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TECHNOLOGY OPPORTUNITY

## 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.

### Description:

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



### 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.

### Validation:

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.

