News Release

MyoKardia Announces Initiative to Create Hypertrophic Cardiomyopathy Patient Community in Collaboration with 23andMe

First-of-its-kind Digital Platform for HCM Patients Intended to Provide a Powerful Patient Resource to Improve Disease Understanding and Deepen Patient Engagement and Advocacy

SOUTH SAN FRANCISCO, Calif., Oct. 25, 2018 (GLOBE NEWSWIRE) -- MyoKardia, Inc., (Nasdaq: MYOK), a clinical stage biopharmaceutical company pioneering precision medicine for the treatment of cardiovascular diseases, today announced that it has entered into a collaboration with 23andMe to advance research for patients with hypertrophic cardiomyopathy (HCM), a progressive and frequently debilitating disease characterized by excessive contractility of the heart.

MyoKardia is committed to raising awareness and supporting patients and families suffering from this devastating disease, a commitment shared by 23andMe. The goal of this initiative is to deepen disease understanding and build resources intended to benefit patients and their caregivers. Together, the companies will collaborate to improve the baseline understanding of unmet need in the HCM disease area, including patient burden, quality of life, standard of care and variability of symptoms, improve knowledge of disease progression and natural history, and empower patient advocacy.

“We are proud to continue our history of investing in resources for HCM patients and their caregivers, furthering our core value of Patients First. By providing opportunities for patients to learn and to contribute to the overall body of knowledge about this devastating disease, we hope to make meaningful contributions to the HCM community,” said Jake Bauer, chief business officer of MyoKardia.

The companies will create a patient community where 23andMe customers can access regularly-updated disease information and HCM research opportunities. More than 6,000 HCM patients are currently 23andMe customers. In addition, a custom survey will be deployed to collect baseline and follow-up longitudinal data from participants with HCM who have opted-in to participate in further research.

MyoKardia and 23andMe intend to study this set of de-identified, population-scale phenotypic and genotypic data in order to gain unique insights that may support the broader HCM ecosystem. Summary results will be made available to patients through the 23andMe platform.

“By collaborating with 23andMe, we strive to share new insights into how HCM manifests across diverse patient groups through a unique combination of genotypic and phenotypic data. This important resource can help support patients as they navigate the challenges of daily life with HCM,” said Richey Neuman, MD, MPH, FACP, vice president of medical affairs at MyoKardia. “We are excited to lead this important effort launching in 2019 in the hopes of informing and potentially improving the lives of HCM patients.”

About HCM
Hypertrophic cardiomyopathy (HCM) is the most common genetic cause of heart disease, in which the walls of the heart thicken and prevent the left ventricle from expanding, resulting in a reduced pumping capacity. HCM is a chronic, progressive disease that can be extremely disabling. According to recent research published in the journal *Circulation*[(1)], HCM patients are at substantially elevated risks of long-term complications and comorbidities, such as atrial fibrillation and heart failure. HCM patients also have significantly higher mortality rates compared to that of the general U.S. population.

About MyoKardia
MyoKardia is a clinical-stage biopharmaceutical company pioneering a precision medicine approach to discover, develop and commercialize targeted therapies for the treatment of serious and rare cardiovascular diseases. MyoKardia's initial focus is on the treatment of heritable cardiomyopathies, a group of rare, genetically driven forms of heart failure that result from biomechanical defects in cardiac muscle contraction. MyoKardia has used its precision medicine platform to generate a pipeline of therapeutic programs for the chronic treatment of two of the most prevalent forms of heritable cardiomyopathy – hypertrophic cardiomyopathy (HCM), and dilated cardiomyopathy (DCM). MyoKardia's most advanced product candidate is mavacamten (formerly MYK-461), a novel, oral, allosteric modulator of cardiac myosin intended to reduce hypercontractility. Mavacamten is being studied in a pivotal Phase 3 clinical trial, known as EXPLORER-HCM, in patients with symptomatic, obstructive HCM. MyoKardia is also developing mavacamten in a second indication, non-obstructive HCM, in the Phase 2 MAVERICK-HCM clinical trial. MYK-491, MyoKardia's second product candidate, is designed to increase the overall extent of the heart's contraction in DCM patients by increasing cardiac contractility. MyoKardia is currently evaluating MYK-491 in a Phase 1b study in DCM patients. A cornerstone of the MyoKardia platform is the Sarcomeric Human Cardiomyopathy Registry (SHaRe), a multi-center, international repository of clinical and laboratory data on individuals and families with genetic heart disease, which MyoKardia helped form in 2014. MyoKardia's mission is to change the world for patients with serious cardiovascular disease through bold and innovative science.

[(1)] Ho, et al, *Circulation* 2018

Forward Looking Statement
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 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 the viability of the collaboration between MyoKardia and 23andMe, the potential of the collaboration to create a patient community and the ability of the collaboration to improve the understanding of HCM, 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, as well as those set forth in our Quarterly Report on Form 10-Q for the quarter ended June 30, 2018, 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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