

Abstract Title: Results of exon skipping and other gene targeted therapies in Duchenne Muscular Dystrophy

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Abstract: Duchenne Muscular Dystrophy (DMD) is a relatively common X linked genetic disorder affecting the skeletal and cardiac muscles. The average age of diagnosis is around 4-6 years age. As the disease advances and patients enter a non-ambulatory phase, cardiac and respiratory decline also follows leading to early death. At present there are many therapies being targeted to reduce the rate of decline and to increase life expectancy. Amongst those at advanced stages of research or available commercially are Ataluren, Exon skipping drugs for Exon 45,51 and 53 and Gene therapy using micro-dystrophin. We discuss the results of clinical trials so far and personal experience of using these drugs.

Area of expertise: Child Neurology with special interest in Neuromuscular, Stroke and Neurogenetics