Retrophin

Agreement to Acquire Orphan Technologies & OT-58

Delivering Life-Changing Therapies to People Living with Rare Diseases

October 22, 2020

Forward-Looking Statements

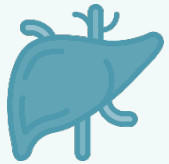
This presentation contains "forward-looking statements" as that term is defined in the Private Securities Litigation Reform Act of 1995. Without limiting the foregoing, these statements are often identified by the words "may", "might", "believes", "thinks", "anticipates", "plans", "expects", "intends" or similar expressions. In addition, expressions of our strategies, intentions or plans are also forward-looking statements.

Such forward-looking statements include, but are not limited to, references related to Retrophin's expectations with respect to the closing of its planned acquisition of Orphan Technologies; the potential impact upon and benefits to Retrophin from the proposed acquisition; the potential for OT-58 to ultimately become the first disease modifying therapy for HCU; and references to future expectations, plans and prospects for Retrophin. Such forward-looking statements are based on current information available to Retrophin and involve inherent risks and uncertainties, including factors that could delay, divert or change any such forward-looking statements, and could cause actual outcomes and results to differ materially from current expectations. No forward-looking statement can be guaranteed. Retrophin faces risks associated with, but not limited to: the parties' ability to complete the proposed transaction in a timely manner, if at all, considering the various closing conditions; if consummated, Retrophin's ability to realize the anticipated benefits of the proposed transaction, including the potential developmental and commercial success of the OT-58 product candidate; significant and unknown transaction costs; actual or contingent liabilities; the risk of litigation and/or regulatory actions related to the proposed transaction; other business effects outside of either company's control, including the effects of industry, market, economic, political or regulatory conditions or the ongoing COVID-19 pandemic; as well as negative impacts that could result from changes in tax and other laws, regulations, rates and policies. In addition, such risks and uncertainties may include those described in Retrophin's annual, quarterly and current reports (i.e., Form 10-K, Form 10-Q and Form 8-K) as filed or furnished with the Securities and Exchange Commission, which are available at Retrophin's website (www.retrophin.com) under "Investors & Media".

You are cautioned not to place undue reliance on any forward-looking statements as there are important factors that could cause actual results to differ materially from those in any forward-looking statements, many of which are beyond our control. Except to the extent required by law, Retrophin undertakes no obligation to publicly update any forward-looking statement.

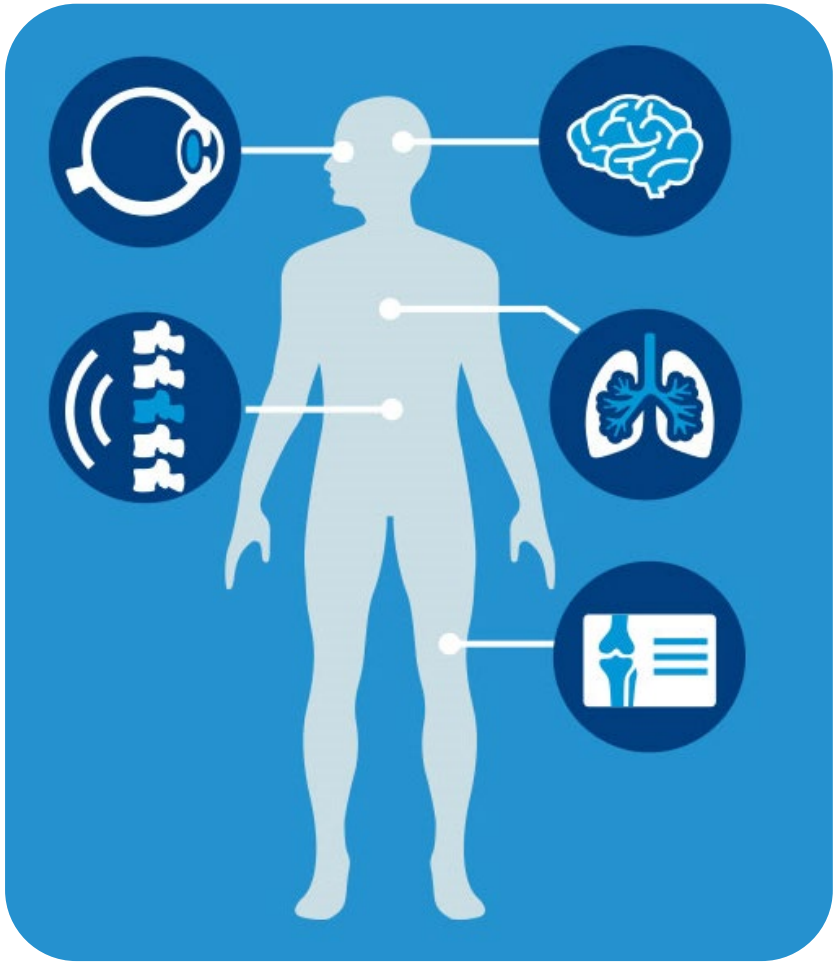

Retrophin®
Our #1 priority will always be the patients we serve.
Retrophin is a biopharmaceutical company dedicated to identifying, developing and delivering life-changing therapies to people living with rare disease.

OT-58 Will Expand Portfolio of Potential First-in-Class Programs Targeting Rare Disease

Therapeutic Area	Program	Indication	Pre-Clinical	Phase 1	Phase 2	Phase 3
Rare Nephrology 	Sparsentan	FSGS				
	Sparsentan	IgAN				
Rare Metabolic 	OT-58*	Classical Homocystinuria (HCU)				
Rare Hepatology 	NIH CRADA	NGLY1 Deficiency				
	NIH CRADA	Alagille Syndrome				

*subject to the closing of the transaction entered into in October 2020

Classical Homocystinuria (HCU) is a Rare Disorder That Can Lead to Life-Threatening Complications



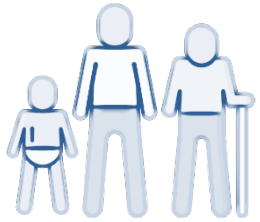
- **Rare autosomal recessive disorder caused by mutations in cystathionine beta-synthase (CBS) gene, leading to deficient activity of CBS**
  - Metabolic deficiency of CBS leads to bodily buildup of toxic homocysteine (Hcy)
- **Toxic levels of Hcy can lead to serious complications for people living with classical HCU**
  - Continuous risk of developing life-threatening thrombotic events including heart attack and stroke
  - Other symptoms of classical HCU include dislocation of the eye lens and extreme nearsightedness, skeletal complications including osteoporosis, and developmental delay
- **There are no approved treatments that address the underlying genetic cause of HCU**
  - Current standard of care includes vitamin B6, low-protein diet + supplements, betaine
- **Estimates suggest at least 3,500 patients in US, similar number in Europe**

With Largely Ineffective Treatment Options, a Significant Unmet Need Remains for People Living with HCU

- **Generally accepted therapeutic goal is to reduce total homocysteine (tHcy) levels but current treatment options rarely sustain reductions in tHcy**

- **Significant challenges for patients to maintain compliance; periods of poor metabolic control have a cumulative deleterious effect**

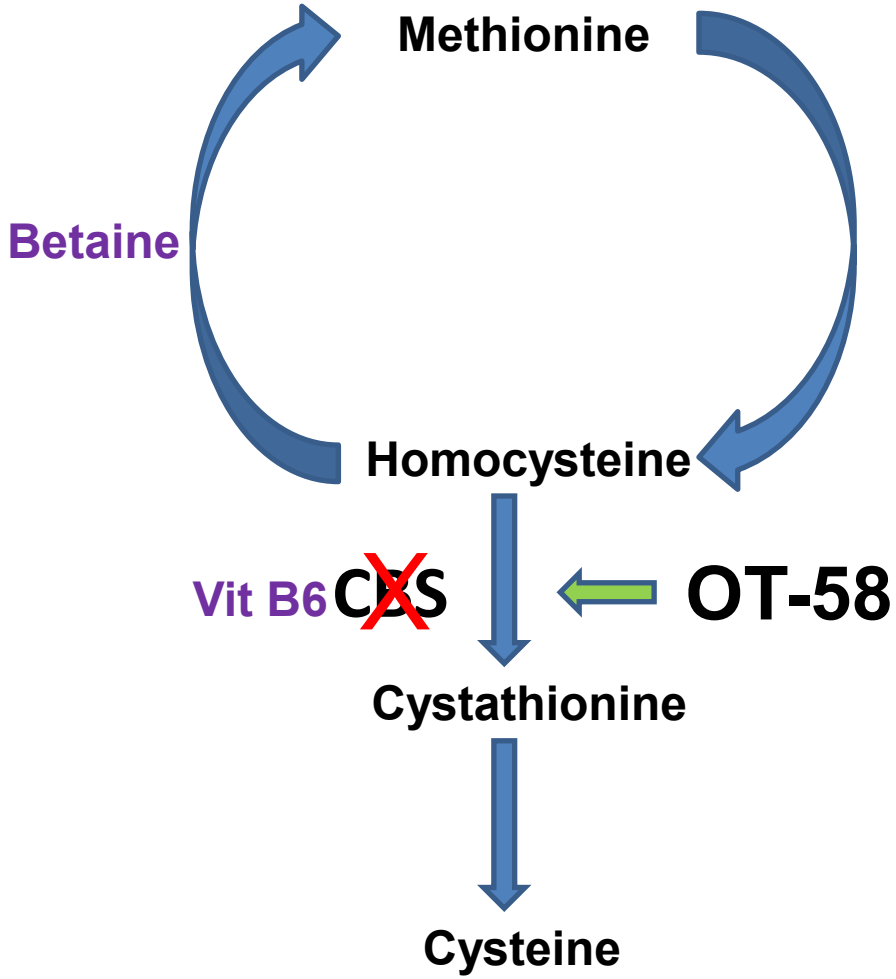
- **Patients struggle with severe dietary protein restrictions as they age; liberalized diet is amongst top needs**



- **Inability to sustain reductions in Hcy results in life-long risk of thrombotic and cardiac events + cognitive impairment**

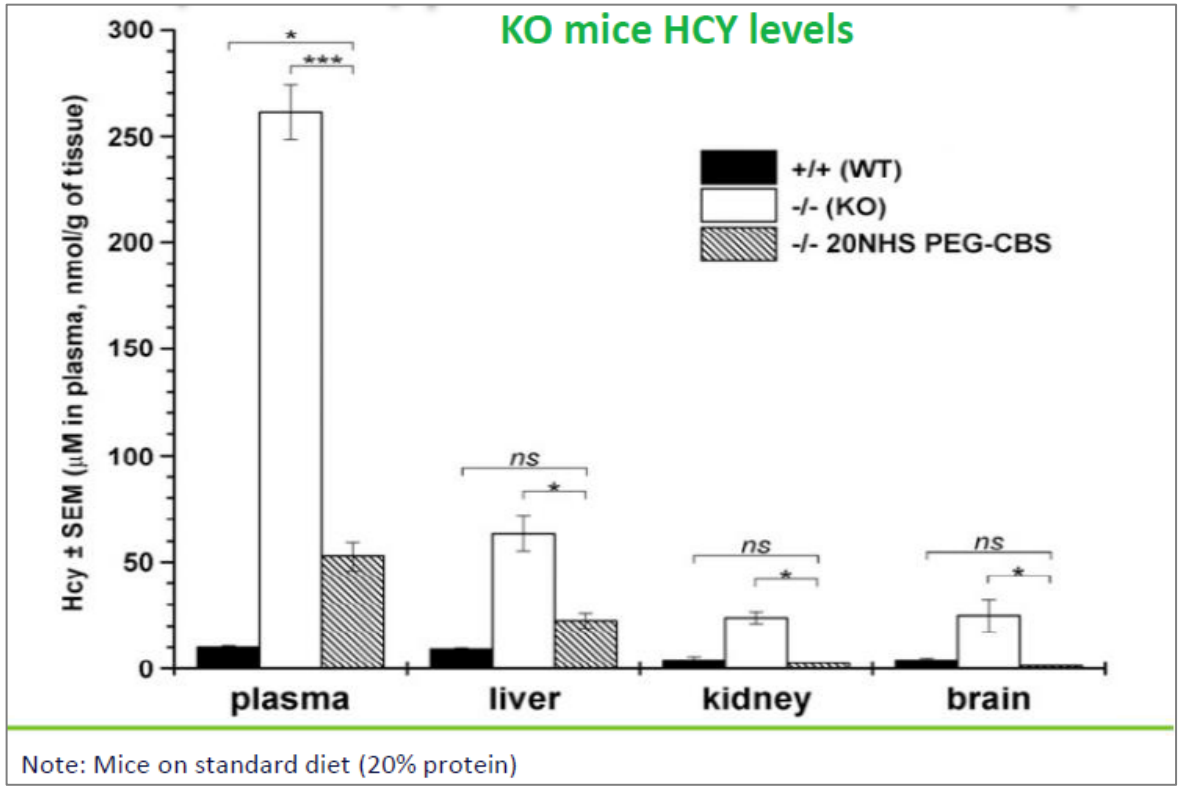
OT-58 is a novel, investigational modified recombinant CBS enzyme therapy

- **OT-58 is a pegylated, modified recombinant truncated human enzyme, designed to address the underlying genetic cause of HCU**
  - Mechanism of action is pathology agnostic
- **OT 58 is 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**
- **OT-58 has been granted multiple regulatory designations:**
  - FDA Rare Pediatric Disease designation
  - FDA Fast Track designation
  - Orphan Drug designation in the US and Europe.



Administration of OT-58 resulted in up to 70-90% reduction of plasma and tissue Hcy levels in mouse models

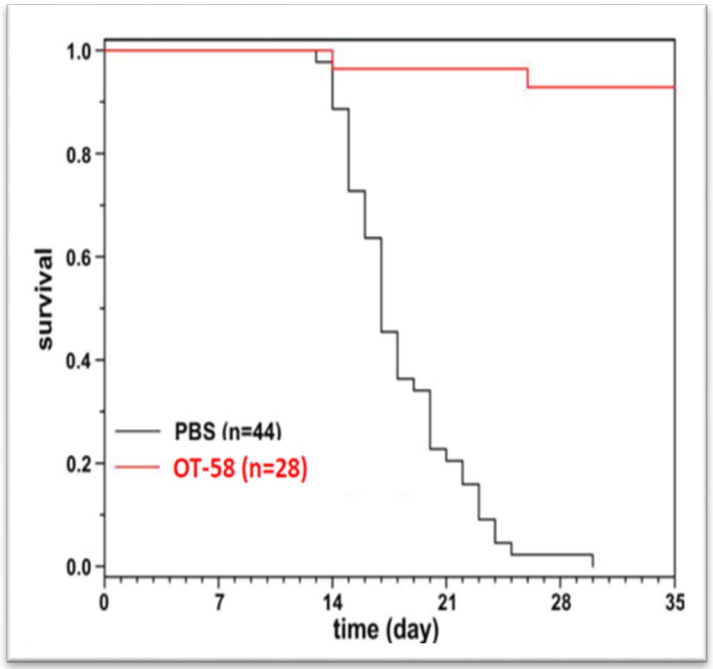
- Dosing with OT-58 resulted in a decrease of extracellular Hcy

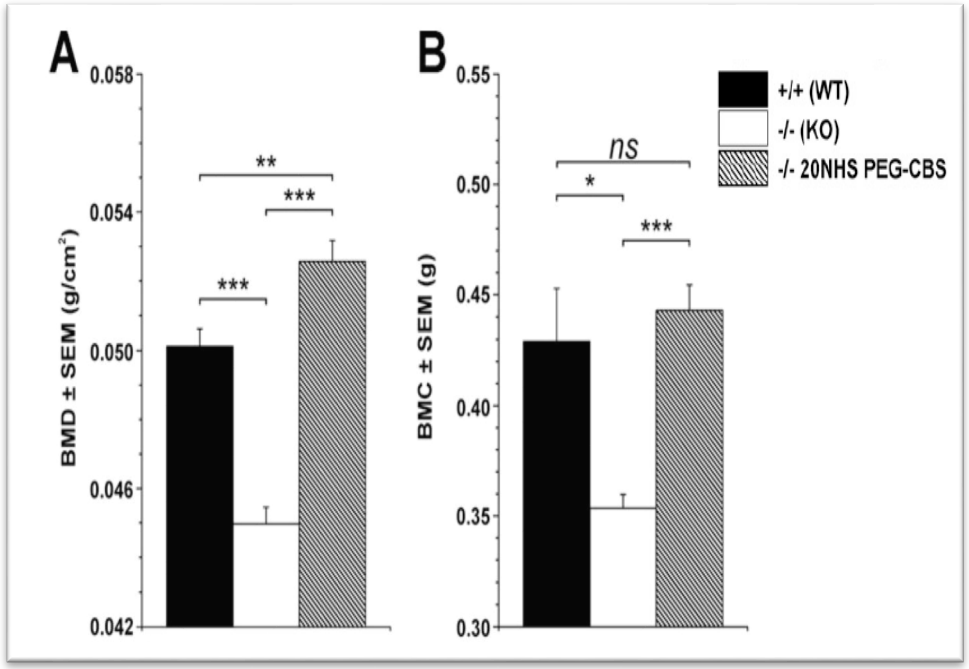


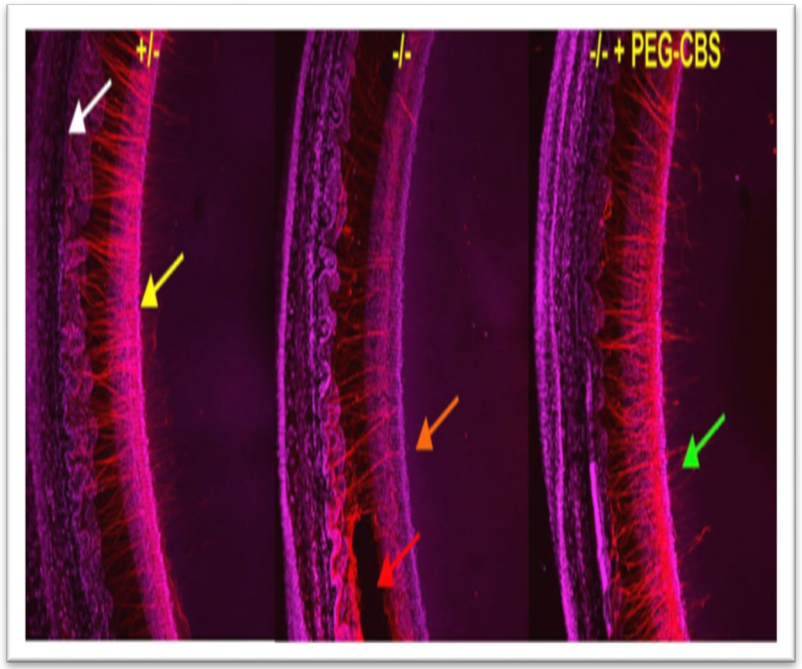
- "Metabolic Sink"
  - By lowering the Hcy levels in the blood, OT-58 has been shown to create a concentration gradient that causes excess Hcy in tissues to move to plasma, where it is metabolized

Source: Majtan,T.,Hulkova,H.,Park,I.,Krijt,J.,Kozich,V.,Bublil,E.M.,andKraus,J.P.(2017)Enzyme replacement prevents neonatal death, liver damage, and osteoporosis in murine homocystinuria FASEBJ

Treatment with OT-58 appeared to prolong survival, prevent osteoporosis and rescue ocular structure in mouse models







Negative Control **Untreated** **OT-58 treated**

- Treatment with OT-58 appeared to prolong survival in KO mouse models[1]
- Untreated KO mouse models resulted in significant liver damage and death within 20-30 days

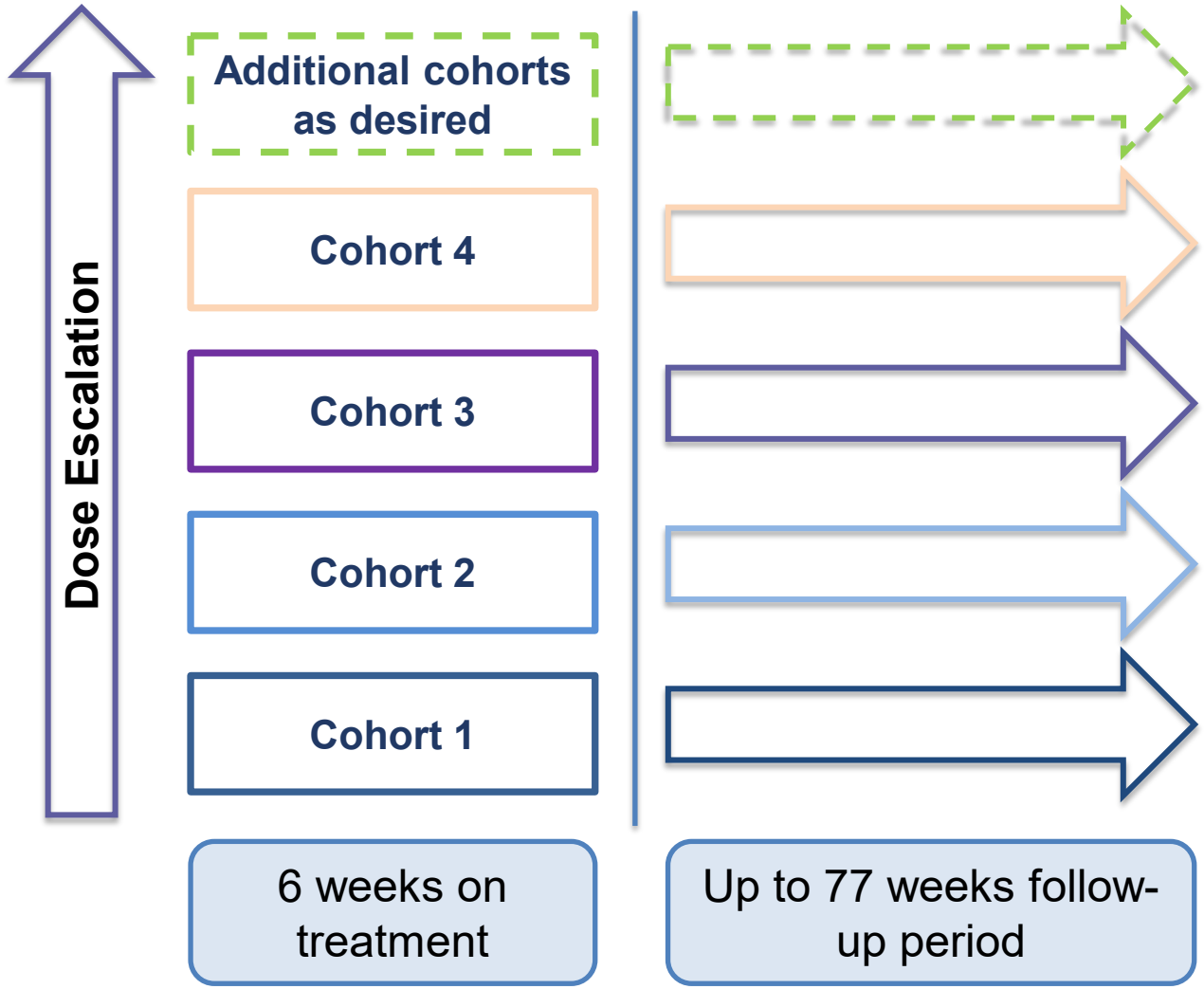
- Early treatment with OT-58 appeared to prevent loss of bone mineralization and fat content in KO mice[1]

- Treatment with OT-58 appeared to preserve fiber integrity and prevent the degradation of the structure that secures the lens in the eye[2]

Sources: 1. Majtan, et al.(2017) Enzyme replacement prevents neonatal death, liver damage, and osteoporosis in murine homocystinuria FASEBJ
2. Majtan,T., et al (2018) Enzyme replacement therapy ameliorates multiple symptoms of murine homocystinuria

OT-58 is Advancing in Ongoing Phase 1/2 Clinical Proof-of-Concept Study in HCU

- OT-58 is advancing in a Phase 1/2 double blind, randomized, placebo-controlled study
- Cohorts are enrolled in a dose escalating fashion; following completion of each cohort, unblinded safety data are reviewed by DMC prior to activating next cohort
- Primary endpoint
  - Incidence of treatment-emergent adverse events
- Secondary endpoints
  - Total plasma homocysteine (tHcy)
  - Cognitive function changes
  - Ocular assessment
  - Bone mineral density
  - Patient QoL scales: Neuro-QoL, EQ-5D & SF-36



Terms of Agreement to Acquire OT-58 and Orphan Technologies

- **Retrophin to receive global rights to OT-58, a novel, potential first-in-class enzyme therapy in Phase 1/2 development for classical HCU**
- **Retrophin is utilizing its balance sheet to fund transaction from position of strength**
  - ~$457 million in cash and marketable securities as of June 30, 2020
- **Transaction expected to close in 4Q20**

- **Upon completion of the transaction, Orphan Technologies shareholders will receive:**
  - $90 million upfront cash payment
  - Up to $427 million in payments contingent upon achievement of key milestones in the development and commercialization of OT-58
  - Tiered mid-single digit royalty on future net sales of OT-58 in the US and Europe
    - ROW economics shared
  - A separate, preclinical OT-15 program is subject to concurrent spin-out and not part of acquisition transaction

Anticipated Milestones to Drive Value for Retrophin in the Coming Years

Key Milestones in 2020

- 190th Patient Enrolled in Pivotal Phase 3 DUPLEX Study in FSGS – **1Q20** ✓
- 280th Patient Enrolled in Pivotal Phase 3 PROTECT Study in IgAN – **3Q20** ✓
- Closing of Orphan Technologies Acquisition – **4Q20**

Key Milestones in 2021

- Topline Results from Proteinuria Endpoint in Pivotal Phase 3 DUPLEX Study in FSGS – **1Q21**
- Topline Results from Proteinuria Endpoint in Pivotal Phase 3 PROTECT Study in IgAN – **3Q21**
- NDA (Subpart H) and CMA Filings for Sparsentan in FSGS – **2021**
- Results from OT-58 Phase 1/2 Study in HCU – **2021**

Key Milestones in 2022

- NDA (Subpart H) and CMA Filings for Sparsentan in IgAN – **2022**
- Potential Commercial Launch of Sparsentan in FSGS – **2022**
- Potential Commercial Launch of Sparsentan in IgAN – **2022**

Key Milestones in 2023

- Topline Results from eGFR Endpoint in Pivotal Phase 3 DUPLEX Study in FSGS – **1H23**
- Topline Results from eGFR Endpoint in Pivotal Phase 3 PROTECT Study in IgAN – **2H23**

Retrophin