Children's Hospital LOS ANGELES
THE SABAN RESEARCH INSTITUTE

Discovery and Innovation Speaker Series

"Correction of Duchenne Muscular Dystrophy by Genome Editing"

Eric N. Olson, PhD

Annie and Willie Nelson
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The Robert A. Welch Distinguished
Chair in Science
The University of Texas Southwestern
Medical Center

Wednesday, April 17, 2019

12-1 p.m.
The Saban Research Building Auditorium
4661 Sunset Blvd., Los Angeles, CA 90027

Lunch will be provided to seminar guests, first come, first served.
Help us save plastic! Bring your own water bottles. Water will be available to fill your bottles.

Duchenne muscular dystrophy (DMD) is a severe, progressive muscle disease caused by mutations in the Dystrophin gene, which encodes a large intracellular protein that maintains integrity of muscle cell membranes. More than 4,000 DMD mutations have been identified in humans. The majority of mutations are deletions that cluster in hot spots, such that skipping of out-of-frame exons can potentially restore the reading frame of the Dystrophin protein. We have used CRISPR/Cas9 to generate new mouse models of DMD lacking the most prominently deleted Dystrophin exons in humans. To permanently correct DMD by skipping mutant dystrophin exons in postnatal muscle tissue in vivo, we have used adeno-associated virus-9 (AAV9) to deliver CRISPR/Cas9 gene editing components to dystrophic mice, a method we refer to as Myoediting. We have also optimized Myoediting of many types of DMD mutations in muscle cells derived from iPS cells generated from blood samples of DMD patients. In a path toward clinical translation of gene editing for DMD, current efforts are directed toward correction of DMD mutations in large mammals. Opportunities and challenges in the path toward permanent correction of disease-causing mutations responsible for DMD and other monogenic disorders by genomic editing will be discussed.

Hosted by Ching-Ling (Ellen) Lien, PhD
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