Technology Ventures Office

Beth Israel Deaconess Medical Center

Harvard Medical School Teaching Affiliate

Stem Cell Therapy for Basement Membrane Diseases

BIDMC #905

Background

Certain kidney diseases result from defects in the glomerular basement membranes (GBM), crucial to proper kidney function. Alport's Syndrome, for example, is an inherited disease of kidney inflammation (nephritis) resulting from a mutation in collagen, a key GBM component. Treatment options for diseases with impaired GBM are dialysis or renal transplantation. While these treatments may increase a patient's survival, quality of life remains poor.

Invention

A novel therapy to prevent or repair the damaged GBM by administering stem cells which then become kidney epithelial cells and provide normal GBM components, such as collagen.

Stage of Development

Pre-clinical studies with stem cells transplanted into a mouse model of Alport Syndrome:

- Restored kidney function as a measure of urine albumin excretion
- Restored kidney cell morphology
- Inhibited progression of tubular damage & fibrosis
- Attenuated kidney disease & decreased proteinuria
- Stem cells differentiated into kidney cells & expressed GBM proteins

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Market / Commercialization

This technology is broadly applicable to diseases which exhibit other basement membrane defects, beyond GBM, including:

- Knoblach Syndrome
- Hematuria
- Epidermolysis bullosa
- Diabetic Nephropathy

➢ Opportunities for Licensing Available

Lead Investigator

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Patent / Publication

➢ US National Phase Pending

➢ "Bone-marrow-derived stem cells repair basement membrane collagen defects and reverse genetic kidney disease"
PNAS May 9, 2006 (103), 19, pgs 7321-7326

Competitive Advantages:

- ✓ **Stem cells can be administered into the blood stream or by bone marrow transplant**
- ✓ **Eliminate the need for dialysis**
- ✓ **Only a small percentage of cells are needed to provide the proper GBM components**