

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**

Washington, D.C. 20549

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**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): **April 28, 2020**

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**Blueprint Medicines Corporation**

(Exact name of registrant as specified in its charter)

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

**001-37359**  
(Commission File Number)

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

**45 Sidney Street**  
**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)

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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:

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

Securities registered pursuant to Section 12(b) of the Exchange Act:

Title of each class	Trading symbol(s)	Name of each exchange on which registered
Common stock, par value \$0.001 per share	BPMC	Nasdaq Global Select Market

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**Item 8.01 Other Events.**

On April 28, 2020, Blueprint Medicines Corporation hosted an investor call and live webcast to discuss the top-line data from its Phase 3 VOYAGER clinical trial of avapritinib versus regorafenib in patients with locally advanced unresectable or metastatic gastrointestinal stromal tumor. A copy of the presentation from the investor call is filed herewith as Exhibit 99.1 to this Current Report on Form 8-K and incorporated herein by reference.

**Item 9.01 Financial Statements and Exhibits.**

(d) Exhibits.

<b>Exhibit No.</b>	<b>Description</b>
99.1	<a href="#">Presentation by Blueprint Medicines Corporation at investor call on April 28, 2020</a>
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

**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 28, 2020

By: /s/ Jeffrey W. Albers  
Jeffrey W. Albers  
Chief Executive Officer



# VOYAGER clinical trial top-line results

CONFERENCE CALL

APRIL 28, 2020

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# Forward-looking statements

This presentation contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended. The words "aim," "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. In this presentation, forward-looking statements include, without limitation, statements regarding the plans, strategies, timelines and expectations of Blueprint Medicines Corporation (the "Company") for the preclinical and clinical development and commercialization of AYWAKIT™ (avapritinib) and pralsetinib; plans and timelines for submitting marketing applications for avapritinib and pralsetinib; the potential benefits of any of the Company's current or future approved drugs or drug candidates in treating patients; plans to discontinue further development of avapritinib for GIST indications other than PDGFRA exon 18 mutant GIST; expectations regarding the Company's existing cash, cash equivalents and investments; and the Company's strategy, goals and anticipated milestones, business plans and focus.

The Company has based these forward-looking statements on management's current expectations, assumptions, estimates and projections. While the Company believes these expectations, assumptions, estimates and projections are reasonable, such forward-looking statements are only predictions and involve known and unknown risks, uncertainties and other important factors, many of which are beyond the Company's control and may cause actual results, performance or achievements to differ materially from those expressed or implied by any forward-looking statements. These risks and uncertainties include, without limitation, risks and uncertainties related to the impact of the COVID-19 pandemic to the Company's business, operations, strategy, goals and anticipated milestones, including the Company's ongoing and planned research and discovery activities, ability to conduct ongoing and planned clinical trials, clinical supply of current or future drug candidates, commercial supply of current or future approved drugs, and launching, marketing and selling current or future approved drugs; the delay of any current or planned clinical trials or the development of the Company's drug candidates, including avapritinib for additional indications, pralsetinib, fisogatinib and BLU-263, or the licensed drug candidate; the Company's advancement of multiple early-stage efforts; the Company's ability to successfully demonstrate the efficacy and safety of its drug candidates and gain approval of its drug candidates on a timely basis, if at all; the preclinical and clinical results for the Company's drug candidates, which may not support further development of such drug candidates; actions or decisions of regulatory agencies or authorities, which may affect the initiation, timing and progress of clinical trials or marketing applications; the Company's ability to obtain, maintain and enforce patent and other intellectual property protection for any drug candidates it is developing or AYWAKIT; the Company's ability and plans for maintaining a commercial infrastructure, and successfully launching, marketing and selling its current or future approved drugs; the Company's ability to successfully expand the approved indications for AYWAKIT, including the Company's ability to obtain U.S. Food and Drug Administration approval for its pending new drug application for avapritinib for the treatment of adults with unresectable or metastatic fourth-line GIST, or obtain marketing approval for AYWAKIT in additional geographies; the Company's ability to develop and commercialize companion diagnostic tests for any of the Company's current or future approved drugs or drug candidates; and the success of the Company's current and future collaborations, partnerships and licenses.

These and other risks and uncertainties are described in greater detail under "Risk Factors" in the Company's filings with the Securities and Exchange Commission ("SEC"), including its most recent Annual Report on Form 10-K and any other filings it has made or may make with the SEC in the future. The Company cannot guarantee future results, outcomes, levels of activity, performance, developments, or achievements, and there can be no assurance that its expectations, intentions, anticipations, beliefs, or projections will result or be achieved or accomplished. The forward-looking statements in this presentation are made only as of the date hereof, and except as required by law, the Company undertakes no obligation to update any forward-looking statements contained in this presentation as a result of new information, future events or otherwise.

This presentation also contains estimates, projections and other statistical data made by independent parties and by the Company relating to market size and growth and other data about the Company's industry. These data involve a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. In addition, projections, assumptions and estimates of the Company's future performance and the future performance of the markets in which the Company operates are necessarily subject to a high degree of uncertainty and risk.



# VOYAGER trial top-line results conference call

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**Introductory remarks**

**Jeff Albers**, Chief Executive Officer

**Summary of top-line VOYAGER trial data**

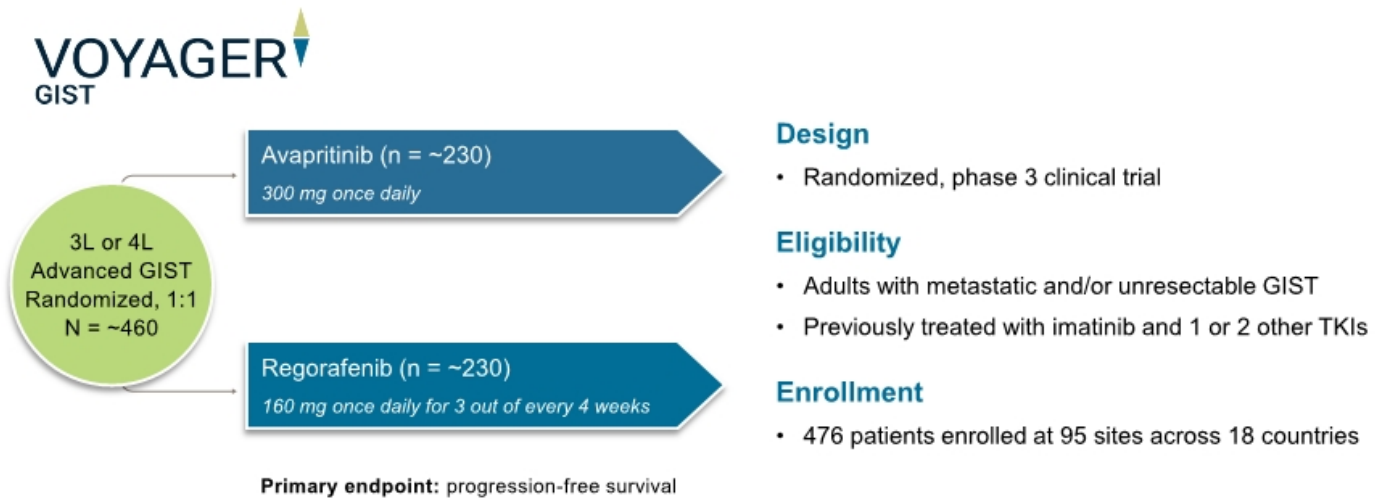
**Andy Boral**, MD, PhD, Chief Medical Officer

**Q&A**



# VOYAGER was a well-designed and well-conducted clinical trial

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GIST, gastrointestinal stromal tumor; TKI, tyrosine kinase inhibitor; 3L, third-line; 4L, fourth-line.

# VOYAGER did not meet the primary endpoint

3L AND 4L GIST (PRIMARY EFFICACY POPULATION)		Avapritinib (n=240)	Regorafenib (n=236)
		mPFS	4.2 months
ORR	17%	7%	

## TOP-LINE EFFICACY

- Difference in mPFS between avapritinib and regorafenib was not statistically significant
- Additional data analyses are ongoing with plans to present results at a future scientific meeting

## TOP-LINE SAFETY

- Consistent with previously reported data for avapritinib
- Avapritinib was generally well-tolerated and most AEs were grade 1 or 2
- 8% discontinued avapritinib due to treatment-related AEs
- 26% rate of cognitive effects in the avapritinib group, compared to a 41% rate in the NAVIGATOR trial



AE, adverse event; ORR, overall response rate; mPFS, median progression-free survival.

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## Summary and next steps for the avapritinib program in advanced GIST

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- Avapritinib did not demonstrate an improvement in mPFS over regorafenib in 3L or 4L GIST
  - Efficacy results highlight the complexity of treatment-resistant advanced GIST
- Top-line safety results for avapritinib were consistent with previously reported data
- Planned next steps:
  - Continue to commercialize AYVAKIT™ (avapritinib) in the United States for PDGFRA exon 18 mutant GIST
  - Continue to seek marketing authorization in the European Union for PDGFRA D842V mutant GIST
  - Discontinue further development of avapritinib for all other GIST indications



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# Portfolio opportunities in systemic mastocytosis and RET-altered cancers remain our priorities, with multiple planned regulatory submissions in 2020

## AVAPRITINIB



Systemic mastocytosis

- sNDA to FDA for advSM in 2H 2020

## PRALSETINIB



Non-small cell lung cancer

- ✓ NDA to FDA for RET+ NSCLC in Q1 2020
- MAA to EMA for RET+ NSCLC in Q2 2020



Medullary thyroid cancer

- NDA to FDA for RET+ MTC in Q2 2020

CONTINUE TO EXPECT EXISTING CASH BALANCE WILL FUND OPERATIONS INTO THE 2H OF 2022, BASED ON CURRENT OPERATING PLANS<sup>1</sup>



1. Includes estimated net proceeds of \$308.4M from January 2020 follow-on public offering and anticipated product revenues. Excludes any potential option fees, milestone payments or other payments under collaboration or license agreements, advSM, advanced systemic mastocytosis; EMA, European Medicines Agency; FDA, U.S. Food and Drug Administration; MAA, marketing authorization application; MTC, medullary thyroid cancer; NDA, new drug application; NSCLC, non-small cell lung cancer; sNDA, supplemental NDA.



Q&A

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