PRECISION THAT MOVES™

Staying one step ahead of disease

October 5, 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), GAVRETO™ (pralsetinib) and other current or future drug candidates; plans, timing, design, initiation, enrollment, expectations and announcement of results for the Company's ongoing and planned clinical trials; plans and timelines for additional marketing applications for avapritinib and pralsetinib and, if approved, commercializing avapritinib and pralsetinib in additional geographies or for additional indications; the potential benefits of any of the Company's current or future approved drugs or drug candidates in treating patients; the potential benefits of the Company's collaboration with Roche and Genentech for pralsetinib, including anticipated milestone payments and other financial terms of the collaboration agreement; 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 AYVAKIT, GAVRETO or future approved drugs, and launching, marketing and selling AYVAKIT, GAVRETO or future approved drugs; the delay of any current or planned clinical trials or the development of the Company's drug candidates or any 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 AYVAKIT, GAVRETO or any drug candidates it is developing; the Company' ability and plans for maintaining a commercial infrastructure, and successfully launching, marketing and selling AYVAKIT, GAVRETO or future approved drugs; the Company's ability to develop and commercialize companion diagnostic tests for AYVAKIT, GAVRETO or any of the Company's future approved drugs or drug candidates; and the success of the Company's current and future collaborations, partnerships and licenses, including the Company's global collaboration with Roche for the development and commercialization of pralsetinib.

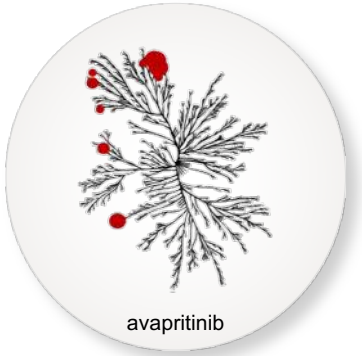
These and other risks and uncertainties are described in greater detail under "Risk Factors" in the Company's filings with the Securities and Exchange Commission ("SEC"), including its most recent Annual Report on Form 10-K, as supplemented by its most recent Quarterly Report on Form 10-Q, and any other 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.

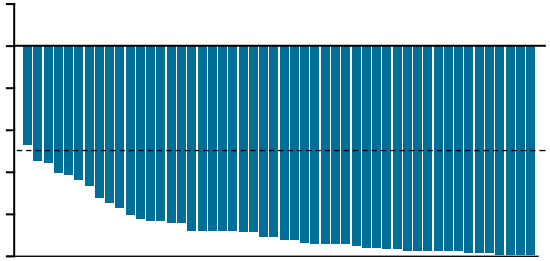
The rapid evolution of Blueprint Medicines

IMAGINING A NEW PLATFORM	BUILDING THE PIPELINE	REALIZING THE VISION
2011 – 2014	2015 – 2019	2020 – FUTURE

HIGHLY SELECTIVE KINASE MEDICINE DISCOVERY PLATFORM



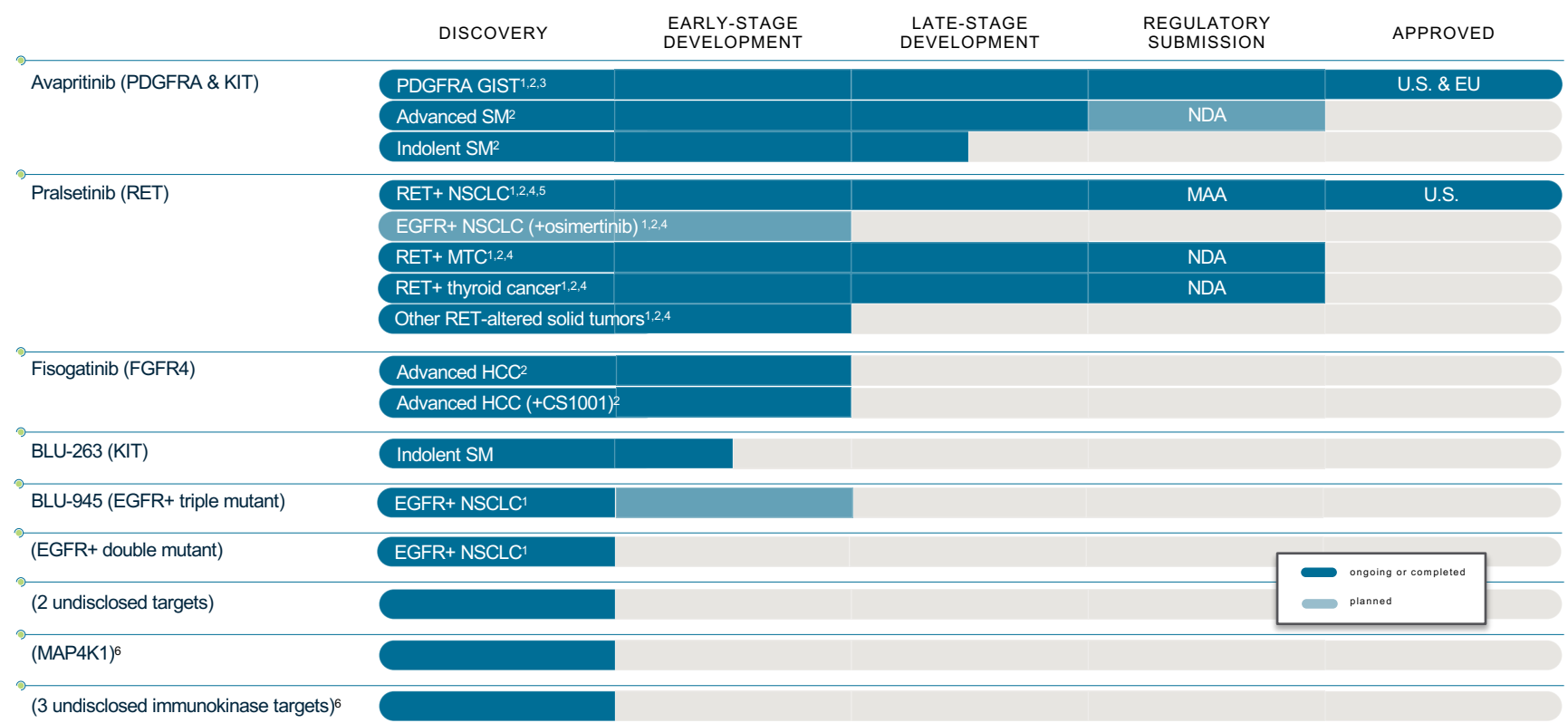
RAPID CLINICAL PROOF-OF-CONCEPT ACROSS MULTIPLE PROGRAMS



Avapritinib in advanced systemic mastocytosis: change in serum tryptase[1]

- Integrated commercialization
- Indication expansion
- Therapeutic area leadership
- Innovative kinase biology

1 Data presented at the European Hematology Association Annual Meeting in June 2019. Data cutoff date: January 2, 2019. 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.

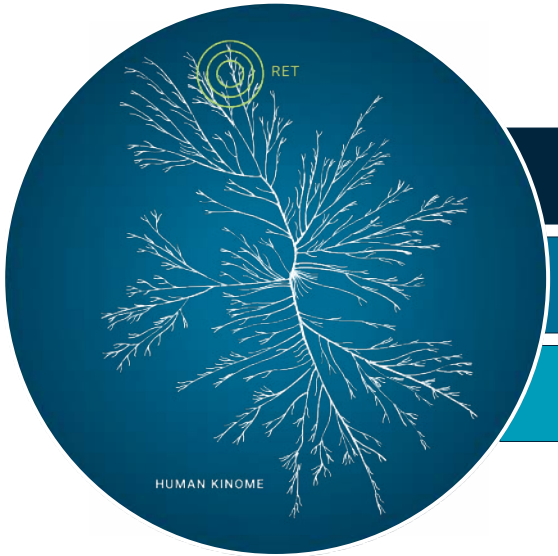


1. Unresectable or metastatic disease. 2. CStone Pharmaceuticals has exclusive rights to develop and commercialize avapritinib, pralsetinib and fisogatinib in Mainland China, Hong Kong, Macau and Taiwan. 3. Approved in the U.S. for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA exon 18 mutation, including PDGFRA D842V mutations. Received conditional marketing authorization in Europe for the treatment of adults with unresectable or metastatic GIST harboring the PDGFRA D842V mutation. 4. In collaboration with Roche. Blueprint Medicines and Roche have co-exclusive rights to develop and commercialize pralsetinib in the U.S., and Roche has exclusive rights to develop and commercialize pralsetinib outside the U.S., excluding the CStone territory. 5. Received accelerated approval in the U.S. for the treatment of adults with metastatic RET fusion-positive NSCLC. Continued approval may be contingent on a confirmatory trial. The proposed indication for the MAA is locally advanced or metastatic RET fusion-positive NSCLC previously treated with platinum-based chemotherapy. 6. 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. GIST, gastrointestinal stromal tumors; HCC, hepatocellular carcinoma; MAA, marketing authorization application; MTC, medullary thyroid cancer; NDA, new drug application; NSCLC, non-small cell lung cancer; SM, systemic mastocytosis.

Updated as of September 25, 2020.

blueprint™ MEDICINES

Pralsetinib: a precision therapy for RET-altered cancers



	LATE CLINICAL DEVELOPMENT	U.S. REGULATORY SUBMISSION STATUS
RET+ NSCLC		APPROVED
RET+ thyroid cancer		NDA accepted
Other RET+ solid tumors		

PRALSETINIB

POTENT AND HIGHLY SELECTIVE RET INHIBITOR

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.

GAVRETO: a precision therapy that can transform NSCLC patients' lives now approved in the United States

GAVRETO™
pralsetinib | 100mg capsules

- Selective and potent mechanism of action inhibiting RET fusions and mutations
- Deep and durable responses regardless of patient baseline characteristics
- Predictable and manageable safety profile that is familiar to oncologists
- Once-daily dosing format that optimizes adherence and ease of dose modification

GAVRETO is indicated for the treatment of adult patients with metastatic RET fusion-positive non-small cell lung cancer (NSCLC) as detected by an FDA approved test. This indication is approved under accelerated approval based on overall response rate and duration of response. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial. FDA, U.S. Food and Drug Administration; RET, rearranged during transfection.

Not for promotional use.

Accelerated approval of GAVRETO based on Phase 1/2 ARROW trial in patients with RET fusion+ NSCLC

EFFICACY PARAMETER	TREATMENT NAÏVE (N=27)	PRIOR PLATINUM (N=87)
Overall response rate (95% confidence interval)	70% (50%, 86%)	57% (46%, 68%)
Complete response	11%	5.7%
Partial response	59%	52%
Duration of response	n=19	N=50
Median in months (range)	9 (6.3, NE)	NE (15.2, NE)
Patients with DOR ≥ 6-mo (%)	58%	80%

Full prescribing information is available at www.GAVRETO.com. DOR, duration of response; NE, not evaluable.

Safety highlights from GAVRETO prescribing information

MOST COMMON ADVERSE REACTIONS (≥25%; ANY GRADE):[1]

- Fatigue, constipation, musculoskeletal pain and hypertension

MOST COMMON LABORATORY ABNORMALITIES (≥2%; GRADE 3-4):[1]

- Decreased lymphocytes, decreased neutrophils, decreased phosphate, decreased hemoglobin, decreased sodium, decreased calcium (corrected) and increased alanine aminotransferase (ALT)

WARNINGS AND PRECAUTIONS:

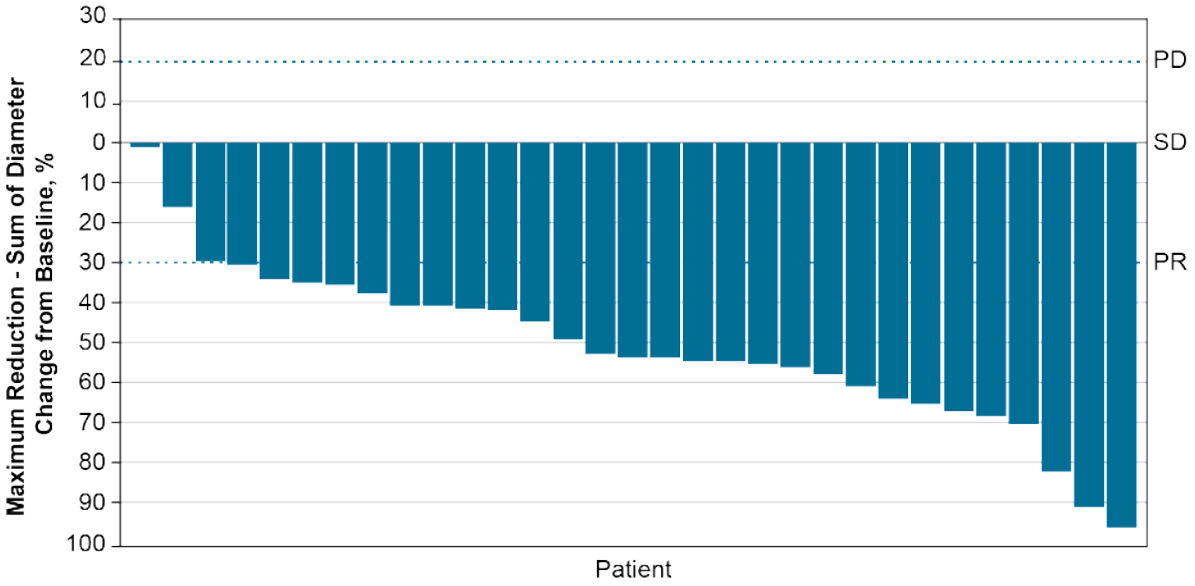
- Interstitial Lung Disease (ILD)/Pneumonitis
- Hypertension
- Hepatotoxicity
- Hemorrhagic Events
- Risk of Impaired Wound Healing
- Embryo-Fetal Toxicity

Important safety information and full prescribing information are available at www.GAVRETO.com. 1. Adverse reactions in 220 patients with metastatic RET fusion-positive NSCLC who received GAVRATO at 400 mg orally once daily.

Not for promotional use.

High response rates in treatment-naïve NSCLC populations consistent with real-world patients

TUMOR SHRINKAGE PER INVESTIGATOR ASSESSMENTS



OVERALL RESPONSE RATE

TREATMENT-NAÏVE PATIENTS

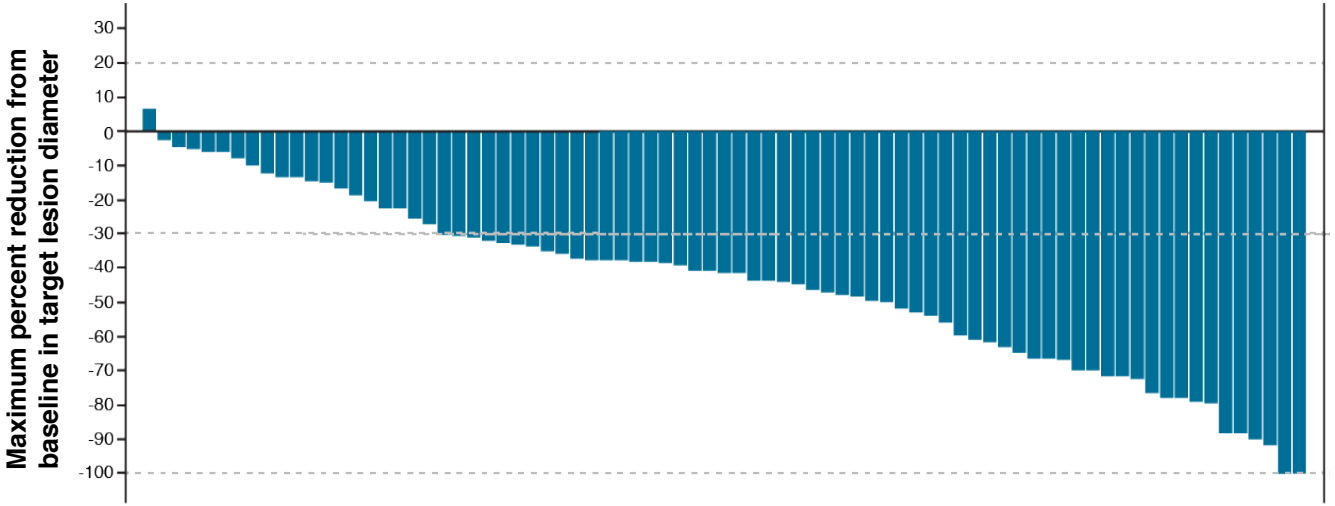
88% **Eligible for standard therapy (n=32)**

Target population for actively enrolling AcceleRET Lung Phase 3 trial in treatment-naïve NSCLC

Patients evaluable for response and enrolled after July 11, 2019. Data cut off: April 24, 2020. Data presented in May 2020 at ASCO 2020 virtual annual meeting.
PD, progressive disease; PR, partial response; SD, stable disease.

Robust clinical activity in MTC patients regardless of prior therapy

TUMOR SHRINKAGE PER CENTRAL RADIOLOGY



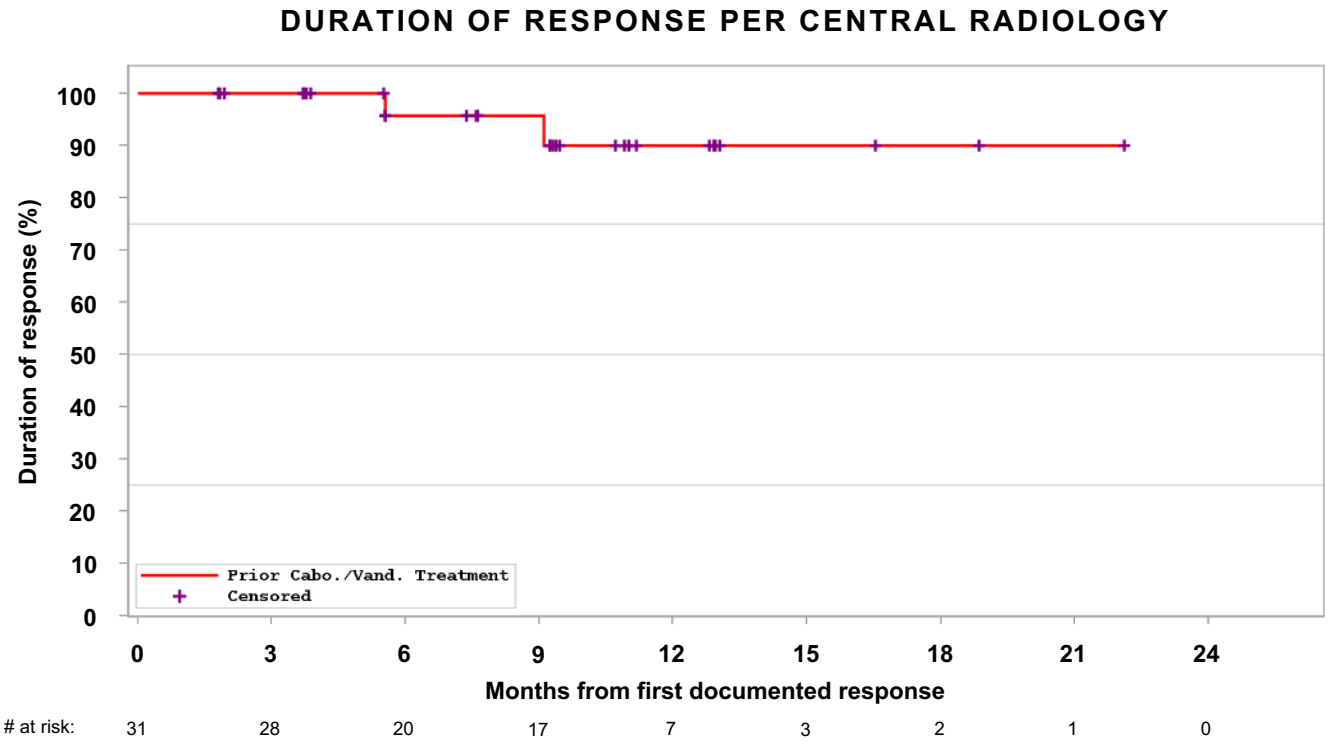
OVERALL RESPONSE RATE

60% (95% CI: 46-74%)	**Prior MKI treatment (n=53)[1]**
74% (95% CI: 49-91%)	**No prior systemic therapy (n=19)**

Tumor shrinkage in 99% of patients regardless of prior therapy

Top-line ARROW trial data in patients with RET+ MTC reported on April 1, 2020. Data cutoff: February 13, 2020. 1. One response pending confirmation. MKI, multi-kinase inhibitor.

Prolonged duration of response in patients with previously treated MTC



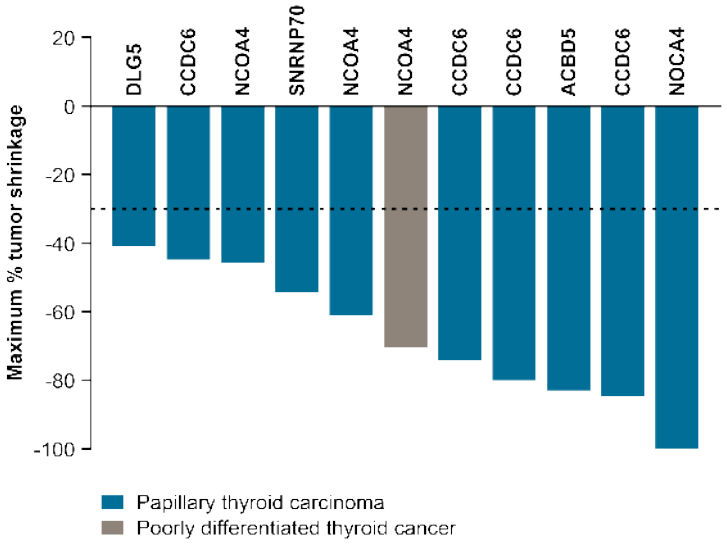
NR
(95% CI: NE-NE)
Median DOR

96%
6-month DOR

Top-line ARROW trial data in patients with RET+ MTC reported on April 1, 2020. Data cutoff: February 13, 2020. NE, not estimable.

Deep and durable responses in patients with RET fusion+ thyroid cancer

TUMOR SHRINKAGE PER CENTRAL RADIOLOGY



RET FUSION+ THYROID CANCER ALL DOSES (N=11)

91% (95% CI: 59-100%) **ORR**

100% **6-month DOR**

10/11 PATIENTS PREVIOUSLY TREATED WITH SYSTEMIC THERAPY

Data presented in May 2020 at ASCO 2020 virtual annual meeting. Data cutoff: February 13, 2020.

Blueprint Medicines and Roche: a transformative partnership for pralsetinib

STRATEGIC IMPERATIVES

1 Bring pralsetinib faster to more patients globally

2 Continue to build best-in-class precision medicine capabilities

3 Transform Blueprint Medicines with path to financial independence

COLLABORATION STRUCTURE & IMPACT

- Co-commercialize pralsetinib in the U.S., equally sharing responsibilities, profits and losses
- Roche granted exclusive license to commercialize pralsetinib outside of the U.S., excluding Greater China[1]
- Co-develop pralsetinib globally, expanding into new treatment settings
- Explore co-development of a next-generation selective RET inhibitor

- Reduce reliance on capital markets to finance company
- Expand investment in systemic mastocytosis and pipeline programs

1. Greater China comprises Mainland China, Hong Kong, Macau and Taiwan.

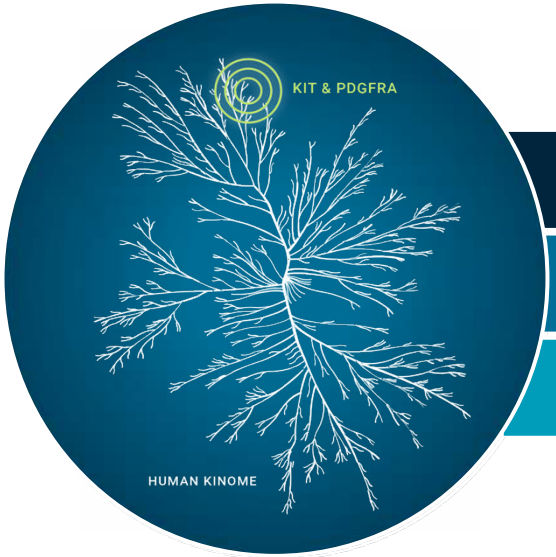
Summary of financial terms

2ND LARGEST BIOPHARMACEUTICAL COLLABORATION IN 2020 TO DATE[1]

UPFRONT	$775M, with $675M in cash and $100M equity investment at $96.57 per share
MILESTONES	Up to $927M in contingent payments, with approximately $90M in potential near-term milestones[2]
ROYALTIES	Tiered high-teens to mid-twenties on aggregate net sales outside U.S.
U.S. P&L	Companies to equally share profits and losses
DEVELOPMENT	Costs shared 45% Blueprint / 55% Roche up to pre-specified limit, then % reduction for Blueprint

1. Based on upfront payment. Source: company information, third party press releases and SEC filings. 2. Blueprint Medicines will receive $40.0 million in specified regulatory and commercialization milestones under the Roche collaboration as a result of FDA approval of GAVRETO (pralsetinib).

Avapritinib: a precision therapy with broad potential



LATE CLINICAL DEVELOPMENT	U.S. REGULATORY SUBMISSION STATUS
PDGFRA exon 18 mutant GIST	APPROVED[1]
Advanced SM	Q4 2020*
Indolent and smoldering SM	

AVAPRITINIB

POTENT AND HIGHLY SELECTIVE KIT AND PDGFRA INHIBITOR

Received European Commission approval for PDGFRA D842V GIST on September 25, 2020

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. Received conditional marketing authorization in Europe for the treatment of adults with unresectable or metastatic GIST harboring the PDGFRA D842V mutation.
* Planned supplemental 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. CHMP, EMA Committee for Medicinal Products for Human Use.

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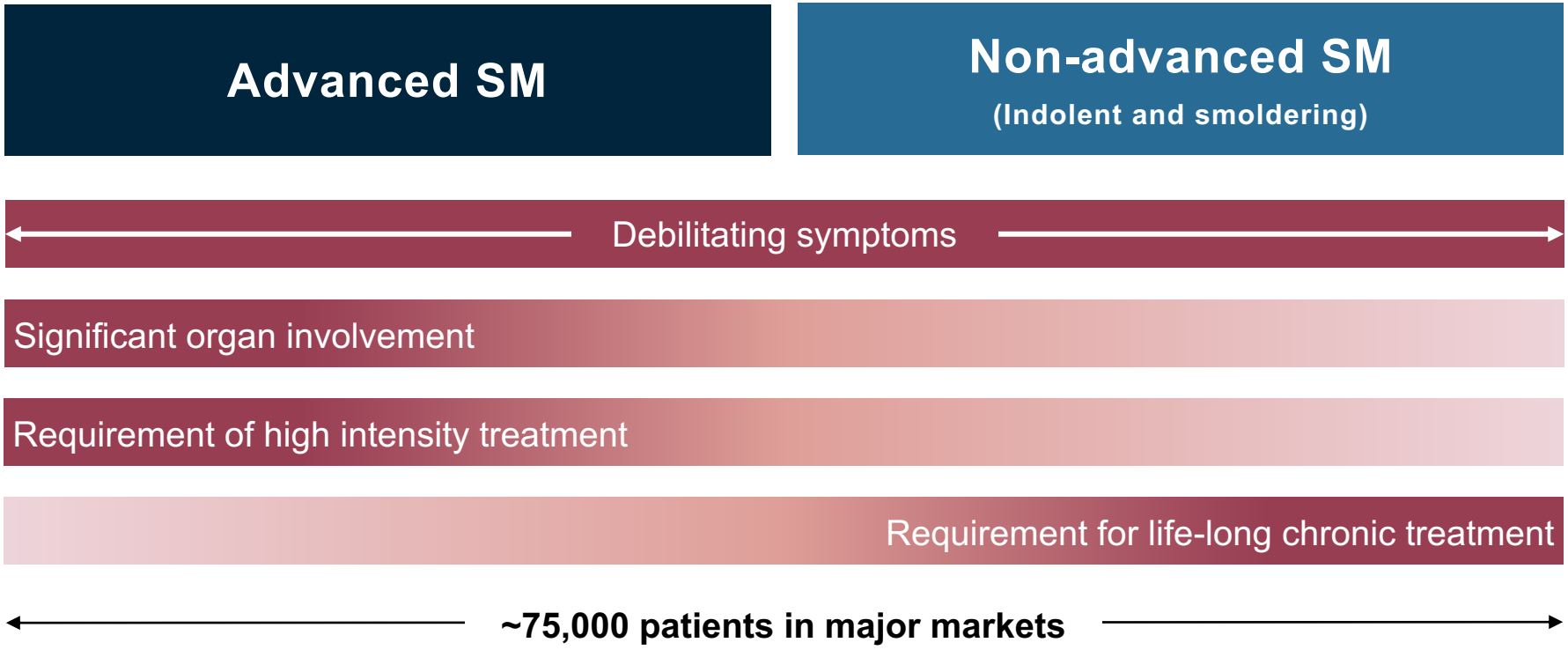
AYVAKIT launch has established a strong foundation for Blueprint Medicines' commercial execution

- ✓ Establish Blueprint Medicines with key centers of excellence
- ✓ Drive broad access to therapy quickly
- ✓ Support patients to start and stay on therapy
- ✓ Strong field engagement to identify new prescribers and drive patient demand

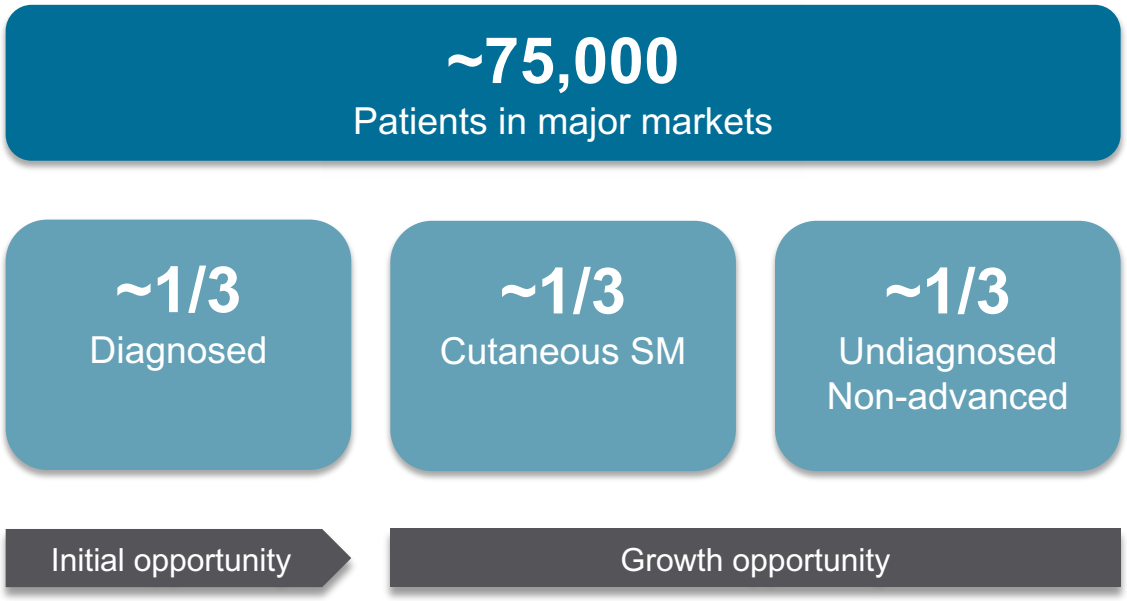
$5.7M in Q2 2020 sales ($9.1M since launch)

Systemic mastocytosis is one disease driven by KIT D816V



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

Significant initial target patient population with additional growth potential

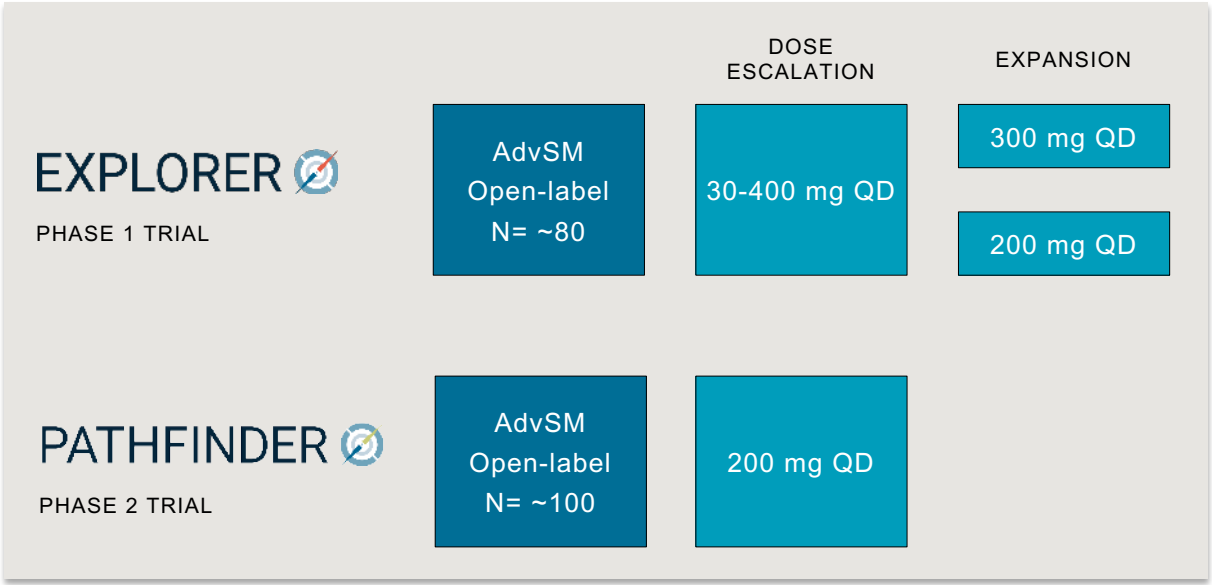


Estimated prevalence[1]
- ~5% advanced SM
- ~95% non-advanced SM

Estimated addressable patients (extrapolated from U.S. claims data and market research analyses)

Major markets include US, EU5 and Japan. 1. Cohen S et al Br J Haematol (2014) 166(4):521-8 and World Bank Population estimates.

Comprehensive development program designed to support avapritinib registration in advanced systemic mastocytosis



PRIMARY ENDPOINT FOR APPROVAL:

OVERALL RESPONSE RATE PER mIWG

mIWG, modified International Working Group-Myeloproliferative Neoplasms Research and Treatment (IWG-MRT) & European Competence Network on Mastocytosis (ECNM) response criteria. AdvSM, advanced systemic mastocytosis; QD, once daily.

Planned avapritinib sNDA submission for advanced SM to include pooled data from the EXPLORER and PATHFINDER trials

	EXPLORER	PATHFINDER	200 MG QD POOLED GROUP
Enrolled	86 patients	62 patients	81 patients
200 mg QD mIWG evaluable	13 patients	31 patients	44 patients
All doses mIWG evaluable	53 patients	32 patients	
Prior midostaurin	32%	53%	

Top-line EXPLORER and PATHFINDER data presented in September 2020. Data cutoff: May 27, 2020 for EXPLORER and June 23, 2020 for PATHFINDER, with response assessments per central review completed in September 2020. sNDA, supplemental NDA.

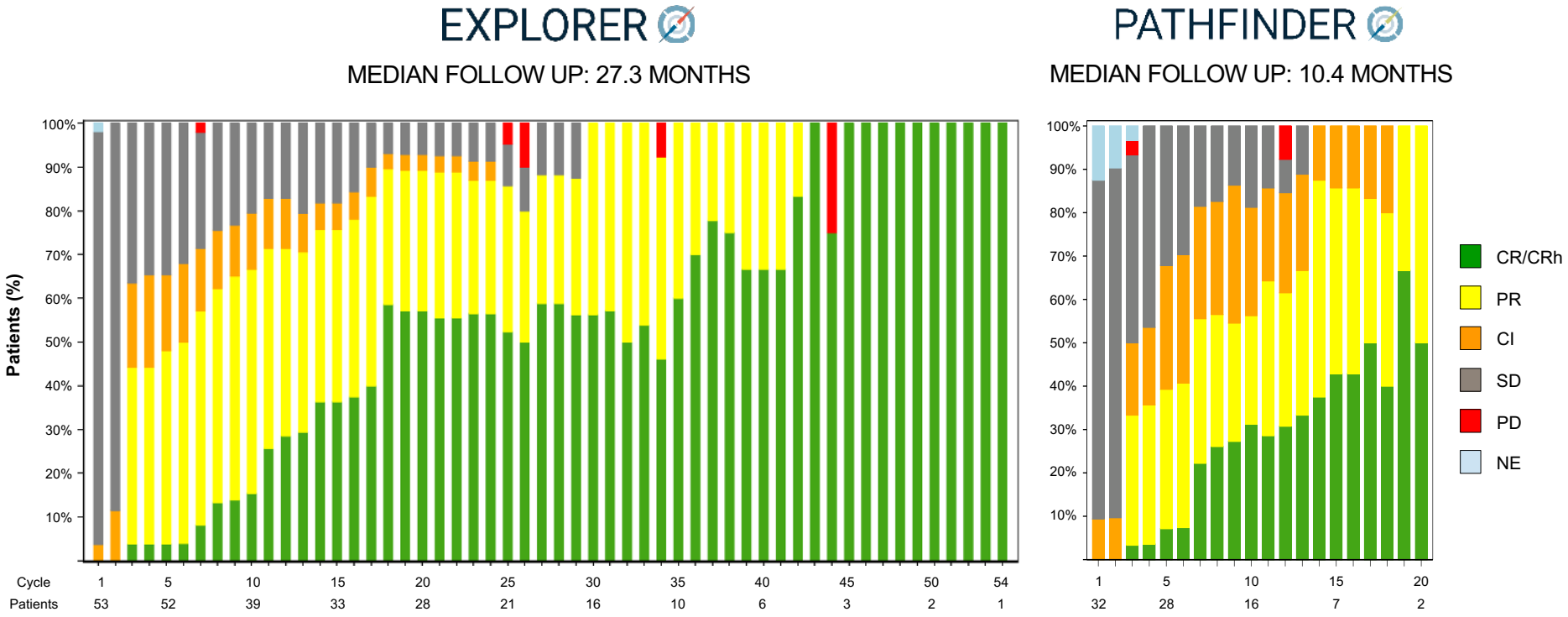
Consistently high ORRs and prolonged duration of response across trials

	EXPLORER	PATHFINDER	200 MG QD POOLED GROUP
ORR (CR+CRh+PR+CI)	75.5% (61.7- 86.2)	75.0 (56.6 – 88.5)	68.2%
CR+CRh	35.8%	18.8%	18.2%
mDOR (months)	38.3 (21.7 - NE)	NE (NE - NE)	
mOS (months)	NE (46.9 - NE)	NE	
	Median follow up: 27.3 months	Median follow up: 10.4 months	Median follow up: 10.4 months

PATHFINDER interim analysis was positive (p-value=0.0000000016)

Top-line EXPLORER and PATHFINDER data presented in September 2020. Data cutoff: May 27, 2020 for EXPLORER and June 23, 2020 for PATHFINDER, with response assessments per central review completed in September 2020.
CR, complete remission; CRh, CR with partial hematologic recovery; CI, clinical improvement; mDOR, median duration of response; mOS, median overall survival; PR, partial remission.

EXPLORER and PATHFINDER response kinetics are similar, with deepening responses over time with longer-term follow up



Top-line EXPLORER and PATHFINDER data presented in September 2020. Data cutoff: May 27, 2020 for EXPLORER and June 23, 2020 for PATHFINDER, with response assessments per central review completed in September 2020.
PD, progressive disease; SD, stable disease.

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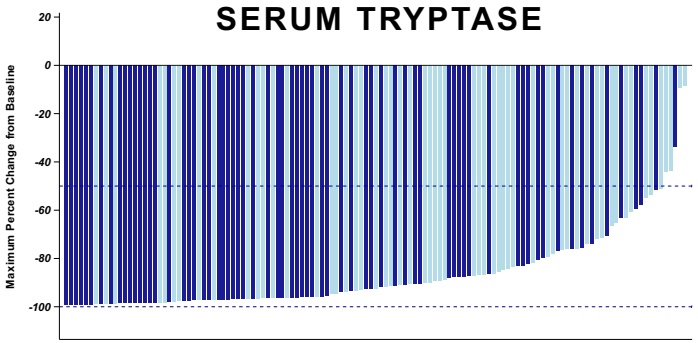
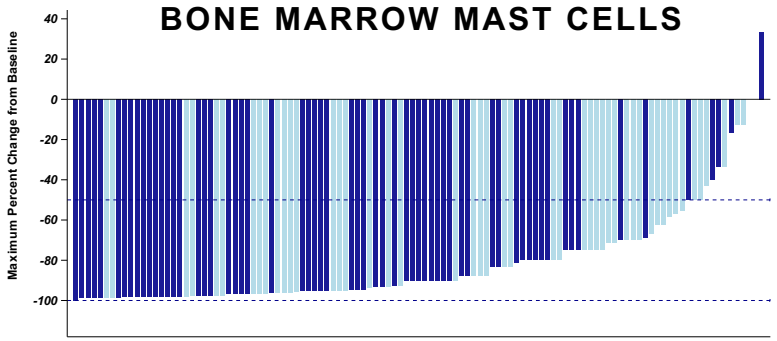
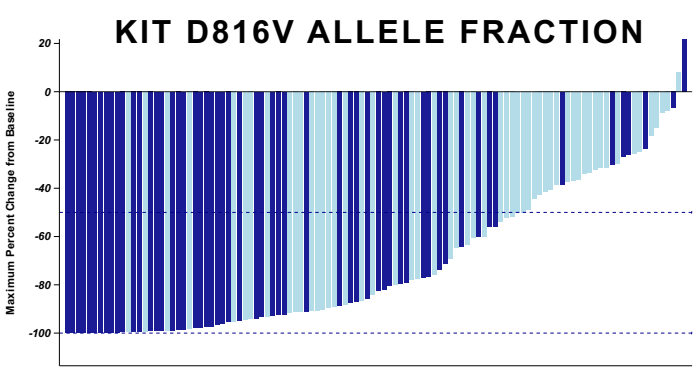
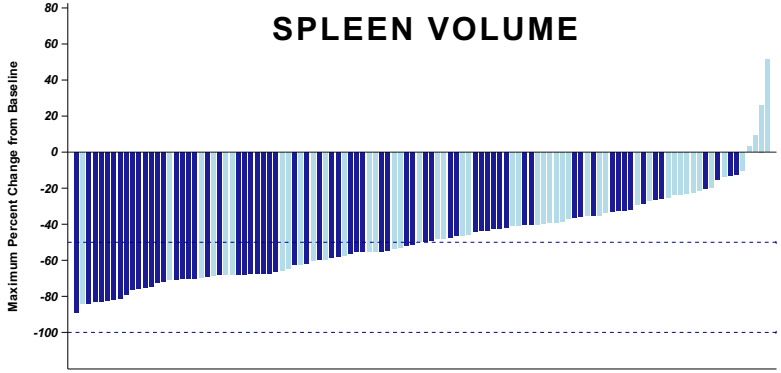
Consistent impact on objective measures of mast cell burden across trials









Explorer

Pathfinder

Top-line EXPLORER and PATHFINDER data presented in September 2020. Data cutoff: May 27, 2020 for EXPLORER and June 23, 2020 for PATHFINDER, with response assessments per central review completed in September 2020.

Avapritinib demonstrated improved tolerability at 200 mg QD

Treatment Emergent AEs ≥ 20%, All Grades*	200 mg n=81 (%)	All doses N=148 (%)
Peripheral Edema	39 (48.1)	65 (43.9)
Periorbital Edema	32 (39.5)	81 (54.7)
Thrombocytopenia	28 (34.6)	55 (37.2)
Anemia	26 (32.1)	65 (43.9)
Diarrhea	23 (28.4)	53 (35.8)
Nausea	20 (24.7)	49 (33.1)
Fatigue	15 (18.5)	44 (29.7)
Vomiting	15 (18.5)	42 (28.4)

* Most common AEs in patients treated at 200mg in EXPLORER and PATHFINDER

Cognitive effects	10 (12.3)	37 (25.0)
≥Grade 2	2 (2.5)	13 (8.8)

- Overall, 8.1% of patients discontinued treatment due to treatment-related AEs
- ICB risk mitigations implemented
  - Starting dose of 200 mg QD
  - Exclusion criteria for pre-existing severe thrombocytopenia
  - Increased platelet monitoring
  - Mandatory dose interruption for severe thrombocytopenia
- ICB events in patients without pre-existing severe thrombocytopenia
  - Pooled 200 mg group (n=76): 2 (2.6%)†
  - PATHFINDER (n=57): 0‡

Top-line EXPLORER and PATHFINDER data presented in September 2020. Data cutoff: May 27, 2020 for EXPLORER and June 23, 2020 for PATHFINDER, with response assessments per central review completed in September 2020.

† Both ICB events in EXPLORER patients were Grade 1 and asymptomatic. ‡ 1 ICB event occurred in a PATHFINDER patient with pre-existing severe thrombocytopenia prior to exclusion of such patients for 1/62 (1.6%) overall. AE, adverse event; ICB, intracranial bleed.

Summary of top-line EXPLORER and PATHFINDER trial results

1	Consistently high IWG response rates across trials and at the proposed labeled 200 mg QD dose, with prolonged overall survival in EXPLORER
2	Avapritinib was generally well-tolerated, with improved safety at 200 mg QD and with platelet management
3	Detailed results expected to be presented at a future scientific congress
4	Plan to submit sNDA to FDA for avapritinib for advanced SM in Q4 2020

Top-line EXPLORER and PATHFINDER data presented in September 2020. Data cutoff: May 27, 2020 for EXPLORER and June 23, 2020 for PATHFINDER, with response assessments per central review completed in September 2020.

Registration-enabling PIONEER Part 2 clinical trial ongoing in non-advance/indolent systemic mastocytosis

PRIMARY ENDPOINT FOR APPROVAL:

CHANGE IN ISM-SAF TOTAL SYMPTOM SCORE

PIONEER REGISTRATION-ENABLING PART 2

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

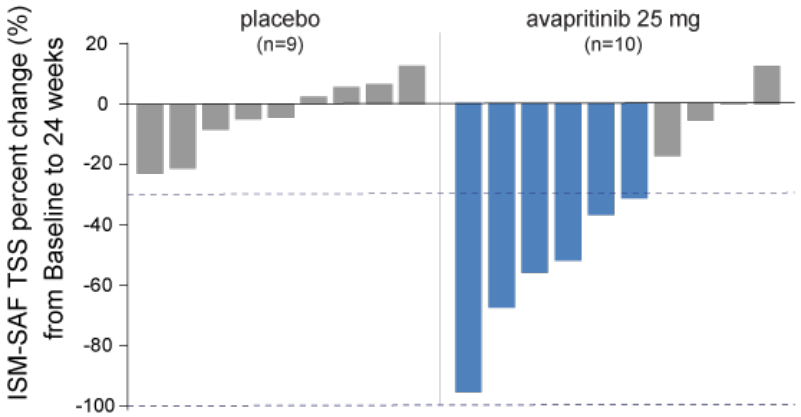
Key endpoints: Response rate defined as ≥30% reduction in ISM-SAF total symptom score (primary), measures of mast cell burden, quality of life, concomitant medications

Duration: 24 weeks

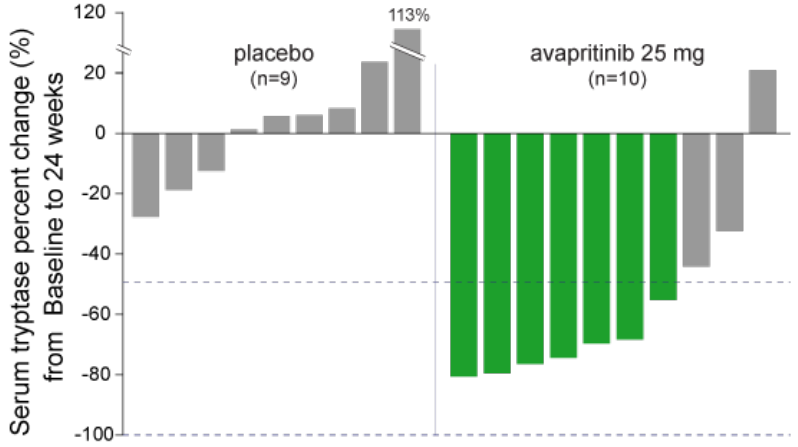
ISM, indolent system mastocytosis; ISM-SAF, indolent systemic mastocytosis – symptom assessment form; RP2D, recommended phase 2 dose.

Avapritinib 25 mg QD reduces symptoms and mast cell burden in indolent SM

Part 2 primary endpoint
≥30% reduction in ISM-SAF Total Symptom Score at 24 weeks



Part 2 first key secondary endpoint
≥50% tryptase reduction at 24 weeks*



Response rate: **0%** **60%** **0%** **70%**

Presented at EAACI Virtual 2020 Congress in June 2020. Data cutoff: March 31, 2020. *24 weeks or last assessment before, if 24 weeks not available. EAACI, European Academy of Allergy and Clinical Immunology.

Safety results for avapritinib 25mg QD are similar to placebo at 16 weeks[1]

AE in >15% of placebo or avapritinib arms	Placebo n=9		avapritinib 25 mg n=10	
Preferred term				
% of subjects with ≥1 AE	any grade	grade 3	any grade	grade 3
	89	**22**	**100**	**0**
Nausea	22	0	10	0
Dizziness	22	0	30	0
Headache	11	0	30	0
Diarrhea	11	0	0	0
Fatigue	11	0	40	0
Face edema	0	0	10	0
Peripheral edema	0	0	10	0
Periorbital edema	0	0	0	0
Bone Pain	22	0	0	0

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**

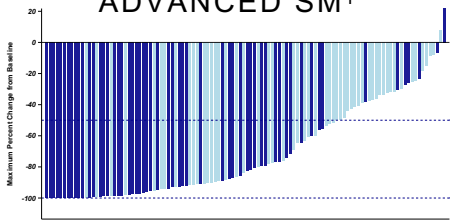
Follow up at 24 weeks showed no ≥grade 3 AEs or discontinuations due to AEs for avapritinib 25 mg QD[2]

1. Data presented in March 2020 at AAAAI annual meeting. Data cutoff: December 27, 2019. 2. Data cutoff: March 31, 2020.

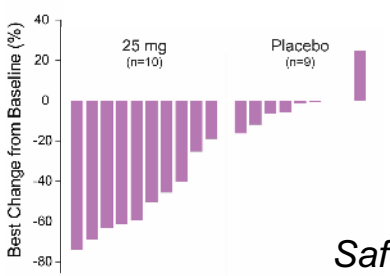
Avapritinib is the only clinically validated, highly potent inhibitor of KIT D816V, the genetic driver of SM

REDUCE MASK CELL BURDEN

ADVANCED SM[1]

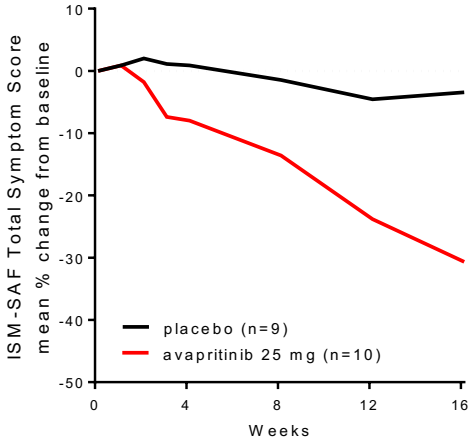


NON-ADVANCED SM[2]



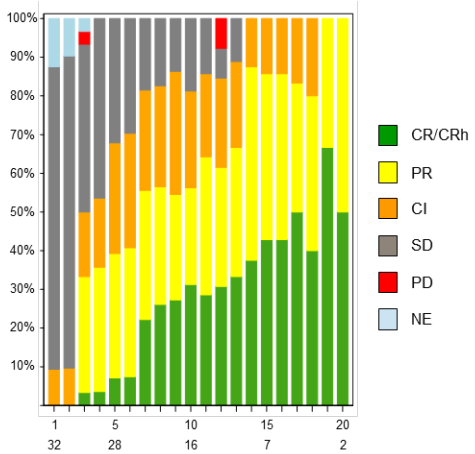
IMPROVE DISEASE SYMPTOMS

NON-ADVANCED SM[2]



INDUCE DEEP AND DURABLE RESPONSES

ADVANCED SM[1]



Safety profile enables tailored dosing based on patient need

1. Top-line EXPLORER and PATHFINDER data presented in September 2020. Data cutoff: May 27, 2020 for EXPLORER and June 23, 2020 for PATHFINDER, with response assessments per central review completed in September 2020.
2. Data reported at AAAAI Annual Meeting in March 2020. Data cutoff: December 27, 2019.

BLU-945 is the first of two 4th-gen inhibitors in development targeting EGFR-driven NSCLC

Comprehensive program targeting osimertinib-resistant EGFRm NSCLC

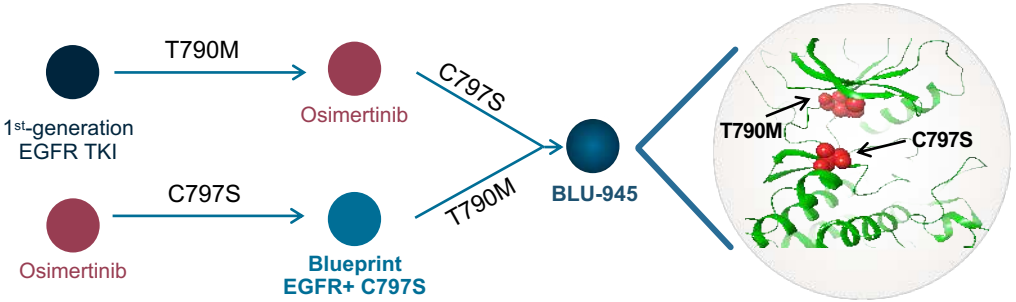
- Multiple research programs targeting the most common on-target resistance mutations

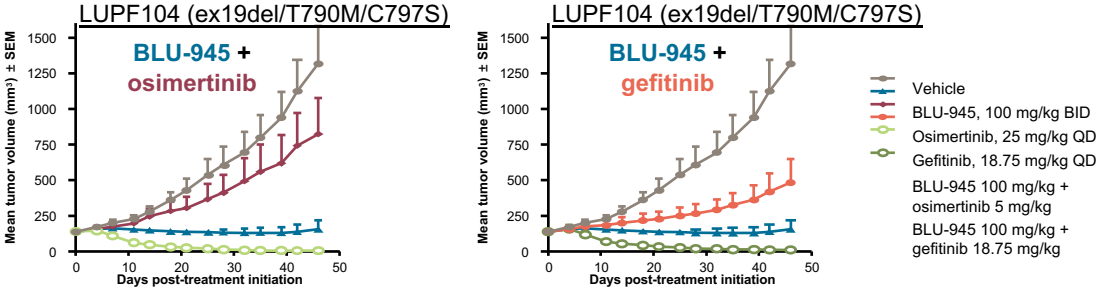
BLU-945 is a potent and selective inhibitor of triple mutant EGFRm NSCLC

- Excellent wild-type and kinome selectivity
- Preclinical single agent and combination activity
- Preclinical CNS activity

Plan to nominate double mutant EGFR development candidate in fourth quarter 2020

Rationale for development of BLU-945 targeting EGFR+/T790M/C797S





BLU-945 showed significant tumor regression alone and in combination with osimertinib or gefitinib

BID, twice daily; EGFR, epidermal growth factor receptor; NSCLC, non-small cell lung cancer; QD, once daily; PDX, patient-derived xenograft; SEM, standard error of the mean.

Second quarter 2020 financial results

Statement of Operations (unaudited)	Three Months Ended 6/30/2020	Three Months Ended 6/30/2019
Total revenue	$8.3M	$5.1M
Collaboration revenue	$2.6M	$5.1M
AYVAKIT net sales	$5.7M	--
Cost of sales	$0.1M	--
Research & development expense[1]	$91.1M	$87.1M
Selling, general & administrative expense[2]	$42.2M	$21.9M
Net loss	$(123.5)M	$(99.7)M

Balance Sheet (unaudited)	6/30/2020	12/31/2019
Cash, cash equivalents and investments	$650.3M[3]	$548.0M

Received $775M in payments under Roche collaboration in Q3 2020

1. Includes stock-based compensation expense of $8.7M in 2020 and $7.5M in 2019. 2. Includes stock-based compensation expense of $10.8M in 2020 and $6.2M in 2019. 3. Does not include $775.0M in upfront payments received under the Roche collaboration for pralsetinib in Q3 2020 or $40.0 million in specified regulatory and commercialization milestones that Blueprint Medicines will receive under the Roche collaboration as a result of FDA approval of GAVRETO (pralsetinib).

Significant progress across portfolio in 2H 2020

ADVANCE REGISTRATION PROGRAMS FOR SM

- ✓ Report Part 1 PIONEER data in ISM and select recommended Part 2 dose
- ✓ Initiate registration-enabling Part 2 of PIONEER trial of avapritinib in ISM
- ✓ Report top-line data for avapritinib in advSM
- • Submit sNDA to FDA for avapritinib for advSM in Q4 2020

BUILD COMMERCIAL MOMENTUM

- ✓ Enter global collaboration with Roche to develop and commercialize GAVRETO
- ✓ Obtain U.S. approval of GAVRETO for RET+ NSCLC
- ✓ Obtain EU approval for avapritinib for PDGFRA D842V GIST in Q3 2020
- • Obtain U.S. approval of GAVRETO for RET+ thyroid cancers in Q1 2021

STRENGTHEN PIPELINE WITH NEW PROGRAMS

- ✓ Present preclinical data for BLU-945 at ESMO 2020 Congress
- • Nominate up to two additional development candidates in Q4 2020
- • Complete Phase 1 healthy volunteer trial of BLU-263 in Q4 2020

1. Full prescribing information is available at www.GAVRETO.com. ESMO, European Society of Medical Oncology.