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(71) Applicant: **MEIRAGTX UK LIMITED** [GB/GB]; 92 Britannia Walk, London N1 7NQ (GB).

(72) Inventors: **BOYNE, Alex, R.**; 198 Van Vorst Street, Apt. 415, Jersey City, NJ 07302 (US). **DANOS, F., Olivier**; 49 Hancock Street, Boston, MA 02114 (US). **VOLLES, J., Michael**; 69 Bolton Street, Apt. 102, Cambridge, MA 02140 (US). **GUO, Xuecui**; 1 Bungtown Road, Cold Spring Harbor, NY 11724 (US).

(74) Agents: **SOMERVILLE, Deborah, A.** et al.; Fox Rothschild LLP, Princeton Pike Corporate Center, 997 Lenox Drive, Bldg. #3, Lawrenceville, NJ 08648-2311 (US).

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(54) Title: REGULATION OF GENE EXPRESSION BY APTAMER-MEDIATED MODULATION OF ALTERNATIVE SPLICING

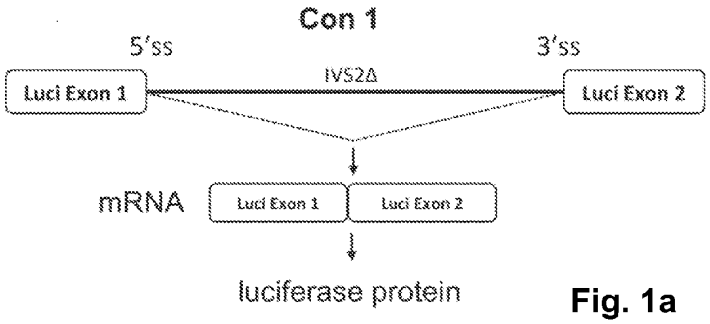


Fig. 1a

(57) Abstract: The invention provides a platform and methods of using the platform for the regulation of the expression of a target gene using exposure to an aptamer ligand (for example, a small molecule). The platform features a polynucleotide gene regulation cassette that is placed in the target gene and includes a synthetic riboswitch positioned in the context of a 5' intron-alternative exon-3' intron. The riboswitch comprises an effector region and a sensor region (e.g., an aptamer that binds a small molecule ligand) such that the alternative exon is spliced into the target gene mRNA when the ligand is not present thereby preventing expression of the target gene. When the ligand is present, the alternative exon is not spliced into the target gene mRNA thereby providing expression of the target gene.

WO 2016/126747 A1