**Spinal Muscular Atrophy - Emerging therapies: Experience from a tertiary care hospital**

**Objective:** The newer emerging therapies in spinal muscular atrophy(SMA) have shown encouraging short-term outcomes in terms of motor function scores and life expectancy. Nusinersen, Zolgensma and Risdiplam are the drugs that have been approved by the US-FDA for SMA. Due to their prohibitive exorbitant costs, these are being mostly provided to SMA patients under humanitarian access programs. We present our data of children who have received these medications at our center over the last 2 years and 3 months.

**Methods:** Two groups of patients(12 and 35) have been receiving intrathecal Nusinersen and are under follow-up. Change in the Modified Hammersmith functional motor scale extended version(HFMSE) is being evaluated. Nine patients(humanitarian-access:7) received intravenous Zolgensma and are under follow-up. Nine children(humanitarian-access:7) have been receiving Risdiplam, out of which 3 have a follow-up of more than 1-year.

**Results:** Twelve children have been receiving Nusinersen for >2 years, out of which 11 have shown improvement in HFMSE scores(non-compliance: 1). 35 children have a follow up of <6 months. Nine children who have received Zolgensma, showed significant improvement in motor function (those with SMA-II can now stand with orthoses and those with SMA-I can sit independently without support). There has been reduction in the incidence of lower respiratory tract infections. Three children who have been receiving Risdiplam for > 1 year, have shown significant improvement in HFMSE scores, with gain in motor milestones.

**Conclusion:** The newer emerging therapies in SMA have shown favorable results in improving the short-term functional outcome.