PRECISION THAT MOVES™
Staying one step ahead of disease

APRIL 1, 2020

R.S., living with systemic mastocytosis
Forward-looking statements

This presentation contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended. The words "aim," "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. In this presentation, forward-looking statements include, without limitation, statements regarding the plans, strategies, timelines and expectations of Blueprint Medicines Corporation (the "Company") for the preclinical and clinical development and commercialization of AYVAKIT™ (avapritinib), pralsetinib, fisoatinib, and BLU-263; the plans, timing, design, initiation, enrollment, expectations and announcement of results for the Company's ongoing and planned clinical trials; plans and timelines for submitting marketing applications for avapritinib and pralsetinib and, if approved, commercializing avapritinib for additional indications or pralsetinib; the potential benefits of any of the Company's current or future approved drugs or drug candidates in treating patients; expectations regarding the Company's existing, cash, cash equivalents and investments; and the Company's strategy, goals and anticipated milestones, business plans and focus.

The Company has based these forward-looking statements on management's current expectations, assumptions, estimates and projections. While the Company believes these expectations, assumptions, estimates and projections are reasonable, such forward-looking statements are only predictions and involve known and unknown risks, uncertainties and other important factors, many of which are beyond the Company's control and may cause actual results, performance or achievements to differ materially from those expressed or implied by any forward-looking statements. These risks and uncertainties include, without limitation, risks and uncertainties related to the impact of the COVID-19 pandemic to the Company's business, operations, strategy, goals and anticipated milestones, including the Company's ongoing and planned research and discovery activities, ability to conduct ongoing and planned clinical trials, clinical supply of current or future drug candidates, commercial supply of current or future approved drugs, and launching, marketing and selling current or future approved drugs; the delay of any current or planned clinical trials or the development of the Company's drug candidates, including avapritinib for additional indications, pralsetinib, fisoatinib and BLU-263, or the licensed drug candidate; the Company's advancement of multiple early-stage efforts; the Company's ability to successfully demonstrate the efficacy and safety of its drug candidates and gain approval of its drug candidates on a timely basis, if at all; the preclinical and clinical results for the Company's drug candidates, which may not support further development of such drug candidates; actions or decisions of regulatory agencies or authorities, which may affect the initiation, timing and progress of clinical trials or marketing applications; the Company's ability to obtain, maintain and enforce patent and other intellectual property protection for any drug candidates it is developing or AYVAKIT; the Company' ability and plans for maintaining a commercial infrastructure, and successfully launching, marketing and selling its current or future approved drugs; the Company's ability to successfully expand the approved indications for AYVAKIT or obtain marketing approval for AYVAKIT in additional geographies; the Company's ability to develop and commercialize companion diagnostic tests for any of the Company's current or future approved drugs or drug candidates; and the success of the Company's current and future collaborations, partnerships and licenses.

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Leadership in a time of challenge and uncertainty

OUR APPROACH TO NAVIGATING THE COVID-19 PANDEMIC

PATIENT CENTERED
Stay focused on the patients who need access to our innovation, perhaps now more than ever

VIGILANT
Constantly assess and customize approaches to potential business impacts

NIMBLE
Leverage global infrastructure including external collaborators and adapt to new ways of working

RESILIENT
Provide support and flexibility to our employees to enable resiliency
3 clinical datasets reported in 2020 to date, with additional disclosures planned

Q1 2020
- Top-line ARROW data for pralsetinib in RET+ NSCLC
- Updated PIONEER data for avapritinib in ISM

Q2 2020
- Top-line ARROW data for pralsetinib in RET+ MTC
- Top-line VOYAGER data for avapritinib in 3L GIST

Q3 2020
- Top-line EXPLORER and PATHFINDER data for avapritinib in advanced SM

On track to lock VOYAGER trial database in April 2020 and provide top-line data to FDA to enable action on avapritinib NDA for 4L GIST by May 14 PDUFA date

FDA, U.S. Food and Drug Administration; ISM, indolent systemic mastocytosis; GIST, gastrointestinal stromal tumors. MTC, medullary thyroid cancer; NDA, new drug application; NSCLC, non-small cell lung cancer; PDUFA, the Prescription Drug User Fee Act; SM, systemic mastocytosis; 3L, third-line; 4L, fourth-line.

Not for promotional use.
1. Approved in the U.S. for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA exon 18 mutant, including PDGFRA D842V mutations. 2. Proposed MAA indication is unresectable or metastatic GIST harboring a PDGFRA D842V mutation. 3. Planned NDA or MAA submissions. MAA, marketing authorization application; 2L, second-line. *All planned commercial launches are subject to regulatory review and approval of marketing applications currently under review or planned. Not for promotional use.
<table>
<thead>
<tr>
<th>Discovery</th>
<th>Early-Stage Development</th>
<th>Late-Stage Development</th>
<th>Regulatory Submission</th>
<th>Approved</th>
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<tbody>
<tr>
<td><strong>Avapritinib (KIT &amp; PDGFRA)</strong></td>
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<td>U.S.</td>
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<tr>
<td>PDGFRA GIST</td>
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<td>MAA</td>
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<td>4L GIST</td>
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<td>2L GIST</td>
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<td>Advanced SM</td>
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<td>Indolent SM</td>
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<td><strong>Pralsetinib (RET)</strong></td>
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<tr>
<td>2L RET+ NSCLC</td>
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<td>NDA / MAA</td>
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<tr>
<td>1L RET+ NSCLC</td>
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<td>EGFR+ NSCLC (+osimertinib)</td>
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<td>2L MTC</td>
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<td>NDA</td>
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<td>1L MTC</td>
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<td>Other RET-altered solid tumors</td>
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<td><strong>Fisogatinib (FGFR4)</strong></td>
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<td>Advanced HCC</td>
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<td>Advanced HCC (+CS-1001)</td>
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<td><strong>BLU-263 (KIT)</strong></td>
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<td>Indolent SM</td>
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<td><strong>BLU-945 (EGFR+ triple mutant)</strong></td>
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<td>EGFR+ NSCLC</td>
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<td>(EGFR+ double mutant)</td>
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<td>(2 undisclosed targets)</td>
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<td>(MAP4K1)</td>
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<td>(3 undisclosed immunokinase targets)</td>
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1. Unresectable or metastatic disease. 2. CStone Pharmaceuticals has exclusive rights to develop and commercialize avapritinib, pralsetinib and fisogatinib in Mainland China, Hong Kong, Macau and Taiwan. Blueprint Medicines retains all rights in the rest of the world. 3. Approved in the U.S. for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA exon 18 mutation, including PDGFRA D842V mutations. The proposed MAA indication is unresectable or metastatic GIST harboring a PDGFRA D842V mutation. 4. NDA submitted to FDA in March 2020; plan to submit MAA to EMA in Q2 2020. 5. In collaboration with Roche. Blueprint Medicines has U.S. commercial rights for up to two programs. Roche has worldwide commercialization rights for up to two programs and ex-U.S. commercialization rights for up to two programs. 1L, first line; HCC, hepatocellular carcinoma

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Pralsetinib: an investigational precision therapy for RET-altered cancers

HUMAN KINOME

Pralsetinib
Potent and highly selective RET inhibitor

LATE CLINICAL DEVELOPMENT

U.S. REGULATORY SUBMISSION STATUS

RET fusion-positive NSCLC
Submitted

Previously treated MTC
Q2 2020*

Other RET-altered tumors

* Planned NDA submission. Kinome illustration reproduced courtesy of Cell Signaling Technology, Inc. (www.cellsignal.com) (CSTI). The foregoing website is maintained by CSTI, and Blueprint Medicines is not responsible for its content.

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RET alterations: oncogenic drivers lacking a targeted therapeutic approach

Non-small cell lung cancer: ~1-2% RET fusions\(^1,2\)

Advanced medullary thyroid cancer: ~90% RET mutations\(^3\)

Papillary thyroid cancer: ~20% RET fusions\(^4\)

Multiple other tumor types <1% RET-altered, including:\(^5,6\)
- esophageal
- pancreatic
- breast
- melanoma
- colorectal
- leukemia

Top-line ARROW trial data support registration plans for NSCLC and MTC

Part 1 dose escalation

RET-altered solid tumors

RP2D 400 mg QD

Part 2 expansion

RET-fusion NSCLC, prior platinum

RET-fusion NSCLC, no prior platinum

MTC, prior MKI

MTC, no prior MKI

RET-fusion solid tumors, prior standard of care

RET-altered solid tumors, prior selective RET TKI

RET-mutated tumors, prior standard of care

Trial endpoints:
ORR, duration of response, safety

Top-line safety
(n=438; 400 mg QD)

- Top-line safety results consistent with prior data
- Pralsetinib was well-tolerated and most AEs were Grade 1 or 2
- Across all patients, 4% discontinued due to treatment-related AEs

1. Phase 1/2 ARROW trial data in patients treated with pralsetinib 400 mg QD reported on April 1, 2020. Data cutoff: February 13, 2020. AE, adverse event; MKI, multi-kinase inhibitor; ORR, overall response rate; QD, once daily; RP2D, recommended phase 2 dose; TKI, tyrosine kinase inhibitor

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NSCLC patients with RET fusions have no highly effective treatment options

- **Chemotherapy**: nonspecific, low response rates, significant toxicity
- **Checkpoint inhibition**: Preliminary evidence for lack of benefit in RET-altered NSCLC\(^1\)
- **Multi-kinase inhibitors**: ↓ activity, ↑ off-target toxicity\(^2,3\)
- Growing understanding of RET-driven resistance
- No selective RET inhibitors are approved

Top-line ARROW trial data: RET fusion-positive NSCLC

- **61% ORR¹** for RET-fusion NSCLC with prior platinum chemotherapy
  - 400 mg QD, N=80

- **73% ORR²** for RET-fusion NSCLC with no prior systemic therapy
  - 400 mg QD, N=26

Median DOR not reached (95% CI: 11.3 months, NE) in patients treated with 400 mg QD.

ALL RET FUSION-POSITIVE NSCLC PATIENTS (400 MG QD) PER CENTRAL RADIOLOGY

12% COMPLETE RESPONSE RATE IN PATIENTS WITH NO PRIOR SYSTEMIC THERAPY
Multi-kinase inhibitors are approved for MTC, but have important limitations:\(^1\)

- 25-44% ORR
- Off-target toxicity often requiring dose modification or discontinuation
- Emergence of resistance
- No selective RET inhibitors are approved

Top-line ARROW trial data: RET mutant medullary thyroid cancer

- **60% ORR**
  - RET-mutated MTC with prior cabozantinib and/or vandetinib treatment
  - 400 mg QD, N=53

- **74% ORR**
  - RET-mutated MTC with no prior systemic therapy
  - 400 mg QD, N=19

**ALL RET MUTANT MTC PATIENTS (400 MG QD) PER CENTRAL RADIOLOGY**

99% OF EVALUABLE PATIENTS HAD TUMOR REDUCTIONS

Median DOR not reached (95% CI: NE, NE) in patients treated with 400 mg QD

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Prolonged duration of response in patients with previously treated MTC


18-month duration of response rate of 90%

No. at risk: 31 28 20 17 7 3 2 1 0

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Top-line ARROW trial data: RET fusion-positive thyroid cancer

89% ORR\(^1\)

RET fusion-positive thyroid cancer with prior systemic therapy
400 mg QD, N=9

RET FUSION-POSITIVE THYROID CANCER PATIENTS (400 MG QD) PER CENTRAL RADIOLOGY

100% OF EVALUABLE PATIENTS HAD TUMOR REDUCTIONS

Median DOR not reached (95% CI: 8.2, NE) in patients treated with 400 mg QD


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Pralsetinib is a potential best-in-class selective RET inhibitor and the cornerstone of our lung cancer portfolio.

**EQUIPOTENT INHIBITION** of RET fusions and mutations, including predicted gatekeeper resistance mutations.

**HIGH RESPONSE RATES AND DURABLE ACTIVITY** in RET+ NSCLC\(^1\) and MTC\(^2\) patients.

**STRONG ACTIVITY AGAINST BRAIN METASTASES** in patients with RET+ NSCLC\(^3\).

**CLINICAL RESPONSES** in 2 of 4 patients previously treated with selpercatinib\(^3\).

**FDA BREAKTHROUGH THERAPY DESIGNATIONS** for RET+ NSCLC and MTC\(^4\).

**WELL-TOLERATED WITH LOW DISCONTINUATION RATES** in advanced cancer populations\(^1,2,3\).


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Avapritinib: a precision therapy with broad potential

<table>
<thead>
<tr>
<th>LATE CLINICAL DEVELOPMENT</th>
<th>U.S. REGULATORY SUBMISSION STATUS</th>
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<tbody>
<tr>
<td>PDGFRA exon 18 mutant GIST</td>
<td>APPROVED¹</td>
</tr>
<tr>
<td>4L GIST</td>
<td>SUBMITTED</td>
</tr>
<tr>
<td>3L GIST</td>
<td>2H 2020*</td>
</tr>
<tr>
<td>Advanced SM</td>
<td>2H 2020*</td>
</tr>
<tr>
<td>Indolent and smoldering SM</td>
<td></td>
</tr>
</tbody>
</table>

¹. Approved in the U.S. for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA exon 18 mutation, including PDGFRA D842V mutations. * Planned NDA submission. Kinome illustration reproduced courtesy of Cell Signaling Technology, Inc. (www.cellsignal.com) (CSTI). The foregoing website is maintained by CSTI, and Blueprint Medicines is not responsible for its content.

Avapritinib
Potent and highly selective KIT and PDGFRA inhibitor

Not for promotional use.
AYVAKIT™ (avapritinib) is now approved in the United States

**INDICATION**

AYVAKIT is indicated for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA exon 18 mutation, including PDGFRA D842V mutations.

**AVAILABLE DOSE STRENGTHS**

100, 200 and 300 mg tablets

First precision therapy for GIST • Approved regardless of line of therapy

Only highly effective treatment for PDGFRA exon 18 mutant GIST

Full prescribing information is available at www.AYVAKIT.com.
Full approval of AYVAKIT based on Phase 1 NAVIGATOR trial

<table>
<thead>
<tr>
<th>EFFICACY PARAMETER</th>
<th>PDGFRA EXON 18 (N=43)</th>
<th>PDGFRA D842V (N=38)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Overall response rate (95% CI)</td>
<td>84% (69%, 93%)</td>
<td>89% (75%, 97%)</td>
</tr>
<tr>
<td>Complete response, n (%)</td>
<td>3 (7%)</td>
<td>3 (8%)</td>
</tr>
<tr>
<td>Partial response, n (%)</td>
<td>33 (77%)</td>
<td>31 (82%)</td>
</tr>
<tr>
<td>Duration of response</td>
<td>n=36</td>
<td>N=34</td>
</tr>
<tr>
<td>Median in months (range)</td>
<td>Not reached (1.9+, 20.3+)</td>
<td>Not reached (1.9+, 20.3+)</td>
</tr>
</tbody>
</table>

Full prescribing information is available at [www.AYVAKIT.com](http://www.AYVAKIT.com). CI, confidence interval.

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Safety highlights from AYVAKIT prescribing information

MOST COMMON ADVERSE REACTIONS (≥20%; ANY GRADE):¹

- Edema, nausea, fatigue/asthenia, cognitive impairment, vomiting, decreased appetite, diarrhea, hair color changes, increased lacrimation, abdominal pain, constipation, rash, and dizziness

WARNINGS AND PRECAUTIONS:

- Intracranial hemorrhage
  - Occurred in 1% of 267 patients with GIST who received AYVAKIT
- CNS adverse reactions
  - Occurred in 58% of 335 patients who received AYVAKIT
    - Cognitive impairment: 41% (3.6% Grade 3 or 4)
  - Overall, 3.9% of patients required treatment discontinuation due to a CNS adverse reaction
- Embryo-fetal toxicity
Systemic mastocytosis is one disease driven by KIT D816V

- **Advanced SM**
  - Debilitating symptoms
  - Significant organ involvement
  - Requirement of high intensity treatment
  - Requirement for life-long chronic treatment
  - ~75,000 patients in major markets

- **Non-advanced SM** (Indolent and smoldering)

Patient numbers in major markets based on estimated prevalence for advanced, indolent and smoldering systemic mastocytosis in the US, EU5 and Japan.

Not for promotional use.
Avapritinib is the only highly potent inhibitor of KIT D816V, the common disease driver across systemic mastocytosis.

1. Analysis of trial data from EXPLORER and PATHFINDER (data cutoff: August 30, 2019) and PIONEER (data cutoff: December 27, 2019).

≥25% reduction in KIT D816V MAF is correlated with improved overall survival in advanced SM.
PIONEER trial results: unparalleled clinical profile in patients with indolent SM

Reduces mast cell burden

Kit D816V mutant allele fraction

Improves disease symptoms

ISM-SAF total symptom score

Improves quality of life

MC-QoL total score

Favorable safety profile supports the selection of avapritinib 25 mg QD as recommended Part 2 dose


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Avapritinib improves all symptoms assessed by the ISM-SAF


Not for promotional use.
Avapritinib improves all quality of life domains measured by the MC-QoL

Data cutoff: December 27, 2019. MC-QoL, Mastocytosis Quality of Life Questionnaire.

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Avapritinib demonstrates clinically meaningful changes in disease severity, as measured by the MC-QoL

**MC-QoL DISEASE SEVERITY**

*(Baseline to Week 16)*

<table>
<thead>
<tr>
<th>Severe</th>
<th>Moderate</th>
<th>Mild</th>
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</thead>
<tbody>
<tr>
<td>Affected multiple times per day</td>
<td>Affected multiple times per week</td>
<td>Affected multiple times per month</td>
</tr>
</tbody>
</table>

**Avapritinib 25 mg QD**
- 71% with mild disease at 16 weeks
- 86% improved

**Placebo**
- 0% with mild disease at 16 weeks
- 50% worsened

Avapritinib improves objective measures of mast cell burden assessed

Data reported at AAAAI Annual Meeting in March 2020. Data cutoff: December 27, 2019. *Bone marrow MC assessment in SM may have variability in sampling due to patchy nature of disease. No patient on study has progressed to advanced disease. #patient received high dose IV steroids.

Not for promotional use.
Safety results for avapritinib 25mg QD are similar to placebo at 16 weeks

**AE in >15% of placebo or avapritinib arms**

<table>
<thead>
<tr>
<th>Preferred term</th>
<th>Placebo n=9</th>
<th>25 mg n=10</th>
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<tbody>
<tr>
<td></td>
<td>any grade</td>
<td>grade 3</td>
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<tr>
<td>% of subjects with ≥1 AE</td>
<td>89</td>
<td>22</td>
</tr>
<tr>
<td>Nausea</td>
<td>22</td>
<td>0</td>
</tr>
<tr>
<td>Dizziness</td>
<td>22</td>
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<td>Headache</td>
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<td>Face edema</td>
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<tr>
<td>Periorbital edema</td>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td>Bone Pain</td>
<td>22</td>
<td>0</td>
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**AVAPRITINIB 25 MG QD**

- No patients had serious AEs
  - 2 patients treated with placebo had serious AEs, 1 with psychogenic seizure and 1 with diffuse cutaneous mastocytosis
- No patients had dose modifications
- No patients discontinued due to AEs

Data presented in March 2020 at AAAAI annual meeting. Data cutoff: December 27, 2019.

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Next steps for PIONEER trial of avapritinib in indolent SM

PIONEER REGISTRATION-ENABLING PART 2

**Design:** Randomized, double-blind, placebo-controlled treatment period, followed by open-label expansion

**Key endpoints:** ISM- SAF total symptom score (primary), measures of mast cell burden, quality of life, concomitant medications

**Sample size:** ~200 patients

**Duration:** ~6 months

**Timeline:** Plan to initiate patient screening in June 2020
EXPLORER trial results: Remarkable response rate and prolonged duration of response in patients with advanced SM

- FDA breakthrough therapy designation
- Robust activity across all disease subtypes
- Median follow up of 21 months with ongoing treatment up to ~3.5 years

**BEST RESPONSE PER IWG-MRT-ECNM CRITERIA**
**ALL DOSES (N=48)**

- Confirmed ORR
- Median DOR and OS not reached

-SAFETY
**ALL DOSES (N=80)**

- Avapritinib was generally well-tolerated, and most AEs were grade 1 or 2
- Most common treatment-emergent AEs were periorbital edema, anemia, diarrhea, fatigue, peripheral edema, nausea, thrombocytopenia, vomiting and cognitive effects
- Across all doses, 6 patients discontinued treatment due to treatment-related AEs

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1. EXPLORER trial data reported on December 8, 2019. Data cutoff: August 30, 2019. 2. ORR defined as complete remission with full or partial recovery of peripheral blood counts, partial remission or clinical improvement. 3. Avapritinib granted Breakthrough Therapy Designation for the treatment of advanced SM, including the subtypes of aggressive SM, SM with an associated hematologic neoplasm and mast cell leukemia. 4. After the data cutoff date, one patient with SM and an associated hematologic neoplasm (SM-AHN) of myelodysplastic syndrome had a Grade 5 intracranial bleed. At the time of the bleeding event, the patient had severe thrombocytopenia and experienced a serious injury involving head trauma. DOR, duration of response; OS, overall survival.

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## Fourth quarter & full year 2019 financial results

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<th>Balance Sheet (unaudited)</th>
<th>FY ‘19</th>
<th>FY ‘18</th>
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<tbody>
<tr>
<td>Cash, Cash Equivalents and Investments</td>
<td>$548.0M</td>
<td>$494.0M</td>
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<thead>
<tr>
<th>Statement of Operations (unaudited)</th>
<th>FY ‘19</th>
<th>FY ‘18</th>
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<tbody>
<tr>
<td>Collaboration Revenue</td>
<td>$66.5M</td>
<td>$44.5M</td>
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<tr>
<td>Research &amp; Development Expenses</td>
<td>$331.5M</td>
<td>$243.6M</td>
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<td>General &amp; Administrative Expenses</td>
<td>$96.4M</td>
<td>$47.9M</td>
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<td>Net Loss</td>
<td>$(347.7)M</td>
<td>$(236.6)M</td>
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<thead>
<tr>
<th>Q4 ‘19</th>
<th>Q4 ‘18</th>
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<tbody>
<tr>
<td>Collaboration Revenue</td>
<td>$51.5M</td>
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<td>Research &amp; Development Expenses</td>
<td>$88.6M</td>
</tr>
<tr>
<td>General &amp; Administrative Expenses</td>
<td>$32.3M</td>
</tr>
<tr>
<td>Net Loss</td>
<td>$(66.3)M</td>
</tr>
</tbody>
</table>

Estimated net proceeds of $308.2M from January 2020 follow-on public offering
Based on current operating plans, expect existing cash balance will fund operations into 2H of 2022*

* Includes January 2020 follow-on public offering and anticipated product revenues. Excludes any potential option fees, milestone payments or other payments under collaboration or license agreements.