Dyne Therapeutics Reports First Quarter 2023 Financial Results and Business Highlights

May 11, 2023

- On Track to Report Initial Data from ACHIEVE Clinical Trial of DYNE-101 in DM1 and DELIVER Clinical Trial of DYNE-251 in DMD During the Second Half of 2023 -

WALTHAM, Mass., May 11, 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 first quarter of 2023 and business highlights.

"We are making excellent progress in both our ACHIEVE and DELIVER trials and are on track to report initial data from both in the second half of 2023, including evaluating the key disease-driving biomarkers of splicing in DM1 and dystrophin in DMD," said Joshua Brumm, president and chief executive officer of Dyne. "Additionally, we are pleased to be presenting on our FORCE™ platform and clinical programs at premiere scientific meetings, engaging with the communities treating and living with DM1 and DMD. In particular, we are excited about the preclinical data that are being shared at ASGCT this month which, for the first time, demonstrate that FORCE leveraged TfR1 to deliver to the CNS, reinforcing the potential broad applicability of the platform to address multiple clinical manifestations of neuromuscular diseases."

Business Highlights

- Enrollment continues in ACHIEVE, a Phase 1/2 global clinical trial evaluating DYNE-101 in adult patients with myotonic dystrophy type 1 (DM1). 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 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.
- Enrollment continues in DELIVER, a Phase 1/2 global clinical trial evaluating DYNE-251 in males with Duchenne muscular dystrophy (DMD) who 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 open-label extension 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.
- DYNE-251 was granted orphan drug and rare pediatric disease designations by the U.S. Food and Drug Administration (FDA) in March 2023.
- Additional preclinical data supporting Dyne's DMD program as well as overviews of the DELIVER and ACHIEVE trial designs were featured in poster presentations during the Muscular Dystrophy Association (MDA) Clinical & Scientific Conference, held March 19-22, 2023:
  - "FORCE™ Platform Achieves Robust Exon Skipping, Restores Dystrophin at the Sarcolemma and Halts Progression of Fibrosis in the D2-*mdx* Model of DMD"
  - "ACHIEVE Trial, a Randomized, Placebo-Controlled, Multiple Ascending Dose Study of DYNE-101 in Individuals with Myotonic Dystrophy Type 1 (DM1)"
  - "DELIVER, a Randomized, Double-blind, Placebo-Controlled, Multiple Ascending Dose Study of DYNE-251 in Boys with DMD Amenable to Exon 51 Skipping"
- An overview of the ACHIEVE trial design was also featured in an oral presentation at the 75th American Academy of Neurology Annual Meeting, held April 22-27, 2023.

Upcoming Events & Key 2023 Milestones

- Dyne anticipates reporting initial data in the second half of 2023 from:
  - the MAD placebo-controlled portion of the ACHIEVE trial of DYNE-101 in DM1 on safety, tolerability and splicing; and
  - the MAD placebo-controlled portion of the DELIVER trial of DYNE-251 in DMD on safety, tolerability and dystrophin.
- Preclinical data from the FORCE platform have been selected for an oral presentation during the American Society of Gene & Cell Therapy (ASGCT) 26th Annual Meeting being held May 16-20, 2023 in Los Angeles. The presentation, "FORCE™ Platform Delivers Oligonucleotides to the Brain in a DM1 Mouse Model and in NHPs" (abstract #82), is scheduled for May 17 at 4:00 p.m. PT.

First Quarter 2023 Financial Results

Cash position: Cash, cash equivalents and marketable securities were $238.2 million as of March 31, 2023. In addition, subsequent to March 31, 2023, the Company raised $24.2 million from the sale of stock through its "at the market" offering program. The Company's cash, cash equivalents and marketable securities are anticipated to fund operations through 2024.

Research and development (R&D) expenses: R&D expenses were $37.5 million for the quarter ended March 31, 2023, compared to $28.2 million

for the quarter ended March 31, 2022.

General and administrative (G&A) expenses: G&A expenses were $7.9 million for the quarter ended March 31, 2023, compared to $7.5 million for the quarter ended March 31, 2022.

Net loss: Net loss for the quarter ended March 31, 2023 was $44.2 million, or $0.78 per basic and diluted share. This compares with a net loss of $35.6 million, or $0.69 per basic and diluted share, for the quarter ended March 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 Twitter, 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 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 March 31,	
	2023	**2022**
Operating expenses:		
Research and development	$ 37,536	$ 28,236
General and administrative	7,928	7,547
Total operating expenses	**45,464**	**35,783**
Loss from operations	(45,464)	(35,783)
Other (expense) income, net	1,277	200
Net loss	**$ (44,187)**	**$ (35,583)**
Net loss per share—basic and diluted	$ (0.78)	$ (0.69)
Weighted-average common shares outstanding used in net loss per share—basic and diluted	56,325,864	51,601,444

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

	March 31, 2023	December 31, 2022
Assets		
Cash, cash equivalents and marketable securities	$ 238,211	$ 256,012
Other assets	50,431	50,313

Total assets	**$ 288,642**	**$ 306,325**
Liabilities and Stockholders' Equity		
Liabilities	46,707	53,961
Stockholders' equity	241,935	252,364
Total liabilities and stockholders' equity	**$ 288,642**	**$ 306,325**

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