



Pioneering the Science of Time

COMPANY OVERVIEW

JANUARY 2022



Cyndi N.
Systemic mastocytosis patient

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, express or implied 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 and data publications; the anticipated benefits of the Company's ISM Symptom Assessment Form; the Company's scientific platform expansion plans; 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; preliminary financial results; 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. If such expectations, assumptions, estimates and projections do not fully materialize or prove incorrect, the events or circumstances referred to in the forward-looking statements may not occur. 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 timing and results of preclinical and clinical studies for the Company's drug candidates, which may not support further development of such drug candidates or may impact the anticipated timing of data or regulatory submissions; 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; the Company's ability to successfully expand its operations and scientific platform and the costs thereof 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. Accordingly, readers are cautioned not to place undue reliance on these forward-looking statements.

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.

Blueprint Medicines is a global leader in precision therapy



Ongoing global collaboration with Roche and Genentech for the development and commercialization of GAVRETO

OUR FIRST DECADE OF ACHIEVEMENT

2 internally discovered medicines

FDA approved across **5** indications

within **10** years, and with

5 breakthrough therapy designations

BROAD AND GROWING PORTFOLIO WITH 10 PRECISION THERAPIES IN DEVELOPMENT



AYVAKIT is approved for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA exon 18 mutation, including PDGFRA D842V mutations, and adult patients with advanced SM, including aggressive SM, SM with an associated hematologic neoplasm and mast cell leukemia. GAVRETO is approved for the treatment of adult patients with RET-fusion positive NSCLC, adult and pediatric patients with advanced or metastatic RET-mutant medullary thyroid cancer who require systemic therapy and adult, and pediatric patients with advanced or metastatic RET fusion-positive thyroid cancer who require systemic therapy and who are radioactive iodine-refractory. FDA, U.S. Food and Drug Administration; GIST, gastrointestinal stromal tumor; NSCLC, non-small cell lung cancer; SM, systemic mastocytosis.

Not for promotional use.

Our path to potential transformative growth and an independent financial profile

YEAR END 2021

- AYWAKIT/AYWAKYT and GAVRETO approved with ongoing global expansion
- A leading precision therapy research platform
- Strong financial position with ~\$1B cash and cash equivalents

NEAR-TERM PLANS • 2022-2023

- Constellation of clinical data catalysts across strategic therapeutic areas
- AYWAKIT launch in non-advanced systemic mastocytosis
- Continued product revenue growth plus collaboration milestones and royalties

FUTURE GOALS • 2024-2025+

- Broad portfolio of marketed medicines in precision oncology and hematology
- Diversified research platform with unparalleled productivity
- Independent financial profile

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AYVAKIT launch update: strong performance with significant growth potential

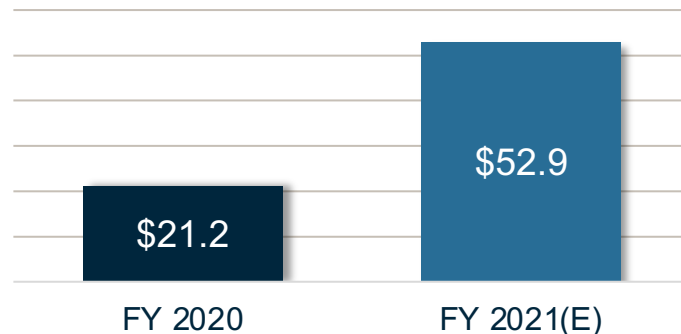


~550 patients with GIST or advanced SM treated with commercial AYVAKIT in the U.S. to date

ANTICIPATED GROWTH CATALYSTS

- ▶ Obtain EMA decision for advanced SM in 1H 2022
- ▶ Report top-line registration-enabling PIONEER trial data in non-advanced SM in mid-2022
- ▶ Submit sNDA to FDA for non-advanced SM in 2H 2022

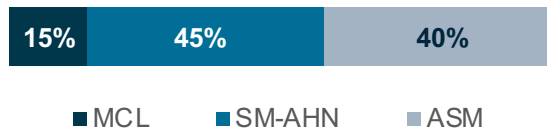
AYVAKIT GLOBAL REVENUE (\$, MILLIONS)¹



ESTIMATED \$20.0M AYVAKIT REVENUE IN Q4 2021¹

Breadth of early AYVAKIT prescribing for advanced SM in the U.S.

DISEASE SUBTYPES¹



PRIOR TREATMENT¹

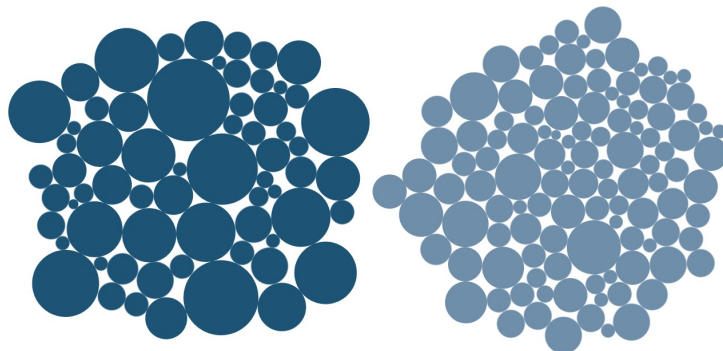
55% TREATMENT NAIVE

PATIENT HISTORY¹

20% WITH FIRST OBSERVED SM DIAGNOSIS IN 2021

QUANTITATIVE VISUALIZATION OF INSTITUTIONS PRESCRIBING AYVAKIT²

55%
VOLUME FROM
ACADEMIC CENTERS
& TEACHING HOSPITALS

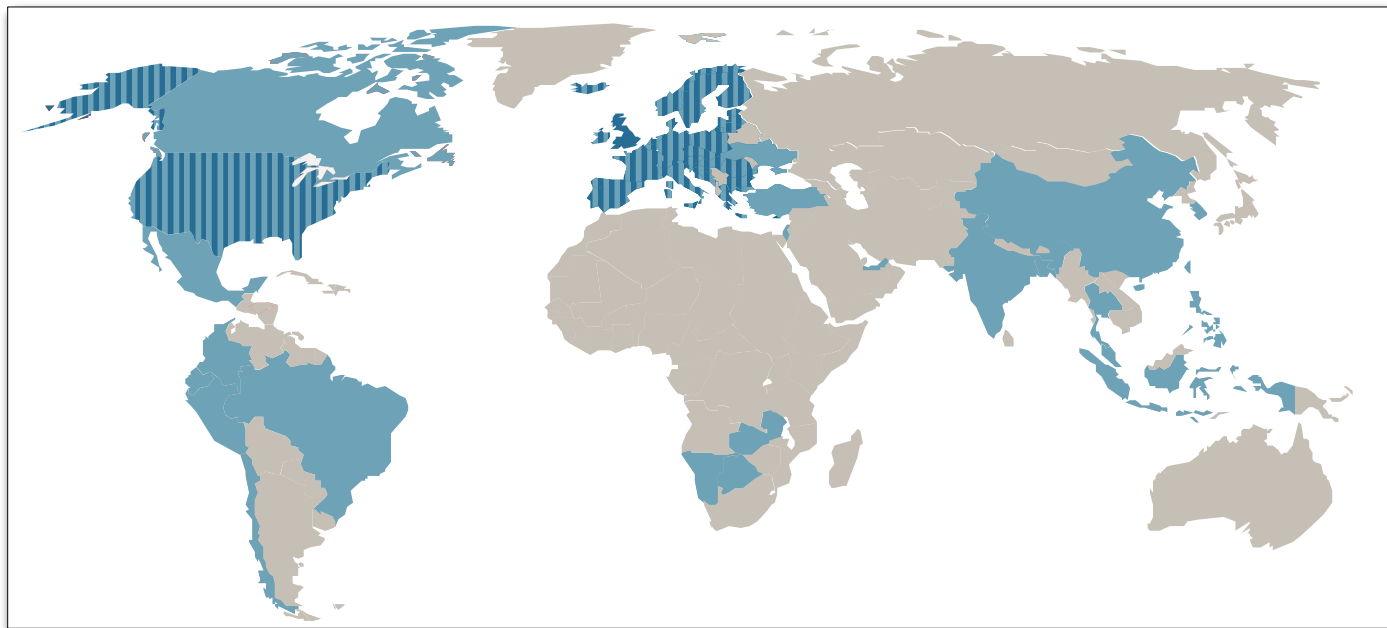


45%
VOLUME FROM
COMMUNITY PRACTICES
& CLINICS

Over **180** healthcare providers have prescribed AYVAKIT for advanced SM since launch

Two years after our first approval, we're bringing medicines to patients globally

~50 COUNTRIES WITH ≥ 1 APPROVAL OR MARKETING APPLICATION UNDER REVIEW



AYVAKIT®
avapritinib | tablets

GAVRETO®
pralsetinib | tablets

-  Blueprint Medicines
-  Strategic partner

We're primed to bring the promise of precision therapy to broad populations



Upcoming wave of clinical data milestones has the potential to dramatically expand our impact

BLU-222: Breast, ovarian and other cyclin E aberrant cancers

BLU-945 / BLU-701 / BLU-451*: EGFR+ NSCLC

AYVAKIT / BLU-263: Non-advanced SM

AYVAKIT: Advanced SM

GAVRETO: RET+ NSCLC and thyroid cancer

AYVAKIT: PDGFRA exon 18 mutant GIST

OPPORTUNITY SIZE

TIMING OF DATA CATALYSTS

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SYSTEMIC MASTOCYTOSIS
STRATEGIC THERAPEUTIC AREA

Pioneering the Science of Time

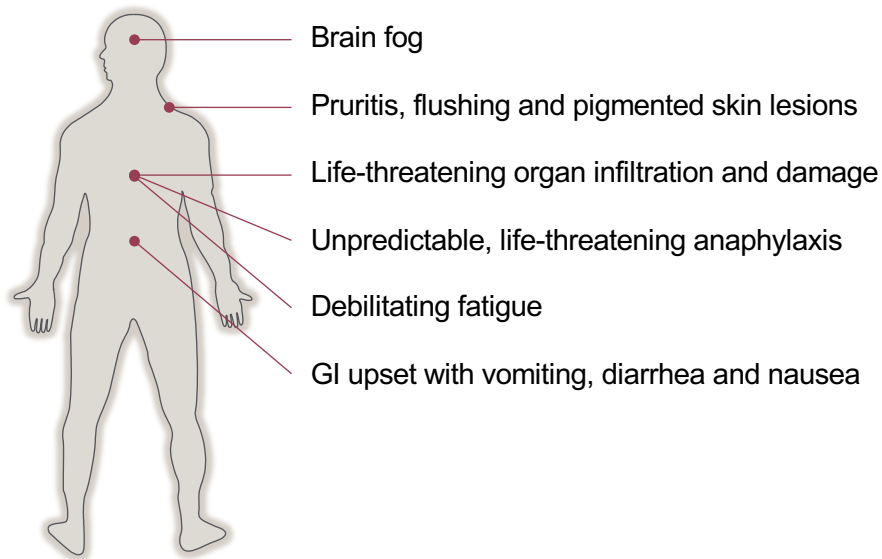
Every day, we seek to transform science into more time for life. Time to be with families, time to be productive community members, time to feel the small moments of joy that shape our lives.



Kristine G.
Systemic mastocytosis patient

Systemic mastocytosis, a rare mast cell disease with high medical need

SYSTEMIC MASTOCYTOSIS SYMPTOMS



95% of SM cases driven by the KIT D816V mutation

ADVANCED SM¹

6 months to 3.5 years median overall survival based on disease subtype

NON-ADVANCED SM²

30% had ≥1 emergency room visit in prior year

51% take ≥3 prescription medicines for SM

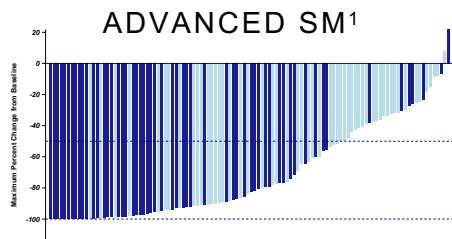
65% reported SM impacted their ability to work

90% feel SM controls their life to some extent

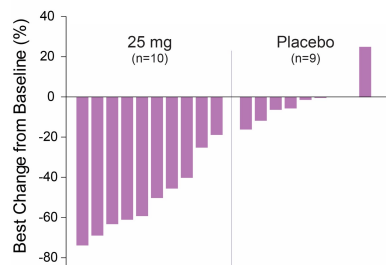
Worse physical functioning and mental health reported than patients with colorectal or lung cancer

Avapritinib is a clinically validated, highly potent inhibitor of KIT D816V

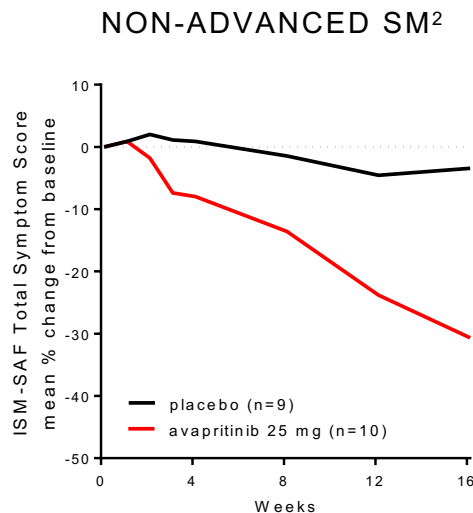
REDUCED MAST CELL BURDEN



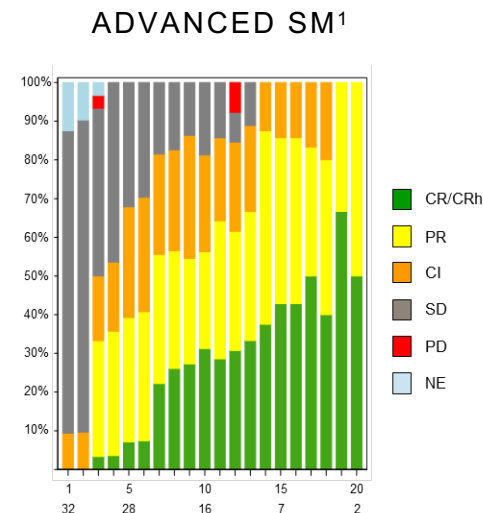
NON-ADVANCED SM²



IMPROVED DISEASE SYMPTOMS



INDUCED DEEP AND DURABLE RESPONSES



Safety data support evaluation of chronic treatment

Broad clinical strategy designed to address the spectrum of medical need in non-advanced SM

Avapritinib

- FDA breakthrough therapy designation granted for moderate to severe indolent SM

PIONEER

Phase 2 trial in non-advanced SM

PART 1 DOSE
ESCALATION

25 mg QD
selected as
RP2D

REGISTRATIONAL
PART 2

Fully enrolled
N= ~200

PRIMARY ENDPOINT
FOR sNDA SUBMISSION

Change in
ISM-SAF TSS
at 24 weeks

Plan to report top-line registration-enabling Part 2 data in mid-2022

BLU-263

- Next-generation KIT D816V inhibitor
- Opportunity to reach a broader population of patients with SM, based on potential for optimized risk-benefit profile

HARBOR

Phase 2/3 trial in non-advanced SM

PART 1 DOSE
ESCALATION

Now
enrolling

REGISTRATIONAL
PART 2

Planned
enrollment
N= ~350

PRIMARY ENDPOINT
FOR sNDA SUBMISSION

Change in
ISM-SAF TSS
at 24 weeks

Plan to present data in 2H 2022

ISM-SAF is a patient-reported outcomes tool developed with input from SM patients, disease experts and global regulatory authorities

ISM-Symptom Assessment Form

- Clinical benefit measure and primary endpoint for PIONEER trial
- Designed with input from disease experts, patients and regulatory authorities to support regulatory approval¹
- ISM-SAF produced reliable, construct-valid, sensitive scores when administered in PIONEER Part 1 to patients with indolent SM²

Symptom	Domains	Score
Abdominal pain	GI (0 – 30)	Scored 0 – 10 daily (24-hour recall) on a handheld device 0 is no symptoms 10 is worst Analyzed as a 14-day moving average
Diarrhea		
Nausea		
Spots	Skin (0 – 30)	
Itching		
Flushing		
Brain Fog	Neurocognitive (0 – 30)	
Headache		
Dizziness		
Bone pain		
Fatigue		

Total Symptom Score (0-110)

ISM-SAF TSS correlates with symptom and quality of life measures

CLINICALLY IMPORTANT TSS OUTCOMES



~30% reduction in ISM-SAF TSS

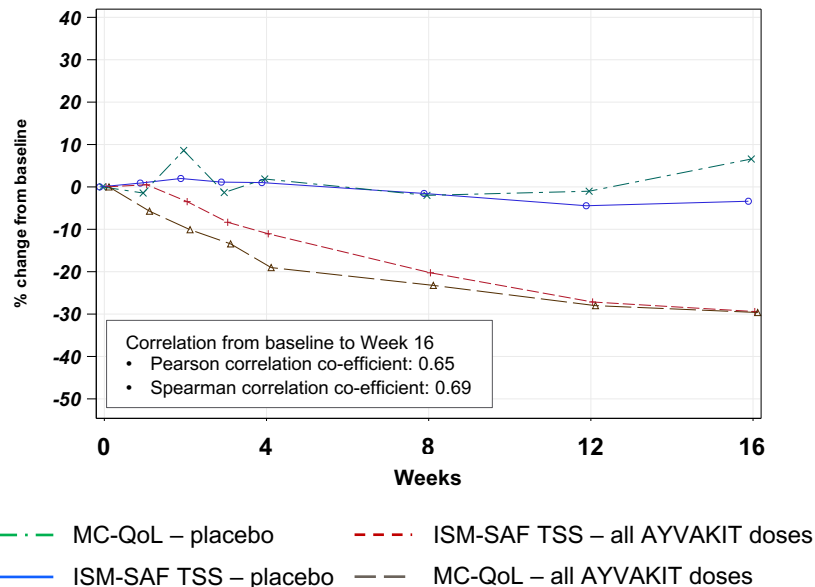
- 30% reduction in TSS is correlated with 1 to 2-point change on PGIS symptom questionnaire¹
- 2-point reduction on PGIS is associated with change from severe to mild symptoms²



~30% difference in ORR versus placebo

- Registration-enabling symptom assessment tools have shown ORR differences of ~15-40% versus placebo (e.g., linaclotide, ruxolitinib)²

PIONEER PART 1 DATA SHOW TSS CORRELATES WITH MC-QOL³



Significant initial target SM patient population, with high growth potential

75,000 SM PATIENTS
IN MAJOR MARKETS

 **5-10%**
ADVANCED SM

 **90-95%**
NON-ADVANCED SM



1/3 DIAGNOSED

1/3 MISDIAGNOSED

1/3 UNDIAGNOSED

← Initial opportunity → ← Growth opportunity →

We're executing a comprehensive plan to accelerate SM patient identification

RAISE AWARENESS VIA EDUCATION

- › Multiple ongoing healthcare provider and patient education programs designed to unmask signs and symptoms of disease

GENERATE EVIDENCE

- › PROSPECTOR screening study initiated to assess KIT D816V prevalence in patients with evidence of mast cell activation

SUPPORT ACCESS TO TESTING

- › Sponsored no-charge KIT D816V testing program with LabCorp Oncology now available for patients with suspected SM

ENHANCE TESTING INFRASTRUCTURE

- › Highly sensitive blood-based KIT D816V testing is recommended and available at laboratories covering >80% of SM patients
- › Ongoing community engagement to generate testing and treatment algorithms

LUNG CANCER
STRATEGIC THERAPEUTIC AREA

Precision that Moves

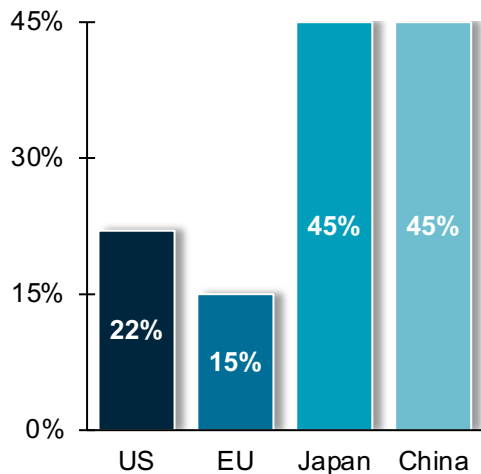
We help patients stay one step ahead with therapies that adapt to disease evolution. This includes solving for treatment resistance and intractable sites of progression, as well as pioneering innovative combinations to prolong benefit.



Diane L.
Lung cancer patient

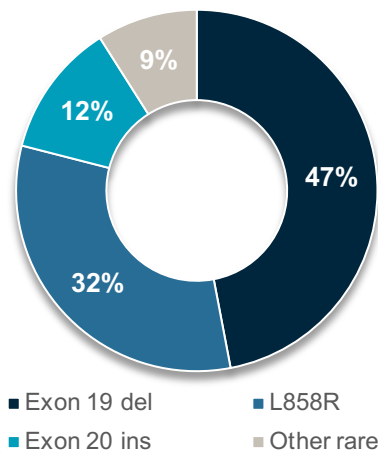
Significant medical needs in EGFR-driven non-small cell lung cancer

EGFR MUTATIONS ARE THE SECOND MOST COMMON ONCOGENIC DRIVER IN NSCLC¹



Estimated EGFR mutation rates in NSCLC adenocarcinoma

>90% OF ACTIVATING EGFR MUTATIONS ARE EXON 19 DELETIONS, L858R OR EXON 20 INSERTIONS²



Frequency of activating EGFR mutations in a recent U.S. multi-center study

EXON 19 DELETION & L858R

- Treatment resistance is a major issue, and no therapies are approved post osimertinib
- CNS is a common site of progression

EXON 20 INSERTION MUTATIONS

- All approved and investigational therapies have important safety, efficacy and/or CNS activity limitations

Commercial foundation in RET+ NSCLC, with a comprehensive EGFR pipeline

	Therapeutic targets	Program status
 pralsetinib 100mg capsules	RET fusions and predicted resistance mutations	Approved for first-line treatment of metastatic RET fusion+ NSCLC in the US and EU
BLU-945	EGFR L858R or exon 19 deletion plus on-target resistance mutations	Phase 1/2 SYMPHONY trial enrolling
BLU-701	- EGFR L858R or exon 19 deletion plus on-target resistance mutations - Highly brain penetrant*	Phase 1/2 HARMONY trial enrolling
BLU-451 (formerly LNG-451)	- EGFR exon 20 insertion mutations - Highly brain penetrant*	IND submitted to FDA

COMPLETED ACQUISITION OF LENGO THERAPEUTICS IN DECEMBER 2021

BLU-945 and BLU-701 designed to provide broad coverage of EGFR mutations

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

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							

$IC_{50} \leq 10$ nM

10 nM < $IC_{50} \leq 50$ nM

$IC_{50} > 50$ nM

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

POTENCY ON RESISTANCE MUTANTS¹

	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

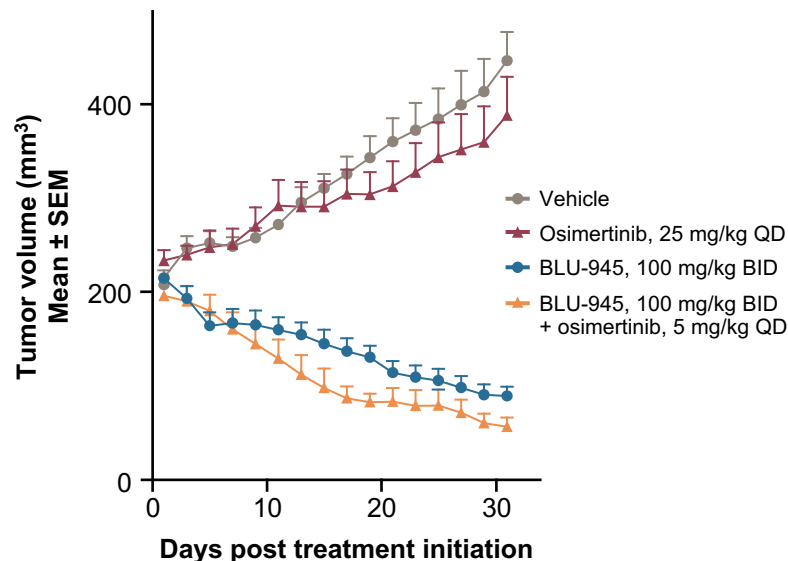
WILD-TYPE SELECTIVITY²

Wild-type EGFR	544.4	16.5	115.9
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➤ BLU-945 demonstrated robust CNS activity in preclinical models

ANTI-TUMOR ACTIVITY ALONE AND IN COMBINATION WITH OSIMERTINIB

Ex19del/T790M/C797S triple mutant PDX model



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

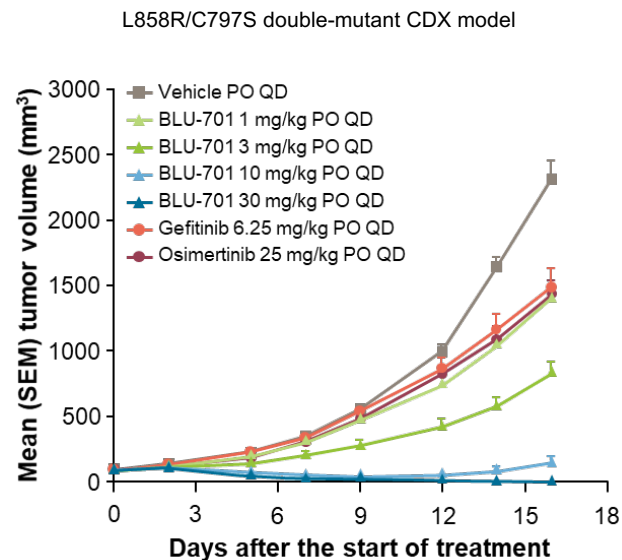
POTENCY ON ACTIVATING & RESISTANCE MUTANTS¹

	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

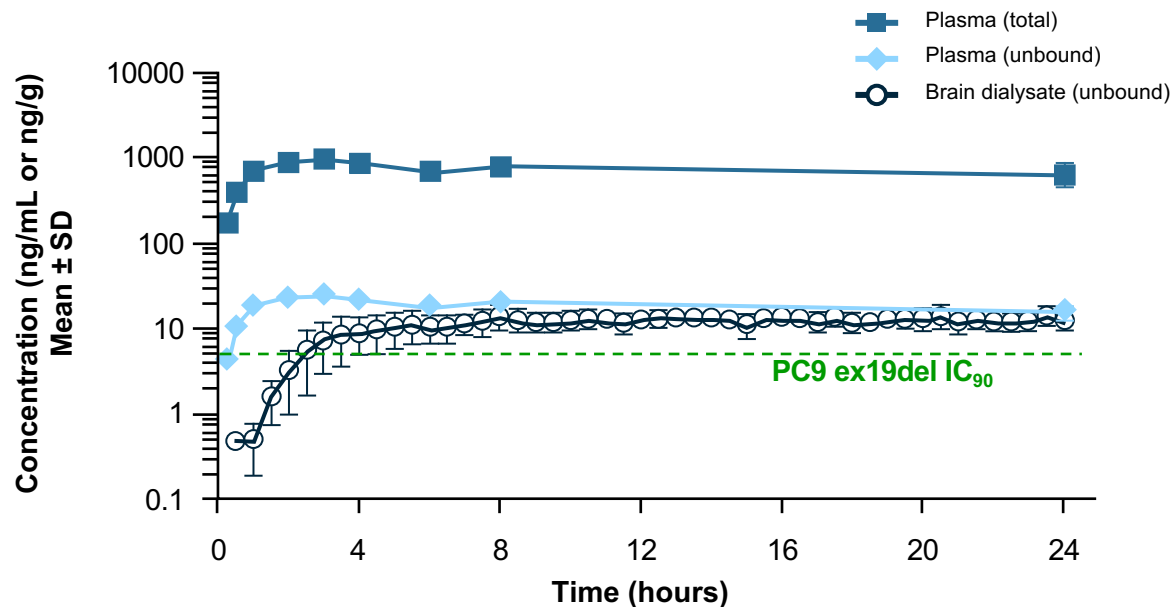
WILD-TYPE SELECTIVITY²

Wild-type EGFR	107.3	16.6	113.6
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SINGLE AGENT ANTI-TUMOR ACTIVITY



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



Compound	IV infusion $K_{p,u,u} (C_{ss})^a$
BLU-701	0.98
Gefitinib	0.11
Osimertinib	0.30

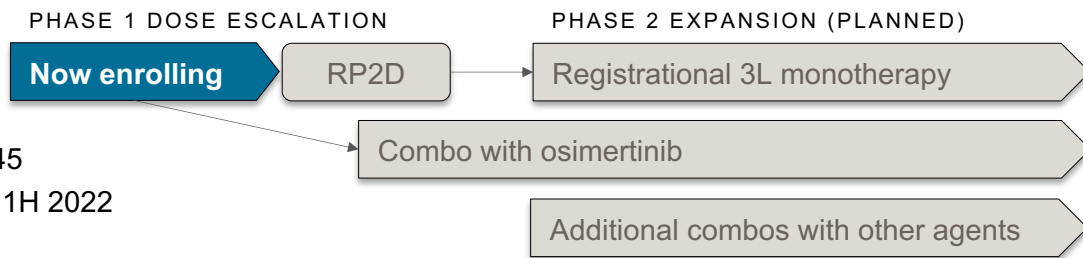
BLU-701 30 MG/KG ACHIEVED CONCENTRATIONS ABOVE IC₉₀ IN PLASMA AND BRAIN DIALYSATE

Ongoing clinical programs designed to rapidly explore combination therapies



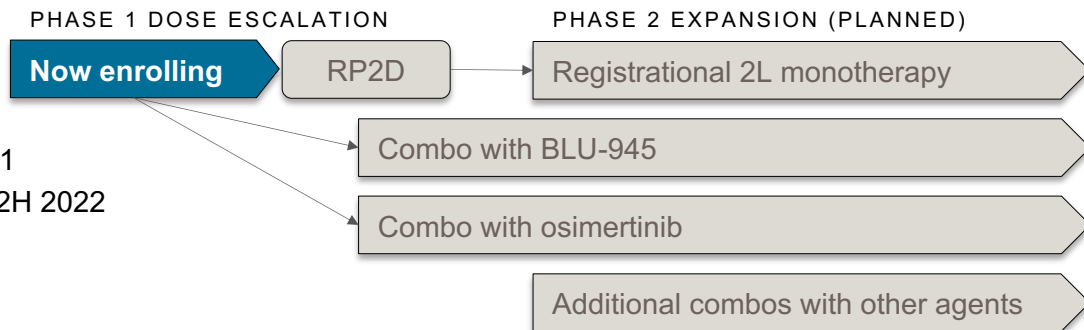
Phase 1/2 trial of BLU-945

- Initial data expected in 1H 2022



Phase 1/2 trial of BLU-701

- Initial data expected in 2H 2022



PLAN TO RAPIDLY INITIATE COMBINATION COHORTS BASED ON INITIAL PHASE 1 DATA

BLU-451: a potential best-in-class EGFR exon 20 precision therapy

-  **Potent inhibition** of all common EGFR exon 20 insertion variants
-  **Brain penetrant** with robust activity in a preclinical intracranial model
-  **Highly selective** over wild-type EGFR and off-target kinases
-  **Oral administration**, with well-characterized preclinical pharmacology

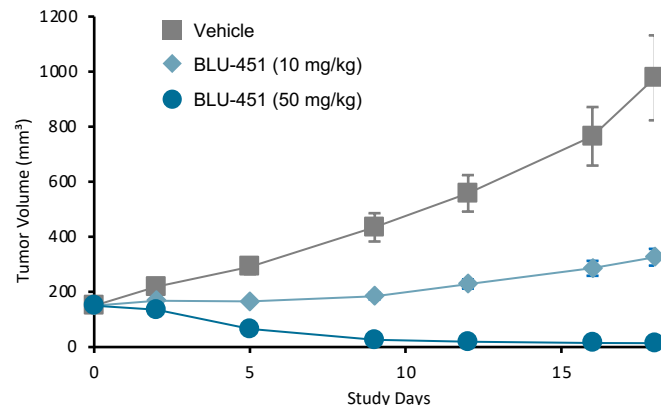
BLU-451 PRECLINICAL PROFILE HAS POTENTIAL TO TRANSLATE INTO IMPROVED SAFETY AND EFFICACY, INCLUDING IN PATIENTS WITH BRAIN METASTASES

Preclinical data show BLU-451 is highly selective, potent and CNS penetrant

HIGHLY SELECTIVE OVER WILD-TYPE EGFR (CELLULAR ACTIVITY IC₅₀, NM)

EGFR Exon 20 insertion variants	BLU-451
	WT EGFR
	1,630
	SVD
	53
	ASV
	78
	NPH
	75
	FQEA
	61
	NPG
	7

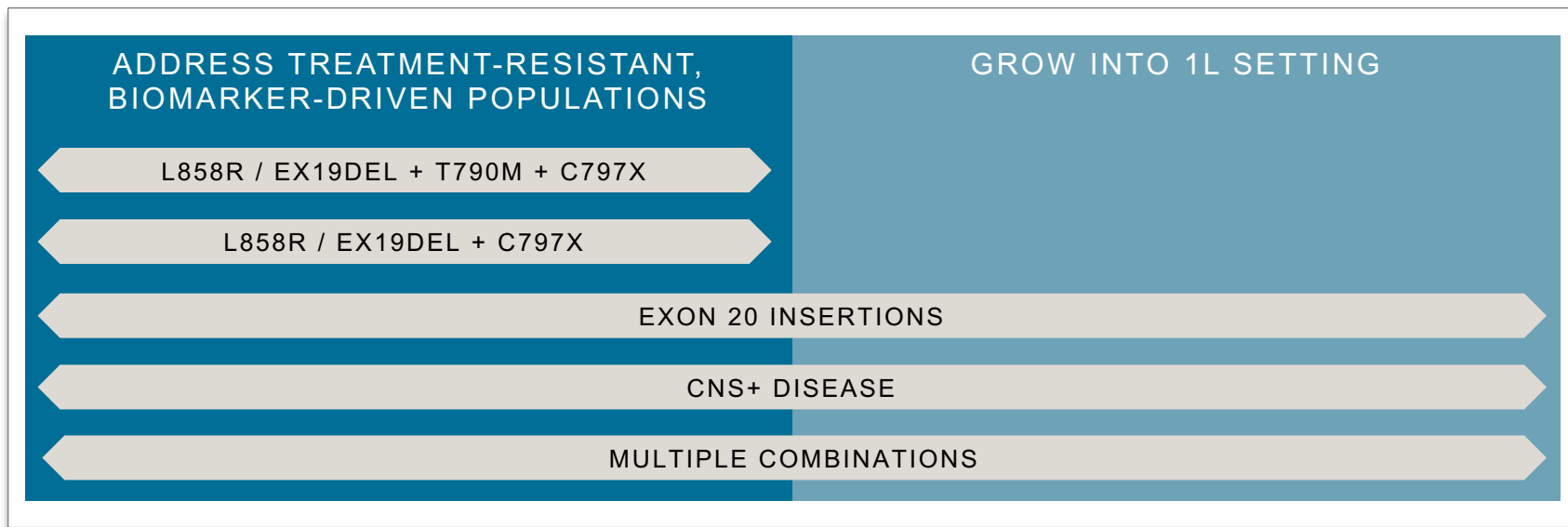
ROBUST ANTI-TUMOR ACTIVITY IN AN EGFR EXON 20 MODEL



HIGHLY CNS PENETRANT: $KP_{U,U} = 0.66$

PLAN TO PRESENT DETAILED PRECLINICAL DATA FOR BLU-451 IN 1H 2022

Broad EGFR-driven NSCLC franchise strategy with paths to first-line setting



INITIATED STRATEGIC COLLABORATION FOR BLU-701 & BLU-945 WITH ZAI LAB IN GREATER CHINA IN Q4 2021

ADDITIONAL PIPELINE
PROGRAMS

The Urgency of Now

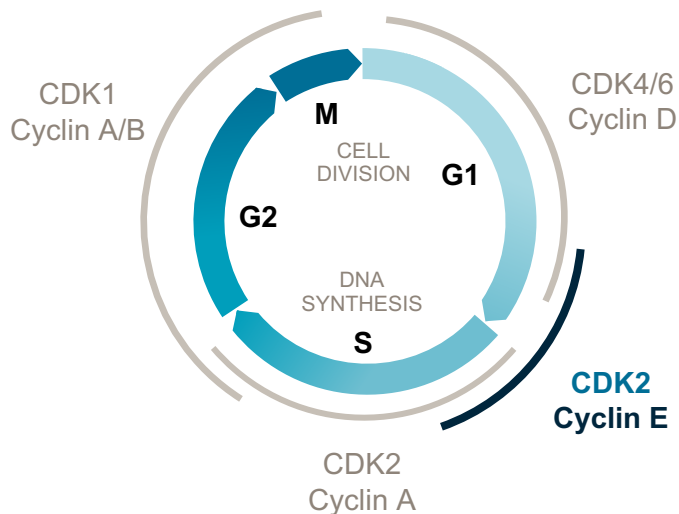
We are constantly on the move, relentless in our determination to accelerate development of new therapies, expedite clinical trials and quickly bring approved medicines to patients worldwide.



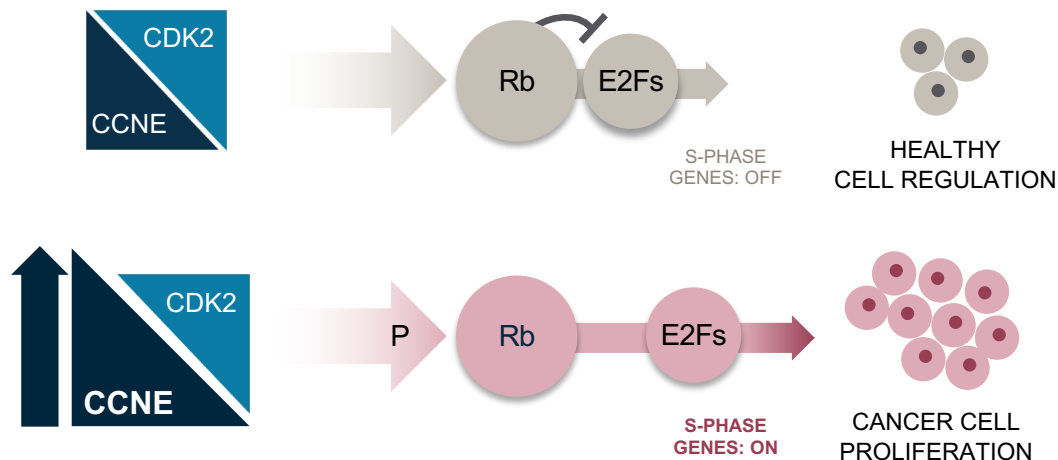
Rob T.
Advanced cancer patient

Aberrant cyclin E unleashes cellular proliferation

CDK-CYCLIN COMPLEXES
REGULATE THE CELL CYCLE



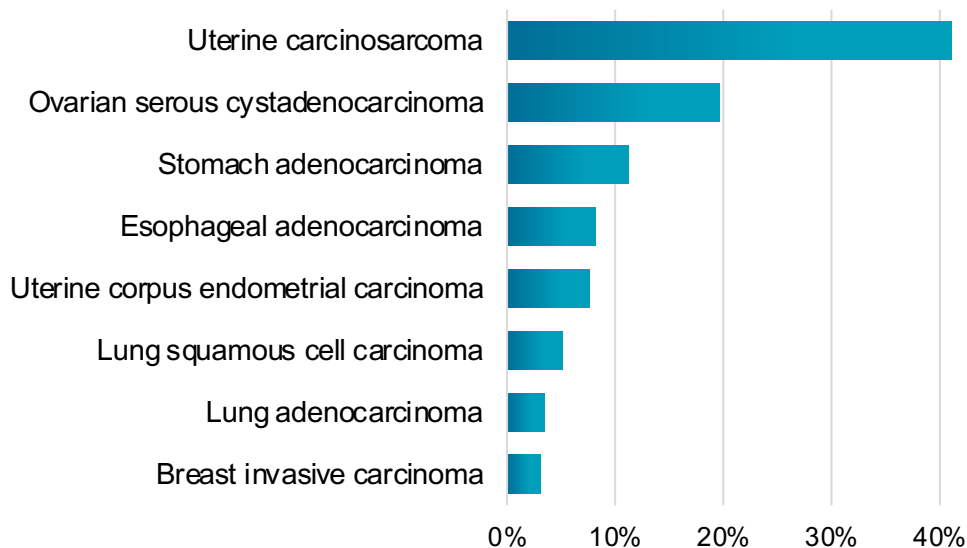
ABERRANT CYCLIN E (CCNE1)
DRIVES PROLIFERATION



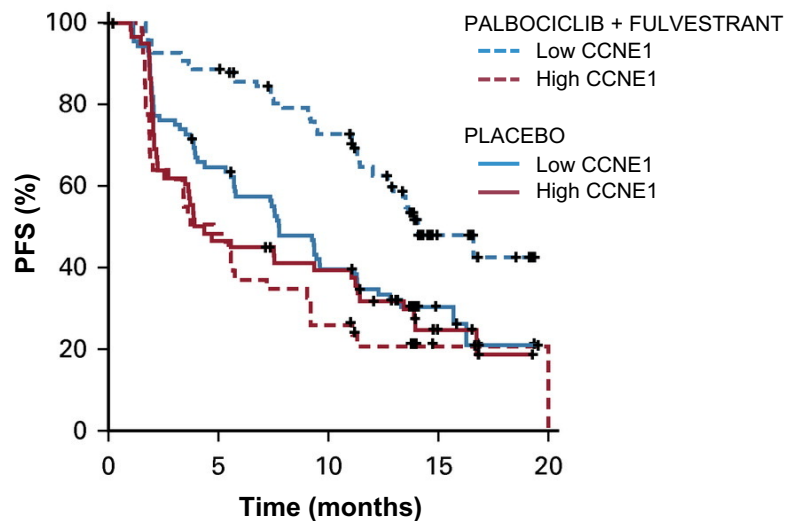
Aberrant CCNE hyperactivates CDK2, dysregulating Rb protein phosphorylation and E2F transcription factor activation of S-phase genes, resulting in cancer cell proliferation

Aberrant CCNE is a disease driver in multiple cancers

REPRESENTATIVE TUMOR TYPES
BY CCNE1 AMPLIFICATION FREQUENCY¹



LOWER PFS IN PALBOCICLIB-TREATED
HR+ BREAST CANCER WITH HIGH CCNE1²



Our highly selective and potent CDK2 inhibitors spare CDK anti-targets

← KEY ANTI-TARGETS →

		ENZYME ACTIVITY IC ₅₀ (NM)					
	Kinome S (10)	CDK2	CDK1	CDK4	CDK6	CDK7	CDK9
BLU0298	0.045	2.6	233.6	377.4	275.2	6941.2	6115.1
BLU1954	0.055	0.2	110.1	114.5	190.6	3928.9	849.1
BLU2256	0.040	0.1	152.9	116.9	393.2	4826.4	9063.2

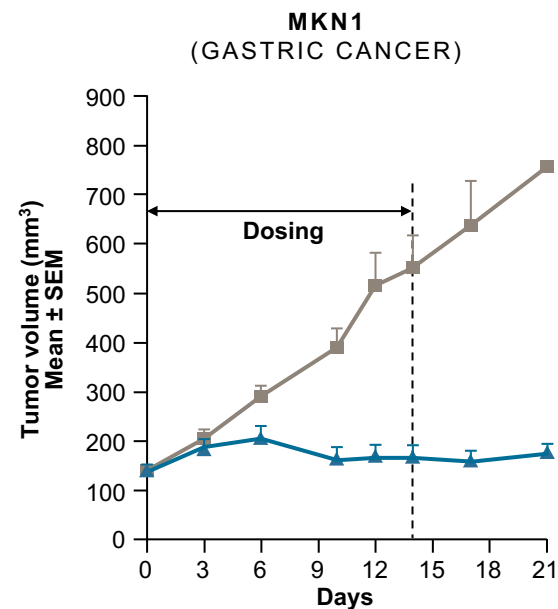
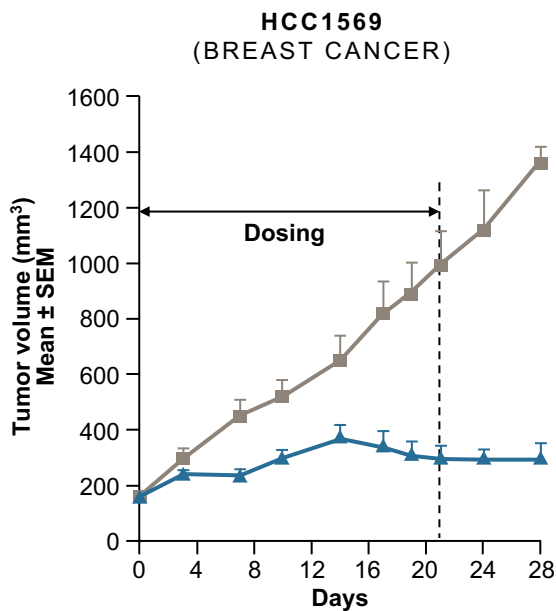
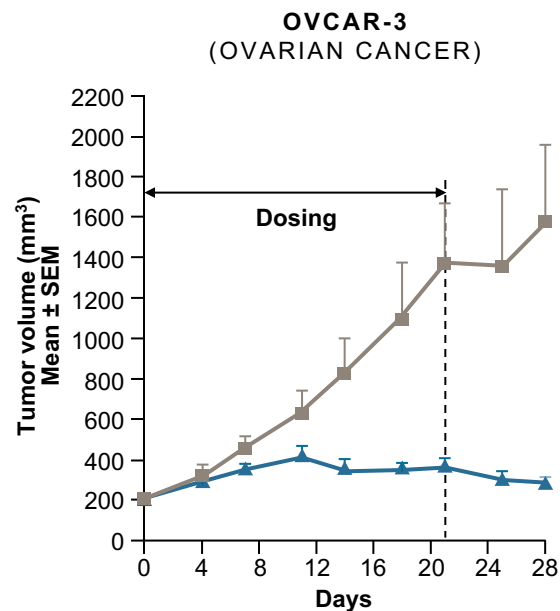
ASSOCIATED TOXICITIES:

Gastrointestinal

Hematologic

PLAN TO PRESENT PRECLINICAL DATA FOR BLU-222 IN 1H 2022

Selective CDK2 inhibition led to sustained anti-tumor activity in CCNE1-amplified *in vivo* models



■ Vehicle ▲ BLU2256 5 mg/kg BID

PLAN TO PRESENT PRECLINICAL DATA FOR BLU-222 IN 1H 2022

On track to initiate broad BLU-222 clinical development program



PHASE 1/2 TRIAL OF BLU-222 IN CCNE1 ABERRANT CANCERS

PHASE 1 DOSE ESCALATION

Multiple dose cohorts

- Safety
- Preliminary clinical activity
- Patient selection strategy

RP2D

PHASE 2 EXPANSION

Monotherapy – CCNE1 tumor(s) with accelerated development path

Monotherapy – multiple other CCNE1 tumors (basket cohort)

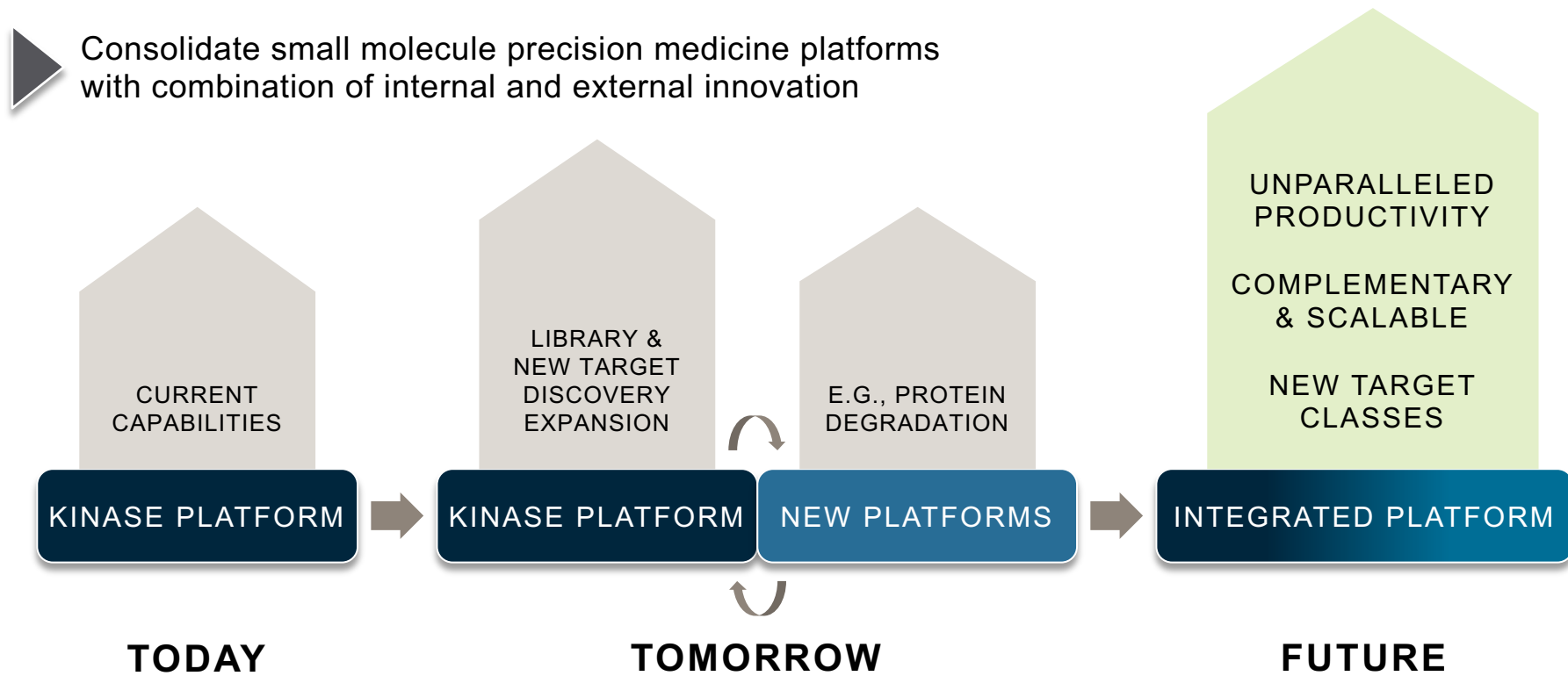
Combo with chemotherapy – multiple CCNE1 tumors

Combo with CDK4/6i + ER antagonist – ER+/HER2- breast cancer

PLAN TO INITIATE VELA TRIAL OF BLU-222 IN Q1 2022

Planned research platform expansion to drive innovation & expanded productivity

Consolidate small molecule precision medicine platforms with combination of internal and external innovation



		DISCOVERY	EARLY-STAGE DEVELOPMENT	LATE-STAGE DEVELOPMENT	REGULATORY SUBMISSION	APPROVED
Hematologic disorders	AYVAKIT® (avapritinib): KIT	Advanced SM ^{1,2}			MAA	U.S.
		Non-advanced SM ¹				
	BLU-263: KIT	Non-advanced SM				
Genomically defined cancers	AYVAKIT® (avapritinib): PDGFRA	PDGFRA GIST ^{1,3,4}				U.S., Europe
	GAVRETO® (pralsetinib): RET	RET+ NSCLC ^{1,3,5,6}				U.S., Europe
		RET+ thyroid cancer ^{1,3,5,7}			MAA	U.S.
		Other RET+ solid tumors ^{1,3,5}				
	Fisogatinib: FGFR4	Advanced HCC (+/- sugemalimab) ¹				
	BLU-701: EGFR	EGFR+ NSCLC ^{3,8}				
	BLU-945: EGFR	EGFR+ NSCLC ^{3,8}				
	BLU-451: EGFR exon 20 insertions	EGFR+ NSCLC ³				
Cancer immunotherapy	BLU-222: CDK2	Cyclin E aberrant cancers				
	BLU-852: MAP4K1	Advanced cancers ⁹				
	Multiple undisclosed research programs					

 ongoing or completed
 planned

1. CStone Pharmaceuticals has exclusive rights to develop and commercialize avapritinib, pralsetinib and fisogatinib in Mainland China, Hong Kong, Macau and Taiwan. 2. Approved in the U.S. for the treatment of adults with advanced SM, including aggressive SM, SM with an associated hematologic neoplasm and mast cell leukemia. 3. Unresectable or metastatic disease. 4. 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. 5. 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. 6. 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. Received conditional marketing authorization in Europe for the treatment of adults with advanced RET fusion-positive NSCLC not previously treated with a RET inhibitor. 7. 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. 8. Zai Lab has exclusive rights to develop and commercialize BLU-701 and BLU-945 in Mainland China, Hong Kong, Macau and Taiwan. 9. 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; NSCLC, non-small cell lung cancer; SM, systemic mastocytosis.



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Updated as of January 10, 2022.

Not for promotional use.

A breadth of planned value-driving milestones across our portfolio

Expand SM leadership	• Obtain EMA approval and launch AYWAKYT in advanced SM in Europe in 1H 2022 • Report topline registration-enabling PIONEER Part 2 trial data for AYWAKIT in non-advanced SM in mid-2022 • Submit sNDA to the FDA for AYWAKIT in non-advanced SM in 2H 2022 • Present data from the HARBOR trial of BLU-263 in non-advanced SM in 2H 2022
Advance programs toward registration	• Present preclinical data for the combination of BLU-945 and BLU-701 in EGFR-driven NSCLC in 1Q 2022 • Present initial SYMPHONY trial data for BLU-945 in EGFR-driven NSCLC in 1H 2022 • Present initial HARMONY trial data for BLU-701 in EGFR-driven NSCLC in 2H 2022 • Initiate Phase 1/2 trial of BLU-451 in Exon 20 insertion positive NSCLC in 1Q 2022 • Present preclinical data for BLU-451 in Exon 20 insertion positive NSCLC in 1H 2022 • Initiate VELA trial of BLU-222 in cyclin-E aberrant cancers in 1Q 2022 • Present preclinical data for BLU-222 in cyclin-E aberrant cancers in 1H 2022 • Submit additional marketing applications for GAVRETO for RET-altered NSCLC and thyroid cancers across multiple global geographies in 2022, via ongoing Roche collaboration
Grow R&D pipeline	• Nominate two new development candidates in 2022 • Share the company's precision therapy research vision, including scientific platform expansion plans, at an R&D Day in 2H 2022

Our path to potential transformative growth and an independent financial profile

YEAR END 2021

- AYYAKIT/AYVAKYT and GAVRETO approved with ongoing global expansion
- A leading precision therapy research platform
- Strong financial position with ~\$1B cash and cash equivalents

NEAR-TERM PLANS

- Constellation of clinical across strategic therapies
- AYYAKIT launch in non-systemic mastocytosis
- Continued product revenue collaboration milestones

FUTURE GOALS • 2024-2025+

- Broad portfolio of marketed medicines in precision oncology and hematology
- Diversified research platform with unparalleled productivity
- Independent financial profile