Dyne Therapeutics Reports Third Quarter 2024 Financial Results and Provides Corporate Update

November 12, 2024

- IND Application for DYNE-101 for DM1 Cleared by FDA -

- New Clinical Data from DYNE-101 ACHIEVE Trial Expected in Early January 2025 -

- Enrolling Registrational Cohort of DYNE-251 DELIVER Trial in DMD -

WALTHAM, Mass., Nov. 12, 2024 (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 2024 and provided a corporate update.

"We've made significant progress in our ACHIEVE and DELIVER trials. We are very pleased to have received IND clearance for DYNE-101 and plan to report in early January additional data from the ACHIEVE trial in DM1, including from the 6.8 mg/kg cohort, which will inform our go-forward dose and dose regimen. Based on the encouraging biomarker and functional data from the DELIVER trial of DYNE-251 in DMD, we are enrolling patients in a registrational cohort at 20 mg/kg. We continue to pursue expedited approval pathways for both DYNE-101 and DYNE-251," said John Cox, president and chief executive officer of Dyne. "Our strong financial foundation positions us to advance our clinical programs as well as our pipeline to address the significant unmet needs of people living with neuromuscular diseases."

ACHIEVE Trial of DYNE-101 in DM1

- The U.S. Food and Drug Administration (FDA) has cleared the Investigational New Drug (IND) application for DYNE-101, which is being evaluated in the ongoing, global Phase 1/2 ACHIEVE trial in adults with myotonic dystrophy type 1 (DM1). The ACHIEVE trial currently includes 56 participants and is fully enrolled through the 6.8 mg/kg Q8W cohort (approximate ASO dose).
- Dyne anticipates reporting in early January 2025 new data from the ACHIEVE trial, including safety and tolerability, change from baseline in splicing, video hand opening time (vHOT) assessment, functional measures, as well as patient-reported outcomes.
    - Efficacy data will be shared from the 6.8 mg/kg cohort up to 6 months and the 5.4 mg/kg and 3.4 mg/kg cohorts up to 12 months.
    - Longer-term safety and tolerability data from all participants will be reported.
- Dyne continues to pursue expedited approval pathways globally for DYNE-101 utilizing splicing as a surrogate endpoint.

DELIVER Trial of DYNE-251 in DMD

- In September 2024, Dyne reported positive efficacy data and a favorable safety profile[1] from the ongoing Phase 1/2 global DELIVER trial of DYNE-251 in males with Duchenne muscular dystrophy (DMD) mutations amenable to exon 51 skipping. Based on these data, Dyne has begun enrolling a 20 mg/kg Q4W (approximate PMO dose) registrational cohort of 32 participants as part of the DELIVER trial. The Company continues to pursue expedited approval pathways for DYNE-251, including accelerated approval in the U.S. based on dystrophin as a surrogate endpoint.

Third Quarter 2024 Financial Results

Cash position: Cash, cash equivalents and marketable securities were $723.7 million as of September 30, 2024, which is anticipated to fund operations at least into the second half of 2026.

Research and development (R&D) expenses: R&D expenses were $92.8 million for the quarter ended September 30, 2024, compared to $55.3 million for the quarter ended September 30, 2023.

General and administrative (G&A) expenses: G&A expenses were $12.9 million for the quarter ended September 30, 2024, compared to $7.0 million for the quarter ended September 30, 2023.

Net loss: Net loss for the quarter ended September 30, 2024 was $97.1 million, or $0.96 per basic and diluted share. This compares with a net loss of $60.2 million, or $0.99 per basic and diluted share, for the quarter ended September 30, 2023.

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 potential of DYNE-101 and DYNE-251, the anticipated timelines for reporting additional data from the ACHIEVE and DELIVER clinical trials, the availability of expedited approval pathways for DYNE-101 and DYNE-251, expectations regarding the timing and outcome of interactions with global regulatory authorities, the sufficiency of Dyne's cash resources for the period anticipated, 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.

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,	
	2024	2023	2024	2023
Operating expenses:				
Research and development	$ 92,800	$ 55,251	$ 199,601	$ 151,918
General and administrative	12,859	7,022	47,177	22,556
Total operating expenses	**105,659**	**62,273**	**246,778**	**174,474**
Loss from operations	(105,659)	(62,273)	(246,778)	(174,474)
Other income (expense), net	8,534	2,063	18,902	5,175
Net loss	**$ (97,125)**	**$ (60,210)**	**$ (227,876)**	**$ (169,299)**
Net loss per share—basic and diluted	$ (0.96)	$ (0.99)	$ (2.49)	$ (2.86)
Weighted average common shares outstanding used in net loss per share—basic and diluted	100,882,042	61,109,917	91,511,621	59,107,795

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

	September 30, 2024	December 31, 2023
Assets		
Cash, cash equivalents and marketable securities	$ 723,674	$ 123,100
Other assets	45,170	41,982
Total assets	**$ 768,844**	**$ 165,082**
Liabilities and Stockholders' Equity		
Liabilities	63,304	73,790
Stockholders' equity	705,540	91,292
Total liabilities and stockholders' equity	**$ 768,844**	**$ 165,082**

1. DYNE-251 safety data as of August 21, 2024.

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