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NUSINERSEN FOR SPINAL MUSCULAR ATROPHY TYPE 1: REAL-WORLD RESPIRATORY EXPERIENCE

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BACKGROUND and AIM

- The emergence of new treatments for spinal muscular atrophy (SMA) is revolutionary, especially for SMA type 1 (SMA1).
- Data on respiratory outcomes remain sparse and rely mostly on randomized clinical trials.
- We report our experience of Nusinersen-treated SMA1 patients in real-world settings.

METHODS

- Data from SMA1 patients treated with Nusinersen were prospectively collected between 1/2017 and 1/2020.
- Respiratory variables included the use of assisted ventilation, the use of mechanical insufflation-exsufflation (MIE), respiratory complications, and death or treatment cessation due to respiratory reasons.

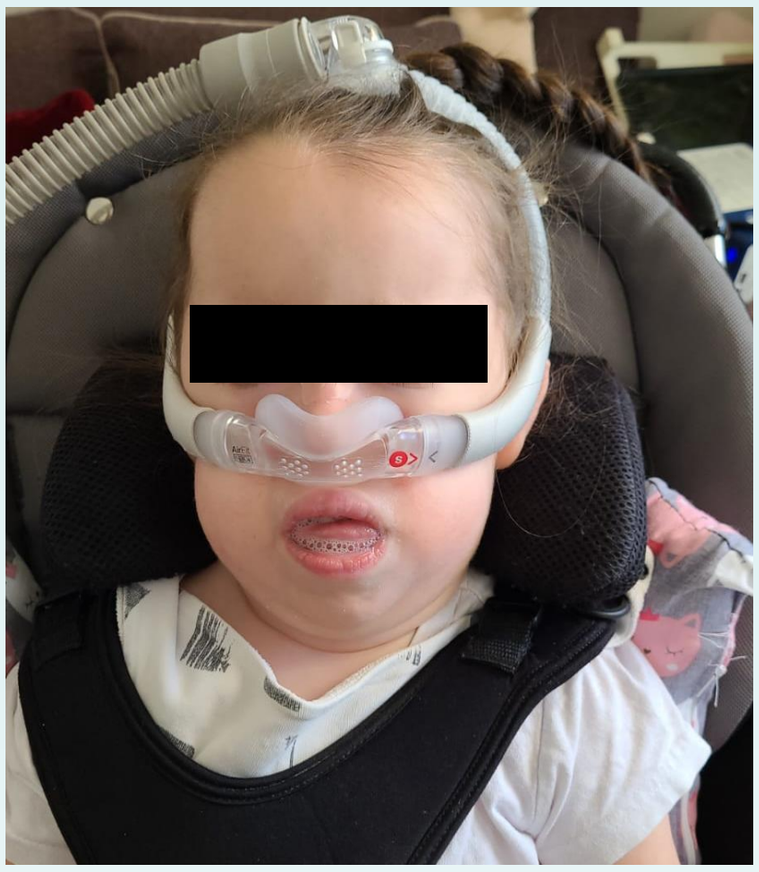

Figure – SMA type 1 patient with ventilation support

RESULTS

- Twenty SMA1 patients were assessed before and after 2 years of Nusinersen treatment which was initiated at a median age of 13.5 months (range, 1-184).
- At baseline, 16 patients were using assisted ventilation, eight noninvasive and eight invasive. Twelve patients were using permanent ventilation and four partial ventilation.
- After 2 years of treatment, there was no change in respiratory support among ventilated patients.
- All four patients who were free from respiratory support at baseline required the initiation of assisted ventilation during the study period.
- All 20 patients used MIE after 2 years of treatment.
- Two patients died from acute respiratory failure and one sustained severe brain injury.
- Four patients had chronic and/or recurrent atelectasis.

CONCLUSIONS

- Most of our patients were stable in their need for assisted ventilation and did not worsen as expected in SMA1, nor did they improve as might be hoped.
- Future studies are needed to determine if earlier treatment with Nusinersen might result in respiratory outcomes superior to those reported in this real-life study.
- Figure – SMA type 1 patient, with ventilation support