

10th International Conference on **Mental Health and Psychiatry**
11th World Summit on **Neonatal Nursing and Health Care**

May 20, 2025 | Webinar

ACCEPTED ABSTRACTS

SMA (Spinal muscular atrophy) incidence and treatment with addressing our experience in Saudi

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Introduction: Spinal muscular atrophy (SMA) is a major autosomal recessive neuromuscular disorder SMA causes motor neuron degeneration in the brain stem and spinal-cord, leading to progressive muscle weakness and atrophy. The standard of care in SMA is mainly supportive and consists of nutritional and respiratory support, as needed, in addition to management of muscle weakness complications.

Currently, there is Novel of therapy approved and present for

SMA, showed their efficacy and progress of improvement. Based on one study done in Saudi addressed the cases recived therapy either Gene or Intrathecal Spinraza...

We believe that, the entry of such novel, innovative, and high-cost treatments should be through an outcome-based agreement until the long-term follow-updata confirm real-world effectiveness.

Received Date: December 16, 2024; Accepted Date: December 18, 2024; Published Date: May 30, 2025