Dyne Therapeutics Reports Third Quarter 2023 Financial Results and Provides Update on Significant Progress for ACHIEVE and DELIVER Trials and Upcoming Clinical Milestones

October 30, 2023

- Initial ACHIEVE and DELIVER Data to be Presented at a Company Event Around the J.P. Morgan Healthcare Conference in Early January 2024, Including Safety, Biomarker of Splicing and Functional Outcome of Myotonia in DM1; Safety and Dystrophin Expression in DMD -

- ACHIEVE Trial of DYNE-101 in DM1 Fully Enrolled Through 3.4 mg/kg Cohort; DELIVER Trial of DYNE-251 in DMD Fully Enrolled Through 10 mg/kg Cohort -

- Safety Profile in ACHIEVE and DELIVER Has Supported Dose Escalation to a Combined Nine Cohorts with Over 300 Doses Administered to Date -

WALTHAM, Mass., Oct. 30, 2023 (GLOBE NEWSWIRE) -- Dyne Therapeutics, Inc. (Nasdaq: DYN), a clinical-stage muscle disease company focused on advancing innovative life-transforming therapeutics for people living with genetically driven diseases, today reported financial results for the third quarter of 2023 and provided an update on progress and upcoming milestones for its Phase 1/2 ACHIEVE and DELIVER clinical trials.

"We've made tremendous progress in our ACHIEVE and DELIVER trials with more than 72 patients enrolled and over 300 doses administered thus far. To date, the safety profile in the clinic has been favorable for DYNE-101 and DYNE-251 and has supported dose escalation to a combined nine cohorts across both trials. The enthusiasm amongst the DM1 and DMD communities reflects the significant unmet need and the potential for our investigational therapies to deliver transformative outcomes for patients," said Joshua Brumm, president and chief executive officer of Dyne. "We are moving with great excitement towards reporting initial data from ACHIEVE and DELIVER, including the important biomarker of splicing and functional outcome of myotonia in DM1 and dystrophin in DMD. We look forward to sharing both readouts around the time of the J.P. Morgan Healthcare Conference in early January 2024."

ACHIEVE Trial of DYNE-101 in DM1

Clinical Update

- ACHIEVE is a Phase 1/2 global clinical trial evaluating DYNE-101 in adult patients with myotonic dystrophy type 1 (DM1) who are 18 to 49 years of age. ACHIEVE, which is designed to be a registrational trial, consists of a 24-week multiple ascending dose (MAD), randomized, placebo-controlled period, a 24-week open-label extension (OLE) and a 96-week long-term extension. The primary endpoints are safety and tolerability, with secondary endpoints of pharmacokinetics and pharmacodynamics, including change from baseline in splicing, as well as measures of muscle strength and function.

- In the MAD portion of ACHIEVE:
  - Enrollment is complete in the 1.8 mg/kg (approximate ASO dose) cohort (n=16) evaluating once every four-week dosing and the 3.4 mg/kg cohort evaluating once every four-week dosing (n=16) and every eight-week dosing (n=8). A total of 40 patients have been enrolled in these two cohorts with over 150 doses administered to date.
  - All patients who completed the 24-week MAD portion of ACHIEVE have entered the 24-week OLE.
  - The safety profile of DYNE-101 supported dose escalation to cohort 3 (up to 6.8 mg/kg), which is currently enrolling participants. Per the protocol, further dose escalation to cohort 4 (up to 10.2 mg/kg) will be based on review of safety data from cohort 3, when available, and the trial overall.
    - To date, no participants have demonstrated treatment-emergent anemia and there have been no discontinuations.

Planned Reporting of Initial Data

- In early January 2024 at a company event around the time of the 42nd Annual J.P. Morgan Healthcare Conference, Dyne anticipates reporting from the ACHIEVE trial:
  - Safety and tolerability data from multiple cohorts.
  - Splicing data and analysis of the video hand opening time (vHOT) myotonia functional assessment from at least the 1.8 mg/kg cohort at 6 months.

DELIVER Trial of DYNE-251 in DMD

Clinical Update

- DELIVER is a Phase 1/2 global clinical trial evaluating DYNE-251 in ambulant and non-ambulant males with Duchenne muscular dystrophy (DMD) who are ages 4 to 16 and have mutations amenable to exon 51 skipping. DELIVER, which is designed to be a registrational trial, consists of a 24-week MAD, randomized, placebo-controlled period, a 24-week OLE and a 96-week long-term extension. The primary endpoints are safety, tolerability and change from baseline in dystrophin

levels as measured by Western blot. Secondary endpoints include measures of muscle function, exon skipping and pharmacokinetics.

- In the MAD portion of DELIVER:
  - Enrollment is complete through the 10 mg/kg (approximate PMO dose) cohort evaluating once every four-week dosing. A total of 32 patients have been enrolled across the first five cohorts (0.7 mg/kg (n=6), 1.4 mg/kg (n=6), 2.8 mg/kg (n=6), 5 mg/kg (n=6) and 10 mg/kg (n=8)) with over 175 doses administered to date.
  - All patients who completed the 24-week MAD portion of DELIVER have entered the 24-week OLE.
  - The safety profile of DYNE-251 supported dose escalation to cohort 6 (up to 20 mg/kg), which is currently enrolling participants. Per the protocol, further dose escalation to cohort 7 (up to 40 mg/kg) will be based on review of safety data from cohort 6, when available, and the trial overall.
    - To date, no participants have demonstrated treatment-emergent anemia and there have been no discontinuations.

Planned Reporting of Initial Data

- In early January 2024 at a company event around the time of the 42nd Annual J.P. Morgan Healthcare Conference, Dyne anticipates reporting from the DELIVER trial:
  - Safety and tolerability data from multiple cohorts.
  - Dystrophin expression data from the 5 mg/kg cohort at 6 months.

Third Quarter 2023 Financial Results

Cash position: Cash, cash equivalents and marketable securities were $157.8 million as of September 30, 2023, which is anticipated to fund operations through 2024.

Research and development (R&D) expenses: R&D expenses were $55.3 million for the quarter ended September 30, 2023, compared to $34.7 million for the quarter ended September 30, 2022.

General and administrative (G&A) expenses: G&A expenses were $7.0 million for the quarter ended September 30, 2023, compared to $7.6 million for the quarter ended September 30, 2022.

Net loss: Net loss for the quarter ended September 30, 2023 was $60.2 million, or $0.99 per basic and diluted share. This compares with a net loss of $41.4 million, or $0.80 per basic and diluted share, for the quarter ended September 30, 2022.

About Dyne Therapeutics

Dyne Therapeutics is a clinical-stage muscle disease company focused on advancing innovative life-transforming therapeutics for people living with genetically driven diseases. With its proprietary FORCE™ platform, Dyne is developing modern oligonucleotide therapeutics that are designed to overcome limitations in delivery to muscle tissue. Dyne has a broad pipeline for serious muscle diseases, including clinical programs for myotonic dystrophy type 1 (DM1) and Duchenne muscular dystrophy (DMD) and a preclinical program for facioscapulohumeral muscular dystrophy (FSHD). For more information, please visit 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 anticipated timelines for reporting data from the DYNE-251 and DYNE-101 clinical trials, the trial design of the DYNE-251 and DYNE-101 clinical trials, and the sufficiency of Dyne's existing cash resources for the period anticipated, 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 initiate and enroll patients in clinical trials; whether results from preclinical studies and clinical trials will be predictive of the results of later preclinical studies and clinical trials; whether Dyne's cash resources will be sufficient to fund the Company's 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.

Dyne Therapeutics, Inc.
Condensed Consolidated Statement of Operations (Unaudited)

(in thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	**2022**	**2023**	**2022**
Operating expenses:				
Research and development	$ 55,251	$ 34,670	$ 151,918	$ 109,570
General and administrative	7,022	7,609	22,556	21,247
Total operating expenses	**62,273**	**42,279**	**174,474**	**130,817**
Loss from operations	(62,273)	(42,279)	(174,474)	(130,817)
Other (expense) income, net	2,063	894	5,175	1,545
Net loss	**$ (60,210)**	**$ (41,385)**	**$ (169,299)**	**$ (129,272)**
Net loss per share—basic and diluted	$ (0.99)	$ (0.80)	$ (2.86)	$ (2.50)
Weighted-average common shares outstanding used in net loss per share—basic and diluted	61,109,917	51,795,446	59,107,795	51,692,899

Dyne Therapeutics, Inc.
Condensed Consolidated Balance Sheet Data (Unaudited)
(in thousands)

	September 30, 2023	December 31, 2022
Assets		
Cash, cash equivalents and marketable securities	$ 157,823	$ 256,012
Other assets	47,421	50,313
Total assets	**$ 205,244**	**$ 306,325**
Liabilities and Stockholders' Equity		
Liabilities	53,206	53,961
Stockholders' equity	152,038	252,364
Total liabilities and stockholders' equity	**$ 205,244**	**$ 306,325**

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