MyoKardia Announces Receipt of Breakthrough Therapy Designation from FDA for Mavacamten for the Treatment of Symptomatic, Obstructive Hypertrophic Cardiomyopathy

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BRISBANE, Calif., July 23, 2020 (GLOBE NEWSWIRE) -- MyoKardia, Inc. (Nasdaq: MYOK) today announced that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation to mavacamten, a novel, oral, allosteric modulator of cardiac myosin, for the treatment of symptomatic, obstructive hypertrophic cardiomyopathy (HCM). The FDA’s Breakthrough Therapy Designation is intended to expedite the development and review of a drug candidate that is planned for use to treat a serious or life-threatening disease or condition when clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints.

“Receipt of Breakthrough Therapy Designation from the FDA acknowledges both the significant unmet need among patients with obstructive HCM, for whom there are currently no targeted therapies, and the highly encouraging clinical results generated by mavacamten. We look forward to working closely with the FDA in pursuit of regulatory approval so that we may bring mavacamten to people with HCM and remain appreciative of the collaborative approach and valued input the agency has provided throughout mavacamten’s development,” said Tassos Gianakakos, CEO of MyoKardia.

In May, MyoKardia announced topline results from the company’s pivotal Phase 3 EXPLORER clinical trial of mavacamten for the treatment of symptomatic patients with obstructive HCM. Mavacamten was well tolerated, with a safety profile comparable to placebo, and demonstrated a robust treatment effect, with patients experiencing clinically meaningful responses to treatment, including reductions in symptoms, improvements in cardiac function and reduction or elimination of the obstruction of the left ventricle. MyoKardia is currently preparing a New Drug Application (NDA) for mavacamten, with plans to submit to the FDA in the first quarter of 2021.

About HCM
Hypertrophic cardiomyopathy (HCM) is a chronic, progressive disease in which excessive contraction of the heart muscle and reduced ability of the left ventricle to fill can lead to the development of debilitating symptoms and cardiac dysfunction. HCM is estimated to affect one in every 500 people. The most frequent cause of HCM is mutations in the heart muscle proteins of the sarcomere. In approximately two-thirds of HCM patients, the path followed by blood exiting the heart, known as the left ventricular outflow tract (LVOT), becomes obstructed by the enlarged and diseased muscle, restricting the flow of blood from the heart to the rest of the body (obstructive HCM). In other patients, the thickened heart muscle does not block the LVOT, and their disease is driven by diastolic impairment due to the enlarged and stiffened heart muscle (non-obstructive HCM). In either obstructive or non-obstructive HCM patients, exertion can result in fatigue or shortness of breath, interfering with a patient’s ability to participate in activities of daily living. HCM has also been associated with increased risks of atrial fibrillation, stroke, heart failure and sudden cardiac death.

About Mavacamten (MYK-461)
An investigational, novel, oral, allosteric modulator of cardiac myosin, mavacamten reduces cardiac muscle contractility by inhibiting excessive myosin-actin cross-bridge formation that results in hypercontractility, left ventricular hypertrophy and reduced compliance. In clinical and preclinical studies, mavacamten has consistently reduced biomarkers of cardiac wall stress, lessened excessive cardiac contractility and increased diastolic compliance. MyoKardia is developing mavacamten for the treatment of conditions in which excessive cardiac contractility and impaired diastolic filling of the heart are the underlying cause. Mavacamten is initially being developed for the treatment of symptomatic, obstructive hypertrophic cardiomyopathy (HCM). Based on its mechanism of action and evidence of therapeutic activity, mavacamten is also being studied in the clinic for the treatment of symptomatic non-obstructive HCM and among a targeted population of patients with heart failure with preserved ejection fraction (HfPEF).

About MyoKardia
MyoKardia is a clinical-stage biopharmaceutical company discovering and developing targeted therapies for the treatment of serious cardiovascular diseases. The company is pioneering a precision medicine approach to its discovery and development efforts by 1) understanding the biomechanical underpinnings of disease; 2) targeting the proteins that modulate a given condition; 3) identifying patient populations with shared disease characteristics; and 4) applying learnings from research and clinical studies to inform and guide pipeline growth and product advancement. MyoKardia’s initial focus is on small molecule therapeutics aimed at the proteins of the heart that modulate cardiac muscle contraction to address diseases driven by excessive contraction, impaired relaxation, or insufficient contraction. Among its discoveries are three clinical-stage therapeutics: mavacamten (formerly MYK-461); danicamtiv (formerly MYK-491) and MYK-224.

MyoKardia’s mission is to change the world for people with serious cardiovascular disease through bold and innovative science.

Forward-Looking Statements
Statements we make in this press release may include statements which are not historical facts and are considered forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, which are usually identified by the use of words such as “anticipates,” “believes,” “estimates,” “expects,” “intends,” “may,” “plans,” “projects,” “seeks,” “should,” “will,” and variations of such words or similar expressions. We intend these forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act and are making this statement for purposes of complying with those safe harbor provisions. These forward-looking statements, including statements regarding our expectations regarding our continuation of discussions with the FDA and our plan to submit a New Drug Application for mavacamten, as well as the timing of such submission, reflect our current views about our plans, intentions, expectations, strategies and prospects, which are based on the information currently available to us and on assumptions we have made. Although we believe that our plans, intentions, expectations, strategies and prospects as reflected in or suggested by those forward-looking statements are reasonable, we can give no assurance that the plans, intentions, expectations or strategies will be attained or achieved. Furthermore, actual results may differ materially from those described in the forward-looking statements and will be affected by a variety of risks and factors that are beyond our control including, without limitation, risks associated with the
development and regulation of our product candidates, as well as those set forth in our Quarterly Report on Form 10-Q for the quarter ended March 31, 2020, and our other filings with the SEC. Except as required by law, we assume no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

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