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(71) Applicants: **THE BROAD INSTITUTE INC.** [US/US]; 415 Main Street, Cambridge, MA 02142 (US). **MASSACHUSETTS INSTITUTE OF TECHNOLOGY** [US/US]; 77 Massachusetts Ave., Cambridge, MA 02142 (US). **UNIVERSITY OF IOWA RESEARCH FOUNDATION** [US/US]; 112 N. Capitol Street, 6 Gilmore Hall, Iowa City, IA 52242-5500 (US).

(72) Inventors: **DAVIDSON, Beverly**; C/o University Of Iowa Research Foundation, 112 N. Capitol Street, 6 Gilmore Hall, Iowa City, IA 52242-5500 (US). **LIN, Chie-yu**; 30 Clarendon Street, Boston, MA 02116 (US). **RODRIGUEZ, Edgardo**; C/o University Of Florida, 1275 Center Drive, Bms J-487, Po Box 100159, Gainesville, FL 32610 (US). **ZHANG, Feng**; 100 Pacific Street, Apt. 11, Cambridge, MA 02139 (US).

(74) Agents: **LU, Deborah, L.** et al.; Vedder Price P.C., 1633 Broadway, New York, NY 10019 (US).

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(54) Title: COMPOSITIONS AND METHODS OF USE OF CRISPR-CAS SYSTEMS IN NUCLEOTIDE REPEAT DISORDERS

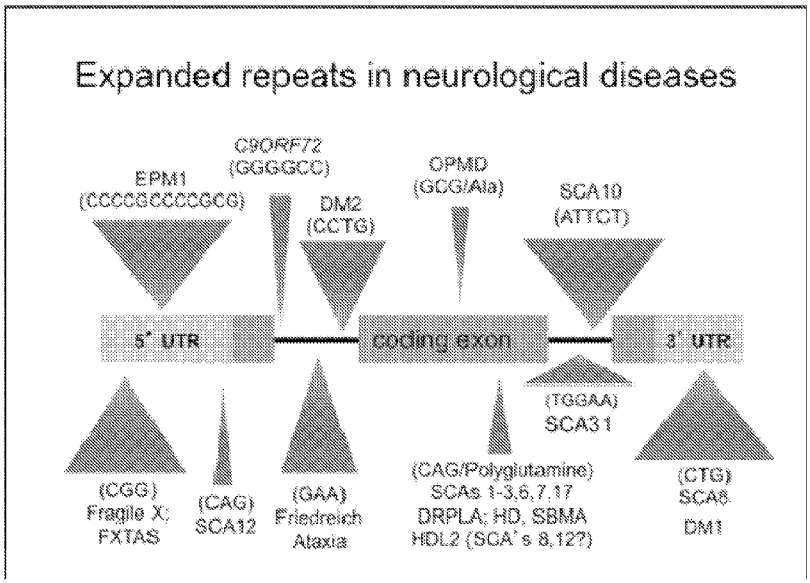


FIG. 1

(57) Abstract: The invention provides for delivery, engineering and optimization of systems, methods, and compositions for manipulation of sequences and/or activities of target sequences especially for use as to nucleotide repeat disorders. Provided are delivery systems and tissues or organ which are targeted as sites for delivery especially for use as to nucleotide repeat disorders. Also provided are vectors and vector systems some of which encode one or more components of a CRISPR complex or system especially for use as to nucleotide repeat disorders, as well as methods for the design and of such. Also provided are methods of directing CRISPR complex or system formation in eukaryotic cells especially for use as to nucleotide repeat disorders including with consideration of specificity for target recognition and avoidance of toxicity and editing or modifying a target site in a genomic locus of interest to alter or improve the status of a disease or a condition.

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