Retrophin

Thiola® License
May 30, 2014

Retrophin

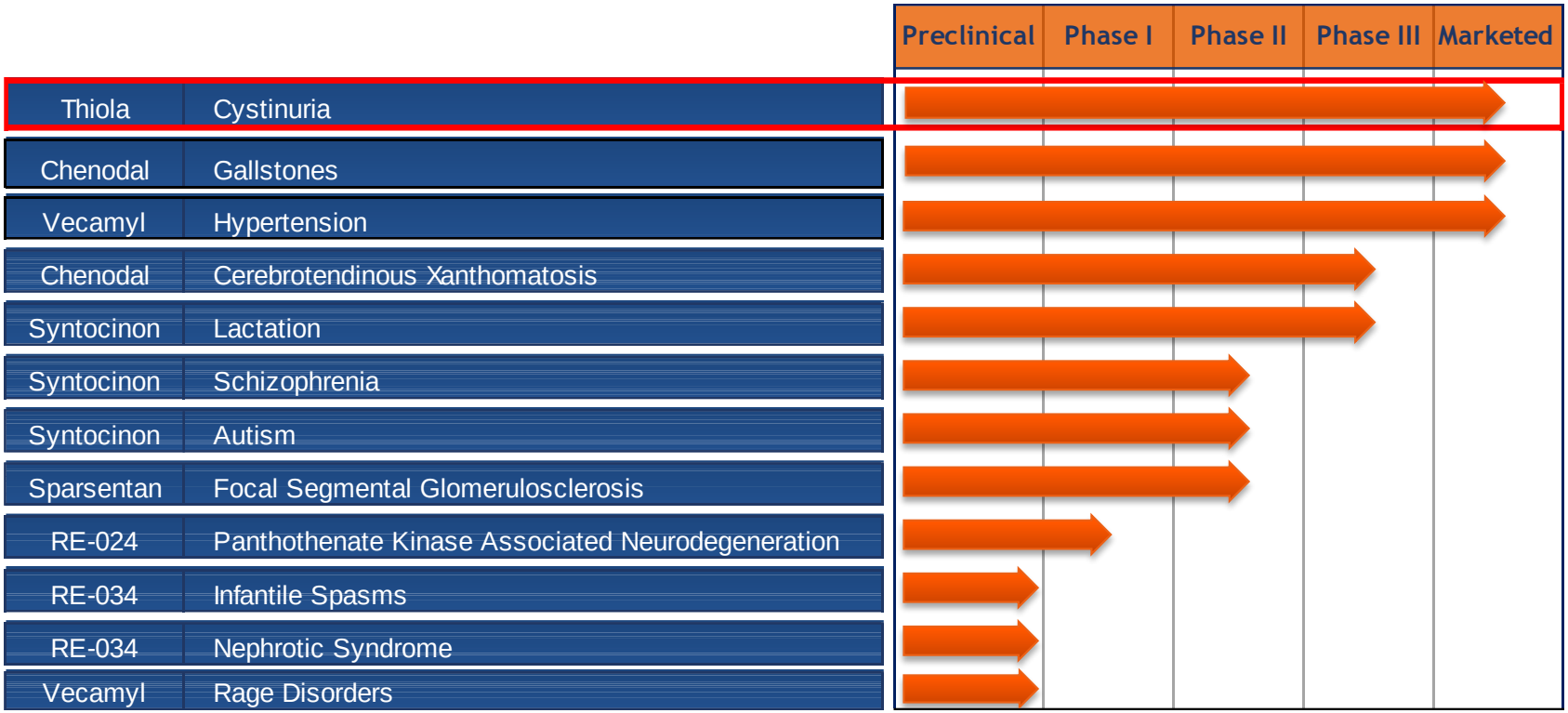
Forward-Looking Statements

This presentation contains forward-looking statements, including statements about our prospects, competitive position, 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, the occurrence of adverse safety events with our products, adverse market and economic conditions, 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 does not undertake any obligation to publicly update any forward-looking statements.

Assets

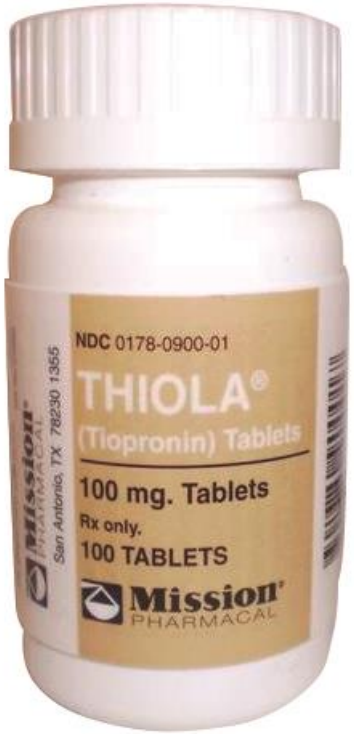

Preclinical
Phase I
Phase II
Phase III
Marketed
Thiola
Cystinuria
Chenodal
Gallstones
Vecamyl
Hypertension
Chenodal
Cerebrotendinous Xanthomatosis
Syntocinon
Lactation
Syntocinon
Schizophrenia
Syntocinon
Autism
Sparsentan
Focal Segmental Glomerulosclerosis
RE-024
Panthothenate Kinase Associated Neurodegeneration
RE-034
Infantile Spasms
RE-034
Nephrotic Syndrome
Vecamyl
Rage Disorders

Transaction Overview

- Retrophin has entered into a trademark license and supply agreement with Mission Pharmacal for Thiola® (tiopronin)
- Thiola® is approved to prevent the formation of cystine stones in patients with cystinuria
  - Thiola significantly reduces the number of stone events in patients with cystinuria
  - Thiola® is taken prophylactically
  - FDA approved in 1988
- Retrophin has identified several potential strategies to grow revenue
  - Product has no active sales force
  - New indications
  - Cystinuria is highly underdiagnosed
  - Increased pricing
  - Opportunity to expand to other geographical regions
- Strong fit with Retrophin's focus on rare and catastrophic diseases, particularly in nephrology

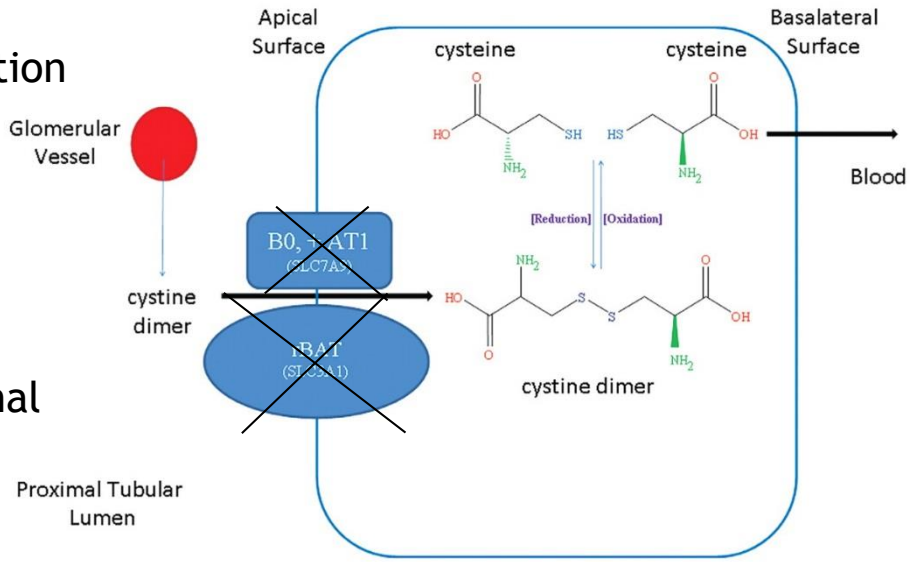
Thiola® (tiopronin)

- Thiola® is an FDA approved small molecule for the prevention of cystine formation
  - Granted Orphan Drug Designation (expired)
  - No approved generics
- Thiola® is the only approved form of tiopronin in the world
- Thiola® binds cystine and creates a more soluble molecule, which is excreted in the urine
- Thiola® is one of two products approved to prevent stone formation in cystinuria patients
  - Penicillamine is approved to treat cystinuria
  - Nearly a third of people are allergic to penicillamine
  - Thiola® is the preferred therapy due to its reduced risk of adverse events



Cystinuria

- Cystinuria is a rare genetic disease
  - Autosomal recessive inheritance
- Mutations in SLC3A1 and SLC7A9
  - Transporters of cystine, ornithine, lysine, and arginine in the kidney
  - Cystine is a dimer composed of two cysteine residues bound by a disulfide bond
  - Cystine is not readily soluble and accumulation leads to the formation of cystine stones
- Kidney stones are typically removed via lithotripsy or nephrolithotomy
  - Cystine stones are resistant to lithotripsy
- Chronic kidney stones can cause long term renal damage
  - Potentially culminating in loss of kidney function
- Kidney stones are extremely painful
  - Results in loss of productivity and diminished quality of life



Epidemiology

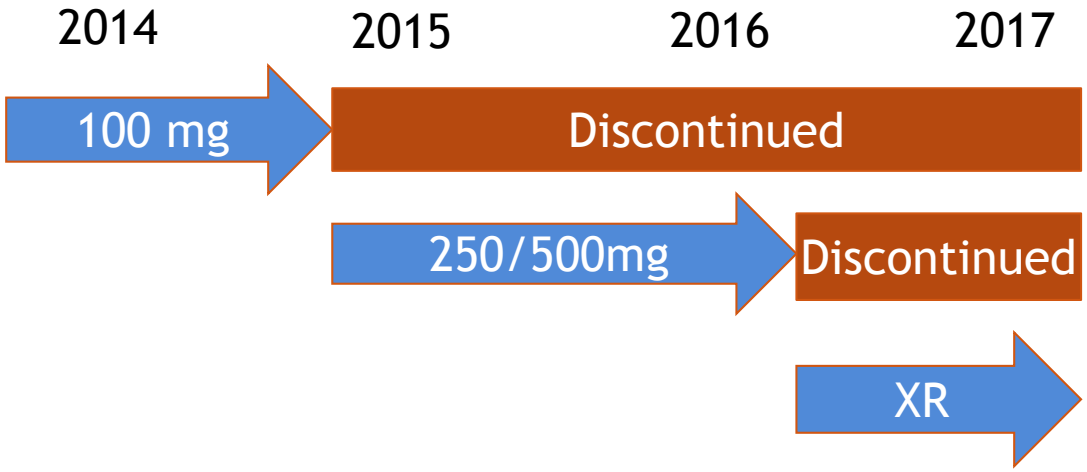
- The incidence of cystinuria is 1:7,000 worldwide
  - There are believed to be ~20,000 cystinuria patients in the US
  - Some of these patients can have their cystinuria controlled with diet, increased fluid intake, and alkalization therapy
  - Others are still unable to manage their disease using these methods
- Thiola® helps these resistant patients control the formation of stones
- If left untreated, cystinuria patients can have up to five stone events per year

Pharmacoeconomics

- Current pricing of Thiola® - $4,000 PPPY
  - Penicillamine pricing- $80,000-$140,000
- Thiola could support a significant price increase
  - $2.1 billion spent treating kidney stones in 2010
- Cost of a single stone removal
  - Lithotripsy: $10,000-$20,000 (generally ineffective in cystinuria)
  - Nephrolithotomy: $20,000-$60,000
  - ER visits
  - Lost of productivity
  - Pain medicines, mental anguish, depression

Distribution and Intellectual Property

- Similar to Chenodal®, Retrophin will move Thiola® into closed distribution
- Retrophin will also increase the number of available dosage forms
  - 100mg capsule is currently the only available dose
  - Retrophin will develop 250mg and 500mg doses
  - Retrophin will discontinue the 100mg dose
- Retrophin also plans to develop a long-acting version of Thiola® for once daily dosing



Forecasts

- 2014
  - Previous Guidance: $20mm - $22mm
  - New Guidance: $30mm - $35mm
- 2015
  - Previous Guidance: $36mm - $41mm
  - New Guidance: $60mm - $70mm
- Thiola® EBITDA margins of 70 - 75%
- Earnings power per share potential $1.50 - $2.00
- Potential Thiola® peak sales of $100mm

We estimate the NPV of license of Thiola® to be at least $10 per Retrophin share

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