Kadmon Announces Pivotal Trial Data Published in the Journal Blood for REZUROCK(TM) (Belumosudil) in Chronic Graft-Versus-Host Disease (cGVHD)

July 19, 2021

- REZUROCK is approved by the U.S. FDA for the treatment of adult and pediatric patients 12 years and older with cGVHD after failure of at least two prior lines of systemic therapy -

NEW YORK, NY / ACCESSWIRE / July 19, 2021 / Kadmon Holdings, Inc. (NASDAQ:KDMN) today announced that data from the pivotal ROCKstar clinical trial of REZUROCK™ (belumosudil) for the treatment of chronic graft-versus-host disease (cGVHD) were published in the journa*Blood*. The ROCKstar data served as the basis for the Company's New Drug Application (NDA) for REZUROCK for the treatment of cGVHD, which was granted full approval by the U.S. Food and Drug Administration (FDA) on July 16, 2021 for the treatment of adult and pediatric patients 12 years and older with cGVHD after failure of at least two prior lines of systemic therapy.

"Belumosudil is very effective in inducing responses across all organs affected by cGVHD and is safe and well tolerated. It is exciting to see the development of new interventions that can help patients," said Corey Cutler, MD, MPH, FRCPC, Associate Professor of Medicine, Harvard Medical School; Medical Director, Adult Stem Cell Transplantation Program, Dana-Farber Cancer Institute and ROCKstar study investigator.

"Leveraging ROCK inhibition to address both the immune and fibrotic components of cGVHD is a welcome step forward in the treatment of this debilitating disease."

"The results from ROCKstar showcase the benefit that belumosudil can provide to cGVHD patients, including reducing and eliminating corticosteroid and calcineurin inhibitor doses and achieving improvements in quality of life," said Harlan W. Waksal, M.D., President and CEO of Kadmon. "We are pleased by the recent FDA approval of REZUROCK and look forward to making the drug available to patients in the near term."

As previously reported, data from ROCKstar, the randomized, Phase 2 clinical trial of belumosudil, which enrolled 132 cGVHD patients who had received two to five prior lines of therapy, demonstrated an Overall Response Rates (ORR) of 74% (95% Confidence Interval (CI): 62, 84; p<0.0001) with 200 mg once daily (QD) and 77% (95% CI: 65, 87; p<0.0001) with 200 mg twice daily (BID). Responses were achieved across patient subgroups and complete responses were observed in all organ systems, including in lung. Responses were durable, with a median duration of 54 weeks, which represents the total combined response periods for each patient. Forty-four percent (44%) of responders maintained their response for ≥1 year. In addition, clinically meaningful improvement from baseline in the Lee Symptom Scale (LSS) score, a chronic GVHD symptom measurement, with belumosudil was observed in 61% of patients. During treatment, 65% of patients were able to reduce their corticosteroid dose, with 21% of patients completely discontinuing corticosteroid therapy. In addition, 22% of patients completely discontinued calcineurin inhibitor therapy. Belumosudil was well tolerated and adverse events have been consistent with those expected in patients with advanced cGVHD receiving corticosteroids and/or other immunosuppressants.

The *Blood* publication of the ROCKstar data is available online here.

About cGVHD

cGVHD is a complication that can occur following allogeneic stem cell transplantation, resulting in significant morbidity and mortality. 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 living with cGVHD.

About REZUROCK™ (belumosudil)

REZUROCK™ (belumosudil) is the first and only approved therapy targeting Rho-associated coiled-coil kinase 2 (ROCK2), a signaling pathway that modulates inflammatory response and fibrotic processes. REZUROCK is approved in the United States for the treatment of adult and pediatric patients 12 years and older with cGVHD after failure of at least two prior lines of systemic therapy. For more information, visit www.REZUROCK.com.

Kadmon is also developing belumosudil for the treatment of systemic sclerosis. The FDA has granted Orphan Drug Designation to belumosudil for the treatment of systemic sclerosis.

INDICATIONS AND USAGE

REZUROCK is a kinase inhibitor indicated for the treatment of adult and pediatric patients 12 years and older with chronic graft-versus-host disease (chronic GVHD) after failure of at least two prior lines of systemic therapy.

SELECT IMPORTANT SAFETY INFORMATION

Warnings and Precautions

Embryo-Fetal Toxicity: Based on findings in animals and its mechanism of action, REZUROCK can cause fetal harm when administered to a pregnant woman. Advise females of reproductive potential and males with female partners of reproductive potential of the potential risk to a fetus and to use effective contraception.

Adverse Reactions

The most common (≥20%) adverse reactions, including laboratory abnormalities, in patients receiving REZUROCK were infections, asthenia, nausea,

diarrhea, dyspnea, cough, edema, hemorrhage, abdominal pain, musculoskeletal pain, headache, phosphate decreased, gamma glutamyl transferase increased, lymphocytes decreased, and hypertension.

To report suspected adverse reactions, contact Kadmon at 1-877-377-7862 or the FDA at (800) FDA-1088 or www.fda.gov/medwatch.

Use in Specific Populations

Lactation: Advise not to breastfeed.

Please visit www.REZUROCK.com to see the full Prescribing Information for REZUROCK.

About Kadmon

Kadmon is a biopharmaceutical company that discovers, develops and delivers transformative therapies for unmet medical needs. REZUROCK™ (belumosudil), an oral, once-daily, tablet, is approved in the United States for the treatment of adult and pediatric patients 12 years and older with chronic graft-versus-host disease (cGVHD) after failure of at least two prior lines of systemic therapy. Kadmon's clinical pipeline includes treatments for immune and fibrotic diseases as well as immuno-oncology therapies. For more information, please visit www.kadmon.com.

Forward Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, our expectations regarding REZUROCK™ (belumosudil) as a new available therapy, the timing of commercial availability of REZUROCK in the U.S., the commercial launch of REZUROCK in the U. S. and the potential benefit of our clinical and preclinical development programs for immune and fibrotic diseases as well as immuno-oncology therapies. The words "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target," "contemplate" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statement contained in this press release, including, without limitation, (i) our ability to commercialize REZUROCK and execute on our marketing plans for any other drugs or indications that may be approved in the future, (ii) risks that REZUROCK revenue, expenses and costs may not be as expected, (iii) risks relating to REZUROCK's market acceptance, competition, reimbursement and regulatory actions, (iv) risks and uncertainties related to the severity and duration of the impact of COVID-19 on our business and operations, including, without limitation, commercial and clinical drug supply chain continuity and the commercial launch of REZUROCK, (v) our ability to obtain and maintain reimbursement for REZUROCK and any approved product and the extent to which patient assistance programs and copay programs are utilized, (vi) our ability to successfully demonstrate the efficacy and safety of our product candidates including in later-stage studies, (vii) availability and timing of data from our preclinical and clinical trials, which may not support further development of our product candidates, (viii) our ability to manage our reliance on sole-source third parties such as our third party drug substance and drug product contract manufacturers, (ix) actions of regulatory agencies, (x) the inherent uncertainty in estimates of patient populations and incidence and prevalence estimates, (xi) competition from other products, (xii) our ability to comply with healthcare regulations and laws, (xiii) our ability to obtain, maintain and enforce our intellectual property rights, (xiv) our ability to maintain and establish strategic agreements and collaborations and the potential benefits of those arrangements and (xv) other risks, including active or potential litigation risks, any or all of which of the foregoing may affect the initiation, timing and progress of clinical studies and the timing of and our ability to obtain additional regulatory approvals, and make our investigational drugs and REZUROCK available to patients, and to derive revenue from product sales. More detailed information about us and the risk factors that may affect the realization of our forward-looking statements are set forth in our Securities and Exchange Commission (SEC) filings, including our Annual Report on Form 10-K for the fiscal year ended December 31, 2020, 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. We caution you not to place undue reliance on any forward-looking statements, which speak only as of the date they are made. We disclaim any obligation to publicly update or revise any such statements to reflect any change in expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements, except as may be required by law. Any forward-looking statements contained in this press release represent our views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date.

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