Pioneering Precision Cardiovascular Medicine

MyoKardia

PIONEER-HCM Data Summary
March 2018

Forward-Looking Statements

Statements we make in this presentation 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 clinical and therapeutic potential of mavacamten (formerly MYK-461) and MYK-491, the Company's ability to continue to advance mavacamten in the PIONEER-HCM study and MYK-491 in its Phase 1 study in healthy volunteers, the Company's expectations with respect to the release of data from these studies and the timing thereof, the Company's ability to initiate its planned Phase 3 EXPLORER-HCM study of mavacamten in symptomatic oHCM and its planned Phase 2 MAVERICK-HCM study of mavacamten in nHCM and the timing thereof, the anticipated numbers of patients expected to be enrolled in these studies, the Company's ability to initiate its planned Phase 1b trial of MYK-491 in DCM patients and generate data therefrom, and the timing of these events, as well as the requirements for registration of the Company's product candidates and the Company's projected cash runway, 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 Annual Report on Form 10-K for the year ended December 31, 2017, 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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Cohort B Results Overview

- Statistically significant reductions in the primary endpoint of a reduction in left ventricular outflow (LVOT) gradient were achieved
- Mavacamten treatment resulted in improvement of patient symptoms across several measures
  - NYHA class
  - Peak VO_2
  - Dyspnea
- Ejection fraction maintained at or above the normal range
- Use of background beta blockers did not seem to impact mavacamten's safety or pharmacodynamics profile
- A desired target concentration range is emerging that will inform and guide dosing in our planned Phase 3 pivotal study, known as EXPLORER

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Baseline Characteristics and Trial Design

Baseline Characteristics

	Cohort A	Cohort B
N	11	10
Age (years) mean (min-max)	56 (22–70)	57 (22–71)
Sex, % male	64%	50%
NYHA Class	64% Class II; 36% Class III	50% Class II; 50% Class III
Concomitant β-blockers, N (%)	0 (0)	9 (90)

Parameter (Mean ±SD)	Cohort A	Cohort B
Resting LVEF (%)	70 ± 7	75 ± 5
Exercise LVEF (%)	76 ± 8	76 ± 8
Resting LVOT Gradient (mm Hg)	60 ± 28	86 ± 63
Exercise LVOT Gradient (mm Hg)	103 ± 50	86 ± 43
Peak VO_2 (mL/kg/min)	20.7 ± 7.4	19.4 ± 4.6

Trial Design

Cohort A (n=11)

- Doses of 10mg, 15mg, 20mg
- Starting dose based on weight
  - 10 mg ≤ 60kg
  - 15 mg >60kg
- Dose adjustment at week 4 based on percentage decrease from baseline in LVEF

Cohort B (n=10)

- Doses of 2mg, 5mg
- Starting dose = 2mg
- Dose adjustment at week 4 based on percentage decrease from baseline in resting LVOT peak gradient

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PIONEER-HCM Data Summary

	Cohort A		Cohort B	
Number of patients	n=11		n=10	
Mavacamten doses studied	10, 15, 20 mg QD		2, 5 mg QD	
Change, from baseline to W12	Mean ± SD	p-value	Mean ± SD	p-value
Post-exercise LVOT gradient (mmHg)	-112 ± 63.8	0.002	-25 ± 28.7	0.020
Resting LVOT gradient (mmHg)	-55 ± 41.8	0.006	-49 ± 48.0	0.004
Resting LVEF (%)	-16 ± 14.1	0.008	-5.5 ± 6.0	0.002
NYHA class	-0.9 ± 0.7	0.016	-1.0 ± 0.5	0.004
Peak VO2 (mL/kg/min)	+3.5 ± 3.25	0.004	+1.7 ± 2.3	0.121
Dyspnea numerical rating	-3.1 ± 1.4	0.002	-3.0 ± 2.8	0.008

Additional Cohort B Observations

- Mavacamten was generally well tolerated
  - Most AEs were mild (80%) to moderate (19%)
  - Most AEs were deemed unrelated to mavacamten (79%)
- No discernable impact on mavacamten's pharmacodynamics profile were observed with use of background beta blockers
- LVOT gradient, NYHA class, LVEF measures reverted towards baseline during four-week washout period

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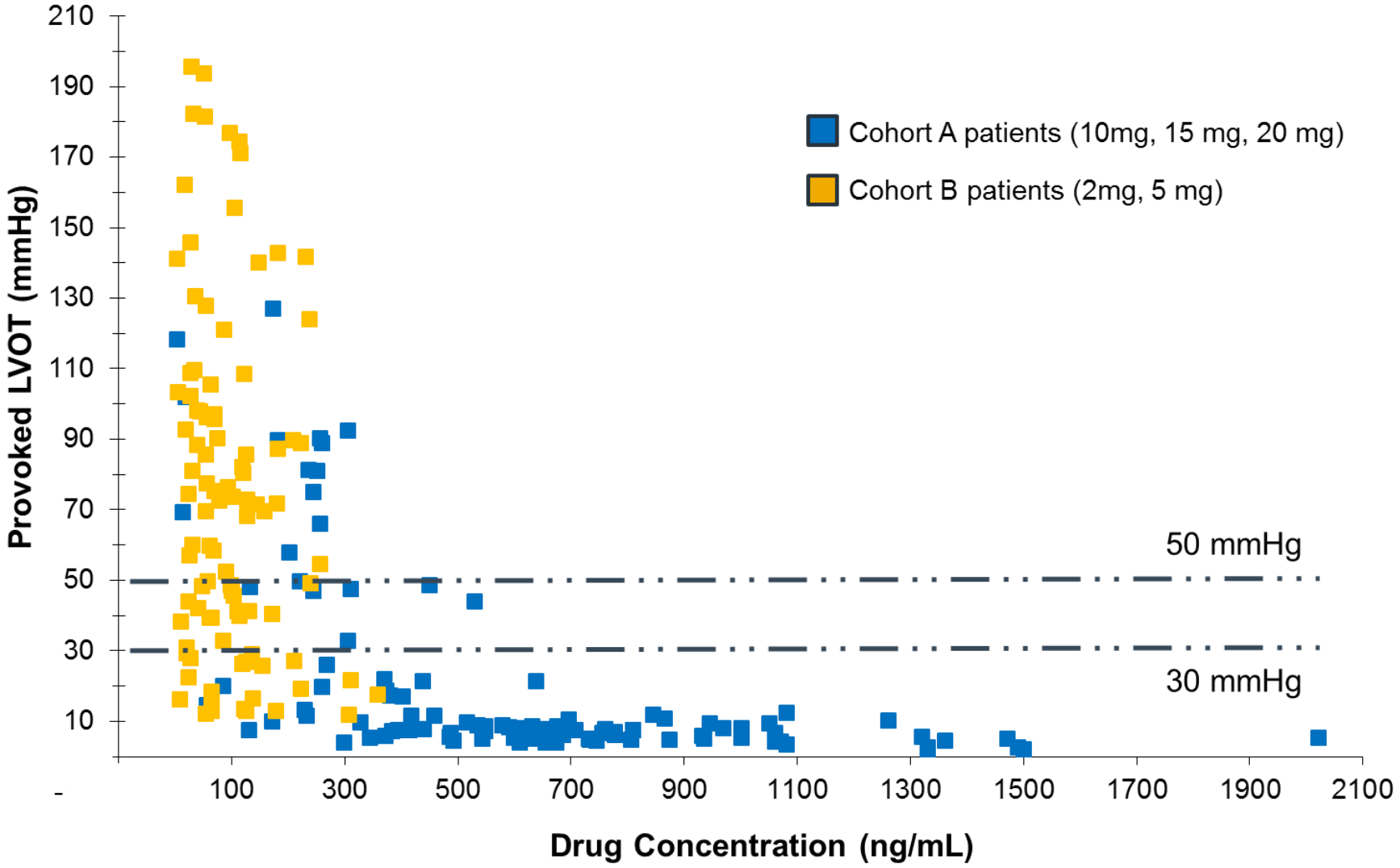
Mavacamten Concentration vs. Provoked LVOT



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Thank You

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PATIENTS FIRST

PASSION FOR SCIENCE

SUCCEED TOGETHER

IMAGINE & INNOVATE

LIFELONG LEARNING

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