Docket #: S14-300

Compositions and methods for regulation of gene expression with and detection of folinic acid and folates

Researchers in Dr. Christina Smolke's lab have generated novel RNA aptamer sensors that can be used to regulate gene expression in response to the clinically relevant drug folinic acid. Chemical induction of gene expression is a regulatory tool used in nature to control biological systems. Methods have been developed to repurpose such a tool to engineer and control synthetic systems in biotechnology. However, these methods are limited as they can- cause off-target effects, restrict use to specific organisms, have difficulty uncoupling from native cellular regulation, and/or be difficult to integrate into a regulatory platform. To overcome these limitations the inventors have generated these RNA aptamer sensors. The sensors can serve as regulatory switches to control gene expression in response to folinic acid. As such they have great potential for many applications including gene therapy. This technology provides powerful biologic sensing and control capabilities to program gene expression and cellular behavior.

Applications

- Control RNA-based, viral or cellular therapeutics using externally administered folinic acid
- Detect and measure folinic acid and folate derivatives
- General inducible gene expression system for a variety of applications

Advantages

- Broad utility
- Aptamer sensors exhibit high affinity and sensitivity
- Can be used in many different organisms
- Enables control of gene expression in response to folinic acid input
- Folinic acid is FDA approved, clinically useful, non-toxic, bioavailable, a heterologous input signal for controlling gene expression

Patents

• Published Application: WO2016025750

• Published Application: 20180237781

• Published Application: 20200255835

• Issued: 10,450,573 (USA)

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