



FDA Accepts New Drug Application and Grants Priority Review for Enasidenib in Relapsed or Refractory AML with an IDH2 Mutation

March 1, 2017

PDUFA date set for Aug. 30, 2017

SUMMIT, N.J. & CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Celgene Corporation ([CELG](#)) and Agios Pharmaceuticals ([AGIO](#)) today announced that the U.S. Food and Drug Administration (FDA) has accepted Celgene's New Drug Application (NDA) for enasidenib (AG-221/CC-90007) for the treatment of patients with relapsed or refractory acute myeloid leukemia (AML) with an isocitrate dehydrogenase 2 (IDH2) mutation. The NDA was granted Priority Review and has been given a Prescription Drug User Fee Act (PDUFA) action date of Aug. 30, 2017. Celgene completed the NDA submission in late December 2016.

"We accelerated this application – submitting the NDA just three years after the first patient was treated in the enasidenib pivotal investigational trial – because we believe that there is a significant unmet need for people with relapsed or refractory AML," said Michael Pehl, president, Hematology/Oncology for Celgene. "The acceptance of the enasidenib NDA is a significant milestone in what we hope will be a new era of molecularly targeted therapies for patients with this devastating disease."

Enasidenib is a first-in-class, oral, targeted inhibitor of mutant IDH2. The NDA submission is based on results from AG221-C-001, a single-arm phase I/II study of enasidenib in patients with advanced hematologic malignancies with an IDH2 mutation. Early data from the relapsed or refractory AML patients in this study were presented at the 2015 American Society of Hematology (ASH) Annual Meeting.

"Having received NDA acceptance and priority review for enasidenib, we look forward to working with our partner Celgene and the FDA to advance a first-in-class therapy for relapsed or refractory AML with an IDH2 mutation," said David Schenkein, M.D., chief executive officer at Agios. "We hope that the continued adoption of molecular profiling and availability of new targeted therapies such as enasidenib will have a significant impact on patients living with AML."

Additionally, Abbott has submitted a Premarket Approval (PMA) application for the FDA approval of an IDH2 assay on the Abbott *m2000 RealTime* System, a polymerase chain reaction (PCR) molecular diagnostics instrument. IDH2 mutations occur in about 8 to 19 percent of AML patients. Recent publications have highlighted the advances in the understanding of the genetics underlying AML and the need for routine mutational analysis at diagnosis and relapse.

Celgene is also evaluating enasidenib compared with conventional therapy in older patients with an IDH2 mutation and relapsed or refractory AML in the ongoing phase III IDHENTIFY trial (NCT02577406).

Enasidenib is an investigational drug that has not been approved for any use in any country.

About Acute Myelogenous Leukemia (AML)

AML, a cancer of blood and bone marrow characterized by rapid disease progression, is the most common acute leukemia affecting adults. Undifferentiated blast cells proliferate in the bone marrow rather than mature into normal blood cells. AML incidence significantly increases with age, and according to the American Cancer Society, the median age of onset is 66. Less than 10 percent of U.S. AML patients are eligible for bone marrow transplant and the vast majority of patients do not respond to chemotherapy and progress to relapsed/refractory AML. The five-year survival rate for AML is approximately 20 to 25 percent. IDH2 mutations are present in about 8 to 19 percent of AML cases.

About Celgene

Celgene Corporation, headquartered in Summit, New Jersey, is an integrated global biopharmaceutical company engaged primarily in the discovery, development and commercialization of innovative therapies for the treatment of cancer and inflammatory diseases through next-generation solutions in protein homeostasis, immuno-oncology, epigenetics, immunology and neuro-inflammation. For more information, please visit www.celgene.com. Follow Celgene on Social Media: [@Celgene](#), [Pinterest](#), [LinkedIn](#), [Facebook](#) and [YouTube](#).

About Agios

Agios is focused on discovering and developing novel investigational medicines to treat cancer and rare genetic diseases through scientific leadership in the field of cellular metabolism. In addition to an active research and discovery pipeline across both therapeutic areas, Agios has multiple first-in-class investigational medicines in clinical and/or preclinical development. All Agios programs focus on genetically identified patient populations, leveraging our knowledge of metabolism, biology and genomics. For more information, please visit the company's website at www.agios.com.

About Agios/Celgene Collaboration

Enasidenib is part of Celgene's global strategic collaboration with Agios focused on cancer metabolism. Under the terms of the 2010 collaboration agreement, Celgene has worldwide development and commercialization rights for enasidenib. Agios continues to conduct clinical development activities within the enasidenib development program and is eligible to receive reimbursement for those development activities and up to \$120 million in payments assuming achievement of certain milestones and royalties on net sales. Celgene and Agios intend to co-commercialize enasidenib in the U.S. Celgene will reimburse Agios for costs incurred for its co-commercialization efforts.

Forward-Looking Statements

This press release contains forward-looking statements, which are generally statements that are not historical facts. Forward-looking statements can

be identified by the words "expects," "anticipates," "believes," "intends," "estimates," "plans," "will," "outlook" and similar expressions. Forward-looking statements are based on management's current plans, estimates, assumptions and projections, and speak only as of the date they are made. Neither Celgene nor Agios undertake any obligation to update any forward-looking statement in light of new information or future events, except as otherwise required by law. Forward-looking statements involve inherent risks and uncertainties, most of which are difficult to predict and are generally beyond each company's control. Actual results or outcomes may differ materially from those implied by the forward-looking statements as a result of the impact of a number of factors, many of which are discussed in more detail in the Annual Report on Form 10-K and other reports of each company filed with the Securities and Exchange Commission.

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