UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934

Date of Report (Date of Earliest Event Reported): **April 15, 2018**

Blueprint Medicines Corporation
(Exact name of registrant as specified in its charter)

Delaware	**001-37359**	**26-3632015**
(State or other jurisdiction of incorporation)	(Commission File Number)	(I.R.S. Employer Identification No.)

45 Sidney Street **Cambridge, Massachusetts**	**02139**
(Address of principal executive offices)	(Zip Code)

Registrant's telephone number, including area code: **(617) 374-7580**

(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- o Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- o Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- o Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- o Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 7.01 Regulation FD Disclosure.

On April 15, 2018, Blueprint Medicines Corporation (the "Company") issued a press release announcing data from its ongoing Phase 1 clinical trial evaluating BLU-667 for the treatment of RET-altered non-small cell lung cancer, medullary thyroid cancer and other advanced solid tumors. The data were presented on Sunday, April 15, 2018 in an oral presentation during the clinical trials plenary session at the American Association for Cancer Research ("AACR") Annual Meeting in Chicago, Illinois. A copy of the press release is furnished as Exhibit 99.1 to this Current Report on Form 8-K, and a copy of the presentation at the AACR Annual Meeting is furnished as Exhibit 99.2 to this Current Report on Form 8-K.

In addition, on April 15, 2018, the Company hosted an investor event and live webcast to discuss the data presented at the AACR Annual Meeting. A copy of the presentation from the investor event is furnished as Exhibit 99.3 to this Current Report on Form 8-K.

The information in Item 7.01 of this Current Report on Form 8-K, including Exhibits 99.1, 99.2 and 99.3, is intended to be furnished and shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits.

Exhibit No.	Description
99.1	Press release issued by Blueprint Medicines Corporation on April 15, 2018
99.2	Presentation by Blueprint Medicines Corporation at the AACR Annual Meeting on April 15, 2018
99.3	Presentation by Blueprint Medicines Corporation at investor event on April 15, 2018

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

BLUEPRINT MEDICINES CORPORATION

Date: April 16, 2018

By: /s/ Tracey L. McCain
Tracey L. McCain
Chief Legal Officer

Blueprint Medicines Announces Proof-of-Concept Data for Highly Selective RET Inhibitor BLU-667 from Phase 1 ARROW Clinical Trial in Patients with RET-Altered Solid Tumors

– Broad Anti-Tumor Activity Observed across Multiple Tumor Types and RET Genotypes, with Radiographic Tumor Reductions in 84% of Evaluable Patients with RET-Altered Tumors –
– Expansion Portion of ARROW Trial Initiated and Actively Enrolling Patients Globally –
– Blueprint Medicines to Host Investor Event and Webcast on Sunday, April 15, 2018 –

CAMBRIDGE, Mass., April 15, 2018 – Blueprint Medicines Corporation (NASDAQ:BPMC), a leader in discovering and developing targeted kinase medicines for patients with genomically defined diseases, today announced proof-of-concept data from the ongoing Phase 1 ARROW clinical trial of BLU-667 in patients with RET-altered solid tumors. Designed and developed by Blueprint Medicines, BLU-667 is a potent and highly selective inhibitor targeting oncogenic RET fusions and mutations, which are key drivers across multiple cancers, including subsets of patients with non-small cell lung cancer (NSCLC) and medullary thyroid cancer (MTC). The data will be presented today in a clinical trials plenary session at the American Association for Cancer Research (AACR) Annual Meeting in Chicago, Illinois.

The data from the dose escalation portion of the ARROW trial showed broad and robust clinical activity for once-daily (QD) dosing of BLU-667 across multiple tumor types and RET genotypes, including in patients whose disease had progressed on prior multi-kinase inhibitor therapy. As of the data cutoff date of April 6, 2018, the data showed radiographic tumor reductions in 84 percent of patients with RET-altered solid tumors with measurable target lesions. In patients evaluable for response, preliminary overall response rates (ORR) were 50 percent in patients with NSCLC and 40 percent in patients with MTC. As of the data cutoff date, QD dosing of BLU-667 was well-tolerated, and most adverse events (AEs) reported by investigators were Grade 1 or 2.

“The data announced today reveal the broad clinical potential of BLU-667, a potent and highly selective RET inhibitor, and further demonstrate the power and reproducibility of Blueprint Medicines’ proprietary drug discovery platform,” said Andy Boral, M.D., Ph.D., Chief Medical Officer at Blueprint Medicines. “We believe the safety, clinical activity and pharmacodynamic results from the dose escalation portion of the Phase 1 ARROW trial demonstrate compelling proof-of-concept for BLU-667. We are particularly encouraged by the consistency of these early BLU-667 data across multiple tumor types, RET alterations and prior lines of therapy. Based on these data, we are excited to rapidly advance the global expansion portion of the trial, which will further evaluate an optimized dose of BLU-667 across a broad patient population with a focus on durability of activity.”

Data from the Ongoing Phase 1 ARROW Clinical Trial

As of the data cutoff date of April 6, 2018, 53 patients had been treated with BLU-667 in the dose escalation portion of the Phase 1 ARROW clinical trial across multiple dose levels ranging from 30 mg to 600 mg QD, including 19 patients with NSCLC, 29 patients with MTC and five patients with other solid tumors. Of these 53 patients, 27 patients (51 percent) had been previously treated with a multi-kinase inhibitor and 18 patients (34 percent) had been previously treated with an immunotherapy.

Pharmacokinetic (PK) data across all QD dose levels demonstrated rapid absorption of BLU-667 and a mean half-life greater than 12 hours, supporting a QD dosing regimen.

Preliminary Safety Data:

As of the data cutoff date, QD dosing of BLU-667 was observed to be well-tolerated. The maximum tolerated dose (MTD) for BLU-667 was determined to be 400 mg QD using a Bayesian optimal interval design. At QD dose levels up to and including the MTD, the majority of AEs reported by investigators were Grade 1 or 2. AEs reported by investigators (≥20 percent) most commonly included constipation (24 percent), increased alanine aminotransferase (ALT) (22 percent) and increased aspartate aminotransferase (20 percent). Investigators reported treatment-related Grade 3 AEs in eight patients (16 percent). Treatment-related Grade 3 AEs occurring in two or more patients included hypertension and neutropenia. There were no treatment-related Grade 4 or 5 AEs.

Across all QD dose levels up to 600 mg QD, seven patients experienced dose-limiting toxicities. Only one patient discontinued treatment with BLU-667 due to a dose-limiting toxicity (Grade 3 ALT increase). An additional 11 patients discontinued treatment, including eight patients due to progressive disease, one patient due to an AE unrelated to BLU-667 and one patient due to non-compliance. One patient passed away, and the death was deemed unrelated to BLU-667. Among all 53 enrolled patients, 41 patients (77 percent) remained on BLU-667 as of the data cutoff date. Duration of treatment ranged from 0.3 to 11.5 months.

Preliminary Clinical Activity Data:

As of the data cutoff date, 40 patients with RET-altered tumors were evaluable for response assessment, including 14 patients with NSCLC, 25 patients with MTC and one patient with papillary thyroid cancer (PTC). CT and MRI imaging was used to measure clinical activity by Response Evaluation Criteria in Solid Tumors (RECIST) version 1.1. Of the remaining 13 enrolled patients who were not evaluable for response assessment, two patients did not have RET-altered tumors, one patient died due to an AE unrelated to BLU-667 prior to any response assessment and 10 recently enrolled patients had not been evaluated for response by the data cutoff date.

Across all evaluable patients, the preliminary ORR was 45 percent. Responses were observed in patients previously treated with multi-kinase therapy, immunotherapy and chemotherapy.

RET-altered NSCLC
- 85% of NSCLC patients with measurable target lesions had radiographic tumor reductions.
- Seven patients achieved a partial response (PR) (five confirmed, two pending confirmation), representing a preliminary ORR of 50 percent.
- Responses were observed in patients with the most common RET alterations in NSCLC, including RET-KIF5B and RET-CCDC6 fusions.
- Preliminary evidence of anti-tumor activity in the brain was observed in metastatic NSCLC.

RET-altered MTC
- 83% of MTC patients with measurable target lesions had radiographic tumor reductions.
- One patient achieved a confirmed complete response, nine patients achieved a PR (five confirmed, four pending confirmation), representing a preliminary ORR of 40 percent.

- Responses were observed in patients with the most common activating RET mutations in MTC, including the RET-M918T mutation.

Other RET-altered solid tumors
- One patient with RET-altered PTC achieved a PR (pending confirmation).

Based on the favorable tolerability and encouraging clinical activity observed for BLU-667 to date, Blueprint Medicines initiated and is actively enrolling patients in the global expansion portion of the ARROW trial.

Investor Event and Webcast Information

Blueprint Medicines will host an investor event on Sunday, April 15, 2018 beginning at 7:00 p.m. CT (8:00 p.m. ET) in Chicago to review the preliminary clinical data presented at AACR for BLU-667. Formal presentations and the live webcast will begin at 7:30 p.m. CT (8:30 p.m. ET). The event can be accessed by dialing 1-855-728-4793 (domestic) or 1-503-343-6666 (international) and providing the passcode 6080608. A live webcast will also be available under "Events & Presentations" in the Investors section of Blueprint Medicines' website at http://ir.blueprintmedicines.com. The archived webcast will be available on Blueprint Medicines' website approximately two hours after the event concludes and will be available for 30 days following the event.

About the Phase 1 ARROW Clinical Trial of BLU-667

ARROW is a Phase 1 clinical trial of BLU-667 designed to evaluate the safety and tolerability of BLU-667 in multiple ascending doses in adults with RET-altered NSCLC, MTC and other advanced solid tumors. The trial consists of two parts: a dose escalation portion and an expansion portion. Enrollment in the dose escalation portion is complete, and the expansion portion has been initiated and is actively enrolling patients in four defined cohorts at the MTD of 400mg QD: (1) RET-altered NSCLC patients previously treated with a tyrosine kinase inhibitor (TKI), (2) RET-altered NSCLC patients who have not previously received any TKI treatment, (3) patients with medullary thyroid cancer, and (4) patients with other RET-altered solid tumors. Trial objectives include assessing response, pharmacokinetics, pharmacodynamics and safety.

Patients and physicians interested in the ARROW trial can contact the Blueprint Medicines study team at studydirector@blueprintmedicines.com or 1-617-714-6707. More information about the ARROW trial is also available at www.arrowtrial.com or www.clinicaltrials.gov (Identifier: NCT03037385).

About RET-Altered NSCLC, MTC and Other Solid Tumors

RET activating fusions and mutations are a key disease driver in multiple cancers, including NSCLC and MTC. RET fusions are implicated in approximately 1-2% of patients with NSCLC, while RET mutations are implicated in approximately 60% of patients with MTC. In addition, genomic analyses published by scientists at Blueprint Medicines have identified RET fusions at low frequencies in colon and breast cancer. Currently, there are no approved therapies that selectively target RET-driven cancers, though there are several approved multi-kinase inhibitors with RET activity being evaluated in clinical trials. Thus far, clinical activity attributable to RET inhibition has been uncertain for these inhibitors, likely due to insufficient inhibition of RET and off-target toxicities.

About BLU-667

BLU-667 is an orally available, potent and highly selective inhibitor designed to target RET fusions, mutations and predicted resistance mutations. Blueprint Medicines is developing BLU-667, an investigational medicine, for the treatment of patients with RET-altered NSCLC, MTC and other solid tumors. BLU-667 was discovered by Blueprint Medicine's research team leveraging its proprietary compound library, and Blueprint Medicines retains worldwide development and commercialization rights for BLU-667.

About Blueprint Medicines

Blueprint Medicines is developing a new generation of targeted and potent kinase medicines to improve the lives of patients with genomically defined diseases. Its approach is rooted in a deep understanding of the genetic blueprint of cancer and other disease driven by the abnormal activation of kinases. Blueprint Medicines is advancing multiple programs in clinical development for subsets of patients with gastrointestinal stromal tumors, hepatocellular carcinoma, systemic mastocytosis, non-small cell lung cancer, medullary thyroid cancer and other advanced solid tumors, as well as multiple programs in research and preclinical development. For more information, please visit www.blueprintmedicines.com.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, statements regarding plans and timelines for the clinical development of BLU-667, including plans and timelines for advancing the expansion portion of the Phase 1 ARROW trial; expectations regarding the safety and efficacy of BLU-667 and the potential benefits of BLU-667 in treating patients with RET-altered cancers; plans to advance a QD dosing regimen for BLU-667; plans and timelines for activating additional clinical sites in the Phase 1 ARROW trial; expectations regarding enrollment in the expansion portion of the Phase 1 ARROW trial; and Blueprint Medicines' strategy, business plans and focus. The words "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, risks and uncertainties related to the delay of any current or planned clinical trials or the development of Blueprint Medicines' drug candidates, including avapritinib, BLU-554, BLU-667 and BLU-782; Blueprint Medicines' advancement of multiple early-stage efforts; Blueprint Medicines' ability to successfully demonstrate the safety and efficacy of its drug candidates; the preclinical and clinical results for Blueprint Medicines' drug candidates, which may not support further development of such drug candidates; and actions of regulatory agencies, which may affect the initiation, timing and progress of clinical trials; Blueprint Medicines' ability to develop and commercialize companion diagnostic tests for its current and future drug candidates, including companion diagnostic tests for BLU-554 for FGFR4-driven HCC, avapritinib for PDGFRα D842V-driven GIST and BLU-667 for RET-driven NSCLC; and the success of Blueprint Medicines' cancer immunotherapy collaboration with F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc. These and other risks and uncertainties are described in greater detail in the section entitled "Risk Factors" in Blueprint Medicines' Annual Report on Form 10-K for the year ended December 31, 2017, as filed with the Securities and Exchange Commission

(SEC) on February 21, 2018, and other filings that Blueprint Medicines has made or may make with the SEC in the future. Any forward-looking statements contained in this press release represent Blueprint Medicines' views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date. Blueprint Medicines explicitly disclaims any obligation to update any forward-looking statements.

Investor and Media Relations Contacts

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Highly potent and selective RET inhibitor, BLU-667, achieves proof of concept in ARROW, a phase 1 study of advanced, RET-altered solid tumors

Vivek Subbiah[1], Matthew Taylor[2], Jessica Lin[3], Mimi Hu[1], Sai-Hong Ignatius Ou[4], Marcia S. Brose[5], Elena Garralda[6], Corinne Clifford[7], Michael Palmer[7], Meera Tugnait,[7] Erica Evans[7], Hongliang Shi[7], Beni Wolf[7], and Justin Gainor[3]

[1]Department of Investigational Cancer Therapeutics, The University of Texas MD Anderson Cancer Center, Houston, United States;
[2]The Knight Cancer Institute, Oregon Health & Science University, Portland, United States;
[3]Department of Medicine, Massachusetts General Hospital, Boston, United States,
[4]Chao Family Comprehensive Cancer Center, University of California Irvine Medical Center, United States;
[5]Abramson Cancer Center, University Of Pennsylvania, Philadelphia, United States;
[6]Vall d'Hebron Institute of Oncology, Vall d'Hebron University Hospital, Barcelona, Spain;
[7]Blueprint Medicines Corporation, Cambridge, United States;

NCT03037385

Disclosures

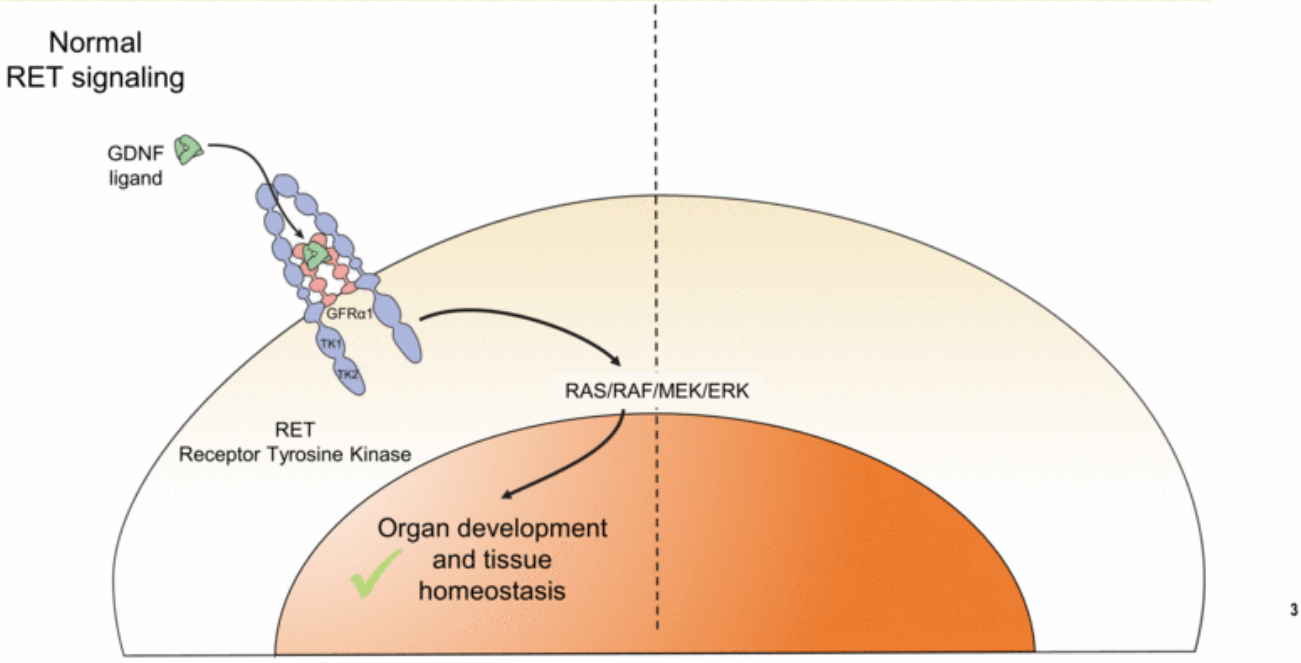
I have the following financial relationships to disclose:

Grant/Research support from:

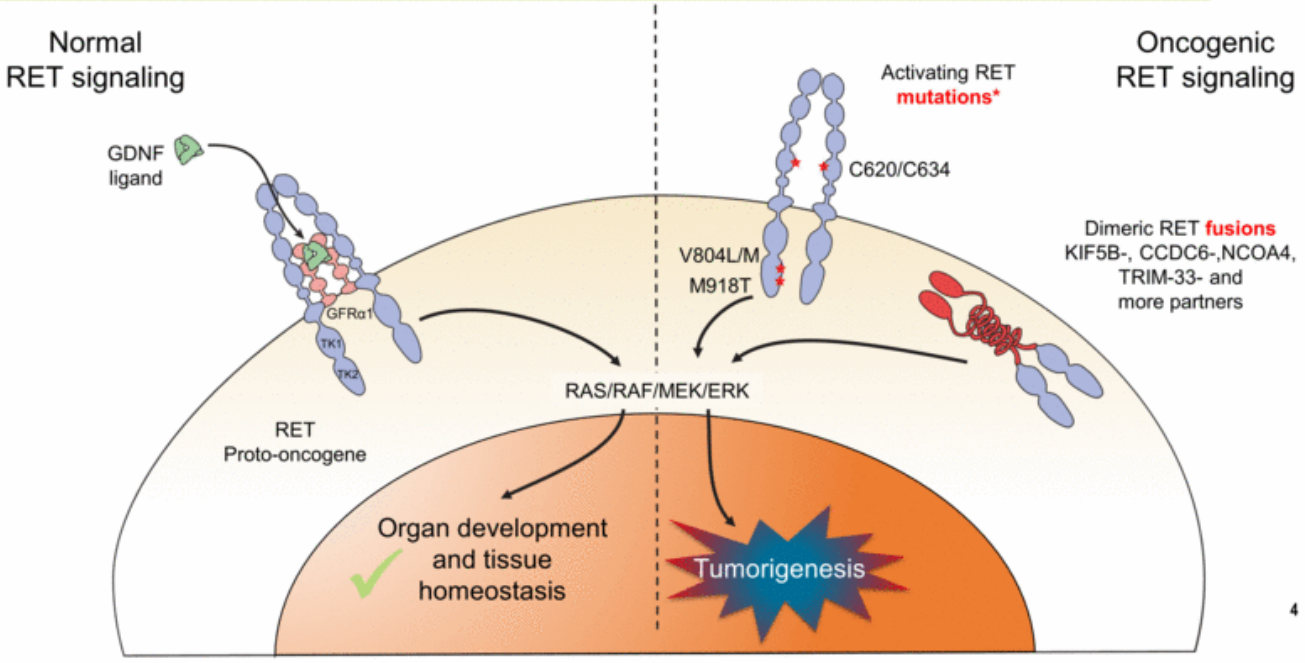
- **Blueprint Medicines Corporation**
- Novartis International AG
- Bayer AG
- GlaxoSmithKline plc
- NanoCarrier Co. Ltd
- Vegenics Pty Ltd
- Northwest Biotherapeutics
- Boston Biomedical Inc
- Berg
- Incyte Corporation
- Fujifilm Holdings Corporation
- PharmaMar
- D3
- Pfizer Inc
- MultiVir Inc
- Amgen Inc
- AbbVie Inc
- Loxo Oncology
- F. Hoffmann-La Roche AG / Genentech Inc
- National Comprehensive Cancer Network
- National Cancer Institute-Cancer Therapy Evaluation Program

BLU-667 is an investigational agent discovered and currently in development by Blueprint Medicines Corporation (Blueprint Medicines)

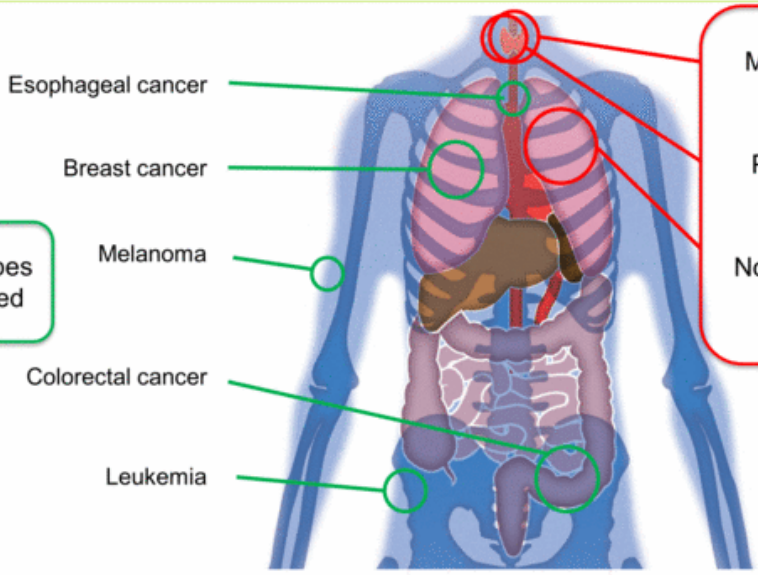
Receptor tyrosine kinase, *RE*arranged during *T*ransfection (*RET*)



Receptor tyrosine kinase, *RE*arranged during *T*ransfection (*RET*)



RET is a rare driver of multiple, diverse tumor types[1,2]



Other tumor types
≤1% *RET*-altered

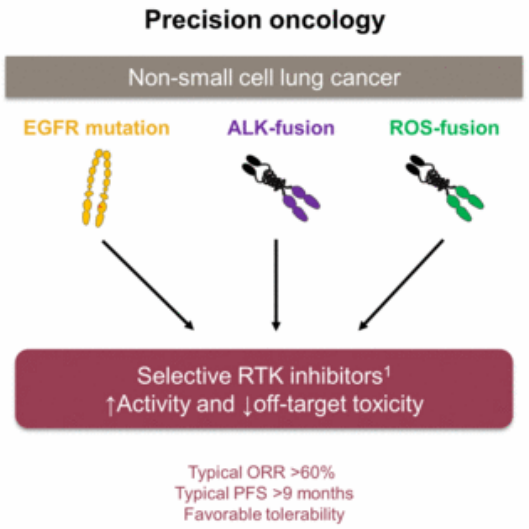
Medullary thyroid cancer
>60% *RET*-mutations

Papillary thyroid cancer
~10% *RET*-fusions

Non-small cell lung cancer
~1-2% *RET*-fusions

1. Drilon A et al. *Nat Rev Clin Oncol*. 2018;15:151-67 2.Kato S, et al. *Clin Cancer Res* 2017;23:1988-1997.

Patients with *RET*-alterations have not benefited from precision oncology

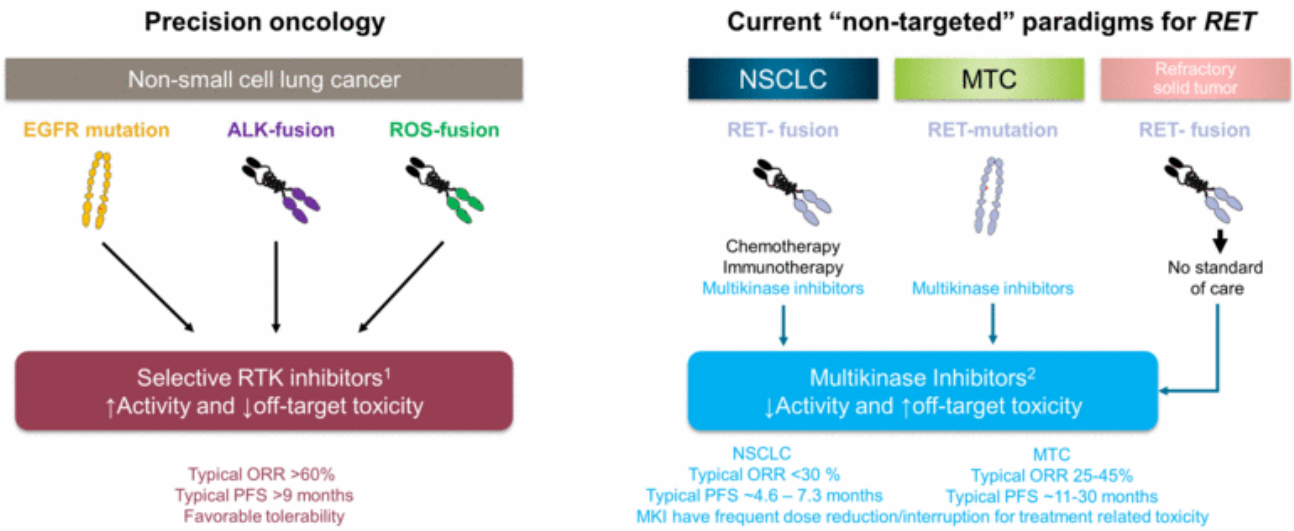


Typical ORR >60%
Typical PFS >9 months
Favorable tolerability

MKI, multikinase inhibitors; MTC, medullary thyroid cancer; NSCLC, non-small cell lung cancer; ORR, overall response rate; PFS, progression-free survival; RTK, receptor tyrosine kinase

1. Herbst RS et al. *Nature* 2018; 553:446-54; 2. Drilon A et al. *Nat Rev Clin Oncol*. 2018;15:151-67.

Patients with *RET*-alterations have not benefited from precision oncology



MKI, multikinase inhibitors; MTC, medullary thyroid cancer; NSCLC, non-small cell lung cancer; ORR, overall response rate; PFS, progression-free survival; RTK, receptor tyrosine kinase

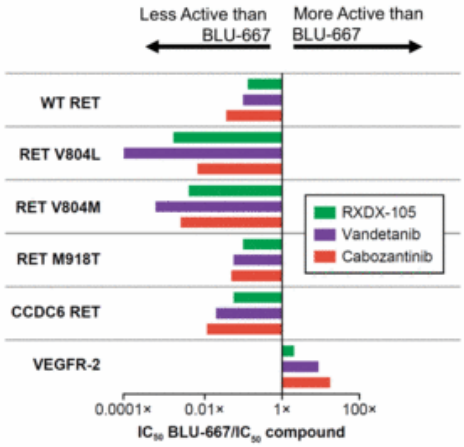
1. Herbst RS et al. *Nature* 2018; 553:446-54; 2. Drilon A et al. *Nat Rev Clin Oncol*. 2018;15:151-67.

BLU-667 was designed to treat RET-altered cancers

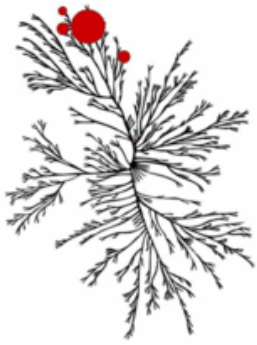
Subnanomolar potency[1]

Variant	Biochemical IC_{50} (nM)
RET wildtype	0.4
RET V804L	0.3
RET V804M	0.4
RET M918T	0.4
CCDC6-RET	0.4

More Potent than MKI

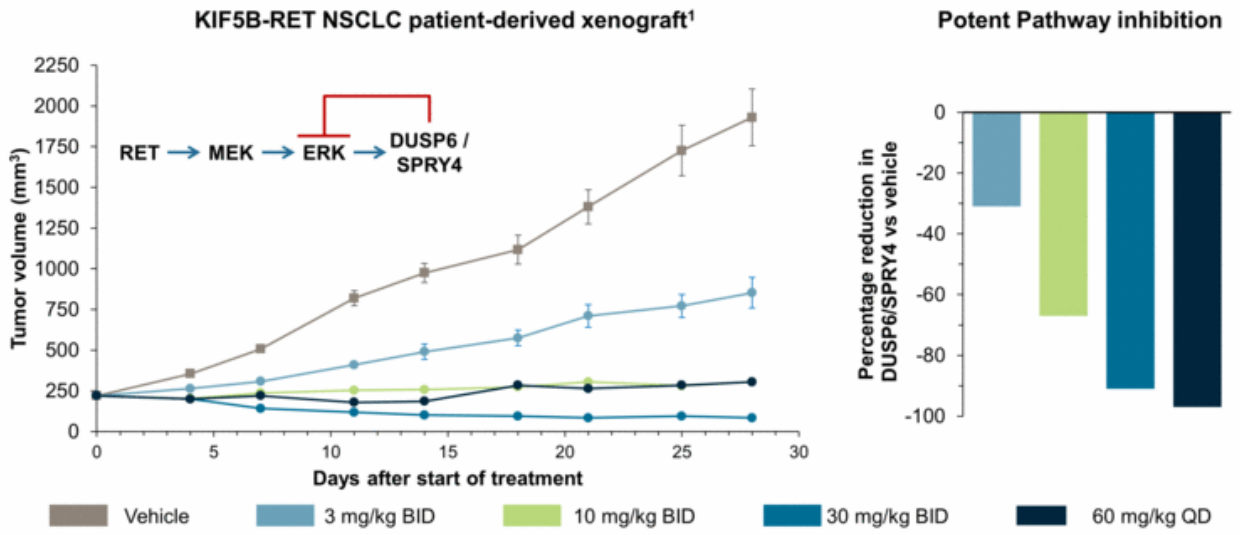


Kinome selectivity for RET



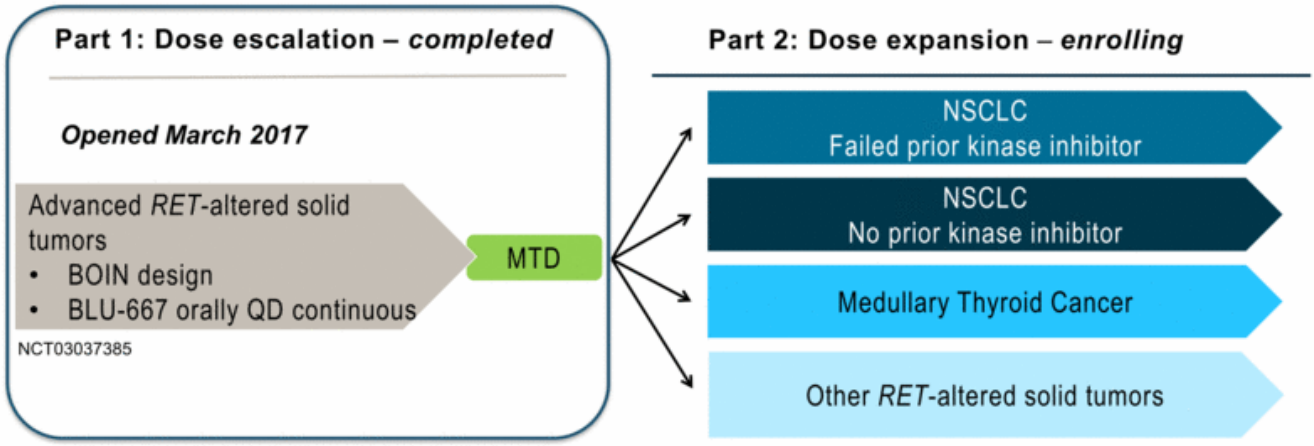
1. Subbiah V et al. *Cancer Discovery* April 15 2018

BLU-667 potently inhibits RET-driven tumor growth



BID, two times per day; QD, once daily 1. Subbiah V et al. *Cancer Discovery* April 15 2018

BLU-667 ARROW first-in-human study



Key objectives

- MTD, safety, pharmacokinetics, pharmacodynamics, anti-tumor activity

BOIN, Bayesian optimal interval; MTD, maximum tolerated dose

Demography and baseline characteristics

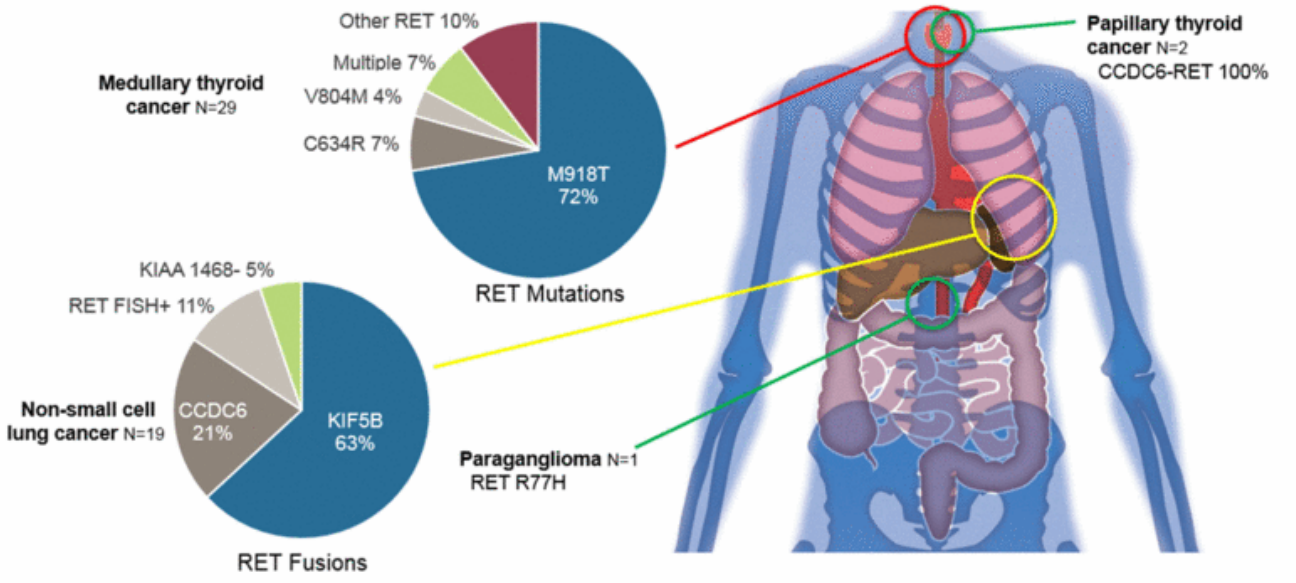
Parameter	(N=53)
Age, years; median (range)	56 (19-83)
Sex, male; n (%)	30 (57)
ECOG PS; n (%)	
0	21 (40)
1	32 (60)
Metastatic disease; n (%)	50 (94)
Tumor type; n (%)	
RET-alteration	51 (96)
Medullary thyroid cancer	29 (55)
Non-small cell lung cancer	19 (36)
Papillary thyroid cancer	2 (4)
Retroperitoneal Paraganglioma	1 (2)
Non-*RET* altered solid tumor	2 (4)

Parameter	(N=53)
Prior systemic therapy; n (%)	41 (77)
Multikinase inhibitor; n (%)	27 (51)
Chemotherapy; n (%)	19 (36)
Immunotherapy; n (%)	18 (34)
# of lines, median (range)	1 (0-8)

ECOG PS, Eastern Cooperative Oncology Group performance score

Data cut-off: April 6, 2018

Diverse *RET* genotypes enrolled



Data cut-off: April 6, 2018

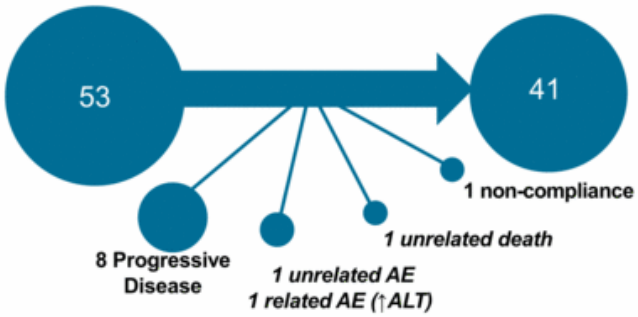
Dose escalation results

Maximum Tolerated Dose – 400 mg QD

Dose (mg QD)	# Evaluable (N=49)	Dose limiting toxicity
30	1	None
60	6	None
100	5	Alanine transaminase increased (1)
200	12	None
300	11	Tumor lysis syndrome (1) Hypertension (1)
400	10	Asthenia (1) Hypertension (1)
600	4	Hyponatremia (1) Hypertension (1)

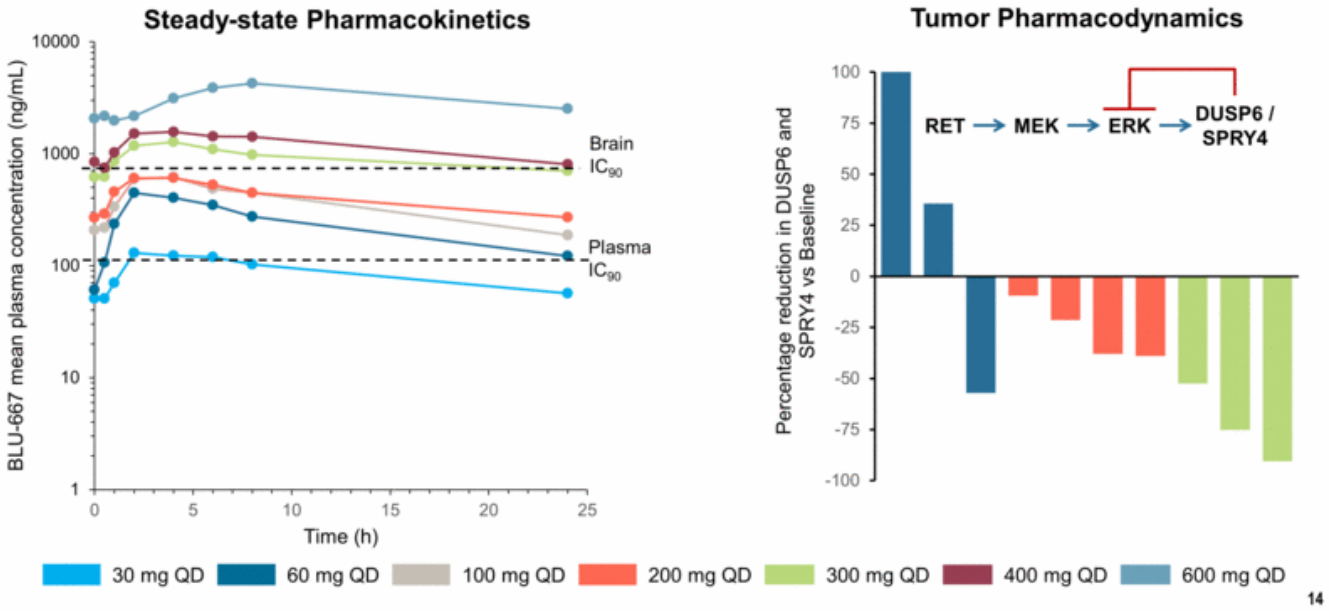
ALT, alanine aminotransferase

41 of 53 patients remain on treatment (median 3.9 months [range: 0.3–11.5])

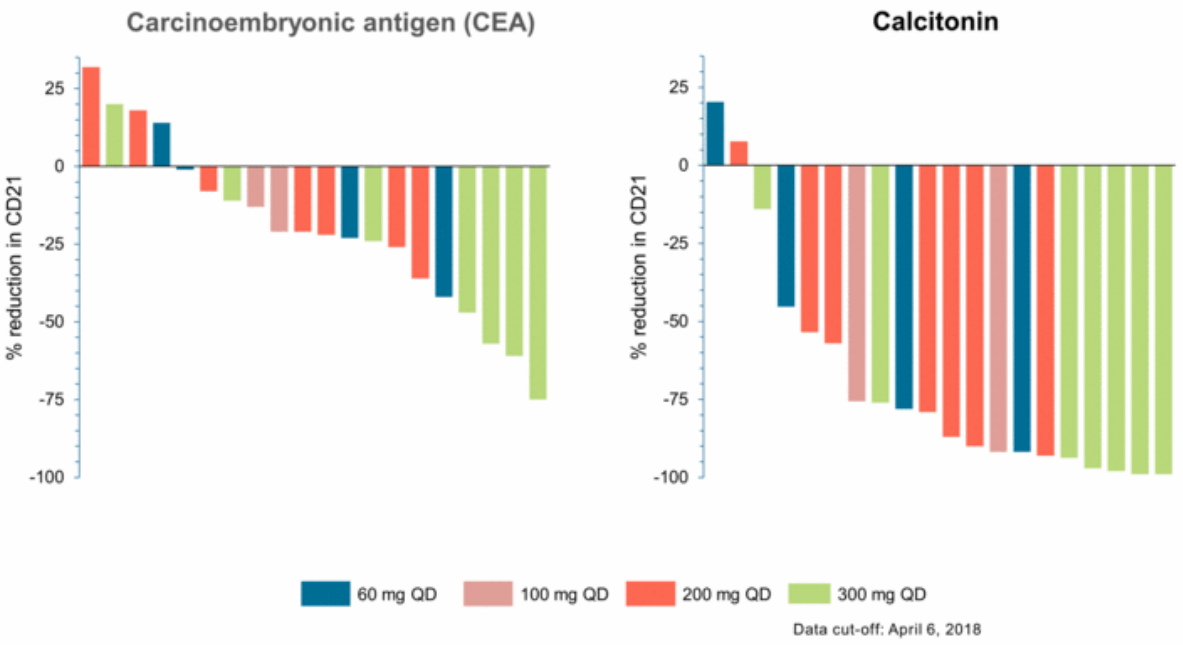


Data cut-off: April 6, 2018

Dose-dependent exposure and RET pathway inhibition

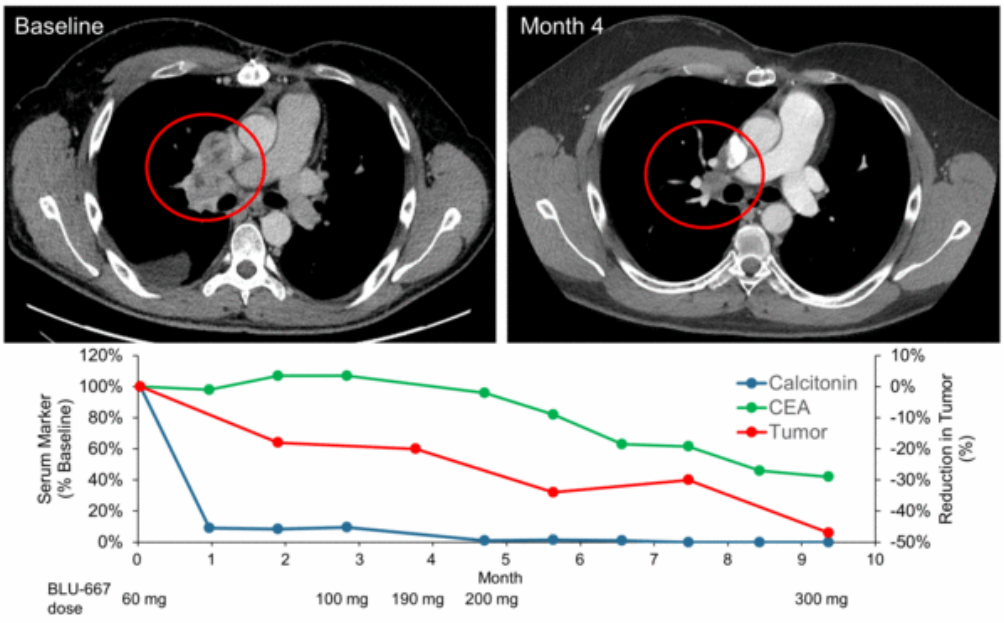


Dose-dependent decline in MTC tumor markers



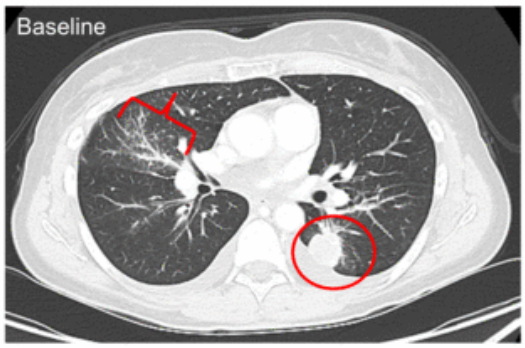
Data cut-off: April 6, 2018

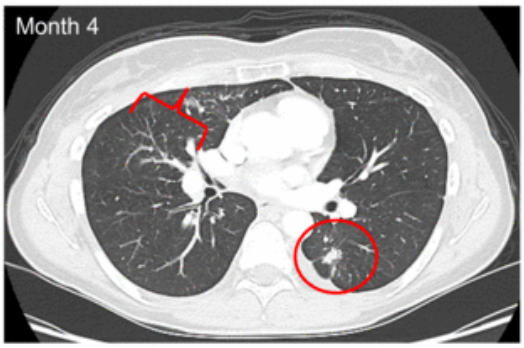
Potent activity against highly invasive *RET*-mutant MTC



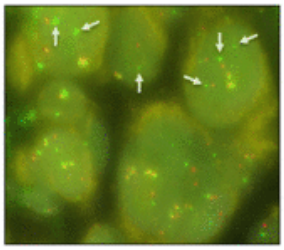
27-year-old male; *RET* L629-D361 Del; initiated at 60 mg; ongoing at 400 mg with confirmed PR

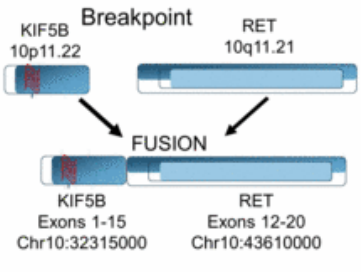
Potent activity against KIF5B-RET NSCLC – post chemotherapy

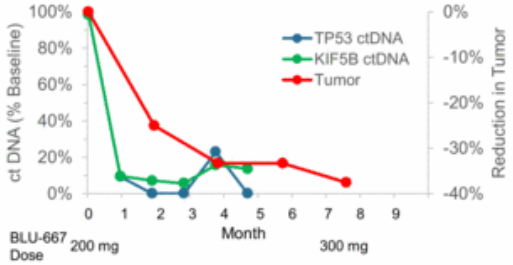




FISH







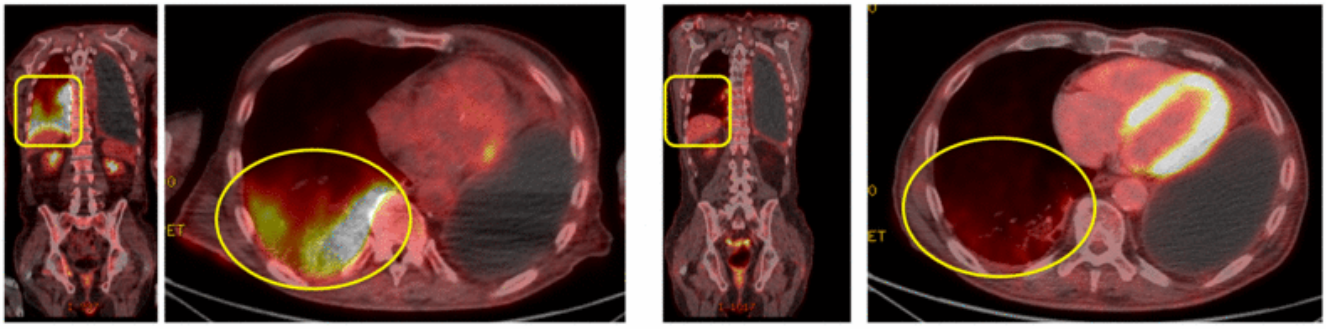
37-year-old female; ongoing at 400 mg with confirmed PR

Subbiah V et al. *Cancer Discovery* April 15 2018

Potent activity against KIF5B-RET NSCLC – post-vandetinib+everolimus

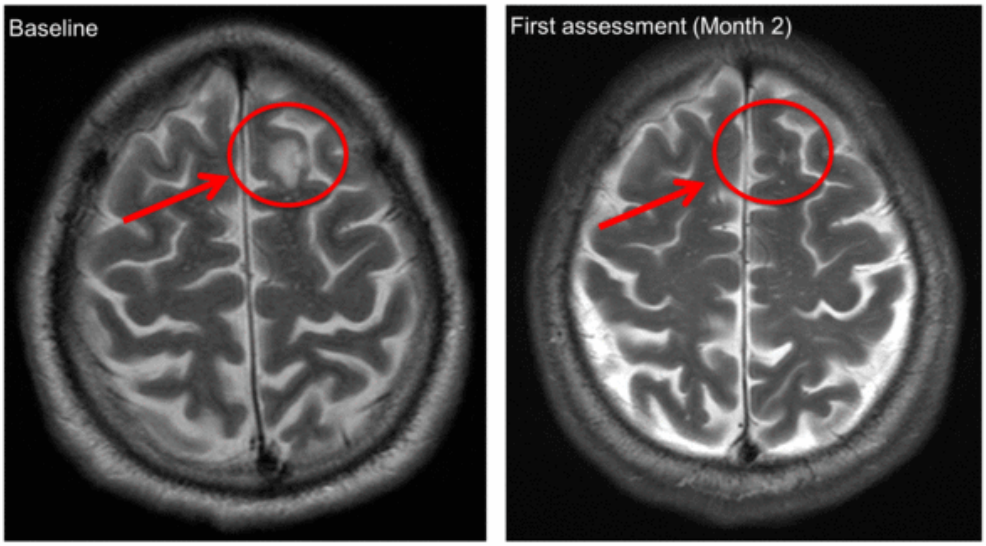
Baseline

First Assessment (Month 2)



74-year-old male; initiated at 300 mg; ongoing at 400 mg; PR at month 5 pending confirmation Subbiah V et al. *Cancer Discovery* April 15 2018

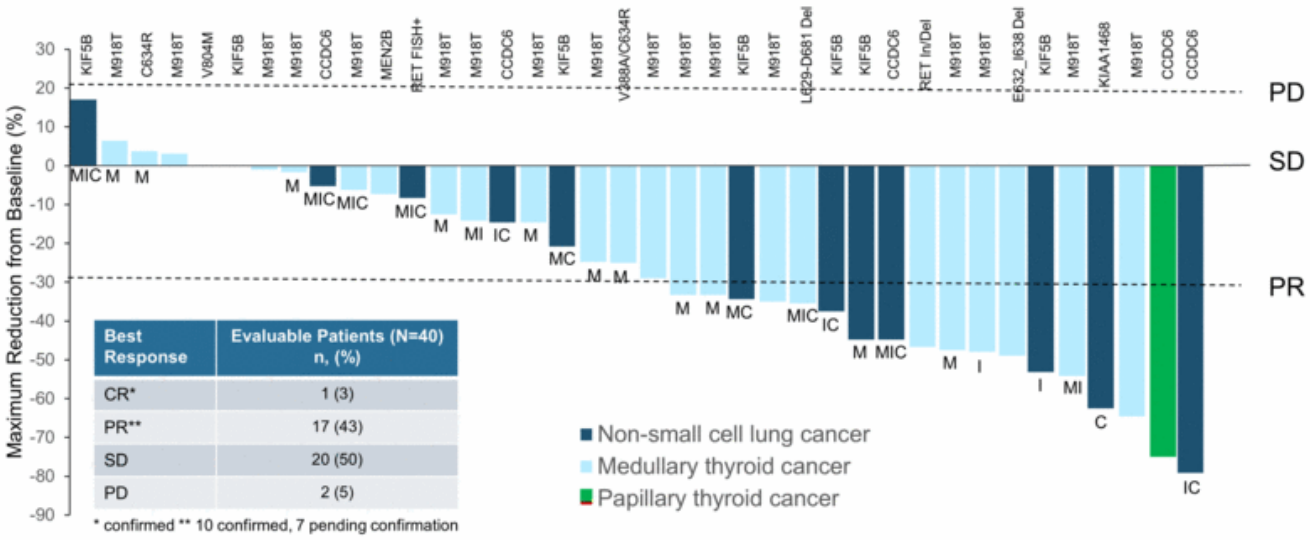
Activity against KIF5B-RET NSCLC brain metastases



69-year-old male; initiated at 400 mg; ongoing at month 4

Images courtesy of Drs. Gainor, J and Lin, J of MGH

BLU-667 has broad anti-tumor activity against RET-altered cancers



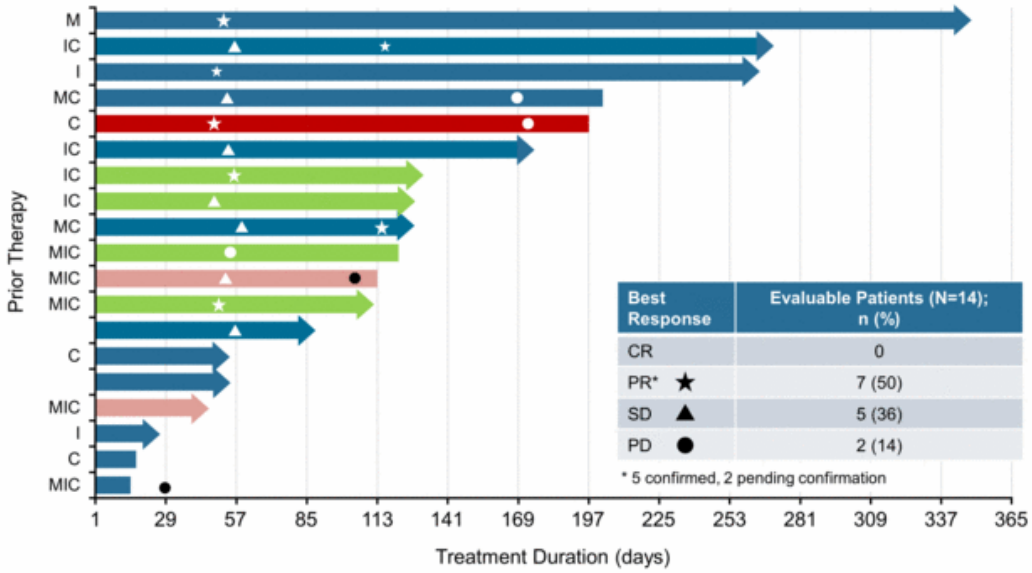
Best Response	Evaluable Patients (N=40) n, (%)
CR*	1 (3)
PR**	17 (43)
SD	20 (50)
PD	2 (5)

* confirmed ** 10 confirmed, 7 pending confirmation

C, prior chemotherapy; CR, complete response; I, prior immunotherapy; M, prior MKI therapy; MKI, multikinase inhibitor; PD, progressive disease; PR, partial response; SD, stable disease

Data cut-off: April 6, 2018

BLU-667 has durable activity and high response rate in RET-altered NSCLC



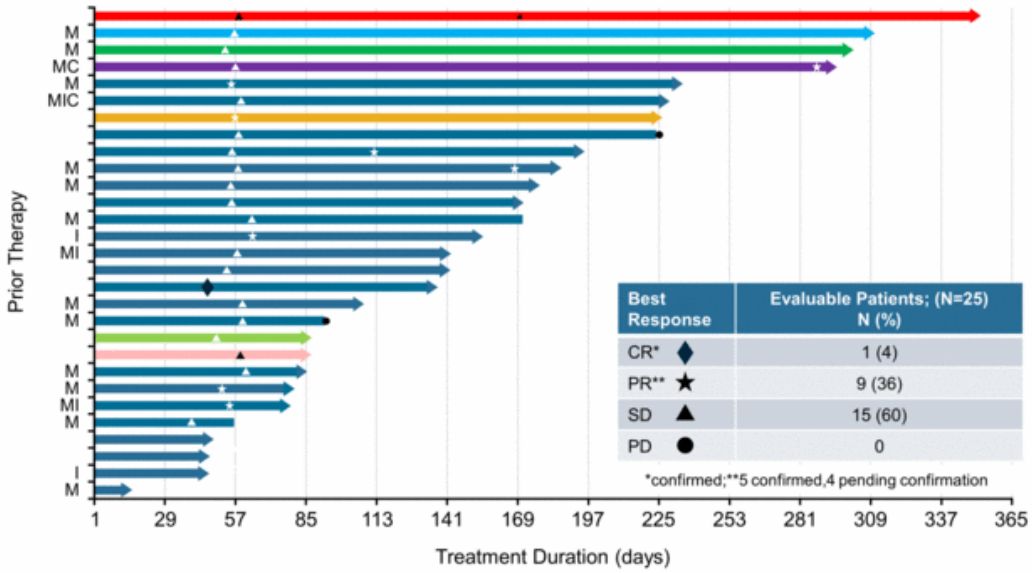
Best Response	Evaluable Patients (N=14); n (%)
CR	0
PR* ★	7 (50)
SD ▲	5 (36)
PD ●	2 (14)

* 5 confirmed, 2 pending confirmation

Treatment duration:
Median 3.9 months
Range 0.4–11.4 months
13/19 (68%) on treatment

Data cut-off: April 6, 2018

BLU-667 has durable activity and high response rate in RET-altered MTC



Best Response	Evaluable Patients; (N=25) N (%)
CR*	1 (4)
PR**	9 (36)
SD	15 (60)
PD	0

*confirmed;**5 confirmed,4 pending confirmation

L629-D361 Del
V388A/C634R
C634R
RET In/Del
M918T
E632_1638 Del
Other RET
V804M

Treatment duration:
Median 4.7 months
Range 0.5–11.5 months
25/29 (86%) on treatment

Data cut-off: April 6, 2018

BLU-667 is well tolerated

Treatment-emergent Adverse Events ≥10% per CTCAE
(30-400 mg Safety Population, N=49)

Adverse event, n (%)	Grade 1	Grade 2	Grade 3	Grade 4/5
Constipation	10 (20)	2 (4)	0	0
ALT increased	10 (20)	0	1 (2)	0
AST increased	8 (16)	2 (4)	0	0
Hypertension	2 (4)	2 (4)	4 (8)	0
Fatigue	5 (10)	1 (2)	1 (2)	0
Edema peripheral	6 (12)	1 (2)	0	0
Diarrhea	4 (8)	1 (2)	1 (2)	0
Blood creatinine increased	6 (12)	0	0	0
Hyperphosphatemia	4 (8)	2 (4)	0	0
Headache	5 (10)	1 (2)	0	0
Leukopenia	5 (10)	0	0	0
Neutropenia	2 (4)	1 (2)	2 (4)	0
White blood cell decreased	2 (4)	2 (4)	1 (2)	0
Insomnia	5 (10)	0	0	0
Cough	3 (6)	2 (4)	0	0

Most adverse events were Grade 1

8 (16%) patients had Grade 3 treatment-related AE

No Grade 4/5 treatment-related AEs

AE, adverse event; ALP, alkaline phosphatase; ALT, analine aminotransferase;
AST, aspartate aminotransferase; CTCAE, common terminology criteria for adverse events

Data cut-off: April 6, 2018

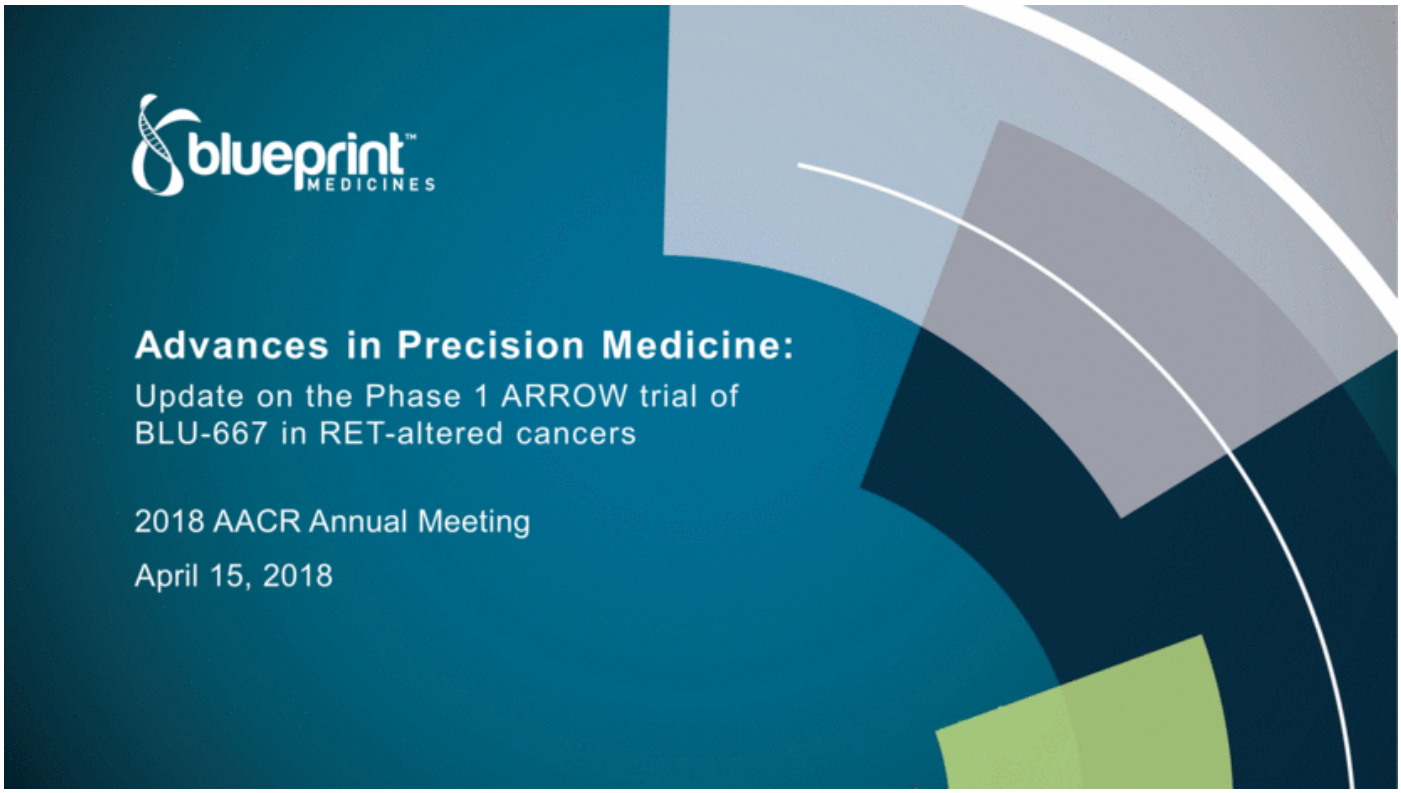
Conclusions

- BLU-667 delivers:
  - Potent RET pathway inhibition with favorable tolerability
  - Broad anti-tumor activity regardless of *RET* genotype, indication and prior therapy
  - High preliminary response rates and durable activity
    - ORR: RET-fusion NSCLC 50%
    - ORR: RET-mutant MTC 40%
    - ORR: *RET*-fusions and mutations (NSCLC, MTC and PTC) 45%
    - 41 of 51 *RET*-altered patients remain on treatment
- ARROW dose escalation data validate BLU-667 as a promising precision therapy for *RET*-altered cancers
- ARROW dose expansion is open and enrolling globally
- BLU-667 manuscript published today in Cancer Discovery
  - Foundational preclinical work and clinical translation

Data cut-off: April 6, 2018

Acknowledgements

- We thank the participating patients, their families, all study co-investigators, and research co-ordinators at the following institutions:
  - Department of Investigational Cancer Therapeutics, The University of Texas MD Anderson Cancer Center, Houston, United States
  - The Knight Cancer Institute Oregon Health & Science University Portland, United States
  - Department of Medicine, Massachusetts General Hospital Cancer Center, Boston, United States
  - Chao Family Comprehensive Cancer Center University of California Irvine Medical Center, United States
  - Abramson Cancer Center, University Of Pennsylvania, United States
  - Vall d'Hebron Institute of Oncology Vall d'Hebron University Hospital, Barcelona, Spain


blueprint MEDICINES
Advances in Precision Medicine:
Update on the Phase 1 ARROW trial of
BLU-667 in RET-altered cancers
2018 AACR Annual Meeting
April 15, 2018

Forward-looking statements

This presentation contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended. The words "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

In this presentation, forward-looking statements include, without limitation, statements about plans and timelines for the development of avapritinib, BLU-554, BLU-667 and BLU-782 and the ability of Blueprint Medicines Corporation (the "Company") to implement those clinical development plans; the potential benefits of the Company's current and future drug candidates in treating patients; plans and timelines for regulatory submissions, filings or discussions; plans and timelines for the development and commercialization of companion diagnostics for the Company's current or future drug candidates; expectations regarding potential milestones; and the Company's strategy, 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 delay of any current or planned clinical trials or the development of the Company's drug candidates, including avapritinib, BLU-554, BLU-667 and BLU-782; the Company's advancement of multiple early-stage efforts; the Company's ability to successfully demonstrate the efficacy and safety of its drug candidates; 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; the Company's ability to obtain, maintain and enforce patent and other intellectual property protection for any drug candidates it is developing; the Company's ability to develop and commercialize companion diagnostic tests for its current and future drug candidates, including companion diagnostic tests for BLU-554 for FGFR4-driven HCC, avapritinib for PDGFRα D842V-driven GIST and BLU-667 for RET-driven non-small cell lung cancer; and the success of the Company's cancer immunotherapy collaboration with F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc.

These and other risks and uncertainties are described in greater detail under "Risk Factors" in the Company's Annual Report on Form 10-K for the year ended December 31, 2017, as filed with the Securities and Exchange Commission ("SEC") on February 21, 2018, and any other filings the Company 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 the Company's 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.

Agenda

Welcome	**Jeff Albers** Chief Executive Officer, Blueprint Medicines
ARROW trial results	**Vivek Subbiah, M.D.** Assistant Professor, Department of Investigational Cancer Therapeutics, Division of Cancer Medicine, MD Anderson Cancer Center
BLU-667 program vision	**Ben Wolf, M.D., Ph.D.** Vice President Clinical Development, Blueprint Medicines
Question and answers	**All**
Closing remarks	**Jeff Albers** Chief Executive Officer, Blueprint Medicines

A new way of looking at kinase medicines

Highly selective kinase medicines offer potential for improved potency, less off-target activity and increased probability of clinical success

SELECTIVE

AVAPRITINIB

NON-SELECTIVE

SUNITINIB

MIDOSTAURIN

Realizing our vision for Blueprint Medicines

DRUG CANDIDATE (TARGET)	DISCOVERY	PRECLINICAL	PHASE 1-2	PIVOTAL	COMMERCIAL RIGHTS
avapritinib (KIT & PDGFRα)	Phase 1 NAVIGATOR – Advanced PDGFRα-driven GIST				
	Phase 1 NAVIGATOR – Advanced 3L+ (KIT-driven) GIST				
	Phase 1 NAVIGATOR – 2L (KIT-driven) GIST				
	Phase 3 VOYAGER – Advanced 3L GIST (planned 1H 2018)				
	Phase 1 EXPLORER – Advanced systemic mastocytosis (SM)				
	Phase 2 – Advanced systemic mastocytosis (planned 1H 2018)				
	Phase 2 – Indolent and smoldering systemic mastocytosis (planned 2H 2018)				
BLU-554 (FGFR4)	Phase 1 – Advanced hepatocellular carcinoma				
BLU-667 (RET)	Phase 1 ARROW – Advanced NSCLC, thyroid and other cancers[1]				
BLU-782 (ALK2)	Fibrodysplasia ossificans progressiva				
2 undisclosed kinase targets					
Immunokinase targets	Up to 5 cancer immunotherapy programs; development stage undisclosed[2]				

2L, second-line; 3L, third-line; GIST, gastrointestinal stromal tumors; NSCLC, non-small cell lung cancer; SM, systemic mastocytosis.
[1] Phase 1 trial includes a basket cohort that consists of other advanced solid tumors with RET alterations.
[2] Blueprint Medicines has U.S. commercial rights for up to two programs. Roche has worldwide commercialization rights for up to three programs and ex-U.S. commercialization rights for up to two programs.

The power and reproducibility of our scientific platform

Internally designed and wholly owned therapeutic candidates with rapid clinical proof-of-concept

Avapritinib

KIT/PDGFRA mutations

- Gastrointestinal stromal tumors
- Systemic mastocytosis

BLU-554

Activated FGFR4 pathway

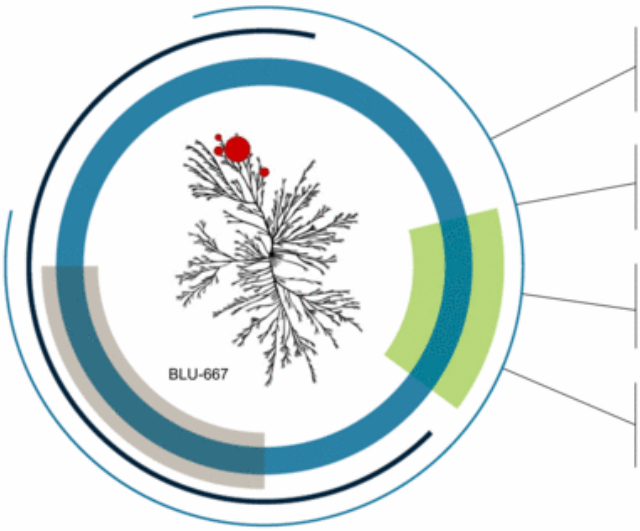
- Hepatocellular carcinoma

BLU-667

RET alterations

- Non-small cell lung cancer
- Medullary thyroid cancer
- Other solid tumors

BLU-667 is a model Blueprint Medicines program



Discovery profile focused on selective targeting of RET alterations including resistant mutants

Drug candidate designed via direct hit from Blueprint Medicines proprietary library

Expedited development through effective patient identification

Initial clinical data provide clear proof-of-concept, supporting further development

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.

ARROW trial results

Vivek Subbiah, M.D.
MD Anderson Cancer Center

Highly potent and selective RET inhibitor, BLU-667, achieves proof of concept in ARROW, a phase 1 study of advanced, RET-altered solid tumors

Vivek Subbiah[1], Matthew Taylor[2], Jessica Lin[3], Mimi Hu[1], Sai-Hong Ignatius Ou[4], Marcia S. Brose[5], Elena Garralda[6], Corinne Clifford[7], Michael Palmer[7], Meera Tugnait,[7] Erica Evans[7], Hongliang Shi[7], Beni Wolf[7], and Justin Gainor[3]

[1]Department of Investigational Cancer Therapeutics, The University of Texas MD Anderson Cancer Center, Houston, United States;
[2]The Knight Cancer Institute, Oregon Health & Science University, Portland, United States;
[3]Department of Medicine, Massachusetts General Hospital, Boston, United States,
[4]Chao Family Comprehensive Cancer Center, University of California Irvine Medical Center, United States;
[5]Abramson Cancer Center, University Of Pennsylvania, Philadelphia, United States;
[6]Vall d'Hebron Institute of Oncology, Vall d'Hebron University Hospital, Barcelona, Spain;
[7]Blueprint Medicines Corporation, Cambridge, United States;

AACR American Association for Cancer Research ANNUAL MEETING 2018 • CHICAGO APRIL 14-18

NCT03037385

Disclosures

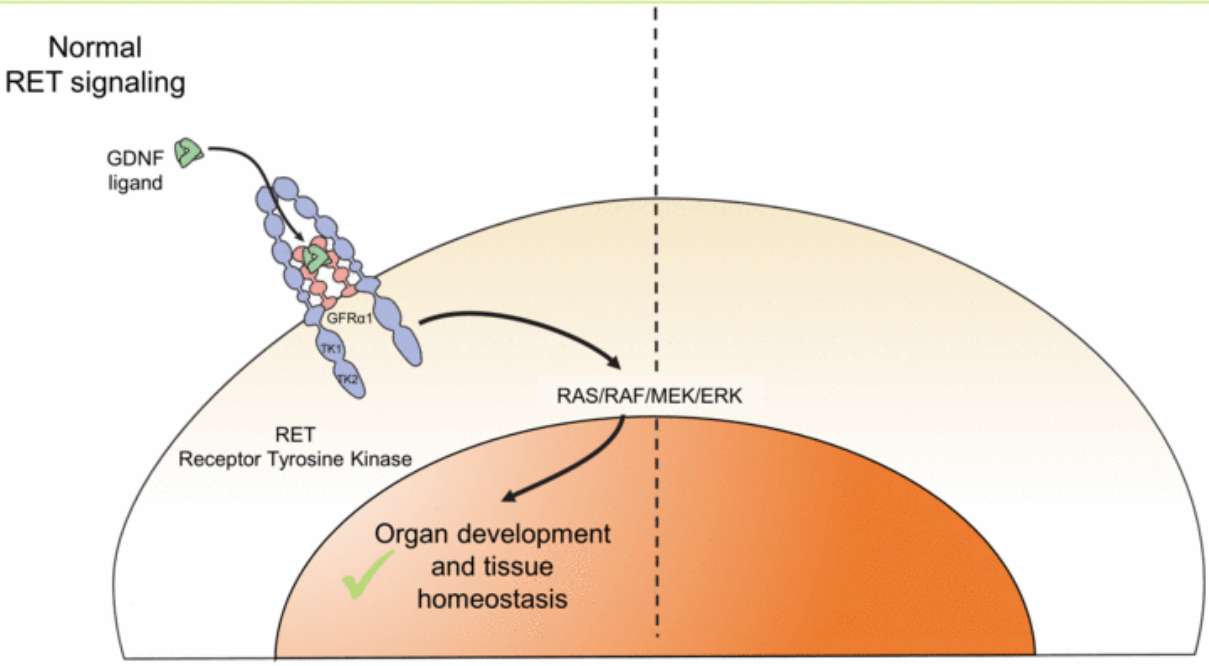
I have the following financial relationships to disclose:

Grant/Research support from:

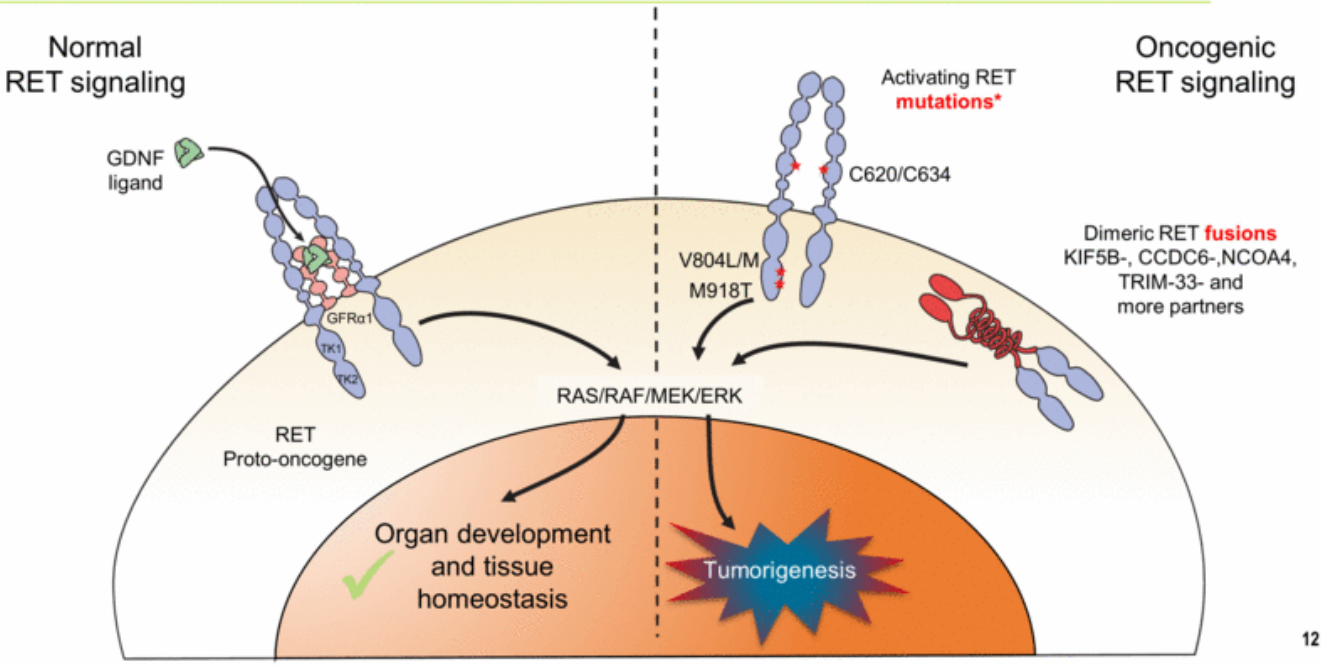
- **Blueprint Medicines Corporation**
- Novartis International AG
- Bayer AG
- GlaxoSmithKline plc
- NanoCarrier Co. Ltd
- Vegenics Pty Ltd
- Northwest Biotherapeutics
- Boston Biomedical Inc
- Berg
- Incyte Corporation
- Fujifilm Holdings Corporation
- PharmaMar
- D3
- Pfizer Inc
- MultiVir Inc
- Amgen Inc
- AbbVie Inc
- Loxo Oncology
- F. Hoffmann-La Roche AG / Genentech Inc
- National Comprehensive Cancer Network
- National Cancer Institute-Cancer Therapy Evaluation Program

BLU-667 is an investigational agent discovered and currently in development by Blueprint Medicines Corporation (Blueprint Medicines)

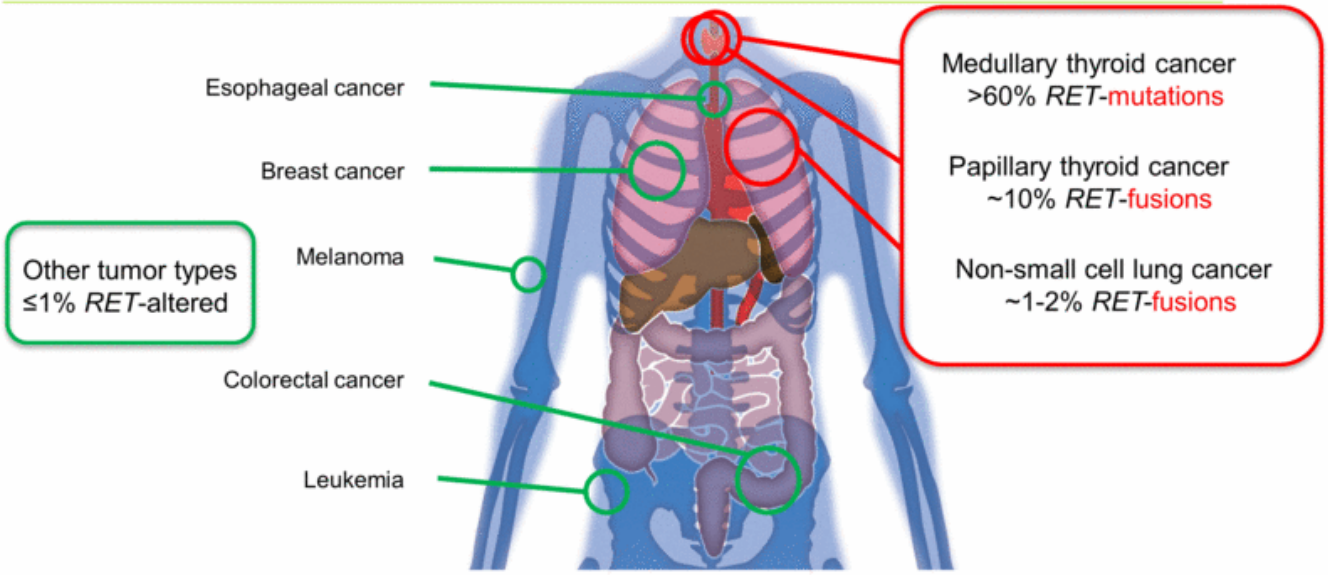
Receptor tyrosine kinase, *RE*arranged during *T*ransfection (*RET*)



Receptor tyrosine kinase, *RE*arranged during *T*ransfection (*RET*)



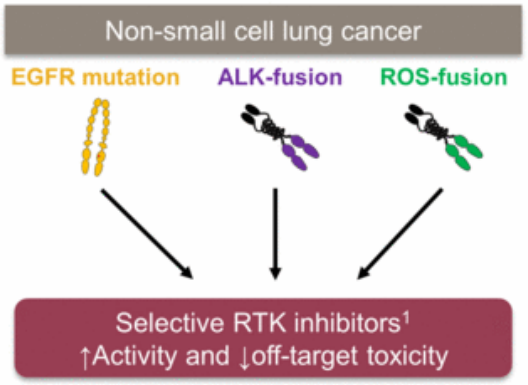
RET is a rare driver of multiple, diverse tumor types[1,2]



1. Drilon A et al. *Nat Rev Clin Oncol.* 2018;15:151-67 2.Kato S, et al. *Clin Cancer Res* 2017;23:1988-1997.

Patients with *RET*-alterations have not benefited from precision oncology

Precision oncology

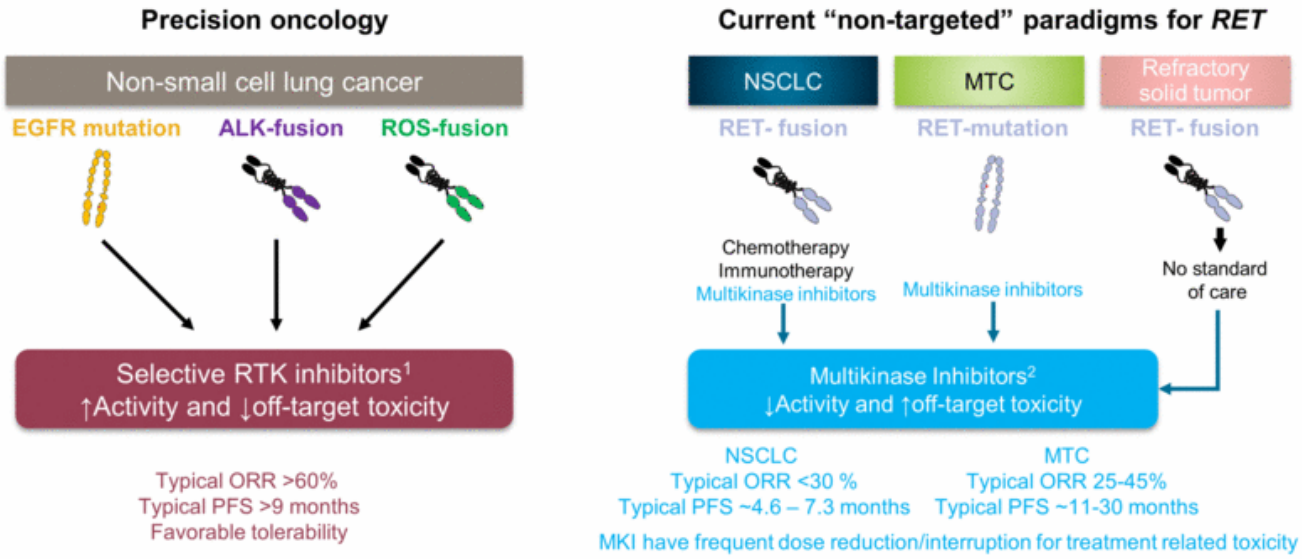


Typical ORR >60%
Typical PFS >9 months
Favorable tolerability

MKI, multikinase inhibitors; MTC, medullary thyroid cancer; NSCLC, non-small cell lung cancer; ORR, overall response rate; PFS, progression-free survival; RTK, receptor tyrosine kinase

1. Herbst RS et al. *Nature* 2018; 553:446-54; 2. Drilon A et al. *Nat Rev Clin Oncol.* 2018;15:151-67.

Patients with *RET*-alterations have not benefited from precision oncology



MKI, multikinase inhibitors; MTC, medullary thyroid cancer; NSCLC, non-small cell lung cancer; ORR, overall response rate; PFS, progression-free survival; RTK, receptor tyrosine kinase

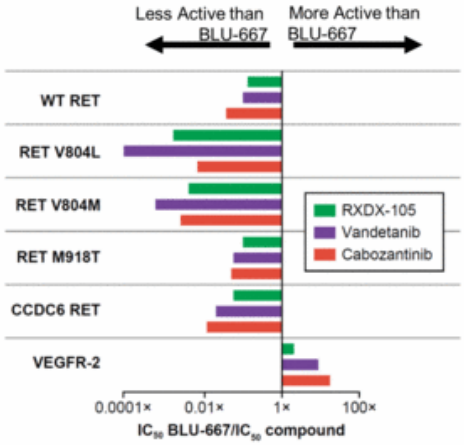
1. Herbst RS et al. *Nature* 2018; 553:446-54; 2. Drilon A et al. *Nat Rev Clin Oncol.* 2018;15:151-67.

BLU-667 was designed to treat RET-altered cancers

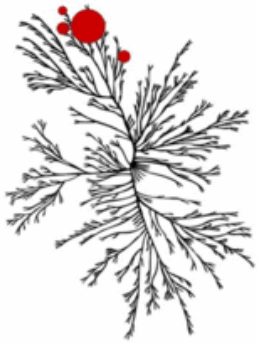
Subnanomolar potency[1]

Variant	Biochemical IC_{50} (nM)
RET wildtype	0.4
RET V804L	0.3
RET V804M	0.4
RET M918T	0.4
CCDC6-RET	0.4

More Potent than MKI

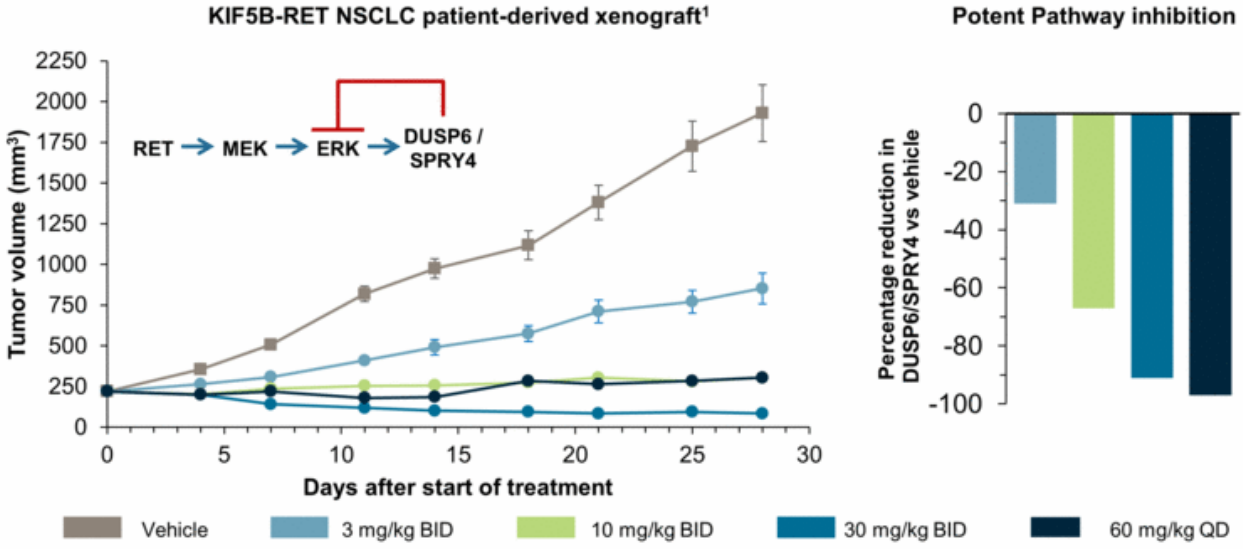


Kinome selectivity for RET



1. Subbiah V et al. *Cancer Discovery* April 15 2018.

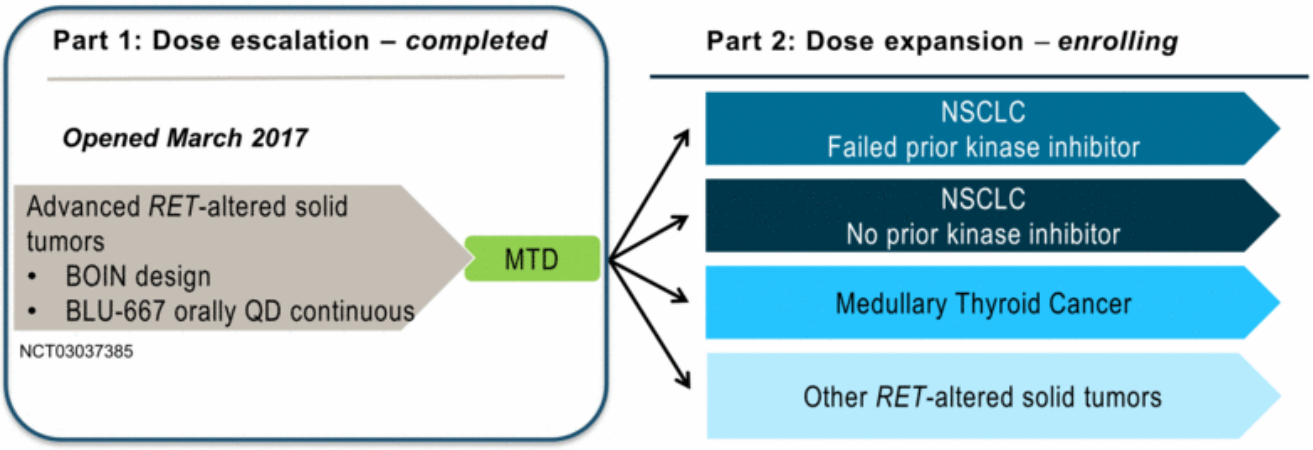
BLU-667 potently inhibits RET-driven tumor growth



BID, two times per day; QD, once daily

1. Subbiah V et al. *Cancer Discovery* April 15 2018.

BLU-667 ARROW first-in-human study



Key objectives

- MTD, safety, pharmacokinetics, pharmacodynamics, anti-tumor activity

BOIN, Bayesian optimal interval; MTD, maximum tolerated dose

Demography and baseline characteristics

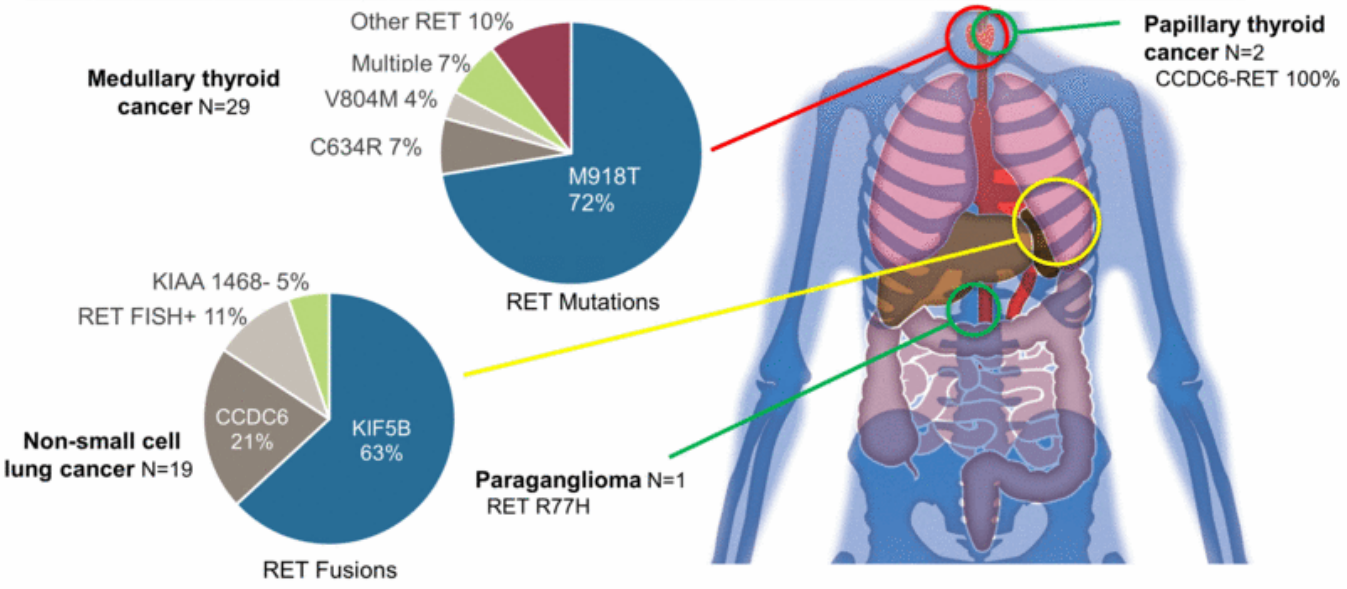
Parameter	(N=53)
Age, years; median (range)	56 (19-83)
Sex, male; n (%)	30 (57)
ECOG PS; n (%)	
0	21 (40)
1	32 (60)
Metastatic disease; n (%)	50 (94)
Tumor type; n (%)	
RET-alteration	51 (96)
Medullary thyroid cancer	29 (55)
Non-small cell lung cancer	19 (36)
Papillary thyroid cancer	2 (4)
Retroperitoneal Paraganglioma	1 (2)
Non-*RET* altered solid tumor	2 (4)

Parameter	(N=53)
Prior systemic therapy; n (%)	41 (77)
Multikinase inhibitor; n (%)	27 (51)
Chemotherapy; n (%)	19 (36)
Immunotherapy; n (%)	18 (34)
# of lines, median (range)	1 (0-8)

ECOG PS, Eastern Cooperative Oncology Group performance score

Data cut-off: April 6, 2018

Diverse *RET* genotypes enrolled



Data cut-off: April 6, 2018

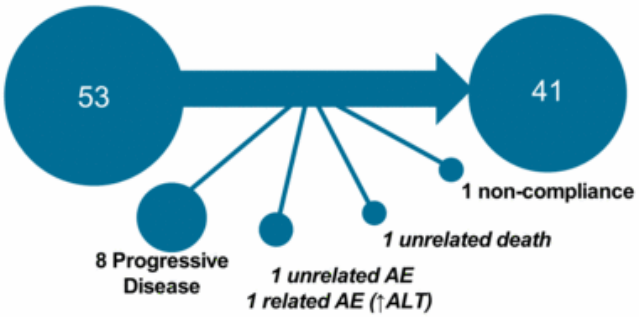
Dose escalation results

Maximum Tolerated Dose – 400 mg QD

Dose (mg QD)	# Evaluable (N=49)	Dose limiting toxicity
30	1	None
60	6	None
100	5	Alanine transaminase increased (1)
200	12	None
300	11	Tumor lysis syndrome (1) Hypertension (1)
400	10	Asthenia (1) Hypertension (1)
600	4	Hyponatremia (1) Hypertension (1)

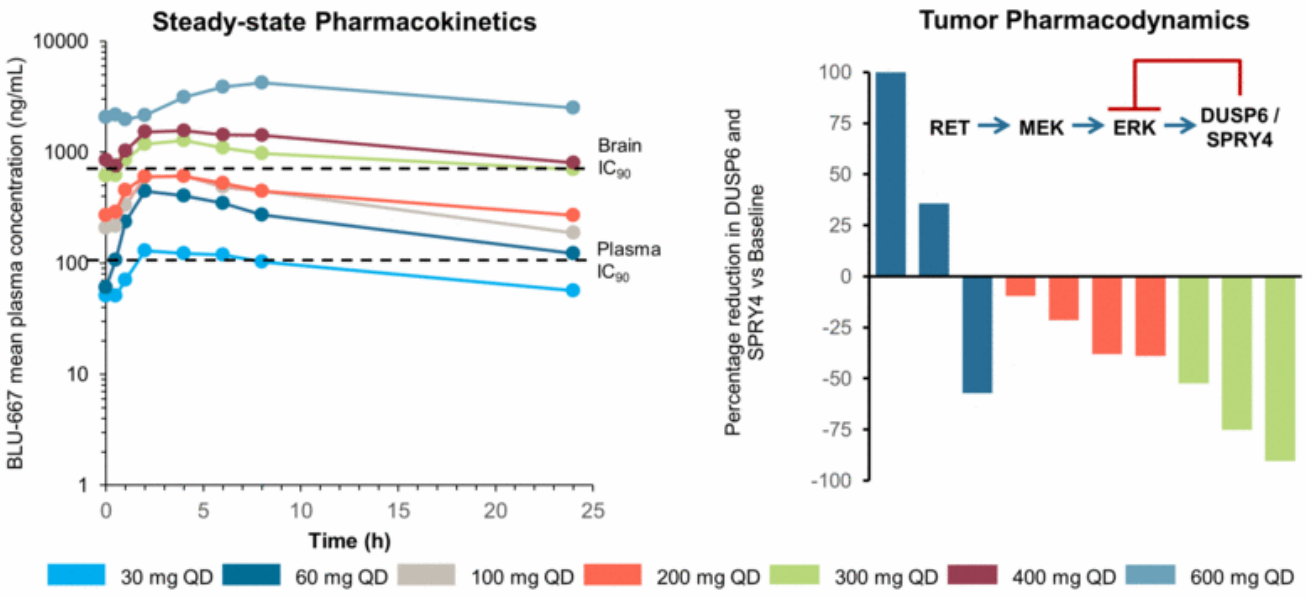
ALT, alanine aminotransferase

41 of 53 patients remain on treatment (median 3.9 months [range: 0.3–11.5])

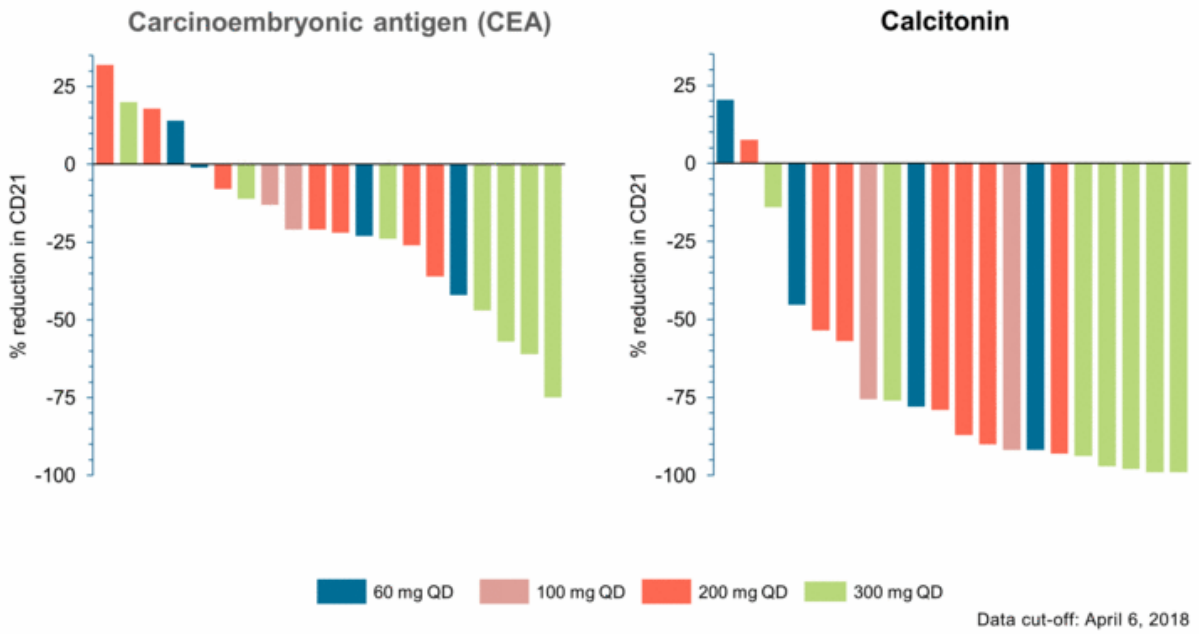


Data cut-off: April 6, 2018

Dose-dependent exposure and RET pathway inhibition

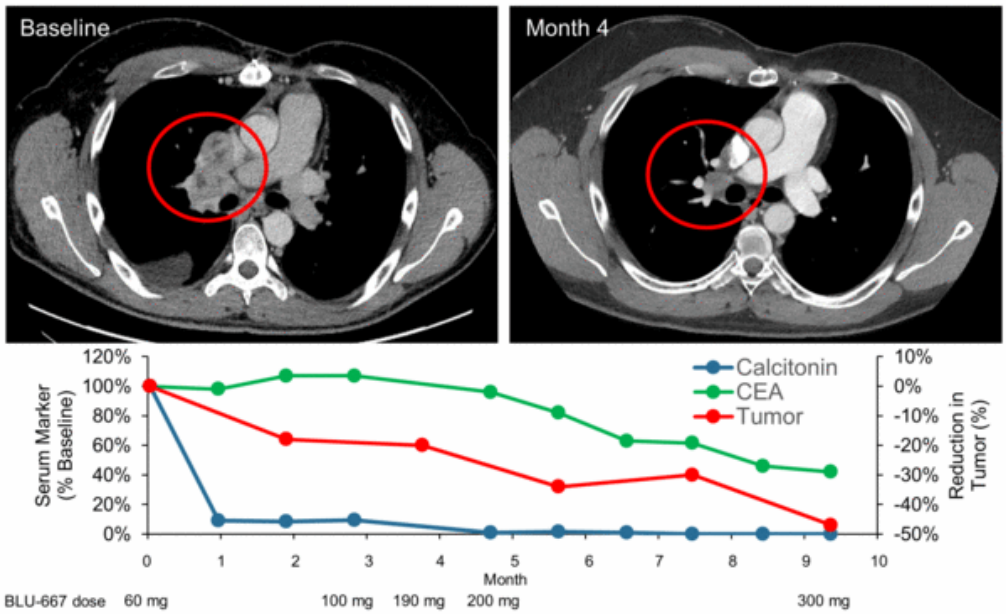


Dose-dependent decline in MTC tumor markers



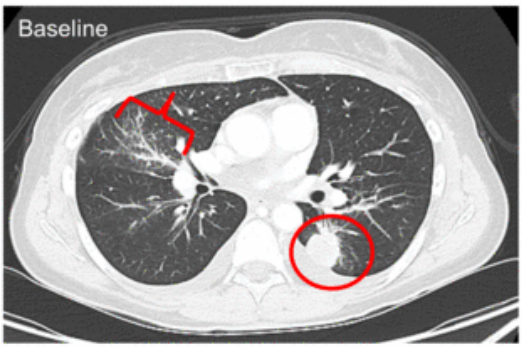
Data cut-off: April 6, 2018

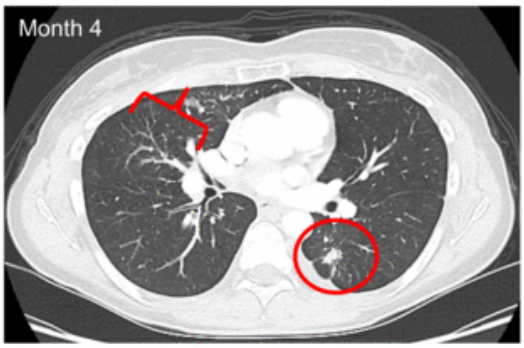
Potent activity against highly invasive *RET*-mutant MTC



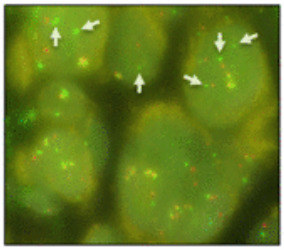
27-year-old male; *RET* L629-D361 Del; initiated at 60 mg; ongoing at 400 mg with confirmed PR

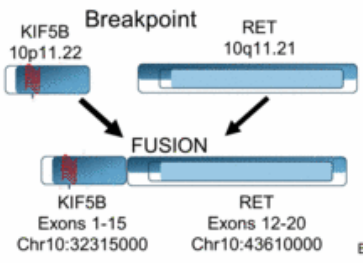
Potent activity against KIF5B-RET NSCLC – post chemotherapy

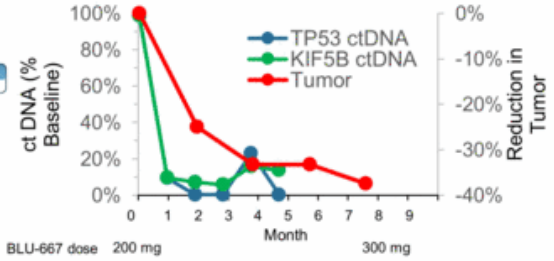




FISH







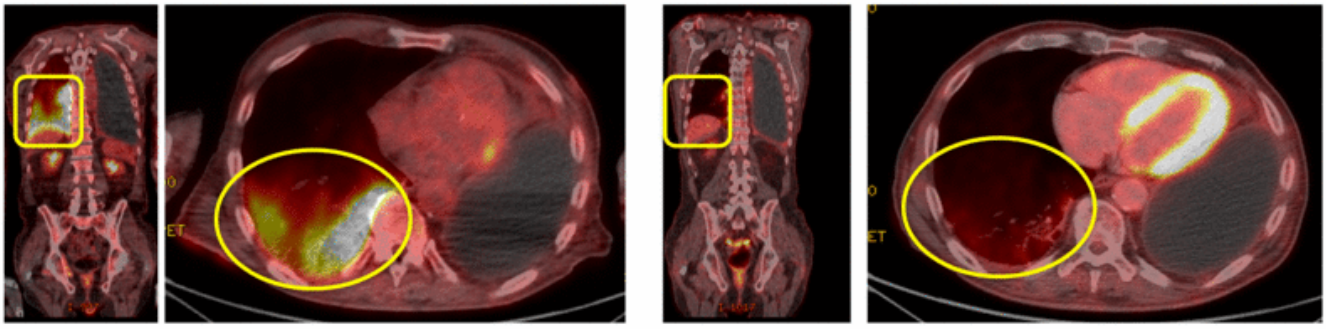
37-year-old female; ongoing at 400 mg with confirmed PR.

Subbiah V et al. *Cancer Discovery* April 15 2018.

Potent activity against KIF5B-RET NSCLC – post-vandetinib+everolimus

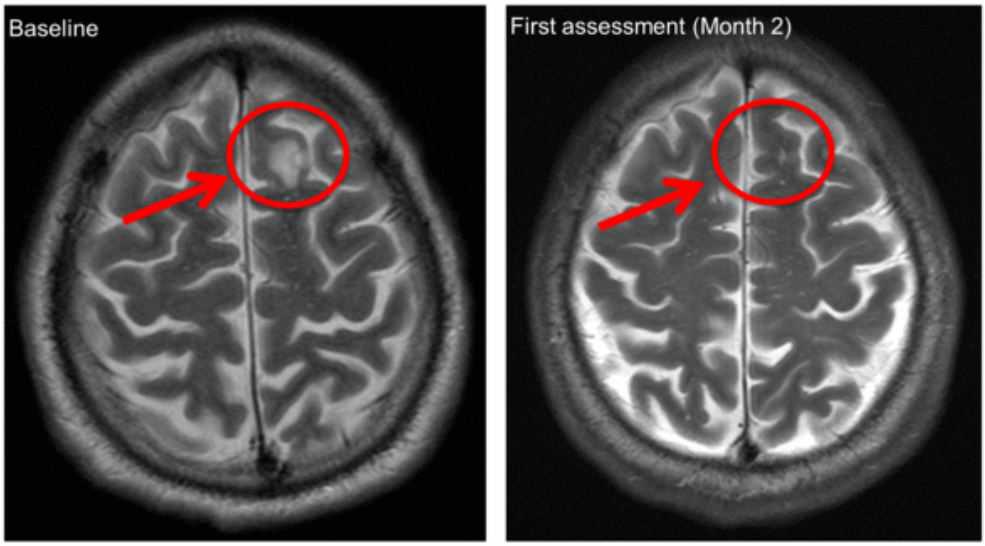
Baseline

First Assessment (Month 2)



74-year-old male; initiated at 300 mg; ongoing at 400 mg; PR at month 5 pending confirmation Subbiah V et al. *Cancer Discovery* April 15 2018.

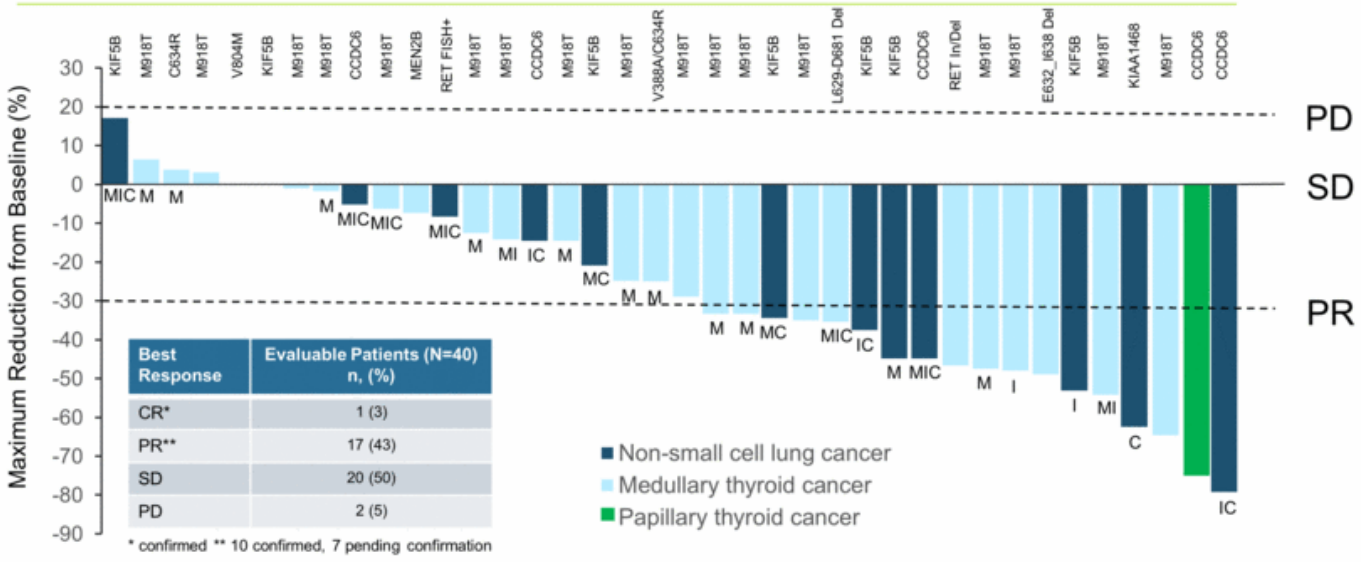
Activity against KIF5B-RET NSCLC brain metastases



69-year-old male; initiated at 400 mg; ongoing at month 4

Images courtesy of Drs of Gainor, J and Lin, J of MGH

BLU-667 has broad anti-tumor activity against RET-altered cancers



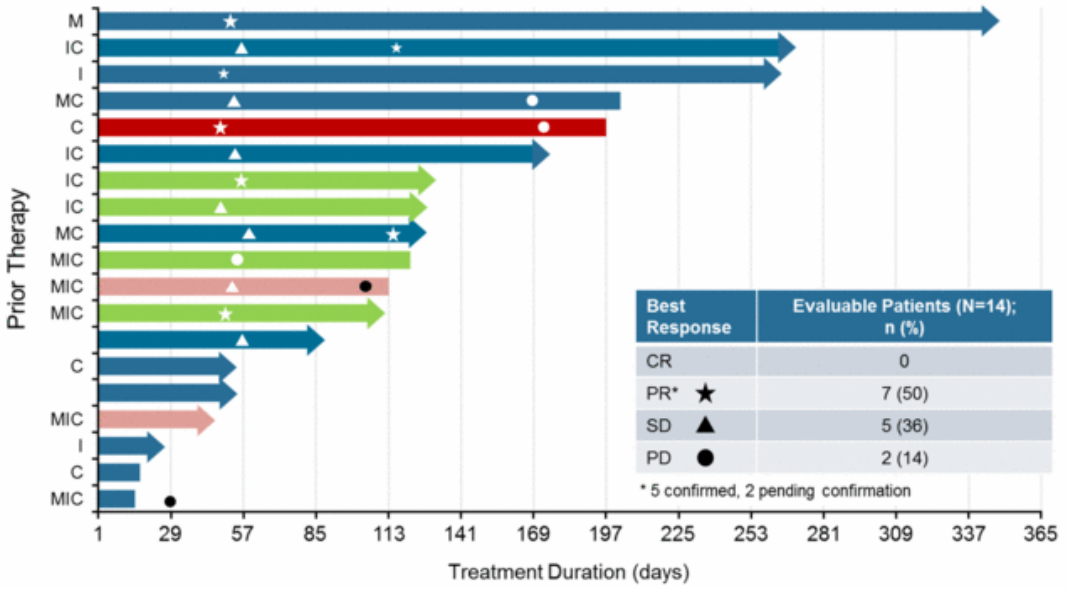
Best Response	Evaluable Patients (N=40) n, (%)
CR*	1 (3)
PR**	17 (43)
SD	20 (50)
PD	2 (5)

* confirmed ** 10 confirmed, 7 pending confirmation

C, prior chemotherapy; CR, complete response; I, prior immunotherapy; M, prior MKI therapy; MKI, multikinase inhibitor; PD, progressive disease; PR, partial response; SD, stable disease

Data cut-off: April 6, 2018

BLU-667 has durable activity and high response rate in RET-altered NSCLC



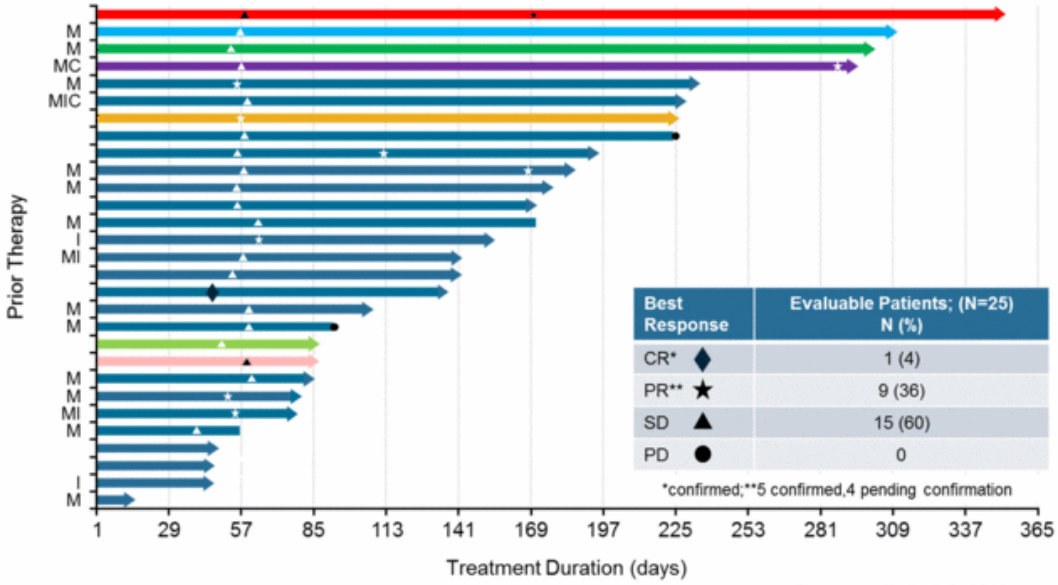
Best Response	Evaluable Patients (N=14); n (%)
CR	0
PR* ★	7 (50)
SD ▲	5 (36)
PD ●	2 (14)

* 5 confirmed, 2 pending confirmation

Treatment duration:
Median 3.9 months
Range 0.4–11.4 months
13/19 (68%) on treatment

Data cut-off: April 6, 2018

BLU-667 has durable activity and high response rate in RET-altered MTC



Best Response	Evaluable Patients; (N=25) N (%)
CR*	1 (4)
PR**	9 (36)
SD	15 (60)
PD	0

*confirmed;**5 confirmed,4 pending confirmation

L629-D361 Del
V388A/C634R
C634R
RET In/Del
M918T
E632_1638 Del
Other RET
V804M

Treatment duration:
Median 4.7 months
Range 0.5–11.5 months
25/29 (86%) on treatment

Data cut-off: April 6, 2018

BLU-667 is well tolerated

Treatment-emergent Adverse Events ≥10% per CTCAE
(30-400 mg Safety Population, N=49)

Adverse event, n (%)	Grade 1	Grade 2	Grade 3	Grade 4/5
Constipation	10 (20)	2 (4)	0	0
ALT increased	10 (20)	0	1 (2)	0
AST increased	8 (16)	2 (4)	0	0
Hypertension	2 (4)	2 (4)	4 (8)	0
Fatigue	5 (10)	1 (2)	1 (2)	0
Edema peripheral	6 (12)	1 (2)	0	0
Diarrhea	4 (8)	1 (2)	1 (2)	0
Blood creatinine increased	6 (12)	0	0	0
Hyperphosphatemia	4 (8)	2 (4)	0	0
Headache	5 (10)	1 (2)	0	0
Leukopenia	5 (10)	0	0	0
Neutropenia	2 (4)	1 (2)	2 (4)	0
White blood cell decreased	2 (4)	2 (4)	1 (2)	0
Insomnia	5 (10)	0	0	0
Cough	3 (6)	2 (4)	0	0

Most adverse events were Grade 1

8 (16%) patients had Grade 3 treatment-related AE

No Grade 4/5 treatment-related AEs

AE, adverse event; ALP, alkaline phosphatase; ALT, analine aminotransferase;
AST, aspartate aminotransferase; CTCAE, common terminology criteria for adverse events

Data cut-off: April 6, 2018

Conclusions

- BLU-667 delivers:
  - Potent RET pathway inhibition with favorable tolerability
  - Broad anti-tumor activity regardless of *RET* genotype, indication and prior therapy
  - High preliminary response rates and durable activity
    - ORR: RET-fusion NSCLC 50%
    - ORR: RET-mutant MTC 40%
    - ORR: *RET*-fusions and mutations (NSCLC, MTC and PTC) 45%
    - 41 of 51 *RET*-altered patients remain on treatment
- ARROW dose escalation data validate BLU-667 as a promising precision therapy for *RET*-altered cancers
- ARROW dose expansion is open and enrolling globally
- BLU-667 manuscript published today in Cancer Discovery
  - Foundational preclinical work and clinical translation

Data cut-off: April 6, 2018

Acknowledgements

We thank the participating patients, their families, all study co-investigators, and research co-ordinators at the following institutions:

- Department of Investigational Cancer Therapeutics, The University of Texas MD Anderson Cancer Center, Houston, United States
- The Knight Cancer Institute Oregon Health & Science University Portland, United States
- Department of Medicine, Massachusetts General Hospital Cancer Center, Boston, United States
- Chao Family Comprehensive Cancer Center University of California Irvine Medical Center, United States
- Abramson Cancer Center, University Of Pennsylvania, United States
- Vall d'Hebron Institute of Oncology Vall d'Hebron University Hospital, Barcelona, Spain

BLU-667 program vision

Ben Wolf, M.D., Ph.D.

VP of Clinical Development

blueprint™ MEDICINES

Strategy to rapidly bring transformative kinase medicines to patients

GENOMIC DRIVER OF DISEASE ❯ **HIGHLY SELECTIVE KINASE MEDICINE** ❯ **SELECTED PATIENT POPULATION** ❯ **TARGET OUTCOMES**

Proprietary library of kinome-annotated compounds

- Rapid clinical proof-of-concept
- Early go/no-go decisions
- Expedited development & regulatory approval
- Clear commercial value proposition

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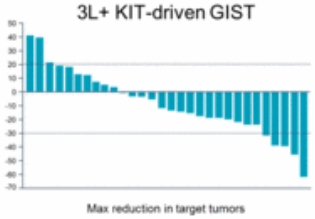
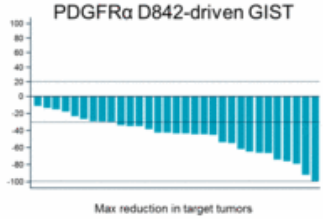
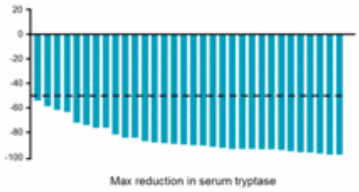
Realizing the promise: five compelling proof-of-concept datasets

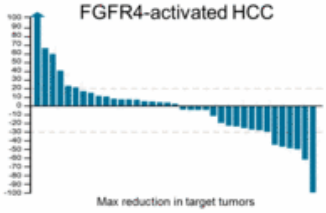
avapritinib



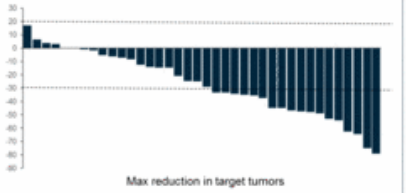




BLU-554



BLU-667



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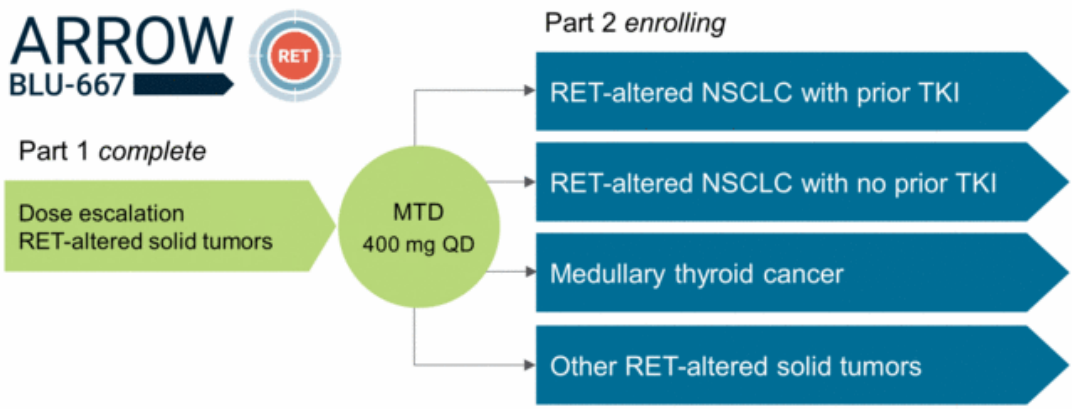
Avapritinib GIST data presented at November 2017 CTOS Annual Meeting. Data cutoff: October 11, 2017; Avapritinib systemic mastocytosis data presented at December 2017 ASH Annual Meeting. Data cutoff: October 4, 2017; BLU-554 data presented at September 2017 ESMO Congress. Data cutoff: August 18, 2017; BLU-667 data presented at April 2018 AACR Annual Meeting. Data cutoff: April 6, 2018. 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; HCC, hepatocellular carcinoma; 3L+, third-line or later treatment.

Significant opportunity for selective RET inhibitor, anchored by NSCLC

	RET opportunity in major geographies[1]	Regulatory precedent
Non-small cell lung cancer	~10,000 patients[2]	Single-arm trial *EGFR and ALK inhibitors*
Medullary thyroid cancer	~800 patients[2]	Randomized controlled trial *Vandetinib and cabozantinib (first-line)*
Tumor agnostic	Under evaluation *Frequency of RET alterations vary across multiple solid tumors: papillary thyroid, colorectal, breast, melanoma, esophageal and others*	Emerging precedent *Pembrolizumab (MSI-H or dMMR cancers)*

[1] Major geographies include United States, France, Germany, Italy, Spain, the United Kingdom and Japan.
[2] Epidemiology based on estimated incidence for NSCLC and estimated prevalence for MTC.

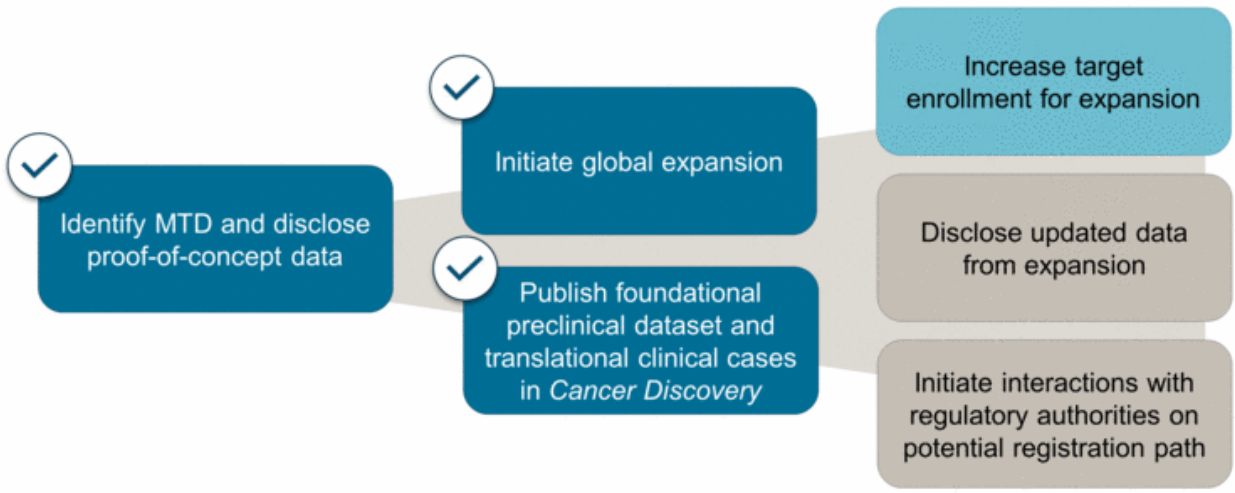
Expansion portion of ARROW trial initiated and enrolling globally



Key Part 2 objectives: evaluate efficacy (ORR and DOR) and safety at the MTD

DOR, duration of response; MTD, maximum tolerated dose; ORR, overall response rate; TKI, tyrosine kinase inhibitor

Promising early clinical activity supports rapid development of BLU-667



Questions & Answers

Closing Remarks

Jeff Albers

Chief Executive Officer

Upcoming anticipated milestones

Avapritinib	• Complete enrollment of registration-enabling trial in PDGFRα-driven GIST by mid-2018 • Engage global regulatory authorities on potential registration pathways in GIST and SM in 1H 2018 • Initiate registration-enabling trials in 3L GIST and advanced SM in 1H 2018 • Initiate Phase 2 trial in indolent and smoldering SM in 2H 2018 • Present updated Phase 1 data across multiple GIST and SM patient populations in 2018
Portfolio	• Present data from Phase 1 trial of BLU-667 in RET-altered cancers and initiate expansion in 1H 2018 • Initiate TKI-naïve cohort in Phase 1 trial of BLU-554 in HCC in 1Q 2018 • Present data from expansion and TKI-naïve cohort of BLU-554 in HCC in 2H 2018
Research	• Initiate IND-enabling studies for BLU-782 in fibrodysplasia ossificans progressiva (FOP) in 1H 2018 • Present preclinical data supporting development of BLU-782 in FOP in 2018 • Nominate at least 2 additional discovery programs in 2018

Realizing our vision for Blueprint Medicines

Deliver transformational genomically targeted medicines to patients

2014 – 2015

- STRATEGY
- LIBRARY
- TEAM

2016 – 2017

3 CLINICAL CANDIDATES WITH PROOF-OF-CONCEPT

2018 – 2019

- **4** CLINICAL CANDIDATES
- **6+** TUMOR TYPES & RARE DISEASES
- **2** TUMOR AGNOSTIC CLINICAL TRIALS
- **~9** RESEARCH PROGRAMS*

blueprint MEDICINES

* Includes up to 5 programs under the cancer immunotherapy collaboration with Roche.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Thank you