

Agenda



INTRODUCTION

Kate HavilandChief Executive Officer



AYVAKIT PERFORMANCE

Philina Lee, PhD
Chief Commercial Officer



AAAAI 2024 PREVIEW

Fouad Namouni, MD
President, Research &
Development



Q4/FY 2023 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 for growth in 2024; AYVAKIT's potential as a blockbuster market opportunity in SM; whether the any of the company's product candidates will address unmet medical needs; reduction of the company's opex and cash burn in 2024; 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.

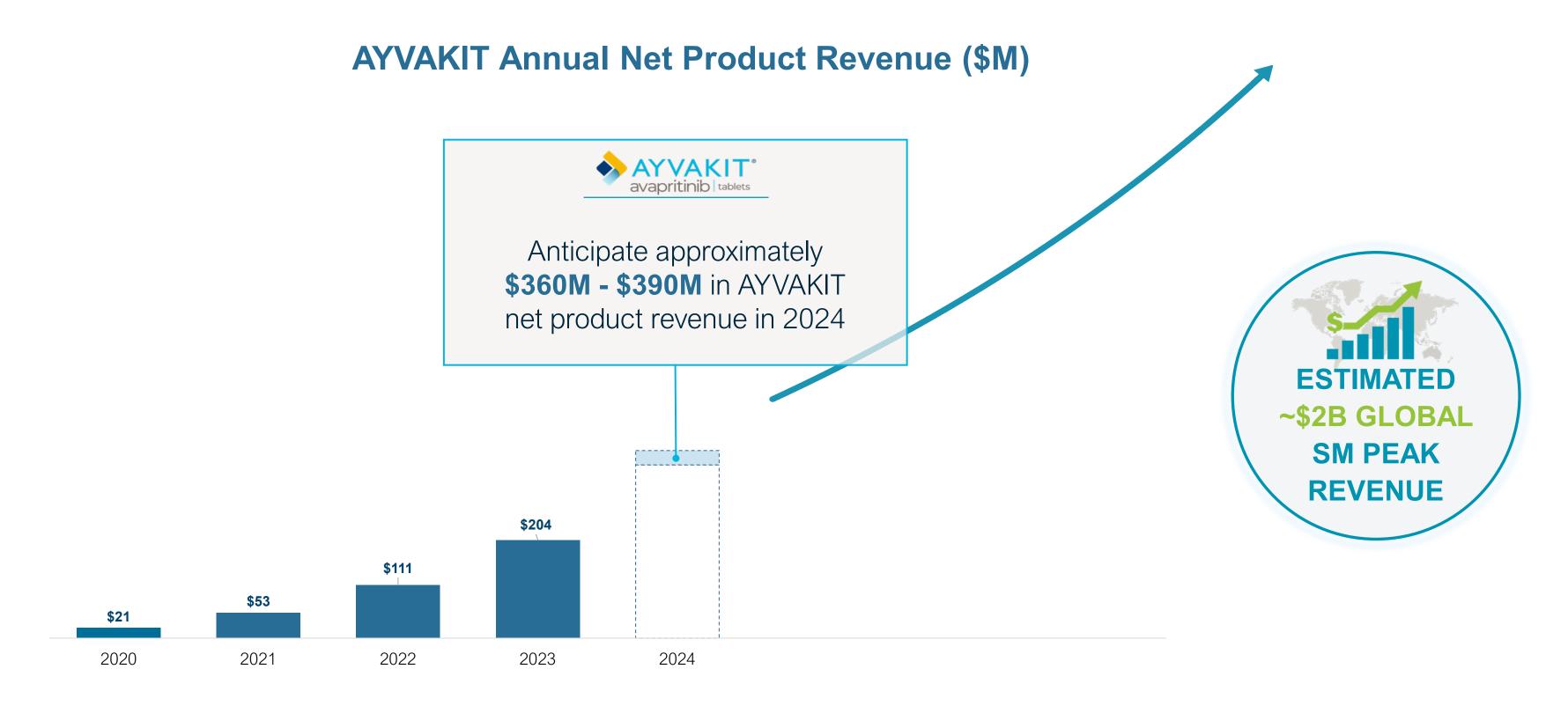
The words "aim," "may," "will," "could," "should," "expect," "plan," "anticipate," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying 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, 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 successfully expand the approved indications for AYVAKIT/AYVAKYT or obtain marketing approval for AYVAKIT/AYVAKYT in additional geographies in the future; 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. 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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AYVAKIT net product revenue anticipated to nearly double in 2024





Blueprint Medicines' accelerating growth profile



Capturing a Blockbuster Opportunity

\$204.2M in 2023 revenue

Anticipate **\$360 - \$390M** in **2024** revenue

1,000 patients on AYVAKIT in U.S.



Investing in Sustainable Innovation

AYVAKIT long-term safety and efficacy data at AAAAI

BLU-808 for chronic urticaria and other mast cell-driven diseases

BLU-222 as the backbone of combination therapy in breast cancer



Maintaining Financial Strength

> 80% YoY AYVAKIT revenue growth at guidance midpoint

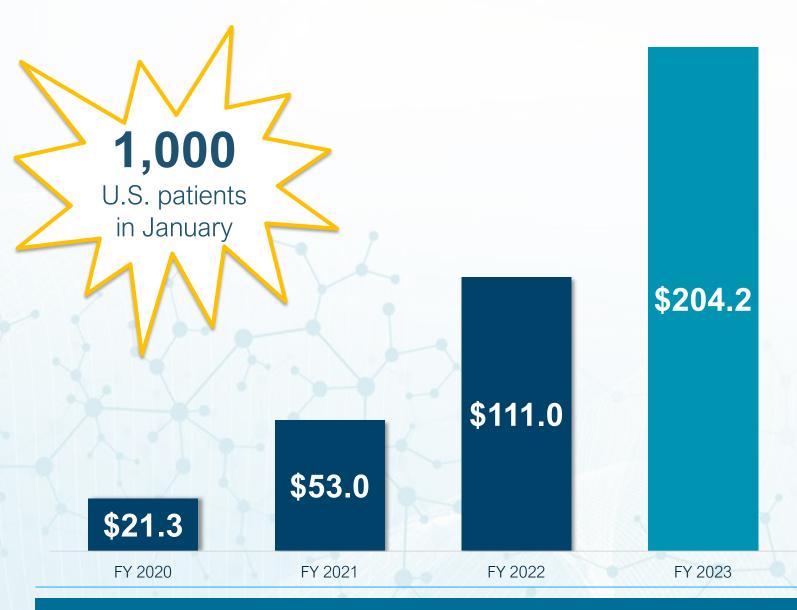
Strong and durable financial position with \$767.2M cash

Continued reduction in opex and cash burn in 2024



AYVAKIT revenue grew 84 percent in 2023

Global Net Revenues (\$, Millions)





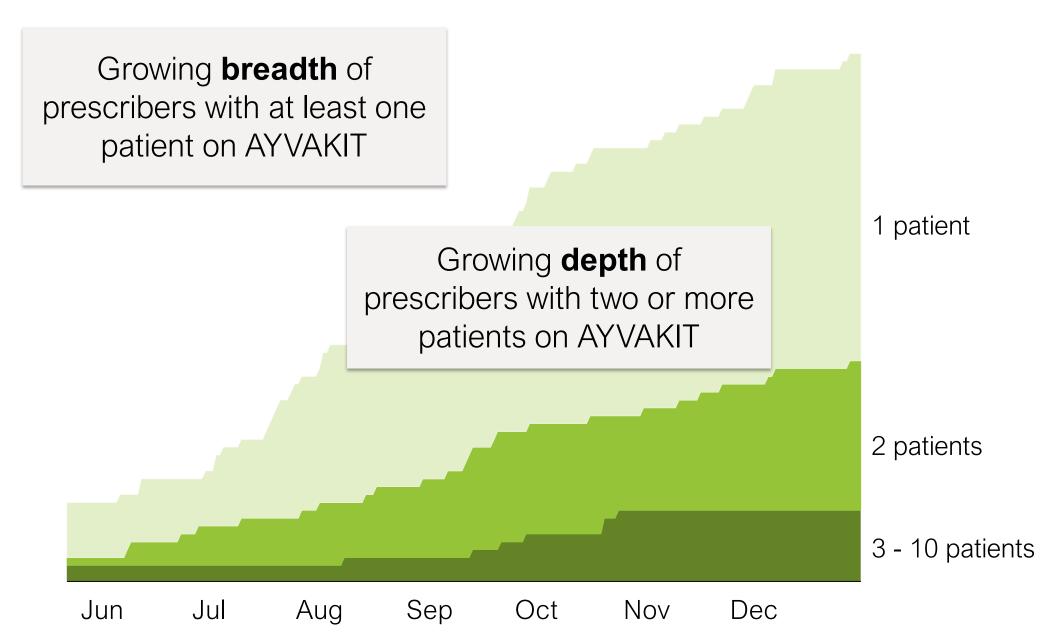
- Free goods decreased to ~25%
- Low discontinuation rates
- High compliance

Achieved \$71.0M in AYVAKIT revenue in Q4 2023



Sustaining growth in 2024 and beyond

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



- Continued growth in share of allergist prescribing
- 50/50% split in prescribing at academic vs. community accounts
- 60/40% of volume driven by new vs. existing prescribers
- 73% of new SM starts at 25 mg dose



Leadership in the science of mast cell biology and inflammatory disease





Broad and Durable Efficacy

Durable improvement across broad range of symptoms; reductions in best supportive care



Safety Profile Supporting Chronic Treatment

Treatment durations up to 4+ years in PIONEER¹; consistent safety profile at long-term follow-up



BLU-808

Highly selective and very potent wild-type

KIT inhibitor; preclinical profile and in vivo data in disease models

Leadership in SM and mast cell biology highlighted across breadth of data at AAAAI



Q4 and FY 2023 financial results

Statement of Operations (unaudited)	Three Months Ended 12/31/2023	Three Months Ended 12/31/2022	FY Ended 12/31/2023	FY Ended 12/31/2022
Total revenue	\$72.0M	\$38.8M	\$249.4M	\$204.0M
Net product sales Collaboration revenue License revenue – related party	\$71.0M \$1.0M -	\$30.1M \$8.7M -	\$204.2M \$45.2M -	\$111.0M \$65.5M \$27.5M
Cost of sales	\$0.3M	\$4.8M	\$8.5M	\$17.8M
Collaboration loss sharing	-	\$1.9M	\$4.3M	\$8.9M
Research & development expense ¹	\$97.5M	\$117.8M	\$427.7M	\$477.4M
Selling, general & admin expense ²	\$79.3M	\$64.0M	\$295.1M	\$237.4M
Net loss	\$(110.9)M	\$(158.6)M	\$(507.0)M	\$(557.5)M
Balance Sheet (unaudited)			12/31/2023	12/31/2022
Cash, cash equivalents, and investments			\$767.2M	\$1,078.5M



Expect operating expenses and cash burn will continue to decline in 2024

- Anticipate \$360 \$390 million in 2024 AYVAKIT revenue on path to blockbuster opportunity
- Prioritized capital allocation and continued reduction in R&D opex
- Increasing SG&A operating leverage from commercial infrastructure
- Declining cash burn and durable capital position on path to profitability

Operating Cash Burn Will Continue to Decline in 2024+

