

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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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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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 | 161 |
| Number of Patents as Third Party | 1 |
| Number of Deals | 8 |
| Key Indications | Neuropathic pain,Epilepsy,Renal disease,Inflammatory disease,Pain,Cancer,Multiple myeloma,Cystic fibrosis,Anxiety disorder,Brain |
| Key Target-based Actions | NMDA receptor antagonist,GABA A receptor modulator,GABA B receptor agonist,CFTR stimulator,Calcium channel inhibitor T-type,PDE inhibitor,HIV protease inhibitor,Opioid receptor sigma agonist 1,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 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 overallotments. 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.

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.

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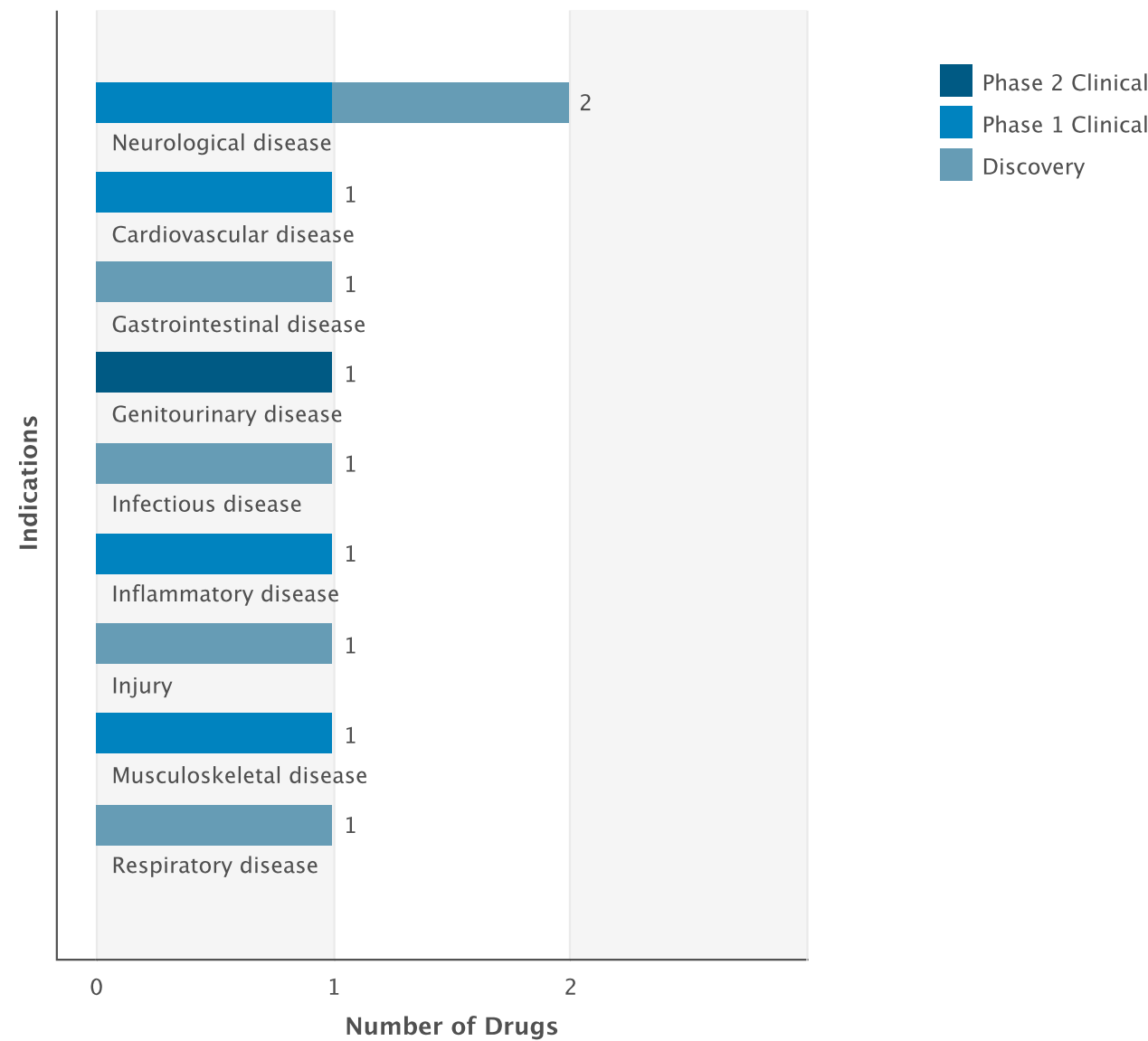


PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



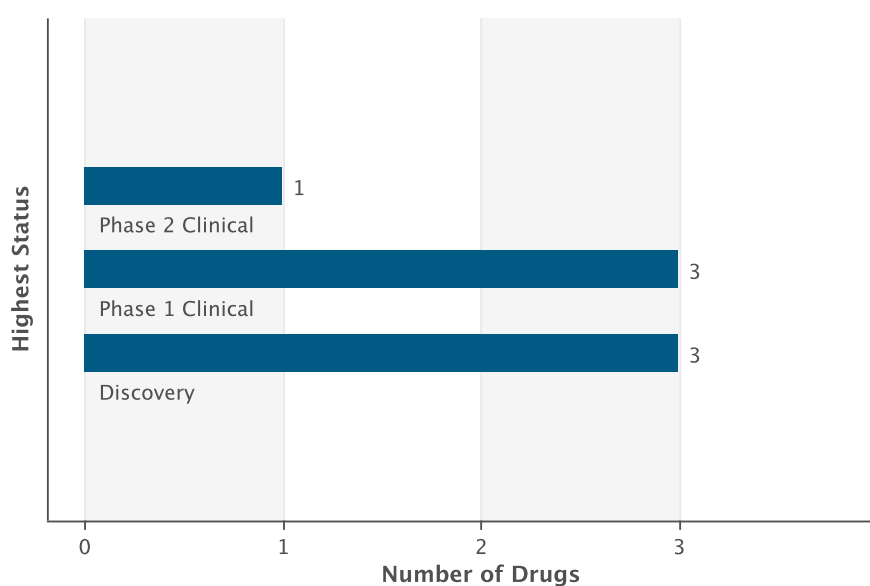
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Drugs by Indication Table

| 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 |
| Psychiatric disorder | 0 | 3 | 3 |
| Musculoskeletal disease | 1 | 2 | 3 |
| Genitourinary disease | 1 | 1 | 2 |
| Gastrointestinal disease | 1 | 1 | 2 |
| Respiratory disease | 1 | 1 | 2 |
| Immune disorder | 0 | 2 | 2 |
| Hematological disease | 0 | 1 | 1 |
| Injury | 1 | 0 | 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 - Screening/Evaluation | 1 | 0 | 1 | 0 | 2 |
| Drug - Development/Commercialization License | 4 | 0 | 0 | 0 | 4 |
| Drug - CRADA | 1 | 0 | 0 | 0 | 1 |
| Drug - Funding | 1 | 0 | 0 | 0 | 1 |

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 | 71 | 0 | 71 |
| Genitourinary disease | 65 | 0 | 65 |
| Growth disorder | 11 | 0 | 11 |
| Hematological disease | 35 | 0 | 35 |
| Degeneration | 14 | 0 | 14 |
| Andrology | 22 | 0 | 22 |
| Immune disorder | 57 | 0 | 57 |
| Psychiatric disorder | 33 | 0 | 33 |
| Musculoskeletal disease | 30 | 0 | 30 |
| Neoplasm | 55 | 0 | 55 |
| Ocular disease | 18 | 0 | 18 |
| Genetic disorder | 19 | 0 | 19 |
| Metabolic disorder | 41 | 0 | 41 |
| Mouth disease | 1 | 0 | 1 |
| Neurological disease | 80 | 0 | 80 |
| Nutritional disorder | 16 | 0 | 16 |
| Respiratory disease | 35 | 0 | 35 |
| Infectious disease | 41 | 0 | 41 |
| Injury | 6 | 0 | 6 |
| Toxicity and intoxication | 12 | 0 | 12 |
| Inflammatory disease | 47 | 0 | 47 |

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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

| | |
|-----------------------------|--|
| Drug Name | CTP-499 |
| Key Synonyms | |
| Originator Company | CoNCERT Pharmaceuticals Inc |
| Active Companies | GlaxoSmithKline plc;CoNCERT Pharmaceuticals Inc |
| Inactive Companies | |
| Highest Status | Phase 2 Clinical |
| Active Indications | Renal disease |
| Target-based Actions | PDE inhibitor |
| Other Actions | Antioxidant agent;Renal system agent;Fibrosuppressant;Anti-inflammatory |
| Technologies | Oral formulation;Controlled release formulation;Small molecule therapeutic |
| Last Change Date | 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 |
|---|---------------|
| CTP-499 | Research Code |
| deuterated undisclosed antiinflammatory/antioxidant/antifibrotic agent (controlled release formulation, chronic kidney disease), CoNCERT/GSK | |
| deuterated analog of 1-((S)-5-hydroxyhexyl)-3,7- dimethylxanthine (HDX) (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

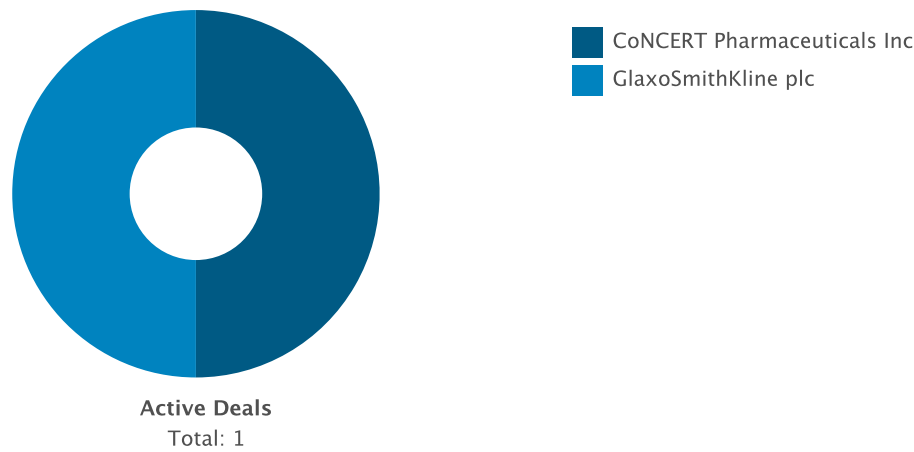
Includes Phase 1, Phase 1a, Phase 1, Phase 1/2 (where enrolment count is under 100 or not specified), Phase 0

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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 |

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Deals by Type Chart



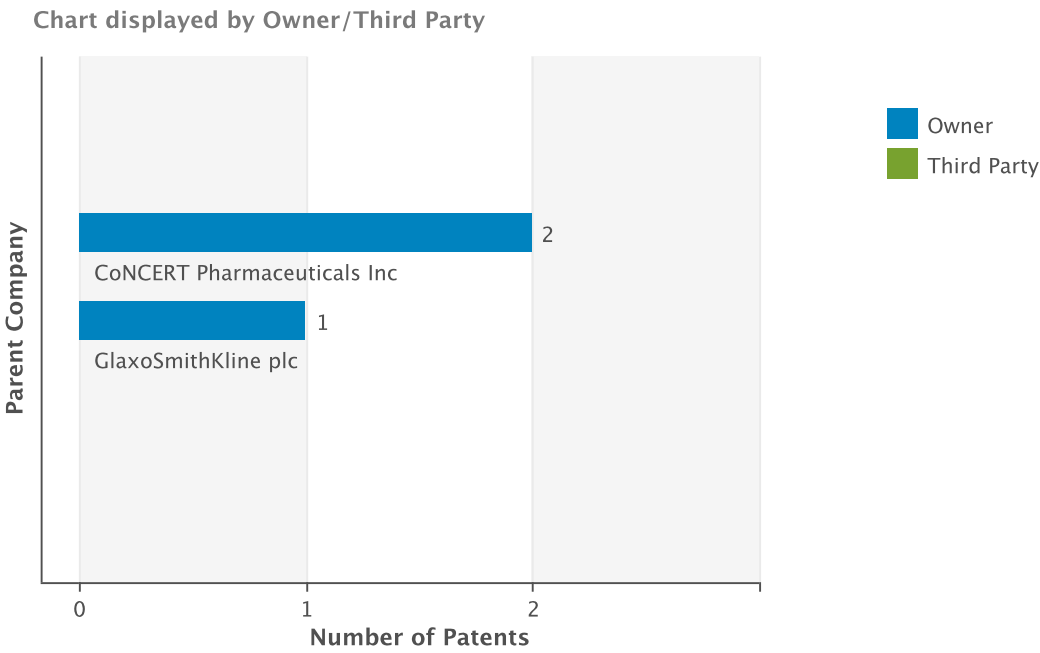
Deals by Type Table

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

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PATENTS

Patents by Parent Company Chart

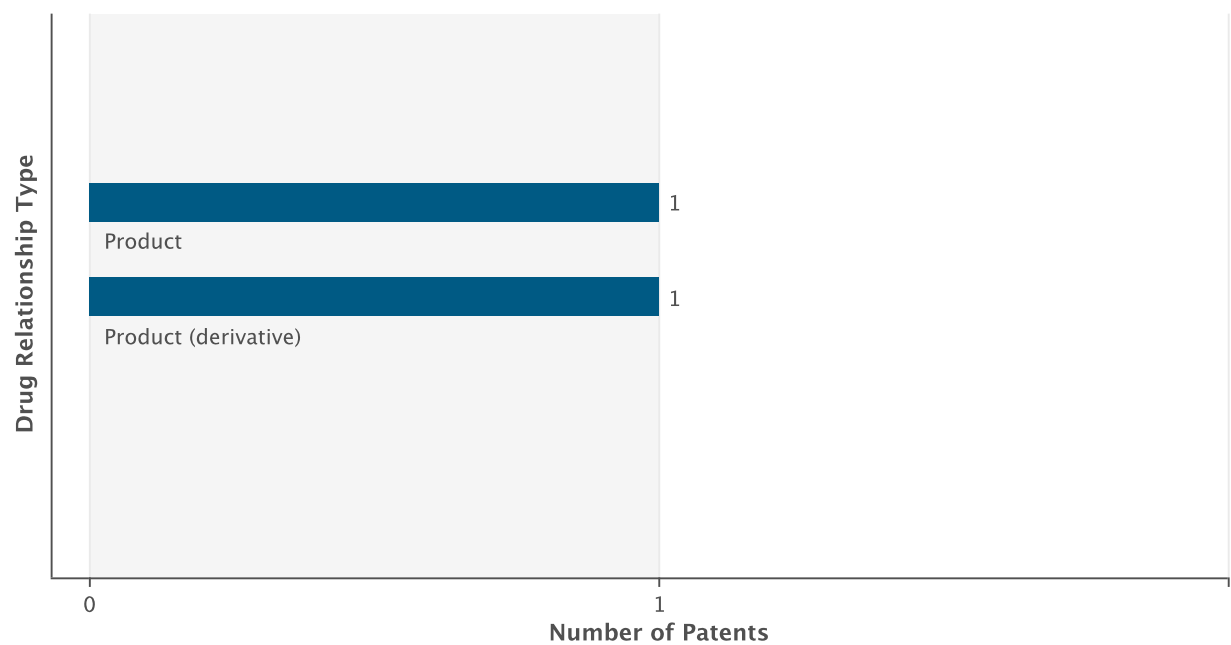


Patents by Parent Company Table

| Company Name | As Owner | As Third Party | Total |
|-----------------------------|----------|----------------|-------|
| 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 (derivative) | 1 |
| Product | 1 |

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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

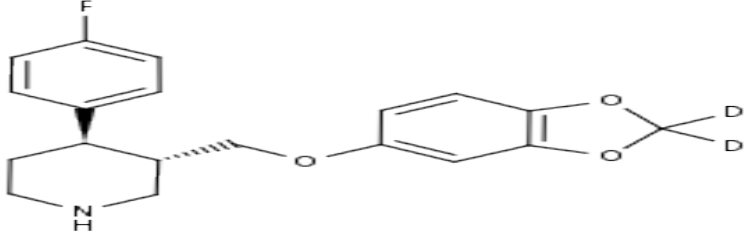
CURRENT DEVELOPMENT STATUS

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

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)

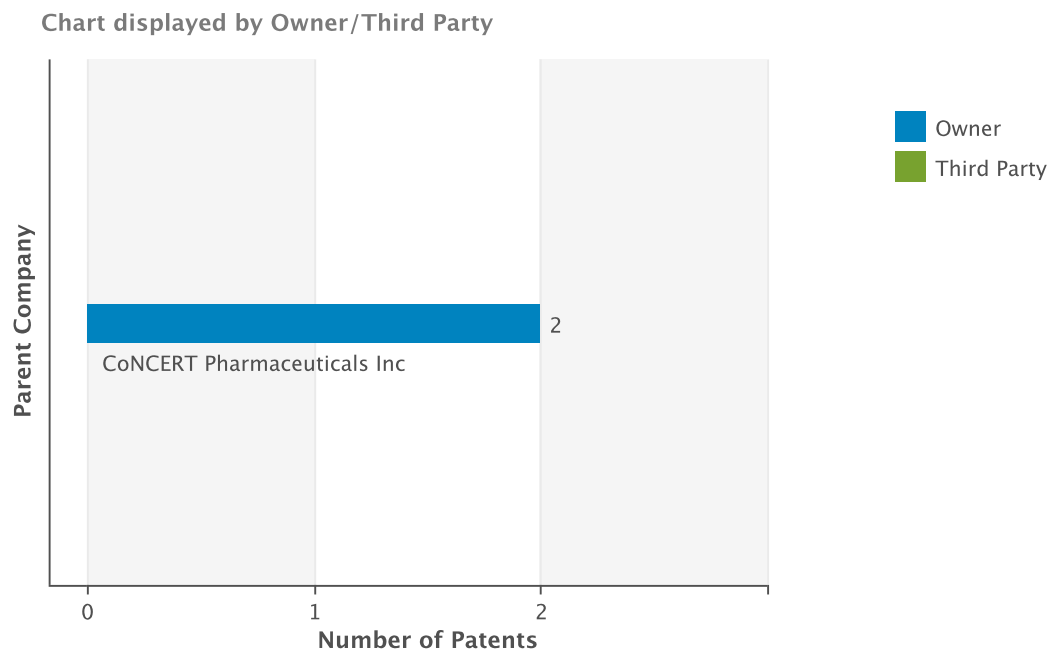
Phase 1 Clinical

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

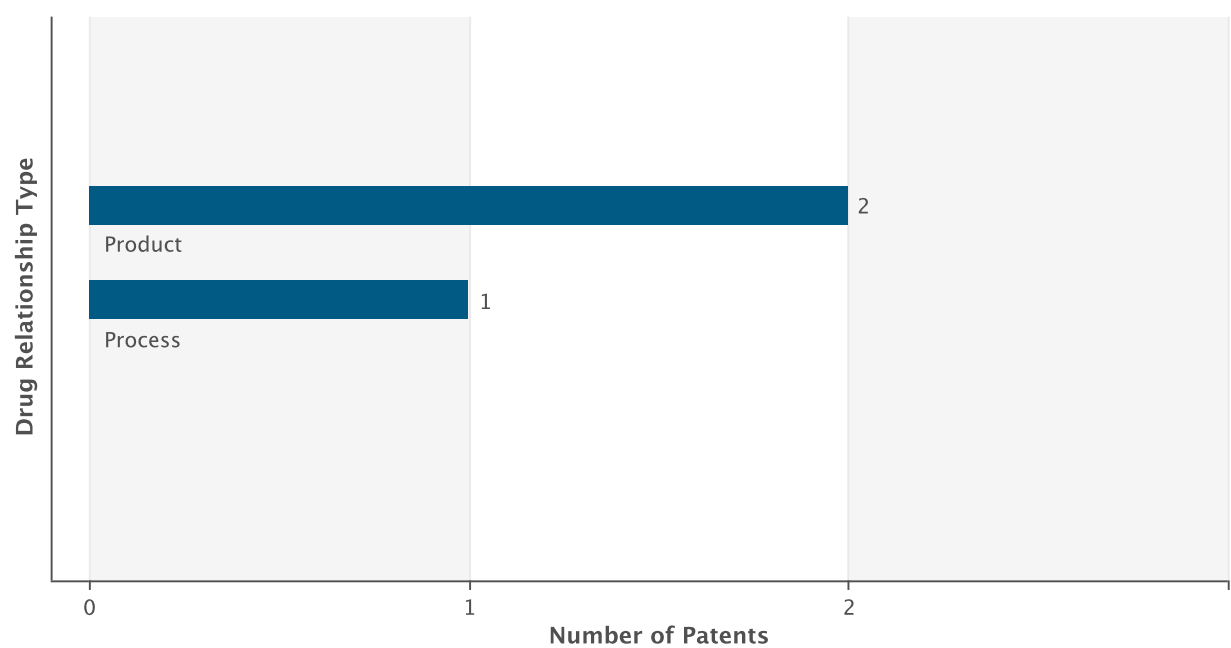


Patents by Parent Company Table

| 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 |
| 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 | Anti-inflammatory; Anticancer; Unspecified drug target |
| Technologies | Oral formulation; Suspension; Small molecule therapeutic |
| Last Change Date | 13-Nov-2014 |

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 | US | Phase 1 Clinical | 10-Sep-2014 |
| CoNCERT Pharmaceuticals Inc | Inflammatory disease | US | 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

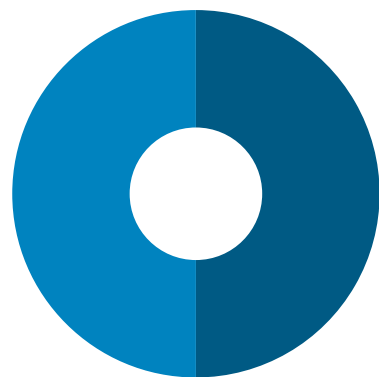
| Phase 4 Clinical | | Phase 3 Clinical | | Phase 2 Clinical | | Phase 1 Clinical | | Phase Unspecified | | Total | |
|---------------------------|-----|------------------|-----|------------------|-----|------------------|-----|-------------------|-----|----------|-----|
| On-going | All | On-going | 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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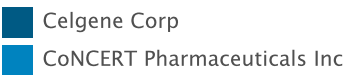
CTP-730 DEALS AND PATENTS

DEALS

Deals by Parent Company Chart



Active Deals
Total: 1

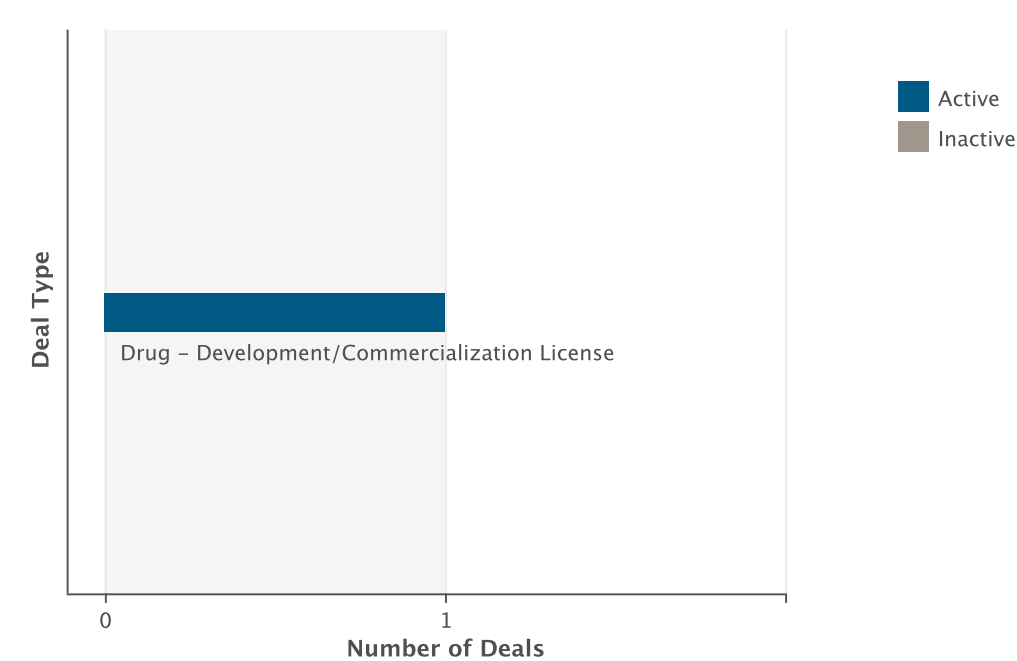


Deals by Parent Company Table

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

CTP-354 CLINICAL TRIALS

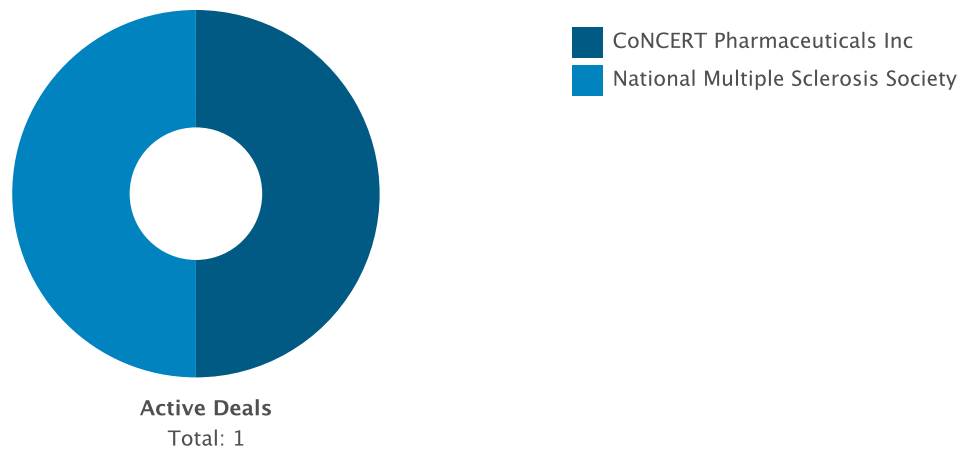
Total Trials by Phase and Status

| 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

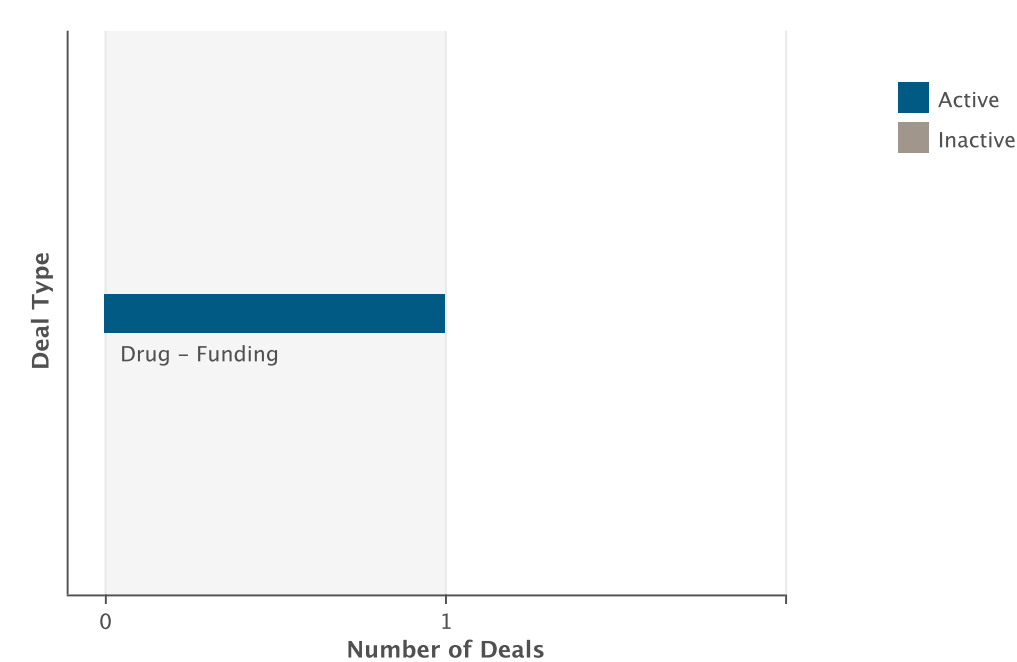


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Deals by Parent Company Table

| 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 |

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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 | Antidepressant;Analgesic;Neuroprotectant;Anticonvulsant agent |
| 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 |

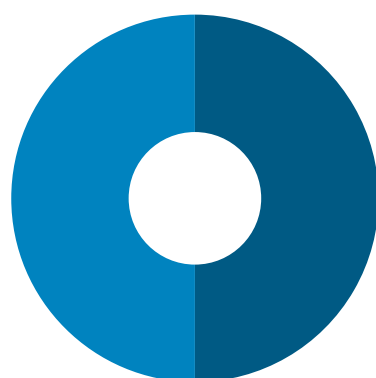
C-10068 DRUG NAMES

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

C-10068 DEALS AND PATENTS

DEALS

Deals by Parent Company Chart



Active Deals
Total: 2

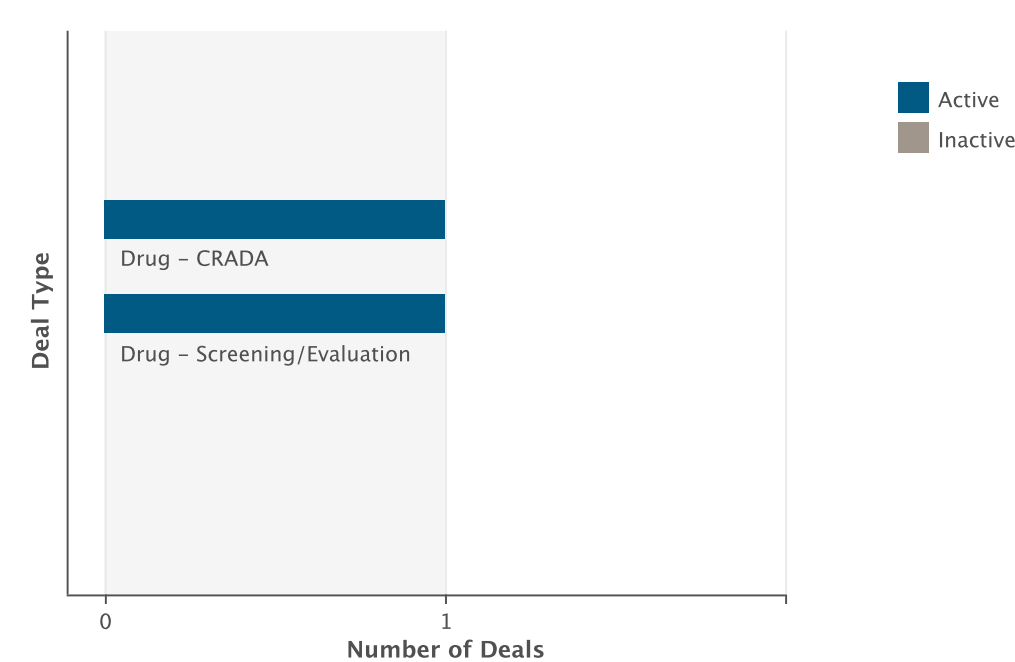


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Deals by Parent Company Table

| 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



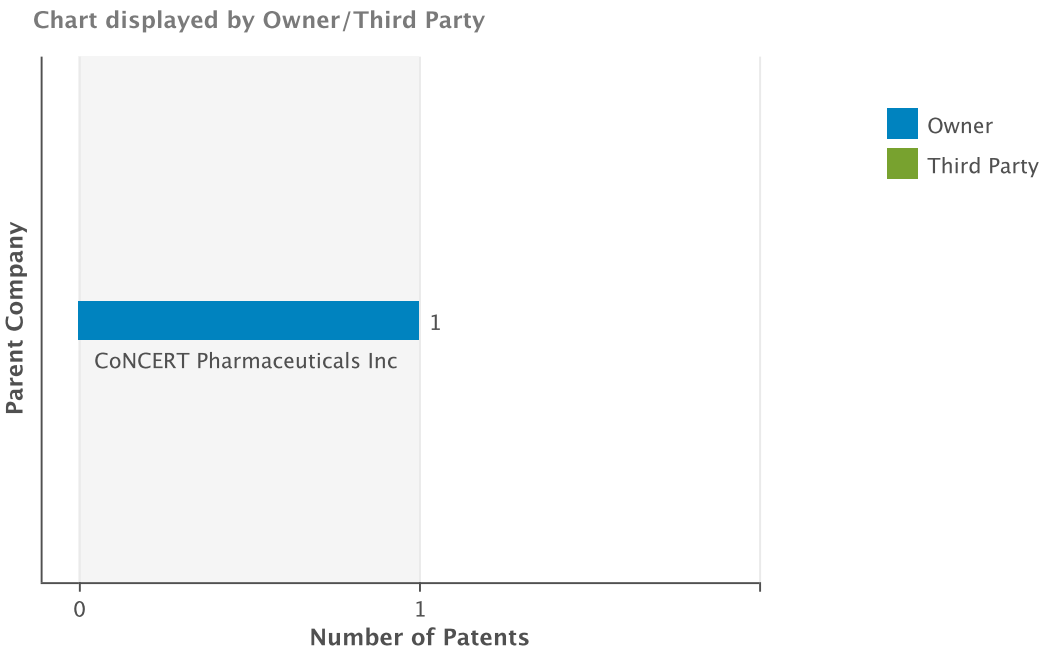
Deals by Type Table

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

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PATENTS

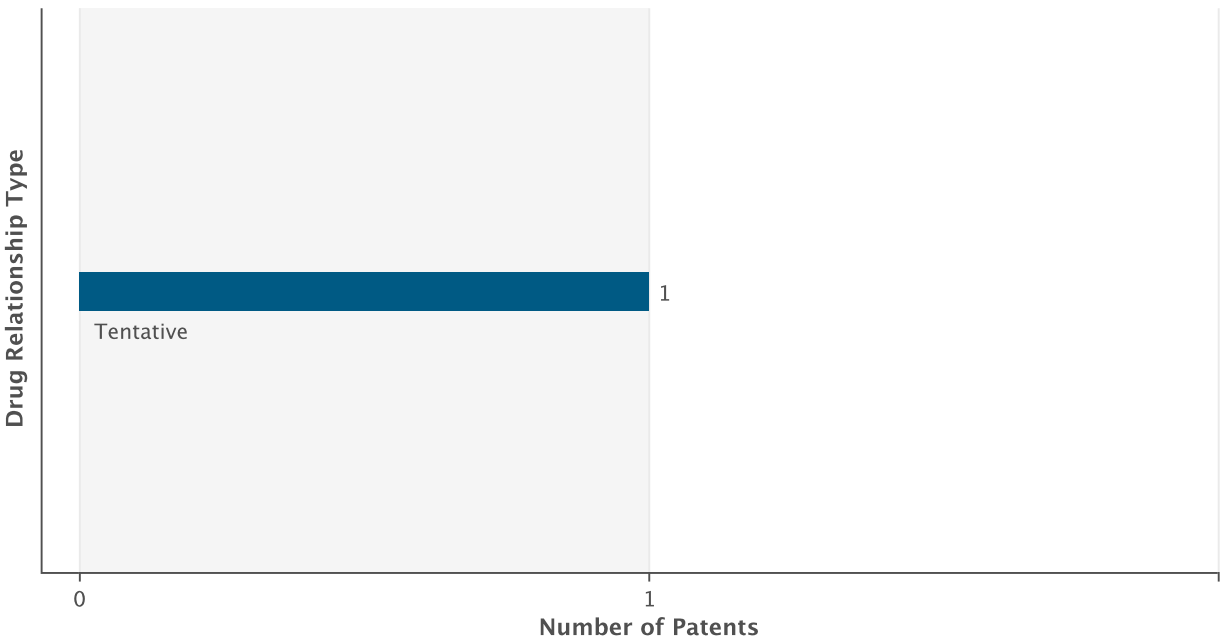
Patents by Parent Company Chart



Patents by Parent Company Table

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

Patents by Drug Relationship Type Chart



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Patents by Drug Relationship Type Table

| Drug Relationship | Total |
|-------------------|-------|
| Tentative | 1 |

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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

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

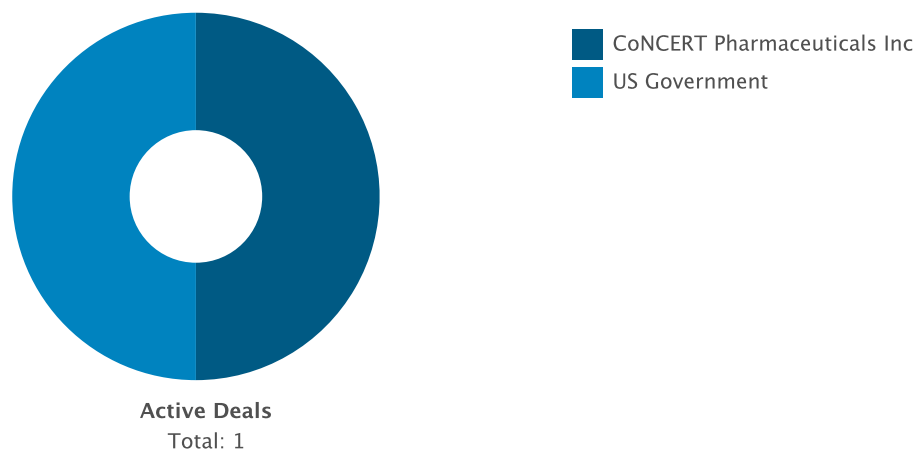
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deuterated praziquantel analogs (schistosomiasis), Therapeutics for Rare and Neglected Diseases/CoNCERT Pharmaceuticals DEALS AND PATENTS

DEALS

Deals by Parent Company Chart

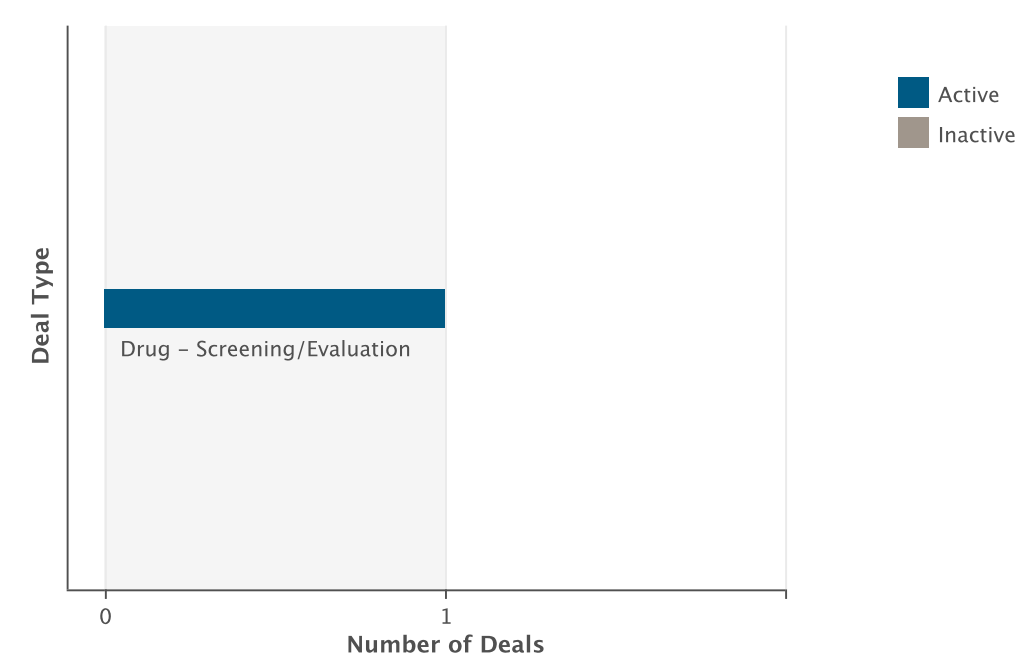


Deals by Parent Company Table

| Company Name | Principal | | Partner | | Total |
|-----------------------------|-----------|----------|---------|----------|-------|
| | Active | Inactive | Active | Inactive | |
| CoNCERT Pharmaceuticals Inc | 1 | 0 | 0 | 0 | 1 |
| 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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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: |
|--|-------------------|
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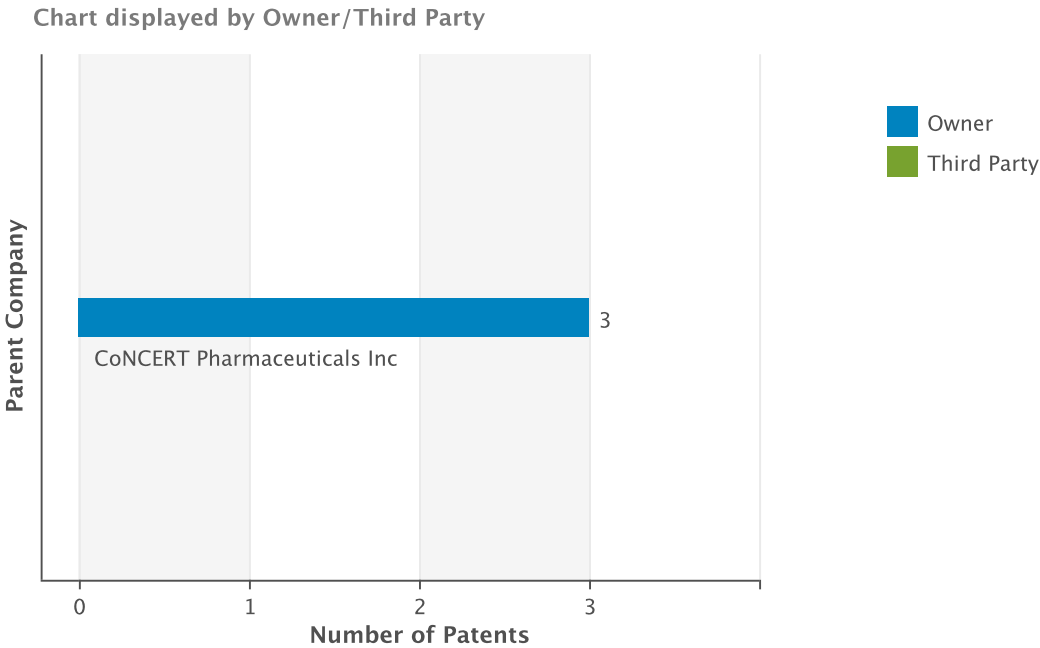
deuterated ivacaftor analogs (cystic fibrosis/COPD), Concert Pharmaceuticals DRUG NAMES

| 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

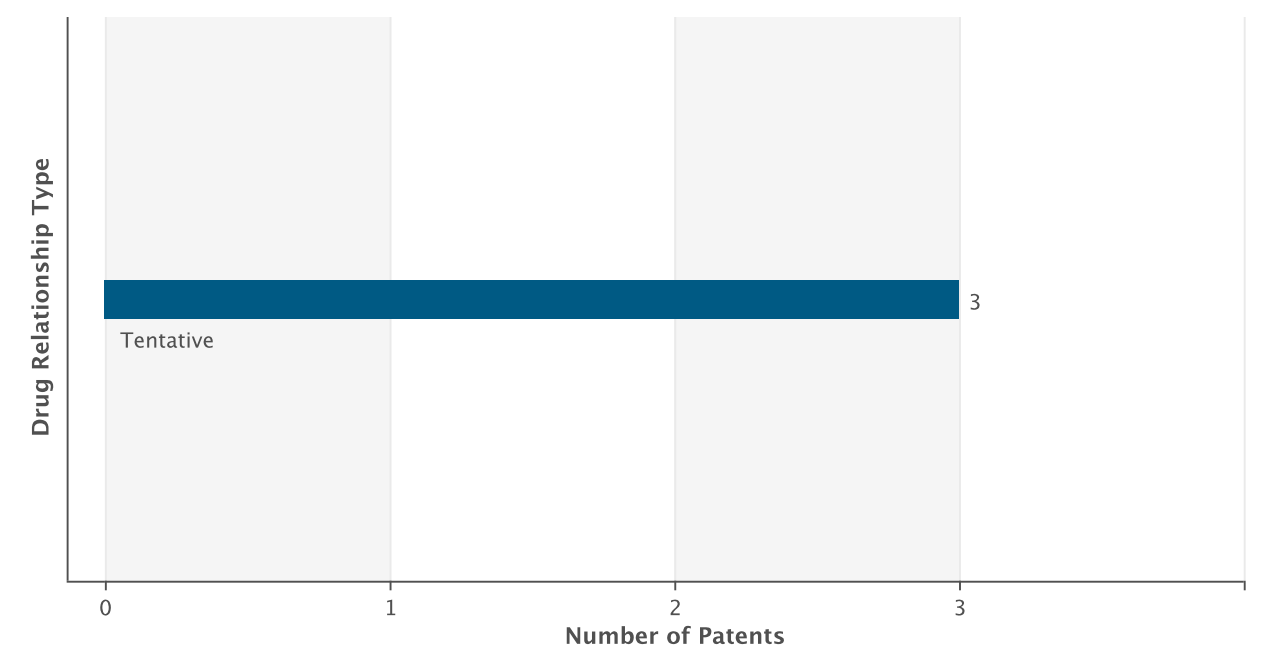


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Patents by Parent Company Table

| 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 |

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