

# Concert Pharmaceuticals, Inc. (CNCE)

Favorable Safety Threshold Advances Higher Dose in Phase I Trial

## MARKET DATA

Price	\$8.75
52-Week Range:	\$7.12 - \$16.26
Shares Out. (M):	17.9
Market Cap (\$M):	\$156.6
Average Daily Vol. (000):	208.0
Cash (M):	\$108
Cash/Share:	\$6.04
Enterprise Value (M):	\$274
Float (M):	16.2
LT Debt (M):	\$9

Source: Thomson Reuters and JMP Securities LLC

**MARKET OUTPERFORM** | Price: \$8.75 | Target Price: \$28.00

## INVESTMENT HIGHLIGHTS

**Phase I trial continues on track as the FDA lifts its partial clinical hold on Concert Pharmaceuticals' CTP-354; reiterate Market Outperform rating and \$28 price target based on DCF and SOTP valuation methodologies.** Today, CNCE announced that the company has completed the necessary pre-clinical testing for CTP-354, a novel non-sedating treatment for spasticity for use in patients with multiple sclerosis and spinal cord injury, and the FDA has lifted its partial clinical hold. CNCE plans to initiate the 12mg per day arm of its Phase I multiple ascending-dose trial in 3Q14.

**Favorable pre-clinical safety and tolerability initially leaves the FDA wanting more.** CTP-354 has demonstrated excellent pre-clinical PK/PD and safety with minimal to no toxicity. Previously, in absence of a determined maximum tolerated dose, the FDA would not allow trials in normal volunteers beyond the tested 6mg dose. With the potential of CTP-354 to be used in a number of indications outside of spasticity, including anxiety, chronic pain, muscle tension and epilepsy, CNCE expanded pre-clinical testing and determined the 12mg dose to display a favorable toxicity profile supporting the expanded multiple ascending-dose Phase I trial.

**Significant upside potential for this deuterated drug company.** We remain bullish on Concert Pharmaceuticals shares, as we see several opportunities for growth over the next several years in addition to CTP-499, including: CTP-354, JZP-386, AVP-786 and CTP-730. In our opinion, the company's DCE Platform® has all the requisite ingredients to allow CNCE to become one of the leading developers of unique, deuterated-therapeutic compounds. We reiterate our Market Outperform rating and \$28 price target for CNCE shares based on our DCF and SOTP valuation methodologies.

FY DEC		2013A	2014E	2015E
Revenue (\$M)	1Q	--	\$1.6A	\$0.0
	2Q	\$24.0	\$0.0	\$0.0
	3Q	\$24.0	\$0.0	\$0.0
	4Q	\$1.4	\$0.0	\$1.6
	<b>FY</b>	<b>\$25.4</b>	<b>\$1.6</b>	<b>\$14.0</b>
EPS	1Q	--	(\$0.76)A	--
	2Q	(\$0.01)	(\$0.53)	--
	3Q	(\$0.01)	(\$0.64)	--
	4Q	(\$4.66)	(\$0.70)	--
	<b>FY</b>	<b>(\$4.99)</b>	<b>(\$2.27)</b>	<b>(\$1.81)</b>

Source: Company reports and JMP Securities LLC

## STOCK PRICE PERFORMANCE



Michael G. King, Jr.  
mking@jmpsecurities.com  
(212) 906-3520

Eric Joseph, PhD  
ejoseph@jmpsecurities.com  
(212) 906-3514

FOR DISCLOSURE AND FOOTNOTE INFORMATION, REFER TO JMP FACTS AND DISCLOSURES SECTION.

**FIGURE 1. Upcoming CNCE Milestones**

Timing	Drug	Milestones
3Q14	CTP-354	Phase I MAD continuation
1H15	CTP-354	Phase II clinical trial in MS and SCI expected to begin
2H14	AVP-786	Phase II trial for treatment of resistant major depressive disorder
2014	JZP-386	First Phase I in-human trial
2014	CTP-730	Clinical trials expected to begin

Source: CNCE company presentations

## Company Description

Concert Pharmaceuticals is a clinical stage biopharmaceutical company seeking to discover and develop novel small molecule drugs through the improvement of existing drugs and clinical candidates, via deuterium substitution. Deuterium substitution can lead to drugs with superior pharmacokinetic or metabolic properties, improved clinical safety, tolerability, and/or efficacy. Deuterated analogs of approved drugs may also be able to enjoy expedited pathways to FDA approval. The firm currently has a trio of clinical-stage product candidates, including CTP-354, for spasticity associated with multiple sclerosis, CTP-499 for diabetic kidney disease, and AVP-786 for neurologic and psychiatric disorders, through a collaboration with Avanir Pharmaceuticals. The firm is also in ongoing collaboration with Celgene Corporation for deuterated compounds, including CTP-730 for inflammatory diseases, and with Jazz Pharmaceuticals for JZP-386, the active ingredient in Xyrem, which is in pre-clinical development for narcolepsy.

## Investment Risks

**Clinical risk.** Products undergoing clinical trials may have serious safety concerns, lack efficacy, or fail to demonstrate statistical significance, any of which would preclude them from continuing clinical development and eventual commercialization. If the company's Deuterated Chemical Entity (DCE) Platform® technology is not proven, there will likely be downside to the share price as well as risk to the viability of the company. In addition, CNCE has not yet demonstrated an ability to successfully conduct a large-scale pivotal clinical trial, obtain marketing approvals, manufacture a commercial scale product, or to conduct the sales and marketing activities necessary for successful product commercialization.

**Collaboration risk.** CNCE will depend upon collaborations with third parties for the development and commercialization of some of the company's product candidates and expects to continue to do so in the future. CNCE's business model relies on making use of its DCE platform to partner with Pharmaceutical and Biotechnology companies to improve existing drug candidates. CNCE's prospects with respect to those product candidates will depend in significant part on the success of those collaborations.

**Manufacturing risk.** CNCE is a clinical-stage biopharmaceutical company applying its extensive knowledge of deuterium chemistry to discover and develop novel small molecule drugs. Because there are limited sources of deuterium, CNCE and its collaborators are exposed to a number of risks and uncertainties associated with the company's deuterium supply. In particular, manufacturing processes for many drug candidates, including those for CTP-499 and certain others, are projected to require large quantities of deuterium for late-stage clinical trials and for commercialization. Consequently, any adverse impact on CNCE's ability to obtain deuterium oxide could have a significant impact on the company's ability to develop or commercialize product candidates. Similarly, CNCE's collaborators will also need to obtain supplies of deuterium and will be subject to risks and requirements in connection with sourcing deuterium similar to the ones the company faces.

**Intellectual property risk.** As of December 31, 2013, CNCE held 100 issued patents worldwide, including 50 issued patents in the United States. CNCE's patents and patent applications for its lead programs are set to expire between 2028 and 2034. The company may be sued by a competitor on patent infringement or have to undergo litigation that would incur substantial fees. The company could lose a case, which would make it susceptible to generic risk.

**Financial risk.** Concert Pharmaceuticals currently derives revenue from research and development funding and from license or collaboration agreements. The company is not yet profitable and has a history of operating losses that are expected to continue in the near future. Developing pharmaceutical products, including conducting pre-clinical studies and clinical trials, is a time-consuming, expensive, and uncertain process that takes years to complete. CNCE needs to continue financing clinical trials through to completion and it may be unable to secure additional funding, forcing it to delay, reduce, or eliminate product development programs or commercialization efforts. The company has incurred significant losses since its inception and should expect losses to occur for the next several years.

**Competitive risk.** CNCE faces competition from marketers of other treatments for the indications that it seeks to develop drugs for, including major pharmaceutical firms and biotech firms. The firm's products will also have to compete with existing treatments that have already become generically available (e.g., CTP-354 will have to compete with other spasticity drugs, such as baclofen, tizanidine, diazepam, and dantrolene).

**Regulatory risk.** CNCE or its collaborators, may, in some instances, be able to secure clearances from the FDA or comparable foreign regulatory authorities to use expedited development pathways. If it is unable to obtain such clearances, CNCE or its collaborators may be required to conduct additional pre-clinical studies or clinical trials beyond those contemplated, which could increase the expense of obtaining, and delay the receipt of, necessary marketing approvals.

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Market Outperform (MO): JMP Securities expects the stock price to outperform relevant market indices over the next 12 months.

Market Perform (MP): JMP Securities expects the stock price to perform in line with relevant market indices over the next 12 months.

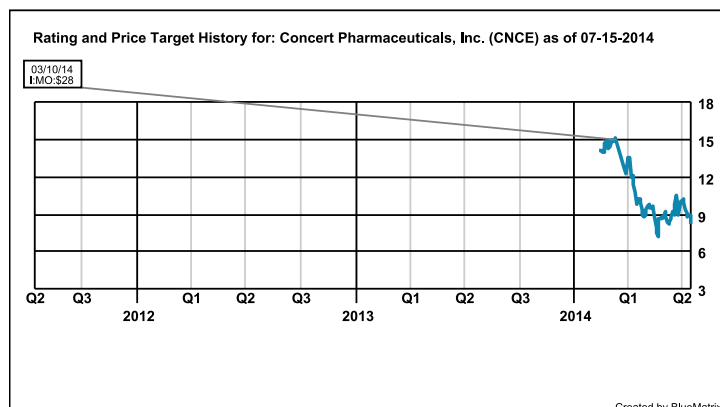
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JMP Rating	Regulatory Equivalent	# Co's Under Coverage	% of Total	Regulatory Equivalent	# Co's Under Coverage	% of Total	# Co's Receiving IB Services in Past 12 Months	% of Co's With This Rating
MARKET OUTPERFORM	Buy	267	59.73%	Buy	267	59.73%	103	38.58%
MARKET PERFORM	Hold	139	31.10%	Hold	139	31.10%	17	12.23%
MARKET UNDERPERFORM	Sell	4	0.89%	Sell	4	0.89%	0	0%
COVERAGE IN TRANSITION		37	8.28%		37	8.28%	0	0%
TOTAL:		447	100%		447	100%	120	26.85%

### Stock Price Chart of Rating and Target Price Changes:

Note: First annotation denotes initiation of coverage or 3 years, whichever is shorter. If no target price is listed, then the target price is N/A. In accordance with NASD Rule 2711, the chart(s) below reflect(s) price range and any changes to the rating or price target as of the end of the most recent calendar quarter. The action reflected in this note is not annotated in the stock price chart. Source: JMP Securities.



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Jeffrey H. Spurr  
Director of Research  
(415) 835-3903

## RESEARCH PROFESSIONALS

### FINANCIAL SERVICES

#### Alternative Asset Managers

Devin Ryan	(212) 906-3578
Brian McKenna	(212) 906-3545

#### Commercial & Specialty Finance

Christopher York	(415) 835-8965
Hannah Kim, CFA	(415) 835-8962

#### Consumer Finance

David M. Scharf	(415) 835-8942
Jeremy Frazer	(312) 768-1796

#### Financial Processing & Outsourcing

David M. Scharf	(415) 835-8942
Jeremy Frazer	(312) 768-1796

#### Insurance

Matthew J. Carletti	(312) 768-1784
Christine Worley	(312) 768-1786

#### Investment Banks & Brokers

Devin Ryan	(212) 906-3578
Brian McKenna	(212) 906-3545

#### Mortgage Operating Companies

##### REITs: Agency, Hybrid, & Commercial Mortgage

Steven C. DeLaney	(404) 848-7773
Trevor Cranston, CFA	(415) 869-4431
Charter Robinson	(757) 613-8955
Benjamin Zucker	(212) 906-3529

### HEALTHCARE

#### Biotechnology

Liisa A. Bayko	(312) 768-1785
Andrew Prigodich	(312) 768-1788
Jason N. Butler, PhD	(212) 906-3505
Caroline Palomeque	(212) 906-3509
Michael G. King, Jr.	(212) 906-3520
Eric Joseph, PhD	(212) 906-3514

#### Healthcare Services & Facilities

Peter L. Martin, CFA	(415) 835-8904
Aaron Hecht	(415) 835-3963
Arthur Kwok	(415) 835-8908

#### Life Science Tools & Diagnostics

J. T. Haresco, III, PhD	(415) 869-4477
Marie T. Casey, PhD	(415) 835-3955

#### Medical Devices

J. T. Haresco, III, PhD	(415) 869-4477
Marie T. Casey, PhD	(415) 835-3955

#### Medical Devices & Supplies

David Turkaly	(212) 906-3563
John Gillings	(212) 906-3564

#### Specialty Pharmaceuticals

Oren G. Livnat, CFA	(212) 906-3566
Nazibur Rahman	(212) 906-3519

### REAL ESTATE

#### Housing & Land Development

Peter L. Martin, CFA	(415) 835-8904
Aaron Hecht	(415) 835-3963
Bharathwajan Iyengar	(415) 835-3902

#### Lodging & Leisure

Robert A. LaFleur	(212) 906-3510
Whitney Stevenson	(212) 906-3538

#### Property Services

Mitch Germain	(212) 906-3546
Peter Lunenburg	(212) 906-3537

#### REITs: Healthcare, Residential, & Specialty

Peter L. Martin, CFA	(415) 835-8904
Aaron Hecht	(415) 835-3963
Arthur Kwok	(415) 835-8908

#### REITs: Office, Industrial, & Diversified

Mitch Germain	(212) 906-3546
Peter Lunenburg	(212) 906-3537

#### Residential Services

Peter L. Martin, CFA	(415) 835-8904
Aaron Hecht	(415) 835-3963
Bharathwajan Iyengar	(415) 835-3902

### TECHNOLOGY

#### Communications Equipment & Internet Security

Erik Suppiger	(415) 835-3918
John Lucia	(415) 835-3920

#### Internet & Digital Media

Ronald V. Josey III	(212) 906-3528
Andrew Boone, CFA	(415) 835-3957
Michael Wu	(415) 835-8996

#### Software

Patrick Walravens	(415) 835-8943
Peter Lowry	(415) 869-4418
Greg McDowell	(415) 835-3934

#### Wireless & Cloud Computing Technologies

Alex Gauna	(415) 835-8998
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## ADDITIONAL CONTACTS

Thomas R. Wright  
Director of Equities  
(212) 906-3599

Dan Wychulis  
Director of Institutional Sales  
(617) 235-8530

600 Montgomery Street, Suite 1100  
San Francisco, CA 94111  
[www.jmpsecurities.com](http://www.jmpsecurities.com)