THOMSON REUTERS
CORTELLIS™

Hyperion Therapeutics Inc

CORTELLIS COMPANY DETAILED PIPELINE REPORT

A comprehensive coverage of the the company's drug pipeline portfolio including detailed product records.

Publication Date: 06-Jan-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

THOMSON REUTERS

ABOUT CORTELLIS COMPANY DETAILED PIPELINE 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 accurateand 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 Detailed Pipeline reports are the second in a series of that track pharmaceutical and biotechnology companies worldwide. 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 Drug Pipeline Detail..... 9
Launched..... 10
Pre-registration..... 14

Return to Table of Contents

Hyperion Therapeutics Inc

COMPANY OVERVIEW

Company Name	Hyperion Therapeutics Inc
Parent Company Name	Hyperion Therapeutics Inc
Website	http://www.hyperiontx.com/
Country	US
Number of Drugs in Active Development	2
Number of Inactive Drugs	0
Number of Patents as Owner	3
Number of Patents as Third Party	0
Number of Deals	1
Key Indications	Hepatic encephalopathy,Hyperammonemia,Uremia
Key Target-based Actions	
Key Technologies	Prodrug

COMPANY PROFILE

SUMMARY

Hyperion Therapeutics Inc focuses on gastrointestinal and hepatology programs and products that address unmet medical needs to improve patient care.

FINANCIAL

In April 2012, Hyperion filed a registration statement with the SEC for the issual of initial public offering of its common stock; at that time, the offering details were not disclosed; in July 2012, the company priced its initial public offering of 5 million shares of its common stock at $10 per share. At that time, underwriters were granted 30-day option to purchase up to an additional 750,000 shares of common stock to cover any over-allotments. The offering was expected to close on July 31, 2012; later that month, the offering was completed, with underwriters exercising their overallotment option in full.

In June 2009, Hyperion rasied $60 million from a series C financing.

In September 2007, Hyperion raised $4 million from a series B financing.

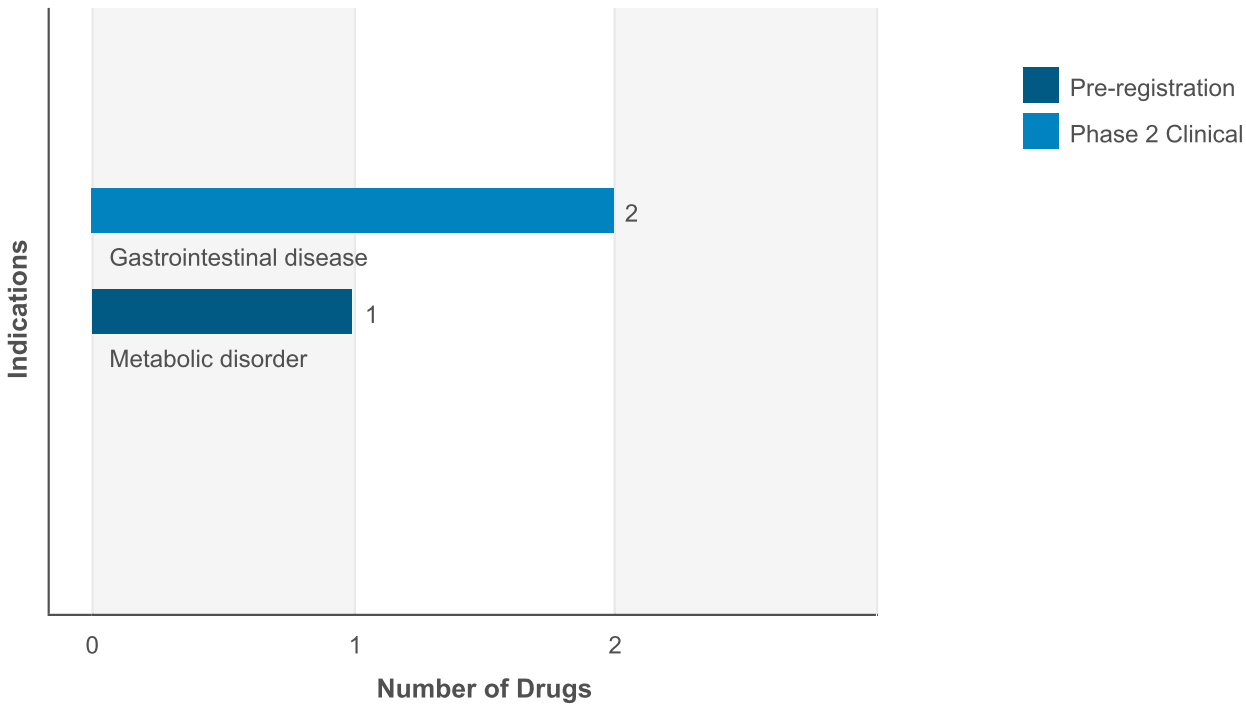
Return to Table of Contents

PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



Drugs by Indication Table

Indication	Active	Inactive	Total
Gastrointestinal disease	2	0	2
Metabolic disorder	1	0	1

Return to Table of Contents

Drugs by Highest Status

Active Drugs by Highest Status Chart

Drugs by Highest Status Table

Development Status	Number of Drugs
Pre-registration	1
Phase 2 Clinical	1

DEALS

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

CLINICAL TRIALS

Trials by Condition Studied

Condition Studied	Ongoing	All
Metabolic disorder	1	9
Genitourinary disease	1	7
Gastrointestinal disease	1	7
Genetic disorder	0	1

Return to Table of Contents

Trials by Phase

Phase	Ongoing	All
Phase 4	1	1
Phase 3	0	3
Phase 2	0	5
Phase 1	0	4

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

PATENTS *

Indication	As Owner	As Third Party	Total
Neoplasm	1	0	1
Metabolic disorder	2	0	2
Neurological disease	1	0	1
Degeneration	1	0	1
Genitourinary disease	2	0	2
Gastrointestinal disease	2	0	2
Hematological disease	1	0	1

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

Return to Table of Contents

PRODUCT PORTFOLIO DRUG PIPELINE DETAIL

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

sodium phenylacetate + sodium benzoate (hyperammonemia), Ucyclyd

sodium phenylacetate + sodium benzoate (hyperammonemia), Ucyclyd SNAPSHOT

Drug Name	sodium phenylacetate + sodium benzoate (hyperammonemia), Ucyclyd
Key Synonyms	Ammonul
Originator Company	Ucyclyd Pharma Inc
Active Companies	Hyperion Therapeutics Inc;Ucyclyd Pharma Inc
Inactive Companies	
Highest Status	Launched
Active Indications	Hepatic encephalopathy;Hyperammonemia
Target-based Actions	
Other Actions	Nitrogen metabolism modulator
Technologies	Drug combination;Intravenous formulation;Small molecule therapeutic
Last Change Date	18-Oct-2011

sodium phenylacetate + sodium benzoate (hyperammonemia), Ucyclyd DEVELOPMENT PROFILE

SUMMARY

Ucyclyd Pharma has developed and launched Ammonul, a combination of sodium phenylacetate + sodium benzoate, as an ammonia scavenger for the iv treatment of hyperammonemia. Licensee Hyperion Therapeutics is developing the drug for the potential treatment of hepatic encephalopathy. In December 2007, Hyperion began a phase II trial in patients with hepatic encephalopathy which was expected to be completed in February 2009.

sodium phenylacetate + sodium benzoate (hyperammonemia), Ucyclyd DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Ucyclyd Pharma Inc	Hyperammonemia	US	Launched	28-Aug-2007
Hyperion Therapeutics Inc	Hepatic encephalopathy	US	Phase 2 Clinical	31-Dec-2007

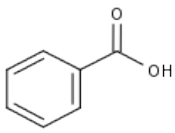
sodium phenylacetate + sodium benzoate (hyperammonemia), Ucyclyd CHEMICAL STRUCTURES

Return to Table of Contents

CAS Registry Number:	Confidence Level:
	2

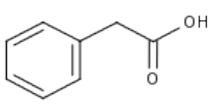
Name	Type
Ammonul	Trade Name
sodium phenylacetate + sodium benzoate	

CAS Registry Number:	Confidence Level:
65-85-0	2



Name	Type
benzoic acid	

CAS Registry Number:	Confidence Level:
103-82-2	2



Name	Type
phenylacetic acid	

Return to Table of Contents

sodium phenylacetate + sodium benzoate (hyperammonemia), Ucyclyd DRUG NAMES

Names	Type
sodium phenylacetate + sodium benzoate (hyperammonemia), Ucyclyd	
sodium phenylacetate + sodium benzoate	
Ammonul	Trade Name

sodium phenylacetate + sodium benzoate (hyperammonemia), Ucyclyd CLINICAL TRIALS

Trials by Phase and Condition Studied

Phase 4 Clinical		Phase 3 Clinical		Phase 2 Clinical		Phase 1 Clinical		Phase Unspecified		Total	
On-going	All	On-going	All	On-going	All	On-going	All	On-going	All	On-going	All
Hepatic encephalopathy											
0	0	0	0	0	1	0	0	0	0	0	1

Total Trials by Phase and Status

Phase 4 Clinical		Phase 3 Clinical		Phase 2 Clinical		Phase 1 Clinical		Phase Unspecified		Total	
On-going	All	On-going	All	On-going	All	On-going	All	On-going	All	On-going	All
Total by Phase and Status											
0	0	0	0	0	1	0	0	0	0	0	1

Phase Definitions

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

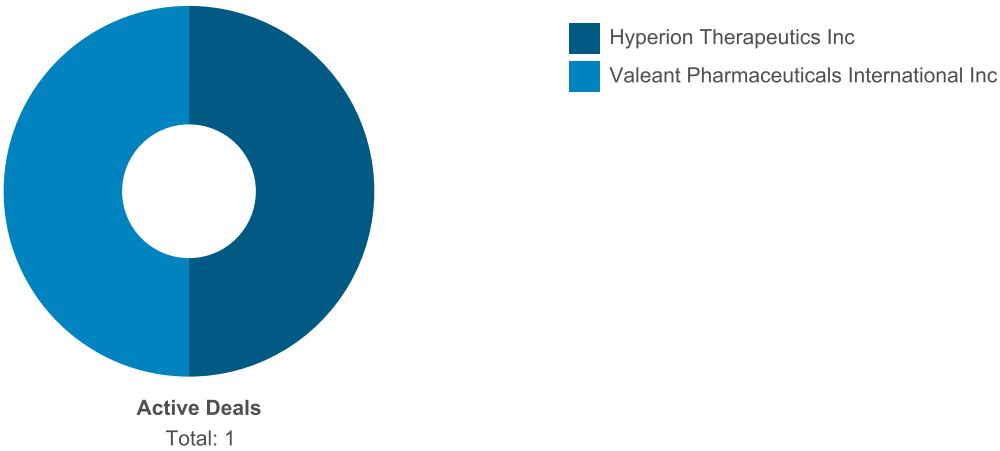
Phase 2 Clinical
Includes Phase 2, Phase 2a, Phase 2b, Phase 1/2 (where enrolment count is 100 or over), Phase 2/3 (where enrolment count is under 300 or not specified)

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

DEALS

Deals by Parent Company Chart

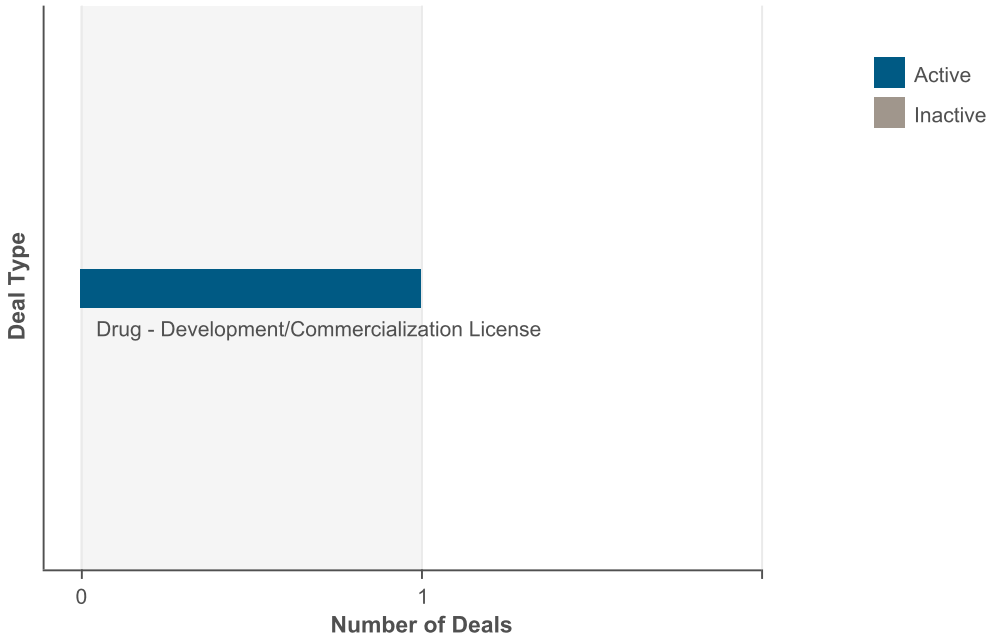


Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Hyperion Therapeutics Inc	0	0	1	0	1
Valeant Pharmaceuticals International Inc	1	0	0	0	1

Return to Table of Contents

Deals by Type Chart



Deals by Type Table

Deal Type	Active	Inactive	Total
Drug - Development/Commercialization License	1	0	1

Return to Table of Contents

glycerol phenylbutyrate

glycerol phenylbutyrate SNAPSHOT

Drug Name	glycerol phenylbutyrate
Key Synonyms	glycerol phenylbutyrate;Ravicti
Originator Company	Ucyclyd Pharma Inc
Active Companies	Hyperion Therapeutics Inc
Inactive Companies	Ucyclyd Pharma Inc
Highest Status	Pre-registration
Active Indications	Hyperammonemia;Hepatic encephalopathy
Target-based Actions	
Other Actions	Nitrogen metabolism modulator
Technologies	Prodrug;Oral formulation;Oral liquid formulation;Small molecule therapeutic
Last Change Date	08-Dec-2012

glycerol phenylbutyrate DEVELOPMENT PROFILE

SUMMARY

Hyperion Therapeutics, under license from Ucyclyd Pharma, has developed Ravicti, an oral liquid formulation of ammonia-scavenging glycerol phenylbutyrate (HPN-100, glyceryl tri-(4-phenylbutyrate), GT4P), a prodrug of phenylbutyrate (pre-prodrug of phenylacetate), for the potential treatment of urea cycle disorders (UCD) and hepatic encephalopathy (HE),. By March 2012, an NDA for use of drug as an adjunctive therapy for chronic management of urea cycle disorders was under review by the FDA. The PDUFA date was set for October 23, 2012 ; in September 2012, the FDA extended the PDUFA date to January 23, 2013. In October 2009, a US phase III trial for UCD began. In October 2009, a phase II trial was initiated for hepatic encephalopathy in the US and Eastern Europe ; in June 2012, data were reported.

glycerol phenylbutyrate DEVELOPMENT STATUS

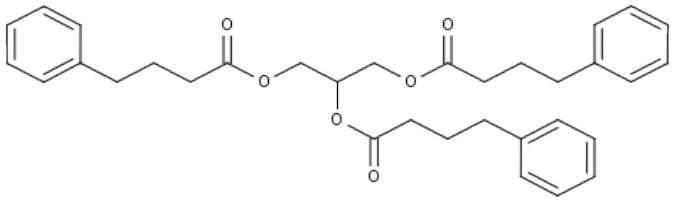
CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Hyperion Therapeutics Inc	Hyperammonemia	US	Pre-registration	22-Mar-2012
Hyperion Therapeutics Inc	Hepatic encephalopathy	Eastern Europe	Phase 2 Clinical	03-Dec-2009
Hyperion Therapeutics Inc	Hepatic encephalopathy	US	Phase 2 Clinical	08-Oct-2009
Ucyclyd Pharma Inc	Hepatic encephalopathy	US	Discontinued	28-Aug-2007
Ucyclyd Pharma Inc	Hyperammonemia	US	Discontinued	28-Aug-2007

Return to Table of Contents

glycerol phenylbutyrate CHEMICAL STRUCTURES

CAS Registry Number:	Confidence Level:
611168-24-2	2



Name	Type
glycerol phenylbutyrate	INN; USAN
GT4P	Research Code
HPN-100	Research Code
glyceryl tri-(4-phenylbutyrate)	

glycerol phenylbutyrate DRUG NAMES

Names	Type
HPN-100	Research Code
glyceryl tri-(4-phenylbutyrate)	
glycerol phenylbutyrate	INN, USAN
GT4P	Research Code
Ravicti	Trade Name

glycerol phenylbutyrate CLINICAL TRIALS

Trials by Phase and Condition Studied

Phase 4 Clinical		Phase 3 Clinical		Phase 2 Clinical		Phase 1 Clinical		Phase Unspecified		Total	
On-going	All	On-going	All	On-going	All	On-going	All	On-going	All	On-going	All
Uremia											
1	1	0	1	0	2	0	3	0	0	1	7

Return to Table of Contents

Hepatic encephalopathy											
1	1	0	0	0	2	0	3	0	0	1	6
Hyperammonemia											
1	1	0	0	0	2	0	2	0	0	1	5
Liver cirrhosis											
0	0	0	0	0	1	0	0	0	0	0	1
Genetic disorder											
0	0	0	1	0	0	0	0	0	0	0	1
Metabolic disorder											
0	0	0	1	0	0	0	0	0	0	0	1

Total Trials by Phase and Status

Phase 4 Clinical		Phase 3 Clinical		Phase 2 Clinical		Phase 1 Clinical		Phase Unspecified		Total	
On-going	All	On-going	All	On-going	All	On-going	All	On-going	All	On-going	All
Total by Phase and Status											
1	1	0	3	0	4	0	4	0	0	1	12

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)

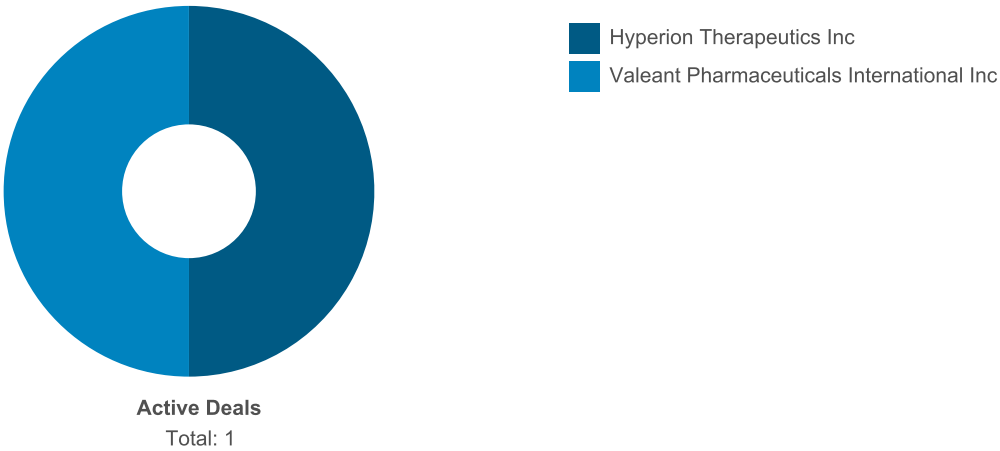
Phase 1 Clinical
Includes Phase 1, Phase 1a, Phase 1, Phase 1/2 (where enrolment count is under 100 or not specified), Phase 0

Return to Table of Contents

glycerol phenylbutyrate DEALS AND PATENTS

DEALS

Deals by Parent Company Chart

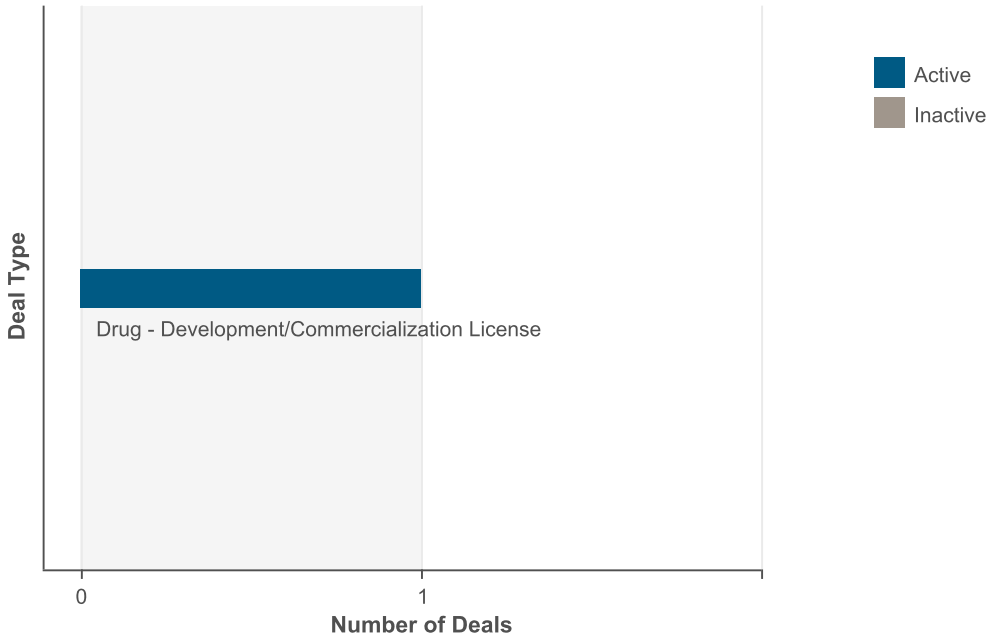


Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Hyperion Therapeutics Inc	0	0	1	0	1
Valeant Pharmaceuticals International Inc	1	0	0	0	1

Return to Table of Contents

Deals by Type Chart



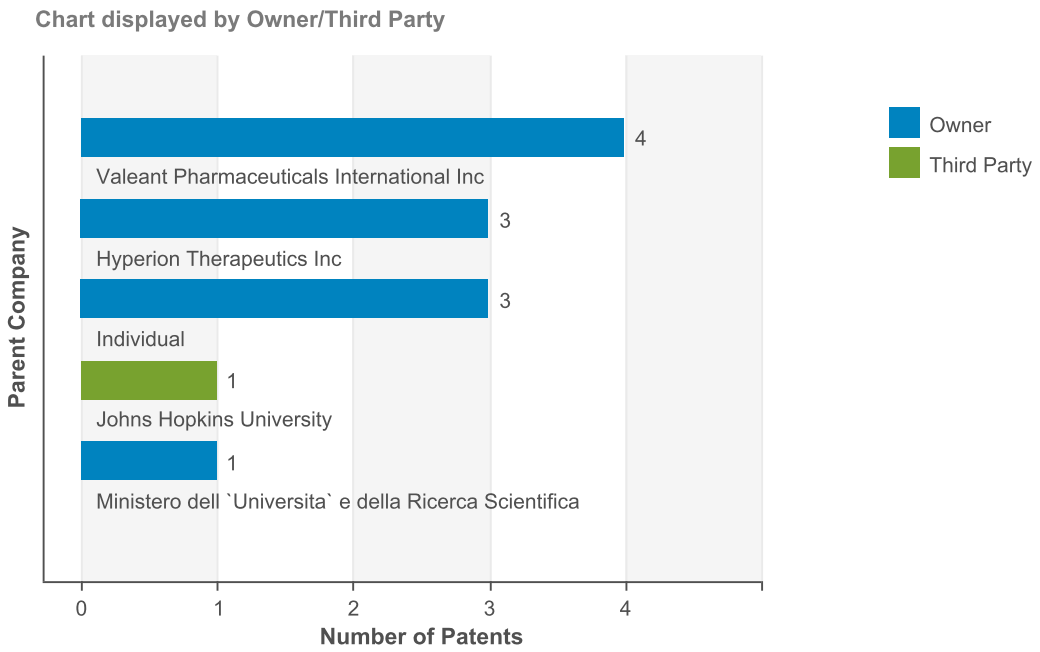
Deals by Type Table

Deal Type	Active	Inactive	Total
Drug - Development/Commercialization License	1	0	1

Return to Table of Contents

PATENTS

Patents by Parent Company Chart

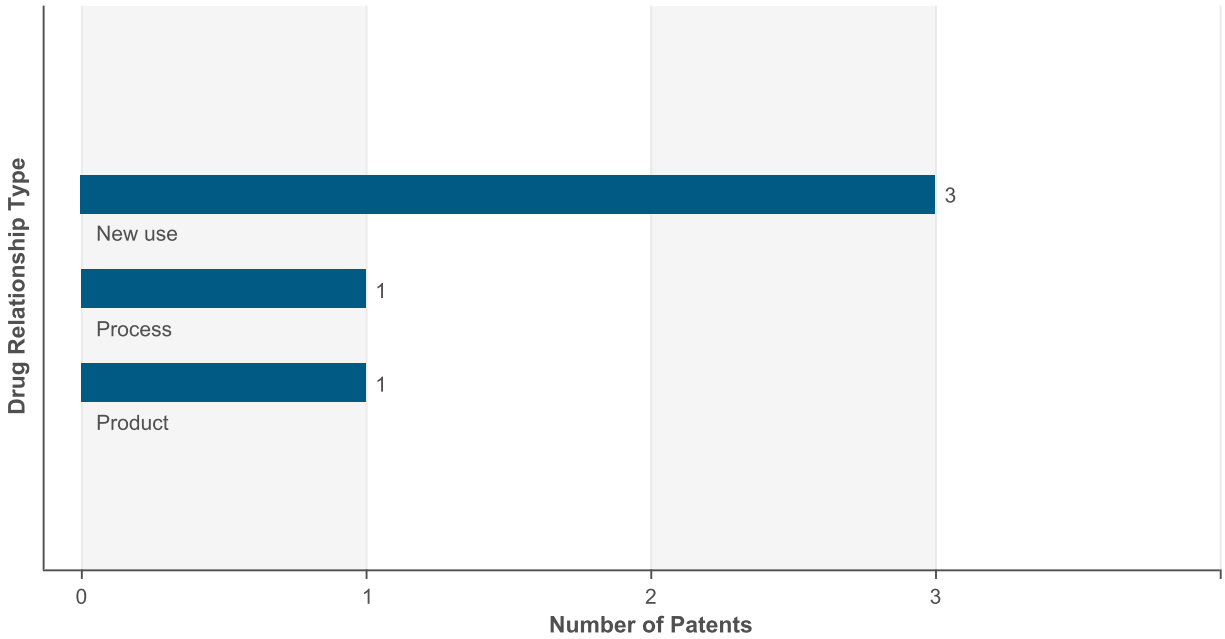


Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
Valeant Pharmaceuticals International Inc	4	0	4
Individual	3	0	3
Hyperion Therapeutics Inc	3	0	3
Ministero dell `Universita` e della Ricerca Scientifica	1	0	1
Johns Hopkins University	0	1	1

Return to Table of Contents

Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
New use	3
Process	1
Product	1

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.

Return to Table of Contents