Description
Inventors discovered a novel use for platelets as a protein expression vehicle for the delivery of proteins which are not well tolerated by the immune system. Like a shell game at a carnival, platelets can be used as the shell to hide proteins of interest from the immune system. The discovery is a lentiviral based gene therapy that has been tested using clotting factors in mice lacking these proteins. Not only do the animals resolve their bleeding phenotype, but they also do not develop an immune reaction, as control animals do to the immunogenic protein. The platelets help cloak or mask the target protein from the immune system. The invention covers both a permanent gene therapy approach where stem cells are transduced with a protein of interest and a transient cell therapy approach where transduced stem cells are driven \textit{ex vivo} to differentiate into platelets and the platelets transfused.

Potential uses
Conditions to target include diseases where a specific protein or set of proteins is missing or for conditions where auto-antibody formation is a serious complication. This approach may be useful when establishing immune tolerance is critical, such as in patients with severe allergies, auto-immune diseases, specific protein deficiencies and in transplant recipients. Conditions include: Bernard Soulier Syndrome, achondroplasia, lysosomal storage diseases, sickle cell disease, Coeliac disease, diabetes, lupus, Sjogren’s syndrome, Churg-Strauss Syndrome, Hashimoto’s thyroiditis, Graves’ disease, idiopathic thrombocytopenia purpura, arthritis, allergies, solid organ transplantation, and bone marrow transplantation.

Benefits
- Can use either a transient cell therapy approach or a permanent gene therapy approach
- Induction of immune tolerance to proteins that were previously immunogenic
- Gene therapy approach would correct deficiency
- Using a cell type that circulates throughout the body as a natural delivery system
- Delivery using platelets, a short lived cell, may offer safety advantages

Publications
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