March 6, 2017

Concert Pharmaceuticals Reports Year Ended 2016 Financial Results

- Vertex Pharmaceuticals to Acquire Cystic Fibrosis Candidate, CTP-656, from Concert Pharmaceuticals for up to $250 Million -

- CTP-543 On Track to Advance Into Phase 2a Trial -

- Conference Call Scheduled Today at 8:30 a.m. EST -

LEXINGTON, Mass.--(BUSINESS WIRE)-- Concert Pharmaceuticals, Inc. (NASDAQ:CNCE) today reported financial results for the year ended December 31, 2016, as well as provided an update on its product pipeline and corporate activities.

"We are excited to continue the progression of our clinical candidates, CTP-656 and CTP-543. These two drug candidates, CTP-656 for cystic fibrosis and CTP-543 for alopecia areata, further our goal of developing innovative treatments with the potential to provide important benefits to patients," said Roger Tung, Ph.D. President and Chief Executive Officer of Concert Pharmaceuticals. "I am very pleased that Vertex intends to integrate CTP-656 within their portfolio of investigational and approved CF medicines. We believe this agreement provides the optimal pathway to broadly and rapidly advance the development of CTP-656 for patients."

Recent Business Highlights and Upcoming Milestones

- ***CTP-656 Asset Purchase Agreement.*** On March 6, 2017, Concert announced that it entered into an asset purchase agreement worth up to $250 million with Vertex Pharmaceuticals, Inc., under which Vertex will acquire all rights to CTP-656 and other assets related to cystic fibrosis. The asset purchase agreement is subject to the approval of Concert's shareholders and other customary conditions. As part of the agreement, Vertex will pay Concert $160 million in cash upon closing for all worldwide development and commercialization rights to CTP-656. If CTP-656 is approved as part of a combination regimen to treat CF, Concert could receive up to an additional $90 million in milestones based on regulatory approval in the U.S. and reimbursement in the UK, Germany or France.
- ***CTP-656 Phase 2 Development for Cystic Fibrosis in U.S.*** Concert is conducting a randomized, placebo-controlled Phase 2 clinical trial evaluating CTP-656 in the U.S. in cystic fibrosis patients with gating mutations. The Company continues to expect topline data by year end 2017.
- ***CTP-656 Orphan Drug Designation.*** In January 2017, the U.S. Food and Drug Administration (FDA) granted orphan drug designation for CTP-656. The Orphan Drug Act provides incentives for companies to develop products for rare diseases affecting fewer than 200,000 people in the United States.
- ***CTP-543 Phase 1 Evaluation Complete.*** The Phase 1 single and multiple ascending dose trial evaluating CTP-543 in healthy volunteers is complete. The pharmacokinetic measurements showed increased exposure with increasing doses of CTP-543. In the study, CTP-543 was well tolerated across all dose groups and there were no serious adverse events reported in subjects who received CTP-543. The Phase 1 pharmacokinetic findings were presented at the American Academy of Dermatology Annual Meeting taking place March 3-7, 2017 in Orlando, FL.
- ***CTP-543 Phase 1 Crossover Study***. In a Phase 1 crossover study evaluating the metabolite profiles of CTP-543 and ruxolitinib, as previously demonstrated with other deuterium-modified compounds, the Company confirmed that, except for the presence of deuterium, no new metabolites were observed with CTP-543.
- ***CTP-543 Phase 1 Pharmacodynamic Results.*** In the multiple ascending dose Phase 1 trial of CTP-543, pharmacodynamic analyses were performed to assess the inhibition of IL-6- and IFN-γ-mediated JAK/STAT signaling. Consistent with the established pharmacological activity of CTP-543, a dose-related reduction in IL-6-stimulated phosphorylated STAT3 was observed. Also, IFN-γ-mediated STAT1 signaling, which is believed to play a key role in the pathogenesis of alopecia areata, was significantly inhibited in disease-relevant immune cell types at all doses evaluated.
- ***CTP-543 Phase 2 Initiation.*** The Company intends to advance CTP-543 into a Phase 2a trial in patients with moderate-to-severe alopecia areata in the first quarter of 2017. Topline data from the trial is expected by year-end 2017. The primary outcome measure of the Phase 2a trial will be the effect on treating hair loss as measured by the Severity of Alopecia Tool (SALT) after 24 weeks of dosing. The trial will include an additional 28 weeks of dosing where all patients enrolled in the study will receive CTP-543.

- ***General Counsel Transition***. Dr. Robert Silverman, General Counsel of Concert, has decided to transition from the Company. Effective June 1, 2017, Lynette Herscha, Vice President, Legal Affairs and Associate General Counsel, will be promoted to General Counsel and Secretary, succeeding Dr. Silverman. Dr. Silverman will remain a part-time employee with the Company in a senior legal advisory role.

Full Year 2016 Financial Results

- ***Cash and Investments Position.*** Cash, cash equivalents and investments as of December 31, 2016, totaled $96.2 million as compared to $142.2 million as of December 31, 2015. Concert expects its cash, cash equivalents and investments as of December 31, 2016, to be sufficient to fund the Company through the second quarter of 2018. Upon closing of the CTP-656 asset purchase agreement, pro forma cash is expected to be sufficient to fund the Company into 2021.
- ***Revenues.*** Revenue was $174,000 for the year ended December 31, 2016, compared to $66.7 million for the year ended December 31, 2015. The decrease in revenue relates primarily to a one-time $50.2 million change in control payment received from Auspex Pharmaceuticals in June 2015 as a result of their acquisition by Teva Pharmaceuticals Ltd. The decrease in revenue also relates to the completion of the Phase 1 clinical evaluation under our strategic collaborations with Celgene Corporation and Jazz Pharmaceuticals in 2015.
- ***R&D Expenses.*** Research and development expenses were $37.0 million for the year ended December 31, 2016, compared to $28.9 million for the year ended December 31, 2015, an increase of $8.1 million. The increase in research and development expenses was primarily due to expenses associated with the development of the Company's proprietary programs CTP-656 and CTP-543.
- ***G&A Expenses.*** General and administrative expenses were $14.4 million for the year ended December 31, 2016, compared to $13.1 million for the year ended December 31, 2015, an increase of $1.3 million. The increase in general and administrative expenses was primarily attributable to a $1.2 million increase in non-cash stock-based compensation expense.
- ***Net (Loss) Income.*** For the year ended December 31, 2016, net loss was $50.7 million, or $2.28 per basic and diluted share, as compared to net income of $24.2 million, or $1.14 and $1.09 per basic and diluted share, respectively, for the year ended December 31, 2015.

Conference Call and Webcast

The Company will host a conference call and webcast today at 8:30 a.m. EST to provide an update on the Company and discuss full year 2016 financial results. To access the conference call, please dial (855) 354-1855 (U.S. and Canada) or (484) 365-2865 (International) five minutes prior to the start time.

A live webcast may be accessed in the Investors section of the company's website at www.concertpharma.com. Please log on to the Concert website approximately 15 minutes prior to the scheduled webcast to ensure adequate time for any software downloads that may be required. A replay of the webcast will be available on Concert's website for three months.

Concert Pharmaceuticals, Inc.
Condensed Consolidated Statements of Operations
(in thousands, except per share amounts)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2016	2015	2016	2015
Revenue:				
License and research and development revenue	$ 21	$ 305	$ 174	$ 6,574
Other revenue	—	—	—	50,155
Milestone revenue	—	10,000	—	10,000
Total revenue	21	10,305	174	66,729
Operating expenses:				
Research and development	8,658	6,386	36,983	28,885
General and administrative	3,515	3,256	14,358	13,056
Total operating expenses	12,173	9,642	51,341	41,941
(Loss) Income from operations	(12,152)	663	(51,167)	24,788
Interest and other income (expense), net	109	44	447	(185)

(Loss) Income before income taxes	(12,043)	707	(50,720)	24,603
Provision for income taxes	—	23	—	429
Net (loss) income	$ (12,043)	$ 684	$ (50,720)	$ 24,174
Net (loss) income per share applicable to common stockholders - basic	$ (0.54)	$ 0.03	$ (2.28)	$ 1.14
Net (loss) income per share applicable to common stockholders - diluted	$ (0.54)	$ 0.03	$ (2.28)	$ 1.09
Weighted-average number of common shares used in net (loss) income per share applicable to common stockholders - basic	22,287	22,123	22,233	21,152
Weighted-average number of common shares used in net (loss) income per share applicable to common stockholders - diluted	22,287	23,302	22,233	22,267

Concert Pharmaceuticals, Inc.
Summary Balance Sheet Data
(in thousands)

	December 31, 2016	December 31, 2015
Cash and cash equivalents	$ 40,555	$ 92,510
Investments, available for sale	55,630	49,680
Working capital	92,159	137,481
Total assets	100,395	146,932
Deferred revenue	10,050	10,170
Total stockholders' equity	$ 85,594	$ 130,635

About CTP-656 and Cystic Fibrosis

CTP-656 is a novel CFTR potentiator that may offer next generation, once-daily dosing and was developed by Concert's novel application of deuterium chemistry to modify ivacaftor. Ivacaftor is marketed by Vertex Pharmaceuticals under the brand name Kalydeco. Concert is initially developing CTP-656 as a potential monotherapy treatment for cystic fibrosis due to gating mutations of the gene that encodes for the cystic fibrosis transmembrane conductance regulator (CFTR), a protein, which regulates components of sweat, mucus clearance and digestion.

Cystic fibrosis is a life-threatening, hereditary genetic disease that has systemic effects and can cause significantly reduced lung and digestive system function. According to the Cystic Fibrosis Foundation, an estimated 70,000 people worldwide have cystic fibrosis.

About CTP-543 and Alopecia Areata

CTP-543 was discovered by applying Concert's deuterium chemistry technology to modify ruxolitinib, which is commercially available under the name Jakafi® in the United States for the treatment of certain blood disorders. Ruxolitinib has been used to treat alopecia areata in academic settings, including an investigator-sponsored clinical trial, and has been shown to promote hair growth in individuals with moderate to severe disease.

Alopecia areata is an autoimmune disease that results in partial or complete loss of hair on the scalp and body that may affect up to 650,000 Americans at any given time. The scalp is the most commonly affected area, but any hair-bearing site can be affected alone or together with the scalp. Onset of the disease can occur throughout life and affects both women and men. Alopecia areata can be associated with serious psychological consequences, including anxiety and depression. There are currently no drugs approved by the U.S. Food and Drug Administration (FDA) for the treatment of alopecia areata.

Additional Information about the Transactions and Where to Find It

This press release is being made in respect of the proposed asset sale with Vertex. The proposed asset sale and the asset purchase agreement will be submitted to the shareholders of the Company for their consideration and approval. In connection with the proposed asset sale, the Company will file a proxy statement with the SEC. This press release does not constitute a solicitation of any vote or proxy from any shareholder of the Company. Investors are urged to read the proxy statement carefully and in its entirety when it becomes available and any other relevant documents or materials filed or to be filed with the SEC or incorporated by reference in the proxy statement, because they will contain important information about the proposed asset sale. The definitive proxy statement will be mailed to the Company's shareholders. In addition, the

proxy statement and other documents will be available free of charge at the SEC's internet website, www.sec.gov. When available, the proxy statement and other pertinent documents may also be obtained free of charge at the Investors section of the Company's website, www.concertpharma.com, or by directing a written request to Concert Pharmaceuticals, Inc., Attn: Investor Relations, in writing, at 99 Hayden Ave, #500, Lexington, MA 02421.

The Company and its directors, executive officers and other members of management and employees may be deemed to be participants in the solicitation of proxies in connection with the proposed asset sale. Information about the Company's directors and executive officers is included in the Company's Annual Report on Form 10-K for the year ended December 31, 2016 filed with the SEC on March 6, 2017. Additional information regarding these persons and their interests in the transaction will be included in the proxy statement relating to the proposed asset sale when it is filed with the SEC. These documents can be obtained free of charge from the sources indicated above.

About Concert

Concert Pharmaceuticals is a clinical stage biopharmaceutical company focused on applying its DCE Platform® (deuterated chemical entity platform) to create novel medicines designed to address unmet patient needs. The Company's approach starts with approved drugs in which deuterium substitution has the potential to enhance clinical safety, tolerability or efficacy. Concert has a broad pipeline of innovative medicines targeting pulmonary diseases, including cystic fibrosis, autoimmune and inflammatory diseases and central nervous systems (CNS) disorders. For more information please visit www.concertpharma.com.

Cautionary Note on Forward Looking Statements

Any statements in this press release about our future expectations, plans and prospects, including statements about the asset purchase agreement, including risks related to the satisfaction of the conditions to closing the acquisition, the clinical development of our therapeutic candidates and expectations regarding the sufficiency of our cash balance to fund operating expenses and capital expenditures, and other statements containing the words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: the uncertainties inherent in the initiation of future clinical trials, availability and timing of data from ongoing and future clinical trials and the results of such trials, whether preliminary results from a clinical trial will be predictive of the final results of that trial or whether results of early clinical trials will be indicative of the results of later clinical trials, expectations for regulatory approvals, availability of funding sufficient for our foreseeable and unforeseeable operating expenses and capital expenditure requirements and other factors discussed in the "Risk Factors" section of our most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission and in other filings that we make with the Securities and Exchange Commission. In addition, any forward-looking statements included in this press release represent our views only as of the date of this release and should not be relied upon as representing our views as of any subsequent date. We specifically disclaim any obligation to update any forward-looking statements included in this press release.

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Investors
Concert Pharmaceuticals, Inc.
Justine Koenigsberg, 781-674-5284
ir@concertpharma.com
or
Media
The Yates Network
Kathryn Morris, 845-635-9828

Source: Concert Pharmaceuticals, Inc.