Concert Pharmaceuticals, Inc. (CNCE)

New Biomarker Data in Diabetic Kidney Disease

MARKET OUTPERFORM | Price: $13.76 | Target Price: $28.00

MARKET DATA

Price	$13.76
52-Week Range:	$7.12 - $16.26
Shares Out. (M):	17.9
Market Cap ($M):	$246.3
Average Daily Vol. (000):	77.0
Cash (M):	$18
Cash/Share:	$1.00
Enterprise Value (M):	$52
Float (M):	16.2
LT Debt (M):	$5

Source: Thomson Reuters and JMP Securities LLC

FY DEC		2013A	2014E	2015E
Revenue ($M)	1Q	--	$1.6A	$1.2
	2Q	$24.0	$1.2A	$0.0
	3Q	$24.0	$0.0	$0.0
	4Q	$1.4	$0.0	$2.8
	FY	**$25.4**	**$2.8**	**$2.0**
EPS	1Q	--	($0.76)A	--
	2Q	($0.01)	($0.45)A	--
	3Q	($0.01)	($0.65)	--
	4Q	($4.66)	($0.71)	--
	FY	**($4.99)**	**($2.20)**	**($2.39)**

Source: Company reports and JMP Securities LLC

STOCK PRICE PERFORMANCE

INVESTMENT HIGHLIGHTS

Concert Pharmaceuticals will present new data from the Phase II trial for CTP-499 in diabetic kidney disease demonstrating reduction in inflammatory biomarkers of kidney fibrosis; reaffirm Market Outperform rating and $28 price target. In the American Society of Nephrology abstract release, the company details plans to present a poster demonstrating additional encouraging results from its 48-week Phase II clinical trial of CTP-499 in patients with diabetic kidney disease supporting an anti-inflammatory and antifibrotic mechanism.

Previously, CNCE had reported the longer-term, 48-week treatment suggests a favorable trend in UACR for patients receiving CTP-499 as compared to placebo. At 48 weeks, UACR in patients receiving CTP-499 increased 24 mg/g from baseline compared to a 223 mg/g increase in patients receiving placebo ($p = 0.097$) with significant reductions in circulating collagen IV and urinary fibronectin. These data may indicate a stabilization of UACR in patients treated with CTP-499 compared to those who received placebo. This abstract explored several other biomarkers of inflammation and fibrosis including IL-18 and clusterin, both of which were significantly decreased (25% and 42%, respectively, $P < 0.05$; N = 65 CTP-499, 57-58 placebo). Additionally, in a high-risk subgroup (baseline UACR $\geq$ 850 mg/g), tubule injury markers MCP-1 and N-OPN decreased by 34% and 55%, respectively ($P < 0.05$, N = 32 CTP-499, 27 placebo). Finally, a statistically significant reduction of the fibrotic biomarkers collagen IV and urinary fibronectin and laminin was observed (13-68%; $P < 0.05$, N=65 CTP-499, 57-58 placebo).

Taken together, these data support the hypothesis that the protective anti-fibrotic effects on kidney function of CTP-499 occurs through an anti-inflammatory mechanism. CTP-499, when used in addition to the standard of care ACE or ARB therapy, may therefore be used to slow the progression of kidney disease to end-stage renal failure, which results in a lifetime of dialysis or kidney transplantation. We also note that each of these biomarkers have also shown elevations in a number of inflammatory and fibrotic conditions, including nonalcoholic steatohepatitis (NASH) and multiple sclerosis.

Upside potential for deuterated drug company. We recommend the purchase of CNCE shares to those investors who have a long-term perspective and a vision toward the kind of company that we believe CNCE can grow into over the course of the next several years. In addition to CTP-499, CNCE has several in-house and partnered deuterium based agents in clinical trials including: CTP-354, JZP-386, AVP-786 and CTP-730. In our view, CNCE's DCE Platform® has all the requisite ingredients to allow it to become one of the leading developers of unique, deuterated-therapeutic compounds.

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FIGURE 1. Upcoming CNCE Milestones

Timing	Drug	Milestones
2H2014	CTP-354	Phase II clinical trial in MS and SCI expected to begin
2H2014	CTP-499	Phase II meeting with the FDA
2H2014	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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JMP Securities was manager or co-manager of a public offering of securities for Concert Pharmaceuticals, Inc. (CNCE) in the past 12 months, and received compensation for doing so.

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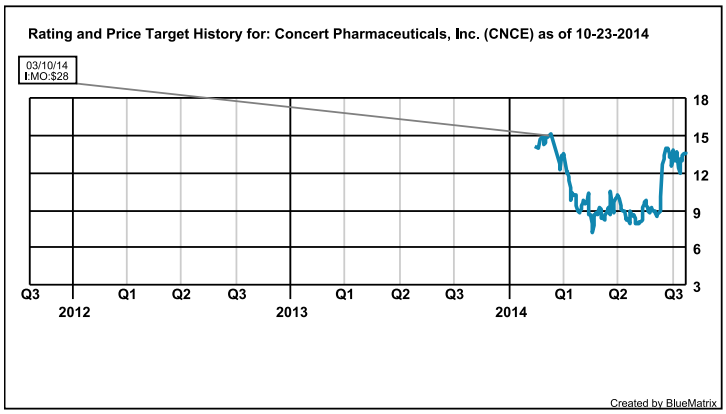
JMP Rating	Regulatory Equivalent	# Co's Under Coverage	*% of Total*	Regulatory Equivalent	# Co's Under Coverage	*% of Total*	# Co's Receiving IB Services in Past 12 Months	*% of Co's With This Rating*
MARKET OUTPERFORM	Buy	284	*61.34%*	Buy	284	*61.34%*	106	*37.32%*
MARKET PERFORM	Hold	140	*30.24%*	Hold	140	*30.24%*	16	*11.43%*
MARKET UNDERPERFORM	Sell	2	*0.43%*	Sell	2	*0.43%*	0	*0%*
COVERAGE IN TRANSITION		36	*7.78%*		36	*7.78%*	0	*0%*
TOTAL:		463	*100%*		463	*100%*	123	*26.57%*

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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