A FUNDAMENTALLY DIFFERENT APPROACH TO TREATING CANCER & RARE GENETIC DISEASES

Inspired by patients and frustrated by the limitations of conventional approaches to treatment, Agios pioneered a novel path to treating cancer and rare genetic diseases by targeting cellular metabolism. In our first decade, Agios brought two precision oncology medications from our own labs to patients – TIBSOVO® (ivosidenib tablets) for acute myeloid leukemia (AML) patients with an IDH1 mutation and IDHIFA® (enasidenib) for AML patients with an IDH2 mutation. During that same time period, the Agios team discovered and developed six additional investigational new drug (IND) candidates, including the first pyruvate kinase R (PKR) activator in development as a potential treatment for a rare hemolytic anemia known as pyruvate kinase deficiency. Agios is leading the way in advancing PKR activation for additional non-malignant hematologic diseases, including thalassemia and sickle cell disease, and the company continues to foster a productive research engine that yields new insights and potential therapeutic approaches. Now entering our next decade, Agios has a differentiated portfolio of preclinical, clinical and commercial programs, unparalleled expertise in cellular metabolism and a committed team that will continue to drive the company’s mission to make a difference for patients with hematologic malignancies, solid tumors and rare genetic diseases.

UNWAVERING COMMITMENT TO SCIENCE AND PATIENTS

Agios is a biopharmaceutical company passionately committed to applying our leadership in the field of cellular metabolism to transform the lives of patients with hematologic malignancies, solid tumors and rare genetic diseases. Agios believes that dysregulation of normal cellular metabolism plays a crucial role in many genetic diseases, and it is among the first in using cellular metabolism as a platform for developing potentially transformative medicines.
In early disease, and the company plans to initiate pivotal Phase 3 data, proof-of-concept has been established for mitapivat deficiencies in red blood cell glycolysis, which lead to pyruvate kinase R (PKR) enzymes. Mutations in PKR cause PKR Activator

Mitapivat

Metabolic

Medicines

Vorasidenib

IDHIFA

Mitapivat

Mitapivat is an investigational, wholly owned, first-in-class, novel, oral activator of both wild-type and mutated PKR enzymes. It is developing as a potential treatment for thalassemia and for sickle cell disease, and the company plans to initiate pivotal Phase 3 studies in both indications.

Vorasidenib is an investigational, orally available, selective inhibitor of the mutated IDH1 and IDH2 enzymes. In early clinical studies, vorasidenib demonstrated full blood-brain barrier penetration, robust suppression of the oncometabolite 2-HG and encouraging preliminary efficacy. Vorasidenib is currently being studied in the registration-enabling Phase 3 INDIGO study in patients with IDH-mutant low-grade glioma.

IDHIFA is an investigational first-in-class methionine adenosyltransferase 2a (MAT2A) inhibitor being evaluated in combination with taxanes in patients with methylthioadenosine phosphorylase (MATAP)-deleted non-small cell lung cancer and pancreatic cancer. MAT2A is a component of a novel pathway in MTAP-deleted tumors which, when modulated by small molecule inhibitors, results in robust anti-tumor activity.

AG-270 is an investigational first-in-class methionine adenosyltransferase 2a (MAT2A) inhibitor being evaluated in combination with taxanes in patients with methylthioadenosine phosphorylase (MATAP)-deleted non-small cell lung cancer and pancreatic cancer. MAT2A is a component of a novel pathway in MTAP-deleted tumors which, when modulated by small molecule inhibitors, results in robust anti-tumor activity.

Preclinical Programs

Agios has led the field of cancer metabolism with its novel IDH and MTAP programs and continues to make important advances in the field of rare genetic diseases with its PKR enzymes. The initial focus of the AG-946 development program is to establish that it is safe and well-tolerated in healthy volunteers.

Preclinical programs exemplify our strategy of applying our foundational expertise in cellular metabolism and precision medicine to translated science from our labs into first-of-their-kind experimental therapies. In addition to advancing our lead programs, we continue to discover novel metabolic targets that meet a high bar for future development across all three of our core focus areas: malignant hematology, solid tumors and rare genetic diseases.

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