KemPharm Reports Third Quarter 2022 Results

November 9, 2022

Conference Call and Live Audio Webcast with Slide Presentation Scheduled for Today, November 9, 2022, at 5:00 p.m. ET

- Significant activities underway for preparation of the arimoclomol NDA resubmission
- Advancing key activities to initiate a Phase 2 clinical trial of KP1077 in IH by year-end 2022
- Total cash (cash, cash equivalents and long-term investments) of $107.4M as of Sep 30, 2022; based on current operating forecast, cash runway extends into 2026

CELEBRATION, Fla., Nov. 09, 2022 (GLOBE NEWSWIRE) -- KemPharm, Inc. (NasdaqGS: KMPH) (KemPharm, or the Company), a biotechnology company focused on the discovery, development and commercialization of novel treatments for rare central nervous system (CNS) and neurodegenerative diseases, lysosomal storage disorders and related treatment areas, today reported its financial results for the quarter ended September 30, 2022.

"During Q3, we made substantial progress with our two lead programs, arimoclomol, our NDA-stage product candidate for Niemann-Pick Type C (NPC), an ultra-rare lysosomal disease, and KP1077, our product candidate based on our prodrug of d-methylphenidate, serdexmethylphenidate (SDX), which is intended for the treatment of two rare sleep disorders, idiopathic hypersomnia (IH) and narcolepsy," stated Travis Mickle, Ph.D., President and Chief Executive Officer of KemPharm. "For arimoclomol, our team has made considerable progress characterizing the substantial data repository and generating a host of summary reports designed to present meaningful evidence of safety and efficacy as part of the NDA resubmission. Based on the recent completion of the 4-year open-label safety trial, the ongoing and constructive dialogue with the FDA and the new wealth of data generated since the CRL, we now anticipate resubmitting the updated NDA as early as Q3 2023. And, while no new or unanticipated issues related to resubmission have arisen, we believe the added time will be well-spent in preparation of an NDA filing with the highest likelihood of approval."

Dr. Mickle continued, "For KP1077, the initial results we reported in October 2022 from the Phase 1 cardiovascular safety trial of SDX demonstrated the potential for 'higher dose' SDX to be safe and well-tolerated. We believe this could position KP1077 as an advancement in the treatment of IH, and we remain on track to initiate the Phase 2 clinical trial by the end of 2022."

Dr. Mickle concluded, "We believe KemPharm is well-positioned with a strong investment thesis that we expect to be validated as the Company executes on a deep and differentiated development pipeline supported by a strong operational and financial foundation. This includes a cash runway that is forecasted to extend into 2026, which could be further bolstered by the potential to realize sales milestone and royalty revenue from AZSTARYS® as Corium executes its commercialization strategy. Altogether, we believe there are multiple catalysts for KemPharm during the remainder of 2022 and throughout 2023."

Recent Business and Corporate Highlights:

- Continuing activities to bolster the arimoclomol New Drug Application (NDA) for resubmission to the U.S. Food and Drug Administration (FDA):
    - Working to amass and characterize a substantial data repository from a 4-year arimoclomol safety study, and pinpointing key elements to include in the NDA resubmission for arimoclomol based on new data generated since June 2021;
    - Ongoing collaborative dialogue and periodic meetings with the FDA intended to ensure an optimal NDA data package that demonstrates arimoclomol to be a safe and effective therapy for NPC, if approved; and
    - Currently anticipating resubmission of the updated NDA as early as Q3 2023, with plans to provide updated guidance if needed based on ongoing dialogue with the FDA as we seek to compile an optimal data package for resubmission.
- Progress in advancing investigational candidate KP1077, an SDX-based product being developed as a treatment for IH and narcolepsy:
    - Completed Phase 1 cardiovascular safety clinical trial of SDX which confirmed the initial dosing strengths for the Phase 2 clinical trial of KP1077 in IH;

- Data suggest that SDX can be safely dosed at levels higher than currently available methylphenidate-based products, which is expected to result in improved efficacy while avoiding the potential for greater cardiovascular safety risk; and

- Preparing to initiate a Phase 2 clinical trial of KP1077 in patients with IH prior to year-end 2022 and a second trial in patients with narcolepsy in 2023.

- Strong operational and financial foundation, including $107.4 million in cash, cash equivalents and investments as of September 30, 2022:

  - Based on current operating forecast, cash runway is expected to continue into 2026; and

  - The potential to realize milestone and royalty revenue from AZSTARYS® as Corium executes its commercialization strategy could provide further capital flexibility and extend the operating cash runway.

Overview of Third Quarter 2022 Financial Results:

Net revenue for Q3 2022 was $2.9 million, as compared to Q3 2021 net revenue of $2.0 million. The period-over-period increase was primarily attributed to revenue from the arimoclomol Early Access Program (EAP) in France, partially offset by a decrease in revenue from consulting arrangements period over period.

Research and development expenses were $5.4 million for Q3 2022, as compared to $2.2 million in Q3 2021. The period-over-period increase was primarily driven by the KP1077 clinical development program, the arimoclomol program, increased depreciation/amortization related to the arimoclomol asset acquisition in the second quarter of 2022, and increased compensation costs, including non-cash stock-based compensation expense.

General and administrative expenses were $4.0 million for Q3 2022, as compared to $1.9 million in Q3 2021. The period-over-period increase was primarily driven by increased compensation costs, including non-cash stock-based compensation expense, as well as increased professional fees and depreciation/amortization related to the arimoclomol asset acquisition in the second quarter of 2022.

Net loss attributable to common stockholders for Q3 2022 was ($6.6) million, or ($0.19) per basic and diluted share, compared to a net loss attributable to common stockholders of ($1.8) million, or ($0.05) per basic and diluted share for the same period in 2021. Net loss for Q3 2022 was driven primarily by research and development expense of $5.4 million, and general and administrative expense of $4.0 million, partially offset by net revenues of $2.9 million.

As of September 30, 2022, total cash, cash equivalents and investments were $107.4 million, which was a decrease of $7.1 million compared to $114.5 million as of June 30, 2022, driven in part by increased third-party research and development costs related to the KP1077 clinical trial program, the arimoclomol program, other expenses, as well as investment of working capital related to the collection of accounts receivable due from French EAP reimbursements. Based on the Company's current operating forecast, existing cash, cash equivalents and investments are expected to be sufficient to continue operations into 2026.

As of September 30, 2022, total shares of common stock outstanding was 34,501,144 shares, and fully diluted common shares outstanding was 47,076,872 shares, which included 4,252,600 shares issuable upon exercise of warrants.

Conference Call Information:

KemPharm will host a conference call and live audio webcast with a slide presentation today at 5:00 p.m. ET, to discuss its corporate and financial results for the third quarter of 2022.

The audio webcast with slide presentation will be accessible via the Investor Relations section of the Company's website, http://investors.kempharm.com/. An archive of the webcast and presentation will be available for 90 days beginning at approximately 6:00 p.m. ET, on November 9, 2022.

Additionally, interested participants and investors may access conference call by dialing either:

- (800) 225-9448 (U.S.)
- (203) 518-9708 (International)
- Conference ID: KMPHQ322

About KemPharm:

KemPharm is a biotechnology company focused on the discovery, development and commercialization of novel treatments for rare CNS and neurodegenerative diseases, lysosomal storage disorders and related treatment areas. KemPharm has a diverse product portfolio, combining a clinical-stage development pipeline with NDA-stage and commercial assets. The pipeline includes arimoclomol, an orally-delivered, first-in-class investigational product candidate for Niemann-Pick disease type C (NPC), and KP1077, which the Company is developing as a treatment for idiopathic hypersomnia (IH), a rare neurological sleep disorder, and narcolepsy. In addition, the U.S. Food and Drug Administration (FDA) has approved AZSTARYS®, a once-daily treatment for

ADHD in patients age six years and older containing KemPharm's prodrug, serdexmethylphenidate (SDX), which is being commercialized by Corium, Inc. in the U.S. The FDA has also approved APADAZ®, an immediate-release combination product containing benzhydrocodone, KemPharm's prodrug of hydrocodone, and acetaminophen, which is being commercialized by KVK-Tech, Inc. in the U.S. For more information on KemPharm and its pipeline of product candidates visit www.kempharm.com or connect with us on Twitter, LinkedIn, Facebook and YouTube.

Early access programs are made available by KemPharm, Inc. and its affiliates, and are subject to the Company's Early Access Program (EAP) policy as published on its website at www.kempharm.com. Participation in these programs is subject to the laws and regulations of each jurisdiction under which each respective program is operated. Eligibility for participation in any such program is at the discretion of the treating physician.

Caution Concerning Forward Looking Statements:

This press release may contain forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements include all statements that do not relate solely to historical or current facts, including without limitation and which can be identified by the use of words such as "may," "will," "expect," "project," "estimate," "anticipate," "plan," "believe," "potential," "should," "continue," "could," "intend," "target," "predict," or the negative versions of those words or other comparable words or expressions, although not all forward-looking statements contain these identifying words or expressions. Forward-looking statements are not guarantees of future actions or performance. These forward-looking statements include statements regarding the promise and potential impact of our preclinical or clinical trial data, including without limitation the timing and results of any clinical trials or readouts, the timing or results of any Investigational New Drug applications and NDA submissions, including the resubmission of the NDA for arimoclomol, communications with the FDA, the potential uses or benefits of arimoclomol, KP1077, SDX, or any other product candidates for any specific disease indication or at any dosage, the potential benefits of any of KemPharm's product candidates, the sufficiency of cash to fund operations, the potential for and timing of milestone and/or royalty revenues, our plans or ability to seek funding, and our strategic and product development objectives. These forward-looking statements are based on information currently available to KemPharm and its current plans or expectations and are subject to a number of known and unknown uncertainties, risks and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. These and other important factors are described in detail in the "Risk Factors" section of KemPharm's Annual Report on Form 10-K for the year ended December 31, 2021, as updated by the Quarterly Report on Form 10-Q for the three months ended September 30, 2022, and KemPharm's other filings with the Securities and Exchange Commission. While we may elect to update such forward-looking statements at some point in the future, except as required by law, we disclaim any obligation to do so, even if subsequent events cause our views to change. Although we believe the expectations reflected in such forward-looking statements are reasonable, we can give no assurance that such expectations will prove to be correct. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to the date of this press release.

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KEMPHARM, INC.
UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(in thousands, except share and per share amounts)

	Three months ended September 30,		Nine months ended September 30,	
	2022	2021	2022	2021
Revenue, net	$ 2,874	$ 1,965	$ 8,139	$ 26,068
Operating expenses:				
Cost of revenue	141	0	200	2,000
Research and development	5,385	2,239	13,262	7,352
General and administrative	3,974	1,948	10,266	6,145
Acquired in-process research and development	—	—	17,663	—
Total operating expenses	9,500	4,187	41,391	15,497
(Loss) income from operations	(6,626)	(2,222)	(33,252)	10,571
Other (expense) income:				
Loss on extinguishment of debt	—	—	—	(16,096)
Interest expense related to amortization of debt issuance costs and discount	—	—	—	(150)

Interest expense on principal	(124)	(6)	(165)	(221)
Fair value adjustment related to derivative and warrant liability	22	332	295	(92)
Interest and other (expense) income, net	79	137	(152)	136
Total other (expense) income	(23)	463	(22)	(16,423)
Loss before income taxes	(6,649)	(1,759)	(33,274)	(5,852)
Income tax benefit	33	—	752	—
Net loss	$ (6,616)	$ (1,759)	$ (32,522)	$ (5,852)
Deemed dividend	—	—	—	(54,342)
Net loss attributable to common stockholders	$ (6,616)	$ (1,759)	$ (32,522)	$ (60,194)
Basic and diluted net loss per share of common stock:				
Net loss attributable to common stockholders	$ (0.19)	$ (0.05)	$ (0.94)	$ (2.16)
Weighted average number of shares of common stock outstanding:				
Basic and diluted	34,494,702	35,217,953	34,482,791	27,904,711

KEMPHARM, INC.
UNAUDITED CONDENSED CONSOLIDATED BALANCE SHEETS
(in thousands, except share and par value amounts)

	September 30, 2022	December 31, 2021
Assets		
Current assets:		
Cash and cash equivalents	$ 70,059	$ 112,346
Short-term investments	5,832	—
Accounts and other receivables	6,583	1,528
Prepaid expenses and other current assets	2,659	1,182
Total current assets	85,133	115,056
Inventories	596	—
Property and equipment, net	852	884
Operating lease right-of-use assets	1,068	1,141
Long-term investments	31,463	15,422
Other long-term assets	439	438
Total assets	$ 119,551	$ 132,941
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 4,279	$ 3,038
Current portion of operating lease liabilities	474	356
Current portion of discount and rebate liabilities	2,825	—
Other current liabilities	853	836
Total current liabilities	8,431	4,230
Line of credit payable	12,800	—
Derivative and warrant liability	35	330
Operating lease liabilities, less current portion	956	1,232
Discount and rebate liabilities, less current portion	3,509	—
Other long-term liabilities	26	31
Total liabilities	25,757	5,823
Commitments and contingencies (Note D)		
Stockholders' equity:		
Preferred stock:		
Undesignated preferred stock, $0.0001 par value, 10,000,000 shares authorized, no shares issued or outstanding as of September 30, 2022 or December 31, 2021	—	—

Common stock, $0.0001 par value, 250,000,000 shares authorized, 35,411,097 shares issued and 34,501,144 shares outstanding as of September 30, 2022; 35,325,801 shares issued and 35,005,640 shares outstanding as of December 31, 2021	3	4
Additional paid-in capital	400,677	396,957
Treasury stock, at cost	(7,536)	(2,814)
Accumulated deficit	(299,551)	(267,029)
Accumulated other comprehensive income	201	—
Total stockholders' equity	93,794	127,118
Total liabilities and stockholders' equity	$ 119,551	$ 132,941

Source: KemPharm