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# Delivering on the RNA Revolution

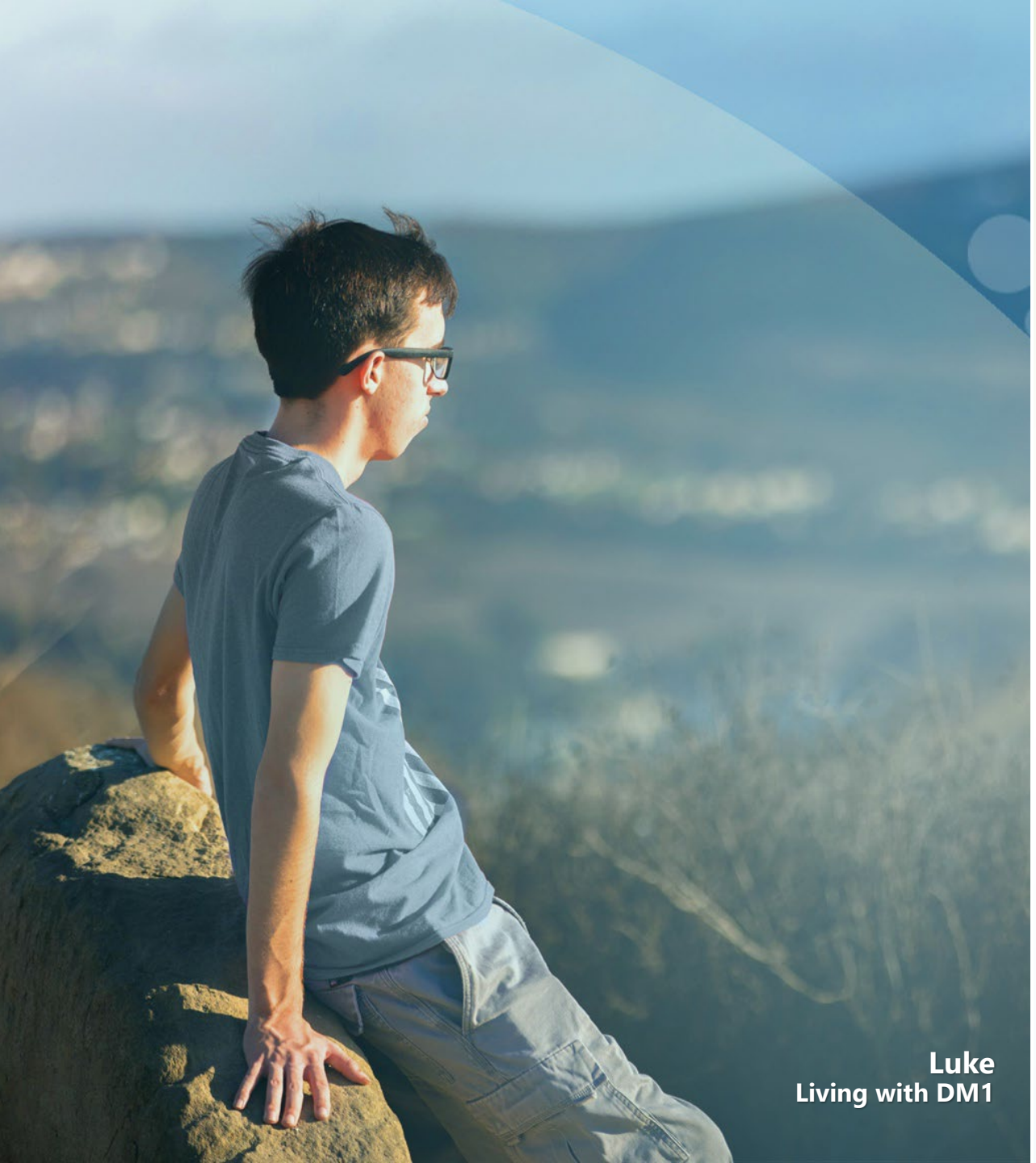
## March 2024

NASDAQ: RNA | 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 future results of operations and financial position; our business strategy; the anticipated timing, costs, design and conduct of our ongoing and planned preclinical studies and clinical trials; the timing of release of data from our ongoing clinical programs; the characterization of data and results from preclinical studies and clinical trials, and conclusions drawn therefrom; research and development plans; plans and projected timelines for *delpacibart etedesiran (del-desiran, or* AOC 1001), AOC 1020 and AOC 1044; safety and tolerability profiles of our product candidates; the potential of the AOC platform and specific product candidates; the ability of our product candidates to treat rare diseases; timing and likelihood of success; product approvals; plans and objectives of management for future operations; collaborations with third parties and expected benefits therefrom; the partial clinical hold related to *del-desiran*; and cash position and our ability to fund our planned operations. 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 based on factors beyond our control, including, without limitation: we may not be able to fully resolve the partial clinical hold related to *del-desiran*, which may result in delays in the clinical development of *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; additional participant data related to *del-desiran* and our other product candidates that continues to become available may be inconsistent with the data produced as of the most recent respective 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, recalls or product liability claims; 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 results of preclinical studies and early clinical trials are not necessarily predictive of future results; potential delays in the commencement, enrollment and completion of 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 Designation 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 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.




## OUR VISION

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

# Building a New Class of RNA Therapeutics

## Well positioned for next phase of growth

**BROAD & DISRUPTIVE PLATFORM**

- AOC platform led to historical first-ever successful delivery of RNA to muscle; successful delivery repeated with AOC 1044 in healthy volunteers
- Expanding our therapeutic expertise, particularly in precision cardiology, through research collaborations and internal discovery efforts

**WORLD CLASS TEAM OF RNA & RARE DISEASE EXPERTS**

- Committed to innovative science matched by passion to improve people's lives
- Building an integrated and diverse company in service of our patients

**STRONG FINANCIALS & INVESTOR CONFIDENCE**

- Strong pro forma cash position of ~$975 million with funding into late 2026*
- Continue to execute on our three clinical development programs for DM1, DMD44 and FSHD and broaden our AOC platform into the precision cardiology therapeutic area

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*** Including anticipated receipt of $380M net proceeds raised in private placement announced on Feb 29, 2024, and expected to close on March 4, 2024**

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

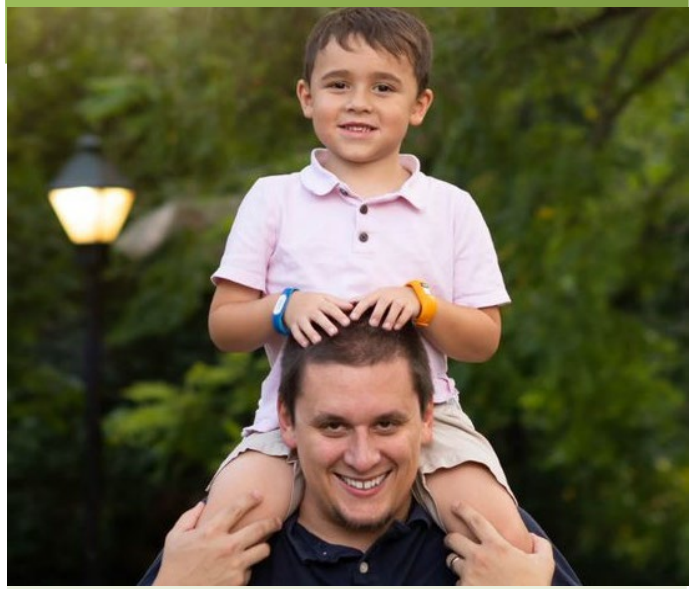
## *Del-desiran™* in **DM1**

***>40,000 patients in U.S.***

On track for initiation of global Phase 3 HARBOR trial Q2 2024

## **AOC 1044** in **DMD44**

***~900 patients in U.S.***



Anticipate Phase 1/2 EXPLORE44 patient data in 2H 2024

## **AOC 1020** in **FSHD**

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

Anticipate Phase 1/2 FORTITUDE preliminary data in ~half of participants in Q2 2024

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# Diverse and Expanding AOC Pipeline

| PROGRAM / INDICATION | TARGET | LEAD OPTIMIZATION | IND ENABLING | PHASE 1/2 | PHASE 3 |
|---|---|---|---|---|---|
| ***Del-desiran™* (AOC 1001)**<br>Myotonic Dystrophy Type 1 (DM1) | DMPK | | | MARINA® | MARINAOLE™ |
| **AOC 1044**<br>Duchenne Muscular Dystrophy (DMD) | Exon 44 | | | explore 44™ | |
| **AOC 1020**<br>Facioscapulohumeral Muscular Dystrophy (FSHD) | DUX4 | | | FORTITUDE™ | |
| **Additional DMD Programs** | Exon 45 & Undisclosed | | | | |
| **Rare Skeletal Muscle Program** | Undisclosed | | | | |
| **Rare Precision Cardiology Program** | Undisclosed | | | | |

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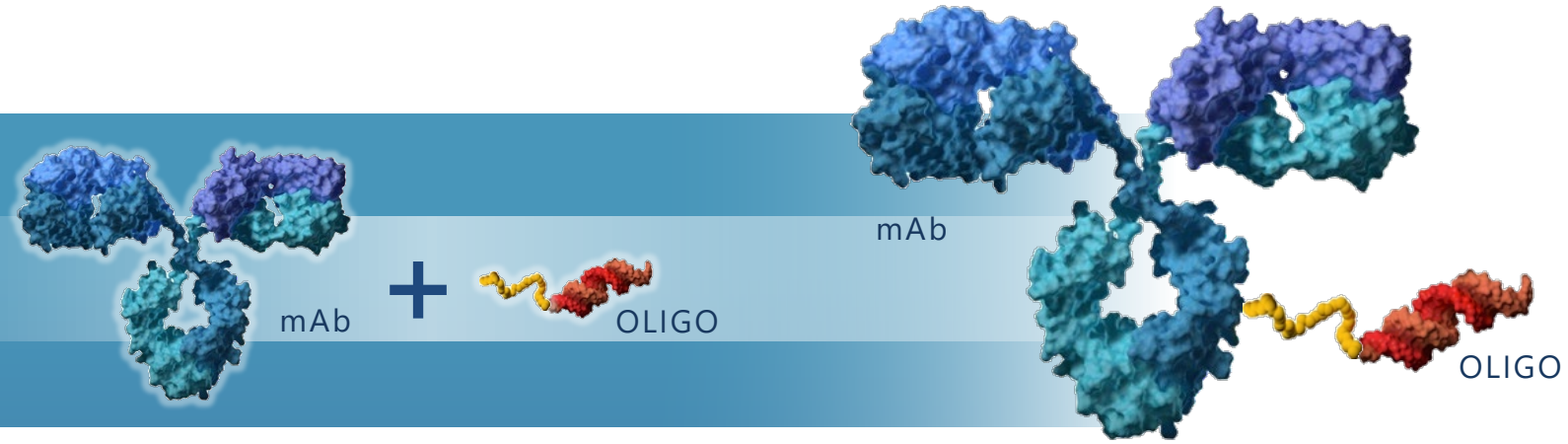
# Key to Our Success: Proprietary AOC™ Platform

Combining the specificity of mAbs with the precision of oligonucleotide therapies




**MONOCLONAL ANTIBODIES** | **OLIGONUCLEOTIDE THERAPIES** | **ANTIBODY OLIGONUCLEOTIDE CONJUGATE (AOC)**

## AOC platform advantages:

- ✓ Ability to target new tissue and cell types beyond the liver
- ✓ Flexibility to select and deploy the most potent oligonucleotides (e.g., siRNAs, PMOs)
- ✓ Maximizes therapeutic durability, enabling infrequent dosing
- ✓ Readily reproducible and scalable

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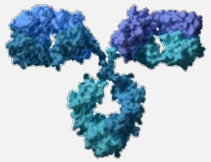
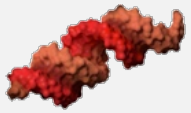
# The Optimal AOC for Each Target

| AOC COMPONENTS | | DATA-DRIVEN COMPONENT CHARACTERISTICS | OUR ENGINEERING IMPACT |
|---|---|---|---|
| **mAb**  | | • Well-established safety profile<br>• High specificity and affinity<br>• Long half-life | • Designed to be effector function null<br>• Epitope selection designed for optimal activity |
| **Linker**  | | • Known linker<br>• Applicable to multiple oligo modalities | • Enhanced for durability<br>• Engineered sites of conjugation<br>• Optimized ratio of oligonucleotides to mAbs |
| **Oligonucleotide** | **siRNA**  | • Attractive safety profiles<br>• Potency in the nanomolar range<br>• Sustained activity in the cytoplasm and nucleus | • Engineered to withstand lysosomal enzymes<br>• Selected and modified to diminish off-target effects |
| | **PMO**  | • Attractive safety profile<br>• Potency in the nanomolar range<br>• Sustained activity | • Engineered for efficient delivery to muscle – increased drug to antibody ratio |

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# Avidity Is Opening the Possibilities of RNA Delivery

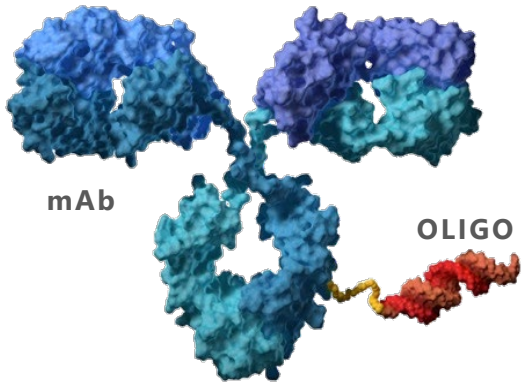
## First-ever company to demonstrate successful targeted delivery of RNA to muscle

**Past 30 Years**

RNA Therapeutics

Focused on delivery to the liver or local delivery

**2021**
**First AOC Dosed in Humans**




AOC

**Today**

Skeletal Muscle

Precision Cardiology

**2024 and Beyond**

Immunology

Other Indications

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# *Del-desiran* Program for Myotonic Dystrophy Type 1 (DM1)

***"Some days I don't have the energy to take another step."***

*— Karin, living with DM1*

# DM1: Significant Patient Burden and Unmet Need

**>40,000**
PEOPLE WITH DM1 IN THE US

**0**
APPROVED THERAPIES

- Underrecognized, progressive & often fatal neuromuscular disease that primarily affects skeletal, cardiac & smooth muscle
- Increases in severity from generation to generation
- Significant impact on quality of life
- *Del-desiran* is designed to address root cause of DM1


Loraine, Kristl & Zen
Living with DM1

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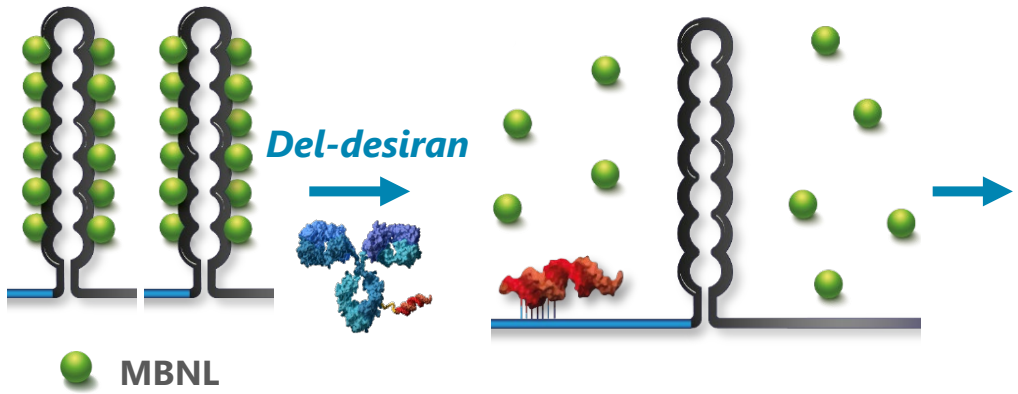
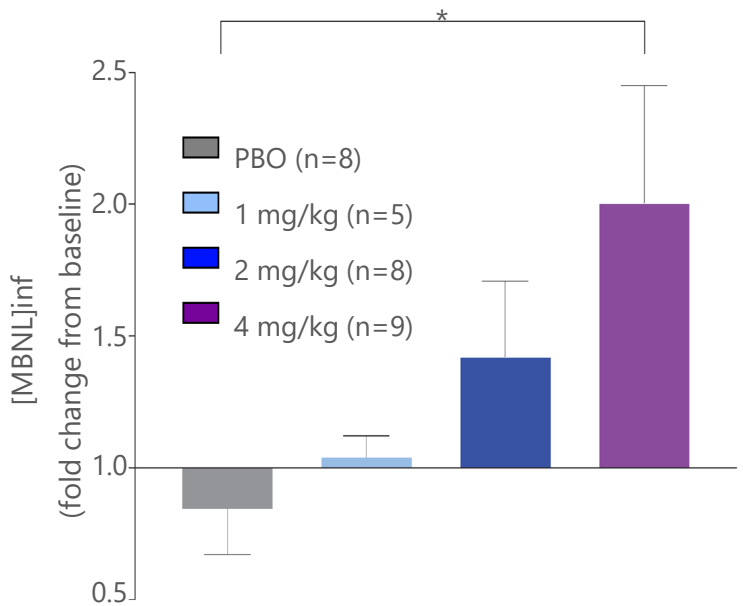
# *Del-desiran* Designed to Address Underlying Cause of Myotonic Dystrophy by Liberating Free MBNL




MARINA®
MARINA OLE™

**Video Hand Opening Time (vHOT)**

**MYOTONIA**

**Hand Grip**

**Quantitative Muscle Testing**

**STRENGTH**

**DM1-Activ**

**ACTIVITIES OF DAILY LIVING**

MBNL sequestered by CUG repeats of mutant DMPK

*Del-desiran* (AOC 1001) treatment Reduced mutant DMPK

**Del-desiran* (AOC 1001) Leads to Dose-dependent increase in MBNL

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#Data shown as mean and standard error. Fold change is calculated per subject as post-treatment relative to baseline;
*P<0.05, unpaired t-test
Wagner, SD, et al. *PLOS Genet.* 2016;12(9):e1006316

# Recent *Del-desiran* Program Updates

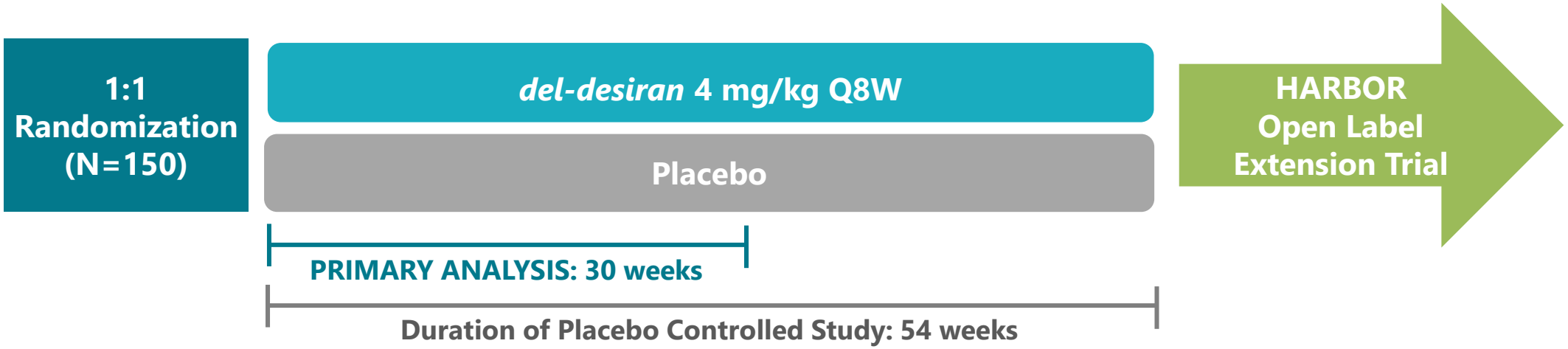
- Initiation of global Phase 3 HARBOR™ trial of *del-desiran* for myotonic dystrophy type 1 (DM1) on-track for Q2 2024
- In March 2024, presented first-look at long-term efficacy and safety data from MARINA-OLE™ trial in people living with DM1 at MDA Clinical & Scientific Conference
  - Data from MARINA-OLE™ showed reversal of disease progression in multiple functional measures in people living with DM1 compared to END-DM1 natural history data
  - Data demonstrated consistent and durable improvements in myotonia, muscle strength and activities of daily living in people with DM1 in new long-term data from MARINA-OLE™
- In January 2024, commenced dose-escalation of remaining study participants from 2 mg/kg to 4 mg/kg of *del-desiran* in the MARINA-OLE™ trial

# Global Phase 3 HARBOR™ Trial Design for Myotonic Dystrophy Type 1 (DM1)

March 2024

# HARBOR™ Initiating Global Phase 3 Study

- Regulatory agreement on study design
- HARBOR™ study designed for efficiency and speed of execution
- On track to initiate in Q2 2024

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# HARBOR™ Phase 3 Trial: Design & Objectives

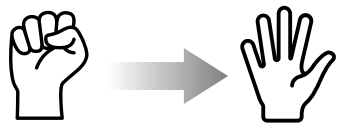
## Optimized for efficiency and speed of execution

### Pivotal Study Design

- 4 mg/kg every 8 weeks; first dose of 2 mg/kg
- N=150; Ages 16+
- 1:1 randomization
- Primary analysis at Week 30; Placebo-control out to week 54
- Participants eligible to roll-over into an open label extension
- ~40 global sites

### Primary Endpoint

**Video Hand Opening Time (vHOT)**



**MYOTONIA**

### Key Secondary Endpoints

**Hand Grip**

**STRENGTH**

**Quantitative Muscle Testing**

**DM1-Activ**

**ACTIVITIES OF DAILY LIVING**

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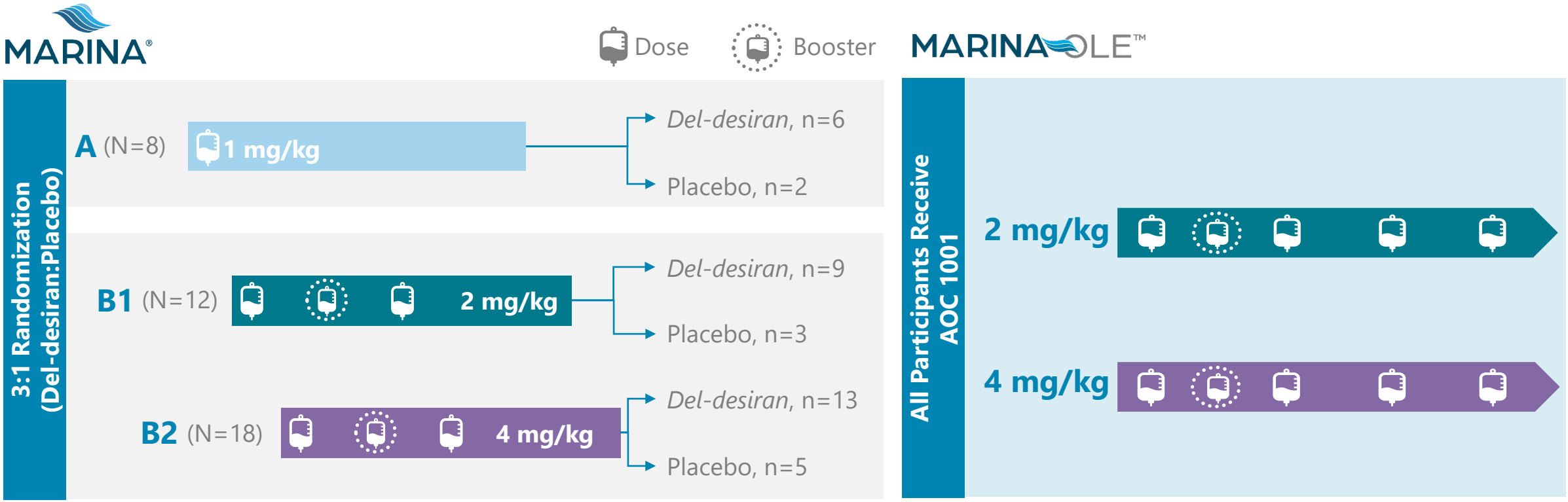
# MARINA-OLE™ Long-term Safety & Efficacy Data in Patients with DM1 Presented at MDA Scientific Conference

March 2024

# Phase 1/2 MARINA® and MARINA-OLE™ Trial Design




- In MARINA, one participant receiving 4 mg/kg *del-desiran* discontinued treatment due to SAE
- All eligible participants (N=37) have enrolled in the MARINA-OLE™

Dose listed is siRNA. The diagram for the MARINA-OLE™ trial includes the first 12 of the 24 months with quarterly dosing.
‡Booster dose was only given to participants who were in Cohort A1 and placebo B1/B2.
SAE: serious adverse event; siRNA: small inhibitory ribonucleic acid.

# MARINA-OLE™ Favorable Long-term Safety and Tolerability

## Over 265 infusions of *del-desiran* totaling 61.1 patient-years of exposure

| MARINA-OLE™ | Number (%) with AE N=37 |
|---|---|
| Subjects with ≥ 1 AE | |
| Any AE | 35 (94.6%) |
| AE related to study drug | 9 (24.3%) |
| Unrelated serious AE (SAE) | 4 (10.8) |
| SAE related to study drug | 0 |
| AE leading to treatment discontinuation | 0 |
| AE leading to death | 0 |

MARINA-OLE™

- **All 37 participants enrolled remain on study**
- **All related AEs were mild or moderate**
  - Most common related AEs reported in 2 or more participants:
    - Nausea
    - Headache
  - No discontinuations
  - No study drug related SAEs; unrelated SAEs are consistent with DM1

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As of January 2024, data from MARINA-OLE™

SAEs considered unrelated to treatment included nausea/vomiting, worsening of atrial fibrillation, and chest pain; and one participant had acute cholelithiasis and biliary pancreatitis.

# END-DM1 Natural History Study: Understanding DM1 Disease Progression

- Ongoing non-interventional NHS aimed to advance the understanding of disease progression in DM1 patients
- Focuses on clinical outcome assessments to support development of therapies for DM1
- 700 patient, 2-year study, ~ 20 centers
- Designed and run by the Myotonic Dystrophy Clinical Research Network (DMCRN)
- Supported by FDA, MDA, MDF; Avidity is one of several sponsoring organizations

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# END-DM1 Data Informed Design of the MARINA® & Phase 3 HARBOR Trials

## Presenting one-year data for the first time today

Same endpoints measured

Clinical trial sites overlap with MARINA® & HARBOR

Contemporary data set based upon standard of care

Hundreds of patients with at least one-year of follow-up in END-DM1 natural history study

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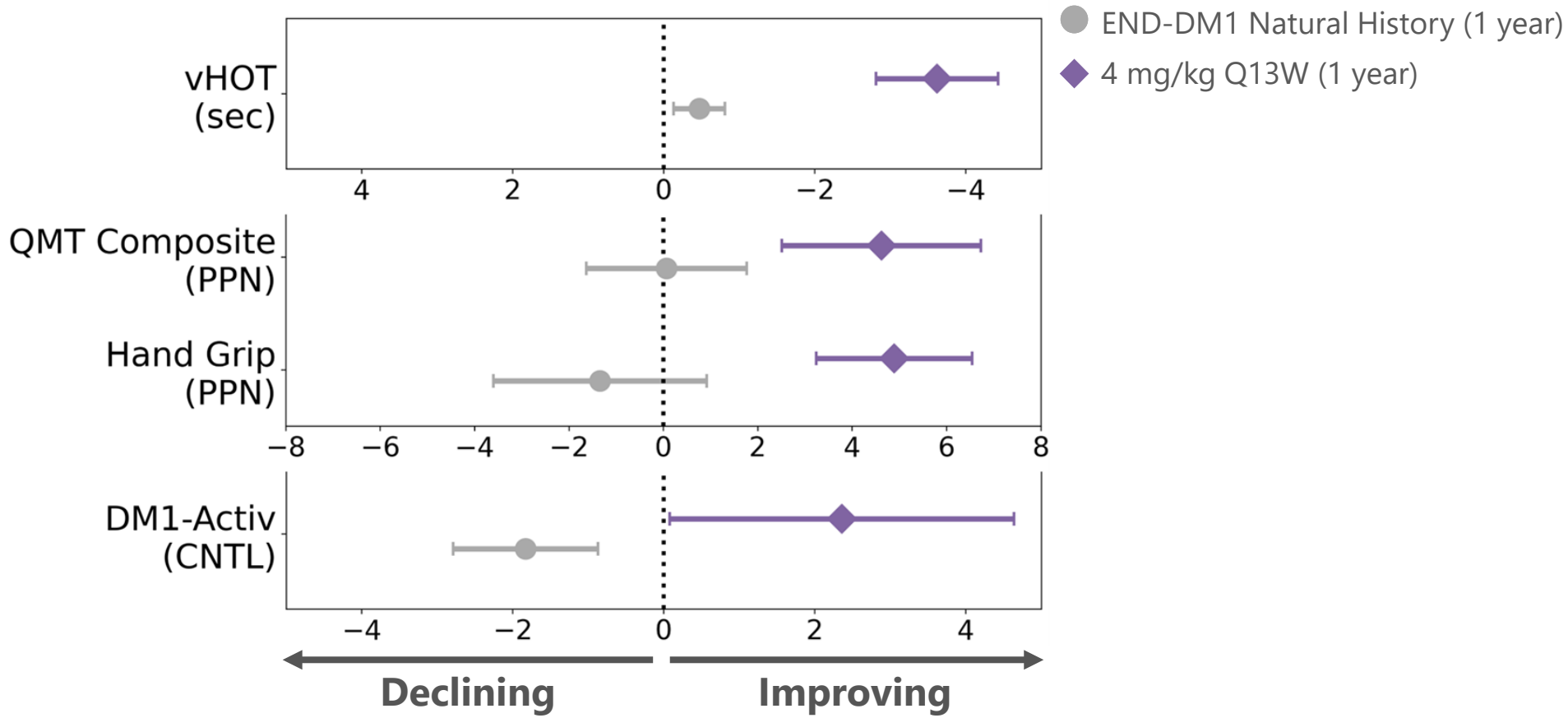
# *Del-desiran*: Reversal of Disease Progression as Measured by MARINA-OLE™ vs. Natural History

## Key endpoints to be used in pivotal HARBOR study




Thanks to END-DM1 physicians for reviewing and approving use of this Avidity analysis; END-DM1 subpopulation matched to MARINA® (n ~ 60)

In MARINA-OLE™ data 4 mg/kg, n=12 for vHOT, QMT composite, hand grip; n=11 for DM1-Activ

PPN = Percent predicted normal
CNTL= percentile
Error bars = SEM (standard error of the mean)

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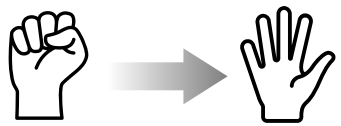
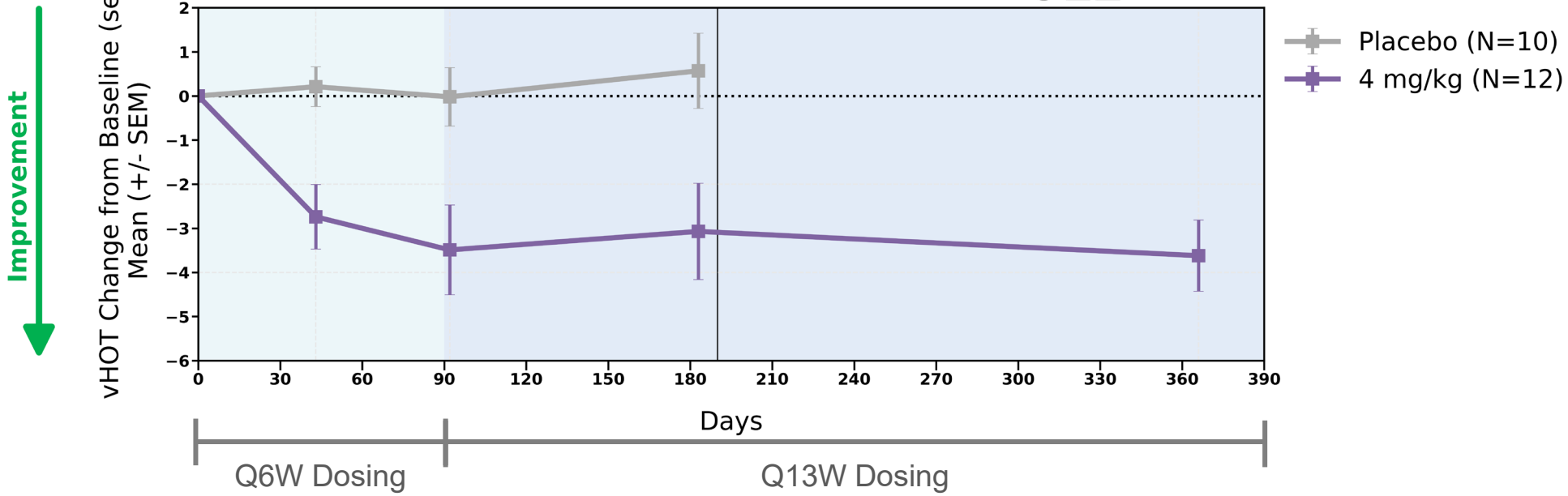
# *Del-desiran*: Long-term Improvement in Myotonia

## Measured by video hand opening time (vHOT) in MARINA® and MARINA-OLE™

**Video Hand Opening Time (vHOT)**



**Independently adjudicated**




**MARINA® data statistically significant at all assessment time points***

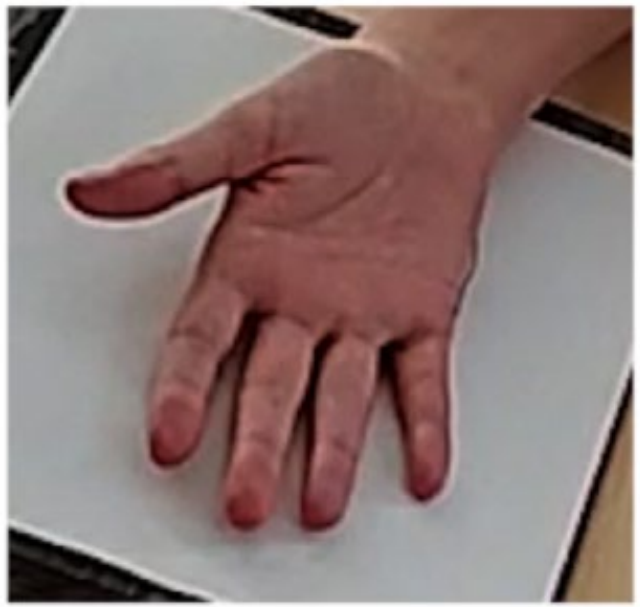
SEM=standard error of the mean
*Based on a post-hoc analysis

# *Del-desiran*: Long-term Improvement in Myotonia

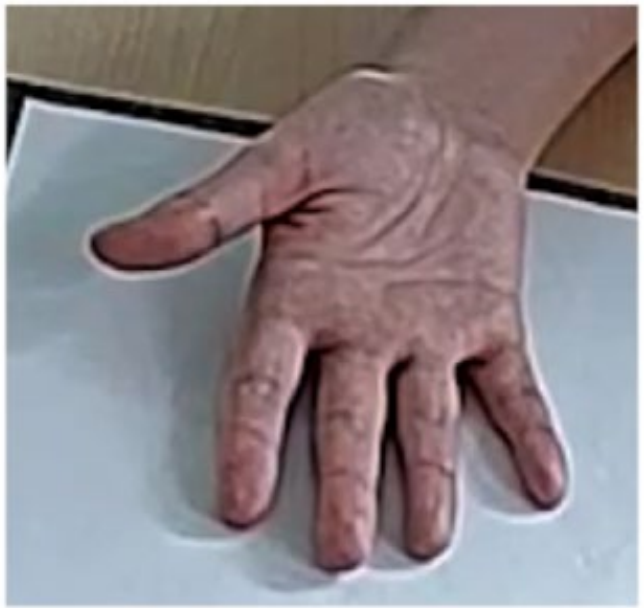
## Measured by video hand opening time (vHOT) in MARINA® and MARINA-OLE™

**Participant from *del-desiran* 4 mg/kg**

**Baseline vHOT**



**MARINA-OLE™ (1 year of 4 mg/kg)**



Timepoint at Day 183 in MARINA-OLE™

# *Del-desiran*: Long-term Improvement in Muscle Strength

## Measured by Hand Grip and Quantitative Muscle Testing in MARINA® and MARINA-OLE™

### Hand Grip Strength

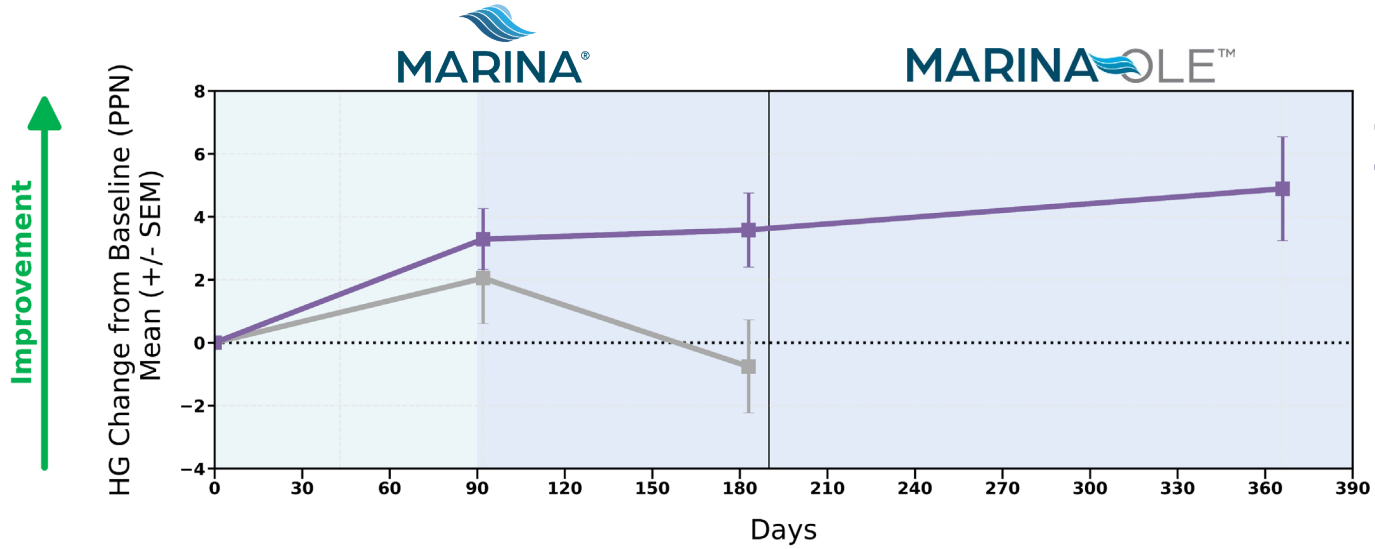
### Quantitative Muscle Testing (QMT)

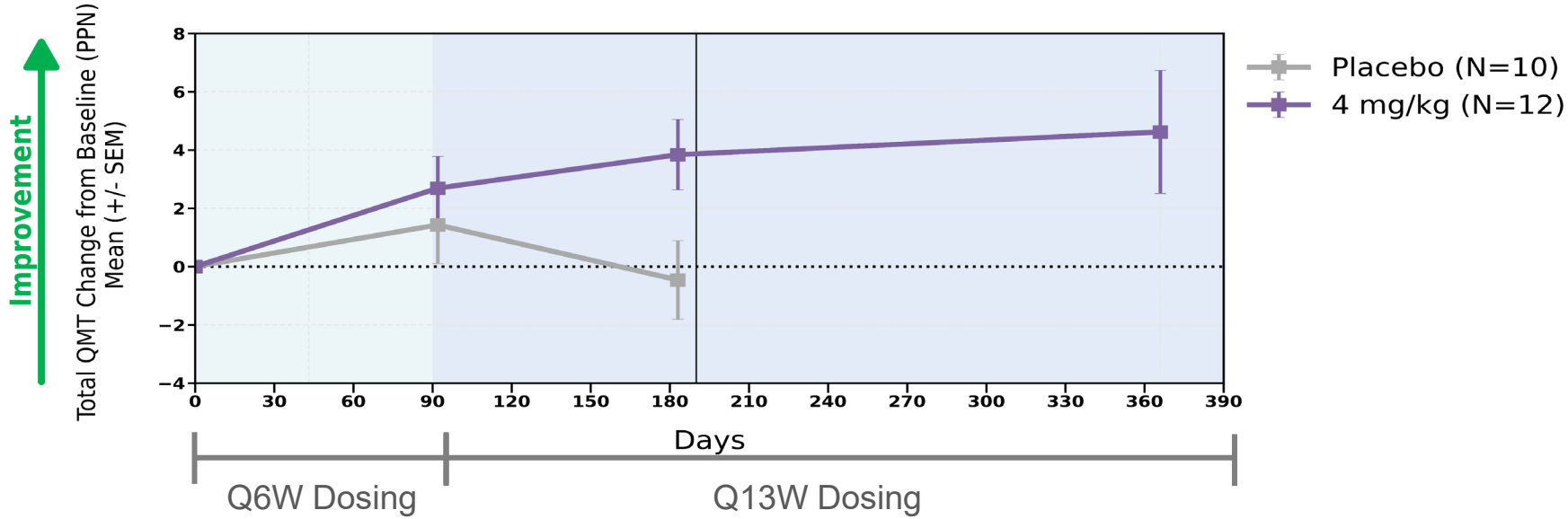
Hand Grip

Elbow Extension & Elbow Flexion

Knee Extension & Knee Flexion

Ankle Dorsiflexion

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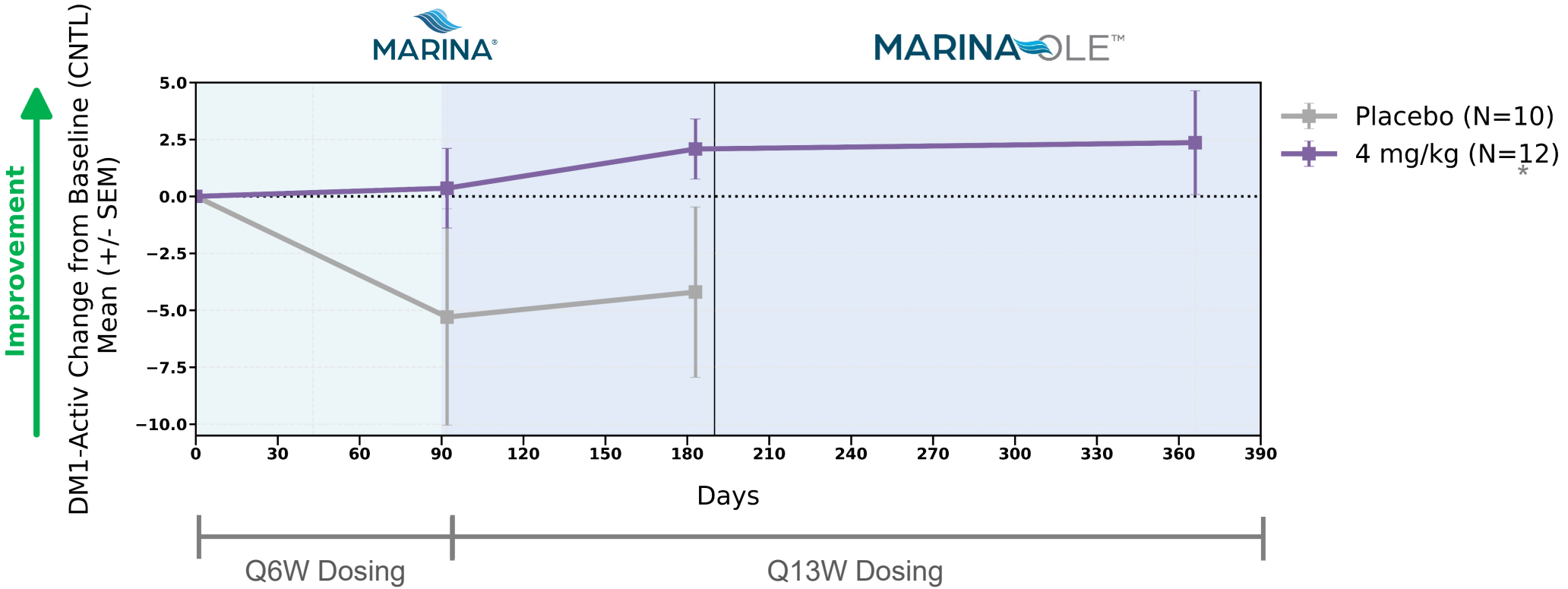
HG=hand grip; PPN=percent predicted normal; SEM=standard error of the mean

# *Del-desiran*: Long-term Improvement in Activities of Daily Living

## Measured by DM1-Activ Patient Reported Outcomes in MARINA® and MARINA-OLE™

**DM1-Activ**

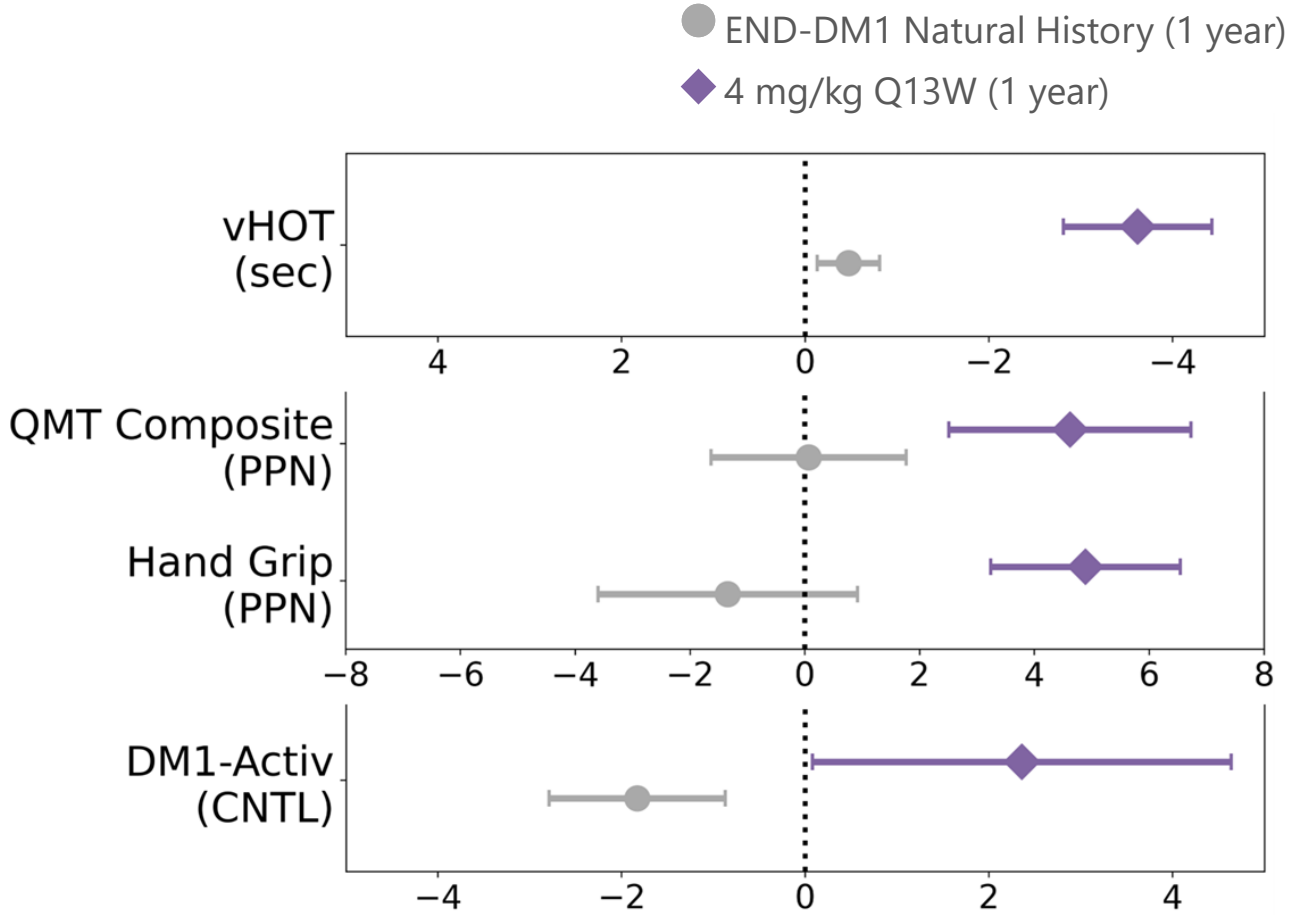



*Day 360 excludes one participant who experienced an injury impairing their ability to perform mobility measures.
CNTL= percentile
SEM = standard error of the mean

# MARINA-OLE™ Data Summary

## Potential of *del-desiran* to be a transformational therapy for DM1 patients

- *Del-desiran* 4 mg/kg
  - Demonstrated favorable long-term safety and tolerability
  - Showed reversal of disease progression in MARINA® and MARINA-OLE™ compared to END-DM1 natural history data
  - Provided consistent and durable improvements in multiple clinical endpoints
- Global HARBOR™ trial on-track to initiate in Q2 2024

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# Patient Experiences: Impact of *del-desiran* on their life

MARINA OLE™

"I started this drug in June and like, two weeks after I took the first infusion, I went to open up a pop bottle, which I never would've been able to do. It **was a twist pop bottle**...**and it opened right up**.

My **strength was better, my outlook was better**, **my hands were working**.
I had more strength, and I could stretch them out. I could **open things and I could turn door knobs** and all these things that were harder.

Like, my **upper arm strength was better**. I could **walk better**.

I didn't need to wear my neck brace all the time and **everything just improved a lot**."

"Before the study I couldn't stand on my toes and since I've been **going back to working out, I can actually stand on my toes again.** So hopefully **building up some strength.**

The myotonia, if I would make a fist, I wouldn't be able to open my hand...I was able to squeeze my fist and **open my hand with no problems.**

My **tongue would cramp up when I would speak**, and I have not had any signs of that happening since the very first dose."

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# Patient Experience: Impact of *del-desiran* on their life

MARINA OLE™

"I've noticed a really big difference in the fact that I used to be a really active person before I got more symptomatic. After a few rounds of the infusion, I've actually been able to **get back to the gym and start working out**, working with a trainer. That's all because **my mobility has definitely increased.** My **range of motion has also increased.**

I think that it's amazing that when I was diagnosed, I was told there's no treatment, no cure. The study has **given me a lot of hope**. I would love for that to be able to be shared with other people in the community who have DM1."

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# Delivering on DM1


- ❑ Initiation of global Phase 3 HARBOR trial – **mid-2024**
- ✓ First look at MARINA-OLE long-term efficacy and safety data - **Complete**
- ✓ Demonstrated first-ever successful targeted delivery of RNA to muscle - **Complete**
- ✓ FDA & EMA Orphan Drug designation - **Complete**
- ✓ FDA Fast Track designation - **Complete**

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Nathan,
Living with DMD, and
his father, Brad

# AOC 1044 Program for Duchenne Muscular Dystrophy (DMD)

***"My advice to any other family dealing with this is to take it day by day, do as much research as possible, and connect with others. I hope that someday there will be a cure for DMD, and no other family will have to go through this"***

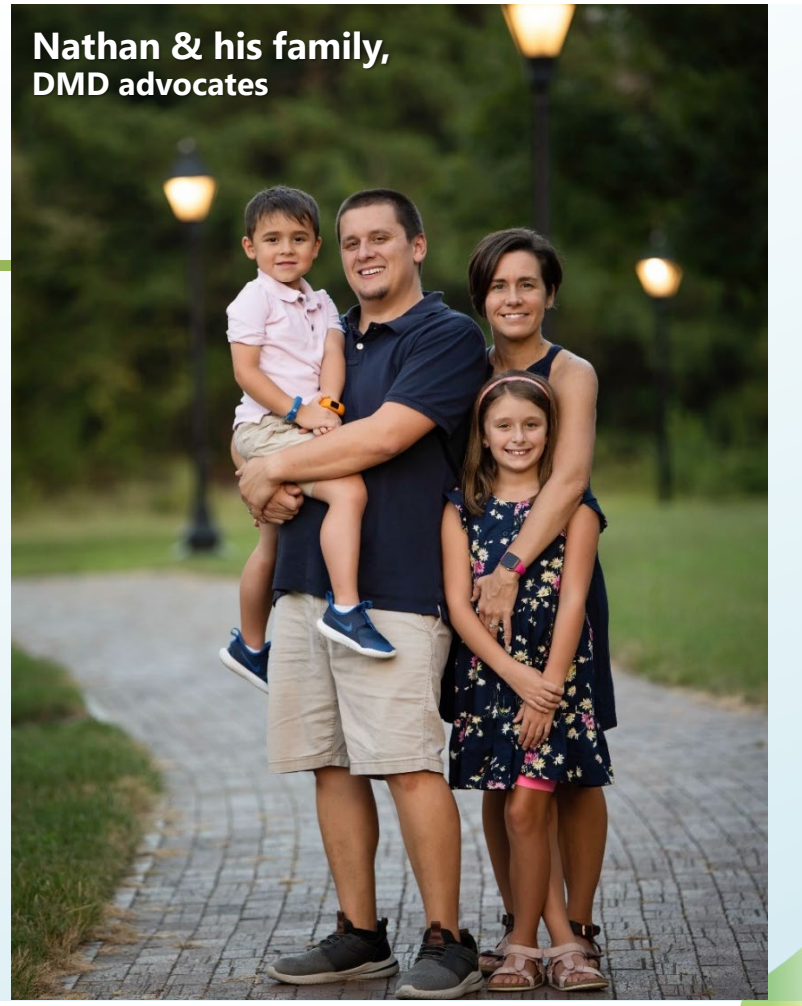
*— Brad, Nathan's Father, DMD Advocate*

# DMD: Characterized By Progressive Muscle Damage and Weakness

**~10,000 - 15,000**
PEOPLE WITH DMD IN THE US
SIMILAR PREVALENCE IN EUROPE

**0**
APPROVED THERAPIES
FOR DMD44

- Monogenic, X-linked, recessive condition characterized by progressive muscle damage and weakness
- Primarily affects males, loss of ambulation by teenage years
- Significantly reduced life expectancy
- Caused by mutations in the DMD gene, which encodes for the dystrophin protein
  - **~ 7%** of DMD skip-amenable patients have mutations amenable to exon 44 skipping (DMD44)
  - **~900** with DMD44 in US (ultra rare)
- AOC 1044: designed to specifically skip exon 44 of dystrophin gene to enable dystrophin production

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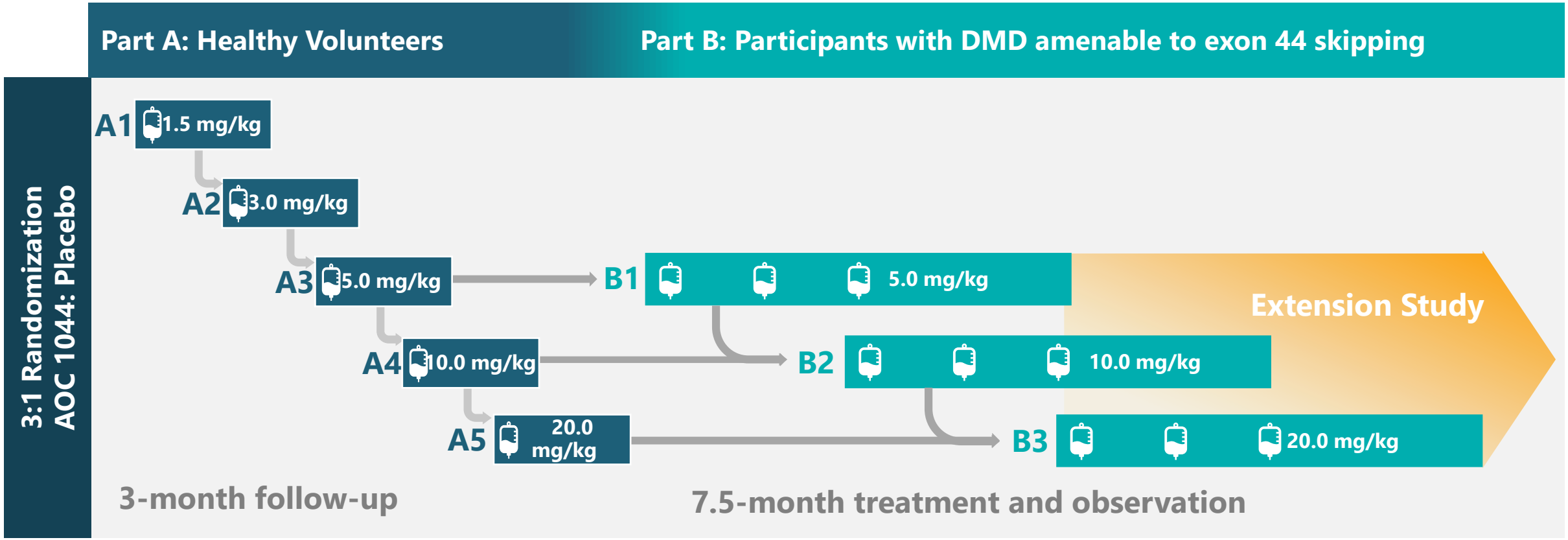
# Phase 1/2 EXPLORE44 Trial Design




Dose – dose listed is PMO

Initiation of next dose level cohort

Enables initiation of Part B cohort at corresponding dose level

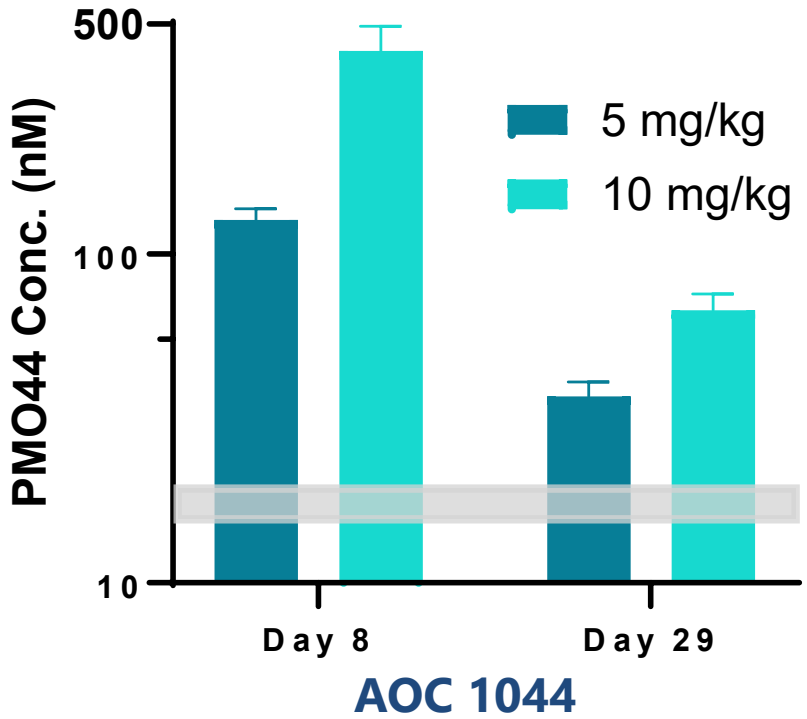
# Phase 1/2 AOC 1044 Healthy Volunteer Data

- In the second half of 2024, planning to share 5 mg/kg cohort data from the Phase 1/2 EXPLORE44™ trial of people living with Duchenne muscular dystrophy mutations amenable to exon 44 skipping (DMD44)
- In December 2023, announced positive AOC 1044 data in healthy volunteers
  - Delivered unprecedented concentrations of PMO into skeletal muscle
    - Up to 50-times greater concentrations of phosphorodiamidate morpholino oligomers (PMO) in skeletal muscle following a single dose compared to peptide conjugated PMOs in healthy volunteers*
  - Demonstrated statistically significant exon 44 skipping compared to placebo in healthy volunteers and increased exon skipping in all participants
    - Up to 1.5% exon skipping after a single dose of 10 mg/kg AOC 1044
  - AOC 1044 was well tolerated

*Comparative statements are based on cross-trial comparisons with publicly-available data for other exon skipping approaches that have been assessed following a single dose in healthy volunteers.

# AOC 1044 Delivered Unprecedented Dose-dependent Concentrations of PMO in Skeletal Muscle in Healthy Volunteers

## Achieved PMO Muscle Concentrations well above those that produce dystrophin in preclinical models



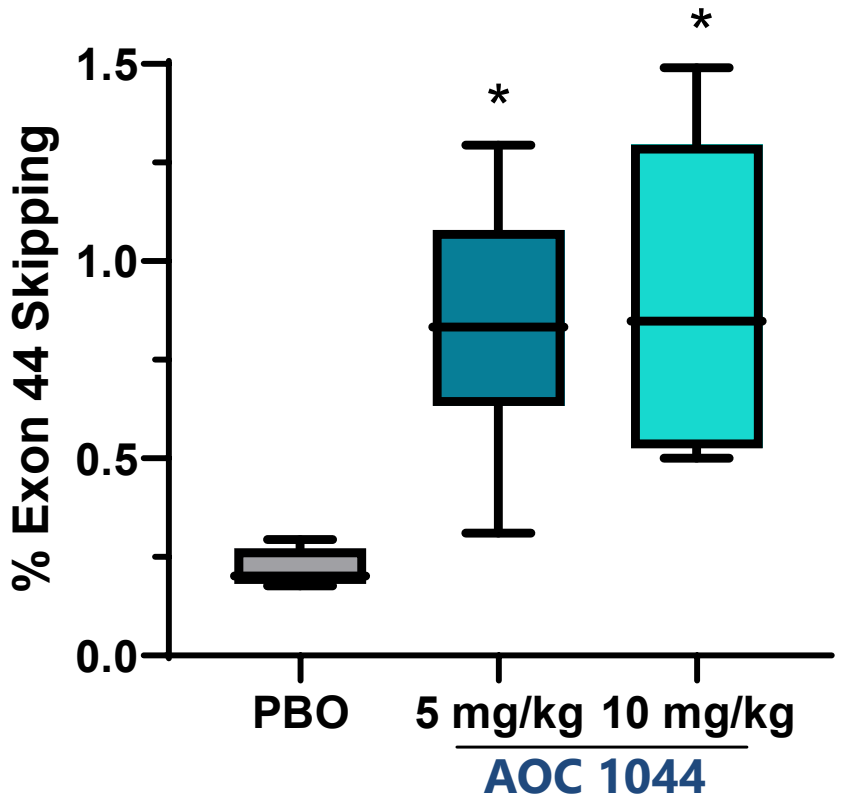

**PMO44 muscle concentration associated with 5% dystrophin production in preclinical DMD model***

PMO44 muscle tissue concentrations were determined utilizing HPLC. N=8/cohort except for 10 mg/kg (n=7) where a single participant did not have a Day 29 biopsy. Doses expressed as PMO component.

*Based on PKPD modelling of a mouse active AOC (utilizing PMO44) in a human DMD exon 45 del/mdx mouse model

# AOC 1044 Provides Statistically Significant Exon 44 Skipping of Up to 1.5% After Single Dose in Healthy Volunteers

## AOC 1044 increased exon skipping in all participants compared to placebo




Exon skipping was determined using ddPCR at Day 29 post dose. Data presented as a boxplot: 25th and 75th quartiles, line represents mean with 5% and 95% confidence interval. N=4 (PBO), N=8 (5 mg/kg), N=7 (10 mg/kg, a single participant did not have a Day 29 biopsy). Doses expressed as PMO component.

*Statistically significant difference relative to PBO utilizing Mann-Whitney test ($p < 0.05$)

# AOC 1044 Well Tolerated in Healthy Volunteers

## Summary of Treatment Emergent Adverse Events (TEAEs)*

| Subjects with ≥ 1 AE n (%) | Placebo N=8 | 1.5 mg/kg N=6 | 3.0 mg/kg N=6 | 5.0 mg/kg N=8 | 10 mg/kg N=8 | Total AOC 1044 N=28 |
|---|---|---|---|---|---|---|
| Any AE | 2 (25%) | 0 | 3 (50%) | 6 (75%) | 7 (88%) | 16 (57%) |
| AE related to study drug | 0 | 0 | 2 (33%) | 3 (38%) | 5 (63%) | 10 (36%) |
| Serious AE (SAE) | 0 | 0 | 0 | 0 | 0 | 0 |
| Severe AE | 1 (13%) | 0 | 0 | 0 | 0 | 0 |
| AE leading to discontinuation of infusion | 0 | 0 | 0 | 0 | 0 | 0 |
| AE leading to death | 0 | 0 | 0 | 0 | 0 | 0 |

*Data as of 23 October 2023; trial ongoing

### AOC 1044

- **All TEAEs mild or moderate**
- **No serious or severe TEAEs**
- **No symptomatic hemoglobin changes**
- **No hypomagnesemia**
- **No renal events**

### Placebo Group

- **One severe adverse event in placebo group**

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# Collaboration with DMD Community to Advance New Treatment Options

Brad and Nathan
DMD Advocates

## Patients and their families are at the heart of everything we do

- Deepening our understanding of the patient and caregiver journey with ongoing discussions
- Engaging with community to better understand challenges and opportunities in drug development
- Continually seeking community input to best meet patient needs and inform trial design and participant support services
- EXPLORE44™ and extension study designed for potential accelerated approval

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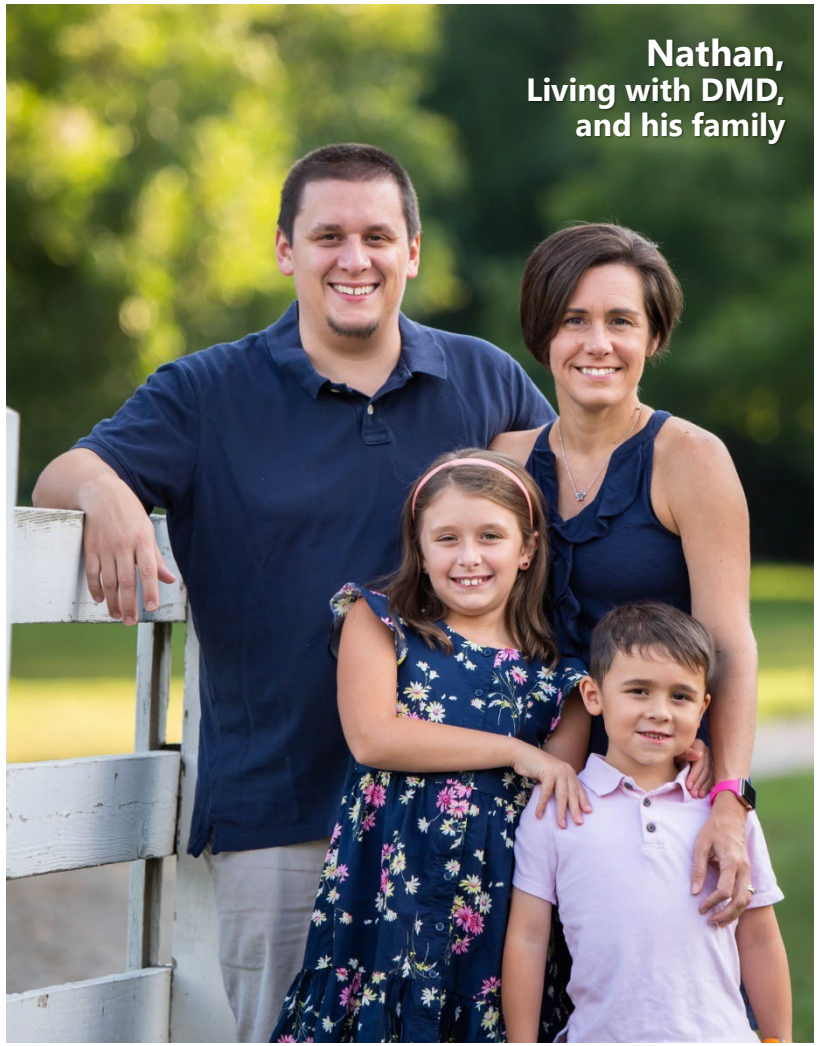
MDA
Parent Project Muscular Dystrophy
TREAT-NMD Neuromuscular Network
Cure Duchenne
Jett Foundation
Duchenne Parent Project aps
Global Genes
EveryLife Foundation for Rare Diseases
CURE 44

# Delivering on DMD




- ❑ EXPLORE44 patient data – **2H 2024**
- ❑ Preclinical development of additional DMD programs - **Ongoing**
- ✓ Reported EXPLORE44 healthy volunteer data - **Complete**
- ✓ Initiated enrollment of participants with DMD44 in EXPLORE44 - **Complete**
- ✓ FDA Rare Pediatric Disease Designation - **Complete**
- ✓ FDA & EMA Orphan Drug designation - **Complete**
- ✓ FDA Fast Track designation - **Complete**

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# AOC 1020 Program for Facioscapulohumeral Muscular Dystrophy (FSHD)

***"Living with FSHD feels like an imprisonment in your own body."***

— *Amy, Living with FSHD*

# FSHD: Lifelong, Progressive and Variable Loss of Muscle Function

**~16,000 - 38,000**
PEOPLE WITH FSHD IN THE US

**0**
APPROVED THERAPIES

- One of the most common forms of muscular dystrophy
- Rare, progressive and variable hereditary muscle-weakening condition marked by significant pain, fatigue and disability
- Onset often in teenage and 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
- AOC 1020: designed to reduce abnormal expression of DUX4 mRNA and DUX4 protein


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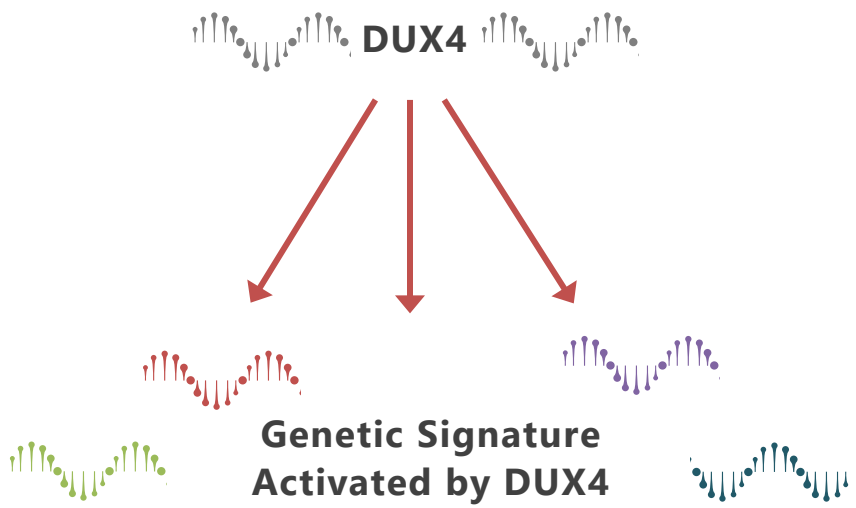
# AOC 1020: A Novel RNA Therapeutic for FSHD

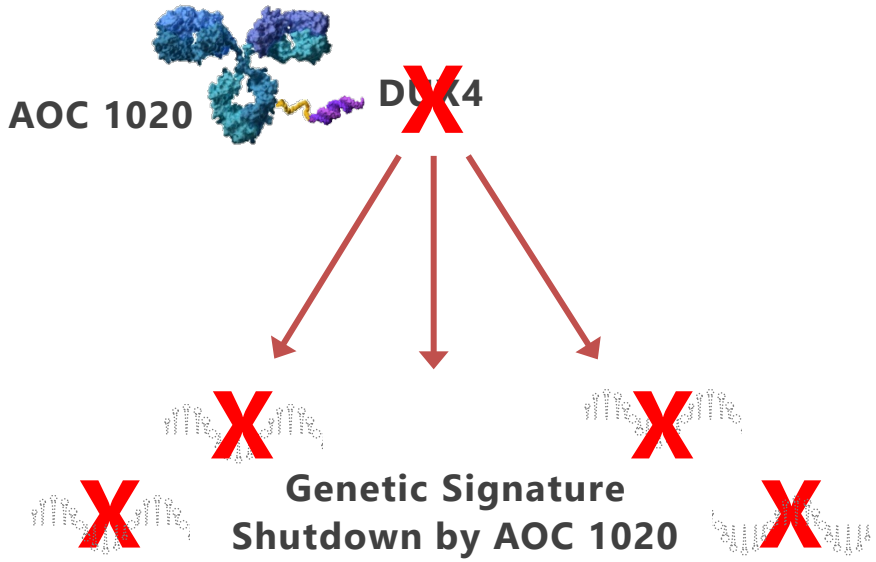
## Targets abnormal expression of DUX4 mRNA in muscle

### MECHANISM OF DISEASE[1,2]




**Apoptosis, immune signaling altered**
**myogenesis inhibited**

### AOC APPROACH[3,4]




**Apoptosis, immune signaling altered**
**myogenesis inhibited**

**Small reductions in DUX4 expression may have large clinical benefit**

1. Lemmers RJLF, et al. *Science*. 2010;329(5999):1650–1653; 2. Snider L, et al. *PLoS Genet*. 2010;6(10):e1001181;
3. Ansseau E, et al. *Genes (Basel)*. 2017;8(3):93; 4. Jiang S, et al. *PLoS Genet*. 2020;16(5):e1008754.

# Phase 1/2 FORTITUDE™ Trial Overview & Objectives

## Key Information

- Randomized, double blinded, placebo controlled
- Multiple dose
- N=72; Ages 18-65
- Follow-up of up to 12 months
- Biopsies in all cohorts

## Stages

- Part A: single-cohort dose titration
- Part B: 2 multiple ascending dose cohorts
- Expansion: single-cohort, parallel-group, multiple-dose
- Eligible to roll-over into OLE

## Primary & Secondary Objectives

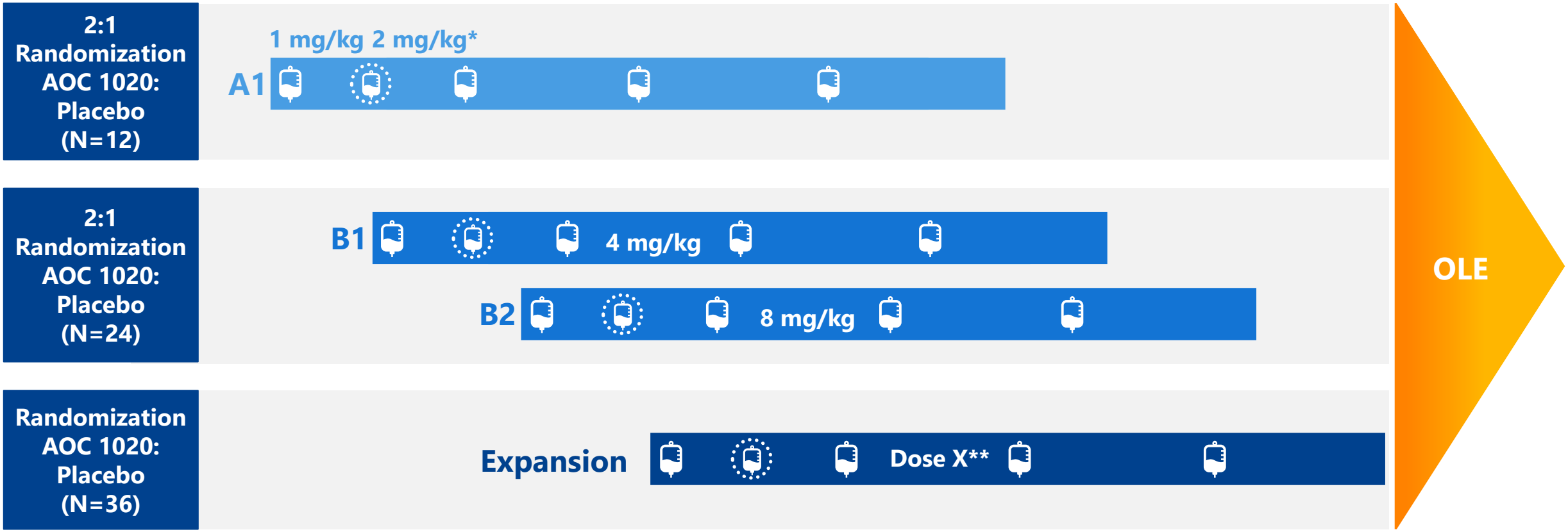
- Safety and tolerability of ascending doses of AOC 1020 in participants with FSHD
- Pharmacokinetics

## Key Exploratory Objectives

- Pharmacodynamics
- Measures of clinical activity
  - Muscle strength
  - Muscle function
  - Muscle composition
- Patient-reported outcomes (PRO)

# Phase 1/2 FORTITUDE Trial Design

FORTITUDE™




*Participants in A1 receive a first dose of 1mg/kg and then receive the 2mg/kg dose for the remainder of the study.

**Dose to be determined based on emerging data

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Dose Booster Multidose quarterly with 1 booster after first 6 weeks; Dose listed is siRNA

# Collaboration with FSHD Community to Advance New Treatment Options


Josh and his brother Jared – FSHD Advocates

## Patients and their families are at the heart of everything we do

- Actively working with and supporting FSHD advocacy groups
- Investing in gaining a deep understanding of patient and caregiver perspectives and journey with FSHD
- Continually seeking community input throughout the drug development process to best meet patient needs
- Collaborating with community leaders to address gaps in support services and resources
- Supporting natural history studies with FSHD Clinical Trial Research Network (**ReSolve, MOVE-FSHD** and **MOVE+** studies)

MDA
FSHD SOCIETY LIGHTING THE WAY TO A CURE
TREAT-NMD Neuromuscular Network
AVIDITY BIOSCIENCES
FSHD CTRN FSHD CLINICAL TRIAL RESEARCH NETWORK
Global Genes Allies in Rare Disease
EVERYLIFE FOUNDATION FOR RARE DISEASES

# Delivering on FSHD


- ❑ FORTITUDE preliminary data in ~half of study participants – **Q2 2024**
- ❑ Enrollment of participants with FSHD in FORTITUDE trial - **Ongoing**
- ✓ FDA & EMA Orphan Drug designation - **Complete**
- ✓ FDA Fast Track Designation - **Complete**

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# Expanding Use of AOCs Beyond Skeletal Muscle

## Industry-leading partners validate broad potential of AOC platform; including precision cardiology and immunology

### PRECISION CARDIOLOGY

Bristol Myers Squibb

Global licensing & research collaboration focused on up to five cardiovascular indications

Expansion of our Bristol Myers Squibb/MyoKardia single target research arrangement

**$100M up-front plus potential for ~$2.2B**

$60M upfront payment

$40M equity investment at a 40% premium

Up to ~$1.35B in R&D milestone payments, up to ~$825 million in commercial milestone payments and tiered royalties on net sales

### IMMUNOLOGY

Lilly

Global licensing & research collaboration focused on immunology and other select indications

**Up to $405M**

Potential milestone payments per target, plus mid-single to low double-digit tiered royalties

# The Experience to Deliver a New Class of RNA Therapeutics

## AVIDITY MANAGEMENT TEAM

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**Art Levin, PhD**
Distinguished Scientist & Strategic Leader

**Steve Hughes, MD**
Chief Medical Officer

**W. Michael Flanagan, PhD**
Chief Scientific & Technical Officer

**Michael MacLean**
Chief Financial & Business Officer

**Teresa McCarthy**
Chief Human Resources Officer

**Eric Mosbrooker**
Chief Strategy Officer

**John Wallen III, PhD, JD**
General Counsel

**Kath Gallagher**
SVP, Global Program Head DM1

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# Strong Cash Position – Funded Into Late 2026

## Q4 2023 financial results

| In millions | Q423 | Q323 | Q422 | Q423 vs Q323 | Q423 vs Q422 |
|---|---|---|---|---|---|
| Collaboration revenue | $2.2 | $2.8 | $2.7 | ($0.6) | ($0.5) |
| R&D expenses | 52.8 | 47.7 | 45.6 | 5.1 | 7.2 |
| G&A expenses | 16.1 | 13.7 | 10.4 | 2.4 | 5.7 |
| Total operating expenses | 68.9 | 61.4 | 56.0 | 7.5 | 12.9 |
| Loss from operations | (66.7) | (58.6) | (53.3) | (8.1) | (13.4) |
| Other income/expenses, net | 6.3 | 6.2 | 2.8 | 0.1 | 3.5 |
| Net loss | ($60.4) | ($52.4) | ($50.5) | ($8.0) | ($9.9) |

| In millions | Q423 | Q422 |
|---|---|---|
| Cash, cash equivalents and marketable securities | $595.4 | $610.7 |

**Strong pro forma cash position of ~$975 million with funding into late 2026***
**Continue to execute on our three clinical development programs for DM1, DMD44 and FSHD and broaden our AOC platform into the precision cardiology therapeutic area**

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*** Including anticipated receipt of $380M net proceeds raised in private placement announced on Feb 29, 2024, and expected to close on March 4, 2024.**

# Delivering on the RNA Revolution

## DM1

On-track for initiation of global Phase 3 HARBOR trial in Q2 2024

## DMD44

Anticipate EXPLORE44 patient data in 2H 2024

## FSHD

Anticipate FORTITUDE preliminary data in ~half of participants in Q2 2024

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