Dyne Therapeutics Reports Fourth Quarter and Full Year 2023 Financial Results and Recent Business Highlights

March 5, 2024

- Positive Initial Clinical Data from ACHIEVE Trial in DM1 Patients and DELIVER Trial in DMD Patients Demonstrated Proof-of-Concept, Validating the Promise of the FORCE™ Platform and Targeted Delivery to Muscle -

- Strengthened Balance Sheet with $345 Million Public Offering Extending Projected Cash Runway Through 2025 -

WALTHAM, Mass., March 05, 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 fourth quarter and full year 2023 and recent business highlights.

"We've had an exciting start to 2024 highlighted by Dyne's first clinical data demonstrating proof-of-concept in our DM1 and DMD programs that validated the promise of the FORCE™ platform in developing targeted therapeutics for people living with rare muscle diseases. We were thrilled to see dose-dependent results in our ACHIEVE trial in DM1 as well as meaningful improvement in myotonia at the lowest dose, and in our DELIVER trial to exceed the level of dystrophin production reported for the standard of care for DMD exon 51 with a fraction of the dose. The strength of these data formed the foundation of the $345 million offering we completed in January 2024, which has extended our projected cash runway through 2025," said Joshua Brumm, president and chief executive officer of Dyne. "In 2024, we are focused on leveraging the favorable safety profiles for both DYNE-101 and DYNE-251 and the adaptive nature of our ACHIEVE and DELIVER trials to optimize dose and dose regimen. We plan to provide an update from both trials in the second half of 2024, including reporting data from multiple, higher dose cohorts. We also continue to pursue expedited regulatory pathways as part of our commitment to move with a sense of urgency to deliver potentially transformative therapies for the DM1 and Duchenne communities."

Highlights from Clinical Programs

Phase 1/2 ACHIEVE Trial of DYNE-101 in DM1

- In January 2024, Dyne reported initial clinical data from the Phase 1/2 ACHIEVE trial of DYNE-101 in adult patients with myotonic dystrophy type 1 (DM1).
    - The initial efficacy assessment was based on data from 32 patients enrolled in the randomized, placebo-controlled multiple ascending dose (MAD) portion of the trial, including 6-month data from the 1.8 mg/kg (approximate ASO dose) cohort and 3-month data from the 3.4 mg/kg Q4W cohort. DYNE-101 demonstrated a dose-dependent splicing correction as well as an increase in muscle delivery and *DMPK* knockdown. Patients treated with 1.8 mg/kg Q4W of DYNE-101 also experienced functional improvement in myotonia as measured by video hand opening time (vHOT) and overall improvement in the Myotonic Dystrophy Health Index (MDHI) patient reported outcome, including the fatigue subscale, suggesting potential benefit in the central nervous system.
    - Safety and tolerability data were based on 45 patients enrolled through the 5.4 mg/kg Q8W cohort of the MAD portion. DYNE-101 demonstrated a favorable safety profile as of the data cutoff date[1].
- The ACHIEVE trial is fully enrolled through the 5.4 mg/kg Q8W cohort and is currently enrolling participants in the 6.8 mg/kg Q8W cohort.

Phase 1/2 DELIVER Trial of DYNE-251 in DMD

- In January 2024, Dyne reported initial clinical data from the Phase 1/2 DELIVER trial of DYNE-251 in Duchenne muscular dystrophy (DMD).
    - The initial efficacy assessment was based on 6-month data from 6 male patients with DMD amenable to exon 51 skipping enrolled in the 5 mg/kg (approximate PMO dose) cohort of the randomized, placebo-controlled MAD portion of the trial. Once every 4-week administration of DYNE-251 reached levels of dystrophin expression, exon skipping and percent dystrophin positive fibers that exceeded levels reported in a clinical trial for the current weekly standard of care for DMD exon 51, eteplirsen, at 6 months[2] with a 24-fold lower total PMO dose.
    - Safety and tolerability data were based on 37 patients enrolled through the 20 mg/kg cohort of the MAD portion. DYNE-251 demonstrated a favorable safety profile as of the data cutoff date[1].
- The DELIVER trial is fully enrolled through the 20 mg/kg Q4W cohort and is currently enrolling participants in the 40 mg/kg Q8W cohort.

Other Business Highlights

- In January 2024, Dyne completed an underwritten public offering of 19,722,500 shares of its common stock at a public offering price of $17.50 per share, which included 2,572,500 shares issued upon the exercise in full by the underwriters of their option to purchase additional shares of common stock in the offering. The gross proceeds to Dyne from the offering were approximately $345.1 million, before deducting underwriting discounts and commissions and offering expenses payable by Dyne.

Upcoming Events & Key 2024 Milestones

- Initial data from ACHIEVE and DELIVER reported in January 2024 will be featured in oral presentations at the Muscular Dystrophy Association (MDA) Clinical and Scientific Conference on March 6, 2024.
- Dyne anticipates reporting data from multiple, higher dose cohorts from both the ACHIEVE and the DELIVER trials in the second half of 2024 with the goal of initiating registrational cohorts by the end of 2024.

Fourth Quarter and Full Year 2023 Financial Results

Cash position: Cash, cash equivalents and marketable securities were $123.1 million as of December 31, 2023. In January 2024, the Company completed an underwritten public offering of 19,722,500 shares of its common stock for net proceeds of approximately $323.9 million. The Company expects that its cash, cash equivalents and marketable securities as of December 31, 2023, together with the net proceeds from the January 2024 underwritten public offering, will be sufficient to fund its operations through 2025.

Research and development (R&D) expenses: R&D expenses were $58.8 million and $33.2 million for the quarters ended December 31, 2023 and 2022, respectively. R&D expenses were $210.8 million and $142.8 million for the years ended December 31, 2023 and 2022, respectively.

General and administrative (G&A) expenses: G&A expenses were $8.8 million and $7.0 million for the quarters ended December 31, 2023 and 2022, respectively. G&A expenses were $31.4 million and $28.2 million for the years ended December 31, 2023 and 2022, respectively.

Net loss: Net loss for the quarter ended December 31, 2023 was $66.6 million, or $1.09 per basic and diluted share. This compares with a net loss of $38.8 million, or $0.74 per basic and diluted share, for the quarter ended December 31, 2022. Net loss for the year ended December 31, 2023 was $235.9 million, or $3.95 per basic and diluted share. This compares with a net loss of $168.1 million, or $3.23 per basic and diluted share, for the year ended December 31, 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 trial design of the DYNE-101 and DYNE-251 clinical trials, the anticipated timelines for reporting data from the DYNE-101 and DYNE-251 clinical trials and initiating registrational cohorts, plans to optimize dose and dose regimen for DYNE-101 and DYNE-251 and the sufficiency of Dyne's 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 initial data from early clinical trials will be predictive of the final results of the clinical trials or future 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-K 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. Safety data as of December 6, 2023

2. No head-to-head trials have been conducted comparing DYNE-251 to eteplirsen. Eteplirsen data may not be directly comparable due to differences in trial protocols, dosing regimens and patient populations. Accordingly, these cross-trial comparisons may not be reliable. Eteplirsen data from *J Neuromuscul Dis*. 2021; 8(6): 989–1001

Dyne Therapeutics, Inc.
Condensed Consolidated Statement of Operations

(in thousands, except share and per share data)

	Three Months Ended December 31,		Year Ended December 31,	
	2023	**2022**	**2023**	**2022**
Operating expenses:				
Research and development	$ 58,843	$ 33,191	$ 210,762	$ 142,760
General and administrative	8,846	6,955	31,400	28,202
Total operating expenses	**67,689**	**40,146**	**242,162**	**170,962**
Loss from operations	(67,689)	(40,146)	(242,162)	(170,962)
Other (expense) income, net	1,050	1,319	6,225	2,863
Net loss	**$ (66,639)**	**$ (38,827)**	**$ (235,937)**	**$ (168,099)**
Net loss per share, basic and diluted	$ (1.09)	$ (0.74)	$ (3.95)	$ (3.23)
Weighted average common shares outstanding, basic and diluted	61,393,409	52,817,413	59,683,851	51,976,343

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

	December 31,	
	2023	**2022**
Assets		
Cash, cash equivalents and marketable securities	$ 123,100	$ 256,012
Other assets	41,982	50,313
Total Assets	**$ 165,082**	**$ 306,325**
Liabilities and Stockholders' Equity		
Liabilities	73,790	53,961
Stockholders' equity	91,292	252,364
Total liabilities and stockholders' equity	**$ 165,082**	**$ 306,325**

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