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## **STEM CELLS OFFER NEW HOPE FOR KIDNEY DISEASE PATIENTS**

*For Alport Syndrome, Several Cell-Based Therapies Show Promise*

**Washington, DC (October 13, 2009)** — Several cell-based therapy approaches could provide new treatments for patients with Alport syndrome, reports an upcoming paper in the *Journal of the American Society of Nephrology* (JASN). "Our study opens up many considerations of how new therapies related to the use of stem cells can be devised for our kidney patients with chronic disease," comments Raghu Kalluri, MD, PhD (Harvard Medical School, Boston, MA).

Led by Valerie LeBleu, PhD (also of Harvard Medical School), the researchers tested various types of cell-based therapy in mice with a gene defect similar to that causing Alport syndrome, a genetic kidney disease. Most often occurring in boys, Alport syndrome causes progressive kidney disease leading to kidney failure at a young age. Patients may develop hearing loss and eye disease as well. Although treatment can slow the progression of kidney disease, there is currently no cure for Alport syndrome.

The experiments provide evidence that stem cell treatments could repair the kidney defects associated with Alport syndrome. "We found that stem cells derived from adult bone marrow are equally useful as embryonic stem cells," says Kalluri. "This will make it easier to translate these scientific discoveries to a treatment protocol for patients with Alport syndrome."

Transplantation of bone marrow from unaffected animals significantly improved kidney function in mice in the late stages of disease. Importantly, the results suggested that the beneficial effects of bone marrow transplantation may be achieved without the need for radiation.

Simple blood transfusion from healthy mice also achieved dramatic improvements in kidney function and survival in the mice with end-stage kidney disease. "Bone marrow transplant and blood transfusion protocols have already been approved for previous clinical use in patients with end-stage kidney disease and Alport syndrome," says Kalluri. "Therefore, clinical application of this procedure is quite feasible and may help our patients immediately." Although the results in mice are promising, real

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effectiveness can only be tested in human clinical trials. Kalluri adds, "Our study is an important step towards that goal."

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The study entitled, "Stem Cell Therapies Benefit Alport Syndrome," will appear online at <http://jasn.asnjournals.org/> on October 15, 2009 5:00 PM ET, doi 10.1681/ASN.2009010123.

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