Retrophin

Corporate Overview

Delivering Life-Changing Therapies to People Living with Rare Diseases

June 2017

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.

Our Mission

Retrophin is a fully integrated biopharmaceutical company dedicated to delivering life-changing therapies to people living with rare diseases who have few, if any, treatment options. The Company is advancing a robust, clinical-stage pipeline, including sparsentan for FSGS and RE-024 for PKAN, supported by revenues from the Company's commercial products Thiola®, Cholbam®, and Chenodal®.

Retrophin: Innovating and Marketing Rare Disease Therapies

Innovative, Late-Stage Product Pipeline	• Research and development efforts largely focus on clinical-stage, disease-modifying assets that address rare diseases with no effective therapeutic options • Two product candidates entering Phase 3 development; expected to drive substantial future value
Revenue-Generating Commercial Portfolio	• Three commercial products with strong year-over-year organic growth trends • Full year 2016 revenue of $134mm; Full year 2017 revenue expected to be $150mm to $160mm
Focused Business Development Strategy	• Actively exploring strategic business development options, including external partnerships, acquisitions, and out-licensing opportunities • Strong balance sheet to support BD strategy

Development Pipeline & Product Portfolio

Program / Product	Indication	Pre-Clinical	Phase 1	Phase 2	Phase 3	Marketed
Sparsentan (RE-021)	FSGS	→	→	→		
RE-024	PKAN	→	→	→		
NGLY1 Collaboration & Discovery Efforts	NGLY1 Deficiency	→				
Thiola® (tiopronin) tablets	Cystinuria	→	→	→	→	→
Cholbam® (cholic acid) capsules	Bile Acid Synthesis Disorders Due to Single Enzyme Defects	→	→	→	→	→
	Zellweger Spectrum Disorders (ZSDs)	→	→	→	→	→
CHENODAL™ (Chenodiol Tablets 250 mg)	Gallstones/CTX*	→	→	→	→	→

**Indicated for gallstones; recognized as standard of care for CTX*

Development Pipeline Highlights & Milestones

	2015-2017	Future Milestones
Sparsentan	✓ Granted both U.S. and EU orphan drug designation for treatment of FSGS ✓ Positive results from Phase 2 DUET study reported ✓ End of Phase 2 meeting provided regulatory guidance for pivotal Phase 3 study ✓ U.S. and European patents granted; exclusivity expected to at least 2030	• Finalize Phase 3 protocol and confirm with FDA in 2H17; expect to initiate trial thereafter • Potential to develop sparsentan in additional glomerular nephropathies • Interim analysis expected to serve as basis for NDA filing for accelerated approval
RE-024	✓ Granted both US and EU orphan drug designation for treatment of PKAN ✓ Fast Track designation in the U.S. for treatment of PKAN ✓ Positive case report data presented at ACMG in March and MDS in June 2016 ✓ Reached SPA agreement on pivotal Phase 3 trial with FDA	• First patient dosed in pivotal FORT Phase 3 trial expected mid-year 2017 • Complete enrollment of FORT study • Potential NDA filing

Sparsentan

Focal Segmental Glomerulosclerosis (FSGS)

- **Disease where the glomeruli become progressively scarred and the kidney is dysfunctional**
  - Damaging symptoms including large amounts of protein leaking into the urine (proteinuria); increased risk of infection and blood clots
- **No FDA-approved treatments for FSGS**
  - Current standard of care: steroids, ACE/ARBs, calcineurin inhibitors, dialysis, and renal transplant
- **Many FSGS patients will eventually progress to End-Stage Renal Disease (ESRD)**
  - ~19,306 people are living with ESRD caused by FSGS
  - ~1,000 FSGS patients a year receive kidney transplants

Incidence & Prevalence

- **There are up to 40,000 FSGS patients in the U.S.**
- **~5,400 patients are diagnosed with FSGS every year**
- **Prevalence is increasing**

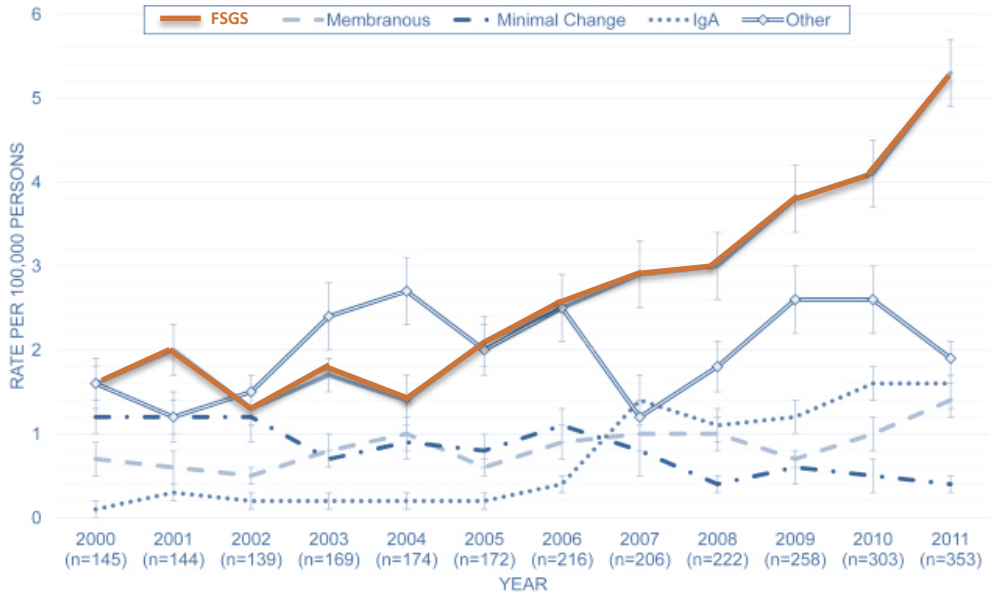

Figure is reprinted from Sim JJ, et al. *Am J Kidney Dis.* 2016;68:533-544. doi: 10.1053/j.ajkd.2016.03.416. Open access.

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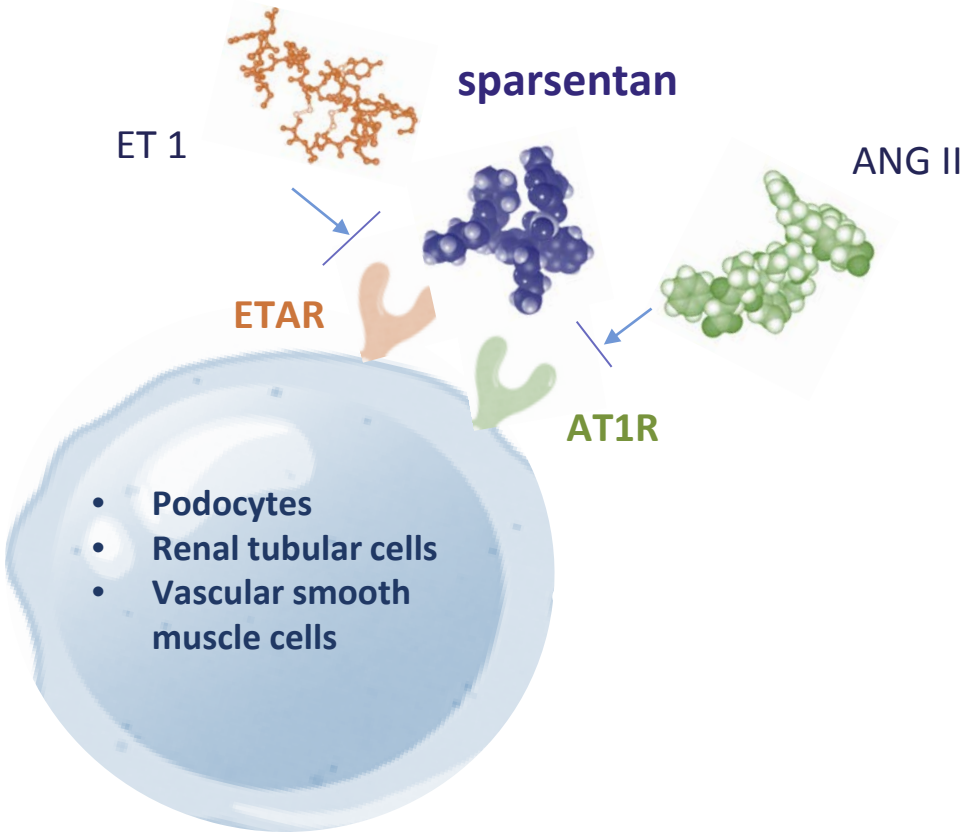
Sparsentan – Dual Mechanism of Action

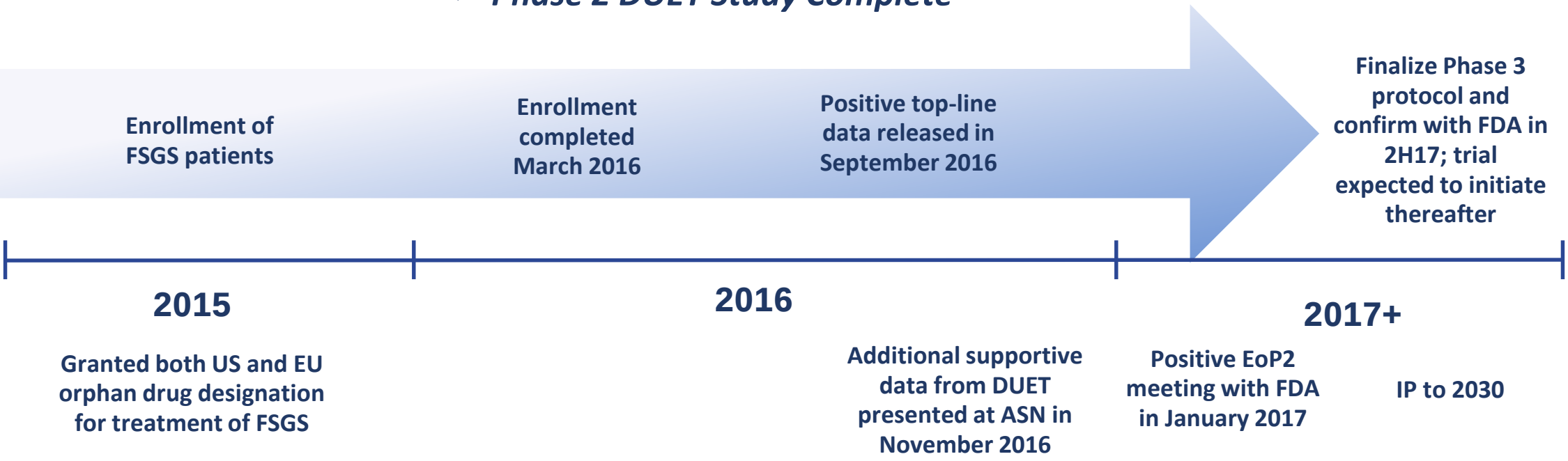
- **Dual mechanism: endothelin type A (ETA) activity + angiotensin receptor blocker (ARB)**
- **Sparsentan has a unique profile: believed to have high selectivity for endothelin type A receptor (ETAR) blockade combined with angiotensin receptor blockade**
- **Well-tolerated with favorable safety profile in over 500 subjects in nine Phase 1 or Phase 2 studies conducted by Bristol Myers Squibb (BMS)**



- ETA: emerging evidence of protective effects in kidney and cardiovascular system
- ARB: established evidence of protective effects in kidney and cardiovascular system

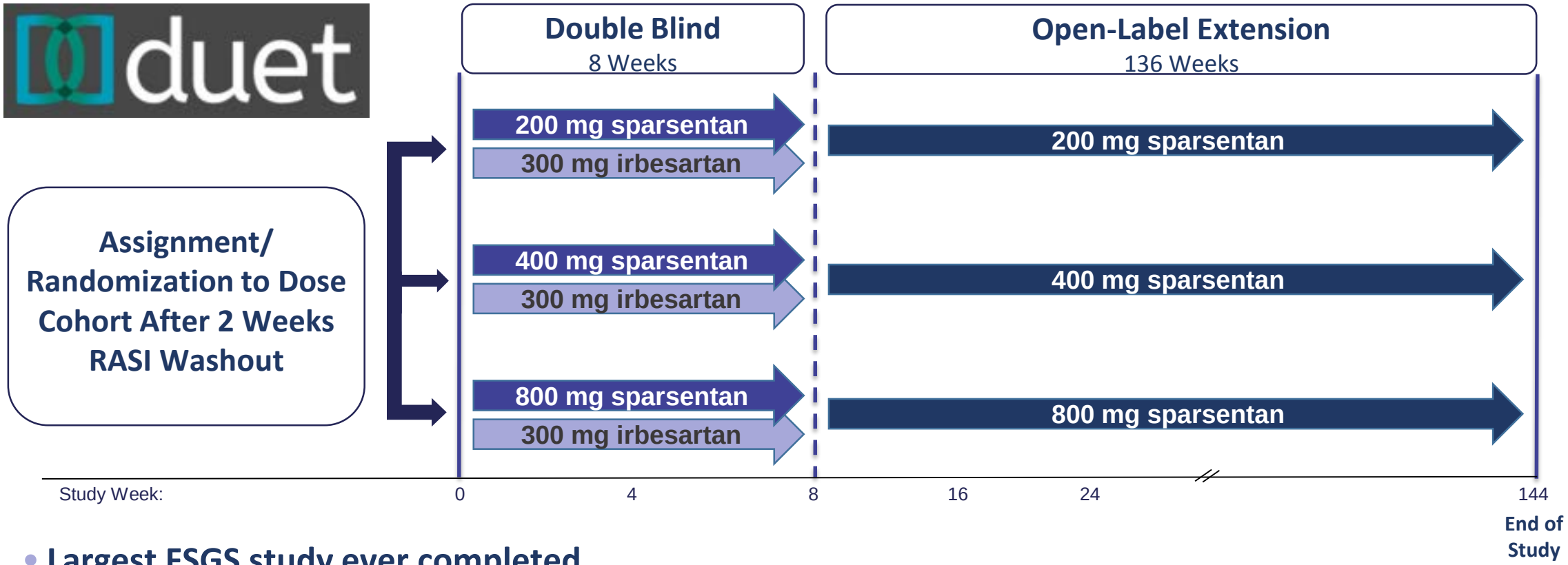
Sparsentan Advancing To Phase 3

✓ ***Phase 2 DUET Study Complete***



- **Strong intellectual property estate expected to provide market exclusivity to at least 2030**
  - U.S. and Europe patents directed to the use of sparsentan for treating glomerulosclerosis, including FSGS, issued in May 2017; stated expiration of March 2030 with potential for extension
  - Orphan designation in both the U.S. and Europe

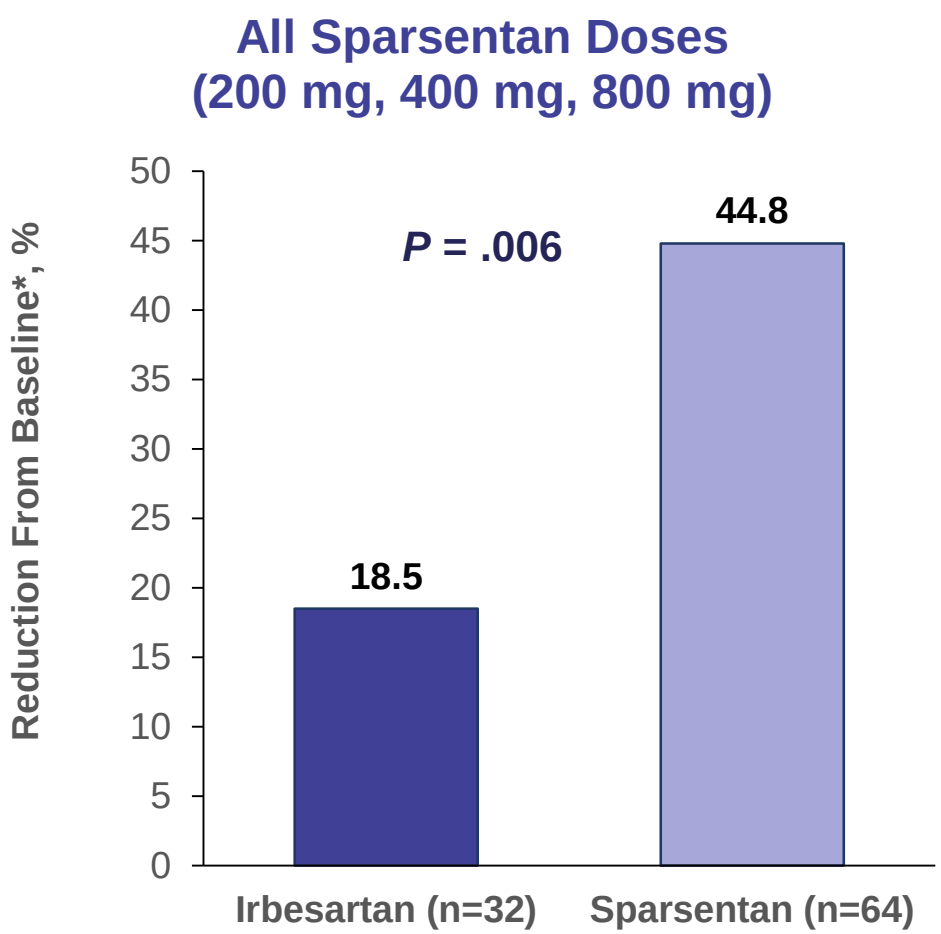
Sparsentan Phase 2 Study – DUET

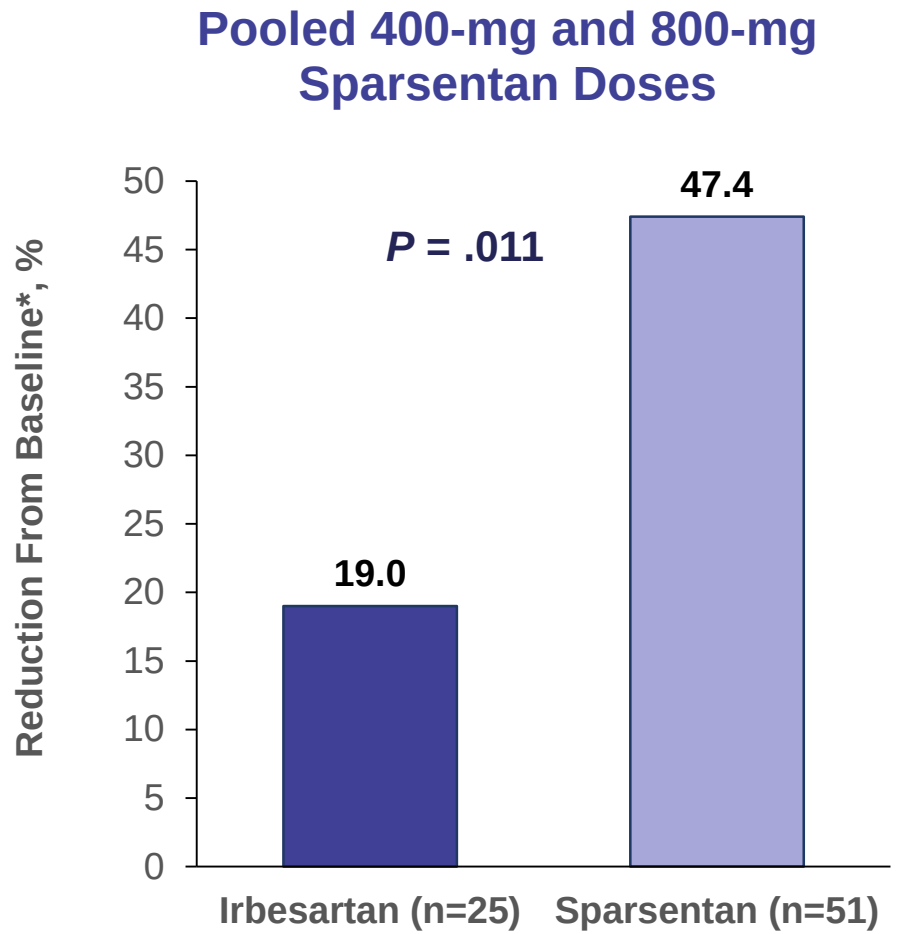


- **Largest FSGS study ever completed**
  - N=109 (96 evaluable for efficacy); 40+ U.S. and E.U. sites
- **Reduction of proteinuria at 8 weeks was primary endpoint**

Patients were assigned to dose cohort, then randomized to sparsentan or irbesartan within the dose cohort
Study drug administered orally, once daily. Patients who weighed < 50 kg received half of the daily dose of sparsentan or irbesartan according to the assigned dose cohort. RASI = renin-angiotensin system inhibitor.

DUET: Sparsentan More Than Doubled Reduction of Proteinuria vs. Irbesartan





- **Individual dose cohorts showed clear signals of relative improvement, but did not reach statistical significance**

*Geometric least squares mean reduction. *P* values from analysis of covariance. Analyses based on the EES.

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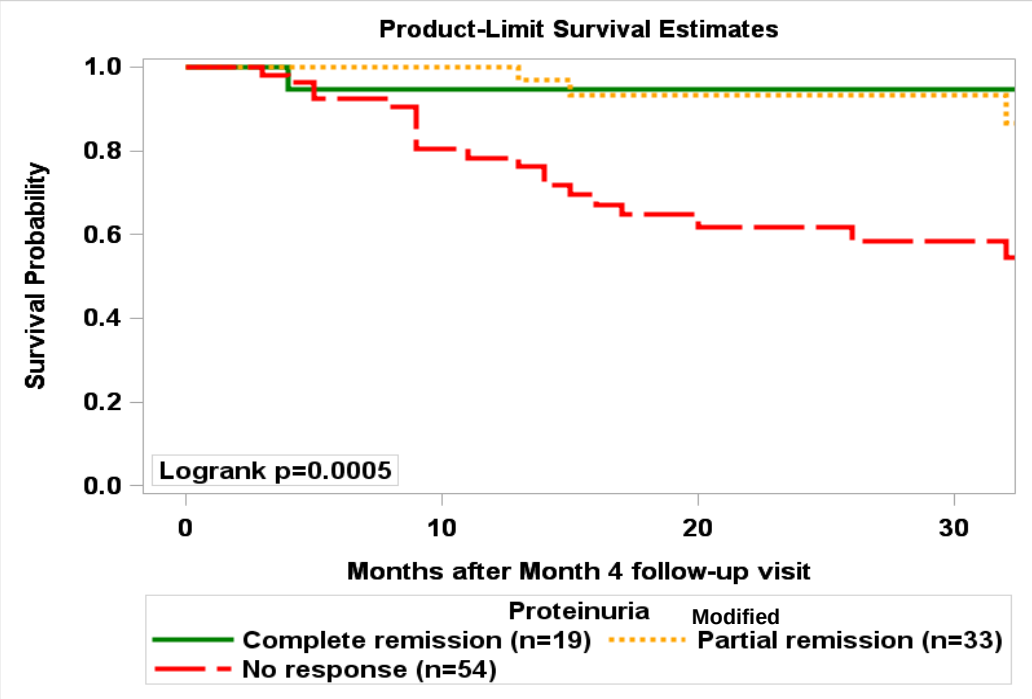
Modified Partial Remission Leads to Better Outcomes

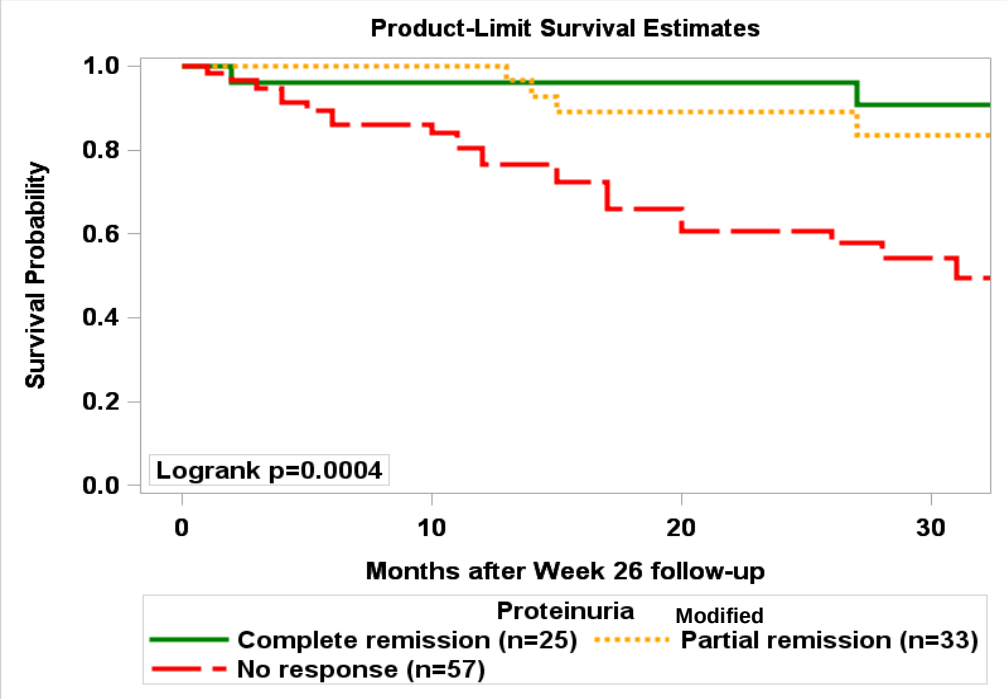
- **Secondary endpoint based on data from the Nephrotic Syndrome Study Network (NEPTUNE) and FSGS Clinical Trial (FSGS-CT)[1]**
  - Complete remission: UPC < 0.3 g/g
  - Modified partial remission (mPR): UPC < 1.5 g/g and 40% reduction in UPC

Proteinuria and Progression to Kidney Failure

NEPTUNE



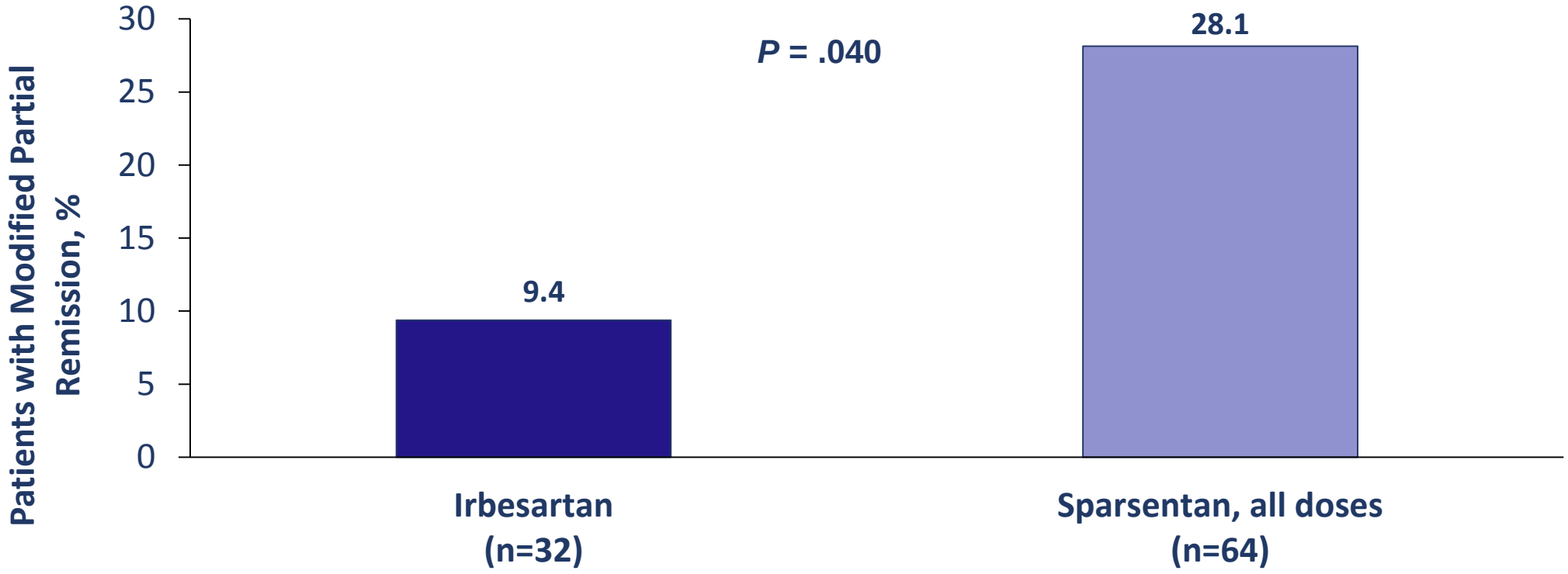
FSGS-CT



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.

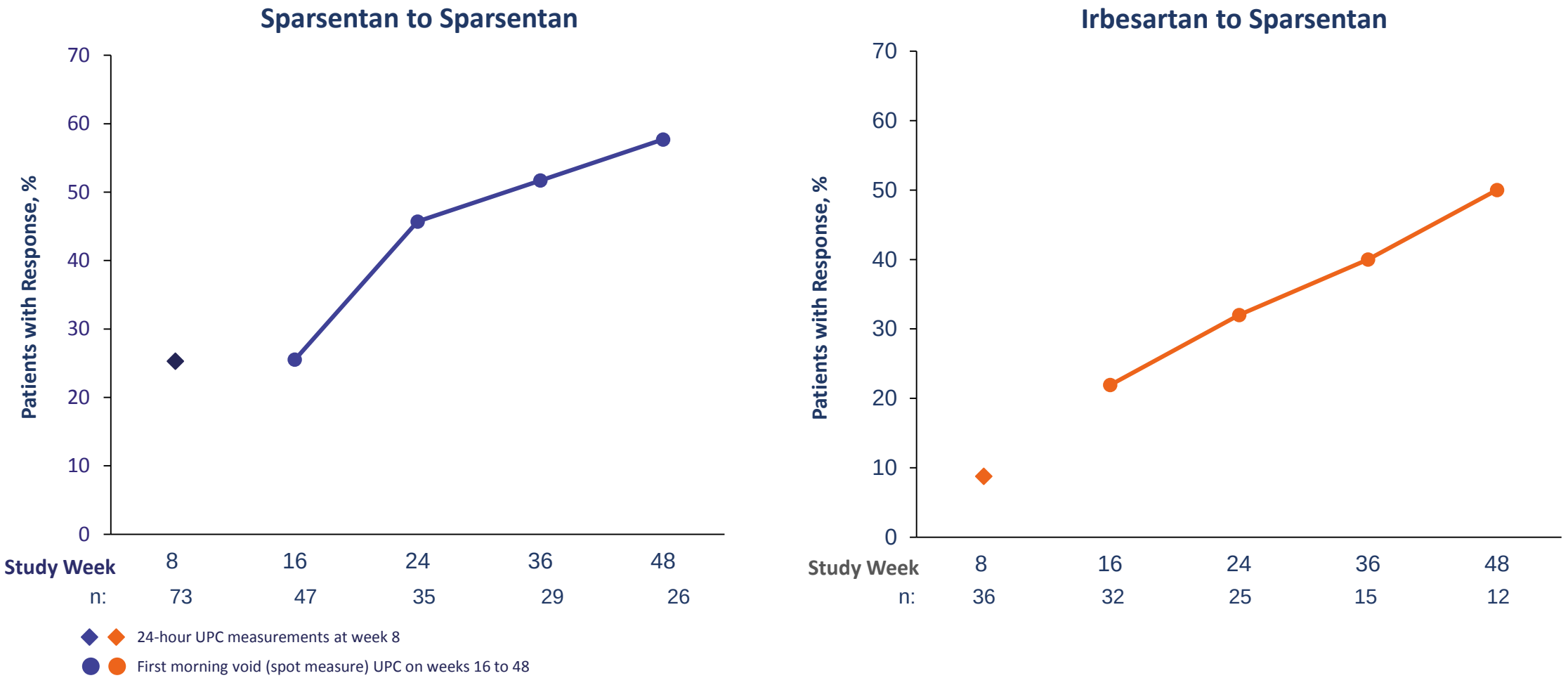
Promising mPR Rates in DUET Study

- mPR defined as UPC ≤ 1.5 g/g and > 40% reduction in UPC



Complete remission (UPC < 0.3 g/g) was achieved by 4 sparsentan-treated patients vs 0 irbesartan-treated patients

mPR Sustained During Open Label Period



Response defined as UPC ≤ 1.5 g/g and > 40% reduction in UPC (first morning void) 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). 46% of sparsentan-treated patient provided spot measure (first morning void) at week 8. Percent of patients that provided spot measure (first morning void) at week 8 is not available for irbersartan-treated patients. Based on the full analysis set.

Similar Incidence of TEAEs Between Irbesartan and Sparsentan-treated Patients

TEAE	Patients with TEAEs During the Double-Blind Period, %	
	Irbesartan (n = 36)	Sparsentan, 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

Edema Incidence and Severity Manageable and Similar Across Treatment Groups

Edema Severity Grade	Patients with Edema During the Double-Blind Period, %			
	Irbesartan		Sparsentan, All Doses	
	Baseline (n = 29)	Week 8 (n = 28)	Baseline (n = 53)	Week 8 (n = 60)
0	76	86	66	65
1+ to 2+	21	14	32	30
3+ to 4+	3	0	2	5

P value = NS

Sparsentan generally safe and well tolerated

Phase 2 DUET Study – Key Conclusions

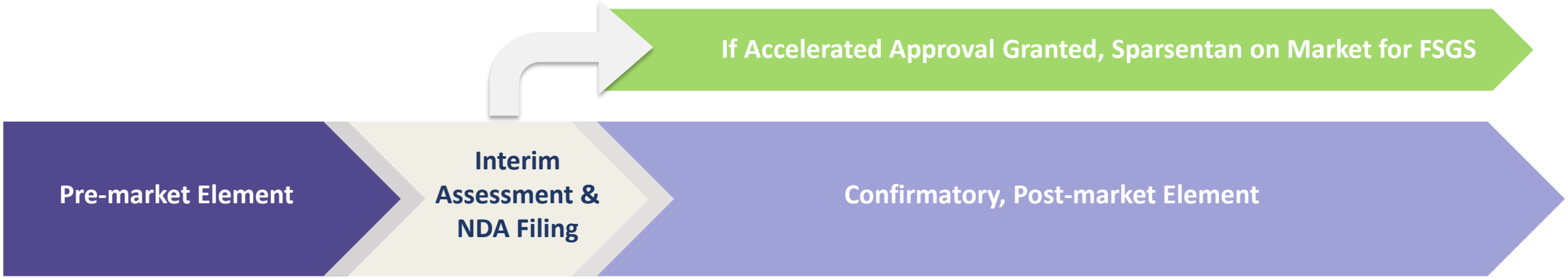
- **Sparsentan achieved a significant reduction in proteinuria compared with irbesartan in patients with FSGS**

- **The proportion of patients who achieved a mPR of proteinuria (UPC ≤ 1.5 g/g and > 40% reduction in UPC) was significantly greater in the sparsentan-treated group and increased throughout the open-label period**

- **Sparsentan was generally safe and well-tolerated in the study**
  - No withdrawals due to fluid retention during the eight-week blinded treatment period
  - 84% of patients who completed the 8-week double-blind period remained on sparsentan in the open-label extension at the time of data presentation

Sparsentan - Advancing Towards NDA Filing With Single Phase 3 Trial

- **Following FDA guidance received during the Company's End of Phase 2 meeting, Retrophin plans to initiate a single Phase 3 clinical trial to support registration in the U.S. and EU**
  - Protocol development ongoing; expect to confirm plans with FDA in 2H17 and initiate pivotal trial thereafter
  - Preliminary identification of global sites has resulted in the identification of significant numbers of potential trial candidates



1. Pre-marketing portion of the trial will focus on an analysis of proteinuria, mPR of proteinuria expected

2. Substantial treatment effect in pre-market portion would enable an NDA filing for accelerated approval under Subpart H

3. Confirmatory, post-marketing portion of trial is expected to monitor changes in estimated glomerular filtration rate (eGFR)

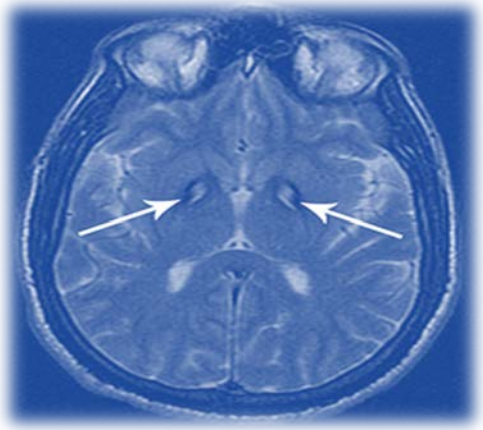
RE-024

Pantothenate Kinase-Associated Neurodegeneration (PKAN)

- **PKAN is a rare autosomal recessive disorder**
  - Caused by mutations in *PanK2*, which encodes an enzyme essential to coenzyme A (CoA) synthesis
  - Diagnosis occurs via genetics or imaging
  - Approximately 2,500 – 5,000 patients worldwide

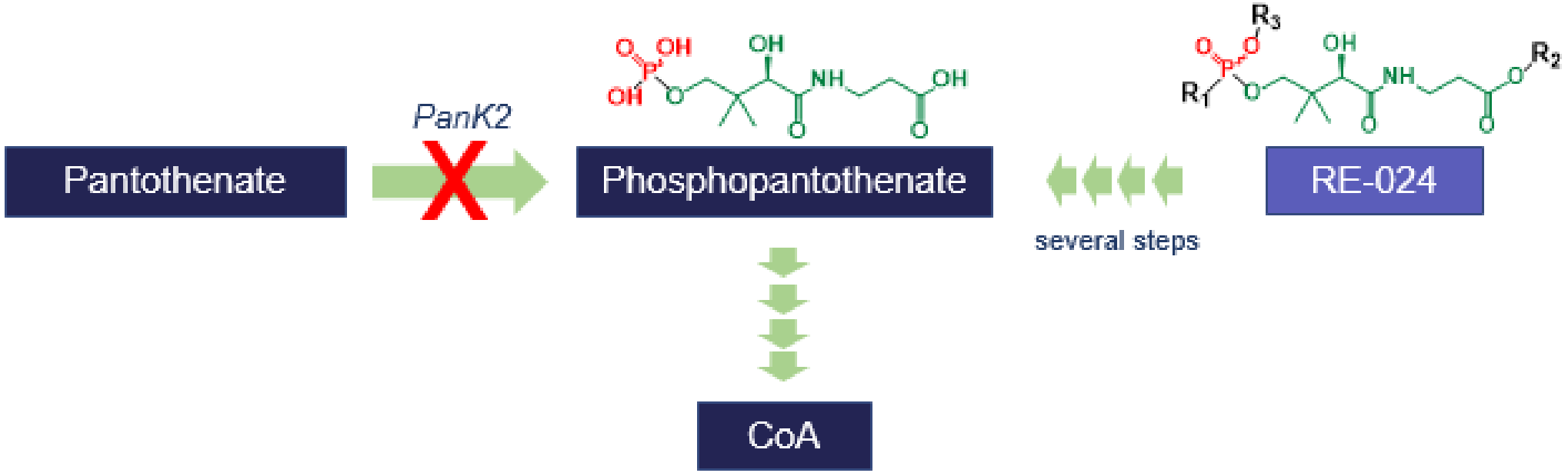
- **PKAN presents as chronic, progressive neurodegeneration with a variable clinical course**
  - Parkinsonian phenotype with loss of ambulation and early mortality due to disease complications & sequelae
  - Dystonia is another cardinal symptom that results in significant morbidity

- **No FDA/EMA approved treatments**



PKAN specific “eye of the tiger” sign caused by iron deposits on MRI

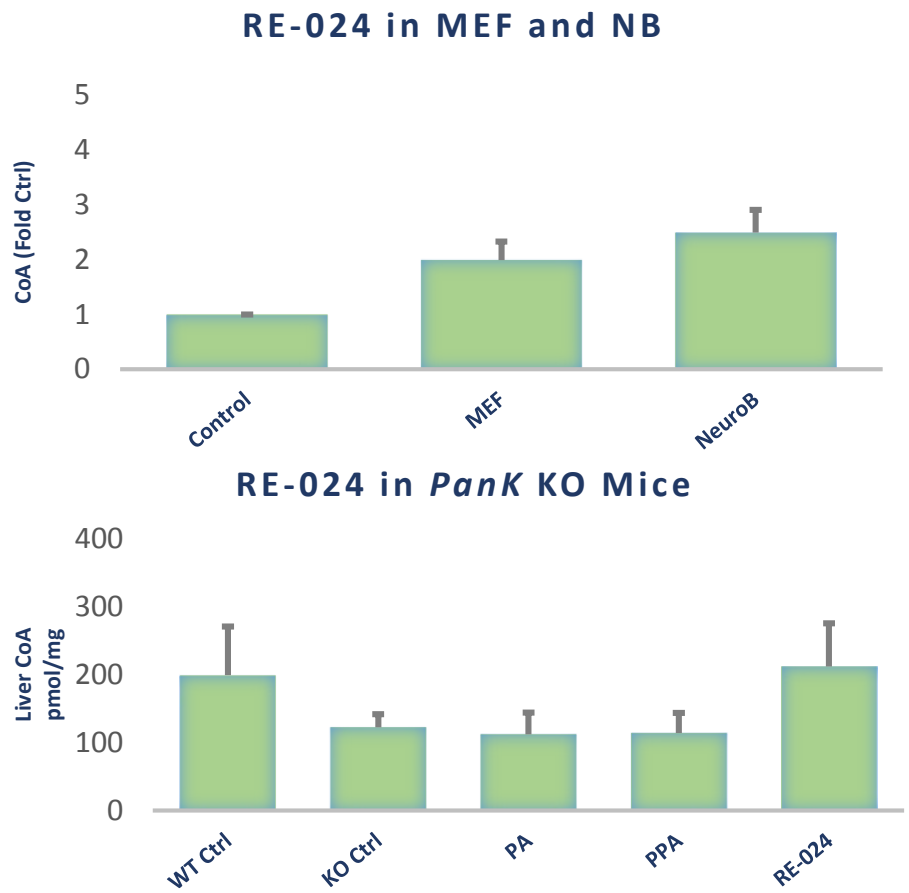
PKAN Biology and Novel Mechanism of RE-024

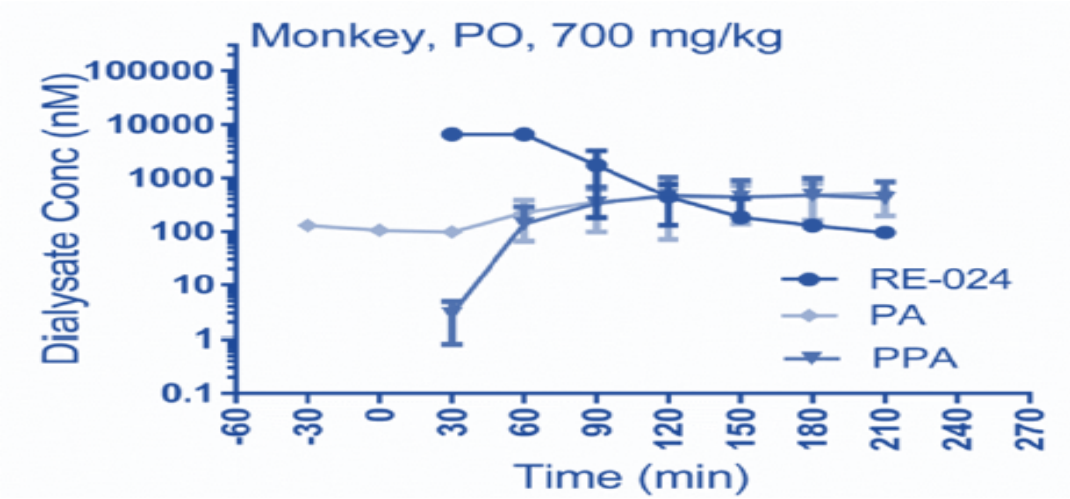


- **RE-024 is a novel phosphopantothenate replacement therapy developed internally by Retrophin R&D**
- **Pantothenate kinase (*PanK*) phosphorylates pantothenate (PA) to yield phosphopantothenate (PPA)**
- **It is hypothesized that *PanK2* deficient patients have decreased or no ability to synthesize PPA, resulting in low CoA levels**
- **CoA is important in many functions, mostly related to mitochondrial energy metabolism**

RE-024 Preclinical Evidence & Mechanism of Action

- **RE-024 has been shown to increase coenzyme A levels in nonclinical PKAN disease models, and demonstrate distribution to the brain in monkeys**



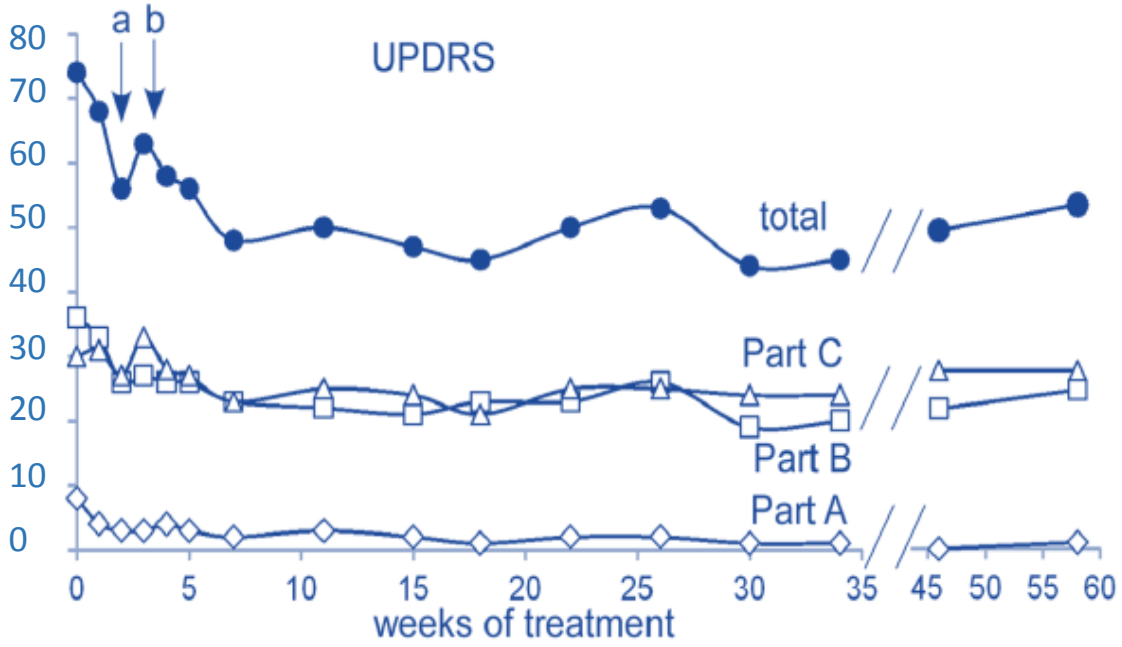


Conclusions

- RE-024 was measurable in brain dialysate in monkeys after an oral dose
- Given the similarities in the plasma half-life between monkeys and humans, RE-024 is expected to reach the human brain

RE-024 Clinical Activity in Physician-Initiated Treatment

- **12-month open, uncontrolled treatment of one PKAN patient with RE-024 was associated with clinical improvement followed by stabilization of symptoms**
  - RE-024 was demonstrated in the single patient to be safe and well-tolerated at 240mg total daily dose
  - Patient showed rapid improvement in clinical parameters including the Unified Parkinson's Disease Rating Scale (UPDRS), followed by a period of stabilization
  - Patient regained ability to walk independently for short distances

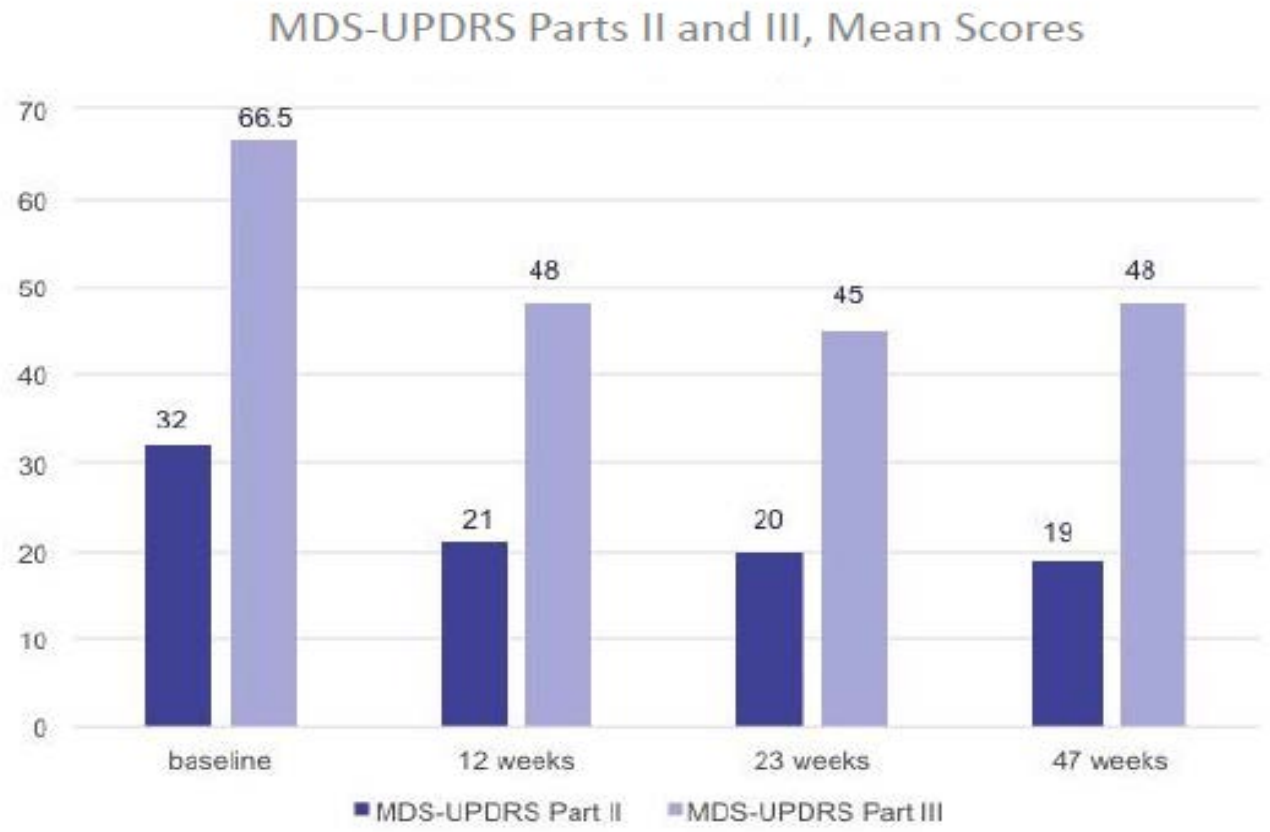


UPDRS including parts A-C and total score (arrows a and b indicate a) interruption of treatment; a) restarting at half dose

*Physician-reported outcomes in uncontrolled setting; Yiolanda-Panayiota Christou, MD et al. ACMG 2016

RE-024 Clinical Activity in Physician-Initiated Treatment

- **Open, uncontrolled treatment with RE-024 in two adult PKAN patients over 47 weeks was associated with clinical improvement followed by stabilization of symptoms**
  - RE-024 was demonstrated to be safe and well-tolerated with no treatment-associated adverse events in the two patients
  - Both patients regained ability to walk unassisted for short distances



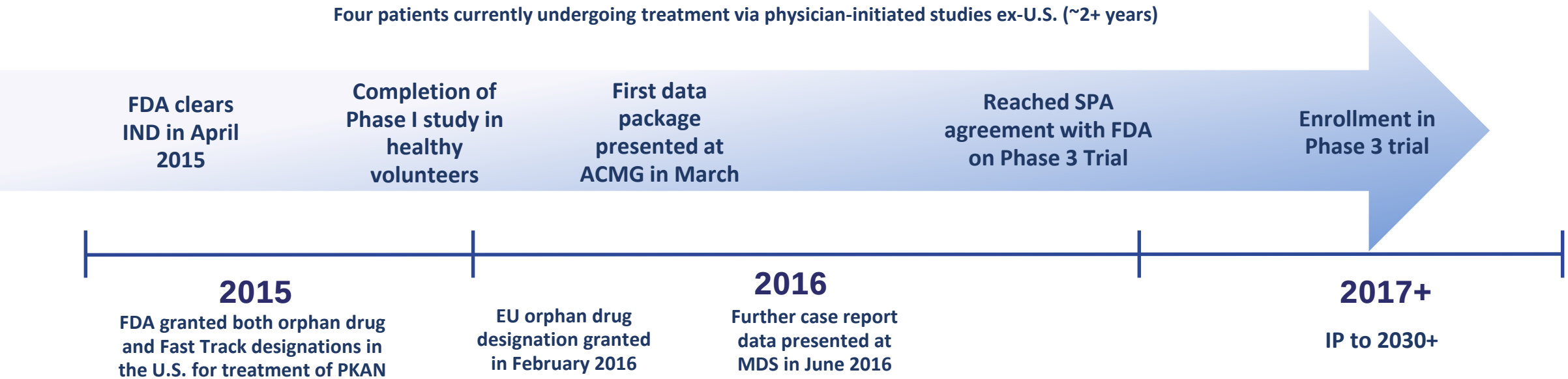
Mean change from Part 2 baseline	-34%	-38%	-41%
Mean change from Part 3 baseline	-27%	-31%	-27%

*Physician-reported outcomes in uncontrolled setting; Pedro Roa, MD et al. MDS 2016

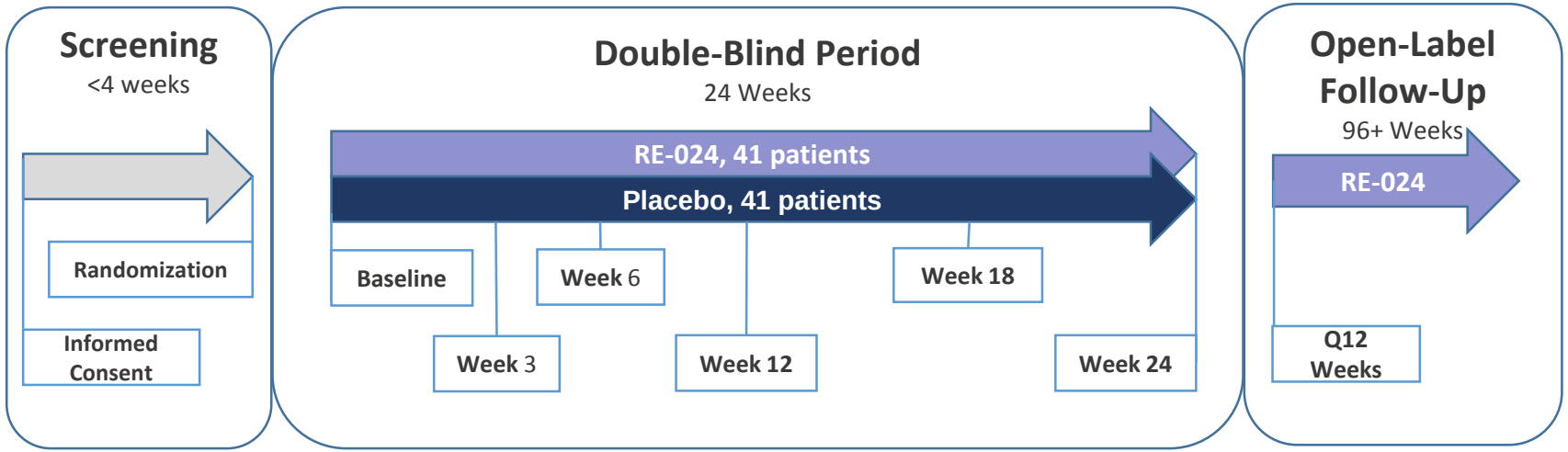
Retrophin

RE-024 Phase 3 Study Expected to Begin Dosing Mid-2017

- **Phase 1 study of RE-024 in healthy volunteers (N=40) was determined to be safe and well tolerated, no SAEs reported and no patients dropped from study**
- **Reached Special Protocol Assessment (SPA) agreement with FDA on single Phase 3 trial evaluating efficacy in PKAN patients in November 2016**
- **Multiple IRB approvals obtained; expected to dose first patient in Phase 3 FORT study mid-2017**



RE-024 Phase 3 Trial Design



- **International, randomized, double-blind, placebo-controlled pivotal study**
- **Plan to enroll approximately 82 patients with PKAN aged 6 to 65 years**
- **Primary endpoint: change in Pantothenate Kinase-associated Neurodegeneration Activities of Daily Living (PKAN-ADL) scale from baseline through 24 weeks of treatment**
  - PKAN-ADL scale is a novel, PKAN-specific, patient-reported outcome scale measuring motor abilities to function in daily living for patients with PKAN
  - Adaptation of Part II of the comprehensive and widely-referenced Unified Parkinson's Disease Rating Scale (UPDRS)

Commercial Products

Thiola® (tiopronin)

Thiola® (tiopronin) tablets Rx Only

- **Thiola is an FDA approved small molecule for the prevention of cystine stone formation**
  - Binds with cystine to form a more soluble molecule which can be excreted in the urine
  - Not a typical kidney stone; generally does not respond to lithotripsy
  - Percutaneous nephrolithotomy or surgical intervention often required to manage stones
  - An average of one surgical procedure every 3 years, with 7 surgical procedures by middle age[1]

- **Thiola significantly reduces the number of stone events in patients with cystinuria**
  - Chronic therapy taken prophylactically
  - If left untreated, cystinuria patients can have multiple stone events per year

- **Recently initiated development of a new formulation**

- **An estimated 4,000 to 5,000 cystinuria patients are potential candidates for Thiola therapy**

Reference: 1.Barbey F, Joly D, Rieu P, et al. Medical treatment of cystinuria: critical reappraisal of long-term results. J Urol. May 2000;163(5):1419-23.

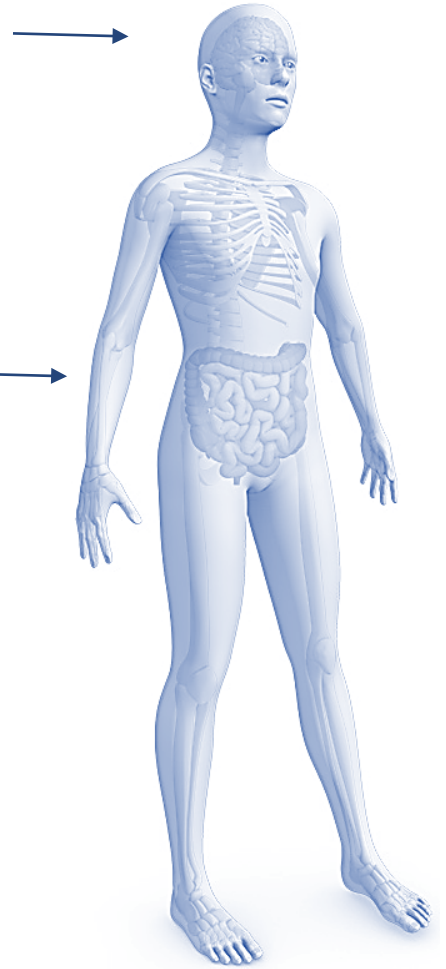
Cholbam® (cholic acid)

- **First FDA approved naturally-occurring primary bile acid for pediatric and adult patients with bile acid synthesis disorders due to single enzyme defects and Zellweger Spectrum Disorders (ZSDs)**
  - Seven years of U.S. market exclusivity via orphan drug designation (approved March 2015)
  - Oral dosing of 10 to 15 mg/kg/day
- **The absence of cholic acid in patients leads to:**
  - Accumulation of potentially toxic bile acid intermediates in the liver (cholestasis)
  - Malabsorption of fats and fat-soluble vitamins in the diet
  - If untreated, these 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 200 to 300 patients are current candidates for therapy;**
  - New patients continue to be identified and begin therapy

Chenodal® (chenodeoxycholic acid)

CHENODAL™
(Chenodiol Tablets 250 mg)

- **Chenodal is a synthetic bile acid approved for the treatment of gallstones, but usage is exclusively in cerebrotendinous xanthomatosis (CTX)**
  - CTX is a rare autosomal recessive lipid storage disease with multi-organ onset
  - Chenodal is the only FDA-approved formulation of chenodeoxycholic acid, which is the current standard of care
- **Retrophin is seeking U.S. regulatory approval for the addition of the CTX indication to the Chenodal label**
  - Following FDA interaction, the Company is assessing clinical efforts that could enable the addition of CTX to the Chenodal label
- **Chenodal received orphan designation for CTX in 2010 and could gain seven years of U.S. market exclusivity with changes to the current label**



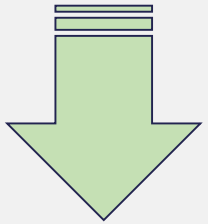
Striving for Improved Therapeutic Outcomes

TOTAL CARE HUB

A Comprehensive Patient Support, Education, Access, and Delivery Program

- **24/7 access to hub counselors for treatment support**
- **Financial support and reimbursement options boost access**
- **Patient education and access to support groups**
- **24/7 access to medical information**
- **Home delivery**

- **Improved Access**
- **Improved Support**
- **Improved Education**



- **Better Compliance**
- **Better Adherence**
- **More Prescribers**
- **Better Outcomes**

Financial Performance

Retrophin Financial Profile

GAAP Reported Financials	1Q17	FY16	FY15
Net Sales	$33.6mm	$133.6mm	$99.9mm
Operating Expenses	$44.0mm	$163.7mm	$130.0mm
Operating Income/(Loss)	($14.4mm)	($58.2mm)	($50.7mm)
Net Income/(Loss)	($11.1mm)	($47.9mm)	$117.2mm
Cash and net receivable	$295.3mm	$302.7mm	$322.0mm

Non-GAAP Reported Financials	1Q17	FY16	FY15
Operating Expenses	$32.6mm	$118.4mm	$90.7mm
Operating Income	$0.3mm	$5.4mm	$7.0mm
Net Income	$0.3mm	$4.4mm	$7.5mm

- **Shares outstanding as of March 31, 2017: basic ~38mm, diluted ~47mm**
- **Debt outstanding as of March 31, 2017: $46 million of convertible notes**

*As presented, GAAP operating expenses includes SG&A and R&D only. Non-GAAP adjustments include: bargain purchase gain, change in fair value of the Company's derivative instruments, stock compensation, depreciation and amortization, finance expense, and transaction & license fees. Net receivable includes the present value of the $47.5 million owed to Retrophin as a result of the sale of its PRV.

Ample Cash on Hand; Extensive Catalysts Ahead

Pipeline to Shape Promising Future

- **Two first-in-class Phase 3 programs expected to create significant value for patients and shareholders in the near future**

Growing Commercial Portfolio

- **Double-digit organic revenue growth expected in 2017, supporting investment in R&D**

Strong Balance Sheet to Provide Flexibility

- **Strong balance sheet with ~$300mm in cash + receivable to fund current programs while providing flexibility to accelerate growth and diversify the business**

Retrophin