
# Second Quarter 2024 Financial Results

AUG 1, 2024

# Agenda

INTRODUCTION

**Kate Haviland**
Chief Executive Officer

AYVAKIT PERFORMANCE

**Philina Lee, PhD**
Chief Commercial Officer

CORPORATE PROGRESS

**Christy Rossi**
Chief Operating Officer

Q2 2024 FINANCIAL PERFORMANCE

**Mike Landsittel**
Chief Financial Officer

# Forward-looking statements

This presentation 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 the company's future business growth, including its expectations regarding continued growth in the breadth and depth of prescribing; its plans to initiate registration-enabling Part 2 of the HARBOR trial in ISM by the end of 2024; its goal of executing a partnership for BLU-222 by the end of 2024; its expectations related to the markets for the company's current or future approved drugs and drug candidates; statements regarding the continued reduction of the company's operating expenses and cash burn; statements regarding plans and expectations for the company's current or future approved drugs and drug candidates; the potential benefits of any of the company's current or future approved drugs or drug candidates in treating patients; statements related to the company's liquidity and capital position, including expectations that its cash, cash equivalents and investments will provide a durable capital position which, together with anticipated product revenues, will enable it to reach a self-sustainable financial profile; and the company's product revenues, 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 presentation 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 presentation, including, without limitation: the risk that the marketing and sale of AYVAKIT/ AYVAKYT or any future approved drugs may be unsuccessful or less successful than anticipated, or that AYVAKIT/ AYVAKYT may not gain market acceptance by physicians, patients, third-party payors and others in the medical community; the risk that the market opportunities for AYVAKIT/ AYVAKYT or the company's drug candidates are smaller than it estimates or that any approval it obtains may be based on a narrower definition of the patient population that it anticipates; the risk that the company may be unable to execute a partnership for BLU-222 on a timely basis or at all; the risk of delay of any current or planned clinical trials or the development of the company's current or future drug candidates; risks related to the company's 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; the risk that preclinical and clinical results for the company's 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 risk that 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; the risk that actions of regulatory agencies may affect the company's approved drugs or its current or future drug candidates, including affecting the initiation, timing and progress of clinical trials; risks related to the company's ability to obtain, maintain and enforce patent and other intellectual property protection for its products and current or future drug candidates it is developing; risks related to the success of the company's current and future collaborations, financing arrangements, partnerships, licensing and other arrangements; risks related to the company's liquidity and financial position, including the risk that it may be unable to generate sufficient future product revenues to achieve and maintain a self-sustainable financial profile; and risks related to the accuracy of the company's estimates of revenues, expenses and capital requirements. These and other risks and uncertainties are described in greater detail in the section entitled "Risk Factors" in the company's filings with the Securities and Exchange Commission (SEC), including the company's most recent Annual Report on Form 10-K, as supplemented by its most recent Quarterly Report on Form 10-Q and any other filings that the company has made or may make with the SEC in the future. The forward-looking statements in this presentation are made only as of the date hereof, and except as required by law, the company undertakes no obligation to update any forward-looking statements contained in this presentation as a result of new information, future events or otherwise. Accordingly, readers are cautioned not to place undue reliance on these forward-looking statements.

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# Blueprint Medicines Q2 2024 highlights


## Driving AYVAKIT® (avapritinib) Revenue Inflection

**Achieved $114.1 M in AYVAKIT revenue** in Q2, representing >185% YoY growth

**Raising AYVAKIT revenue guidance** to **$435 - $450M** for 2024

**Continued strength** across revenue drivers


## Building a Synergistic R&D Portfolio

Leveraging **mast cell expertise** to expand R&D in allergy and inflammation

**Initiated BLU-808 HV study**

**Continue to advance strategic partnering discussions** for CDK2i BLU-222

## Maintaining Financial Strength

Strong and **durable financial position** with $868.5 M in cash

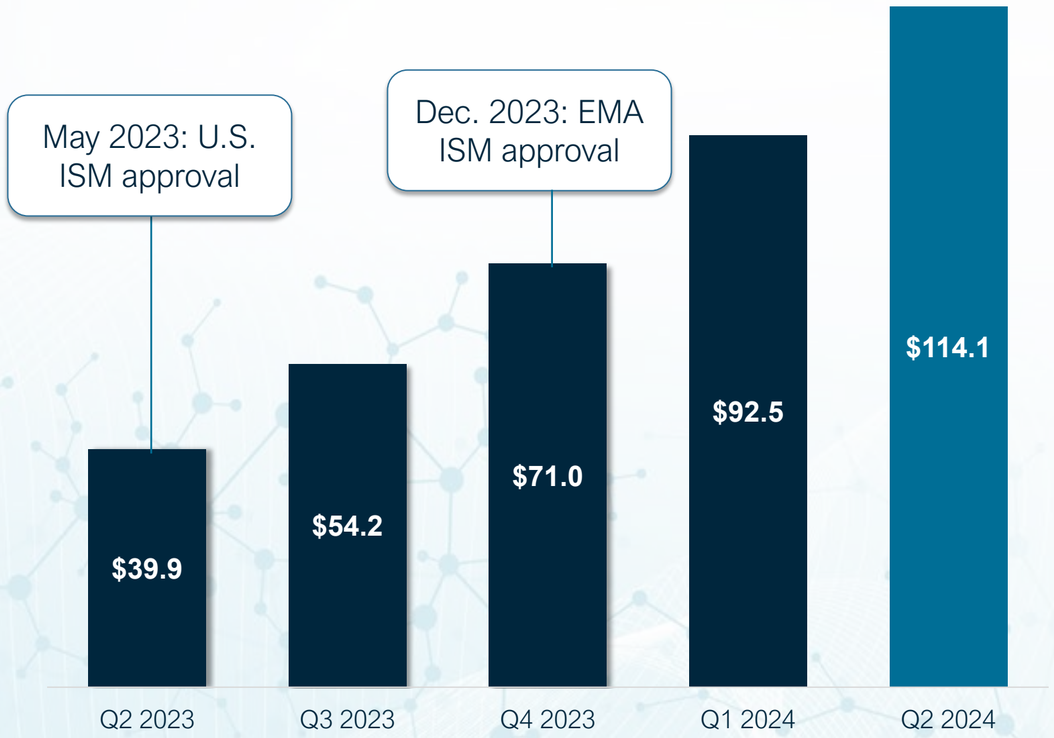
A financial profile that enables us to **invest sustainably in innovation**



YoY, year-over year; R&D, research and development; IND, investigational new drug; HV, healthy volunteer; CDK2i, cyctin-dependent kinase 2 inhibitor

# AYVAKIT revenue has grown more than 185% year-over-year

## AYVAKIT Global Net Revenues ($, Millions)




AYVAKIT® avapritinib | tablets

**Q2 highlights**

- Strong and steady growth in patients on therapy, driven by new patient starts and low discontinuation rates
- Continued high compliance
- Trend towards multi-year duration of therapy
- Free goods <20% since ISM approval
- Strong international performance, including ISM launch in Germany

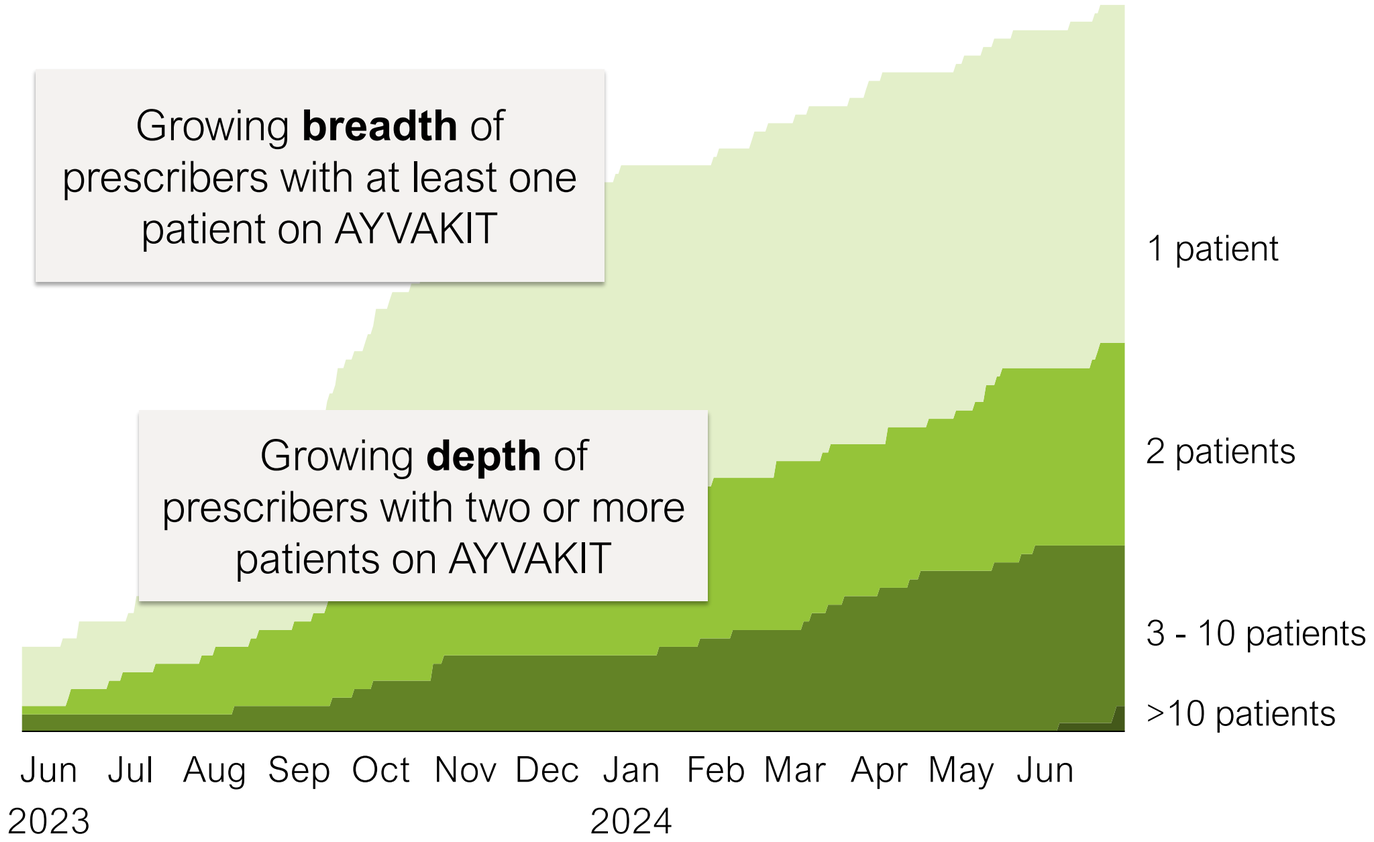
**AYVAKIT net product revenue guidance updated to $435 - $450M for FY 2024**



ISM, indolent systemic mastocytosis; EMA, European Medicines Agency; DOT, duration of therapy

# Driving breadth and depth with significant headroom for future growth

## GROWING BREADTH AND DEPTH AMONG TOP 400 TREATERS BY SM PATIENT VOLUME




- Continued **growth in overall breadth of prescribing,** including allergists
- **~60/40%** split in prescribing at **academic vs. community** accounts
- **~40/60%** of volume driven by **new vs. existing prescribers**
- **~75%** of new SM starts at 25 mg dose

1. Blueprint Medicines data on file. Cumulative 25 mg AYVAKIT prescribers within the top 400 targets since ISM approval in May 2023. Data based upon SP/HUB prescriptions which represent ~70% of total AYVAKIT volume in U.S.

# Supporting AYVAKIT patient activation through education and programming

*"I was teaching classes and getting pulled out weekly for anaphylaxis for almost six weeks straight and **I haven't been to ER in about three months**."*

**Patient on AYVAKIT**

*"I just **jumped in 100%,** ready to try anything, and it was the **best decision I've ever made**."*

**Patient on AYVAKIT**

*"**I would consider it**…recently found an AI who treats people with mast cell disorders, and I **feel like I am getting a team to know me** and be more knowledgeable and it took a while to get my diagnosis."*

**Patient Considering AYVAKIT**

1. Blueprint Medicines patient market research, fielded May 2024.

# Pipeline leadership across allergy/inflammation and oncology

## Elenestinib

Next-generation KIT D816V inhibitor

Part 2 of HARBOR study in ISM expected to initiate by end of year

## BLU-808

Wild-type KIT inhibitor for chronic urticaria and other mast cell disorders

HV study initiated

## Cell cycle inhibition

BLU-222 is validating the importance of complete cell cycle inhibition

Advancing cell cycle degraders

**Driving the next wave of innovation and growth at Blueprint Medicines**

ISM, indolent systemic mastocytosis; HV, healthy volunteer

# Strong financial position driven by growing product revenue and continued operating expense reduction

| Statement of Operations (unaudited) | Three Months Ended 6/30/2024 | Three Months Ended 6/30/2023 | Six Months Ended 6/30/2024 | Six Months Ended 6/30/2023 |
|---|---|---|---|---|
| Total revenue | $138.2M | $57.6M | $234.3M | $120.9M |
| Net product sales | $114.1M | $39.9M | $206.7M | $78.9M |
| Collaboration, license and other revenue | $24.0M | $17.7M | $27.6M | $41.9M |
| Cost of sales | $7.6M | $2.3M | $10.8M | $5.5M |
| Collaboration loss sharing | $0.0M | $1.2M | $0.0M | $2.5M |
| Research & development expense[1] | $84.3M | $110.1M | $172.5M | $222.1M |
| Selling, general & admin expense[2] | $89.3M | $71.9M | $172.9M | $142.9M |
| Other income (expense), net[3] | $(6.8)M | $(4.6)M | $161.3M | $(9.5)M |
| Net income (loss) | $(50.0)M | $(132.8)M | $39.1M | $(262.4)M |
| **Balance Sheet (unaudited)** | | | **6/30/2024** | **12/31/2023** |
| Cash, cash equivalents, and investments | | | $868.5M | $767.2M |

1. Includes stock-based compensation expense of $12.3M and $23.2M for the three and six months ended 6/30/24, and $10.2M and $20.3M for the three and six months ended 6/30/23, respectively.
2. Includes stock-based compensation expense of $15.7M and $29.1M for the three and six months ended 6/30/24, and $13.6M and $26.7M for the three and six months ended 6/30/23, respectively.
3. Includes debt extinguishment gain of $173.7 million in the six months ended 06/30/24.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Thank You