

Travere Therapeutics Corporate Overview

January 2025



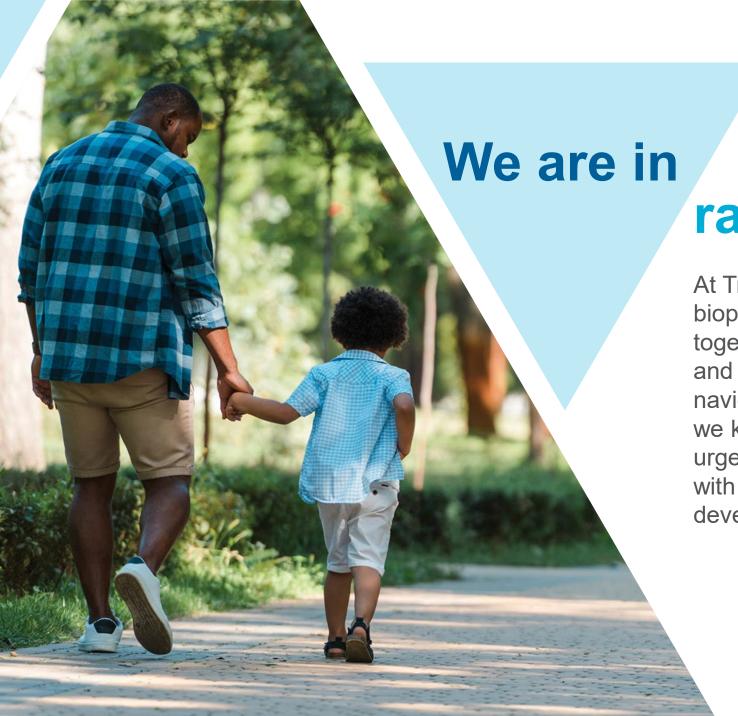
Forward-Looking Statements

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.





rare for life.

At Travere Therapeutics, we are a biopharmaceutical company that comes together every day to help patients, families, and caregivers of all backgrounds as they navigate life with a rare disease. On this path, we know the need for treatment options is urgent — that is why our global team works with the rare disease community to identify, develop, and deliver life-changing therapies.

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

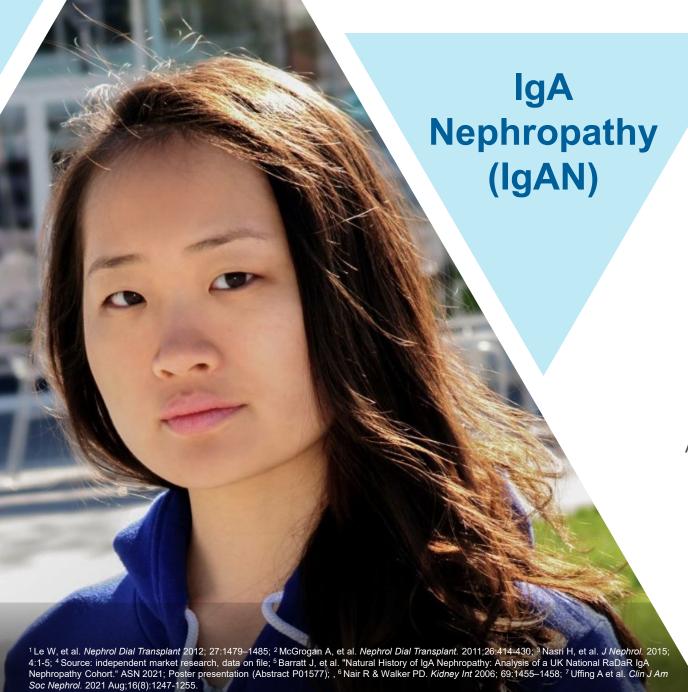




FILSPARI® (sparsentan)

First and only endothelin and angiotensin II receptor antagonist for rare kidney disorders





is a Serious Unmet Rare Kidney Disease (RKD)

IgAN is the most prevalent primary glomerulonephritis worldwide¹

Often uncontrolled, progressive IgAN is a major cause of kidney failure^{2,3}

>70k

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

~11 years

median time to kidney failure in high-risk adult patients⁵

25-39

peak incidence age of IgAN⁶

30-40%

of transplants fail due to disease recurrence⁷



Draft KDIGO Guidelines: The IgAN Treatment Paradigm is Evolving

Earlier Treatment, Lower Proteinuria Targets and Simultaneous Therapy



Earlier diagnosis



At risk of progressive loss of kidney function requiring treatment



Treatment goal



Kidney biopsy in all adults with proteinuria ≥0.5g/d¹



At risk if proteinuria ≥0.5g/d while on or off treatment

Combination treatment should be started simultaneously in all cases



Proteinuria should be maintained at <0.5g/d, or preferably <0.3g/d

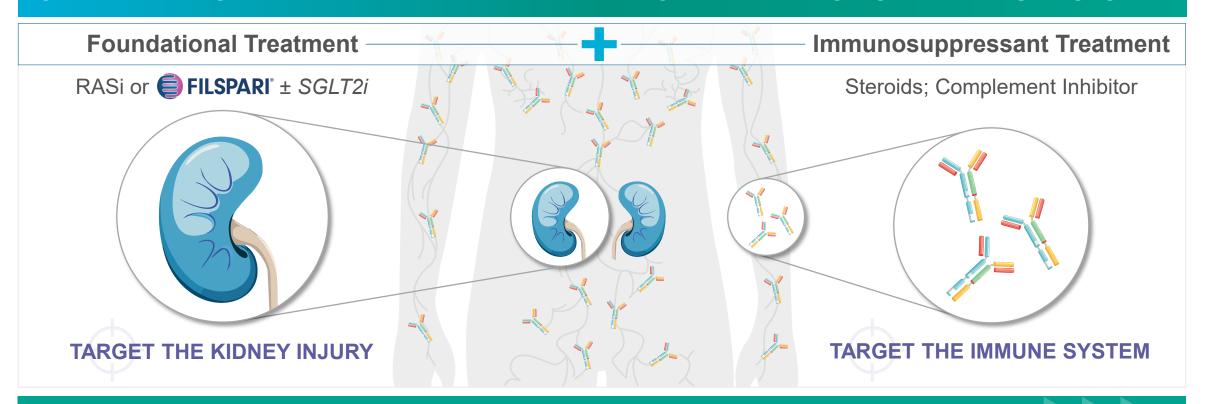
Proteinuria is the only validated early biomarker to help guide clinical decision-making



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*



The Only Non-Immunosuppressive Treatment Proven to Significantly Slow Kidney Function Decline in IgA Nephropathy



Overview of Prescribing Information

Indication Statement

FILSPARI is indicated to **slow kidney function decline** in adults with primary IgAN who are at
risk for disease progression

Dosing and Administration Tablets: 200mg and 400mg, for once-a-day oral dose

Most Common
Adverse Reactions
(≥5%)

Hyperkalemia, hypotension (including orthostatic hypotension), peripheral edema, dizziness, anemia, and acute kidney injury

For full prescribing information including boxed warning, visit filspari.com



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



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



as foundational treatment in IgAN



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



Two-year safety data with no new

safety signals, comparable to irbesartan

IgAN Induced Nephron Loss is Driven by Two Critical Pathways - Endothelin-1 (ET-1) and Angiotensin II (ANG-II)¹⁻³

Galactose-deficient, IgA-containing immune complexes are deposited in the mesangium⁴ ET-1 levels ET-1 and ANG-II act Causes Rapid ET-1 and together to damage proteinuria decline in Progression **ANG-II** the glomerular to kidney kidney to rise to mutually filtration barrier and function failure⁸ detrimental upregulate tubulointerstitium^{1,2} levels1,2 (eGFR)⁵⁻⁸ one another² ANG-II levels **FILSPARI Preserves** Blocks actions of Reduces **Dual Endothelin Angiotensin** kidnev **ET-I and ANG-II** proteinuria Receptor Antagonist function

Abbreviations: Ang II: angiotensin II, ET-1: endothelin-1, IgAN: immunoglobulin A nephropathy, eGFR: estimated glomerular filtration rate. Figure adapted from Lai K, et al. Nat Rev Dis Primers. 2016;16001.

¹Komers R, et al. Am J Physiol Regul Integr Comp Physiol. 2016;310(10):R877-R884. ²Kohan DE, et al. Kidney Int. 2014;86(5):896-904. ³ Raina R, et al. Kidney Dis. 2020;6(1):22-34. ⁴ Ebefors K, Bergwall L, Nyström J. Front Med (Lausanne). 2022;8:740527. doi:10.3389/fmed.2021.740527. ⁵ Zoja C, Morigi M, Figliuzzi M, et al. Am J Kidney Dis. 1995;26(6):934-941. ⁶ Morigi M, Buelli S, Angioletti S, et al. Am J Pathol. 2005;166(5):1309-1320. ⁷ Tejera N, Gómez-Garre D, Lázaro A, et al. Am J Pathol. 2004;164(5):1817-1826. ⁸ Lai K, et al. Nat Rev Dis Primers. 2016;2:160001. © 2025 Travere Therapeutics, Inc.



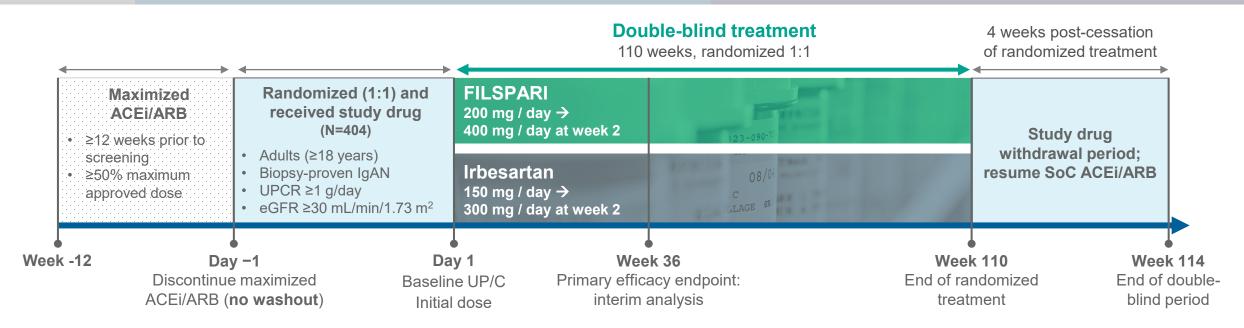
The Only Head-to-Head, Active-Controlled Trial in IgAN to Date: Phase 3 PROTECT Study



Test the efficacy and safety of FILSPARI vs. active control (irbesartan) in a global, multicenter, double-blind, randomized study of 404 patients with IgAN, ages 18+



- Primary efficacy endpoint: change in UPCR from baseline to week 36
- Key secondary efficacy endpoint: eGFR slope: total (day 1 - week 110) and chronic (week 6 - 110)



Abbreviations: UPCR: urine protein/creatinine ratio, g/day: grams per day, eGFR: estimated glomerular filtration rate, ACEs: angiotensin converting enzyme inhibitors, ARBs: angiotensin receptor blockers, SoC: standard of care.



^{*} ClinicalTrials.gov ID: NCT03762850. © 2025 Travere Therapeutics, Inc.

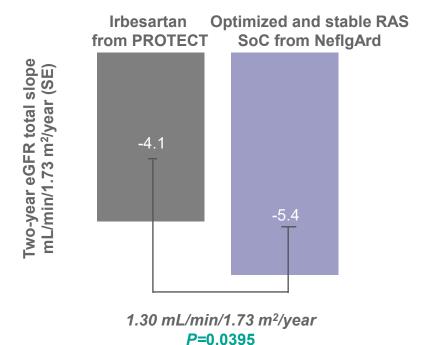
Active Control is Not Placebo: Matching-Adjusted Indirect Comparisons Show Irbesartan Significantly Outperformed Standard of Care in Other Studies

Rate of kidney function decline: maximally dosed irbesartan vs standard of care in real-world setting

> 1.12 mL/min/1.73 m²/year P=0.0239

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Rate of kidney function decline: maximally dosed irbesartan vs standard of care in clinical trial setting



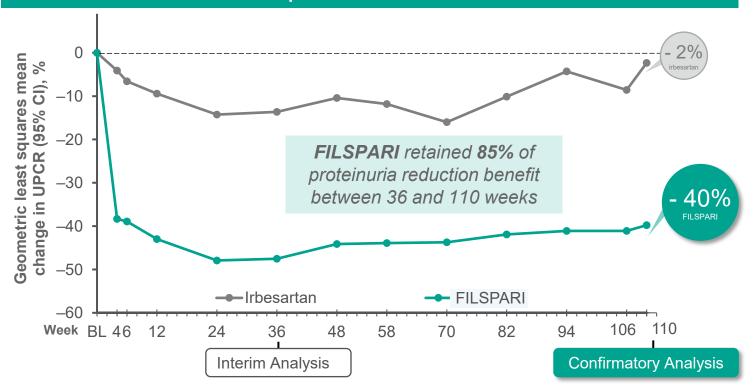
Maximally tolerated irbesartan was associated with slower decline in kidney function vs real-world SoC treatment in RaDaR and physician defined, optimized SoC in NeflgArd*



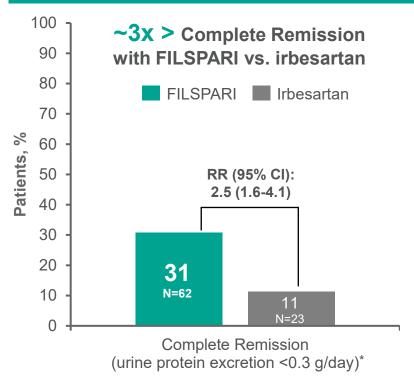
FILSPARI Showed Superior Proteinuria Reduction in a Phase 3 Study vs. Active Control, Sustained Over Two Years

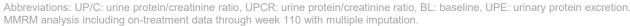
FILSPARI demonstrated a statistically significant reduction in proteinuria of ~40% after 110 weeks of treatment

FILSPARI showed 20x better proteinuria reduction vs irbesartan at Week 110



Complete Remission UPE<0.3g/day





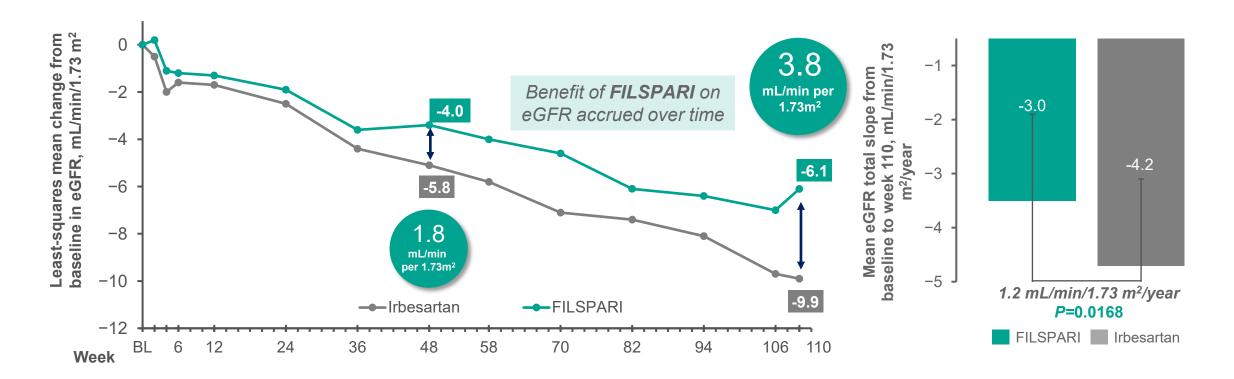
^{*} Achieved complete remission at any time while on study medication during the double-blind period. © 2025 Travere Therapeutics, Inc.



FILSPARI Demonstrated Significant Long-Term Kidney Function Preservation in IgAN Patients

Long-term FILSPARI treatment showed significant preservation of kidney function that accrued over time

Annual rate of decline in kidney function from baseline to Week 110



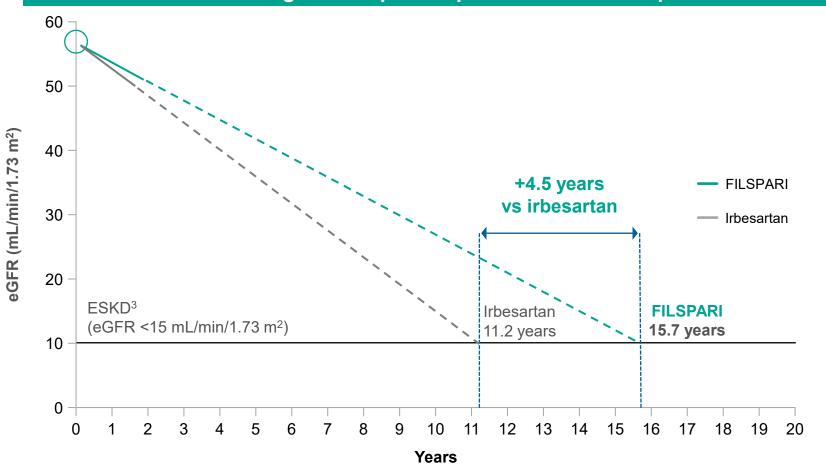


^{*} The analysis includes eGFR data during the double-blind period up to Week 110 regardless of treatment discontinuation or immunosuppressive therapy initiation.

^{**} LS Means and 95% CI from a random coefficient analysis including available on-treatment eGFR data through week 110 with multiple imputation; mL/min/1.73m² per year. © 2025 Travere Therapeutics, Inc.

Treatment with FILSPARI May Potentially Delay Dialysis or Transplant

Potential long-term impact of preserved eGFR slope^{1,2}



Based on extrapolation of eGFR slope data from PROTECT, FILSPARI may potentially delay dialysis or transplant by 4.5 years

when compared to maximum-labeled dose irbesartan¹⁻³

Abbreviations: eGFR: estimated glomerular filtration rate, ESKD: end-stage kidney disease.



¹ FILSPARI Prescribing Information. San Diego, CA: Travere Therapeutics, Inc.

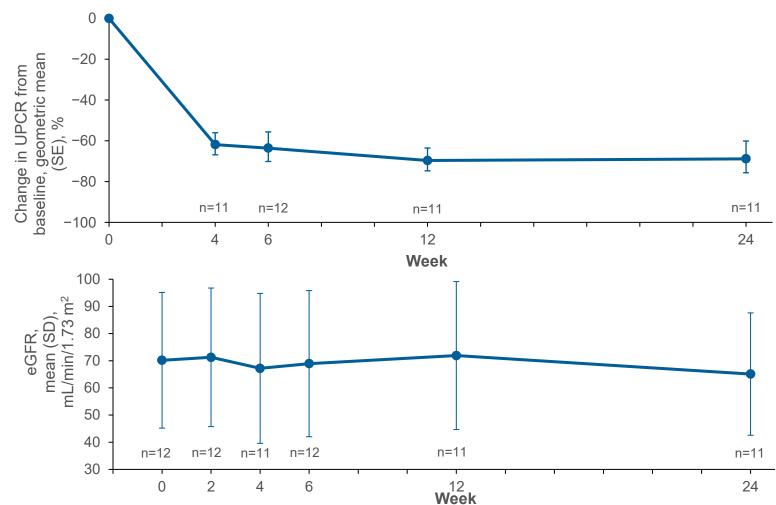
² Data on file, Travere Therapeutics, Inc.

³ United States Renal Data System. 2023 USRDS Annual Data Report: Epidemiology of kidney disease in the United States. NIH, NIDDK, Bethesda, MD, 2023. © 2025 Travere Therapeutics, Inc.

SPARTAN Study Shows Rapid and Sustained Impact of FILSPARI as First-Line Treatment in Newly Diagnosed Patients

Preliminary clinical findings at 24-weeks in treatmentnaïve patients on FILSPARI

- ➤ Sparsentan, led to rapid and sustained reductions in proteinuria (~70% from baseline at week 24)
- within 24 weeks of starting sparsentan, ~60% of patients achieved complete remission of proteinuria, a treatment goal recommended in the draft 2024 KDIGO guidelines¹
- Sparsentan was generally well tolerated over 24 weeks of treatment, with no evidence of fluid retention. Safety was consistent with the Phase 3 PROTECT Study^{2,3}



Abbreviations: UPCR: urine protein-to-creatine ratio, eGFR: estimated glomerular filtration rate. Source: Cheung CK, et al. presented at ASN 2024: October 23-27, 2024: San Diego, CA, FR-OR63.

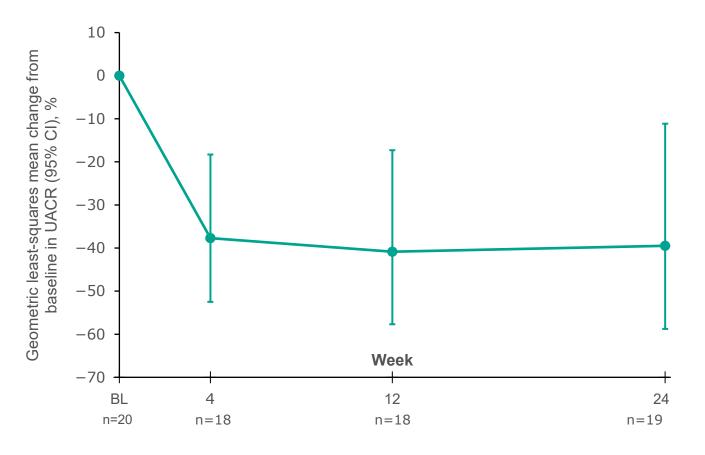


¹ KDIGO 2024 Clinical Practice Guideline for the Management of Immunoglobulin A Nephropathy (IgAN) and Immunoglobulin A Vasculitis (IgAV), public review draft, 8/30/2024.

² Heerspink HJL, et al. Lancet. 2023;401(10388):1584-1594.

³ Rovin BH, et al. Lancet. 2023;402(10417):2077-2090.

SPARTACUS Study: FILSPARI Added to SGLT2i Resulted in Further Proteinuria Reduction and Was Generally Well Tolerated



TEAEs*	Patients (N=20)
Any TEAE, n (%)	12 (60)
Any TEAEs in >1 patient, n (%)	
Dizziness	2 (10)
Headache	2 (10)
Hypertension	2 (10)
Hypotension	2 (10)
Edema	2 (10)
Peripheral edema	2 (10)
Osteoarthritis	2 (10)
Any severe TEAE, n (%)	1 (5)
Gout	1 (5)
Any serious AE, n (%)	2 (10)
Acute kidney injury**	1 (5)
Cerebrovascular accident	1 (5)
Osteoarthritis**	1 (5)

After replacing RASi with FILSPARI, patients experienced a mean reduction in UACR of ~40% at 24 weeks

Abbreviations: UACR: urine albumin-to-creatinine ratio, SGLT2i: soldium-glucose cotransporter-2 inhibitor, TEAE: treatment-emergent adverse event, AE: adverse event. Ayoub I., et al. presented at ASN 2024, October 23-27, 2024; San Diego, CA. Poster FR-PO849.



^{*} TEAEs were based on MedDRA preferred terms.

^{**} Reported in the same patient.
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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



^{*} 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 guidelines² 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.¹

TRAVERE THERAPEUTICS

¹ Source: independent market research, data on file.

² 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 patients¹

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 Norway² 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 payments³ + tiered double-digit royalties on global net sales of FILSPARI

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

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¹ Source: independent market research, data on file.

² License to CSL Vifor also covers other territories including the United Kingdom, Switzerland, Australia, and New Zealand, with potential to expand. ³ 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.¹

~5-10 years

Median time to kidney failure for 30-60% of patients²

0

Approved treatments indicated for this condition

40%

of transplant patients experience disease recurrence²



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.¹

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



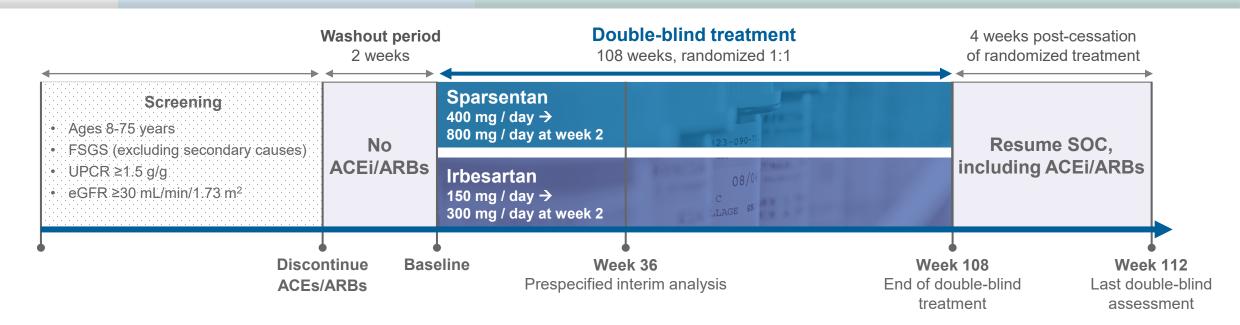
The DUPLEX Study of Sparsentan is the Largest Active-Controlled Interventional Phase 3 Trial in FSGS to Date



Evaluate the efficacy and safety of sparsentan vs. the active control irbesartan in patients with focal segmental glomerulosclerosis (FSGS)



- Phase 3, double-blind, active-controlled global trial in patients with biopsy-proven FSGS or genetic FSGS, N=371 patients (ages 8 to 75 years)*
- The only head-to-head Phase 3 study of its kind in FSGS
- Surrogate efficacy endpoint: (36-week interim analysis) = proportion of patients achieving FPRE at week 36 (UPCR ≤ 1.5 g/g and ≥ 40% reduction from baseline)
- Primary endpoint: eGFR total slope: From day 1 to week 108 of treatment (U.S. primary), eGFR chronic slope: From week 6 to week 108 of treatment (EU primary)

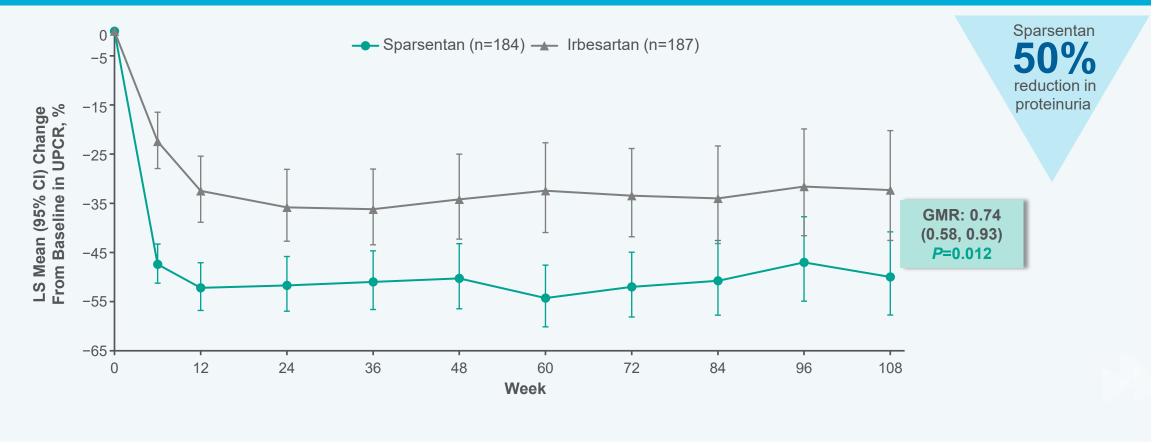


Abbreviations: ACEi: angiotensin converting enzyme inhibitors, ARBs: angiotensin receptor blockers, UPCR: urine protein/creatinine ratio, g/g: grams per gram, eGFR: estimated glomerular filtration rate, FPRE: FSGS partial remission endpoint, SOC: standard of care.



Results from the Phase 3 DUPLEX Study of Sparsentan in FSGS – Rapid Decline in Proteinuria Sustained Through 108 Weeks

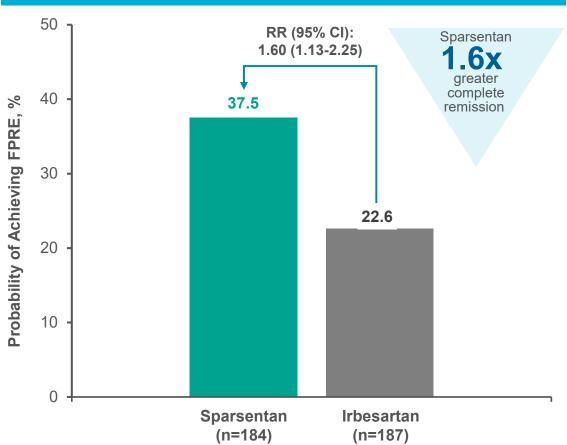
Sparsentan resulted in a rapid decline in UPCR that was sustained through the duration of the trial



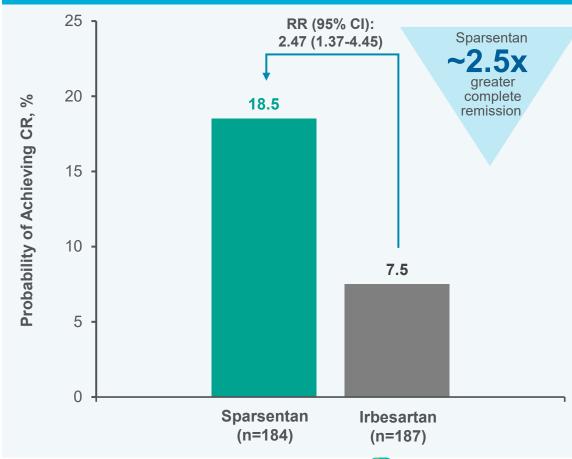


Sparsentan Demonstrated Significantly Higher Probability of Achieving Partial and Complete Remission at Week 108

Patients achieving FPRE at any time during the double-blind period

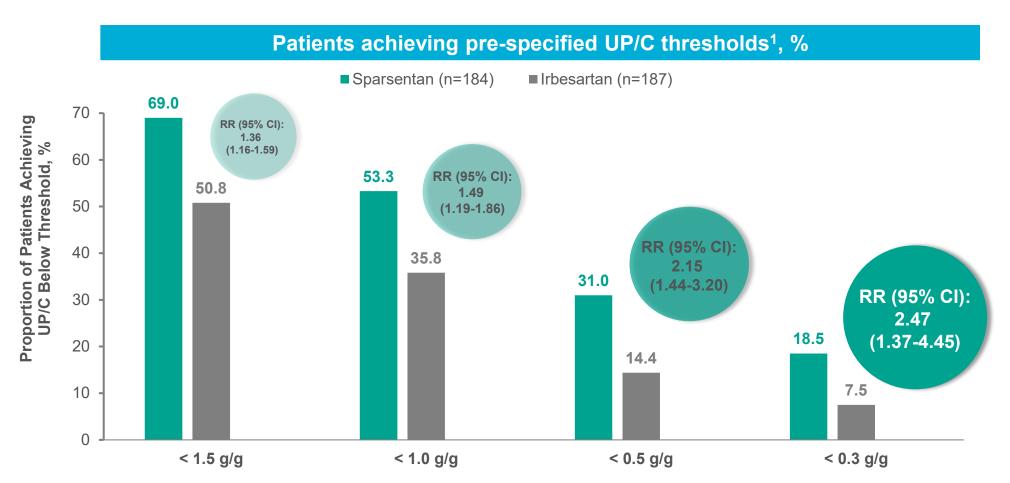


Patients achieving CR at any time during the double-blind period





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





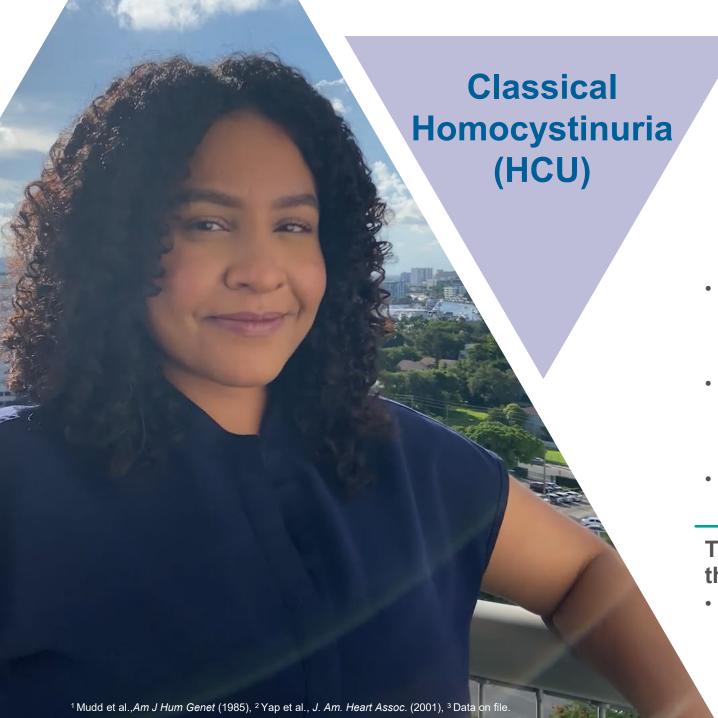




Pegtibatinase

The Only Potential Disease Modifying Therapy for Classical Homocystinuria (HCU)





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.^{1,2}
- Estimates suggest 7,000 to 10,000 patients living with HCU in U.S.; similar number in Europe.³

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.



The HCU Market is Expected to Grow with Better Diagnostics, Awareness and Effective Treatment Options

Disease education/awareness, enhanced diagnostics and better treatment options are expected to lead to **increased patient identification**, **earlier diagnosis**, **and better outcomes** - driving growth in addressable market



Diagnosed prevalence rates are highest in U.S., EU, and Middle East

HCU patients actively managed by an HCP in the U.S are expected to increase

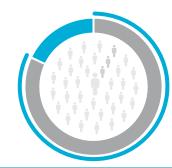


At-launch Future

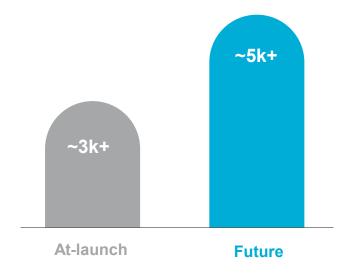


Despite newborn screening for HCU in the U.S., it is estimated that fewer than 50% of people with HCU are diagnosed at birth¹

Today, ~80% of HCU patients are partially or non-responsive to B6 therapy (current standard of care)²



Expected growth in addressable HCU patients in U.S.



Pegtibatinase has the potential to become the **only disease-modifying therapy** in a market with significant growth expected.



Pegtibatinase is an Investigational, Modified, Recombinant CBS Human Enzyme Therapy

Pegtibatinase is designed to address the underlying genetic cause of HCU



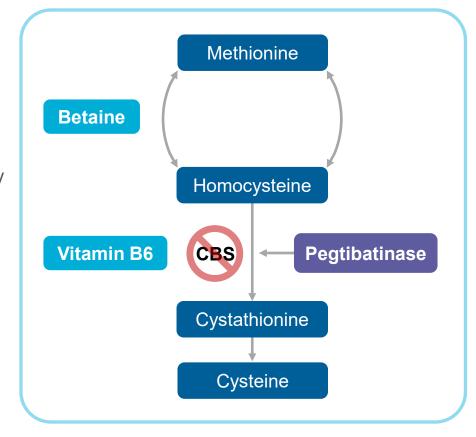
Mechanism of action is expected to have broad effect across HCU population



Administered subcutaneously and designed to be active and stable in plasma, unlike native CBS



Designed to introduce the CBS enzyme into circulation and reduce intracellular and plasma Hcy levels



Pegtibatinase has been granted multiple regulatory designations for the treatment of classical HCU

- FDA Breakthrough
 Therapy designation
- FDA Rare Pediatric
 Disease designation
- FDA Fast Track designation
- Orphan Drug designation in the U.S. and Europe



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

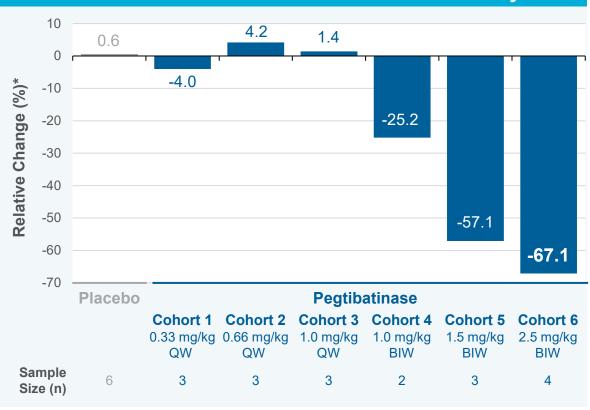


Methionine and cystathionine biomarkers suggest that pegtibatinase acts similar to the native CBS enzyme and can restore the metabolic dysregulation in patients with HCU



Pegtibatinase was generally well-tolerated at all doses tested





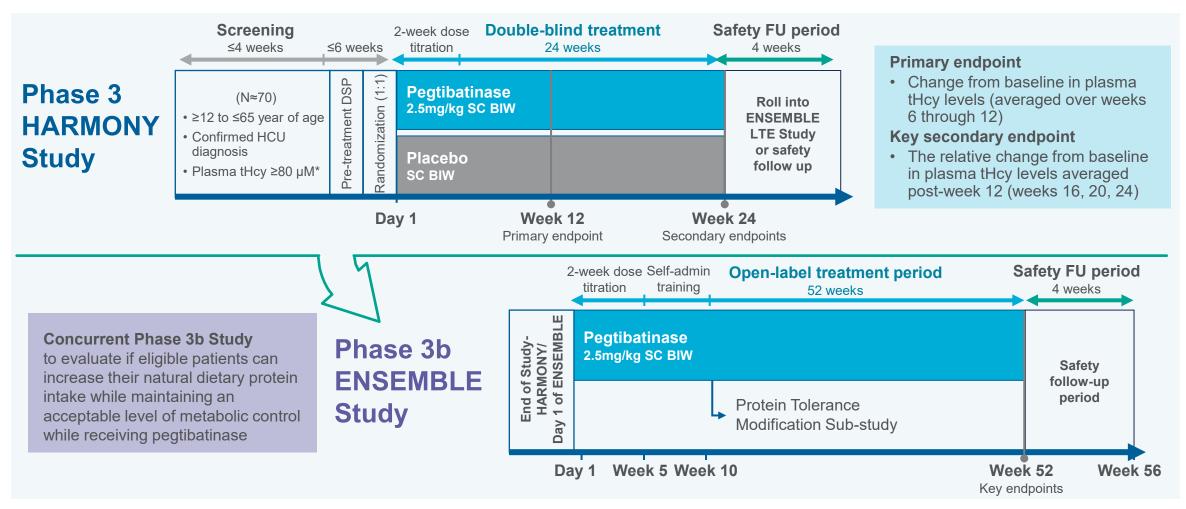
Abbreviations: QW: once weekly, BIW: twice weekly.

^{*} 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.



^{*} Protocol allows for ~25% of patients with tHcy ≥50 to <80μM.

^{**} ClinicalTrials.gov ID: NCT06247085.

^{***} In September 2024, Travere voluntarily paused the enrollment in the HARMONY Study due to delays in commercial manufacturing scale-up. © 2025 Travere Therapeutics, Inc.

Pegtibatinase Offers A Promising Approach to Address the Unmet Need in Patients with Classical Homocystinuria

Our goal is to deliver pegtibatinase as the first disease-modifying treatment for patients living with HCU

Clinical Conclusions from COMPOSE Study



A ~67% post-treatment relative change from baseline of plasma tHcy levels was achieved at the highest dose of pegtibatinase; reductions were evident from week 2 and sustained throughout the 12-week study period.



All participants in cohorts 5 and 6 achieved mean post-treatment tHcy levels below the key clinical threshold of 100 μM; tHcy reductions below 50 μM were observed, including one patient with a lower tHcy level at baseline that achieved normalization (<15 μM) of tHcy.



Pegtibatinase was generally well-tolerated at all doses tested; no reports of anaphylaxis or severe immune reactions due to pegtibatinase or discontinuations associated with the study drug.

Milestones/ Next Steps



The Company successfully completed its end of Phase 2 meeting with the FDA.



In December 2023, the pivotal HARMONY Study was initiated to support potential regulatory submissions.



The Company is making progress on necessary process improvements in manufacturing scale-up and is on track to restart enrollment in the Phase 3 HARMONY Study in 2026.



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 ~\$227M¹



~77M

basic shares
outstanding for nine
months ended
September 30, 2024;
diluted ~92mm²



~\$371M

in cash and cash equivalents as of 12/31¹



~\$69M

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

¹Based on preliminary and unaudited financial data for period ending 12/31/24.

² Weighted average share count. Diluted share count calculation includes all outstanding equity awards but excludes convertible notes.

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





