



G E N E T I X  
P H A R M A C E U T I C A L S , I N C .

**FOR IMMEDIATE RELEASE:**

**GENETIX PHARMACEUTICALS' ADRENOLEUKODYSTROPHY (ALD) PRODUCT  
RECOGNIZED AS ONE OF *SCIENCE MAGAZINE'S* TOP 10 SCIENTIFIC  
BREAKTHROUGHS OF 2009**

*ALD Patients Featured in Science Article Now Living Healthy and Normal Lives  
Due to Groundbreaking Gene Therapy*

**Cambridge, MA – January 12, 2010** – Genetix Pharmaceuticals, a leader in gene therapy of somatic stem cells, announced today that *Science Magazine* has honored the ALD gene therapy research recently published by its collaborators at National Institute of Health and Medical Research (INSERM), placing it on their list of ten “Scientific Breakthroughs of 2009.” Genetix is developing this gene therapy product in collaboration with Professor Patrick Aubourg and Doctor Nathalie Cartier of INSERM, and planning to file an IND to expand the development in the US, as well as to expand the ongoing trial in France. The results spotlighted in the *Science* article show that this innovative therapy has been used to stabilize an otherwise devastating disease in two boys called adrenoleukodystrophy (ALD), or Lorenzo’s Oil disease, in addition to providing a safer alternative to previous therapies.

Adrenoleukodystrophy (ALD) affects 1 in 17,000 males, resulting in severe cognitive and neurologic deficits and eventually leading to death if untreated. It currently is treated with allogeneic bone marrow transplantation, often leading to further complications for the afflicted. In 50% of the patients who survive, graft-versus-host disease or delayed hematological reconstitution delay the time at which disease is stabilized, allowing the disease to have progressed further before stabilization.

Alfred Slanetz, Chief Executive Officer of Genetix, commented, “We are excited and honored that *Science Magazine* has recognized the ALD gene therapy product. We look forward to further developing this cutting-edge gene therapy in our own clinical trials, and bringing along a therapy that may be curative to this devastating and often terminal disease. I too would like to acknowledge the pioneering and steadfast work of Patrick Aubourg and Doctor Nathalie Cartier, with whom we will continue to work closely as our research and development progresses.”

To view the *Science* article that recognizes the work of Genetix and INSERM, please click on the following link. <http://www.sciencemag.org/cgi/content/full/326/5960/1600>

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**About Genetix Pharmaceuticals**

Genetix is the leading global gene therapy company developing curative therapies for genetic diseases. The company's proprietary platform treats the cause of genetic diseases by placing a healthy gene into the patient's extracted bone marrow stem cells, and transplanting these corrected stem cells back into the patient. Genetix's lead clinical product is Lenti-D™ for Adrenoleukodystrophy (ALD), also known as Lorenzo's Oil disease, which is now in clinical development. The results of the ongoing ALD trial, based upon work conducted by the National Institute of Health and Medical Research (INSERM), were named to *Science Magazine's* "Scientific Breakthroughs of 2009." Genetix has in-licensed the technology from INSERM, and plans to file for pivotal studies in 2010 in both the U.S. and Europe. In addition to ALD, Genetix is conducting Phase I/II trials with its LentiGlobin™ product in Thalassemia/Sickle Cell Disease, the two most prevalent human genetic disorders. To date, all patients receiving the full therapy with both products have been cured and are healthy. The company's proprietary stem cell processing, GMP manufacturing and gene therapy vector technologies are also applicable to treating other genetic diseases moving forward. Genetix is privately financed by an experienced syndicate of healthcare investors, including TVM Capital, Forbion Capital Partners and Easton Capital. For more information, please visit <http://www.genetixpharm.com/>.

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