HUDSON INSTITUTE OF MEDICAL RESEARCH

COMMERCIALISATION OPPORTUNITY

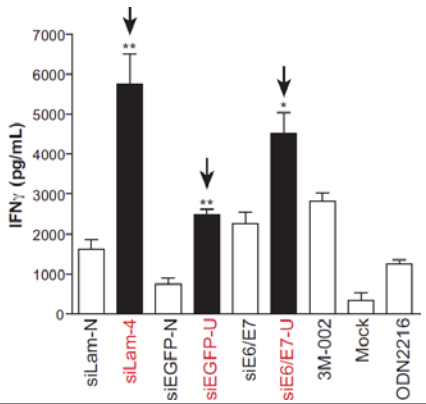
RNA interference with Potent Immune System Activation

Summary

Gene silencing therapy with RNA interference (RNAi) methods hold significant opportunities for many diseases, including cancer, heart disease, ophthalmology and viral infections. At least two rare disease gene therapy treatments have been recently approved in Europe.

Short-interfering RNAs (siRNAs) have the ability to specifically silence disease-causing genes. Prof B. Williams and Dr M. Gantier have rationally designed an siRNA motif modification that can be applied to any siRNA scaffold which potently stimulates the innate immune system, without impairing gene silencing capacity. Harnessing the immune system can increase the therapeutic potential of gene silencing treatment. Immunostimulatory siRNAs have enhanced therapeutic antiviral and antitumor functions. Multiple *in vivo* studies have now demonstrated that combining silencing of a cancer-related gene with immune stimulation, mediated by the same siRNA sequence, elicits therapeutic benefits such as decreased metastasis and inhibition of tumor growth. As many cancers are caused by specific, known gene mutations this approach is highly applicable for immuno-oncology.

Key Data



Proprietary immuno-stimulatory siRNA (red), increases interferon-γ production (and -α, and other cytokines) in human blood.

Advantages and research strengths

- Immunostimulatory motif can be applied to any siRNA construct
- Strong oncology and immunology expertise
- Range of disease models available

IP position

A US national phase patent has been granted (8,318,924) with priority date Nov 2012.

Market

This technology has multiple market applications, including:

1. **RNAi immuno-oncology** is a newly emerging field with strong interest from experienced life science investors. Many patients do not respond to current immuno-oncology treatments so alternative combination therapies are being investigated. The field of immuno-oncology has grown exponentially over recent years and potential combination treatments are in high demand.
2. Chronic **hepatitis B** affects approximately 400 million people worldwide. The virus infects liver cells and can lead to cirrhosis and liver cancer, resulting in more than 780,000 deaths annually. Currently, the standard of care for chronic hepatitis B infection is interferon-α treatment. Dual specific gene targeting and immune activation has great potential in treating chronic viral infections. siRNA can be designed to target potential viral infections and stimulate a therapeutic innate immune response.
3. Patented **modifications to siRNA** design can strengthen freedom to operate for gene therapy companies looking to enhance their gene therapy platform technology.

Publication

Rational design of immunostimulatory siRNAs. *Molecular Therapy*, 2010 Apr 18(4) 785-795, Gantier M. et al.

Opportunity

Hudson welcomes opportunities for co-investment or collaboration to further develop this project.

For further information

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