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A Chemistry Platform for Gene Silencing: Antisense and siRNA

Overview:

McGill University is seeking to outlicense a broad patent portfolio relating to a chemistry platform for gene silencing and oligonucleotide therapeutics. The opportunity resides in the unique attributes of the "**FANA CONSTRUCT**". Multiple strategies for silencing gene expression using nucleic acid-based compounds are in development. The FANA Antisense and short interfering RNAs architectures offer high potency, specificity, and a catalytic mode of action, ideally suited for biological targets not amenable to "small molecule" drug candidates.

Opportunity:

Antisense and siRNA have become widely accepted across the biotech and pharmaceutical spectrum. The number of companies either developing or collaborating with developers of Oligonucleotide therapeutics has more than doubled since Y 2001. Product pipelines presently span numerous indications including: Cancer (>35%); infectious diseases (17%); cardiovascular (10%); respiratory (8%); ocular (8%) and others.

Description:

The FANA CONSTRUCTS comprise four architectures for which over thirty patents have issued worldwide

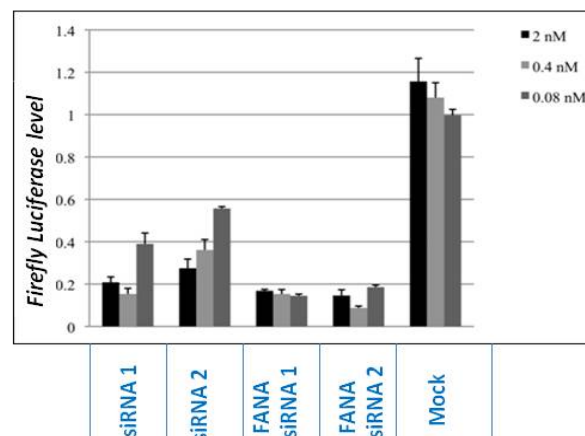
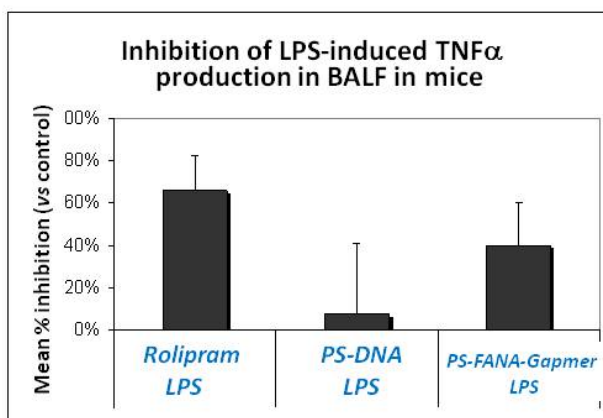


Technology

The FANA Constructs have been validated in numerous cellular and rodent models of disease including cancer, infectious diseases and COPD. Ancillary pharmacology and toxicology study data is also available.

- A: Rolipram: 30 mg/kg i.p. 45 min. pre LPS challenge
- B: PS-DNA antisense: 10 ug i.t. aerosol 16 h pre LPS challenge ;
- C: PS-FANA-Gapmer antisense: 10 ug i.t. aerosol 16 h pre LPS challenge.

Gene silencing of firefly luciferase mRNA using FANA modified siRNAs. Two siRNA sequences were tested, demonstrating that FANA modification does not reduce potency.



The Inventor



Dr. Masad J. Damha is James McGill Professor in the Department of Chemistry of McGill University. He obtained his PhD in Chemistry from McGill University in 1988. Dr. Damha's research focuses on nucleic acid chemistry. Professor Damha has 25 years experience in nucleic acid chemistry and has authored more than 130 publications and patents worldwide. Prof. Damha's research group has made important contributions to nucleic acid chemistry at the interface between chemistry and molecular biology. His research is directed to DNA mimics as model systems for down-regulating gene expression. Dr. Damha has received many distinctions including: the John Charles Polanyi Chemistry Prize (Ministry of Colleges and Universities, 1989), The IUPAC Award (Chemical Institute of Canada, 1991), Ichikizaki Award for Young Chemist (1989-94), the Merck-Frosst Award for Therapeutic Research (Canadian Society for Chemistry, 1999), Fellowship of the Chemical Institute of Canada (F.C.I.C., since 1999) and the Bernard Belleau Award (Canadian Society for Chemistry, 2007).

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Reference code: ROI 09065, 97032, 01023, 02166, 09003
Opportunity: Exclusive license or R&D collaboration



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