

Effect of Nusinersen treatment on motor functions in children and adolescents with spinal muscular atrophy who gave a break to physiotherapy during COVID-19 pandemic

Gülten Öztürk¹, Evrim Karadağ Saygı², Olcay Ünver¹, Dilşad Türkođan¹

¹Department of Pediatric Neurology, Marmara University Pendik Training and Research Hospital, Istanbul, Turkey

²Department of Physical Medicine and Rehabilitation, Marmara University Pendik Training and Research Hospital, Istanbul, Turkey

Received: June 03, 2021 Accepted: August 14, 2021 Published online: March 01, 2022

During the novel coronavirus-2019 (COVID-19) pandemic which is still ongoing, the patients with chronic neuromuscular disorders have been considered as a risky subgroup in terms of susceptibility to infection and treatment approach.^[1,2] Effective physiotherapy, which is the only supportive treatment option for most disease subgroups, was disrupted in many clinical centers due to extraordinary pandemic regulations or parental concerns about taking their child to the centers. The preferred approach was to continue home-based physiotherapy either with solely parental involvement or in combination with healthcare professional support online or by home visits. Studies with the Nusinersen which was approved by the United States Food and Drug Administration (FDA) in 2016 for the treatment of spinal muscular atrophy (SMA) patients has shown favorable long-term outcomes in motor function and prognosis.^[3]

Spinal muscular atrophy patients have been treated by intrathecal Nusinersen therapy in our neuromuscular center since 2017. Physiotherapy given by a physiotherapist in a medical center (either private clinic or state hospital) has always been included in the treatment protocol as supportive treatment in addition to Nusinersen; however, some of the patients

followed in our clinic preferred home-based approach during the pandemic. Guidelines developed during the COVID-19 pandemic strongly recommended avoidance of any interruption of Nusinersen treatment due to its proven positive effects on prognosis and the devastating effects of lack of treatment.^[4] We aimed to evaluate the impact of Nusinersen therapy, which was continued to be performed according to schedules during the pandemic on motor functions, specifically on SMA patients whose effective physiotherapy has been disrupted due to the extraordinary conditions and caregivers' choice.

Forty-eight patients (22 type 1, 14 type 2, and 12 type 3) who preferred home-based physiotherapy after March 2020 were included in the study. Patients who continued physiotherapy by the help of a healthcare professional (nurse or physiotherapist) were excluded from the study. The Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND) Scale was used for SMA type 1 patients and HFMS (Hammersmith Functional Motor Scale) for SMA type 2 and 3 patients. Baseline CHOP/HFMS scores used in the study were defined as the latest score of the patient before COVID-19 pandemic in March 2020 (December

Corresponding author: Gülten Öztürk, MD. Marmara Üniversitesi Tıp Fakültesi Pendik Eğitim ve Araştırma Hastanesi Nöroloji Anabilim Dalı, Çocuk Nörolojisi Bilim Dalı, 34899 Pendik, İstanbul, Türkiye

e-mail: gulten@thomas.md

Cite this article as:

Öztürk G, Karadağ Saygı E, Ünver O, Türkođan D. Effect of Nusinersen treatment on motor functions in children and adolescents with spinal muscular atrophy who gave a break to physiotherapy during COVID-19 pandemic. Turk J Phys Med Rehab 2022;68(1):157-158.

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2019-August 2020). Follow-up scores were taken as the last measured score before May 2021. The mean baseline CHOP score was 33.0 ± 20.9 (n=22) and the mean baseline HFMS score was 28.4 ± 19.8 (n=26). The following mean CHOP-INTEND scores were as 38.8 ± 18.0 (n=22) and the mean HFMS scores were 32.1 ± 20.2 (n=26). The mean baseline dose numbers for SMA type 1 and type 2-3 were 5.3 ± 3.5 (range, 0 to 11) and 2.1 ± 1.9 (range, 0 to 4), respectively. The mean follow-up dose numbers for these two groups were 7.5 ± 3.1 (range, 2 to 13) and 4.5 ± 0.9 (range, 2 to 6), respectively. Fifteen patients started treatment during pandemic. The mean age of the patients was 65 ± 59.6 (range, 0 to 222) months at the evaluation of baseline scores. We observed that all of the patients had an increase in their scores or kept the scores stable. According to local governmental guidelines, none of the patients had a decline of the scores that would prevent their legal drug application procedures. The SMA type 1 patients had a relatively better increase in their scores (6 points), compared to type 2 and 3 patients (4 points); however, their mean baseline Nusinersen dose number was higher than type 2 and 3 patients.

In conclusion, prevalent data of the present study suggest that SMA is considered among the more vulnerable group to severe COVID-19 infection and complications.^[5] However, avoidance of Nusinersen

treatment due to pandemic concerns is likely to have more devastating effects on the patients' motor functions; as our study showed, timely administered, Nusinersen treatment has noticeable positive effects on motor functions independent of physiotherapy.

Declaration of conflicting interests

The authors declared no conflicts of interest with respect to the authorship and/or publication of this article.

Funding

The authors received no financial support for the research and/or authorship of this article.

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