

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934

Date of report (Date of earliest event reported): September 29, 2026

Dyne Therapeutics, Inc.
(Exact Name of Registrant as Specified in Charter)

Delaware
(State or Other Jurisdiction
of Incorporation)

001-39509
(Commission
File Number)

36-4883909
(IRS Employer
Identification No.)

1560 Trapelo Road
Waltham, Massachusetts
(Address of Principal Executive Offices)

02451
(Zip Code)

Registrant's telephone number, including area code: (781) 786-8230

Not applicable
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

- ☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- ☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- ☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- ☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading symbol(s)	Name of each exchange on which registered
Common stock, \$0.0001 par value per share	DYN	Nasdaq Global Select Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 7.01 Regulation FD Disclosure.

On September 29, 2026, Dyne Therapeutics, Inc. (the “Company”) issued a press release and presentation announcing additional one-year clinical data from the multiple ascending dose (“MAD”) portion of its Phase 1/2 ACHIEVE clinical trial (the “ACHIEVE trial”) evaluating zecicimant basivarsen (z-basivarsen, also known as DYNE-101) for myotonic dystrophy type 1 (“DM1”). Additionally, the Company announced the mean baseline value for video hand-opening time (“vHOT”) for the participants enrolled in the registrational expansion cohort (“REC”) of the ACHIEVE trial. A copy of the press release and data presentation are furnished as Exhibit 99.1 and Exhibit 99.2 hereto respectively and are incorporated herein by reference.

The information furnished under this Item 7.01, including Exhibit 99.1 and Exhibit 99.2, shall not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing.

Item 8.01 Other Events.

On September 29, 2026, the Company issued a press release and presentation announcing additional one-year clinical data from the ACHIEVE trial evaluating z-basivarsen in individuals with DM1. The new data come from a pooled dose group of participants (N=25-26) from the 3.4 mg/kg every four weeks, 5.4 mg/kg every 8 weeks and 6.8 mg/kg every 8 weeks cohorts of the MAD portion of the ACHIEVE trial (the “pooled dose group”), including participants originally assigned to the placebo group (re-baselined to the visit prior to their first active dose). The pooled dose group includes a mixture of dose exposures and does not reflect the effect of maintaining the registrational dose of 6.8 mg/kg every 8 weeks for the entire treatment duration, as only 8 of 26 participants in the pooled dose group received the registrational dose for the full 12 months analyzed, and most did not receive the registrational dose during this timeframe. For certain measurements at 12 months, these data were compared to a cohort of participants (N=41-46) from the ongoing END-DM1 natural history study who were propensity-matched to the ACHIEVE pooled dose group (the “matched natural history cohort”).

These data include:

- **Myotonia:** Sustained improvement from baseline in hand myotonia, as measured by vHOT. The pooled dose group had a mean baseline vHOT of 8.2 seconds, which decreased by 3.2 seconds at 6 months (standard deviation of change from baseline: 3.5 seconds). This is compared to an increase of 0.4 seconds in the placebo group from the ACHIEVE MAD at 6 months (N=14), representing a 3.6 second relative improvement. The change from baseline in vHOT at 6 months is the primary endpoint of the ACHIEVE REC, from which topline data are expected in the first quarter of 2027 to support a potential Biologics License Application (“BLA”) submission for U.S. Accelerated Approval in the third quarter of 2027.
 - **Function:** Sustained improvement from baseline in function, as assessed by the 5 times sit to stand (“5xSTS”) test and the 10-meter walk/run (“10MWR”) test. In the pooled dose group at 12 months, participants improved on 5xSTS by 1.2 seconds and on 10MWR by 0.3 seconds relative to baseline, showing a divergence from the matched natural history cohort on 10MWR. 5xSTS was not included in the END-DM1 natural history study, so no comparisons could be made. The change from baseline in 5xSTS at 12 months is the primary endpoint of the ongoing global Phase 3 HARMONIA clinical trial, which is intended to serve as a confirmatory trial for traditional approval in the U.S. and support ex-U.S. marketing applications.
 - **Strength:** Sustained improvement from baseline on muscle strength as assessed by the quantitative muscle testing (“QMT”) total score and the individual QMT hand grip score. In the pooled dose group at 12 months, participants improved on both QMT total and hand grip by 4.8% predicted relative to baseline, showing a divergence from the matched natural history cohort on both measures.
 - **Patient Reported Outcomes:** Sustained improvement from baseline in the Myotonic Dystrophy Health Index (“MDHI”) patient reported outcome measure, including in 6 subscales assessing central nervous system disease manifestations (cognitive impairment, sleep disturbances, fatigue, communication, emotional issues and pain). In the pooled dose group at 12 months, participants improved on the MDHI total score by 25.2% relative to baseline, showing a divergence from an unmatched END-DM1 natural
-

history cohort (N=410).

- **Safety and Tolerability:** Updated safety and tolerability data as of April 20, 2026, from participants initially enrolled in the MAD portion of the ACHIEVE trial. Z-basivarsen continued to demonstrate a favorable safety profile, and no related serious treatment emergent adverse events were identified.

Additionally, the Company announced that the mean baseline value for vHOT of the 71 participants enrolled in the ACHIEVE REC is 8.3 seconds (standard deviation at baseline: 6.0 seconds). These baseline data remain blinded to treatment assignment. Topline data from the ACHIEVE REC, which is intended to support a potential application for U.S. Accelerated Approval, are planned for the first quarter of 2027.

Cautionary Note Regarding Forward-Looking Statements

This Current Report on Form 8-K contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Current Report on Form 8-K, including statements regarding the Company's strategy, future operations, prospects and plans, objectives of management, the potential of the FORCE platform, the clinical potential of z-basivarsen and its potential to mitigate central nervous system-related manifestations of DM1, expectations regarding the timing and outcome of data from clinical trials, expectations regarding the timing and outcome of submissions to and interactions with global regulatory authorities, the availability of accelerated approval pathways for z-basivarsen, and the potential of video hand opening time to serve as an intermediate clinical endpoint for U.S. accelerated approval constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "might," "objective," "ongoing," "plan," "predict," "project," "potential," "should," "will" or "would," or the negative of these terms, or other comparable terminology are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. The Company may not actually achieve the plans, intentions or expectations disclosed in these forward-looking statements, and you should not place undue reliance on these forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in these forward-looking statements as a result of various important factors, including: uncertainties inherent in the identification and development of product candidates, including the initiation and completion of preclinical studies and clinical trials; uncertainties as to the availability and timing of results from preclinical studies and clinical trials; the timing of and the Company's ability to enroll patients in clinical trials; whether results from preclinical studies and initial data from early clinical trials will be predictive of the final results of the clinical trials or future trials; uncertainties as to the FDA's and other regulatory authorities' interpretation of the data from the Company's clinical trials and acceptance of the Company's clinical programs and the regulatory approval process; whether the Company's cash resources will be sufficient to fund its foreseeable and unforeseeable operating expenses and capital expenditure requirements; as well as the risks and uncertainties identified in the Company's filings with the Securities and Exchange Commission (SEC), including the Company's most recent Form 10-Q and in subsequent filings the Company may make with the SEC. In addition, the forward-looking statements included in this Current Report on Form 8-K represent the Company's views as of the date hereof. The Company anticipates that subsequent events and developments will cause its views to change. However, while the Company may elect to update these forward-looking statements at some point in the future, it specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing the Company's views as of any date subsequent to the date hereof.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Press Release, dated September 29, 2026
99.2	Presentation, dated September 29, 2026
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

DYNE THERAPEUTICS, INC.

Date: September 29, 2026

By: /s/ Erick Lucera
Name: Erick Lucera
Title: Treasurer and Chief Financial Officer



Dyne Therapeutics Announces Additional One-Year Clinical Data from Phase 1/2 ACHIEVE Trial of Z-Basivarsen (DYNE-101) for Myotonic Dystrophy Type 1 (DM1)

- Data from a pooled dose group of participants initially enrolled in the multiple ascending dose (MAD) portion of ACHIEVE showed functional improvement versus baseline, versus placebo and versus a matched natural history cohort across multiple measures at 12 months -
- Data continued to support improvement at 6 months in video hand opening time (vHOT) as an early indicator of clinical benefit with z-basivarsen in DM1 -
- Dyne continues to expect topline data in Q1 2027 from the ACHIEVE registrational expansion cohort (REC), with a primary endpoint of change from baseline in vHOT at 6 months, intended to support a potential submission for U.S. Accelerated Approval in Q3 2027 -
- The mean baseline vHOT in the ACHIEVE REC is 8.3 seconds -

WALTHAM, Mass., Sept. 29, 2026 – Dyne Therapeutics, Inc. (Nasdaq: DYN), a clinical-stage company focused on delivering functional improvement for people living with genetically driven neuromuscular diseases, today announced additional one-year efficacy data from the MAD portion of its ongoing Phase 1/2 ACHIEVE clinical trial of zeleciment basivarsen (z-basivarsen, also known as DYNE-101) for DM1, showing functional improvement across multiple measures. These data are being presented at the 31st Annual International Congress of the World Muscle Society (WMS) being held September 29, 2026, through October 3, 2026, virtually and in Hiroshima, Japan, as well as the 2026 Annual Meeting of the American Association of Neuromuscular & Electrodiagnostic Medicine (AANEM) being held September 29, 2026, through October 2, 2026, virtually and in Orlando, Florida.

“This week we are presenting data at 6 and 12 months from a larger pooled dose group of participants from ACHIEVE, which further support our confidence in the results we have previously presented from smaller individual dose cohorts,” said Doug Kerr, M.D., Ph.D., chief medical officer of Dyne. “In addition, for the first time, we are showing functional improvement across multiple measures compared to a matched natural history cohort, not just compared to placebo and compared to baseline. We attribute these results to the differentiated properties of our FORCE platform and z-basivarsen, which is designed to correct the core underlying splicing abnormalities in DM1 by delivering a targeted therapeutic payload to the nucleus in both muscle and the CNS. We aim to confirm these findings through both the ACHIEVE REC and the Phase 3 HARMONIA study, which is currently recruiting participants at over 35 active global sites.”

The analyses being presented at WMS and AANEM were designed to evaluate whether a larger treated group showed the same positive efficacy trends previously observed in a smaller cohort of participants receiving the registrational dose of 6.8 mg/kg Q8W (N=6). Data from a pooled dose group of participants (N=25-26) from the 3.4 mg/kg Q4W, 5.4 mg/kg Q8W and 6.8 mg/kg Q8W cohorts of the MAD portion of the ACHIEVE trial, including participants originally assigned to the

placebo group (re-baselined to the visit prior to their first active dose), were assessed through 12 months of treatment.

The pooled dose group includes a mixture of dose exposures and does not reflect the effect of maintaining the registrational dose of 6.8 mg/kg for the entire treatment duration, as only 8 of 26 participants in the pooled dose group received the registrational dose for the full 12 months analyzed and most did not receive the registrational dose during this timeframe.

At 12 months, these data were compared to a cohort of participants (N=41-46) from the ongoing END-DM1 natural history study who were propensity-matched to the ACHIEVE pooled dose group.

The new results to be presented at WMS and AANEM include:

- **Myotonia:** Sustained improvement from baseline in hand myotonia, as measured by vHOT. The pooled dose group had a mean baseline vHOT of 8.2 seconds, which decreased by 3.2 seconds at 6 months (standard deviation of change from baseline: 3.5 seconds). This is compared to an increase of 0.4 seconds in the placebo group from the ACHIEVE MAD at 6 months (N=14), representing a 3.6 second relative improvement. The change from baseline in vHOT at 6 months is the primary endpoint of the ACHIEVE REC, from which topline data are expected in Q1 2027 to support a potential Biologics License Application (BLA) submission for U.S. Accelerated Approval in Q3 2027.
- **Function:** Sustained improvement from baseline in function, as assessed by the 5 times sit to stand (5xSTS) test and the 10-meter walk/run (10MWR) test. In the pooled dose group at 12 months, participants improved on 5xSTS by 1.2 seconds and on 10MWR by 0.3 seconds relative to baseline, showing a divergence from the matched natural history cohort on 10MWR. 5xSTS was not included in the END-DM1 natural history study, so no comparisons could be made. The change from baseline in 5xSTS at 12 months is the primary endpoint of the ongoing global Phase 3 HARMONIA clinical trial, which is intended to serve as a confirmatory trial for traditional approval in the U.S. and support ex-U.S. marketing applications.
- **Strength:** Sustained improvement from baseline on muscle strength as assessed by the quantitative muscle testing (QMT) total score and the individual QMT hand grip score. In the pooled dose group at 12 months, participants improved on both QMT total and hand grip by 4.8% predicted relative to baseline, showing a divergence from the matched natural history cohort on both measures.
- **Patient Reported Outcomes:** Sustained improvement from baseline in the Myotonic Dystrophy Health Index (MDHI) patient reported outcome measure, including in 6 subscales assessing central nervous system (CNS) disease manifestations (cognitive impairment, sleep disturbances, fatigue, communication, emotional issues and pain). In the pooled dose group at 12 months, participants improved on the MDHI total score by 25.2% relative to baseline, showing a divergence from an unmatched END-DM1 natural history cohort (N=410).

- **Safety and Tolerability:** Updated safety and tolerability data from participants initially enrolled in the MAD portion of the ACHIEVE trial. Z-basivarsen continued to demonstrate a favorable safety profile, and no related serious treatment emergent adverse events were identified.¹

These data will be presented in an oral presentation at WMS titled “Long-term functional improvement with zecicement basivarsen in the Phase 1/2 ACHIEVE trial.” The slides for this presentation are now available in the Scientific Publications & Presentations section of Dyne’s website. Additionally, these data will be presented in a poster presentation at AANEM titled “Zecicement basivarsen targets the underlying cause of myotonic dystrophy type 1 to enable functional improvement in the Phase 1/2 ACHIEVE trial,” which will be available in the Scientific Publications & Presentations section of Dyne’s website on Wednesday, September 30 at 6:15 p.m. ET.

Baseline vHOT of the ACHIEVE Registrational Expansion Cohort (REC)

The mean baseline value for vHOT of the 71 participants enrolled in the ACHIEVE REC is 8.3 seconds (standard deviation at baseline: 6.0 seconds). These baseline data remain blinded to treatment assignment. Topline data from the ACHIEVE REC, which is intended to support a potential application for U.S. Accelerated Approval, are planned for Q1 2027.

About the ACHIEVE Trial

ACHIEVE is a global, randomized, placebo-controlled, double-blind, Phase 1/2 clinical trial evaluating the safety, tolerability and efficacy of zecicement basivarsen (z-basivarsen, also known as DYNE-101) in patients with myotonic dystrophy type 1 (DM1). The multiple ascending dose (MAD) portion of the study resulted in the selection of a registrational dose and regimen of 6.8 mg/kg z-basivarsen administered every eight weeks. A registrational expansion cohort to support potential regulatory submissions, including Accelerated Approval in the U.S., is fully enrolled. The primary endpoint for this cohort is the change from baseline in middle finger myotonia as measured by video hand opening time (vHOT) at 6 months, compared to placebo. For more information on the ACHIEVE trial, visit www.clinicaltrials.gov (NCT05481879) and euclinicaltrials.eu (EUCT2023-510353-42-00).

About zecicement basivarsen (z-basivarsen, also known as DYNE-101)

Z-basivarsen is an investigational therapeutic being evaluated in the fully enrolled global Phase 1/2 ACHIEVE clinical trial and the global confirmatory Phase 3 HARMONIA clinical trial for people living with DM1. Z-basivarsen consists of an antisense oligonucleotide (ASO) conjugated to an antigen-binding fragment (Fab) that binds to the transferrin receptor 1 (TfR1) to enable delivery to muscle and the central nervous system. It is designed to deliver functional improvement in individuals living with DM1 by reducing toxic nuclear *DMPK* RNA to release splicing proteins and allow normal mRNA processing. Z-basivarsen has been granted Breakthrough Therapy, Orphan Drug and Fast Track designations by the U.S. Food and Drug Administration (FDA), as well as Orphan Drug designation from the European Medicines Agency (EMA) and the Ministry of Health, Labour and Welfare (MHLW) in Japan for the treatment of DM1.

About Myotonic Dystrophy Type 1 (DM1)

Myotonic dystrophy type 1 (DM1) is a rare, progressive, genetic neuromuscular disease with high morbidity and early mortality. DM1 affects ~40,000 people in the U.S. and ~55,000 people in the EU. The severity of symptoms and rate of progression vary. Symptoms can begin at any point in an affected person's life, depending on the DM1 subtype. Adult-onset DM1 symptoms typically appear between 20 to 40 years of age. DM1 is caused by mutations in the *DMPK* gene, leading to a widespread disruption of RNA splicing, known as spliceopathy, which drives the multi-system manifestations of the disease. People experience a broad spectrum of symptoms, including: muscle weakness throughout the body, myotonia or difficulty relaxing muscles, excessive daytime sleepiness, fatigue, dysregulated sleep, cognitive impairments, cardiac arrhythmias, respiratory issues and gastrointestinal dysfunction. Although the genetic cause of DM1 is well understood, there are currently no approved disease-modifying treatments for DM1.

About Dyne Therapeutics

Dyne Therapeutics is focused on delivering functional improvement for people living with genetically driven neuromuscular diseases. We are developing therapeutics that target muscle and the central nervous system (CNS) to address the root cause of disease. The company is advancing clinical programs for Duchenne muscular dystrophy (DMD) and myotonic dystrophy type 1 (DM1) as well as preclinical programs for facioscapulohumeral muscular dystrophy (FSHD), Pompe disease and multiple DMD mutations. At Dyne, we are on a mission to deliver functional improvement for individuals, families and communities. Learn more at <https://www.dyne-tx.com/>, and follow us on X, LinkedIn and Facebook.

Forward-Looking Statements

This press release contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this press release, including statements regarding Dyne's strategy, future operations, prospects and plans, objectives of management, the potential of the FORCE platform, the clinical potential of zeleciment basivarsen ("z-basivarsen," also known as DYNE-101) and its potential to mitigate central nervous system-related manifestations of myotonic dystrophy type 1, expectations regarding the timing and outcome of data from clinical trials, expectations regarding the timing and outcome of submissions to and interactions with global regulatory authorities, the availability of accelerated approval pathways for z-basivarsen, and the potential of video hand opening time to serve as an intermediate clinical endpoint for U.S. accelerated approval, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "might," "objective," "ongoing," "plan," "predict," "project," "potential," "should," "will" or "would," or the negative of these terms, or other comparable terminology are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Dyne may not actually achieve the plans, intentions or expectations disclosed in these forward-looking statements, and you should not place undue reliance on these forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in these forward-looking statements as a result of various important factors, including: uncertainties inherent in the identification and development of

product candidates, including the initiation and completion of preclinical studies and clinical trials; uncertainties as to the availability and timing of results from preclinical studies and clinical trials; the timing of and Dyne's ability to enroll patients in clinical trials; whether results from preclinical studies and initial data from early clinical trials will be predictive of the final results of the clinical trials or future trials; uncertainties as to the FDA's and other regulatory authorities' interpretation of the data from Dyne's clinical trials and acceptance of Dyne's clinical programs and the regulatory approval process; whether Dyne's cash resources will be sufficient to fund its foreseeable and unforeseeable operating expenses and capital expenditure requirements; as well as the risks and uncertainties identified in Dyne's filings with the Securities and Exchange Commission (SEC), including the Company's most recent Form 10-Q and in subsequent filings Dyne may make with the SEC. In addition, the forward-looking statements included in this press release represent Dyne's views as of the date of this press release. Dyne anticipates that subsequent events and developments will cause its views to change. However, while Dyne may elect to update these forward-looking statements at some point in the future, it specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing Dyne's views as of any date subsequent to the date of this press release.

¹. Z-basivarsen safety data as of April 20, 2026.

Contacts:

Investors

Mia Tobias
ir@dyne-tx.com
781-317-0353

Media

Stacy Nartker
media@dyne-tx.com
781-317-1938

Long-Term Functional Improvement With Zeleciment Basivarsen in the Phase 1/2 ACHIEVE Trial

Karlién Mul, MD, PhD¹, Guillaume Bassez², Jordi Diaz-Manera³, James Lilleker⁴, Marika Pane⁵, Richard Roxburgh⁶, Benedikt Schoser⁷, Christopher Turner⁸, Allison Kreuzer⁹, Alexandra Lovett⁹, Soma Ray⁹, Huaihou Chen⁹, Shauna Andersson⁹, Douglas Kerr⁹, Valeria Sansone¹⁰

¹Radboud University Medical Center, Nijmegen, the Netherlands; ²Institut de Myologie, Paris, France; ³John Walton Muscular Dystrophy Research Centre, Newcastle University, Newcastle-Upon-Tyne, UK; ⁴Muscle Disease Unit, Northern Care Alliance NHS Foundation Trust, Manchester Academic Health Science Centre, Manchester, UK; ⁵Fondazione Policlinico Universitario A. Gemelli, Rome, Italy; ⁶Neurogenetics Clinic Centre for Brain Research, University of Auckland, Auckland, NZ; ⁷Friedrich-Baur-Institute, Dep. of Neurology LMU Clinics, Ludwig-Maximilians University, Munich, Germany; ⁸University College London Hospitals, London, UK; ⁹Dyne Therapeutics, Inc., Waltham, MA, USA; ¹⁰Centro Clinico NeMO, University of Milan, Milan, Italy

WMS2026
HIROSHIMA

**The 31st Annual Congress of
the World Muscle Society**

29 September – 3 October 2026, Hiroshima, Japan

To view an electronic version of this presentation and the related appendix, scan the QR codes below:

Presentation



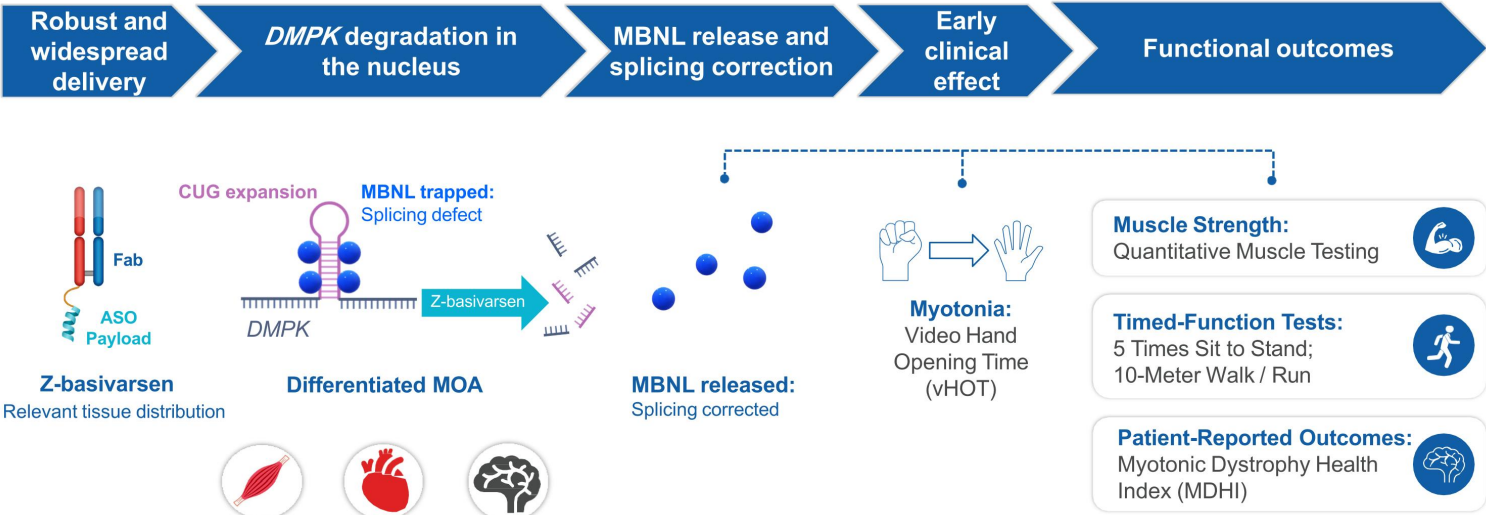
Appendix



Disclosure

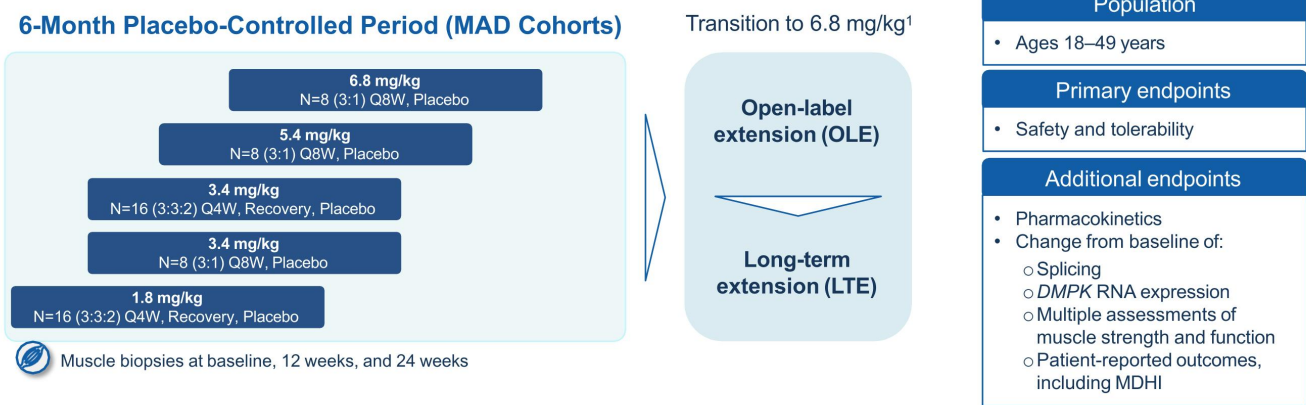
- Dr. Mul has served as a paid consultant for Dyne Therapeutics, Avidity Biosciences, and Sarepta Therapeutics. She received research funding from Vertex Pharmaceuticals and PepGen Inc.
- Zeleciment basivarsen (z-basivarsen, also known as DYNE-101) is investigational or otherwise in development and has not been approved as safe or effective by the US FDA, EMA, or any other regulatory authority.

Z-Basivarsen Addresses the Central Pathobiology of DM1 to Enable Broad Functional Improvement¹



¹Image depicts the intended z-basivarsen mechanism of action.
ASO, antisense oligonucleotide; CUG, cytosine, uracil, and guanine; DM1, myotonic dystrophy type 1; DMPK, dystrophin myotonia protein kinase; Fab, antigen-binding fragment. MBNL, muscleblind-like; MDHI, Myotonic Dystrophy Health Index; MOA, mechanism of action; vHOT, video hand opening time; z-basivarsen, zeleciment basivarsen

ACHIEVE Multiple Ascending Dose Study



Data from the MAD portion of the ACHIEVE study was used to select the registrational dose of 6.8 mg/kg Q8W that is being evaluated in the Registrational Expansion Cohort of the study^{2,3}

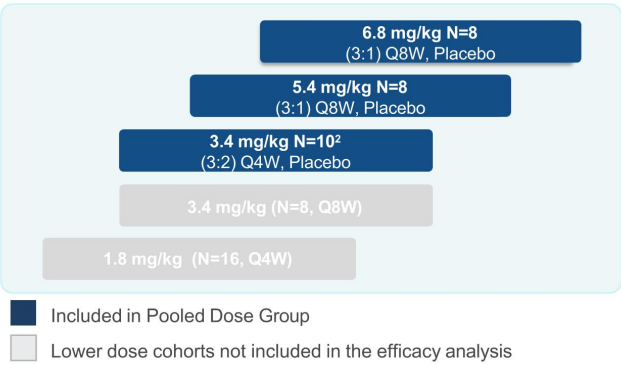


Doses provided refer to approximate antisense oligonucleotide component of DYNE-101. Recovery cohort Q4W x 2 doses then placebo for the remainder of the 24-week placebo-controlled period. Q8W, every 8 weeks with a loading dose given 4 weeks after first dose of active drug. Additional endpoints include select secondary and exploratory endpoints. ¹Transition to 6.8 mg/kg dose occurs at non-uniform times during OLE or LTE. ²Lilleker J. 2025 Annual MDA Clinical and Scientific Conference, March 16-19, Dallas, TX. ³Sansone V, et al. Poster presentation at the Annual International Congress of the WMS, Vienna, Austria, October 7–11, 2025. Poster 380P. DMPK, dystrophin myotonic protein kinase; MAD, multiple ascending dose; MDHI Myotonic Dystrophy Health Index; LTE, long-term extension; OLE, open-label extension; Q4W, every 4 weeks; Q8W, every 8 weeks; RNA, ribonucleic acid.

ACHIEVE MAD Study: Efficacy Analysis of Pooled Dose Group

- Previous long-term analysis included N=6 assigned to 6.8 mg/kg (registrational dose) at baseline¹
- To determine if similar trends hold in a larger sample, we analyzed 12mo efficacy of a pooled dose group (N = 26)
- The pooled dose group includes a mixture of dose exposures and does not reflect the effect of maintaining 6.8 mg/kg for the entire treatment duration
- Only 8/26 participants in the pooled dose group received the registrational dose for the full 12 months analyzed, and most did not receive the registrational dose during this timeframe

Pooled Dose Group 1-Year Efficacy Analysis (N=26)



Pooled Dose Group includes participants from the 6.8 mg/kg Q8W, 5.4 mg/kg Q8W, and 3.4 mg/kg Q4W cohorts, including participants who initially received placebo within those cohorts and subsequently received active treatment. The participants who originally received placebo were re-baselined to the visit prior to their first active dose. 6.8 mg/kg and 5.4 mg/kg dose cohorts were treated with Q8W, every 8 weeks with a loading dose given 4 weeks after first dose of active drug. ¹Sansone V, et al. Poster presentation at the Annual International Congress of the WMS, Vienna, Austria, October 7–11, 2025. Poster 380P. ²Patients assigned to recovery were not included in this analysis. MAD, multiple ascending dose; Q4W, every 4 weeks; Q8W, every 8 weeks; z-bas, zeleciment basivarsen.

Z-Basivarsen ACHIEVE MAD Clinical Update



Safety

Data from ACHIEVE MAD as of **April 20, 2026**



Efficacy

Data from **6.8 mg/kg** cohort and analysis of **Pooled Dose Group**:

- **vHOT**
- Clinical outcome measures of **muscle strength** and **function**
- **Patient-reported outcomes**



Divergence From Natural History¹

Analysis of **ACHIEVE** versus **END-DM1** natural history



¹END-DM1 data transfer as of July 2026. Analysis presented with permission from the Myotonic Dystrophy Clinical Research Network (DMCRN). END-DM1 remains ongoing. DM1, myotonic dystrophy type 1; MAD, multiple ascending dose; vHOT, video hand opening time; z-basivarsen, zeleciment basivarsen.

ACHIEVE MAD Baseline Characteristics

Mean (SD)	Placebo (N=14)	6.8 mg/kg Q8W (N=6)	Pooled dose group (N=26)
Age (years)	32.6 (9.6)	37.2 (9.7)	35.6 (8.0)
BMI (kg/m ²)	24.4 (4.7)	23.4 (5.6)	22.7 (3.9)
CASI-22	0.68 (0.20)	0.74 (0.25)	0.73 (0.19)
CTG repeats	597 (246)	542 (191)	527 (243)
vHOT (middle finger) (sec)	7.5 (3.0)	7.8 (3.8)	8.2 (4.4)
QMT total (% predicted)	51.5 (14.3)	51.3 (10.4)	48.0 (14.3)
Hand grip (% predicted)	44.8 (16.9)	49.0 (12.9)	41.5 (16.3)
10-meter walk/run (sec)	3.34 (0.48)	3.94 (1.56)	3.99 (1.49)
5 Times sit-to-stand (sec)	9.24 (2.03)	9.98 (3.33)	10.20 (3.94)
MDHI total ¹	18.7 (13.8)	26.5 (13.7)	24.4 (19.8)

Pooled Dose Group includes participants from the 6.8 mg/kg Q8W, 5.4 mg/kg Q8W, and 3.4 mg/kg Q4W cohorts, including participants who initially received placebo within those cohorts and subsequently received active treatment. The participants who originally received placebo were re-baselined to the visit prior to their first active dose. Only 8/26 participants in the pooled dose group received the registrational dose for the full 12 months analyzed, and most did not receive the registrational dose during this timeframe.



¹Scored from 0–100, with 0 representing no disease burden, 100 maximal disease burden.
BMI, body mass index; CASI, composite alternative splicing index; CTG, cytosine, thymine, and guanine; MAD, multiple ascending dose; MDHI, Myotonic Dystrophy Health Index; Q8W, every 8 weeks; QMT, quantitative muscle testing; SD, standard deviation; sec, seconds; vHOT, video hand opening time.

Favorable Safety Profile With No Serious Related TEAEs¹

Summary of treatment-emergent adverse events (TEAEs)¹

TEAE category	Participants with ≥1 TEAE, n (%)					Overall (N=56)
	1.8 mg/kg Q4W+Rec. N=16	3.4 mg/kg Q4W+Rec. N=16	3.4 mg/kg Q8W N=8	5.4 mg/kg Q8W N=8	6.8 mg/kg Q8W N=8	
Any TEAE	16 (100)	16 (100)	8 (100)	8 (100)	8 (100)	56 (100)
Any related TEAE	10 (62.5)	10 (62.5)	4 (50)	6 (75)	7 (87.5)	37 (66.1)
Any serious TEAE	5 (31.3)	0	2 (25)	1 (12.5)	0	8 (14.3)
Any serious related TEAE	0	0	0	0	0	0
Any TEAE leading to withdrawal from study	0	0	0	2 (25)	0	2 ² (3.6)
Any TEAE leading to death	0	0	0	0	0	0

Most TEAEs were mild or moderate in intensity¹

- **10 serious TEAEs unrelated to study drug**
 - Atrioventricular block first degree (1)³
 - Pneumonia (2 events in same participant)
 - Pulmonary embolism (1)⁴
 - Hyponatraemia (1)
 - Influenza B virus test positive (1)
 - Fall (1)
 - Gastroenteritis (1)
 - Meningioma (1)
 - Uterine leiomyoma (1)
- **Most common TEAEs (≥30% participant incidence)⁵**
 - Infusion-related reaction (50%)⁶
 - Nasopharyngitis (46.4%)
 - Diarrhoea (35.7%)
 - Headache (33.9%)
 - Procedural pain (33.9%)
 - Influenza (32.1%)
 - Back pain (30.4%)

Additional safety data

- **Liver enzyme elevations have been observed in a minority of participants**
 - No impact on liver function (bilirubin or coagulation)
 - Interpretation is complicated by underlying disease and elevated baseline values up to ~2.5x greater than the upper limit of normal
- **No participants have demonstrated persistent related anemia or thrombocytopenia**

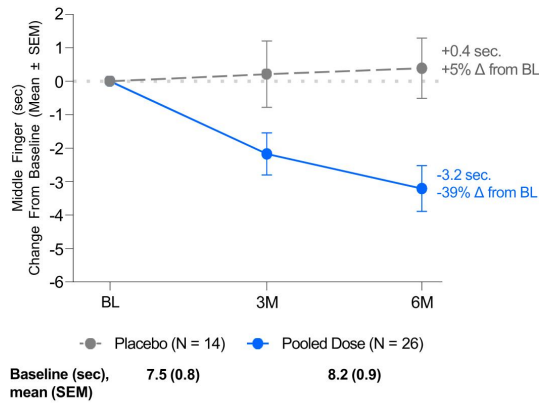
~1300 doses of study drug administered to date representing over 143 patient-years of follow-up¹



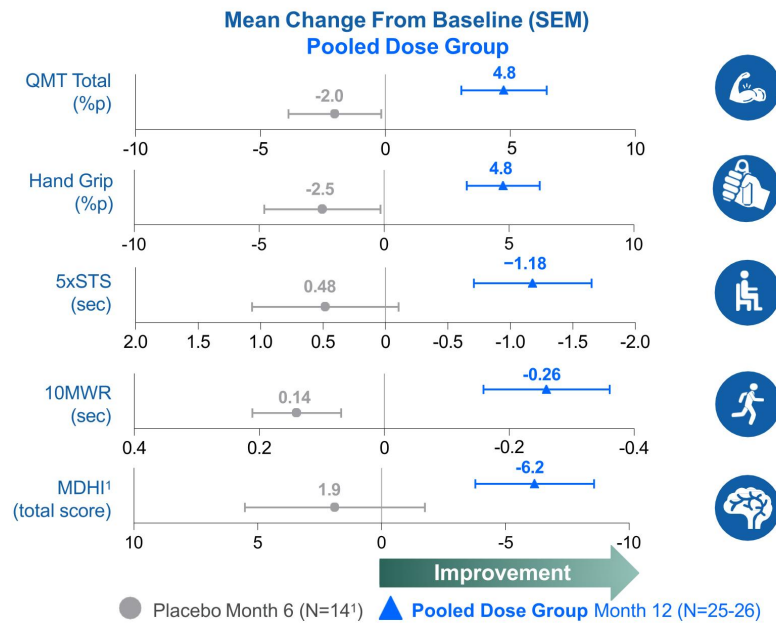
¹Data as of April 20, 2026. ²TEAEs leading to study withdrawal were IRRs that resolved within 2-3 days in 2 participants who had received z-basivarsen for over 12 months. ³Transient worsening of atrioventricular (AV) block in a participant with ongoing medical history of first-degree AV block. ⁴Attributed to risk factors for pulmonary embolism. ⁵All cohorts combined. ⁶Most IRRs were mild or moderate in severity. IRR, infusion-related reactions; Q4W, every 4 weeks; Q8W, every 8 weeks; Rec, Recovery Arm; TEAE, treatment-emergent adverse event; z-basivarsen, zeleciment basivarsen.

Pooled Efficacy Data Continue to Support vHOT as an Early Indicator of Functional Improvement

vHOT Middle Finger: Pooled Dose Group



Improvement



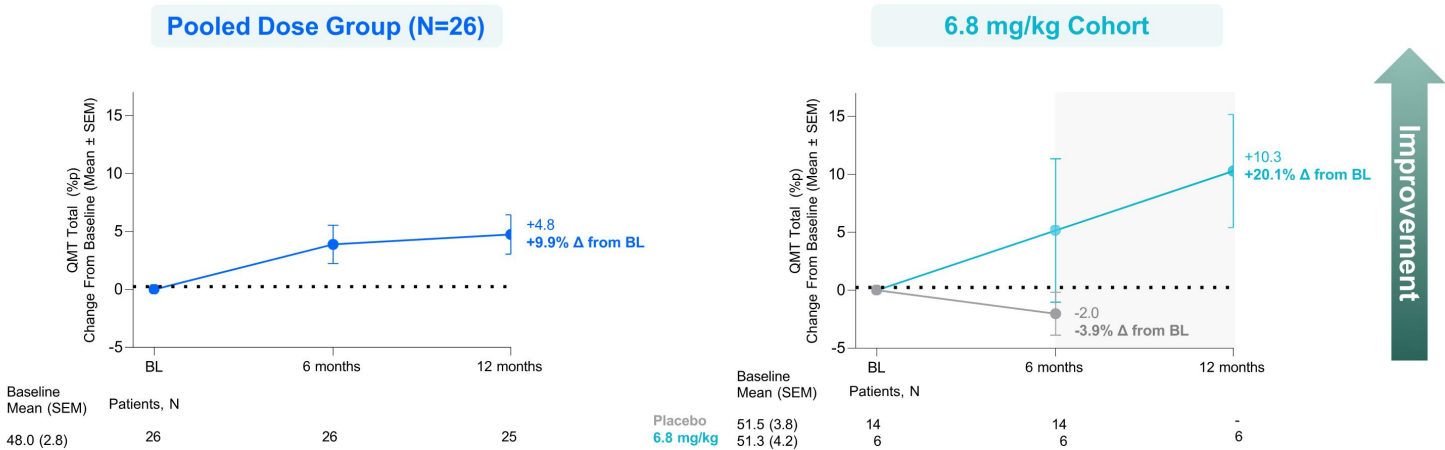
¹MDHI, N=13. Percent mean change from baseline = (mean change from baseline/baseline mean) x 100. 6 months = 169 days; 12 months = 337 days.

For information on the Pooled Dose Group, see footnotes on slide 7

%p, percent predicted; 10MWR, 10-Meter Walk/Run; 5xSTS, 5 Times Sit to Stand; BL, baseline; M, months; MDHI, myotonic dystrophy health index; Q4W, every 4 weeks; Q8W, every 8 weeks; QMT, quantitative muscle testing; sec, seconds; SEM, standard error of the mean; vHOT, video hand opening time.

Improvement in Strength Is Sustained at 12 Months, as Measured by QMT (%p)

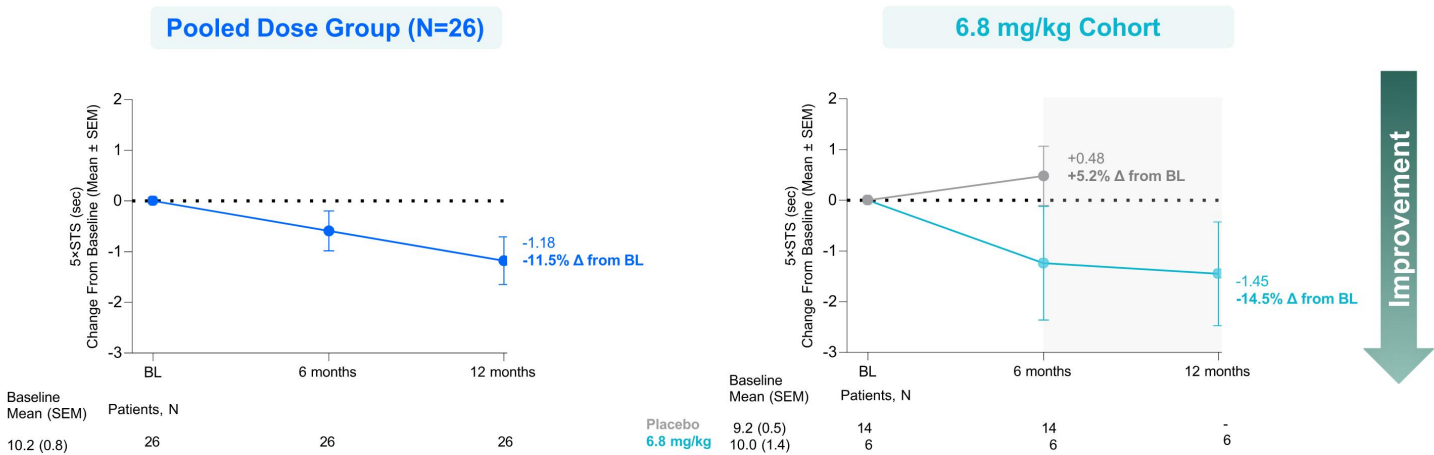
Quantitative Muscle Testing (QMT) Total Score



For information on the Pooled Dose Group, see footnotes on slide 7.
Percent mean change from baseline = (mean change from baseline/baseline mean) x 100. 6 months = 169 days; 12 months = 337 days.
%p, percent predicted; BL, baseline; Q4W, every 4 weeks; Q8W, every 8 weeks; QMT, quantitative muscle testing; SEM, standard error of the mean.

Improvement in Motor Function Is Sustained at 12 Months, as Measured by 5xSTS Timed Function Test

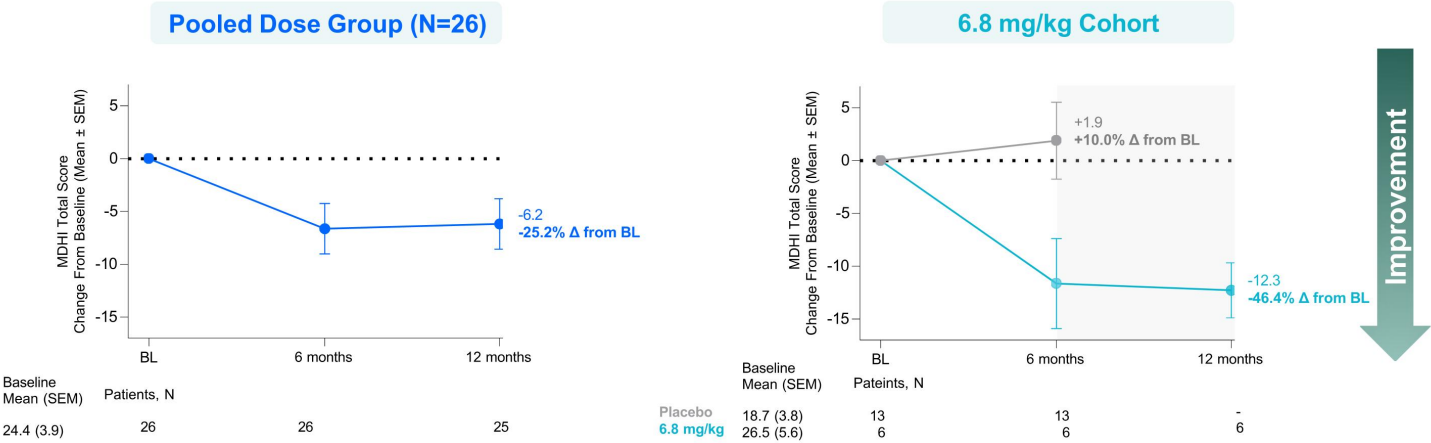
5 Times Sit to Stand



For information on the Pooled Dose Group, see footnotes on slide 7.
Percent mean change from baseline = (mean change from baseline/baseline mean) x 100. 6 months = 169 days; 12 months = 337 days.
5xSTS, 5 Times Sit to Stand; BL, baseline; Q4W, every 4 weeks; Q8W, every 8 weeks; sec, seconds; SEM, standard error of the mean.

Improvement in Patient-Reported Disease Burden Is Sustained at 12 Months, as Measured by MDHI Total Score

Myotonic Dystrophy Health Index (MDHI) Total Score



For information on the Pooled Dose Group, see footnotes on slide 7.
Percent mean change from baseline = (mean change from baseline/baseline mean) x 100. 6 months = 169 days; 12 months = 337 days.
BL, baseline; MDHI, Myotonic Dystrophy Health Index; Q4W, every 4 weeks; Q8W, every 8 weeks; SEM, standard error of the mean.

END-DM1¹ Assesses Natural DM1 Progression

END-DM1 is an ongoing, international, prospective DM1 natural history study designed to expand understanding of DM1 disease progression and to establish biomarkers and endpoints appropriate for interventional studies^{1,2}

Propensity-Matched Baseline Characteristics

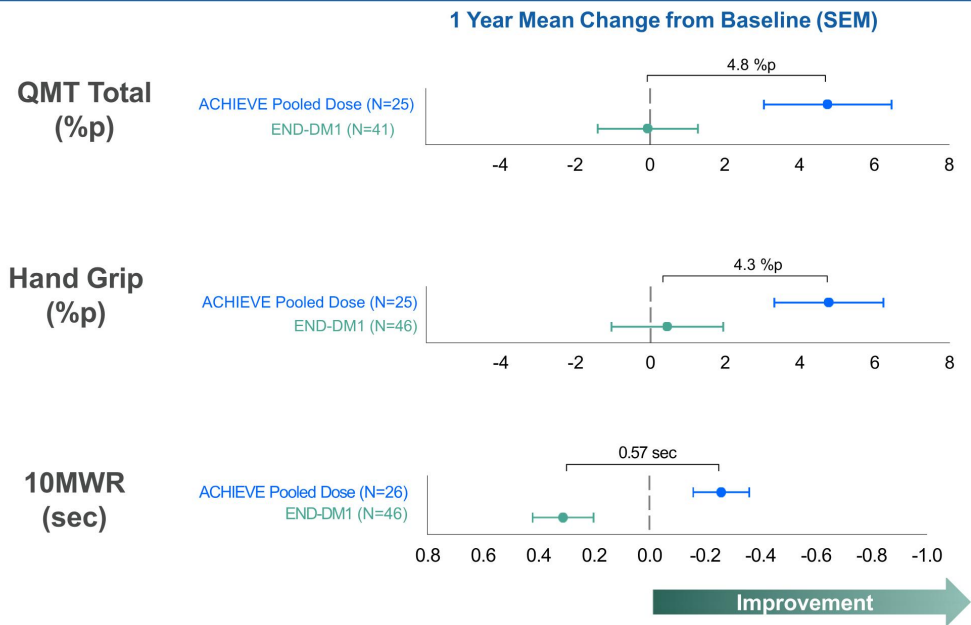
	END-DM1 Matched Cohort ³ Mean (SD)	ACHIEVE Pooled Dose Group Mean (SD)
N	49	26
Age at diagnosis (years)	27.5 (11.9)	23.7 (8.3)
Male (%)	19 (38.8)	14 (53.8)
Age at baseline (years)	36.1 (10.7)	35.6 (8.0)
BMI (kg/m ²)	23.3 (4.8)	22.7 (3.9)
vHOT (sec)	7.7 (4.6)	8.2 (4.4)
QMT Total (%p)	53.9 (17.6)	48.0 (14.3)
QMT hand grip (%p)	43.2 (22.8)	41.5 (16.3)
10MWR (sec)	4.19 (1.91)	3.99 (1.49)

Parameters for propensity matching included age, sex, and measures of strength and function.



Dyne Therapeutics is one of multiple companies funding the END-DM1 study, along with the US Food and Drug Administration (FDA) and the Myotonic Dystrophy Foundation (MDF). The Myotonic Dystrophy Clinical Research Network (DMCRN), consisting of END-DM1-participating centers, has extended permission to present these analyses. END-DM1 data transfer July 2026. Note: END-DM1 is an ongoing natural history study, and data comparisons are subject to change. For information on the Pooled Dose Group, see footnotes on slide 7.
¹ClinicalTrials.gov NCT03981575. <https://clinicaltrials.gov/study/NCT03981575>. Accessed September 14, 2026. ²Mul K, et al. *PLoS One*. 2025;20(12):e0331163.³The 5xSTS assessment was not included in the original END-DM1 protocol.
%p, percent predicted; 10MWR, 10-Meter Walk/Run; BMI, body mass index; DM1, myotonic dystrophy type 1; QMT, quantitative muscle testing; SD, standard deviation; sec, seconds; vHOT, video hand opening time.

ACHIEVE Pooled Dose Group Improves From Baseline and Diverges From Natural History in Strength and Ambulation

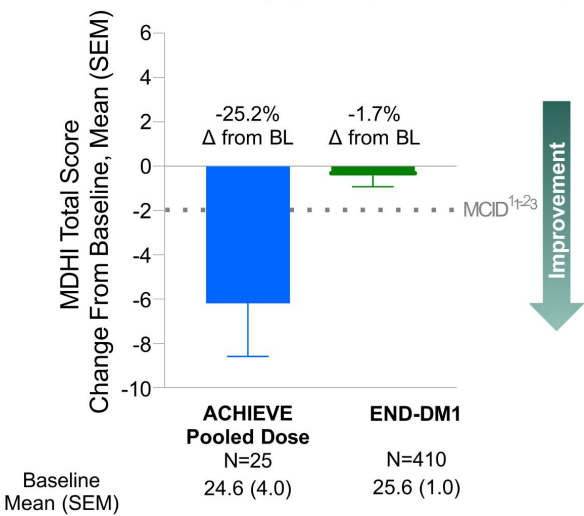


ACHIEVE

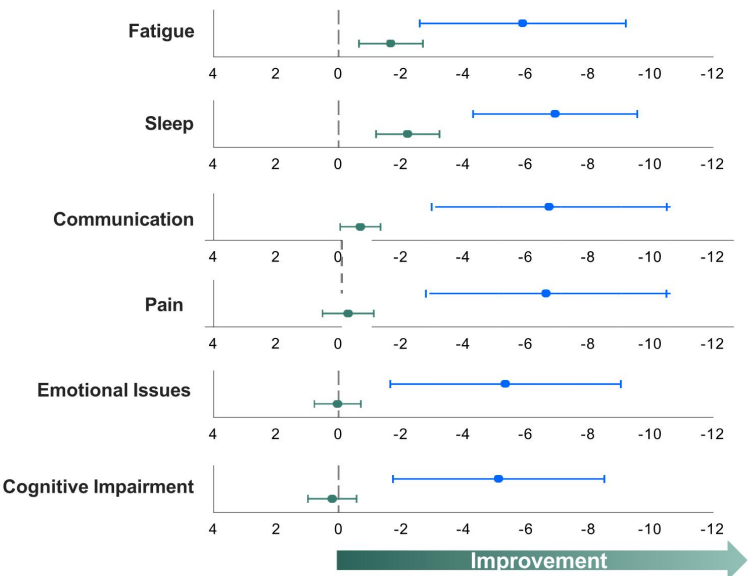
Thank you to END-DM1 Executive Committee for reviewing and approving use of this analysis.
N's reflect number of participants with assessments at baseline and Year 1. END-DM1 remains ongoing. 12 months = 337 days.
The 5xSTS assessment was not included in the original END-DM1 protocol. For information on the Pooled Dose Group, see footnotes on slide 7.
%p, percent predicted; 5xSTS, 5 Times Sit to Stand; 10MWR, 10-Meter Walk/Run; DM1, myotonic dystrophy type 1; Q4W, every 4 weeks; Q8W, every 8 weeks; QMT quantitative muscle testing; sec, second; SEM, standard error of the mean.

Improvement in MDHI in ACHIEVE Pooled Dose Group Exceeded MCID

MDHI Total Score Year 1
Pooled Dose Group (N=25) vs END-DM1 (N=410)



CNS-Related MDHI Subscales



Thank you to END-DM1 Executive Committee for reviewing and approving use of this analysis. For information on the Pooled Dose Group, see footnotes on slide 7.

¹Sansone V, et al. *Muscle & Nerve*. 2026;1–11. ²MDHI Total Score MCID values by Sansone, et al. 2026 were calculated utilizing an anchor-based approach with the domain delta questionnaire related to "overall health" at Year 2 upon study completion. MCID values ranged from 4.71 to -2.06. The mean change in MDHI Total Score for the ACHIEVE pooled dose group also exceeds MDC ESch-based proposed by Sansone et al. 2026. Mean change from baseline (SEM) is presented.

BL, baseline; DM1, myotonic dystrophy type 1; MCID, Minimal Clinically Important Difference; MDC, Minimal Detectable Change; MDHI, Myotonic Dystrophy Health Index; SEM, standard error of the mean; Y, year.

Summary: Z-Basivarsen ACHIEVE MAD Clinical Update



Safety

Safety findings through April 20, 2026, remain favorable



Efficacy

- **6.8 mg/kg cohort** demonstrated **durable functional improvement** in myotonia, motor function, and strength¹
- Data continues to support **vHOT as an early indicator of functional improvement**
- **Pooled dose analysis** (N=26) showed functional improvement at 1 year, with **consistent efficacy** trends observed across endpoints
- **Participants reported improvements in disease burden**, including CNS-related impacts



Divergence From Natural History²

- Z-basivarsen–treated participants **showed divergence from natural history** across functional and strength outcomes
- **Functional improvements** in MDHI Total Score exceeded MCID and diverged from natural history control



¹Sansone V, et al. Poster presentation at the Annual International Congress of the WMS, Vienna, Austria, October 7–11, 2025. Poster 380P. ²END-DM1 data transfer as of July 2026. Analysis presented with permission from the Myotonic Dystrophy Clinical Research Network (DMCRN). For information on the Pooled Dose Group, see footnotes on slide 7.

CNS, central nervous system; DM1, myotonic dystrophy type 1; MAD, multiple ascending dose; MDHI, Myotonic Dystrophy Health Index; vHOT, video hand opening time; z-basivarsen, zeleciment basivarsen.

Acknowledgments

- ACHIEVE participants, families, investigators, and site staff
- Myotonic Dystrophy Clinical Research Network (DMCRN) and END-DM1 participants, families, and research personnel
- Members of the DM1 community who have provided feedback, insights, and questions

To view the presentation, related, or the HARMONIA study design poster, please scan the QR codes below:

Presentation



Appendix



HARMONIA study design poster
Enrollment ongoing



DM1, myotonic dystrophy type 1.

Long-Term Functional Improvement With Zeleciment Basivarsen in the Phase 1/2 ACHIEVE Trial

Karlién Mul, MD, PhD¹, Guillaume Bassez², Jordi Diaz-Manera³, James Lilleker⁴, Marika Pane⁵, Richard Roxburgh⁶, Benedikt Schoser⁷, Christopher Turner⁸, Allison Kreuzer⁹, Alexandra Lovett⁹, Soma Ray⁹, Huaihou Chen⁹, Shauna Andersson⁹, Douglas Kerr⁹, Valeria Sansone¹⁰

¹Radboud University Medical Center, Nijmegen, the Netherlands; ²Institut de Myologie, Paris, France; ³John Walton Muscular Dystrophy Research Centre, Newcastle University, Newcastle-Upon-Tyne, UK; ⁴Muscle Disease Unit, Northern Care Alliance NHS Foundation Trust, Manchester Academic Health Science Centre, Manchester, UK; ⁵Fondazione Policlinico Universitario A. Gemelli, Rome, Italy; ⁶Neurogenetics Clinic Centre for Brain Research, University of Auckland, Auckland, NZ; ⁷Friedrich-Baur-Institute, Dep. of Neurology LMU Clinics, Ludwig-Maximilians University, Munich, Germany; ⁸University College London Hospitals, London, UK; ⁹Dyne Therapeutics, Inc., Waltham, MA, USA; ¹⁰Centro Clinico NeMO, University of Milan, Milan, Italy

WMS2026
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**The 31st Annual Congress of
the World Muscle Society**
29 September - 3 October 2026, Hiroshima, Japan

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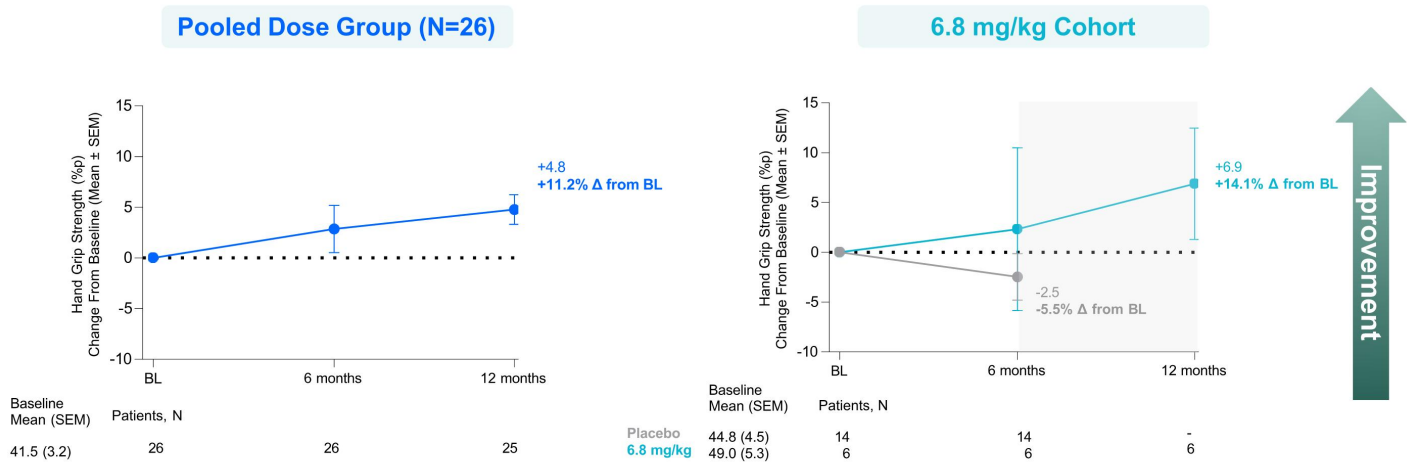


Appendix



Improvement in Strength Is Sustained at 12 Months, Including Hand Grip, as Measured by QMT (%p)

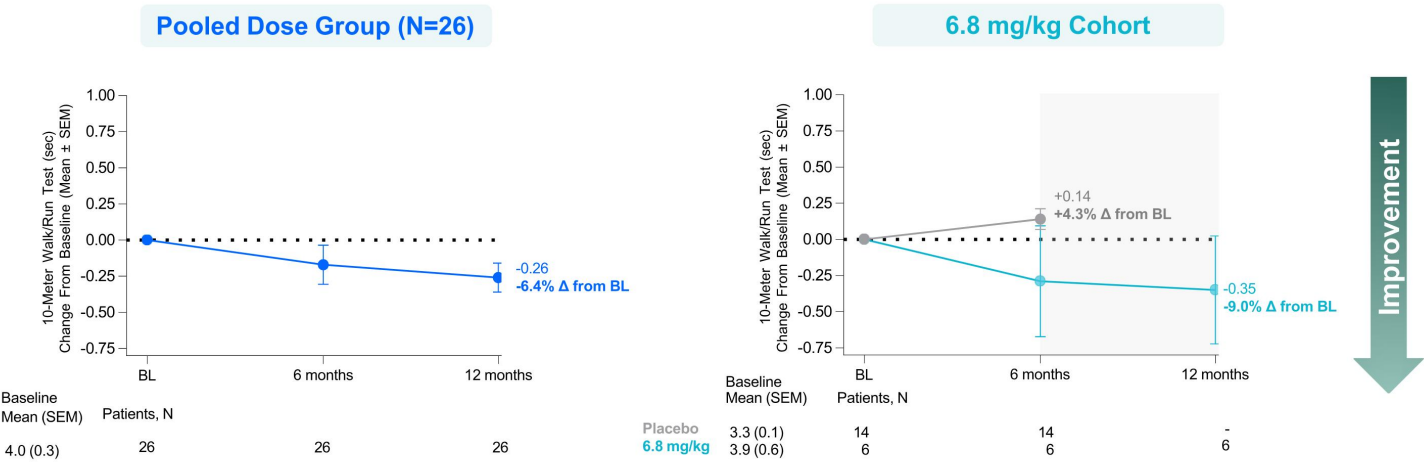
Quantitative Muscle Testing (QMT) Hand Grip Strength



For information on the Pooled Dose Group, see footnotes on slide 2.
Percent mean change from baseline = (mean change from baseline/baseline mean) x 100. 6 months = 169 days; 12 months = 337 days.
%p, percent predicted; BL, baseline; Q4W, every 4 weeks; Q8W, every 8 weeks; QMT, quantitative muscle testing; SEM, standard error of the mean.

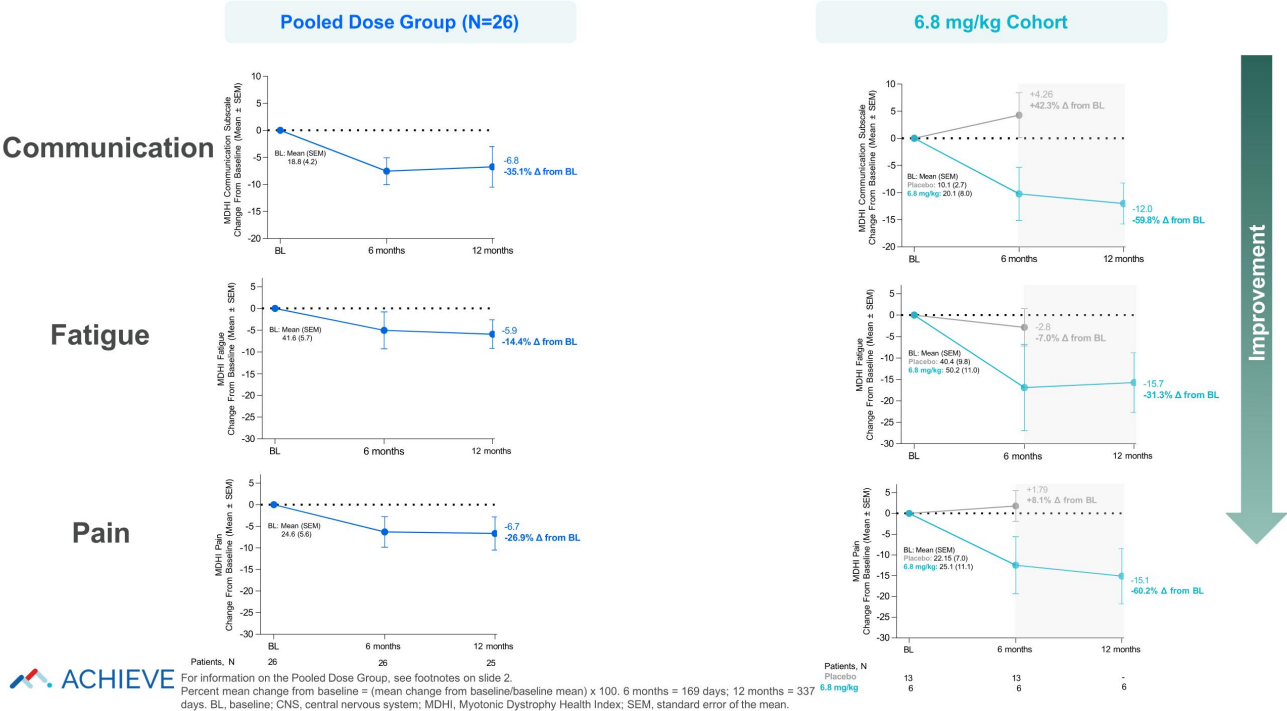
Improvement in Motor Function Is Sustained at 12 Months, as Measured by 10MWR Timed Function Test

10-Meter Walk/Run Test



For information on the Pooled Dose Group, see footnotes on slide 2.
Percent mean change from baseline = (mean change from baseline/baseline mean) x 100. 6 months = 169 days; 12 months = 337 days.
10MWR, 10-Meter Walk/Run; BL, baseline; Q4W, every 4 weeks; Q8W, every 8 weeks; sec, seconds; SEM, standard error of the mean.

Improvement Is Observed Across CNS-Related MDHI Subscales Through 12 Months



Improvement Is Observed Across CNS-Related MDHI Subscales Through 12 Months

