



Forma Therapeutics Highlights Etavopivat Development Expansion and Introduces New Oncology Program from Research Pipeline at Inaugural Research and Development (R&D) Day

May 26, 2022

Expanding etavopivat development in 2022 with Phase II trial exploring transfusion burden across sickle cell disease (SCD), thalassemia and myelodysplastic syndromes (MDS)

Phase I trial of FT-7051 in mCRPC proceeding with predicted efficacious dose range under evaluation and exploring alternative dosing schedule

FT-3171 (USP1 inhibitor) introduced targeting BRCA mutant tumors with investigational new drug (IND) filing expected in the first half of 2023

Early stage research pipeline focusing on red blood cell (RBC) health and novel mechanisms in rare hematology and targeted oncology indications

Forma well-positioned with \$441 million in cash to progress programs in rare hematologic diseases and cancers

WATERTOWN, Mass.--(BUSINESS WIRE)--May 26, 2022-- [Forma Therapeutics Holdings, Inc.](#) (Nasdaq: FMTX), a clinical-stage biopharmaceutical company focused on sickle cell disease (SCD), prostate cancer and other rare hematologic diseases and cancers, will hold a virtual R&D Day today to provide a comprehensive update on its pipeline and strategic vision.

The R&D event is taking place today at 8:00 a.m. ET. A live webcast will be available in the "[News & Investors](#)" section of Forma's website.

"We are pleased to share insights into Forma's unique commitment to patients and expansion of etavopivat development into transfusion-dependent populations that have tremendous unmet need across sickle cell disease, thalassemia and MDS," said Frank Lee, president and chief executive officer of Forma.

"In our early stage research pipeline, we are excited to introduce a USP1 program that targets a novel DNA damage repair pathway relevant to a broad range of tumor types," said David Cook, chief scientific officer of Forma. "We are also capitalizing on our knowledge of the emerging science of red blood cell health by exploring areas beyond hematologic disease."

Etavopivat (oral PKR activator) Program in SCD, thalassemia and MDS:

In addition to ongoing enrollment in the Phase II/III Hibiscus Study in SCD, Forma will outline ongoing and anticipated future development plans:

- A Phase II trial is enrolling patients in three cohorts: SCD with chronic transfusion, and both transfusion-dependent and non-transfusion-dependent thalassemia. Etavopivat has the potential to address RBC health, hemolytic anemia and/or ineffective erythropoiesis in these populations, leading to reduction of transfusion burden and associated iron overload and improvement of anemia.
- A Phase II trial in lower-risk MDS is planned to commence in the second half of 2022. Dr. Michael Savona of Vanderbilt University will review the significant unmet need in lower-risk MDS and the potential for etavopivat to provide a well-tolerated oral treatment that may be able to improve the ability of bone marrow to produce healthier RBCs.
- Analyses from the completed Phase I trial in SCD show benefit in both pain events reported in the trial and vaso-occlusive crises (VOC's) occurring during treatment.
- Enrollment in the Phase II/III Hibiscus Study in SCD is on track for the first interim analysis (IA1) in late 2022.

FT-7051 (oral CBP/p300 inhibitor) in prostate cancer:

Forma's Phase I trial continues to enroll men with metastatic castration-resistant prostate cancer (mCRPC).

- As of May 12, 2022, 25 patients have enrolled in the Phase I dose escalation trial, assessing the predicted efficacious exposure range supported by target engagement.
- The trial population is heavily pre-treated, with a high AR-v7 positivity rate and mutation burden.
- Future trial enrollment to include less heavily pre-treated patients and alternative dosing schedules to address adverse events, with updated results expected in the first half of 2023.

Research Pipeline/RBC Health:

Forma's ongoing research efforts are focused on novel mechanisms of action in oncology and expansion in red blood cell health and hematologic diseases:

- Forma's research pipeline is led by the USP1 program (FT-3171), targeting a novel DNA damage repair pathway with the potential to address multiple tumor types in both poly ADP ribose polymerase inhibitor (PARPi)-sensitive and resistant

settings.

- FT-3171 IND filing is expected in the first half of 2023.
- Ongoing pre-clinical research is targeting areas of expansion for RBC health, novel mechanisms that may be complementary to etavopivat in rare hematology, and targeted oncology.

About Forma Therapeutics

Forma Therapeutics is a clinical-stage biopharmaceutical company focused on the research, development and commercialization of novel therapeutics to transform the lives of patients with rare hematologic diseases and cancers. Our R&D engine combines deep biology insight, chemistry expertise and clinical development capabilities to create drug candidates with differentiated mechanisms of action focused on indications with high unmet need. Our work has generated a broad proprietary portfolio of programs with the potential to provide profound patient benefit. For more information, please visit www.FormaTherapeutics.com or follow us on Twitter @FORMAInc and LinkedIn.

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, express or implied statements regarding the company's beliefs and expectations regarding its: business plans and objectives; future plans for etavopivat, FT-7051 and FT-3171, including expectations regarding potential development expansion plans as well as regulatory filings, enrollment, timing, success and data announcements of planned and ongoing clinical and pre-clinical trials; therapeutic potential, clinical benefits, mechanisms of action and safety of our product candidates; plans for the USP1 program (FT-3171) and other research pipeline expansion efforts; upcoming milestones and planned additional trials for the company's product candidates; growth as a company; upcoming presentations of our R&D programs, including the introduction of a new molecule and related studies; uses and need of capital, expenses and other financial results currently or in the future. 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, those risks and uncertainties associated with the following: the impact of the COVID-19 pandemic on the company's business, operations, supply chain, patient enrollment and retention, clinical trials, strategy, goals and anticipated milestones, as well as global economies and financial markets; the therapeutic potential of our product candidates and the timing and completion of our clinical trials and related data analyses; positive results from a clinical study may not necessarily be predictive of the results of future or ongoing clinical studies; any one or more of our product candidates may not be successfully developed and commercialized; regulatory developments in the United States and foreign countries; our ability to protect and maintain our intellectual property position; and our ability to fund operations; as well as those risks and uncertainties set forth more fully under the caption "Risk Factors" in our most recent annual report on Form 10-Q filed with the United States Securities and Exchange Commission (SEC) and subsequent filings with the SEC. We disclaim any obligation to publicly update or revise any such statements to reflect any change in expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements. Any forward-looking statements contained in this press release represent our views only as of the date hereof and should not be relied upon as representing our views as of any subsequent date.

View source version on [businesswire.com](https://www.businesswire.com/news/home/20220526005218/en/): <https://www.businesswire.com/news/home/20220526005218/en/>

Media Contact:

Caitlin Hunt, +1 781-985-5967
Porter Novelli
caitlin.hunt@porternovelli.com

Investor Contact:

Mario Corso, +1 781-366-5726
Forma Therapeutics
mcorso@formatherapeutics.com

Source: Forma Therapeutics Holdings, Inc.