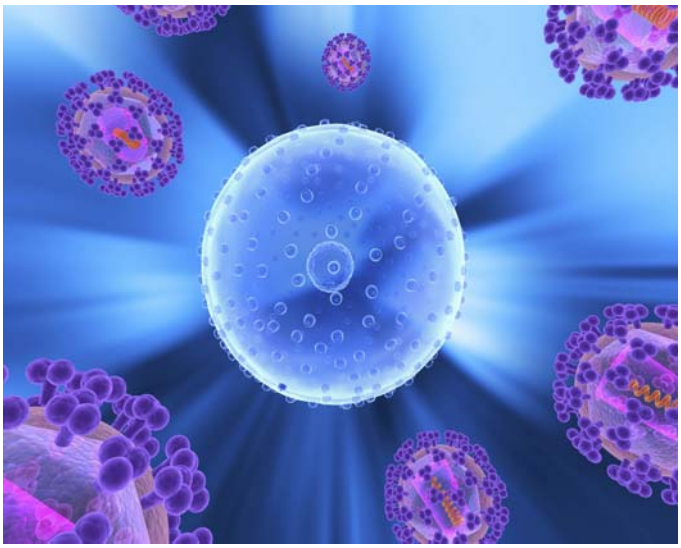
Intellectual Property
(Non-confidential)

City of Hope
NATIONAL MEDICAL CENTER AND
BECKMAN RESEARCH INSTITUTE

Specific siRNA Therapeutic System for the Treatment of HIV-1



DESCRIPTION

Current therapeutic strategies designed to combat HIV-1/AIDS are limited by viral drug resistance and toxicity issues, thus highlighting the need for new approaches to combat this disease. This technology is a novel combinatorial approach for the treatment of HIV-1 infection in which an RNA aptamer with high binding affinity to the HIV-1 envelope glycoprotein (gp120) is covalently linked to a small interfering RNA (siRNA) that triggers sequence specific degradation of HIV-1 RNAs. HIV-1-infected cells display gp120 on their cell surface, which makes the anti-gp120 aptamer an effective siRNA delivery vehicle to HIV-1-infected cells. Thus, the combined aptamer-siRNA approach is an attractive, non-toxic therapeutic approach for treatment of HIV-1 infection. Additionally, multiple different aptamers can be employed to circumvent viral resistance and maximize treatment efficacy.

KEY ASPECTS

- Novel therapeutic approach that circumvents viral drug resistance in current HIV therapies
- Non-toxic molecules can specifically target HIV-1-infected cells for delivery of siRNA payload
- Multiple aptamers can be interchanged for versatility and maximal effectiveness
- Powerful data for this technology can be found in C.P. Neff & J.J. Rossi, et al., "An Aptamer-siRNA Chimera Suppresses HIV-1 Viral Loads and Protects from CD4-CD3 T Cell Decline in Humanized Mice" available in the next issue of *Science Translational Medicine* or upon request.

INTELLECTUAL PROPERTY

Title	US Patent Application	Filed
Cell-Type Specific Aptamer-siRNA Delivery System for HIV-1 Therapy	12/328,994	12/5/2008

CONTACT

Ryan Kelly, Ph.D.
Manager, Office of Technology Licensing
Telephone: (626) 471 9359 | Email: rykelly@coh.org