



## Retrophin, Inc. Reports Third Quarter 2013 Operational and Financial Results

November 18, 2013

NEW YORK--(BUSINESS WIRE)-- Retrophin, Inc. (OTCQB: RTRX) today announced the Company's financial results for the quarter ended September 30, 2013. The Company's Form 10-Q was filed on November 13, 2013.

### Third Quarter 2013 and Recent Highlights

- Phase II trial initiation activities for RE-021 in focal segmental glomerulosclerosis (FSGS) are underway, with "first-patient-in" expected in December 2013. FDA has indicated the results of this Phase II trial may serve as the basis for accelerated approval.
- Entered into exclusivity agreement with a major pharmaceutical company to negotiate a license to a product to be developed for Autism and Schizophrenia. Retrophin expects to close and announce the license agreement in December 2013.
- Reported positive survival data from preclinical trial of RE-024 for the treatment of the ultra-orphan disease, Pantothenate Kinase-Associated Neurodegeneration (PKAN). A Phase I emergency and compassionate use trial of RE-024 is expected to begin enrolling in December 2013 or January 2014.
- Expanded management team and Board of Directors with additions of Steven R. Eby, R.Ph as Vice President, Global Strategy and Program Management; Maria Beconi, Ph.D., as Vice President of Preclinical Development; Ronald Guido as Vice President of Regulatory Affairs; Jennifer Hunt as Vice President of Clinical Operations; Nils Olsson, Ph.D., as Vice President, Chemistry, Manufacturing and Control (CMC); Ryan Bucco Pharm.D., as Director of Medical Strategy; Kristyn Bogli, as Director of Clinical Logistics; and Cornelius E. Golding and Jeffrey Paley, MD as Directors.

Commenting on the quarter, Martin Shkreli, Founder and Chief Executive Officer of Retrophin, noted, "The third quarter represented a period of continued growth for Retrophin, both operationally and financially. During this time, we completed a successful PIPE financing, welcoming new investors to the Company and raising approximate gross proceeds of \$25 million to fund the continued research and development of our pipeline.

"We have also been active in our pursuit of new product candidates that may fit our long-term strategic objective of focusing on treatments for serious, unmet diseases. Toward this end, in mid-August we entered into an exclusivity agreement with a major pharmaceutical company allowing Retrophin to negotiate a U.S. license to a product to be developed for Autism and Schizophrenia. Given that there are no disease-modifying therapies available that treat autism, and there has not been a drug with a new mechanism of action to treat Schizophrenia in decades, both of these diseases represent important opportunities to fill an urgent, unmet medical need. Our discussions with the major pharmaceutical company have progressed significantly and we expect to announce the license agreement in December.

"Retrophin is also progressing toward initiation of enrollment for the Company's Phase II trial for RE-021 for the treatment of focal segmental glomerulosclerosis (FSGS). This potentially pivotal Phase 2 trial will be an eight-week, 100-patient, randomized, double-blind study, with a 40 week open-label extension period. The primary endpoint of the study is mean reduction in proteinuria from baseline compared to placebo. Study initiation activities are underway and Retrophin expects to initiate enrollment in December 2013. FDA has stated that the results of this Phase II trial may serve as the basis for accelerated approval of RE-021.

"RE-024 is Retrophin's pipeline candidate for the treatment of the ultra-orphan disease, pantothenate kinase-associated neurodegeneration (PKAN), a degenerative disease of the brain that primarily affects young children. In August 2013, Retrophin reported positive survival results from interim preclinical tests of RE-024, used as a replacement therapy for phosphopantothenate, the substrate that PKAN patients are unable to synthesize. Results indicated that RE-024 could be a potential, promising new approach to treating this debilitating and life-threatening disease. The Company's preclinical work continues to show promise, and we intend to initiate a Phase 1 emergency and compassionate use human clinical trial in December 2013 or January 2014."

Mr. Shkreli continued, "We are pleased with the pace of growth we have achieved during the most recent quarter, and as part of our strategy, we continue to seek out potentially transformative transactions that can leverage Retrophin's core strengths and expand our pipeline and commercialization opportunities.

"In tandem with our increasing activity level, we have expanded our management team with the addition of a number of highly talented employees, all of whom bring a wealth of experience in the biotechnology space, to execute on our strategic objectives. Specifically, we are pleased to welcome Steven R. Eby, R.Ph, Vice President; Global Strategy and Program Management; Maria Beconi, Ph.D., Vice President of Preclinical Development; Ronald Guido, Vice President of Regulatory Affairs; Jennifer Hunt, Vice President of Clinical Operations; Nils Olsson, Ph.D., Vice President, Chemistry, Manufacturing and Control (CMC); Ryan Bucco, Pharm.D., Director of Medical Strategy; and Kristyn Bogli, Director of Clinical Logistics. We have also added to our Board with newly appointed and distinguished members, Cornelius E. Golding and Dr. Jeff Paley. Neal and Jeff have more than 60 years' combined experience in healthcare and finance and will be invaluable members of our Board of Directors."

## Financial Status

Cash, cash equivalents and marketable securities at September 30, 2013 totaled approximately \$16.4 million, compared to \$11,388 as of December 31, 2012. Since inception, Retrophin has raised approximately \$38.5 million. Retrophin filed its third quarter 2013 financial results with the U.S. Securities and Exchange Commission on November 13, 2013.

## Conference Call Information

Management will hold a conference call today, November 18, 2013 at 4:30pm EST, to discuss the Company's third quarter operational and financial results. Access information is as follows:

Time: 4:30 pm EST  
Dial-in numbers: 877-703-6109 (U.S. and Canada) or 857-244-7308  
Conference ID number: 63839921  
Live web cast): [www.retrophin.com](http://www.retrophin.com), under "Investor Relations"

The teleconference replay will be available from 8:30 pm EST on Monday, November 18, 2013 through 11:59 pm EST on Monday, November 25, 2013. The replay number is 888-286-8010 (U.S. and Canada) or 617-801-6888, confirmation code 59828006.

## About Retrophin

Retrophin is a pharmaceutical company focused on the discovery and development of drugs for the treatment of debilitating and often life-threatening diseases for which there are currently no viable patient options. The Company is currently focused on several catastrophic diseases affecting children, including Focal Segmental Glomerulosclerosis (FSGS), Pantothenate Kinase-Associated Neurodegeneration (PKAN) and Duchenne Muscular Dystrophy. Retrophin's lead compound, RE-021, is scheduled to begin enrollment in a potentially pivotal Phase 2 clinical trial for the treatment of FSGS during 2013. For additional information, please visit [www.retrophin.com](http://www.retrophin.com).

## Forward-Looking Statements

This press release 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 pharmaceutical products and the future financial performance of the Company. 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 are based on current expectations and involve inherent risks and uncertainties, including factors that could delay, divert or change any of them, and could cause actual outcomes and results to differ materially from current expectations. No forward-looking statement can be guaranteed. Forward-looking statements in the press release should be evaluated together with the many uncertainties that affect the Company's business. 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. Any forward-looking statement speaks only as of the date on which such statement is made, and the Company undertakes no obligation to publicly update any forward-looking statement, whether as a result of new information, future events, or otherwise, to reflect any change in expectations or in events, conditions or circumstances on which any such statement may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements. Investors are referred to the full discussion of risks and uncertainties as included in the Company's filings with the Securities and Exchange Commission.

-Financial Tables to Follow -

### RETROPHIN, INC. AND SUBSIDIARY (A DEVELOPMENT STAGE COMPANY) CONDENSED CONSOLIDATED BALANCE SHEETS

	<u>September 30, 2013</u> (unaudited)	<u>December 31, 2012</u>
<b>Assets</b>		
Current assets:		
Cash	\$ 13,409,825	\$ 11,388
Marketable securities, available-for-sale	2,957,376	-
Prepaid expenses and other current assets	<u>480,647</u>	<u>21,830</u>
Total current assets	16,847,848	
Property and equipment, net	38,437	23,790
Patents pending	23,793	18,093
Due from affiliate	-	137,547
Security deposits	177,547	-
Deposits on license agreements	2,250,000	-
Technology license, net	<u>2,027,085</u>	<u>2,178,617</u>
Total assets	<u>\$ 21,364,710</u>	<u>\$ 2,358,047</u>
<b>Liabilities and Stockholders' Deficit</b>		
Current liabilities:		
Technology license liability	\$ -	\$ 1,300,000

Accounts payable	1,721,511	1,023,320
Accrued expenses	1,511,848	2,467,796
Settlements payable	1,691,400	-
Note payable - related party	-	884,764
Investors' deposits	-	100,000
Due to related parties	10,000	23,200
Derivative financial instruments, at estimated fair value - warrants	22,234,325	-
Total current liabilities	27,169,084	5,799,080
Stockholders' Deficit:		
Preferred stock Series A \$0.001 par value; 20,000,000 shares authorized; 0 issued and outstanding	-	-
Common stock \$0.0001 par value; 100,000,000 shares authorized; 18,376,363 and 8,952,905 issued and outstanding, respectively	1,838	895
Additional paid-in capital	48,649,970	30,203,402
Deficit accumulated during the development stage	(54,301,348)	(33,612,112)
Accumulated other comprehensive income	(154,834)	-
Total stockholders' deficit	(5,804,374)	(3,407,815)
Total liabilities and stockholders' deficit	\$ 21,364,710	\$ 2,391,265

**RETROPHIN, INC. AND SUBSIDIARY**  
**(A DEVELOPMENT STAGE COMPANY)**  
**UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS**

	For the three months ended		For the nine months ended		For the period from
	September 30,		September 30,		March 11, 2011
	2013	2012	2013	2012	(inception) through
					September 30, 2013
Operating expenses:					
Compensation and related costs - inclusive of share base compensation \$28,519, \$4,780,383, \$70,189, \$7,724,150 and \$17,808,002	\$ 478,741	\$ 5,068,707	\$ 1,767,195	\$ 8,371,481	\$ 22,127,948
Professional fees - inclusive of share based compensation \$1,640,501, \$2,247,292, \$1,849,745, \$6,290,252 and \$8,501,449	2,463,804	3,167,242	4,392,673	7,761,899	13,602,012
Research and development - inclusive of share based compensation \$72,888, \$0, \$109,571, \$0 and \$109,571	1,399,875	110,656	2,113,813	286,889	3,008,326
Selling, general and administrative	812,066	113,140	4,131,193	337,622	5,487,301
Technology license fee	-	-	100,000	-	1,800,000
Total operating expenses	5,154,486	8,459,745	12,504,874	16,757,891	46,025,587
Operating loss	(5,154,486)	(8,459,745)	(12,504,874)	(16,757,891)	(46,025,587)
Other income (expense):					
Interest income	4	6,049	9	15,781	21,914
Interest expense	-	(26,761)	(41,563)	(70,559)	(147,480)
Registration payment obligation income	360,000	-	360,000	-	360,000
Registration payment obligation expense	(360,000)	-	(360,000)	-	(360,000)
Realized gain on sale of marketable securities	59,737	-	59,737	-	59,737
Change in fair value of derivative financial instruments - warrants	(5,803,054)	-	(8,198,672)	-	(8,198,672)
Loss on transactions denominated in foreign currencies	-	-	(3,873)	-	(11,260)
Total other expense, net	(5,743,313)	(20,712)	(8,184,362)	(54,778)	(8,275,761)

Net loss	\$ <u>(10,897,799)</u>	\$ <u>(8,480,457)</u>	\$ <u>(20,689,236)</u>	\$ <u>(16,812,669)</u>	\$ <u>(54,301,348)</u>
Net loss per common share, basic and diluted	\$ <u>(0.71)</u>	\$ <u>(2.42)</u>	\$ <u>(1.62)</u>	\$ <u>(5.55)</u>	
Weighted average common shares outstanding, basic and diluted	<u>15,365,631</u>	<u>3,510,415</u>	<u>12,797,714</u>	<u>3,027,468</u>	
Comprehensive Loss:					
Net loss	\$ (10,897,799)	\$ (8,480,457)	\$ (20,689,236)	\$ (16,812,669)	\$ (54,301,348)
Unrealized loss on marketable securities	<u>(154,834)</u>	<u>-</u>	<u>(154,834)</u>	<u>-</u>	<u>(154,834)</u>
Comprehensive Loss	\$ <u>(11,052,633)</u>	\$ <u>(8,480,457)</u>	\$ <u>(20,844,070)</u>	\$ <u>(16,812,669)</u>	\$ <u>(54,456,182)</u>

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