

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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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

## COMPANY OVERVIEW

<b>Company Name</b>	Marinus Pharmaceuticals Inc
<b>Parent Company Name</b>	Marinus Pharmaceuticals Inc
<b>Website</b>	<a href="http://www.marinuspharma.com/">http://www.marinuspharma.com/</a>
<b>Country</b>	US
<b>Number of Drugs in Active Development</b>	3
<b>Number of Inactive Drugs</b>	1
<b>Number of Patents as Owner</b>	7
<b>Number of Patents as Third Party</b>	2
<b>Number of Deals</b>	7
<b>Key Indications</b>	Central nervous system disease,Epilepsy,Fragile X syndrome,Post traumatic stress disorder,Cancer,Allergic rhinitis,Attention deficit hyperactivity disorder,Bipolar disorder,Brain injury,Inflammatory disease,Nasal polyps,Schizophrenia,Seasonal allergic rhinitis
<b>Key Target-based Actions</b>	GABA A receptor agonist,AKT1 protein kinase modulator,Alpha 1 antichymotrypsin modulator,Angiotensinogen ligand modulator,Clusterin modulator,Contactin 1 modulator,Contactin 2 modulator,Dickkopf-3 ligand modulator,ErbB2 tyrosine kinase receptor modulator,Extracellular signal related kinase-1 modulator,Extracellular signal related kinase-4 modulator,MAP kinase modulator,Monocyte differentiation antigen CD14 modulator,Neuronal cell adhesion molecule modulator,Protein kinase C inhibitor,Serum amyloid P modulator,Topoisomerase I inhibitor,Topoisomerase II inhibitor,VEGF receptor modulator
<b>Key Technologies</b>	Small molecule therapeutic,Steroid,Oral formulation,Capsule formulation,Intravenous formulation,Nanoparticle formulation oral,Oral liquid formulation,Oral suspension formulation,Fluorescence,Formulation preservation,Nucleophilic substitutional synthesis,Stereochemical synthesis,Steroid synthesis

## COMPANY PROFILE

### SUMMARY

Marinus Pharmaceuticals focuses on the identification, development and commercialization of drugs to treat serious neurological, psychiatric and pain disorders.

### FINANCIAL

In July 2014, Marinus priced its IPO of 5,625,000 shares at \$8 per share. Underwriters were granted a 30-day overallotment option to buy an additional 843,750 shares. The IPO was expected to close on August 05, 2014. In September 2014, the underwriters exercised their option to purchase an additional 133,000 shares at a price of \$8 each, which bought the total shares issued by the company in the offering to 5,758,000. Net proceeds were approximately \$41.3 million.

In January 2013, Marinus raised \$21 million from a series C financing.

In April 2009, Marinus raised \$20 million from a series B financing round.

In October 2005, Marinus raised \$29.4 million in a series A financing.

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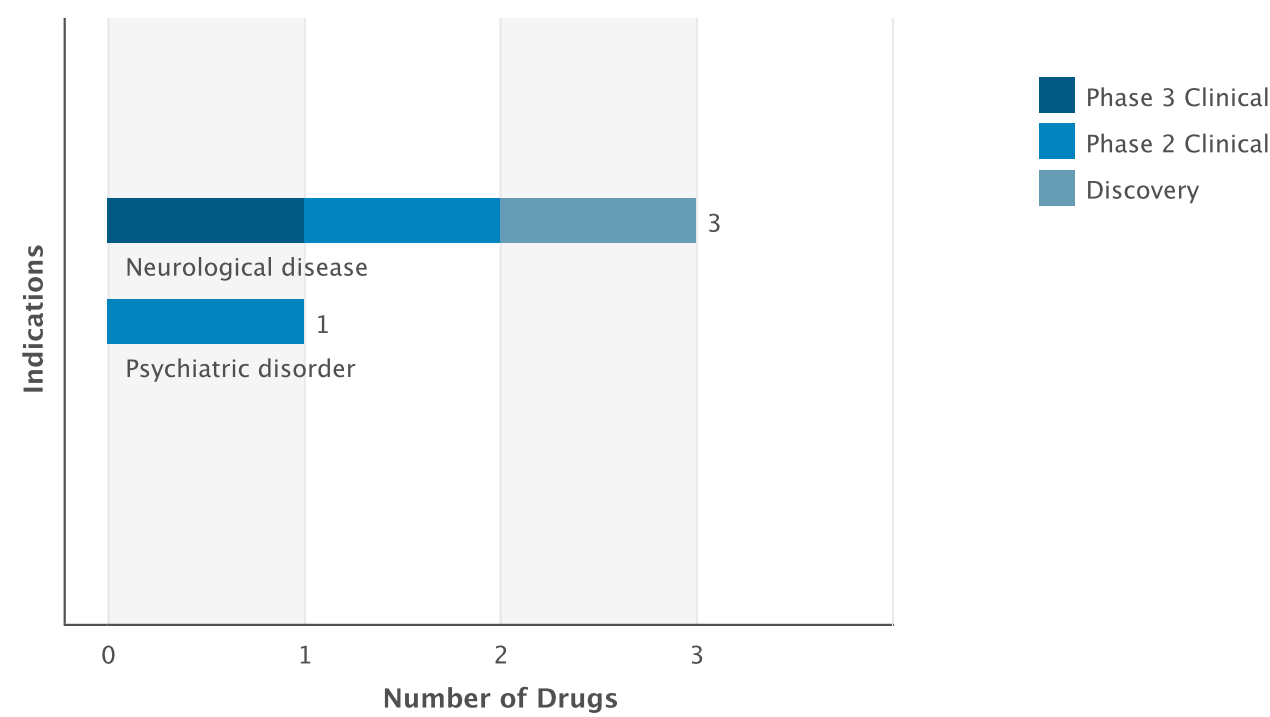


# PRODUCT PORTFOLIO SUMMARY

## DRUGS

### Drugs by Indication

Active Drugs by Indication Chart



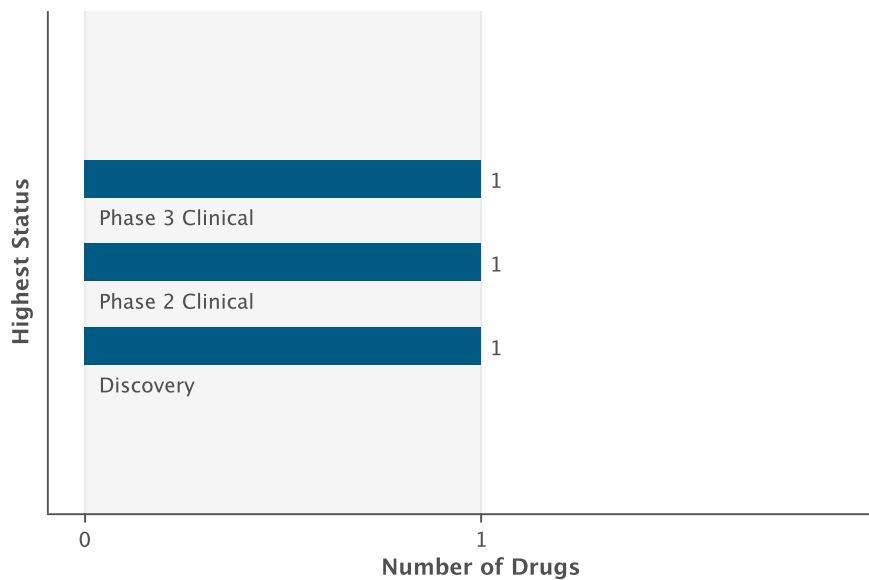
Drugs by Indication Table

Indication	Active	Inactive	Total
Neurological disease	3	0	3
Psychiatric disorder	1	1	2
Genetic disorder	0	1	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
Phase 3 Clinical	1
Phase 2 Clinical	1
Discovery	1
No Development Reported	1

## DEALS

Deal Type	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Drug - Funding	1	0	0	0	1
Drug - Early Research/Development	0	0	1	0	1
Drug - Development/Commercialization License	1	0	1	0	2
Drug - Development Services	0	0	2	0	2
Technology - Delivery/Formulation	0	0	1	0	1

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## CLINICAL TRIALS

### Trials by Condition Studied

Condition Studied	Ongoing	All
Neurological disease	1	6
Psychiatric disorder	1	2

### Trials by Phase

Phase	Ongoing	All
Phase 2	2	8

### 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
Immune disorder	1	0	1
Psychiatric disorder	2	0	2
Musculoskeletal disease	1	0	1
Neoplasm	2	1	3
Genetic disorder	1	0	1
Neurological disease	4	1	5
Respiratory disease	1	0	1
Infectious disease	2	0	2
Injury	0	1	1
Inflammatory disease	2	0	2
Otorhinolaryngological 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

### ganaxolone (oral capsule/nanoparticle, PTSD/CNS disorders), Marinus/NovaMedica

#### ganaxolone (oral capsule/nanoparticle, PTSD/CNS disorders), Marinus/NovaMedica SNAPSHOT

<b>Drug Name</b>	ganaxolone (oral capsule/nanoparticle, PTSD/CNS disorders), Marinus/NovaMedica
<b>Key Synonyms</b>	ganaxolone
<b>Originator Company</b>	Marinus Pharmaceuticals Inc
<b>Active Companies</b>	Marinus Pharmaceuticals Inc;NovaMedica
<b>Inactive Companies</b>	
<b>Highest Status</b>	Phase 3 Clinical
<b>Active Indications</b>	Post traumatic stress disorder;Epilepsy;Central nervous system disease
<b>Target-based Actions</b>	GABA A receptor agonist
<b>Other Actions</b>	CNS modulator;Anticonvulsant agent
<b>Technologies</b>	Nanoparticle formulation oral;Oral formulation;Capsule formulation;Small molecule therapeutic;Steroid
<b>Last Change Date</b>	14-Nov-2014

### ganaxolone (oral capsule/nanoparticle, PTSD/CNS disorders), Marinus/NovaMedica DEVELOPMENT PROFILE

#### SUMMARY

Marinus Pharmaceuticals is developing a solid nanoparticle capsule formulation of ganaxolone, for the potential oral treatment of post-traumatic stress disorder (PTSD) and epilepsy,. Presumably, the company is also investigating the drug for the potential treatment of other central nervous system disorders, including fragile X syndrome (FXS). In November 2013, a phase IIb/III study for epilepsy was initiated and at that time, the study was expected to complete in July 2016, ; in November 2014, data was expected in the second half of 2015. In April 2011, a phase II study was initiated in PTSD patients in the US; in April 2012, the trial was ongoing. In December 2012, it was reported that NovaMedica would commercialize ganaxolone in Russia and CIS countries. At that time, Marinus was planning to conduct phase II studies in Russia.

Marinus is also developing an oral liquid suspension formulation of ganaxolone.

### ganaxolone (oral capsule/nanoparticle, PTSD/CNS disorders), Marinus/NovaMedica DEVELOPMENT STATUS

#### CURRENT DEVELOPMENT STATUS

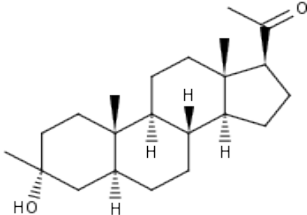
Company	Indication	Country	Development Status	Date
Marinus Pharmaceuticals Inc	Epilepsy	US	Phase 3 Clinical	06-Nov-2013
Marinus Pharmaceuticals Inc	Post traumatic stress disorder	US	Phase 2 Clinical	19-Apr-2011

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Company	Indication	Country	Development Status	Date
Marinus Pharmaceuticals Inc	Central nervous system disease	US	Discovery	07-Apr-2011
NovaMedica	Central nervous system disease	Russian Federation	Discovery	19-Dec-2012
NovaMedica	Epilepsy	Russian Federation	Discovery	19-Dec-2012
NovaMedica	Post traumatic stress disorder	Russian Federation	Discovery	19-Dec-2012

### ganaxolone (oral capsule/nanoparticle, PTSD/CNS disorders), Marinus/NovaMedica CHEMICAL STRUCTURES

CAS Registry Number: 38398-32-2	Confidence Level: 1
	
Name ganaxolone	Type INN
CCD-1042	Research Code

### ganaxolone (oral capsule/nanoparticle, PTSD/CNS disorders), Marinus/NovaMedica DRUG NAMES

Names	Type
ganaxolone (oral capsule/nanoparticle, PTSD/CNS disorders), Marinus/NovaMedica	
ganaxolone	INN
ganaxolone (oral solid nanoparticle/capsule, PTSD/epilepsy/CNS disorders), Marinus	

### ganaxolone (oral capsule/nanoparticle, PTSD/CNS disorders), Marinus/NovaMedica 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

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Epilepsy											
0	0	0	0	1	1	0	0	0	0	1	1
Post traumatic stress disorder											
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	1	2	0	0	0	0	1	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

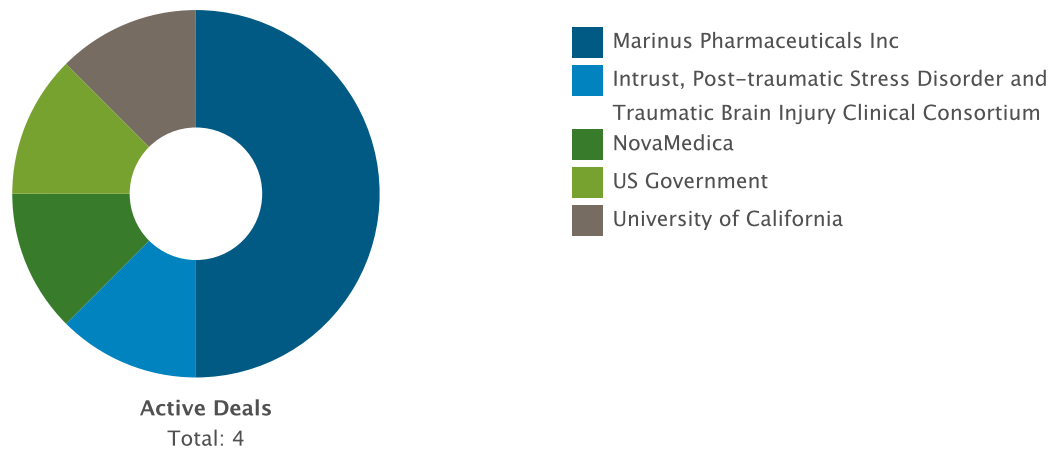
Includes Phase 1, Phase 1a, Phase 1, Phase 1/2 (where enrolment count is under 100 or not specified), Phase 0

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ganaxolone (oral capsule/nanoparticle, PTSD/CNS disorders), Marinus/NovaMedica DEALS AND PATENTS

DEALS

Deals by Parent Company Chart

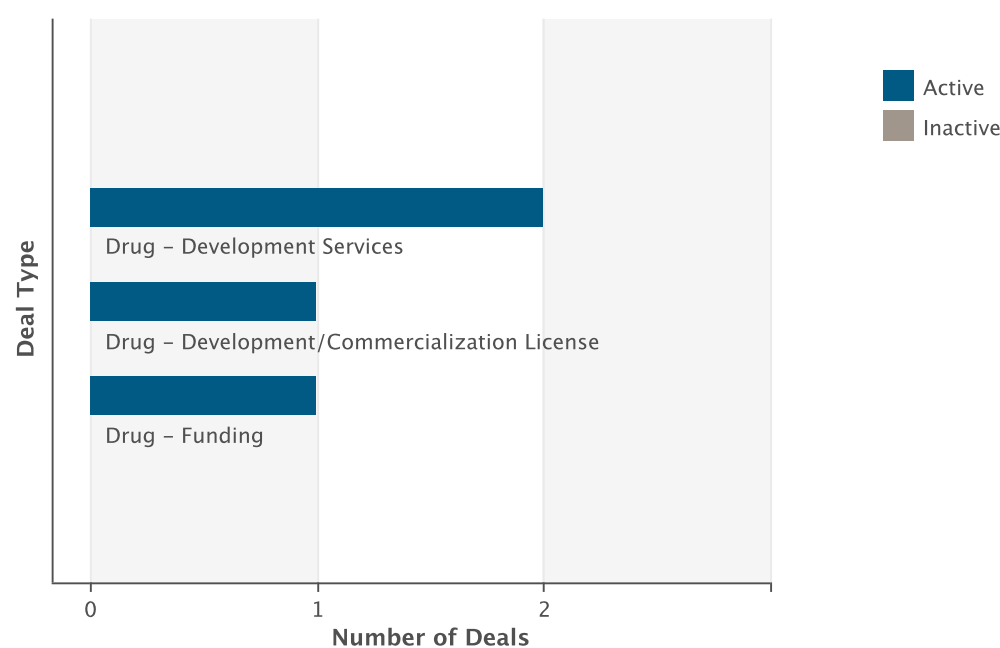


Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Marinus Pharmaceuticals Inc	2	0	2	0	4
NovaMedica	0	0	1	0	1
Intrust, Post-traumatic Stress Disorder and Traumatic Brain Injury Clinical Consortium	1	0	0	0	1
US Government	0	0	1	0	1
University of California	1	0	0	0	1

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Deals by Type Chart

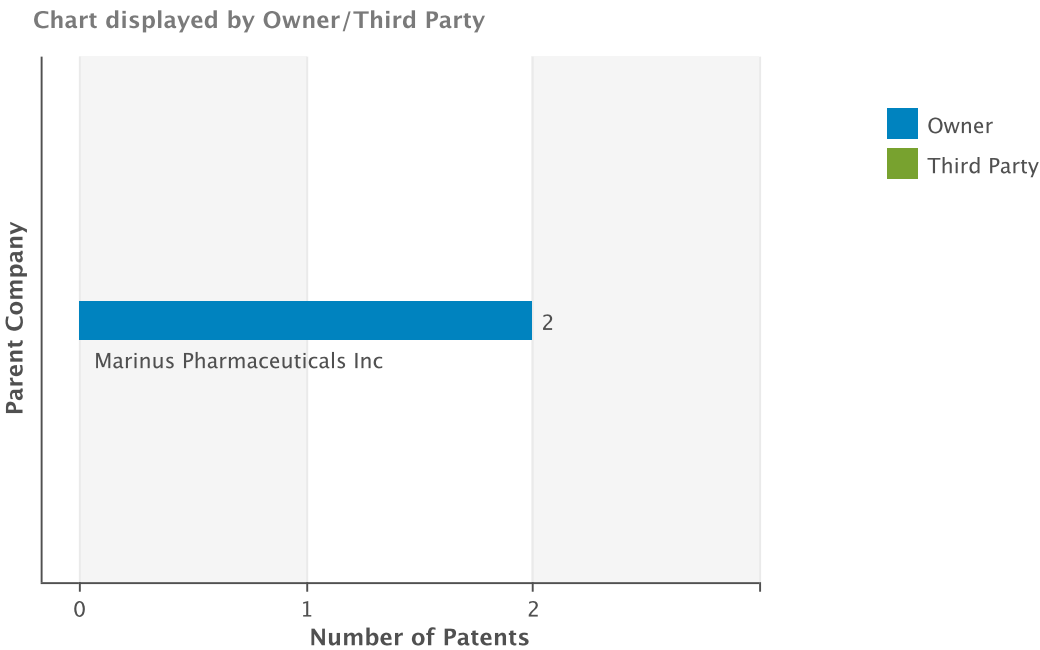


Deals by Type Table

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

PATENTS

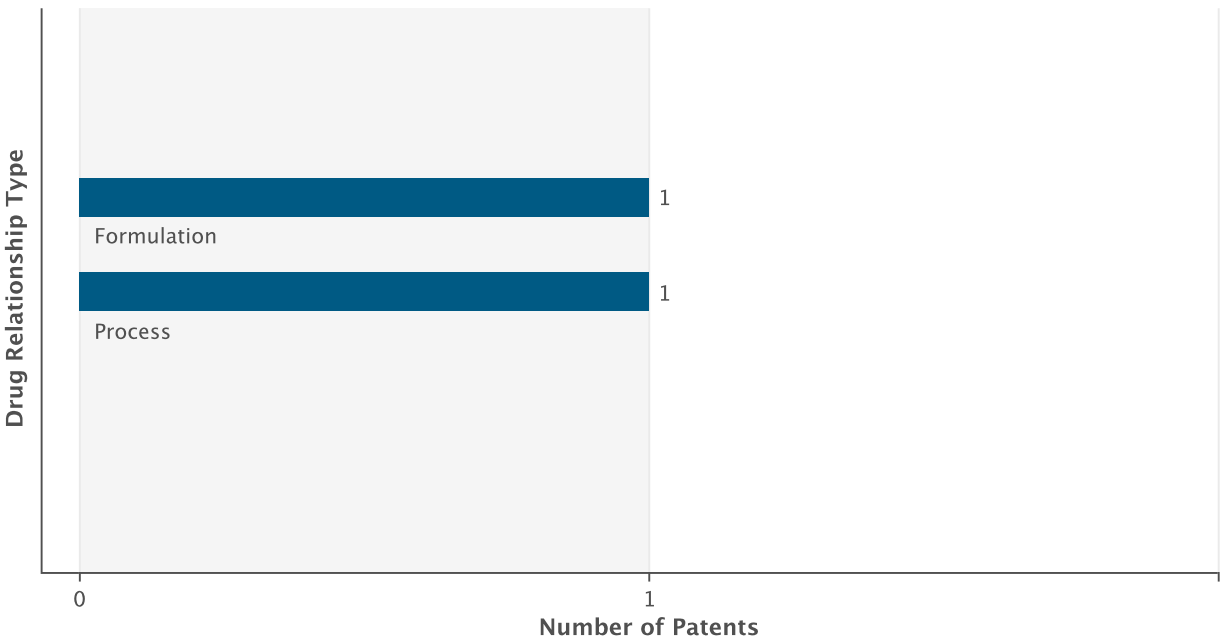
Patents by Parent Company Chart



Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
Marinus Pharmaceuticals Inc	2	0	2

Patents by Drug Relationship Type Chart



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Patents by Drug Relationship Type Table

Drug Relationship	Total
Process	1
Formulation	1

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## ganaxolone (oral liquid suspension, epilepsy/fragile X syndrome), Marinus/NovaMedica

### ganaxolone (oral liquid suspension, epilepsy/fragile X syndrome), Marinus/NovaMedica SNAPSHOT

<b>Drug Name</b>	ganaxolone (oral liquid suspension, epilepsy/fragile X syndrome), Marinus/NovaMedica
<b>Key Synonyms</b>	ganaxolone
<b>Originator Company</b>	Marinus Pharmaceuticals Inc
<b>Active Companies</b>	Marinus Pharmaceuticals Inc;NovaMedica
<b>Inactive Companies</b>	
<b>Highest Status</b>	Phase 2 Clinical
<b>Active Indications</b>	Fragile X syndrome;Epilepsy;Central nervous system disease
<b>Target-based Actions</b>	GABA A receptor agonist
<b>Other Actions</b>	CNS modulator;Anticonvulsant agent
<b>Technologies</b>	Oral liquid formulation;Oral suspension formulation;Oral formulation;Small molecule therapeutic;Steroid
<b>Last Change Date</b>	14-Nov-2014

### ganaxolone (oral liquid suspension, epilepsy/fragile X syndrome), Marinus/NovaMedica DEVELOPMENT PROFILE

#### SUMMARY

Marinus is developing an oral liquid suspension formulation of ganaxolone, a positive allosteric modulator of the GABA A receptor, for the potential treatment of epilepsy and fragile X syndrome (FXS). In January 2007, a phase IIb trial for pediatric spasms was initiated. A second phase II trial for adult partial seizures began in February 2007; positive data were reported in March 2009; at that time, a dose-ranging phase IIb trial was planned. In November 2012, a phase II trial in children with FXS was initiated ; in November 2014, top-line data were expected in mid 2015. In March 2009, Marinus intended to explore the use of the drug in other CNS indications such as post-traumatic stress disorder (PTSD) and postpartum depression. In September 2014, a phase II proof-of-concept trial in PCDH19 gene mutation female pediatric epilepsy patients was expected to begin later that year ; In November 2014, top-line data from the initial tranche of patients was expected in the first half of 2015. In December 2012, it was reported that NovaMedica would commercialize the drug in Russia and CIS countries. At that time, Marinus was planning to conduct phase II studies in Russia.

Marinus is also developing an oral capsule formulation of ganaxolone for the potential treatment of PTSD.

Ganaxolone was previously being developed by Purdue, but development was discontinued by July 2002.

### ganaxolone (oral liquid suspension, epilepsy/fragile X syndrome), Marinus/NovaMedica DEVELOPMENT STATUS

#### CURRENT DEVELOPMENT STATUS

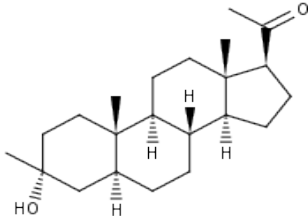
Company	Indication	Country	Development Status	Date
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Company	Indication	Country	Development Status	Date
Marinus Pharmaceuticals Inc	Epilepsy	Europe	Phase 2 Clinical	28-Feb-2007
Marinus Pharmaceuticals Inc	Epilepsy	US	Phase 2 Clinical	31-Jan-2007
Marinus Pharmaceuticals Inc	Fragile X syndrome	US	Phase 2 Clinical	30-Nov-2012
Marinus Pharmaceuticals Inc	Central nervous system disease	US	Discovery	03-Mar-2009
NovaMedica	Epilepsy	Russian Federation	Discovery	19-Dec-2012
NovaMedica	Fragile X syndrome	Russian Federation	Discovery	19-Dec-2012

### ganaxolone (oral liquid suspension, epilepsy/fragile X syndrome), Marinus/NovaMedica CHEMICAL STRUCTURES

CAS Registry Number:	Confidence Level:
38398-32-2	1
	
Name	Type
ganaxolone	INN
CCD-1042	Research Code

### ganaxolone (oral liquid suspension, epilepsy/fragile X syndrome), Marinus/NovaMedica DRUG NAMES

Names	Type
ganaxolone (oral liquid suspension, epilepsy/fragile X syndrome), Marinus/NovaMedica	
ganaxolone (oral liquid suspension, epilepsy/fragile X syndrome), Marinus	
ganaxolone	INN
ganaxolone (liquid-suspension, epilepsy), Marinus	

### ganaxolone (oral liquid suspension, epilepsy/fragile X syndrome), Marinus/NovaMedica 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
Seizure disorder											
0	0	0	0	0	3	0	0	0	0	0	3
Epilepsy											
0	0	0	0	0	3	0	0	0	0	0	3
Attention deficit hyperactivity disorder											
0	0	0	0	1	1	0	0	0	0	1	1
Anxiety disorder											
0	0	0	0	1	1	0	0	0	0	1	1
Infantile spasms											
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	1	6	0	0	0	0	1	6

### 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)

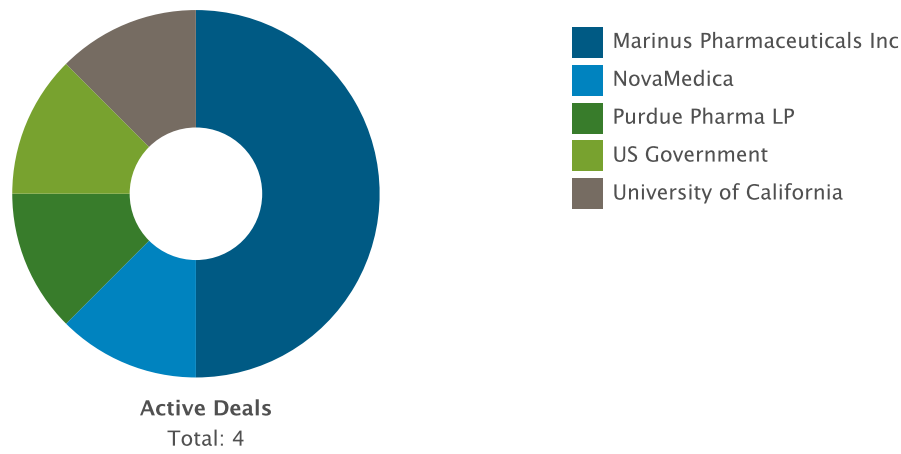
#### Phase 1 Clinical

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

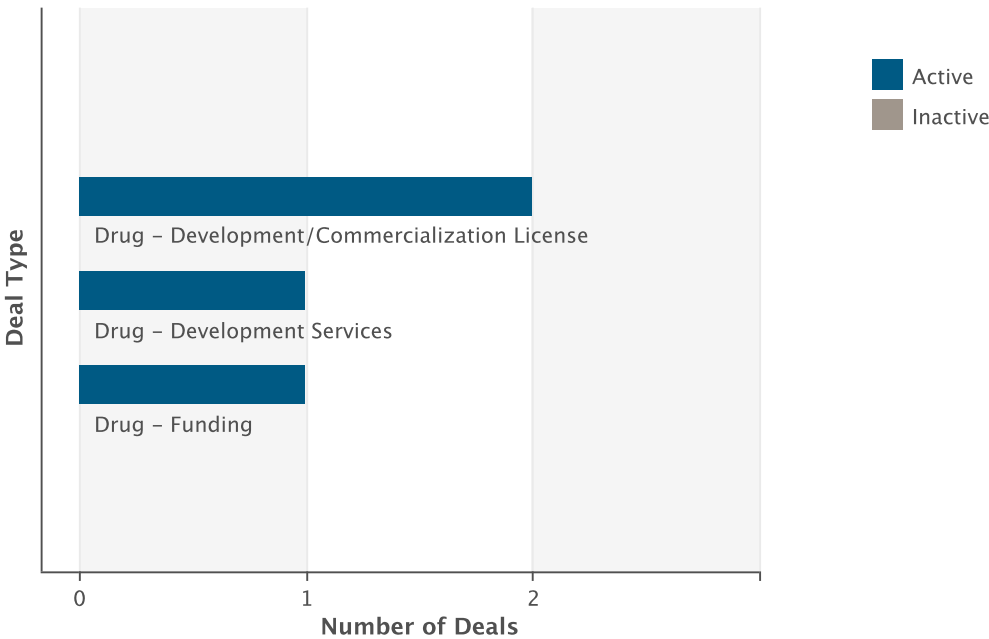


Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Marinus Pharmaceuticals Inc	2	0	2	0	4
NovaMedica	0	0	1	0	1
University of California	1	0	0	0	1
Purdue Pharma LP	1	0	0	0	1
US Government	0	0	1	0	1

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Deals by Type Chart



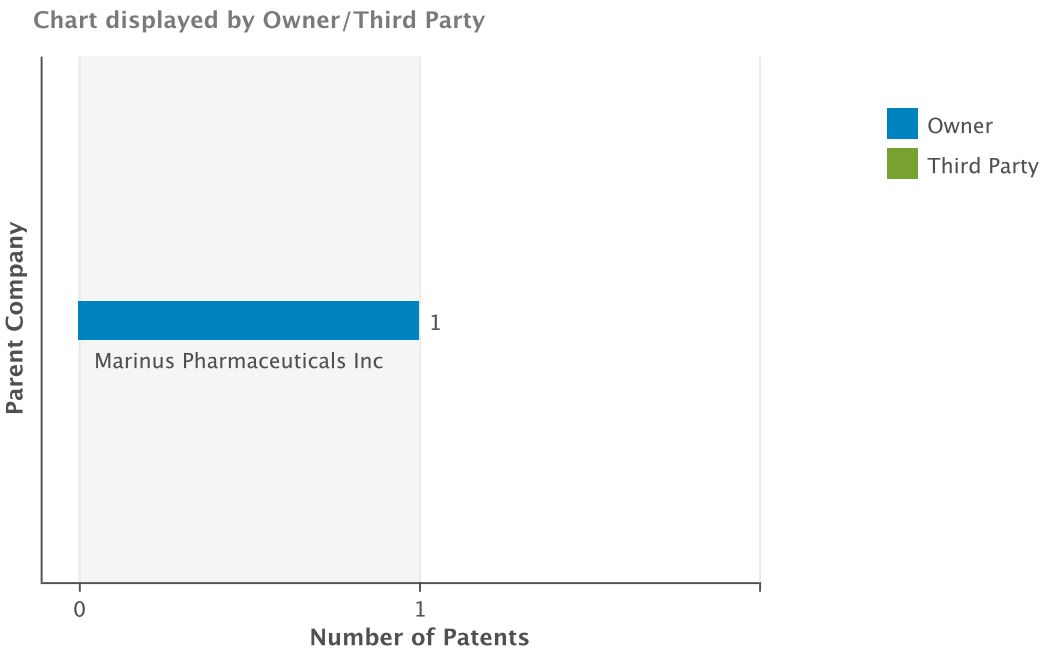
Deals by Type Table

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

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PATENTS

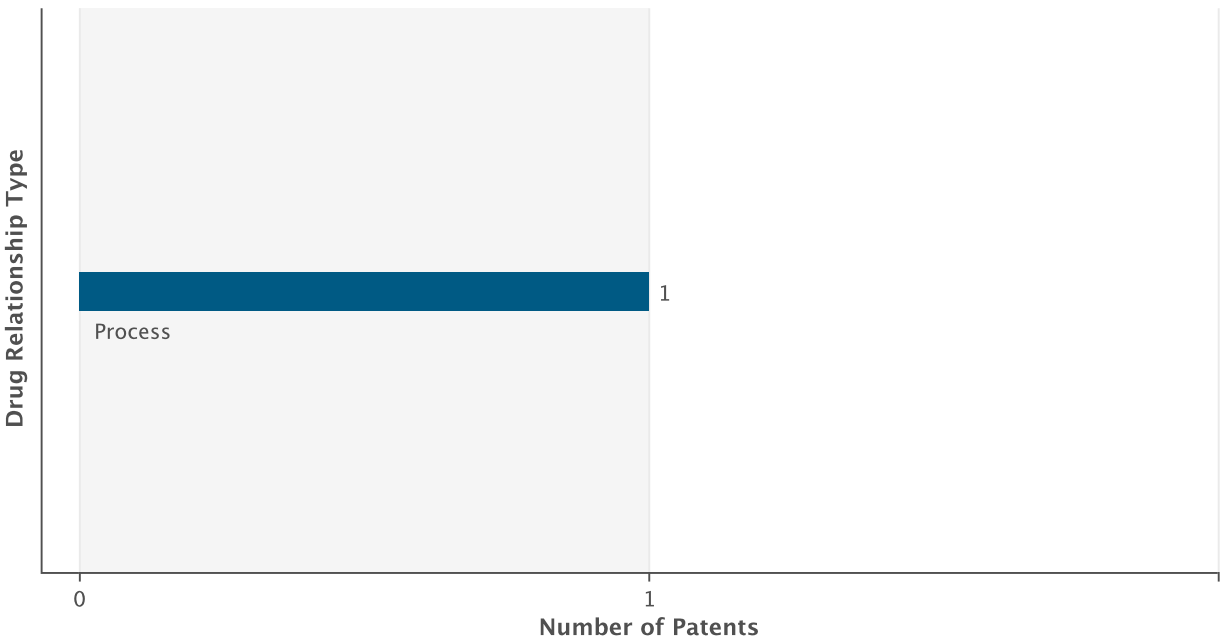
Patents by Parent Company Chart



Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
Marinus Pharmaceuticals Inc	1	0	1

Patents by Drug Relationship Type Chart



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Patents by Drug Relationship Type Table

Drug Relationship	Total
Process	1

## ganaxolone (intravenous/Captisol, central nervous system diseases), Marinus

### ganaxolone (intravenous/Captisol, central nervous system diseases), Marinus SNAPSHOT

Drug Name	ganaxolone (intravenous/Captisol, central nervous system diseases), Marinus
Key Synonyms	ganaxolone
Originator Company	Marinus Pharmaceuticals Inc
Active Companies	Marinus Pharmaceuticals Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Central nervous system disease
Target-based Actions	GABA A receptor agonist
Other Actions	CNS modulator
Technologies	Small molecule therapeutic;Intravenous formulation;Steroid
Last Change Date	14-Nov-2014

### ganaxolone (intravenous/Captisol, central nervous system diseases), Marinus DEVELOPMENT PROFILE

#### SUMMARY

Marinus Pharmaceuticals is investigating an iv formulation of ganaxolone, using CyDex's Captisol technology, presumably for the potential treatment of central nervous system (CNS) disorders. In November 2014, preclinical studies were ongoing.

Marinus is also developing oral liquid and oral capsule formulations for CNS disorders.

### ganaxolone (intravenous/Captisol, central nervous system diseases), Marinus DEVELOPMENT STATUS

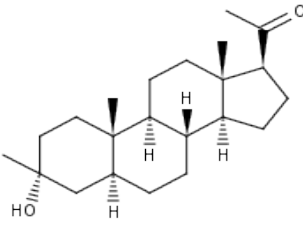
#### CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Marinus Pharmaceuticals Inc	Central nervous system disease	US	Discovery	12-Aug-2014

### ganaxolone (intravenous/Captisol, central nervous system diseases), Marinus CHEMICAL STRUCTURES

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CAS Registry Number:	Confidence Level:
38398-32-2	1
	
Name	Type
ganaxolone	INN
CCD-1042	Research Code

**ganaxolone (intravenous/Captisol, central nervous system diseases), Marinus DRUG NAMES**

Names	Type
ganaxolone	INN
ganaxolone (intravenous/Captisol, central nervous system diseases), Marinus	

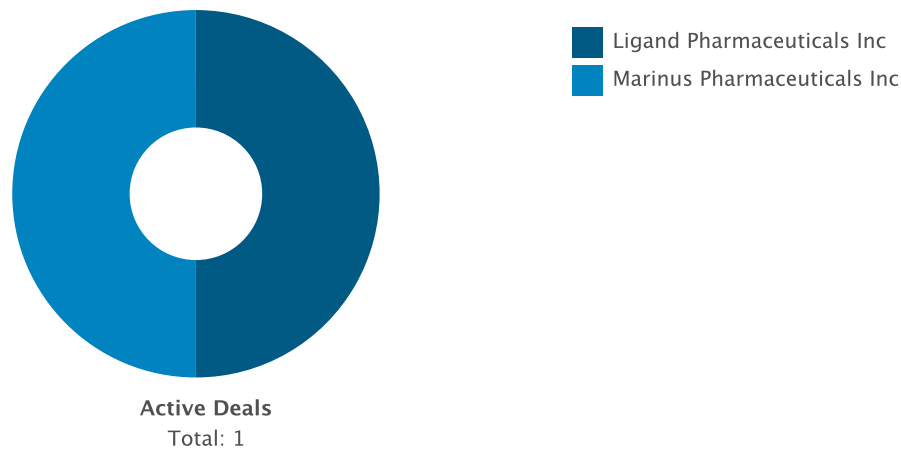
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ganaxolone (intravenous/Captisol, central nervous system diseases), Marinus DEALS AND PATENTS

DEALS

Deals by Parent Company Chart



Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Marinus Pharmaceuticals Inc	0	0	1	0	1
Ligand Pharmaceuticals Inc	1	0	0	0	1

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Deals by Type Chart



Deals by Type Table

Deal Type	Active	Inactive	Total
Technology - Delivery/Formulation	1	0	1

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