# UNITED STATES SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 8-K
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CURRENT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934

Date of Report (Date of Earliest Event Reported): January 11, 2016

# **Blueprint Medicines Corporation**

(Exact name of registrant as specified in its charter)

**Delaware** (State or other jurisdiction of incorporation)

001-37359

(Commission File Number)

26-3632015 (I.R.S. Employer Identification No.)

38 Sidney Street, Suite 200
Cambridge, Massachusetts
(Address of principal executive offices)

**02139** (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))

#### Item 7.01. Regulation FD Disclosure.

Blueprint Medicines Corporation (the "Company") from time to time presents and/or distributes to the investment community at various industry and other conferences slide presentations to provide updates and summaries of its business. The Company is posting to the "Investors" portion of its website at http://ir.blueprintmedicines.com/ a copy of its current corporate slide presentation. These slides are attached to this Current Report on Form 8-K as Exhibit 99.1. The Company undertakes no obligation to update, supplement or amend the materials attached hereto as Exhibit 99.1.

The information in Item 7.01 of this Current Report on Form 8-K, including Exhibit 99.1 attached hereto, 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 Exhibit
--------------------------------------------

(d) Exhibits.

xhibit No. Description

#### **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: January 11, 2016

By: /s/ Michael Landsittel

Michael Landsittel Senior Director of Finance

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#### EXHIBIT INDEX

Exhibit No.

99.1 Description

Corporate slide presentation of Blueprint Medicines Corporation dated January 11, 2016

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# Crafting Highly Selective Kinase Medicines for Patients With Genomically Defined Diseases

January 11, 2016

### 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," "project," "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 clinical development of BLU-554 and BLU-285; the potential benefits of BLU-554 in treating patients with hepatocellular carcinoma; the potential benefits of BLU-285 in treating patients with gastrointostinal stromal tumors and systemic mastocytosis; the potential of RET and NTRK fusions and their producted resistant mutants as therapeutic targets; the potential of PRKACA fusions as therapeutic targets; the potential of kinases as therapeutic targets in tumor immunity; the timing of clinical data or proof of concept for preclinical and clinical programs; the timing of regulatory submissions or filings, including, without limitation, an investigational new drug application for BLU-667; plans and timelines for the development of companion diagnostics for BLU-554 and BLU-285, plans and timelines for additional discovery programs; the future financial performance of Blueprint Medicines Corporation (the "Company"); 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 are reasonable, such floward-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 product candidates, including BLU-285 and BLU-534; the Company's ability to successfully demonstrate the efficacy and safety of its drug product candidates, including BLU-285 and BLU-534; the Company's ability to successfully demonstrate the efficacy and safety of its drug product can

These and other risks and uncertainties are described in greater detail under "Risk Factors" in the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2015, as filed with the Securities and Exchange Commission ("SEC") on November 9, 2015, and any other filings the Company 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.

# Crafting Highly Selective Kinase Medicines for Patients With Genomically Defined Diseases



#### Novel Selective Kinase Platform

- Systematic, reproducible approach to identify kinase drivers of disease and craft drug candidates
- Integrated new target discovery engine and proprietary compound library
- Demonstrated productivity including Alexion collaboration

# Two Lead Programs: 3 Clinical Trials

- Three Phase I trials
  - BLU-285 for gastrointestinal stromal tumor (GIST): Phase 1 enrolling
  - BLU-285 for advanced systemic mastocytosis (SM): IND accepted, clinical sites opening
  - BLU-554 for hepatocellular carcinoma (HCC): Phase 1 enrolling

### Near-Term Milestones

- Initial data potential by end of 2016 from 3 Phase 1 trials
- IND filing for BLU-667 (RET fusions / resistant mutants) anticipated by end of 2016
- Nomination of additional discovery programs with rapid development potential through enriched clinical trials

### Large Opportunity

- >\$17bn in small molecule kinase drug sales in 2014
- Continued platform potential to generate new programs in 2016 and beyond

### Highly Experienced Team

- Deep expertise in oncology and rare genetic diseases
- Successful track records in discovery and development of drugs to genomic drivers of disease

# Blueprint Medicines is Positioned to Build a Sustainable Business



- ✓ Untapped kinase opportunity in oncology, rare genetic diseases and beyond
- ✓ Focused on kinase-driven diseases with high medical need
- ✓ Potential for transformative impact in genomically defined patient populations

### **Prolific Discovery Engine**

- 1 IND per year on average
- 6 programs; plan to nominate
   2 more in 2016
- Expertise to mine potential of Kinases of Unknown Biology
- Strategic collaboration opportunities

### **Clinical Stage Pipeline**

2016 expected milestones:

- BLU-285 POC in GIST
- BLU-285 POC in SM
- BLU-554 POC in HCC
- Develop CDx's for patient selection
- BLU-667 RET IND filing

### Poised to Build a Fully Integrated Business

- Potential for rapid clinical path in defined patient groups
- Multiple wholly-owned assets enabling a staged approach to potential commercialization in as little as 5 years

Strong financial position with cash through at least early 2017

# Led by a Team of Industry Innovators With Deep Expertise and Track Records of Success



# **Executive Management**



Jeff Albers Chief Executive Officer and President



Andy Boral, M.D., Ph.D. Chief Medical Officer



Kate Haviland Chief Business Officer



Christoph Lengauer, Ph.D. Chief Scientific Officer



















# Raised \$169M Gross in 2015 IPO (NASDAQ: BPMC)

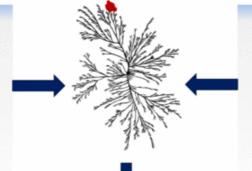


# Platform Integrates Innovative Target Discovery Engine and Proprietary Compound Library to Yield Novel Drug Candidates



### **Compound Library**

- Novel chemical matter
- Broad, diverse kinome coverage
- Annotated for rapid prosecution of medicinal chemistry
- Precisely tailored target product profiles



# **Target Discovery Engine**

- Proprietary genomic analyses algorithms
- Precision functional genomics
- Specific emphasis on kinases of unknown biology (KUBs)
- Proven expertise in predicting drug resistance

Novel kinase targets of disease
Highly selective kinase medicines
Unique chemistry for difficult to drug targets

Gnome illustration reproduced courtesy of CSTI (www.cellsignal.com). The foregoing website is maintained by CSTI, and Blueprint Medicines is not responsible for its content.

# Diverse Portfolio With Clinical Stage Assets



	Initial Indications	Stage of Development	Commercial Rights
BLU-285 (KIT Exon 17 Mutants	GIST	Phase 1 Enrolling	
and PDGFRα D842V Inhibitor)	SM	IND Accepted	Opinebulg
BLU-554 (FGFR4 Inhibitor)	HCC	Phase 1 Enrolling	<b>Solueprint</b>
BLU-667 (RET Fusions & Resistant Mutants)	NSCLC, thyroid	Pre-clinical	Solveprint
Rare Genetic Disease Target	Rare genetic disease	Undisclosed	ALEXION

Ongoing discovery: NTRK fusions / resistant mutants & undisclosed 6<sup>th</sup> program Platform Potential: 1 IND per Year on Average

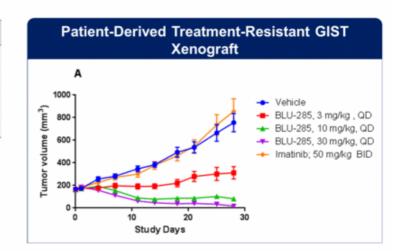
# BLU-285 is a Selective and Potent Inhibitor of Exon 17 Mutant KIT and D842V Mutant PDGFRα in GIST Models



#### **Gastrointestinal Stromal Tumor**

Advanced GIST	Details	Patients*
PDGFRα D842V	5-6%	500
Relapsed/ Refractory GIST	2L and beyond	12,000
1L GIST	1L	8,000

- Sarcoma of the digestive tract
- Overall survival of ~5 years
- Imatinib and other kinase inhibitors do not provide a cure, as resistance mutations in KIT Exon 17 ultimately arise
- No therapy addresses PDGFRα D842 mutations



\*GIST patient estimates represent incidence in US, EU5 and Japan

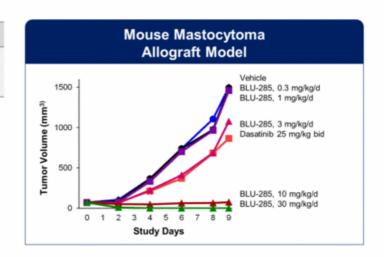
# BLU-285 is a Selective and Potent Inhibitor of D816V Mutant KIT in SM Models



### **Systemic Mastocytosis**

SM Subtypes	Disease Burden	Patients*
Advanced SM	≥94% with KIT	4,500
Indolent SM	D816V mutation	16,000

- Mast cell disorder
- Patients experience severe allergy symptoms
- Advanced SM patients suffer from organ dysfunction due to mast cell infiltration – overall survival of 3 – 5 years
- No therapy addresses the underlying disease biology



SM patient estimates represent prevalence in these markets.

# BLU-285: Two Phase 1 Clinical Studies Underway



2015 **Enrolling** 

2017 2016

- GIST Expansion **GIST Dose Escalation**
- 3+3 design: ~25 patients
- Starting dose: 30 mg / day
- Retrospective KIT Exon 17 and PDGFRa testing
- Two groups of patients
  - KIT Exon 17 mutations
  - PDGFRα D842V mutation
- ~35 patients

GIST Key Endpoints		
Primary	<ul><li>Safety</li><li>MTD</li></ul>	
Secondary	<ul><li>Response rate</li><li>Pharmacokinetics</li><li>Allele burden</li></ul>	

2015

Sites opening

2016

2017

## **SM Dose Escalation**

- 3+3 design: ~25 patients
- Starting dose: 30 mg / day
- Retrospective KIT D816V testing

# **SM Expansion**

- Three groups of patients
  - Aggressive SM
  - Aggressive SM with another hematologic disorder
  - Mast cell leukemia
- ~35 patients

### SM Key Endpoints

**Primary** 

- Safety
- MTD

Secondary

- Response rate
- Serum tryptase
- Allele burden
- Symptoms

Potential for POC by end of 2016

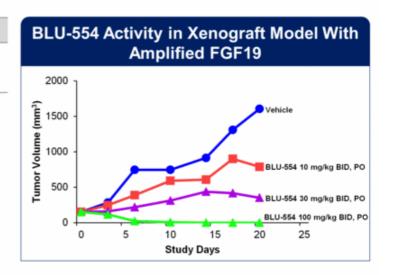
# BLU-554 is a Selective and Potent Inhibitor of FGFR4 in HCC models



### **Hepatocellular Carcinoma**

Advanced HCC Subsets	Patients*
1L with FGFR4 activation	18,000
2L with FGFR4 activation	6,000

- Liver cancer, most often HCC, is 2<sup>nd</sup> leading cause of cancer death worldwide
- Sorafenib only approved drug, no approved 2<sup>nd</sup> line therapy
- Up to 30% of HCC patients have abnormally active FGFR4 pathway, a validated driver
- No genomically targeted therapies available



Represents prevalence in key markets including US, EU5 and Japan

# BLU-554: Phase 1 Clinical Study Underway



2015 Enrolling 2016 2017

# **HCC Dose Escalation**

- 3+3 design: ~25 patients
- Starting dose: 140 mg / day
- Retrospective FGF19 expression and amplification testing

# **Expansion**

- Patients selected for FGF19 expression by IHC
  - ~25 HCC patients
  - ~10 cholangiocarcinoma patients

# Key Endpoints

### **Primary**

Secondary

- Safety
- MTD

#### -----

- Response rate
- Pharmacokinetics
- Alpha fetoproteinFGF19 in blood
- CYP7A1 RNA in tumor

Potential for POC by end of 2016

# BLU-667 is a Selective and Potent Inhibitor of RET in Non-Clinical Development for RET-driven Tumors



#### **RET Initial Indications**

Disease	Frequency*	Patients**
Lung Adenocarcinoma	1-2%	2,500
Medullary Thyroid Cancer	60%	400
Papillary Thyroid Cancer	10%	500

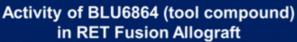
# Approach Differentiated from Multi-kinase **Inhibitors With RET Activity**

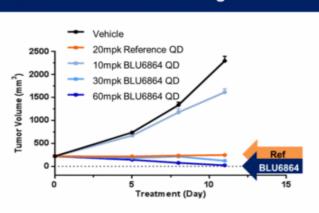
- Robust activity against wild-type RET as well as known and predicted resistance mutations
- Tumor regression in animal models driven by **RET** fusion proteins
- Initiate 28-day GLP toxicology 1H 2016 with IND filing anticipated by end of 2016

<sup>&</sup>quot;Represents estimated frequency of RET fusion for Lung and PTC and estimated frequency of activating point mutations for MTC 
\*\*Represents estimated incidence in US, EU5 and Japan.

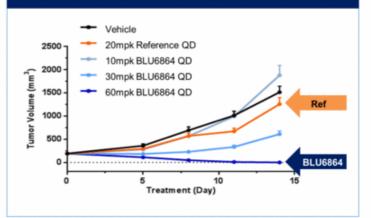
# RET Program: Potent In Vivo Activity in RET and Predicted Resistance Mutants







# Activity of BLU6864 (tool compound) in RET V804L Mutant Fusion Allograft



# Alexion Collaboration Leverages Blueprint Platform in Rare Genetic Diseases



### Collaboration Highlights

- Objective is to develop the first kinase inhibitor specifically designed for a rare and devastating genetic disease
- Blueprint Medicines is responsible for:
  - Identification & optimization of drug candidates
  - Biomarker discovery
  - All pre-IND activities
- Alexion is responsible for:
  - Clinical development
  - Commercialization
- >\$250M in potential milestones

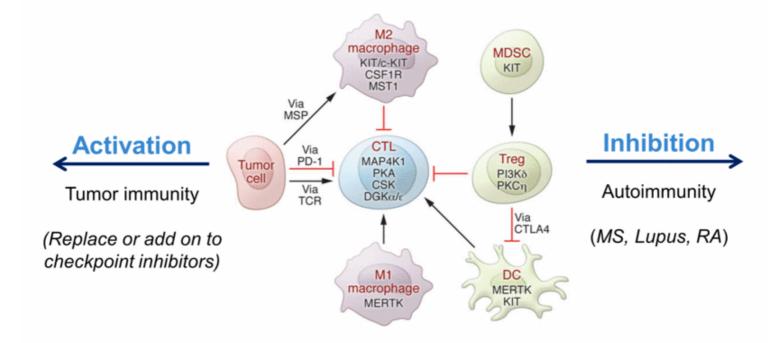




# Immunokinase Opportunity

J Clin Invest. (2015) 125:1780-9.





Kinases are critically involved in all aspects of immune system signaling

# Blueprint Medicines Achieved 2015 Goals



Clinical Development	<ul> <li>BLU-285 GIST IND accepted; Phase I trial enrolling patients in dose escalation</li> <li>BLU-285 SM IND accepted; Phase 1 clinical sites opening</li> <li>BLU-554 HCC IND accepted; Phase I trial enrolling patients in dose escalation</li> <li>Received orphan drug designation for BLU-554 in HCC</li> </ul>
Discovery	<ul> <li>Selected development candidates for RET and NTRK</li> <li>Initiated undisclosed 6<sup>th</sup> discovery program</li> <li>Presented at five major medical conferences and published two peer-reviewed publications</li> </ul>
Corporate	<ul> <li>Raised \$169M gross in upsized IPO</li> <li>Announced collaboration with Alexion for rare genetic disease program         <ul> <li>Received \$15M upfront payment and achieved first preclinical milestones</li> </ul> </li> <li>Continued to attract top discovery, clinical and business talent</li> </ul>

# Blueprint Medicines (BPMC): Entering 2016 in a Position of Financial Strength



Financial Overview*		Ownership Summary*
Market Cap	~\$580M	5% 3%
Shares Outstanding - Basic - Fully Diluted	27.1M 29.1M	21% ■ Top 10 holders
Outstanding Debt	\$7.9M	■ Other Institutional ■ Retail/Other ■ Insider
Cash Position	\$179.8M	

Existing cash balance expected to be sufficient to fund operations and capital expenditures through at least early 2017

"As of 9/30/2015, with the exception of the Market Cap, which is as of 1/6/201

# 2016 Goals: Advance 3 Trials to Clinical POC While Continuing to Progress Diversified Discovery Portfolio



Clinical Development	<ul> <li>Report preliminary data from three ongoing trials</li> <li>BLU-285 in GIST</li> <li>BLU-285 in SM</li> <li>BLU-554 in HCC</li> </ul>
	<ul> <li>Finalize companion diagnostic development for BLU-285 and BLU-554 programs</li> </ul>
	Advance RET BLU-667 to IND filing
Discovery	Disclose 6 <sup>th</sup> discovery program
	<ul> <li>Internally nominate 2 new discovery programs including at least one in immuno-oncology</li> </ul>
Corporate	<ul> <li>Maintain financial strength with cash for clinical proof of concept for three lead programs</li> </ul>
	Continue to explore strategic collaborations that allow us to maximize the value of our platform