



# ANNUAL REPORT 2018

NASDAQ: BPMC



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## Dear Shareholders

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When I first joined Blueprint Medicines in 2014, the company was only three years old. In those early years, our scientists were singularly focused on building a new discovery platform, anchored in the core belief that there was a faster and more reproducible way to design highly selective and transformative kinase medicines. It was an exciting time, and our team worked hard to establish an early vision for building our business for the long-term. We set out to design medicines that exquisitely targeted the underlying molecular cause of cancer or rare diseases in specific populations and, with a portfolio-based business strategy, deliver multiple therapies to patients globally.

Now, only five years later, we are preparing to bring our first medicine directly to patients in the United States and Europe. This quarter, we plan to submit a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for our lead therapeutic candidate avapritinib, based on compelling data from our Phase 1 NAVIGATOR clinical trial. This first NDA for avapritinib – a highly selective KIT and PDGFRA inhibitor – will focus on two well-defined subsets of patients with advanced gastrointestinal stromal tumors (GIST) who have no approved treatment options: PDGFRA Exon 18 mutant GIST and fourth-line GIST. On the heels of this NDA, we also plan to submit a Marketing Authorization Application for avapritinib to the European Medicines Agency in the third quarter of 2019.

While this progress for avapritinib alone is significant, our vision has always been to deliver a portfolio of precision therapies to patients and their physicians. To this end, we announced our “2020 Blueprint” global business strategy earlier this year. Under this plan, by the end of 2020, we expect to have two marketed products in the U.S. and one marketed product in the EU, four additional marketing applications pending in the U.S. or EU, six therapeutic candidates in global clinical development, and up to eight research programs that leverage our strategic areas of focus.

Broad development programs for avapritinib and our second therapeutic candidate BLU-667, a highly selective RET inhibitor, are core components of this portfolio strategy. Beyond the initial indications for avapritinib for PDGFRA Exon 18 GIST and fourth-line GIST, we plan to submit marketing applications for third-line GIST and advanced systemic mastocytosis in 2020. In addition, registration-enabling trials for avapritinib are ongoing or planned in indolent SM and second-line GIST.

Similarly, we are rapidly advancing the development of BLU-667. In March, we announced encouraging top-line interim data and the early achievement of an enrollment target for our Phase 1 ARROW trial. These achievements have enabled us to accelerate plans to submit an NDA for previously treated RET-fusion non-small cell lung cancer (NSCLC) into the first quarter of 2020. In addition, we plan to submit a marketing application for BLU-667 for previously treated medullary thyroid cancer in the first half of 2020, while advancing the development of BLU-667 for first-line NSCLC and other RET-altered cancers.

In addition, we have two more therapeutic candidates – BLU-554 for advanced hepatocellular carcinoma and BLU-782 for fibrodysplasia ossificans progressiva – in early-stage trials and multiple undisclosed discovery programs either wholly owned or partnered under our cancer immunotherapy collaboration with Roche. Altogether, these programs position us well to rapidly advance and expand our portfolio. Later this year, we plan to highlight our portfolio expansion strategy at our first Research and Development Day for the investor community.

With potential approvals for avapritinib and BLU-667 on the horizon, we are increasingly focused on building effective and scalable commercial capabilities. In addition to establishing a commercial leadership team – led by our Chief Commercial Officer, Christina Rossi – we are engaging with physicians and patients to better understand their needs and tailor our commercial strategy to meet them. These efforts include a focus on increasing rates of tumor mutation testing, which has the potential to enable a more complete diagnosis, identify patients eligible for treatment and improve outcomes.

Underpinning our portfolio expansion and commercial plans is a strong financial foundation. We ended 2018 with \$494.0 million in cash, cash equivalents and investments, and in April 2019, we closed an underwritten public offering with estimated net proceeds of \$327.2 million. Altogether, based on our existing operating plans, we expect our existing cash will be sufficient to fund operating expenses and capital expenditure requirements into the middle of 2021, through multiple potential regulatory approvals and the recognition of potentially meaningful product revenue. Ultimately, we believe our “2020 Blueprint” strategy has the potential to spark a sustainable innovation cycle, with reinvestment of product revenue in our scientific platform and expansion of our portfolio with new medicines that can benefit even more patients.

Reflecting on these last five years, it’s exciting to see our original vision for Blueprint Medicines come into focus, as we prepare for multiple potential regulatory approvals and the launch of an integrated, global business. We are grateful to our employees, scientific and clinical collaborators, board members and stockholders for their continued support as we work to make a meaningful impact on the lives of patients. Most importantly, I want to thank the patients and families who have participated in our clinical trials, without whom we would not be able to bring forward new treatment innovations.



Jeff Albers

President and Chief Executive Officer



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## Our 2020 vision

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A broad global portfolio of precision therapies:



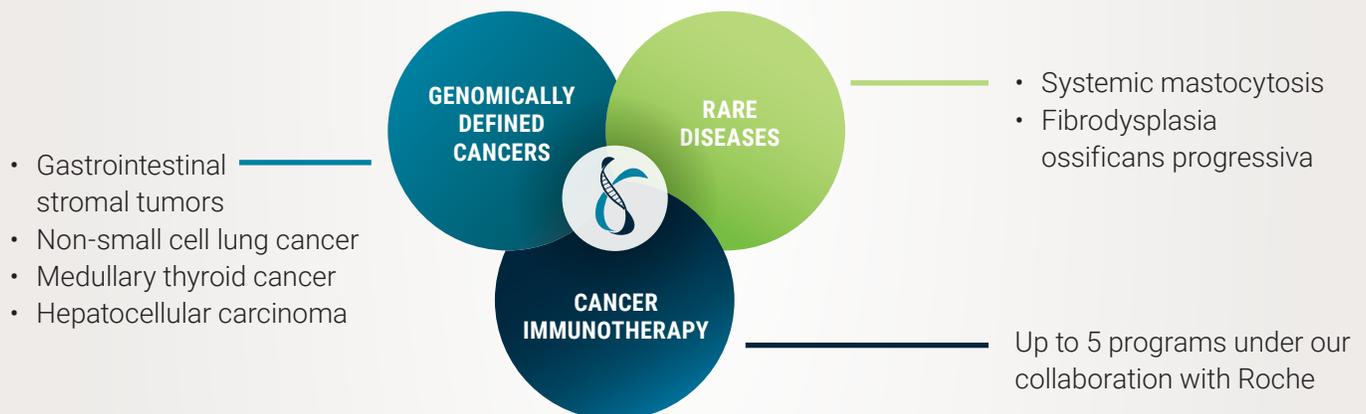
Our “2020 Blueprint” strategy establishes a planned path to transform Blueprint Medicines, by the end of 2020, into a global commercial enterprise focused on delivering a portfolio of precision therapies to patients with cancer and rare diseases.

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## Therapeutic areas of focus

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Our research focuses on distinct areas where we believe we can significantly advance medicine and improve patient outcomes. Across these areas, we combine a rich universe of kinase targets with significant scientific, clinical and commercial expertise.



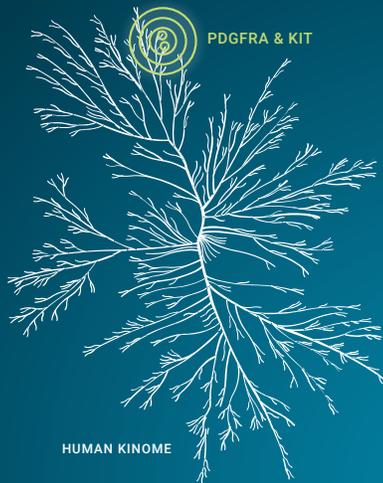
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## Lead investigational medicines

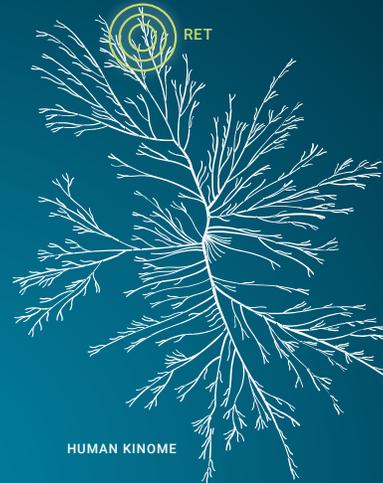
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Our proprietary scientific platform empowers the rapid and reproducible design of precision therapies that selectively target kinase drivers of disease. Currently, we are evaluating four investigational medicines in clinical trials across multiple patient populations.

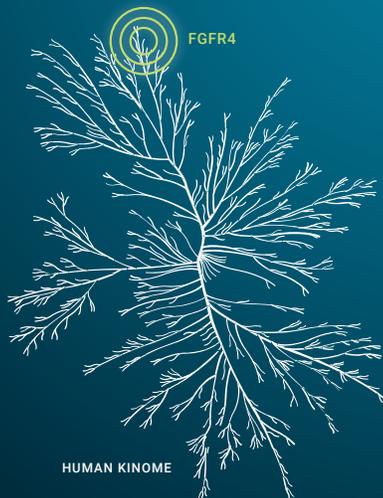
**AVAPRITINIB**



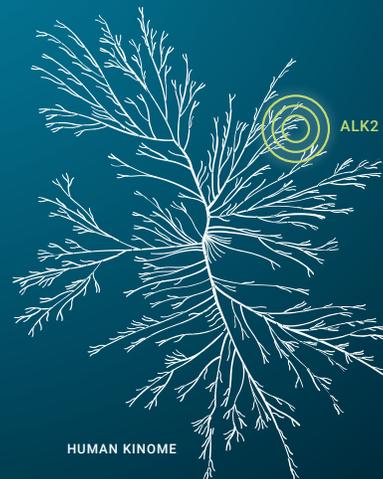
**BLU-667**



**BLU-554**

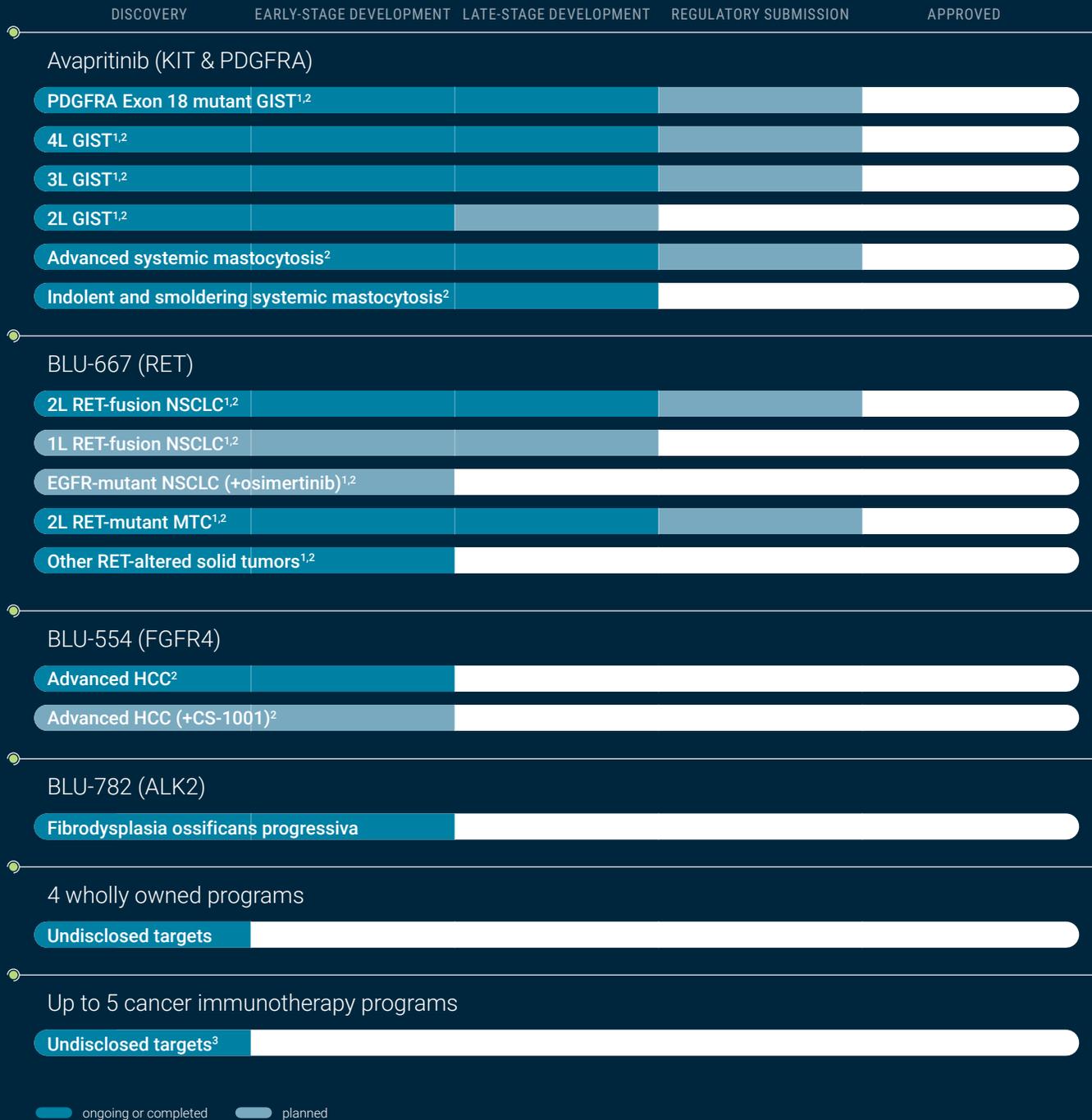


**BLU-782**



## An expansive pipeline

We are rapidly advancing multiple investigational medicines across a broad range of genetically defined cancers and rare diseases:



<sup>1</sup>Unresectable or metastatic disease.

<sup>2</sup>CStone Pharmaceuticals has exclusive rights to develop and commercialize avapritinib, BLU-554 and BLU-667 in Mainland China, Hong Kong, Macau and Taiwan. Blueprint Medicines retains all rights in the rest of the world.

<sup>3</sup>In collaboration with Roche. 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.

1L = first-line. 2L = second-line. 3L = third-line. 4L = fourth-line. GIST = gastrointestinal stromal tumors. HCC = hepatocellular carcinoma. MTC = medullary thyroid cancer. NSCLC = non-small cell lung cancer.

## Executive Leadership

### Jeff Albers

Chief Executive Officer and President

### Anthony L. Boral, M.D., Ph.D.

Chief Medical Officer

### Marion Dorsch, Ph.D.

Chief Scientific Officer

### Debbie Durso-Bumpus

Senior Vice President,  
Human Resources

### Kate Haviland

Chief Operating Officer

### Mike Landsittel

Chief Financial Officer

### Christoph Lengauer, Ph.D.

Executive Vice President

### Tracey L. McCain, Esq.

Executive Vice President,  
Chief Legal and Compliance Officer

### Christopher K. Murray, Ph.D.

Senior Vice President,  
Technical Operations

### Christina Rossi

Chief Commercial Officer

## Board of Directors

### Daniel Lynch

Chairman, Blueprint Medicines

### Jeff Albers

Chief Executive Officer, President and  
Board Member, Blueprint Medicines

### Alexis Borisy

Partner, Third Rock Ventures

### Lonnel Coats

Chief Executive Officer, President and Board  
Member, Lexicon Pharmaceuticals, Inc.

### George D. Demetri, M.D.

Professor of Medicine at Harvard  
Medical School, Director of the  
Center for Sarcoma and Bone  
Oncology and Physician at the  
Dana-Farber Cancer Institute

### Mark Goldberg, M.D.

Associate Professor of Medicine,  
Harvard Medical School

### Nicholas Lydon, Ph.D.

Founder, Granite Biopharma LLC

### Charles A. Rowland, Jr.

Former Chief Executive Officer,  
Aurinia Pharmaceuticals Inc.

### Lynn Seely, M.D.

Chief Executive Officer, President  
and Board Member, Myovant  
Sciences, Ltd.

## Annual Meeting of Stockholders

The 2019 annual meeting of  
stockholders will be held on Tuesday,  
June 18, 2019, at 3:30 p.m. ET at  
Blueprint Medicines' headquarters,  
which are located at 45 Sidney Street,  
Cambridge, MA 02139.

## Stock Listing

NASDAQ: BPMC

## Independent Auditors

Ernst & Young LLP

## SEC Form 10-K

A copy of Blueprint Medicines' Form  
10-K filed with the Securities and  
Exchange Commission is available  
free of charge from the company's  
Investor Relations Department by  
calling (617) 714-6674, emailing  
ir@blueprintmedicines.com or  
sending a written request to:

Investor Relations  
Blueprint Medicines Corporation  
45 Sidney Street  
Cambridge, MA 02139

## Transfer Agent

The transfer agent is responsible, among  
other things, for handling stockholder  
questions regarding lost stock certificates,  
address changes, including duplicate  
mailings, and changes in ownership or  
name in which shares are held. These  
requests may be directed to the transfer  
agent at the following address:

Computershare Trust Company, N.A.  
Meidinger Tower, 462 South 4th Street  
Louisville, KY 40202  
www-us.computershare.com/contactus

## Cautionary Note Regarding Forward-Looking Statements

This annual report contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, statements regarding plans and timelines for the clinical development of avapritinib, BLU-667, BLU-554 and BLU-782; the potential benefits of Blueprint Medicines' current and future drug candidates in treating patients; plans and timelines for regulatory submissions, filings or discussions; plans and timelines for current or future discovery programs; Blueprint Medicines' future financial performance; expectations regarding potential milestones in 2019 and 2020; expectations regarding Blueprint Medicines' existing cash, cash equivalents and investments; 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 annual report 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 annual report, 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-667, BLU-554 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; 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; and the success of Blueprint Medicines' cancer immunotherapy collaboration with F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc and its collaboration with CStone Pharmaceuticals.

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, 2018, as filed with the Securities and Exchange Commission (SEC) on February 26, 2019, and other filings that Blueprint Medicines has made or may make with the SEC in the future. Any forward-looking statements contained in this annual report represent Blueprint Medicines' views only as of April 29, 2019, and should not be relied upon as representing its views as of any subsequent date. Except as required by law, Blueprint Medicines assumes no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.



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