Retrophin

Corporate Overview

Delivering Life-Changing Therapies
to People Living with Rare Diseases

August 2020

Forward-Looking Statements

This presentation contains forward-looking statements, including statements about our prospects, products, growth projections, competitive position, potential regulatory filings and agency actions, and the anticipated development, timing, data readouts and therapeutic scope of programs in our clinical pipeline. These forward-looking statements may be accompanied by such words as "anticipate," "believe," "estimate," "expect," "forecast," "intend," "may," "plan," "project," "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, including the safety and efficacy of our product candidates, product competition, market acceptance, the occurrence of adverse safety events with our products or product candidates, clinical trials risk, adverse market and economic conditions, regulatory uncertainty, our dependence on collaborations and other third parties over which we may not always have full control, failure to comply with government regulation, our ability to protect our intellectual property rights, and have sufficient rights to market our products and services together with the cost of doing so, problems with our manufacturing processes and our reliance on third parties, our ability to attract and retain qualified personnel, our level of indebtedness, environmental risks, change of control provisions in our collaborations 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.

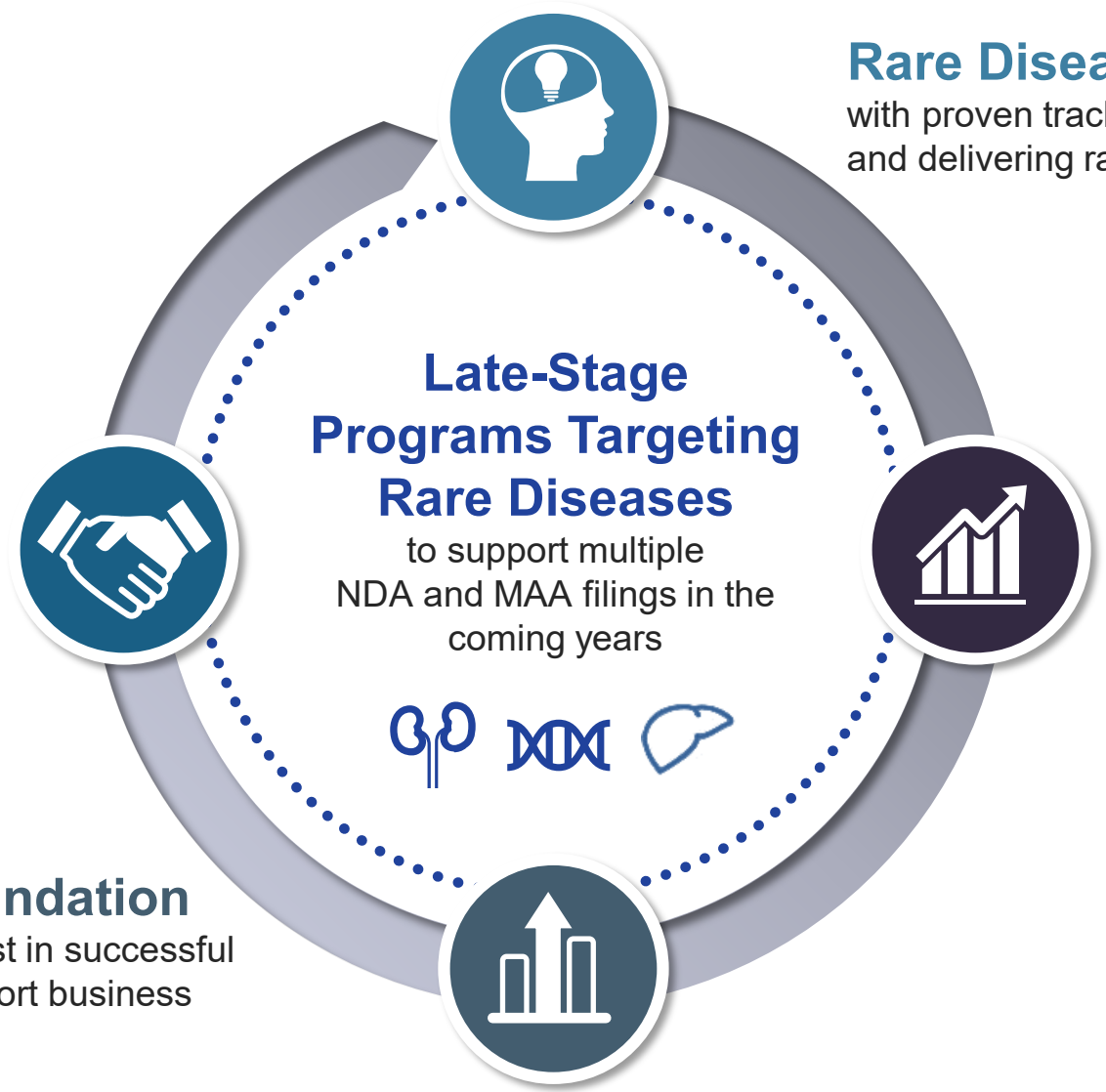
These statements are based on our current beliefs and expectations and speak only as of the date of this presentation. We do not undertake any obligation to publicly update any forward-looking statements.


Retrophin®
Our #1 priority will always be the patients we serve.
Retrophin is a biopharmaceutical company dedicated to identifying, developing and delivering life-changing therapies to people living with rare disease.

Retrophin: Innovating and Delivering Rare Disease Therapies

Rare Disease Expertise
with proven track record of developing and delivering rare disease therapies

Disciplined Business Development
to diversify and accelerate Retrophin's growth potential



Proven Commercialization Capabilities
with track record of delivering organic growth; strategic advantage when bringing our product candidates from the pipeline to patients

Strong Financial Foundation
to fund late-stage pipeline, invest in successful commercial launches, and support business development activities

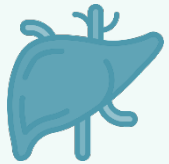
Our COVID-19 Response

- **Dedicated to supporting the needs of our team members, rare patients and caregivers**
  - Implemented work-from-home policy to safeguard our team members
  - Prioritizing heightened safety measures for patients in our clinical trials and receiving our approved products
  - Focused on maintaining continuity of supply chain
  - Created an emergency assistance fund to provide COVID-19 grant support for rare patient communities

- **Committed to our ongoing development plans and clinical programs**
  - Working in alignment with recent COVID-19 related FDA and EMA guidance
  - Prioritizing patient safety, continuous supply of investigational medicine, preserving trial conduct and documentation
  - We continue to believe the timelines for our pivotal Phase 3 studies are achievable
    - Seeing encouraging signs of clinical restrictions easing throughout global network

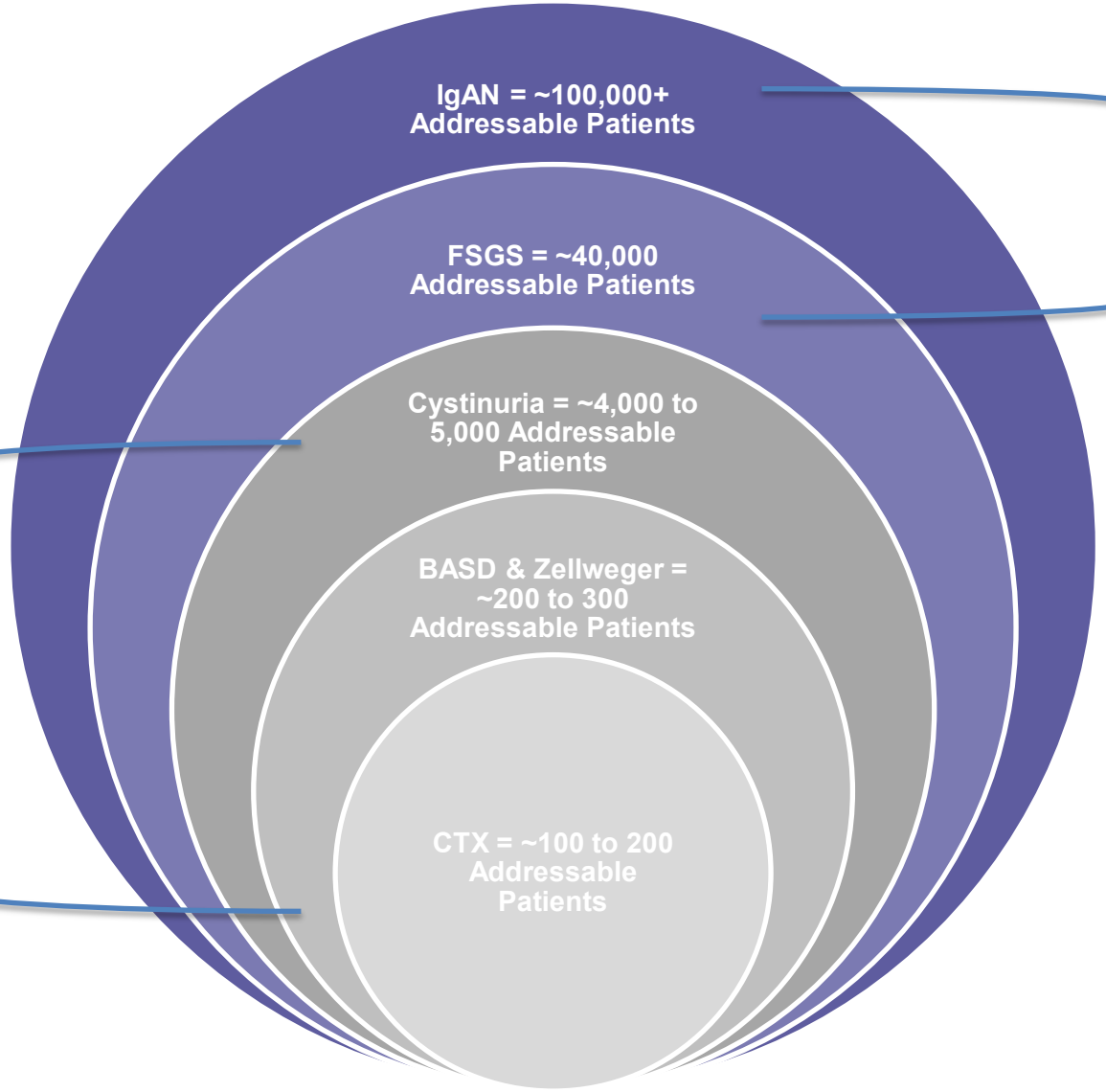
- **Identifying new patients and delivering our approved therapies**
  - Effectively supporting the needs of physicians through virtual interactions
  - Total Care HUB has remained fully operational and has been able to meet the needs of our patients during this time
  - Strong demand through early onset of pandemic; however, fewer patients may visit their HCPs during the year

Pipeline Advancing Programs With Potential To Be First Treatment Indicated for Two Rare Kidney Disorders

Therapeutic Area	Program	Indication	Pre-Clinical	Phase 1	Phase 2	Phase 3
Rare Nephrology 	Sparsentan	FSGS				
	Sparsentan	IgAN				
Rare Hepatology 	NIH CRADA	NGLY1 Deficiency				
	NIH CRADA	Alagille Syndrome				

Potential to Significantly Increase the Number of Patients Treated with Retrophin Therapies in the Coming Years

Potential for continued organic growth with the approved product portfolio



If approved for FSGS and IgAN, sparsentan provides a transformational opportunity to expand our growth in the coming years

Sparsentan

There is a Significant Need for Therapies in FSGS and IgAN

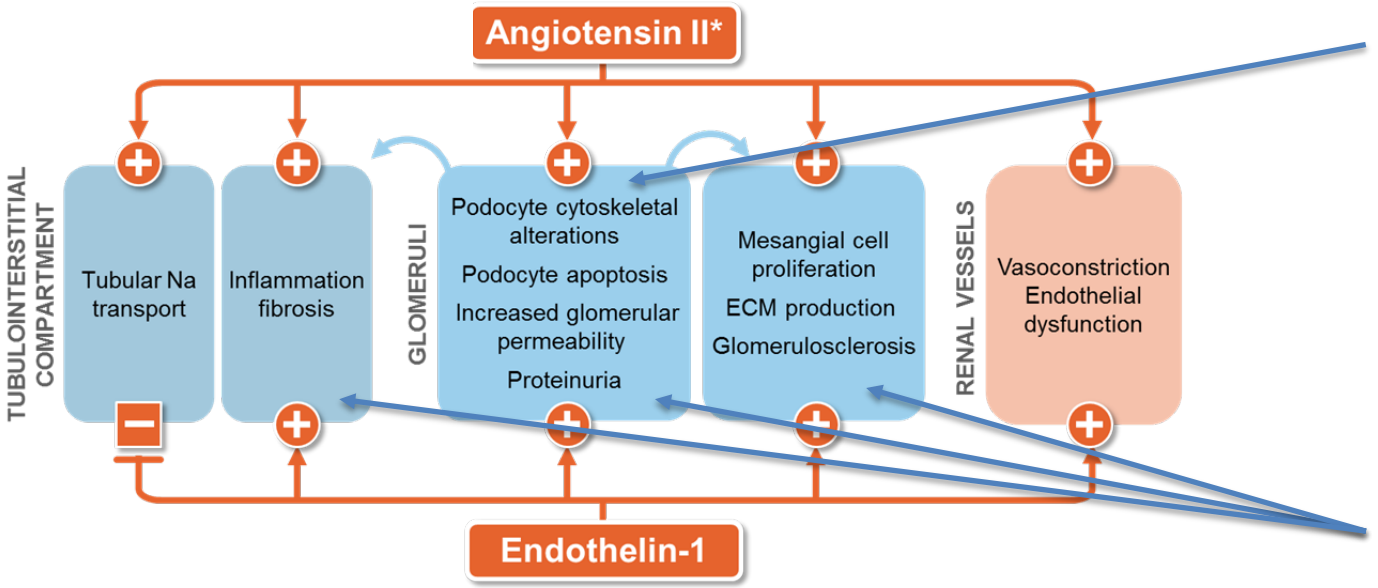
- **High unmet need to improve outcomes and delay progression to End Stage Renal Disease (ESRD) in focal segmental glomerulosclerosis (FSGS) and IgA nephropathy (IgAN)[1–3]**
  - An estimated 40,000 people are living with FSGS in the U.S. and a similar number estimated in Europe*
  - Estimated prevalence of more than 100,000 people with IgAN in the U.S., greater numbers in Europe and Asia*
- **These diseases reduce life expectancy and quality of life[1–5]**
  - Both FSGS and IgAN are characterized by renal function decline that is often recognized by elevated levels of protein leaking into the urine (**proteinuria**) and reduced glomerular filtration rate (**GFR**)
  - Up to 40% of patients diagnosed with FSGS and IgAN will progress to renal failure within 15 years requiring dialysis or transplantation[1–4]
- **As of August 2020 there are no FDA or EMA approved medicines indicated for FSGS or IgAN[1,4]**
  - Current standard of care in FSGS is steroids, ACE/ARBs, calcineurin inhibitors, dialysis, and renal transplant
  - Current standard of care in IgAN is RAAS blockade

*An estimated 50% of this patient population constitutes addressable market.

ESRD, end-stage renal disease; FSGS, focal segmental glomerulosclerosis; IgAN, immunoglobulin A nephropathy.

1. AZ Rosenberg & JB Kopp. *Clin J Am Soc Nephrol*. 2017; 12:502–517; 2. JC Rodrigues *et al. Clin J Am Soc Nephrol.* 2017; 12:677–686; 3. M-Y Wu *et al. J Clin Med* 2018; 7:225; 4. H Trachtman, *et al. J Am Soc Nephrol* 2018; 29:2745–2754. 5. Knoop, et al 2013

FSGS and IgAN Have Overlapping Pathologies



Primary FSGS
putative permeability factors

IgAN
glomerular immune-complex deposition

- **It has been well-established that stimulation of endothelin receptors (ET_A) and angiotensin II (AT_1) leads to detrimental effects on renal structure and function, and contributes to the development and progression of kidney disease**
- **Disorders are often characterized by progressive loss of functional glomerular tissue, defects in the glomerular filter function, and subsequent proteinuria**
- **Both FSGS and IgAN exhibit glomerulosclerosis and tubulointerstitial fibrosis**
  - Damaged glomeruli result in proteinuria and decreased GFR

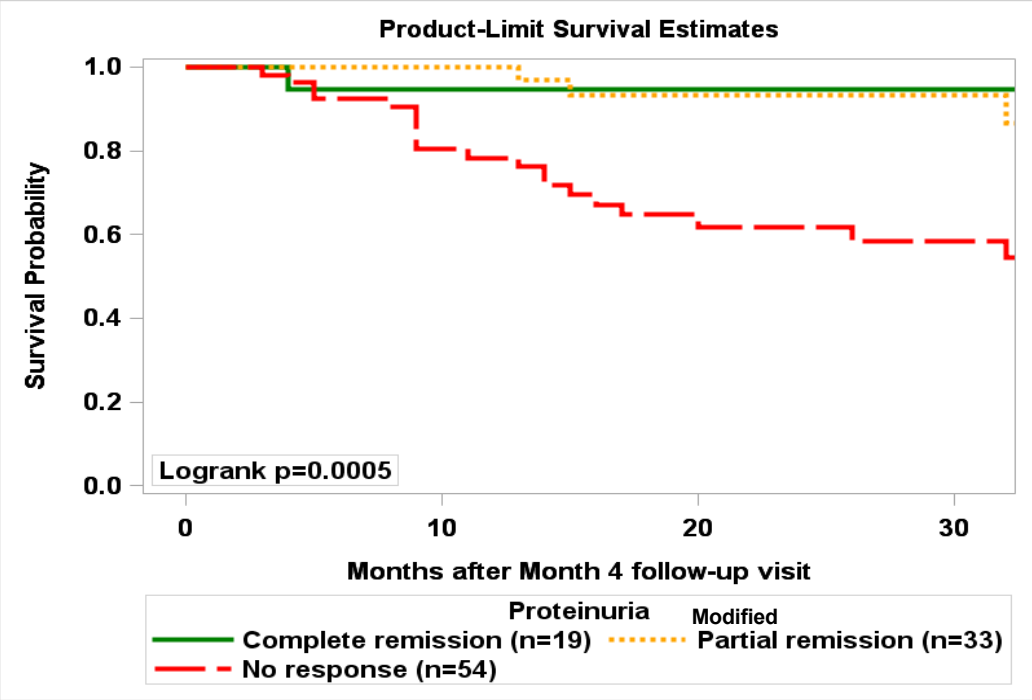
*Also applies to aldosterone. AT1, angiotensin II Type 1; ECM, extracellular matrix; ERA, endothelin receptor antagonist; ETA, endothelin type A; FSGS, focal segmental glomerulosclerosis; RAASi, renin-angiotensin-aldosterone system inhibitor. Image adapted from Komers R, Plotkin H. Am J Physiol Regul Integr Comp Physiol. 2016;310:R877-84

Significantly Reducing Proteinuria in FSGS Has Been Shown to Correlate With Improved Kidney Survival

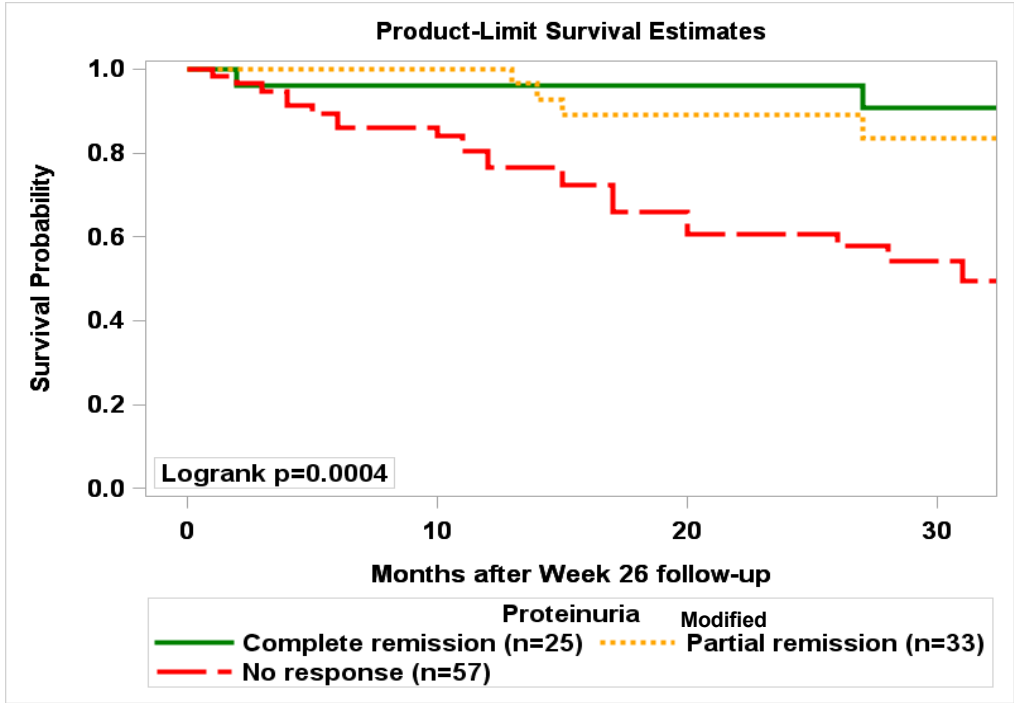
- **Analyses from the largest available clinical outcome assessment of proteinuria demonstrate kidney survival in patients achieving FSGS partial remission of proteinuria or complete remission, compared to no remission**
  - FSGS partial remission of proteinuria endpoint (FPRE): UP/C < 1.5 g/g and >40% reduction in UP/C
  - Complete remission: UP/C < 0.3 g/g

Proteinuria and Progression to Kidney Failure

NEPTUNE

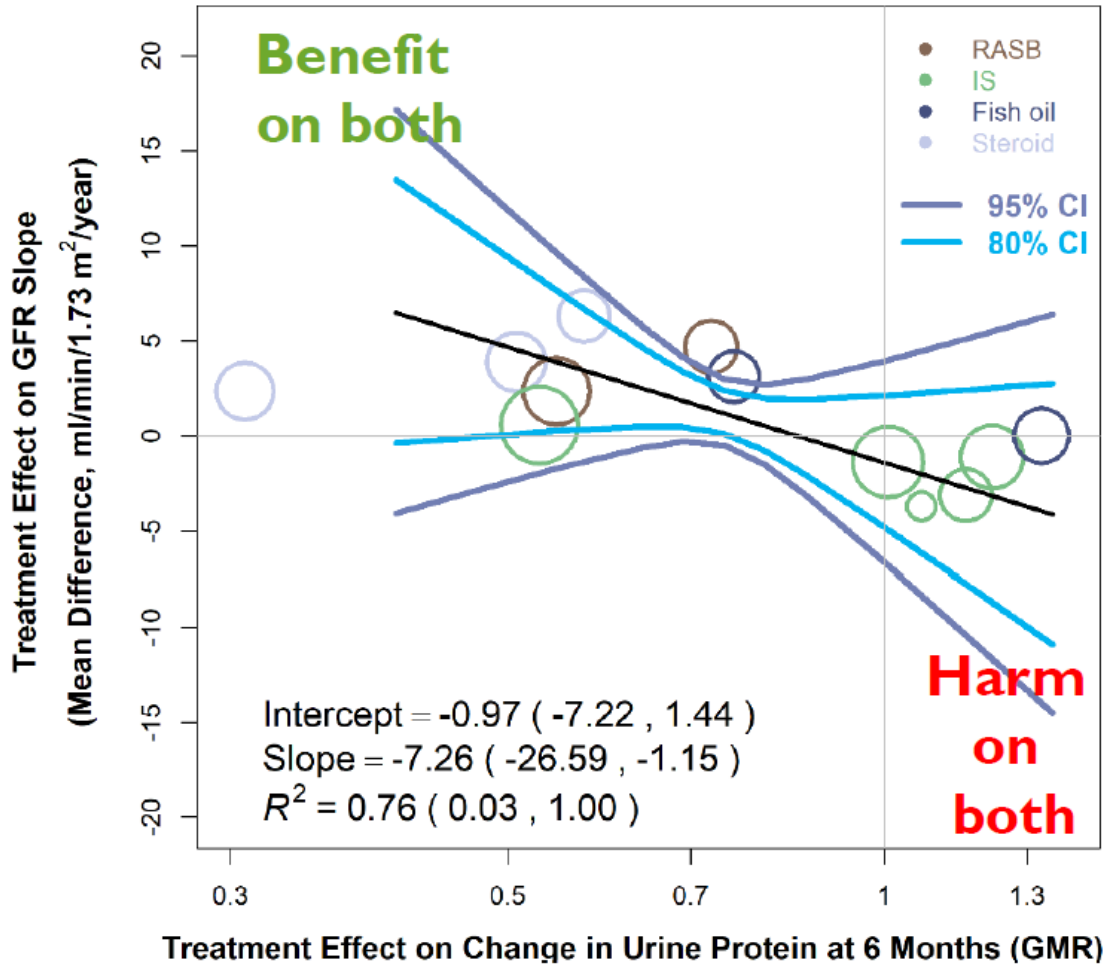


FSGS-CT



1. Troost JP, et al. A Clinical Outcome Assessment of Proteinuria in Patients with Focal Segmental Glomerulosclerosis. American Society of Nephrology Kidney Week; 2016. Abstract #FR-OR117.

Studies Suggested Greater Reduction in Proteinuria Also Resulted in Slower Progression of IgAN

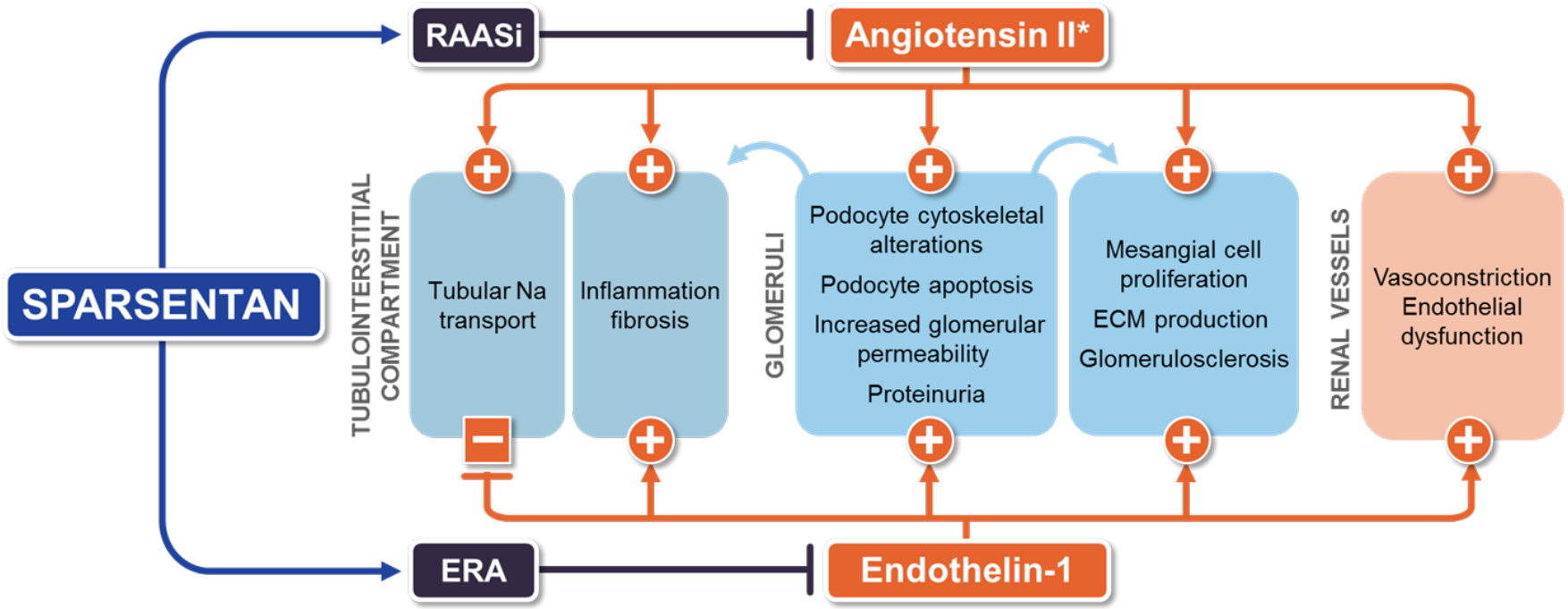


- **The most widely recognized and well-studied risk factor for progression to ESRD is proteinuria[1]**
- **Recent analyses of trial-level data from ~1,000 patients with IgAN show associations between treatment effect on change in proteinuria and rate of change in GFR[2]**
  - Predicted treatment effects on GFR slope were strongest for larger treatment effects on change in UP/C
  - KDIGO guidelines recommend antiproteinuric and antihypertensive therapy through renin-angiotensin system blockade as first-line therapy for IgAN patients with proteinuria >1 g/day[3]
- **It has been demonstrated that an oral selective ET_A receptor antagonist reduced proteinuria in patients with proteinuric nephropathy, including IgAN[3]**
  - These effects were seen in patients already receiving optimal treatment with ACE inhibitors and ARBs and were at least in part BP independent.

1. Thompson et al. CJASN 2019 2. Inker on behalf of CKD-EPI et al. ASN Kidney Week 2019; 3. KDIGO Clinical Practice Guidelines for Glomerulonephritis. Kidney Int Suppl. 2012;2(2):139-274 4. Dhaun et al. Hypertension. 2011;57:772-779;

Sparsentan – Dual Mechanism of Action With Potential to Confer Protective Actions in the Kidney and Reduce Proteinuria

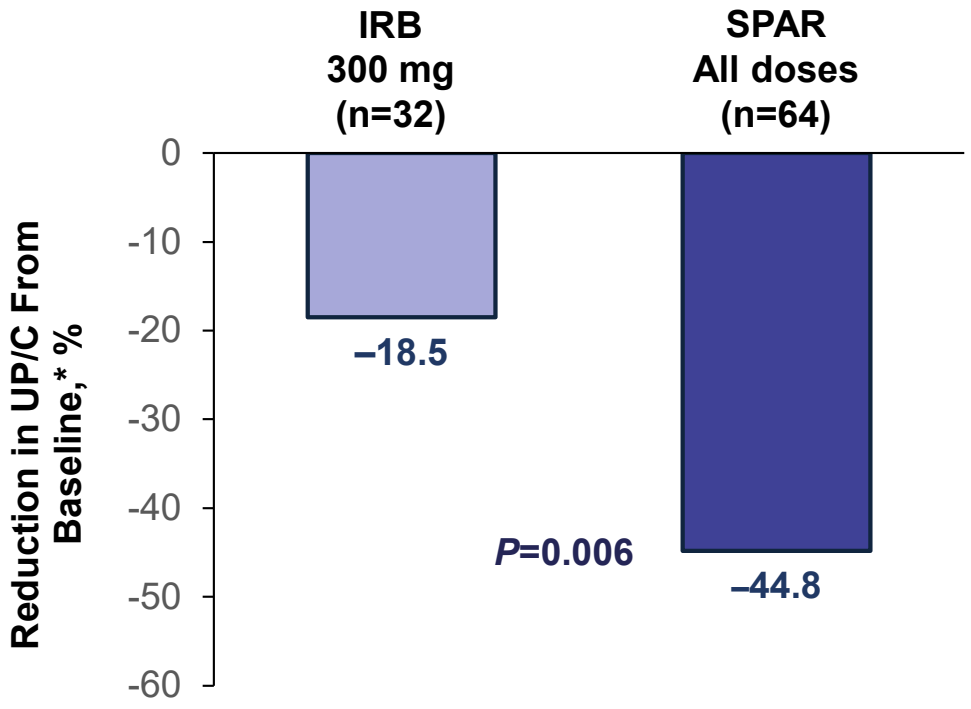
- **Dual mechanism:** designed to include orally active, endothelin type A (ET_A) + angiotensin receptor blocker (AT_1) activity in a single molecule; orphan drug designation for FSGS in U.S. and EU
- **Sparsentan has shown distinct selectivity:** high affinity selective antagonist at both the ET_A and AT_1 receptors with greater than 500-fold selectivity ET_A/ET_B
- **Generally well-tolerated to date:** evaluated in more than 500 subjects in nine Phase 1 or Phase 2 studies



Blocking both receptors may be more effective on the origins of underlying progressive kidney disease compared to either ET_A or AT_1 receptor alone

*Also applies to aldosterone. AT1, angiotensin II Type 1; ECM, extracellular matrix; ERA, endothelin receptor antagonist; ETA, endothelin type A; FSGS, focal segmental glomerulosclerosis; RAASi, renin-angiotensin-aldosterone system inhibitor. Image adapted from Komers R, Plotkin H. Am J Physiol Regul Integr Comp Physiol. 2016;310:R877-84

Phase 2 DUET Study: Overall Sparsentan Treatment Group Met Primary Endpoint; More Than Doubled Reduction of Proteinuria vs. Irbesartan



Significant reduction in proteinuria after 8 weeks of sparsentan vs irbesartan treatment in primary and genetic FSGS patients

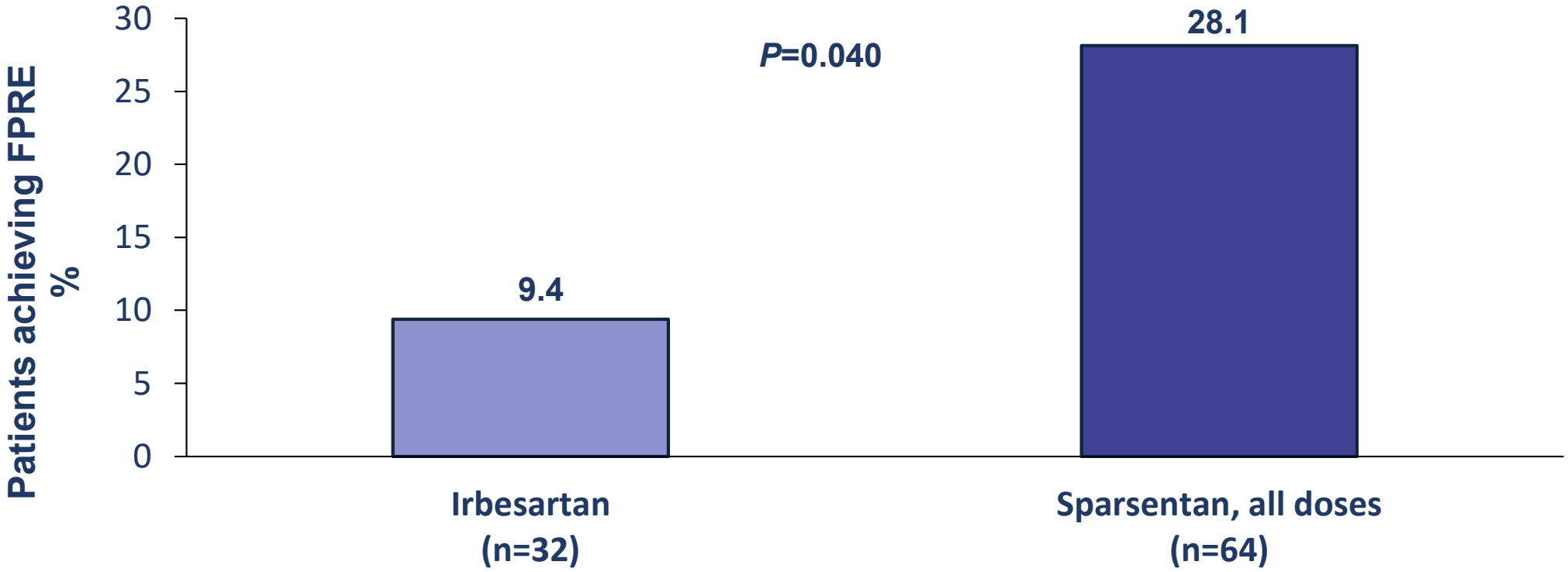
TEAE	Patients with TEAEs During the Double-Blind Period, %	
	IRB (n = 36)	SPAR, All Doses (n = 73)
Any	72.2	76.7
Drug-related	36.1	43.8
Serious	2.8	2.7
Leading to dose change or interruption	8.3	23.3
Leading to drug discontinuation	2.8	4.1
Leading to study withdrawal	2.8	2.7
Death	0	0

Similar incidence of TEAEs between irbesartan and sparsentan-treated patients

*Geometric least squares mean reduction. P values from analysis of covariance. Analyses based on the efficacy evaluable set. UP/C based on 24-hour urine. Individual dose cohorts showed clear signals of relative improvement, but did not reach statistical significance; H Trachtman, *et al*. *J Am Soc Nephrol* 2018; 29:2745–2754. Loreto G, *et al. ERA-EDTA* 2017 Oral presentation TO042

Promising Response in FPRE During DUET Study

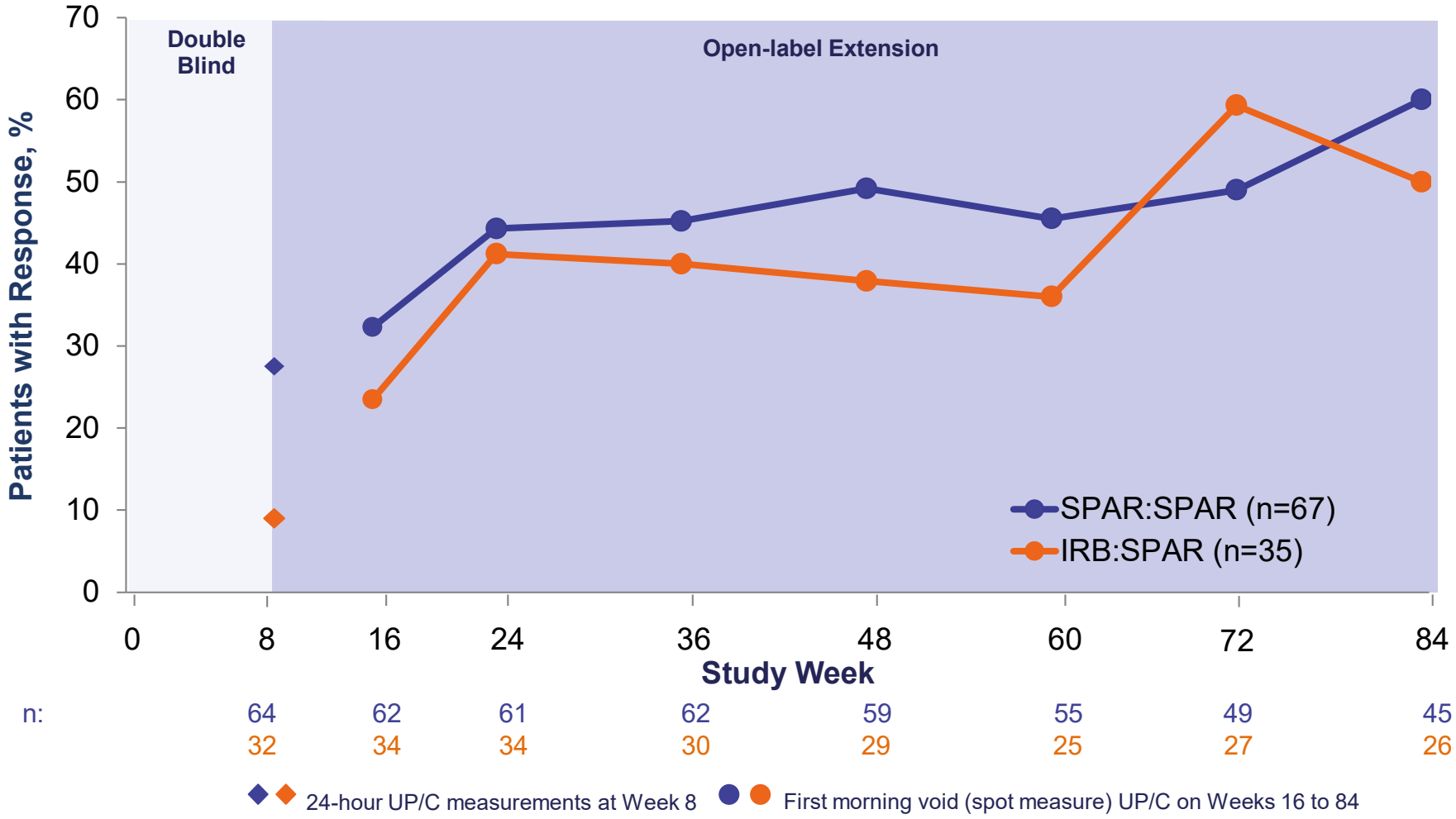
- FPRE defined as urinary protein-to-creatinine ratio (UP/C) ≤ 1.5 g/g and > 40% reduction in UP/C



- **Reaching either a complete remission (defined as UP/C < 0.3 g/g) or FPRE has been associated with better long-term outcomes in patients with FSGS[1]**
  - After 8-weeks, four sparsentan-treated patients achieved UP/C of 0.3 g/g or less vs. 0 irbesartan-treated patients

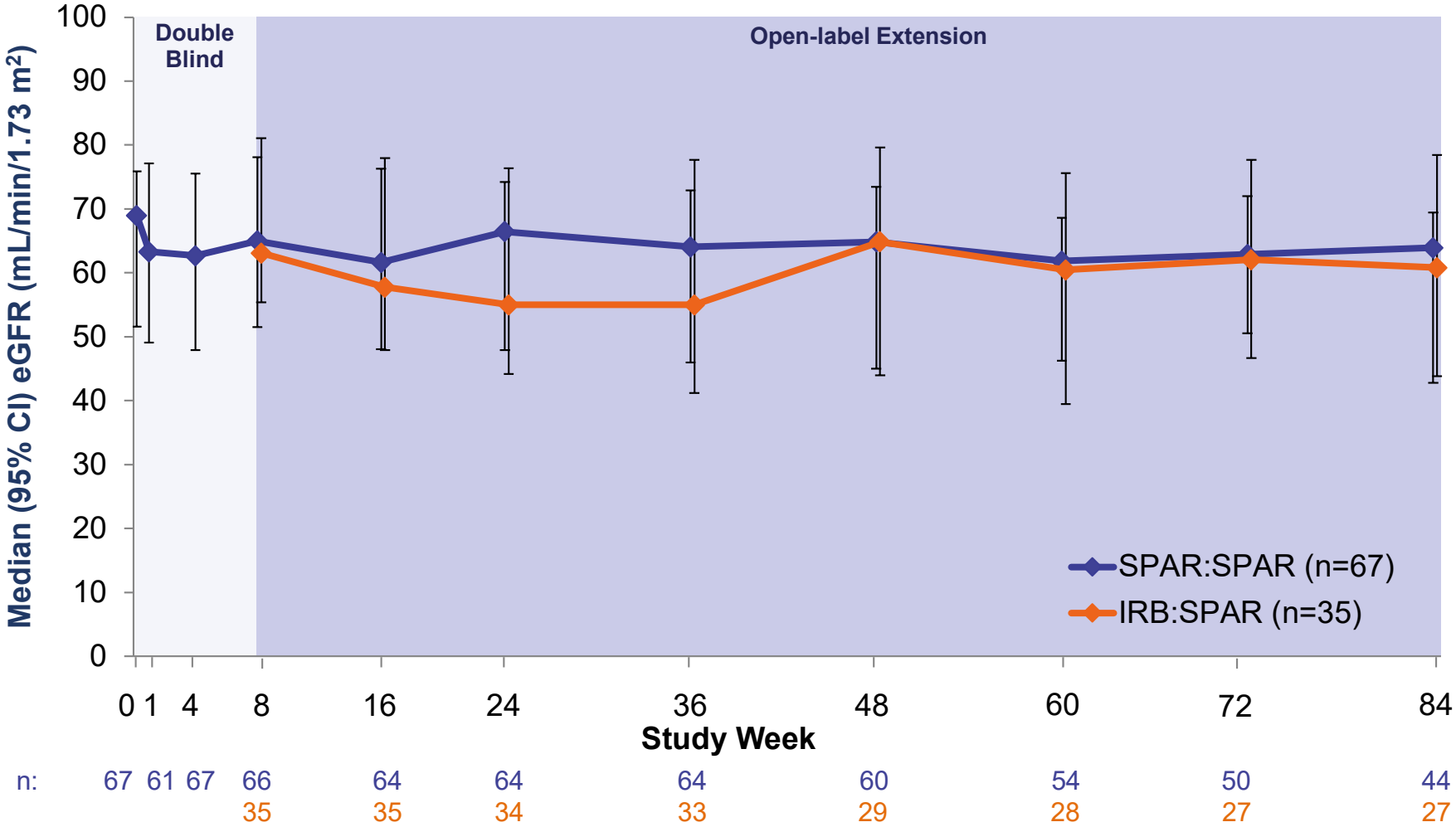
1. Troost JP, et al. An Outcomes-Based Definition of Proteinuria Remission in Focal Segmental Glomerulosclerosis. Clin J Am Soc Nephrol 13 2017.

Increasing Proportion of Patients Achieved FPRE During Open Label Period



ASN 2018 Oral Presentation, Long-term Effects of Sparsentan, a Dual Angiotensin and Endothelin Receptor Antagonist in Primary Focal Segmental Glomerulosclerosis (FSGS): Interim 84-Week Analysis of the DUET Trial - FPRE is defined as UP/C ≤1.5 g/g and >40% reduction in UP/C from baseline. Baseline in the double-blind period defined as Week 0; baseline for the open-label period defined as last observation before start of open-label sparsentan treatment (ie, Week 8). Data for Week 8 is based on the efficacy evaluable set. Data for Weeks 16 to 84 are based on the full analysis set.

eGFR Remained Stable in Sparsentan-Treated Patients Over 84 Weeks

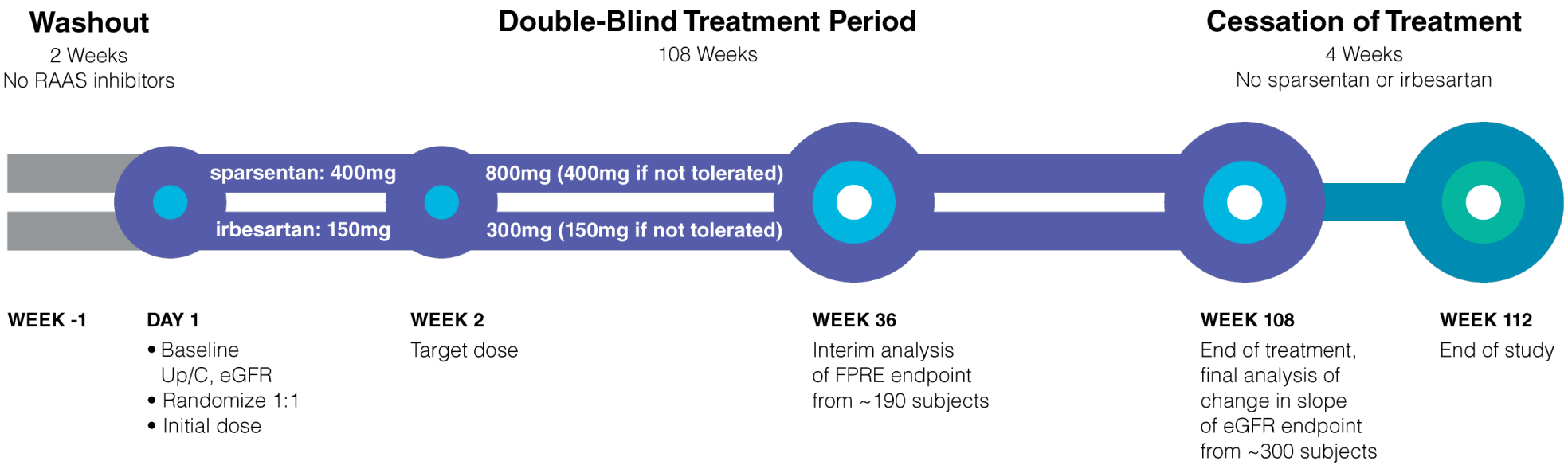


The observed beneficial effects of sparsentan on proteinuria were associated with stable estimated glomerular filtration rate (eGFR) during the open label period

- The median change from baseline experienced some variability in both groups, reflecting expected hemodynamic effects in patients with hyperfiltration

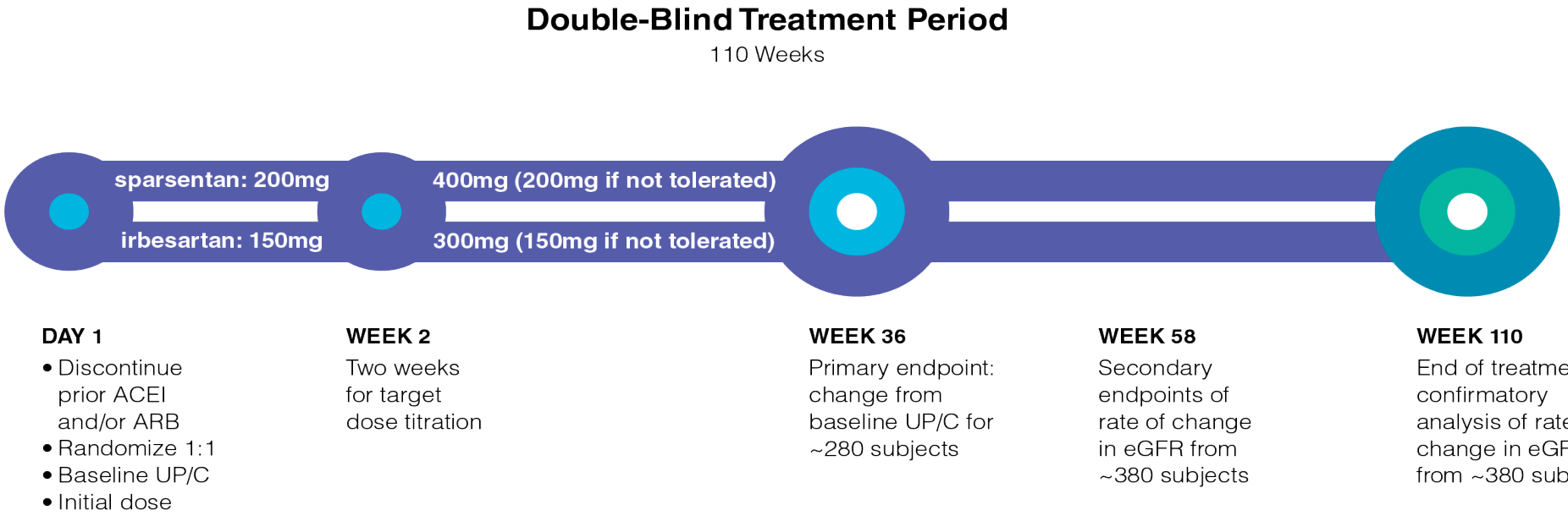
ASN 2018 Oral Presentation, Long-term Effects of Sparsentan, a Dual Angiotensin and Endothelin Receptor Antagonist in Primary Focal Segmental Glomerulosclerosis (FSGS): Interim 84-Week Analysis of the DUET Trial - Based on the full analysis set. 95% CIs were calculated for the median value at each time point. Note: There were 9 study withdrawals due to renal causes during the follow-up period, including 1 case of end-stage renal disease.

Enrolling Phase 3 DUPLEX Study to Support Registration of Sparsentan for FSGS in U.S. and EU



- **Global, randomized, multi-center, double-blind, parallel-arm, active controlled pivotal Phase 3 clinical trial designed in alignment with FDA and EMA feedback**
- **The DUPLEX Study reached enrollment of the first 190 patients in March 2020 - successful achievement of the 36-week proteinuria endpoint for ~190 patients is expected to serve as the basis for submission of an NDA under the Subpart H accelerated approval pathway in the U.S. and CMA consideration in Europe**
- **We currently believe top-line data from the 36-week proteinuria endpoint analysis remain achievable in 1Q21, but we are continuing to monitor the impact of the evolving COVID-19 pandemic**

Enrolling Phase 3 PROTECT Study to Support Registration of Sparsentan for IgAN in U.S. and EU



- **Global, randomized, multi-center, double-blind, parallel-arm, active controlled pivotal Phase 3 clinical trial designed in conjunction with FDA and EMA feedback**
- **Successful achievement of the 36-week proteinuria endpoint for ~280 patients is expected to support submission of an NDA under the Subpart H accelerated approval pathway in the U.S., as well as an application for CMA consideration in Europe**
- **We currently believe top-line data from the 36-week proteinuria efficacy analysis are achievable in 2H21, but we are continuing to monitor the impact of the evolving COVID-19 pandemic**

Commercial Portfolio & Financial Profile

Diversified Product Portfolio for Rare Diseases

CHENODAL™
(Chenodiol Tablets 250 mg)

- **Chenodal is a synthetic bile acid approved for the treatment of gallstones, but usage is primarily in cerebrotendinous xanthomatosis (CTX)**
  - CTX is a rare autosomal recessive lipid storage disease with multi-organ onset
  - Chenodal is not labeled or marketed for CTX
- **Retrophin is seeking U.S. regulatory approval for the addition of a CTX indication to the Chenodal label**
  - The Company recently randomized the first patients in the Phase 3 RESTORE Study, which is anticipated to ultimately support an NDA submission for marketing authorization of Chenodal for CTX in the United States

Cholbam™
(cholic acid) capsules

- **Cholbam is a cholic acid capsule approved for pediatric and adult patients with bile acid synthesis disorders due to single enzyme defects and Zellweger Spectrum Disorders (ZSDs)**
  - If untreated, patients fail to grow and may develop life-threatening liver injury, potentially resulting in liver transplant or death
- **Treatment with Cholbam may:**
  - Prevent progression of liver disease
  - Normalize liver biochemical and histological abnormalities
- **An estimated addressable population of 200 to 300 patients**

Thiola® (tiopronin) tablets
Rx Only

Thiola® EC (tiopronin)
Delayed-Release Tablets 100mg|300mg

- **Thiola is FDA-approved for the prevention of cystine stone formation**
  - Not a typical kidney stone; generally does not respond to lithotripsy
  - An estimated average of one surgical procedure every 3 years, with 7 surgical procedures by middle age
- **An estimated addressable population of 4,000 to 5,000 cystinuria patients**
- **Thiola EC became available in July 2019 and offers the potential for administration with or without food, and the potential to reduce the number of tablets necessary to manage cystinuria**

Strong Commercial Capabilities Provide Robust Foundation in Nephrology to Launch Sparsentan, if Approved

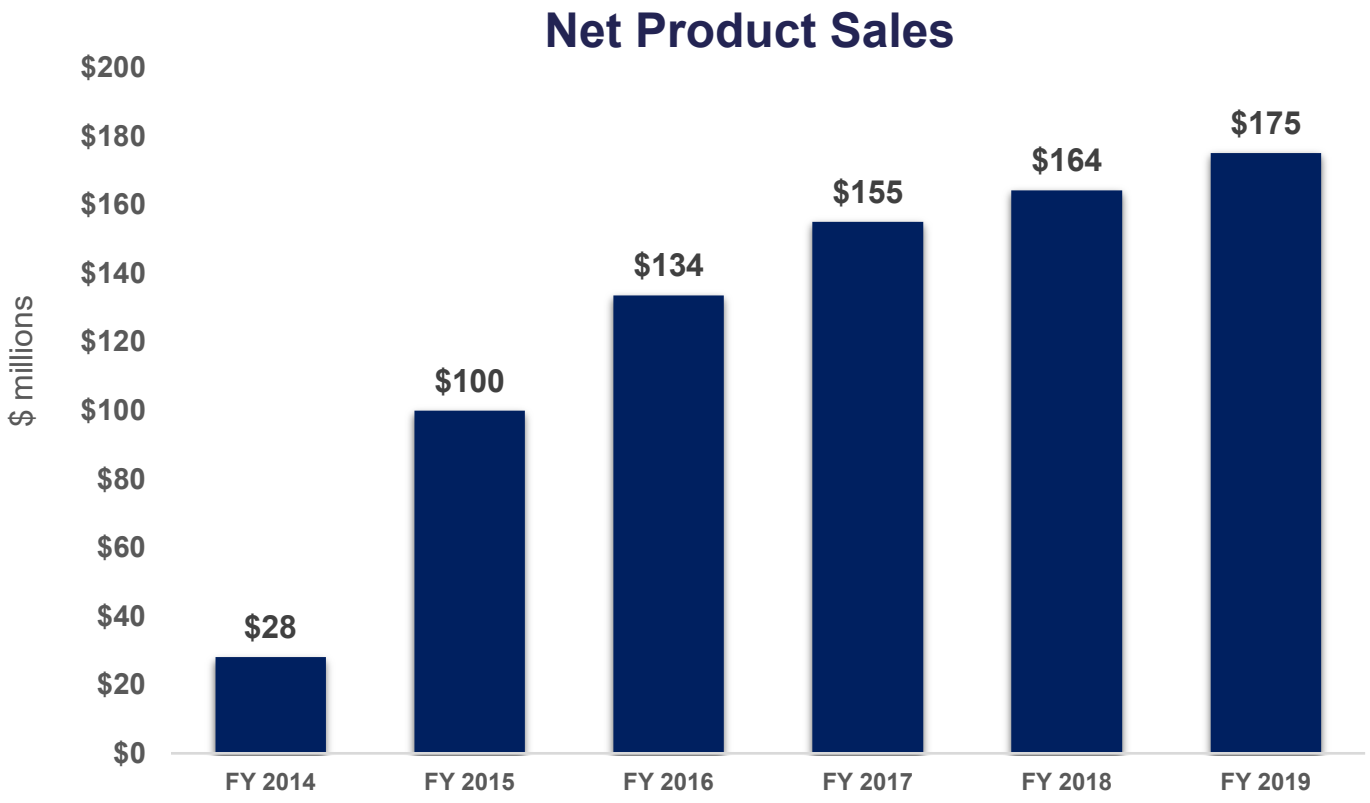
- **Dedicated sales teams with extensive experience in rare disease**
- **Multiple successful product launches – Thiola EC launch progressing well with strong patient engagement and payer access**
- **Dedicated Total Care Hub to ensure:**

- Broad access

- 24/7 counseling and support

- Direct delivery to patients

- Educational resources for patients and HCPs



Five-year history of identifying and treating new patients

Retrophin Financial Profile

GAAP Reported Financials	2Q20	FY19	FY18	FY17	FY16
Net Sales	$48.4mm	$175.3mm	$164.2mm	$154.9mm	$133.6mm
Operating Expenses	$71.5mm	$312.7mm	$244.3mm	$208.7mm	$191.8mm
Operating Income / (Loss)	($23.1mm)	($137.4mm)	($80.0mm)	($53.8mm)	($58.2mm)
Net Income / (Loss)	($26.1mm)	($146.4mm)	($102.7mm)	($59.7mm)	($47.9mm)
Cash and Equivalents*	$457.4mm	$398.5mm	$471.5mm	$300.6mm	$255.9mm

- **Basic shares outstanding as of June 30, 2020 ~50.9mm**
- **Convertible notes: $276 million due September 2025**

*Includes available-for-sale securities

Retrophin: Innovating and Delivering Rare Disease Therapies

Positively transforming the lives of people with rare diseases

- We focus on differentiated therapeutics in areas of high unmet medical needs
- Our team has a proven track record of developing and commercializing rare disease therapies

Sparsentan is advancing in Phase 3 development for indications with no approved medicines

- Robust Phase 3 studies ongoing to support NDA/CMA submissions for FSGS and IgAN starting next year
- Potential to reach an aggregate of ~40,000 patients with FSGS in U.S. and EU; ~100,000+ patients with IgAN in U.S. and EU

We have a strong financial foundation to execute on our strategy

- Cash balance of ~$457.4mm as of 6/30/20 anticipated to be sufficient to fund operations beyond the planned proteinuria readout of our pivotal studies and into 2023
- Potential for business development to further diversify and expand pipeline

Retrophin