Short Communication

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Spinal Muscular Atrophy

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Abstract

Spinal Muscular Atrophy (SMA) is a very severe neurodegenerative disease which causes progressive muscle atrophy and weakness due to loss of the anterior horn cells in the spinal cord and the lower brain stem nuclei. SMA is second most common autosomal recessive disorder and the most common genetic cause of death in infancy. It is caused bydeficiency of survival of motor neuron (SMN) protein due to pathogenic variant in the SMN1 gene. SMA is classified clinically into four subtypes based on the age of onset and maximum motor function achieved. SMA type 1 (most severe form) is present with weakness and hypotonia before six months of age. Type II manifest between ages six and 12 months. Type III manifests between ages 18 to 36 months.Type IV (adult onset) develops muscle weakness in second or third decade. There is no cure for SMA and until recently only supportive treatment was available but the approval of Nusinersen and Zolgesma, has revolutionized the outcome of SMA. Nusinersen (Spinraza) is an antisense oligonucleotide forthe treatment of SMA that increases the production of full-length SMN protein. Zolgesma is a gene therapy that restores the deleted SMN1 gene. Both Nusinersen and Zolgesma have demonstrated significant and clinically meaningful efficacy on the achievement of motor milestones and measures of motor function, as well as favorable safety across all types of SMA and significantly greater event-free survival in infants with infantile-onset SMA. It's important to recognize some of the early signs and symptoms of SMA and diagnose presymptomatic patients by Newborn screen for better outcome of this devastating disease.

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Biography

Farida Abid serves as an Assistant Professor in the Department of Pediatric Neurology at Baylor College of Medicine. She is a graduate of Dow Medical College in Karachi, Pakistan. She then moved to the USA. She completed her Pediatric Residencyat the University of Medicine and Dentistry at New Jersey (UMDNJ), followed by a Pediatric Neurology fellowship at Baylor College of Medicine.

