomes
- Favorable safety and tolerability
- Looking forward to rapidly advancing FORTITUDE trial

# Agenda/Outline

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**Revolutionizing the Delivery of RNA**

**Sarah Boyce**, President & CEO

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**Delivering for People Living with FSHD**

**Steve Hughes, M.D.**, CMO

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**Leading the Field: Directly Targeting DUX4**

**Michael Flanagan, Ph.D.**, CSO & CTO

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**FORTITUDE™ Preliminary Data Assessment**

**Jeffrey M. Statland, M.D.**, Professor of Neurology, University of Kansas Medical Center

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**Closing Remarks**

**Sarah Boyce**, President & CEO

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**Q&A Session**

**Avidity Management & Dr. Statland**  
Moderator: **Geoff Grande, CFA**, VP of IR/CC

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# Del-brax: Transforming the Treatment of FSHD

First-in-class and best-in-class: greater than 50% reduction in DUX4 regulated genes

Unprecedented & Consistent Reduction in DUX4 Regulated Genes	Signs of Functional Improvement and Reported Outcomes	Favorable Safety and Tolerability
<ul style="list-style-type: none"><li>• Greater than 50% reduction across multiple DUX4 gene panels</li><li>• All treated participants showed reductions greater than 20%</li><li>• Reduction of a newly-identified DUX4 circulating biomarker &amp; creatine kinase</li></ul>	<ul style="list-style-type: none"><li>• Improved muscle strength</li><li>• Increased reachable workspace compared to placebo and natural history study</li><li>• Positive patient and clinician reported outcomes</li></ul>	<ul style="list-style-type: none"><li>• All adverse events (AEs) were mild or moderate</li><li>• No serious AE, No severe AE</li><li>• No discontinuations</li></ul>

**Accelerating *Del-brax* Toward Approval**

# Delivering on the RNA Revolution

PROGRAM / INDICATION	TARGET	LEAD OPTIMIZATION	IND ENABLING	PHASE 1/2	PHASE 3
Myotonic Dystrophy Type 1 (DM1)	DMPK	<i>Del-desiran</i> <sup>TM</sup> (AOC 1001)			HARBOR <sup>TM</sup>
Facioscapulohumeral Muscular Dystrophy (FSHD)	DUX4	<i>Del-brax</i> <sup>TM</sup> (AOC 1020)			
Duchenne Muscular Dystrophy (DMD)	Exon 44	AOC 1044			
DMD Exon 45	Exon 45				
Additional DMD Programs	Undisclosed				
Rare Skeletal Muscle	Undisclosed				
Rare Precision Cardiology	To be disclosed in 2H 24				

# Delivering in 2024: 3 Data Readouts in 3 Clinical Programs in 3 Rare Diseases

## *Del-desiran* in DM1

>40,000 patients in U.S.



- ✓ MARINA-OLE™ data (Q1 2024)
- ✓ Initiation of global Phase 3 HARBOR trial (Q2 2024)

## *Del-brax* in FSHD

~16,000-38,000 patients in U.S.



- ✓ Phase 1/2 FORTITUDE initial data (Q2 2024)

Initiate Phase 3 cohorts

- Biomarker cohort (2H 2024)
- Functional cohort (1H 2025)

## AOC 1044 in DMD44

~900 patients in U.S.



Phase 1/2 EXPLORE44 5mg/kg patient data (Anticipated in Q3 2024)



Amy  
Living with FSHD

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## OUR VISION

**To profoundly improve  
people's lives by  
revolutionizing  
the delivery of  
RNA therapeutics**

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Investor & Analyst Event Series – Volume 9

# Transforming Facioscapulohumeral Muscular Dystrophy (FSHD)

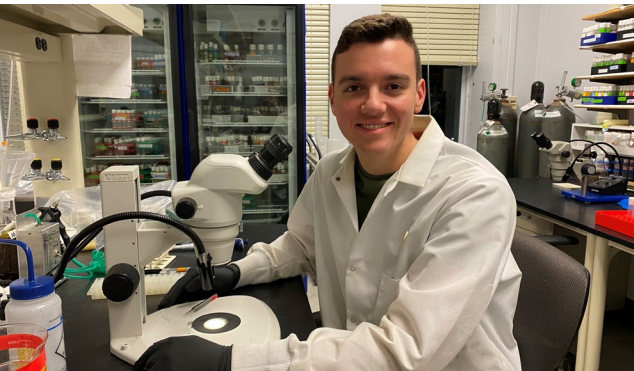
AOC 1020 FORTITUDE™ Phase 1/2 Initial Data

Q&A

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June 12, 2024

NASDAQ: RNA | [aviditybio.com](https://aviditybio.com)





Amy  
Living with FSHD

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Investor & Analyst Event Series – Volume 9

# Transforming Facioscapulohumeral Muscular Dystrophy (FSHD)

## AOC 1020 FORTITUDE™ Phase 1/2 Initial Data

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