Forma Therapeutics to Present Data from Pivotal Phase 2 Trial of Olutasidenib at ASCO 2021

May 20, 2021

Olutasidenib demonstrated a 33.3% composite complete remission rate (CR/CRh) in people living with R/R AML with the IDH1 mutation

Among those with CR/CRh, estimated 18-month survival is 87%; median overall survival has not yet been reached

Duration of response of 13.8 months is longest reported in this treatment setting to date

Favorable tolerability profile following continuous oral treatment with olutasidenib 150mg BID

WATERTOWN, Mass.--(BUSINESS WIRE)--May 20, 2021-- Forma Therapeutics Holdings, Inc. (Nasdaq: FMTX) today announced that topline interim data from its Phase 2 trial of olutasidenib in relapsed/refractory acute myeloid leukemia (R/R AML) will be presented at the upcoming 2021 American Society of Clinical Oncology (ASCO) Annual Meeting taking place June 4-8. Olutasidenib, Forma's selective inhibitor for cancers with mutations in isocitrate dehydrogenase 1 (IDH1m), demonstrated positive efficacy and a favorable tolerability profile as a monotherapy in patients with IDH1m R/R AML, achieving the primary endpoint of a composite complete remission (CR) or CR plus CR with partial hematologic recovery (CRh) rate of 33.3% (30% CR and 3% CRh).

The presentation is based on an interim analysis from a pivotal trial arm evaluating continuous treatment with 150 mg twice daily of oral olutasidenib. The data indicate the duration of CR/CRh for people on treatment was 13.8 months. Among patients with a complete remission (CR) who were transfusion-dependent at baseline, 56-day transfusion independence was achieved in 100% of patients as measured by platelets and 83% as measured by red blood cells.

"The data being presented at ASCO showcase olutasidenib's meaningful progress for this patient population, which currently has limited options to extend life expectancy," said Patrick Kelly, M.D., chief medical officer of Forma Therapeutics. "The safety data from the treatment cohort are consistent with the findings from our Phase 1 evaluation in this high-risk AML patient population. The data highlight the duration of response, which is nearly six months longer than current standard of care."

Oral Abstract Session – June 4, 2:30 p.m. ET

- Abstract #7006: Effect of olutasidenib (FT-2102) on complete remissions in patients with relapsed/refractory (R/R) mIDH1 acute myeloid leukemia (AML): Results from a planned interim analysis of a phase 2 clinical trial

About the Phase 1/2 Study

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, 2102-HEM-101, was an open-label, dose-escalation and expansion study of olutasidenib alone and in combination with azacitidine (AZA). The Phase 2 portion was an open-label, fixed-dose study of olutasidenib as a monotherapy and in combination with AZA in multiple IDH1m AML/MDS populations. The primary efficacy evaluable population is comprised of 123 R/R AML patients enrolled in Cohort 1, who received 150 mg of olutasidenib BID at least six months prior to the interim analysis cutoff date of June 18, 2020. The primary endpoint of the Phase 2 pivotal study is a complete remission (CR) plus a complete remission with partial hematological recovery (CRh) that is defined as <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).

Key Study Findings

Efficacy (n=123)

- Olutasidenib induced a durable CR/CRh rate of 33.3% (95% CI 25.1, 42.2), which is the primary endpoint of the study:
  - The CR rate was 30.0% (37 of 123 patients) and the CRh rate was 3.0% (4 of 123 patients)
- While the median duration of response was not yet reached, in a sensitivity analysis with hematopoietic stem cell transplant considered as the end of a response, the median duration was 13.8 months.
- The median duration of overall response was 11.7 months.
- The median overall survival (OS) was 10.5 months. The median OS for non-CR/CRh responders was 15.0 months. A median OS has not yet been reached for the CR/CRh population, with an 18-month survival estimate of 87.0%.
- Transfusion independence was achieved in all response groups at 56 days, particularly in those achieving CR, with 100% independence for platelet transfusions and 83.0% independence for red blood cells.

Safety (n=153)

- Olutasidenib was well-tolerated, with adverse events (AEs) consistent with the late stage disease and the heavily pre-treated population. A safety analysis for all 153 patients enrolled in the Phase 2 Cohort 1 found the most common

grade 3/4 (≥ 20% or ≥ 10%) treatment-emergent adverse events (TEAEs) were febrile neutropenia (20%), anemia (19%), thrombocytopenia (16%), neutropenia (13%), leukocytosis (9%) and fatigue (<1%). AEs of interest were the following:

- 14% of patients reported AEs due to Differentiation syndrome, including 7% Grade 3 and 1% Grade 4 AEs. Most resolved with corticosteroids and treatment interruption. However, 1 fatal event was reported.
- 8% of patients reported AEs due to QTc prolongation, with <1% Grade 3 or 4. No events led to discontinuation.
- Grade 3 or 4 elevation in liver parameters (ALT/AST/total bilirubin) occurred in 10% and 2% of patients, respectively. Most resolved with treatment interruption and dose reduction. Seven patients (<5%) discontinued study treatment due to LFT abnormalities. No Hy's law cases reported.

About Olutasidenib

Olutasidenib is an oral, potent, 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. With the conclusion of the Phase 2 R/R AML trial, Forma has begun preparing a new drug application (NDA) for submission to the U.S. Food and Drug Administration (FDA).

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 beliefs and expectations regarding: interim data analysis for olutasidenib in the Phase 2 trial in R/R AML; the therapeutic potential and clinical benefits and safety related to olutasidenib; whether interim results from our clinical trials are predictive of final trial results or future clinical studies; our planned presentation of data at ASCO; and planned regulatory submissions, including the preparation and submission of an NDA for olutasidenib with the FDA. 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 our ability to execute on our strategy; the finalization of our Phase 2 study in R/R AML and final audit and quality controlled verification of interim data and related analyses; positive results from interim data analyses may not be predictive of final results; risks related to our planned regulatory submissions and developments; 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 March 31, 2021, 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.

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Media:
Adam Silverstein, +1 917-697-9313
Porter Novelli
adam.silverstein@porternovelli.com

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

Source: Forma Therapeutics Holdings, Inc.