Real-world safety data of the orphan drug Onasemnogene Abeparvovec (Zolgensma®) for the SMA rare disease: a pharmacovigilance study based on the EMA adverse event reporting system

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The recent introduction of the innovative therapy with onasemnogene abeparvovec (Zolgensma®) revolutionized the spinal muscular atrophy (SMA) therapeutic landscape. Even if it had proven to lead to functional improvements in SMA children, some gaps in its safety profile still need to be investigated. To better characterize the Zolgensma® safety profile, we conducted a retrospective observational study, analyzing all the Individual Case Safety Reports (ICSR) referred to it and collected in the European pharmacovigilance database. Our period was between 1st January 2019 and 22th September 2023. We found 661 ICSRs related to Zolgensma®, with a growing trend in the annual reporting. The majority of the reports were sent by healthcare professionals and were referred to infant females. In more than 90% of the cases, Zolgensma® was the only reported suspected drug. Out of a total of 2,744 reported ADRs, increased hepatic enzymes, pyrexia, vomiting, and thrombocytopenia were the most reported ones. The 56.9% of the adverse drug reactions (ADRs) were serious, causing or prolonging the patient's hospitalization. A total of 39 ICSRs described 130 ADRs with fatal outcome. Alterations of the heart rhythm, acute hepatic failure, and hepatic cytolysis emerged among the cardiac and hepatic disorders, respectively.

Biography

Ruggiero Rosanna is a Researcher from University of Campania “Luigi Vanvitelli” in Italy and her scientific works cover the field of Pharmacovigilance and Pharmacoepidemiology. On 2014 she graduated in Pharmacy at the University of Naples Federico II. From 2016 she is a member of the Italian Society of Pharmacology (SIF). On 2016, she got a II level Master in “Pre-clinical and clinical development of the drug and post-marketing monitoring” at the University of Naples Federico II, focusing her research in pharmacovigilance field. On 2021 she completed her PhD in Translational Medicine by conducting researches on drug safety at the Regional Center of Pharmacovigilance and Pharmacoepidemiology – Department of Experimental Medicine of the University of Campania “Luigi Vanvitelli”. From 2023, she is resident in Clinical Pharmacology and Toxicology at the University of Campania “Luigi Vanvitelli”..