Dysmegakaryopoiesis and Transient Mild Increase in Bone Marrow Blasts in Patients With Aplastic Anemia Treated With Eltrombopag May Be Signs of Hematologic Improvement and Not Portend Clonal Evolution

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ABSTRACT

Objectives: Eltrombopag, a thrombopoietin-receptor agonist, stimulates hematopoiesis in patients with acquired aplastic anemia (AA). Cytomorphologic changes in bone marrow after eltrombopag administration are still unclear. This study examined the effect of eltrombopag on cytomorphologic findings using data from prior phase 2 studies (E1201 and E1202).

Methods: Microscopic examinations were performed in 31 patients with AA (E1201 [n = 21], E1202 [n = 10]). The relationship between hematologic improvement and morphologic findings was also investigated.

Results: In 5 patients (E1201 [n = 3], E1202 [n = 2]), the bone marrow blast count increased after initiation of eltrombopag treatment compared with screening values. The blast count was less than 5%, and the increase in bone marrow blasts was transient in all 4 patients who had bone marrow examinations at follow-up. In 8 patients (E1201 [n = 5], E1202 [n = 3]), dysplastic forms of megakaryocytes were found in the bone marrow following treatment initiation. Dysmegakaryopoiesis of 10% or more was found in 3 patients. None of the patients revealed micromegakaryocytes. Ten patients showed an increase in bone marrow blasts and/or dysmegakaryopoiesis following treatment initiation. Nine of 10 patients showed hematologic improvement in 1 or more lineages.

Conclusions: Dysmegakaryopoiesis without micromegakaryocytes and a transient increase of less than 5% in bone marrow blast count may be signs of hematologic improvement with eltrombopag for patients with AA.

KEY POINTS

- Clonal evolution after eltrombopag treatment in patients with aplastic anemia (AA) is reported, but cytomorphologic changes in bone marrow during administration are unknown.
- Dysmegakaryopoiesis without micromegakaryocytes and a transient increase of <5% in bone marrow blast count may be a marker of hematologic improvement following eltrombopag treatment in patients with AA.
- Even if these findings are confirmed in AA patients receiving eltrombopag therapy, they may not be progressing to myeloid neoplasms.

KEY WORDS

Aplastic anemia; Blast count; Bone marrow; Eltrombopag; Hematologic improvement; Dysmegakaryopoiesis

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