PRECISION THAT MOVES™

Staying one step ahead of disease

May 19, 2021

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 plans, strategies, timelines and expectations for the current or future approved drugs and drug candidates of Blueprint Medicines Corporation (the "Company"), including timelines for marketing applications and approvals, the initiation of clinical trials or the results of ongoing and planned clinical trials; the Company's plans, strategies and timelines to nominate development candidates; 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; and the Company's financial performance, 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 Company's ability and plans in establishing a commercial infrastructure, and successfully launching, marketing and selling current or future approved products; the Company's ability to successfully expand the approved indications for AYVAKIT™/AYVAKYT® (avapritinib) and GAVRETO™(pralsetinib) or obtain marketing approval for AYVAKIT/AYVAKYT in additional geographies in the future; the delay of any current or planned clinical trials or the development of the Company's drug candidates 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 AYVAKIT/AYVAKYT, GAVRETO or any drug candidates it is developing; 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.

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2011

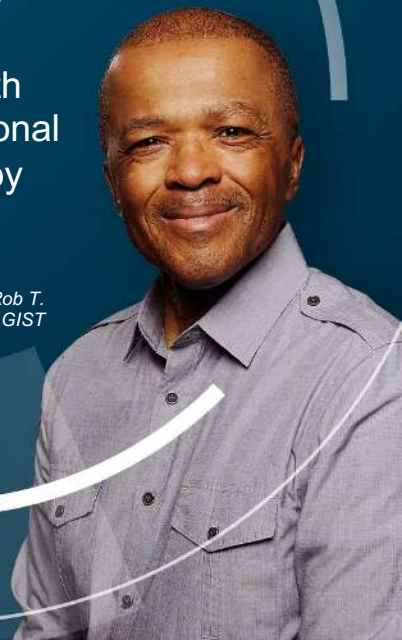
Hopeful foundation

A new precision therapy platform

2021

Hopeful reality

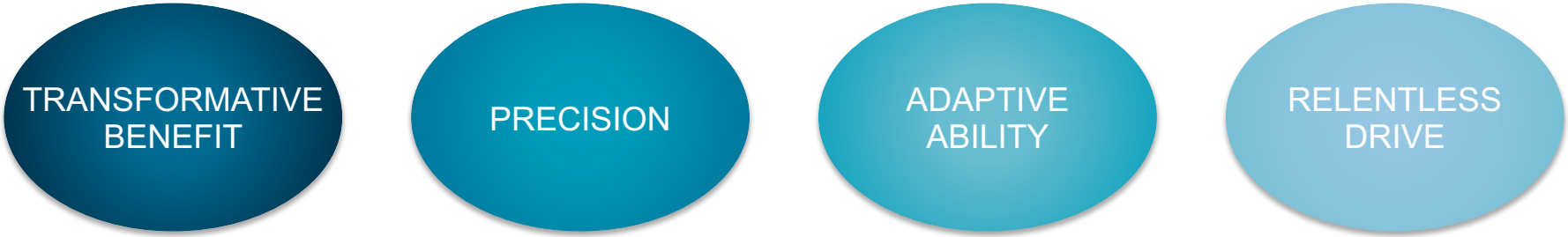
~2,600 patients treated with an approved or investigational Blueprint Medicines therapy



Rob T.
Living with GIST

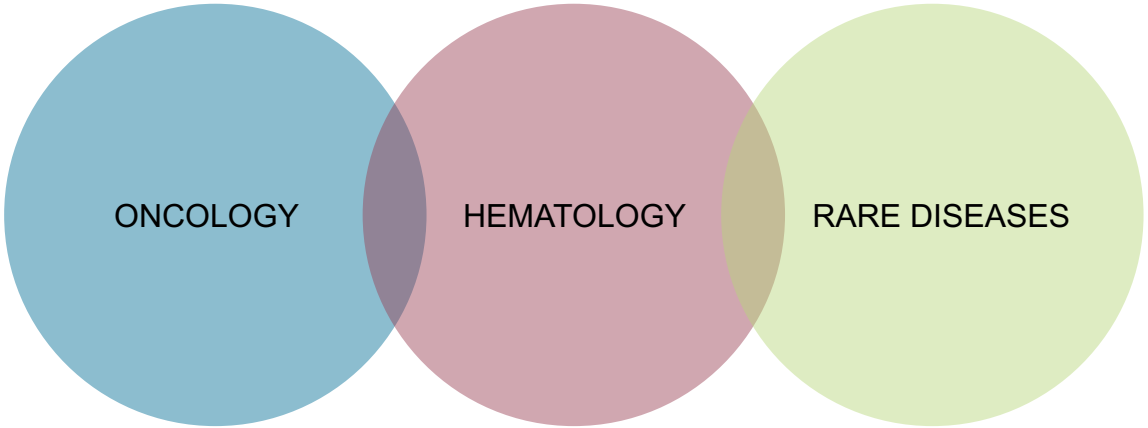
Blueprint Medicines' core mission and foundational principles

We aim to make real the promise of precision therapy to improve and extend life for as many people with cancer and hematologic disorders as possible



A leader in precision oncology and hematology

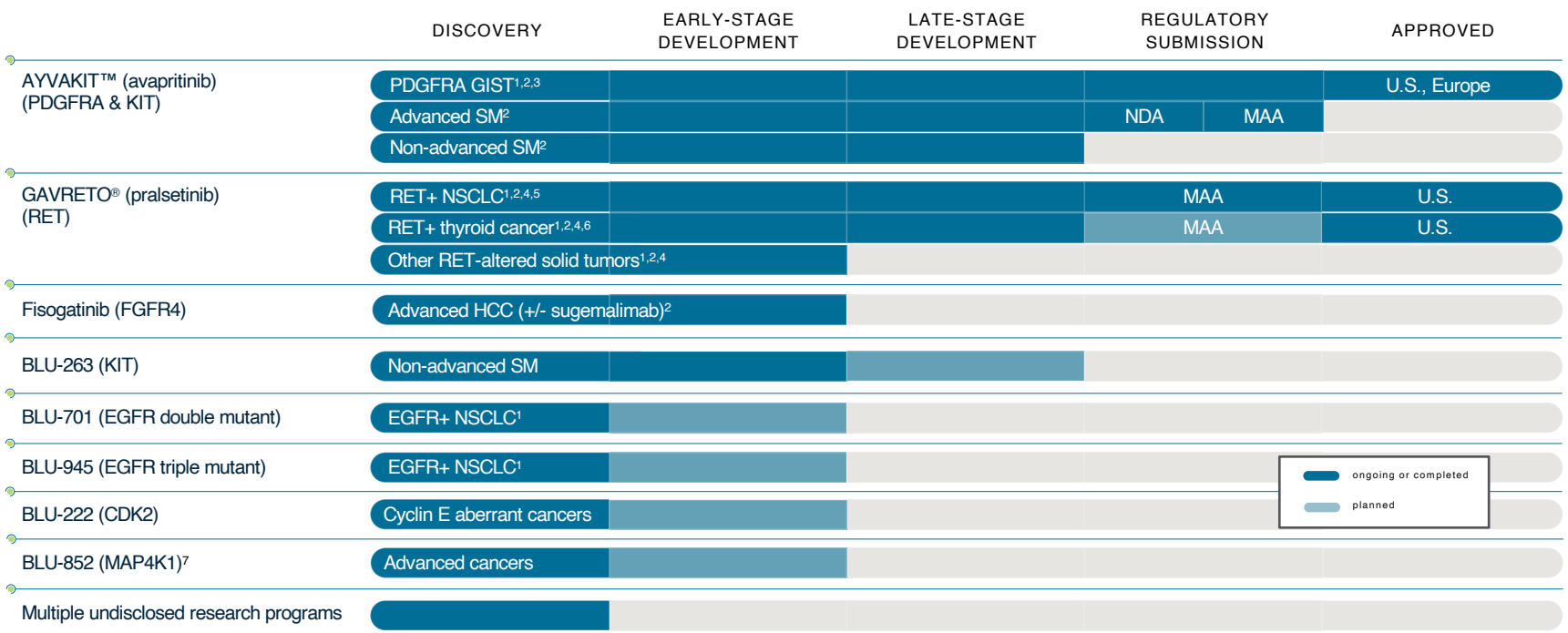
PORTFOLIO AREAS OF FOCUS



THERAPEUTIC AREA LEADERSHIP

- SYSTEMIC MASTOCYTOSIS
- LUNG CANCER

Rapidly expanding portfolio highlights precision therapy leadership



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 under the brand name AYVAKYT® 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. Received accelerated approval in the U.S. for the treatment of patients with advanced or metastatic RET-mutant medullary thyroid cancer and RET fusion-positive thyroid cancer. Continued approval may be contingent on confirmatory trials. 7. In collaboration with Roche. Blueprint Medicines and Roche are conducting activities for up to two programs under the collaboration, including the program targeting MAP4K1. For one of the programs, Blueprint Medicines has U.S. commercial rights and Roche has ex-U.S. commercialization rights. For one of the programs, Roche has worldwide commercialization rights. GIST, gastrointestinal stromal tumors; HCC, hepatocellular carcinoma; MAA, marketing authorization application; NDA, new drug application; NSCLC, non-small cell lung cancer; SM, systemic mastocytosis.

Updated as of April 12, 2021

2021 roadmap for precision medicine leadership

Accelerate global adoption of AYVAKIT and GAVRETO

Advance a new wave of therapeutic candidates toward clinical proof-of-concept

Further expand the company's precision therapy pipeline

2021 roadmap for precision medicine leadership

Accelerate global adoption of AYVAKIT and GAVRETO

Advance a new wave of therapeutic candidates toward clinical proof-of-concept

Further expand the company's precision therapy pipeline

Two approved precision therapies poised for growth

AYVAKIT™ avapritinib | tablets

- Approved for unresectable or metastatic PDGFRA GIST
- 1Q 2021 net product sales: $7.1M

KEY PRIORITIES AND AREAS OF GROWTH

- Plan to launch in advanced SM in U.S. in Q2
  - Target PDUFA action date: June 16, 2021
  - MAA submitted to EMA in Q1 2021
- Registrational PIONEER trial in non-advanced SM enrolling
- FDA breakthrough therapy designations granted for advanced SM and moderate to severe indolent SM

GAVRETO® pralsetinib | 100mg capsules

- Approved for advanced or metastatic RET-altered NSCLC, MTC and other thyroid cancers[1]
- 1Q 2021 net product sales: $1.8M

KEY PRIORITIES AND AREAS OF GROWTH

- Continue to advance U.S. launch, with focus on growing share of new patient starts and identified RET patients
- First selective RET inhibitor approved in China
- MAA for RET fusion-positive NSCLC under review by EMA
- Plan to develop in additional treatment settings

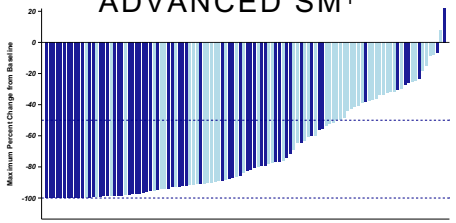
blueprint™ MEDICINES

EMA, European Medicines Agency; FDA, U.S. Food and Drug Administration; GIST, gastrointestinal stromal tumors; MAA, marketing authorization application; NDA, new drug application.
1. In collaboration with Roche. Blueprint Medicines and Roche have co-exclusive rights to develop and commercialize GAVRETO in the U.S., and Roche has exclusive rights to develop and commercialize pralsetinib outside the U.S., excluding Greater China.

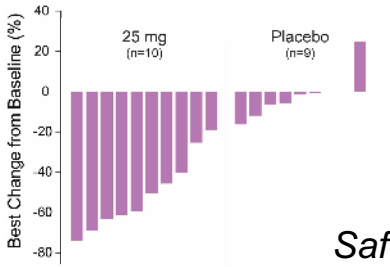
AYVAKIT is a clinically validated, highly potent inhibitor of KIT D816V, the genetic driver of SM

REDUCE MAST CELL BURDEN

ADVANCED SM[1]

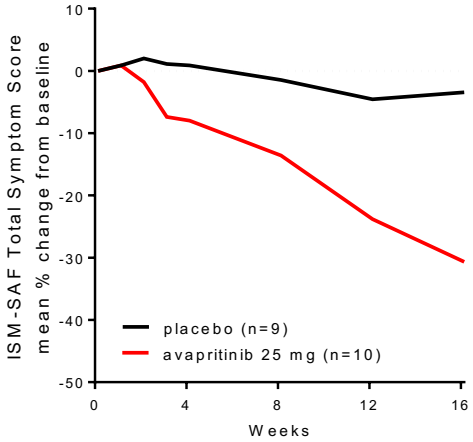


NON-ADVANCED SM[2]



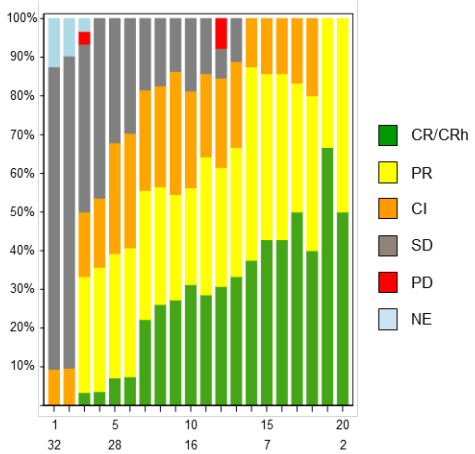
IMPROVE DISEASE SYMPTOMS

NON-ADVANCED SM[2]



INDUCE DEEP AND DURABLE RESPONSES

ADVANCED SM[1]



Safety profile enables tailored dosing based on patient need

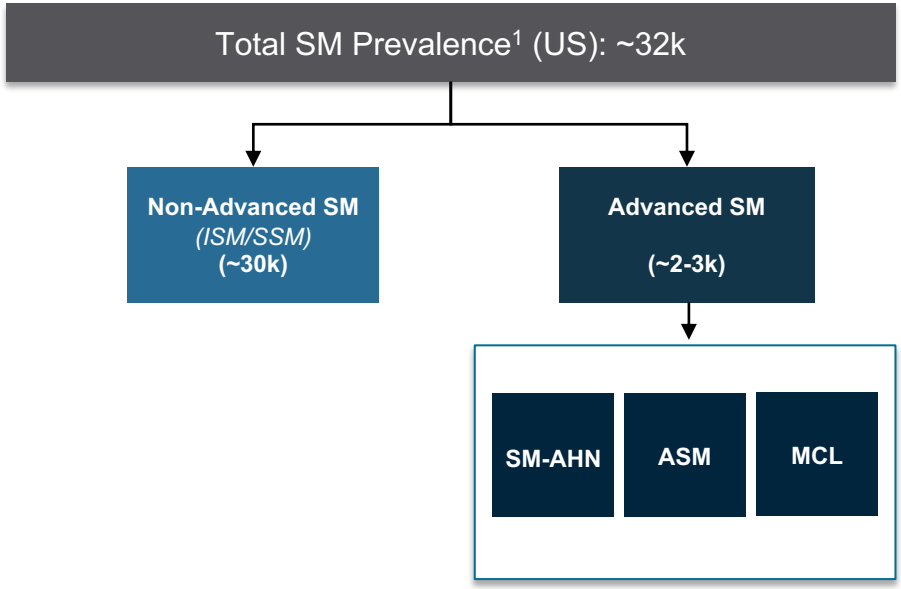
1. Top-line EXPLORER and PATHFINDER data presented in September 2020. Data cutoff: May 27, 2020 for EXPLORER and June 23, 2020 for PATHFINDER, with response assessments per central review completed in September 2020.
2. Data reported at AAAAI Annual Meeting in March 2020. Data cutoff: December 27, 2019.

Not for promotional use.

Significant initial target patient population with additional growth potential

Major markets include U.S., France, Germany, Italy, Spain, the United Kingdom and Japan. 1. Cohen S et al Br J Haematol (2014) 166(4):521-8 and World Bank Population estimates.

Advanced SM is estimated to represent ~5-10% of the total SM opportunity

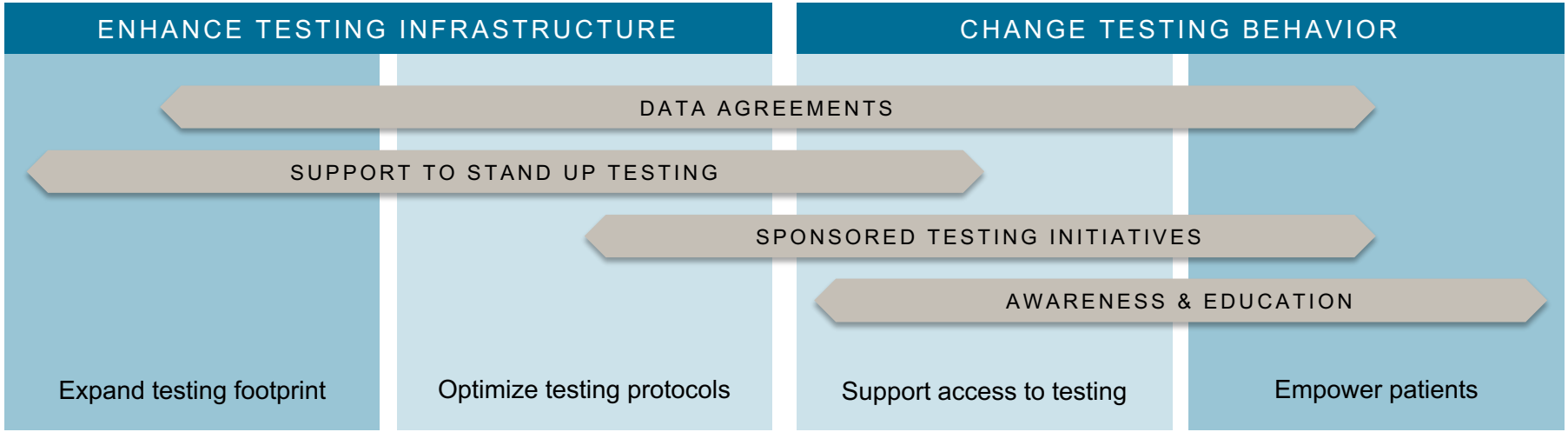


- Majority of advanced SM target population is diagnosed
- Initial focus on top potential treatment sites and prescribers
  - ~70 centers of excellence treat ~50% of advanced SM patients[2]

1. Estimated SM prevalence and patient subtypes based on internal claims analysis and epidemiology reported in S.S Cohen et al. 2014 and Orphanet.
2. Information on key SM centers of excellence identified based on depth of experience managing SM patients, trial site participation, disease area thought leadership and potential commercial opportunity, if approved.

Pursuing a range of testing initiatives to facilitate SM patient identification

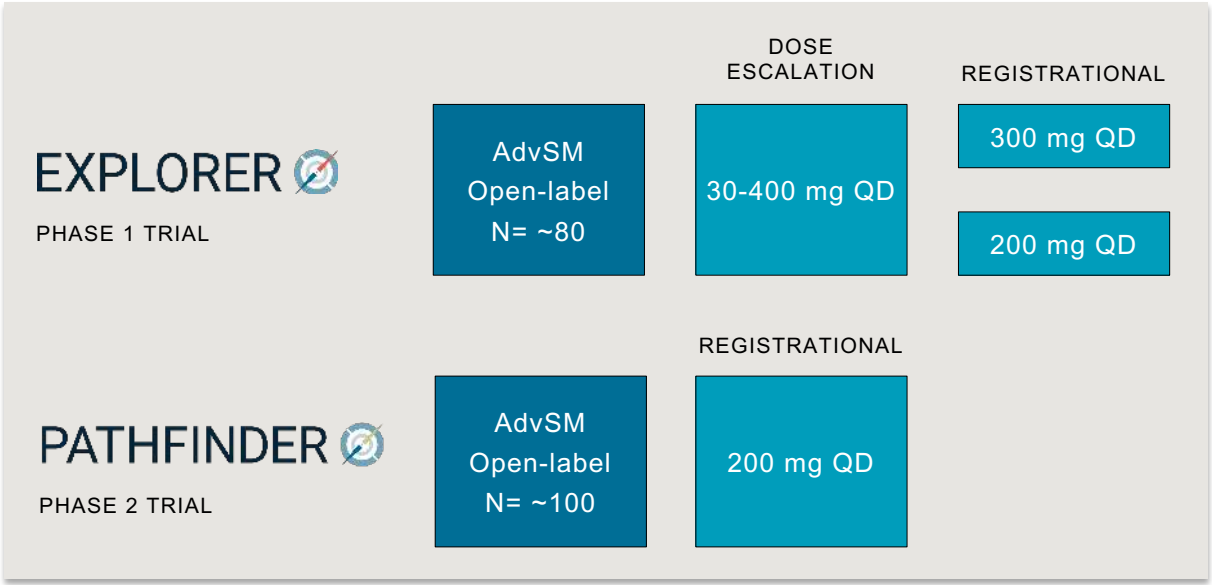
DATA SHOW HIGHLY SENSITIVE ddPCR TESTING DETECTS KIT D816V IN ~95% OF PATIENTS[1]



Anticipate highly sensitive ddPCR KIT D816V testing to be widely available in 2021 at laboratories currently covering ~80% of SM patients in U.S.[2]

1. Data in patients with non-advanced SM presented at the American Society of Hematology Annual Meeting in December 2020. 2. Based on internal market research.

AYVAKIT registration program in advanced systemic mastocytosis



PRIMARY ENDPOINT FOR APPROVAL:

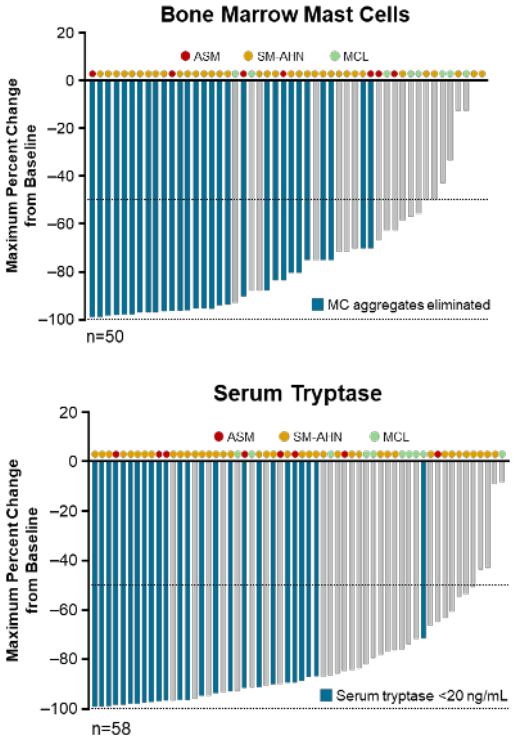
OVERALL RESPONSE RATE PER mIWG

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.

Interim analysis from PATHFINDER trial of AYAVKIT in advanced SM

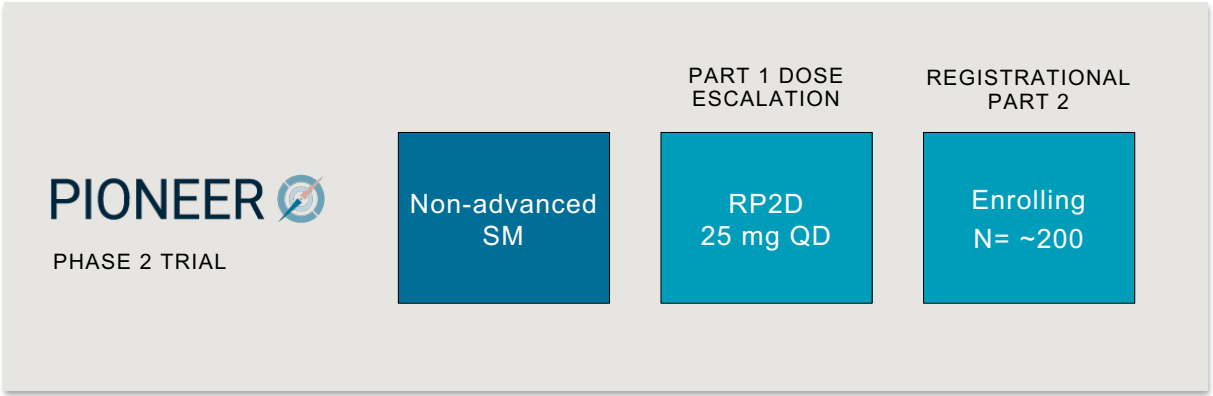
AYVAKIT
SELECTIVE AND POTENT KIT INHIBITOR

- Interim analysis consistent with prior data
- 75% ORR[1] (95% CI: 57%, 89%) per IWG-MRT-ECNM response criteria
- Robust reductions in mast cell burden and improvements in disease symptoms and patient-reported quality of life
- Generally well-tolerated at 200 mg QD proposed dose, based on the observed benefit-risk profile
- Supplementary marketing applications under review in U.S. and Europe, with FDA PDUFA action date on June 16, 2021



Data presented at AACR 2021 Annual Meeting. Data cut-off: June 23, 2020. 1. ORR defined as complete remission with full or partial recovery of peripheral blood counts, partial remission or clinical improvement. ASM, aggressive SM; CI, confidence interval; IWG-MRT-ECNM, International Working Group-Myeloproliferative Neoplasms Research and Treatment and European Competence Network on Mastocytosis; MC, mast cell; MCL, mast cell leukemia; ng/mL, nanograms per milliliter; ORR, overall response rate; PDUFA, Prescription Drug User Fee Act; QD, once daily; SM-AHN, SM with an associated hematologic neoplasm.

Plan to complete enrollment of registrational Part 2 of PIONEER trial of AYVAKIT in non-advanced SM in mid-2021



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

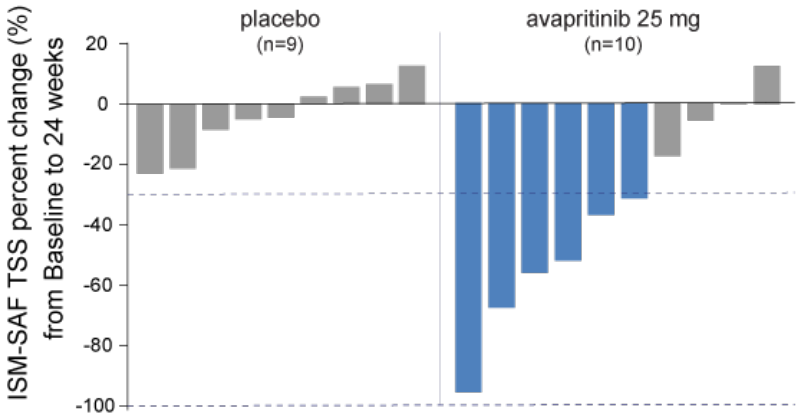
Key endpoints: Response rate defined as ≥30% reduction in ISM-SAF total symptom score (primary), measures of mast cell burden, quality of life, concomitant medications

Duration: 24 weeks

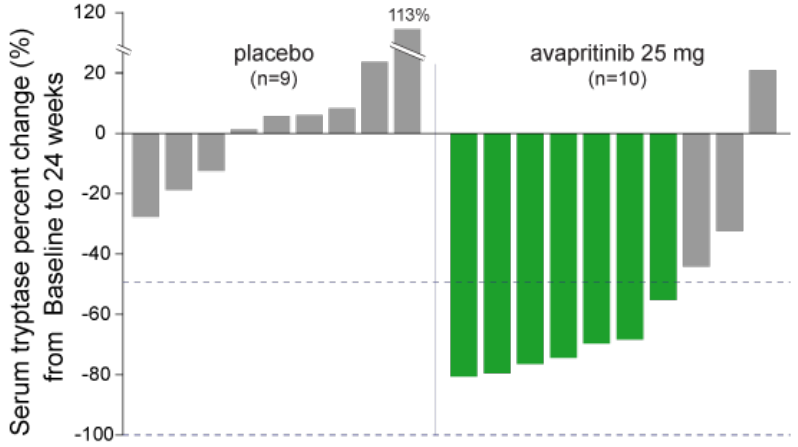
ISM, indolent system mastocytosis; ISM-SAF, indolent systemic mastocytosis – symptom assessment form; RP2D, recommended phase 2 dose.

PIONEER Part 1 data showed AYVAKIT 25 mg QD reduces symptoms and mast cell burden in non-advanced 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 AYVAKIT 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 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.

2021 roadmap for precision medicine leadership

Accelerate global adoption of AYVAKIT and GAVRETO

Advance a new wave of therapeutic candidates toward clinical proof-of-concept

Further expand the company's precision therapy pipeline

Extraordinary productivity of scientific platform

5 DEVELOPMENT CANDIDATES NOMINATED IN LAST ~18 MONTHS

Program	Target	Therapeutic area focus	Trial	Planned initiation
BLU-263	KIT D816V	Non-advanced SM	Phase 2/3	Mid-2021
BLU-945	EGFR triple mutant	EGFRm NSCLC	Phase 1	Q2 2021
BLU-701	EGFR double mutant	EGFRm NSCLC	Phase 1	2H 2021
BLU-222	CDK2	Cyclin E aberrant cancers	Phase 1	1H 2022
BLU-852*	MAP4K1	Cancer immunotherapy	Phase 1	2022

MULTIPLE CLINICAL PROOF-OF-CONCEPT DATASETS ANTICIPATED IN 2022

blueprint™ MEDICINES

KIT, KIT proto-oncogene receptor kinase; MAP4K1, hematopoietic progenitor kinase 1.
*In collaboration with Roche. For one of the programs, Blueprint Medicines has U.S. commercial rights and Roche has ex-U.S. commercialization rights. For one of the programs, Roche has worldwide commercialization rights.

Results from a Phase 1 trial of BLU-263 in healthy volunteers

BLU-263
NEXT-GENERATION KIT INHIBITOR

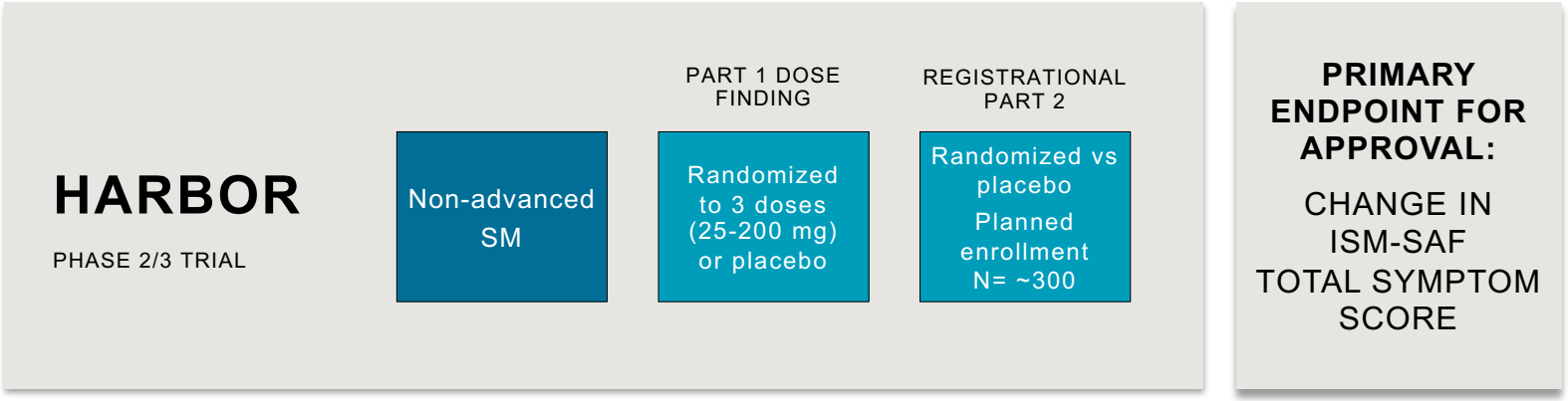
- BLU-263 was generally well-tolerated at all doses tested in healthy volunteers
- Pharmacokinetics were linear and dose-dependent
- Half-life supports once-daily dosing
- Plan to initiate Phase 2/3 HARBOR trial in non-advanced SM, at doses ranging 25-100 mg QD, in mid 2021

Treatment-related AEs, N of subjects	Single ascending dose cohorts	
	All other doses N=24	200 mg N=6
Any TRAE	0	1
Upper abdominal pain	0	1
Decreased appetite	0	1
Somnolence	0	0
Headache	0	0

Treatment-related AEs, N of subjects	Multiple ascending dose cohorts		
	25 mg N=6	50 mg N=6	100 mg N=6
Any TRAE	1	0	0
Upper abdominal pain	1	0	0
Fatigue	1	0	0
Chapped lips	1	0	0
Nausea	1	0	0
Headache	1	0	0

Data presented at AACR 2021 Annual Meeting. Data cut-off: November 9, 2020. AE, adverse event; TRAE, treatment-related AE.

Plan to initiate HARBOR trial of BLU-263 in non-advanced SM in mid-2021



HARBOR PARTS 1 AND 2

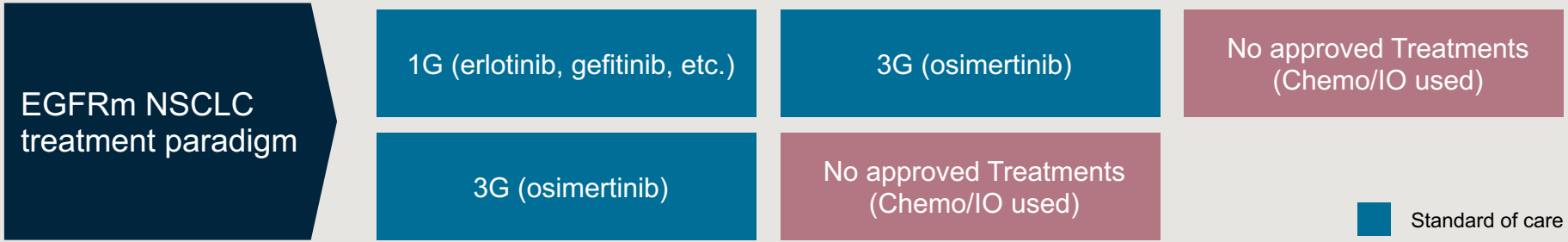
Design: Randomized, double-blind, placebo-controlled treatment period, followed by open-label expansion

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

Study includes an exploratory arm in monoclonal mast cell activation syndrome

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

Significant patient needs remain in EGFRm NSCLC across all lines of therapies



- Treatment resistance emerges over time which represents a significant barrier to durable benefit
- Chemotherapy +/- IO are the main treatment options post-osimertinib[1]
- CNS is a common site of metastases in EGFR driven NSCLC that needs to be targeted[2]
- **To improve durability and overall treatment outcome, we need effective, highly tolerated, brain-penetrant treatment options that target the most common on-target mutations early in initial therapy**

1. Piper-Vallillo, et al. Emerging treatment paradigms for EGFR-mutant lung cancers progressing on osimertinib. Journal of Clinical Oncology, 2020. 2. Remon and Besse. Brain metastases in oncogene-addicted NSCLC patients: incidence and treatment. Frontiers in Oncology, 2018. CNS, central nervous system; 1G, first-generation; 3G, third-generation; Chemo/IO, chemotherapy/immunotherapy.

BLU-701: potential best-in-class coverage of activating EGFR mutations, plus C797S osimertinib-resistant mutants

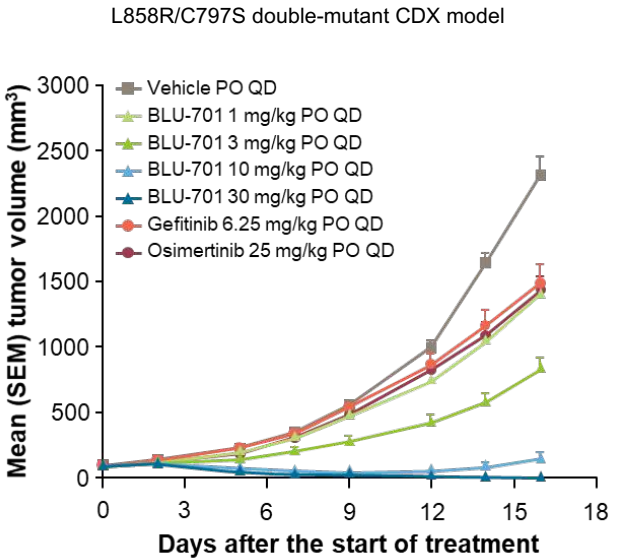
POTENCY ON ACTIVATING & RESISTANCE MUTANTS[1]

	BLU-701	gefitinib	osimertinib
ex19del	**3.3**	4.6	5.0
L858R	**3.3**	4.2	10.3
ex19del/C797S	**1.8**	6.1	>8000
L858R/C797S	**3.3**	3.8	>7000

WILD-TYPE SELECTIVITY[2]

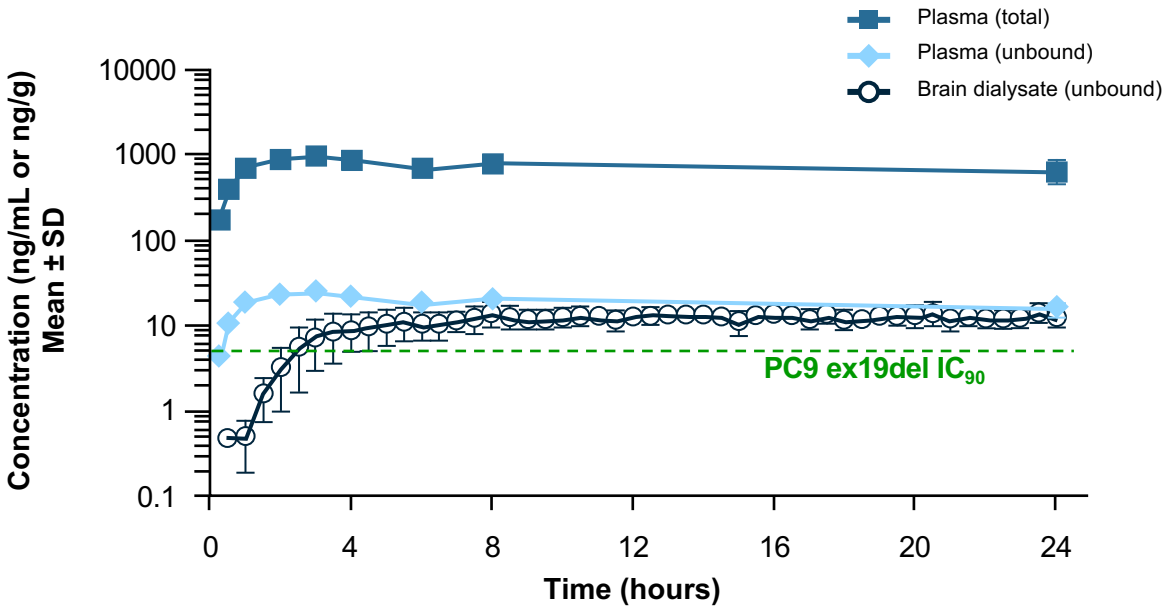
Wild-type EGFR	**107.3**	16.6	113.6

SINGLE AGENT ANTI-TUMOR ACTIVITY



Data presented at AACR 2021 Annual Meeting. 1. Cellular inhibition IC_{50} (nM) in Ba/F3 cell lines. 2. Cellular inhibition IC_{50} in A431 (wild-type EGFR) cell line. Wild-type EGFR selectivity shading: green = >50 nM; yellow = >10 nM, ≤50 nM. PO, oral administration. CDX, cell-line derived xenograft.

BLU-701 plasma and brain concentrations are comparable in preclinical models, suggesting significant brain penetration



Compound	IV infusion $Kp_{u,u}$ (C_{ss})[a]
BLU-701	0.98
Gefitinib	0.11
Osimertinib	0.30

BLU-701 30 MG/KG ACHIEVED CONCENTRATIONS ABOVE IC90 IN PLASMA AND BRAIN DIALYSATE

Data presented at AACR 2021 Annual Meeting.

BLU-945: potential first-in-class triple-mutant EGFR inhibitor, with exceptional wild-type EGFR selectivity to enable combinations

POTENCY ON RESISTANCE MUTANTS[1]

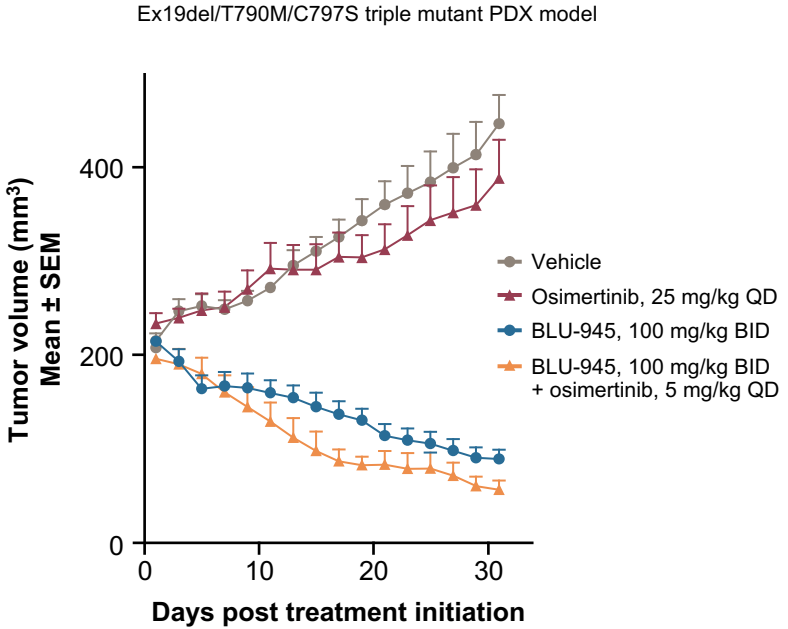
	BLU-945	gefitinib	osimertinib
L858R/T790M	**1.2**	4679.8	4.7
ex19del/T790M/C797S	**4.4**	4864.7	>10000
L858R/T790M/C797S	**2.9**	6707.7	7754.6

WILD-TYPE SELECTIVITY[2]

Wild-type EGFR	**544.4**	16.5	115.9

- BLU-945 demonstrated robust CNS activity in preclinical models

ANTI-TUMOR ACTIVITY ALONE AND IN COMBINATION WITH OSIMERTINIB



Data presented at AACR 2021 Annual Meeting. 1. Cellular inhibition IC_{50} (nM) in NCI-H1975 (EGFR double mutant) and Ba/F3 (EGFR triple mutant) cell lines. 2. Cellular inhibition IC_{50} in A431 (wild-type EGFR) cell line. Wild-type EGFR selectivity shading: green = >50 nM; yellow = >10 nM, ≤50 nM. PDX, patient-derived xenograft.

BLU-701 and BLU-945 provide comprehensive EGFR mutational coverage

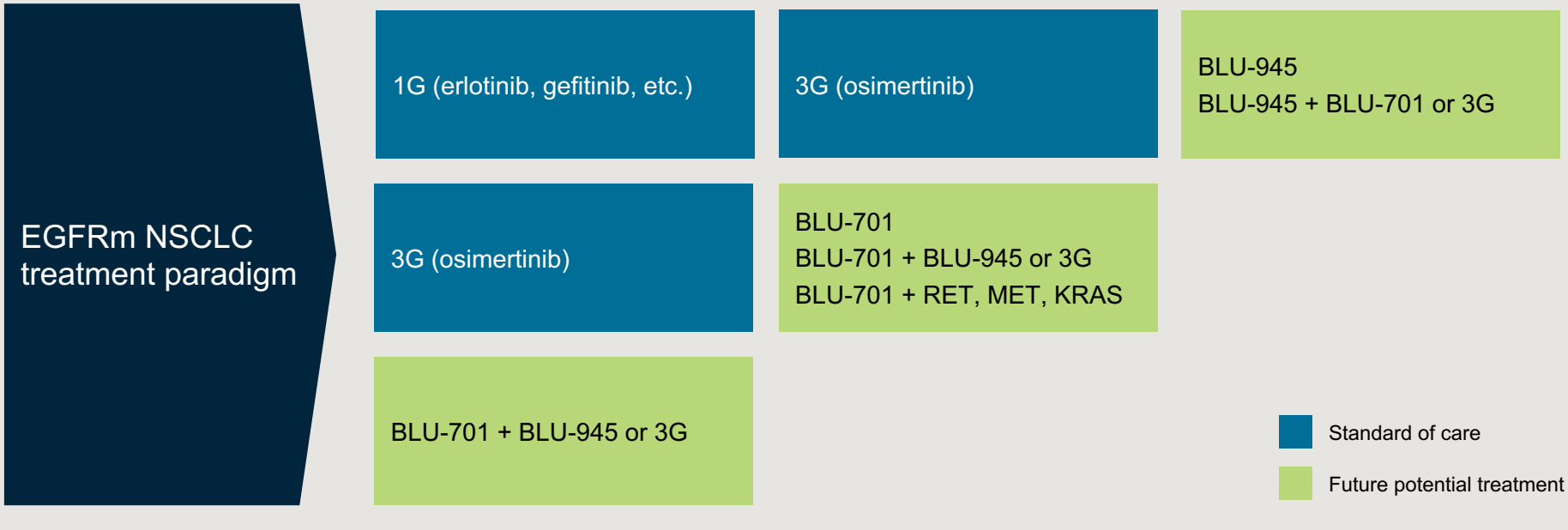
T790M & C797S: MOST COMMON ON-TARGET RESISTANCE TO 1G AND 3G, RESPECTIVELY

		1G	3G	4G		Potential Combinations		
	*EGFR mutational coverage**	*Gefitinib*	*Osimertinib*	*BLU-701*	*BLU-945*	*BLU-701 + osimertinib*	*BLU-945 + osimertinib*	*BLU-701 + BLU-945*
1L	L858R							
1L	ex19del							
2L	L858R or ex19del / T790M							
2L	L858R or ex19del / C797S							
3L	L858R or ex19del / T790M / C797S							

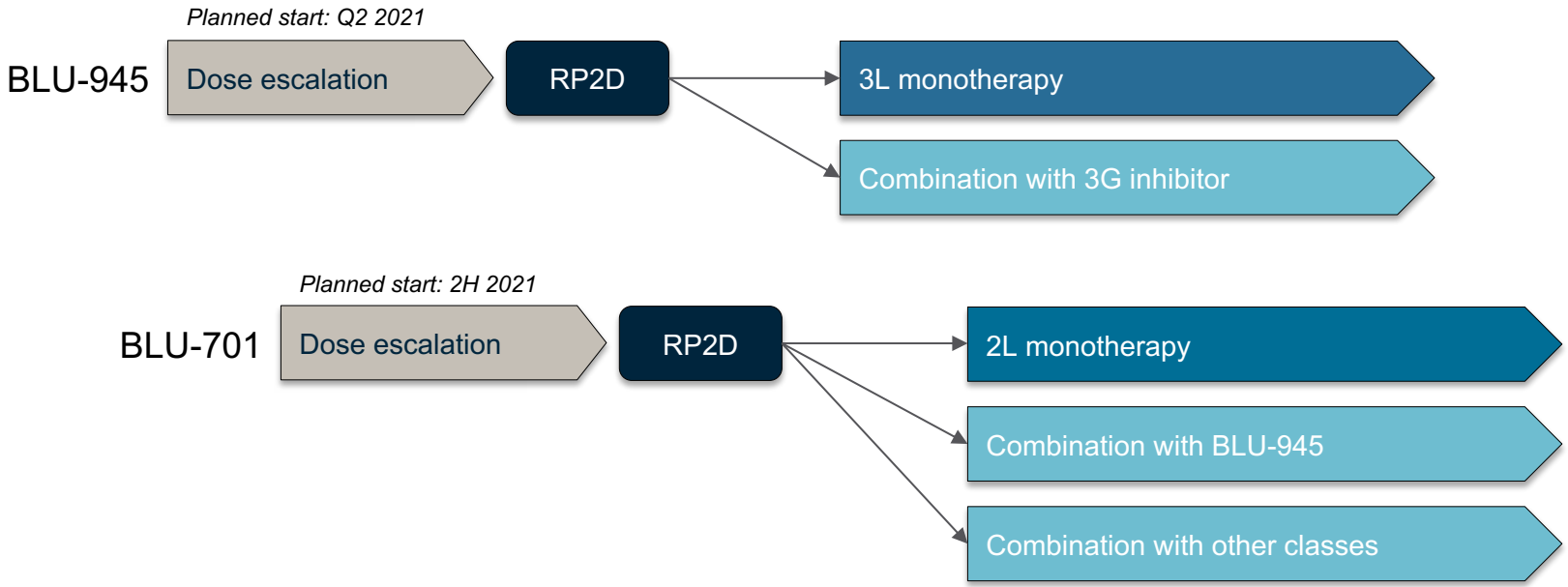
IC_{50} ≤10 nM 10 nM< IC_{50} ≤50 nM IC_{50} >50 nM

* Based on biochemical IC_{50}. 1L, first line; 2L, second line; 3L, third line; 4G, fourth generation.

We aim to transform EGFRm NSCLC treatment with 4G inhibitors that overcome or prevent on-target resistance across treatment lines



Plan to rapidly develop BLU-945 and BLU-701 monotherapy and combination regimens



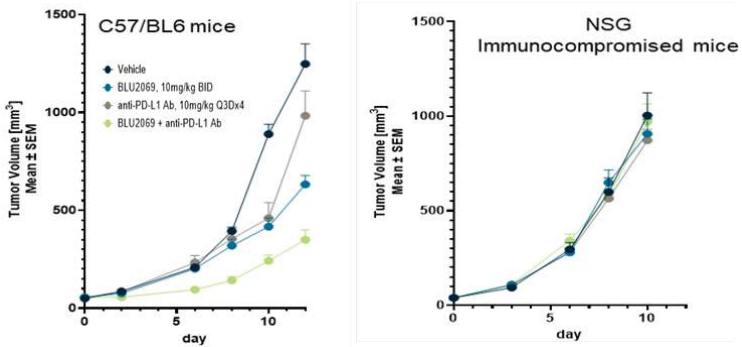
BLU-945 IND APPLICATION RECENTLY CLEARED BY U.S. FDA

2L, second line; 3L, third line; 3G, third generation; IND, investigational new drug application; RP2D, recommended Part 2 dose.

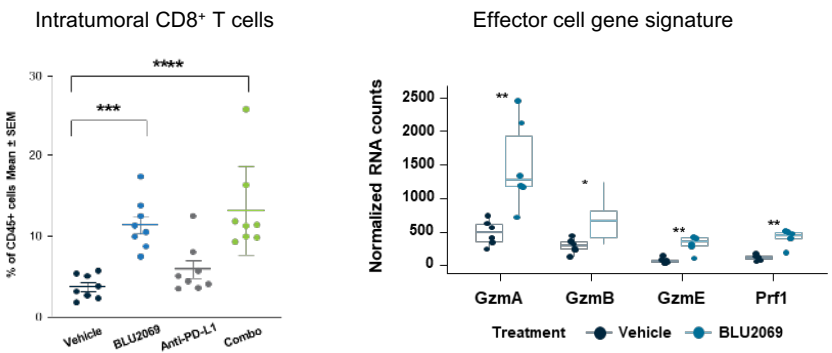
BLU-852, the first development candidate nominated under our cancer immunotherapy collaboration with Roche, has best-in-class potential

	Enzyme activities IC_{50} (nM)			Cell activity IC_{50} or EC_{50} (nM)		Whole Blood activity IC_{50} or EC_{50} (nM)		Selectivity
Compound	MAP4K1	LCK	MAP4K4	pSLP76*	IL-2†	pSLP76*	IL-2†	% kinome >100x
BLU2069	0.17	19	45	29	16	615	517	95%
BLU6348	0.13	78	73	27	11	1033	1194	96%
BLU-852	0.11	502	1196	40	11	851	1240	97%

ANTI-TUMOR ACTIVITY IN AN MCA-205 SARCOMA MODEL



INCREASED CD8 T CELL FREQUENCY & ACTIVATION



Data presented at AACR 2021 Annual Meeting. * IC_{50} values. † EC_{50} values. EC_{50}, half-maximal effective concentration; IC_{50}, half-maximal inhibitory concentration; nM, nanomolar.

Multiple pipeline programs driving to planned clinical data disclosures in 2022

BLU-263
NEXT-GENERATION KIT INHIBITOR

- Initiate Phase 2/3 HARBOR trial of BLU-263 in non-advanced systemic mastocytosis in mid 2021

BLU-701 AND BLU-945
POTENTIAL FIRST- AND BEST-IN-CLASS 4G EGFR INHIBITORS

- Initiate Phase 1 trial of BLU-945 in Q2 2021
- Initiate Phase 1 trial of BLU-701 in 2H 2021
- Report preclinical combination data in 2H 2021

BLU-222
POTENTIAL BEST-IN-CLASS CDK2 INHIBITOR

- Initiate IND-enabling studies in Q2 2021
- Report additional preclinical data in 2H 2021
- Initiate Phase 1 trial of BLU-222 in 1H 2022

2021 roadmap for precision medicine leadership

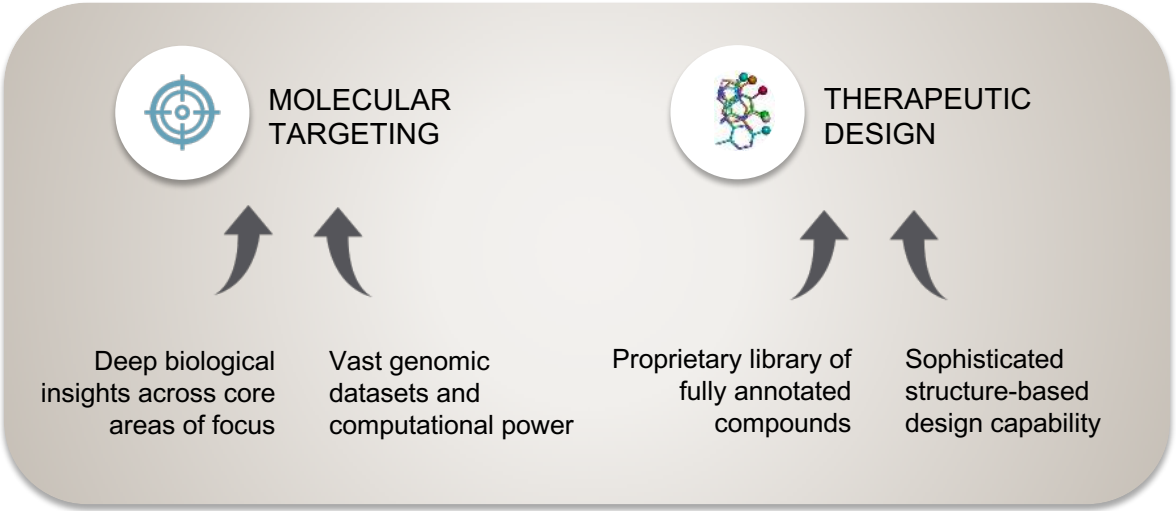
Accelerate global adoption of AYVAKIT and GAVRETO

Advance a new wave of therapeutic candidates toward clinical proof-of-concept

Further expand the company's precision therapy pipeline

Constant expansion of highly productive research platform

WORLD-CLASS EXPERTISE IN CATALYTIC KINASE INHIBITION



PLANNED FUTURE

EXPANDED INTERNAL CAPABILITY ACROSS ADDITIONAL PRECISION THERAPY MODALITIES

+

POTENTIAL COMPLEMENTARY EXTERNAL INNOVATION

Significant progress made towards 2021 goals

Accelerate global adoption of AYVAKIT and GAVRETO

- ✓ Submit MAA to EMA for AYAVKIT for advanced SM in Q1 2021
- ✓ Present registrational PATHINDER trial data for AYVAKIT in advanced SM in 1H 2021
- • Obtain FDA approval of AYVAKIT for advanced SM in the U.S. in Q2 2021
- • Complete enrollment of registration-enabling PIONEER trial in mid-2021
- • Obtain EMA approval of GAVRETO for RET fusion-positive NSCLC in 2H 2021
- • Submit MAA to EMA for GAVRETO for RET-altered thyroid cancers in 2H 2021
- • Initiate GAVRETO cohort in Roche's TAPISTRY tumor-agnostic platform trial in 2H 2021
- • Submit multiple marketing applications for GAVRETO across multiple additional geographies

Advance a new wave of therapeutic candidates toward clinical proof-of-concept

- ✓ Present preclinical data for BLU-701 and MAP4K1 inhibitors in 1H 2021
- • Initiate Phase 1 trial of BLU-945 in EGFR-driven NSCLC in Q2 2021
- • Initiate Phase 2/3 HARBOR trial of BLU-263 in non-advanced SM in mid-2021
- • Initiate Phase 1 trial of BLU-701 in EGFR-driven NSCLC in 2H 2021
- • Present preclinical data for combo of BLU-945 and BLU-701 in 2H 2021

Further expand the company's precision therapy pipeline

- ✓ Expand pipeline with one or more development candidates (BLU-222)
- • Pursue external opportunities to complement the company's precision therapy pipeline

NSCLC, non-small cell lung cancer; SM, systemic mastocytosis

Blueprint Medicines is in a strong financial position

Statement of Operations (unaudited)	Three Months Ended 3/31/2021	Three Months Ended 3/31/2020
Total revenue	$21.6M	$6.2M
Collaboration revenue	$12.6M	$2.7M
Net product sales	$9.0M	$3.5M
Cost of sales	$0.1M	--
Research & development expense[1]	$79.7M	$84.1M
Selling, general & admin expense[2]	$42.0M	$35.7M
Net income (loss)	$(99.7)M	$(111.0)M

Balance Sheet (unaudited)	3/31/2021	12/31/2020
Cash, cash equivalents and investments	$1,430.1M	$1,549.7M

1. Includes stock-based compensation expense of $8.9M and $7.8M in the three months ended 3/31/21 and 3/31/20, respectively. 2. Includes stock-based compensation expense of $11.7M and $9.1M in the three months ended 3/31/21 and 3/31/20, respectively.