

## **CoNCERT Pharmaceuticals Inc**

### **COMPANY AND PIPELINE OVERVIEW REPORT**

Coverage of the company and a summary of the drug pipeline portfolio.

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#### **THOMSON REUTERS**

3 Times Square  
New York, New York 10036  
United States

Tel: +1 646 223 4000

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

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## GLOSSARY

### Number of Drugs in Active Development

Number of drugs associated with the company or subsidiary that are currently in active development, i.e. the development status for the drug(s) is one of the following: Discovery, Clinical, Phase I, Phase II, Phase III, Pre-registration, Registered, Launched, or Suspended.

### Number of Inactive Drugs

Number of drugs associated with the company or subsidiary that are currently classified as inactive, i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.

### Number of Patents as Owner

Number of patents associated with the company where the company is listed as owner; i.e. the relationship type (or way the patent refers to the company) is: Patent Assignee/Owner, Patent owner (not assignee), Licensee for development and marketing, Licensee – marketing only (Distributor), Patent assignee of family member, Inferred assignee.

### Number of Patents as Third Party

Number of patents associated with the company where the company is listed as third party; i.e. the relationship type (or way the patent refers to the company) is: Patent assignee (not owner), Ex-Licensee for development and marketing, Ex-Licensee marketing only (Distributor), Customer of technology, Ex-Customer of technology, Patent opponent or infringer, Affiliate organization of inventor, Owner of underlying technology.

### Patents summary table

This table represents a summary of the core patent coverage for this company covering Therapeutic EP, US and WO patents since 1990 only.

### Number of Deals

A count of deals where the company or one of its subsidiaries is the primary company.

### Key Indications

Displays top ten key indications for the company and its subsidiaries based on frequency (indications occurring with high and identical frequency are always included, and this may result in more than ten Key Indications being listed). Includes both indications associated with patents where the company is patent owner and indications associated with drugs in active development. A drug is classified as 'active' if it features on a row (or rows) in the current development status table where the status is one of the following: Discovery, Clinical, Phase I, Phase II, Phase III, Pre-registration, Registered, Launched, or Suspended.

### Key Target-based Actions

Displays top ten key target-based actions for the company and its subsidiaries based on frequency (actions occurring with high and identical frequency are always included, and this may result in more than ten Key Target-based Actions being listed). Includes both target-based actions associated with patents where the company patent owner and target-based actions associated with drugs in active development. A drug is classified as 'active' if it features on a row (or rows) in the current development status table where the status is one of the following: Discovery, Clinical, Phase I, Phase II, Phase III, Pre-registration, Registered, Launched, or Suspended. A target-based action is one that is associated with a target.

### Key Technologies

Displays top ten key technologies for the company and its subsidiaries based on frequency (technologies occurring with high and identical frequency are always included, and this may result in more than ten Key Technologies being listed). Includes both key technologies associated with patents where the company relationship is patent owner and key technologies associated with drugs in active development. A drug is classified as 'active' if it features on a row (or rows) in the current development status table where the status is one of the following: Discovery, Clinical, Phase I, Phase II, Phase III, Pre-registration, Registered, Launched, or Suspended.

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# CoNCERT Pharmaceuticals Inc

## COMPANY OVERVIEW

|                                       |   |
|---------------------------------------|---|
| Company Name                          | CoNCERT Pharmaceuticals Inc   |
| Parent Company Name                   | CoNCERT Pharmaceuticals Inc   |
| Website                               | <a href="http://www.concertpharma.com/">http://www.concertpharma.com/</a>   |
| Country                               | US  |
| Number of Drugs in Active Development | 16  |
| Number of Inactive Drugs              | 7   |
| Number of Patents as Owner            | 130   |
| Number of Patents as Third Party      | 1   |
| Number of Deals                       | 7   |
| Key Indications                       | HIV infection, Cardiovascular disease, Neuropathic pain, Renal disease, Cancer, Anxiety disorder, Epilepsy, Pain, Major depressive disorder, Hepatitis C virus infection  |
| Key Target-based Actions              | NMDA receptor antagonist, GABA A receptor modulator, HIV protease inhibitor, CCR5 chemokine antagonist, EGFR family tyrosine kinase receptor inhibitor, HIV-1 protease inhibitor, Calcium channel inhibitor T-type, GABA A receptor alpha-5 subunit modulator, Opioid receptor sigma agonist 1, PDE 3 |
| Key Technologies                      | Small molecule therapeutic, Oral formulation, Antibiotic, Controlled release formulation, Systemic formulation unspecified, Drug combination, Formulation preservation, Prodrug, Labeling synthesis, Labeling system  |

## 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 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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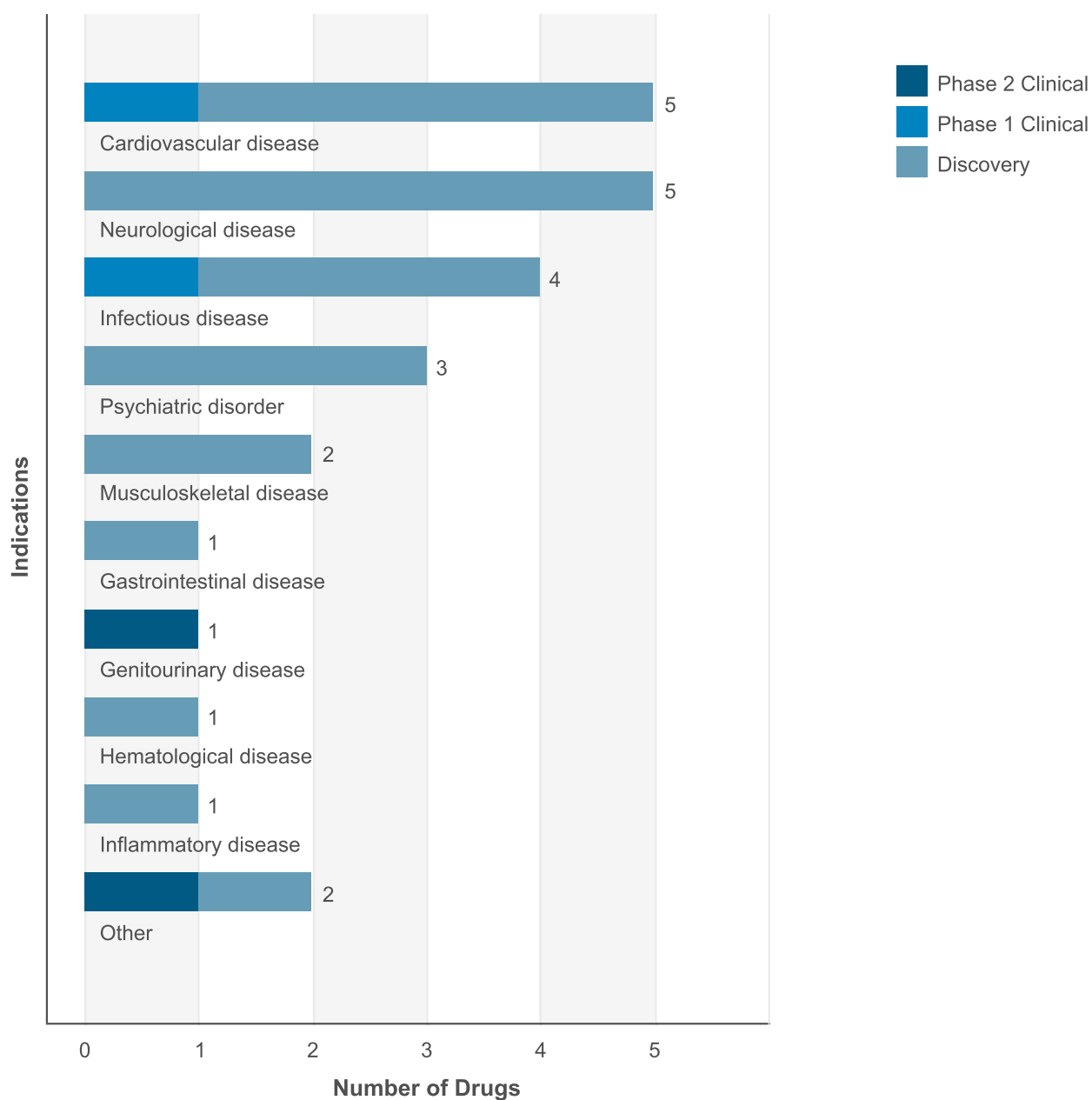


## PRODUCT PORTFOLIO SUMMARY

### DRUGS

#### Drugs by Indication

Active Drugs by Indication Chart



Drugs by Indication Table

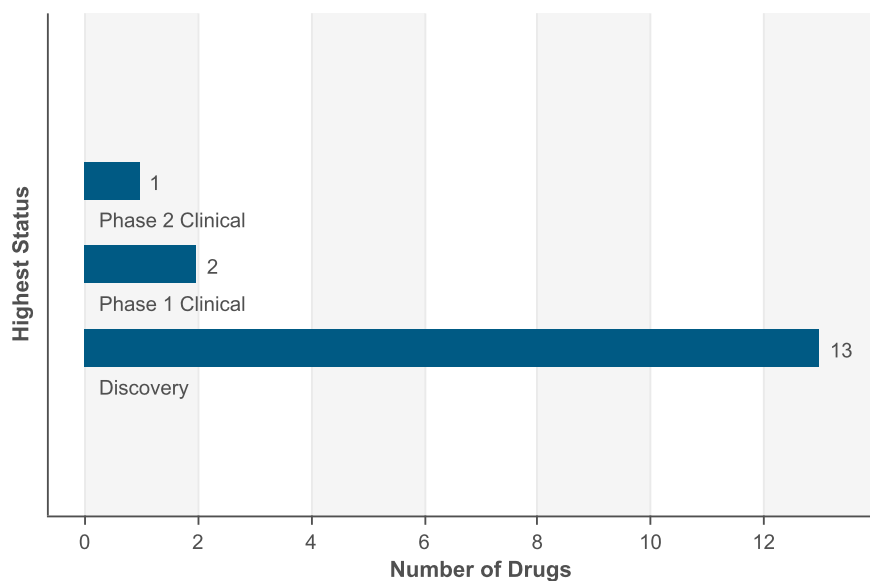
| Indication           | Active | Inactive | Total |
|----------------------|--------|----------|-------|
| Neurological disease | 5      | 3        | 8     |

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|                          |   |   |   |
|--------------------------|---|---|---|
| Infectious disease       | 4 | 2 | 6 |
| Cardiovascular disease   | 5 | 0 | 5 |
| Psychiatric disorder     | 3 | 1 | 4 |
| Inflammatory disease     | 1 | 2 | 3 |
| Musculoskeletal disease  | 2 | 1 | 3 |
| Neoplasm                 | 1 | 2 | 3 |
| Immune disorder          | 0 | 2 | 2 |
| Genitourinary disease    | 1 | 1 | 2 |
| Gastrointestinal disease | 1 | 0 | 1 |
| Injury                   | 1 | 0 | 1 |
| Respiratory disease      | 0 | 1 | 1 |
| Hematological disease    | 1 | 0 | 1 |

### Drugs by Highest Status

Active Drugs by Highest Status Chart



Drugs by Highest Status Table

| Development Status | Number of Drugs |
|--------------------|-----------------|
| Phase 2 Clinical   | 1               |
| Phase 1 Clinical   | 2               |

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|                         |    |
|-------------------------|----|
| Discovery               | 13 |
| Discontinued            | 2  |
| No Development Reported | 5  |

## 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 | 3         | 0        | 0       | 0        | 3     |

## CLINICAL TRIALS

### Trials by Condition Studied

| Condition Studied      | Ongoing | All |
|------------------------|---------|-----|
| Genitourinary disease  | 1       | 3   |
| Infectious disease     | 0       | 2   |
| Cardiovascular disease | 0       | 1   |
| Psychiatric disorder   | 0       | 1   |

### Trials by Phase

| Phase   | Ongoing | All |
|---------|---------|-----|
| Phase 3 | 0       | 1   |
| Phase 2 | 1       | 1   |
| Phase 1 | 0       | 5   |

### 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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## PATENTS \*

| Indication                     | As Owner | As Third Party | Total |
|--------------------------------|----------|----------------|-------|
| Cardiovascular disease         | 56       | 0              | 56    |
| Endocrine disease              | 37       | 0              | 37    |
| Gastrointestinal disease       | 56       | 0              | 56    |
| Genitourinary disease          | 54       | 0              | 54    |
| Growth disorder                | 10       | 0              | 10    |
| Hematological disease          | 20       | 0              | 20    |
| Degeneration                   | 13       | 0              | 13    |
| Andrology                      | 20       | 0              | 20    |
| Immune disorder                | 44       | 0              | 44    |
| Psychiatric disorder           | 30       | 0              | 30    |
| Musculoskeletal disease        | 25       | 0              | 25    |
| Neoplasm                       | 39       | 0              | 39    |
| Ocular disease                 | 15       | 0              | 15    |
| Genetic disorder               | 18       | 0              | 18    |
| Metabolic disorder             | 34       | 0              | 34    |
| Neurological disease           | 66       | 0              | 66    |
| Nutritional disorder           | 13       | 0              | 13    |
| Respiratory disease            | 26       | 0              | 26    |
| Infectious disease             | 38       | 0              | 38    |
| Injury                         | 6        | 0              | 6     |
| Toxicity and intoxication      | 7        | 0              | 7     |
| Inflammatory disease           | 43       | 0              | 43    |
| Fatigue                        | 2        | 0              | 2     |
| Otorhinolaryngological disease | 2        | 0              | 2     |
| Gynecology and obstetrics      | 21       | 0              | 21    |

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|                        |    |   |    |
|------------------------|----|---|----|
| Dermatological disease | 27 | 0 | 27 |
| 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 DRUGS

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

### CTP-499

|                             |  |
|-----------------------------|--|
| <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> |  |
| <b>Other Actions</b>        | Fibrosuppressant, Anti-inflammatory, Antioxidant agent, Renal system agent |
| <b>Technologies</b>         | Controlled release formulation, Small molecule therapeutic                 |
| <b>Last Change Date</b>     | 02-Nov-2012  |

### CTP-347

|                             |  |
|-----------------------------|--|
| <b>Drug Name</b>            | CTP-347                                      |
| <b>Key Synonyms</b>         |  |
| <b>Originator Company</b>   | CoNCERT Pharmaceuticals Inc                  |
| <b>Active Companies</b>     | CoNCERT Pharmaceuticals Inc                  |
| <b>Inactive Companies</b>   |  |
| <b>Highest Status</b>       | Phase 1 Clinical                             |
| <b>Active Indications</b>   | Hot flashes                                  |
| <b>Target-based Actions</b> |  |
| <b>Other Actions</b>        | 5-HT uptake inhibitor                        |
| <b>Technologies</b>         | Oral formulation, Small molecule therapeutic |
| <b>Last Change Date</b>     | 15-Dec-2011                                  |

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**CTP-298**

|                             |  |
|-----------------------------|--|
| <b>Drug Name</b>            | CTP-298  |
| <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 1 Clinical                                 |
| <b>Active Indications</b>   | HIV infection                                    |
| <b>Target-based Actions</b> | HIV protease inhibitor                           |
| <b>Other Actions</b>        | Antiviral  |
| <b>Technologies</b>         | Oral formulation, Small molecule therapeutic     |
| <b>Last Change Date</b>     | 07-Sep-2012                                      |

**deuterated boceprevir analogs (HCV infection), CoNCERT**

|                             |  |
|-----------------------------|--|
| <b>Drug Name</b>            | deuterated boceprevir analogs (HCV infection), CoNCERT |
| <b>Key Synonyms</b>         |  |
| <b>Originator Company</b>   | CoNCERT Pharmaceuticals Inc                            |
| <b>Active Companies</b>     | CoNCERT Pharmaceuticals Inc                            |
| <b>Inactive Companies</b>   |  |
| <b>Highest Status</b>       | Discovery  |
| <b>Active Indications</b>   | Hepatitis C virus infection                            |
| <b>Target-based Actions</b> | Hepatitis C virus protease inhibitor                   |
| <b>Other Actions</b>        |  |
| <b>Technologies</b>         | Small molecule therapeutic                             |
| <b>Last Change Date</b>     | 14-Dec-2011  |

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### prostacyclin analog (deuterated, pulmonary hypertension), CoNCERT

|                             |   |
|-----------------------------|---|
| <b>Drug Name</b>            | prostacyclin analog (deuterated, pulmonary hypertension), CoNCERT |
| <b>Key Synonyms</b>         |   |
| <b>Originator Company</b>   | CoNCERT Pharmaceuticals Inc                                       |
| <b>Active Companies</b>     | CoNCERT Pharmaceuticals Inc                                       |
| <b>Inactive Companies</b>   |   |
| <b>Highest Status</b>       | Discovery   |
| <b>Active Indications</b>   | Pulmonary artery hypertension                                     |
| <b>Target-based Actions</b> |   |
| <b>Other Actions</b>        | Antihypertensive, Prostacyclin agonist                            |
| <b>Technologies</b>         | Small molecule therapeutic  |
| <b>Last Change Date</b>     | 16-Sep-2011   |

### C-10068

|                             |   |
|-----------------------------|---|
| <b>Drug Name</b>            | C-10068   |
| <b>Key Synonyms</b>         |   |
| <b>Originator Company</b>   | CoNCERT Pharmaceuticals Inc   |
| <b>Active Companies</b>     | Walter Reed Army Institute of Research, CoNCERT Pharmaceuticals Inc |
| <b>Inactive Companies</b>   |   |
| <b>Highest Status</b>       | Discovery   |
| <b>Active Indications</b>   | Brain injury, Neuropathic pain, Epilepsy, Major depressive disorder |
| <b>Target-based Actions</b> | Opioid receptor sigma agonist 1                                     |
| <b>Other Actions</b>        | Neuroprotectant, Analgesic, Antidepressant, Anticonvulsant agent    |
| <b>Technologies</b>         | Small molecule therapeutic  |
| <b>Last Change Date</b>     | 06-Sep-2012   |

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**CTP-354**

|                             |   |
|-----------------------------|---|
| <b>Drug Name</b>            | CTP-354   |
| <b>Key Synonyms</b>         |   |
| <b>Originator Company</b>   | CoNCERT Pharmaceuticals Inc                           |
| <b>Active Companies</b>     | CoNCERT Pharmaceuticals Inc                           |
| <b>Inactive Companies</b>   |   |
| <b>Highest Status</b>       | Discovery   |
| <b>Active Indications</b>   | Muscle hypertonia, Neuropathic pain, Anxiety disorder |
| <b>Target-based Actions</b> | GABA A receptor modulator                             |
| <b>Other Actions</b>        | Anxiolytic, Analgesic                                 |
| <b>Technologies</b>         | Small molecule therapeutic                            |
| <b>Last Change Date</b>     | 06-Sep-2012   |

**deuterated darunavir analogs (HIV infection), CoNCERT**

|                             |   |
|-----------------------------|---|
| <b>Drug Name</b>            | deuterated darunavir analogs (HIV infection), CoNCERT |
| <b>Key Synonyms</b>         |   |
| <b>Originator Company</b>   | CoNCERT Pharmaceuticals Inc                           |
| <b>Active Companies</b>     | CoNCERT Pharmaceuticals Inc                           |
| <b>Inactive Companies</b>   |   |
| <b>Highest Status</b>       | Discovery   |
| <b>Active Indications</b>   | HIV infection   |
| <b>Target-based Actions</b> | HIV-1 protease inhibitor                              |
| <b>Other Actions</b>        |   |
| <b>Technologies</b>         | Small molecule therapeutic                            |
| <b>Last Change Date</b>     | 21-Sep-2011   |

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### deuterated ranolazine analogs (cardiovascular disease), CoNCERT

|                             |  |
|-----------------------------|--|
| <b>Drug Name</b>            | deuterated ranolazine analogs (cardiovascular disease), CoNCERT    |
| <b>Key Synonyms</b>         |  |
| <b>Originator Company</b>   | CoNCERT Pharmaceuticals Inc  |
| <b>Active Companies</b>     | CoNCERT Pharmaceuticals Inc  |
| <b>Inactive Companies</b>   |  |
| <b>Highest Status</b>       | Discovery  |
| <b>Active Indications</b>   | Cardiovascular disease   |
| <b>Target-based Actions</b> |  |
| <b>Other Actions</b>        | Antiarrhythmic agent, Cardioprotectant, Lipid metabolism modulator |
| <b>Technologies</b>         | Small molecule therapeutic   |
| <b>Last Change Date</b>     | 16-Sep-2011  |

### deuterated cilostazol analogs (cardiovascular disease), CoNCERT

|                             |   |
|-----------------------------|---|
| <b>Drug Name</b>            | deuterated cilostazol analogs (cardiovascular disease), CoNCERT |
| <b>Key Synonyms</b>         |   |
| <b>Originator Company</b>   | CoNCERT Pharmaceuticals Inc                                     |
| <b>Active Companies</b>     | CoNCERT Pharmaceuticals Inc                                     |
| <b>Inactive Companies</b>   |   |
| <b>Highest Status</b>       | Discovery   |
| <b>Active Indications</b>   | Cardiovascular disease  |
| <b>Target-based Actions</b> | PDE 3 inhibitor   |
| <b>Other Actions</b>        | Vasodilator, Platelet aggregation inhibitor, Cardioprotectant   |
| <b>Technologies</b>         |   |
| <b>Last Change Date</b>     | 16-Sep-2011   |

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### deuterated praziquantel analogs (schistosomiasis), CoNCERT Pharmaceuticals

|                             |  |
|-----------------------------|--|
| <b>Drug Name</b>            | deuterated praziquantel analogs (schistosomiasis), 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>     | 15-Feb-2013  |

### deuterated GABAA receptor inverse agonists (cognition enhancement), CoNCERT

|                             |   |
|-----------------------------|---|
| <b>Drug Name</b>            | deuterated GABAA receptor inverse agonists (cognition enhancement), CoNCERT |
| <b>Key Synonyms</b>         |   |
| <b>Originator Company</b>   | CoNCERT Pharmaceuticals Inc   |
| <b>Active Companies</b>     | CoNCERT Pharmaceuticals Inc   |
| <b>Inactive Companies</b>   |   |
| <b>Highest Status</b>       | Discovery   |
| <b>Active Indications</b>   | Cognitive disorder  |
| <b>Target-based Actions</b> | GABA A receptor alpha-5 subunit modulator                                   |
| <b>Other Actions</b>        | Nootropic agent, GABA A receptor inverse agonist                            |
| <b>Technologies</b>         | Small molecule therapeutic  |
| <b>Last Change Date</b>     | 25-Nov-2011   |

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**C-10291**

|                             |                             |
|-----------------------------|-----------------------------|
| <b>Drug Name</b>            | C-10291                     |
| <b>Key Synonyms</b>         |                             |
| <b>Originator Company</b>   | CoNCERT Pharmaceuticals Inc |
| <b>Active Companies</b>     | CoNCERT Pharmaceuticals Inc |
| <b>Inactive Companies</b>   |                             |
| <b>Highest Status</b>       | Discovery                   |
| <b>Active Indications</b>   | Fibromyalgia, Narcolepsy    |
| <b>Target-based Actions</b> |                             |
| <b>Other Actions</b>        | Neuroprotectant             |
| <b>Technologies</b>         | Small molecule therapeutic  |
| <b>Last Change Date</b>     | 20-Dec-2011                 |

**CTP-221**

|                             |  |
|-----------------------------|--|
| <b>Drug Name</b>            | CTP-221  |
| <b>Key Synonyms</b>         |  |
| <b>Originator Company</b>   | CoNCERT Pharmaceuticals Inc                          |
| <b>Active Companies</b>     | CoNCERT Pharmaceuticals Inc                          |
| <b>Inactive Companies</b>   |  |
| <b>Highest Status</b>       | Discovery  |
| <b>Active Indications</b>   | Hematological neoplasm, Myelodysplastic syndrome     |
| <b>Target-based Actions</b> |  |
| <b>Other Actions</b>        | Unspecified drug target, Immunomodulator, Anticancer |
| <b>Technologies</b>         | Small molecule therapeutic                           |
| <b>Last Change Date</b>     | 21-Dec-2012  |

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**CTP-786**

|                             |   |
|-----------------------------|---|
| <b>Drug Name</b>            | CTP-786   |
| <b>Key Synonyms</b>         |   |
| <b>Originator Company</b>   | CoNCERT Pharmaceuticals Inc                             |
| <b>Active Companies</b>     | CoNCERT Pharmaceuticals Inc, AVANIR Pharmaceuticals Inc |
| <b>Inactive Companies</b>   |   |
| <b>Highest Status</b>       | Discovery   |
| <b>Active Indications</b>   | Psychiatric disorder, Pain                              |
| <b>Target-based Actions</b> |   |
| <b>Other Actions</b>        | Unspecified drug target, Analgesic                      |
| <b>Technologies</b>         | Small molecule therapeutic                              |
| <b>Last Change Date</b>     | 16-Aug-2012   |

**deuterated mibefradil analogs (cardiovascular disease), CoNCERT**

|                             |   |
|-----------------------------|---|
| <b>Drug Name</b>            | deuterated mibefradil analogs (cardiovascular disease), CoNCERT |
| <b>Key Synonyms</b>         |   |
| <b>Originator Company</b>   | CoNCERT Pharmaceuticals Inc                                     |
| <b>Active Companies</b>     | CoNCERT Pharmaceuticals Inc                                     |
| <b>Inactive Companies</b>   |   |
| <b>Highest Status</b>       | Discovery   |
| <b>Active Indications</b>   | Cardiovascular disease  |
| <b>Target-based Actions</b> | Calcium channel inhibitor T-type                                |
| <b>Other Actions</b>        | Antihypertensive, Class IV antiarrhythmic agent                 |
| <b>Technologies</b>         | Small molecule therapeutic                                      |
| <b>Last Change Date</b>     | 26-Oct-2012   |

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