Epizyme Provides Business Update and Reports Fourth Quarter and Full Year 2019 Financial Results

February 24, 2020

TAZVERIK™ (tazemetostat) Commercial Launch Underway for Epithelioid Sarcoma

TAZVERIK sNDA for Follicular Lymphoma Accepted for Filing by FDA with Priority Review; PDUFA Target Action Date of June 18, 2020

Expanding Future TAZVERIK Value through Clinical Development into New Combinations and Indications

Conference Call to be Held Today, Feb. 24 at 8:30 a.m. ET

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 24, 2020-- Epizyme, Inc. (Nasdaq: EPZM), a fully integrated, commercial-stage biopharmaceutical company developing novel epigenetic therapies, today provided business and pipeline updates and reported fourth quarter and full year 2019 financial results.

"We began 2020 with the most meaningful milestone for the company to date – the accelerated approval of TAZVERIK, making it the first and only commercially available EZH2 inhibitor and the only approved product specifically indicated for epithelioid sarcoma patients. We executed a strong start to our commercial launch, in which we made TAZVERIK available to patients within one week of approval," said Robert Bazemore, president and chief executive officer of Epizyme. "Following the acceptance of our sNDA filing for TAZVERIK for follicular lymphoma, we are preparing to quickly address the more prevalent FL patient population, if approved. In parallel, we are continuing our in-house and clinical collaboration efforts to demonstrate the full potential of tazemetostat for a range of cancer indications, with 12 trials underway and four more planned for initiation this year, making for a robust development expansion program."

Recent Progress

- **TAZVERIK Commercial Launch Underway in Epithelioid Sarcoma (ES) and Added to NCCN Clinical Practice Guidelines:** TAZVERIK became commercially available to patients on February 1, 2020, following its accelerated approval on January 23, 2020, for the treatment of adults and pediatric patients aged 16 years and older with metastatic or locally advanced ES not eligible for complete resection. The approval was based on overall response rate and duration of response observed in a Phase 2 clinical trial. In addition, the National Comprehensive Cancer Network has updated its Clinical Practice Guidelines in Oncology (NCCN Guidelines®) for Soft Tissue Sarcoma to include TAZVERIK as a recommended category 2A treatment for metastatic or locally advanced ES. The NCCN Guidelines are the recognized clinical standard for cancer care by U.S. healthcare providers and payers, and are maintained by a committee of expert physicians from leading U.S. cancer centers.
- **TAZVERIK sNDA Submission for Follicular Lymphoma (FL) Accepted for Filing with Priority Review:** The U.S. Food and Drug Administration (FDA) has accepted the company's supplemental NDA (sNDA) for filing for the accelerated approval of TAZVERIK for a proposed indication of patients with relapsed or refractory FL who have received at least two prior lines of systemic therapy. The FDA granted Priority Review for the sNDA and has set a Prescription Drug User Fee Act (PDUFA) target action date of June 18, 2020. The submission is based primarily on Phase 2 data, which demonstrated clinical benefit as assessed by both investigators and an Independent Review Committee (IRC) and a generally well-tolerated profile in this patient population, as presented at the 2019 American Society of Hematology (ASH) Annual Meeting.
- **Exercise of $50 Million Put Option with Royalty Pharma Supports Runway into at least 2022:** Pursuant to the terms of its agreements with Royalty Pharma and Pharmakon Advisors, in January 2020, Epizyme exercised its option to sell $50 million of its common stock to Royalty Pharma. The cash, cash equivalents and marketable securities as of December 31, 2019, together with the $50 million raised from the exercise of the put option, allowed the company to start 2020 with approximately $431 million, which will fund the current operating runway into at least 2022.

2020 Company Priorities

Epizyme has outlined the following key milestones for 2020:

Epithelioid Sarcoma

- Optimize the commercial launch of TAZVERIK for ES to seamlessly expand to the FL opportunity, if approved;
- Complete the safety portion of the ongoing global, Phase 1b/3 confirmatory trial assessing TAZVERIK in combination with doxorubicin compared with doxorubicin plus placebo as a front-line treatment for ES, and advance into the efficacy portion of the trial.

Follicular Lymphoma

- Gain FDA approval for and launch TAZVERIK for patients in the U.S. with relapsed or refractory FL who have received at least two prior lines of systemic therapy;
- Complete the safety portion of the ongoing global Phase 1b/3 confirmatory trial assessing TAZVERIK in combination with "R ^{2}" (Revlimid® plus Rituximab®) compared with R^2 plus placebo in the second-line treatment setting and advance into the efficacy portion of the trial;
- Expand clinical investigation of tazemetostat in combination with R-CHOP in the high-risk front-line treatment setting for patients with FL; and
- Support the investigator-sponsored studies assessing tazemetostat in combination with rituximab, venetoclax and BTK inhibitors in the third-line and later FL treatment setting.

Additional Indications

Epizyme plans to explore the utility of tazemetostat in additional indications, as a monotherapy and in combinations, through in-house efforts, investigator-sponsored studies and clinical collaborations. Based on scientific hypotheses and unmet need, these areas include:

- Hematological malignancies, such as lymphomas and other b-cell malignancies, in which EZH2 plays an important role in B cell biology;
- Mutationally defined solid tumors, such as chordoma, melanoma and tumors with a SWI/SNF alteration or other mutations;
- Metastatic castration-resistant prostate cancer;
- Solid tumors that are resistant to chemotherapy or PARP inhibitors, such as triple negative breast, small cell lung and ovarian cancers, as well as mesothelioma; and
- Solid tumors in which EHZ2 inhibition can augment the response to immune-oncology treatments.

Preclinical Pipeline

- Pursue additional development candidates for its preclinical programs.

Financial Guidance

Based on its current operating plans, Epizyme expects its current cash runway to extend into at least 2022. Additionally, the company expects its GAAP operating expenses for 2020 to be between $300 and $330 million inclusive of the milestones due to Eisai for the FDA approval of TAVERIK for epithelioid sarcoma and for follicular lymphoma, if approved.

Fourth Quarter and Full Year 2019 Financial Results

- **Cash Position**: Cash, cash equivalents and marketable securities were $381.1 million as of December 31, 2019, as compared to $240.3 million as of December 31, 2018.
- **Revenue**: Collaboration revenue for the fourth quarter of 2019 was $4.3 million and $23.8 million for the full year ended December 31, 2019, compared to collaboration revenue of $9.7 million for the fourth quarter of 2018 and $21.7 million for the full year ended December 31, 2018. The increase reflects the recognition of revenue for services performed under the company's multi-target research collaboration with Boehringer Ingelheim offset by development milestones with GlaxoSmithKline achieved in 2018 for its PRMT1 and PRMT 5 inhibitors, which were invented at Epizyme.
- **R&D Expenses**: Research and development (R&D) expenses were $38.3 million for the fourth quarter of 2019 and $132.6 million for the full year ended December 31, 2019, compared to $21.8 million for the fourth quarter of 2018 and $105.8 million for the full year ended December 31, 2018. The increase is due to the initiation of the global confirmatory trials in ES and FL and early expansion of the tazemetostat program into new indications and combinations. The primary driver of the increase in R&D expenses for 2019 was the expense of $20 million in milestones paid to Eisai for the ES and FL NDA submissions. These milestones were funded through the loan facility provided by Pharmakon.
- **G&A Expenses**: General and administrative (G&A) expenses were $23.5 million for the fourth quarter of 2019 and $68.3 million for the full year ended December 31, 2019, compared to $12.2 million for the fourth quarter of 2018 and $44.0 million for the full year ended December 31, 2018. G&A expenses for 2019 included the buildout of the infrastructure to support the launch and commercialization of TAZVERIK for the ES indication, as well as initial expansion to prepare for FL commercialization in the US.
- **Net Loss Attributable to Common Stockholders:** Net loss attributable to common stockholders was $56.4 million, or $0.59 per share, for the fourth quarter of 2019 and $173.2 million, or $1.93 per share, for the full year ended December 31, 2019, compared to $22.9 million, or $0.29 per share, for the fourth quarter of 2018 and $123.6 million, or $1.72 per share, for the full year ended December 31, 2018.

Conference Call Information

Epizyme will host a conference call today, Feb. 24, at 8:30 a.m. ET. To participate in the conference call, please dial (877) 844-6886 (domestic) or (970) 315-0315 (international) and refer to conference ID 9045277. A live webcast and slides will be available in the investor section of the company's website at www.epizyme.com, and will be archived for 60 days following the call and presentation.

About TAZVERIK

TAZVERIK™ (tazemetostat) is the first EZH2 inhibitor approved by the U.S. Food and Drug Administration (FDA). TAZVERIK™ is an enhancer of zeste homolog 2 (EZH2) methyltransferase inhibitor indicated for the treatment of adults and pediatric patients aged 16 years and older with metastatic or locally advanced epithelioid sarcoma who are not eligible for complete resection. This indication is approved under accelerated approval based on overall response rate and duration of response. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s). For more information, visit TAZVERIK.com.

About Epizyme, Inc.

Epizyme, Inc. is a fully integrated, commercial-stage biopharmaceutical company committed to its mission of rewriting treatment for cancer and other serious diseases through novel epigenetic medicines. In addition to an active research and discovery pipeline, Epizyme has one U.S. FDA approved product, TAZVERIK™ (tazemetostat), for the treatment of adults and pediatric patients aged 16 years and older with metastatic or locally advanced epithelioid sarcoma (ES) who are not eligible for complete resection. This indication is approved under accelerated approval based on overall response rate and duration of response. Continued approval for this indication is contingent upon verification and description of clinical benefit in an ongoing confirmatory trial. The company is also exploring the treatment potential of tazemetostat in investigational clinical trials in other solid tumors and hematological malignancies, as a monotherapy and combination therapy in both relapsed and front-line disease settings. By focusing on the genetic drivers of disease, Epizyme seeks to match medicines with the patients who need them. For more information, visit www.epizyme.com.

Cautionary Note on Forward-Looking Statements

Any statements in this press release about future expectations, plans and prospects for Epizyme, Inc. and other statements containing the words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: whether tazemetostat will receive marketing approval for epithelioid sarcoma in other jurisdictions, full approval in the United States or approval in any other indication; whether results from preclinical studies or earlier clinical studies will be predictive of the results of future trials, such as the ongoing confirmatory trial; whether results from clinical studies will warrant meetings with regulatory authorities, submissions for regulatory approval or review by governmental authorities under the accelerated approval process; expectations for regulatory approvals, including accelerated approval, to conduct trials or to market products; whether the company's cash resources will be sufficient to fund the company's foreseeable and unforeseeable operating expenses and capital expenditure requirements; other matters that could affect the availability or commercial success of tazemetostat or the company's therapeutic candidates; and other factors discussed in the "Risk Factors" section of the company's most recent Form 10-Q filed with the SEC and in the company's other filings from time to time with the SEC. In addition, the forward-looking statements included in this press release represent the company's views as of the date hereof and should not be relied upon as representing the company's views as of any date subsequent to the date hereof. The company anticipates that subsequent events and developments will cause the company's views to change. However, while the company may elect to update these forward-looking statements at some point in the future, the company specifically disclaims any obligation to do so.

TAZVERIK™ is a trademark of Epizyme, Inc.

EPIZYME, INC.
CONSOLIDATED BALANCE SHEET DATA (UNAUDITED)
(Amounts in thousands)

	December 31, 2019	December 31, 2018
Consolidated Balance Sheet Data:		
Cash and cash equivalents	$ 139,482	$ 86,671
Marketable securities	241,605	153,633
Total assets	424,589	275,501
Total current liabilities	34,386	37,833
Deferred revenue	3,806	17,106
Long-term debt, net of debt discount	23,309	—
Liability related to sale of future royalties	12,793	—
Total stockholders' equity	$ 331,137	$ 233,009

EPIZYME, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS (UNAUDITED)
(Amounts in thousands except per share data)

	Three Months Ended December 31,		Year Ended December 31,	
	2019	2018	2019	2018
Collaboration revenue	$ 4,294	$ 9,700	$ 23,800	$ 21,700
Operating expenses:				
Research and development	38,257	21,838	132,639	105,833
General and administrative	23,530	12,170	68,303	43,972
Total operating expenses	61,787	34,008	200,942	149,805

Operating loss	(57,493)	(24,308)	(177,142)	(128,105)
Other income, net:				
Interest income, net	1,320	1,420	7,110	4,557
Other income (expense), net	21	—	(13)	(25)
Non-cash interest expense related to sale of future royalties	(192)	—	(192)	—
Other income, net	1,149	1,420	6,905	4,532
Loss before income taxes	(56,344)	(22,888)	(170,237)	(123,573)
Income tax (provision)	(58)	(57)	(58)	(57)
Net loss	$ (56,402)	$ (22,945)	$ (170,295)	$ (123,630)
Other comprehensive income (loss):				
Unrealized (loss) gain on available-for-sale securities	(130)	(31)	73	(5)
Comprehensive loss	$ (56,532)	$ (22,976)	$ (170,222)	$ (123,635)

Reconciliation of net loss to net loss attributable to common stockholders:				
Net loss	$ (56,402)	$ (22,945)	$ (170,295)	$ (123,630)
Accretion of convertible preferred stock	—	—	(2,940)	—
Net loss attributable to common stockholders	$ (56,402)	$ (22,945)	$ (173,235)	$ (123,630)
Net loss per share attributable to common stockholders - basic and diluted	$ (0.59)	$ (0.29)	$ (1.93)	$ (1.72)
Weighted-average common shares outstanding used in net loss per share attributable to common stockholders - basic and diluted	95,074	78,962	89,891	71,864

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