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General AML

Ivosidenib approved as first-line treatment for AML with IDH1 mutation

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Results of an open-label, multicenter, single-arm study (NCT 02074839) have led to the approval of ivosidenib for the treatment of newly diagnosed acute myeloid leukemia (AML).

On 2nd May 2019, the U.S. Food and Drug Administration (FDA) granted approval for the use of ivosidenib as the first-line therapy for newly diagnose AML with a susceptible IDH1 mutation. Patients should be at least 75 years old, or have comorbidities that preclude the use of intensive induction chemotherapy.

Patients enrolled in the study were at least 75, or had severe pulmonary or cardiac disease, creatine clearance at <45ml/min, or hepatic impairment with bilirubin >1.5 times the upper limit of normal. Ivosidenib was administered orally at 500mg once per day until progression of the disease, development of unacceptable toxicity, or hematopoietic stem cell transplantation (HSCT). Of the 28 patients enrolled in the study, the median age was 77 years (range, 64—87), and 22 (79%) had myelodysplasia-related changes or therapy-related AML.

Fatigue, diarrhea, edema, decreased appetite, leucocytosis, nausea, arthralgia, abdominal pain, dyspnea, myalgia and differentiation syndrome were the side effects seen in at least 25% of patients. As differentiation syndrome could be potentially life threatening, a boxed warning is issued with the medication to alert healthcare professionals.

The recommended dose of ivosidenib is 500mg orally, once daily with or without food, until disease progression or unacceptable toxicity. Treatment is recommended for a minimum of six months for patients without toxicity or disease progression, allowing time for clinical response.

In the study, 42.9% (n = 12) of patients achieved complete remission (CR) or CR with partial hematologic recovery (CRh).