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Letter to the Editor

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Acquired amegakaryocytic thrombocytopenia: a diagnostic pitfall in refractory thrombocytopenia

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Dear Editor,

Acquired amegakaryocytic thrombocytopenia (AAT) is a rare hematologic disorder characterized by severe isolated thrombocytopenia caused by a selective reduction or complete absence of bone marrow megakaryocytes while other hematopoietic lineages remain relatively preserved. Because its initial clinical presentation frequently overlaps with immune thrombocytopenia (ITP), delayed diagnosis and prolonged exposure to ineffective ITP-directed therapies are common in clinical practice. Early recognition is essential, as untreated AAT may progress to irreversible marrow failure or evolve into clonal hematologic disorders in refractory cases.[1]

A 21-year-old female presented with severe mucocutaneous bleeding and isolated thrombocytopenia. Initial peripheral blood smear confirmed marked thrombocytopenia without schistocytes or circulating blasts. Based on isolated thrombocytopenia, she was initially diagnosed with ITP and treated sequentially with corticosteroids, intravenous immunoglobulin, and eltrombopag over approximately two months. The patient had no previous exposure to immune checkpoint inhibitors, cytotoxic chemotherapy, or other known marrow-suppressive medications. Her treatment before the diagnosis of AAT consisted of corticosteroids, intravenous immunoglobulin, and eltrombopag, which were administered after the initial clinical diagnosis of ITP. However, no hematologic response was achieved, and the patient became entirely transfusion dependent for platelets and red blood cells. Given this refractory course, a bone marrow evaluation was performed. Bone marrow examination demonstrated a hypocellular marrow with marked megakaryocytic depletion and hypoplastic myeloid and erythroid compartments with preserved maturation. The myeloid-to-erythroid ratio was 64:4 (16:1), and blasts accounted for 2% of nucleated cells. No significant dysplasia was identified. Although the aspirate was limited for megakaryocyte assessment, the biopsy demonstrated marked megakaryocytic reduction, further supported by CD61/CD42b immunohistochemical staining. The marked and disproportionate depletion of megakaryocytes in the setting of severe thrombocytopenia argued against the initial diagnosis of ITP and supported AAT, further supported by CD61/CD42b immunohistochemical staining. Cytogenetics, FISH, and a comprehensive 141-gene myeloid NGS panel showed no clonal abnormalities. Comprehensive workup successfully excluded HIV, HBV, HCV, CMV, EBV, parvovirus B19, autoimmune disorders, thymoma, large granular lymphocytic leukemia, and PNH. The patient had no history of exposure to immune checkpoint inhibitors, cytotoxic chemotherapy, or other marrow-suppressive medications before the onset of thrombocytopenia. Thus, a drug-induced etiology was considered unlikely. Congenital etiologies were considered unlikely given the normal previous hematologic history and negative MPL mutation analysis.

The pathogenesis of AAT involves immune-mediated suppression of megakaryopoiesis and stem-cell dysfunction.[1] Distinguishing AAT from ITP is essential because management strategies differ substantially. Unlike ITP, AAT patients show limited responses to standard immunosuppressants or TPO receptor agonists. First-line cyclosporine or antithymocyte globulin yields variable responses, and many patients ultimately require allogeneic hematopoietic stem cell transplantation (HSCT).[2] HLA-matched siblings are traditionally preferred; however, recent evidence increasingly supports alternative strategies including haploidentical HSCT in selected amegakaryocytic settings.[3] Although the hypocellular marrow raised the differential diagnoses of hypoplastic myelodysplastic syndrome and early aplastic anemia, the absence of significant dysplasia, the low blast percentage (2%), the marked megakaryocytic depletion, and the absence of demonstrable clonal abnormalities favored AAT in the clinical context.

This case highlights the importance of timely recognition of severe, refractory AAT and the potential role of allogeneic HSCT as a definitive treatment in selected patients. Our

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patient remained profoundly thrombocytopenic, with severe refractory disease and no HLA-matched sibling donor. She underwent haploidentical allogeneic HSCT from her father on 28 April 2024. The available records did not contain sufficient information regarding the specific conditioning regimen and doses, neutrophil and platelet engraftment dates, or post-transplant donor chimerism; therefore, these parameters could not be reliably reported. Following transplantation, the patient achieved hematologic recovery and became transfusion independent. At the latest documented assessment, hemoglobin was 13.5 g/dl, platelet count was 175×10^3/μL, and white blood cell count was 7.61×10^3/μL. No graft-versus-host disease or other major transplant-related complication was documented. Persistent isolated thrombocytopenia unresponsive to standard ITP therapies should prompt timely bone marrow evaluation to avoid prolonged ineffective treatment and facilitate prompt access to definitive therapies including allogeneic HSCT.[4]

ETHICAL DECLARATIONS

Informed Consent

Written informed consent was obtained from the patient for the publication of this correspondence and any related clinical details.

Peer Review Process

This letter was externally peer-reviewed.

Conflict of Interest

The author declares no conflicts of interest.

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Author Contributions

The author is solely responsible for the conception, data collection, analysis, and writing of this manuscript.

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