Kadmon Announces Publication of Phase 2a Clinical Trial Results of Belumosudil for cGVHD in the Journal of Clinical Oncology

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NEW YORK, NY / ACCESSWIRE / April 21, 2021 / Kadmon Holdings, Inc. (NASDAQ:KDMN) today announced the publication of results from KD025-208, the Phase 2a clinical trial of belumosudil (KD025) for chronic graft-versus-host disease (cGVHD). The results were published in the *Journal of Clinical Oncology*. A New Drug Application (NDA) for belumosudil is currently under Priority Review by the U.S Food and Drug Administration (FDA) for the treatment of patients with cGVHD, with a Prescription Drug User Fee Act (PDUFA) goal date of August 30, 2021.

"The results from our first clinical study in cGVHD demonstrated that belumosudil achieves meaningful, long-lasting responses and is very well tolerated in refractory cGVHD patients," said Harlan W. Waksal, M.D., President and CEO of Kadmon. "These positive results were replicated in the ROCKstar pivotal trial and support belumosudil's ability to offer meaningful clinical benefit, if approved, to patients with cGVHD, for which new therapies are urgently needed."

Results from KD025-208, the open-label, Phase 2a clinical trial of belumosudil, which enrolled 54 cGVHD patients who had received one to three prior lines of therapy, demonstrated a pooled Overall Response Rate (ORR) of 65% across the three patient cohorts. Responses were achieved across patient subgroups, including in patients with four or more organs involved. Responses were durable, with a median duration of 35 weeks in responders. In addition, 50% of responders experienced a clinically meaningful improvement in symptoms, as measured by at least a 7-point decrease in the Lee cGVHD Symptom Scale score. Belumosudil was also shown to be steroid sparing: 67% of all patients were able to reduce steroid doses and 19% of patients completely discontinued steroids. Belumosudil was well tolerated across all cohorts, with no drug-related serious adverse events and no increased risk of infection observed.

The publication, titled "ROCK2 Inhibition with Belumosudil (KD025) for the Treatment of Chronic Graft-Versus-Host Disease," is available online here as well as on the Kadmon website.

About Belumosudil

Belumosudil (KD025) is a selective oral inhibitor of Rho-associated coiled-coil kinase 2 (ROCK2), a signaling pathway that modulates inflammatory response and pro-fibrotic processes. The FDA granted Priority Review for the NDA for belumosudil for the treatment of cGVHD and assigned a PDUFA target action date of August 30, 2021. The NDA is being reviewed under the FDA's Real-Time Oncology Review (RTOR) and Project Orbis pilot programs. The FDA has granted Breakthrough Therapy Designation to belumosudil for the treatment of patients with cGVHD after failure of two or more lines of systemic therapy as well as Orphan Drug Designation to belumosudil for the treatment of cGVHD.

About cGVHD

cGVHD is a common and often fatal complication following hematopoietic stem cell transplantation. In cGVHD, transplanted immune cells (graft) attack the patient's cells (host), leading to inflammation and fibrosis in multiple tissues, including skin, mouth, eye, joints, liver, lung, esophagus and gastrointestinal tract. Approximately 14,000 patients in the United States are currently living with cGVHD.

About Kadmon

Kadmon is a clinical-stage biopharmaceutical company that discovers, develops and delivers transformative therapies for unmet medical needs. Kadmon's clinical pipeline includes treatments for immune and fibrotic diseases as well as immuno-oncology therapies.

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. Among those risks and uncertainties are risks related to market conditions, including market interest rates, and the trading price and volatility of Kadmon's common stock. 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) the impact of the COVID-19 pandemic on our business, workforce, patients, collaborators and suppliers, including delays in anticipated timelines and milestones of our clinical trials and on various government agencies who we interact with and/or are governed by; (iv) our reliance on the success of our product candidates; (v) the timing or likelihood of regulatory filings and approvals, especially in light of the COVID-19 pandemic; (vi) our ability to expand our sales and marketing capabilities; (vii) the commercialization, 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; (xvi) the rate and degree of market acceptance of our product candidates, if approved; (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; (xx) our expected use of cash and cash equivalents and other sources of liquidity;

(xxi) our expected use for the proceeds from the offering of our convertible senior notes; (xxii) the potential benefits of any of our product candidates being granted orphan drug designation; (xxiii) the future trading price of the shares of our common stock and impact of securities analysts' reports on these prices; (xxiv) our ability to apply unused federal and state net operating loss carryforwards against future taxable income and/or (xv) other risks and uncertainties. More detailed information about the Company 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 (the "SEC"), including Kadmon's Annual Report on Form 10-K for the fiscal year ended December 31, 2020. Investors and security holders are urged to read these documents free of charge on the SEC's website 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.

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