
UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

SCHEDULE 14A INFORMATION

Proxy Statement Pursuant to Section 14(a) of the
Securities Exchange Act of 1934 (Amendment No.)

Filed by the Registrant

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Check the appropriate box:

- Preliminary Proxy Statement
- Confidential, for Use of the Commission Only (as permitted by Rule 14a-6(e)(2))**
- Definitive Proxy Statement
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MyoKardia, Inc.

(Name of Registrant as Specified In Its Charter)

(Name of Person(s) Filing Proxy Statement, if other than the Registrant)

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- No fee required.
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To MyoKardia Shareholders,

April 2020

It is my sincere hope that this annual update finds you and your loved ones in good health.

Since we implemented a work-from-home policy in early March, I have been inspired by the commitment, camaraderie and creativity of the MyoKardia team. We continue to drive forward our efforts to develop important therapies, and 2020 promises to be an incredibly eventful year for our company. Our Phase 3 pivotal

trial of mavacamten for obstructive hypertrophic cardiomyopathy (HCM) remains on track to report topline data in the second quarter of 2020. Given the unprecedented circumstances caused by the pandemic, this would not have been possible without the dedication and experience of our team, and the motivation of our investigators and their staffs, who completed enrollment ahead of schedule last August.

As we await the results of the EXPLORER-HCM pivotal trial, we have begun to assemble the full registration package for mavacamten's New Drug Application (NDA). The NDA package tells the scientific and medical story of mavacamten, starting from its discovery in 2013. And as we look across the years of studies, results

and analyses, we are encouraged and excited by mavacamten's potential to help people with HCM and by the breadth

Results from our clinical studies added important evidence that mavacamten is addressing the key driver of HCM – cardiac muscle hypercontractility

and consistency of the data across our considerable body of research. This was evident in 2019, as results from our clinical studies added important evidence that mavacamten is addressing the key driver of HCM – cardiac muscle hypercontractility. That hypercontractility, caused by mutations in the heart muscle proteins, leads to the down-stream consequences of HCM: chronic and potentially debilitating symptoms such as shortness of breath, fatigue, chest pain, and palpitations as well as serious complications such as heart failure, arrhythmias, stroke, sudden cardiac death, and mortality rate three times higher than that of the general population.

In 2019, we also reported long-term results from the PIONEER-OLE study of mavacamten treatment in people with obstructive HCM. The 12 participants from the original PIONEER Phase 2 clinical trial have all now completed 60 weeks on mavacamten, with the majority having been on therapy for over 84 weeks. The one-year data shared at last year's American Heart Association (AHA) meeting showed that treatment with mavacamten resulted in sustained improvements,

including reductions in left ventricular outflow tract gradient and improvements in New York Heart Association functional class, while being well tolerated and maintaining ejection fractions well above normal. Continued treatment with mavacamten shows evidence of bringing the heart closer to a normal state, including the sustained reduction in biomarkers of cardiac wall stress, diastolic filling pressure, as well as left atrial volume and septal wall thickness. Left untreated, the latter two biomarkers are associated with increased risk for atrial fibrillation and sudden cardiac death.

Among the most important insights gained from our work with mavacamten in 2019 was evidence of its ability to improve diastolic relaxation. Diastolic impairment – which results in less oxygenated blood being available in the left ventricle and therefore to the rest of the body – brings about much of the disease burden in non-obstructive HCM and contributes to worsening health in obstructive HCM. The results from our MAVERICK-HCM study of patients with non-obstructive HCM showed that mavacamten can lower important biomarkers of cardiac wall stress and injury that are unnaturally elevated in non-obstructive HCM patients. Among those patients with the greatest diastolic impairment or with signs of higher risk of mortality,

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mavacamten demonstrated encouraging trends toward potential benefits across several, diverse clinical measurements. MAVERICK also answered important questions about how to dose and what patients to study in our subsequent non-obstructive HCM trials, which was the main objective of the study.

MyoKardia can make an important difference in the treatment of diseases of diastolic dysfunction

Beyond HCM, impairment of diastolic relaxation underlies heart failure with preserved ejection fraction, or HFpEF. HFpEF affects approximately 13 million people worldwide, and treatment options for HFpEF have proven elusive. Based on the evidence provided by MAVERICK, we are increasingly optimistic that MyoKardia can make an important difference in the treatment of diseases of diastolic dysfunction, including a targeted subset of HFpEF patients whose disease characteristics align with the benefits that were observed in our MAVERICK study of non-obstructive HCM patients.

Taken together, the concordance of our mavacamten data underpin our confidence in mavacamten's mechanism and its ability to help people with HCM. 2019 saw the build out of a commercial leadership team, led by Bill Fairey, who joined us early last year as Chief Commercial Officer. We have added leaders in marketing, market access, commercial operations and real-world evidence (RWE) & health economics and continue to grow our commercial infrastructure to ensure readiness for the launch of our first therapy.

Recognizing that HCM remains an underappreciated and underdiagnosed disease, we debuted the "Expose HCM" disease awareness campaign at the AHA meeting last November and have recently released ExposeHCM.com - an accompanying online resource for physicians.

In parallel, our medical and advocacy teams have been partnering with the HCM community. Together with the AHA, we're aiming to heighten awareness and clarify misconceptions about HCM among cardiologists, patients and their families. These new initiatives complement our other long-standing efforts to support the HCM community, such as the SHaRe patient registry, the development of the HCM Cares app with Duke, and our partnership with 23andMe.

2019 was also a breakout year for danicamtiv (MYK-491), our potential therapy for patients with dilated cardiomyopathy (DCM). In our Phase 2a clinical trial of 26 patients with stable heart failure, danicamtiv showed improvement in systolic function – including stroke volume – while preserving diastolic function. Given the favorable safety profile seen in the study, we extended the trial to evaluate higher doses of danicamtiv and plan to initiate a Phase 2 study in people with dilated cardiomyopathy caused by genetic mutations in the proteins of the heart muscle, allowing us to study danicamtiv in a well-defined patient population who we believe are well matched to danicamtiv's mechanism of action.

Danicamtiv showed improvement in systolic function – including stroke volume – while preserving diastolic function

Our progress so far brings us closer to broadly enabling an era of precision medicine in cardiovascular disease treatment, with an opportunity to bring functional cures to many of the millions of people with heart failure. We've seen precision approaches in other disease areas, such as oncology, autoimmune and inflammatory conditions, change the lives of so many people. It's time we make this happen in cardiovascular medicine.

As I reflect on the tremendous progress that we've made in 2019 and the opportunities just ahead, I am overcome with a sense of pride and gratitude. Pride in our collective commitment to our mission to improve the lives of people with serious cardiovascular disease. And gratitude for the dedication of our employees, as well as for the patients who participate in our studies, their caregivers, the clinical investigators and their staff, and you – our shareholders.

We look forward to your ongoing support as we continue this incredible journey into 2020 and beyond.



Sincerely,

Tassos Gianakakos
President and CEO, MyoKardia

