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Title: Hematopoietic Stem Cell Gene Therapy

#### Abstract

Transplantation of hematopoietic stem cells (HSCs) or bone marrow cells has long been an effective therapy in the clinic for various hematologic malignancies, as well as disorders of blood cell, bone marrow, and immune function. More recently, HSC gene therapy has been explored as a promising treatment for a number of inherited diseases, including severe combined immunodeficiency and adrenoleukodystrophy. In this talk, I will present data from our studies on the application of HSC gene therapy to some common human diseases, with particular emphasis on efforts to develop a novel neuroprotective and disease-modifying therapy for Parkinson's disease.