



Forma Therapeutics Announces Positive Top-line Olutasidenib Data From a Planned Interim Analysis of a Registrational Phase 2 Clinical Trial in Acute Myeloid Leukemia (AML)

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FT2102-HEM-101 clinical trial data demonstrate a 30% CR and 3% CRh rate with olutasidenib monotherapy in 123 relapsed or refractory IDH1m AML patients

While median duration of CR/CRh has not been reached, sensitivity analysis indicates the median duration of CR/CRh to be 13.8 months

Favorable tolerability profile consistent with Phase 1 study results

Data will be submitted for discussion at an upcoming medical meeting

WATERTOWN, Mass.--(BUSINESS WIRE)--Oct. 26, 2020-- [Forma Therapeutics Holdings, Inc.](#) (Nasdaq: FMTX), a clinical-stage biopharmaceutical company focused on rare hematologic diseases and cancers, today announced positive top-line data from a planned interim analysis of a registrational Phase 2 clinical trial of olutasidenib, Forma's selective inhibitor for hematological malignancy cancers with mutations in isocitrate dehydrogenase 1 (IDH1m). Olutasidenib demonstrated a favorable tolerability profile as a monotherapy in patients with IDH1m relapsed/refractory acute myeloid leukemia (R/R AML), and achieved a composite complete remission (CR+CRh, or complete remission plus complete remission with partial hematologic recovery) rate of 33.3% (30% CR and 3% CRh), the primary efficacy endpoint. While a median duration of CR/CRh has not been reached, a sensitivity analysis (with a hematopoietic stem cell transplant or HCST as the end of a response) indicates the median duration of CR/CRh to be 13.8 months.

Safety results are consistent with previously reported Phase 1 clinical trial results^{1,2}. The most common adverse events (AEs) observed were nausea, constipation, increased white blood cell count, decreased red blood cell count, fever, febrile neutropenia and fatigue.

"We are pleased to announce these compelling top-line data," said Patrick Kelly, MD, chief medical officer of Forma Therapeutics. "The safety profile and the duration of the response we're seeing supports the potential for olutasidenib to become a leading therapy for R/R IDH1m AML patients. While the multi-cohort Phase 2 trial is ongoing, this specific cohort was designed to serve as a pivotal study; these efficacy data support an early stop in enrollment in favor of moving the program forward."

Additional analyses and other outcome measures will be presented at an upcoming medical meeting.

Study Design

The Phase 1/2 study is a multicenter, open-label, multi-cohort evaluation of the safety, efficacy and pharmacokinetics/pharmacodynamics (PK/PD) of olutasidenib for patients with AML or myelodysplastic syndrome (MDS) with an IDH1 mutation. Phase 1 of the trial, FT2102-HEM-101, was an open-label, dose-escalation and expansion study of olutasidenib alone and in combination with azacitidine (AZA). The pivotal Phase 2 study is an open-label, fixed-dose study of olutasidenib as a monotherapy in IDH1m AML patients. The Phase 2 study includes other cohorts of olutasidenib in combination with AZA in IDH1m AML/MDS populations. The primary efficacy-evaluable population of the pivotal phase 2 study is comprised of 123 R/R AML patients, who received olutasidenib 150 mg BID at least six months prior to the interim analysis cutoff date of June 18, 2020. The primary endpoint is a composite of a complete remission (CR) plus a complete remission with partial hematological recovery (CRh), defined as less than 5% blasts in the bone marrow, no evidence of disease and partial recovery of peripheral blood counts (platelets >50,000/microliter and ANC >500/microliter).

About Olutasidenib

Olutasidenib is an oral, potent and small molecule investigational agent designed to selectively bind to and inhibit mutated IDH1 enzymes. This targeted treatment has the potential to provide therapeutic benefit by reducing 2-HG levels and restoring normal cellular differentiation. Forma is currently evaluating olutasidenib in a registrational Phase 2 trial for relapsed/refractory AML and in an exploratory Phase 1 trial for glioma and other solid tumors.

IDH1 is a natural enzyme that is part of the normal metabolism of all cells; when mutated, its activity can promote blood malignancies and solid tumors. IDH1 mutations are present in 6-8% of patients with AML and as many as 70 to 80% of patients with grade II/III gliomas and secondary glioblastoma. In gliomas, IDH1 mutations occur early in the tumor pathogenesis and persist throughout progression from a neural stem or progenitor cell. Gliomas are the most common, aggressive and difficult-to-treat primary brain tumors, and high-grade gliomas are associated with poor long-term prognosis. Treatment options for relapsed glioma are limited.

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: our guidance regarding our business plans and objectives for olutasidenib, including the therapeutic potential and clinical benefits thereof as well as the planned study design, the interim results of the Phase 2 clinical trial of olutasidenib, including its initial primary efficacy, safety and tolerability results, the planned additional analyses of the 2102-HEM-101 study, the timing and success of ongoing clinical trials, our growth as a company, and the potential impact of COVID-19 on patient retention, strategy, future operations and clinical trials. 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 related to the advancement of our clinical programs, our ability to execute on our strategy, that positive interim results from a clinical study may not be necessarily predictive of the results of future or ongoing clinical studies, the regulatory developments in the United States, the risks related to the competitive landscape, and other risks identified in our SEC filings, including those risks discussed under the heading “Risk Factors” in our Quarterly Report on Form 10-Q for the quarter ended June 30, 2020, as well as other risks detailed in our subsequent filings with the SEC. We caution you not to place undue reliance on any forward-looking statements, which speak only as of the date they are made. 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 its views as of any subsequent date. We explicitly disclaim any obligation to update any forward-looking statements.

¹ J. Watts et al., ASH 2019

[Olutasidenib, an IDH1 Inhibitor as a single agent or in combination with azacitidine, induces deep clinical remissions with mutation clearance in patients with acute myeloid leukemia treated in a Phase I dose escalation and expansion study.](#)

² J. Cortes et al., ASH 2019

[Olutasidenib induces rapid remissions in patients with IDH1-mutant myelodysplastic syndrome: Results of Phase 1/2 single-agent treatment and combination with azacitidine](#)

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