

## Review Form 1.6

Journal Name:	<b>Journal of Pharmaceutical Research International</b>
Manuscript Number:	<b>Ms_JPRI_93538</b>
Title of the Manuscript:	<b>Gene therapies for Spinal muscular atrophy and Duchenne muscular dystrophy : a pathbreaking moment in therapeutics</b>
Type of the Article	<b>Review Article</b>

### **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.journalpri.com/index.php/JPRI/editorial-policy>

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**PART 1: Review Comments**

	<b>Reviewer's comment</b>	<b>Author's comment</b> (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)
<b>Compulsory</b> REVISION comments	<p>1. The English need improvement since there are some grammatical and syntax errors in the manuscript. For example, the words "delivery" may be as "the delivery"; ", cultured" as ", and cultured"; "the Caucasians" as "Caucasians"; "in degeneration" as "in the degeneration"; ", greater" as ", the greater"; "milder the severeness" as "the milder the severity"; "is complete" as "is a complete"; "as sooner" as "as the sooner"; "better" as "the better"; "Multidisciplinary" as "A multidisciplinary"; "inclusion" as "the inclusion"; "Discovery" as "The discovery"; "for the" as "in the"; "promotion" as "the promotion"; "and effective" as "and most effective"; "that are" as "are"; "and cap" as "and a cap"; "for packaging" as "for the packaging"; "absence" as "an absence"; "so that it" as "so it"; "which is" as "which are"; "of an" as "an"; "(AAV) are" as "(AAV) is"; "trials is" as "trials are"; "oligonucleotides has" as "oligonucleotides have"; "53% participants" as "53% of participants"; "Oral route" as "The oral route"; "from the" as "in the"; "widening" as "a widening"; "to risk" as "to the risk"; "blood-brain-barrier" as "the blood-brain-barrier"; ", systemic" as ", the systemic"; "of central" as "of the central"; "route having" as "route has"; "the inflammatory" as "inflammatory"; "for treatment" as "for the treatment"; "in stage" as "in a stage"; "Substantial" as "A substantial"; "in stage" as "in a stage"; "to expression" as "to the expression"; "double-blind" as "a double-blind"; "placebo" as "a placebo"; "on lower" as "on the lower"; "have lower" as "have a lower"; "57% patients" as "57% of patients"; "with increase" as "with an increase"; "Randomised" as "A randomised"; "for 30s" as "for the 30s"; "at 14th" as "in the14th"; "Until age" as "Until the age"; "for 30s" as "for the 30s"; "Open-label" as "The open-label"; "ventilation required" as "ventilation is required"; "with nonsense" as "with the nonsense"; "taken of" as "was taken off"; "Till 28th" as "Till the 28th"; "a quantitative" as "quantitative"; "assessing ratio" as "assessing the ratio"; "6-Minute" as "the 6-Minute"; "above a" as "over a"; "Treated" as "The treated"; "baseline" as "the baseline"; "in untreated" as "in the untreated"; "difference" as "the difference"; "of 48th" as "of the 48th"; "commencement" as "the commencement"; "at 48th" as "at the 48th"; "ambulatory" as "an ambulatory"; "hope for patients" as "hope in patients"; "for their" as "to their"; "treatment" as "the treatment"; "from 2022" as "in 2022"; "precedes over" as "precedes"; "is important" as "is more important"; "a case of 25" as "the case of a 25"; "of SMA" as "of the SMA"; "higher" as "a higher"; "motor" as "a motor"; "better" as "a better"; "administered with" as "administered"; "improvement" as "the improvement"; "myostatin" as "the myostatin"; "in initial" as "in an initial"; "in cytoplasm" as "in the cytoplasm"; "inhibitor" as "an inhibitor"; "increase of" as "an increase in"; "also shown" as "also been shown"; "a pharmacological" as "pharmacological"; "death of" as "the death of"; "of ROCK" as "of the ROCK"; "in activation" as "in the activation"; "maturation" as "the maturation"; "RhoA-Rho" as "the RhoA-Rho"; "activation of" as "the activation of"; ", increase" as ", an increase"; "into CSF" as "into the CSF"; "under experimental" as "in under the experimental"; "of viral" as "of the viral"; "of CRISPR" as "of the CRISPR"; "restoration" as "the restoration"; "at genomic" as "at the genomic"; "a partially" as "partially"; "targeting the" as "to target the"; "has shown" as "has been shown"; "for longer" as "for a longer"; "dystrophin are" as "dystrophin is"; "In coming" as "In the coming". The grammar mistakes which are not mentioned here are also to be checked and corrected properly.</p> <p>2. There are some typing mistakes as well, and authors are advised to carefully proof-read the text. For example, the word "Szy-balski" may be as "Szy-Balski"; "first ever" as "first-ever"; "cis- elements and trans- factors" as "cis-elements and trans-factors"; "single stranded" as "single-stranded"; "read through" as "read-through"; "produces a" as "produce a"; "per genome" as "per-genome"; "prognosed" as "prognosis"; "makes risdiplam" as "make risdiplam"; "into the" as "to the"; "instead it" as "instead, it"; "dependent of" as "dependent on"; "Multiple exon" as "Multiple exons"; "at forty eighth" as "in forty-eighth"; "one time" as "one-time"; "twenty four" as "twenty-four"; "of regular" as "of a regular"; "nusinersen related" as "nusinersen-related"; "18th month" as "18th months"; "long term" as "long-term"; "96 week" as "96 weeks"; "placebo controlled" as "placebo-controlled"; "open label" as "open-label"; "twenty eight" as "twenty-eight"; "one-time-infusion" as "one-time infusion"; "16 crore" as "16 crores"; "an US" as "a US"; "orphan drug" as "orphan drugs"; "focuses" as "focus"; "thought on" as "thoughts on"; "inhibits myostatin" as "inhibit myostatin"; "evaluating in" as "evaluated in"; "SMA independent" as "SMA-independent"; "compromise on" as "compromising on"; "double strand" as "double-strand"; "to improve" as "improved". The typos not mentioned here are also to be checked and corrected properly.</p>	

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	3. Check the abbreviations throughout the manuscript and introduce the abbreviation when the full word appears the first time in the text and then use only the abbreviation (For example, Becker's muscular dystrophy (BMD), creatine kinase muscle type isoform (CK-MM), Adeno-Associated Virus (AAV), etc.). And it should be in both abstract as well as in the remaining part of the manuscript.	
<b>Minor</b> REVISION comments	1. The literature search should be described in detail. The authors are encouraged to include the database, search engines (like PubMed, ScienceDirect, Google scholar etc.), the keywords used etc., which may be included in the introduction section or separately in the materials heading since it is a review article. 2. In conclusion, the authors may also be included the limitation of the present findings for a better understanding of the manuscript.	
<b>Optional/General</b> comments	The authors have put effort to evaluate the "Gene therapies for Spinal muscular atrophy and Duchenne muscular dystrophy : a pathbreaking moment in therapeutics". The authors describe the research undertaken with this in an organized manner, emphasizing the results obtained by them. The article needs modification for better cohesion of information to achieve the goal and the minor shortcomings which need to be considered. Hence, the paper can be accepted after MAJOR REVISIONS are carried out.	

### **PART 2:**

	<b>Reviewer's comment</b>	<b>Author's comment</b> (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)
<b>Are there ethical issues in this manuscript?</b>	<i>(If yes, Kindly please write down the ethical issues here in details)</i>	

### **Reviewer Details:**

Name:	<b>A. Vijaya Anand</b>
Department, University & Country	<b>Bharathiar University, India</b>