KemPharm Reports Second Quarter 2022 Financial Results and Corporate Updates

August 11, 2022

Conference Call and Live Audio Webcast with Slide Presentation Scheduled for Today, August 11, 2022, at 5:00 p.m. ET

Perry Sternberg, Corium, Inc.'s President and CEO, to Participate and Provide Update on the Commercialization of AZSTARYS ®

- Acquired substantially all the assets and operations of Orphazyme A/S, including arimoclomol, for a cash payment of $12.8 million
- Resubmission of the arimoclomol NDA for the treatment of NPC as early as Q1 2023
- Filed IND application for KP1077, an SDX-based product candidate for the treatment of IH
- Initiation of a Phase 2 trial (KP1077.D01) expected by the end of 2022
- Topline data from Phase 1 clinical trial evaluating cardiovascular safety of SDX expected as early as Q3 2022
- Total cash, cash equivalents and investments were $114.5 million as of June 30, 2022

CELEBRATION, Fla., Aug. 11, 2022 (GLOBE NEWSWIRE) -- KemPharm, Inc. (NasdaqGS: KMPH) (KemPharm, or the Company), a specialty pharmaceutical company focused on the discovery, development and commercialization of novel treatments for rare central nervous system (CNS), neurodegenerative and lysosomal storage diseases, today reported its financial results for the second quarter ended June 30, 2022.

"KemPharm's recent acquisition of arimoclomol, along with substantially all of Orphazyme's assets and operations, provided an exclamation point to the first half of 2022 as we shifted our strategic focus to the development of therapies targeting rare central nervous system (CNS), neurodegenerative, and lysosomal storage diseases," stated Travis Mickle, Ph.D., President and Chief Executive Officer of KemPharm. "We believe this orientation towards rare disease classification with limited treatment options and no approved treatments in the U.S. provides a significant opportunity for KemPharm to make an impact in an area of high-need and to validate the potential of our strategy to create both near and longer-term shareholder value."

Dr. Mickle continued, "The acquisition of arimoclomol is a unique and potentially game-changing opportunity for KemPharm. Arimoclomol is an NDA-stage product candidate being developed for the treatment of Niemann-Pick disease type C (NPC), a rare neurodegenerative disease for which no approved therapy exists in the U.S. We acquired this asset for total consideration of $18.0 million, which included a cash payment of $12.8 million and the assumption of an estimated reserve liability equal to approximately $5.2 million. The cash payment was funded through a line of credit secured by our balance sheet, making this transaction very capital efficient. Another important part of this transaction is maintaining the early access programs in the U.S. and the E.U. while we support the ongoing work of seeking regulatory approval. KemPharm is concentrating more of our resources towards the resubmission of the arimoclomol NDA with the U.S. Food and Drug Administration (FDA), which we expect to complete as early as the first quarter of 2023."

Dr. Mickle continued, "In parallel with our work on arimoclomol is the ongoing development of KP1077, our lead clinical candidate, which we are advancing as a treatment for idiopathic hypersomnia (IH) and narcolepsy. As announced in May, the Investigational New Drug (IND) application to initiate a clinical program investigating KP1077 for the treatment of IH has been successfully filed with the FDA. We expect to initiate the Phase 2 trial of KP1077 in IH by the end of 2022, with a second trial focused on narcolepsy commencing soon thereafter. Additionally, we expect to report topline results from the cardiovascular safety study involving serdexmethylphenidate (SDX) as soon as Q3 2022. We believe that demonstrating an improved cardiovascular safety profile compared to current stimulants could be a key potential differentiator for KP1077."

Dr. Mickle continued, "The execution of our strategy is continuing as KemPharm seeks to build a diverse and unique product portfolio combining an NDA-stage product with a rapidly advancing clinical-stage pipeline targeting multiple disease indications. Additionally, KemPharm continues to be excited by the commercialization of AZSTARYS® by Corium. We are pleased that Perry Sternberg, Corium's President and CEO, will join our second quarter results conference call to discuss ongoing commercialization activities and review the substantial progress made, including the recent national expansion of the launch of AZSTARYS."

Dr. Mickle concluded, "Looking ahead, we believe KemPharm is well positioned for growth on multiple fronts, while possessing a strong operational and financial foundation, including $114.5 million in cash, cash equivalents and investments as of June 30, 2022. We believe these attributes, combined with the numerous milestone opportunities anticipated for 2022 and beyond, position KemPharm for continued growth despite current macroeconomic and global equity market challenges. This is a very exciting time for KemPharm."

Q2 2022 Financial Results:

KemPharm's net revenue for Q2 2022 was $1.3 million, as compared to Q2 2021 net revenue of $12.0 million. The Q2 2021 net revenue included a one-time regulatory payment of $10 million for the DEA scheduling of AZSTARYS.

Research and development expenses were $4.8 million for Q2 2022, as compared to $2.8 million in Q2 2021, driven primarily by spending on the KP1077 clinical development program and, increased compensation costs, including non-cash stock-based compensation expense.

General and administrative expenses were $3.6 million for Q2 2022, as compared to $2.3 million in Q2 2021. The period-over-period increase was primarily driven by increased compensation costs, including non-cash stock-based compensation expense.

In addition, KemPharm recognized $17.7 million of expense during Q2 2022 related to acquired in-process research and development from the arimoclomol asset acquisition during the quarter, which was immediately expensed.

Net loss attributable to common stockholders for Q2 2022 was ($24.0) million, or ($0.70) per basic and diluted share, compared to a net loss attributable to common stockholders of ($10.7) million, or ($0.40) per basic and diluted share for the same period in 2021. Net loss for Q2 2022 was driven primarily by the one-time non-cash expense recognized in Q2 2022 for the arimoclomol asset acquisition of $17.7 million, research and development expense of $4.8 million, and general and administrative expense of $3.6 million, partially offset by an income tax benefit of $0.7 million. Excluding the one-time $17.7 million of non-cash expense related to the arimoclomol asset acquisition recognized during Q2 2022, adjusted net loss was ($6.4) million, or ($0.19) per basic and diluted share.

As of June 30, 2022, total cash, cash equivalents and investments were $114.5 million, which was a decrease of $4.6 million compared to $119.1 million as of March 31, 2022, driven in part by increased spending on third-party research and development costs related to the KP1077 clinical trial program, and other expenses related to the arimoclomol asset acquisition. Based on the Company's current operating forecast, existing cash, cash equivalents and investments are expected to be sufficient to continue operations beyond 2025.

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 second 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 August 11, 2022.

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

- (800) 245-3047 (U.S.)
- (203) 518-9765 (International)
- Conference ID: KMPHQ222

About KemPharm:

KemPharm is a specialty pharmaceutical company focused on the discovery, development and commercialization of novel treatments for rare central nervous system (CNS), neurodegenerative and lysosomal storage diseases. 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., and 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.

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 IND applications and NDA submissions, including the resubmission of the NDA for arimoclomol, 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 success or timing of the launch or commercialization of AZSTARYS or any other products or related sales milestones, the sufficiency of cash to fund operations, 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 June 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.

KemPharm Contacts:

Tiberend Strategic Advisors, Inc.
Jason Rando/Daniel Kontoh-Boateng
jrando@tiberend.com
dboateng@tiberend.com

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

	Three months ended June 30,		Six months ended June 30,	
	2022	2021	2022	2021
Revenue, net	$ 1,300	$ 11,986	$ 5,265	$ 24,103
Operating expenses:				
Cost of revenue	51	1,000	59	2,000
Research and development	4,795	2,848	7,877	5,113
General and administrative	3,558	2,305	6,292	4,197
Acquired in-process research and development	17,663	—	17,663	—
Total operating expenses	26,067	6,153	31,891	11,310
(Loss) income from operations	(24,767)	5,833	(26,626)	12,793
Other income (expense):				
Gain (loss) on extinguishment of debt	—	789	—	(16,096)
Interest expense related to amortization of debt issuance costs and discount	—	—	—	(150)
Interest expense on principal	(36)	(16)	(41)	(215)
Fair value adjustment related to derivative and warrant liability	32	(394)	273	(424)
Interest and other income (expense), net	14	(9)	(231)	(1)
Total other income (expense)	10	370	1	(16,886)
(Loss) income before income taxes	(24,757)	6,203	(26,625)	(4,093)
Income tax benefit	715	—	719	—
Net (loss) income	$ (24,042)	$ 6,203	$ (25,906)	$ (4,093)
Deemed dividend	—	(16,898)	—	(54,342)
Net loss attributable to common stockholders	$ (24,042)	$ (10,695)	$ (25,906)	$ (58,435)
Basic and diluted net loss per share of common stock:				
Net loss attributable to common stockholders	$ (0.70)	$ (0.40)	$ (0.75)	$ (2.42)
Weighted average number of shares of common stock outstanding:				
Basic and diluted	34,447,206	29,174,565	34,476,737	24,187,484

KEMPHARM, INC.

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

	June 30, 2022	December 31, 2021
Assets		
Current assets:		
Cash and cash equivalents	$ 76,779	$ 112,346
Short-term investments	4,199	—
Accounts and other receivables	2,820	1,528
Prepaid expenses and other current assets	3,637	1,182
Total current assets	87,435	115,056
Inventories	779	—
Property and equipment, net	904	884
Operating lease right-of-use assets	1,165	1,141
Long-term investments	33,535	15,422
Other long-term assets	440	438
Total assets	$ 124,258	$ 132,941
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 3,600	$ 3,038
Current portion of operating lease liabilities	469	356
Current portion of discount and rebate liabilities	1,796	—
Other current liabilities	1,294	836
Total current liabilities	7,159	4,230
Line of credit payable	12,800	—
Derivative and warrant liability	57	330
Operating lease liabilities, less current portion	1,082	1,232
Discount and rebate liabilities, less current portion	3,900	—
Other long-term liabilities	27	31
Total liabilities	25,025	5,823
Stockholders' equity:		
Preferred stock:		
Undesignated preferred stock, $0.0001 par value, 10,000,000 shares authorized, no shares issued or outstanding as of June 30, 2022 (unaudited) or December 31, 2021	—	—
Common stock, $0.0001 par value, 250,000,000 shares authorized, 35,399,267 shares issued and 34,489,314 shares outstanding as of June 30, 2022 (unaudited); 35,325,801 shares issued and 35,005,640 shares outstanding as of December 31, 2021	3	4
Additional paid-in capital	399,701	396,957
Treasury stock, at cost	(7,536)	(2,814)
Accumulated deficit	(292,935)	(267,029)
Total stockholders' equity	99,233	127,118
Total liabilities and stockholders' equity	$ 124,258	$ 132,941

Source: KemPharm