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Exploratory Profiling of Urine MicroRNAs in the *dy*^{2J}/*dy*^{2J} Mouse Model of LAMA2-CMD: Relation to Disease Progression.

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Abstract

Circulating microRNAs (miRNAs) are being considered as non-invasive biomarkers for disease progression and clinical trials. Congenital muscular dystrophy with deficiency of laminin $\alpha 2$ chain (LAMA2-CMD) is a very severe form of muscular dystrophy, for which no treatment is available. In order to identify LAMA2-CMD biomarkers we have profiled miRNAs in urine from the *dy*^{2J}/*dy*^{2J} mouse model of LAMA2-CMD at three distinct time points (representing asymptomatic, initial and established disease). We demonstrate that unique groups of miRNAs are differentially expressed at each time point. We suggest that urine miRNAs can be sensitive biomarkers for different stages of LAMA2-CMD.

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