



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 .

11000 Cedar Avenue, Suite 145
Cleveland, Ohio 44106-3052

Contact: Robert C. Moen, M.D., Ph.D.
President and CEO
(216) 231-0227
rmoen@cgsys.com

Mark J. Cooper, M.D.
Senior VP, Science and Medical Affairs
(216) 231-0227
mcooper@cgsys.com

For Immediate Release

Copernicus Reports Targeting of Liver Cells by Non-Viral Compacted DNA Complexes

Cleveland, Ohio, November 2, 2001 – Copernicus Therapeutics, Inc. announced that the size and shape of compacted DNA particles markedly influences the efficiency of targeted gene transfer to liver cells. As reported in *Journal of Biological Chemistry* (276:34379-34387, 2001), “Biological Properties of Poly-L-lysine-DNA Complexes Generated by Cooperative Binding of the Polycation,” receptor-mediated uptake in hepatoma cells only occurs when compacted DNA particles are spherical and are smaller than 30 nm.

Mark J. Cooper, M.D., Senior Vice President of Science and Medical Affairs said, “This manuscript provides a detailed correlation between the size and shape of compacted DNA complexes and successful targeting of liver cells expressing the asialoglycoprotein receptor. The key to facilitating receptor-mediated uptake and transgene expression is to compact single molecules of DNA using polycations; only unimolecularly condensed complexes can achieve the critical size requirements necessary for efficient cellular uptake and transgene expression. Single molecules of plasmid DNA can be compacted by several formulation methods, and cooperative binding of DNA and polylysine can achieve unimolecular DNA compaction, as reported in this manuscript. There is a growing appreciation that the optimal formulation of compacted DNA complexes varies according to the site of administration *in vivo*, although unimolecular condensation is an absolute requirement for biologically significant gene transfer.”

“Copernicus has established platform gene transfer and expression technologies that are effective and safe,” said Robert C. Moen, M.D., Ph.D., President and CEO of Copernicus. “Compaction of single molecules of DNA with polycations produces single genome equivalents of DNA that are stable in serum, permit targeting and uptake by specific cell types, traffic effectively in the cell, and cross the intact nuclear membrane. The modular design of our technology gives us the flexibility to codevelop gene therapies for a variety of clinical indications, including hemophilia and cystic fibrosis. We expect to broadly partner this delivery platform with other pharmaceutical and biotechnology companies wishing to exploit the advantages of our proprietary technology.”

Copernicus Therapeutics, Inc. is advancing novel targeting and delivery systems with broad applications in human therapeutics and vaccines. Copernicus’ technologies include a multi-component delivery platform that can be applied to nucleic acids to develop therapies for a variety of human diseases and a targeting platform enabling the efficient uptake of drugs by specific cells and tissues. The Company’s targeting and delivery platforms are complementary and can be combined to enhance the efficacy and safety of existing drugs or to create novel therapeutics.

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