Kadmon Announces 12 Month Data from Pivotal Trial of Belumosudil for cGVHD at the 62nd ASH Annual Meeting

December 6, 2020

- ORRs of 73% and 77% with Belumosudil 200 mg QD and 200 mg BID, Respectively -

- cGVHD Key Opinion Leader Webcast Today at 2:15 p.m. Eastern Time (11:15 a.m. Pacific Time) -

NEW YORK, NY / ACCESSWIRE / December 6, 2020 / Kadmon Holdings, Inc. (Nasdaq:KDMN) today announced 12-month data from ROCKstar, its ongoing pivotal trial of belumosudil for the treatment of patients with chronic graft-versus-host disease (cGVHD), in an oral session at the 62nd American Society of Hematology (ASH) Annual Meeting.

At the 12-month follow-up analysis, belumosudil achieved clinically meaningful and statistically significant Overall Response Rates (ORRs) of 73% with 200 mg once daily (QD) (95% Confidence Interval (CI): 60%, 83%; p<0.0001) and 77% with 200 mg twice daily (BID) (95% CI: 65%, 87%; p<0.0001) in patients with cGVHD who have received at least two prior lines of systemic therapy. Responses were achieved across key patient subgroups and complete responses were observed in all organ systems, including in lung.

Belumosudil performed well across all secondary endpoints:

- Responses were durable, with a median duration of response of 50 weeks; 60% of responders have maintained their responses for 20 weeks or longer
- A Failure-Free Survival (FFS) rate of 58% was maintained at 12 months
- Clinically meaningful improvement from baseline in the Lee Symptom Scale (LSS) score, a quality-of-life measurement, was observed in 60% of patients
- 64% of patients were able to reduce their corticosteroid dose, with 21% of patients completely discontinuing corticosteroid therapy
- Belumosudil has been well tolerated and adverse events have been consistent with those expected in the cGVHD patient population

"The positive results achieved with belumosudil have continually demonstrated its ability to meaningfully address the broad spectrum of cGVHD manifestations, including difficult-to-treat organs like lung," said Corey Cutler, MD, MPH, FRCPC, Dana-Farber Cancer Institute. "If we were to translate this data to a real-world setting, with two-thirds of patients able to reduce corticosteroid doses and achieve 50-week median duration of responses, belumosudil, if approved, would potentially offer benefit to the thousands of patients currently living with cGVHD."

"We are extremely pleased with the 12-month results from this trial, as the Overall Response Rates have consistently strengthened, the durability has proven to be robust, the drug continues to perform across a number of key secondary endpoints and has been well tolerated," said Harlan W. Waksal, M.D., President and CEO at Kadmon. "With the FDA's recent acceptance of our NDA filing and a PDUFA date of May 30, 2021, we are moving forward with our commercial preparation efforts, having hired two-thirds of our planned field force. We look forward to continuing to work closely with the FDA as they complete their review of belumosudil in cGVHD."

Key Opinion Leader Event Details

Kadmon will host a key opinion leader event on December 6th at 2:15 p.m., Eastern time (11:15 a.m., Pacific time), to discuss the 12-month data of the ROCKstar trial. To listen online via webcast, please visit: https://openexc.zoom.us/webinar/register/WN_NbmeCyjmQ1iQVOvy0FlGaA

The event will be archived and will be available at https://investors.kadmon.com/presentations-and-events. Replays of the event will be available for 60 days.

About ROCKstar

ROCKstar (KD025-213) is an ongoing open-label trial of belumosudil in patients with cGVHD who have received at least two prior lines of systemic therapy. Patients were randomized to receive belumosudil 200 mg once daily or 200 mg twice daily, enrolling 66 patients per arm. The primary endpoint of the study is Overall Response Rate (ORR). The ORR endpoint was met at the interim analysis, conducted two months after completion of enrollment. At the study's follow-up analysis, conducted twelve months after completion of enrollment, belumosudil achieved ORRs of 73% and 77% in the respective arms. Belumosudil has been well tolerated and adverse events have been consistent with those expected in the patient population.

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. In November 2020, the U.S. Food and Drug Administration (FDA) accepted the NDA for belumosudil for the treatment of patients with cGVHD. The FDA granted Priority Review for the NDA for belumosudil and assigned a Prescription Drug User Fee Act (PDUFA) target action date of May 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. Our 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. 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) our ability to expand our sales and marketing capabilities; (viii) the commercialization, pricing and reimbursement of our product candidates, if approved; (ix) the implementation of our business model, strategic plans for our business, product candidates and technology; (x) the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates and technology; (xi) our ability to operate our business without infringing the intellectual property rights and proprietary technology of third parties; (xii) costs associated with defending intellectual property infringement, product liability and other claims; (xiii) regulatory developments in the United States, Europe, and other jurisdictions; (xiv) estimates of our expenses, future revenues, capital requirements and our needs for additional financing; (xv) the potential benefits of strategic collaboration agreements and our ability to enter into strategic arrangements; (xvi) our ability to maintain and establish collaborations; (xvii) the rate and degree of market acceptance of our product candidates, if approved; (xviii) developments relating to our competitors and our industry, including competing therapies; (xix) our ability to effectively manage our anticipated growth; (xx) our ability to attract and retain qualified employees and key personnel; (xxi) our expected use of cash and cash equivalents and other sources of liquidity; (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 (xxv) 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, 2019 and subsequent filings with the SEC. 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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