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ESPS PEER-REVIEW REPORT

Name of journal: World Journal of Stem Cells

ESPS manuscript NO: 22635

Title: Human induced pluripotent stem cells for monogenic disease modelling and therapy

Reviewer's code: 00573611

Reviewer's country: Taiwan

Science editor: Xue-Mei Gong

Date sent for review: 2015-09-10 14:43

Date reviewed: 2015-09-13 18:46

CLASSIFICATION	LANGUAGE EVALUATION	SCIENTIFIC MISCONDUCT	CONCLUSION
<input type="checkbox"/> Grade A: Excellent	<input type="checkbox"/> Grade A: Priority publishing	Google Search:	<input type="checkbox"/> Accept
<input type="checkbox"/> Grade B: Very good	<input checked="" type="checkbox"/> Grade B: Minor language polishing	<input type="checkbox"/> The same title	<input type="checkbox"/> High priority for publication
<input checked="" type="checkbox"/> Grade C: Good	<input type="checkbox"/> Grade C: A great deal of language polishing	<input type="checkbox"/> Duplicate publication	<input type="checkbox"/> Rejection
<input type="checkbox"/> Grade D: Fair	<input type="checkbox"/> Grade D: Rejected	<input type="checkbox"/> Plagiarism	<input checked="" type="checkbox"/> Minor revision
<input type="checkbox"/> Grade E: Poor		<input checked="" type="checkbox"/> No	<input type="checkbox"/> Major revision
		BPG Search:	
		<input type="checkbox"/> The same title	
		<input type="checkbox"/> Duplicate publication	
		<input type="checkbox"/> Plagiarism	
		<input checked="" type="checkbox"/> No	

COMMENTS TO AUTHORS

In this review, the authors provided an overview on the current state of modeling monogenic disease and the ultimate application of these in vitro models for cell therapy. The authors considered and summarized some peculiar aspects such as the type of parental cell used for reprogramming, the methods currently used to induce expression of the reprogramming factors, and the type of iPSC-derived differentiated cells, relating them to the genetic basis of diseases and to their inheritance model. Comments This is an interesting review article. The authors provide the information about recent development on human induced pluripotent stem cells for monogenic disease modeling and therapy. The reviewer has only some minor concerns: The editing of manuscript needs to be revised. The punctuation of sentence or paragraph is not clear that may cause reading difficulty. Moreover, there is no page number in the manuscript.



ESPS PEER-REVIEW REPORT

Name of journal: World Journal of Stem Cells

ESPS manuscript NO: 22635

Title: Human induced pluripotent stem cells for monogenic disease modelling and therapy

Reviewer's code: 01851506

Reviewer's country: Japan

Science editor: Xue-Mei Gong

Date sent for review: 2015-09-10 14:43

Date reviewed: 2015-09-16 15:40

Table with 4 columns: CLASSIFICATION, LANGUAGE EVALUATION, SCIENTIFIC MISCONDUCT, CONCLUSION. It contains checkboxes for various evaluation criteria like 'Grade A: Excellent', 'Priority publishing', 'Google Search', etc.

COMMENTS TO AUTHORS

The article by Spitalieri et al. has reviewed the usefulness of iPSC for modeling the monogenic diseases and the potential use of iPSC for therapy. The problems relevant to obtaining the therapeutic grade iPSC (i.e. DNA modification free iPSC) and the possible resolutions were discussed. Examples for monogenic disease specific iPSCs were described such as autosomal dominant, recessive, and X-linked disorders. Finally the authors discussed the potential use of DNA editing technologies such as ZNFs, TALENs, and CRISPR/Cas 9 to repair the DNA mutation in the disease. The reviewer felt that the manuscript is, in general, well written. However, English is to be further polished. My concern is that although the authors wrote "Current researches focus on the use of Adenovirus and Sendai virus due to their ability to avoid exogenous DNA integration into the host genome. The results, however, have been unsuccessful most of the time (page 5, line 26 -), the reviewer do not find any reference which underpin their claim. The reviewer rather thinks it to be opposite. There are many examples in which Sendai virus is successfully used to generate scar-free iPSC (an recent example: Ando M. et al. Stem Cell Report 5, 1-12 (2015)). Please check the literatures



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and correct the sentence with appropriate references.



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ESPS PEER-REVIEW REPORT

Name of journal: World Journal of Stem Cells

ESPS manuscript NO: 22635

Title: Human induced pluripotent stem cells for monogenic disease modelling and therapy

Reviewer's code: 02446119

Reviewer's country: China

Science editor: Xue-Mei Gong

Date sent for review: 2015-09-10 14:43

Date reviewed: 2015-09-21 11:56

CLASSIFICATION	LANGUAGE EVALUATION	SCIENTIFIC MISCONDUCT	CONCLUSION
<input type="checkbox"/> Grade A: Excellent	<input type="checkbox"/> Grade A: Priority publishing	Google Search:	<input type="checkbox"/> Accept
<input type="checkbox"/> Grade B: Very good	<input type="checkbox"/> Grade B: Minor language polishing	<input type="checkbox"/> The same title	<input type="checkbox"/> High priority for publication
<input type="checkbox"/> Grade C: Good	<input type="checkbox"/> Grade C: A great deal of language polishing	<input type="checkbox"/> Duplicate publication	<input type="checkbox"/> Rejection
<input type="checkbox"/> Grade D: Fair	<input type="checkbox"/> Grade D: Rejected	<input type="checkbox"/> Plagiarism	<input type="checkbox"/> Minor revision
<input type="checkbox"/> Grade E: Poor		<input type="checkbox"/> No	<input type="checkbox"/> Major revision
		BPG Search:	
		<input type="checkbox"/> The same title	
		<input type="checkbox"/> Duplicate publication	
		<input type="checkbox"/> Plagiarism	
		<input type="checkbox"/> No	

COMMENTS TO AUTHORS

The establish of human iPS is now a promising source of treatment cells. After gene editing with different technique, iPS will replace the abnormal cells to restore the expression of the critical gene(s). The authors summarized the present advances in fetal tissue-derived iPS, the monogenic disease models and the techniques to correct gene expression. The content of this manuscript is well-designed with extensive information of the field. Its fluent writing is also easy to read and understand. This manuscript is very useful for the readers to get a close look at the development of iPS and its application.