New Data From Adrenoleukodystrophy (ALD) Pilot Study Presented at American Society of Gene and Cell Therapy Show Three-Year Disease Stabilization of Patients

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NEW DATA FROM ADRENOLEUKODYSTROPHY (ALD) PILOT STUDY PRESENTED AT AMERICAN SOCIETY OF GENE AND CELL THERAPY SHOW THREE-YEAR DISEASE STABILIZATION OF PATIENTS

Cambridge, Mass. and Washington, D.C. – May 20, 2010 – Nathalie Cartier-Lacave, M.D., study investigator and a director of research at the National Institute of Health and Medical Research in France (INSERM), and collaborator of Genetix Pharmaceuticals, Inc., today presented additional data from a pilot study treating X-linked adrenoleukodystrophy (ALD) at the American Society of Gene and Cell Therapy's 13th Annual Meeting. The findings demonstrate continued stable expression of ALD protein and disease stabilization at three years in two ALD patients. Initial data on a third patient show stable expression of the protein at 20 months; however, current data are insufficient to determine disease stabilization. Genetix, an emerging leader in developing breakthrough gene therapies for severe genetic disorders, plans to initiate larger clinical studies in ALD in both the United States and Europe in 2011.

"Although early, these follow-up data on the ALD patients are promising and we congratulate our colleagues at the INSERM," said Mitchell Finer, Ph.D., chief scientific officer of Genetix Pharmaceuticals, Inc. "These data continue to highlight the potential of the Genetix lentiviral product platform as an important, one-time therapy in treating a broad range of severe genetic disorders. We are excited to further investigate these results in larger clinical trials."

ALD is a rare, inherited neurological disorder that, in its most severe form, causes damage to the myelin sheath (an insulating membrane that surrounds nerve cells in the brain) and progressive dysfunction of the adrenal glands. ALD affects one in every 17,900 boys worldwide. In the cerebral childhood-onset form, symptoms usually occur between the ages of 4 and 10. Boys afflicted with this form of ALD develop normally until the onset of symptoms. The symptoms of this disorder progress often rapidly and, in a matter of years, can lead to a vegetative state and ultimately death. Current treatment options are limited to allogeneic bone marrow transplantation when there is an appropriate donor, which often leads to further complications for the afflicted.

In this pilot study, blood stem cells were removed from the patients and genetically modified in the lab using a lentiviral vector to introduce a working copy of the ALD gene into the cells. The patients' modified cells were then infused back into the patients after receiving a treatment to allow engraftment of the corrected cells. Initial results from this study published in the November 2009 issue of the journal *Science* demonstrated disease stabilization and no further disease progression in two patients monitored for two years. Study results presented today were based on continued follow-up of the first two patients as well as 20-month data on a third patient. The new data show that at three years the two patients' functional ALD proteins remain stably expressed at 10-15 percent of circulating monocytes. Both continue to show neurological stabilization with no biological adverse effects to date. In the third patient, functional ALD protein remains stably expressed at 20 months. New findings demonstrate the third patient's cognitive functioning within normal range at 16 months. However, current data are not yet sufficient to determine that there has been disease stabilization. All patients have random integration of the ALD gene with polyclonal hematopoiesis.

"Genetix remains committed to building our unique product platform to develop breakthrough gene therapy treatments for patients with severe genetic disorders," said Nick Leschly, interim president of Genetix Pharmaceuticals, Inc. and partner of Third Rock Ventures. "What's exciting to the Genetix team is to be at the forefront of the maturation of gene therapy as a powerful therapeutic modality that can help patients with a class of severe genetic disorders lacking any safe or widely available treatment options."

About Lenti-D™

Genetix's lead clinical product in development is Lenti-D™ for ALD. The results of the ongoing ALD trial, based upon work conducted by INSERM, were named as one of the "Scientific Breakthroughs of 2009" by the journal *Science*. Genetix is also conducting Phase I/II trials with its LentiGlobin™ product in beta-Thalassemia, a rare blood disorder that reduces the production of hemoglobin or the iron-containing protein in red blood cells that carries oxygen to cells throughout the body leading to a lack of oxygen in many parts of the body.

About Genetix Pharmaceuticals, Inc.

Genetix Pharmaceuticals, Inc. is an emerging leader in treatments for severe genetic disorders based on a breakthrough gene therapy platform that uses a patient's own genes to treat his/her disorder. Taking stem cells from a patient's own bone marrow, Genetix uses a proprietary lentiviral technology to modify the genes and deliver corrective stem cells back into the patient, providing the potential for a one-time transformative therapy. Current treatment of severe genetic disorders relies solely on transplanting donor stem cells, when there is an appropriate donor, and can lead to graft versus host disease or disease progression before gene stabilization. Genetix's novel approach using a patient's own stem cells represents a true paradigm shift in the treatment of severe genetic diseases as it eliminates the potential complications associated with donor cell transplantation. Genetix is privately held and backed by top-tier life sciences investors, including Third Rock Ventures, TVM Capital, Forbion Capital Partners, Easton Capital and Genzyme Ventures. Its operations are located in Cambridge, Mass. and Paris, France. For more information, please visit http://www.genetixpharm.com/.

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