DukeNUS Medical School

Signature Seminar Series

Correction of Duchenne Muscular Dystrophy by Genome Editing

ABOUT THE LECTURE

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. 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.

Speaker: **Prof Eric Olson**
Professor and Chairman, Department of Molecular Biology
University of Texas Southwestern Medical Center at Dallas

Host: **Dr Manvendra Singh**
Assistant Professor
Cardiovascular & Metabolic Disorders Programme
Duke-NUS Medical School

Date: **Thursday, 8 February 2018**

Time: **12.00 PM - 1.00 PM**
(Light refreshments will be served at 11.30 AM)

Venue: **Duke-NUS Medical School**
Amphitheatre, Level 2

Contact Person: **Ms Cynthia Lim, Duke-NUS Research Affairs Department**
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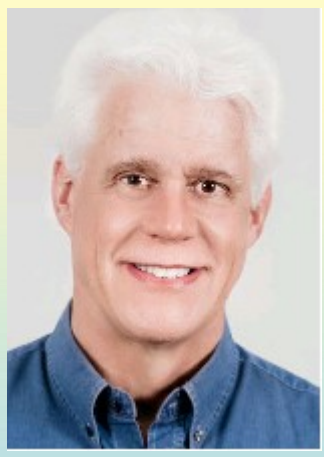
ABOUT THE SPEAKER

Eric Olson chairs the Department of Molecular Biology at University of Texas Southwestern Medical Center. He also directs the Hamon Center for Regenerative Science and Medicine and the Wellstone Center for Muscular Dystrophy Research. He holds the Annie and Willie Nelson Professorship in Stem Cell Research, the Robert A. Welch Distinguished Chair, and the Pogue Distinguished Chair. Eric Olson and his trainees discovered many of the genes that control heart and muscle development and disease. His most recent work has provided a new strategy for correction of Duchenne muscular dystrophy using CRISPR gene editing. Dr. Olson is a member of the U.S. National Academy of Sciences, the National Academy of Medicine, and the American Academy of Arts and Sciences. He has co-founded multiple biotechnology companies to develop new therapies for heart and muscle disease.



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