Day One Reports Fourth Quarter and Full Year 2022 Financial Results and Corporate Progress

Mar 6, 2023

First patient dosed in pivotal Phase 3 FIREFLY-2/LOGGIC trial evaluating tovorafenib (DAY101) as a frontline therapy for patients newly diagnosed with pediatric low-grade glioma (pLGG)

Reported topline data in January 2023 from ongoing, pivotal Phase 2 FIREFLY-1 trial demonstrating meaningful responses with tovorafenib (DAY101) in relapsed or progressive pLGG

Additional data from FIREFLY-1 planned for presentation at a medical meeting in second quarter of 2023

New Drug Application (NDA) submission planned for tovorafenib (DAY101) in first half of 2023

BRISBANE, Calif., March 06, 2023 (GLOBE NEWSWIRE) -- Day One Biopharmaceuticals (Nasdaq: DAWN), a clinical-stage biopharmaceutical company dedicated to developing and commercializing targeted therapies for people of all ages with life-threatening diseases, today announced its fourth quarter and full year 2022 financial results and highlighted recent corporate achievements.

“Day One made tremendous progress in 2022 on our mission of bringing novel targeted therapies to children with brain cancer and people of all ages with life-threatening diseases,” said Jeremy Bender, Ph.D., chief executive officer of Day One. “With positive topline results from the FIREFLY-1 study and commercial launch planning well underway, we believe we are on track to submit our first New Drug Application in the first half of this year. Given the significant unmet need for new therapies in children with relapsed or progressive pediatric low-grade gliomas, our team is laser focused on executing on our mission.”

Program Highlights

- In March 2023, Day One dosed the first patient the pivotal Phase 3 FIREFLY-2/LOGGIC clinical trial evaluating tovorafenib as a frontline therapy for patients newly diagnosed with pLGG.
    - The study is a randomized, monotherapy, open-label trial aiming to enroll approximately 400 patients aged 6 months to 25 years across approximately 100 sites globally, including in the United States, Europe and Asia.
    - The primary endpoint will be the overall response rate (ORR) based upon Response Assessment for Neuro-Oncology (RANO) criteria as reported by Blinded Independent Central Review.
    - Secondary endpoints will include safety, progression-free survival, overall survival, duration of response, functional outcomes and quality of life measures.

- In January 2023, Day One announced positive topline results from the ongoing, open-label, pivotal Phase 2 FIREFLY-1 trial evaluating tovorafenib (DAY101) as a monotherapy in relapsed or progressive pLGG. The primary endpoint of the FIREFLY-1 trial is ORR by RANO criteria as assessed by Blinded Independent Central Review. Topline results as of September 28, 2022 include:
    - Among 69 RANO-evaluable patients:
        - 64% ORR and 91% clinical benefit rate (complete response + partial response/unconfirmed partial response + stable disease)
            - 4% (n=3) confirmed complete responses
            - 59% (n=41) partial responses (31 confirmed and 10 unconfirmed)
            - 28% (n=19) patients with stable disease
        - 86% (n=59) of patients had a BRAF fusion alteration, for which there are no approved systemic therapies, while the remaining 14% (n=10) had a BRAF mutation
    - Safety data, based on 77 treated patients, indicated monotherapy tovorafenib (DAY101) to be generally well-tolerated. The most common side effects reported related to tovorafenib (DAY101) were change in hair color (75%), increased creatine phosphokinase (64%), anemia (46%), fatigue (42%), and maculopapular rash (42%).
    - Among a total of 77 treated patients:
        - Participants were heavily pretreated, with a median of three prior lines of systemic therapy (range: 1-9)
        - The median duration of tovorafenib (DAY101) treatment was 8.4 months, with 77% (n=59) of patients on treatment at the time of the data cutoff
        - Nearly 60% (n=46) of patients had already received at least one prior MAPK inhibitor prior to study participation

- Patient enrollment continues in the Phase 1b/2 FIRELIGHT-1 trial evaluating tovorafenib (DAY101) as a monotherapy and

as a combination with the company's investigational MEK inhibitor, pimasertib, in adults and adolescents with relapsed, progressive, or refractory solid tumors harboring MAPK pathway aberrations.

Upcoming Milestones

- Additional follow-up data from the full FIREFLY-1 study population is planned for presentation at a medical meeting in the second quarter of 2023.

- Anticipated submission of an NDA for tovorafenib (DAY101) to the United States Food and Drug Administration (FDA) in the first half of 2023.

Fourth Quarter and Full Year 2022 Financial Highlights

- **Cash Position:** Cash, cash equivalents and short-term investments totaled $342.3 million on December 31, 2022. Based on Day One's current operating plan, management believes it has sufficient capital resources to fund anticipated operations into 2025.

- **R&D Expenses:** Research and development expenses were $26.0 million and $85.6 million for the fourth quarter and full year ended December 31, 2022, respectively, as compared to $11.2 million and $43.6 million for the same periods in 2021. The increase was primarily due to additional employee compensation costs, clinical trial and pre-commercial manufacturing activities related to Day One's lead product candidate, tovorafenib (DAY101).

- **G&A Expenses:** General and administrative expenses were $16.7 million and $61.3 million for the fourth quarter and full year ended December 31, 2022, respectively, as compared to $10.8 million and $29.2 million for the same periods in 2021. The increase was primarily due to additional employee compensation costs, an ongoing commercial buildout, and professional service expenses to support company growth.

- **Net Loss:** Net loss totaled $40.1 million for the fourth quarter of 2022 with non-cash stock compensation expense of $6.8 million, compared to $21.9 million for the fourth quarter of 2021 with non-cash stock compensation expense of $5.1 million. Net loss was $142.2 million for the year ended December 31, 2022, with non-cash stock compensation expense of $27.2 million, compared to $72.8 million for the year ended December 31, 2021, with non-cash stock compensation expense of $13.3 million.

Upcoming Events

- **43rd Annual TD Cowen Health Care Conference**
  - Management will participate in a fireside chat on March 7 at 9:10 a.m. ET. A live and archived audio webcast of the discussion will be available by visiting the Events & Presentations section of the Company's website.

About Tovorafenib
Tovorafenib is an investigational, oral, brain-penetrant, highly-selective type II pan-RAF kinase inhibitor designed to target a key enzyme in the MAPK signaling pathway, which is being investigated in primary brain tumors or brain metastases of solid tumors. Tovorafenib has been studied in over 325 patients to date. Currently tovorafenib is under evaluation in a pivotal Phase 2 clinical trial (FIREFLY-1) among pediatric, adolescent and young adult patients with relapsed or progressive pLGG, which is an area of considerable unmet need with no approved therapies. Tovorafenib is also being evaluated alone or as a combination therapy for adolescent and adult patient populations with recurrent or progressive solid tumors with MAPK pathway aberrations (FIRELIGHT-1).

Tovorafenib (DAY101)has been granted Breakthrough Therapy and Rare Pediatric Disease designations by the U.S. Food and Drug Administration (FDA) for the treatment of patients with pLGG harboring an activating RAF alteration. Tovorafenib (DAY101) has also received Orphan Drug designation from the FDA for the treatment of malignant glioma, and from the European Commission (EC) for the treatment of glioma.

About Day One Biopharmaceuticals
Day One Biopharmaceuticals is a clinical-stage biopharmaceutical company that believes when it comes to pediatric cancer, we can do better. We put kids first and are developing targeted therapies that deliver to their needs. Day One was founded to address a critical unmet need: the dire lack of therapeutic development in pediatric cancer. The Company's name was inspired by "The Day One Talk" that physicians have with patients and their families about an initial cancer diagnosis and treatment plan. Day One aims to re-envision cancer drug development and redefine what's possible for all people living with cancer—regardless of age—starting from Day One.

Day One partners with leading clinical oncologists, families, and scientists to identify, acquire, and develop important emerging cancer treatments. The Company's lead product candidate, tovorafenib (DAY101), is an investigational, oral, brain-penetrant, highly-selective type II pan-RAF kinase inhibitor. The Company's pipeline also includes pimasertib, an investigational, oral, highly-selective small molecule inhibitor of mitogen-activated protein kinases 1 and 2 (MEK-1/-2). Day One is based in Brisbane. For more information, please visit www.dayonebio.com or find the company on LinkedIn or Twitter.

Cautionary Note Regarding Forward-Looking Statements

This press release contains “forward-looking” statements within the meaning of the “safe harbor” provisions of the Private Securities Litigation Reform Act of 1995, including, but not limited to: Day One’s plans to develop cancer therapies, expectations from current clinical trials, the execution of the Phase 2 and Phase 3 clinical trial for DAY101 as designed, any expectations about safety, efficacy, timing and ability to complete clinical trials, release data results and to obtain regulatory approvals for DAY101 and other candidates in development, and the ability of DAY101 to treat pLGG or related indications.

Statements including words such as “believe,” “plan,” “continue,” “expect,” “will,” “develop,” “signal,” “potential,” or “ongoing” and statements in the future tense are forward-looking statements. These forward-looking statements involve risks and uncertainties, as well as assumptions, which, if they do not fully materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such forward-looking statements.

Forward-looking statements are subject to risks and uncertainties that may cause Day One’s actual activities or results to differ significantly from those expressed in any forward-looking statement, including risks and uncertainties in this press release and other risks set forth in our filings with the Securities and Exchange Commission, including Day One’s ability to develop, obtain regulatory approval for or commercialize any product candidate, Day One’s ability to protect intellectual property, the potential impact of global business or macroeconomic conditions, including as a result of the COVID-19 pandemic, inflation and rising interest rates and the sufficiency of Day One’s cash, cash equivalents and investments to fund its operations. These forward-looking statements speak only as of the date hereof and Day One specifically disclaims any obligation to update these forward-looking statements or reasons why actual results might differ, whether as a result of new information, future events or otherwise, except as required by law.

Day One Biopharmaceuticals, Inc.
Consolidated Statements of Operations
(unaudited)
(In thousands)

	Year Ended December 31,		
	2022	**2021**	**2020**
Operating expenses:			
Research and development	$ 85,618	$ 43,584	$ 9,100
General and administrative	61,291	29,159	4,682
Total operating expenses	146,909	72,743	13,782
Loss from operations	(146,909)	(72,743)	(13,782)
Interest income (expense), net	4,746	4	(30)
Other expense, net	(18)	(15)	(31)
Changes in fair value of derivative tranche liability	—	—	(30,000)
Net loss	(142,181)	(72,754)	(43,843)
Net loss attributable to redeemable convertible noncontrolling interest	—	(2,109)	(3,336)
Exchange of redeemable noncontrolling interest shares – deemed dividend	—	(99,994)	—
Net loss attributable to common stockholders/members	$ (142,181)	$ (170,639)	$ (40,507)
Net loss per share, basic and diluted	$ (2.17)	$ (4.62)	$ (7.33)
Weighted-average number of common shares used in computing net loss per share, basic and diluted	65,466,773	36,960,569	5,529,519

Day One Biopharmaceuticals, Inc.
Selected Consolidated Balance Sheet Data
(unaudited)
(In thousands)

	December 31, 2022	**December 31, 2021**
Cash, cash equivalents and short-term investments	$ 342,269	$ 284,309
Total assets	349,062	289,821
Total liabilities	17,023	8,673
Accumulated deficit	(269,668)	(127,487)
Total stockholders’ equity	332,039	281,148

DAY ONE MEDIA

Laura Cooper, Head of Communications
media@dayonebio.com

DAY ONE INVESTORS
LifeSci Advisors, PJ Kelleher
pkelleher@lifesciadvisors.com