Fall 2024

Biochemistry and Molecular Biology Brown Bag Series

Sakshi Sakshi
Graduate Student

"MyoAAV-mediated lipin1 restoration reduces dystrophic diaphragm muscle pathology"

Tuesday, November 5, 2024

11:00 AM

Location 105 Biological Sciences Building

Lab: Hongmei Ren, Ph.D.

Boonshoft School of Medicine
WRIGHT STATE UNIVERSITY

WRIGHT STATE UNIVERSITY

https://science-math.wright.edu/biochemistry-and-molecular-biology

Abstract

Respiratory failure is a leading cause of death in Duchenne Muscular Dystrophy (DMD), which is characterized by severe skeletal muscle degeneration, particularly affecting the respiratory muscles like the diaphragm. DMD is caused by mutations in the dystrophin gene, which leads to sarcolemmal instability and structural deterioration of the diaphragm muscle tissues. Our previous studies have shown that lipin1 plays a complementary role to dystrophin in restoring sarcolemmal integrity. This study explores the therapeutic potential of MyoAAV-mediated lipin1 gene therapy in alleviating diaphragm muscle pathology associated with dystrophic conditions. Our research demonstrates that restoration of lipin1 delivered via MyoAAV vector significantly reduced inflammation, fibrosis, and myofiber death. These findings suggest that MyoAAV-mediated lipin1 gene therapy is a promising strategy for treating respiratory complications in DMD, ultimately enhancing the quality of life of the patients.