

## CONCERT PHARMACEUTICALS INC.

### AVP-923's Data in Alzheimer's Agitation Looks Solid; 354 Data Also Supportive

CNCE (NASDAQ)

#### Company & Market Data

|                                   |                  |
|-----------------------------------|------------------|
| Closing Price (as of 10/13/2014): | \$11.96          |
| Rating:                           | BUY              |
| Price Target:                     | \$26.00          |
| 52 Week Range:                    | \$7.12 - \$16.26 |
| Shares Outstanding (MM):          | 18               |
| Market Capitalization (MM):       | \$215            |
| Cash (MM):                        | \$108.0          |
| Debt (MM):                        | \$13.0           |
| Fiscal Year End:                  | Dec              |

#### Estimates

| EPS          | 2013A    | 2014E     | 2015E    |
|--------------|----------|-----------|----------|
| 1Q           | —        | \$(0.76)A | —        |
| 2Q           | —        | \$(0.45)A | —        |
| 3Q           | —        | \$(0.40)  | —        |
| 4Q           | —        | \$(0.32)  | —        |
| Full Year    | \$(4.99) | \$(1.78)  | \$(0.67) |
| Revenue (MM) | \$25.4   | \$9.6     | \$27.5   |

#### Ratios

|     |    |    |    |
|-----|----|----|----|
| P/E | NA | NA | NA |
|-----|----|----|----|

Concert Pharmaceuticals, founded in 2006 by Richard Aldrich, Roger Tung, and Christoph Westphal, creates novel medicines by applying its DCE (Deuterated Chemical Entity) Platform technologies to molecules. The DCE approach involves the selective addition of deuterium, a stable isotope of hydrogen, to molecules, resulting in clinical therapeutic candidates with improved pharmacology. This approach has the potential to enable more efficient drug discovery and clinical development. Concert's proprietary programs include CTP-354, a novel GABA-A modulator for spasticity and anxiety, and CTP-499, a PDE inhibitor for diabetic kidney disease. Promising partnered programs include Jazz's JZP-386, a long-acting Xyrem, Avanir's AVP-786, an improved Neudexta, and multiple programs with Celgene. Concert has also developed a broad portfolio of deuterated molecules that are expected to migrate into and through the clinic over time.

**Data Presented At ANA Supports AVP-923 in Alzheimer's Agitation.** Avanir recently announced favorable top-line data for AVP-923 (Neudexta) in a 220-patient Phase 2 study in agitation associated with Alzheimer's disease. Yesterday at the American Neurological Association (ANA) meeting in Baltimore, Avanir presented more of the data; we were in attendance at the meeting. As previously noted, efficacy for 923 at the primary endpoint appeared impressive, with the details today showing a reduction in the agitation/aggression domain of the NPI scale of -3.3 for 923 vs. -1.7 for placebo in stage 1 of the study, and -2.0 for 923 vs. -0.8 for placebo in stage 2 (P=0.00008).

Adverse events in the study  $\geq 3\%$ , were falls (8.6% for 923 vs. 3.9% for placebo), diarrhea (5.9% vs. 3.1%) and urinary tract infection (5.3% vs. 3.9%). Though the rate of falls appears a question, there was an imbalance at baseline, with 20.4% of 923 patients having a prior history of falls, compared to 12.8% for placebo, perhaps providing an explanation for the results. GI AEs should also be reduced as the quinidine is reduced when AVP-786 is substituted in Phase 3.

As noted before, the results are important for AVP-786 and Concert, because AVP-786 is expected to replace AVP-923/Neudexta in Alzheimer's agitation and all future indications. AVP-786 is a deuterated dextromethorphan that contains less quinidine than Neudexta, allowing broader consideration. Avanir also recently announced 786 is moving into P2 for depression. Concert is to receive \$164mm in additional milestones and royalties from mid-single digit to low double digits on sales of AVP-786.

**CTP-354 Data At ANA Also Supportive.** CTP-354, is a subtype-selective GABA-A modulator, with familiar yet distinctive GABA agonist activity, and a profile of minimal sedation compared to benzodiazepines (BZDs). Data in a multiple ascending dose trial presented at ANA showed evidence that CTP-354 can achieve high plasma levels without causing sedation and ataxia, which limits broader adoption of BZDs. Also, 354's pharmacokinetics support once-daily dosing. We believe '354 has the potential for utility not only in spasticity associated with both multiple sclerosis (MS) and spinal cord injury, but we believe there is significant potential for its development in anxiety and pain settings over time. '354 is moving into Phase 2 for spasticity in spinal cord injury in 2H14, and into other indications during 2015.

**Celgene/Concert's CTP-730: An Improved Otezla?** CPT-730 could be a potential once-daily version of Otezla (apremilast), Celgene's PDE4 inhibitor. Concert's patents cover a deuterated version of that molecule. The doses being examined in Phase 1 for CTP-730 are in the ballpark for Otezla, which is approved in the US for psoriatic arthritis, and is also in development for psoriasis, ankylosing spondylitis, rheumatoid arthritis, inflammatory bowel disease, and atopic dermatitis. With a potential of a multi-billion dollar franchise if all these indications are achieved, if it is Otezla, our estimates for this collaboration could prove conservative. The Celgene agreement is potentially very lucrative, with \$35mm upfront, \$1.4B in milestones, and royalties ranging from the mid-single digits to low double digits (we estimate 13% at the top end), on worldwide sales of the products.

**CNCE Valuation: Attractive In Our View.** We value CNCE shares at \$26, based on fully-taxed, risk-weighted NPV calculations that total \$25.77 (see below). During the next 12 months, we expect continued progress with its partners, and advancements with its proprietary efforts CTP-499 for diabetic kidney disease, and CTP-354 for spasticity and anxiety.

Disclosures and Analyst Certifications can be found in Appendix A.

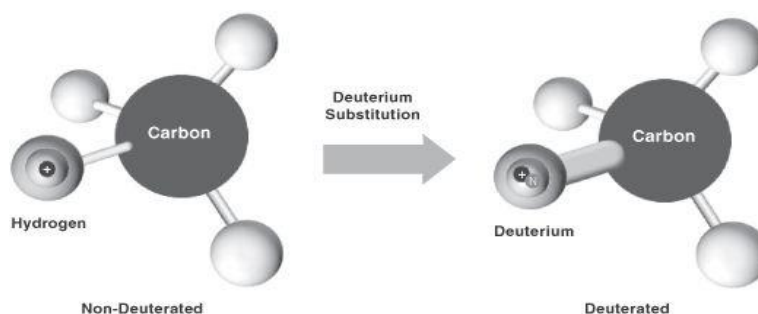
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## Concert Pharmaceuticals – Executive Summary

**Concert Pharmaceuticals: Developing Deuterium-Based Drugs.** Founded in 2006, Concert Pharmaceuticals focuses on creating and developing new medicines through its proprietary DCE (deuterated chemical entity) Platform. The DCE Platform selectively employs deuterium, a naturally-occurring relative of hydrogen. Deuterium modification of a molecule has the potential to improve its metabolic properties, with minimal change to its intrinsic pharmacology. Concert's business strategy initially involved establishing novel intellectual property for a number of deuterium-modified molecules across a wide range of therapeutic classes, and then advancing the most promising initial candidates, even as it continued to develop others. Five of these more mature deuterated assets are now currently in active development under the guidance of either Concert or its strategic partners, and all five should be advancing in the clinic by year-end 2014.

### Exhibit 1: Deuterium Substitution



Source: Concert Corporate Fact Sheet, May 2013.

The application of deuterium medicinal chemistry to compounds with well understood therapeutic utility can potentially provide an approach with modestly reduced risk with regard to creating new drugs. Concert believes (and we concur) that since its assets are generally materially related to a parent molecule, once Phase 1 is established for the deuterated molecule, especially in an indication that the parent shares, the program has been materially de-risked.

**Deuterium Medicinal Chemistry In Brief.** Deuterium is one of two naturally-occurring stable isotopes of hydrogen. Where hydrogen has one electron and one proton, deuterium also has a neutron in its nucleus, resulting in an atomic mass that is double that of hydrogen. Deuterium is not radioactive, and possesses physicochemical properties that are similar to those of hydrogen, but because of its increased mass, bonds involving deuterium are generally stronger than similar bonds with hydrogen. This strengthening can be enough to make significant changes in biological reactions with deuterium-based compounds compared to hydrogen-based ones. Many drugs are metabolized by pathways that involve the breakdown of carbon-hydrogen bonds, and the stronger deuterium-based bonds have the potential to alter or deflect the breakdown of the molecule or its metabolites. Deuterium modification therefore offers an approach to potentially creating significantly differentiated new medicines, and importantly, because the behavior of deuterium based bonds is not inherently predictable, novel intellectual property can be established.

**Concert's Drug Development Approach.** Concert's strategic development of deuterium-based molecules began with management initially broadly examining a host of both approved and novel molecules to selectively apply deuterium and establish intellectual property. Having now established a broad portfolio of deuterated molecules that span multiple therapeutic areas, it continues with that process. We note that

Concert's management team is well-seasoned in therapeutics development, having material experience at Vertex, Merck, Amgen, and other organizations; because of this, we believe Concert should have the ability to successfully navigate the development of its wide range of assets. With existing therapies, Concert is often utilizing significant information regarding the related non-deuterated (parent) compound, allowing it to efficiently identify lead compounds. In some cases, Concert or its partners can truncate the development timeline of a deuterated molecule compared to conventional drug development by sourcing the data from the related parent molecule.

**Concert's Pipeline: Five In The Clinic During 2015.** Concert has a solid pipeline of deuterated compounds as noted in Exhibit 2. Concert's five most advanced programs are all expected to be materially progressing at various stages in the clinic by the end of 2014. Concert has two proprietary compounds and three partnered programs of significant importance. Given the urgency with which Concert has established its intellectual property, we expect continued advancement of new compounds in its proprietary pipeline into the clinic.

*Concert's Proprietary Pipeline: CTP-354 Has A Familiar Yet Distinctive GABA Profile.* CTP-354 is Concert's GABA-A selective modulator moving into Phase 2 for spasticity associated with both multiple sclerosis and spinal cord injury. It also could be considered for other much broader indications such as anxiety and neuropathic pain. CTP-354 is attempting to demonstrate efficacy in these settings without the sedation seen with other common GABA-focused therapies, and the larger opportunities such as anxiety and pain with limited sedation gives this compound considerable potential. Phase 2 should begin in 2H14 for the spasticity settings.

*Concert's Proprietary Pipeline: CTP-499--Through Phase 2 in Diabetic Kidney Disease.* CTP-499 is a PDE-inhibitor with anti-inflammatory and anti-fibrotic characteristics that has shown encouraging results in Phase 2 for diabetic kidney disease, and recent changes in trial endpoints have become helpful to the development of therapeutics for this indication. Also, since its parent molecule is pentoxifylline, CTP-499 could have potential for long-term consideration in hepatic settings such as alcoholic liver disease and NASH. '499 could be partnered after the recent positive end-of-Phase 2 meeting with FDA, and the upcoming issuance of a potential SPA for the program.

**Exhibit 2: Concert Pharmaceuticals - Pipeline**

| Product Candidate                        | Lead Indication(s)                   | Preclinical | Phase 1 | Phase 2 | Anticipated Milestones   | Potential Deal Value | Worldwide Rights             |
|--|--------------------------------------|-------------|---------|---------|--|----------------------|------------------------------|
| CTP-354                                  | Spasticity associated with MS        |             |         |         | <ul style="list-style-type: none"> <li>Ph1 MAD data expected 2H14</li> <li>Ph2 program expected to begin 2H14</li> </ul>             |                      | CoNCERT Pharmaceuticals Inc. |
|  | Spasticity associated with SCI       |             |         |         |  |                      |                              |
| CTP-499                                  | Diabetic Kidney Disease              |             |         |         | <ul style="list-style-type: none"> <li>End of Ph2 FDA meeting expected mid-2014</li> </ul>   |                      | CoNCERT Pharmaceuticals Inc. |
| AVP-786<br>(Deuterated dextromethorphan) | Neurologic and Psychiatric Disorders |             |         |         | <ul style="list-style-type: none"> <li>Ph2 trial for treatment-resistant major depressive disorder expected to begin 2H14</li> </ul> | \$170 Million        | AVANIR pharmaceuticals       |
| CTP-730                                  | Inflammatory Diseases                |             |         |         | <ul style="list-style-type: none"> <li>Phase 1 expected to begin 2014</li> </ul>   | \$1.4 Billion        | Celgene                      |
| JZP-386<br>(Deuterated sodium oxybate)   | Narcolepsy                           |             |         |         | <ul style="list-style-type: none"> <li>Phase 1 expected to begin 2014</li> </ul>   | \$117 Million        | Jazz Pharmaceuticals         |
| C-10068                                  | Pain and Seizures                    |             |         |         |  |                      | CoNCERT Pharmaceuticals Inc. |
| Deuterated Ivacaftor                     | CF and COPD                          |             |         |         |  |                      | CoNCERT Pharmaceuticals Inc. |

Source: Concert Pharmaceuticals corporate presentation; June 2014.

**Partnered Programs With Avanir, Jazz, and Celgene, Are Advancing.** Concert initially implemented a partnering strategy for the development of its molecules in order to defer risk, and help establish and validate its DCE platform. It has three corporate agreements so far, and we believe there is the potential for additional collaborations over time. Concert has structured its partnerships with material developmental milestones, providing the company with potential for material cash and revenue generation from those programs even prior to the introduction of its own proprietary therapies.

*AVP-786 with Avanir for Neurologic/Psychiatric Disorders.* Avanir's (AVNR, \$5.51; Not Rated) AVP-786's is a deuterated version of Avanir's approved Neudexta, indicated for pseudobulbar affect (sudden, frequent episodes of laughing and/or crying). AVP-786 contains much less quinine than Neudexta, which could make its appeal much broader. '786 is being developed in treatment-resistant depression and neuropathic pain, and is now entering Phase 2 for major depressive disorder. Indications such as agitation in Alzheimer's disease and Parkinson's dyskinesia are also likely. Concert can earn up to \$166 million in additional regulatory, development, and sales milestones, and a royalty in the mid-single digits to low double digits on global sales.

*Jazz's JZP-386 – A Better Xyrem?* Jazz's (JAZZ, \$132.89; Not Rated) JZP-386 is a deuterated analog of sodium oxybate that is entering Phase 1 development for narcolepsy; sodium oxybate is the active ingredient in Jazz's large and rapidly growing Xyrem franchise; Xyrem posted revenue of \$569 million in 2013, up +50% from the previous year. JZP-386 has the potential to materially expand the franchise by avoiding the cumbersome middle-of-the-night dosing. Concert can earn up to \$117 million in regulatory, development, and sales milestones, and a royalty in the mid-single digits to low double digits on worldwide sales.

*Celgene's Inflammation/Oncology Programs.* Celgene's (CELG, \$85.90; Not Rated) deuterated CTP-730 is in development for inflammatory diseases, and is expected to enter the clinic this year, with Phase 1 results expected in 2015. Though the specific target and parent molecule of CTP-730 has not been disclosed, milestones of up to \$1.4 billion point to the deal's significance (the deal also includes three other potential assets). In terms of inflammatory indications and franchises of importance to Celgene, we note that Thalomid has one inflammatory indication, and Celgene's pipeline contains Otezla/apremilast, pomalidomide, CC-220, CC-292, and others that are being examined for inflammatory indications. We expect greater visibility for this collaboration in 2015.

### Exhibit 3: Concert – Recent Events/Upcoming Catalysts

| Date   | Event  | Comment   | Significance |
|--------|--|---|--------------|
| Feb-12 | Avanir deal for deuterated dextromethorphan          | Program gives solid PK profile with less quinidine      | ◆◆◆          |
| Feb-13 | Jazz deal for deuterated Xyrem (sodium oxybate)      | Extension of significant Xyrem franchise                | ◆◆◆◆         |
| Apr-13 | Celgene collaboration - CTP-730, others              | Large deal: \$35mm UF, \$1.4B MS, SD/low DD royalty     | ◆◆◆◆◆        |
| Feb-14 | Concert IPO  | Capital to advance internal programs more rapidly       | ◆◆◆◆◆        |
| Apr-14 | P2 data for CTP-499 in diabetic kidney disease       | Showed signal, leading to end of P2 FDA meeting         | ◆◆◆◆         |
| 2H14   | Avanir AVP-786 into Phase 2 in treatment res't dep'n | Phase 2 start triggers \$2mm milestone to Concert       | ◆◆◆          |
| 2H14   | Jazz JZP-386 moves into P1 in narcolepsy             | Completion of P1 in 2015 to trigger milestone           | ◆◆◆          |
| 2H14   | CTP-354 completes MAD study                          | Important for '854 in a range of indications            | ◆◆◆          |
| 2H14   | CTP-354 FDA discussion about higher doses            | Could lead to '854 in pain, other indications           | ◆◆◆◆         |
| 2H14   | CTP-354 into Phase 2 in spasticity (spinal)          | Data for this indication, should help establish PoC     | ◆◆◆          |
| 2H14   | CTP-499 end P2 Meeting w/FDA                         | Should clarify Phase 3, lead to SPA, pot. partner       | ◆◆◆◆         |
| 2H14   | Celgene's CTP-730 to start Phase 1 study             | Program progress good visibility for Concert            | ◆◆◆◆◆        |
| 2015   | CTP-354 moves into Phase 2 in spasticity (MS)        | Data for this indication, should help establish PoC     | ◆◆◆          |
| 2015   | Avanir to move '786 into P3 in Alzheimer's agitation | Phase 3 start triggers \$2mm milestone to Concert       | ◆◆◆◆         |
| 2015   | Additional Concert proprietary assets progress       | Concert's DCE platform should broaden during 2015       | ◆◆◆◆         |
| 2015   | Jazz JZP-386 completes P1 in narcolepsy              | Completion of P1 triggers \$4 milestone                 | ◆◆◆          |
| 2015   | Celgene's CTP-730 Phase 1 data                       | Results trigger \$8mm milestone, visibility for Concert | ◆◆◆◆         |
| 2016   | CTP-354 Phase 2 data in spasticity (MS, spinal)      | Data in the indications could be a major catalyst       | ◆◆◆◆         |

(Significance: ◆ least important., ◆◆◆◆◆ most important.)

Source: Ladenburg Thalmann BioPharmaceuticals Research.

## Concert Pharmaceuticals – Potential Catalyst for 2014, 2015

### *Concert's Proprietary Programs*

- **CTP-354** completed additional preclinical toxicology work, and FDA has recently allowed repeated dosing with the molecule above 6mg. We expect clearance from the agency during mid-2014, providing some room for this drug to be dosed higher, if necessary in the pain indications. '354's top-line receptor occupancy data, important for licensing, should be released mid-year.
- **CTP-354** should also move into phase 2 during 2H14 for spasticity associated with spinal cord injury, and in early 2015 for spasticity associated with multiple sclerosis. Data from these Phase 2 studies should read out during 2016. Other larger opportunities such as anxiety and pain also have the potential to begin during 2015.
- **CTP-499** has shown encouraging results in Phase 2 for diabetic kidney disease, and recent changes in trial endpoints have become helpful to the drug development for this indication. Clarity on Phase 3 trial design, an SPA, and a potential partnership could happen in that order over the next 12 months.

### *Concert's Partnered Programs*

- **Avanir's AVP-786**, a deuterated Neudexta, just entered Phase 2 for treatment resistant major depressive disorders, triggering a \$2 million milestone upon dosing initiation. AVP-923 (Neudexta) just presented solid Phase 2 study in patients who have material agitation associated with Alzheimer's disease. Avanir has stated repeatedly that it intends to move forward with AVP-786 for the indication, creating the potential for '786 to move into Phase 3 during 2015, with a favorable readout for the Phase 2 study. Movement into additional indications for 786 is possible/likely in 2014/15.
- **Jazz's JZP-386**, a deuterated analog of Xyrem is now moving into Phase 1 for narcolepsy; when JZP-386 completes this P1 study, a \$4 million milestone payment is due to Concert. Advancement of this next-gen version of Xyrem, a \$500+mm program, should be an ongoing catalyst for CNCE shares.
- **Celgene's deuterated CTP-730** is in development for inflammatory diseases, and is expected to enter the clinic this year, with Phase 1 completion expected in 2015, triggering an \$8mm milestone. Advancement of this program has the potential to be a material catalyst for CNCE shares, despite not knowing material program specifics at this point.

## Concert Pharmaceuticals - Valuation

**Concert and CNCE Shares Valuation.** Because Concert's operations have the potential for significant revenue and earnings variability over the coming quarters and years, we value the company and its assets using a fully-taxed, risk-weighted net present value methodology for each of its assets. We note that with its multiple partnerships and advancing clinical programs, Concert has a diverse portfolio of therapeutic assets, and we assess the total value of these programs at \$25.77 per CNCE share, which underpins our \$26 CNCE price target (see Exhibit 4 below).

**Valuing Concert's Proprietary Pipeline.** Concert's two most advanced proprietary assets, CTP-354 for spasticity, and CTP-499 for diabetic kidney disease, have material



valuation potential, in our view. CTP-354 for spasticity as a result of multiple sclerosis and/or spinal cord injury and possibly other indications such as anxiety and neuropathic pain, is moving into Phase 2. We believe that a GABA-A modulator with familiar GABA mechanism, yet a distinct profile, with once-daily dosing and less sedation, should have material potential for consideration in spasticity, anxiety, and even pain settings, and have the potential to exceed \$1 billion in peak revenue. We value its collective opportunities at \$7.82 per share. CTP-499, for diabetic kidney disease appears to have achieved material validation after a more full analysis of its Phase 2 trial data, which bodes well for Phase 3 consideration. We assume licensing is to occur after discussions with FDA now that Phase 2 is winding up. Despite risks to the Phase 3 endpoints that remain, we believe more reasonable guidance is emerging along those lines from FDA, and estimate CTP-499 at \$1 billion peak revenue, and value the program at \$5.70 per CNCE share. Other less mature proprietary products are much more modest opportunities at this point and valued at \$0.35 per CNCE share, though proof of concept comes at Phase 1 for many of these deuterated programs, and this part of the portfolio could appreciate quite quickly.

**Exhibit 4: Concert – CNCE Share NPV Summary** (\$000, except per share amts)

| Concert Pharmaceuticals - Assets             | NPValue           | NPV/Share      |
|--|-------------------|----------------|
| <b>CTP-354 - Spasticity, Anxiety, others</b> | \$ 140,798        | \$7.82         |
| <b>CTP-499 - Diabetic Kidney Disease</b>     | \$ 102,577        | \$5.70         |
| <b>Other Proprietary Dueterated Assets</b>   | \$ 6,369          | \$0.35         |
| <b>AVP-786 - CNS indications (Avanir)</b>    | \$ 81,601         | \$4.53         |
| <b>JZP-386 - Sleep indications (Jazz)</b>    | \$ 45,383         | \$2.52         |
| <b>CTP-730 - Inflammation (Celgene)</b>      | \$ 59,885         | \$3.33         |
| <b>Other Celgene - Onc/Inflam (Celgene)</b>  | \$ 12,183         | \$0.68         |
| <b>Other Corporate</b>                       | \$ (94,465)       | (\$5.25)       |
| <b>Net Cash</b>                              | \$ 98,329         | \$5.46         |
| <b>NOLs, Credits, etc.</b>                   | \$ 11,172         | \$0.62         |
| <b>Concert - Company Valuation</b>           | <b>\$ 463,832</b> | <b>\$25.77</b> |

Source: Ladenburg Thalmann BioPharmaceuticals Research.

**Valuing Concert's Collaborations.** Regarding Concert's partnered portfolio, Avanir seems to be operating with a good urgency with AVP-786, and is exploring dextromethorphan's broad neurotransmitter receptor activity with the molecule, looking at the therapy in multiple additional indications in addition to depression, including agitation and dyskinesia. Because of these multiple indications, and its potential for its rapid advancement, the economics of this program are valued at \$4.53 per CNCE share. The Jazz program JZP-386 attempts to provide the Xyrem franchise with a product that doesn't have middle of the night dosing, and can help protect against potential loss of exclusivity. Xyrem is growing rapidly (+50% yr/yr) and is large at \$569 million in 2013, and making a more convenient dosing form with longer patent life appears to be a material opportunity, generating a \$2.52 NPV per CNCE share. Finally Celgene has not disclosed the mechanism of CTP-730 inflammation collaboration, though the very large regulatory milestones and peak revenue along the lines of Xyrem, give this collaboration a \$3.33 NPV, by our calculation. The remainder of the Celgene assets are estimated at \$0.68, and net cash, NOLs and the drag of general corporate expense totals \$0.68 per CNCE share, yielding the \$25.77 total, driving our \$26 CNCE shares price target.

## Concert Pharmaceuticals - Risks

The following Risks include, but are not limited to:

**Regulatory/FDA.** As with any company whose main business focuses on the development of pharmaceuticals, Concert is subject to the strenuous regulatory requirements of the US Food and Drug Administration (FDA) and other international regulatory agencies such as the EMEA to have its new drugs approved. Promotion of its approved drug products is also highly regulated by FDA and related agencies throughout the globe. Also, in general, though the company's specific focus on ethical (prescription) pharmaceuticals places significant risk on its operations due to the scrutiny of FDA and other governmental regulatory bodies, we believe this specific risk over time should be no greater than that for any other research-based drug development company.

**Material Dependence Upon CTP-499, CTP-354 Progress.** CTP-354 and CTP-499 are two of Concert's most advanced proprietary clinical candidates in development. These novel molecules may take material time and resources to finish clinical development, if they are able to complete at all, and there is certainly no guarantee that the company will be successful in doing so. In addition, Concert may seek one or more collaborators for future development of CTP-499. There is a risk that the company may not be able to enter into a collaboration for the therapy, or is able to enter into one with terms that are beneficial to CNCE shareholders. These development programs have garnered major investor interest within Concert's operations; if they do not progress, there is material risk that CNCE shares could trade downward.

**Risks With Partnered Programs.** Concert has a number of partnered programs, including material efforts with Celgene, Jazz Pharmaceuticals, and Avanir Pharmaceuticals. These partnered development programs have garnered major investor interest within Concert's operations. Because these programs are ultimately under the direction of other companies, there is no guarantee those programs will progress at all, or in a way that is beneficial to Concert or CNCE shareholders. If any or all of these programs do not progress in a way that is beneficial to Concert, it is possible that CNCE shares may come under material negative pressure.

**Deuteration: Approval, Manufacturing Risks.** To the best of our knowledge, no deuterated drug has ever been successfully approved or commercialized. There may be specific risks to gaining licensure for these types of agents from regulatory authorities, though these do not appear to have not emerged at this point. In addition, the company may also incur unforeseen manufacturing challenges with deuterated compounds, or manufacturing costs that are required for the production of any product candidate that receives marketing approval may turn out to be substantial, though excessive cost have not specifically manifested at this point.

**Other Risks.** Concert has incurred significant losses since inception, expects to incur losses for at least the next several years, and may never sustain profitability. Concert also has a limited operating history and no history of commercializing pharmaceutical products, which may make it difficult to evaluate the prospects for future viability. It is an "emerging growth company," and the reduced disclosure requirements applicable to emerging growth companies may make its common stock less attractive to investors.

**Concert Pharmaceuticals, Inc.**

|  | 2011A           | 2012A            | 2013A           | 1Q14A           | 2Q14A           | 3Q14E           | 4Q14E           | 2014e              | 2015E            | 2016E            | 2017E            | 2018E             |
|--|-----------------|------------------|-----------------|-----------------|-----------------|-----------------|-----------------|--------------------|------------------|------------------|------------------|-------------------|
| <b>Income Statement (\$000, except per share amts.)</b>    |                 |                  |                 |                 |                 |                 |                 |                    |                  |                  |                  |                   |
| <b>Product Revenue</b>                                     |                 |                  |                 |                 |                 |                 |                 |                    |                  |                  |                  |                   |
| CTP-354 Total (Int'l to partner)                           | \$ -            | \$ -             | \$ -            | \$ -            | \$ -            | \$ -            | \$ -            | \$ -               | \$ -             | \$ -             | \$ -             | \$ -              |
| CTP-499 Total (top line to partner)                        | \$ -            | \$ -             | \$ -            | \$ -            | \$ -            | \$ -            | \$ -            | \$ -               | \$ -             | \$ -             | \$ -             | \$ -              |
| Other/Collab revenue                                       | 0               | 0                | 0               | 0               | 0               | 0               | 0               | 0                  | 0                | 0                | 0                | 0                 |
| <b>Total Proprietary Sales Revenue</b>                     | <b>0</b>        | <b>0</b>         | <b>0</b>        | <b>0</b>        | <b>0</b>        | <b>0</b>        | <b>0</b>        | <b>0</b>           | <b>0</b>         | <b>0</b>         | <b>0</b>         | <b>0</b>          |
| <b>Milestone &amp; Royalty Revenue</b>                     |                 |                  |                 |                 |                 |                 |                 |                    |                  |                  |                  |                   |
| <b>Total Royalties from Partners</b>                       | <b>0</b>        | <b>0</b>         | <b>0</b>        | <b>-</b>        | <b>-</b>        | <b>-</b>        | <b>-</b>        | <b>0</b>           | <b>-</b>         | <b>-</b>         | <b>-</b>         | <b>-</b>          |
| <b>Upfront, Milestones from Partners</b>                   | <b>\$ 5,500</b> | <b>\$ 1,500</b>  | <b>\$ 2,000</b> | <b>\$ -</b>     | <b>\$ -</b>     | <b>\$ -</b>     | <b>\$ 2,000</b> | <b>\$ 2,000</b>    | <b>\$ 19,000</b> | <b>\$ 13,750</b> | <b>\$ 40,750</b> | <b>\$ 152,750</b> |
| <b>License and Development Revenue</b>                     | <b>13,967</b>   | <b>11,349</b>    | <b>23,408</b>   | <b>1,613</b>    | <b>1,235</b>    | <b>2,250</b>    | <b>2,500</b>    | <b>\$ 7,598</b>    | <b>8,500</b>     | <b>8,500</b>     | <b>9,000</b>     | <b>9,000</b>      |
| <b>Total Concert Revenue</b>                               | <b>\$19,467</b> | <b>\$12,849</b>  | <b>\$25,408</b> | <b>\$1,613</b>  | <b>\$1,235</b>  | <b>\$2,250</b>  | <b>\$4,500</b>  | <b>\$9,598</b>     | <b>\$27,500</b>  | <b>\$22,250</b>  | <b>\$49,750</b>  | <b>\$161,750</b>  |
| <b>Expenses:</b>   |                 |                  |                 |                 |                 |                 |                 |                    |                  |                  |                  |                   |
| COGS   | \$0             | \$0              | \$0             | \$0             | \$0             | \$0             | \$0             | \$0                | \$0              | \$0              | \$0              | \$0               |
| R & D  | 23,436          | 24,193           | 21,790          | 5,594           | 6,243           | 6,500           | 7,500           | \$ 25,837          | 28,500           | 30,000           | 30,000           | 35,000            |
| S G & A  | 7,377           | 7,266            | 8,028           | 2,538           | 2,718           | 2,700           | 2,650           | \$ 10,606          | 10,500           | 10,750           | 13,000           | 20,000            |
| <b>Total Expenses</b>                                      | <b>\$30,813</b> | <b>31,459</b>    | <b>29,818</b>   | <b>\$8,132</b>  | <b>\$8,961</b>  | <b>\$9,200</b>  | <b>\$10,150</b> | <b>\$ 36,443</b>   | <b>\$39,000</b>  | <b>\$40,750</b>  | <b>\$43,000</b>  | <b>\$55,000</b>   |
| <b>Operating Income</b>                                    | <b>(11,346)</b> | <b>(18,610)</b>  | <b>(4,410)</b>  | <b>(6,519)</b>  | <b>(7,726)</b>  | <b>(6,950)</b>  | <b>(5,650)</b>  | <b>\$ (26,845)</b> | <b>(11,500)</b>  | <b>(18,500)</b>  | <b>6,750</b>     | <b>106,750</b>    |
| <i>Operating Margin</i>                                    | NM              | NM               | NM              | NM              | NM              | NM              | NM              | NM                 | NM               | NM               | NM               | NM                |
| Interest Income  | 44              | 22               | 20              | 4               | 16              | 50              | 60              | \$ 130             | 150              | 175              | 200              | 800               |
| Interest Expense   | (18)            | (1,856)          | (1,666)         | 0               | 0               | 0               | 0               | \$ -               | 0                | (100)            | (100)            | (100)             |
| Other Income (Expense)                                     | 0               | 0                | 0               | 0               | 0               | 0               | 0               | \$ -               | 0                | 0                | 0                | 0                 |
| Other financing income (expense)                           | 0               | 0                | 0               | (435)           | (280)           | (250)           | (200)           | \$ (1,165)         | (750)            | (400)            | (350)            | 0                 |
| <b>Total Other Income, net</b>                             | <b>26</b>       | <b>(1,834)</b>   | <b>(1,646)</b>  | <b>(431)</b>    | <b>(264)</b>    | <b>(200)</b>    | <b>(140)</b>    | <b>(1,035)</b>     | <b>(600)</b>     | <b>(325)</b>     | <b>(250)</b>     | <b>700</b>        |
| <b>Pretax Income</b>                                       | <b>(11,320)</b> | <b>(20,444)</b>  | <b>(6,056)</b>  | <b>(6,950)</b>  | <b>(7,990)</b>  | <b>(7,150)</b>  | <b>(5,790)</b>  | <b>\$ (27,880)</b> | <b>(12,100)</b>  | <b>(18,825)</b>  | <b>6,500</b>     | <b>107,450</b>    |
| <i>Pretax Margin</i>                                       | NM              | NM               | NM              | NM              | NM              | NM              | NM              | NM                 | NM               | NM               | NM               | NM                |
| <b>Effective Taxes</b>                                     | -               | -                | -               | -               | -               | -               | -               | -                  | -                | -                | 455              | 9,133             |
| <i>Tax Rate</i>  | 0.0%            | 0.0%             | 0.0%            | 0.0%            | 0.0%            | 0.0%            | 0.0%            | 0.0%               | 0.0%             | 0.0%             | 7.0%             | 8.5%              |
| <b>Fully Taxed rate</b>                                    | <b>(4,302)</b>  | <b>(7,769)</b>   | <b>(2,301)</b>  | <b>(2,641)</b>  | <b>(3,036)</b>  | <b>(2,717)</b>  | <b>(2,200)</b>  | <b>\$ (10,594)</b> | <b>(4,598)</b>   | <b>(7,154)</b>   | <b>2,470</b>     | <b>40,831</b>     |
| <i>Tax Rate</i>  | 38.0%           | 38.0%            | 38.0%           | 38.0%           | 38.0%           | 38.0%           | 38.0%           | 38.0%              | 38.0%            | 38.0%            | 38.0%            | 38.0%             |
| Other Convertible Preferred, other securities transactions | (1,069)         | (388)            | (396)           | (55)            | (50)            | (45)            | (40)            | (190)              | -                | -                | -                | -                 |
| <b>Net Income (Loss) - Effective taxed</b>                 | <b>(12,389)</b> | <b>(20,832)</b>  | <b>(6,452)</b>  | <b>(7,005)</b>  | <b>(8,040)</b>  | <b>(7,195)</b>  | <b>(6,830)</b>  | <b>\$ (28,070)</b> | <b>(12,100)</b>  | <b>(18,825)</b>  | <b>6,045</b>     | <b>98,317</b>     |
| <b>Income - Fully taxed</b>                                | <b>(7,018)</b>  | <b>(12,675)</b>  | <b>(3,755)</b>  | <b>(4,309)</b>  | <b>(5,004)</b>  | <b>(4,433)</b>  | <b>(3,590)</b>  | <b>\$ (17,336)</b> | <b>(7,502)</b>   | <b>(11,672)</b>  | <b>4,030</b>     | <b>66,619</b>     |
| <i>Margin</i>  | NM              | NM               | NM              | NM              | NM              | NM              | NM              | NM                 | NM               | NM               | NM               | NM                |
| Other Comprehensive Income (Loss)                          | 16              | (5)              | (1)             | (8)             | -               | -               | -               | (8)                | -                | -                | -                | -                 |
| <b>Comprehensive Income (Loss)</b>                         | <b>(11,304)</b> | <b>(20,449)</b>  | <b>(6,057)</b>  | <b>(6,958)</b>  | <b>(7,990)</b>  | <b>(7,150)</b>  | <b>(5,790)</b>  | <b>\$ (27,888)</b> | <b>(12,100)</b>  | <b>(18,825)</b>  | <b>6,500</b>     | <b>107,450</b>    |
| <b>EPS (ex-charges; eff. taxed)</b>                        | <b>(\$9.66)</b> | <b>(\$16.15)</b> | <b>(\$4.99)</b> | <b>(\$0.76)</b> | <b>(\$0.45)</b> | <b>(\$0.40)</b> | <b>(\$0.32)</b> | <b>(\$1.78)</b>    | <b>(\$0.67)</b>  | <b>(\$1.03)</b>  | <b>\$0.31</b>    | <b>\$5.09</b>     |
| <b>EPS (ex-charges; fully-taxed)</b>                       | <b>(\$5.47)</b> | <b>(\$9.83)</b>  | <b>(\$2.91)</b> | <b>(\$0.47)</b> | <b>(\$0.28)</b> | <b>(\$0.25)</b> | <b>(\$0.20)</b> | <b>(\$1.10)</b>    | <b>(\$0.41)</b>  | <b>(\$0.64)</b>  | <b>\$0.21</b>    | <b>\$3.40</b>     |
| EPS - comprehensive Income (eff taxes)                     | (\$8.81)        | (\$15.85)        | (\$4.69)        | (\$0.76)        | (\$0.45)        | (\$0.40)        | (\$0.32)        | (\$1.77)           | (\$0.67)         | (\$1.03)         | \$0.33           | \$5.48            |
| Shares O/S (000), Basic                                    | 1,283           | 1,290            | 1,292           | 9,188           | 17,937          | 18,000          | 18,050          | 15,794             | 18,100           | 18,200           | 19,200           | 19,300            |
| Shares O/S (000), Diluted                                  | 1,283           | 1,290            | 1,292           | 9,188           | 17,937          | 18,000          | 18,050          | 15,794             | 18,100           | 18,200           | 19,500           | 19,600            |
| <b>-- Expenses (% of sales) --</b>                         |                 |                  |                 |                 |                 |                 |                 |                    |                  |                  |                  |                   |
| Cost of Sales (product sales)                              | 0.0%            | 0.0%             | 0.0%            | 0.0%            | 0.0%            | 0.0%            | 0.0%            | 0.0%               | 0.0%             | NM               | NM               | NM                |
| Gross  | NM              | NM               | NM              | NM              | NM              | NM              | NM              | NM                 | NM               | NM               | NM               | NM                |
| R & D  | NM              | NM               | NM              | NM              | NM              | NM              | NM              | NM                 | 103.6%           | 134.8%           | 60.3%            | 21.6%             |
| S G & A  | 31.0%           | 31.0%            | 31.0%           | 31.0%           | 31.0%           | 31.0%           | 31.0%           | 31.0%              | 31.0%            | 31.0%            | 31.0%            | 31.0%             |
| Total  | 158.3%          | 244.8%           | 117.4%          | 504.2%          | 725.6%          | 408.9%          | 225.6%          | 379.7%             | 141.8%           | 183.1%           | 86.4%            | 34.0%             |
| <b>-- Year / Year Growth --</b>                            |                 |                  |                 |                 |                 |                 |                 |                    |                  |                  |                  |                   |
| Revenue  | NM              | NM               | NM              | NM              | NM              | NM              | NM              | NM                 | 96.4%            | -19.1%           | 123.6%           | 225.1%            |
| Operating Income   | NM              | NM               | NM              | NM              | NM              | NM              | NM              | NM                 | NM               | NM               | NM               | NM                |
| Pretax Income  | NM              | NM               | NM              | NM              | NM              | NM              | NM              | NM                 | NM               | NM               | NM               | NM                |
| Income   | NM              | NM               | NM              | NM              | NM              | NM              | NM              | NM                 | NM               | NM               | NM               | NM                |
| EPS (ex-charges)   | NM              | NM               | NM              | NM              | NM              | NM              | NM              | NM                 | NM               | NM               | NM               | NM                |
| EPS (ex-charges; fully-taxed)                              | NM              | NM               | NM              | NM              | NM              | NM              | NM              | NM                 | NM               | NM               | NM               | NM                |

Source: Concert Pharmaceuticals Inc. SEC documents and Ladenburg Thalmann BioPharmaceuticals estimates.

Contact Information: Robert (Bert) Hazlett, Managing Director, Ladenburg Thalmann, rhazlett@ladenburg.com, 212-409-2062.



**Concert Pharmaceuticals, Inc.****Balance Sheet & Statement of Cash Flow (\$mm)**

|  | 12/31/2011A   | 12/31/2012A   | 12/31/2013A   | 12/31/2014E    | 12/31/2015E    | 12/31/2016E    | 12/31/2017E    |
|--|---------------|---------------|---------------|----------------|----------------|----------------|----------------|
| <b>BALANCE SHEET</b>                             |               |               |               |                |                |                |                |
| <b>ASSETS</b>                                    |               |               |               |                |                |                |                |
| Cash & equivalents                               | \$22.9        | \$7.5         | \$9.6         | \$69.3         | \$75.9         | \$74.3         | \$76.9         |
| Investments/Mktb. Securities                     | \$19.7        | \$20.1        | \$23.0        | \$23.0         | \$23.0         | \$23.0         | \$23.0         |
| Account receivable                               | \$0.6         | \$0.1         | \$0.3         | \$2.1          | \$4.1          | \$3.3          | \$7.5          |
| Prepaid & other current assets                   | \$0.9         | \$1.2         | \$1.1         | \$0.1          | \$0.3          | \$0.2          | \$0.5          |
| <b>Total Current Assets</b>                      | <b>\$44.1</b> | <b>\$28.9</b> | <b>\$34.0</b> | <b>\$94.6</b>  | <b>\$103.4</b> | <b>\$100.9</b> | <b>\$107.9</b> |
| Property & Equipment, net, other                 | \$4.4         | \$3.5         | \$2.5         | \$2.5          | \$2.5          | \$2.5          | \$2.5          |
| Long term investment/Restricted Cash             | \$0.0         | \$0.0         | \$0.7         | \$0.7          | \$0.7          | \$0.7          | \$0.7          |
| Other Assets                                     | \$0.8         | \$0.8         | \$2.5         | \$2.5          | \$2.5          | \$2.5          | \$2.5          |
| <b>Total Assets</b>                              | <b>\$49.4</b> | <b>\$33.1</b> | <b>\$39.8</b> | <b>\$100.3</b> | <b>\$109.1</b> | <b>\$106.6</b> | <b>\$113.6</b> |
| <b>LIABILITIES &amp; S.E.</b>                    |               |               |               |                |                |                |                |
| Accounts payable                                 | \$1.6         | \$0.8         | \$1.0         | \$1.4          | \$2.8          | \$3.3          | \$7.5          |
| Accrued expenses                                 | \$1.5         | \$2.0         | \$2.5         | \$2.7          | \$2.9          | \$3.1          | \$3.4          |
| Deferred short-term revenue                      | \$6.9         | \$0.0         | \$4.3         | \$1.0          | \$0.0          | \$0.0          | \$0.0          |
| Leasehold improvement loan                       | \$0.3         | \$0.3         | \$0.3         | \$0.3          | \$0.3          | \$0.3          | \$0.3          |
| Loans payable, net of discount                   | \$0.0         | \$4.8         | \$7.8         | \$5.6          | \$8.3          | \$3.3          | \$10.0         |
| <b>Total Current Liabilities</b>                 | <b>\$10.3</b> | <b>\$7.9</b>  | <b>\$15.9</b> | <b>\$11.0</b>  | <b>\$14.2</b>  | <b>\$10.1</b>  | <b>\$21.1</b>  |
| Deferred revenue, net of current                 | \$4.1         | \$2.8         | \$15.3        | \$9.0          | \$3.0          | \$0.0          | \$0.0          |
| Leasehold improvement loan, net of current       | \$0.9         | \$0.6         | \$0.2         | \$2.0          | \$2.0          | \$2.0          | \$2.0          |
| Deferred lease incentive                         | \$1.4         | \$0.9         | \$0.4         | \$2.0          | \$2.0          | \$2.0          | \$2.0          |
| Deferred rent, net of current                    | \$0.6         | \$0.5         | \$0.2         | \$2.0          | \$2.0          | \$2.0          | \$2.0          |
| Warrant to purchase redeemable securities        | \$0.2         | \$0.5         | \$0.5         | \$0.5          | \$0.5          | \$0.5          | \$0.5          |
| Note Payable/Long-term Liabilities               | \$7.1         | \$14.9        | \$7.1         | \$7.1          | \$7.1          | \$7.1          | \$7.1          |
| <b>Total Liabilities</b>                         | <b>\$24.7</b> | <b>\$28.0</b> | <b>\$39.6</b> | <b>\$33.6</b>  | <b>\$30.8</b>  | <b>\$23.7</b>  | <b>\$34.7</b>  |
| <b>Total Shareholders Equity</b>                 | <b>\$24.7</b> | <b>\$5.2</b>  | <b>\$0.1</b>  | <b>\$66.7</b>  | <b>\$78.3</b>  | <b>\$82.9</b>  | <b>\$78.9</b>  |
| <b>Total Liabilities and Shareholders Equity</b> | <b>\$49.4</b> | <b>\$33.1</b> | <b>\$39.8</b> | <b>\$100.3</b> | <b>\$109.1</b> | <b>\$106.6</b> | <b>\$113.6</b> |
| <b>CASH FLOW STATEMENT</b>                       |               |               |               |                |                |                |                |
| <b>Cash Flow from Operating Activities</b>       |               |               |               |                |                |                |                |
| Net income (loss)                                | (11.3)        | (\$20.4)      | (6.1)         | (20.3)         | (12.1)         | (18.8)         | 6.5            |
| Other adjustments                                | 0.0           | \$0.0         | 0.0           | 0.0            | 20.0           | 20.0           | (15.0)         |
| Depreciation & Amortization                      | 1.6           | \$1.5         | 1.3           | 1.1            | 1.2            | 1.4            | 2.0            |
| Noncash compensation expense                     | 0.9           | \$0.9         | 1.0           | 1.0            | 1.5            | 2.0            | 2.5            |
| Other non-cash financing expense                 | 0.8           | \$0.4         | 0.3           | 0.0            | 0.0            | 0.0            | 0.0            |
| Amortization of financing costs, warrants        | (0.5)         | (\$0.1)       | (0.4)         | 0.0            | 0.0            | 0.0            | 0.0            |
| Accounts receivable                              | 1.0           | \$0.5         | (0.2)         | (1.8)          | (2.0)          | 0.8            | (4.1)          |
| Interest receivable                              | 0.1           | \$0.0         | 0.0           | 0.0            | 0.0            | 0.0            | 0.0            |
| Prepaid expenses, and other current assets       | (0.0)         | (\$0.4)       | 0.0           | 1.0            | (0.1)          | 0.1            | (0.3)          |
| Other assets                                     | (0.1)         | \$0.0         | 0.1           | 0.0            | 0.0            | 0.0            | 0.0            |
| Accounts payable and accrued expenses            | 0.7           | (\$0.3)       | 0.1           | 0.6            | 1.6            | 0.8            | 4.4            |
| Other oper.activities (deferred rent, revenue)   | (11.2)        | (\$8.4)       | 16.7          | (8.5)          | (3.4)          | (7.9)          | 6.6            |
| <b>Cash Flow from Operating Activities</b>       | <b>(18.1)</b> | <b>(26.4)</b> | <b>13.0</b>   | <b>(26.9)</b>  | <b>6.7</b>     | <b>(1.7)</b>   | <b>2.6</b>     |
| <b>Cash Flow from Investing Activities</b>       |               |               |               |                |                |                |                |
| Maturities of Investments                        | 64            | \$37.7        | 27            | 0              | 0              | 0              | 0              |
| Purchase of investments                          | (41)          | (\$38.4)      | (30)          | 0              | 0              | 0              | 0              |
| Capital Expenditures, net                        | (0.3)         | (\$0.5)       | (0.4)         | 0.0            | 0.0            | 0.0            | 0.0            |
| Other  | -             | \$0.0         | -             | -              | -              | -              | -              |
| <b>Cash Flow from Investing Activities</b>       | <b>22.9</b>   | <b>(1.2)</b>  | <b>(3.64)</b> | <b>0.00</b>    | <b>0.00</b>    | <b>0.00</b>    | <b>0.00</b>    |
| <b>Cash Flow from Financing Activities</b>       |               |               |               |                |                |                |                |
| Issuance of loan payable, net                    | 7.3           | \$12.5        | 0             | 0              | 0              | 0              | 0              |
| Principle pmts of loan payable                   | 0.0           | \$0.0         | (5)           | 0              | 0              | 0              | 0              |
| Repayment of leasehold impt loan                 | (0.3)         | (\$0.3)       | (0)           | 0              | 0              | 0              | 0              |
| Proceeds from iss. of common stock (net)         | 0.0           | \$0.0         | 0             | 86.6           | 0.0            | 0.0            | 0.0            |
| Proceeds/Retirement of Debt, Other               | 0.0           | \$0.0         | 0             | 0.0            | 0.0            | 0.0            | 0.0            |
| Payment of IPO/offering costs                    | 0.0           | \$0.0         | (2)           | 0.0            | 0.0            | 0.0            | 0.0            |
| <b>Cash Flow from Financing Activities</b>       | <b>7.0</b>    | <b>12.2</b>   | <b>(7.2)</b>  | <b>86.6</b>    | <b>0.0</b>     | <b>0.0</b>     | <b>0.0</b>     |
| <b>Beginning cash balance</b>                    | <b>11.1</b>   | <b>\$22.9</b> | <b>7.5</b>    | <b>9.6</b>     | <b>69.3</b>    | <b>75.9</b>    | <b>74.3</b>    |
| Net increase (decrease) in cash                  | 11.8          | (\$15.5)      | 2.1           | 59.7           | 6.7            | (1.7)          | 2.6            |
| <b>Ending cash balance</b>                       | <b>22.9</b>   | <b>7.5</b>    | <b>9.6</b>    | <b>69.3</b>    | <b>75.9</b>    | <b>74.3</b>    | <b>76.9</b>    |

Source: Concert Pharmaceuticals Inc. SEC documents and Ladenburg Thalmann BioPharmaceuticals estimates.

Contact information: Robert (Bert) Hazlett, Managing Director, Ladenburg Thalmann, rhazlett@ladenburger.com, 212-409-2062.

# APPENDIX A: IMPORTANT RESEARCH DISCLOSURES

## ANALYST CERTIFICATION

I, Robert C. Hazlett, attest that the views expressed in this research report accurately reflect my personal views about the subject security and issuer. Furthermore, no part of my compensation was, is, or will be directly or indirectly related to the specific recommendation or views expressed in this research report, provided, however, that:

The research analyst primarily responsible for the preparation of this research report has or will receive compensation based upon various factors, including the volume of trading at the firm in the subject security, as well as the firm's total revenues, a portion of which is generated by investment banking activities.

Additional information regarding the contents of this publication will be furnished upon request. Please contact Ladenburg Thalmann, Compliance Department, 570 Lexington Avenue, 11th floor, New York, New York 10022 (or call 212-409-2000) for any information regarding current disclosures, and where applicable, relevant price charts, in regard to companies that are the subject of this research report.

## COMPANY BACKGROUND

Concert Pharmaceuticals, founded in 2006 by Richard Aldrich, Roger Tung, and Christoph Westphal, creates novel medicines by applying its DCE (Deuterated Chemical Entity) Platform technologies to molecules. The DCE approach involves the selective addition of deuterium, a stable isotope of hydrogen, to molecules, resulting in clinical therapeutic candidates with improved pharmacology. This approach has the potential to enable more efficient drug discovery and clinical development. Concert's proprietary programs include CTP-354, a novel GABA-A modulator for spasticity and anxiety, and CTP-499, a PDE inhibitor for diabetic kidney disease. Promising partnered programs include Jazz's JZP-386, a long-acting Xyrem, Avanir's AVP-786, an improved Neudexta, and multiple programs with Celgene. Concert has also developed a broad portfolio of deuterated molecules that are expected to migrate into and through the clinic over time.

## VALUATION METHODOLOGY

**Concert Pharmaceuticals Valuation.** Concert and CNCE Shares Valuation. Because Concert's operations have the potential for significant revenue and earnings variability over the coming quarters and years, we value the company and its assets using a fully-taxed, risk-weighted net present value methodology for each of its assets. We note that with its multiple partnerships and emerging clinical programs, Concert has a diverse portfolio that contributes to its valuation. Our total of the programs yield a value of \$25.77 per CNCE share, driving our \$26 CNCE shares price target.

**Concert's Proprietary Pipeline.** Concert's two most advanced proprietary assets, CTP-354 for spasticity, and CTP-499 for diabetic kidney disease, have material valuation potential, in our view. CTP-354 for spasticity as a result of multiple sclerosis and/or spinal cord injury and possibly other indications such as anxiety and neuropathic pain, is moving into Phase 2. We believe that a GABA-A modulator with familiar GABA mechanism, yet a distinct profile, with once-daily dosing and less sedation, should have material potential for consideration in spasticity, anxiety, and even pain settings, and have the potential to exceed \$1 billion in peak revenue. We value its collective opportunities at \$7.82 per share. CTP-499, for diabetic kidney disease appears to have achieved material validation after a more full analysis of its Phase 2 trial data, which bodes well for Phase 3 consideration. We assume licensing is to occur after discussions with FDA now that Phase 2 is winding up. Despite risks to the Phase 3 endpoints that remain, we believe more reasonable guidance is emerging along those lines from FDA, and estimate CTP-499 at \$1 billion peak revenue, and value the program at \$5.70 per CNCE share. Other less mature proprietary products are much more modest opportunities at this point and valued at \$0.35 per CNCE share, though proof of concept comes at Phase 1 for many of these deuterated programs, and this part of the portfolio could appreciate quite quickly.

**Concert's Collaborations.** Regarding Concert's partnered portfolio, Avanir seems to be operating with a good urgency with AVP-786, and is exploring dextromethorphan's broad neurotransmitter receptor activity with the molecule, looking at the therapy in multiple additional indications in addition to depression, including agitation and dyskinesia. Because of these multiple indications, and its potential for its rapid advancement, the economics of this program are valued at \$4.53 per CNCE share. The Jazz program JZP-386 attempts to provide the Xyrem franchise with a product that doesn't have middle of the night dosing, and can help protect against potential loss of exclusivity. Xyrem is growing rapidly (+50% yr/yr) and is large at \$569 million in 2013, and making a more convenient dosing form with longer patent life appears to be a material opportunity, generating a \$2.52 NPV per CNCE share. Finally Celgene has not disclosed the mechanism of CTP-730 inflammation collaboration, though the very large regulatory milestones and peak revenue along the lines of Xyrem, give this collaboration a \$3.33 NPV, by our calculation. The remainder of the Celgene assets are estimated at \$0.68, and net cash, NOLs and the drag of general corporate expense totals \$0.68 per CNCE share, yielding the \$25.77 total, driving our \$26 CNCE shares price target.

Factors which could impede CNCE shares from reaching our price target include the lack of progress for Concert's proprietary therapeutics CTP-499 and CTP-354 in their respective indications. Progress by indirect competition in indications for chronic kidney failure or spasticity could also impede CNCE shares from reaching our target. Concert has a number of partnered programs, including efforts with Celgene, Jazz Pharmaceuticals, and Avanir, and there is no guarantee those programs will progress at all or in a way that is beneficial to Concert; a lack of progress with any or all of these partnered programs could be an impediment to CNCE shares reaching our target price. In addition, negative

equity market conditions overall, or in particular with regard to the biotechnology sector, or healthcare in general, could be an impediment to CNCE shares reaching our target. Also a change in the regulatory requirements for drugs in development could be an impediment to the advancement in CNCE shares. These risks listed are merely a sample of the types of issues that could impede CNCE shares from advancing, and are not meant to be all inclusive.

## RISKS

**Regulatory/FDA.** As with any company whose main business focuses on the development of pharmaceuticals, Concert is subject to the strenuous regulatory requirements of the US Food and Drug Administration (FDA) and other international regulatory agencies such as the EMEA to have its new drugs approved. Promotion of its approved drug products is also highly regulated by FDA and related agencies throughout the globe. **Dependence on Proprietary Programs.** CTP-354 and CTP-499 are two of Concert's most advanced proprietary clinical candidates in development. These novel molecules may take material time and resources to finish clinical development, if they are able to complete is at all, and there is certainly no guarantee that the company will be successful in doing so. In addition, Concert may seek one or more collaborators for future development of CTP-499. There is a risk that the company may not be able to enter into a collaboration for the therapy, or is able to enter into one with terms that are beneficial to CNCE shareholders. **Risks With Partnered Programs.** Concert has a number of partnered programs, including material efforts with Celgene, Jazz Pharmaceuticals, and Avanir Pharmaceuticals. Because these programs are ultimately under the direction of other companies, there is no guarantee those programs will progress at all, or in a way that is beneficial to Concert or CNCE shareholders. **Deuteration: Approval, Manufacturing Risks.** To the best of our knowledge, no deuterated drug has ever been successfully approved or commercialized. There may be specific risks to gaining licensure for these types of agents from regulatory authorities, though these do not appear to have not emerged at this point.

## STOCK RATING DEFINITIONS

Buy: The stock's return is expected to exceed 12.5% over the next twelve months.

Neutral: The stock's return is expected to be plus or minus 12.5% over the next twelve months.

Sell: The stock's return is expected to be negative 12.5% or more over the next twelve months.

Investment Ratings are determined by the ranges described above at the time of initiation of coverage, a change in risk, or a change in target price. At other times, the expected returns may fall outside of these ranges because of price movement and/or volatility. Such interim deviations from specified ranges will be permitted but will become subject to review.

## RATINGS DISPERSION AND BANKING RELATIONSHIPS AS OF (October 14, 2014)

| Rating  | %    | IB % |
|---------|------|------|
| BUY     | 74.2 | 57.8 |
| NEUTRAL | 25.8 | 39.3 |
| SELL    | 0.0  | 0.0  |

## COMPANIES UNDER ROBERT'S COVERAGE

Acadia Pharmaceuticals Inc. (ACAD)  
 Cemptra Inc. (CEMP)  
 CTI BioPharma Corp. (CTIC)  
 OvaScience, Inc. (OVAS)  
 Targacept, Inc. (TRGT)

Auspex Pharmaceuticals, Inc. (ASPX)  
 Concert Pharmaceuticals Inc. (CNCE)  
 Nektar Therapeutics (NKTR)  
 Prothena Corporation plc (PRTA)

## COMPANY SPECIFIC DISCLOSURES

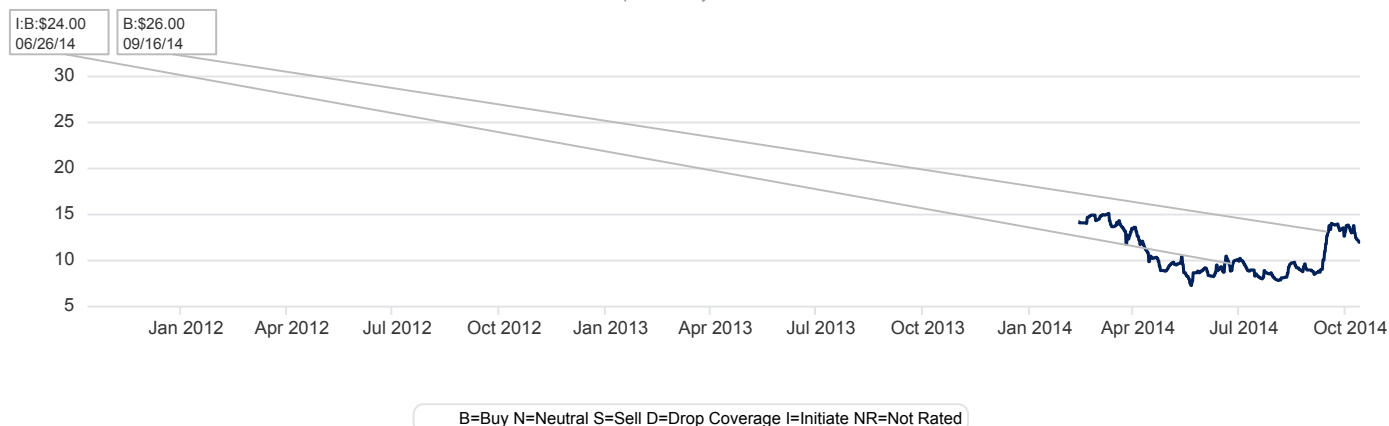
Ladenburg Thalmann & Co. Inc. makes a market in Concert Pharmaceuticals Inc..

Ladenburg Thalmann & Co. Inc. intends to seek compensation for investment banking and/or advisory services from Concert Pharmaceuticals Inc. within the next 3 months.

## OTHER COMPANIES MENTIONED

**INVESTMENT RATING AND PRICE TARGET HISTORY****Concert Pharmaceuticals Inc. Rating History as of 10/13/2014**

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## EQUITY RESEARCH

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#### Water & Sustainable Infrastructure

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#### Healthcare Equipment & Medical Technologies

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