Title: Novel Therapies for Hemophilia

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Medical Student Research Project

Hemophilia is the X-linked bleeding disorder caused by mutations in coagulation factor VIII or IX. Patients with severe disease require frequent intravenous injections of clotting factor concentrate. My lab is developing gene therapies for hemophilia based on adeno-associated viral (AAV) gene transfer. Furthermore, we are developing immune tolerance protocols to prevent immune-mediated rejection of gene or protein replacement therapies in hemophilia. These protocols are based on in vivo induction of regulatory T cells (Treg) using drugs, cell transplant of expanded Treg, specific tolerogenic routes of gene transfer, and on oral tolerance. Medical students will participate in coagulation studies, experiments to measure humoral immune responses (such as ELISA), and analyses of cellular immune responses using flow cytometry, tissue culture, RT-PCR, and immunohistochemistry techniques. The students may also learn viral vector production techniques. Funding for the project is provided by the National Heart Lung and Blood Institute and by the National Institute of Allergy and Infectious Diseases.

