November 7, 2023

CymaBay Reports Third Quarter and Nine Months Ended September 30, 2023 Financial Results and Provides Corporate Update

Announced top-line results from RESPONSE Phase 3 study in PBC where seladelpar met the primary and two key secondary endpoints with high statistical significance.

Seladelpar granted revised Breakthrough Therapy Designation for the treatment of PBC including pruritus in patients without cirrhosis or with compensated cirrhosis and are inadequate responders to or intolerant to UDCA.

Announced oral late-breaking presentation of results from the RESPONSE global Phase 3 study evaluating seladelpar for Primary Biliary Cholangitis at The Liver Meeting® 2023.

Completed a public offering of common stock and pre-funded warrants with net proceeds of $242.8 million.

Initiated AFFIRM, a Phase 3b/4 study evaluating the effect of seladelpar on clinical outcomes in patients with cirrhosis due to PBC.

Conference call and webcast today at 4:30 p.m. ET

NEWARK, Calif., Nov. 07, 2023 (GLOBE NEWSWIRE) -- CymaBay Therapeutics, Inc. (NASDAQ: CBAY), a biopharmaceutical company focused on innovative therapies for patients with liver and other chronic diseases, today announced corporate updates and financial results for the third quarter ended September 30, 2023.

Sujal Shah, President and CEO of CymaBay, stated, “This past quarter has been a momentous one for CymaBay where we achieved multiple significant milestones advancing us towards our goal of improving lives of people living with PBC. The consistency and depth of the clinical data set generated from Phase 2, ENHANCE and now RESPONSE demonstrates that seladelpar has the potential to be the first ever approved treatment for patients with PBC to significantly reduce both markers related to the risk of disease progression and symptoms. I am incredibly proud of the team here at CymaBay and commend them for the progress they helped achieve and equally grateful to patients and patient advocacy groups, their caregivers and our investigators for their partnership and support. We are eager to continue the positive momentum and are working diligently on our near-term milestones for seladelpar.”

Corporate Updates:

- On September 7, 2023, we announced topline results from our seladelpar Phase 3 RESPONSE study. The study evaluated the safety and efficacy of seladelpar for the

treatment of PBC. The trial achieved the primary and all key secondary endpoints.

  - Primary composite endpoint at 12 months of serum alkaline phosphatase and bilirubin was met by 61.7% of patients treated with seladelpar 10 mg vs. 20.0% of placebo treated patients ($p<0.0001$)
  - Normalization of alkaline phosphatase at 12 months was achieved by 25.0% of patients treated with seladelpar vs. 0% on placebo ($p<0.0001$)
  - In patients having moderate-to-severe itch at baseline, the seladelpar treated group improved their pruritus at 6 months compared to those in the placebo group ($p<0.005$)
  - Overall safety and tolerability were comparable between placebo and seladelpar groups and consistent with previous studies
  - Treatment-emergent adverse events, serious adverse events, and patient discontinuations were generally balanced across the treatment and placebo arms. There were no treatment-related serious adverse events in the study.

- On October 23, 2023, we announced that the U.S. Food and Drug Administration (FDA) has revised the originally granted Breakthrough Therapy Designation for seladelpar to now reflect treatment of primary biliary cholangitis (PBC) including pruritus in adults without cirrhosis or with compensated cirrhosis (Child Pugh A) and are inadequate responders to or intolerant to UDCA. Seladelpar is the only potent, selective, orally active PPARδ agonist, or delpar, with Phase 3 results demonstrating a statistically significant improvement in PBC-related cholestatic pruritus.

- A late-breaking presentation highlighting results from the RESPONSE Phase 3 study of seladelpar in patients with PBC will be presented at The Liver Meeting® of the American Association for the Study of Liver Diseases (AASLD), in Boston, MA (November 10th – 14th).

- Announced the initiation of AFFIRM, a randomized, placebo-controlled confirmatory study to evaluate the effect of seladelpar on clinical outcomes in patients with compensated cirrhosis due to PBC. The AFFIRM study is planned to enroll approximately 192 patients with PBC who have compensated cirrhosis (Child-Pugh A or Child-Pugh B) based on prespecified clinical criteria. Patients will be randomly assigned using a 2:1 ratio to oral, once daily seladelpar or placebo for a fixed duration of three years. The primary outcome measure is the time from start of treatment to the first occurrence of clinical events (all-cause death, liver transplant, hospitalization for other serious liver-related events, and progression to Child-Pugh C decompensated cirrhosis). Additional key outcomes include overall survival, liver transplant-free survival, and time to hospitalization for serious liver-related events.

- Continued enrollment in ASSURE, an open-label, long-term study of seladelpar in patients with PBC intended to collect additional long-term safety and efficacy data to support registration. There are now over 300 patients in this study taking seladelpar daily, including those from our prior studies of seladelpar and patients who have completed RESPONSE.

- Findings from post-hoc analysis of the Phase 3 ENHANCE study of seladelpar for the treatment of PBC, showing baseline intensity of patient-reported pruritus was

associated with higher levels of serum IL-31 was presented at American College of Gastroenterology (ACG), by Professor Andreas E. Kremer, MD, Ph.D., MHBA, a leading authority in cholestatic pruritus from the University of Zurich. Featured results included novel aspects of the anti-pruritic and anti-cholestatic mechanisms of seladelpar, CymaBay's first-in-class oral, selective PPARδ agonist, or "delpar," being investigated for the treatment of patients with PBC.

- Completed an upsized public equity offering in September 2023, in which we sold 14,521,307 shares of common stock at $17.13 per share and pre-funded warrants to purchase 583,771 shares of common stock at $17.1299 per underlying share. Net proceeds of the offering were $242.8 million, after deducting the underwriting discount and other offering expenses.

- The Phase 2a proof-of-pharmacology study to assess whether MBX-2982 can enhance glucagon secretion during insulin-induced hypoglycemia in subjects with type 1 diabetes (T1D) has been completed. The study found that there was no change in glucagon secretion during clamps in subjects with T1D dosed with MBX-2982 versus placebo. In contrast, healthy volunteers showed a glucose dependent increase in glucagon during hypoglycemia. Further, target engagement by MBX-2982 was demonstrated by increases in GLP-1 levels in subjects with T1D. While disappointing, the results demonstrate a well-designed and executed study that demonstrated pharmacodynamic action of MBX-2982 but without demonstrating the pharmacology needed to benefit the T1D population. The scientific questions were answered and CymaBay is not planning any further studies with MBX-2982. We would like to thank Dr. Richard Pratley and his team at the AdventHealth Research Institute in Orlando, Florida as well as ProSciento Inc., California for conducting a well-designed and executed study and The Leona M. and Harry B. Helmsley Charitable Trust for their support of the study through a grant to AdventHealth. We are also grateful to the patients with T1D and healthy volunteers for their participation in the study.

Financial Updates:

- Held $438.8 million in cash, cash equivalents and investments as of September 30, 2023. We believe that cash and investments on hand are sufficient to fund CymaBay's operating expenses into the first half of 2026.

Third Quarter and Nine Months Ended September 30, 2023 Financial Results

- Collaboration revenue recognized for the nine months ended September 30, 2023 was $31.0 million and was associated with the collaboration and license agreement with Kaken Pharmaceutical Co., Ltd. (Kaken) entered into in January 2023, to develop and commercialize seladelpar in Japan. As reported earlier, this revenue was recognized upon completion of the initial technology transfer to Kaken in the second quarter of 2023. No incremental collaboration revenue was recognized for the three months ended September 30, 2023. Of the $34.2M upfront payment received from Kaken, $2.7 million remains deferred as of September 30, 2023 and will be recognized upon completion of the CymaBay's ongoing clinical data delivery and CMC development performance obligations.
- Research and development expenses for the three months ended September 30, 2023, and 2022 were $20.0 million and $15.5 million, respectively. Research and

development expenses for the nine months ended September 30, 2023 and 2022 were $58.1 million and $51.8 million, respectively. Research and development expenses for the three- and nine-month periods ended September 30, 2023 increased compared to the corresponding periods in 2022 driven by higher clinical activities supporting our clinical studies.

- General and administrative expenses for the three months ended September 30, 2023 and 2022 were $12.2 million and $5.9 million, respectively. General and administrative expenses for the nine months ended September 30, 2023 and 2022 were $32.1 million and $17.9 million, respectively. General and administrative expenses for the three and nine months ended September 30, 2023 were higher than the corresponding period in 2022 driven by investments to prepare for potential commercialization of seladelpar in PBC as well as an increase in other corporate expenses.
- Net loss for the three months ended September 30, 2023 and 2022 was $33.9 million and $24.5 million, or ($0.32) and ($0.28) per share, respectively. Net loss for the nine months ended September 30, 2023 and 2022 was $63.5 million and $79.4 million, or ($0.62) and ($0.90) per share, respectively. Net loss for the three months ended September 30, 2023 was higher than the three months ended September 30, 2022 primarily due to higher operating expenses. Net loss for the nine months ended September 30, 2023 was lower than the corresponding periods in 2022 due primarily to the recognition of $31.0 million of collaboration revenue related to the Kaken upfront payment during the second quarter of 2023 and higher interest income earned on our investments and other income due to refundable tax credits, offset in part by an increase in operating expenses. Overall, we expect operating expenses to increase in the future as we continue to support our ongoing drug development activities and expand on initiatives to prepare for potential commercialization of seladelpar in PBC.

Conference Call Details

CymaBay will host a conference call today at 4:30 p.m. ET to discuss third quarter financial results and provide a business update. To access the live conference call, please dial 1-877-407-0784 from the U.S. and Canada, or 1-201-689-8560 internationally, Conference ID #13740701. To access the live and subsequently archived webcast of the conference call, go to the Investors section of the company's website at http://ir.cymabay.com/events.

About CymaBay

CymaBay Therapeutics, Inc. is a clinical-stage biopharmaceutical company focused on improving the lives of people with liver and other chronic diseases that have high unmet medical need through a pipeline of innovative therapies. Our deep understanding of the underlying mechanisms of liver inflammation and fibrosis, and the unique targets that play a role in their progression, have helped us receive breakthrough therapy designation (U.S. Food and Drug Administration), Priority Medicines status (European Medicines Agency) and orphan drug status (U.S. and Europe) for seladelpar, a first-in-class investigational treatment for people with PBC. Our evidence-based decision-making and commitment to the highest quality standards reflect our relentless dedication to the people, families and communities we serve. To learn more, visit www.cymabay.com and follow us on X (formerly Twitter) and LinkedIn.

Cautionary Statements

Any statements made in this press release regarding the potential approval, launch and commercialization of seladelpar or timing or plans in regard thereto, as well as statements regarding the Company's expected cash runway, potential benefits of seladelpar, the ability to enroll and/or complete the AFFIRM study, completion of ongoing clinical trials and subsequent regulatory submissions are forward-looking statements that are subject to risks and uncertainties. Actual results and the timing of events regarding the further development of seladelpar could differ materially from those anticipated in such forward-looking statements as a result of risks and uncertainties, which include, without limitation, risks related to: the success, cost and timing of any of CymaBay's product development activities, including clinical trials; effects observed in trials to date that may not be repeated in the future; any delays or inability to obtain or maintain regulatory approval of CymaBay's product candidates in the United States or worldwide; and the ability of CymaBay to obtain sufficient financing to complete development, regulatory approval and commercialization of its product candidates in the United States and worldwide. Additional risks relating to CymaBay are contained in CymaBay's filings with the Securities and Exchange Commission, including without limitation its most recent Annual Report on Form 10-K and other documents subsequently filed with or furnished to the Securities and Exchange Commission. CymaBay disclaims any obligation to update these forward-looking statements except as required by law.

For additional information about CymaBay, visit www.cymabay.com.

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CymaBay Therapeutics, Inc.
Financial Results
(In thousands, except share and per share information)

	Quarter Ended September 30,		Nine Months Ended September 30,	
	2023	**2022**	**2023**	**2022**
	(unaudited)	**(unaudited)**	**(unaudited)**	**(unaudited)**
Collaboration revenue	-	-	31,016	$ -
Operating expenses:				
Research and development	19,962	15,459	58,050	51,765
General and administrative	12,245	5,904	32,147	17,869
Total operating expenses	32,207	21,363	90,197	69,634
Loss from operations	(32,207)	(21,363)	(59,181)	(69,634)
Other income (expense), net:				
Interest income	3,170	677	7,810	1,096
Interest expense	(4,843)	(3,819)	(13,864)	(10,832)
Other income	(2)	-	1,767	2
Total other income (expense), net	(1,675)	(3,142)	(4,287)	(9,734)
Net loss	$ (33,882)	$ (24,505)	$ (63,468)	$ (79,368)
Basic and diluted net loss per common share	$ (0.32)	$ (0.28)	$ (0.62)	$ (0.90)
Weighted average common shares outstanding used to calculate basic and diluted net loss per common share	105,717,996	87,804,272	101,974,866	87,803,388

CymaBay Therapeutics, Inc.
Balance Sheet Data
(in thousands)

	September 30, 2023	**December 31, 2022**
	(unaudited)	
Cash, cash equivalents and marketable securities	$ 438,778	$ 135,485
Working capital	427,993	122,632
Total assets	451,048	141,852
Total liabilities	124,857	105,698
Common stock and additional paid-in capital	1,262,578	909,337
Total stockholders' equity	326,191	36,154

Source: CymaBay Therapeutics, Inc.