



Collection of RWD of Nusinersen treatment for SMA patients through a national registry: description of the cohort in the Korea

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INTRODUCTION

- ◆ Nusinersen is one of the drug treatments currently available to patients with SMA in KOREA. The use of nusinersen is conditional to a pre-approval system since April 2019.

AIM

- ◆ This study aims to describe the characteristics of the SMA patients in the nusinersen pre-approval system.

METHODS

- ◆ The HIRA has been operating a pre-approval system since 1992 to prevent the misuse of medical services and protect the financial stability of health insurance. HIRA determines the initial approval of care benefits and the continuation of medication considering the medical validity of high-risk, high-cost procedures and medications.
- ◆ Nusinersen is a drug subject to the pre-approval system, and the HIRA collects RWD from hospitals to inform the decision-making process regarding the injection, maintenance, and discontinuation of Nusinersen.
- ◆ Retrospective data from April 2019 to December 2021 was extracted from the HIRA pre-approval system registry which includes thirteen hospitals in Korea.
- ◆ The RWD collected from hospitals for nusinersen includes patients, hospitals, nusinersen injection, and motor function assessment information.
- ◆ The motor function measures include HINE-2 and HFMSE. To evaluate the motor function of patients with SMA type 1, the HIRA requires the use of the HINE-2. For patients with SMA type 2 or 3, the HFMSE should be used.
- ◆ Patients were evaluated at baseline and four-monthly thereafter for up to 34-months after receiving nusinersen. Annual follow-up assessments were conducted at 10, 22, and 34 months. In this study, we referred to these time points for the 1-, 2- and 3-year follow-up.

RESULTS

- ◆ Based on RWD, 137 patients (52 males, 85 females), comprising 22, 102, and 13 patients with SMA types 1,2, and 3, respectively (16.1%, 74.5%, and 9.5%), were analyzed. The average age of enrollment is 17.9 ± 11.0 years (median: 16.0 years).
- ◆ The average age of symptom onset was 1.0 ± 0.5 years, and the average age of nusinersen injection was 14.3 ± 11.2 years in patients with SMA. The average number of nusinersen injection was 9.6 ± 2.0 times, with a minimum of 5 times and a maximum of 15 times.
- ◆ For patients with SMA type 1, the average HINE-2 score 1.6 ± 1.7 points out of 26 points at baseline, 6.9 ± 4.6 points at 1-year, 10.6 ± 6.2 points at 2-year and 11.4 ± 8.3 points at 3-year. As for patients with SMA type 2, the average HFMSE score were 9.7 ± 11.7 points out of 66 points at baseline, 13.6 ± 13.3 at 1-year, 15.8 ± 13.7 at 2-year, and 21.3 ± 12.0 at 3-year. In SMA 3, the average HFMSE score were 18.5 ± 18.6 points out of 66 points at baseline, 22.8 ± 19.3 at 1-year, 25.7 ± 21.9 at 2-year, and 42.0 at 3-year.

		Total		SMA type 1		SMA type 2		SMA type 3	
SMA patients		137	(100)	22	(100)	102	(100)	13	(100)
Gender	Male	52	(38.0)	8	(36.4)	42	(41.2)	2	(15.4)
	Female	85	(62.0)	14	(63.6)	60	(58.8)	11	(84.6)
Age of symptom onset (years)		1.0 ± 0.5 (0.9)		0.3 ± 0.2 (0.2)		1.1 ± 0.4 (1.0)		1.4 ± 0.5 (1.1)	
Age at start treatment with nusinersen (years)		14.3 ± 11.2 (11.8)		2.3 ± 4.5 (0.6)		15.6 ± 10.0 (13.8)		24.4 ± 12.1 (22.9)	
Number of nusinersen dose (times)		9.6 ± 2.0 (10.0)		9.6 ± 3.4 (9.0)		9.6 ± 1.7 (10.0)		9.1 ± 1.9 (9.0)	
Motor Function Assessment Score									
Base line	HINE-2	1.6 ± 1.7 (1.0)		1.6 ± 1.7 (1.0)		-		-	
	HFMSE	10.6 ± 13.0 (4.0)		-		9.7 ± 11.7 (4.0)		18.5 ± 18.6 (9.0)	
1-year		-		n=18 6.9 ± 4.6 (7.0)		n=100 13.6 ± 13.3 (7.0)		n=12 22.8 ± 19.3 (14.5)	
2-year		-		n=11 10.6 ± 6.2 (13.0)		n=86 15.8 ± 13.7 (10.0)		n=9 25.7 ± 21.9 (16.0)	
3-year		-		n=7 11.4 ± 8.3 (14.0)		n=12 21.3 ± 12.0 (20.0)		n=1 42.0 (42.0)	

* Average $\pm$ Standard Deviation (Median)

CONCLUSIONS

- ◆ As advancements in medical technology continue, there has been a steady increase in the demand for reimbursement of high-risk medical procedures and high-cost medications. This trend is expected to result in the proactive utilization of the pre-approval system. In line with this, recent cases of collecting RWD for nusinersen, included in the pre-approval system, have been shared.

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- ◆ None

REFERENCES

- ◆ A. Rohwer et al(2022). "Collection of real-world evidence of nusinersen treatment for SMA patients through a national registry: description of the paediatric cohort in the UK." Neuromuscular Disorders. 32. S42-S136.

<Abbreviations>

RWD: Real-World Data

SMA: Spinal Muscular Atrophy

HIRA: Health Insurance Review & Assessment service

HINE-2: Hammersmith Infant Neurological Exam Section 2

HFMSE: Hammersmith Functional Motor Scale-Expanded