

Review Form 1.6

Journal Name:	Asian Journal of Research and Reports in Neurology
Manuscript Number:	Ms_AJORRIN_78613
Title of the Manuscript:	Egypt fights Spinal Muscular Atrophy
Type of the Article	Opinion Article

General guideline for Peer Review process:

This journal's peer review policy states that **NO** manuscript should be rejected only on the basis of '**lack of Novelty**', provided the manuscript is scientifically robust and technically sound. To know the complete guideline for Peer Review process, reviewers are requested to visit this link:

(<https://www.journalajorrin.com/index.php/AJORRIN/editorial-policy>)

PART 1: Review Comments

	Reviewer's comment	Author's comment (if agreed with reviewer, correct the manuscript and highlight that part in the manuscript. It is mandatory that authors should write his/her feedback here)
Compulsory REVISION comments	It is necessary to write that there is no existing cure for the patients with SMA type 1 and type 2. Children in cases of SMA type 1 and type 2 die in early age (till the age of 4 years) because of respiratory complications. After that authors may inform the reader about the new drug Zorgensma (AVXS-101 (onasemnogene abeparvovec-xioi, Zolgensma®) which if a preparation of gene therapy –established for the patients with proximal spinal muscular atrophy, caused by abnormality in SMN1 gene. AVXS-101 is delivering synthetic functional copy of SMN1 gene to the cells of motor neurons by means of adeno-associated virus vector of 9 serotype (scAAV9)).	Comment accepted and considered
Minor REVISION comments	It is necessary to write that this medical preparation passed through clinical trials and was affirmed for treatment use in the clinic. Medicine preparation AVXS-101 had been studied in three programs of clinical investigations on several dozens of children with SMA type 1 in their age from 0 to 9 months and demonstrated promising therapy effectiveness and its safety. For a period of June 2020 – this drug had been approved in the USA, European Union for the therapy in patients with SMA aged up to 24 months, including those children who did not have any symptoms of this disease at the moment of diagnosis confirming. Medical drug is administered in single dose by use of intravenous infusions and its dose is defined on account for body mass in the child.	Comment accepted and considered
Optional/General comments	The author can indicate general statistic on SMA disease worldwide using 1-2 sentences.	Ok

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PART 2:

	Reviewer’s comment	Author’s comment <i>(if agreed with reviewer, correct the manuscript and highlight that part in the manuscript. It is mandatory that authors should write his/her feedback here)</i>
Are there ethical issues in this manuscript?	<p><i>(If yes, Kindly please write down the ethical issues here in details)</i></p> <p>To my mind, it is obligatory to specify that this preparation had been clinically tested and demonstrated its safety and effectiveness in treatment of children. Since treatment is performed for very small patients – therefore, we deal here with ethical issues.</p>	