

Retrophin LLC

COMPANY AND PIPELINE OVERVIEW REPORT

Coverage of the company and a summary of the drug pipeline portfolio.

Publication Date: 03-Mar-2013

THOMSON REUTERS

3 Times Square
New York, New York 10036
United States

Tel: +1 646 223 4000

thomsonreuters.com

[Return to Table of Contents](#)



ABOUT COMPANY AND PIPELINE OVERVIEW REPORT

Thomson Reuters provides the knowledge, tools, and expertise to help support drug discovery and development activities, IP portfolio optimization, identification of licensing and partnering opportunities, delivery of successful regulatory submissions, and the ability to keep current with the rapidly-changing pharmaceutical and chemical markets, supporting informed, early decisions.

This report was created by Thomson Reuters, using information from *Thomson Reuters Cortellis™ for Competitive Intelligence*; a comprehensive, proven intelligence solution that leverages the most accurate, complete, and widely respected drug pipeline information. From drug discovery and development activities to patent reports, the latest deals, and partnering opportunities, *Cortellis* can provide the confidence to make the most informed business decisions, faster. *Cortellis for Competitive Intelligence* provides accurate and validated information on pharmaceutical and biotechnology companies globally, their drug pipelines, deals, patents, and clinical trials, plus breaking industry news and conference coverage. All contained in one simple, highly intuitive research platform.

Cortellis Company and Pipeline Overview reports are the first in a series of reports that track pharmaceutical and biotechnology companies worldwide. Further report offerings planned to follow include: Company Detailed Pipeline and Company Competitive Landscape reports. All *Cortellis for Competitive Intelligence* content is subject to the most comprehensive editorial review process available, conducted by scientists, pharma professionals, regulatory experts, and generics specialists. Featuring timely drug pipeline information expertly uncovered and integrated from over 400 global meetings each year, you'll always be on top of the latest developments.

Chosen by leading life sciences companies, their executives and investors, *Cortellis for Competitive Intelligence* accelerates your deal-making and gives you timely insights on the development landscape.

Discover undiscovered opportunities in drug development and licensing faster with *Thomson Reuters Cortellis™ for Competitive Intelligence*

DISCLAIMER

The information contained in this report is based on sources believed to be correct but Thomson Reuters does not guarantee the accuracy, timeliness, or completeness of this information. Opinions, if any, are those held by the author of any individual report or article at the time of initial publication and do not necessarily reflect the views of Thomson Reuters.

Information in this report on companies is intended for reference use only, and does not constitute a recommendation to buy or sell any particular security or other investment and does not constitute an offer to buy from or sell to any particular investor. Any company or securities mentioned in this report may not be suitable for any particular investor, depending on that investor's financial position and needs.

[Return to Table of Contents](#)



GLOSSARY

Number of Drugs in Active Development

Number of drugs associated with the company or subsidiary that are currently in active development, i.e. the development status for the drug(s) is one of the following: Discovery, Clinical, Phase I, Phase II, Phase III, Pre-registration, Registered, Launched, or Suspended.

Number of Inactive Drugs

Number of drugs associated with the company or subsidiary that are currently classified as inactive, i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.

Number of Patents as Owner

Number of patents associated with the company where the company is listed as owner; i.e. the relationship type (or way the patent refers to the company) is: Patent Assignee/Owner, Patent owner (not assignee), Licensee for development and marketing, Licensee – marketing only (Distributor), Patent assignee of family member, Inferred assignee.

Number of Patents as Third Party

Number of patents associated with the company where the company is listed as third party; i.e. the relationship type (or way the patent refers to the company) is: Patent assignee (not owner), Ex-Licensee for development and marketing, Ex-Licensee marketing only (Distributor), Customer of technology, Ex-Customer of technology, Patent opponent or infringer, Affiliate organization of inventor, Owner of underlying technology.

Patents summary table

This table represents a summary of the core patent coverage for this company covering Therapeutic EP, US and WO patents since 1990 only.

Number of Deals

A count of deals where the company or one of its subsidiaries is the primary company.

Key Indications

Displays top ten key indications for the company and its subsidiaries based on frequency (indications occurring with high and identical frequency are always included, and this may result in more than ten Key Indications being listed). Includes both indications associated with patents where the company is patent owner and indications associated with drugs in active development. A drug is classified as 'active' if it features on a row (or rows) in the current development status table where the status is one of the following: Discovery, Clinical, Phase I, Phase II, Phase III, Pre-registration, Registered, Launched, or Suspended.

Key Target-based Actions

Displays top ten key target-based actions for the company and its subsidiaries based on frequency (actions occurring with high and identical frequency are always included, and this may result in more than ten Key Target-based Actions being listed). Includes both target-based actions associated with patents where the company patent owner and target-based actions associated with drugs in active development. A drug is classified as 'active' if it features on a row (or rows) in the current development status table where the status is one of the following: Discovery, Clinical, Phase I, Phase II, Phase III, Pre-registration, Registered, Launched, or Suspended. A target-based action is one that is associated with a target.

Key Technologies

Displays top ten key technologies for the company and its subsidiaries based on frequency (technologies occurring with high and identical frequency are always included, and this may result in more than ten Key Technologies being listed). Includes both key technologies associated with patents where the company relationship is patent owner and key technologies associated with drugs in active development. A drug is classified as 'active' if it features on a row (or rows) in the current development status table where the status is one of the following: Discovery, Clinical, Phase I, Phase II, Phase III, Pre-registration, Registered, Launched, or Suspended.

[Return to Table of Contents](#)



TABLE OF CONTENTS

Company Overview..... 5

Company Profile..... 6

Product Portfolio Summary..... 6

Product Portfolio Drugs..... 9

[Return to Table of Contents](#)

Retrophin LLC

COMPANY OVERVIEW

Company Name	Retrophin LLC
Parent Company Name	Retrophin LLC
Website	http://www.retrophin.com/
Country	US
Number of Drugs in Active Development	6
Number of Inactive Drugs	0
Number of Patents as Owner	0
Number of Patents as Third Party	0
Number of Deals	2
Key Indications	Cystic fibrosis,Duchenne dystrophy,Focal segmental glomerulosclerosis,Hallervorden-Spatz syndrome,IgA nephropathy,Myopathy,Spinal muscular atrophy
Key Target-based Actions	Myotubularin modulator
Key Technologies	Small molecule therapeutic,Biological therapeutic,Protein fusion,Protein recombinant

COMPANY PROFILE

SUMMARY

Retrophin LLC is a biotechnology company focused on discovering and developing treatments for rare and life-threatening diseases.

COMPANY LOCATION

Retrophin is based in New York, NY.

In September 2012, Retrophin planned to relocate its headquarters from New York, NY to San Diego, CA.

ACQUISITIONS & SPIN-OFFS

In December 2012, Retrophin completed a reverse merger with Desert Gateway. The combined company would be publicly-traded on the OTC stock market under the ticker symbol 'RTRX'.

FINANCIAL

In February 2013, the company was to raise a gross proceeds of approximately \$10 million from the issuance of 3,333,332 shares of common stock and warrants to purchase an additional 1,530,559 shares of common stock. At that time, the transaction was expected to occur in the week of February 11, 2013.

In January 2013, Ligand Pharmaceuticals received milestone payment of 620,000 common stock shares in Retrophin, representing 7% of Retrophin's outstanding capital stock; the payment was triggered by the completion of Retrophin's merger with Desert Gateway.

In May 2012, the company raised \$4 million from the completion of its series A financing round led by MSMB Capital.

[Return to Table of Contents](#)

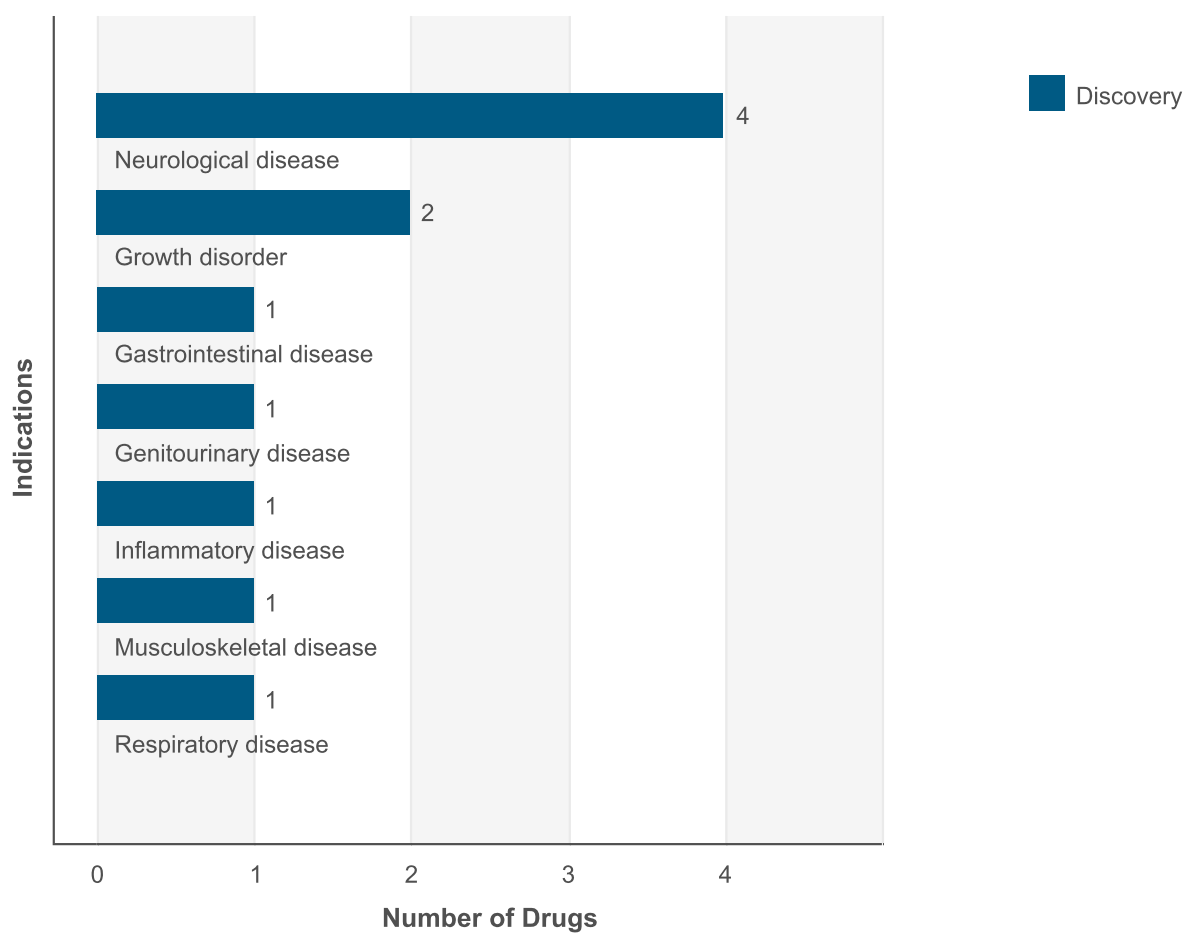


PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



Drugs by Indication Table

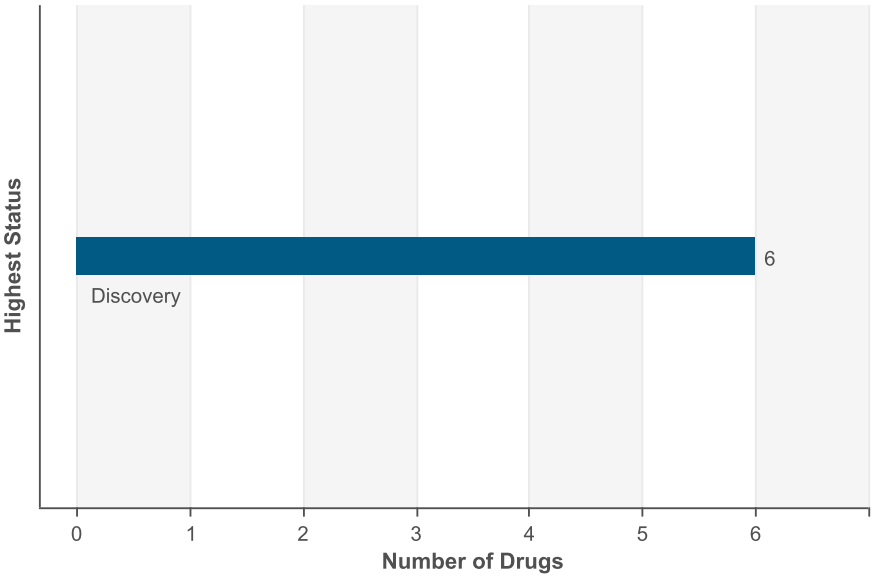
Indication	Active	Inactive	Total
Neurological disease	4	0	4
Growth disorder	2	0	2
Respiratory disease	1	0	1
Genitourinary disease	1	0	1
Inflammatory disease	1	0	1
Gastrointestinal disease	1	0	1

[Return to Table of Contents](#)

Musculoskeletal disease	1	0	1
-------------------------	---	---	---

Drugs by Highest Status

Active Drugs by Highest Status Chart



Drugs by Highest Status Table

Development Status	Number of Drugs
Discovery	6

DEALS

Deal Type	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Drug - Early Research/Development	0	0	1	0	1
Drug - Development/Commercialization License	0	0	1	0	1

CLINICAL TRIALS

Trials by Condition Studied

Condition Studied	Ongoing	All
Inflammatory disease	2	2
Genitourinary disease	2	2

[Return to Table of Contents](#)

Trials by Phase

Phase	Ongoing	All
Phase 2	1	1
Phase not specified	1	1

Phase Definitions

Phase 3 Clinical

Includes Phase 3, Phase 3b, Phase 3a, Phase 2/3 (where enrolment count is 300 or over)

Phase 2 Clinical

Includes Phase 2, Phase 2a, Phase 2b, Phase 1/2 (where enrolment count is 100 or over), Phase 2/3 (where enrolment count is under 300 or not specified)

Phase 1 Clinical

Includes Phase 1, Phase 1a, Phase 1, Phase 1/2 (where enrolment count is under 100 or not specified), Phase 0

[Return to Table of Contents](#)

PRODUCT PORTFOLIO DRUGS

PLEASE NOTE: Highest status refers to highest development of that drug for one of the active companies

RE-021

Drug Name	RE-021
Key Synonyms	
Originator Company	Bristol-Myers Squibb Pharmaceutical Research Institute
Active Companies	Retrophin LLC
Inactive Companies	Ligand Pharmaceuticals Inc, Pharmacopeia Inc, Bristol-Myers Squibb Pharmaceutical Research Institute
Highest Status	Discovery
Active Indications	IgA nephropathy, Focal segmental glomerulosclerosis
Target-based Actions	Angiotensin II receptor antagonist, Endothelin ET-A antagonist
Other Actions	Anti-inflammatory, Cardioprotectant, Antihypertensive
Technologies	Oral formulation, Tablet formulation, Small molecule therapeutic
Last Change Date	10-Jan-2013

RE-001

Drug Name	RE-001
Key Synonyms	
Originator Company	Retrophin LLC
Active Companies	Retrophin LLC
Inactive Companies	
Highest Status	Discovery
Active Indications	Duchenne dystrophy
Target-based Actions	
Other Actions	Muscle system agent, Unspecified drug target
Technologies	Biological therapeutic, Protein fusion, Protein recombinant
Last Change Date	09-May-2012

[Return to Table of Contents](#)



RE-002

Drug Name	RE-002
Key Synonyms	
Originator Company	Retrophin LLC
Active Companies	Retrophin LLC
Inactive Companies	
Highest Status	Discovery
Active Indications	Myopathy
Target-based Actions	Myotubularin modulator
Other Actions	Musculoskeletal system modulator
Technologies	Small molecule therapeutic
Last Change Date	09-May-2012

RE-003

Drug Name	RE-003
Key Synonyms	
Originator Company	Retrophin LLC
Active Companies	Retrophin LLC
Inactive Companies	
Highest Status	Discovery
Active Indications	Spinal muscular atrophy
Target-based Actions	
Other Actions	
Technologies	Small molecule therapeutic
Last Change Date	16-May-2012

[Return to Table of Contents](#)

RE-013

Drug Name	RE-013
Key Synonyms	
Originator Company	Retrophin LLC
Active Companies	Retrophin LLC
Inactive Companies	
Highest Status	Discovery
Active Indications	Cystic fibrosis
Target-based Actions	
Other Actions	Anti-inflammatory, Unspecified drug target
Technologies	Small molecule therapeutic
Last Change Date	16-May-2012

RE-024

Drug Name	RE-024
Key Synonyms	
Originator Company	Retrophin LLC
Active Companies	Retrophin LLC
Inactive Companies	
Highest Status	Discovery
Active Indications	Hallervorden-Spatz syndrome
Target-based Actions	
Other Actions	Neuroprotectant
Technologies	Small molecule therapeutic
Last Change Date	19-Dec-2012

[Return to Table of Contents](#)

This report was created by Thomson Reuters, using information from *Thomson Reuters Cortellis™ for Competitive Intelligence*; a comprehensive, proven intelligence solution that leverages the most accurate, complete, and widely respected drug pipeline information.

For more information about *Cortellis for Competitive Intelligence*, visit:

http://cortellis.thomsonreuters.com/cortellis_for_you/?cid=thomsonone.

For subscription information, e-mail scientific.lifesciences@thomsonreuters.com.

© 2012 Thomson Reuters. All rights reserved.
Republication or redistribution of Thomson Reuters content, including by framing or similar means, is prohibited without the prior written consent of Thomson Reuters. 'Thomson Reuters' and the Thomson Reuters logo are registered trademarks and trademarks of Thomson Reuters and its affiliated companies.

[Return to Table of Contents](#)

