

## CoNCERT 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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## ABOUT CORTELLIS COMPANY DETAILED PIPELINE REPORT

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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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

## COMPANY OVERVIEW

Company Name	CoNCERT Pharmaceuticals Inc
Parent Company Name	CoNCERT Pharmaceuticals Inc
Website	http://www.concertpharma.com/
Country	US
Number of Drugs in Active Development	7
Number of Inactive Drugs	17
Number of Patents as Owner	162
Number of Patents as Third Party	1
Number of Deals	8
Key Indications	Neuropathic pain,Epilepsy,Inflammatory disease,Renal disease,Cancer,Pain,Multiple myeloma,Cystic fibrosis,Brain
Key Target-based Actions	NMDA receptor antagonist,GABA B receptor agonist,GABA A receptor modulator,CFTR stimulator,Calcium channel inhibitor T-type,Opioid receptor sigma agonist 1,HIV protease inhibitor,PDE inhibitor,Sigma opioid receptor agonist,Cytochrome P450 2D6 inhibitor,HIV-1 protease inhibitor
Key Technologies	Small molecule therapeutic,Oral formulation,Drug combination,Formulation preservation,Antibiotic,Capsule formulation,Controlled release formulation,Suspension,Crystalline form,Prodrug

## COMPANY PROFILE

### SUMMARY

CoNCERT Pharmaceuticals Inc is a Boston-based pharmaceutical company which uses chemical methodology, involving an innovative precision deuterium chemistry platform to modify specific properties of validated drug molecules, to create bioavailable drug candidates.

### EARLY R&D

By May 2009, Concert Pharmaceuticals was investigating deuterium analogs of rimonabant and mosapride.

By January 2008, the company was researching an HER2/EGFR inhibitor, an antiviral, a cytokine inhibitor and a number of antibiotics, for deuteration. By June 2008, CoNCERT was also researching a protease inhibitor for the potential treatment of HIV, an antifibrotic agent, an NMDA antagonist/ sigma agonist for the potential treatment of neuropathic pain, an antibacterial for the potential treatment of MRSA, an antipsychotic for the potential treatment for schizophrenia, a PDE-5 inhibitor for the potential treatment pulmonary arterial hypertension and benign prostate hyperplasia, a calcium modulator for the potential treatment of hyperparathyroidism, an EGFR inhibitor for the potential treatment of tumors and a CCR5 antagonist for the potential treatment of HIV.

### FINANCIAL

In December 2014, CoNCERT was selected for addition to the NASDAQ Biotechnology Index, effective from December 22, 2014.

In February 2014, CoNCERT priced a planned IPO of its common stock at \$14 per share. Underwriters would be granted a 30-day option to purchase up to an additional 900,000 shares at the same price to cover over-allotments. The shares would trade on the NASDAQ Global Market under the ticker symbol 'CNCE'. At that time, the offering was expected to close on February 19, 2014. Later that month, the offering was closed; in March 2014, underwriters exercised their over-allotment option and purchased an additional 649,690 shares which increased a capital raise of approximately \$93 million; at that time, net proceeds of \$83.1 million was raised.

In April 2008, CoNCERT Pharmaceuticals raised \$37 million in a series C financing round. The company planned to use the funds to advance its deuterium chemistry platform and product pipeline.

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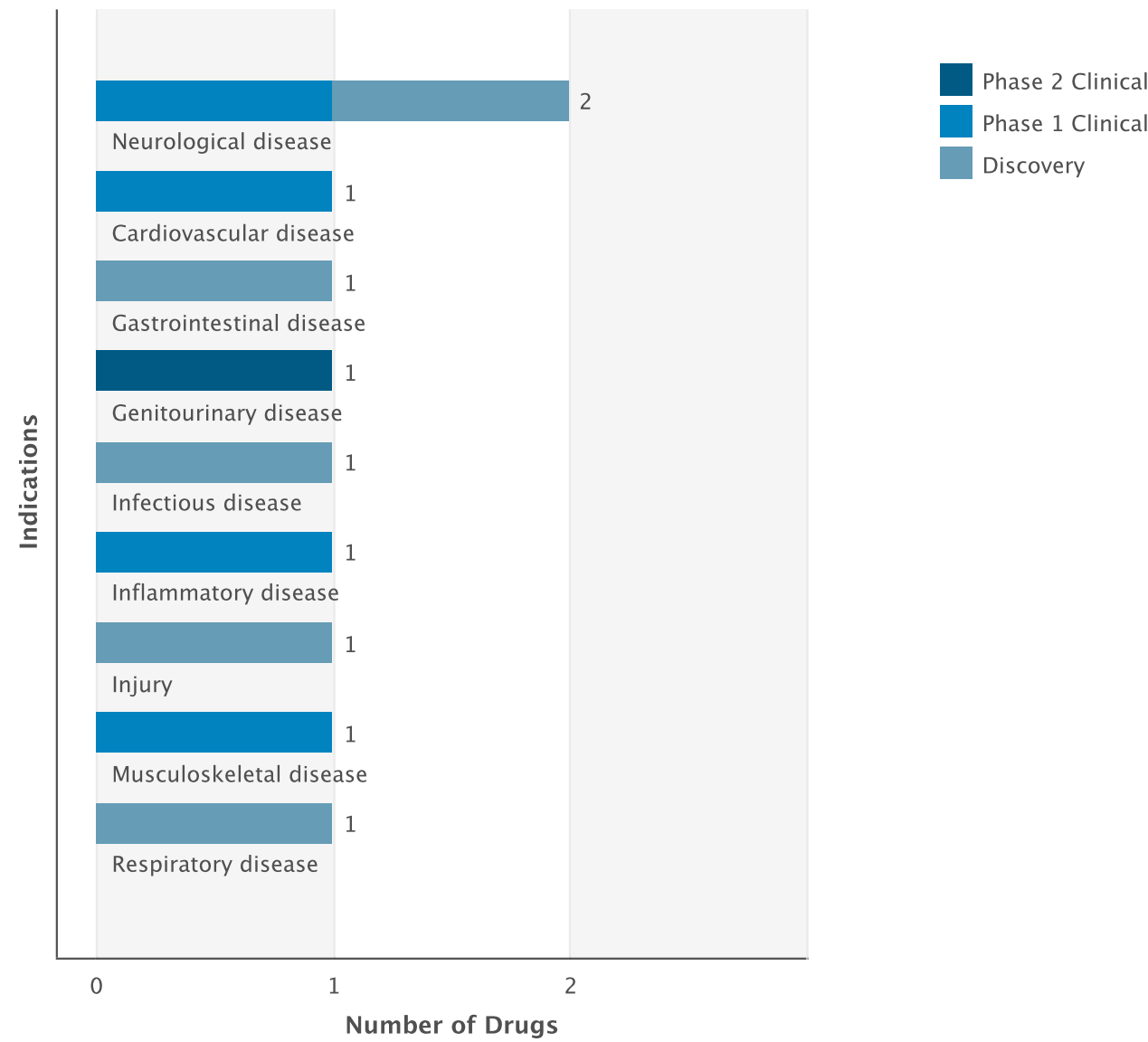
In November 2006, CoNCERT raised \$48.5 million in series B financing.  
In July 2006, CoNCERT raised \$10 million from a series A venture capital financing.

## PRODUCT PORTFOLIO SUMMARY

### DRUGS

#### Drugs by Indication

Active Drugs by Indication Chart



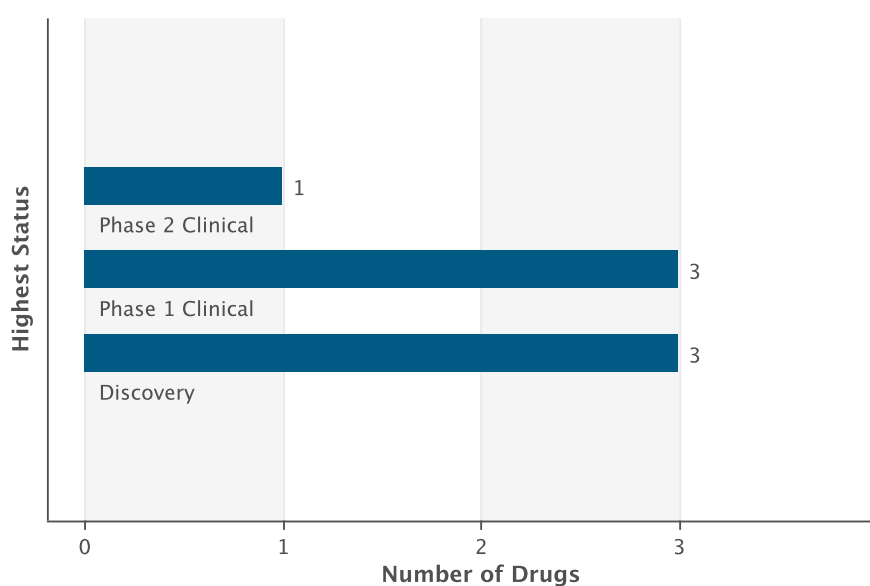
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## Drugs by Indication Table

Indication	Active	Inactive	Total
Neurological disease	2	5	7
Infectious disease	1	5	6
Cardiovascular disease	1	4	5
Inflammatory disease	1	3	4
Neoplasm	0	4	4
Musculoskeletal disease	1	2	3
Psychiatric disorder	0	3	3
Genitourinary disease	1	1	2
Immune disorder	0	2	2
Respiratory disease	1	1	2
Gastrointestinal disease	1	1	2
Injury	1	0	1
Hematological disease	0	1	1

## Drugs by Highest Status

### Active Drugs by Highest Status Chart



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## Drugs by Highest Status Table

Development Status	Number of Drugs
Phase 2 Clinical	1
Phase 1 Clinical	3
Discovery	3
Discontinued	2
No Development Reported	15

## DEALS

Deal Type	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Drug - Funding	1	0	0	0	1
Drug - CRADA	1	0	0	0	1
Drug - Screening/Evaluation	1	0	1	0	2
Drug - Development/Commercialization License	4	0	0	0	4

## CLINICAL TRIALS

### Trials by Condition Studied

Condition Studied	Ongoing	All
Genitourinary disease	0	3
Infectious disease	0	1
Cardiovascular disease	0	1
Psychiatric disorder	0	1

### Trials by Phase

Phase	Ongoing	All
Phase 3	0	1
Phase 2	0	1
Phase 1	0	11

## 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

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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
Cardiovascular disease	61	0	61
Endocrine disease	46	0	46
Gastrointestinal disease	72	0	72
Genitourinary disease	67	0	67
Growth disorder	11	0	11
Hematological disease	37	0	37
Degeneration	14	0	14
Andrology	23	0	23
Immune disorder	58	0	58
Psychiatric disorder	35	0	35
Musculoskeletal disease	31	0	31
Neoplasm	56	0	56
Ocular disease	18	0	18
Genetic disorder	20	0	20
Metabolic disorder	41	0	41
Mouth disease	1	0	1
Neurological disease	82	0	82
Nutritional disorder	16	0	16
Respiratory disease	36	0	36
Infectious disease	41	0	41
Injury	6	0	6
Toxicity and intoxication	12	0	12
Inflammatory disease	49	0	49

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Fatigue	3	0	3
Otorhinolaryngological disease	3	0	3
Gynecology and obstetrics	24	0	24
Dermatological disease	32	0	32
Ulcer	5	0	5

\* 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

### CTP-499

#### CTP-499 SNAPSHOT

<b>Drug Name</b>	CTP-499
<b>Key Synonyms</b>	
<b>Originator Company</b>	CoNCERT Pharmaceuticals Inc
<b>Active Companies</b>	GlaxoSmithKline plc;CoNCERT Pharmaceuticals Inc
<b>Inactive Companies</b>	
<b>Highest Status</b>	Phase 2 Clinical
<b>Active Indications</b>	Renal disease
<b>Target-based Actions</b>	PDE inhibitor
<b>Other Actions</b>	Fibrosuppressant;Anti-inflammatory;Antioxidant agent;Renal system agent
<b>Technologies</b>	Oral formulation;Controlled release formulation;Small molecule therapeutic
<b>Last Change Date</b>	10-Dec-2014

#### CTP-499 DEVELOPMENT PROFILE

##### SUMMARY

CoNCERT, in collaboration with GlaxoSmithKline (GSK), is developing CTP-499, a controlled release, deuterated analog of 1-((S)-5-hydroxyhexyl)-3,7-dimethylxanthine (HDX), which is an active metabolite (M1) of pentoxifylline (PTX) that acts as an anti-inflammatory/antioxidant/antifibrotic agent/PDE inhibitor, created using CoNCERT's deuterated chemical entity platform, for the potential oral treatment of type 2 diabetic nephropathy and other forms of chronic kidney diseases (CKD),,,. In February 2012, a phase II type 2 diabetic nephropathy trial began ; in April 2014, results were presented. In January 2014, the drug was listed as being in phase II development. In July 2014, the company planned to submit a request for a Special Protocol Assessment (SPA) to the FDA, later that year. In November 2014, the company planned to initiate a SPA discussion with the US FDA by the end of 2014 for potential future phase III development of CTP-499. In October 2011, CoNCERT was seeking to outlicense the drug.

#### CTP-499 DEVELOPMENT STATUS

##### CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
CoNCERT Pharmaceuticals Inc	Renal disease	US	Phase 2 Clinical	08-Feb-2012
GlaxoSmithKline plc	Renal disease	US	Discovery	02-Jun-2009

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## CTP-499 DRUG NAMES

Names	Type
deuterated analog of 1-((S)-5-hydroxyhexyl)-3,7-dimethylxanthine (HDX) (controlled release formulation, chronic kidney disease), CoNCERT/GSK CTP-499	Research Code
deuterated undisclosed antiinflammatory/antioxidant/antifibrotic agent (controlled release formulation, chronic kidney disease), CoNCERT/GSK	

## CTP-499 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
End stage renal disease											
0	0	0	0	0	0	0	1	0	0	0	1
Renal disease											
0	0	0	0	0	0	0	1	0	0	0	1
Diabetic nephropathy											
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	2	0	0	0	3

### 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

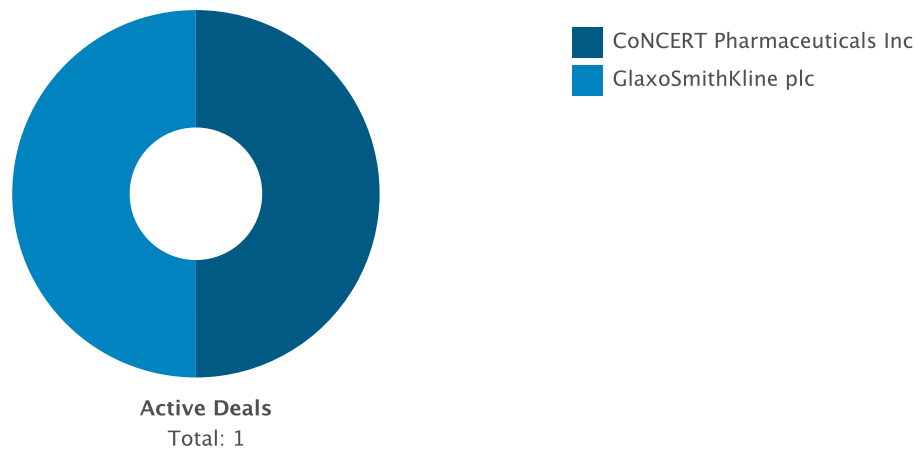
Includes Phase 1, Phase 1a, Phase 1, Phase 1/2 (where enrolment count is under 100 or not specified), Phase 0

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CTP-499 DEALS AND PATENTS

DEALS

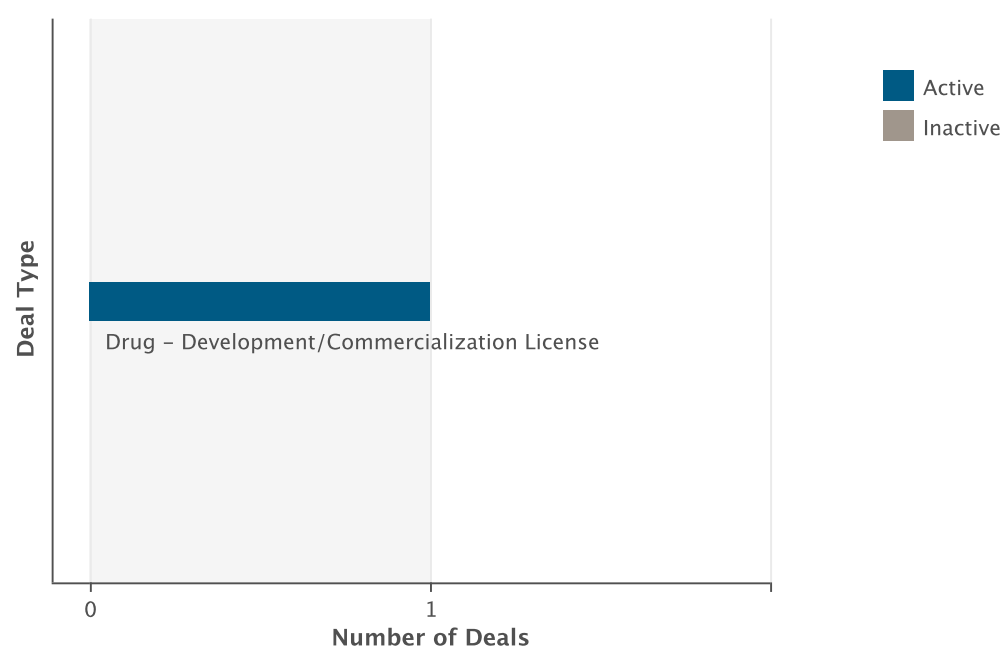
Deals by Parent Company Chart



Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
CoNCERT Pharmaceuticals Inc	1	0	0	0	1
GlaxoSmithKline plc	0	0	1	0	1

Deals by Type Chart



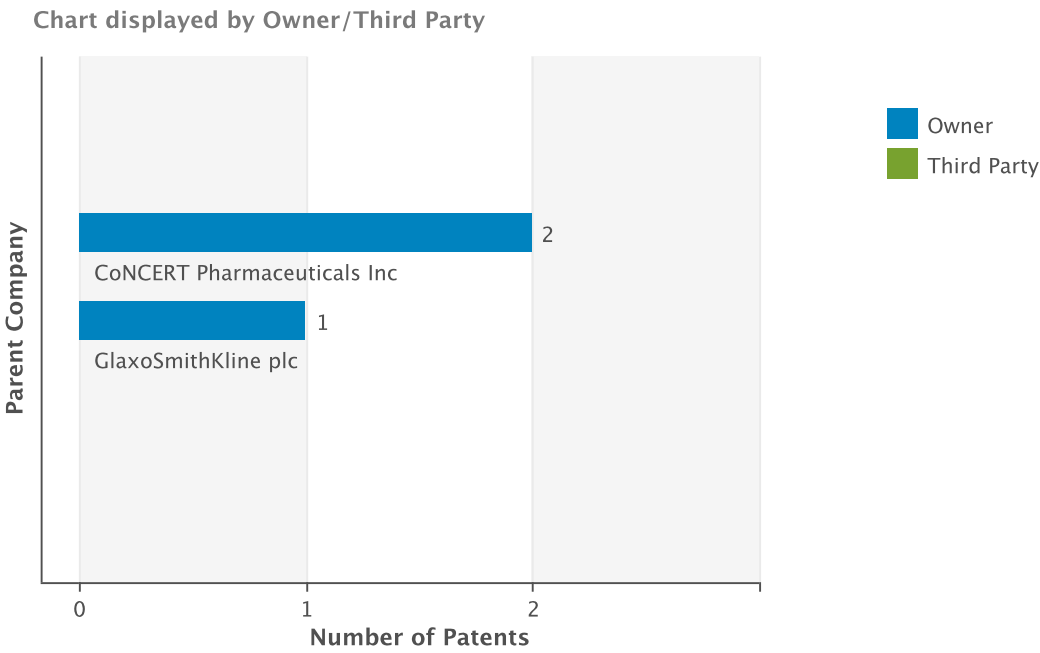
Deals by Type Table

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

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PATENTS

Patents by Parent Company Chart

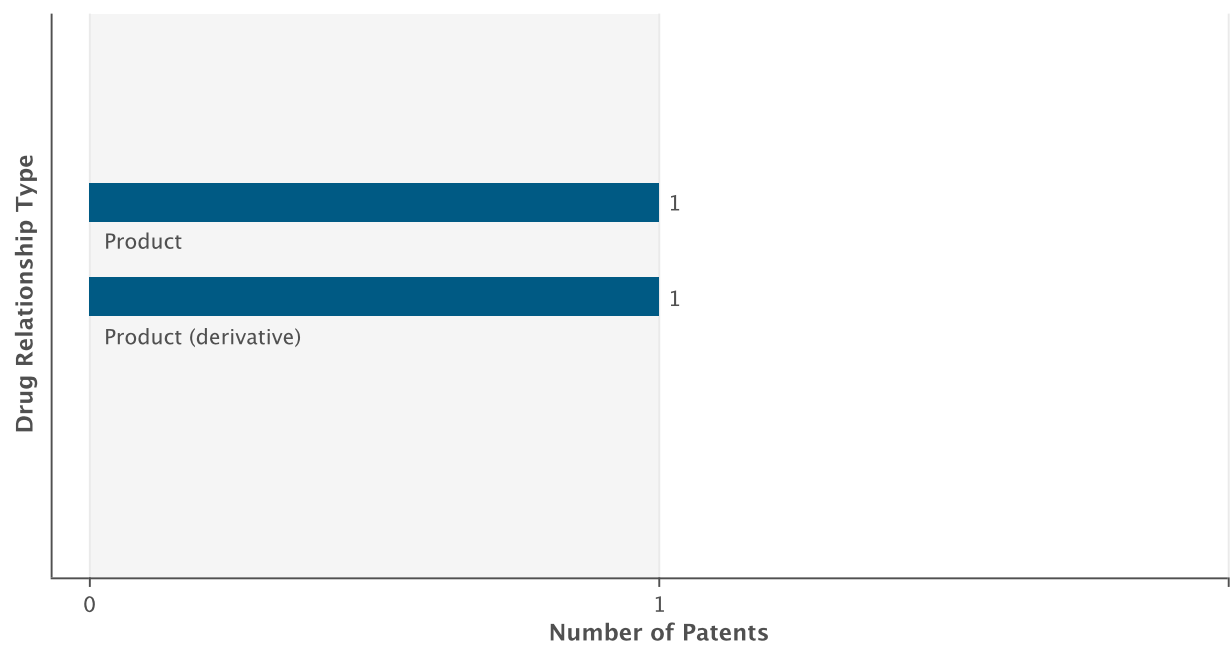


Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
CoNCERT Pharmaceuticals Inc	2	0	2
GlaxoSmithKline plc	1	0	1

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Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
Product	1
Product (derivative)	1



## CTP-347

### CTP-347 SNAPSHOT

Drug Name	CTP-347
Key Synonyms	
Originator Company	CoNCERT Pharmaceuticals Inc
Active Companies	CoNCERT Pharmaceuticals Inc
Inactive Companies	
Highest Status	Phase 1 Clinical
Active Indications	Hot flashes
Target-based Actions	
Other Actions	5-HT uptake inhibitor
Technologies	Oral formulation;Small molecule therapeutic
Last Change Date	09-Apr-2014

### CTP-347 DEVELOPMENT PROFILE

#### SUMMARY

CoNCERT is developing CTP-347, a deuterated form of the selective serotonin reuptake inhibitor paroxetine, for the potential oral treatment of vasomotor symptoms such as hot flashes,. In September 2008, a phase I trial for hot flashes began ; data from the trial were reported in September 2009. In October 2011, the drug was still listed as being in phase I development ; in March 2014, this was still the case. In October 2011, the company was seeking to outlicense the drug.

### CTP-347 DEVELOPMENT STATUS

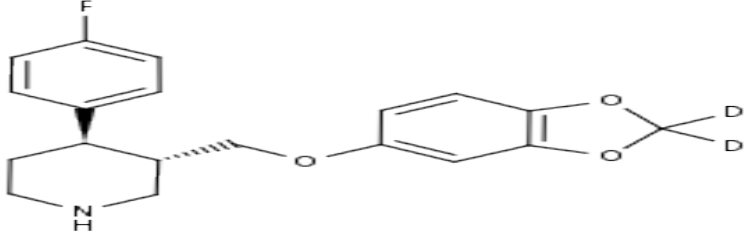
#### CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
CoNCERT Pharmaceuticals Inc	Hot flashes	US	Phase 1 Clinical	25-Sep-2008

### CTP-347 CHEMICAL STRUCTURES

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CAS Registry Number:	Confidence Level:
	4
	
Name	Type
CTP-347	Research Code

### CTP-347 DRUG NAMES

Names	Type
deuterated paroxetine (oral, vasomotor symptoms), CoNCERT	
CTP-347	Research Code

### CTP-347 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
Hot flashes											
0	0	0	0	0	0	0	1	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	0	0	1	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)

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### 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)

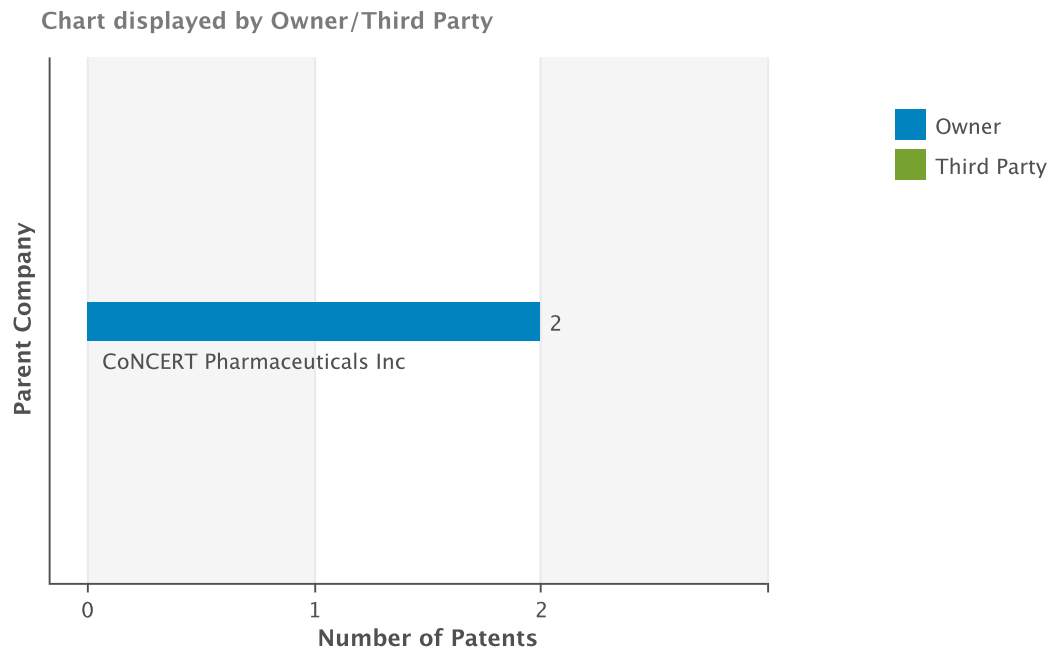
### Phase 1 Clinical

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

## CTP-347 DEALS AND PATENTS

### PATENTS

#### Patents by Parent Company Chart

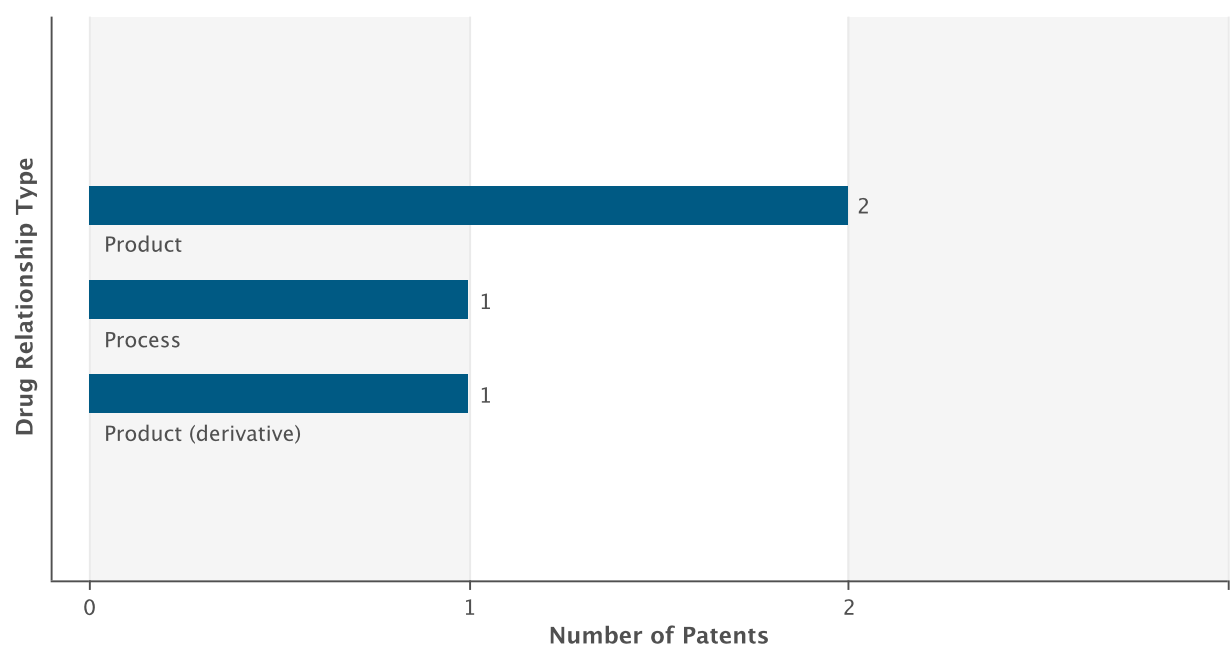


#### Patents by Parent Company Table

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

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Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
Product	2
Product (derivative)	1
Process	1

## CTP-730

### CTP-730 SNAPSHOT

Drug Name	CTP-730
Key Synonyms	
Originator Company	CoNCERT Pharmaceuticals Inc
Active Companies	CoNCERT Pharmaceuticals Inc; Celgene Corp
Inactive Companies	
Highest Status	Phase 1 Clinical
Active Indications	Inflammatory disease
Target-based Actions	
Other Actions	Anticancer; Unspecified drug target; Anti-inflammatory
Technologies	Oral formulation; Small molecule therapeutic; Suspension
Last Change Date	05-Jan-2015

### CTP-730 DEVELOPMENT PROFILE

#### SUMMARY

CoNCERT Pharmaceuticals, in collaboration with Celgene Corp, is developing CTP-730, a lead from deuterium-modified compounds, using the company's deuterated chemical entity platform (DCE) technology, for the potential treatment of inflammation. In September 2014, a phase I trial was initiated. At that time, the trial was expected to complete in 2015. In November 2014, the multiple ascending dose phase I trial was expected to begin in 2015.

CoNCERT Pharmaceuticals, in collaboration with Celgene Corp was previously investigating deuterium-modified compounds, using the company's deuterated chemical entity platform (DCE) technology, for the potential treatment of cancer. In November 2013, the drug was listed as being in preclinical development ; however by January 2014, the program was no longer developed for the treatment of cancer.

#### CLINICAL DATA PHASE I

In January 2014, phase I trials were planned for that year. In May 2014, the company was planning to initiate clinical trials by the end of 2014. In September 2014, a randomized, interventional, double-blind, single ascending, safety, tolerability and pharmacokinetics phase I trial (NCT02239081; CP730.1001) of CTP-730 was initiated in healthy subjects (expected n = 40) in Australia. At that time, the trial was expected to complete in 2015. In November 2014, the multiple ascending dose phase I trial was expected to begin in 2015.

### CTP-730 DEVELOPMENT STATUS

#### CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
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Company	Indication	Country	Development Status	Date
Celgene Corp	Inflammatory disease	Australia	Phase 1 Clinical	10-Sep-2014
CoNCERT Pharmaceuticals Inc	Inflammatory disease	Australia	Phase 1 Clinical	10-Sep-2014
Celgene Corp	Cancer	US	No Development Reported	30-Jan-2014
CoNCERT Pharmaceuticals Inc	Cancer	US	No Development Reported	30-Jan-2014

## CTP-730 DRUG NAMES

Names	Type
CTP-730	Research Code
deuterium-modified compounds (cancer/inflammation), CoNCERT/Celgene	

## CTP-730 CLINICAL TRIALS

### Total Trials by Phase and Status

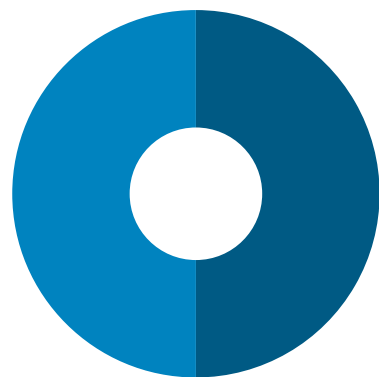
Phase 4 Clinical		Phase 3 Clinical		Phase 2 Clinical		Phase 1 Clinical		Phase Unspecified		Total	
On-going	All	On-going	All	On-going	All	On-going	All	On-going	All	On-going	All
Total by Phase and Status											
0	0	0	0	0	0	0	1	0	0	0	1

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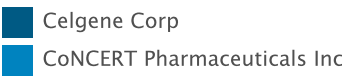
CTP-730 DEALS AND PATENTS

DEALS

Deals by Parent Company Chart



Active Deals  
Total: 1

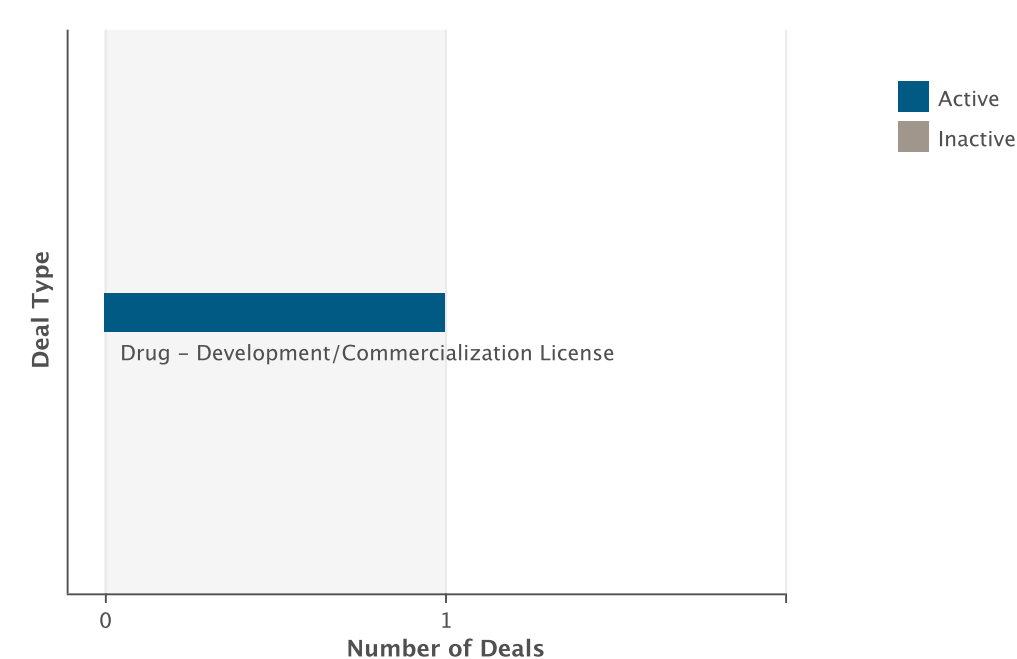


Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
CoNCERT Pharmaceuticals Inc	1	0	0	0	1
Celgene Corp	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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## CTP-354

### CTP-354 SNAPSHOT

Drug Name	CTP-354
Key Synonyms	
Originator Company	CoNCERT Pharmaceuticals Inc
Active Companies	CoNCERT Pharmaceuticals Inc
Inactive Companies	
Highest Status	Phase 1 Clinical
Active Indications	Neuropathic pain;Muscle hypertonia
Target-based Actions	GABA A receptor modulator
Other Actions	Analgesic;Anxiolytic
Technologies	Oral formulation;Small molecule therapeutic
Last Change Date	13-Nov-2014

### CTP-354 DEVELOPMENT PROFILE

#### SUMMARY

CoNCERT Pharmaceuticals is developing CTP-354 (C-21191), the lead from deuterium-modified, non-sedating subtype-selective GABA A modulators using its deuterated chemical entity (DCE) platform, for the potential oral treatment of spasticity and neuropathic pain . In March 2013, the drug was listed as being in phase I development. By March 2014, a multiple-ascending dose phase I study had been initiated. In July 2014, the US FDA lifted a partial clinical hold on the phase I spasticity study ; in August 2014, positive results were reported. In October 2014, positive results were reported and at that time, the company planned to initiate phase II trials for spasticity associated with spinal cord injury and multiple sclerosis by the end of 2014 and in 'early 2015', respectively. In November 2014, the company announced the delay in the initiation of phase II trials and planned to conduct additional non-clinical studies prior to advancing into phase II trials. In March 2013, the company was seeking to outlicense the drug.

The company was previously investigating the drug for the potential treatment of anxiety ; however, no further development had been reported for this indication.

### CTP-354 DEVELOPMENT STATUS

#### CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
CoNCERT Pharmaceuticals Inc	Muscle hypertonia	US	Phase 1 Clinical	13-Mar-2013
CoNCERT Pharmaceuticals Inc	Neuropathic pain	US	Phase 1 Clinical	13-Mar-2013
CoNCERT Pharmaceuticals Inc	Anxiety disorder	US	No Development Reported	14-Mar-2013

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CTP-354 DRUG NAMES

Names	Type
CTP-354	Research Code
C-21191	Research Code
deuterium-modified GABA-A modulator (spasticity/neuropathic pain/anxiety), CoNCERT Pharmaceuticals	

CTP-354 CLINICAL TRIALS

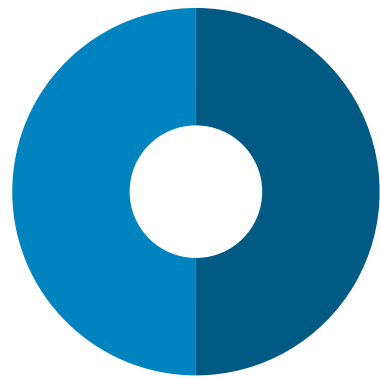
Total Trials by Phase and Status

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	0	0	2	0	0	0	2

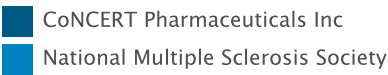
CTP-354 DEALS AND PATENTS

DEALS

Deals by Parent Company Chart



Active Deals  
Total: 1

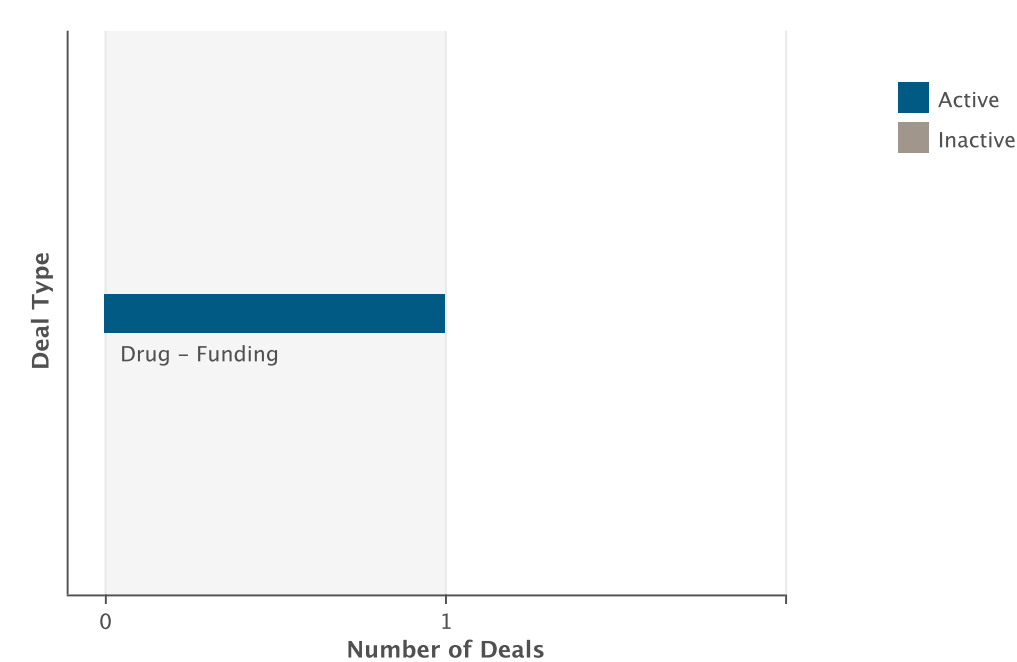


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Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
CoNCERT Pharmaceuticals Inc	1	0	0	0	1
National Multiple Sclerosis Society	0	0	1	0	1

Deals by Type Chart

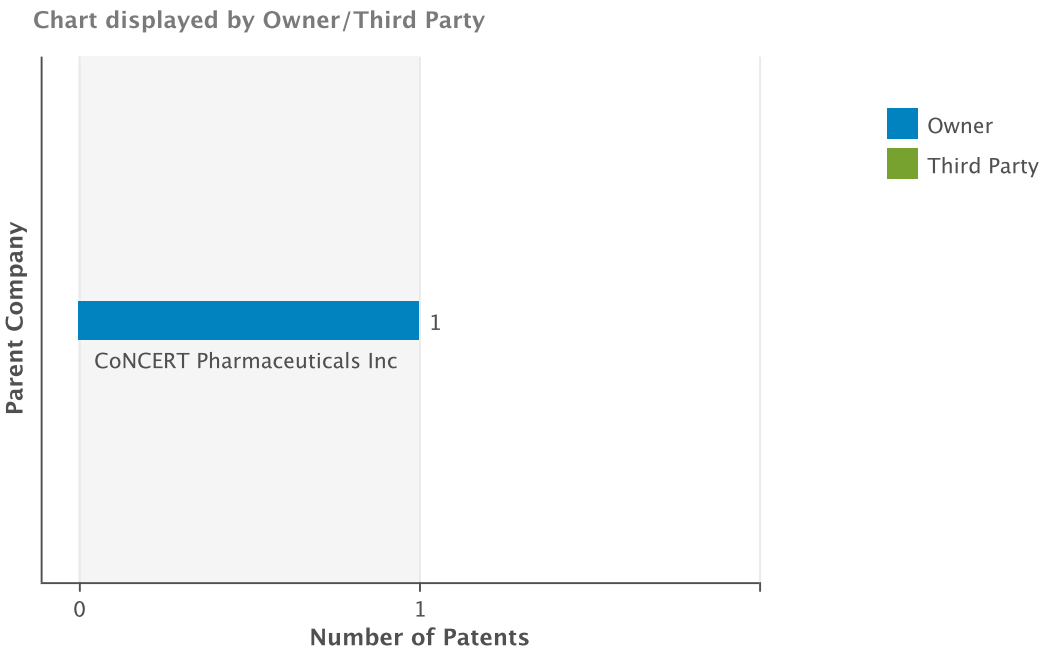


Deals by Type Table

Deal Type	Active	Inactive	Total
Drug - Funding	1	0	1

PATENTS

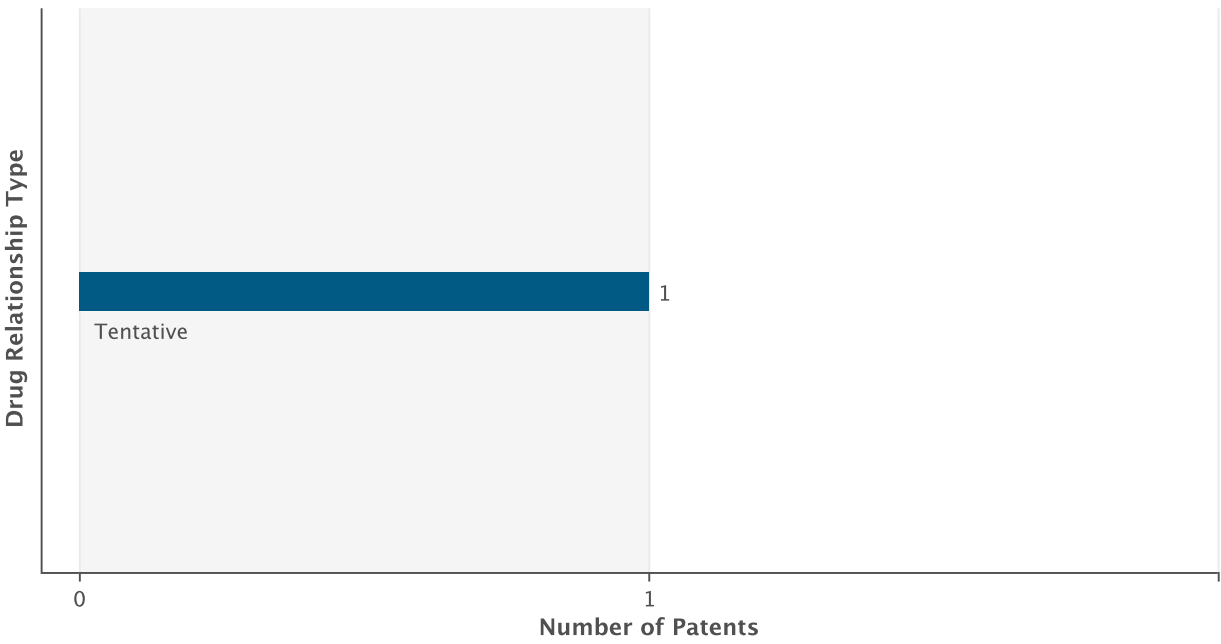
Patents by Parent Company Chart



Patents by Parent Company Table

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

Patents by Drug Relationship Type Chart



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Patents by Drug Relationship Type Table

Drug Relationship	Total
Tentative	1

## C-10068

### C-10068 SNAPSHOT

Drug Name	C-10068
Key Synonyms	
Originator Company	CoNCERT Pharmaceuticals Inc
Active Companies	Walter Reed Army Institute of Research;CoNCERT Pharmaceuticals Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Epilepsy;Neuropathic pain;Brain injury
Target-based Actions	Opioid receptor sigma agonist 1
Other Actions	Analgesic;Antidepressant;Anticonvulsant agent;Neuroprotectant
Technologies	Oral formulation;Small molecule therapeutic
Last Change Date	15-May-2014

### C-10068 DEVELOPMENT PROFILE

#### SUMMARY

CoNCERT Pharmaceuticals and the Walter Reed Army Institute of Research (WRAIR) are investigating C-10068, the lead from deuterium-modified sigma-1 agonists, for the potential oral treatment of neuropathic pain, epilepsy and other seizure-generating diseases and injuries such as ischemic stroke and traumatic brain injury (TBI). By February 2011, development was ongoing. In September 2012, the drug was listed as being in research for epilepsy. In January 2014, the drug was listed as being in preclinical development for pain and seizures. In May 2014, the company was planning to initiate clinical trials by the end of 2014. In October 2011, the company was seeking to outlicense the drug.

CoNCERT and WRAIR were previously investigating the drug for the potential treatment of depression. In September 2012, the drug was listed as being in research for epilepsy and depression; however, in January 2014, the drug was no longer being investigated for depression.

### C-10068 DEVELOPMENT STATUS

#### CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
CoNCERT Pharmaceuticals Inc	Brain injury	US	Discovery	02-Feb-2011
CoNCERT Pharmaceuticals Inc	Epilepsy	US	Discovery	02-Feb-2011
CoNCERT Pharmaceuticals Inc	Neuropathic pain	US	Discovery	02-May-2011
Walter Reed Army Institute of Research	Brain injury	US	Discovery	02-Feb-2011

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Company	Indication	Country	Development Status	Date
Walter Reed Army Institute of Research	Epilepsy	US	Discovery	02-Feb-2011
Walter Reed Army Institute of Research	Neuropathic pain	US	Discovery	02-May-2011
CoNCERT Pharmaceuticals Inc	Depression	US	No Development Reported	31-Jan-2014
Walter Reed Army Institute of Research	Depression	US	No Development Reported	31-Jan-2014

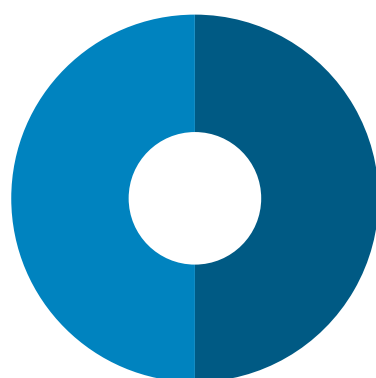
## C-10068 DRUG NAMES

Names	Type
deuterium-modified sigma-1 agonist (epilepsy/depression), CoNCERT	
C-10068	Research Code
deuterated sigma-1 agonists (epilepsy/brain injury/neuropathic pain), CoNCERT/Walter Reed Army Institute	

## C-10068 DEALS AND PATENTS

### DEALS

#### Deals by Parent Company Chart



Active Deals  
Total: 2

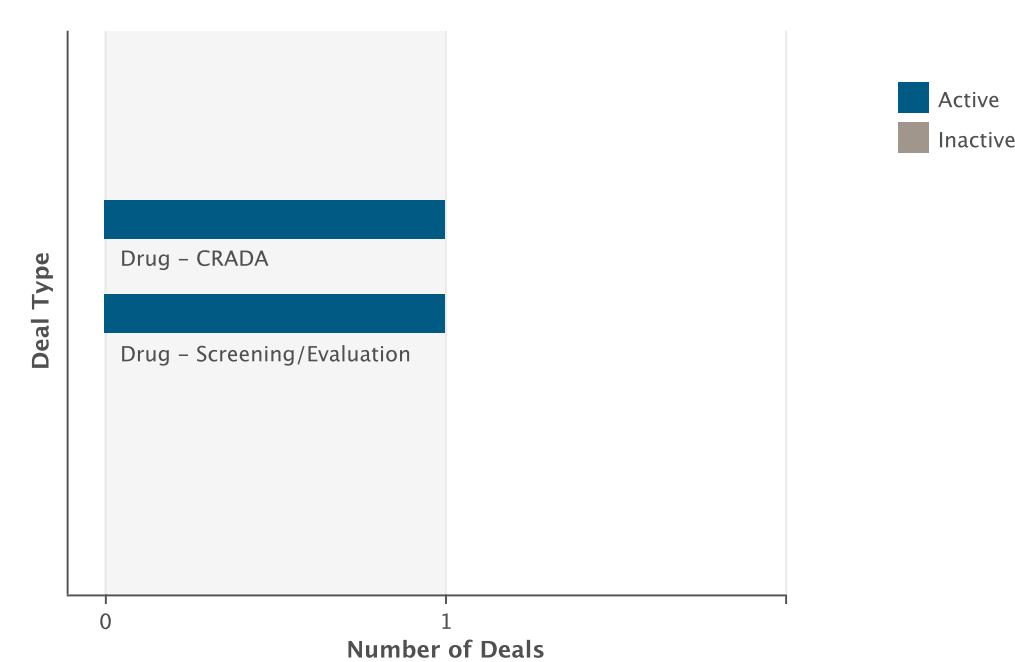


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Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
CoNCERT Pharmaceuticals Inc	1	0	1	0	2
US Government	1	0	1	0	2

Deals by Type Chart



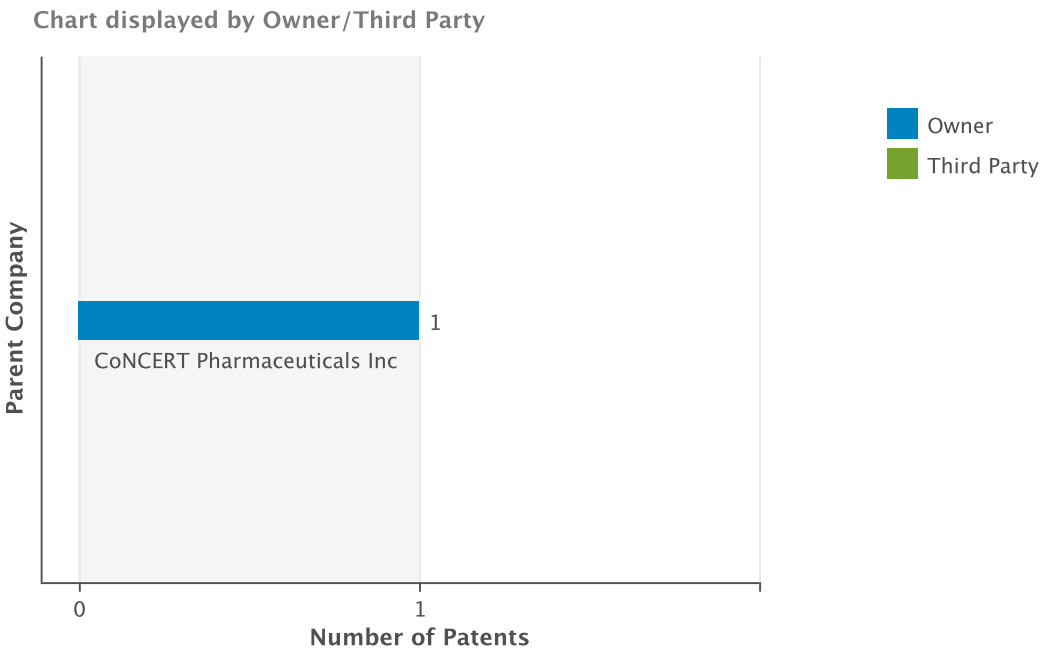
Deals by Type Table

Deal Type	Active	Inactive	Total
Drug - CRADA	1	0	1
Drug - Screening/Evaluation	1	0	1



PATENTS

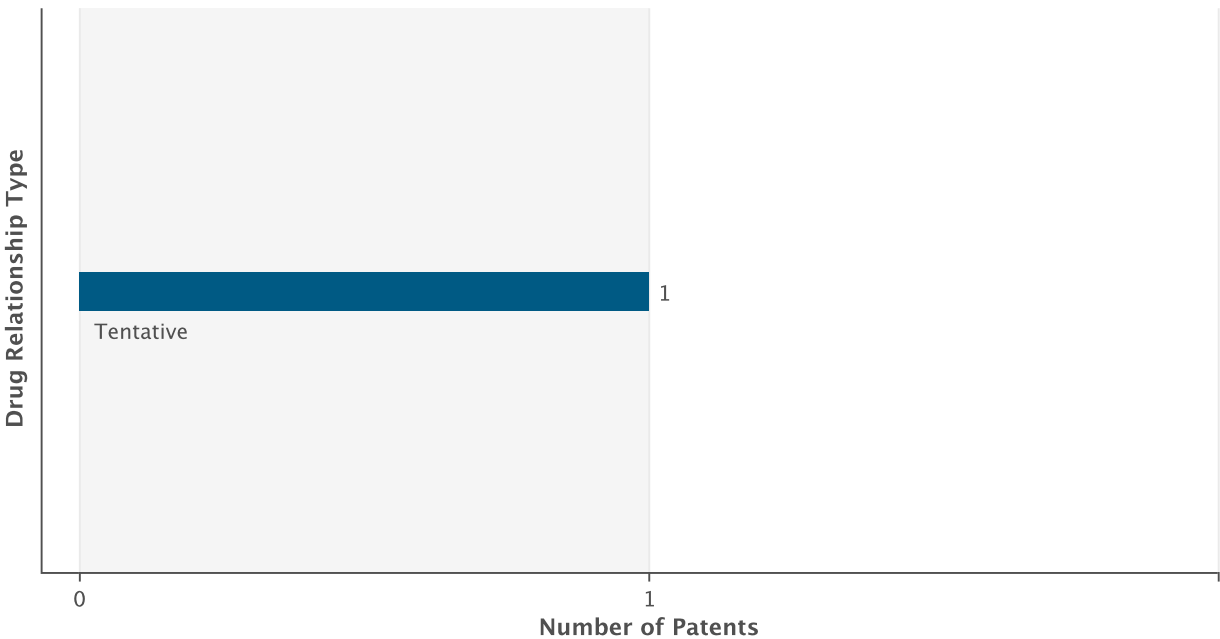
Patents by Parent Company Chart



Patents by Parent Company Table

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

Patents by Drug Relationship Type Chart



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Patents by Drug Relationship Type Table

Drug Relationship	Total
Tentative	1

## deuterated ivacaftor analogs (cystic fibrosis/COPD), Concert Pharmaceuticals

### deuterated ivacaftor analogs (cystic fibrosis/COPD), Concert Pharmaceuticals SNAPSHOT

Drug Name	deuterated ivacaftor analogs (cystic fibrosis/COPD), Concert Pharmaceuticals
Key Synonyms	
Originator Company	CoNCERT Pharmaceuticals Inc
Active Companies	CoNCERT Pharmaceuticals Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Cystic fibrosis;Chronic obstructive pulmonary disease
Target-based Actions	CFTR stimulator
Other Actions	
Technologies	Oral formulation;Small molecule therapeutic
Last Change Date	15-May-2014

### deuterated ivacaftor analogs (cystic fibrosis/COPD), Concert Pharmaceuticals DEVELOPMENT PROFILE

#### SUMMARY

CoNCERT Pharmaceuticals is investigating deuterated ivacaftor analogs as potentiators of cystic fibrosis transmembrane conductance regulator, for the potential oral treatment of cystic fibrosis and COPD,. In September 2013, preclinical data were presented. In May 2014, the company was planning to initiate clinical trials by the end of 2014. In November 2013, the company was seeking to outlicense the drug.

### deuterated ivacaftor analogs (cystic fibrosis/COPD), Concert Pharmaceuticals DEVELOPMENT STATUS

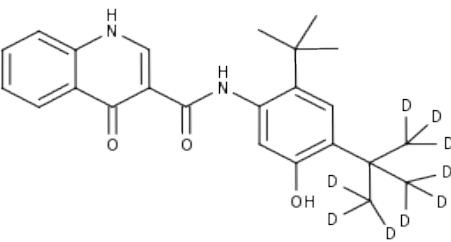
#### CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
CoNCERT Pharmaceuticals Inc	Chronic obstructive pulmonary disease	US	Discovery	14-Mar-2013
CoNCERT Pharmaceuticals Inc	Cystic fibrosis	US	Discovery	14-Mar-2013

### deuterated ivacaftor analogs (cystic fibrosis/COPD), Concert Pharmaceuticals CHEMICAL STRUCTURES

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CAS Registry Number:	Confidence Level:
	4
	

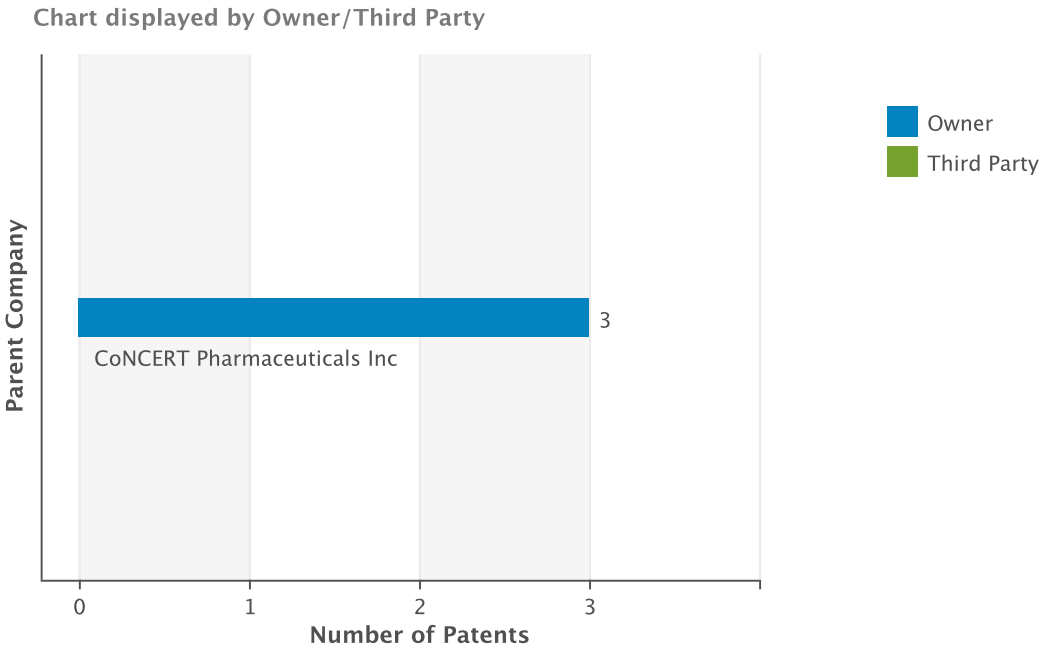
deuterated ivacaftor analogs (cystic fibrosis/COPD), Concert Pharmaceuticals DRUG NAMES

Names	Type
deuterated ivacaftor analogs (cystic fibrosis/COPD), Concert Pharmaceuticals	

deuterated ivacaftor analogs (cystic fibrosis/COPD), Concert Pharmaceuticals DEALS AND PATENTS

PATENTS

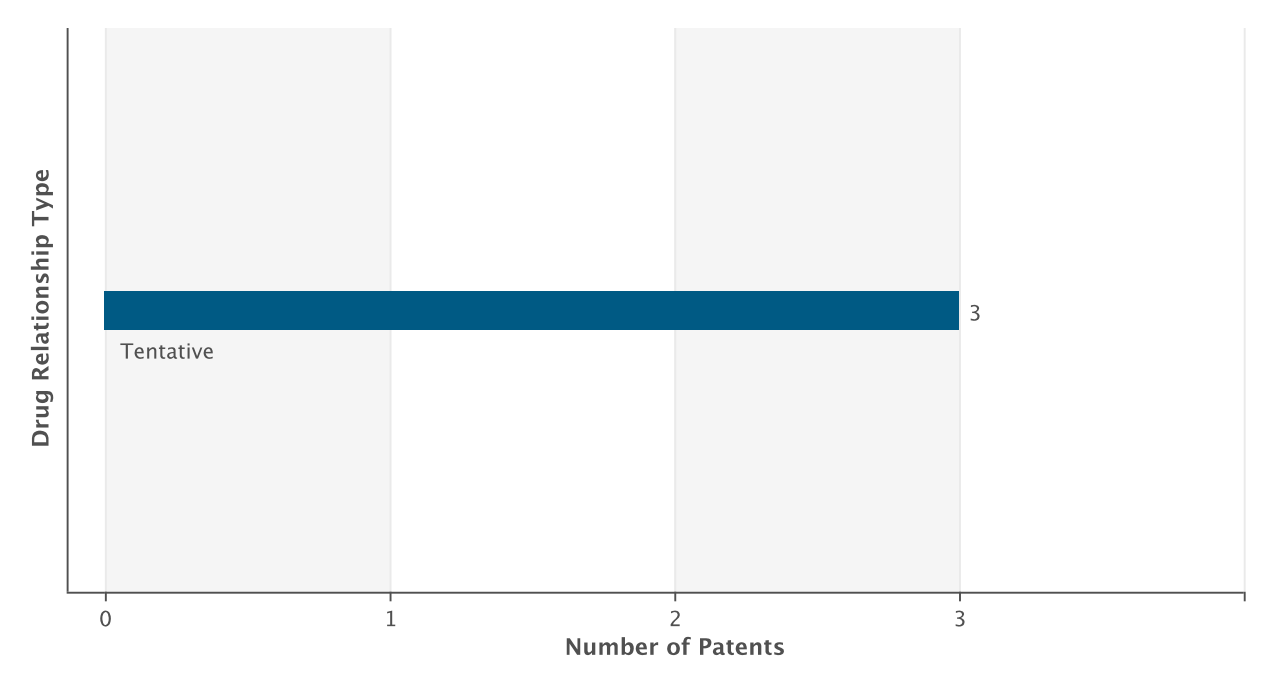
Patents by Parent Company Chart



Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
CoNCERT Pharmaceuticals Inc	3	0	3

Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
Tentative	3

## deuterated praziquantel analogs (schistosomiasis), Therapeutics for Rare and Neglected Diseases/CoNCERT Pharmaceuticals

### deuterated praziquantel analogs (schistosomiasis), Therapeutics for Rare and Neglected Diseases/CoNCERT Pharmaceuticals SNAPSHOT

<b>Drug Name</b>	deuterated praziquantel analogs (schistosomiasis), Therapeutics for Rare and Neglected Diseases/CoNCERT Pharmaceuticals
<b>Key Synonyms</b>	
<b>Originator Company</b>	CoNCERT Pharmaceuticals Inc
<b>Active Companies</b>	Therapeutics for Rare and Neglected Diseases;CoNCERT Pharmaceuticals Inc
<b>Inactive Companies</b>	
<b>Highest Status</b>	Discovery
<b>Active Indications</b>	Schistosomiasis
<b>Target-based Actions</b>	
<b>Other Actions</b>	Unspecified drug target;Antiparasitic
<b>Technologies</b>	Small molecule therapeutic
<b>Last Change Date</b>	04-Sep-2014

### deuterated praziquantel analogs (schistosomiasis), Therapeutics for Rare and Neglected Diseases/CoNCERT Pharmaceuticals DEVELOPMENT PROFILE

#### SUMMARY

Therapeutics for Rare and Neglected Diseases and CoNCERT Pharmaceuticals, are investigating deuterium-modified praziquantel analogs for the potential treatment for schistosomiasis. In February 2013, preclinical studies were ongoing ; in August 2014, this was still the case.

### deuterated praziquantel analogs (schistosomiasis), Therapeutics for Rare and Neglected Diseases/CoNCERT Pharmaceuticals DEVELOPMENT STATUS

#### CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
CoNCERT Pharmaceuticals Inc	Schistosomiasis	US	Discovery	15-Nov-2011
Therapeutics for Rare and Neglected Diseases	Schistosomiasis	US	Discovery	01-Feb-2013

### deuterated praziquantel analogs (schistosomiasis), Therapeutics for Rare and Neglected Diseases/CoNCERT Pharmaceuticals DRUG NAMES

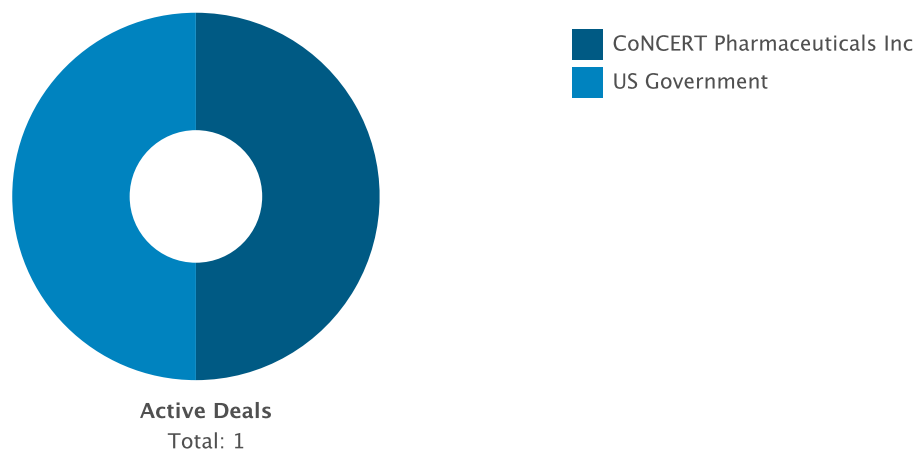
Names	Type
deuterated praziquantel analogs (schistosomiasis), Therapeutics for Rare and Neglected Diseases/CoNCERT Pharmaceuticals	
deuterated praziquantel analogs (schistosomiasis), CoNCERT Pharmaceuticals	

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DEALS

Deals by Parent Company Chart

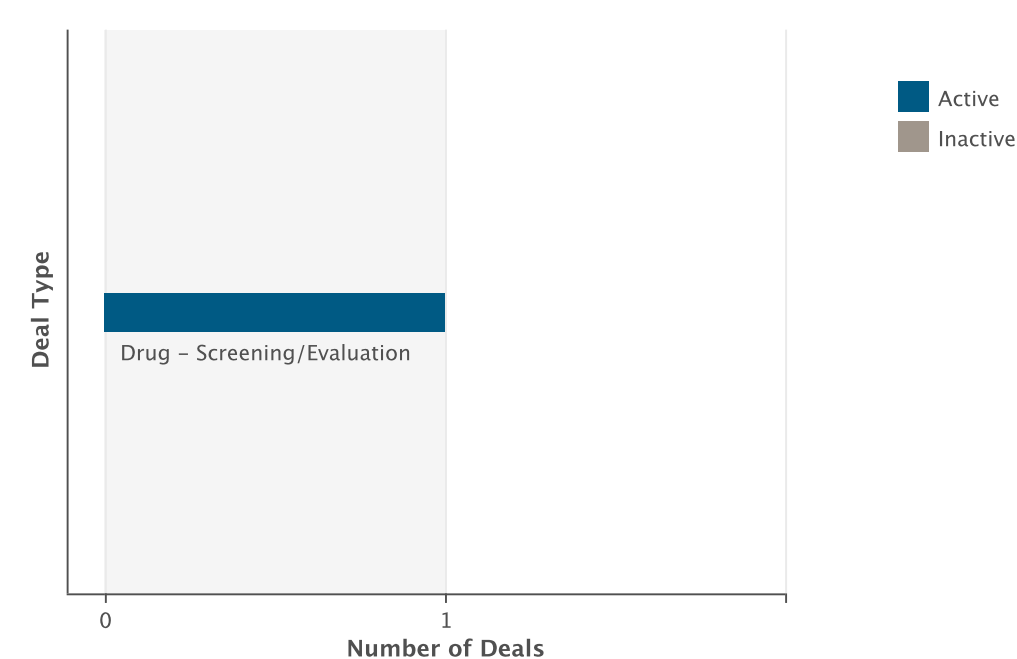


Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
CoNCERT Pharmaceuticals Inc	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 - Screening/Evaluation	1	0	1



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