Fall 2025

Biochemistry and Molecular Biology Brown Bag Series

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Graduate Student

"Therapeutic Evaluation of Lipin1 in the D2.mdx Mouse Model of Duchenne Muscular Dystrophy"

Tuesday, November 4, 2025

11:00 AM

Location 135 Oelman Hall

Lab: Hongmei Ren, Ph.D.

Boonshoft School of Medicine
WRIGHT STATE UNIVERSITY

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

Abstract

Duchenne Muscular Dystrophy (DMD) is a fatal X-linked disorder caused by loss of function mutations in the dystrophin gene, resulting in muscle degeneration, fibrosis, and premature death. The absence of dystrophin compromises sarcolemmal stability, leading to progressive muscle damage and eventually premature death, typically in early adulthood. Despite recent advances, current treatments only slow disease progression and have significant limitations.

Lipin1, a phosphatidic acid phosphatase critical for lipid metabolism and membrane integrity, is markedly reduced in dystrophic muscles of DMD patients as well as in mdx and D2-mdx mouse models. Our recent studies uncovered a novel role for lipin1 in maintaining sarcolemmal integrity. In mdx:lipin1 transgenic mice, lipin1 upregulation reduced muscle pathology and improved both skeletal and respiratory muscle function. To evaluate the translational potential of lipin1 gene therapy, we used the D2-mdx mouse model, which closely recapitulates the severe clinical phenotype of human DMD. We assessed the therapeutic efficacy of MyoAAV-mediated lipin1 gene delivery in restoring muscle function and integrity in D2-mdx mice.