Blueprint Medicines

Driving growth and innovation with operational excellence

Fourth quarter and full-year 2024 financial results

February 13, 2025

Fourth quarter and full-year 2024 financial results

INTRODUCTION

Kate Haviland
Chief Executive Officer

AYVAKIT PERFORMANCE

Philina Lee, PhD
Chief Commercial Officer

CLINICAL PROGRESS

Becker Hewes, MD
Chief Medical Officer

FINANCIAL RESULTS

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 its plans, strategies, timelines and expectations for the company's operations, including its growth strategies; its anticipated 2025 revenue and the peak revenue potential for AYVKIT and the SM franchise; its multiple future data catalysts; its planned capital allocation; the breadth and depth of prescribers; its planned geographic expansion; its expectations related to the markets for the company's current or future approved drugs and drug candidates; statements regarding the benefits plans and expectations for the company's current or future approved drugs and drug candidates; 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..

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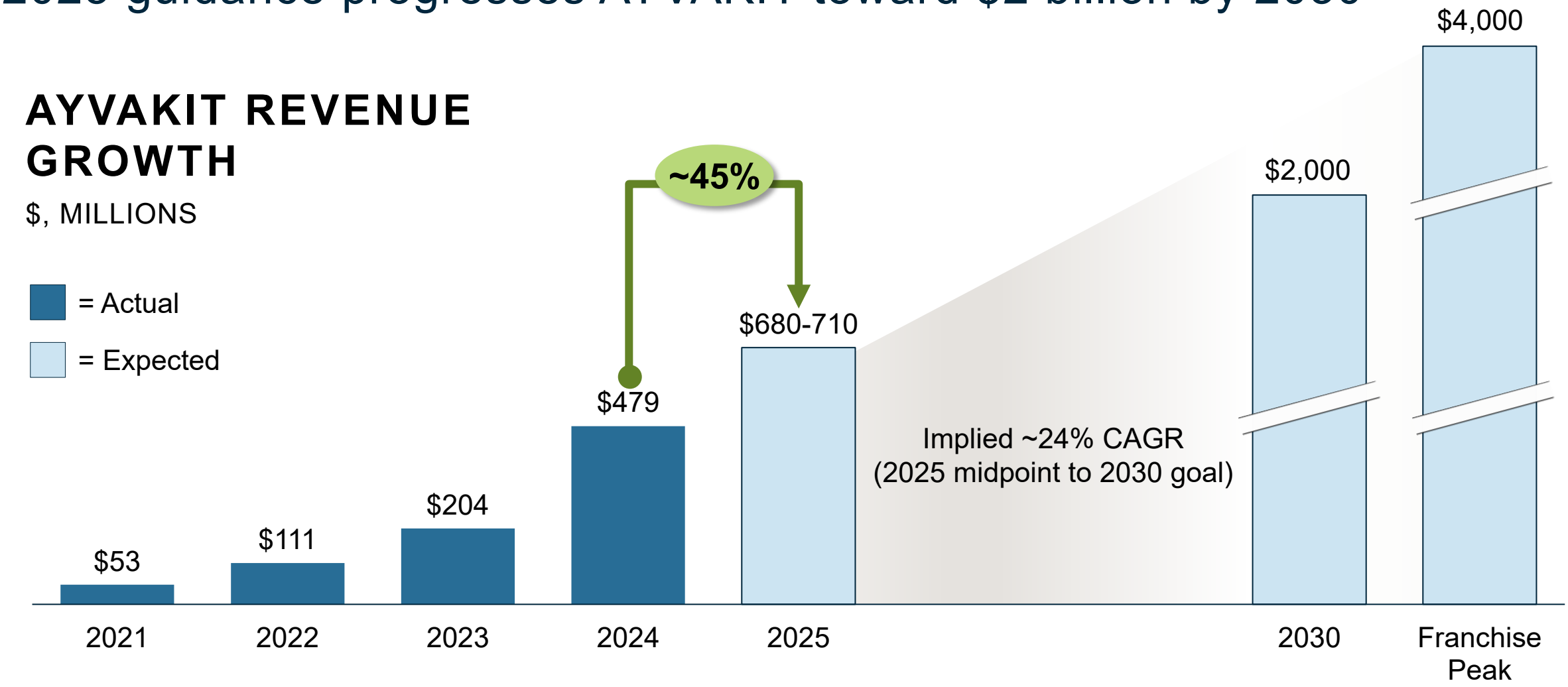
2025 guidance progresses AYVAKIT toward $2 billion by 2030

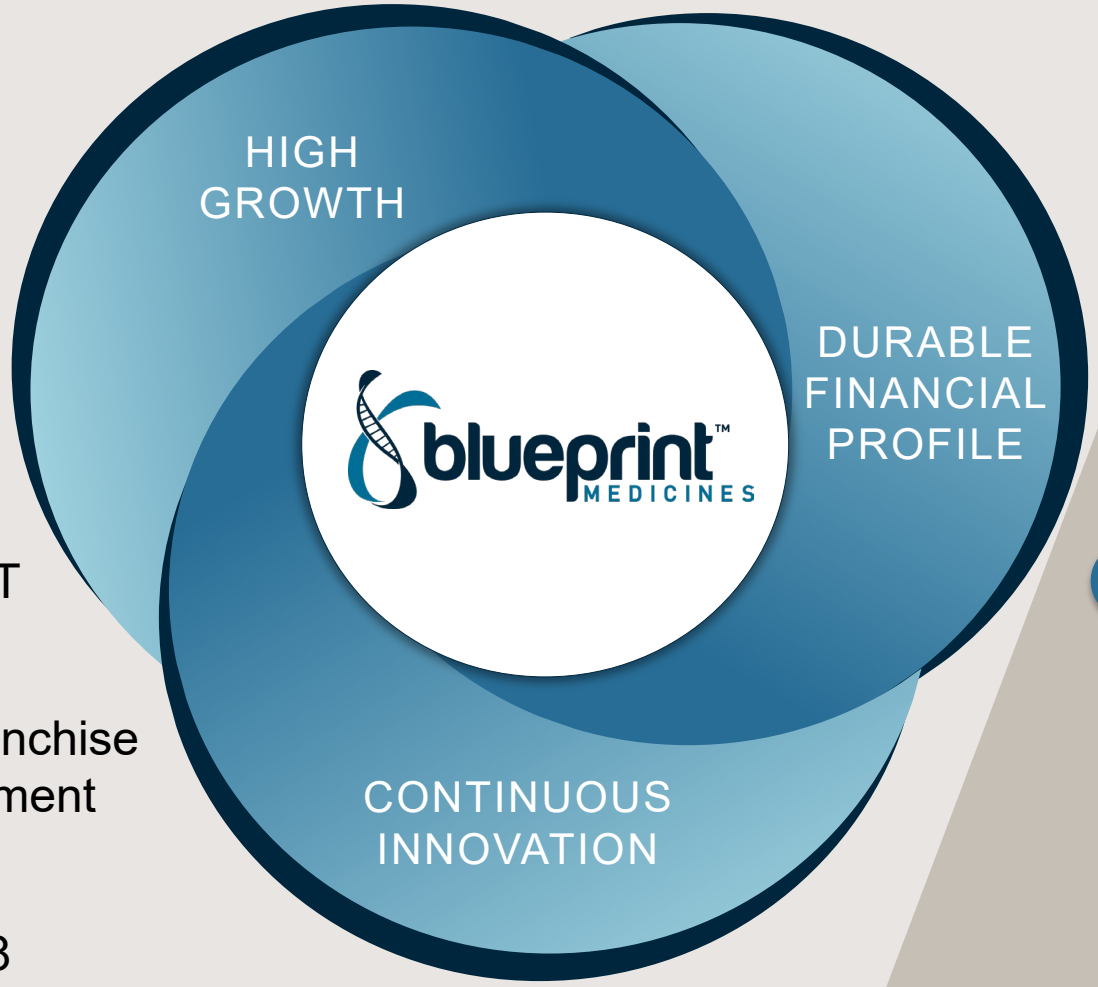


2025 guidance represents robust YoY growth consistent with other high-performing rare disease launches

Our capital allocation strategy aligns with core growth drivers

80%

2025 capital allocation



- Drive continued AYVAKIT revenue growth
- Extend long-term SM franchise with elenestinib development
- Accelerate and expand development of BLU-808

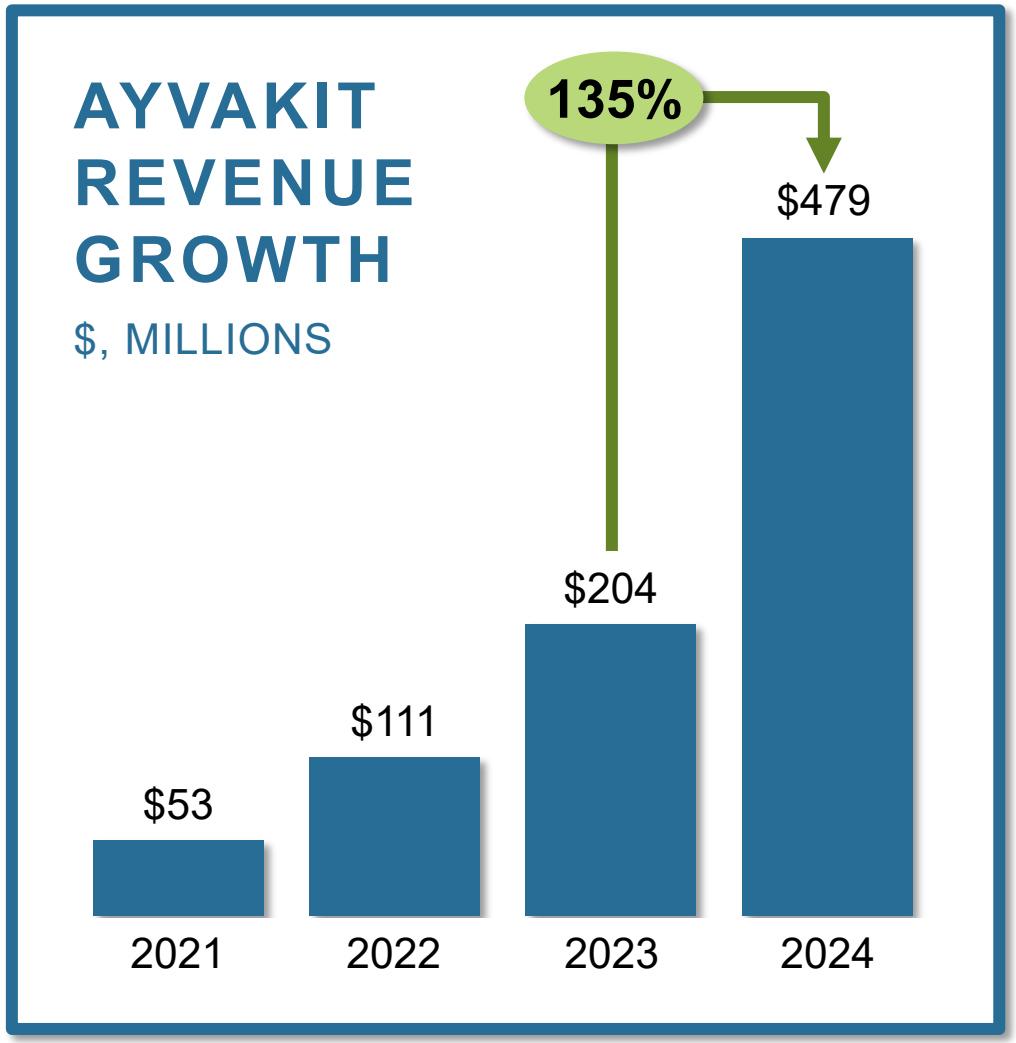
- Advance R&D innovation to fuel long-term growth

20%

2025 capital allocation

SM, systemic mastocytosis.

AYVAKIT achieved 135% year-over-year growth in 2024



Q4 2024 HIGHLIGHTS

PATIENTS

- Continued **growth in patients on therapy**
- Trend toward **multi-year durations of therapy** in ISM
- **~95% strongly agree they are satisfied** with AYVAKIT[1]

PAYERS

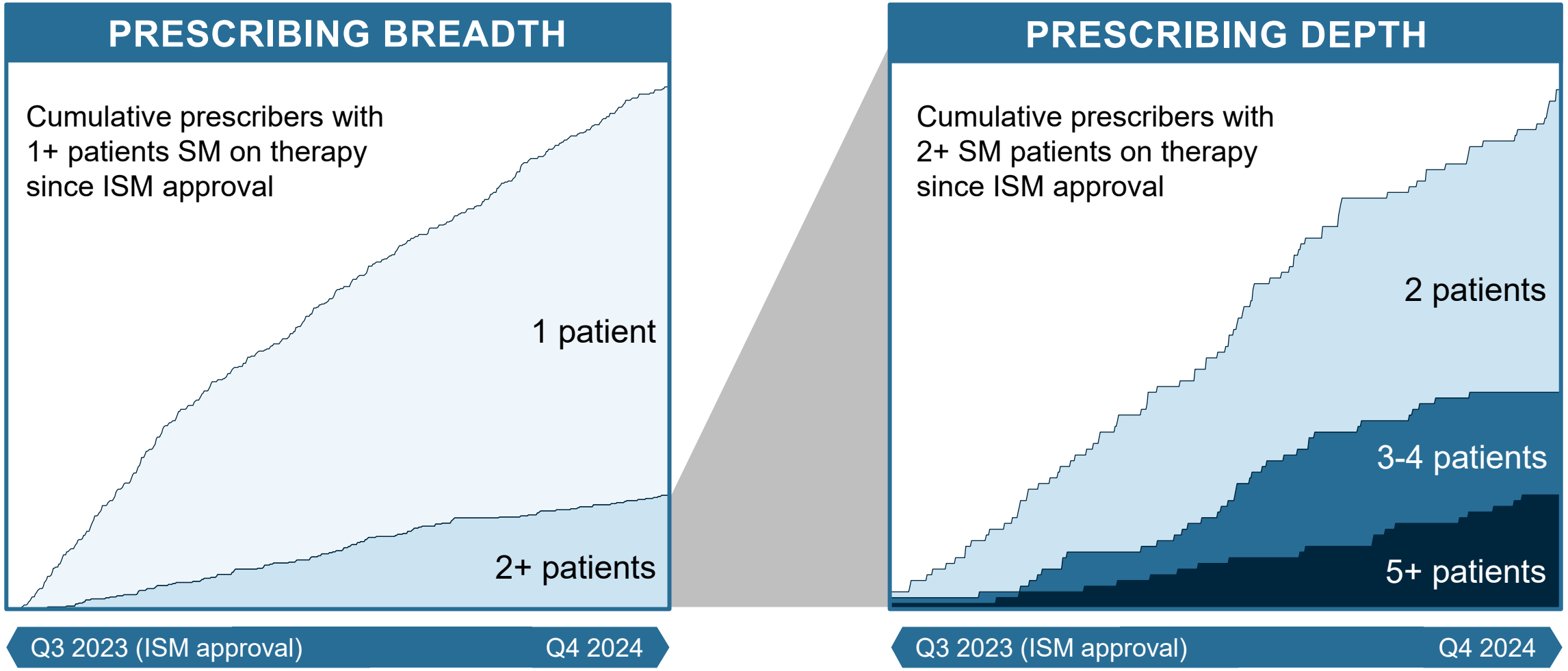
- Continued **strong access** and rapid times to fill

PROVIDERS

- Continued **growth in breadth and depth of prescribing**
- **~40/60%** of volume driven by **new vs existing** prescribers
- **~40/60%** split in new prescribing at **academic vs community** accounts
- **~70%** of new SM starts at **25 mg dose**

[1] Blueprint market research. All statistics reference 4Q24.

Driving breadth and depth with significant headroom for continued growth



Blueprint Medicines data on file. Cumulative SM AYVAKIT prescribers since ISM approval in May 2023. SM new patient starts since ISM launch are included. Data based upon SP/HUB prescriptions which represent ~70% of total AYVAKIT volume in U.S.

Building on our success to drive growth in 2025 and beyond

2025 AYVAKIT investment priorities

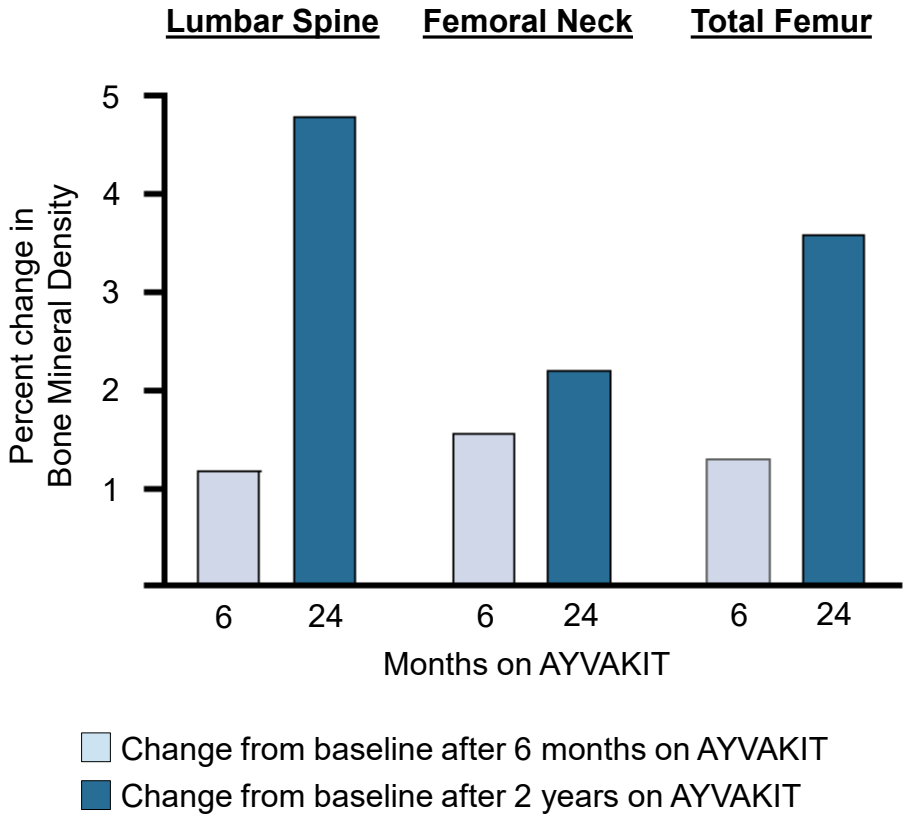
- Activate more patients and providers with new initiatives, including peer-to-peer and direct-to-consumer programs
- Generate AYVAKIT and real-world data catalyzing urgency to treat
- Launch ISM in additional geographies
- Expand commercial and medical field teams to broaden reach to additional providers and specialties

Updated AYVAKIT data support strong safety and efficacy profile plus positive impact on bone health

AYVAKIT 3-YEAR FOLLOW-UP DATA FROM PIONEER TRIAL[1]

- In commercial setting, >90% of patients who initiated treatment at 25 mg stayed at that dose at 12 months; <10% dose escalated to 50 mg[2]
- Updated PIONEER data show consistent safety and durable efficacy in patients with a median duration of 3 years and up to 5+ years
- Among the patient subset in PIONEER who escalated to 50 mg due to high disease burden:
  - Safety at 50 mg was consistent in patients with a median duration of 10 months
  - 93% of patients saw improved (77%) or stable (16%) TSS benefit at 50 mg

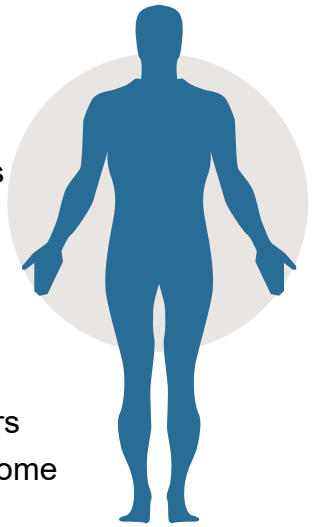
CLINICALLY MEANINGFUL IMPROVEMENT IN BONE DENSITY[3]



[1] Castells et al, AAAAI 2025; [2] Blueprint data on file; [3] Siebenhaar et al, AAAAI 2025

Multiple clinical data milestones anticipated in 2025 and beyond

UNIVERSE OF ALLERGIC & INFLAMMATORY DISEASES



Respiratory
- Allergic asthma
- Allergic rhinitis
- Allergic conjunctivitis
- Nasal polyps
- COPD

Skin
- Chronic urticaria
- Psoriasis
- Atopic dermatitis

Gastrointestinal
- Eosinophilic disorders
- Irritable bowel syndrome
- Food allergy

Multi-system
- Mast cell activation syndrome (MCAS)

PLUS OTHERS…

BLU-808 PROOF-OF-CONCEPT STRATEGY

Move rapidly into areas where targeting KIT has been de-risked

- Chronic spontaneous urticaria
- Chronic inducible urticaria

Explore other biology across organ systems to unlock broader potential

- Allergic asthma
- Allergic rhinitis
- Allergic conjunctivitis
- Mast cell activation syndrome (ISM adjacent)

COPD, chronic obstructive pulmonary disease.

Strong and durable financial profile entering 2025

Statement of Operations (unaudited)	Three Months Ended 12/31/2024	Three Months Ended 12/31/2023	FY Ended 12/31/2024	FY Ended 12/31/2023
Total revenue	$146.4M	$72.0M	$508.8M	$249.4M
Net product sales	$144.1M	$71.0M	$479.0M	$204.2M
Collaboration, license and other revenue	$2.2M	$1.0M	$29.9M	$45.2M
Cost of sales	$7.4M	$0.3M	$20.2M	$8.5M
Collaboration loss sharing	-	-	-	$4.3M
Research & development expense[1]	$83.7M	$97.5M	$341.4M	$427.7M
Selling, general & admin expense[2]	$96.5M	$79.3M	$359.3M	$295.1M
Other income (expense), net[3]	$(8.1)M	$(5.7)M	$146.2M	$(19.7)M
Net income (loss)	$(50.0)M	$(110.9)M	$(67.1)M	$(507.0)M
Balance Sheet (unaudited)			**12/31/2024**	**12/31/2023**
Cash, cash equivalents, and investments			$863.9M	$767.2M

1. Includes stock-based compensation expense of $11.7M and $10.0M in the three months ended 12/31/24 and 12/31/23, respectively, and $47.5M and $41.5M in the full year ended 12/31/24 and 12/31/23, respectively.
2. Includes stock-based compensation expense of $16.6M and $12.6M in the three months ended 12/31/24 and 12/31/23, respectively, and $61.4M and $51.1M in the full year ended 12/31/24 and 12/31/23, respectively.
3. Includes debt extinguishment gain of $173.7 million in the full year ended 12/31/24.
4. Values may not true up due to rounding.

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