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

October 6, 2025

- Robust improvement demonstrated across diverse set of clinical measures -

- Patient-reported outcomes support clinical meaningfulness of improvements in function and strength -

- Meaningful improvements in overall disease burden reported by both patients and physicians -

WALTHAM, Mass., Oct. 06, 2025 (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 data from its ongoing Phase 1/2 ACHIEVE clinical trial of zeleciment basivarsen (z-basivarsen, formerly known as DYNE-101), in patients with myotonic dystrophy type 1 (DM1) demonstrating clinically meaningful improvements in function and strength at the selected registrational dose. These data are being presented at the 30th Annual International Congress of the World Muscle Society (WMS), held virtually and in Vienna, Austria, October 7-11, 2025.

“This week we are presenting additional analyses from the data cut shared in June showing that the improvements in function and strength span both the upper and lower limbs, and are clearly meaningful to both patients and physicians,” said Doug Kerr, M.D., Ph.D., chief medical officer of Dyne. “Z-basivarsen was designed to deliver broad functional improvement to patients, and we believe it has the unique potential to mitigate central nervous system-related manifestations of the disease such as cognitive impairment, sleep disturbances and fatigue.”

“I believe the data for z-basivarsen support its potential to bring a wide range of benefits to DM1 patients, helping to improve their ability to function and carry out their daily lives in a way that will really matter to them,” said Valeria Sansone, M.D., Ph.D., Clinical and Scientific Director at the Clinical Center NeMO in Milan, Professor of Neurology, University of Milan, and principal investigator in the ACHIEVE trial. “The consistency of these data across a variety of endpoints out to one-year increases my level of confidence in the potential of z-basivarsen. The patient perception of improvements are encouraging and provide initial evidence that there may be a broad and meaningful effect with z-basivarsen treatment.”

The one-year data presented at WMS this week come from adults with DM1 (n=6) enrolled in the cohort assessing the selected registrational dose of 6.8 mg/kg Q8W in the randomized, placebo-controlled multiple ascending dose (MAD) portion of the ACHIEVE trial. These results are from an additional analysis of the same cohort and timepoint for which data were previously reported on June 17, 2025.

Data to be presented at WMS include previously disclosed as well as new findings:

- **Myotonia:** Robust and sustained improvement from baseline in hand myotonia, as measured by video hand opening time (vHOT).
- **Function:** Meaningful and sustained improvements from baseline in multiple functional endpoints, including data from the 10-Meter Walk/Run Test (10MWR) and the 5 Times Sit to Stand Test (5xSTS), as well as:
  - **New data** showing improvement in the 9-Hole Peg Test (9HPT), a measure of upper limb function focused on manual dexterity and coordination, which is frequently used across neurological conditions.
- **Strength:** Meaningful and sustained improvement from baseline on muscle strength as assessed by Quantitative Muscle Testing (QMT), as well as:
  - **New data** showing improvements in all QMT scores across both the upper and lower extremities: hand grip strength, elbow flexion, elbow extension, ankle dorsiflexion, knee flexion, and knee extension.
- **Patient Reported Outcomes:** Meaningful and 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), as well as:
  - **New data** showing improvements in other subscales including mobility, ability to do activities, and upper extremity function. These data support the clinical meaningfulness of the improvements seen on QMT, 10MWR, 5xSTS, and 9-HPT.
- **Overall Disease Burden:** Improvements from baseline in both patient and clinician impressions of global function, based on:
  - **New data** from Patient Global Impression of Change (PGI-C) and Clinician Global Impression of Change (CGI-C) scales.
- **Safety and Tolerability:** Previously reported safety and tolerability data from 56 patients enrolled through the 6.8 mg/kg Q8W cohort of the ACHIEVE trial. Z-basivarsen demonstrated a favorable safety profile, and no related serious treatment

emergent adverse events were identified[1].

These data will be presented in a poster titled, “DYNE-101 Targets the Underlying Cause of DM1 to Enable Multi-system Functional Improvement in the ACHIEVE Trial.”

Additional Dyne Presentations at the 30th Annual International Congress of the World Muscle Society

Dyne will also present encore data on zeleciment rostudirsen (z-rostudirsen, formerly known as DYNE-251, an investigational medicine being evaluated for the treatment of individuals with *DMD* mutations amenable to exon 51 skipping) as well as preclinical Duchenne muscular dystrophy (DMD) data with its FORCE platform.

- “DYNE-251 Targets the Underlying Cause of DMD to Enable Sustained Functional Improvement in Males with *DMD* Mutations Amenable to Exon 51 Skipping Enrolled in the Phase 1/2 DELIVER Trial” (encore presentation)
- “The FORCE™ Platform Enables TfR1-mediated Delivery of Exon Skipping PMO to the CNS and Resolves Anxiety in a Mouse Model of DMD” (encore presentation)

All poster presentations, including the poster titled, “DYNE-101 Targets the Underlying Cause of DM1 to Enable Multi-system Functional Improvement in the ACHIEVE Trial,” are now available in the Scientific Publications & Presentations section of Dyne’s website.

Additionally, a symposium titled “Achieving Functional Improvement in Myotonic Dystrophy Type 1 (DM1): Defining Goals of Treatment and a Roadmap to Multidisciplinary Care” will be held on Wednesday, October 8 at 7:45 a.m. CEST. Slides from the symposium will be available in the Scientific Publications & Presentations section of Dyne’s website on Wednesday, October 8 at the commencement of the symposium.

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, formerly 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 has been initiated to support potential regulatory submissions, including Accelerated Approval in the U.S. 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, formerly known as DYNE-101)

Z-basivarsen is an investigational therapeutic being evaluated in the Phase 1/2 global ACHIEVE 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 and Orphan Drug designation by the European Medicines Agency 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 varies. 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, cardiac arrhythmias, respiratory issues, gastrointestinal dysfunction, cognitive impairments, excessive daytime sleepiness, fatigue and dysregulated sleep. 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 myotonic dystrophy type 1 (DM1) and Duchenne muscular dystrophy (DMD), and preclinical programs for facioscapulohumeral muscular dystrophy (FSHD) and Pompe disease. 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”) and its potential to mitigate central nervous system-related manifestations of myotonic dystrophy type 1, expectations regarding the timing and outcome of interactions with global regulatory authorities and the availability of accelerated approval pathways for z-basivarsen, the potential of video hand opening time to serve as an intermediate clinical endpoint for U.S. accelerated approval, and plans to provide future updates on pipeline programs, 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,” 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 (DYNE-101) safety data as of April 23, 2025.

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