# UNITED STATES
# SECURITIES AND EXCHANGE COMMISSION

**Washington, D.C. 20549**

# FORM 8-K

**CURRENT REPORT**
**Pursuant to Section 13 or 15(d)**
**of the Securities Exchange Act of 1934**

Date of Report (Date of Earliest Event Reported): **May 4, 2023**

# Blueprint Medicines Corporation

(Exact name of registrant as specified in its charter)

| **Delaware** | **001-37359** | **26-3632015** |
|---|---|---|
| (State or other jurisdiction of incorporation) | (Commission File Number) | (I.R.S. Employer Identification No.) |

| **45 Sidney Street**<br>**Cambridge, Massachusetts** | **02139** |
|---|---|
| (Address of principal executive offices) | (Zip Code) |

Registrant's telephone number, including area code: **(617) 374-7580**

(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Securities registered pursuant to Section 12(b) of the Exchange Act:

| Title of each class | Trading symbol(s) | Name of each exchange on which registered |
|---|---|---|
| Common stock, par value $0.001 per share | BPMC | Nasdaq Global Select Market |

**Item 2.02 Results of Operations and Financial Condition.**

On May 4, 2023, Blueprint Medicines Corporation announced its financial results for the quarter ended March 31, 2023 and other business highlights. The full text of the press release issued in connection with the announcement is furnished as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated by reference herein.

The information in this Current Report on Form 8-K, including Exhibit 99.1 attached hereto, is intended to be furnished and shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such filing.

(d) Exhibits.

The following exhibit relating to Item 2.02 of this Form 8-K shall be deemed to be furnished and not filed:

**Item 9.01 Financial Statements and Exhibits.**

| **Exhibit No.** | **Description** |
|---|---|
| 99.1 | Press release issued by Blueprint Medicines Corporation on May 4, 2023 |
| 104 | Cover Page Interactive Data File (embedded within the Inline XBRL document and incorporated as Exhibit 101) |

**SIGNATURES**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

**BLUEPRINT MEDICINES CORPORATION**

Date: May 4, 2023

By: /s/ Kathryn Haviland
Kathryn Haviland
Chief Executive Officer

**Exhibit 99.1**

**Blueprint Medicines Reports First Quarter 2023 Results**

*-- Achieved $39.1 million in AYVAKIT®/AYVAKYT® (avapritinib) net product revenues and $63.3 million in total revenues in the first quarter of 2023 --*

*-- Raising 2023 AYVAKIT net product revenue guidance to $135 million to $145 million for advanced SM and GIST, excluding additional revenue associated with anticipated U.S. FDA approval for indolent SM --*

*-- FDA target action date for AYVAKIT supplemental new drug application for indolent SM is May 22, 2023 --*

CAMBRIDGE, Mass., May 4, 2023 – Blueprint Medicines Corporation (NASDAQ: BPMC) today reported financial results and provided a business update for the first quarter ended March 31, 2023.

"Our first quarter was marked by executional progress across the multiple growth opportunities that Blueprint Medicines has to expand our impact and transform outcomes for thousands of patients worldwide. We drove strong AYVAKIT revenue growth, presented seminal data for AYVAKIT in indolent systemic mastocytosis, and worked to progress our broad pipeline by rapidly resolving the partial clinical hold for BLU-222 and generating early clinical data across our portfolio," said Kate Haviland, Chief Executive Officer at Blueprint Medicines. "Blueprint Medicines is ready to further solidify our leadership in systemic mastocytosis and scale the impact of AYVAKIT by addressing the medical needs of a substantially larger group of patients with our anticipated FDA label expansion for indolent systemic mastocytosis in a few weeks."

**First Quarter 2023 Highlights and Recent Progress**

**Systemic mastocytosis (SM)**

- Presented full registrational PIONEER trial data for AYVAKIT in patients with indolent systemic mastocytosis (ISM) at the 2023 American Academy of Asthma, Allergy, and Immunology (AAAAI) Annual Conference. These data included a statistically significant and clinically meaningful improvement in total symptom score that deepened over time, with improvements shown across all individual symptoms and in quality-of-life measures. AYVAKIT showed a favorable safety profile compared to placebo, supporting potential for chronic treatment. Read the press release here.
- U.S. Food and Drug Administration (FDA) target action date for AYVAKIT supplemental new drug application for ISM is May 22, 2023.

**EGFR-driven non-small cell lung cancer (NSCLC)**

- Announced plans to present results from the ongoing dose escalation of the Phase 1/2 CONCERTO trial of BLU-451 in EGFR exon 20 insertion-positive NSCLC, showing early safety and clinical activity, at the 2023 American Society of Clinical Oncology (ASCO) Annual Meeting.
- Announced plans to present updated results from the dose escalation of the Phase 1/2 SYMPHONY trial showing safety and tolerability of BLU-945 both as a monotherapy and in combination with osimertinib in late-line EGFR-driven NSCLC at the 2023 ASCO Annual Meeting.
- Presented real-world data showing that NSCLC patients with an EGFR L858R mutation have worse outcomes compared to patients with an EGFR exon 19 deletion mutation when treated with first-line osimertinib, and new preclinical data showing the combination of BLU-945 and osimertinib enhanced tumor regression and survival compared to osimertinib alone in a treatment-naïve EGFR L858R-mutant cell-derived model at the 2023 American Association for Cancer Research (AACR) Annual Meeting. Read the poster presentation here.
- Today announced acceptance of an investigational new drug (IND) application to the FDA for BLU-525.

**CDK2-vulnerable breast and other cancers**

- Announced the FDA lifted the partial clinical hold on patient enrollment in the VELA trial of BLU-222. Patients already enrolled in the trial have continued on the study and trial sites are working to resume patient enrollment.
- Announced plans to present results from the ongoing dose escalation of the Phase 1/2 VELA trial of BLU-222 in breast cancer and other cancers vulnerable to CDK2 inhibition, showing evidence of monotherapy safety and pathway modulation, at the 2023 ASCO Annual Meeting.

**Key Upcoming Milestones**

The company plans to achieve the following milestones by mid-2023:

- Obtain FDA approval and initiate launch of AYVAKIT in ISM in mid-2023.
- Present initial CONCERTO trial dose escalation data in EGFR exon 20 NSCLC at ASCO 2023.
- Present initial VELA trial dose escalation data at ASCO 2023.
- Nominate a development candidate targeting wild-type KIT for chronic urticaria by mid-2023.

**First Quarter 2023 Results**

- **Revenues**: Revenues were $63.3 million for the first quarter of 2023, including $39.1 million of net product revenues from sales of AYVAKIT/AYVAKYT and $24.2 million in collaboration revenues. Blueprint Medicines recorded revenues of $62.7 million in the first quarter of 2022, including $23.8 million of net product revenues from sales of AYVAKIT/AYVAKIT and $38.9 million in collaboration revenues.
- **Cost of Sales:** Cost of sales was $3.2 million for the first quarter of 2023, as compared to $5.1 million for the first quarter of 2022.
- **R&D Expenses:** Research and development expenses were $112.1 million for the first quarter of 2023, as compared to $103.1 million for the first quarter of 2022. This increase was primarily due to increased compensation related costs driven by increased headcount, along with increased costs related to early discovery efforts. Research and development expenses included $10.1 million in stock-based compensation expenses for the first quarter of 2023.
- **SG&A Expenses:** Selling, general and administrative expenses were $71.0 million for the first quarter of 2023, as compared to $57.1 million for the first quarter of 2022. This increase was primarily due to increased compensation related costs driven by increased headcount, along with increased costs associated with expanding our commercial infrastructure for commercialization of AYVAKIT/AYVAKYT. Selling, general, and administrative expenses included $13.1 million in stock-based compensation expenses for the first quarter of 2023.
- **Net Loss**: Net loss was $129.6 million for the first quarter of 2023, or a net loss per share of $2.15, as compared to a net loss of $106.0 million for the first quarter of 2022, or a net loss per share of $1.79.
- **Cash Position**: As of March 31, 2023, cash, cash equivalents and investments were $961.3 million, as compared to $1,078.5 million as of December 31, 2022.

**2023 Financial Guidance**

Blueprint Medicines has updated its financial guidance and now anticipates approximately $135 million to $145 million in AYVAKIT net product revenues for advanced SM and GIST in 2023, and $40 million to $50 million in collaboration revenues from existing collaborations in 2023. This guidance excludes revenue from the anticipated AYVAKIT indication expansion in ISM in mid-2023. The company continues to expect that its existing cash, cash equivalents and investments, together with anticipated future product revenues, will provide sufficient capital to enable the company to achieve a self-sustainable financial profile.

**Conference Call Information**

Blueprint Medicines will host a live conference call and webcast at 8:00 a.m. ET today to discuss first quarter 2023 financial results and recent business activities. The conference call may be accessed by dialing 833-470-1428 (domestic) or 929-526-1599 (international), and referring to conference ID 668091. A webcast of the call will also be available under "Events and Presentations" in the Investors & Media section of the Blueprint Medicines website at http://ir.blueprintmedicines.com/. The archived webcast will be available on Blueprint Medicines' website approximately two hours after the conference call and will be available for 30 days following the call.

**Upcoming Investor Conferences**

Blueprint Medicines will participate in one upcoming investor conference:

- **Goldman Sachs 44th Annual Global Healthcare Conference** on Wednesday, June 14, 2023 at 12:20 pm ET.

A live webcast of each presentation will be available by visiting the Investors & Media section of Blueprint Medicines' website at http://ir.blueprintmedicines.com. A replay of the webcasts will be archived on Blueprint Medicines' website for 30 days following each presentation.

**About Blueprint Medicines**

Blueprint Medicines is a global precision therapy company that invents life-changing therapies for people with cancer and blood disorders. Applying an approach that is both precise and agile, we create medicines that selectively target genetic drivers, with the goal of staying one step ahead across stages of disease. Since 2011, we have leveraged our research platform, including expertise in molecular targeting and world-class drug design capabilities, to rapidly and reproducibly translate science into a broad pipeline of precision therapies. Today, we are delivering our approved medicines to patients in the U.S. and Europe, and we are globally advancing multiple programs for systemic mastocytosis (SM), lung cancer and other genomically defined cancers, and cancer immunotherapy. For more information, visit www.BlueprintMedicines.com and follow us on Twitter (@BlueprintMeds) and LinkedIn.

**Cautionary Note Regarding Forward-Looking Statements**

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, statements regarding plans, strategies, timelines and expectations for Blueprint Medicines' current or future approved drugs and drug candidates, including approvals and launches, the initiation of clinical trials or the results of ongoing and planned clinical trial; Blueprint Medicines' plans, strategies and timelines to nominate development candidates; timelines and expectations for interactions with the FDA and other regulatory authorities; statements regarding the plans and potential benefits of AYVAKIT in treating patients with indolent SM; statements regarding plans and expectations for Blueprint Medicines' current or future approved drugs and drug candidates; the potential benefits of any of Blueprint Medicines' current or future approved drugs or drug candidates in treating patients; and Blueprint Medicines' financial performance, strategy, goals and anticipated milestones, business plans and focus. The words "aim," "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation: preliminary activity and safety data may not be representative of more mature data; the risk of delay of any current or planned clinical trials or the development of Blueprint Medicines' current or future drug candidates; risks related to Blueprint Medicines' ability to successfully demonstrate the safety and efficacy of its drug candidates and gain approval of its drug candidates on a timely basis, if at all; preclinical and clinical results for Blueprint Medicines' drug candidates may not support further development of such drug candidates either as monotherapies or in combination with other agents or may impact the anticipated timing of data or regulatory submissions; the timing of the initiation of clinical trials and trial cohorts at clinical trial sites and patient enrollment rates may be delayed or slower than anticipated; actions of regulatory agencies may affect the initiation, timing and progress of clinical trials; the success of Blueprint Medicines' current and future collaborations, financing arrangements, partnerships or licensing arrangements the COVID-19 pandemic may impact Blueprint Medicines' business, operations, strategy, goals and anticipated milestones, including ongoing and planned research and discovery activities, Blueprint Medicines' ability to conduct ongoing and planned clinical trials; and risks related to Blueprint Medicines' ability to obtain, maintain and enforce patent and other intellectual property protection for its products and current or future drug candidates it is developing. Any forward-looking statements contained in this press release represent Blueprint Medicines' views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date. Except as required by law, Blueprint Medicines explicitly disclaims any obligation to update any forward-looking statements.

**Trademarks**

Blueprint Medicines, AYVAKIT, AYVAKYT and associated logos are trademarks of Blueprint Medicines Corporation.

**Blueprint Medicines Corporation**
**Selected Condensed Consolidated Balance Sheet Data**
**(in thousands)**
***(unaudited)***

| | March 31, 2023 | December 31, 2022 |
|---|---|---|
| Cash, cash equivalents and marketable securities | $ 961,311 | $ 1,078,472 |
| Working capital (1) | 727,660 | 863,417 |
| Total assets | 1,220,370 | 1,349,902 |
| Liability related to the sale of future royalties and revenues (2) | 434,593 | 430,330 |
| Term loan (2) | 139,512 | 139,083 |
| Deferred revenue | 8,740 | 18,291 |
| Total liabilities | 806,072 | 835,225 |
| Total stockholders' equity | 414,298 | 514,677 |

(1) Blueprint Medicines defines working capital as current assets less current liabilities.
(2) Amount includes both current and non-current portions of the balances.

**Blueprint Medicines Corporation**
**Condensed Consolidated Statements of Operations Data**
**(in thousands, except per share data)**
***(unaudited)***

| | Three Months Ended March 31, | |
|---|---|---|
| | 2023 | 2022 |
| Revenues: | | |
| Product revenue, net | $ 39,069 | $ 23,841 |
| Collaboration revenue | 24,218 | 38,890 |
| Total revenues | 63,287 | 62,731 |
| Cost and operating expenses: | | |
| Cost of sales | 3,175 | 5,079 |
| Collaboration loss sharing | 1,296 | 3,265 |
| Research and development | 112,073 | 103,133 |
| Selling, general and administrative | 70,950 | 57,058 |
| Total cost and operating expenses | 187,494 | 168,535 |
| Other income (expense): | | |
| Interest income (expense), net | (5,819) | 442 |
| Other income (expense), net | 986 | (453) |
| Total other expense, net | (4,833) | (11) |
| Loss before income taxes | (129,040) | (105,815) |
| Income tax expense | 520 | 184 |
| Net loss | $ (129,560) | $ (105,999) |
| Net loss per share — basic and diluted | $ (2.15) | $ (1.79) |
| Weighted-average number of common shares used in net loss per share — basic and diluted | 60,126 | 59,312 |

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