Technology overview

The goal of Dr. Maves' work is to establish zebrafish as a preclinical translation model for evaluating optimal combination drug therapies for Duchenne muscular dystrophy (DMD). Efficient methods for studying drug combinations are particularly important in DMD because many patients take corticosteroids as well as cardiac medication, and any new pharmacological therapies need to be compared to and tested for efficacy in the presence of these current treatments.

The zebrafish model offers several advantages over other animal models for preclinical work: the zebrafish model more closely resembles human DMD in symptom severity; both cardiac and skeletal muscle phenotypes (structure and function) and other biomarkers can be easily monitored; and drug efficacy can be established in studies with duration as short as four days. The zebrafish model can also be used to assess mRNA biomarkers associated with disease progression and drug-induced disease amelioration using qRT-PCR and RNA-seq. Because many of the drugs Dr. Maves assesses are already being used in the clinic or in clinical trials, findings from her zebrafish research could potentially be rapidly incorporated into drug-combination therapies for patients. In addition, the analysis of the DMD cardiac phenotype and cardiac mRNA markers addresses a critical knowledge gap in DMD cardiac issues that may be relevant in the development of new therapies.

The aquatics facility at Seattle Children’s Research Institute is equipped with tools specific to measurement of muscle abnormality in the DMD zebrafish model and allows for screening for DMD therapies, either individually or in combination. Dr. Maves’ research tools can also be applied to facioscapulohumeral muscular dystrophy (FSHD) and other forms of the disease.

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Stage of Development
• Pre-clinical in vivo

Partnering Opportunities
• Collaborative research opportunity
• Sponsored research agreement
• Consultation agreement
• Sample access
• High-throughput screening of DMD therapies

Publications

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