Targeting ABCG4 and other cholesterol transport proteins for treatment of thrombocytopenia

Immune thrombocytopenic purpura (ITP) is a disorder characterized by reduced platelet production and platelet destruction. Currently, treatments for ITP do not address the underlying cause of the disease. Inhibiting the activity of a cholesterol transport pathway could significantly enhance the production of platelets and alleviate the symptoms of the ITP. This technology identifies ABCG4, a protein involved in cholesterol transport, as a potential therapeutic target for ITP. Inhibition of the cholesterol transport protein, ABCG4, has been demonstrated to be able to increase platelet count to alleviate the symptoms of ITP. This technology also identifies two other proteins—Lyn kinase and c-CBL—as potential therapeutic targets for ITP.

Targeting cholesterol transport pathway can be combined with current therapeutics to increase platelet counts in ITP patients

Signaling partners downstream of ABCG4 can also be targeted to treat ITP, increasing the chances for the development of a successful platelet-increasing therapeutic. These multiple targets increase the number of small molecule drugs that can be used to target this disease. Data from a mouse model of ITP demonstrates that antagonizing ABCG4 enhances platelet counts. Furthermore, combining ABCG4 inhibition with currently used therapeutics, such as thrombopoietin (TPO), markedly increases platelet counts. Such results highlight a promising pathway for developing a novel and more effective method of ITP treatment than those that currently exist.

Lead Inventor:
Alan Tall, M.D.

Applications:
- Increase the platelet count of patients with ITP
- Used in combination with current therapies to improve efficacy and reduce off-target effects
• Applied to any disease in which there is low platelet counts
• Applied to any disease in which there is low megakaryocyte progenitor cell proliferation
• Other downstream proteins can potentially be targeted to achieve similar results

Advantages:
• Significantly increases platelet count
• Can be combined with thrombopoietin (TPO) treatment to markedly increase platelet count
• Reduces the amount of required time for therapeutic intervention
• Identifies at least three proteins in a cholesterol transport pathway that can be targeted

Patent Information:
Patent Pending (US 20160067301)
Tech Ventures Reference: IR CU13265

Related Publications:

Inventors

Alan Richard Tall