

# **FOR IMMEDIATE RELEASE**

## **GENETIX PHARMACEUTICALS SECURES \$35M IN SERIES B FUNDING**

Company Focused On Advancing Breakthrough Clinical Programs in Gene Therapy

**Cambridge, MA – March 12, 2010 –** Genetix Pharmaceuticals, a leading gene therapy company developing breakthrough treatments for severe genetic disorders, announced today that it has completed a \$35 million Series B financing with new investors Third Rock Ventures and Genzyme Ventures joining TVM Capital, Forbion and Easton Capital. Proceeds from the financing will be used to advance current clinical programs, strengthen platform capabilities and further expand the team.

"The Genetix platform represents the convergence of the powerful therapeutic modality of gene therapy with a class of severe genetic disorders lacking safe or widely available treatment options," said Nick Leschly, Interim President of Genetix and Partner of Third Rock Ventures. "Genetix not only has a compelling value proposition, but also holds great potential to make a difference for patients. We are very encouraged by the promising clinical data in both Adrenoleukodystrophy (ALD) and beta-Thalassemia showing arrested disease progression in a small number of patients treated to date, and by the opportunity to build a best-in-class company in the field of gene therapy for severe diseases."

"Genetix is making important progress in its gene therapy clinical development programs," said Alan Walts, Managing Director of Genzyme Ventures. "We look forward to joining the company's Board, supporting its growth, and bringing to bear our experience in rare genetic diseases."

## **Gene Therapy Resurgence**

Over the last few years, a small but growing number of gene therapy trials have yielded increasingly promising results. Genetix's proprietary lentiviral technology delivers corrective genes to the patient's own bone marrow cells, providing a one-time effective therapy, a true paradigm shift in the treatment of genetic diseases. Genetix has programs in clinical development in Adrenoleukodystrophy (ALD), a severe neurodegenerative disorder, and beta-Thalassemia, one of the most prevalent human genetic disorders. Both of these programs have shown strong early results to date and stabilization of their respective diseases. Results from the ALD program have been published in a November 2009 issue of *Science*. To view the article, please click on the following link:

http://www.sciencemag.org/cgi/content/abstract/326/5954/818

"Having been in the gene therapy field for over 25 years, it is great to see the new groundswell of excitement from the scientific community and the pharmaceutical industry, reflecting both technology advances and early, but exciting, clinical data," said Mitchell Finer, Ph.D., Chief Scientific Officer of Genetix Pharmaceuticals. "This financing puts us in a great position to expand our team, accelerate the development of our later stage products and further build our unique gene therapy platform so we can pursue our vision of delivering novel, breakthrough treatments for patients."

## **Experienced Team**

Genetix Pharmaceuticals is led by an experienced team with a proven track record of building successful life sciences companies. The new members of the management team include Nick Leschly from Third Rock Ventures as Interim President; Mitchell Finer, Ph.D. as Chief Scientific Officer; Phil Reilly, M.D. from Third Rock Ventures as Interim Chief Medical Officer; and Anup Arora from Easton Capital, who is continuing to lead the business development and operations activities on an interim basis. In parallel with the financing, the current Chief Executive Officer, Alfred Slanetz, Ph.D. will depart from the company in order to pursue other opportunities in the biotech field. Dr. Slanetz has been a key contributor at Genetix and a driver of the Company's accomplishments over the past few years. In addition, the current VP of Corporate Development and Co-Founder, Ron Dorazio, M.D. will transition into a strategic advisor role for the company. Dr. Dorazio's contributions have been fundamental since the inception of the company both scientifically and strategically.

The new Board of Directors will consist of Jim Geraghty of Genzyme; Axel Polack, M.D. of TVM Capital; Geert-Jan Mulder, M.D. of Forbion and Nick Leschly of Third Rock Ventures. The Scientific Advisory Board will continue to be led by Genetix founder Dr. Philippe Leboulch, along with Dr. Patrick Aubourg.

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## **About Genetix Pharmaceuticals**

Genetix Pharmaceuticals is a leading gene therapy company developing breakthrough treatments for severe genetic disorders. Its products treat the underlying cause of genetically based diseases by delivering corrective genes to the patient's own bone marrow, providing the potential for a one-time transformative treatment. Genetix's lead clinical product in development is Lenti-D™ for Adrenoleukodystrophy (ALD), also known as Lorenzo's Oil disease. 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." In addition to ALD, Genetix is conducting Phase I/II trials with its LentiGlobin™ product in beta-Thalassemia. To date, all patients receiving the full therapy with both products have shown disease arrest. In addition to Genetix's lead programs, the company's proprietary stem cell processing, GMP manufacturing and gene therapy vector technologies are applicable to treating other genetic diseases, opening the door to the development of additional therapeutics.

Genetix is privately financed by an experienced syndicate of healthcare investors, including Third Rock Ventures, TVM Capital, Forbion Capital Partners, Easton Capital and Genzyme Ventures. Its operations are located in Cambridge, MA and Paris, France. For more information, please visit <a href="http://www.genetixpharm.com/">http://www.genetixpharm.com/</a>.

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