Kadmon Announces Positive Topline Results from Phase 2 Study of KD025 in Idiopathic Pulmonary Fibrosis

February 12, 2018

-- Conference Call and Webcast Tomorrow at 8:30 a.m. ET --

NEW YORK--(BUSINESS WIRE)-- Kadmon Holdings, Inc. (NYSE:KDMN) today announced topline results from an ongoing Phase 2 clinical trial evaluating KD025, its Rho-associated coiled-coil kinase 2 (ROCK2) inhibitor, in patients with idiopathic pulmonary fibrosis (IPF) who were previously treated with or offered pirfenidone and/or nintedanib. KD025 was well tolerated and demonstrated clinical benefit, with a median decline in forced vital capacity (FVC), a measure of lung function, of 48 mL at week 24, compared to a median decline of 175 mL in patients treated with best supportive care (BSC), an absolute difference of 127 mL and a relative difference of 73%.

“KD025 represents a novel mechanism of action in IPF by inhibiting ROCK, a central regulator of fibrosis that mediates several pro-fibrotic responses, including stress fiber formation, myofibroblast activation and pro-fibrotic gene transcription,” said Kevin F. Gibson, MD, Professor and Medical Director, Dorothy P. and Richard P. Simmons Center for Interstitial Lung Disease, University of Pittsburgh Medical Center and lead investigator of the study. “In this proof-of-concept trial, KD025 has demonstrated clinical activity at 24 weeks and was well tolerated, with no apparent safety signals, potentially offering a new option for patients with IPF.”

In the open-label trial (KD025-207), patients who were previously treated with or offered pirfenidone and/or nintedanib were randomized 2:1 to receive KD025 400 mg QD monotherapy or BSC. The primary endpoints were safety and tolerability of KD025 and change in FVC from baseline to 24 weeks. Patients have the option to continue treatment with KD025 beyond 24 weeks.

As of a data cutoff date of February 1, 2018, 20 evaluable patients have completed 24 weeks of KD025 treatment, and 9 evaluable patients in the BSC arm have completed 24 weeks of follow-up. Approximately 44% of all patients enrolled in the trial had received prior treatment with pirfenidone and/or nintedanib. Following are key results:

- The median decline in FVC at 24 weeks was 48 mL in the KD025 arm, compared to a median decline of 175 mL in the BSC arm, a relative difference of 73%.
- The median decline in FVC % predicted from baseline to week 24 was 1% in KD025 patients, compared to a median decline of 2% in BSC patients, a relative difference of 50%.
- Treatment with KD025 reduced the proportion of patients who experienced IPF progression: At 24 weeks, 20% of KD025 patients experienced FVC % predicted decline ≥5%, compared to 44% of BSC patients, a relative difference of 55%.
- KD025 patients experienced less FVC decline on an annualized basis relative to the year prior to enrollment: Evaluable patients randomized to KD025 had an annualized decline in FVC of 126 mL in the year prior to randomization, compared to an annualized decline of 32 mL at 24 weeks of KD025 treatment.
- KD025 was well tolerated, with no drug-related serious adverse events. In addition, 90% of patients who received KD025 for 24 weeks have elected to continue KD025 treatment beyond week 24.

“We are pleased with today’s results, which demonstrate the activity and tolerability of KD025 in IPF, including in patients who have received prior therapy with approved agents,” said Harlan W. Waksal, M.D., President and CEO at Kadmon. “These findings support the therapeutic potential of ROCK inhibition in IPF and further validate Kadmon’s ROCK inhibitor platform, which is being applied across programs in fibrotic diseases as well as inflammatory diseases.”

Kadmon plans to present the results from this study at the American Thoracic Society (ATS) International Conference in May 2018.

Conference Call and Webcast

Kadmon will host a conference call and webcast on February 13, 2018, at 8:30 a.m. ET to discuss top-line results from the KD025-207 study. To access the webcast, please visit the Investors section of www.kadmon.com, under “Presentations & Events.” A replay of the webcast will be archived on the Company's website for 30 days.

Dial-in Information:
(866) 762-3021
Conference ID: 369-4419

About KD025

KD025 is a selective oral inhibitor of ROCK2, a signaling pathway involved in the pathogenesis of multiple chronic diseases. Published research by Kadmon and academic institutions has demonstrated that KD025 regulates fibrotic processes and aberrant immune responses. Kadmon is conducting Phase 2 clinical trials of KD025, including in idiopathic pulmonary fibrosis (IPF) and chronic graft-versus-host disease (cGVHD).

About IPF

IPF is a progressive fibrotic disease of the lungs, with a median survival of 3 to 5 years from the time of diagnosis. IPF is thought to be caused by...
repetitive environmental injury to the lining of the lung airways and the resulting abnormal wound-healing responses. IPF patients are in need of new therapies that provide clinical benefit.

About Kadmon Holdings, Inc.

Kadmon Holdings, Inc. is a fully integrated biopharmaceutical company developing innovative products for significant unmet medical needs. We have a product pipeline focused on fibrotic and inflammatory diseases.

Forward-Looking Statements

This press release contains forward-looking statements. Such statements may be preceded by the words “may,” “will,” “should,” “expects,” “plans,” “anticipates,” “could,” “intends,” “targets,” “projects,” “contemplates,” “believes,” “estimates,” “predicts,” “potential” or “continue” or the negative of these terms or other similar expressions. Forward-looking statements involve known and unknown risks, uncertainties 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. We believe that these factors include, but are not limited to, (i) the initiation, timing, progress and results of our preclinical studies and clinical trials, and our research and development programs; (ii) our ability to advance product candidates into, and successfully complete, clinical trials; (iii) our reliance on the success of our product candidates; (iv) the timing or likelihood of regulatory filings and approvals; (v) our ability to expand our sales and marketing capabilities; (vi) the commercialization of our product candidates, if approved; (vii) the pricing and reimbursement of our product candidates, if approved; (viii) the implementation of our business model, strategic plans for our business, product candidates and technology; (ix) the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates and technology; (x) our ability to operate our business without infringing the intellectual property rights and proprietary technology of third parties; (xi) costs associated with defending intellectual property infringement, product liability and other claims; (xii) regulatory developments in the United States, Europe and other jurisdictions; (xiii) estimates of our expenses, future revenues, capital requirements and our needs for additional financing; (xiv) the potential benefits of strategic collaboration agreements and our ability to enter into strategic arrangements; (xv) our ability to maintain and establish collaborations or obtain additional grant funding; (xvi) the rate and degree of market acceptance, if any, of our product candidates; (xvii) developments relating to our competitors and our industry, including competing therapies; (xviii) our ability to effectively manage our anticipated growth; (xix) our ability to attract and retain qualified employees and key personnel; and/or (xx) our ability to achieve cost savings and benefits from our efforts to streamline our operations and to not harm our business with such efforts. More detailed information about Kadmon and the risk factors that may affect the realization of forward-looking statements is set forth in the Company's filings with the U.S. Securities and Exchange Commission (“SEC”), including the Company's Quarterly Report on Form 10-Q filed pursuant to Section 13 of the Securities Exchange Act of 1934, as amended, with the SEC on November 9, 2017. Investors and security holders are urged to read these documents free of charge on the SEC's web site at www.sec.gov. The Company assumes no obligation to publicly update or revise its forward-looking statements as a result of new information, future events or otherwise.


Kadmon Holdings, Inc.
Ellen Tremaine, Investor Relations
646-490-2989
ellen.tremaine@kadmon.com

Source: Kadmon Holdings, Inc.