



SFDA SAFETY SIGNAL

“A signal is defined by the SFDA as reported information on a possible causal relationship between an adverse event and a drug, the relationship being unknown or incompletely documented previously. Usually more than a single report is required to generate a signal, depending upon the seriousness of the event and the quality of the information. A signal is a hypothesis together with data and arguments and it is important to note that a signal is not only uncertain but also preliminary in nature”

01-02-2026

Saudi Food and Drug Authority (SFDA) – Safety Signal of Onasemnogene Abeparvovec and the Risk of Pneumonia

*The Saudi Food and Drug Authority (SFDA) recommends all health care professionals to be aware of the safety signal of **Pneumonia** associated with the use of **Onasemnogene Abeparvovec**. The signal has been originated as a result of routine pharmacovigilance monitoring activities.*

Introduction

Onasemnogene abeparvovec-xioi injection is used to treat spinal muscular atrophy (SMA) with bi-allelic mutations in the survival motor neuron 1 (SMN1) gene. It is an adeno-associated virus vector-based gene treatment that works by copying the gene needed for the human SMN protein. [1] Pneumonia is an infection that inflames the air sacs in one or both lungs. The air sacs may fill with fluid or pus (purulent material), causing cough with phlegm or pus, fever, chills, and difficulty breathing. [2] The aim of this review is to evaluate the risk of pneumonia associated with the use of Onasemnogene Abeparvovec and to suggest regulatory recommendations if required.

Methodology

Signal Detection team at SFDA performed a signal review using National Pharmacovigilance Center (NPC) database, and World Health Organization (WHO) database, VigiBase, with literature screening to retrieve all related information to assess the potential link between pneumonia and Onasemnogene Abeparvovec use. The search conducted on December 2025.

Results

Cases Review: Signal detection team at SFDA have searched Saudi national database and WHO database to find individual case safety reports (ICSRs). The WHO database resulted in 97 global case reports while no local cases found. The authors used signal detection tool (Vigilyze) to retrieve global cases. [3] The author applied WHO causality assessment tool on the extracted cases with completeness score (> 0.8) ($n=19$). Among them, 14 cases were possibly linked to Onasemnogene Abeparvovec, while five cases were unassessable due to insufficient information.

Datamining: The disproportionality of the observed and the expected reporting rate for drug/adverse drug reaction pair is estimated using information component (IC), a tool developed by WHO-UMC to measure the reporting ratio. Positive IC reflects higher statistical association while negative values indicates less statistical association. The IC result is (2.6) for this drug/ADR combination which reflects positive statistical association. [3]



Literature: The signal team conducted a literature search to identify publications linking this adverse drug reaction to Onasemnogene Abeparvovec. The search identified one published study suggesting a possible association between the drug and this potential risk. Furthermore, the ADR is listed in Canadian drug monograph. [4], [5].

Conclusion

The weighted cumulative evidence identified from assessed cases, disproportionality analysis and literature are suggestive for causal association between Onasemnogene Abeparvovec and pneumonia. Health care professionals and health regulators must be aware of the potential risk in drug recipients.

Report Adverse Drug Events (ADRs) to the SFDA

The SFDA urges both healthcare professionals and patients to continue reporting adverse drug reactions (ADRs) resulted from using any medications to the SFDA either online, by regular mail or by fax, using the following contact information:

National Pharmacovigilance Center (NPC)
Saudi Food and Drug Authority-Drug sector
4904 northern ring branch rd
Hittin District
Riyadh 13513 – 7148
Kingdom of Saudi Arabia
Toll free number: 19999
Email: NPC.Drug@sfda.gov.sa

References

1. Mayo Clinic- Onasemnogene abeparvovec-xioi (intravenous route) - Side effects & uses - Mayo Clinic: <https://www.mayoclinic.org/drugs-supplements/onasemnogene-abeparvovec-xioi-intravenous-route/description/drg-20465053>
2. Mayo Clinic Diseases- Pneumonia: <https://www.mayoclinic.org/diseases-conditions/pneumonia/symptoms-causes/syc-20354204>
3. Vigilize.who-umc.org. 2025. [online] Available at: <<https://vigilize.who-umc.org/>>
4. Chen, T., Chen, Q., Ye, J. et al. Postmarketing adverse events associated with onasemnogene abeparvovec: a real-world pharmacovigilance study. *Orphanet J Rare Dis* 20, 215 (2025). <https://doi.org/10.1186/s13023-025-03715-2>
5. ZOLGENSMA ® (onasemnogene abeparvovec) (no date). Available at: https://pdf.hres.ca/dpd_pm/00080337.PDF