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## **Penn Medicine presents HIV gene therapy trial data at CROI 2009**

PHILADELPHIA – Researchers from Penn Medicine announced today the results from an ongoing Phase I/II open label clinical trial of VRX496 at the 16th Conference on Retroviruses and Opportunistic Infections (CROI) in Montreal. VRX496 is a gene therapy product being investigated for the treatment of HIV infection. During the trial VRX496 was administered to twelve subjects prior to a controlled scheduled treatment interruption (STI) from their current antiretroviral therapy.

"We continue to see the strong safety and tolerability profile that VRX496 demonstrated in the Phase I clinical trials," said Carl June, MD, Professor of Pathology and Laboratory Medicine at Penn. "What we are seeing now is the potential to generate a sustained immune system response in patients who have stopped taking their anti-retroviral regimens. Potentially this could lead to a shift in the treatment of HIV infection from trying to eradicate the HIV virus; which requires a daily regimen of pills and is ultimately a short-term solution because of the ability of HIV to mutate, to an approach where the body acquires immunity to HIV infection through a series of infusions."

The presentation "Safety, Antiviral Effect and Quantitative Measurement of Modified CD4+ T Cells Trafficking to Gut Associated Lymphoid Tissue in a Phase I/II Open-label Clinical Trial Evaluative Multiple Infusions of Lentiviral Vector-modified CD4+ T Cells Expressing Long env Antisense" was made by Pablo Tebas, MD, at CROI 2009 on February 10th at 10 AM. Tebas is an Assistant Professor of Medicine at the University of Pennsylvania School of Medicine.

"Multiple infusions of lentiviral-vector-modified cells appear to increase CD4+ counts and may decrease viral load set point. We appear to have successfully reconstituted the CD4+ cell population in the gut with VRX496 modified cells, which is a major site of HIV replication and suggests that the modified cells are going where they need to go," said Pablo Tebas MD, Assistant Professor, University of Pennsylvania School of Medicine. "The multiple infusions of lentiviral vector VRX496 with long antisense RNA appear to be safe, and no serious adverse events were noted."

The results presented at CROI are from an ongoing Phase I/II trial of HIV-1 infected subjects fully suppressed on Anti-Retroviral Treatments (ART). Patients received up to six infusions over 14 weeks. At week 18 subjects interrupted their ART. Patients were monitored for adverse events, viral load, CD4+ T cell count, vector-derived replication competent lentivirus (RCL) and immunogenicity, vector DNA integration patterns, and TCR diversity.

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Ronald Collman MD, Farida Shaheen PhD, Jean Boyer PhD, Gwendolyn Binder PhD, Larisa Zifchak RN, Faten Aberra MD, Bruce Levine PhD, Pablo Tebas MD, Carl June MD and Gerard McGarrity PhD are all co-authors of this study. The study was funded by the NIH, NIAID. Dr. June is not affiliated with VIRxSYS in any capacity beyond his role as a clinical collaborator on this project. Gerard McGarrity is an employee of VIRxSYS Corporation. VIRxSYS is commercializing VRX496. For more information about VIRxSYS please contact Russell LaMontagne, [russell@corinthgroup.com](mailto:russell@corinthgroup.com), 917.744.7957.

### **About VRX496**

VRX496 is an investigational gene therapy for the treatment of HIV/AIDS. Current therapies for HIV-infected patients require daily drug regimens and have well documented side effects. It is anticipated that VRX496 will only require a minimal number of infusions. To date VRX496 has been infused in 61 patients, which represents an accumulative safety time period of 150 therapy years. VRX496 also is different from previous gene therapies because it uses a lentiviral vector derived from HIV-1 itself. Unlike other viral

vectors, lentiviral vectors appear to sustain expression of the delivered genes of interest for a longer period of time and do not appear to elicit an inflammatory immune response.

PENN Medicine is a \$3.6 billion enterprise dedicated to the related missions of medical education, biomedical research, and excellence in patient care. PENN Medicine consists of the University of Pennsylvania School of Medicine (founded in 1765 as the nation's first medical school) and the University of Pennsylvania Health System.

Penn's School of Medicine is currently ranked #4 in the nation in U.S. News & World Report's survey of top research-oriented medical schools; and, according to most recent data from the National Institutes of Health, received over \$379 million in NIH research funds in the 2006 fiscal year. Supporting 1,700 fulltime faculty and 700 students, the School of Medicine is recognized worldwide for its superior education and training of the next generation of physician-scientists and leaders of academic medicine.

The University of Pennsylvania Health System (UPHS) includes its flagship hospital, the Hospital of the University of Pennsylvania, rated one of the nation's top ten "Honor Roll" hospitals by U.S. News & World Report; Pennsylvania Hospital, the nation's first hospital; and Penn Presbyterian Medical Center. In addition UPHS includes a primary-care provider network; a faculty practice plan; home care, hospice, and nursing home; three multispecialty satellite facilities; as well as the Penn Medicine at Rittenhouse campus, which offers comprehensive inpatient rehabilitation facilities and outpatient services in multiple specialties.