The Past, Present and Future of Hematopoietic Stem Cell Gene Therapies

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The first human clinical trial of hematopoietic cell directed gene therapy commenced over 35 years ago, and ten years later, the first evidence of unequivocal clinical improvement was reported in X-linked severe combined immunodeficiencies. However, the long road forward to regulatory approval and broader availability of hematopoietic stem cell gene therapies in the past three years has been filled with obstacles as well as revolutionary advances. This talk will discuss a number of both hurdles and solutions, including evolution of integrating vectors and issues surrounding genotoxicity, the targeted gene editing revolution, the importance of predictive animal models, and current considerations in delivering gene therapies to patients, incorporating both the “big picture” and my research group’s contributions.