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Respiratory Follow-up of Patients Treated with Nusinersen

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Objectives: Nusinersen, promising treatment option for spinal muscular atrophy (SMA) patients was approved by the FDA in 2016. In this study, we aimed to evaluate respiratory support need in patients and to report their respiratory follow-up during the treatment period.

Methods: The study included patients treated with 4 and more doses of Nusinersen. Nusinersen treatment was performed on the 1st-15th-29th-64th-183rd days. Patients were evaluated regarding respiratory findings before the initiation of Nusinersen treatment and after the 4.th doses in pediatric pulmonology department. Chop scores were evaluated in pediatric neurology department as well.

Results: Twenty-seven patients who were followed up from our clinic were included in the study. 44% of the patients were female (n=12); The mean age was 47.81 ± 6.27 months (7-120 months). The mean treatment duration of the patients was 36.1 ± 7.57 months (2-111 months). Sixteen patients were on home ventilation via tracheostomy, 6 patients were in ICU and mechanically ventilated (5 of them were entubated and 1 of them was tracheostomized). One patient was on non-invasive ventilation and needed O₂ support. Four patients breathing spontaneously in room air. 56.2% (n=9) of home ventilated patients and 50% (n=3) of patients staying in ICU had clinical improvement. One of the entubated patients was tracheostomized and home ventilation was initiated. Four patients breathing spontaneously did not require any respiratory support. The oxygen need of the non invasive ventilated patient was ended after the 4.th doses of Nusinersen. Home ventilation therapy was stopped by a family without the knowledge of the clinician after the third doses of Nusinersen therapy but unfortunately the patient was passed away.

Conclusion: Respiratory support need was decreased in nearly half of the patients after 4 doses of Nusinersen therapy. Patients should be closely followed up by the neurology, pulmonology, physical therapy and rehabilitation departments with a multidisciplinary approach. Therapy should be discontinued by the decision of three disciplines in patients who do not get benefit. Long term studies including more patients treated with Nusinersen and specified respiratory scoring systems are needed to evaluate the respiratory follow up.

Keywords: Spinal muscular atrophy, nusinersen, tracheostomy, home ventilation