AMP Quick Facts

Retrophin, Inc. (OTCQB: RTRX)

Biotechnology

Symbol:
OTCQB: (RTRX)

Last Price: $4.73
(February 25, 2013)

Sector:
Healthcare

Industry:
Biotechnology & Drugs

52 Week Range:

Low $1.50
High $7.69

Market-Cap: $58.2mm

Shares: O/S 12.3 mm

Insider Ownership: *40%*

Institution Ownership *40%*

Cash: 11.5mm
(Feb. 2013)

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Retrophin, Inc. (***OTCQB: RTRX***) New York-based, biotechnology company focused on discovering and developing treatments for rare and life-threatening diseases. Retrophin is currently developing treatments for Focal Segmental Glomerulosclerosis (FSGS), Pantothenate Kinase Associated Neurodegeneration (PKAN), Duchenne Muscular Dystrophy (DMD), and Spinal Muscular Atrophy (SMA).

Retrophin focuses on diseases that have no currently available treatments or very little competition. RTRX's strategy focuses on "orphan drugs" because of the ability to get an expedited processing through the FDA, and such drugs can command a high price point once on the market. Orphan drug is a special designation that is given to drugs that are intended to treat very rare diseases (in the United States this means fewer than 200,000 patients).

Retrophin intends to advance its pipeline of rare-disease drug candidates. The compound that is furthest along in development is RE-021, a potential treatment for focal segmental glomerulosclerosis (FSGS). RE-021 is on the cusp of beginning a Phase II clinical trial within the first half of 2013. If the results prove to be favorable, the company plans to file for FDA approval based on the Phase II results. This potentially would mean that Retrophin would have their first drug on the market in 2014, with a potential 1.25 billon dollar market.

Understanding FSGS (note that most patients are children) and RE-021:

- The New England Journal of Medicine states that the goal of therapy for FSGS is to induce a full or partial remission of proteinuria. Drugs like RE-021 have been shown to reduce proteinuria by as much as half
- Only 20% to 30% of patients respond to the currently used steroid therapy. Those that do not respond are looking at kidney failure in 5-10 years
- Dialysis costs insurers about $50,000 per patient per year
- A kidney transplant costs insurers about $100,000 per person
- RE-021 at a price point of at least $25,000 per person per year saving insurers a lot of money, and offers a better quality of life to patients
- 50,000 U.S. patients multiplied by $25,000 per patient implies a market of $1.25 billion that Retrophin would be the only player in

Other drugs in development include RE-024 for the treatment of Pantothenate Kinase Associated Neurodegeneration, RE-001 for the treatment of Duchenne Muscular Distrophy, and RE-003 for the treatment of Spinal Muscular Atrophy.

All of these drugs have blockbuster potential' and all are in markets that are underserved by big pharma. Retrophin will essentially be the category creator of these drugs. In each case an expedited FDA process is quite possible.

Drug	Disease	Pre-clinical	Phase I	Phase II	Phase III	Market
RE-021	***Focal Segmental Glomerulosclerosis (FSGS)***	→	→	→		
RE-024	***Pantothenate Kinase Associated Neurodegeneration (PKAN)***	→				
RE-001	***Duchenne Muscular Distrophy (DMD)***	→				
RE-003	***Spinal Muscular Atrophy (SMA)***	→				

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Full Market Awareness

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