PRECISION THAT MOVES™
Staying one step ahead of disease

R.S., living with systemic mastocytosis

APRIL 1, 2020
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, fisogatinib, 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 or 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, 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, fisogatinib 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’s 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.

These and other risks and uncertainties are described in greater detail under "Risk Factors” in the Company's filings with the Securities and Exchange Commission ("SEC"), including its most recent Annual Report on Form 10-K, as supplemented by its most recent Quarterly Report on Form 10-Q and any other filings it has made or may make with the SEC in the future. The Company cannot guarantee future results, outcomes, levels of activity, performance, developments, or achievements, and there can be no assurance that its expectations, intentions, anticipations, beliefs, or projections will result or be achieved or accomplished. The forward-looking statements in this presentation are made only as of the date hereof, and except as required by law, the Company undertakes no obligation to update any forward-looking statements contained in this presentation as a result of new information, future events or otherwise.

This presentation also contains estimates, projections and other statistical data made by independent parties and by the Company relating to market size and growth and other data about the Company's industry. These data involve a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. In addition, projections, assumptions and estimates of the Company's future performance and the future performance of the markets in which the Company operates are necessarily subject to a high degree of uncertainty and risk.
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

Q3 2020

• Top-line VOYAGER data for avapritinib in 3L GIST
• 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.
1. Unresectable or metastatic disease. 2. CStone Pharmaceuticals has exclusive rights to develop and commercialize avapritini, 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

### DISCOVERY

<table>
<thead>
<tr>
<th>Avapritinib (KIT &amp; PDGFRA)</th>
<th>PDGFRA GIST1,2,3</th>
<th>MAA</th>
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<tbody>
<tr>
<td>4L GIST1,2</td>
<td>NDA</td>
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<td>3L GIST1,2</td>
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<td>2L GIST1,2</td>
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<td>Advanced SM2</td>
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<td>Indolent SM2</td>
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<tr>
<th>Pralsetinib (RET)</th>
<th>2L RET+ NSCLC1,2</th>
<th>NDA</th>
<th>MAA4</th>
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<tr>
<td>1L RET+ NSCLC1,2</td>
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<td>EGFR+ NSCLC (+osimertinib)1,2</td>
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<td>2L MTC1,2</td>
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<tr>
<td>1L MTC1,2</td>
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<tr>
<td>Other RET altered solid tumors1,2</td>
<td>NDA</td>
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<th>Fisogatinib (FGFR4)</th>
<th>Advanced HCC2</th>
<th>NDA</th>
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<tbody>
<tr>
<td>Advanced HCC ( +CS-1001)2</td>
<td>NDA</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

| BLU-263 (KIT)             | Indolent SM      | NDA |     |

| BLU-945 (EGFR+ triple mutant) | EGFR+ NSCLC1 | NDA |     |
| (EGFR+ double mutant)        | EGFR+ NSCLC1   | NDA |     |

| (2 undisclosed targets)      | NDA              |     |     |
| (MAP4K1)5                   | NDA              |     |     |

| (3 undisclosed immunokinase targets)5 | NDA              |     |     |

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Not for promotional use.
Pralsetinib: an investigational precision therapy for RET-altered cancers

**Pralsetinib**
Potent and highly selective RET inhibitor

**U.S. REGULATORY SUBMISSION STATUS**

<table>
<thead>
<tr>
<th>Condition</th>
<th>Status</th>
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<tbody>
<tr>
<td>RET fusion-positive NSCLC</td>
<td>Submitted</td>
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<tr>
<td>Previously treated MTC</td>
<td>Q2 2020*</td>
</tr>
<tr>
<td>Other RET-altered tumors</td>
<td></td>
</tr>
</tbody>
</table>

* 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


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

**ARROW**

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

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**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
- **Multi-kinase inhibitors:** ↓ activity, ↑ off-target toxicity
- Growing understanding of RET-driven resistance
- No selective RET inhibitors are approved

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

**61%**

**ORR**

RET-fusion NSCLC with prior platinum chemotherapy
400 mg QD, N=80

**73%**

**ORR**

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


1. Two responses pending confirmation. 2. All responses confirmed. DOR, duration of response; NE, not estimable.

Not for promotional use.
• **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

RET-altered thyroid cancer patients may benefit from highly targeted therapy

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

60% ORR¹
RET-mutated MTC with prior cabozantinib and/or vandetinib treatment
400 mg QD, N=53

74% ORR²
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%
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

- **100%** of evaluable patients had tumor reductions
  - Median DOR not reached (95% CI: 8.2, NE) in patients treated with 400 mg QD


Not for promotional use.
Pralsetinib is a potential best-in-class selective RET inhibitor and the cornerstone of our lung cancer portfolio.


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Avapritinib: a precision therapy with broad potential

Avapritinib
Potent and highly selective
KIT and PDGFRA inhibitor

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.

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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.

Not for promotional use.
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

Important safety information and full prescribing information are available at www.AYVAKIT.com. ¹Adverse reactions in 204 patients with unresectable or metastatic GIST who received 300-400 mg once daily of AYVAKIT. CNS, central nervous system.

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Systemic mastocytosis is one disease driven by KIT D816V

Advanced SM

Non-advanced SM
(Indolent and smoldering)

Debilitating symptoms

Significant organ involvement

Requirement of high intensity treatment

Requirement for life-long chronic treatment

~75,000 patients in major markets

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. Not for promotional use.
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


Not for promotional use.
Avapritinib improves all symptoms assessed by the ISM-SAF

Avapritinib improves all quality of life domains measured by the MC-QoL

Avapritinib 25 mg QD

Placebo

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

Not for promotional use.
Avapritinib demonstrates clinically meaningful changes in disease severity, as measured by the MC-QoL

**MC-QoL DISEASE SEVERITY\(^1,2\)**

(Baseline to Week 16)

- **Severe**
  - Affected multiple times per day

- **Moderate**
  - Affected multiple times per week

- **Mild**
  - Affected multiple times per month

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

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

Avapritinib 25 mg QD (n=7)  
Placebo (n=6)


Not for promotional use.
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.

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

<table>
<thead>
<tr>
<th>Preferred term</th>
<th>Placebo 25 mg</th>
<th>Placebo n=9</th>
<th>Avapritinib 25 mg n=10</th>
</tr>
</thead>
<tbody>
<tr>
<td>% of subjects with ≥1 AE</td>
<td>any grade</td>
<td>grade 3</td>
<td>any grade</td>
</tr>
<tr>
<td>Nausea</td>
<td>22</td>
<td>0</td>
<td>10</td>
</tr>
<tr>
<td>Dizziness</td>
<td>22</td>
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<tr>
<td>Bone Pain</td>
<td>22</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**

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

Not for promotional use.
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

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Fourth quarter & full year 2019 financial results

### Balance Sheet (unaudited)

<table>
<thead>
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<th></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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### Statement of Operations (unaudited)

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<th></th>
<th>Q4 '19</th>
<th>Q4 '18</th>
<th>FY '19</th>
<th>FY '18</th>
</tr>
</thead>
<tbody>
<tr>
<td>Collaboration Revenue</td>
<td>$51.5M</td>
<td>$1.0M</td>
<td>$66.5M</td>
<td>$44.5M</td>
</tr>
<tr>
<td>Research &amp; Development Expenses</td>
<td>$88.6M</td>
<td>$70.5M</td>
<td>$331.5M</td>
<td>$243.6M</td>
</tr>
<tr>
<td>General &amp; Administrative Expenses</td>
<td>$32.3M</td>
<td>$13.6M</td>
<td>$96.4M</td>
<td>$47.9M</td>
</tr>
<tr>
<td>Net Loss</td>
<td>$(66.3)M</td>
<td>$(80.3)M</td>
<td>$(347.7)M</td>
<td>$(236.6)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.