

# **Travere Therapeutics Corporate Overview**

43rd Annual J.P. Morgan Healthcare Conference January 14, 2025



#### **Forward-Looking Statements**

This presentation contains forward-looking statements, including but not limited to statements about: continued progress with the FILSPARI launch; statements regarding our products and products in development as potential foundational treatments and/or treatment standards; additional development and regulatory milestones, including expected data from additional studies and the expected timing thereof; the Company's plans to provide an update on interactions with the FDA regarding establishing a potential regulatory pathway for sparsentan in FSGS and the anticipated timing and outcome thereof; the advancement of our pipeline throughout the year; expectations regarding the Phase 3 HARMONY Study and the other studies described herein, including expectations regarding process improvements and the potential timeline to restart enrollment; statements regarding the potential modification of liver monitoring for FILSPARI in IgAN; statements relating to the KDIGO guidelines; statements regarding potential future milestone and royalty payments; statements regarding potential changes to treatment paradigms; statements regarding estimates of potential addressable market sizes; and statements regarding financial metrics and expectations related thereto. These forward-looking statements may be accompanied by such words as "anticipate," "estimate," "expect," "forecast," "intend," "may," "plan," "project," "schedule," "target," "will," and other words and terms of similar meaning. You should not place undue reliance on these statements.

These statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements. Among the factors that could cause actual results to differ materially from those indicated in the forward-looking statements are risks and uncertainties associated with manufacturing processes and improvements, and risks related to the regulatory review and approval process, as well as risks and uncertainties associated with our business and finances in general, success of our commercial products, and risks and uncertainties associated with our preclinical and clinical stage pipeline. Specifically, we face risks associated with the challenges of manufacturing scale-up, the ongoing commercial launch of FILSPARI, market acceptance of our commercial products including efficacy, safety, price, reimbursement, and benefit over competing therapies, as well as risks associated with the successful development and execution of commercial strategies for such products, including FILSPARI. The risks and uncertainties we face with respect to our preclinical and clinical stage pipeline include risk that our clinical candidates will not be found to be safe or effective and that current or anticipated future clinical trials will not proceed as planned. Specifically, we face risks related to the timing and potential outcome of our Phase 3 HARMONY Study and the other studies described herein, and risks related to the outcome of the Company's interactions with FDA regarding establishing a potential regulatory pathway for sparsentan in FSGS. There is no guarantee that regulators will grant approval of sparsentan for FSGS. We also face the risk that we will not receive some or all of the potential future milestone and/or royalty payments described herein, the risk that our cash runway might not last as long as currently anticipated and the risk that we will be unable to raise additional funding that may be required to complete development of any or all of our product candidates, including as a result of macroeconomic conditions; risks relating to our dependence on contractors for clinical drug supply and commercial manufacturing; uncertainties relating to patent protection and exclusivity periods and intellectual property rights of third parties; risks associated with regulatory interactions; and risks and uncertainties relating to competitive products, including current and potential future generic competition with certain of our products, and technological changes that may limit demand for our products. We also face additional risks associated with global and macroeconomic conditions, including health epidemics and pandemics, including risks related to potential disruptions to clinical trials, commercialization activity, supply chain, and manufacturing operations, and the other risks and uncertainties that are described in the Risk Factors section of our most recent annual or quarterly report and in other reports we have filed with the SEC.

These statements are based on our current beliefs and expectations and speak only as of the date of this presentation. We do not undertake any obligation to publicly update any forward-looking statements.



### Travere Has a Vital Role in Rare Kidney and Metabolic Diseases



With **two future potential treatment standards** for rare kidney and metabolic disorders in global markets projected to exceed \$10B, we are **breaking down barriers** in treating diseases with historically little innovation

>\$10B Market Size >70k
addressable
IgAN patients
in the U.S.1

7k-10k addressable HCU patients globally\*

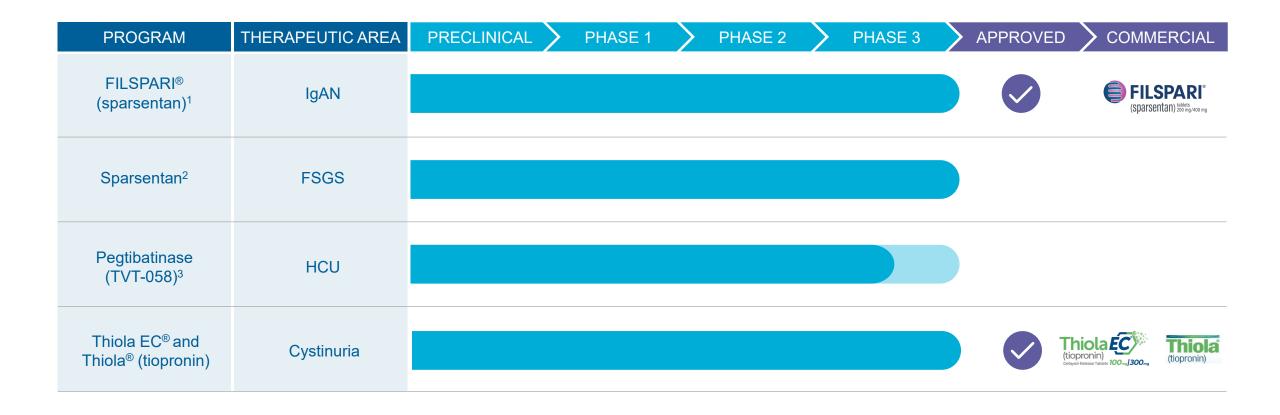
15k-30k addressable FSGS patients in the U.S.\* Through further clinical development and commercial execution, we will solidify our position as a leader in rare kidney and metabolic diseases

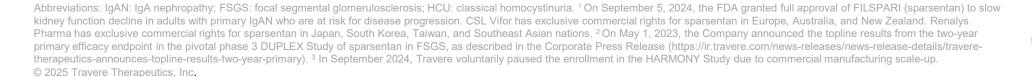


Continue diversifying our growth through **external innovation** and applying our expertise developing therapies through to successful commercialization



# Pipeline of Potential First-in-Class Programs Targeting Rare Kidney and Metabolic Diseases







# **Key 2025 Strategic Priorities and Milestones Driving Our Mission to Deliver Life-Changing Therapies to People Living with Rare Diseases**



## Solidify FILSPARI's placement as foundational care in IgAN

- Full approval with broader label expected to drive significant commercial growth
- Final publication of the updated KDIGO guidelines expected to drive earlier intervention, strengthen FILSPARI's position
- Potential modification of liver monitoring could ease access for certain patients – PDUFA target date of August 28, 2025



# Establish regulatory pathway for sparsentan in FSGS

- Following PARASOL scientific workshop, engaging with FDA to discuss potential regulatory pathway based on proteinuria
   update by 4Q24 earnings call
- If pathway is established, well-positioned to submit sNDA for FSGS indication
- Leverage IgAN commercial success to prepare for a potential launch in FSGS

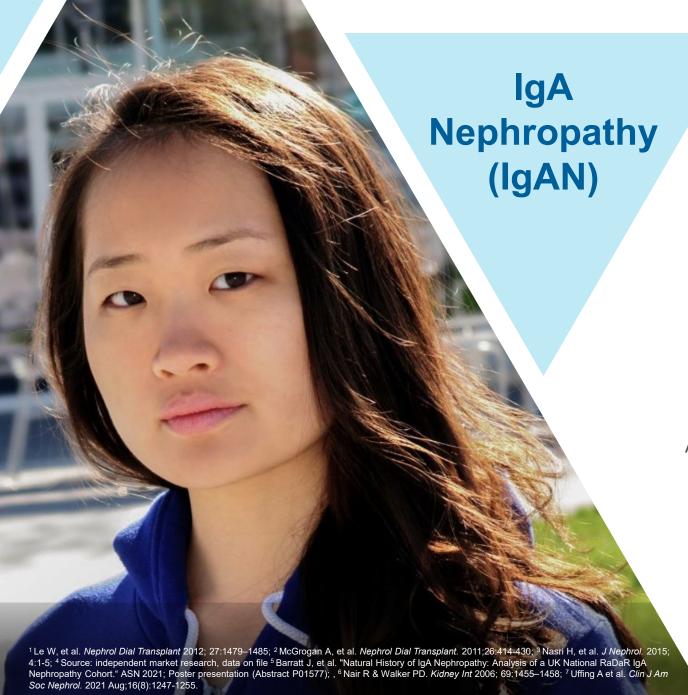


# Advance pegtibatinase development

- Only potentially disease-modifying treatment in clinical development for classical HCU
- Successfully implement process improvements in manufacturing scale up to restart enrollment in pivotal Phase 3 trial in 2026

Continued business development to further diversify pipeline





### is a Serious Unmet Rare **Kidney Disease (RKD)**

IgAN is the most prevalent primary glomerulonephritis worldwide<sup>1</sup>

Often uncontrolled, progressive IgAN is a major cause of kidney failure<sup>2,3</sup>

>70k

Addressable IgAN patients for FILSPARI in the U.S.4

~11 years

median time to kidney failure in high-risk adult patients<sup>5</sup>

**25-39** 

peak incidence age of IgAN<sup>6</sup>

30-40%

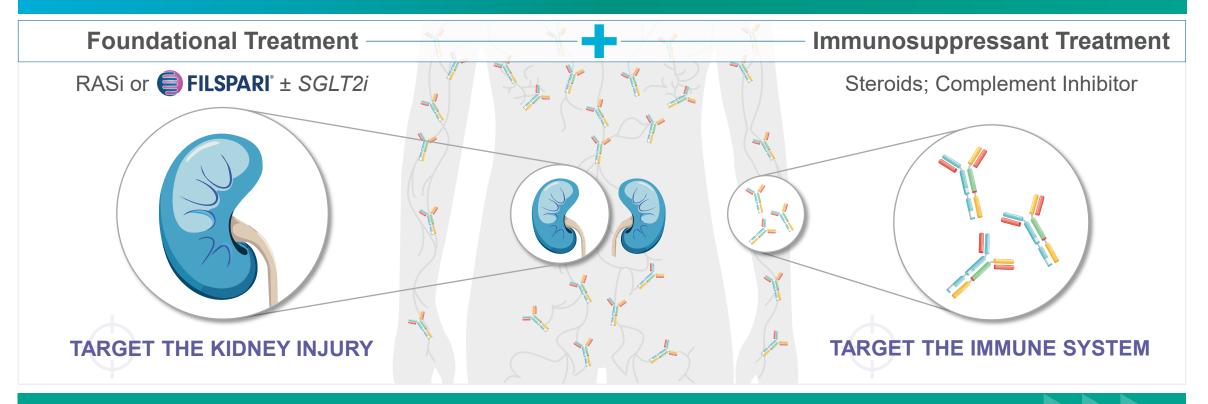
of transplants fail due to disease recurrence<sup>7</sup>



# The IgAN Treatment Paradigm: Two Areas to Target; Two Treatment Categories

#### **OVERACTIVATION IN THE KIDNEY**

#### OVERACTIVATION OF THE IMMUNE SYSTEM



FILSPARI is the only oral non-immunosuppressive, long-term treatment positioned as foundational in preserving kidney function in patients with IgAN\*



# FILSPARI Well-Positioned as a First-in-Class Foundational Treatment in IgAN with Best-in-Class Features

One pill, once daily administration that optimally inhibits the **two critical pathways** driving the progression of IgAN

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**Greatest magnitude of proteinuria reduction** in a Phase 3 study to-date: ~50%
reduction in UP/C at 36 weeks; ~40%
reduction at 2 years

**Two-year safety data** with no new safety signals, comparable to irbesartan





Only non-immunosuppressive treatment to-date to demonstrate statistically significant benefit on kidney function and accrual of benefit over two years



Flexibility for **combination use** in simultaneous treatment; clinical data support use in **newly diagnosed patients** with IgAN



# U.S. Commercial Launch Outperformed Benchmark Launches in First Full Year; Full Approval Strengthens Performance into 2025

### ~\$50M

Preliminary net FILSPARI sales in 4Q24; \$132M net FILSPARI sales in FY24



~40% growth vs 3Q24

693

New PSFs in 4Q24; 3,682 PSFs since launch



~37% growth vs 3Q24

96%

U.S. Patients with Pathway to Access



High compliance and persistence rates



Growth driven by increasing breadth and depth of prescribers, significant increase in new prescribers post full approval



FILSPARI is well established in payer plans and formularies, reflected in payer approval claims



<sup>\*</sup> Benchmark launches are other recent rare nephrology launches.

# **Key Growth Drivers Supporting Continued Execution of Commercial Launch**

Broader label allows for greater number of patients to benefit from FILSPARI

Draft KDIGO guidelines<sup>2</sup> to drive earlier intervention, strengthen FILSPARI's foundational positioning

Opportunity to broaden and deepen FILSPARI's prescriber base

Continue to engage payers to further strengthen coverage/access

Evolving treatment landscape and IgAN awareness to support further growth in addressable patient population



>70k

Addressable Patients with IgAN in the U.S.<sup>1</sup>



<sup>&</sup>lt;sup>1</sup> Source: independent market research, data on file.

<sup>&</sup>lt;sup>2</sup> KDIGO 2024 Clinical Practice Guideline for the Management of Immunoglobulin A Nephropathy (IgAN) and Immunoglobulin A Vasculitis (IgAV), public review draft, 8/30/2024. © 2025 Travere Therapeutics, Inc.

# Paving a Path to Global Access for FILSPARI in IgAN with Established Commercial Partners



>70k addressable IgAN patients<sup>1</sup>

**United States** 





#### **CSL Vifor**

EC granted conditional marketing authorization (CMA) for IgAN; FILSPARI launched in Germany, Austria, and Switzerland

CMA covers all 27 member states of the European Union, plus Iceland, Liechtenstein, and Norway<sup>2</sup>
Recent MHRA approval in UK



Results from registration enabling study for Japan expected in 2H25

License to Renalys covers Japan, South Korea, Taiwan, and Southeast Asian nations





Travere eligible to receive up to \$910 million in potential milestone payments<sup>3</sup> + tiered double-digit royalties on global net sales of FILSPARI

Abbreviations: EC: European Commission, CMA: conditional marketing authorization.

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<sup>&</sup>lt;sup>1</sup> Source: independent market research, data on file.

<sup>&</sup>lt;sup>2</sup> License to CSL Vifor also covers other territories including the United Kingdom, Switzerland, Australia, and New Zealand, with potential to expand. <sup>3</sup> Potential milestone payments include achievements for both IgAN and FSGS indications.



### is a Serious Unmet Rare Kidney Disease (RKD)

A histopathological lesion triggered by podocyte injury and a leading cause of kidney failure worldwide

Severity of proteinuria at onset and during follow up is associated with renal failure

#### 15k-30k

Potential addressable FSGS patients in the U.S.<sup>1</sup>

### ~5-10 years

Median time to kidney failure for 30-60% of patients<sup>2</sup>

#### 0

Approved treatments indicated for this condition

#### 40%

of transplant patients experience disease recurrence<sup>2</sup>



#### **PARASOL** Project: Key Takeaways



FSGS is an important cause of kidney failure in patients of all ages and new therapies are urgently needed to reduce the risk of progression.



Discussion of the findings in an open forum highlighted their broad utility, the **biological role of proteinuria in FSGS** as a podocytopathy, and implications for clinical trial design.



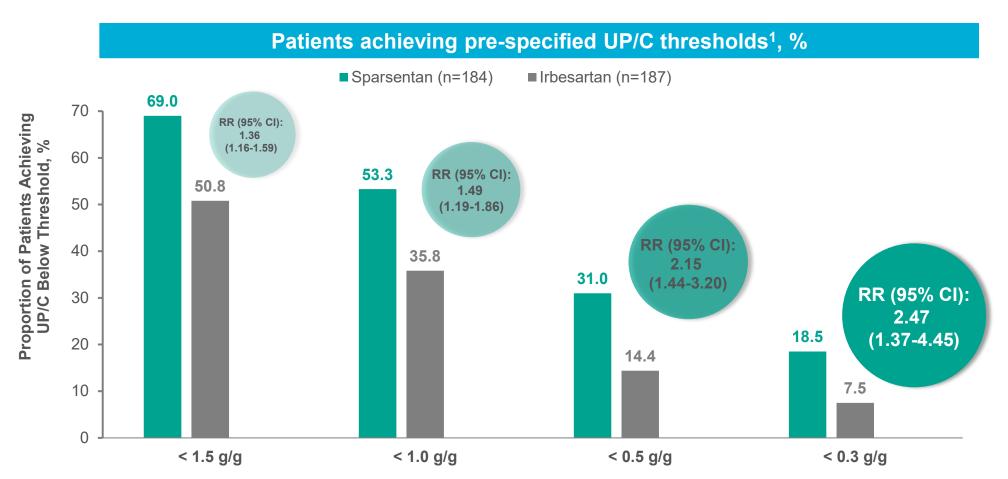
A multi-stakeholder group of rare kidney disease experts aligned around a **potential proteinuria-based clinical trial endpoint**, balancing biological relevance and trial design considerations.

The principal finding is that **reduction in proteinuria** over 24 months is **strongly associated with a reduction in the risk of kidney failure**, and responder definitions based on thresholds of proteinuria are both biologically plausible and strongly supported by epidemiological data.<sup>1</sup>

Abigail Smith, PhD, Northwestern University Feinberg School of Medicine – PARASOL

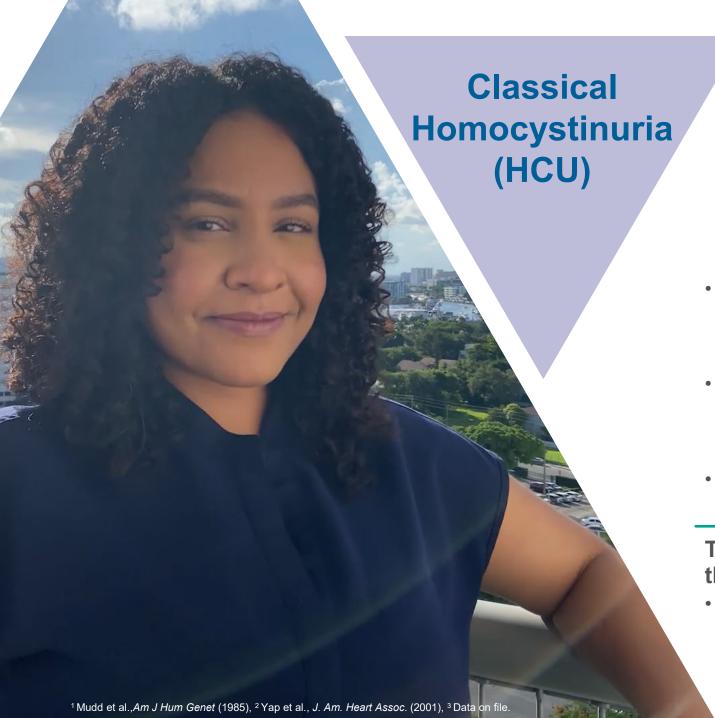


# **Sparsentan Demonstrated Significantly Greater Proteinuria Reduction vs Active Comparator Across Measurement Thresholds**









#### is a Rare Autosomal Recessive Metabolic Disorder that can Lead to Life-Threatening Complications

- Caused by mutations in cystathionine beta-synthase (CBS) gene, leading to deficient activity of CBS, which can result in bodily buildup of toxic homocysteine (Hcy).
- Continuous risk of developing life-threatening thrombotic events, including heart attack and stroke, observed in 25% of HCU patients by age 16 and 50% by age 29.<sup>1,2</sup>
- Estimates suggest at least 12,000 patients living with HCU in U.S.; similar number in Europe.<sup>3</sup>

# There are no approved treatments that address the underlying genetic cause of HCU

 Current standard of care includes vitamin B6, low-protein diet, and supplements, as well as betaine.



# Treatment with Pegtibatinase in the Phase 1/2 COMPOSE Study Showed Rapid and Sustained tHcy Reduction Through 12 Weeks of Treatment



**67.1%** mean relative reduction in total homocysteine from baseline



All patients in highest dose cohort achieved a clinically meaningful threshold in mean tHcy over weeks 6 to 12 of treatment

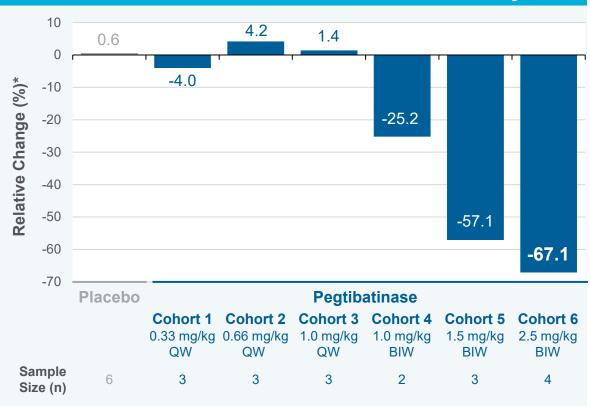


Methionine and cystathionine biomarkers suggest that pegtibatinase acts similar to the native CBS enzyme and can restore the metabolic dysregulation in patients with HCU



Pegtibatinase was generally well-tolerated at all doses tested





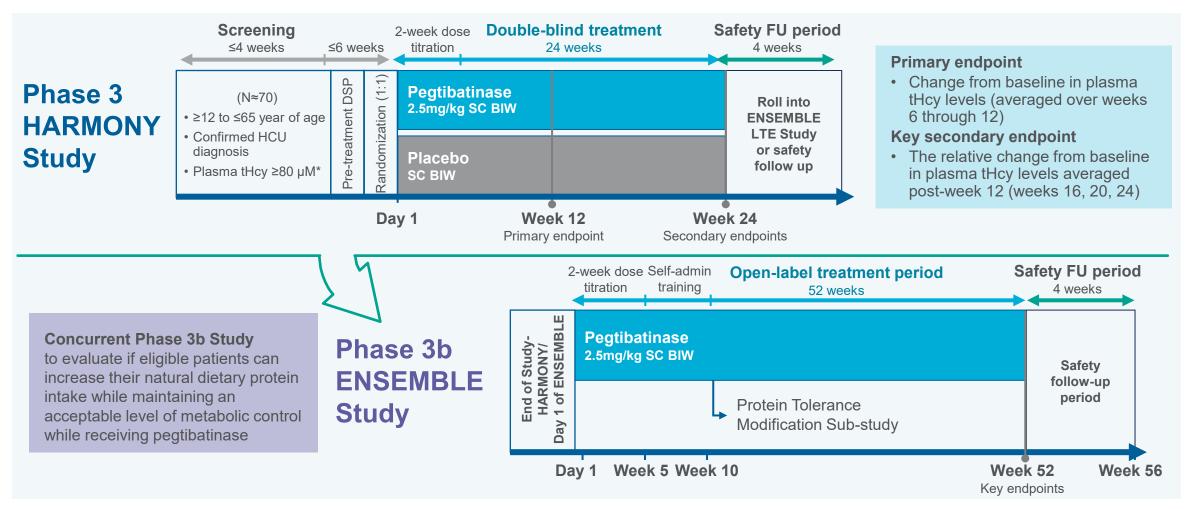
Abbreviations: QW: once weekly, BIW: twice weekly.

TRAVERE®
THERAPEUTICS

<sup>\*</sup> The data referenced in the table above and the analysis conducted in cohort 6 assess the relative reduction in tHcy from baseline in the geometric mean by averaging tHcy over weeks 6, 8, 10, and 12. This measure improves the precision and reliability of assessment of the treatment effect and takes into account that there is some variability in tHcy depending on food intake and diurnal variation. The Company intends to use this measure moving forward.

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#### **Innovative Pegtibatinase Phase 3 Program**



Abbreviations: BIW: twice weekly, DSP: diet standardization period, LTE: long-term (open-label) extension, SC: subcutaneous, tHcy: total homocysteine, FU: follow up.



<sup>\*</sup> Protocol allows for ~25% of patients with tHcy ≥50 to <80µM.

<sup>\*\*</sup> ClinicalTrials.gov ID: NCT06247085.

<sup>\*\*\*</sup> In September 2024, Travere voluntarily paused the enrollment in the HARMONY Study due to delays in commercial manufacturing scale-up. © 2025 Travere Therapeutics, Inc.

# Financial Snapshot – Strong Operational Execution and Balance Sheet to Support Sustainable Growth



~78%

growth in net product sales over 2023; preliminary FY 2024 net product sales ~\$227M<sup>1</sup>



~77M

basic shares
outstanding for nine
months ended
September 30, 2024;
diluted ~92mm<sup>2</sup>



~\$371M

in cash and cash equivalents as of 12/31<sup>1</sup>



~\$69M

in convertible notes due Sept 2025; \$316M due March 2029

<sup>&</sup>lt;sup>1</sup> Based on preliminary and unaudited financial data for period ending 12/31/24.

<sup>&</sup>lt;sup>2</sup> Weighted average share count. Diluted share count calculation includes all outstanding equity awards but excludes convertible notes.



