



TRAVERE[®]
THERAPEUTICS

Traverse Therapeutics Corporate Overview

August 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; plans and expectations regarding our sNDA for traditional approval of FILSPARI in FSGS, expectations regarding the timing and outcome thereof, and statements regarding preparations for a successful launch in FSGS, if approved; 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 and removal of embryo-fetal toxicity monitoring REMS 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,” “believe,” “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 related to our sNDA for FILSPARI in FSGS, including the timing and outcome thereof. There is no guarantee that the FDA will grant approval of FILSPARI for FSGS on the anticipated timeline, or at all. We also face risks related to our business and finances in general, the success of our commercial products, risks and uncertainties associated with our preclinical and clinical stage pipeline, risks and uncertainties associated with the regulatory review and approval process, risks and uncertainties associated with enrollment of clinical trials for rare diseases, and risks that ongoing or planned clinical trials may not succeed or may be delayed for safety, regulatory or other reasons. Specifically, we face risks associated with the ongoing commercial launch of FILSPARI in IgAN, the timing and potential outcome of our and our partners’ clinical studies, market acceptance of our commercial products including efficacy, safety, price, reimbursement, and benefit over competing therapies, risks related to the challenges of manufacturing scale-up, risks associated with the successful development and execution of commercial strategies for such products, including FILSPARI, and risks and uncertainties related to the new administration, including but not limited to risks and uncertainties related to tariffs and the funding, staffing and prioritization of resources at government agencies including the FDA. 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.

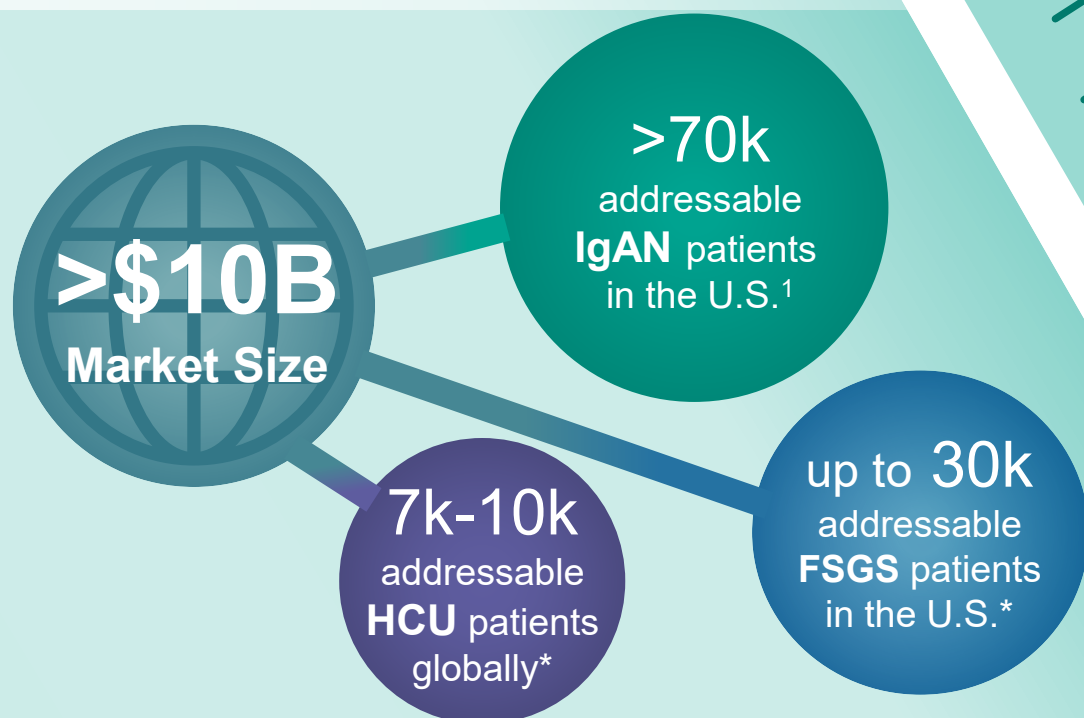
These statements are based on our current beliefs and expectations and speak only as of the date of this presentation. We do not undertake any obligation to publicly update any forward-looking statements.



We are in rare for life.

At Traverre 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.

Traverse Has a Vital Role in Rare Kidney and Metabolic Diseases



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



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

¹ For FILSPARI. Source: independent market research, data on file.

* If approved.

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

PROGRAM	THERAPEUTIC AREA	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3	APPROVED	COMMERCIAL
FILSPARI® (sparsentan) ¹	IgAN					✓	
Sparsentan ²	FSGS						
Pegtibatinase (TVT-058) ³	HCU						
Thiola EC® and Thiola® (tiopronin)	Cystinuria					✓	 

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

- The only fully approved kidney-targeted therapy positioned to replace the historical standard of care in IgAN
- Final publication of the updated KDIGO guidelines expected to drive earlier intervention, strengthen FILSPARI's position
- Potential modification of liver monitoring and removal of pregnancy monitoring could ease access for certain patients – PDUFA target date of August 28, 2025



Position FILSPARI for a potential FDA approval and launch in FSGS

- PDUFA target action date of January 13, 2026
- FILSPARI could become the only FDA-approved medicine indicated for FSGS
- Leverage IgAN commercial success to prepare for commercial launch in FSGS, if approved



Advance pegtibatinase development

- Potential to become the only disease-modifying treatment for classical HCU
- Successfully optimize 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



IgA Nephropathy (IgAN)

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 patients with IgAN 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

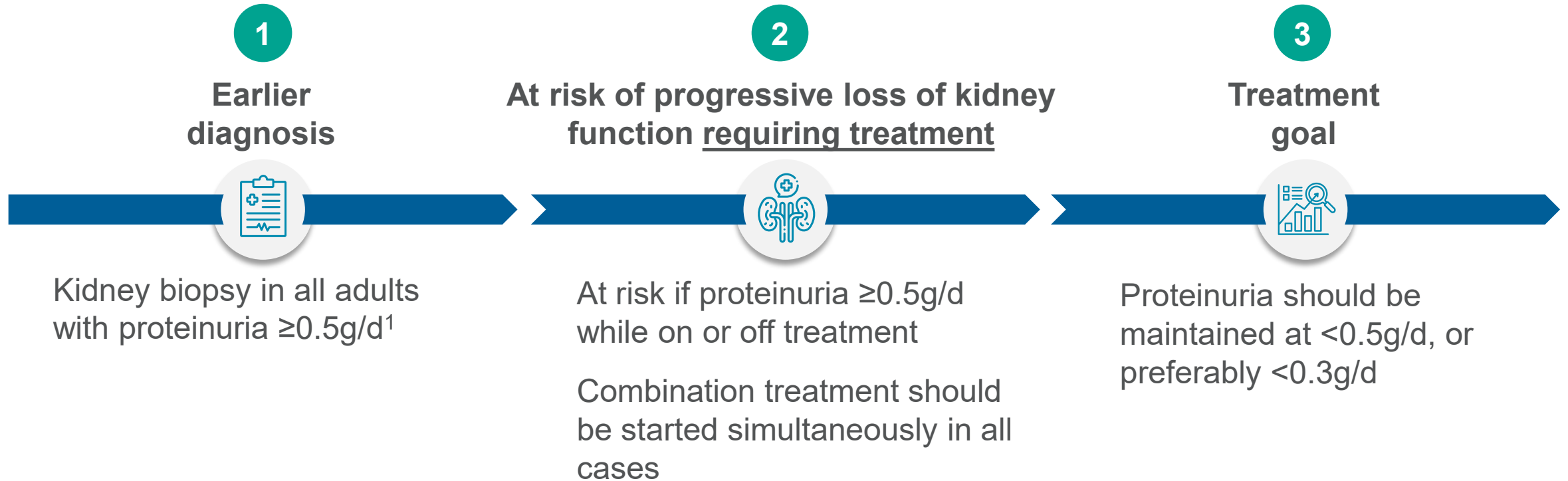
30-40%

of transplants fail due to disease recurrence⁷

¹ Le W, et al. *Nephrol Dial Transplant* 2012; 27:1479–1485; ² McGrogan A, et al. *Nephrol Dial Transplant*. 2011;26:414–430; ³ Nasri H, et al. *J Nephrol*. 2015; 4:1–5; ⁴ Source: independent market research, data on file; ⁵ Barratt J, et al. "Natural History of IgA Nephropathy: Analysis of a UK National RaDaR IgA Nephropathy Cohort." ASN 2021; Poster presentation (Abstract P01577); ⁶ Nair R & Walker PD. *Kidney Int* 2006; 69:1455–1458; ⁷ Uffing A et al. *Clin J Am Soc Nephrol*. 2021 Aug;16(8):1247–1255.

Draft KDIGO Guidelines: The IgAN Treatment Paradigm is Evolving

Earlier Treatment, Lower Proteinuria Targets and Simultaneous Therapy



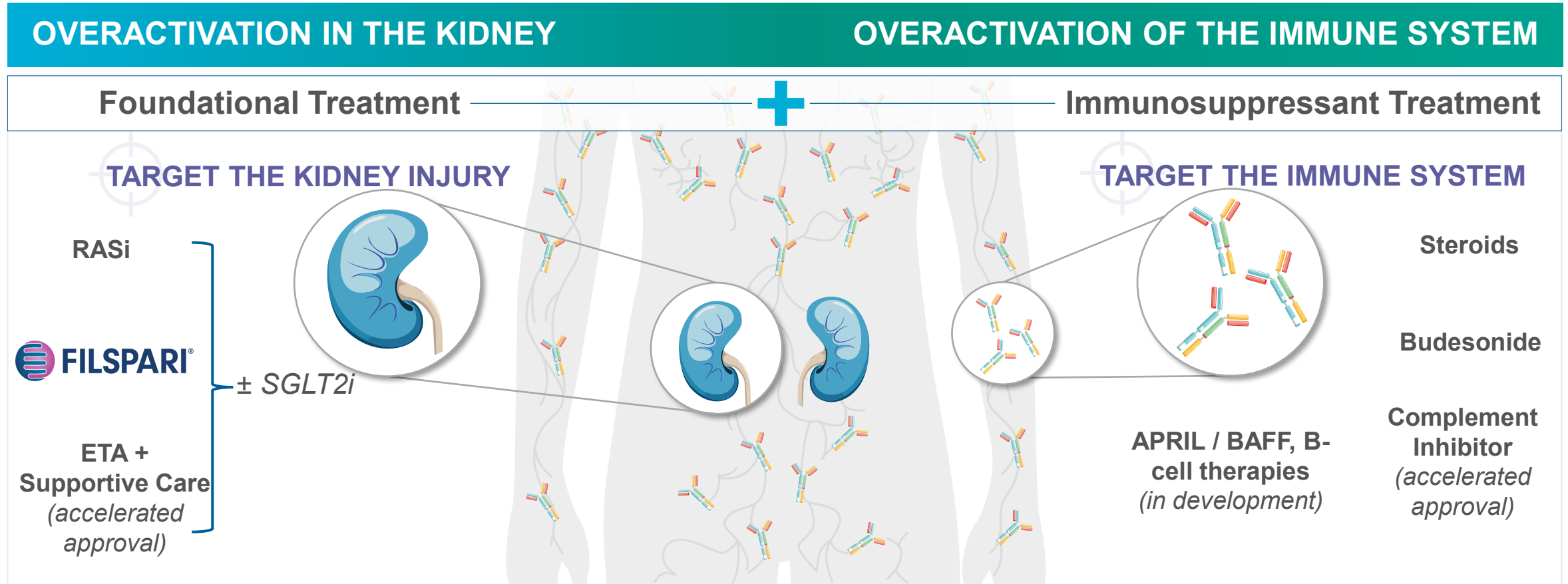
Proteinuria is the only validated early biomarker to help guide clinical decision-making

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

¹ Or equivalent. In whom IgAN is a possible diagnosis and who do not have a contraindication for kidney biopsy.

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



FILSPARI is the only oral non-immunosuppressive, long-term treatment positioned to replace historical standard of care for patients with IgAN*

Abbreviations: RASi: renin-angiotensin system inhibitor.

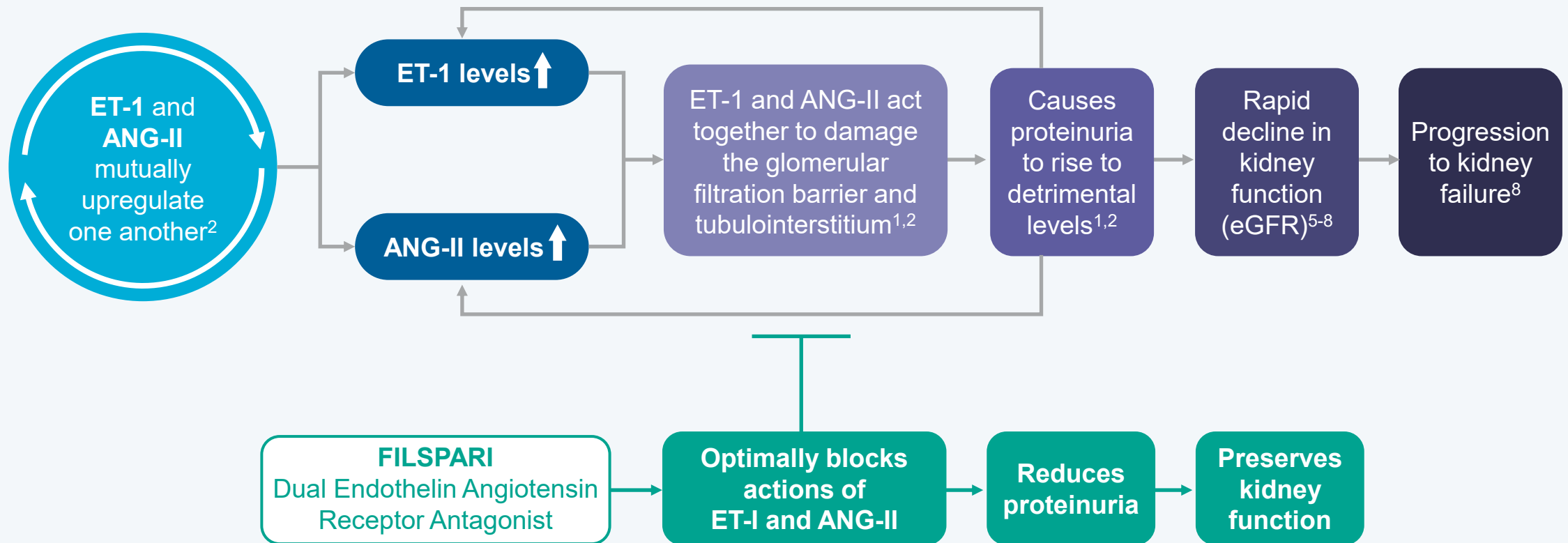
Source: KDIGO 2024 Clinical Practice Guideline for the Management of Immunoglobulin A Nephropathy (IgAN) and Immunoglobulin A Vasculitis (IgAV), public review draft, 8/30/2024.

* Indicated to slow kidney function decline in adults with primary IgAN who are at risk for disease progression.

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Targeting the Kidney: 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



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.

¹ Komers R, et al. Am J Physiol Regul Integr Comp Physiol. 2016;310(10):R877-R884. ² Kohan 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.

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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

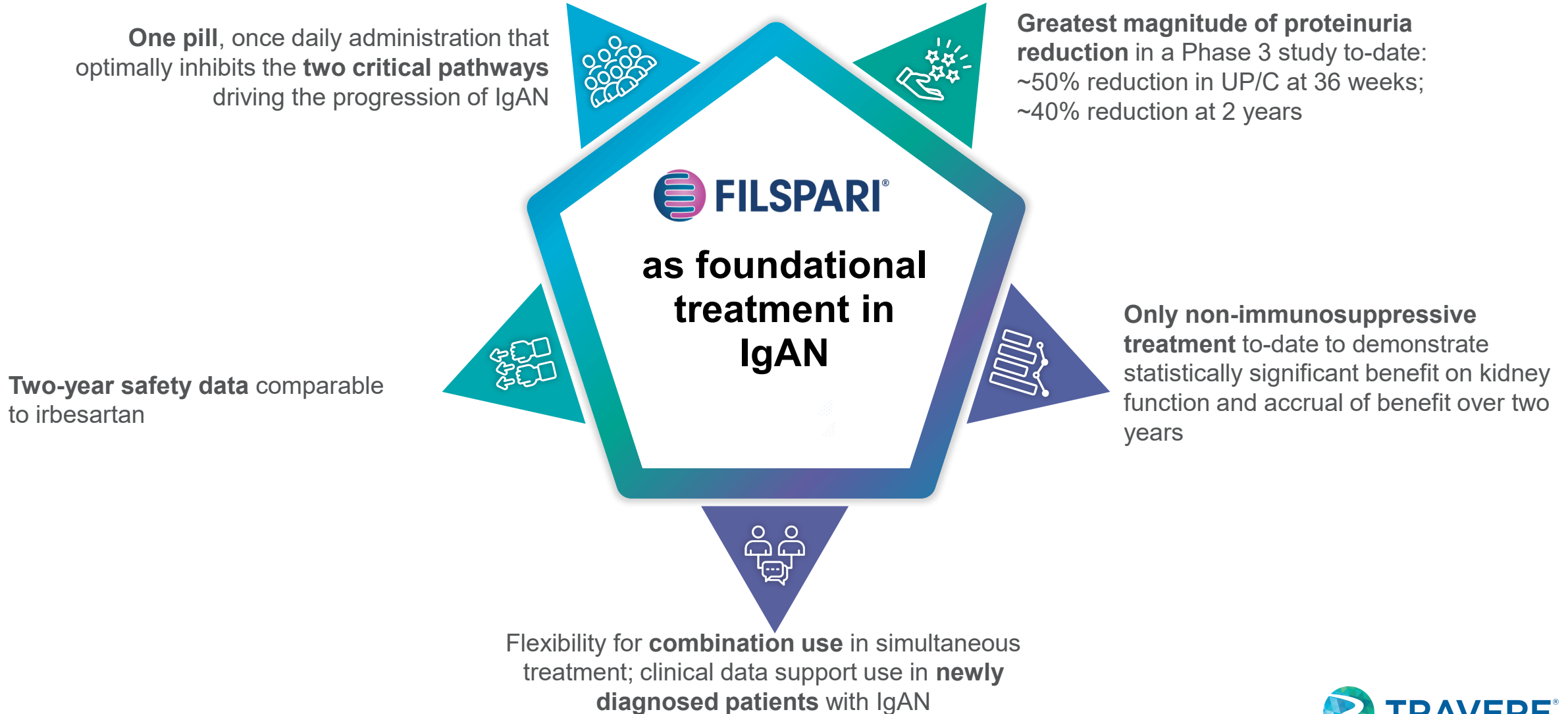
Tablets: 200mg and 400mg, for once-a-day oral dose

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](https://www.filspari.com)

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



Commercial Launch Outperforming Benchmarks; Sustained Momentum Driven by Growing Patient Base and Expanding Prescriber Adoption

~\$72M

U.S. net FILSPARI sales in 2Q25

 **~165% growth vs 2Q24**



High compliance and persistence rates

745

New PSFs in 2Q25

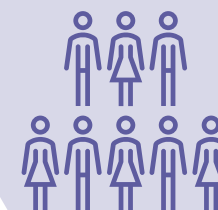
 **Building on strong base post full approval**



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

96%

U.S. Patients with Pathway to Access



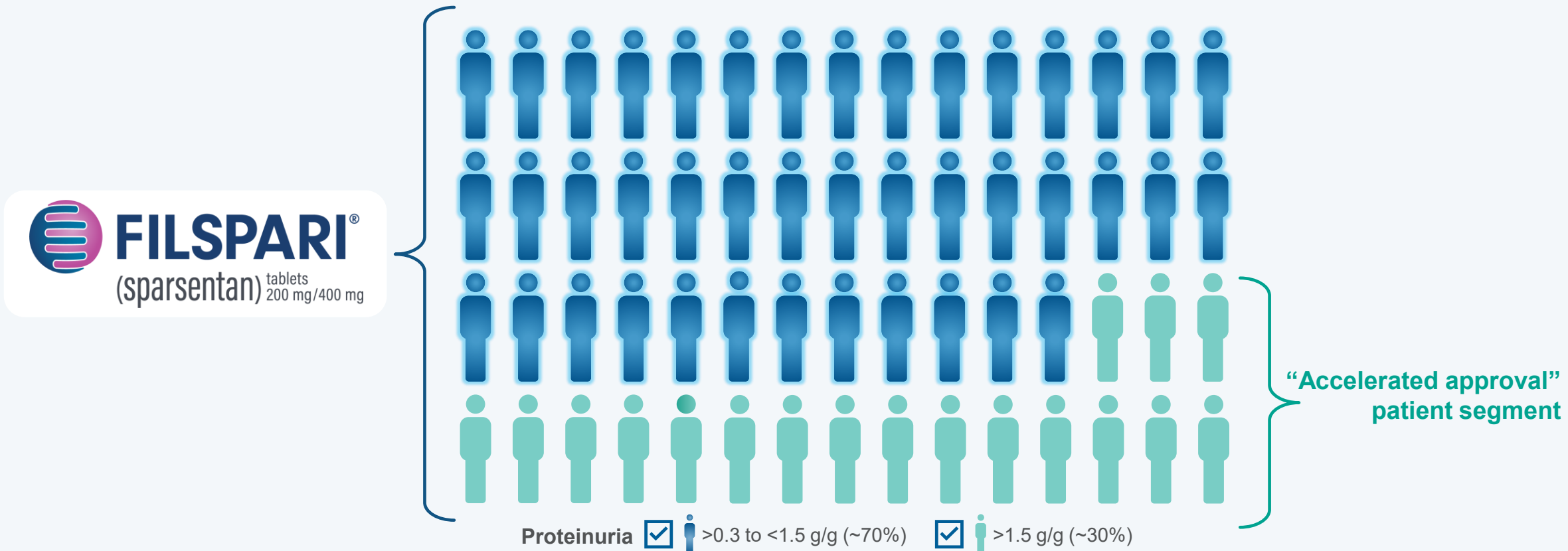
FILSPARI is well established in payer plans and formularies, reflected in payer approval claims

* Benchmark launches are other recent rare nephrology launches.

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FILSPARI is Expected to be the Only Non-Immunosuppressive Treatment Positioned for a Broad Range of Patients with IgAN

FILSPARI is available to patients with IgAN across broad range of proteinuria levels¹
>70,000 addressable patients in the U.S.²



¹ FILSPARI is indicated to slow kidney function decline in adults with primary IgAN who are at risk for disease progression.

² Source: independent market research, data on file.

Key Growth Drivers Supporting Continued Execution of Commercial Launch

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

Draft KDIGO guidelines² drive earlier intervention, strengthen FILSPARI's foundational positioning

Opportunity to broaden and deepen FILSPARI's prescriber base

Continue to simplify access for patients and engage with payers to expand coverage

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

 **FILSPARI[®]**

>70k

**Addressable
Patients with
IgAN in the
U.S.¹** 

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.

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

Objective

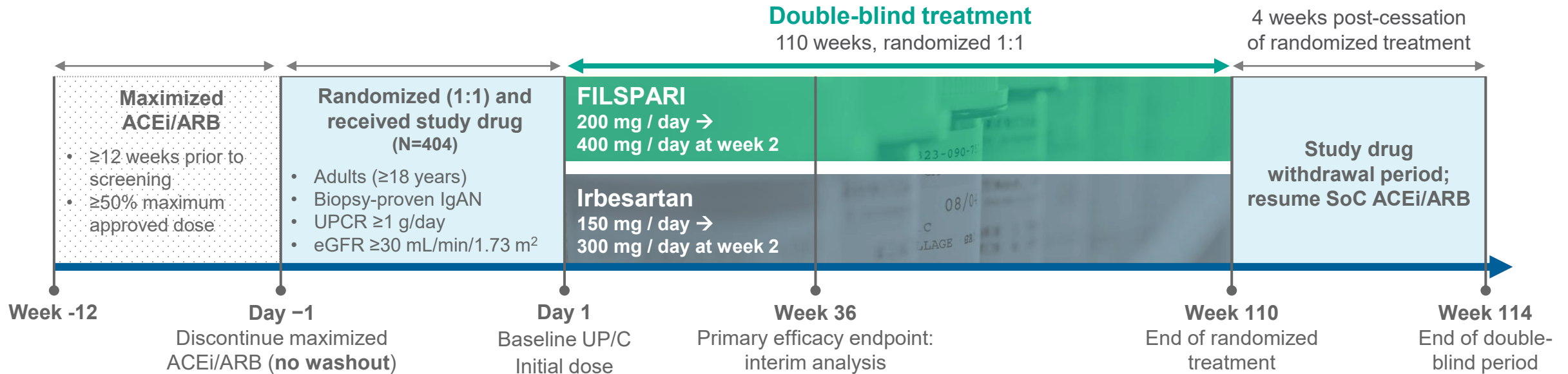


Test the efficacy and safety of FILSPARI vs. active control (irbesartan) in a global, multicenter, double-blind, randomized study of 404 patients with IgAN, ages 18+

Endpoints



- 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)



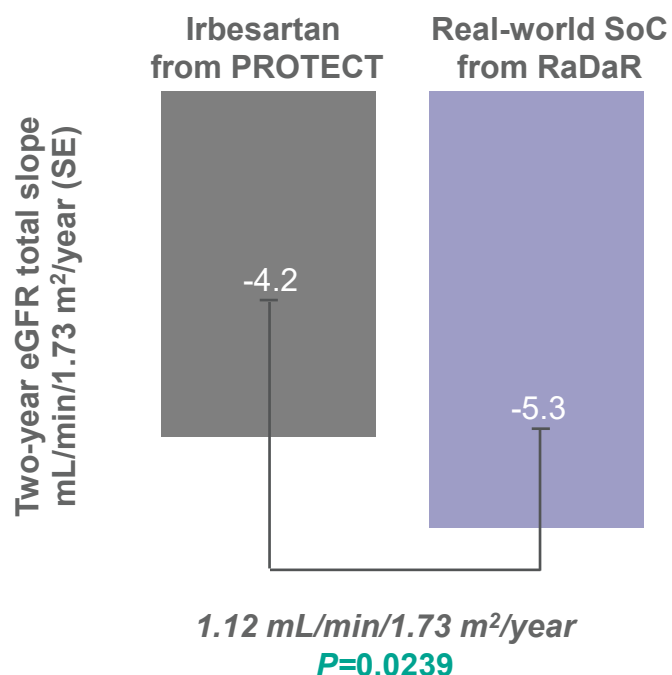
Abbreviations: UPCR: urine protein/creatinine ratio, g/day: grams per day, eGFR: estimated glomerular filtration rate, ACEs: angiotensin converting enzyme inhibitors, ARBs: angiotensin receptor blockers, SoC: standard of care.

* ClinicalTrials.gov ID: NCT03762850.

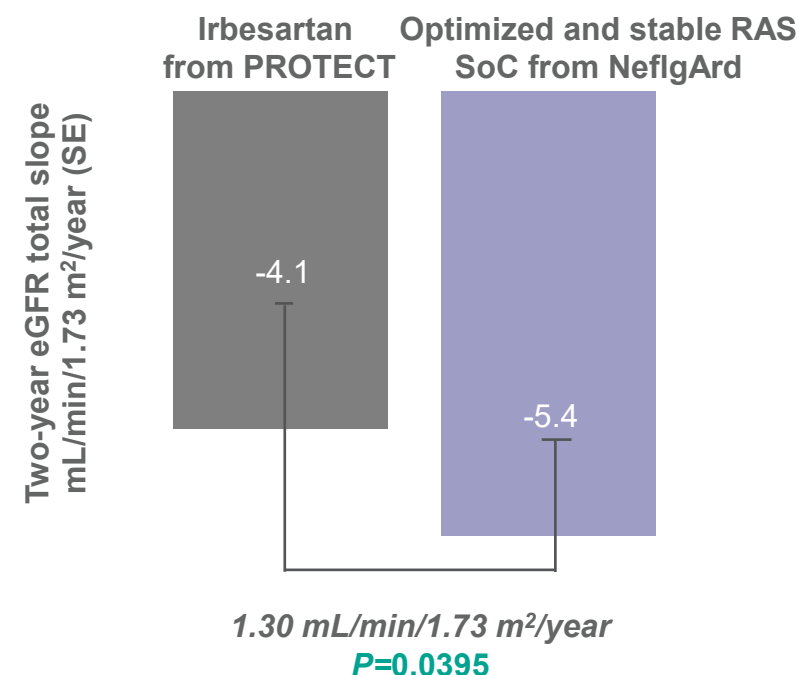
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Active Control is Not Placebo: Matching-Adjusted Indirect Comparisons Show Irbesartan Significantly Outperformed Standard of Care in Other Studies

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



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*

Source: Cheung et al, NKF 2024, Matching-Adjusted Indirect Comparisons of eGFR slopes in the PROTECT study with UK RaDaR IgA Nephropathy population and the control arm of NeflgArd.

Abbreviations: eGFR: estimated glomerular filtration rate, SE: standard error, SoC: standard of care, RaDaR: The UK National Registry of Rare Kidney Diseases.

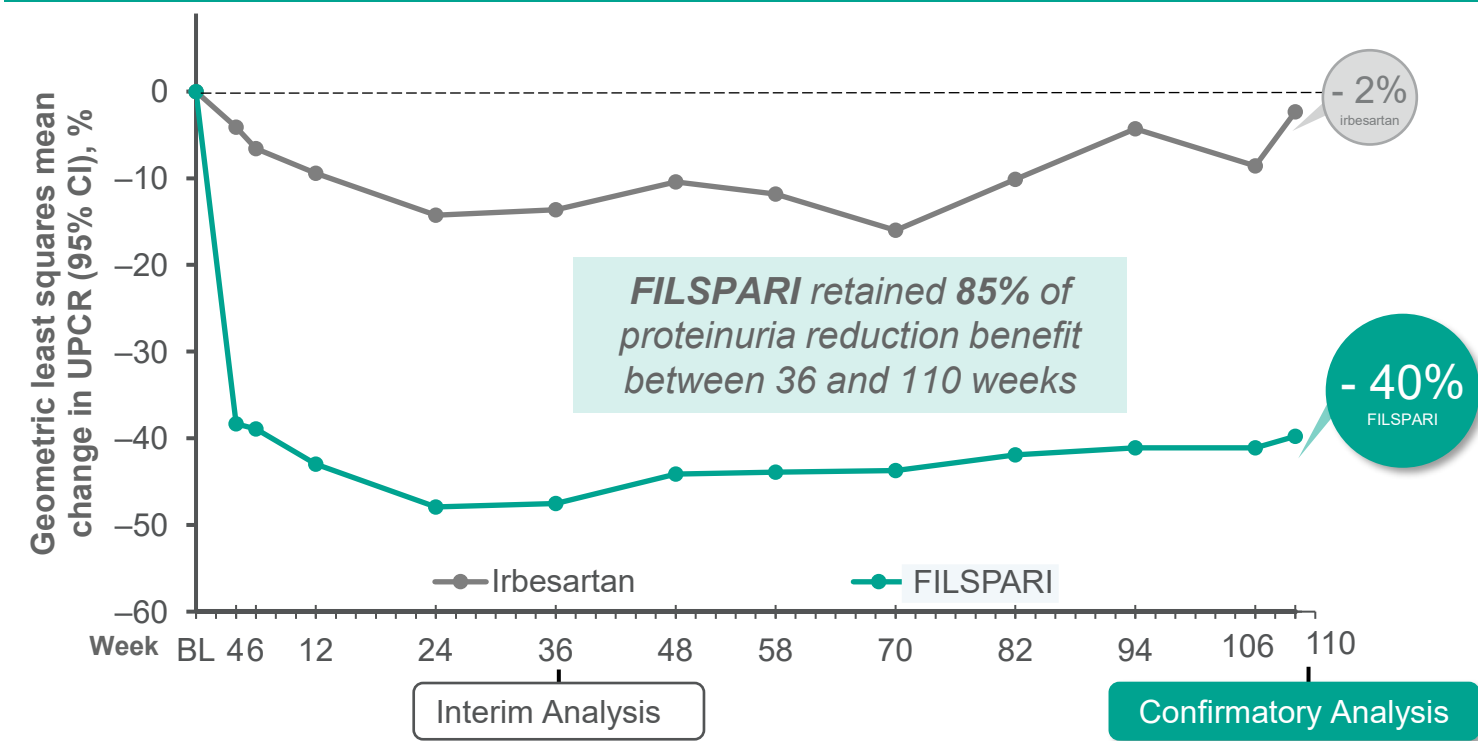
* NeflgArd is a randomized, double-blind, placebo-controlled clinical trial recruiting a total of 360 patients across 155 nephrology clinics in 20 countries.

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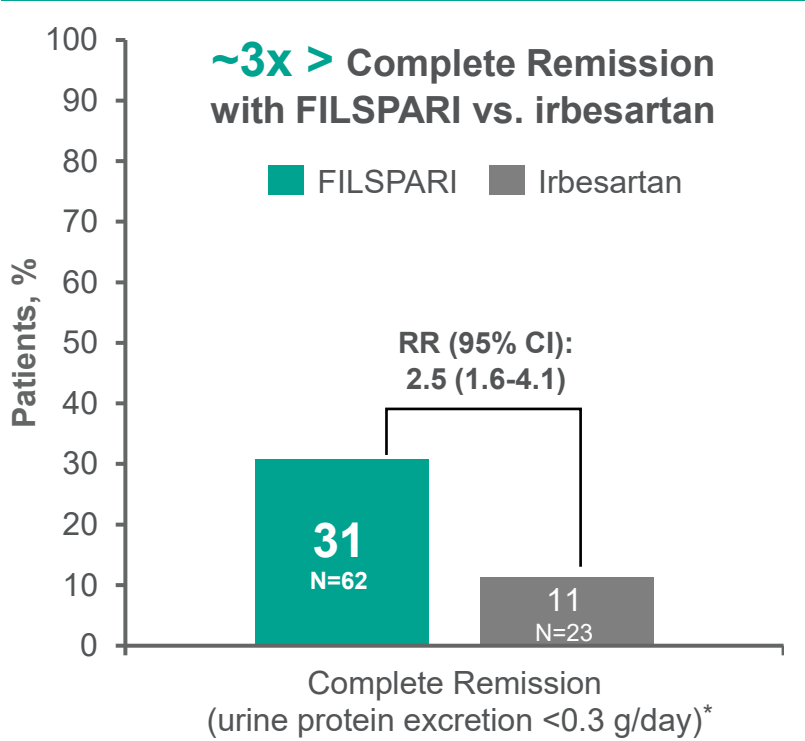
FILSPARI Showed Superior Proteinuria Reduction in a Phase 3 Study vs. Active Control, Sustained Over Two Years

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

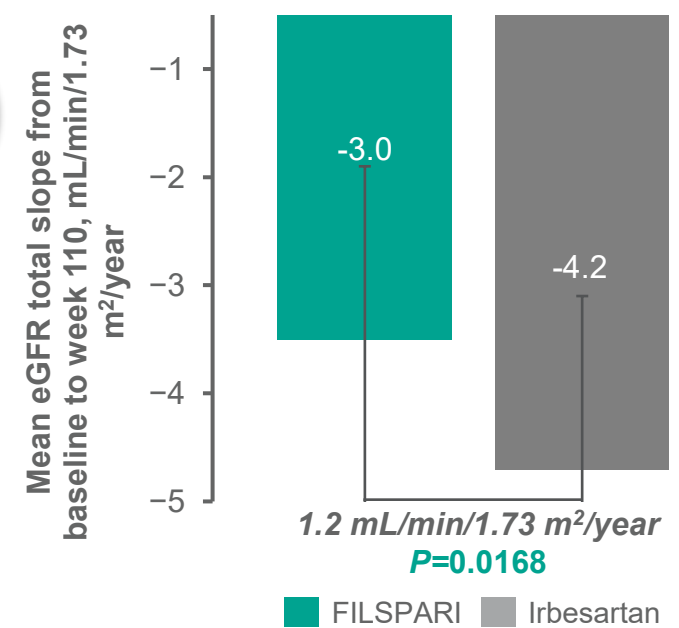
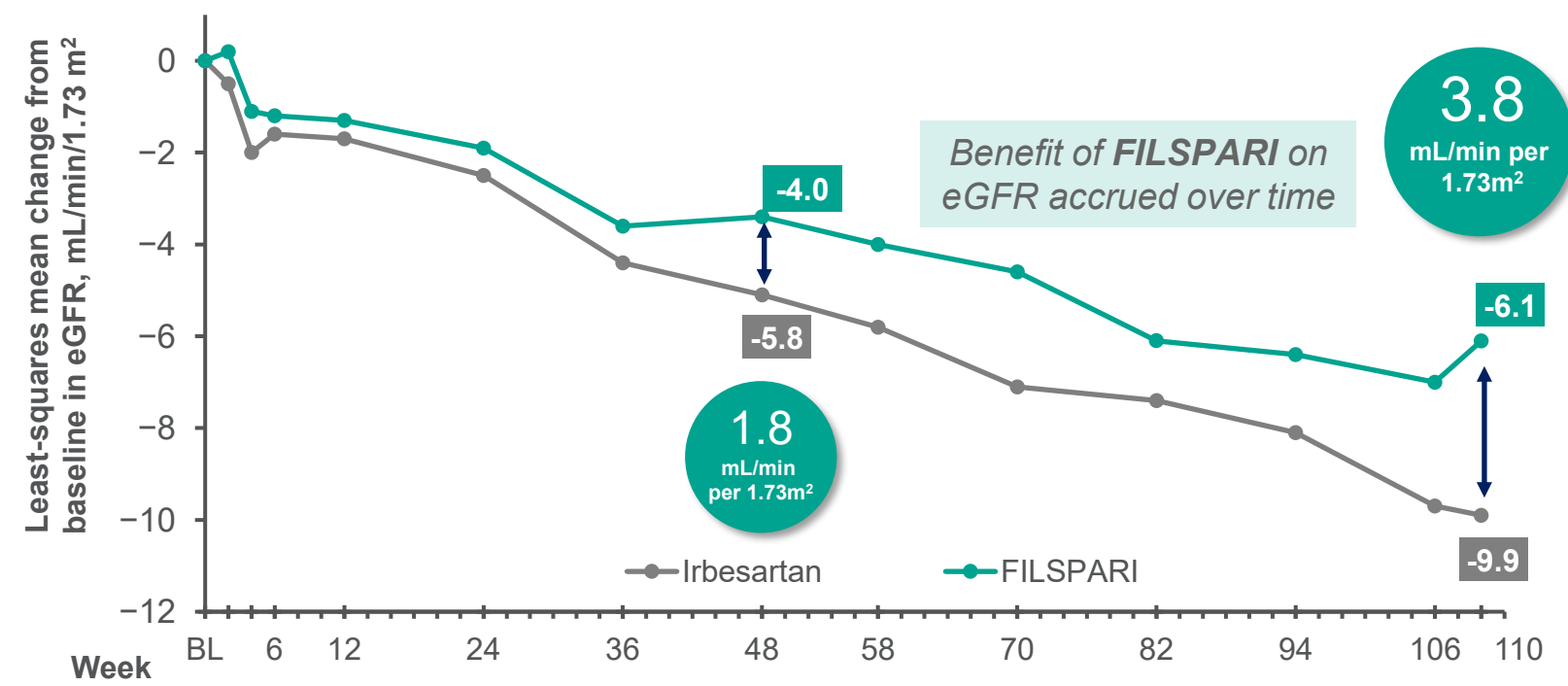


Abbreviations: UP/C: urine protein/creatinine ratio, UPCR: urine protein/creatinine ratio, BL: baseline, UPE: urinary protein excretion.
MMRM analysis including on-treatment data through week 110 with multiple imputation.
* Achieved complete remission at any time while on study medication during the double-blind period.
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FILSPARI Demonstrated Significant Long-Term Kidney Function Preservation in Patients with IgAN

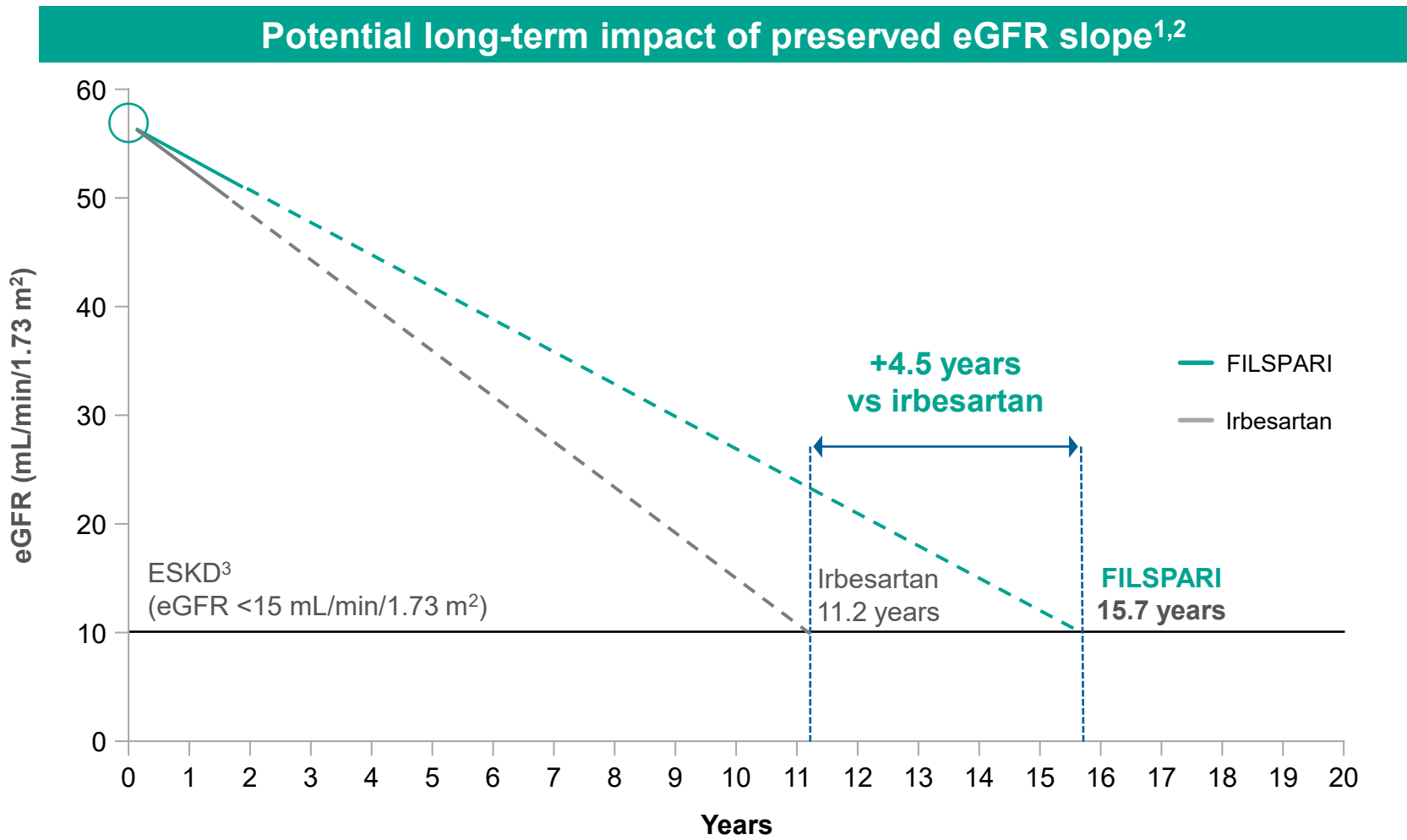
Long-term FILSPARI treatment showed significant preservation of kidney function that accrued over time

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.
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Treatment with FILSPARI May Potentially Delay Dialysis or Transplant



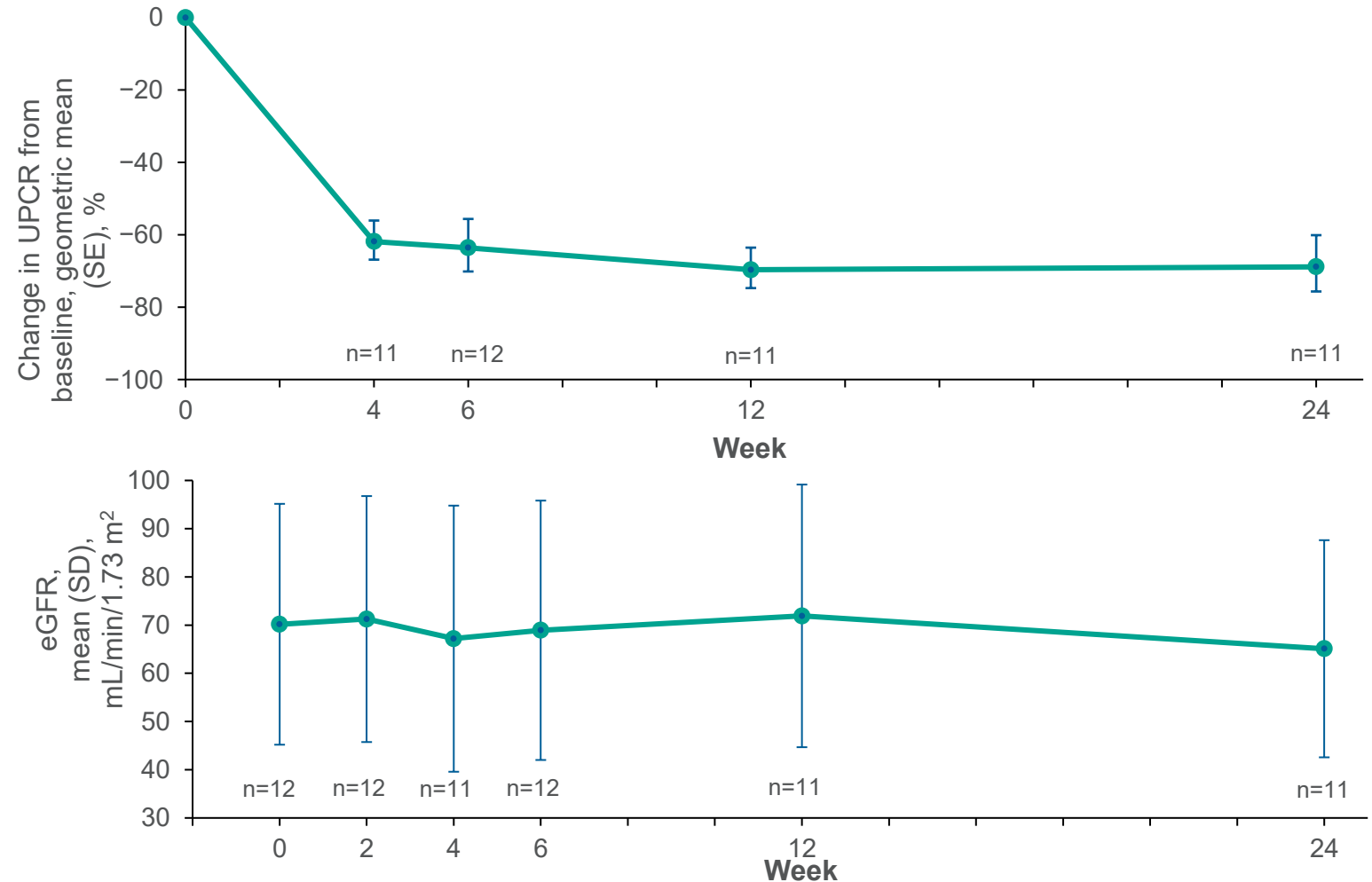
Based on extrapolation of eGFR slope data from PROTECT, FILSPARI may potentially **delay dialysis or transplant by 4.5 years** when compared to maximum-labeled dose irbesartan¹⁻³

Abbreviations: eGFR: estimated glomerular filtration rate, ESKD: end-stage kidney disease.
¹ FILSPARI Prescribing Information. San Diego, CA: Traverre Therapeutics, Inc.
² Data on file, Traverre 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.
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SPARTAN Study: Rapid and Sustained Impact of FILSPARI as First-Line Treatment in Newly Diagnosed Patients

Preliminary clinical findings at 24-weeks in treatment-naïve patients on FILSPARI

- ▶ Sparsentan, led to rapid and sustained reductions in proteinuria (~**70%** from baseline) and stabilization of eGFR 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-creatinine 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.

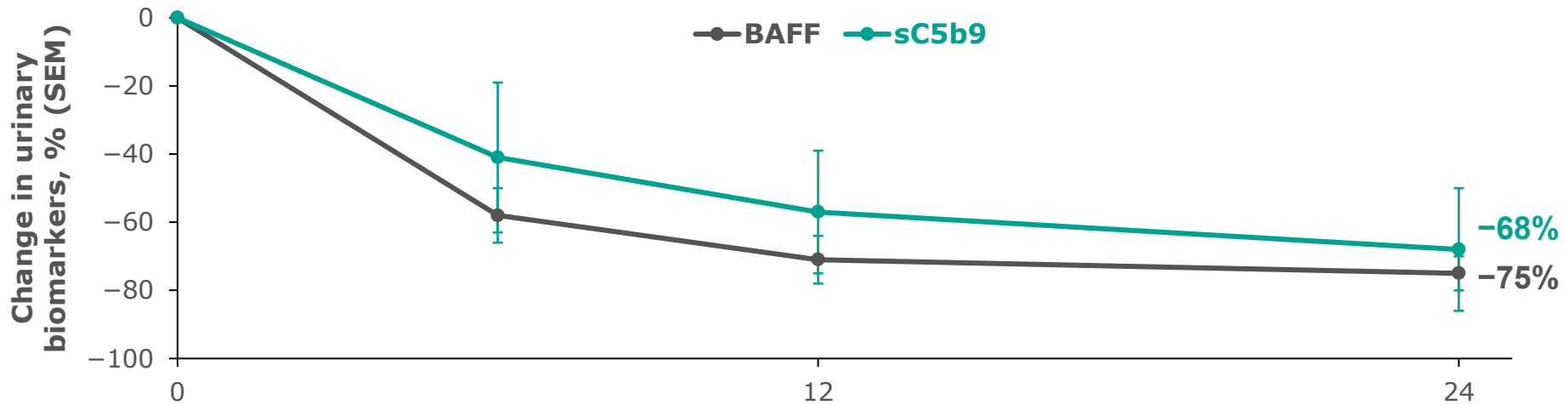
² Heerspink HJL, et al. Lancet. 2023;401(10388):1584-1594.


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

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SPARTAN Study: Urinary Biomarker Analysis Suggests Disease-Modifying Effects of FILSPARI in IgAN

Treatment with FILSPARI resulted in rapid and sustained reductions in urinary biomarkers of inflammation and fibrosis that reveal anti-inflammatory and anti-fibrotic effects of FILSPARI¹

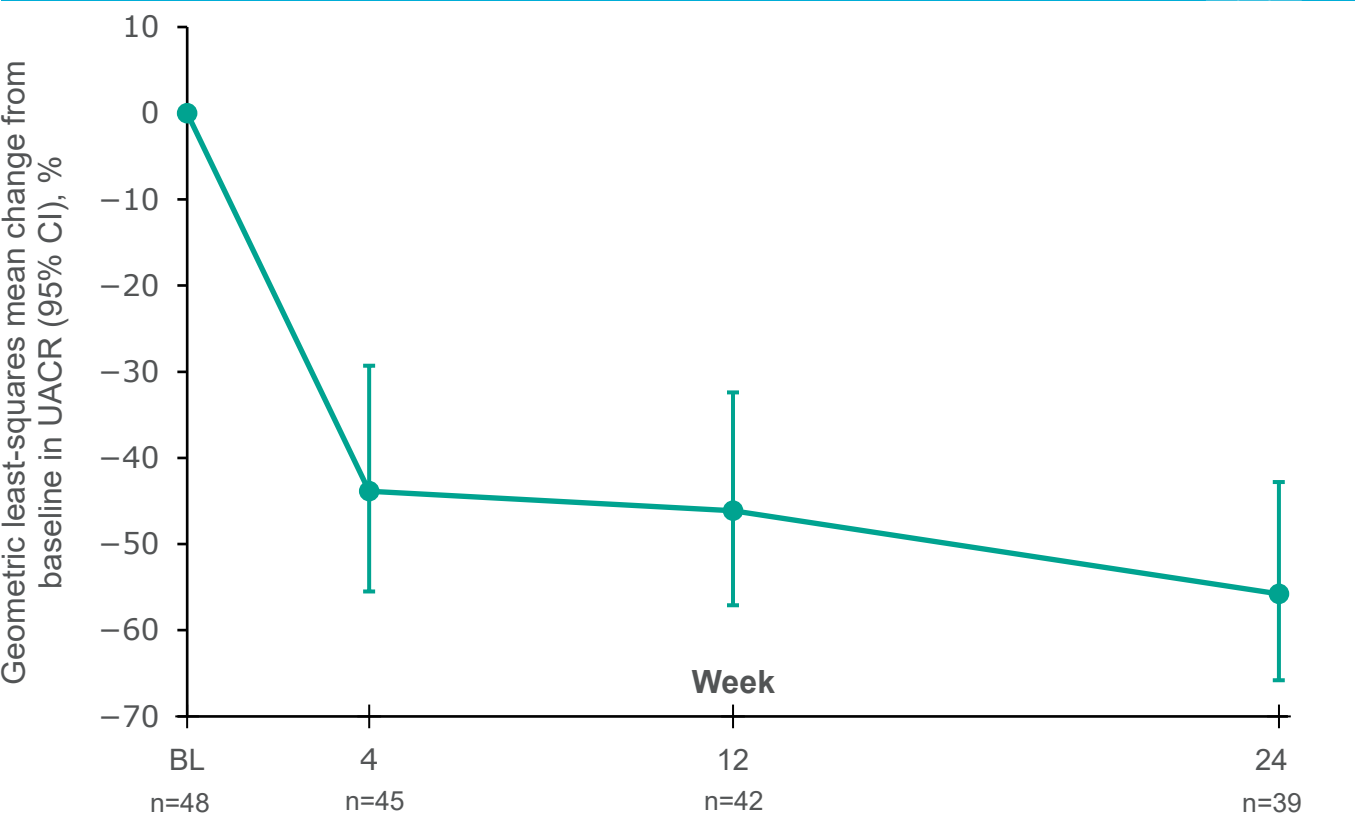




Change in urinary biomarkers from baseline to week 24						
Inflammatory and profibrotic	α2M ² -83%	CHI3L1 -52%	clusterin ² -47%	GDF15 -42%	plasminogen ² -85%	sCD163 -50%
Chemokine and cytokine	CXCL10 -28%	CXCL16 -22%	IL6 -23%	MCP-1 -16%		

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

Transitioning patients from RASi to FILSPARI resulted in a mean reduction in UACR of ~56% at 24 weeks



Abbreviations: BL: baseline, RASi: renin-angiotensin system inhibitor, SGLT2i: sodium-glucose cotransporter-2 inhibitor, SPAR: sparsentan, UACR: urine albumin-to-creatinine ratio, TEAE: treatment-emergent adverse event, AE: adverse event, ULN: upper limit of normal.

Source: Ayoub I., et al. presented at ERA 2025, June 4-7, 2025; Vienna, Austria. Abstract: No. 1916.

* Reported in the same patient. †The incident of acute kidney injury was mild, deemed unrelated to SPAR or SGLT2i treatment, and was resolved after interruption of SPAR and SGLT2i. ‡Abnormal liver function test results met the following criteria: (1) new elevation in ALT or AST >3 × ULN with or without elevation of total serum bilirubin >2 × ULN and (2) 2-fold increase in ALT or AST above the baseline value in patients who had elevated values prior to taking study medication. § One patient each discontinued SPAR treatment due to a TEAE of vertigo, hypotension, peripheral edema, and Henoch-Schönlein purpura.

TEAEs	Patients (N=48)
Any TEAE, n (%)	30 (63)
SPAR related	10 (21)
SGLT2i related	2 (4)
Any TEAEs in >2 patients, n (%)	
Hypotension	7 (15)
Headache	4 (8)
Edema	4 (8)
Peripheral edema	4 (8)
Upper respiratory tract infection	4 (8)
Dizziness	6 (6)
Any severe TEAE, n (%)	2 (4)
Peripheral edema	1 (2)
Gout	1 (2)
Any serious AE, n (%)	4 (8)
Acute kidney injury**	1 (2)
Cerebrovascular accident	1 (2)
Chemical burn	1 (2)
Deep vein thrombosis	1 (2)
Osteoarthritis*	1 (2)
Any abnormal liver function test results >3×ULN, n (%)‡	0 (0)
Any TEAE leading to SPAR discontinuation, n (%)	4§ (8)

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



>70k addressable patients with IgAN¹

United States

CSL Vifor

Standard approval in Europe and the UK; FILSPARI launched in Germany, Austria, and Switzerland

CMA covers all 27 member states of the European Union, plus Iceland, Liechtenstein, and Norway²



Results from registration enabling study for Japan expected in 2H25

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

Traverse 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.

¹ Source: independent market research, data on file.

² License to CSL Vifor covers Europe, Australia, New Zealand, Bahrain, Brazil, Chile, Israel, Kuwait, Oman, Qatar, Saudi Arabia and the United Arab Emirates, with potential to expand. ³ Potential milestone payments include achievements for both IgAN and FSGS indications.

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A photograph of a family of three walking away from the camera on a dirt path through a green field. The father is on the left, wearing a dark jacket and black pants. The mother is in the middle, wearing a black leather jacket and blue jeans, with her arm around the father's shoulder. A young child is on the right, wearing a light-colored jacket and pants, holding the mother's hand.

Focal Segmental Glomerulosclerosis (FSGS)

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

up to 30k

Potential addressable patients with FSGS 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



¹Estimated based on McGrogan A, et al. *Nephrol Dial Transplant*. 2011;26(2):414-430; data on file. ²Kiffel et al. *Adv Chronic Kidney Dis*. 2011;18:332-338.


PARASOL Project: Key Takeaways

- 1 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.
- 2 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.
- 3 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

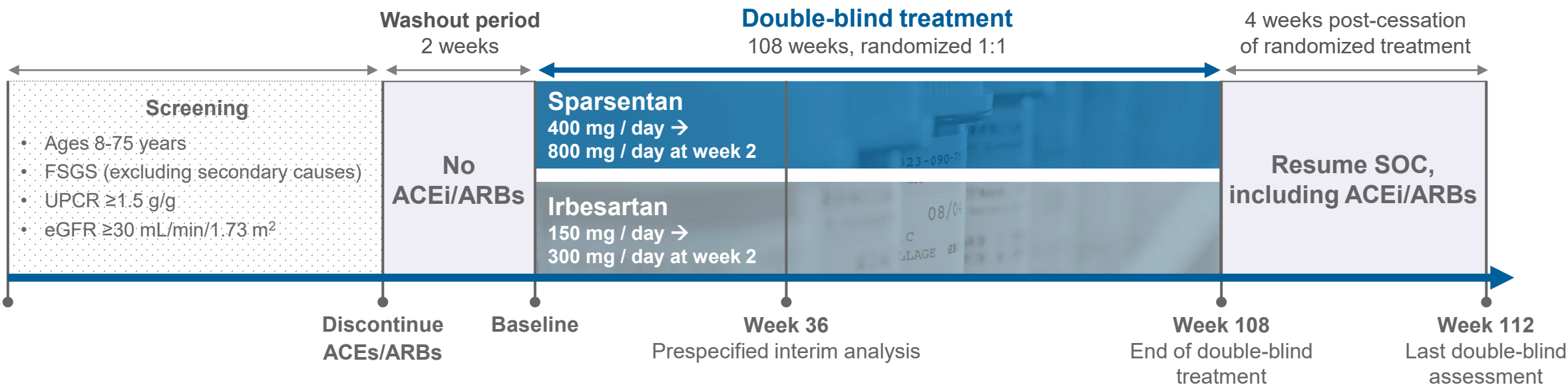
The DUPLEX Study of Sparsentan is the Largest Active-Controlled Interventional Phase 3 Trial in FSGS to Date

Objective


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

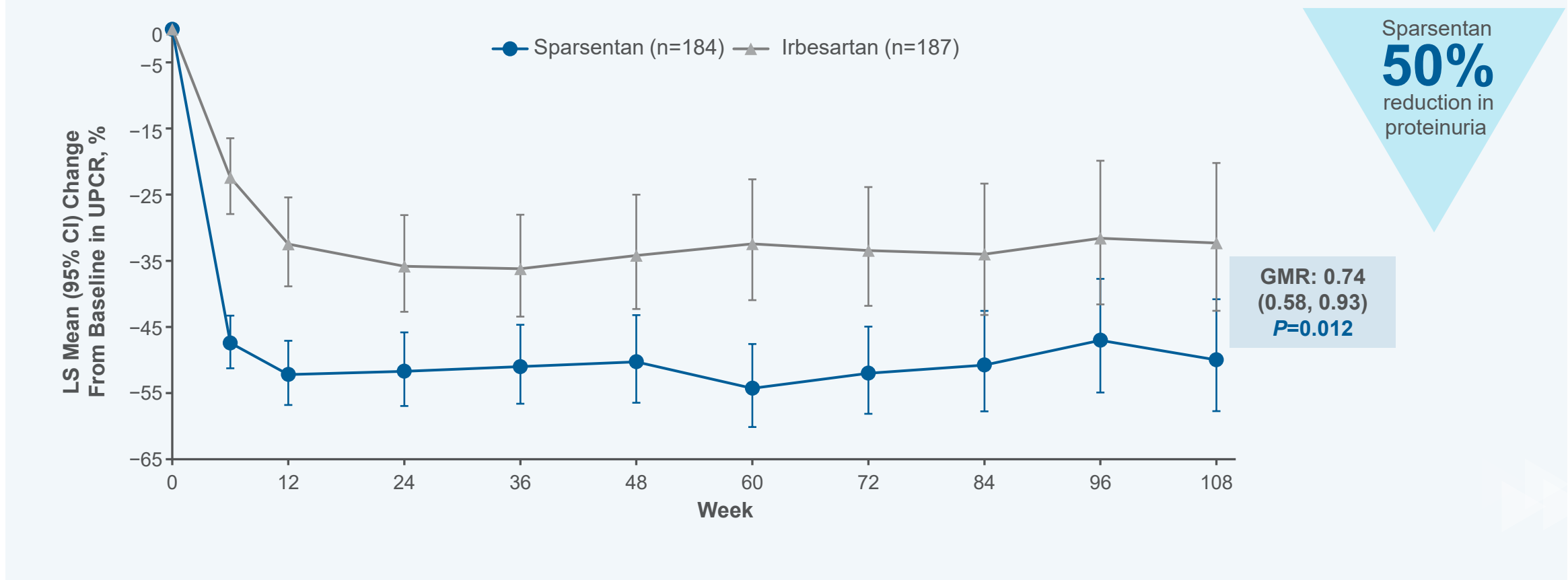
Trial Design


- 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 FPPE at week 36 (UPCR ≤ 1.5 g/g and $\geq 40\%$ 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)

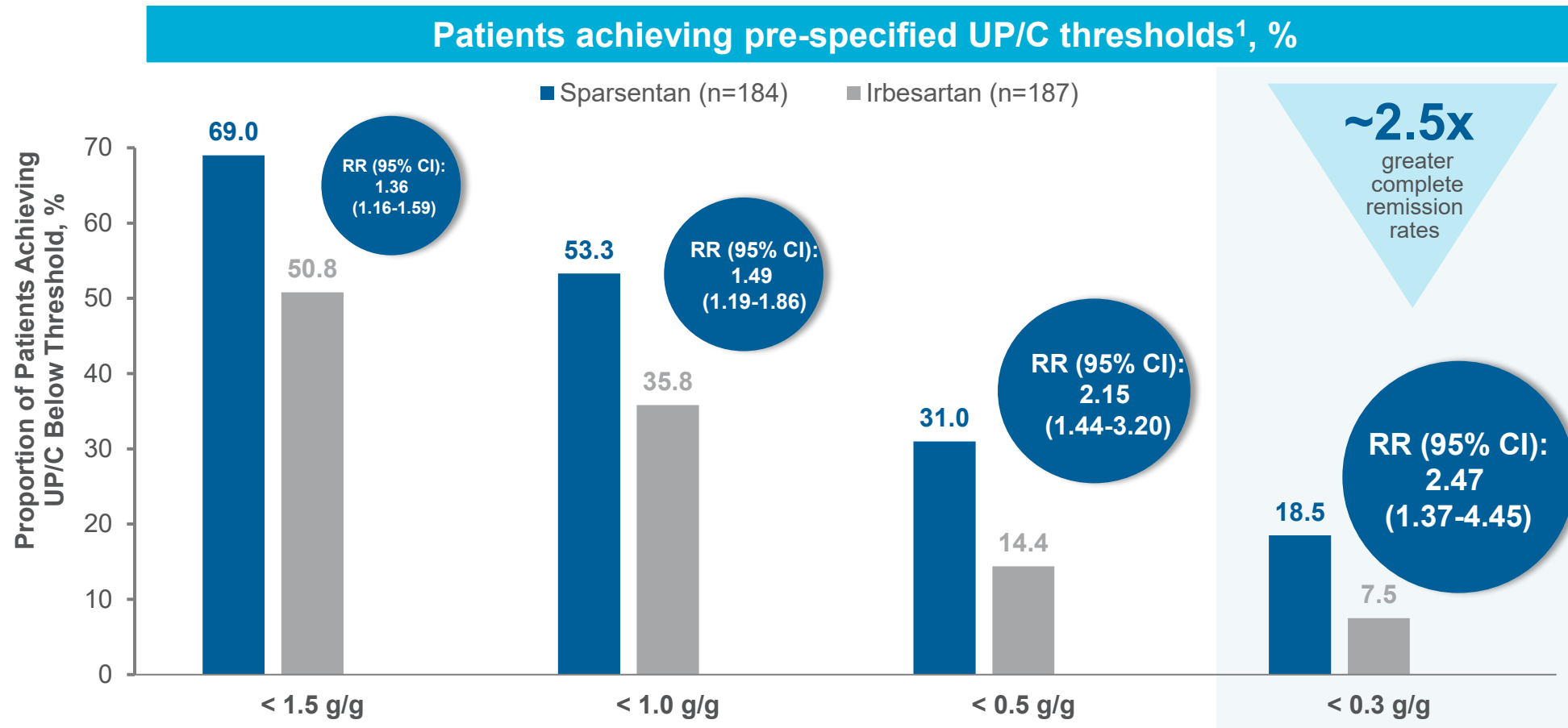


Results from the Phase 3 DUPLEX Study of Sparsentan in FSGS: Rapid Decline in Proteinuria Sustained Through 108 Weeks

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



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



Next Steps for FSGS

- PDUFA target action date of January 13, 2026
- Prepare for commercial launch in early 2026, if approved

Abbreviations: CI: confidence interval, RR: relative risk, UP/C: urine protein/creatinine ratio.

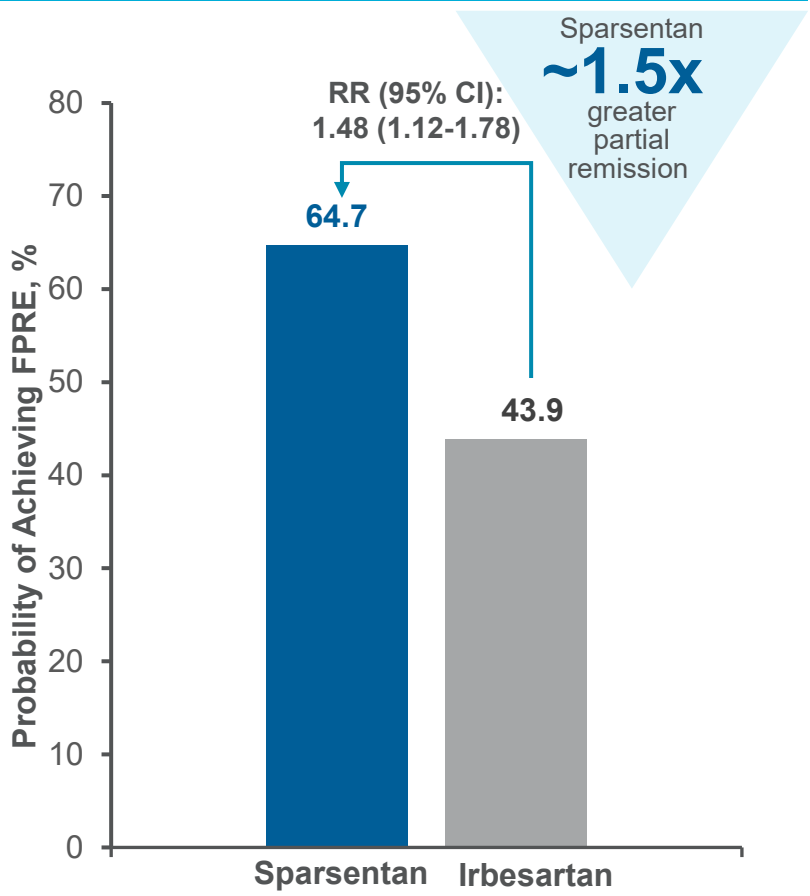
Source: Rheault MN, et al., Sparsentan versus Irbesartan in Focal Segmental Glomerulosclerosis, The New England Journal of Medicine and Supplement, 2023.

¹ At any time during the double-blind period.

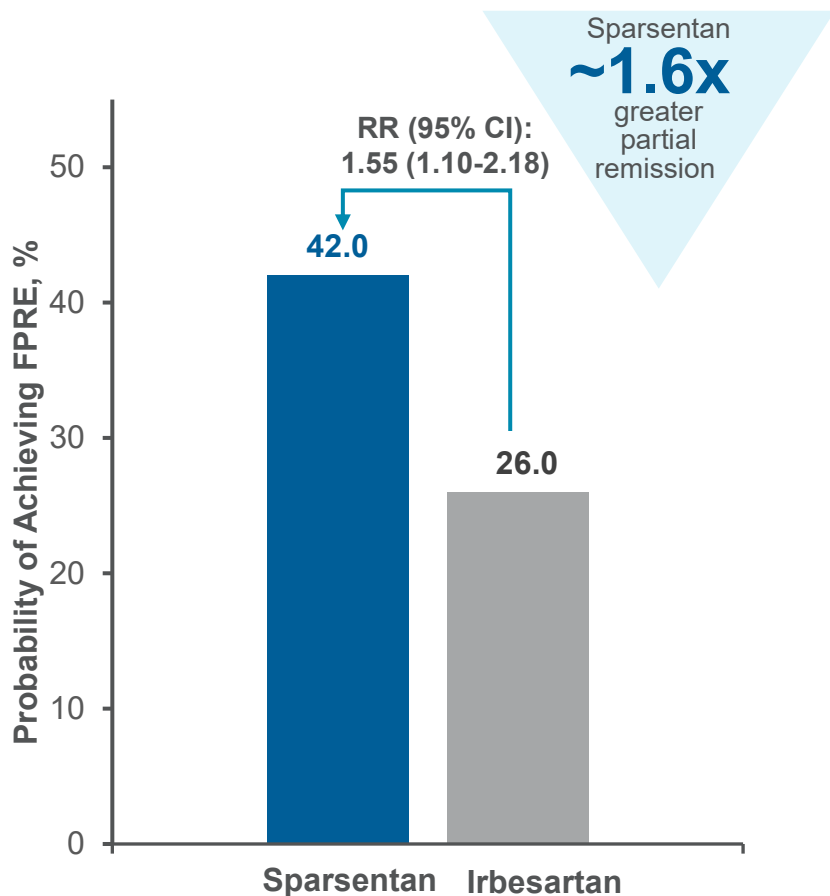
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Sparsentan Demonstrated Consistent Treatment Effect at Interim Analysis and at the End of the Study

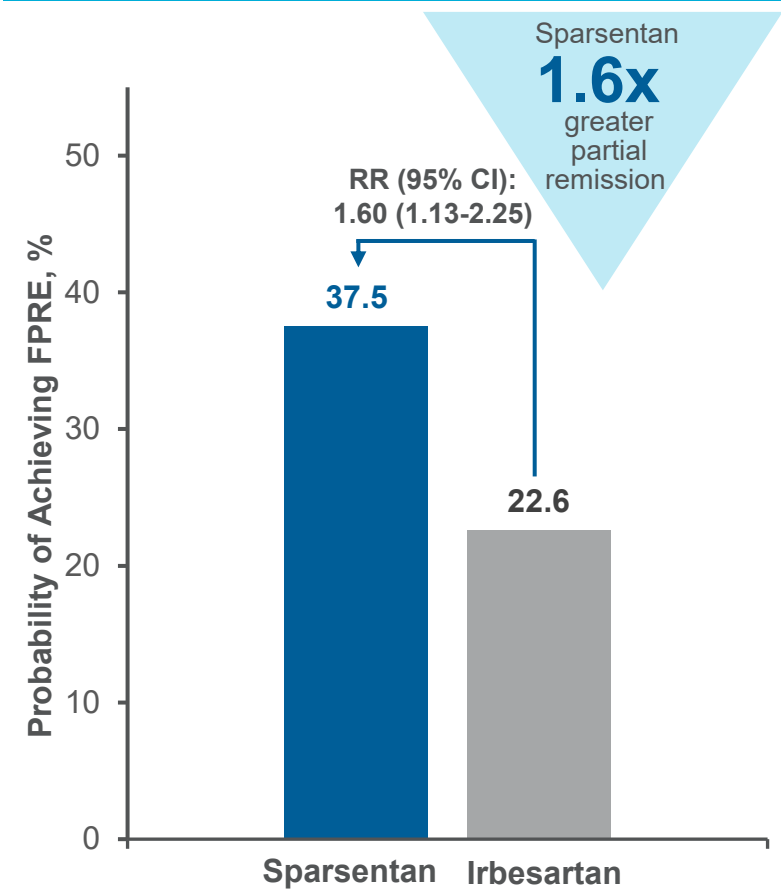
Patients achieving FPRE at any time during the double-blind period



Patients achieving FPRE at Week 36



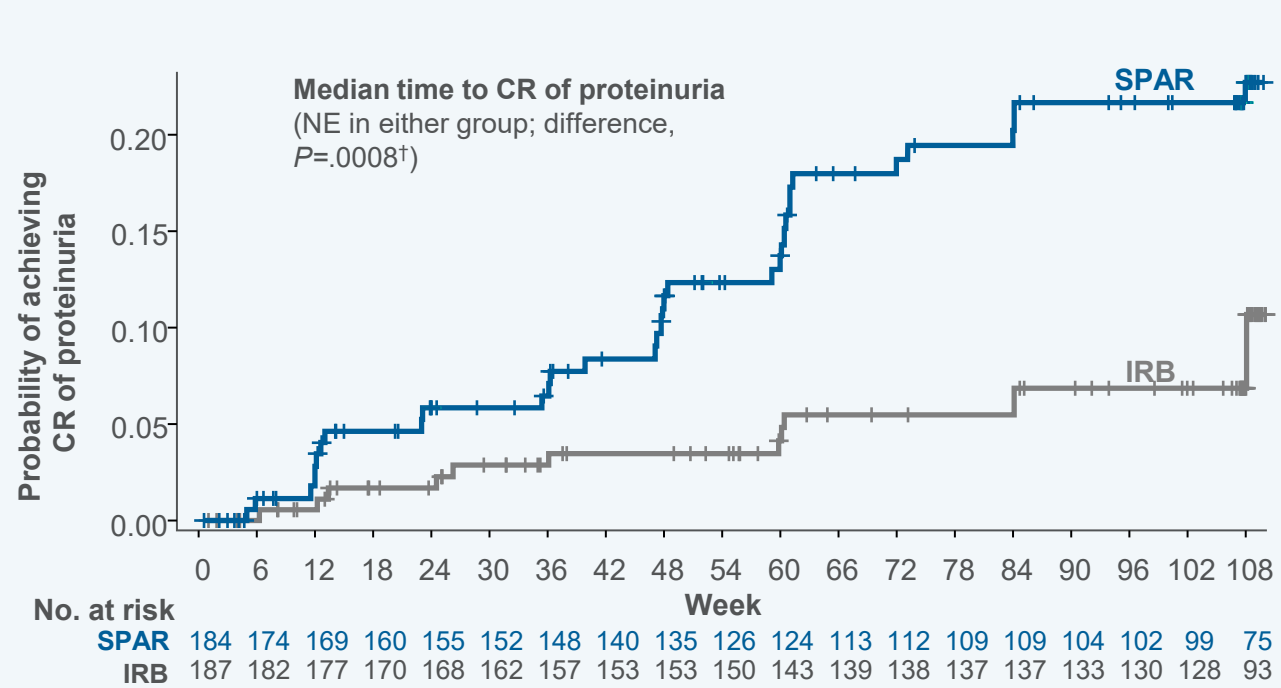
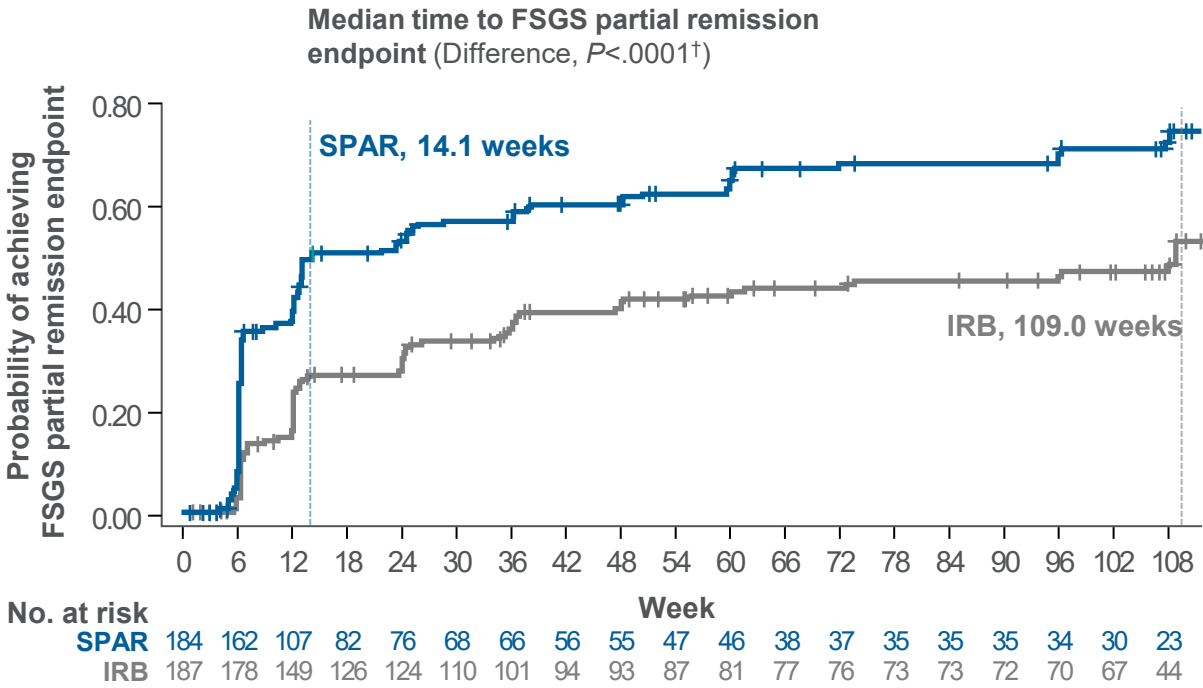
Patients achieving FPRE at Week 108 (final analysis)



Abbreviations: FPRE: FSGS partial remission endpoint, defined as UPCR of ≤ 1.5 g/g and $>40\%$ reduction from baseline,
Source: Rheault MN, et al., Sparsentan versus Irbesartan in Focal Segmental Glomerulosclerosis, The New England Journal of Medicine and Supplement, 2023.

Patients Achieved Partial and Complete Remission Earlier and More Often with Sparsentan vs Irbesartan

Probability of achieving FSGS partial remission endpoint and complete remission of proteinuria, %



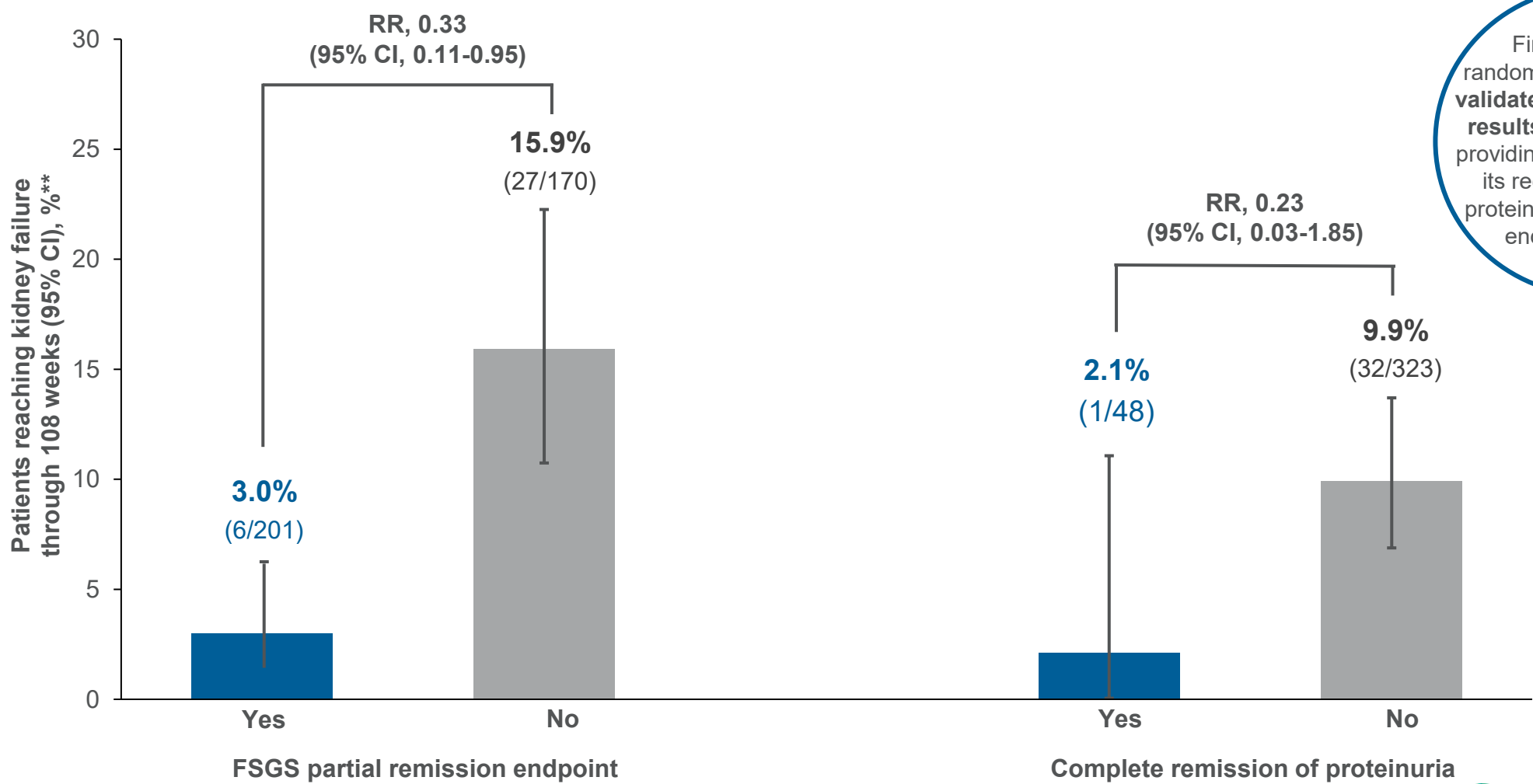
Abbreviations: CR: defined as UPCR of <0.3 g/g, FSGS: focal segmental glomerulosclerosis, FSGS partial remission endpoint: defined as UPCR of ≤ 1.5 g/g and $>40\%$ reduction from baseline, IRB: irbesartan, PR: partial remission, RR: relative risk, SPAR: sparsentan, UPCR: urine protein-to-creatinine ratio.

Source: J Tumlin, et al., presented at the European Renal Association (ERA) Congress 2025; June 4-7, 2025; Vienna, Austria.

† p-value generated from a stratified Cox proportional hazards model with treatment and baseline log (UPCR) as covariates, stratified by randomization stratification factors.



In DUPLEX, Patients That Achieved Proteinuria Remission had Markedly Reduced Risk of Progression to Kidney Failure




First data from a randomized clinical trial to validate the observational results from PARASOL, providing robust support for its recommendation of proteinuria as a surrogate endpoint in FSGS

Abbreviations: CI: confidence interval, eGFR, estimated glomerular filtration rate, RR: relative risk.
* Results from post hoc analyses using pooled data irrespective of treatment arm.
** Confirmed eGFR of <15 mL/min/1.73 m2 or kidney replacement therapy.
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Pegtibatinase

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



Classical Homocystinuria (HCU)

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

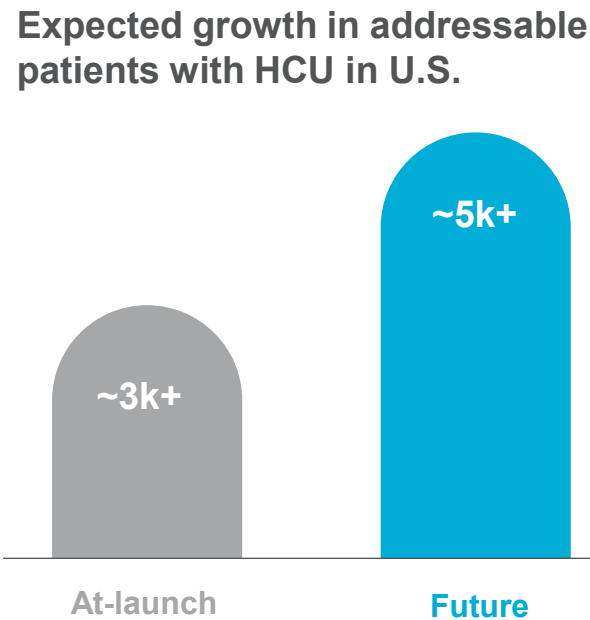
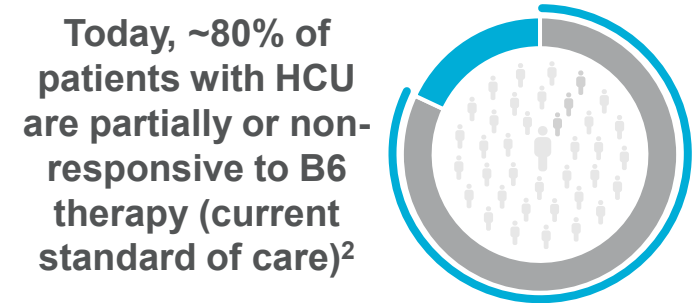
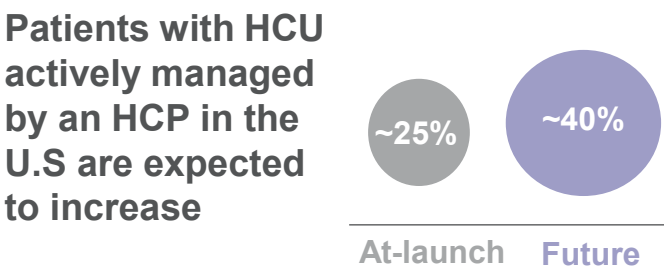
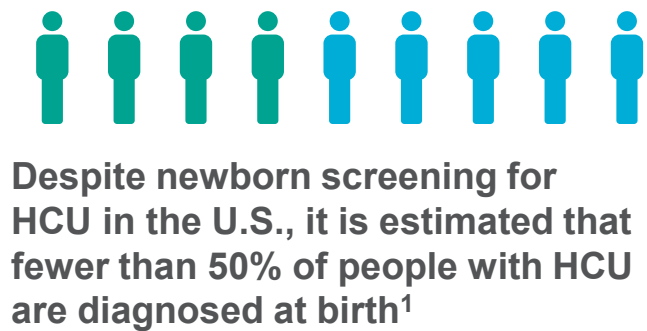
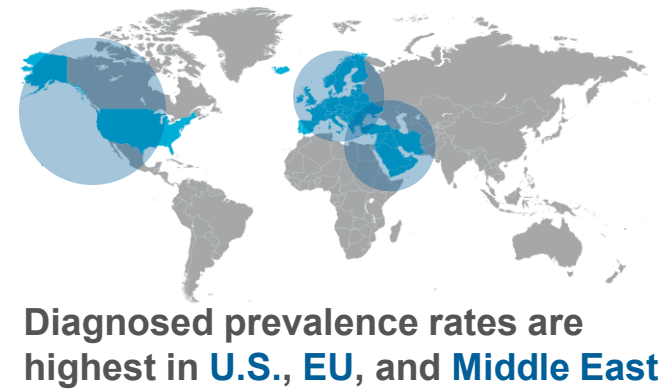
- Caused by mutations in cystathionine beta-synthase (CBS) gene, leading to deficient activity of CBS, which can result in bodily buildup of toxic homocysteine (Hcy).
- Continuous risk of developing life-threatening thrombotic events, including heart attack and stroke, observed in 25% of HCU patients 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



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

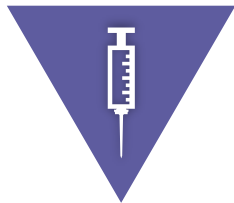
¹ Levy H, et al. *Clinical Chemistry*. 2023;69(5):433-434.
² Kozich V, Sokolova J, Morris AAM, et al. *J Inherit Metab Dis*. 2021;44(3):677-692.
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Pegtibatinase is an Investigational, Modified, Recombinant CBS Human Enzyme Therapy

Pegtibatinase is designed to address the underlying genetic cause of HCU



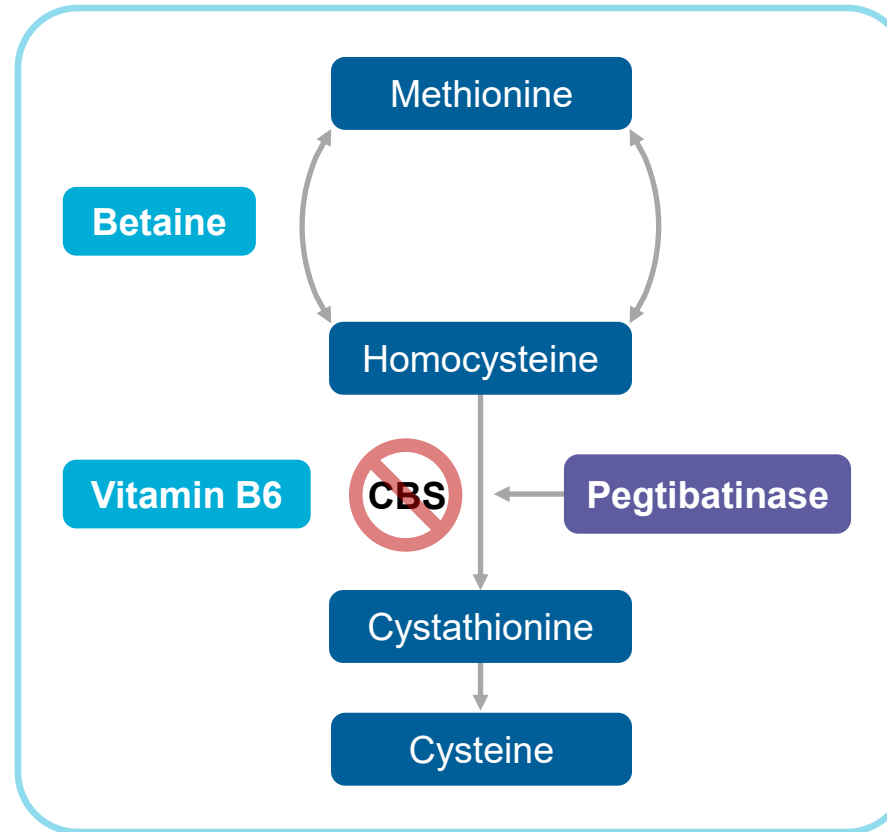
Mechanism of action is expected to have broad effect across HCU population



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 Pegtibatinate 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



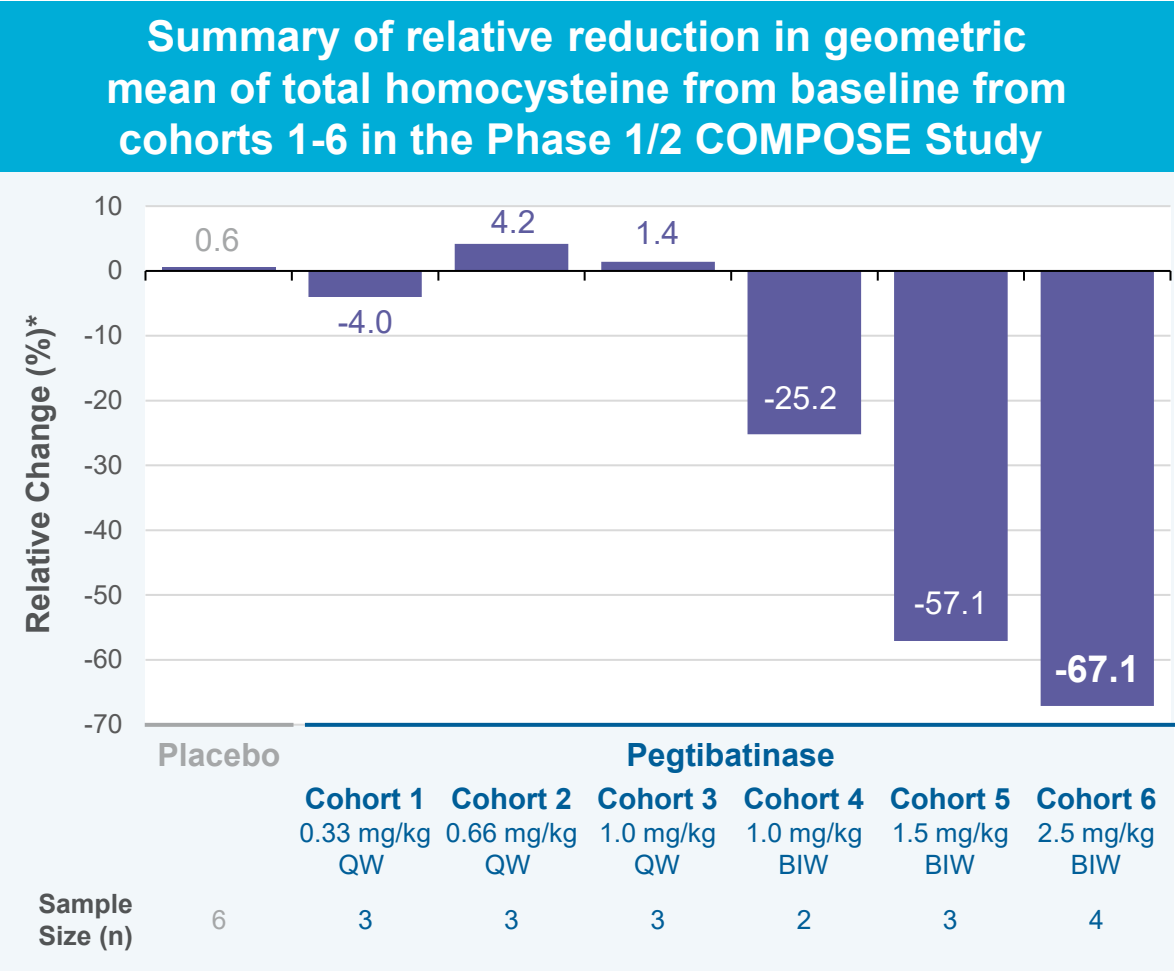
All patients in highest dose cohort achieved a clinically meaningful threshold in mean tHcy over weeks 6 to 12 of treatment



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



Pegtibatinate was generally well-tolerated at all doses tested

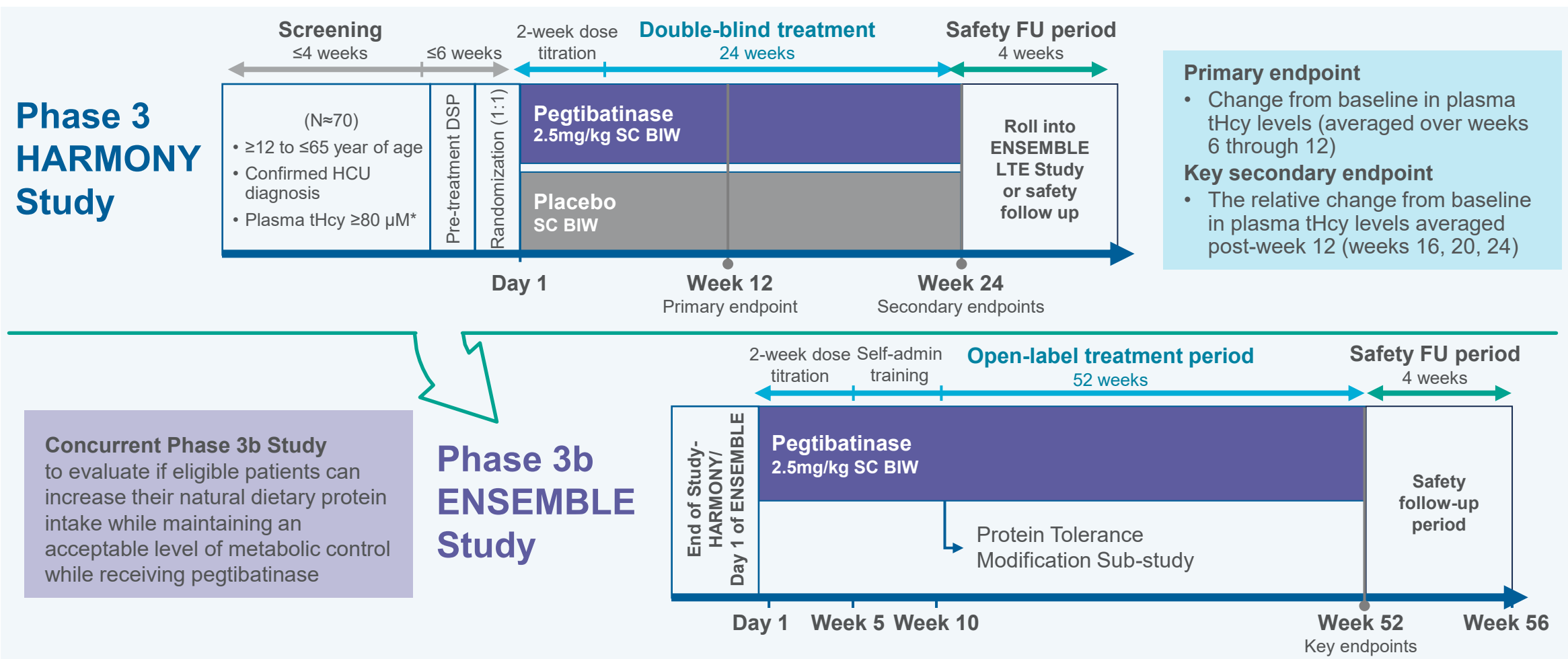


Abbreviations: QW: once weekly, BIW: twice weekly.

* 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 Pegtibatinate Phase 3 Program



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 ($<15 \mu\text{M}$) 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.



On track to restart enrollment in the Phase 3 HARMONY Study in 2026.

Financial Snapshot – Strong Balance Sheet to Support Sustainable Growth



~\$95M

in total U.S. net product sales in 2Q25; represents ~82% growth year-over-year



~89M

basic shares outstanding as of June 30, 2025; diluted ~104mm¹



~\$320M

in cash and cash equivalents as of June 30, 2025



~\$69M

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

¹ 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

- The only fully approved kidney-targeted therapy positioned to replace the historical standard of care in IgAN
- Final publication of the updated KDIGO guidelines expected to drive earlier intervention, strengthen FILSPARI's position
- Potential modification of liver monitoring and removal of pregnancy monitoring could ease access for certain patients – PDUFA target date of August 28, 2025



Position FILSPARI for a potential FDA approval and launch in FSGS

- PDUFA target action date of January 13, 2026
- FILSPARI could become the only FDA-approved medicine indicated for FSGS
- Leverage IgAN commercial success to prepare for commercial launch in FSGS, if approved



Advance pegtibatinase development

- Potential to become the only disease-modifying treatment for classical HCU
- Successfully optimize manufacturing scale up to restart enrollment in pivotal Phase 3 trial in 2026

Continued business development to further diversify pipeline



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