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Therapeutic developments in genetic treatment of Duchenne muscular dystrophy: Kuwait experience

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Duchenne muscular dystrophy (DMD) is a severe muscle wasting disorder, affecting around 1 in 3500 newborn boys, caused by mutations in the *Dystrophin* gene. The disease is characterized by complete loss of muscle *dystrophin* protein causing progressive muscle weakness leading to premature death due to heart and respiratory failure. Two gene therapy modalities, Antisense oligonucleotides (AONs) for frameshift deletions and ataluren for nonsense mutations, are recently introduced to treat the condition. The rationale behind these therapeutic modalities is to restore the reading frame of the *Dystrophin* gene and produce a partially functional *dystrophin* protein isoforms in skeletal muscle. In Kuwait we registered about 100 DMD patients in our institute neuromuscular registry. All patients were confirmed by MLPA or full gene sequencing. We revised the registry and found seven of our patients legible for AONs and three for ataluren therapy. These patients will be included soon in the treatment program supported by Kuwaiti government. In this presentation, I will discuss our experience regarding DMD natural history and the gathered experiences about gene therapy of the selected cases.

Biography

Laila Ali Bastaki completed PhD and currently working as a Director of Kuwait Medical Genetic Center at State of Kuwait.

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