First Quarter 2024 Financial Results

MAY 2, 2024

Agenda

INTRODUCTION

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Chief Executive Officer

AYVAKIT PERFORMANCE

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Chief Commercial Officer

THE POWERFUL MAST CELL

Fouad Namouni, MD
President, Research & Development

Q1 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 that the company expects continued growth in breadth of prescribing, and that there is an approximately $26B market for potential additional indications in 2030; 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; and the company's 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, risks and uncertainties related the company's ongoing and planned research and discovery activities, ability to conduct ongoing and planned clinical trials, clinical supply of current or future drug candidates, commercial supply of current or future approved products, and launching, marketing and selling current or future approved products; the company's ability and plans to continue to expand a commercial infrastructure, and successfully launch, market and sell current or future approved products; the company's ability to obtain marketing approval for AYVAKIT/AYVAKYT in additional geographies in the future; the rate and degree of market acceptance of AYVAKIT/AYVAKYT and any future drug candidates for which we receive marketing approval; the delay of any current or planned clinical trials or the development of the company's current or future drug candidates; the company's advancement of multiple early-stage efforts; 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 preclinical and clinical results for the company's drug candidates, which 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; actions of regulatory agencies, which may affect the initiation, timing and progress of clinical trials; the company's ability to obtain, maintain and enforce patent and other intellectual property protection for AYVAKIT/AYVAKYT or any drug candidates it is developing; the company's ability to successfully expand its operations, research platform and portfolio of therapeutic candidates, and the timing and costs thereof; and the success of the company's current and future collaborations, financing arrangements, partnerships or licensing arrangements; and the accuracy of our 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 Q1 2024 highlights

Driving AYVAKIT® (avapritinib) Revenue Inflection

Achieved $92.5M in AYVAKIT revenue, in Q1, representing >135% YoY growth

Raising AYVAKIT revenue guidance to **$390-$410M** for 2024

Continued strength across revenue drivers, including patient demand

Building a Synergistic R&D Portfolio

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

On track to submit BLU-808 IND in Q2

ASCO data to show **BLU-222 is the first investigational CDK2 inhibitor to demonstrate combination safety** with an approved CDK4/6 inhibitor

Maintaining Financial Strength

Strong and **durable financial position** with $735.6M in cash

Continued **opex reduction and decline in cash burn**

YoY, year-over year; R&D, research and development; IND, investigational new drug

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

AYVAKIT Global Net Revenues ($, Millions)

AYVAKIT® avapritinib | tablets

US Launch

- Strong and steady patient demand
- Growth in commercial patients; free goods ~20% since ISM approval
- Advanced SM duration of therapy trending at ~25 months; early days of ISM trending longer
- Majority of patients on therapy today have ISM

EU Launch

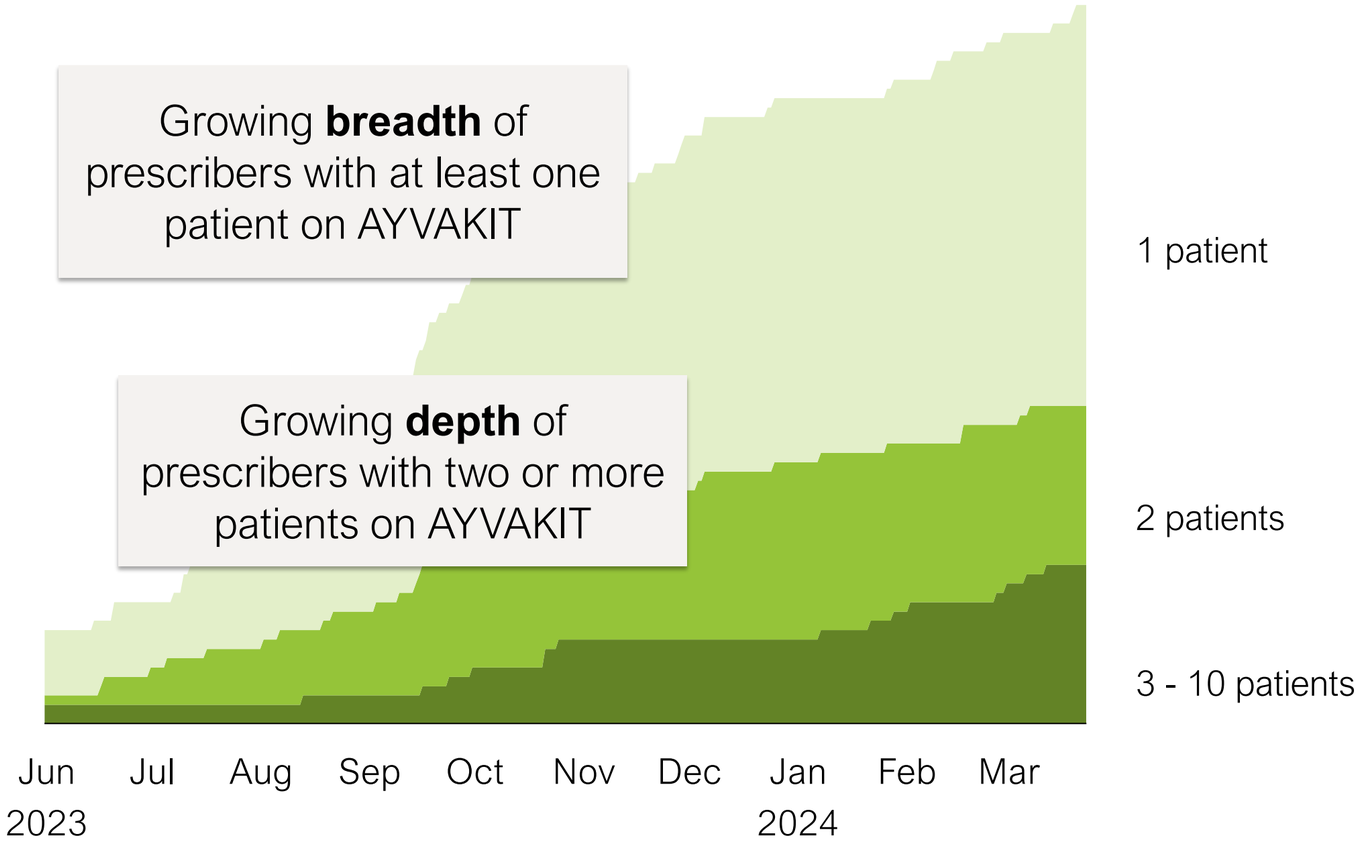
- Strong start in Germany

ISM approval marks clear revenue inflection for AYVAKIT

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
- **~50/50%** split in prescribing at **academic vs. community** accounts
- **45/55%** of volume driven by **new vs. existing prescribers**
- **~70%** 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.

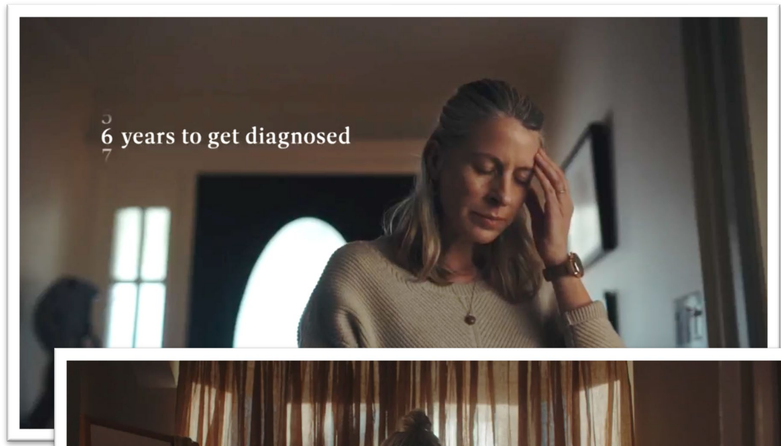
Redefining what “well-controlled” means for people living with ISM

Minimize treatment burden

Achieve broad symptomatic responses

Regain control of their lives

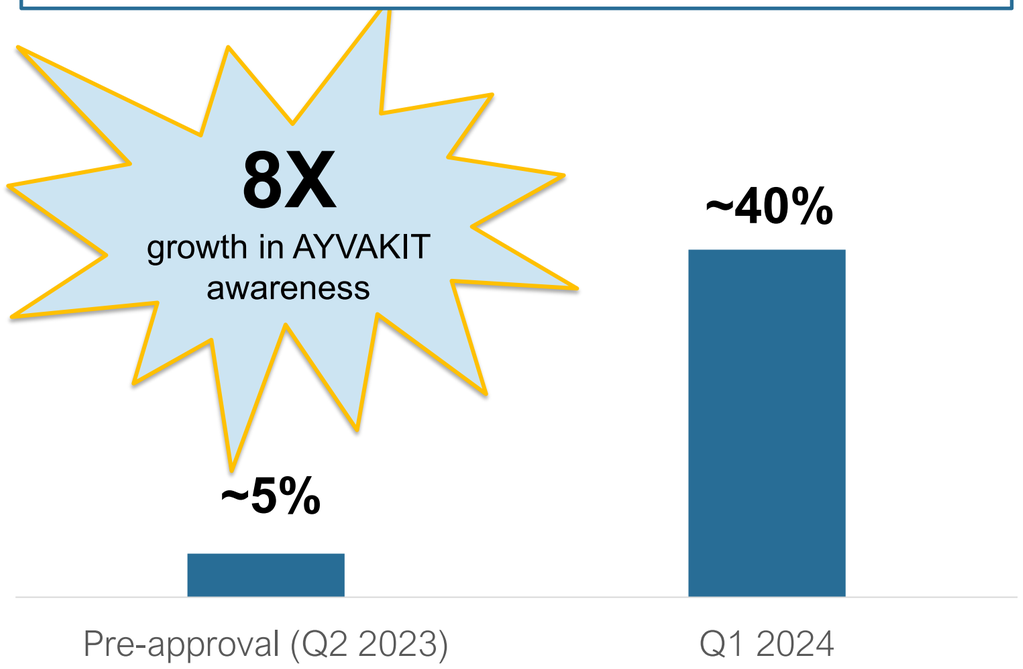
Q2 launch of targeted, unbranded, direct-to-patient media campaign to further **raise awareness** on the *Toll of Living with ISM*



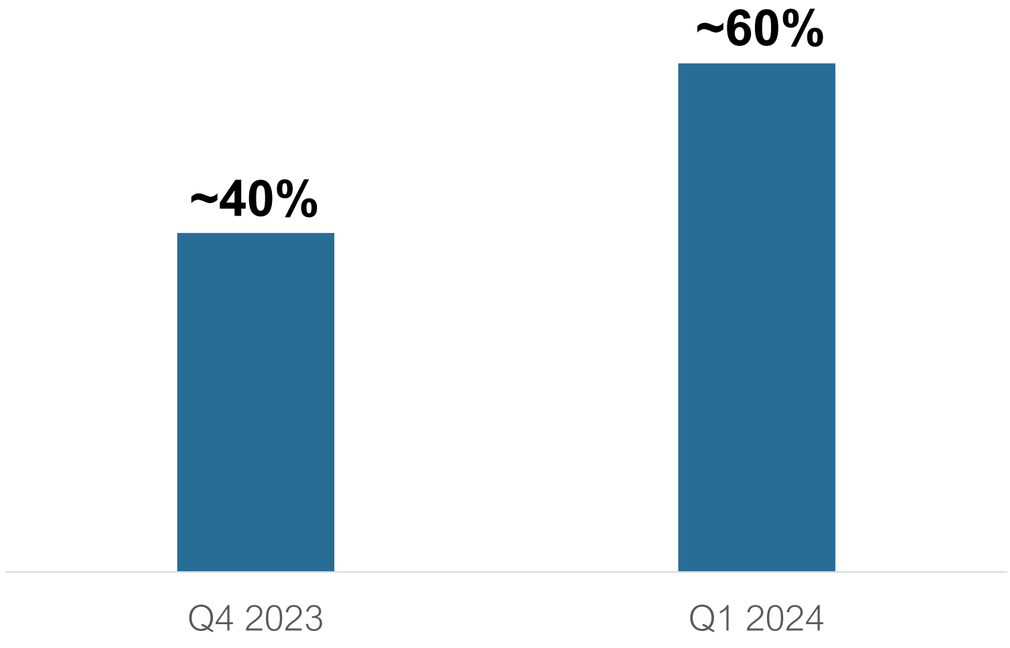
Activating patients to seek treatment with AYVAKIT

AYVAKIT® avapritinib | tablets

Unaided awareness of AYVAKIT (unprompted)

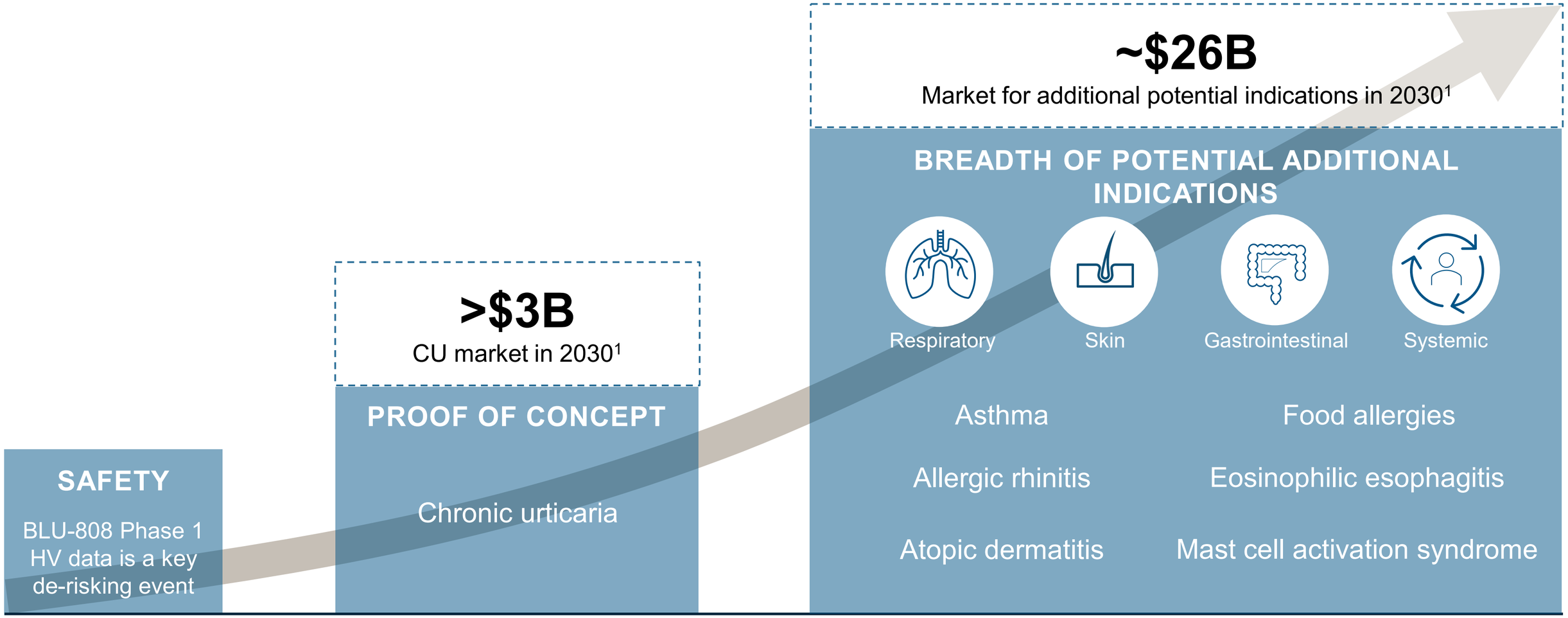


Patients' belief that they could benefit from AYVAKIT and that AYVAKIT may be an appropriate treatment option



1. Blueprint Medicines data on file. ISM Patient Awareness, Treatment, Use survey fielded March 2024.

Mast cell diseases represent significant and growing market opportunity



1. Forecasted sales of approved therapies and projected approved therapies in listed indications in 2030, as reported by EvaluatePharma

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

Statement of Operations (unaudited)	Three Months Ended 3/31/2024	Three Months Ended 3/31/2023
Total revenue	$96.1M	$63.3M
Net product sales	$92.5M	$39.1M
Collaboration revenue	$3.6M	$24.2M
Cost of sales	$3.2M	$3.2M
Collaboration loss sharing	$0.0M	$1.3M
Research & development expense[1]	$88.2M	$112.1M
Selling, general & admin expense[2]	$83.6M	$71.0M
Other income (expense), net[3]	$168.1M	$(4.8)M
Net income (loss)[3]	$89.1M	$(129.6)M
Balance Sheet (unaudited)	**3/31/2024**	**12/31/2023**
Cash, cash equivalents, and investments	$735.6M	$767.2M

1. Includes stock-based compensation expense of $10.9M and $10.1M in the three months ended 3/31/24 and 3/31/23, respectively.
2. Includes stock-based compensation expense of $13.4M and $13.1M in the three months ended 3/31/24 and 3/31/23, respectively.
3. Includes debt extinguishment gain of $173.7 million in the three months ended 03/31/24.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Thank You