

UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
Washington, D.C. 20549

FORM 8-K

CURRENT REPORT  
Pursuant to Section 13 or 15(d) of the  
Securities Exchange Act of 1934

Date of Report (Date of earliest event reported) February 13, 2014

**RETROPHIN, INC.**

(Exact name of registrant as specified in its charter)

Delaware

001-36257

27-4842691

(State or other jurisdiction  
of incorporation)

(Commission  
File Number)

(I.R.S. Employer  
Identification No.)

777 Third Avenue, 22<sup>nd</sup> Floor, New York, NY

10017

(Address of principal executive offices)

(Zip Code)

Registrant's telephone number, including area code (646) 837-5863

(Former name or former address, if changed since last report.)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13a-4(c))

**Item 7.01. Regulation FD Disclosure.**

On February 13, 2014, Retrophin, Inc. released a presentation on its corporate website in connection with its conference call discussing its agreement to acquire Manchester Pharmaceuticals LLC. The presentation is attached hereto as Exhibit 99.1 and is incorporated by reference herein.

**Item 9.01 Financial Statements and Exhibits.**

(d) Exhibits

99.1 Conference call presentation, dated February 13, 2014

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**SIGNATURE**

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

RETROPHIN, INC.

Date: February 13, 2014

By: /s/ Marc Panoff

Name: Marc Panoff

Title: Chief Financial Officer

# Retrophin

Manchester Pharmaceuticals Acquisition  
February 13, 2013

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## 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 do not undertake any obligation to publicly update any forward-looking statements.

# Acquisition – Manchester Pharmaceuticals

		Pre-clinical	Phase I	Phase II	Phase III	Market
Chenodal	Gallstones					
Chenodal	Cerebrotendinous xanthomatosis	Orphan Drug Designation				
Vecamyl	Hypertension					
Vecamyl	Rage Disorders					

# Overview

- Retrophin to acquire Manchester Pharmaceuticals
  - Privately-held specialty pharmaceutical company with two FDA-approved products
    - Chenodal® (chenodeoxycholic acid)
    - Vecamyl® (mecamylamine)
- \$62.5m purchase price
  - \$29.5m paid upfront
  - Remaining payments to be delivered over 2014
  - Ongoing royalty on sales
- Highly accretive acquisition creates a fully-integrated specialty pharmaceutical company focused on catastrophic diseases

# Chenodal® (chenodeoxycholic acid)

- Chenodal (CDCA) is a synthetic bile acid approved for the treatment of gallstones, but...
- ...usage is exclusively in **cerebrotendinous xanthomatosis (CTX)**
  - CDCA is the standard of care for CTX
  - Chenodal is the only FDA-approved formulation of CDCA in the U.S.
    - Manchester received FDA approval of Chenodal in 2009
- Chenodal received Orphan Status for CTX in 2010
- Retrophin will file for approval in CTX in 2014
- Pricing for Chenodal is ~\$110,000 per patient per year
  - Retrophin believes there is upside to this price and will increase price to accommodate product expansion and patient identification efforts



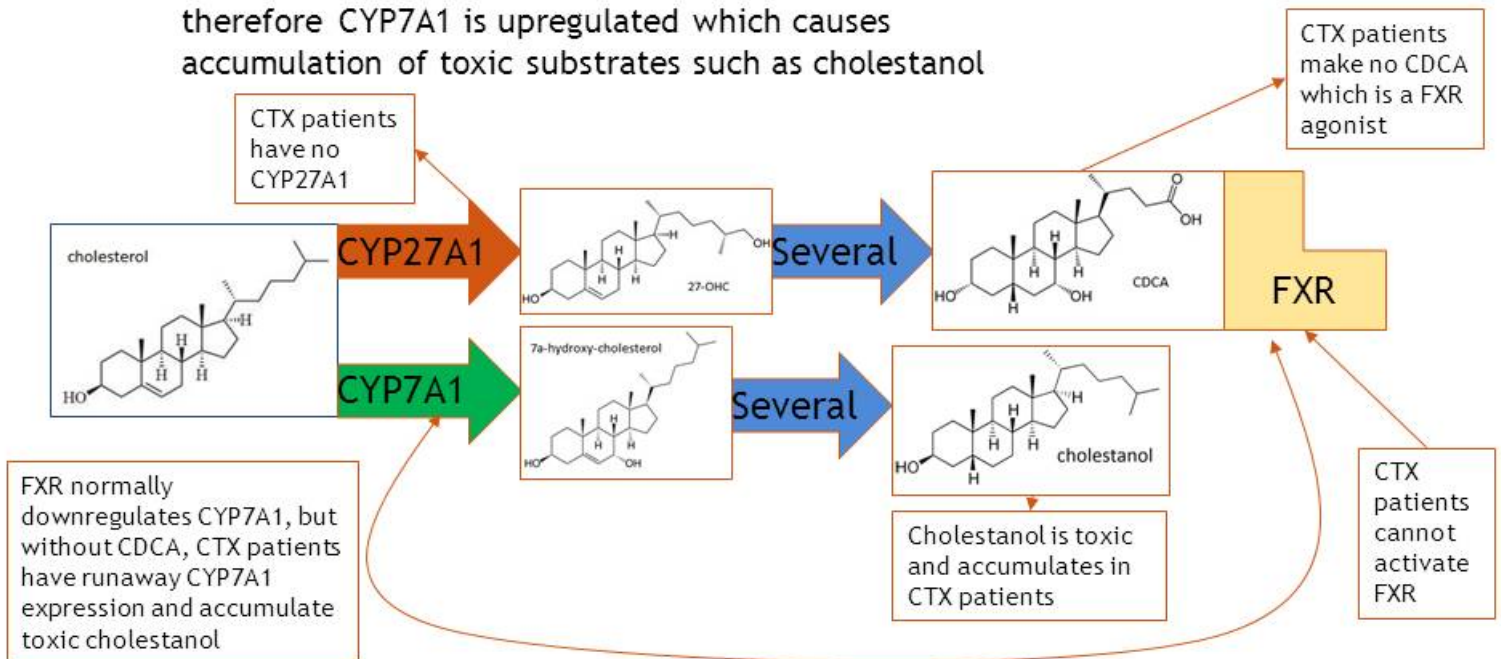


# Cerebrotendinous Xanthomatosis (CTX)

- Inborn error (autosomal recessive) of metabolism

- Mutation in CYP27A (sterol-27-hydroxylase)

- This enzyme converts cholesterol to CDCA
- CDCA binds to FXR and downregulates CYP7A1, which generates bile acids from cholesterol
- Patients with CTX mutations cannot make CDCA and therefore CYP7A1 is upregulated which causes accumulation of toxic substrates such as cholestanol



# Cerebrotendinous Xanthomatosis (CTX)

- CTX is a very difficult diagnosis to make
  - CTX patients begin life with neonatal cholestatic jaundice and refractory diarrhea
    - These common, non-critical and non-specific symptoms rarely lead to a CTX diagnosis
  - Disease progression then occurs with juvenile cataracts, tendon xanthomas (lipid deposition) and neurological deterioration (motor dysfunction, intellectual disabilities)
    - 95-97% of CTX patients have neurological symptoms at diagnosis
  - The disease can be lethal without Chenodal treatment

# CTX Epidemiology

- Due to the underdiagnosis of CTX, epidemiology data are limited
  - Published speculation of 1/1,000,000 to 1/50,000 prevalence
- Retrophin believes there are at least 500 - 1,000 U.S. CTX patients
  - Currently <5% - 10% are diagnosed / treated
- Retrophin believes that many CTX patients are **misdiagnosed** due to a lack of awareness and variable and non-specific presentation
  - An in-house survey of 5 KOLs who treat CTX patients estimate the incidence to be much higher than patients who are currently dosed
- Retrophin is confident that doctor awareness, newborn genetic screening and establishing a patient registry will rapidly identify more patients in the US and ROW

# Chenodal in CTX

- Chenodal replacement therapy is functionally curative for CTX patients
  - Measured by serum cholestanol
  - Healthy patients have little-to-no serum cholestanol
  - After Chenodal treatment, cholestanol drops ~98%
- Chenodal was never subjected to a clinical trial for CTX given its off-label discovery of efficacy
  - We believe a clinical trial would be unethical
  - Standard-of-care-status is unquestioned

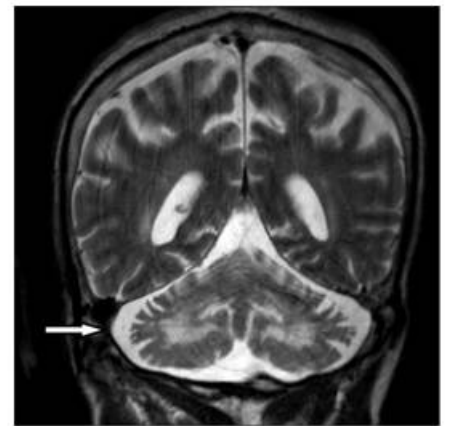


Fig 2. Cerebellar atrophy and hyperintense sign in dentate nuclei and adjacent cerebellar white matter on T2-weight RMI images (arrow), for a patient with cerebrotendinous xanthomatosis.

# Chenodal Pricing

- Price per patient per year (PPPY)
  - Wall Street focuses on this
- % of healthcare spend
  - Insurers focus on this

	US Revenue	Cost to HMO	Annual Cost / Life Covered	% of HMOs drug spend	PPPY	Generic Alternative?
Humira	6,668	1,000	24.40	1.334%		YES
Abilify	6,076	911	22.23	1.215%		YES
Januvia	3,120	468	11.41	0.624%		YES
Soliris	560	84	2.05	0.112%	450,000	NO
Fabrazyme	267	40	0.98	0.053%	300,000	NO
Cerezyme	239	36	0.87	0.048%	300,000	NO
Kalydeco	200	30	0.73	0.040%	307,236	NO
Myozyme	168	25	0.61	0.034%	600,000	NO
Elaprase	150	23	0.55	0.030%	500,000	NO
Vpriv	100	15	0.37	0.020%	300,000	NO
Naglazyme	50	8	0.18	0.010%	750,000	NO
Chenodal	5	1	0.02	0.001%	113,520	NO
<b>Total Ultra-Premium Price Segment</b>				<b>0.348%</b>		

# Global Opportunity

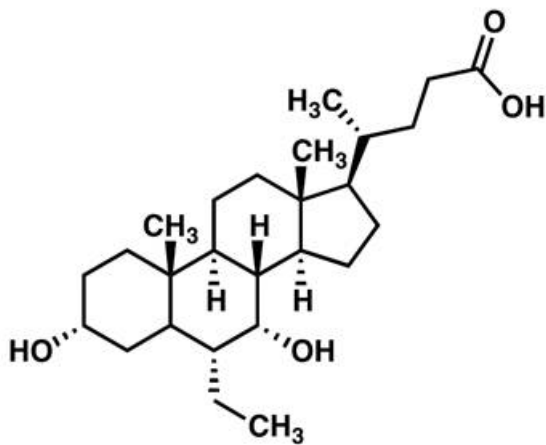
- Current supply of the only other available chenodeoxycholic acid product is limited to ROW markets and is spotty
  - Several product availability complaints
- Retrophin believes a large international opportunity exists
  - At least 1,000 international patients undiagnosed, untreated and without an alternative product
- ROW revenue is usually 50-90% of total revenue for most orphan drugs
  - Zero ROW revenue today
  - Retrophin targets at least 50% ROW revenue long-term

# Intellectual Property

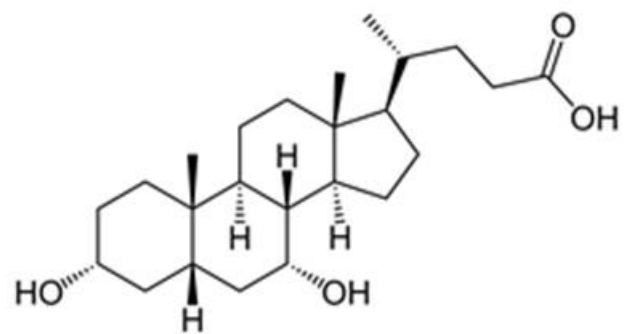
- Retrophin will seek FDA approval for Chenodal in CTX this year
  - Expect rapid approval and 7-year orphan status
- Centric specialty pharmacy distributor
  - Closed distribution system does not allow for generics to access product for bioequivalence study
    - ANDA filings are impossible unless generic company illegally penetrates specialty distributor
    - Recent *Celgene v. Lannett* case establishes precedent
- Retrophin plans to develop a once-a-day chenodeoxycholic acid and remove Chenodal from distribution at the appropriate time

## Other Indications

- FXR agonism has become a popular MOA and reaches across several therapeutic areas including hepatology and nephrology
- Possible additional indications for Chenodal include primary biliary cirrhosis (PBC) and nonalcoholic steatohepatitis (NASH)



Obeticholic acid (OCA)



Chenodeoxycholic acid (CDCA)



- Vecamyl has exhibited strong growth since its reintroduction to the market with no marketing
  - Retrophin is aware of off-label use in rage associated with autism spectrum disorder
- Retrophin plans to continue to make Vecamyl available but does not intend to engage in any marketing or promotional activities



# Forecasts

- 2014 revenue guidance of \$10 - \$12 million
- 2015 revenue guidance of \$19 - \$21 million
- Manchester EBITDA margins of 75-80%
- Potential peak sales of \$100-\$250 million

We estimate NPV of acquisition of Manchester of  
at least ~\$10 per Retrophin share

**Thank You!**

Retrophin

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