



**For Immediate Release**

**CYSTIC FIBROSIS PATIENT RECEIVES FIRST-OF-ITS-KIND GENE TRANSFER**  
*Trial Tackles Root of Gene Defect*

**Cleveland, Ohio, April 3, 2002**—Copernicus Therapeutics, Inc., University Hospitals of Cleveland (UHC), Case Western Reserve University (CWRU) School of Medicine, and Cystic Fibrosis Foundation Therapeutics Inc. (CFFTI), a nonprofit affiliate of the Cystic Fibrosis Foundation, today announced that the first person in a 12 patient study has received a first-of-its-kind non-viral gene transfer vector in an effort to develop gene therapy for cystic fibrosis (CF). This study, being led by Dr. Michael Konstan, aims to use gene transfer technologies to provide a normal copy of the CF gene to correct the underlying defect. The mutations of this gene are the root cause of the disease. This trial is the first step in a long process toward developing a more effective therapy for individuals with CF.

“The CF Foundation’s mission is to assure the development of the means to cure and control cystic fibrosis and to improve the quality of life for those with the disease,” said Robert J. Beall, Ph.D., president and chief executive officer of the Cystic Fibrosis Foundation. “Copernicus is helping us realize this mission by tackling the root cause of CF, rather than only treating the symptoms. We will continue to work closely with Copernicus and are eagerly anticipating the results of this gene transfer trial and moving forward with this approach.”

Cystic fibrosis, which affects approximately 30,000 Americans, causes the body to produce an abnormally thick, sticky mucus, due to the faulty transport of sodium and chloride in cells lining organs such as the lungs and pancreas. Patients with CF develop recurrent lung infections and pulmonary damage and also have difficulty absorbing nutrients from food.

“Gene transfer is one of the most progressive technologies in medicine and healthcare today,” said Michael W. Konstan, M.D. Associate Professor of Pediatrics at CWRU and Director of the LeRoy Matthews Cystic Fibrosis Center, Rainbow Babies and Children’s Hospital/University Hospitals of Cleveland. “We are optimistic that this new non-viral approach will ultimately be an even more effective treatment for CF than those available today. We are excited as well that this study is being undertaken in Cleveland as it represents Cleveland’s emerging status as a leading center for the development of this and similar novel technologies.”

The new gene transfer vector was produced in Cleveland by Copernicus Therapeutics, Inc. whose unique, non-viral approach to deliver the CF gene to affected airway cells of CF patients may eventually provide a long-term treatment for this disease. Funding for this work was obtained from equity investors in Copernicus, as well as an award from CFFTI.

“This trial is the first step and a key indicator of the remarkable potential of Copernicus’ gene delivery technologies,” said Robert C. Moen, M.D., Ph.D., president and chief executive officer of Copernicus. “Copernicus’ approach is designed to bypass the immunological and inflammatory difficulties encountered by other CF gene transfer approaches. Researchers expect that the PLASmin™ compaction technology will enable the delivery and efficient uptake of functional CF genes into epithelial cells and result in a correction of the underlying defect. The initiation of the trial also is an example of how exciting technologies discovered at local academic centers can be transferred to a local biotechnology company for development into a potentially very valuable medical treatment.”

“In recent years, medical research has explored new applications of DNA-based therapeutics for the treatment of CF. For such DNA-based drugs to have a therapeutic effect, however, they must safely and effectively be transported into the nucleus of target cells,” said Mark J. Cooper, M.D., senior vice president of science and medical affairs, Copernicus Therapeutics. “Copernicus’ development of proprietary methods to produce compacted DNA nanoparticles containing only a single nucleic acid molecule puts us at the forefront of using DNA as a pharmaceutical.”

These DNA nanoparticles can much more efficiently cross the cell membrane and enter cell nuclei than DNA that has not been compacted. In addition, Copernicus has demonstrated that compacted DNA nanoparticles are effective in animal airway cells, can uniquely transfer DNA to non-dividing cells, can resist physical and enzymatic destruction, and can be adapted for use with a wide range of nucleic acids. Importantly, these nanoparticles have been shown to be safe, non-immunogenic, and non-toxic in animals, and chronic administration in people with CF may be possible without inciting toxic inflammatory or immune responses.

*Founded in 1843, the Case Western Reserve University School of Medicine is the largest medical research institution in Ohio and the 15th largest among the nation’s medical schools for research funding from the National Institutes of Health. Seven Nobel Laureates have been affiliated with the school. The School of Medicine is recognized throughout the international medical community for outstanding achievements in research, teaching and service. Annually, the School of Medicine trains more than 600 M.D. and M.D./Ph.D. students.*

*University Hospitals Health System (UHHS) is the region’s premier healthcare delivery system, serving patients at more than 150 locations throughout northern Ohio. The System’s 947-bed, tertiary medical center, University Hospitals of Cleveland (UHC), is the primary affiliate of Case Western Reserve University. Together, they form the largest center for biomedical research in the State of Ohio. The System provides the major clinical base for translational researchers at The Research Institute of University Hospitals of Cleveland, as well as a broad and well-characterized patient population for clinical trials involving the most advanced treatments. Included in UHC are Rainbow Babies & Children’s Hospital, among the nation’s best children’s hospitals; Ireland Cancer Center, northern Ohio’s only National Cancer Institute-designated Comprehensive Cancer Center (the nation’s highest designation); and MacDonald Women’s Hospital, Ohio’s only hospital for women.*

*Cystic Fibrosis Foundation Therapeutics, Inc. (CFFTI) is a non-profit affiliate of the Cystic Fibrosis Foundation that directs drug discovery, development and clinical evaluation of CF therapies. For more information on CF or the CF Foundation, call 1-800-FIGHT CF or visit [www.cff.org](http://www.cff.org).*

*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>.*

**Contacts:**

Robert C. Moen, M.D., Ph.D.  
President and CEO  
Copernicus Therapeutics, Inc.  
(216) 231-0227 ext. 26

Mark J. Cooper, M.D.  
Sr. V.P. of Science and Medical Affairs  
Copernicus Therapeutics, Inc.  
(216) 231-0227 ext. 23

George Stamatis  
Director of Public Affairs  
School of Medicine CWRU  
(216) 368-3635

Allison Tobin  
Director of Media Relations  
Cystic Fibrosis Foundation  
(301) 841-2665

Eileen Korey  
Director of News Services  
University Hospitals of Cleveland  
(216) 844-3825

*\*\* All media requests or questions for Dr.  
Konstan should be directed to Eileen Korey.*

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