

## Stem cell model for hereditary disease developed

26 October 2012 | News | By BioSpectrum Bureau

### Stem cell model for hereditary disease developed



**Singapore:** Scientists at the University of Maryland School of Medicine have developed a new method of using adult stem cells as a model for the hereditary condition, Gaucher disease. This development could help accelerate the discovery of new and effective therapies for the disease and similar other conditions such as Parkinson's.

The scientists reprogrammed stem cells to help them develop into cells that are genetically similar and react to drugs in a similar way as cells from patients with Gaucher disease. The stem cells will allow the scientists to test potential new therapies in a dish, accelerating the process toward drug discovery. The research has been published online in the journal the Proceedings of the National Academy of Sciences (PNAS).

"We have created a model for all three types of Gaucher disease, and used stem cell-based tests to evaluate the effectiveness of therapies," said Dr Ricardo Feldman., senior author of the paper and associate professor, microbiology and immunology, University of Maryland School of Medicine.

Dr Feldman, who is also a research scientist at the University of Maryland Center for Stem Cell Biology and Regenerative Medicine, said that, "Our findings have potential to help patients with other neurodegenerative diseases as well. For example, about 10 percent of Parkinson's disease patients carry mutations in the recessive gene for Gaucher disease, making our research possibly significant for Parkinson's disease as well."

The study was funded with \$1.7 million in grants from the Maryland Stem Cell Research Fund. Scientists received a start-up

grant for \$200,000 in 2007 and a larger, five-year grant for \$1.5 million in 2009.