Dyne Therapeutics Reports Second Quarter 2024 Financial Results and Recent Business Highlights

August 12, 2024

- Recent Data from ACHIEVE Trial of DYNE-101 in DM1 and DELIVER Trial of DYNE-251 in DMD Demonstrated Compelling Impact on Key Disease Biomarkers and Improvement in Multiple Functional Endpoints -

- Strengthened Balance Sheet with $374 Million Public Offering Extending Projected Cash Runway At Least Into the Second Half of 2026 -

WALTHAM, Mass., Aug. 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 second quarter of 2024 and recent business highlights.

"The second quarter of 2024 was an exciting one for our clinical programs and pipeline. We reported new clinical data from our ACHIEVE and DELIVER trials, demonstrating meaningful impact on key biomarkers and functional improvements that reflect best-in-class potential. We are evaluating the clinical profile of different dose and dose regimens with the goal of transforming treatment for individuals living with DM1 and DMD. Based on our compelling clinical data and productive regulatory interactions, we continue to pursue expedited approval pathways and plan to provide an update on the path to registration for both programs by year-end," said John Cox, Dyne's president and chief executive officer. "Additionally, we highlighted the modularity of our FORCE™ platform with the presentation of robust preclinical data supporting our FSHD program, and for the first time, showed the ability of our platform to deliver enzyme replacement therapy to muscle and CNS in a Pompe disease model. We also strengthened our balance sheet to support the promise of our portfolio."

Recent Highlights and Anticipated Milestones

Phase 1/2 ACHIEVE Trial of DYNE-101 in myotonic dystrophy type 1 (DM1)

- In May 2024, Dyne reported positive clinical data from the ongoing ACHIEVE trial of DYNE-101 in adult patients with DM1.
  - Efficacy data were based on 40 adult DM1 patients, including 12-month data from the 1.8 mg/kg Q4W (approximate ASO dose) cohort, 6-month data from the 3.4 mg/kg Q4W cohort, and 3-month data from the 5.4 mg/kg Q8W cohort.
  - DYNE-101 demonstrated robust muscle delivery and dose-dependent, consistent splicing correction while also showing improvement in myotonia, muscle strength, and timed function tests and in the Myotonic Dystrophy Type 1 Activity and Participation Scale (DM1-ACTIVc) and the Myotonic Dystrophy Health Index (MDHI) patient reported outcomes.
  - Safety and tolerability data were based on 56 patients enrolled through the 6.8 mg/kg Q8W cohort of the MAD portion of the ACHIEVE trial. DYNE-101 demonstrated a favorable safety profile as of the data cutoff date.[1]
- In addition to the clinical update in May 2024, the Company also noted that based on recent dialogue with the Center for Drug Evaluation and Research (CDER) division of the U.S. Food and Drug Administration (FDA), Dyne continues to pursue an accelerated approval pathway for DYNE-101 in DM1, including leveraging splicing as a potential surrogate biomarker.

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

- In May 2024, Dyne reported positive clinical data from the ongoing DELIVER trial of DYNE-251 in patients with DMD amenable to exon 51 skipping.
  - Efficacy data were based on 8 male patients with DMD enrolled in the 10 mg/kg (approximate PMO dose) cohort of the DELIVER trial. 10 mg/kg of DYNE-251 Q4W demonstrated dose-dependent exon skipping and dystrophin expression. DYNE-251 reached levels of dystrophin expression that exceeded levels reported in a clinical trial for the current weekly standard of care for DMD exon 51, eteplirsen, at 6 months with a 12-fold lower PMO dose.[2] DYNE-251 also demonstrated encouraging trends in multiple functional endpoints, including the North Star Ambulatory Assessment (NSAA), Stride Velocity 95th Centile (SV95C), 10-Meter Walk/Run Time, and Time to Rise from Floor.
  - Safety and tolerability data in the DELIVER trial were based on 48 patients enrolled through the 40 mg/kg Q8W cohort of the MAD portion. DYNE-251 demonstrated a favorable safety profile as of the cutoff date.[3]
- In addition to the clinical update in May 2024, Dyne confirmed that in DMD the FDA precedent for using dystrophin as a surrogate biomarker for accelerated approval remains available.

Path to Registration for ACHIEVE & DELIVER Trials

- Dyne plans to continue to engage with global regulators this year on ACHIEVE and DELIVER and anticipates providing an

update on the path to registration for both DYNE-101 and DYNE-251 by the end of 2024. Both trials are designed to be registrational, and the company is pursuing expedited approval pathways for both programs.

FORCE™ Platform and Pipeline

- In June 2024, Dyne highlighted new data for DYNE-302, its product candidate for facioscapulohumeral muscular dystrophy (FSHD), that demonstrated robust and durable *DUX4* suppression and functional benefit in preclinical models during the 31st Annual FSHD Society International Research Congress.
- In June 2024, Dyne presented new preclinical data in a Pompe disease model demonstrating the potential of the FORCE platform to deliver enzyme replacement therapy to cardiac and skeletal muscle and the central nervous system (CNS) at the New Directions in Biology and Disease of Skeletal Muscle Conference.
- In April 2024, Dyne submitted four abstracts featuring data from the ACHIEVE trial in DM1, DELIVER trial in DMD, and its FSHD and Pompe programs to the 2024 World Muscle Society Annual Congress. These have been accepted for presentation at the meeting, which will take place October 8-12, 2024, in Prague, Czech Republic.

Other Business Highlights

- In May 2024, Dyne completed an underwritten public offering of 12,075,000 shares of its common stock at a public offering price of $31.00 per share. The gross proceeds from the offering before deducting underwriting discounts and commissions were approximately $374.3 million.

Second Quarter 2024 Financial Results

Cash position: Cash, cash equivalents and marketable securities were $778.8 million as of June 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 $62.3 million for the quarter ended June 30, 2024, compared to $59.1 million for the quarter ended June 30, 2023.

General and administrative (G&A) expenses: G&A expenses were $9.7 million for the quarter ended June 30, 2024, compared to $7.6 million for the quarter ended June 30, 2023.

Net loss: Net loss for the quarter ended June 30, 2024, was $65.1 million, or $0.70 per basic and diluted share. This compares with a net loss of $64.9 million, or $1.08 per basic and diluted share, for the quarter ended June 30, 2023.

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 additional data from the ACHIEVE and DELIVER clinical trials and initiating registrational cohorts, expectations regarding the timing and outcome of interactions with global regulatory authorities and the availability of accelerated approval pathways for DYNE-101 and DYNE-251, expectations regarding the initiation of additional preclinical studies or clinical trials of DYNE-302 or, the anticipated timelines for reporting additional data for DYNE-302 or, expectations as to the relationship between data from the company's ongoing ACHIEVE clinical trial in DM1 and DELIVER clinical trial in DMD and existing or additional data for DYNE-302 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 June 30,	Six Months Ended June 30,

	2024	2023	2024	2023
Operating expenses:				
Research and development	$ 62,263	$ 59,130	$ 106,802	$ 96,667
General and administrative	9,699	7,606	34,317	15,533
Total operating expenses	**71,962**	**66,736**	**141,119**	**112,200**
Loss from operations	(71,962)	(66,736)	(141,119)	(112,200)
Other income (expense), net	6,860	1,834	10,368	3,111
Net loss	**$ (65,102)**	**$ (64,902)**	**$ (130,751)**	**$ (109,089)**
Net loss per share—basic and diluted	$ (0.70)	$ (1.08)	$ (1.51)	$ (1.88)
Weighted average common shares outstanding used in net loss per share—basic and diluted	92,507,815	59,835,087	86,777,150	58,090,142

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

	June 30, 2024	December 31, 2023
Assets		
Cash, cash equivalents and marketable securities	$ 778,838	$ 123,100
Other assets	47,046	41,982
Total assets	**$ 825,884**	**$ 165,082**
Liabilities and Stockholders' Equity		
Liabilities	50,691	73,790
Stockholders' equity	775,193	91,292
Total liabilities and stockholders' equity	**$ 825,884**	**$ 165,082**

1. DYNE-101 safety data as of May 8, 2024
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
3. DYNE-251 safety data as of April 30, 2024

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