

## Hyperion Therapeutics Inc

### CORTELLIS COMPANY DETAILED PIPELINE REPORT

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

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#### THOMSON REUTERS

3 Times Square  
New York, New York 10036  
United States

Tel: +1 646 223 4000

[thomsonreuters.com](http://thomsonreuters.com)

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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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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	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 issuance 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 raised \$60 million from a series C financing.

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

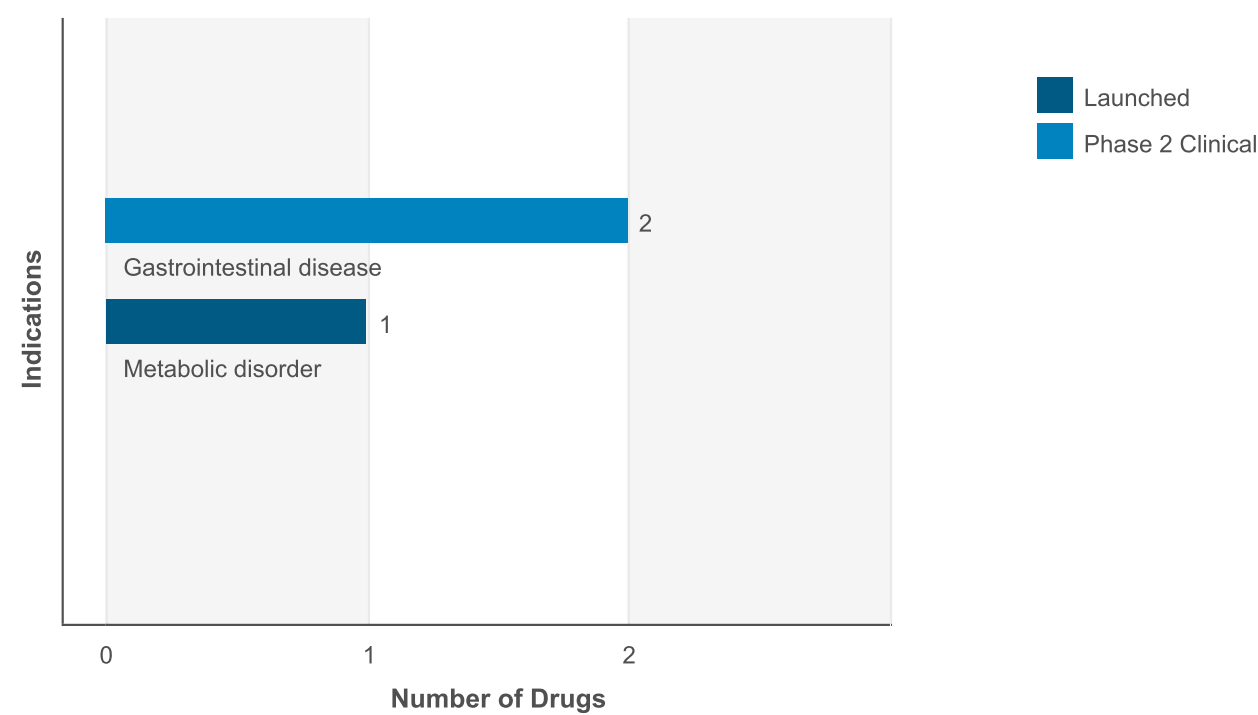
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# PRODUCT PORTFOLIO SUMMARY

## DRUGS

### Drugs by Indication

Active Drugs by Indication Chart



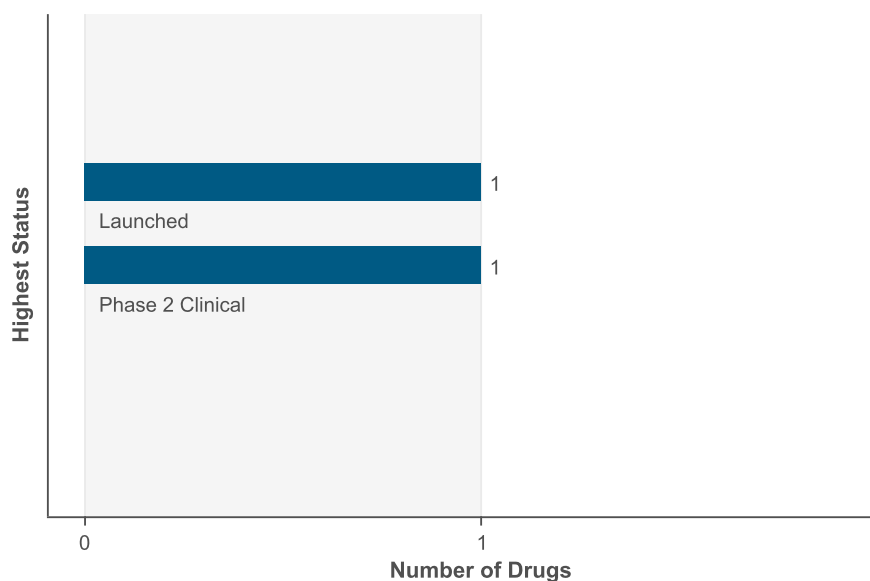
Drugs by Indication Table

Indication	Active	Inactive	Total
Gastrointestinal disease	2	0	2
Metabolic disorder	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	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
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	2	0	2
Genitourinary disease	2	0	2
Hematological disease	1	0	1
Degeneration	1	0	1
Neoplasm	1	0	1
Metabolic disorder	2	0	2
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 phenylacetate + sodium benzoate (hyperammonemia), Ucyglyd

#### sodium phenylacetate + sodium benzoate (hyperammonemia), Ucyglyd SNAPSHOT

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

#### sodium phenylacetate + sodium benzoate (hyperammonemia), Ucyglyd DEVELOPMENT PROFILE

##### SUMMARY

Ucyglyd 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), Ucyglyd DEVELOPMENT STATUS

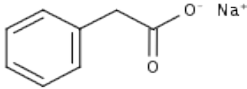
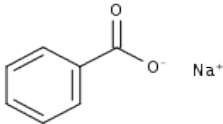
##### CURRENT DEVELOPMENT STATUS

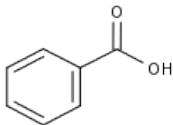
Company	Indication	Country	Development Status	Date
Ucyglyd Pharma Inc	Hyperammonemia	US	Launched	28-Aug-2007
Hyperion Therapeutics Inc	Hepatic encephalopathy	US	Phase 2 Clinical	31-Dec-2007

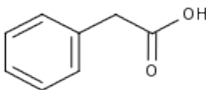
#### sodium phenylacetate + sodium benzoate (hyperammonemia), Ucyglyd CHEMICAL STRUCTURES

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CAS Registry Number:	Confidence Level:
	2
<div style="display: flex; justify-content: space-around; align-items: center;">   </div>	
Name	Type
Ammonul	Trade Name
sodium phenylacetate + sodium benzoate	

CAS Registry Number:	Confidence Level:
65-85-0	2
<div style="display: flex; justify-content: center; align-items: center;">  </div>	
Name	Type
benzoic acid	

CAS Registry Number:	Confidence Level:
103-82-2	2
<div style="display: flex; justify-content: center; align-items: center;">  </div>	
Name	Type
phenylacetic acid	

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## sodium phenylacetate + sodium benzoate (hyperammonemia), Ucyclid DRUG NAMES

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

## sodium phenylacetate + sodium benzoate (hyperammonemia), Ucyclid 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

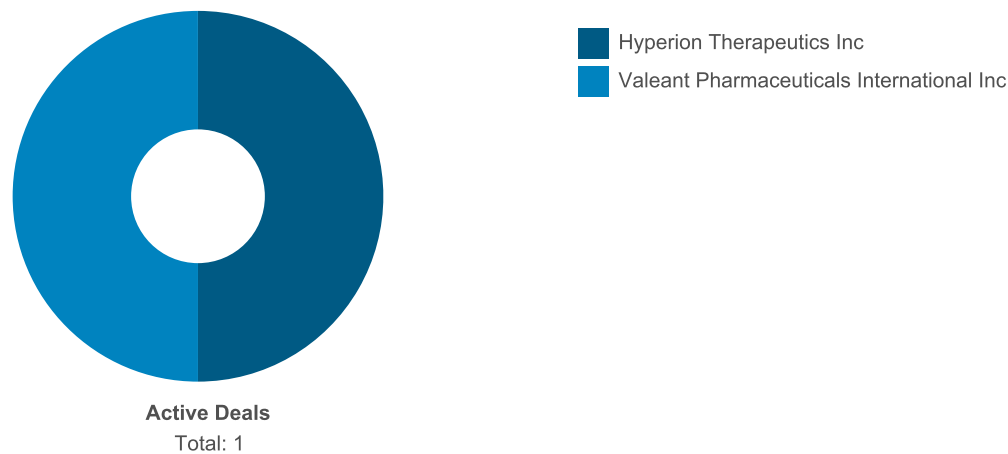
Includes Phase 1, Phase 1a, Phase 1, Phase 1/2 (where enrolment count is under 100 or not specified), Phase 0

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DEALS

Deals by Parent Company Chart

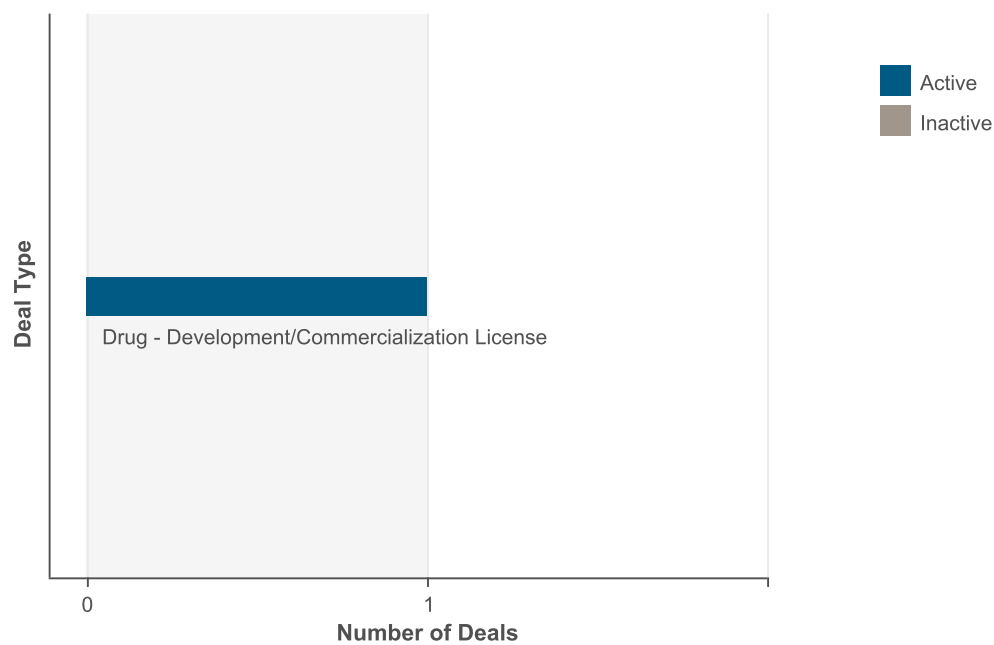


Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
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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## glycerol phenylbutyrate

### glycerol phenylbutyrate SNAPSHOT

<b>Drug Name</b>	glycerol phenylbutyrate
<b>Key Synonyms</b>	glycerol phenylbutyrate;Ravicti
<b>Originator Company</b>	Ucyclyd Pharma Inc
<b>Active Companies</b>	Hyperion Therapeutics Inc
<b>Inactive Companies</b>	Ucyclyd Pharma Inc
<b>Highest Status</b>	Launched
<b>Active Indications</b>	Hyperammonemia;Hepatic encephalopathy
<b>Target-based Actions</b>	
<b>Other Actions</b>	Nitrogen metabolism modulator
<b>Technologies</b>	Prodrug;Oral formulation;Oral liquid formulation;Small molecule therapeutic
<b>Last Change Date</b>	01-Mar-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

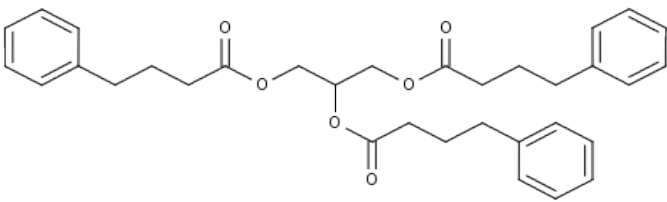
#### CURRENT DEVELOPMENT STATUS

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
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

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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

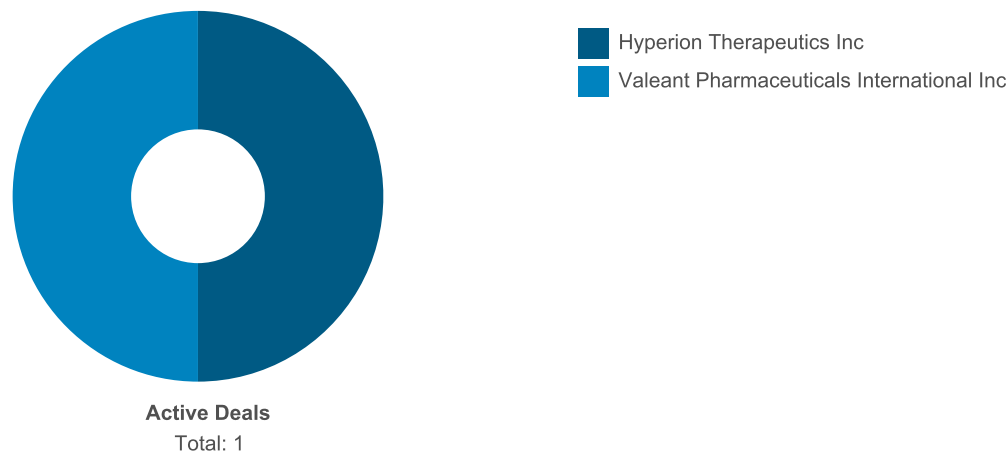
Includes Phase 1, Phase 1a, Phase 1, Phase 1/2 (where enrolment count is under 100 or not specified), Phase 0

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DEALS

Deals by Parent Company Chart

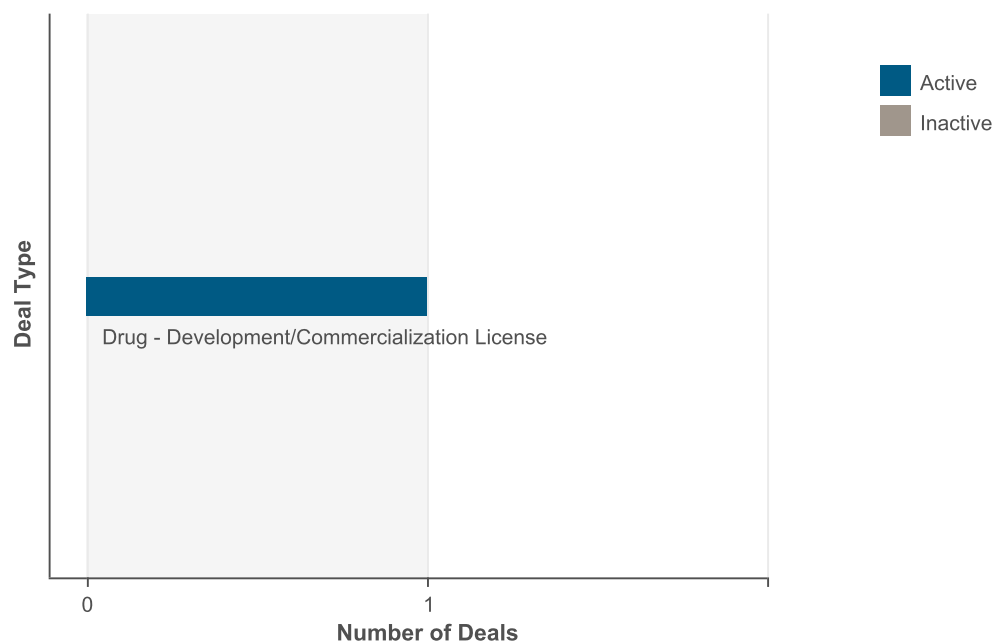


Deals by Parent Company Table

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

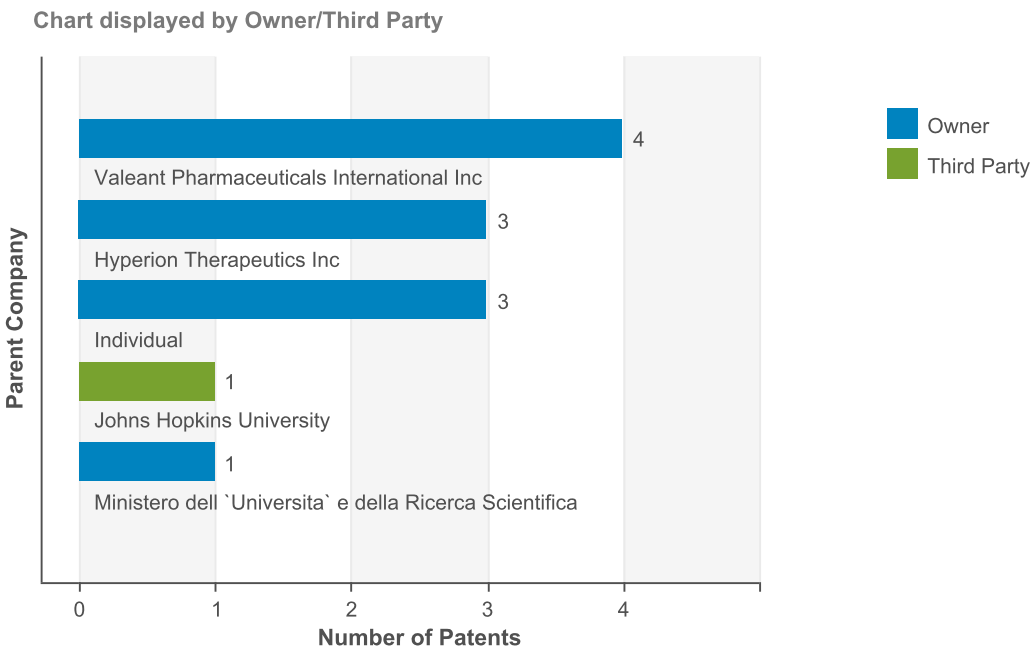


Deals by Type Table

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

PATENTS

Patents by Parent Company Chart

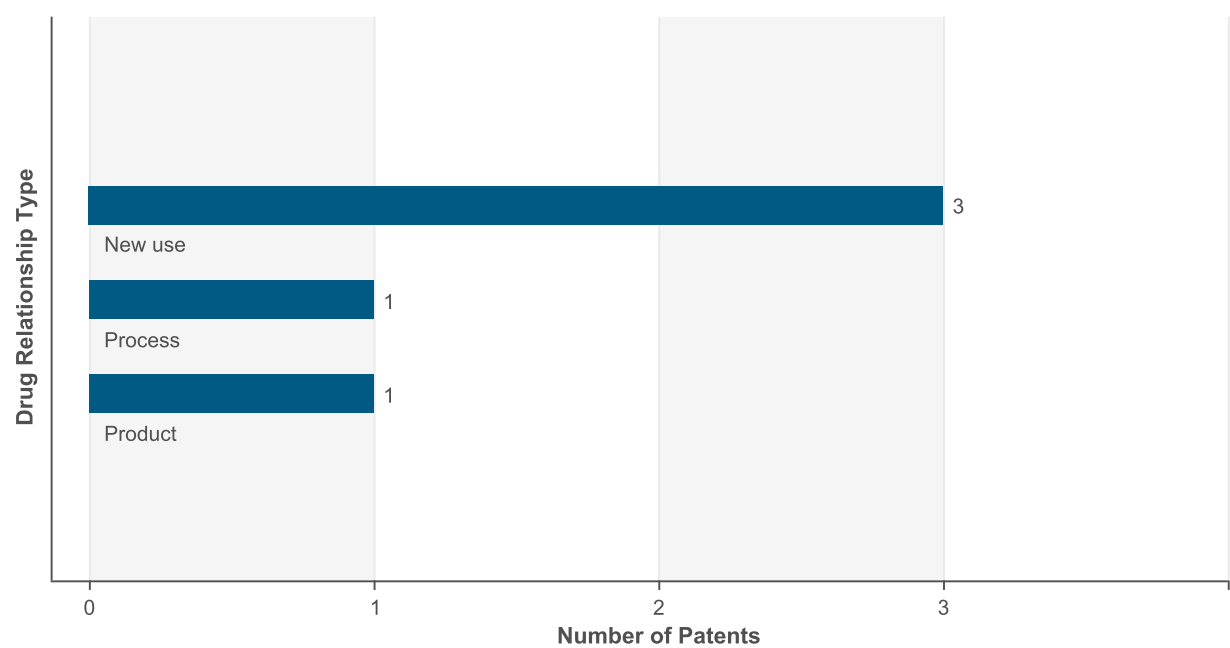


Patents by Parent Company Table

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

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Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
New use	3
Process	1
Product	1

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