PRECISION THAT MOVES™
Staying one step ahead of disease

J.P. Morgan Healthcare Conference
JANUARY 13-16, 2020

R.T., living with GIST
Forward-looking statements

This presentation contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended. The words “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 Blueprint Medicines’ 2020 key milestones; Blueprint Medicines’ plans, strategies, timelines and expectations for the preclinical and clinical development and commercialization of AYVAKIT™ (avapritinib), pralsetinib, fisogatinib, and BLU-263; 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 Blueprint Medicines’ current and future drug candidates in treating patients; expectations regarding Blueprint Medicines’ existing cash, cash equivalents and investments; and Blueprint Medicines’ strategy, goals and anticipated milestones, business plans and focus. Blueprint Medicines has based these forward-looking statements on management’s current expectations, assumptions, estimates and projections. While Blueprint Medicines 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 Blueprint Medicines’ 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 delay of any current or planned clinical trials or the development of Blueprint Medicines’ drug candidates, including avapritinib, pralsetinib, fisogatinib and BLU-263, or the licensed products, including BLU-782; Blueprint Medicines’ advancement of multiple early-stage efforts; Blueprint Medicines’ 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 Blueprint Medicines’ 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; Blueprint Medicines’ ability to obtain, maintain and enforce patent and other intellectual property protection for any drug candidates it is developing; Blueprint Medicines’ ability and plans for establishing a commercial infrastructure, and successfully launching, marketing and selling its current or future approved products; Blueprint Medicines’ ability to successfully expand the indications for AYVAKIT in the future; Blueprint Medicines’ ability to develop and commercialize companion diagnostic tests for its current and future drug candidates; and the success of Blueprint Medicines’ current and future collaborations, partnerships, and license, including its collaborations with F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc. (collectively, “Roche”) and CStone Pharmaceuticals (“CStone”) and its license agreement with Clementia Pharmaceuticals Inc. (“Clementia”).

These and other risks and uncertainties are described in greater detail under “Risk Factors” in Blueprint Medicines’ filings with the Securities and Exchange Commission (“SEC”), including Blueprint Medicines’ most recent Quarterly Report on Form 10-Q and any other filings Blueprint Medicines has made or may make with the SEC in the future. Blueprint Medicines cannot guarantee future results, outcomes, levels of activity, performance, developments, or achievements, and there can be no assurance that Blueprint Medicines’ 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, Blueprint Medicines 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 Blueprint Medicines relating to market size and growth and other data about Blueprint Medicines’ 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 Blueprint Medicines’ future performance and the future performance of the markets in which Blueprint Medicines operates are necessarily subject to a high degree of uncertainty and risk.
2020 Blueprint: three key themes

Fully integrated commercial-stage company, with multiple planned global regulatory submissions for avapritinib and pralsetinib

Expanded strategic focus on systemic mastocytosis and related mast cell disorders

Continuous strengthening of pipeline, with plans to nominate up to 3 development candidates this year

SM, systemic mastocytosis. 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.

Not for promotional use.
The rapid evolution of Blueprint Medicines

IMAGINING A NEW PLATFORM | BUILDING THE PIPELINE | REALIZING THE VISION
---|---|---

HIGHLY SELECTIVE KINASE MEDICINE DISCOVERY PLATFORM

RAPID CLINICAL PROOF-OF-CONCEPT ACROSS MULTIPLE PROGRAMS

Avapritinib in advanced systemic mastocytosis: change in serum tryptase

Integrated commercialization
Indication expansion
Therapeutic area leadership
Innovative kinase biology


Not for promotional use.
A powerful vision for delivering durable benefit with targeted therapy

HIGHLY SELECTIVE INHIBITORS
- Potent inhibition of genetic drivers leads to rapid, deep and durable responses

PATIENT SELECTION
- Understanding of disease heterogeneity enables responder hypotheses

ADAPTIVE ABILITY
- Research engine rapidly empowers solutions for acquired resistance
Expand applications to reach broader patient populations

1. Target single driver
   - Selective and potent inhibitor

2. Fast expansion on early POC

3. Patient selection strategy
   - Address disease heterogeneity

4. Combination and sequential treatment approaches
   - Address disease evolution

5. Tumor agnostic treatment and preventative combinations

Change the paradigm

Expand to new populations

Discrete → Patient population → Expanded

POC, proof of concept
Not for promotional use.
Build therapeutic leadership by leveraging insights and efficiencies

- Next-generation inhibitors
- Combination strategies
- Enhanced patient selection

TRANSLATIONAL INSIGHTS

Not for promotional use.
1. Approved in the U.S. for unresectable or metastatic GIST harboring a PDGFRA exon 18 mutant, including PDGFRA D842V mutations.

2. The proposed MAA indication is unresectable or metastatic GIST harboring a PDGFRA D842V mutation.

3. Represents planned NDA/MAA submissions.

GIST, gastrointestinal stromal tumor; MAA, marketing authorization application; MTC, medullary thyroid cancer; NDA, new drug application; NSCLC, non-small cell lung cancer; SM, systemic mastocytosis; 2L, second-line; 3L, third-line; 4L, fourth-line

MULTIPLE ANTICIPATED COMMERCIAL LAUNCHES THROUGH 2021

<table>
<thead>
<tr>
<th>Year</th>
<th>Q1 2020</th>
<th>Q2 2020</th>
<th>Q3 2020</th>
<th>Q4 2020</th>
<th>2021</th>
</tr>
</thead>
<tbody>
<tr>
<td>GIST</td>
<td>US - PDGFRA GIST&lt;sup&gt;1&lt;/sup&gt;</td>
<td>US - 4L GIST</td>
<td>EU - PDGFRA GIST&lt;sup&gt;2&lt;/sup&gt;</td>
<td>US - 3L GIST</td>
<td></td>
</tr>
<tr>
<td>SM</td>
<td>avapritinib</td>
<td>US - Advanced SM</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>RET</td>
<td>pralsetinib</td>
<td>US - NSCLC</td>
<td>US - 2L MTC</td>
<td>EU - NSCLC</td>
<td></td>
</tr>
</tbody>
</table>

1. Approved in the U.S. for unresectable or metastatic GIST harboring a PDGFRA exon 18 mutant, including PDGFRA D842V mutations. 2. The proposed MAA indication is unresectable or metastatic GIST harboring a PDGFRA D842V mutation. 3. Represents planned NDA/MAA submissions. GIST, gastrointestinal stromal tumor; MAA, marketing authorization application; MTC, medullary thyroid cancer; NDA, new drug application; NSCLC, non-small cell lung cancer; SM, systemic mastocytosis; 2L, second-line; 3L, third-line; 4L, fourth-line

Not for promotional use.
Avapritinib: a precision therapy with broad potential

Avapritinib
Potent and highly selective KIT and PDGFRA inhibitor

<table>
<thead>
<tr>
<th>LATE CLINICAL DEVELOPMENT</th>
<th>U.S. REGULATORY SUBMISSION STATUS</th>
</tr>
</thead>
<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. 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.

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](http://www.AYVAKIT.com).

Not for promotional use.
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>
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

¹ Adverse reactions in 204 patients with unresectable or metastatic GIST who received 300-400 mg once daily of AYVAKIT. CNS, central nervous system.
Strategic imperatives for the AYVAKIT launch

Be recognized as the leader in precision medicine by hematology/oncology centers of excellence

Drive positive first experiences with AYVAKIT among GIST prescribers

Provide best-in-class patient support to optimize patient access and adherence

Catalyze patient identification in GIST and across portfolio therapeutic areas

Focused portfolio field footprint with ~40 area business managers
Systemic mastocytosis is one disease with a common genetic driver. ~95% of patients have KIT D816V mutation.

- **Indolent SM**
  - Debilitating symptoms
  - Requirement for long-term therapy

- **Advanced SM**
  - Life-threatening impact
  - Requirement for high treatment intensity

75,000 prevalent patients in major markets

Comprehensive systemic mastocytosis clinical trial program

**EXPLORER**
Advanced SM
Phase 1 dose-escalation trial with open-label expansion

**PATHFINDER**
Advanced SM
Phase 2 single-arm trial

**PIONEER**
Indolent SM
Phase 2 randomized, double-blind, placebo-controlled trial
EXPLORER trial data for patients with advanced SM:
Profound activity on all measures of mast burden in nearly all patients

EXPLORER trial data reported on December 8, 2019. Data cutoff: August 30, 2019.
Not for promotional use.
EXPLORER trial data for patients with advanced SM:
Remarkable response rate and prolonged duration of response

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

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. AE, adverse events. DOR, duration of response; ORR, overall response rate; OS, overall survival.

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

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

77% Confirmed ORR
Median DOR and OS not reached

SAFETY
ALL DOSES (N=80)

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. AE, adverse events. DOR, duration of response; ORR, overall response rate; OS, overall survival.

Not for promotional use.
Adjustment of NDA submission timing for avapritinib for advanced SM enhances dataset at 200 mg QD dose and increases probability of success

**COMBINED EXPLORER AND PATHFINDER TRIAL DATASET**

- Q1 2020
  - Safety Population
    - N= 101
    - n= 15

- 2H 2020*
  - Safety Population
    - N= ~135
    - n= ~50

* Based on ongoing discussions with FDA, now plan to submit supplemental NDA for avapritinib for advanced SM in 2H 2020

- Plan to include additional patients treated with a starting dose of 200 mg QD, the proposed indicated dose

- Target enrollment for efficacy in PATHFINDER trial is complete and follow-up is ongoing

 Patients with starting dose of 200 mg QD and IWG evaluable

 Patients with all other starting doses or not IWG evaluable

* Estimated based on Blueprint Medicines’ clinical trial plan. QD, once daily.

Not for promotional use.
EXPLORER trial data for patients with indolent SM:
Robust reductions on measures of mast cell burden


Not for promotional use.
EXPLORER trial data for patients with indolent SM: Improvement in disease symptoms and PRO survey total symptom score

EXPLORER trial data analysis. Data cutoff date: August 30, 2019. PRO, patient reported outcomes.

INDOLENT SM PATIENTS (N=5) vs. ALL PATIENTS (N=39)

Radar plot of mean score at baseline and best TSS reduction (ISM patients)

- Total Symptom Score
- Fatigue
- Abdominal
- Diarrhea
- Nausea
- Spots
- Itching
- Vomit

Mean baseline score vs. Best score reduction

Not for promotional use.
EXPLORER trial data for patients with indolent SM:
Prolonged durations of therapy at low doses

- 14 of 15 (93%) remained on treatment up to nearly 3 years (cycle 36)
- Average dose was 126 mg with 73% treated at 100 mg QD
PIioneer trial data for patients with indolent SM:
All avapritinib doses showed rapid and robust reductions in serum tryptase

SAFETY (N=30) ALL DOSES

• Most reported AEs were grade 1 or 2
• No intracranial bleeding, thrombocytopenia or anemia reported
• No patients discontinued treatment due to an AE

BASELINE CHARACTERISTICS

• Significant symptom burden in every patient enrolled
• 84% of screened patients met minimum symptom burden eligibility requirement
• Baseline median Total Symptom Score was 52 (range: 19–100)


Not for promotional use.
Next steps for the PIONEER trial of avapritinib in indolent SM

- Complete enrollment of dose-finding Part 1
- Report initial safety and serum tryptase data at ASH 2019 Annual Meeting
- Plan to report additional Part 1 data in late-breaking oral abstract at AAAAI 2020 on March 14, 2020
- Complete enrollment of the registration-enabling Part 2 by the end of 2020

AAAAl, American Academy of Allergy, Asthma & Immunology; RP2D, recommended part 2 dose.
BLU-263 was advanced based on insights from avapritinib.

**POTENT**
Sub-nanomolar potency against KIT D816V

**SELECTIVE**
Highly selective for KIT, with low off-target activity

**CNS PROFILE**
Designed to not cross blood-brain barrier

---

PLAN TO INITIATE PHASE 1 TRIAL IN HEALTHY VOLUNTEERS IN 1H 2020

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

<table>
<thead>
<tr>
<th>RET fusion NSCLC</th>
<th>Q1 2020</th>
</tr>
</thead>
<tbody>
<tr>
<td>2L MTC</td>
<td>Q2 2020</td>
</tr>
</tbody>
</table>

**Pralsetinib**
Potent and highly selective RET inhibitor

INITIATED ROLLING NDA SUBMISSION TO FDA FOR RET FUSION NSCLC IN JANUARY 2020

Kinome illustration reproduced courtesy of Cell Signaling Technology, Inc. ([www.cellsignal.com](http://www.cellsignal.com)) (CSTI). The foregoing website is maintained by CSTI, and Blueprint Medicines is not responsible for its content.

Not for promotional use.
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

---


Not for promotional use.
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\(^2\)

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

**FDA BREAKTHROUGH THERAPY DESIGNATIONS**
- for RET+ NSCLC and MTC\(^3\)

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

---

1. Top-line NSCLC data reported on January 8, 2020. Data cutoff date: November 18, 2020. 2. Data presented at ASCO Annual Meeting in June 2019. Data cutoff date: April 28, 2019. 3. FDA has granted breakthrough therapy designations to pralsetinib for the treatment of RET fusion-positive NSCLC that has progressed following platinum-based chemotherapy and RET-mutant MTC that requires systemic treatment and for which there are no acceptable alternative treatments.

Not for promotional use.
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

Centrally reviewed top-line ARROW trial data showed robust and durable clinical activity for pralsetinib in RET fusion NSCLC

**61% ORR¹**

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

**73% ORR²**

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
- Safety results (N=354; 400 mg QD) were consistent with prior data; most reported AEs were grade 1 or 2
- Overall, 4% of patients discontinued treatment due a treatment-related AE

Top-line data from the Phase 1/2 ARROW trial in patients with RET fusion NSCLC. Data cutoff date: November 18, 2019. 1. Two responses pending confirmation.
2. All responses confirmed. CR, complete response; NE, not estimable; PD, progressive disease; PR, partial response; SD, stable disease.

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

ARROW trial data presented at ASCO 2019 showed robust and durable clinical activity for pralsetinib in MTC and other RET-altered cancers.

RET-mutant MTC previously treated with an MKI

400 mg QD, n=16

63% ORR\(^1\)

**ADDITIONAL RESULTS**

- Across all MTC patients, 97% disease control rate
- Median duration of response not reached; all responders remain on treatment with durations up to 15.6 months
- 83% ORR in papillary thyroid cancer\(^3\)
- Additional clinical responses observed in pancreatic cancer and intrahepatic bile duct carcinoma

Data presented at ASCO Annual Meeting in June 2019. Includes MTC patients treated at the recommended Phase 2 dose of 400 mg QD and enrolled as of November 14, 2018 with follow-up through a data cutoff date of April 28, 2019. All responses were investigator assessed. \(^1\) Two responses pending confirmation. \(^3\) Six patients were evaluable for response assessment (3 confirmed PRs, 2 PRs pending confirmation). Cabo, cabozantinib; Vand, vandetanib. Not for promotional use.
A powerful scientific platform with a focused research strategy

**Difficult-to-drug**
Kinase targets that are difficult to drug with existing technologies

**Treatment-resistant**
Kinase targets characterized by alterations promoting resistance to existing therapies

**Novel biology**
New kinase targets identified via computational and cell biology

Nominated potential first-in-class development candidate for resistant EGFR+ triple mutant NSCLC
Plan to nominate up to 2 additional development candidates in 2020
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. Expect to complete rolling NDA in Q1 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. Not for promotional use.

<table>
<thead>
<tr>
<th>Avapritinib (KIT &amp; PDGFRA)</th>
<th>DISCOVERY</th>
<th>EARLY-STAGE DEVELOPMENT</th>
<th>LATE-STAGE DEVELOPMENT</th>
<th>REGULATORY SUBMISSION</th>
<th>APPROVED</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>PDGFRA GIST&lt;sup&gt;1,2,3&lt;/sup&gt;</td>
<td>MAA</td>
<td>U.S.</td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>4L GIST&lt;sup&gt;1,2&lt;/sup&gt;</td>
<td>NDA</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>3L GIST&lt;sup&gt;1,2&lt;/sup&gt;</td>
<td>NDA</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>2L GIST&lt;sup&gt;1,2&lt;/sup&gt;</td>
<td>NDA</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Advanced SM&lt;sup&gt;2&lt;/sup&gt;</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Indolent SM&lt;sup&gt;2&lt;/sup&gt;</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Pralsetinib, formerly BLU-667 (RET)</th>
<th>DISCOVERY</th>
<th>EARLY-STAGE DEVELOPMENT</th>
<th>LATE-STAGE DEVELOPMENT</th>
<th>REGULATORY SUBMISSION</th>
<th>APPROVED</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>2L RET+ NSCLC&lt;sup&gt;1,2&lt;/sup&gt;</td>
<td>NDA&lt;sup&gt;4&lt;/sup&gt; / MAA</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>1L RET+ NSCLC&lt;sup&gt;1,2&lt;/sup&gt;</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>EGFR+ NSCLC (+osimertinib)&lt;sup&gt;1,2&lt;/sup&gt;</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>2L MTC&lt;sup&gt;1,2&lt;/sup&gt;</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Other RET-altered solid tumors&lt;sup&gt;1,2&lt;/sup&gt;</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Fisogatinib, formerly BLU-554 (FGFR4)</th>
<th>DISCOVERY</th>
<th>EARLY-STAGE DEVELOPMENT</th>
<th>LATE-STAGE DEVELOPMENT</th>
<th>REGULATORY SUBMISSION</th>
<th>APPROVED</th>
</tr>
</thead>
<tbody>
<tr>
<td>Advanced HCC&lt;sup&gt;2&lt;/sup&gt;</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Advanced HCC (+CS-1001)&lt;sup&gt;2&lt;/sup&gt;</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>BLU-263 (KIT)</th>
<th>DISCOVERY</th>
<th>EARLY-STAGE DEVELOPMENT</th>
<th>LATE-STAGE DEVELOPMENT</th>
<th>REGULATORY SUBMISSION</th>
<th>APPROVED</th>
</tr>
</thead>
<tbody>
<tr>
<td>Indolent SM</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>(EGFR+ C797S double mutant)</th>
<th>DISCOVERY</th>
<th>EARLY-STAGE DEVELOPMENT</th>
<th>LATE-STAGE DEVELOPMENT</th>
<th>REGULATORY SUBMISSION</th>
<th>APPROVED</th>
</tr>
</thead>
<tbody>
<tr>
<td>EGFR+ NSCLC&lt;sup&gt;1&lt;/sup&gt;</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>(EGFR+ T790M/C797S triple mutant)</th>
<th>DISCOVERY</th>
<th>EARLY-STAGE DEVELOPMENT</th>
<th>LATE-STAGE DEVELOPMENT</th>
<th>REGULATORY SUBMISSION</th>
<th>APPROVED</th>
</tr>
</thead>
<tbody>
<tr>
<td>EGFR+ NSCLC&lt;sup&gt;1&lt;/sup&gt;</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>(2 undisclosed targets)</th>
<th>DISCOVERY</th>
<th>EARLY-STAGE DEVELOPMENT</th>
<th>LATE-STAGE DEVELOPMENT</th>
<th>REGULATORY SUBMISSION</th>
<th>APPROVED</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>(MAP4K1)&lt;sup&gt;5&lt;/sup&gt;</th>
<th>DISCOVERY</th>
<th>EARLY-STAGE DEVELOPMENT</th>
<th>LATE-STAGE DEVELOPMENT</th>
<th>REGULATORY SUBMISSION</th>
<th>APPROVED</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>(3 undisclosed immunokinase targets)&lt;sup&gt;5&lt;/sup&gt;</th>
<th>DISCOVERY</th>
<th>EARLY-STAGE DEVELOPMENT</th>
<th>LATE-STAGE DEVELOPMENT</th>
<th>REGULATORY SUBMISSION</th>
<th>APPROVED</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
Strong financial position entering 2020

**Balance Sheet**

<table>
<thead>
<tr>
<th></th>
<th>September 30, 2019*</th>
<th>December 31, 2018</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cash, Cash Equivalents and Investments</td>
<td>$594.5M</td>
<td>$494.0M</td>
</tr>
</tbody>
</table>

**Statement of Operations**

<table>
<thead>
<tr>
<th></th>
<th>Three Months Ended September 30, 2019*</th>
<th>Three Months Ended September 30, 2018*</th>
</tr>
</thead>
<tbody>
<tr>
<td>Collaboration Revenue</td>
<td>$9.1M</td>
<td>$1.1M</td>
</tr>
<tr>
<td>Research &amp; Development Expenses</td>
<td>$81.5M</td>
<td>$64.6M</td>
</tr>
<tr>
<td>General &amp; Administrative Expenses</td>
<td>$25.6M</td>
<td>$12.0M</td>
</tr>
<tr>
<td>Net Loss</td>
<td>$(94.3)M</td>
<td>$(72.7)M</td>
</tr>
</tbody>
</table>

Based on current operating plans, expect existing cash balance will fund operations into 2H 2021**

* Unaudited
** Includes $25.0 million upfront cash payment from Clementia and $8.0 million research milestone achieved in the fourth quarter of 2019 under the Roche collaboration but excludes any additional potential option fees, milestone payments or other payments from Roche, CStone or Clementia.
Anticipated 2020 milestones

**REGULATORY APPROVALS**
- Avapritinib in fourth-line GIST in the U.S. in Q2 2020
- Avapritinib in PDGFRA D842V GIST in the EU in Q3 2020
- Pralsetinib in RET+ NSCLC in the U.S. by the end of 2020

**REGULATORY SUBMISSIONS**
- Pralsetinib NDA to FDA for RET+ NSCLC in Q1 2020
- Pralsetinib NDA to FDA for 2L RET+ MTC in Q2 2020
- Avapritinib sNDA to FDA for advSM in 2H 2020
- Avapritinib sNDA to FDA for 3L GIST in 2H 2020
- Pralsetinib MAA to EMA for RET+ NSCLC in Q2 2020

**TOP-LINE REGISTRATION DATA**
- Avapritinib VOYAGER trial in 3L GIST in Q2 2020

**MEDICAL MEETING PRESENTATIONS**
- Avapritinib PIONEER trial Part 1 in ISM in Q1 2020
- Pralsetinib ARROW trial in RET+ NSCLC in 2020
- Pralsetinib ARROW trial in RET+ MTC in 2020
- Avapritinib VOYAGER trial in 3L GIST in 2020
- Avapritinib EXPLORER and PATHFINDER trials in advSM in 2H 2020

**COMPLETE TRIAL ENROLLMENT**
- Avapritinib PIONEER trial Part 2 in ISM by the end of 2020

**TRIAL INITIATIONS**
- BLU-263 Phase 1 trial in healthy volunteers in 1H 2020
- Pralsetinib Phase 3 trial in 1L MTC in 2H 2020

**RESEARCH PIPELINE**
- Nominate up to 3 development candidates in 2020

advSM, advanced systemic mastocytosis; sNDA, supplemental new drug application.

Not for promotional use.