Concert Pharmaceuticals Reports Third Quarter 2021 Financial Results and Provides Company Update

November 9, 2021

CTP-543 THRIVE-AA1 Phase 3 Trial in Patients with Moderate to Severe Alopecia Areata Fully Enrolled with Topline Data Expected Second Quarter of 2022

CTP-543 THRIVE-AA2 Phase 3 Trial in Patients with Moderate to Severe Alopecia Areata Progressing with Topline Data Expected Second Half of 2022

CTP-543 Treatment Shows Maintenance of Hair Regrowth in Open Label Extension Trial

Conference Call Scheduled Today at 8:30 a.m. ET

LEXINGTON, Mass.--(BUSINESS WIRE)--Nov. 9, 2021-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today reported financial results for the third quarter of 2021.

“The CTP-543 pivotal program is progressing on track to support an NDA filing with the FDA in early 2023,” said Roger Tung, Ph.D., President and Chief Executive Officer of Concert Pharmaceuticals. “We continue to believe that CTP-543, with its Breakthrough Therapy designation, has the potential to be a best in class compound to treat this challenging autoimmune disease and hope to make it available to patients as soon as possible.”

Recent Business Highlights and Upcoming Milestones

CTP-543: An Investigational Treatment in Pivotal Clinical Program for Moderate to Severe Alopecia Areata

- ***Completed Enrollment in the CTP-543 THRIVE-AA1 Phase 3 Trial in October 2021.*** THRIVE-AA1 is a randomized, double-blind, placebo-controlled Phase 3 clinical trial of CTP-543 to evaluate hair regrowth using the Severity of Alopecia Tool (SALT) after 24 weeks of dosing. The study enrolled 708 adult patients with moderate to severe alopecia areata. The trial is evaluating 8 mg and 12 mg twice-daily doses of CTP-543 compared to placebo at sites in the U.S., Canada and Europe. The Company expects to report topline results from the THRIVE-AA1 trial in the second quarter of 2022.

- ***CTP-543 THRIVE-AA2 Phase 3 Trial is Progressing.*** THRIVE-AA2 is a randomized, double-blind, placebo-controlled Phase 3 clinical trial of CTP-543 to evaluate hair regrowth using SALT after 24 weeks of dosing in approximately 440 adult patients with moderate to severe alopecia areata. The trial is evaluating 8 mg and 12 mg twice-daily doses of CTP-543 compared to placebo at sites in the U.S., Canada and Europe. The Company expects to report topline results from the THRIVE-AA2 trial in the second half of 2022.

- ***CTP-543 Long-term Data Presented at JAK Inhibitors Drug Development Summit in Q3 2021.*** Data from an ongoing open label, long-term extension study with CTP-543 were presented at the 2nd JAK Inhibitors Drug Development Summit on July 1, 2021. The presentation highlighted that in the extension study, treatment with CTP-543 showed continued maintenance of hair regrowth relative to the hair growth shown in the Company’s previously conducted Phase 2 trials in patients with alopecia areata. Approximately 57% of participants receiving 12 mg of CTP-543 twice-daily following 52 weeks of dosing achieved a clinically-meaningful SALT score of 20 or less. A SALT score ≤ 20 corresponds to 80% or more hair coverage on the scalp. CTP-543 has been generally well tolerated in the extension study, and adverse events are consistent with those reported in the Phase 2 trials.

- ***New Publication Highlights Adoption of Alopecia Areata Disease Severity Scale.*** Concert participated as a co-author in a new publication in the *Journal of the American Academy of Dermatology (JAAD)* that highlights an academic-industry collaborative effort for the development of a new alopecia areata disease severity scale for use in clinical practice. The new alopecia areata disease severity scale, anchored in the amount of hair loss, captures key features commonly used by experts in clinical practice, and is intended to help physicians to easily and accurately assess alopecia areata severity in patients.

Third Quarter 2021 Financial Results

- ***Cash and Investment Position.*** Cash, cash equivalents and investments as of September 30, 2021 totaled $103.7 million as compared to $130.0 million as of December 31, 2020. In November 2021, the Company raised gross proceeds of $65 million under a financing agreement with BVF Partners L.P. and RA Capital Management. The financing consists of the sale of common and preferred stock, warrants and a portion of Concert’s right to receive potential future AVP-786 royalties under an existing licensing agreement with Avanir Pharmaceuticals, Inc. Concert will have the potential to receive an additional $103 million upon the full exercise of warrants issued in connection with the agreement. Under its current

operating plan, the Company expects its cash and cash equivalents to fund the Company into the fourth quarter of 2022.

- ***Revenues.*** For the quarter ended September 30, 2021, revenue was $0.5 million, compared to $1.5 million for the same period in 2020.
- ***R&D Expenses.*** Research and development expenses were $21.9 million for the quarter ended September 30, 2021, compared to $16.3 million for the same period in 2020. The increase in research and development expenses relates primarily to the clinical development for CTP-543.
- ***G&A Expenses.*** General and administrative expenses were $5.5 million for the quarter ended September 30, 2021, compared to $4.5 million for the same period in 2020.
- ***Net Loss.*** For the quarter ended September 30, 2021, net loss applicable to common stockholders was $26.7 million, or $0.78 per share, as compared to net loss applicable to common stockholders of $18.9 million, or $0.60 per share, for the quarter ended September 30, 2020.

Conference Call and Webcast

The Company will host a conference call and webcast today at 8:30 a.m. ET to provide an update on the Company and discuss third quarter 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 of the third quarter financial results 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.

– Financial Tables to Follow –

Concert Pharmaceuticals, Inc.

Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	**2020**	**2021**	**2020**
Revenue:				
License and research and development revenue	$ 4	$ 1,501	$ 26	$ 7,895
Other revenue	539	—	32,539	—
Total revenue	543	1,501	32,565	7,895
Operating expenses:				
Research and development	21,876	16,347	60,560	45,121
General and administrative	5,462	4,514	16,561	13,917
Total operating expenses	27,338	20,861	77,121	59,038

Loss from operations	(26,795)	(19,360)	(44,556)	(51,143)
Investment income	4	183	44	1,101
Unrealized gain (loss) on marketable equity securities	113	269	590	(2,419)
Loss before income taxes	(26,678)	(18,908)	(43,922)	(52,461)
Income tax benefit	—	—	—	(85)
Net loss	$ (26,678)	$ (18,908)	$ (43,922)	$ (52,376)
Net loss per share applicable to common stockholders - basic and diluted	$ (0.78)	$ (0.60)	$ (1.29)	$ (1.71)
Weighted-average number of common shares used in net loss per share applicable to common stockholders - basic and diluted	34,090	31,547	33,987	30,707

Concert Pharmaceuticals, Inc.

Summary Balance Sheet Data

(in thousands)

(unaudited)

	September 30, 2021	**December 31, 2020**
Cash and cash equivalents	$ 103,664	$ 77,202
Investments, available for sale	—	52,766
Working capital	100,887	132,546
Total assets	128,208	159,263
Deferred revenue	2,750	2,750
Total stockholders' equity	99,162	131,162

About Concert

Concert Pharmaceuticals is a clinical stage biopharmaceutical company that is developing small molecule drugs that it discovered through the application of its DCE Platform® (deuterated chemical entity platform). Selective incorporation of deuterium into known molecules has the potential, on a case-by-case basis, to provide better pharmacokinetic or metabolic properties, thereby enhancing their clinical safety, tolerability or efficacy. Concert's lead product candidate is in late-stage development for the treatment of alopecia areata, a serious autoimmune dermatological condition. Concert is also assessing a number of earlier-stage pipeline candidates. For more information please visit www.concertpharma.com or follow us on Twitter at @ConcertPharma or on LinkedIn.

Cautionary Note on Forward Looking Statements

Any statements in this press release about our future expectations, plans and prospects, including, among others, statements about our expectations regarding the development of CTP-543, the timing of availability of clinical trial data, the timing of regulatory filings and the sufficiency of our cash, cash equivalents and investments to fund our operations, and any other statements containing the words "anticipate," "believe," "continue," "could,"

“estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “will,” “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, timing and design of future clinical trials, the 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, expectations with respect to the protection of our intellectual property afforded by our patents and other factors discussed in the “Risk Factors” section of our most recent Quarterly Report on Form 10-Q 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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