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)

September 29, 2026

- 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 (GLOBE NEWSWIRE) -- 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 zeleciment 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 “Zeleciment 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 zeleciment 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 zeleciment 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