December 9, 2019

FORMA Therapeutics Announces Positive Preliminary Phase 1/2 IDH1m Inhibitor Olutasidenib Results Demonstrating Rapid Clinical Remissions And Mutation Clearance In Patients With Acute Myeloid Leukemia And Myelodysplastic Syndrome

– Phase 2 Pivotal Study is Ongoing with Olutasidenib as a Monotherapy in Patients with Relapsed/Refractory Acute Myeloid Leukemia with an IDH1 Mutation –
– Data Presented at the 61st Annual American Society of Hematology Meeting –

WATERTOWN, Mass. – December 9, 2019 – FORMA Therapeutics Inc., a clinical stage biopharmaceutical company focused on rare hematologic diseases and cancers, today announced positive preliminary Phase 1/2 results from an ongoing study of olutasidenib, a next-generation inhibitor of mutated IDH1 (IDH1m), in patients with IDH1m acute myeloid leukemia (AML) and IDH1m myelodysplastic syndrome (MDS). The data, presented at the 2019 American Society of Hematology Annual Meeting (ASH), demonstrate the potential of olutasidenib to induce rapid remissions and mutation clearance in a percentage of patients with IDH1m AML and MDS.

“We are very pleased with the safety and clinical activity observed in this study of high-risk patients,” said Patrick Kelly, M.D., chief medical officer of FORMA Therapeutics. “As we continue our Phase 2 evaluation, the therapeutic potential of olutasidenib to restore normal cellular differentiation in IDH1m malignancies, as well as to become a best-in-class treatment option, remains promising.”

“In the U.S. alone, more than 20,000 new cases of AML are diagnosed each year, with about one-third of those evolving from patients with MDS,” said Frank Lee, chief executive officer of FORMA Therapeutics. “Despite recent advances in the field, the low median five-year survival rate for AML patients is only 28%, so the need for a new therapy to transform patient outcomes remains significant.”

FORMA is conducting a Phase 1/2 study evaluating the safety, efficacy and pharmacokinetics/pharmacodynamics (PK/PD) of olutasidenib for patients with AML or 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). Phase 2 is an ongoing, open-label, fixed dose study of olutasidenib as a monotherapy and in combination with AZA in multiple IDH1m AML/MDS populations. Phase 2 includes a pivotal arm with olutasidenib as a monotherapy in relapsed and refractory (R/R) AML.

Presentation Overviews

Olutasidenib (FT-2102), an IDH1m Inhibitor as a Single Agent or in Combination with Azacitidine, Induces Deep Clinical Responses with Mutation Clearance in Patients with Acute Myeloid Leukemia Treated in a Phase 1 Dose Escalation and Expansion Study

Oral presentation by Justin Watts, MD, Assistant, Professor University of Miami, Sylvester Comprehensive Cancer Center

Results announced today are based on continuous oral treatment of olutasidenib for 28-day cycles, either alone (n=32) or in combination with AZA (n=46), in patients with IDH1m AML, with a dose evaluation of 300 mg once daily for olutasidenib alone and 150 mg once daily or twice daily for olutasidenib in combination with AZA. The findings indicate:

- Olutasidenib is well tolerated as monotherapy and in combination with AZA;
- No dose-limiting toxicities in dose escalation; 150 mg BID is the RP2D based on optimal exposure and robust 2-HG response;
- Olutasidenib demonstrates clinical activity in a high-risk Phase 1 AML population; and
- Olutasidenib induces IDH1 mutation clearance in a percentage of patients with TN and R/R AML regardless of IWG response.

FORMA's Phase 2 study is ongoing with olutasidenib 150 mg BID as monotherapy and in combination with AZA in multiple IDH1m AML and MDS populations.

Olutasidenib (FT-2102) Induces Rapid Remissions in Patients with IDH1-Mutant Myelodysplastic Syndrome: Results of Phase 1/2 Single Agent Treatment and Combination with Azacitidine

Oral presentation by Jorge E. Cortes, MD, Professor, Augusta University, Director of the Georgia Cancer Center

Results presented today are based on continuous oral treatment of olutasidenib for 28-day cycles, either alone (n=6) or in combination with AZA (n=17) in 23 patients (16 relapsed/refractory, 7 treatment naïve) with IDH1m MDS. The findings indicate:

- Olutasidenib is well-tolerated as a single agent and in combination with AZA;
- Olutasidenib demonstrates preliminary clinical activity as a single agent and in combination with AZA in treatment-naive and relapsed/refractory patients with MDS;
- Mutation clearance was observed in a percentage of evaluable patients; and
- Rapid and sustained reduction of 2-HG was seen by the end of the first cycle.

About Olutasidenib (FT-2102)

FORMA Therapeutics' most advanced clinical asset, olutasidenib, is designed to be a potent and selective next generation inhibitor of mutated isocitrate dehydrogenase 1 (IDH1m) to treat patients with acute myeloid leukemia (AML) or myelodysplastic syndrome (MDS), as well as patients with glioma and other solid tumors with an IDH1 mutation. IDH1 is a natural enzyme that is part of the normal metabolism in all cells; when mutated, its activity can promote blood malignancies and solid tumors. IDH1 mutations are present in 7-14% of patients with AML, 3-4% of patients with MDS, and more than 70% of patients with gliomas. In AML, hypermethylation driven by IDH mutations inhibits normal differentiation of progenitor cells leading to accumulation of immature blasts. Quality of life declines for patients with each successive line of treatment for AML, and well-tolerated treatments in relapsed disease remain an unmet need. In MDS, often a precursor to AML, epigenetic changes from aberrant DNA methylation contribute to the formation of blast cells and the progression of MDS to AML.

About FORMA Therapeutics

FORMA Therapeutics is focused on the discovery, development and commercialization of transformative medicines for patients with rare hematologic diseases and cancers. A fully-integrated biopharmaceutical company, FORMA's validated, proprietary R&D engine combines deep biology insight, chemistry expertise and clinical development capabilities to create differentiated drug candidates with best-in-class or first-in-class potential. FORMA has delivered high-value clinical candidates to its partners and generated a broad proprietary portfolio of programs, ranging from preclinical to pivotal-stage, with the potential to provide profound patient benefit. For more information, please visit the company website at www.formatherapeutics.com or follow us on Twitter @FORMAInc and LinkedIn.

Media Contact:
Kari Watson, +1 781-235-3060
MacDougall
kwatson@macbiocom.com

Investor Contact:
Stephanie Ascher, +1 212-362-1200
Stern Investor Relations
stephanie.ascher@sternir.com