Repurposed Antifibrinolytic Agents as a Substitute for Platelet Transfusions to Prevent Bleeding in Severe Thrombocytopenia Patients

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Technology description

Individuals with leukemia and bone marrow failure often develop thrombocytopenia, or severely low platelet count, as a result of the effects of their underlying disease or the effects of chemotherapy on the blood-forming cells in bone marrow. Thrombocytopenia is often diagnosed when a patient's platelet count is below 50,000, compared to the normal range of 150,000-450,000 platelets per microliter of blood. When platelet count falls below 50,000, bleeding complications can arise. Thrombocytopenia-associated bleeding remains a significant problem (up to 50% of diagnosed patients have bleeding complications). The standard of care for these complications is prophylactic platelet transfusion. However platelet transfusion is expensive and requires repeated weekly medical visits. A substitute standard of care that is at least equal and at best superior to platelet transfusions would be a significant advance in the medical field.

Technical Summary

Epsilon aminocaproic (EACA) inhibits proteolytic enzymes that are responsible for fibrinolysis, the process that prevents blood clots from growing and becoming problematic. EACA is an orally bioavailable antifibrinolytic agent commonly used and FDA approved for hemorrhage patients with congenital bleeding disorders. In fact, EACA has been used as an antifibrinolytic agent for more than 50 years.

Emory researchers administered prophylactic EACA to patients with very severe and chronic thrombocytopenia resulting from hematological malignancies like lymphoma and leukemia. While on EACA, 59% of patients did not experience any bleeding episode and 25% had minor bleeding episodes that resolved themselves and did not require platelet transfusion. Platelets were transfused to only 16% of the total patients due to bleed episodes. EACA was well-tolerated and venous thrombosis was not observed. The results of this preliminary study suggest that EACA is safe, effective, and more cost-efficient compared to prophylactic platelet transfusions to prevent bleeding and manage thrombocytopenia.

Application area

Prophylactic administration of an antifibrinolytic agent, epsilon aminocaproic acid (EACA), to prevent bleeding in patients with thrombocytopenia from hematological malignancies.

Advantages

Orally bioavailable, repurposed drug.
Commonly used and FDA approved to manage hemorrhage in patients with congenital bleeding.
Cheaper and easier to administer to patients compared to platelet replacement therapy.
Results from retrospective human study suggest it is safe and effective for patients with very severe and chronic thrombocytopenia.

Institution

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