Molecularly targeted therapy for spinal muscular atrophy (SMA) and duchene muscular dystrophy (DMD): Kuwait experience

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Spinal muscular atrophy (SMA) is an autosomal recessive, motor neuron disease caused by progressive degeneration of motor neurons in the entire spinal cord and in select brainstem motor nuclei (nuclei of cranial nerves V, VII, IX and XII). The disorder causes weakness and wasting of the voluntary muscles. Duchenne muscular dystrophy (DMD) is a severe degenerative muscle disease that affects young males. It is an X-linked recessive disease caused by a mutation in DMD gene on chromosome Xp21. These mutations prevent the production of a connective protein dystrophin. A lack of this connective protein results in severely weakened muscle cells and loss of muscle functions accompanied by muscle tissue replacement by fat and connective tissue. In September and December 2016, FADA approved the first precise molecularly targeted therapy for DMD (Exondys 51) and SMA (nusinersen). Shortly after the approval of these drugs we, at Kuwait Medical Genetic Center, started treatment of patients fulfilling's the inclusion criteria for therapy. Since then we are now treating 65 patients with SMA and 20 patients with DMD. In my talk I will discuss our experience in this field in more details.

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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