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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: 02-Jun-2013

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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.

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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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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.

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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	3
Number of Inactive Drugs	0
Number of Patents as Owner	4
Number of Patents as Third Party	0
Number of Deals	1
Key Indications	Hepatic encephalopathy,Uremia,Hyperammonemia
Key Target-based Actions	
Key Technologies	Analytical method,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 May 2013, the company was to be listed on the NASDAQ Biotechnology Index under the symbol 'HPTX', following the semi-annual re-ranking of the list, effective from May 20, 2013.

In March 2013, the company priced a follow-on offering of 2,875,000 common stock shares at $20.75 each. At that time, the underwriters were granted a 30-day option to buy an additional 431,250 shares of common stock to cover over-allotments, if any. The offering was expected to close on March 13, 2013; later that month, the offering was closed with net proceeds of approximately $64.5 million; in May 2012, the company reported that the net proceeds from the offering was $63.7 million.

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.

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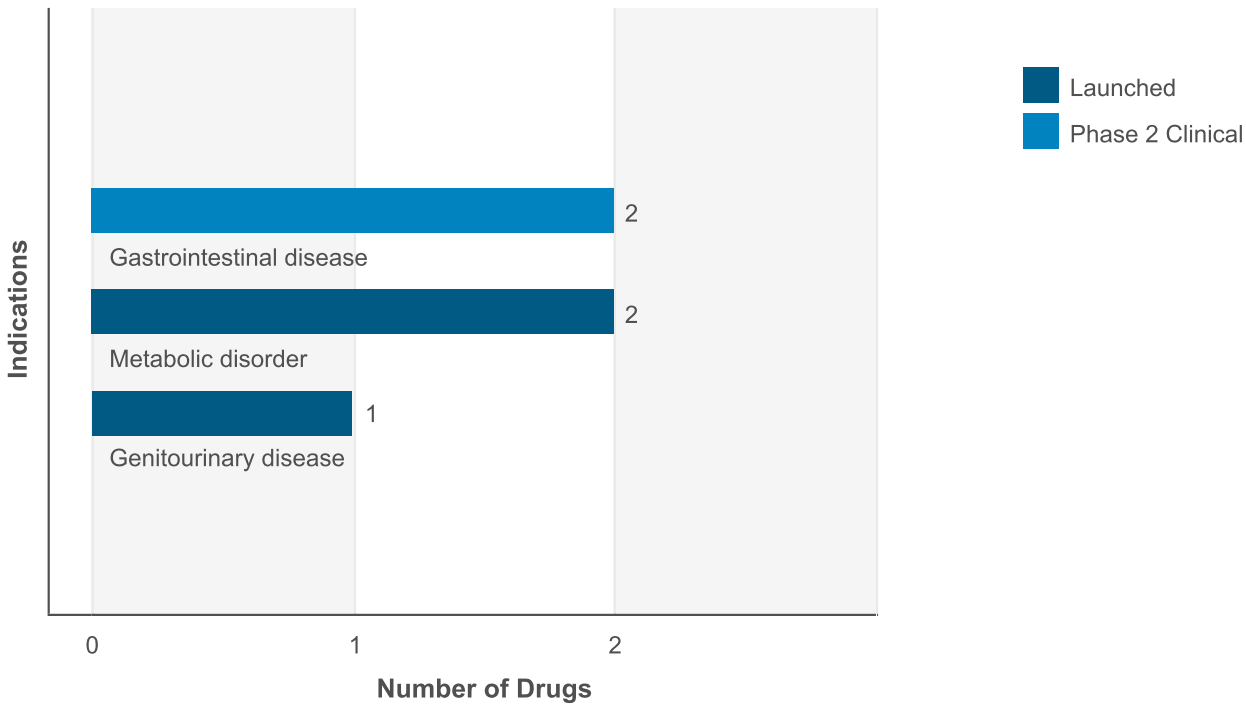
PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



Drugs by Indication Table

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

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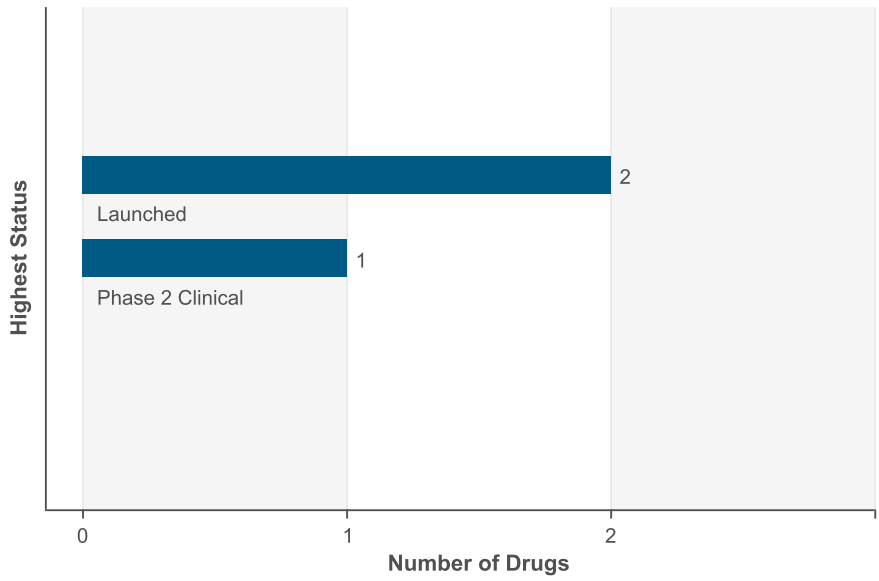
Drugs by Highest Status

Active Drugs by Highest Status Chart



Drugs by Highest Status Table

Development Status	Number of Drugs
Launched	2
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
Gastrointestinal disease	1	7
Genitourinary disease	1	7
Genetic disorder	0	1

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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
Gastrointestinal disease	3	0	3
Genitourinary disease	3	0	3
Hematological disease	1	0	1
Degeneration	1	0	1
Neoplasm	1	0	1
Metabolic disorder	3	0	3
Neurological 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.

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PRODUCT PORTFOLIO DRUG PIPELINE DETAIL

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

sodium phenylbutyrate, Hyperion / Swedish Orphan

sodium phenylbutyrate, Hyperion / Swedish Orphan SNAPSHOT

Drug Name	sodium phenylbutyrate, Hyperion / Swedish Orphan
Key Synonyms	Buphenyl;Ammonaps
Originator Company	Ucyclyd Pharma Inc
Active Companies	Ucyclyd Pharma Inc;Hyperion Therapeutics Inc;Orphan Pacific, Inc;Swedish Orphan Biovitrum AB
Inactive Companies	CMIC Co Ltd
Highest Status	Launched
Active Indications	Uremia
Target-based Actions	Histone deacetylase inhibitor
Other Actions	Renal system agent;Nitrogen metabolism modulator
Technologies	Tablet formulation;Oral formulation;Small molecule therapeutic;Formulation powder
Last Change Date	20-May-2013

sodium phenylbutyrate, Hyperion / Swedish Orphan DEVELOPMENT PROFILE

SUMMARY

Hyperion Therapeutics, following a product acquisition from Ucyclyd Pharma (now part of Medicis (a subsidiary of Valeant)) has developed and launched tablet and powder formulations of sodium phenylbutyrate (Ammonaps, Buphenyl) for the treatment of patients with urea cycle disorders,. In December 1999, the drug was approved in Denmark and was subsequently launched. At that time, the drug was approved in Ireland. By November 2000, the drug had been launched in the US, Canada, Russia, Australia and Europe. In November 2000, the drug was launched in France. In March 2002, tablet formulation was approved in Spain by Swedish Orphan Biovitrum and was subsequently launched. In May 2006, Swedish Orphan Biovitrum was marketing the drug in France. In June 2006, the drug was launched in Sweden. In March 2007, the drug was launched in Portugal by Swedish Orphan Biovitrum. In September 2012, Japanese licensee, CMIC received approval in Japan ; in November 2012, CMIC's marketing rights were transferred to its subsidiary, Orphan Pacific.

sodium phenylbutyrate, Hyperion / Swedish Orphan DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Hyperion Therapeutics Inc	Uremia	Australia	Launched	02-May-2013
Hyperion Therapeutics Inc	Uremia	Canada	Launched	02-May-2013

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Company	Indication	Country	Development Status	Date
Hyperion Therapeutics Inc	Uremia	France	Launched	02-May-2013
Hyperion Therapeutics Inc	Uremia	Germany	Launched	02-May-2013
Hyperion Therapeutics Inc	Uremia	Italy	Launched	02-May-2013
Hyperion Therapeutics Inc	Uremia	Spain	Launched	02-May-2013
Hyperion Therapeutics Inc	Uremia	UK	Launched	02-May-2013
Hyperion Therapeutics Inc	Uremia	US	Launched	02-May-2013
Swedish Orphan Biovitrum AB	Uremia	Denmark	Launched	
Swedish Orphan Biovitrum AB	Uremia	France	Launched	01-May-2006
Swedish Orphan Biovitrum AB	Uremia	Portugal	Launched	21-Mar-2007
Swedish Orphan Biovitrum AB	Uremia	Spain	Launched	
Swedish Orphan Biovitrum AB	Uremia	Sweden	Launched	01-Jun-2006
Ucyclyd Pharma Inc	Uremia	Australia	Launched	08-Nov-2000
Ucyclyd Pharma Inc	Uremia	Canada	Launched	08-Nov-2000
Ucyclyd Pharma Inc	Uremia	France	Launched	08-Nov-2000
Ucyclyd Pharma Inc	Uremia	Germany	Launched	08-Nov-2000
Ucyclyd Pharma Inc	Uremia	Italy	Launched	08-Nov-2000
Ucyclyd Pharma Inc	Uremia	Spain	Launched	08-Nov-2000
Ucyclyd Pharma Inc	Uremia	UK	Launched	08-Nov-2000
Ucyclyd Pharma Inc	Uremia	US	Launched	08-Nov-2000
Orphan Pacific, Inc	Uremia	Japan	Registered	09-Nov-2012
Swedish Orphan Biovitrum AB	Uremia	Ireland	Registered	08-Dec-1999
CMIC Co Ltd	Uremia	Japan	Discontinued	09-Nov-2012

sodium phenylbutyrate, Hyperion / Swedish Orphan CHEMICAL STRUCTURES

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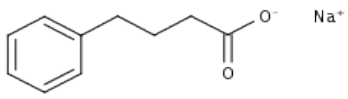
CAS Registry Number:	Confidence Level:
1716-12-7	1



Name	Type
Lunaphen	Trade Name
Buphenyl	Trade Name
triButyrate	Trade Name
sodium phenylbutyrate	
sodium 4-phenylbutyrate	

sodium phenylbutyrate, Hyperion / Swedish Orphan DRUG NAMES

Names	Type
Ammonaps	Trade Name
Buphenyl	Trade Name
sodium 4-phenylbutyrate	
sodium phenylbutyrate, Medicis	
sodium 4-phenylbutyrate, Ucyclyd	
CMK-304	Research Code
sodium phenylbutyrate	
sodium phenylbutyrate, Ucyclyd / Swedish Orphan	
sodium phenylbutyrate, Hyperion / Swedish Orphan	

sodium phenylbutyrate, Hyperion / Swedish Orphan CLINICAL TRIALS

Trials by Phase and Condition Studied

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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
Cholestasis											
0	0	0	0	0	0	0	0	0	1	0	1
Maple syrup urine disease											
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	1	0	2

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

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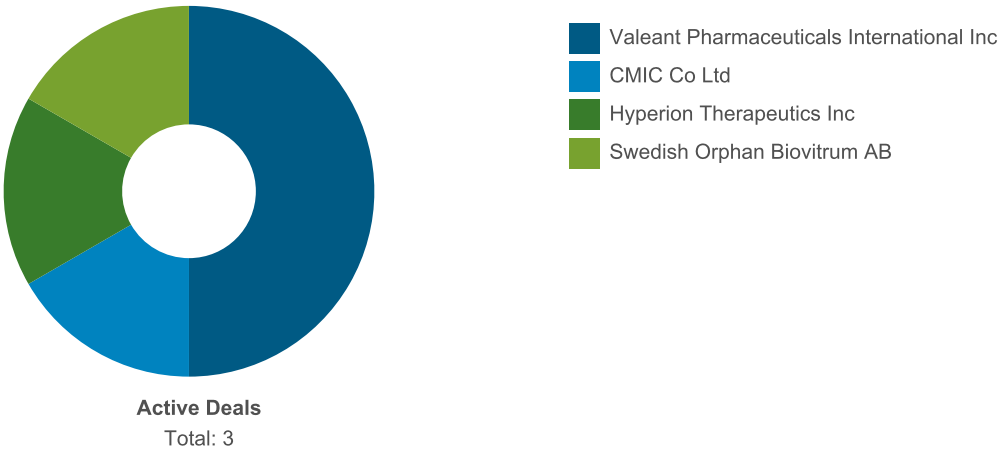
DEALS

Deals by Parent Company Chart



Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Valeant Pharmaceuticals International Inc	3	0	0	0	3
Hyperion Therapeutics Inc	0	0	1	0	1
CMIC Co Ltd	0	0	1	0	1
Swedish Orphan Biovitrum AB	0	0	1	0	1

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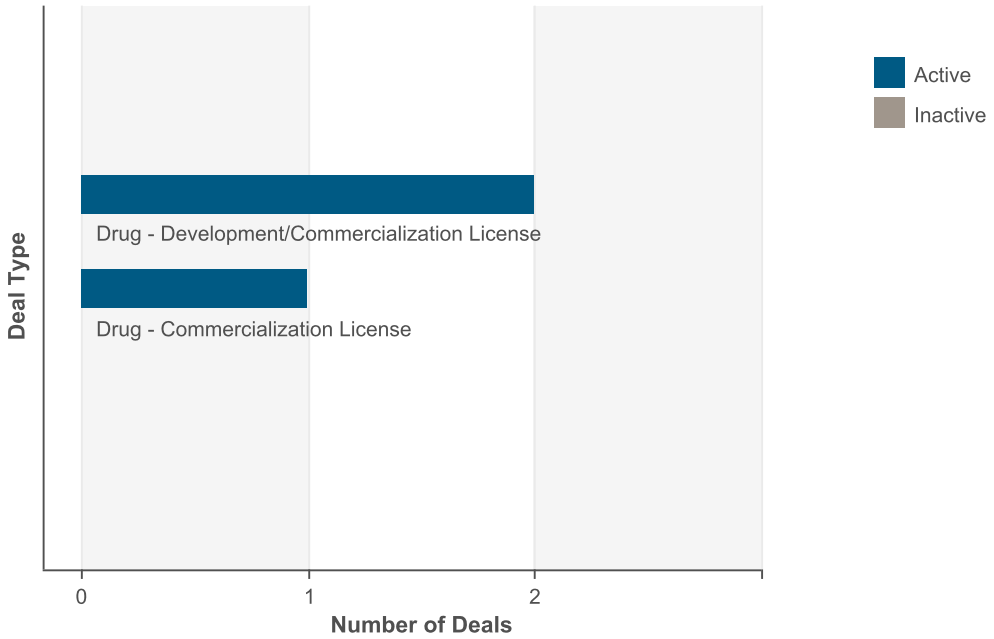
Deals by Type Chart



Deals by Type Table

Deal Type	Active	Inactive	Total
Drug - Development/Commercialization License	2	0	2
Drug - Commercialization License	1	0	1

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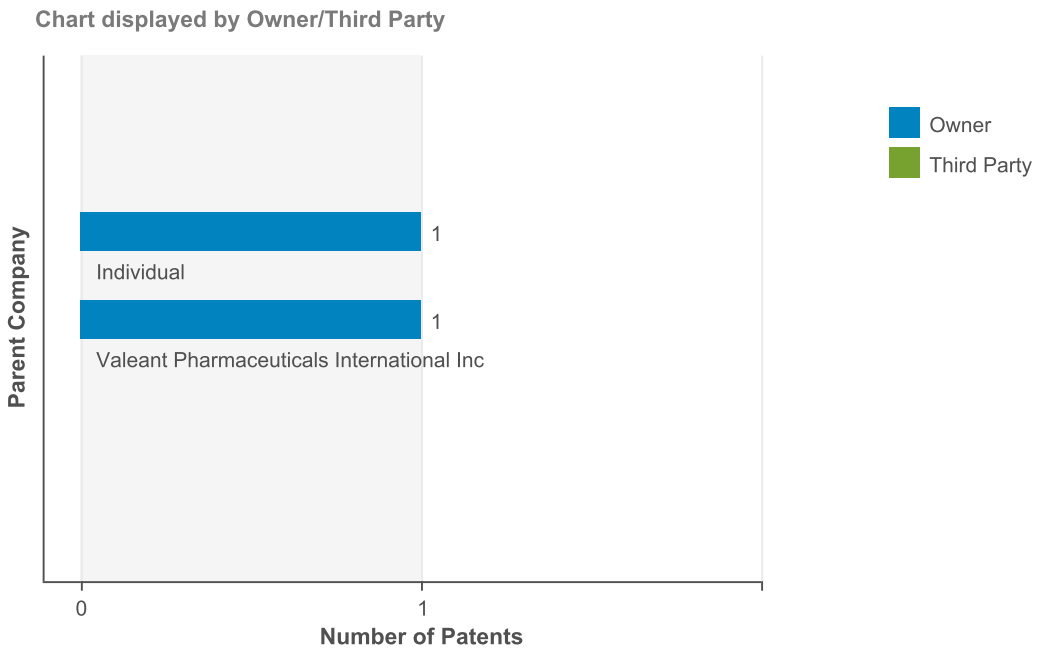
PATENTS

Patents by Parent Company Chart



Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
Valeant Pharmaceuticals International Inc	1	0	1
Individual	1	0	1

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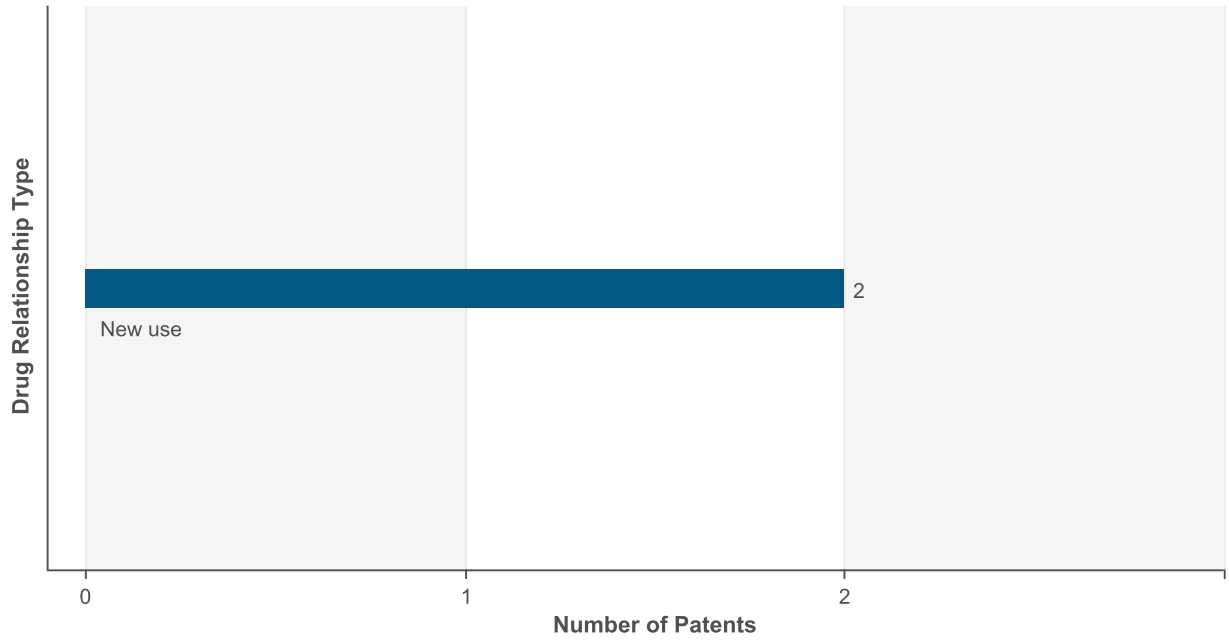
Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
New use	2

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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	Small molecule therapeutic;Intravenous formulation;Drug combination
Last Change Date	03-May-2013

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

SUMMARY

Ucyclyd Pharma (now part of Medicis (a subsidiary of Valeant)) 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

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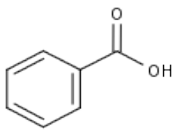
CAS Registry Number:	Confidence Level:
	2

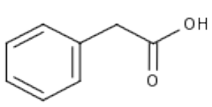
Name	Type
Ammonul	Trade Name
sodium phenylacetate + sodium benzoate	

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	

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

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

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

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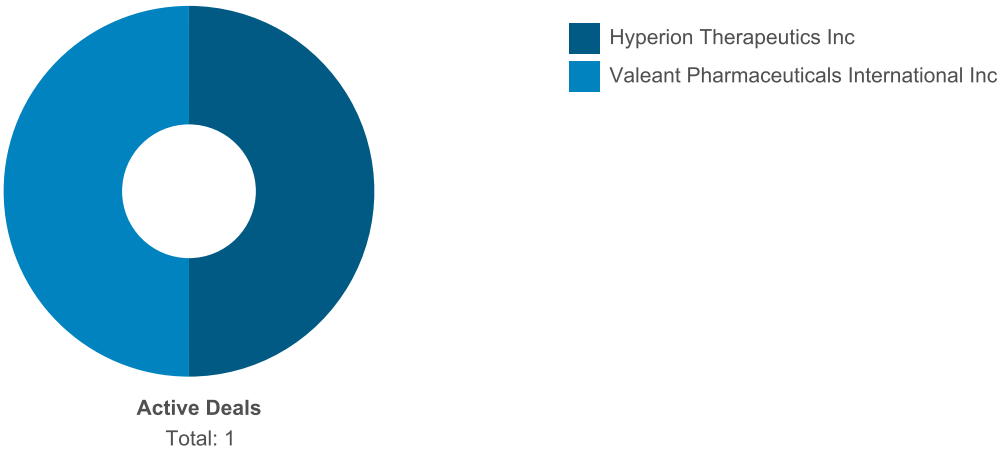
DEALS

Deals by Parent Company Chart



Deals by Parent Company Table

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

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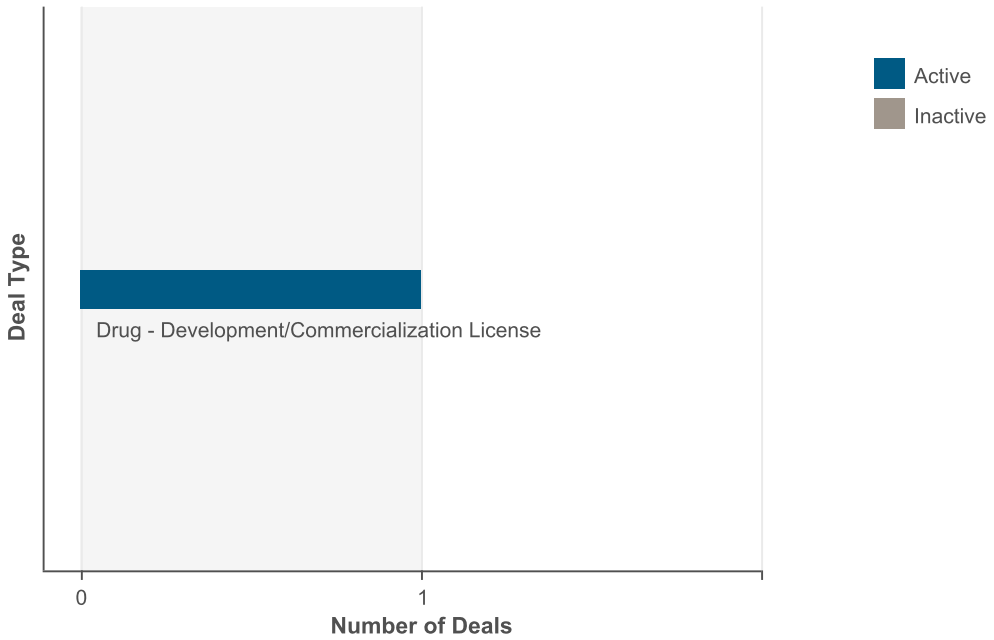
Deals by Type Chart



Deals by Type Table

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

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glycerol phenylbutyrate

glycerol phenylbutyrate SNAPSHOT

Drug Name	glycerol phenylbutyrate
Key Synonyms	Ravicti;glycerol phenylbutyrate
Originator Company	Ucyclyd Pharma Inc
Active Companies	Hyperion Therapeutics Inc
Inactive Companies	Ucyclyd Pharma Inc
Highest Status	Launched
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	03-May-2013

glycerol phenylbutyrate DEVELOPMENT PROFILE

SUMMARY

Hyperion Therapeutics, under license from Ucyclyd Pharma, has developed and launched 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 treatment of urea cycle disorders (UCD),. The drug is also being developed for the potential treatment of hepatic encephalopathy (HE). In February 2013, the drug was launched for UCD. 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	Launched	28-Feb-2013
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

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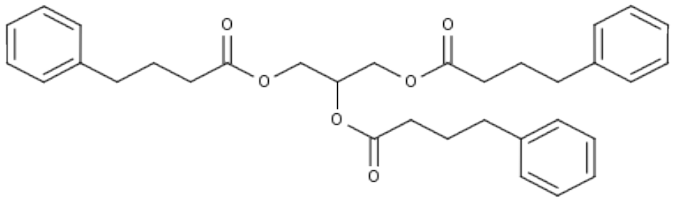
glycerol phenylbutyrate CHEMICAL STRUCTURES

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



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

glycerol phenylbutyrate DRUG NAMES

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

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

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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

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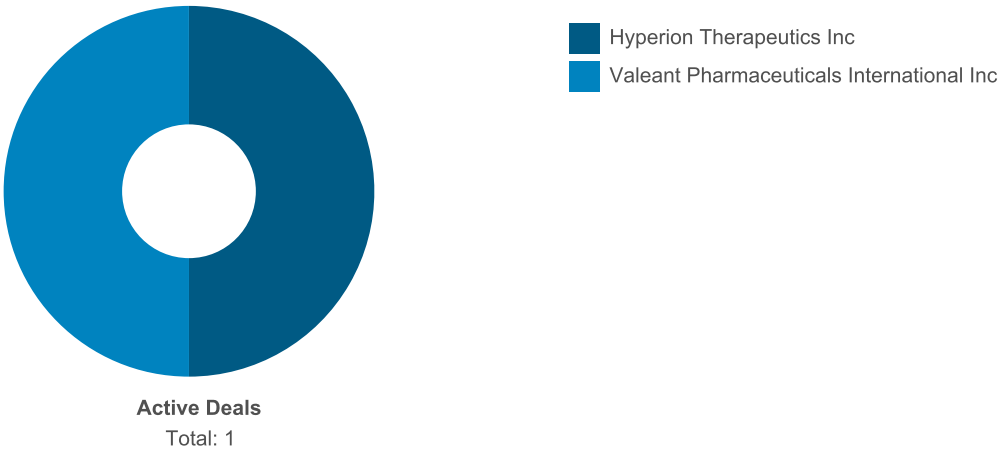
glycerol phenylbutyrate DEALS AND PATENTS

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

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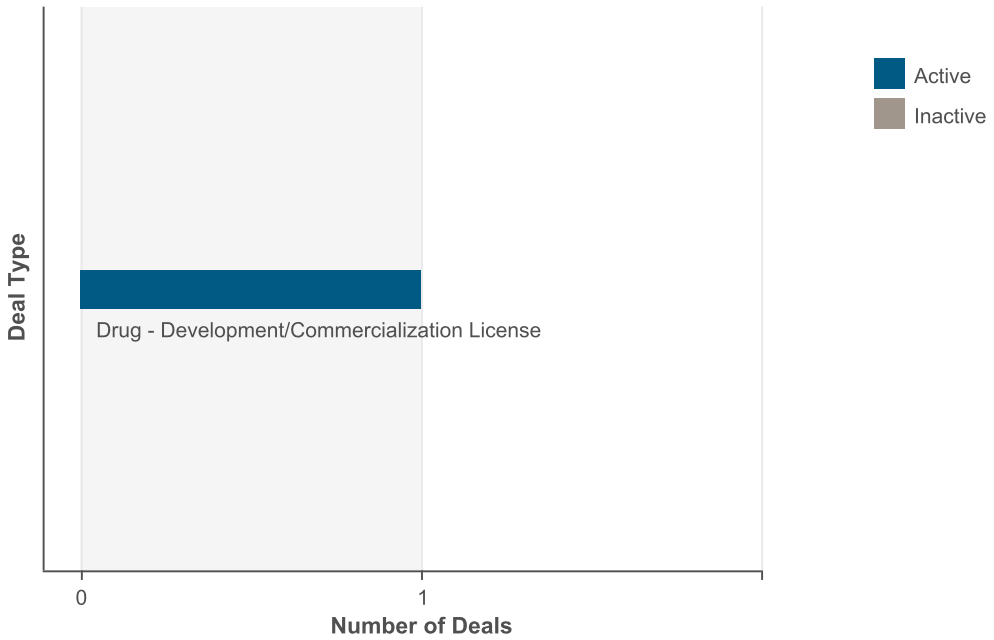
Deals by Type Chart



Deals by Type Table

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

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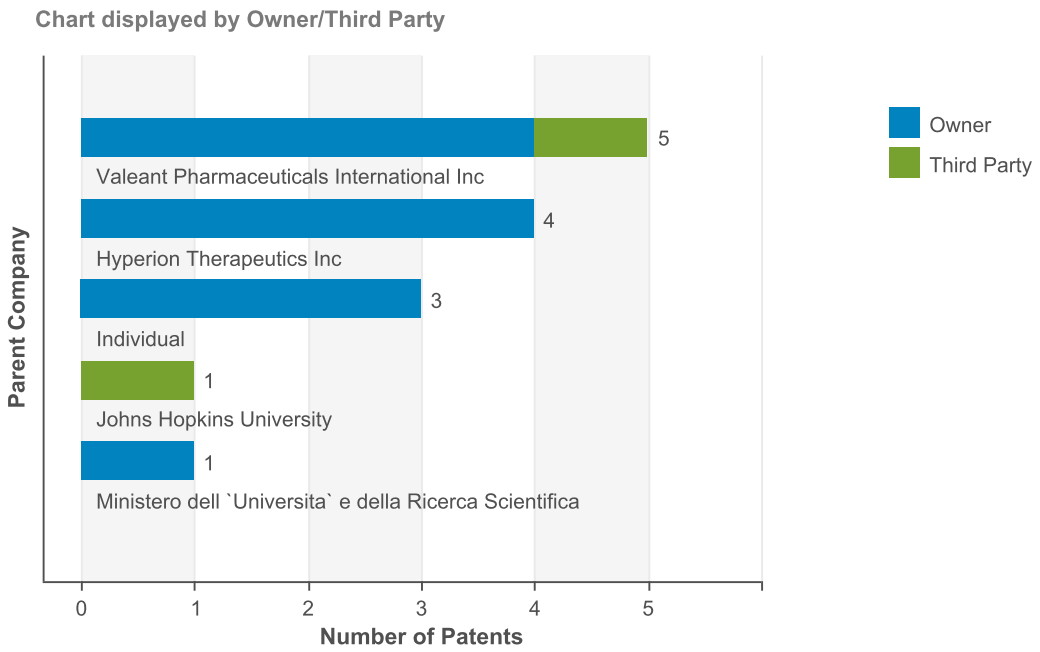
PATENTS

Patents by Parent Company Chart



Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
Valeant Pharmaceuticals International Inc	4	1	5
Hyperion Therapeutics Inc	4	0	4
Individual	3	0	3
Ministero dell `Universita` e della Ricerca Scientifica	1	0	1
Johns Hopkins University	0	1	1

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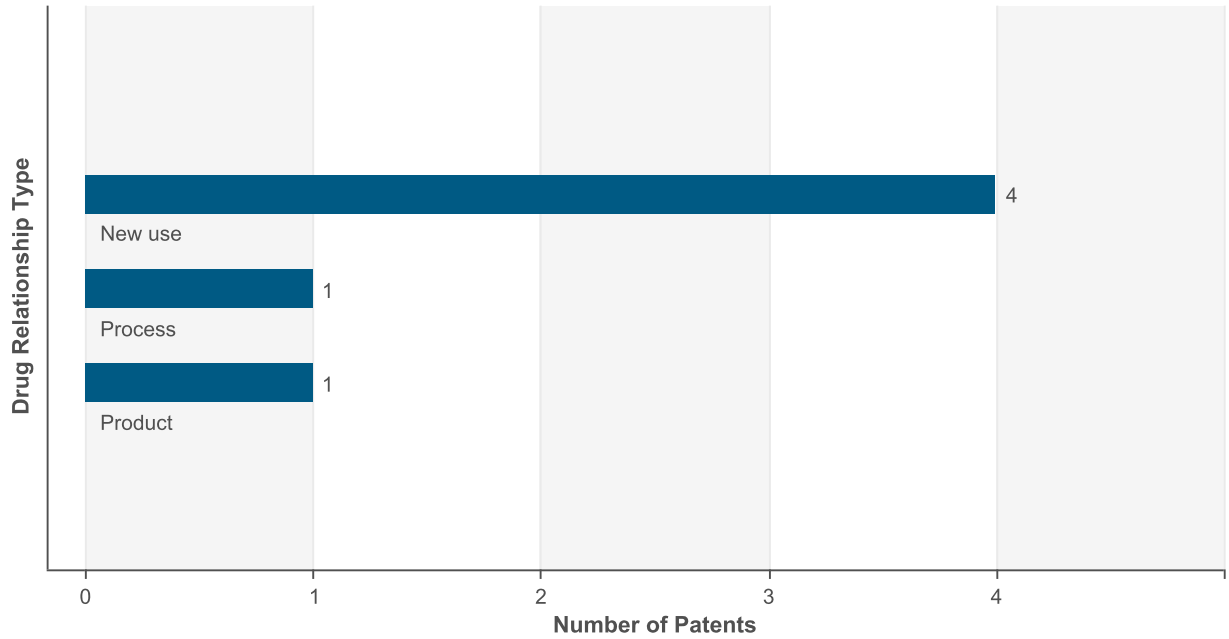
Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
New use	4
Process	1
Product	1

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