



# C O P E R N I C U S T H E R A P E U T I C S , I N C .

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## **For Immediate Release**

### **Phase I/II Clinical Trial Shows Encouraging Results Using Copernicus' Non-Viral Gene Transfer System to Correct the Underlying Gene Defect in Cystic Fibrosis Subjects**

**Cleveland, OH, June 11, 2003** – Copernicus Therapeutics, Inc. presented data at the 6<sup>th</sup> Annual Meeting of the American Society of Gene Therapy showing that their compacted DNA formulation partially restored chloride channel function in the nasal epithelial cells of human subjects with cystic fibrosis (CF). No serious adverse events occurred in the trial, demonstrating that the compacted DNA nanoparticles could be safely administered to the nares of CF subjects. This Phase I/II clinical trial was a joint collaboration of Copernicus Therapeutics, Inc., University Hospitals of Cleveland (UHC), Case Western Reserve University (CWRU) School of Medicine, The Children's Hospital of Denver, and Cystic Fibrosis Foundation Therapeutics, Inc. (CFFTI), a nonprofit affiliate of the Cystic Fibrosis Foundation.

"Copernicus has developed a highly effective non-viral gene transfer formulation that permits the uptake of DNA drugs into lung airway epithelial cells," said Mark J. Cooper, M.D., Senior VP of Science and Medical Affairs at Copernicus. "Our recently completed clinical trial in CF subjects indicates that our DNA drug was safe and well tolerated when applied to the nasal mucosa, and encouraging gene transfer endpoints were observed. Two-thirds of the trial subjects demonstrated evidence of DNA expression, with partial correction of the underlying chloride channel defect that is responsible for CF disease manifestations. We are optimistic that our gene transfer formulation will effectively transfer the CF therapeutic gene to the lung cells of CF subjects in subsequent aerosol-based clinical trials."

Also presented at the ASGT meeting were results indicating that the Copernicus formulation of compacted DNA is stable upon storage at 4°C for well over one year. Given that a successful commercial gene therapy product must remain stable from production to patient administration, these results highlight that the Copernicus compacted DNA formulation has pharmaceutically-relevant properties important for continued commercial development.

"We are quite pleased with our clinical trial results and drug development programs," said Robert C. Moen, M.D., Ph.D., President and CEO of Copernicus. "Our non-viral gene transfer system appears to be safe as both animal and human studies have show no evidence for immune recognition of our complexes or the induction of a significant inflammatory response. We also are encouraged by the efficacy results seen in both animal and human studies. Combined with the storage stability data and other desirable properties of our DNA nanoparticles, these important safety and efficacy results suggest that our DNA drugs could be chronically

administered to treat diseases such as CF. Additional clinical trials will, of course, need to be conducted to ascertain the true potential of our approach in treating CF and other devastating diseases. Copernicus will be seeking additional financing and possible corporate partnerships to help us move these programs forward.”

*Copernicus Therapeutics, Inc., a privately held biotechnology company, is advancing novel targeting and delivery systems with broad applications in human therapeutics, DNA vaccines, and functional genomics. Copernicus' technologies include a targeting platform enabling the efficient uptake of drugs by specific cells and tissues, and a multi-component delivery platform that can be applied to nucleic acids to develop therapies for a variety of human diseases. The Company's targeting, delivery, and expression platforms are complementary and can be combined to enhance the efficacy and safety of existing drugs, to create novel therapeutics, and to speed up the drug discovery process. Additional information about Copernicus is available at <http://www.cgsys.com>*

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