

Retrophin, Inc.
Form 8-K
September 08, 2014

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) September 8, 2014

RETROPHIN, INC.

(Exact name of registrant as specified in its charter)

Delaware
(State or other jurisdiction
of incorporation)

001-36257
(Commission
File Number)

27-4842691
(I.R.S. Employer
Identification No.)

777 Third Avenue, 22nd Floor, New York, NY
(Address of principal executive offices)

10017
(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 8.01 Other Events.

On September 8, 2014, Retrophin, Inc. (the “Company”) provided additional preliminary single patient data from the first Pantothenate kinase-associated neurodegeneration (“PKAN”) patient who began dosing with RE-024 under a physician-initiated research protocol on May 21, 2014. The subject remains on a stable dose of RE-024. The subject is clinically stable and has demonstrated sustained clinical improvement from baseline. Clinical data from this subject and the second subject who began dosing with RE-024 on July 24, 2014 are summarized in Table 1, and biochemical data from the first subject are shown in Table 2.

The Company does not believe data on a small number of patients can be interpreted in a robust fashion and is releasing this data to accommodate investor requests. Further, the results are inconclusive at this time with regard to RE-024’s ability to treat patients that suffer from PKAN. Potential investors should not place undue reliance on these early data points.

TABLE 1: Clinical data summary (as of September 3, 2014)

	Week 0	Week 1	Week 2	Week 3	Week 4	Week 5	Week 7	Week 11	Week 15
SUBJECT 1									
UPDRS1 (A+B+C)	74/118	56/118	56/118	63/118	58/118	56/118	48/118	50/118	47/118
Subscale A	8/10	3/10	3/10	3/10	4/10	3/10	2/10	3/10	2/10
Subscale B	36/52	26/52	26/52	27/52	26/52	26/52	23/52	22/52	21/52
Subscale C	30/56	27/56	27/56	33/56	28/56	27/56	23/56	25/56	24/56
BADS2	14/24	15/24	14/24	16/24	13/24	14/24	13/24	14/24	13/24
EQ-5D-3L	12/15	12/15	10/15	10/15	9/15	9/15	8/15	10/15	10/15
25 foot walk test									
# of steps	25.5	19.5	17.0	16.0	16.5	15.0	14.0	14.5	15.0
Time in seconds	10.5	11.3	8.6	11.1	8.9	8.2	7.6	7.8	6.4
SUBJECT 2									
UPDRS (A+B+C)	Week 0	Week 13							
Subscale A	4/10	4/10							
Subscale B	23/52	17/52							
Subscale C	26/56	15/56							
BADS	14/24	13/24							

1 Unified Parkinson’s Disease Rating Scale

2 Barry-Albright Dystonia Scale

3 Subject 2 Week one measurements were obtained on August 5, 2014

TABLE 2: Biochemical data summary (as of September 3, 2014)

	Week 0	Week 1	Week 2	Week 3	Week 4	Week 5	Week 7	Week 11	Week 15
SUBJECT									
1									
ALT (IU/L)	34	31	85	65	38	35	27	29	27
AST (IU/L)	26	23	55	32	29	30	26	28	27
Lactate (mmol/L)	2.44	1.42	1.46	1.29	1.69	1.07	1.28	1.39	1.7

ABOUT PKAN

Pantothenate kinase-associated neurodegeneration or PKAN is the most common form of neurodegeneration with brain iron accumulation. Classic PKAN is a genetic disorder that is typically diagnosed in the first decade of life. Consequences of PKAN include dystonia, dysarthria, rigidity, retinal degeneration, and severe digestive problems. PKAN is estimated to affect 1 to 3 persons per million. PKAN typically manifests in childhood with a profound, progressive dystonia and is usually lethal. There are currently no viable treatment options for patients with PKAN.

ABOUT RE-024

RE-024 is a phosphopantothenate prodrug replacement therapy with the goal of restoring the supply of this operative substrate in PKAN patients. The results discussed in this Current Report on Form 8-K relate to the first two PKAN patients who have initiated treatment with RE-024 and are not indicative of future responses by these patients or by other patients.

Forward Looking Statements

The foregoing report contains forward-looking statements, as that term is defined in the Private Securities Litigation Reform Act of 1995, regarding the research, development and commercialization of RE-024. These statements involve risks and uncertainties, and actual results may differ. Among other things, the Company faces risk that it will be unable to file an IND for RE-024 or initiate Phase I clinical trials for regulatory or other reasons, that it will be unable to raise additional funding required to complete development of RE-024 and that it is dependent on contractors for RE-024 clinical drug supply, commercial manufacturing and other preclinical development activities. You are cautioned not to place undue reliance on these forward-looking statements as there are important factors that could cause actual results to differ materially from those in forward-looking statements, many of which are beyond our control. The Company undertakes no obligation to publicly update forward-looking statements, whether as a result of new information, future events, or otherwise. Investors are referred to the full discussion of risks and uncertainties as included in the Company's filings with the Securities and Exchange Commission.

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: September 8, 2014

By: /s/ Marc Panoff
Name: Marc Panoff
Title: Chief Financial Officer