Third Quarter 2024 Financial Results

OCT 30, 2024

Agenda

INTRODUCTION

Kate Haviland
Chief Executive Officer

AYVAKIT PERFORMANCE

Philina Lee, PhD
Chief Commercial Officer

CORPORATE PROGRESS

Christy Rossi
Chief Operating Officer

Q3 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 and its net product revenue in 2024; its plans to initiate registration-enabling Part 2 of the HARBOR trial in ISM and complete a Phase 1 trial for BLU-222 to inform registration plans by the end of 2024; its plans to present data from its BLU-808 Phase 1 study in healthy volunteers in early 2025; 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 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.

This presentation also contains estimates, projections and other statistical data made by independent parties and by the company relating to market size and growth and other data about the company's industry. These data involve a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. In addition, projections, assumptions and estimates of the company's future performance and the future performance of the markets in which the company operates are necessarily subject to a high degree of uncertainty and risk.

Blueprint Medicines Q3 2024 highlights

Driving AYVAKIT® (avapritinib) Revenue Growth

Achieved $128.2M in AYVAKIT revenue in Q3, representing >135% YoY growth

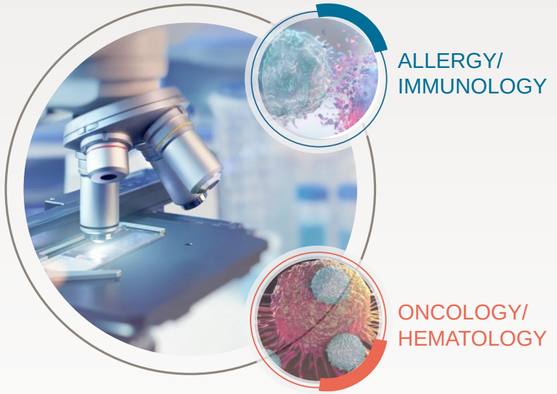
Raising AYVAKIT revenue guidance to **$475-$480M** for 2024

Continued strength across revenue drivers

Maintaining Financial Strength

Strong and **durable financial position** with $882.4M in cash

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



Building a Synergistic R&D Portfolio

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

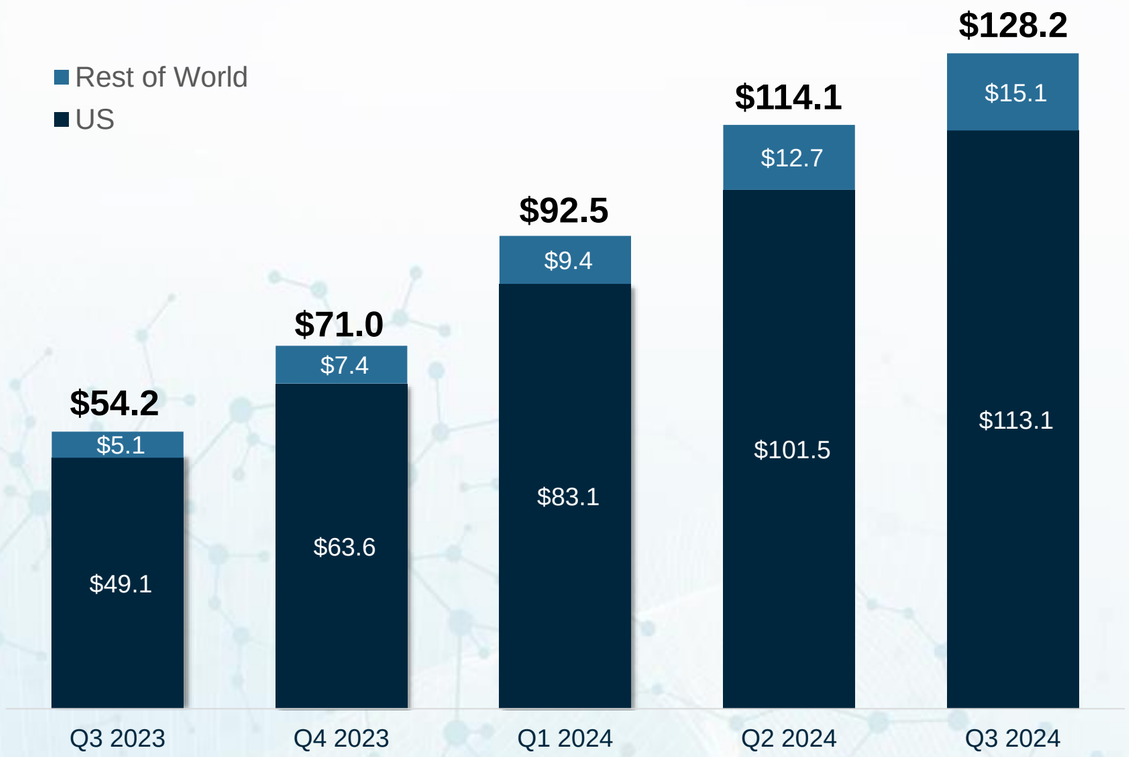
On track to initiate registration-enabling **HARBOR Part 2 study** of elenestinib in ISM

Advancing BLU-808 HV study with data expected in early 2025

YoY, year-over year; R&D, research and development; HV, healthy volunteer; ISM, indolent systemic mastocytosis

AYVAKIT revenue has grown more than 135% year-over-year

AYVAKIT Global Net Revenues ($, Millions)



AYVAKIT® avapritinib | tablets

Q3 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 $475-$480M for FY 2024

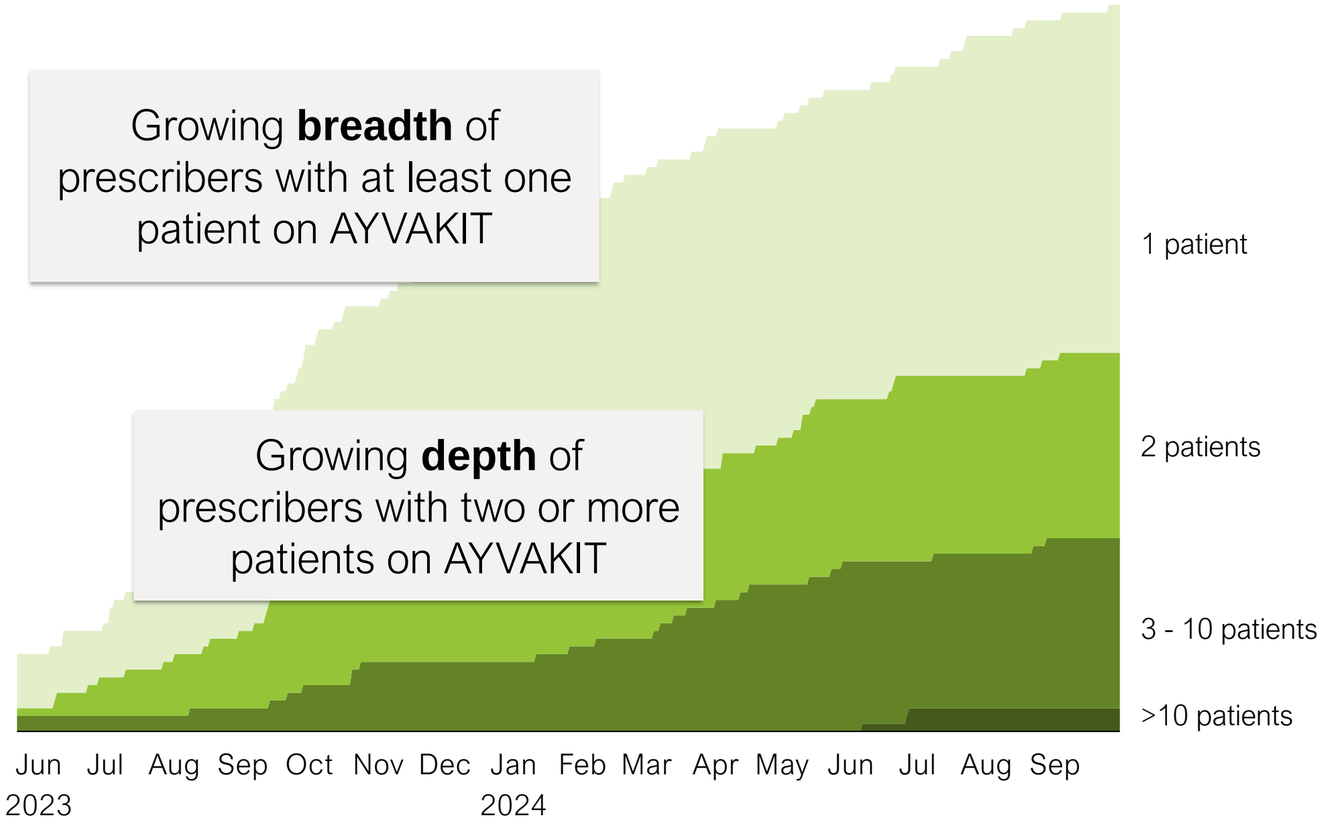
blueprint™ MEDICINES

Not for promotional use

Total revenue may differ from United States plus Rest of World revenue due to rounding.

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 breadth and depth of prescriber base, with **significant opportunity for expansion**
- ✓ Differential diagnosis from other diseases that share similar symptom burden, indicative of the **broadening aperture of who may be a candidate** for AYVAKIT
- ✓ Even split in community and academic prescribing, signaling **reach beyond centers of excellence**

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.

Campaigns to grow AYVAKIT awareness and urgency to treat among providers and patients

Branded campaign for providers

Direct-to-patient ad campaign

Disease awareness plus long-term safety and efficacy data drive urgency to treat

Growing mountain of data demonstrates commitment to community to advance understanding of SM disease and treatment

Pivotal PIONEER data for AYVAKIT in ISM — Q1 2023

AYVAKIT ISM approval (US) — Q2 2023

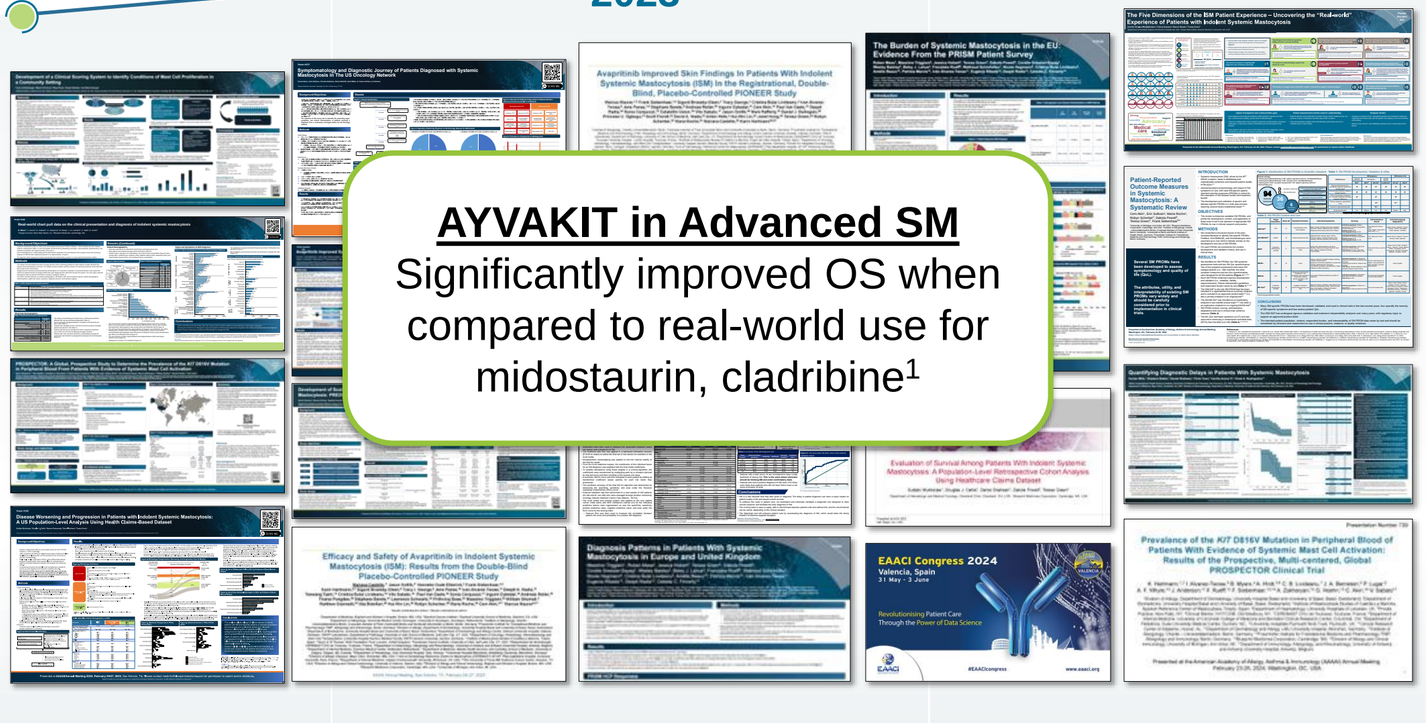
Q3 2023

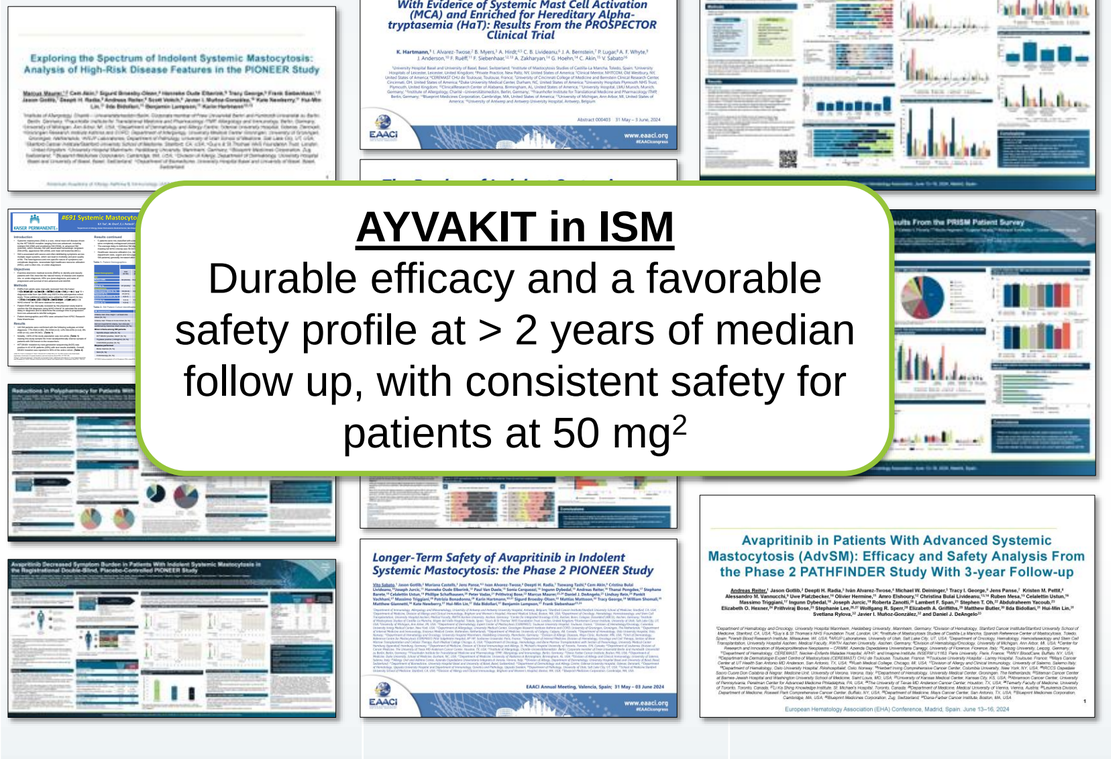
Q4 2023

AYVAKYT ISM approval (EU) — Q1 2024

Q2 2024

Half-billion-dollar revenue run rate — Q3 2024





AYVAKIT in Advanced SM

Significantly improved OS when compared to real-world use for midostaurin, cladribine[1]

AYVAKIT in ISM

Durable efficacy and a favorable safety profile at > 2 years of median follow up, with consistent safety for patients at 50 mg[2]

1. Reiter et al. EHA Hybrid Congress, June 2022. 2. Sabato et al. EAACI Annual Meeting, June 2024.

On track to complete anticipated portfolio milestones in 2024

In addition to achieving **AYVAKIT revenue of $475-480M**, Blueprint expects the following data-related milestones in 2024:

Area	Program	Milestone	Timing
Mast cell disorders	AYVAKIT	Present long-term safety and efficacy data from PIONEER trial in ISM	☑
	BLU-808	IND submission	☑
	Elenestinib	Initiate registration-enabling Part 2 of the HARBOR trial in ISM	On track for EOY
Solid tumors	BLU-222	Present data in combination with ribociclib and fulvestrant for HR+/HER2- breast cancer	☑
		Complete Phase 1 combination dose escalation for BLU-222 by end of year to inform registration plans.	On track for EOY

- Expect to present data from the healthy volunteer study of BLU-808, our oral wild-type KIT inhibitor for chronic urticaria and other mast cell diseases, in early 2025

ISM, indolent systemic mastocytosis; IND, investigational new drug; EOY, end of year

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

Statement of Operations (unaudited)	Three Months Ended 9/30/2024	Three Months Ended 9/30/2023	Nine Months Ended 9/30/2024	Nine Months Ended 9/30/2023
Total revenue	$128.2M	$56.6M	$362.4M	$177.4M
Net product sales	$128.2M	$54.2M	$334.8M	$133.2M
Collaboration, license and other revenue	$0.0M	$2.4M	$27.6M	$44.2M
Cost of sales	$1.9M	$2.8M	$12.7M	$8.3M
Collaboration loss sharing	$0.0M	$1.8M	$0.0M	$4.3M
Research & development expense[1]	$85.3M	$110.3M	$257.8M	$330.2M
Selling, general & admin expense[2]	$89.9M	$70.7M	$262.8M	$215.8M
Other income (expense), net[3]	$(7.0)M	$(4.5)M	$154.3M	$(14.0)M
Net income (loss)	$(56.3)M	$(133.7)M	$(17.1)M	$(396.1)M
Balance Sheet (unaudited)			**9/30/2024**	**12/31/2023**
Cash, cash equivalents, and investments			$882.4M	$767.2M

1. Includes stock-based compensation expense of $12.6M and $35.7M for the three and nine months ended 9/30/24, and $11.2M and $31.5M for the three and nine months ended 9/30/23, respectively.
2. Includes stock-based compensation expense of $15.7M and $44.8M for the three and nine months ended 9/30/24, and $11.9M and $38.6M for the three and nine months ended 9/30/23, respectively.
3. Includes debt extinguishment gain of $173.7 million in the nine months ended 9/30/24.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Thank You