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What if we could turn off the cause of Huntington's disease?

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At Risk for Huntington's Disease

HD is a genetically caused brain disorder that causes uncontrollable bodily movements and robs people's ability to walk, talk, eat, and think. The final result is a slow, ugly death. Children of parents with HD have a 50-50 chance of inheriting the disease. There is no cure or treatment.

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Huntington's Disease Society of America

WEDNESDAY, AUGUST 28, 2019

What if we could turn off the cause of Huntington's disease?

What if scientists could simply switch off a mutated gene causing a debilitating neurodegenerative disorder like Huntington's disease?

Known as gene (or genome) editing, that approach is a current hot research topic, generating hope for sufferers of genetic diseases like HD.

Gene editing will be the focus of a symposium on September 4 sponsored by life science start-up incubator Johnson & Johnson Innovation, JLABS (hereafter simplified as JLABS) and the Janssen Pharmaceutical Companies, the drug-discovery arm of Johnson & Johnson, in San Diego, CA.

At the sponsors' invitation, I will give a presentation, based on my two decades as an HD advocate, on the health and social challenges faced by HD-affected individuals and their families. The two firms have also invited seven leading scientists and biotech executives to speak at the symposium, titled "Science Alliance: Silencing Neurodegenerative Diseases and Sensory Disorders with Gene Editing."

HD community members can watch the live webcast of the event for free by registering at the event website and entering the discount code "HDCOMMUNITY" at check out. Attendance in person is $35 for the general public and $20 for students and academics, at the JLABS facility at 3210 Merryfield Row, San Diego.

Recent milestones in gene therapy "have ignited interest" in the field and "especially its application to neurological disorders," the website states. Gene editing has opened the door to innovation in the treatment of diseases like HD, spinal muscular atrophy, and ALS, according to the organizers.

The website points out that, as the technology progresses, key questions are emerging, such as how to effectively deliver gene editing drugs to the brain.

Owned by Johnson & Johnson, JLABS provides labs, offices, marketing, education, and events for early-stage life-science companies unaffiliated with Johnson & Johnson. In San Diego, one of the world's leading biotech hubs, it offers services to 60 companies; globally, JLABS serves 580 companies.

The pharmaceutical arm of Johnson & Johnson, the Belgium-based Janssen was acquired in 1961.

Advances in gene editing

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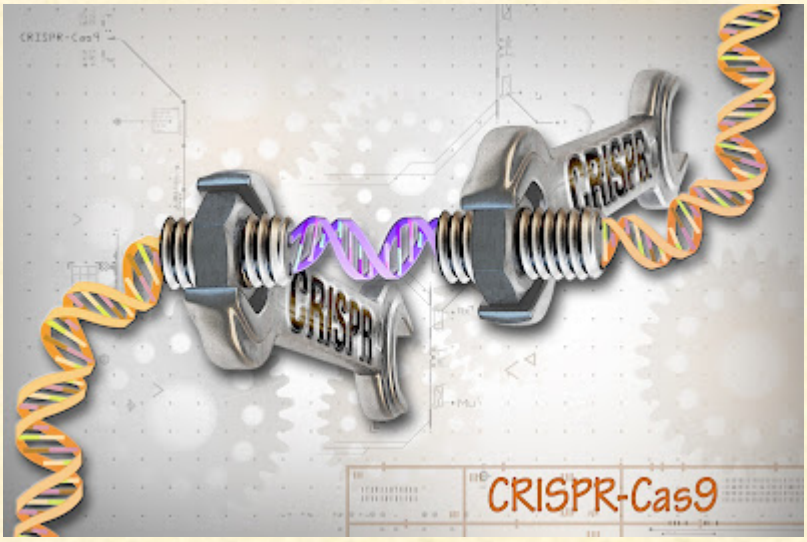
Gene editing is different from gene silencing, the technique used in the Phase 3 Roche clinical trial currently in progress in the U.S. and a projected 17 other countries (click here to read more). Roche's RG6042 is an antisense oligonucleotide, an artificial strand of DNA designed block the production of the huntingtin protein in brain cells.

With gene editing, scientists make changes in the actual DNA – a revolution in biomedical research.

The gene-editing technology currently getting the most attention – one already used in the search for HD treatments – is known as CRISPR. Scientists first observed CRISPR occurring naturally in bacteria in the 1990s. In 2002, scientists discovered additional DNA instructions called "Cas." The combination CRISPR/Cas actually comprises the bacterial immune system. (Click here to read more.)

"There's no equivalent of word processing software to edit genes," then Ph.D. candidate Leora Fox (now a Ph.D.) wrote in HDBuzz in 2017. "To fix genes on a microscopic scale, one cell at a time, the faulty code has to be located and physically cut – and that's what CRISPR/Cas does."

To alter a gene, scientists need to insert CRISPR/Cas into the cells.



(Image credit: Ernesto del Aguila III, National Human Genome Research Institute, and Wikimedia Commons)

In a disease like HD, the goal is to use this mechanism to cut directly (that is, shorten) the defective, elongated gene. Researchers are also looking at other ways to deploy gene editing.

In recent years, HD research groups have used this technology to edit the HD gene in the brains of genetically modified "HD mice". One group developed a technique that led to beneficial effects in mice, including the recovery of older mice that had already developed symptoms. (Click here to read more.)

Chinese researchers have used gene editing in human embryos to fix the mutation behind the blood disease beta-thalassemia, which reduces the amount of red blood cells. However, the embryos were not implanted.

Gene editing is still far from use in human clinical trials. Among the challenges, scientists need to find ways to effectively deliver such a treatment to the brain and avoid inadvertent editing of other genes. (Click here to read more.)

(Late last year a researcher in China claimed to have used CRISPR to alter the genomes of twin baby girls through in vitro fertilization to enable them to resist potential infection from HIV. The news of this development sparked renewed controversy over the use of biotechnology to intervene in human life.)

The symposium participants

To explore gene editing in neurodegenerative and sensory disorders (difficulties with the five senses), JLABS and Janssen have invited seven researchers and executives to the September 4 symposium, including at least two with experience with CRISPR. They include:

Leah Aluisio, Associate Director, Janssen Research and Development;

Alexis C. Komor, Ph.D., Assistant Professor, Department of Chemistry and Biochemistry, UCSD;

Young Jik Kwon, Ph.D., Professor, Department of Pharmaceutical Sciences, University of California, Irvine, and co-founder, Responsive Polymers Therapeutics, Inc., and Jupiter Therapeutics, Inc.;

Sanjay Mistry, Ph.D., Head of JLABS @ San Diego, Johnson & Johnson Innovation, JLABS;

Gerry Rodrigues, Associate Vice President, Allergan;

Arthur Suckow, Ph.D., CEO, DTx Pharma; and

Gene Yeo, Ph.D., MBA, Professor, University of California, San Diego, and co-founder, Locana and Eclipse Bioinnovations.

Their bios are available on the event website.

Imagining a cure?

As a speaker, I hope to portray HD's devastating impact and the urgent need for effective treatment.

In the HD world, scientists avoid the word "cure." HD is so complex that many have said a cocktail of drugs will be needed to target the multiple problems in the brain and elsewhere in the body.

For the first time, actually switching off or completely removing a mutation might enable us to imagine the way to a cure.

Gene Veritas (aka Kenneth P. Serbin) (photo by Yi Sun, Ph.D.)

Posted by Gene Veritas at 9:38 PM

Labels: advocate , brain , CRISPR/Cas , cure , gene editing , gene silencing , genome , huntingtin , Huntington's disease , Janssen , JLABS , Johnson & Johnson , life science start-up incubator , neurodegenerative , Roche , treatments

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