Day One Reports Second Quarter 2023 Financial Results and Corporate Progress

Aug 7, 2023

Results from FIREFLY-1 demonstrate overall response rate (ORR) of 67% and clinical benefit rate (CBR) of 93% in 69 heavily pretreated Response Assessment Neuro-Oncology High-Grade Glioma (RANO-HGG) evaluable patients presented at the 2023 American Society of Clinical Oncology (ASCO) Annual Meeting

Initiated rolling submission of the tovorafenib New Drug Application (NDA) in relapsed or progressive pediatric low-grade glioma (pLGG) in May 2023

The Company expects to complete the rolling submission of the tovorafenib NDA by October 2023

Completed $172.5 million public offering, strengthening balance sheet and extending cash runway into 2026

BRISBANE, Calif., Aug. 07, 2023 (GLOBE NEWSWIRE) -- Day One Biopharmaceuticals (Nasdaq: DAWN) (“Day One” or the “Company”), a clinical-stage biopharmaceutical company dedicated to developing and commercializing targeted therapies for people of all ages with life-threatening diseases, today announced its second quarter 2023 financial results and highlighted recent corporate achievements.

“Day One had a remarkable second quarter, with the initiation of the rolling submission of the tovorafenib NDA, followed by an oral presentation at ASCO with updated data that we anticipate will support our regulatory application to the FDA,” said Jeremy Bender, Ph.D., chief executive officer of Day One. “The majority of children with relapsed or progressive pLGG need new treatment options. With a strong balance sheet, we believe we are well positioned to achieve our key milestones while working towards expanding our pipeline with other innovative therapies.”

Program Highlights

- On June 4, 2023, Day One announced new clinical data from the registrational Phase 2 FIREFLY-1 trial evaluating the investigational agent tovorafenib in relapsed or progressive pLGG in an oral presentation at the 2023 ASCO Annual Meeting. These new data, with a data cutoff of December 22, 2022, included:

RANO-HGG (n=69) data:

- 67% (n=46) ORR by RANO-HGG, the primary endpoint of the trial
- 93% CBR (complete response (CR) + partial response (PR)/unconfirmed partial response (uPR) + stable disease (SD))
  - 6% (n=4) CR
  - 61% (n=42) PR, including 3 uPR
  - 26% (n=18) SD
- At the time of data cutoff, the median duration of response (DOR) based on RANO-HGG criteria was not yet reached (95% CI: 9.0 months, not estimable)

Among a total of 77 treated patients:

- The median duration of tovorafenib treatment was 10.8 months, with 74% (n=57) of patients on treatment at the time of data cutoff

Safety data, based on the 136 patients treated in both Arm 1 and Arm 2 of FIREFLY-1, indicated monotherapy tovorafenib to be generally well-tolerated. The vast majority of adverse events were Grade 1 or Grade 2, with most common side effects reported related to tovorafenib being change in hair color (71%), fatigue (50%), vomiting (43%), maculopapular rash (41%) and headache (39%). The most commonly reported lab abnormalities were CPK elevation, anemia, hypophosphatemia and AST elevation. Nearly all of the lab abnormalities had no clinical manifestations and did not require clinical intervention or change in study treatment.

The Company also shared the evaluation of responses by Response Assessment in Pediatric Neuro-Oncology Low-Grade Glioma (RAPNO-LGG) and Response Assessment Neuro-Oncology Low-Grade Glioma (RANO-LGG). Those results include:

*RAPNO-LGG data (n=69):

- 51% (n=35) ORR by RAPNO-LGG
  - 25% (n=17) PR including 4 uPR

    - 26% (n=18) minor response (MR) including 4 unconfirmed MR (uMR)
    - 36% (n=25) patients with SD
- The median time to response was 5.5 months for confirmed responses
- At the time of data cutoff, Independent Review Committee (IRC)-assessed median DOR based on confirmed RAPNO-LGG responses is 12 months (95% CI: 11.2, not estimable)

**Pending adjudication*

RANO-LGG (n=76) data:

- 49% (n=37) ORR by RANO-LGG
    - 26% (n= 20) PR including 8 uPR
    - 22% (n= 17) MR including 2 uMR
    - 34% (n=26) patients with SD
- The median time to response was 4.2 months for confirmed responses
- At the time of data cutoff, the IRC-assessed median DOR based on confirmed RANO-LGG responses is 14.4 months (95% CI: 8.4, not estimable)

- Two additional posters were presented on June 5, 2023 during the ASCO Pediatric Oncology session, including a trial-in-progress poster for the FIREFLY-2 trial and a poster describing a healthcare resource utilization study conducted for pLGG patients.

- Day One presented two posters at the 2023 American Society of Pediatric Oncology/Hematology Conference on May 10, 2023, focused on the pLGG burden of illness and healthcare utilization data.

- The pivotal Phase 3 FIREFLY-2/LOGGIC clinical trial evaluating tovorafenib as a front-line therapy in patients aged 6 months to 25 years with pLGG continues to enroll in the United States, Canada, Europe, Australia and Asia, with approximately 100 sites planned.

- Patient enrollment continues in the Phase 1b/2 FIRELIGHT-1 trials evaluating tovorafenib 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.

Corporate Highlights and Upcoming Milestones

- In June 2023, Day One announced the successful closing of a public offering including the full exercise of the underwriters' option to purchase additional shares, raising gross proceeds of $172.5 million which strengthens the Company's balance sheet and extends cash runway into 2026.

- The Company anticipates completing the rolling submission of the tovorafenib NDA by October 2023, following submission of an amended clinical study report (CSR) that will include safety and efficacy data from a planned June 2023 data cutoff.

Second Quarter 2023 Financial Highlights

- **Cash Position:** Cash, cash equivalents and short-term investments totaled $442.9 million on June 30, 2023. Based on Day One's current operating plan, management believes it has sufficient capital resources to fund anticipated operations into 2026.

- **R&D Expenses:** Research and development expenses were $32.2 million for the second quarter of 2023 compared to $22.6 million for the second quarter of 2022. The increase was primarily due to additional employee compensation costs, as well as clinical trial and manufacturing activities related to Day One's lead product candidate, tovorafenib.

- **G&A Expenses:** General and administrative expenses were $17.1 million for the second quarter of 2023 compared to $14.2 million for the second quarter of 2022. The increase was primarily due to additional employee compensation costs, as well as the ongoing build-out of commercial capabilities.

- **Net Loss:** Net loss totaled $45.9 million for the second quarter of 2023 with non-cash stock compensation expense of $9.5 million, compared to $36.5 million for the second quarter of 2022 with non-cash stock compensation expense of $5.6 million.

Upcoming Events

- 2023 Wedbush PacGrow Healthcare Conference, August 8-9, 2023

- Morgan Stanley 21st Annual Global Healthcare Conference, September 11-13, 2023

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 for the vast majority of patients. The pivotal Phase 3 FIREFLY-2/LOGGIC clinical trial is evaluating tovorafenib as a front-line therapy versus standard of care chemotherapy. Tovorafenib is also being evaluated alone or as a combination therapy for adolescent and adult patient populations with relapsed or progressive solid tumors with MAPK pathway aberrations (FIRELIGHT-1).

Tovorafenib 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 has also received Orphan Drug designation from the FDA for the treatment of malignant glioma, and from the European Commission 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, 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, California. 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 tovorafenib as designed, any expectations about safety, efficacy, timing and ability to complete clinical trials, release data results and to obtain regulatory approvals for tovorafenib and other candidates in development, and the ability of tovorafenib 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 inflation, rising interest rates, instability in the global banking system, geopolitical conflicts 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, except shares)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Operating expenses:				
Research and development	$ 32,182	$ 22,560	$ 60,010	$ 37,563
General and administrative	17,072	14,159	35,099	26,904
Total operating expenses	49,254	36,719	95,109	64,467
Loss from operations	(49,254)	(36,719)	(95,109)	(64,467)
Investment income, net	3,406	189	6,872	191
Other expense, net	(15)	—	(19)	(1)

Net loss attributable to common stockholders	(45,863)	(36,530)	(88,256)	(64,277)
Net loss per share, basic and diluted	$ (0.61)	$ (0.60)	$ (1.20)	$ (1.08)
Weighted-average number of common shares used in computing net loss per share, basic and diluted	74,964,878	60,760,527	73,478,567	59,586,529

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

	June 30, 2023	December 31, 2022
Cash, cash equivalents and short-term investments	$ 442,882	$ 342,269
Total assets	450,756	349,062
Total liabilities	24,702	17,023
Accumulated deficit	(357,924)	(269,668)
Total stockholders' equity	426,054	332,039

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