

Livin for formation of platelets (Hadasit) code: 9-2014-248 Dina Ben Yehuda, Hadassah Ein Kerem, Hematology

Need:

Numerous conditions and treatments feature decreased platelet (thrombocyte) count with consequent bruising, bleeding & risk of hemorrhage. These include Thrombocytopenic purpura, ITP, Gaucher's disease, Aplastic anemia, Fetomaternal alloimmune thrombocytopenia, Hemolytic-uremic syndrome, chemotherapy and transfusions.

Therapies for disorders with reduced platelet count or platelet dysfunction include chemotherapy and transfusions that result in thrombocytopenia. These conditions can be life-threatening in certain circumstances if not treated promptly and effectively, and such subjects have a low quality of life due to their vulnerabilities to bruising and bleeding. Large numbers of subjects suffer from such a need either acutely or chronically, and would benefit from an effective therapy.

Invention:

Use of the protein Livin and its derivatives as an activator of thrombopoiesis for ex-vivo treatment of blood from thrombocytopenic subjects.

Findings:

Livin is a member of the Inhibitor of Apoptosis Proteins (IAP) family of intracellular anti-apoptotic proteins that acts by binding and inhibiting caspases. Livin or its proteolytic product tLivin can stimulate linage-specific stem cells and megakaryocytes (platelet precursor cells) to thrombopoiesis and the increased production of platelets. This induction can be accomplished in vivo or ex vivo, with ex vivo being preferred for reasons of safety.

Competitive advantage:

Livin is a novel potential ex-vivo therapeutic for thrombocytopenic subjects with a large unmet clinical need, to assist patients in need for platelet donations to maintain homeostasis, and to avoid risk of bruising, bleeding or hemorrhage. This ex-vivo method is expected to be relatively safe. Alternative available treatments (corticosteroids, IVIG, splenectomy, TPO and its mimetics) can be non-specific, unwieldy or expensive. The most direct replacement method using whole blood or platelet transfusion can cause immunologic platelet destruction.

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