

ESPS PEER-REVIEW REPORT

Name of journal: World Journal of Medical Genetics

ESPS manuscript NO: 16286

Title: Adeno-associated virus vectors for human gene therapy

Reviewer's code: 00632006

Reviewer's country: Japan

Science editor: Yue-Li Tian

Date sent for review: 2015-01-08 20:43

Date reviewed: 2015-01-19 10:59

CLASSIFICATION	LANGUAGE EVALUATION	SCIENTIFIC MISCONDUCT	CONCLUSION
<input type="checkbox"/> Grade A: Excellent	<input checked="" type="checkbox"/> Grade A: Priority publishing	Google Search:	<input type="checkbox"/> Accept
<input checked="" 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 checked="" type="checkbox"/> Plagiarism	<input checked="" type="checkbox"/> Minor revision
<input type="checkbox"/> Grade E: Poor		[Y] No	<input type="checkbox"/> Major revision
		BPG Search:	
		<input type="checkbox"/> The same title	
		<input type="checkbox"/> Duplicate publication	
		<input type="checkbox"/> Plagiarism	
		[Y] No	

COMMENTS TO AUTHORS

The review describes the current development of AAV vectors and purification methods of AAV and briefly summarizes the advancement of several clinical trials. The paper is well written and well organized. However, the part of AAV vector description is somewhat redundant. The reviewer recommend the author to present a Table in which tissue tropism, transduction efficiency, immunological properties of AAV vectors are summarized so that the readers can understand them at a glance. Minor comments. 1) Page 12, line 15. "reminder" should be "remainder". 2) Refs 13, 59, and 142. The year of publication is missing. 3) Ref 51. The author's names should be described correctly. The article number of the reference is missing. 4) Ref 78. The year of publication is wrong. The page number is missing.

ESPS PEER-REVIEW REPORT

Name of journal: World Journal of Medical Genetics

ESPS manuscript NO: 16286

Title: Adeno-associated virus vectors for human gene therapy

Reviewer's code: 00503952

Reviewer's country: Canada

Science editor: Yue-Li Tian

Date sent for review: 2015-01-08 20:43

Date reviewed: 2015-03-06 05:50

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	<input type="checkbox"/> The same title	<input type="checkbox"/> High priority for
<input checked="" type="checkbox"/> Grade C: Good	polishing	<input type="checkbox"/> Duplicate publication	publication
<input type="checkbox"/> Grade D: Fair	<input checked="" type="checkbox"/> Grade C: A great deal of	<input type="checkbox"/> Plagiarism	<input type="checkbox"/> Rejection
<input type="checkbox"/> Grade E: Poor	language polishing	<input checked="" type="checkbox"/> No	<input checked="" type="checkbox"/> Minor revision
	<input type="checkbox"/> Grade D: Rejected	BPG Search:	<input type="checkbox"/> Major revision
		<input type="checkbox"/> The same title	
		<input type="checkbox"/> Duplicate publication	
		<input type="checkbox"/> Plagiarism	
		<input checked="" type="checkbox"/> No	

COMMENTS TO AUTHORS

This review provides us with updated information about the progresses of gene therapy using adeno-associated virus vectors. The author describes various AAV vectors. However, the author cited reference 51. I do not believe that the so-called Induced pluripotent stem cells (iPS cells) are real stem cells, and iPS cells would unlikely be a reliable and feasible source of stem cells in the foreseeable future (Journal of Biomedical Research 01/2015; 29(1):1-2). This manuscript has many grammatical problems and typographical errors, for example: On page 9, transduced human hepatocytes rather poorly – approximately 20-fold less efficiently than it does mouse hepatocytes (66); VR-I differences modify the raised region of the capsid surface between the 2-fold and 5-fold depressions. On page 11, when the genome is half wild-type size; monkey and rodent origins and accelerated and long-term transduction in vivo via intramuscular or tail vein delivery in mice. On page 12, in which the rep gene is derived from AAV2 but the cap gen can be any other serotypes On page 16, Density gradient ultracentrifugation with cesium chloride (CsCl) for virus particle purification has been used for more than 50 years On page 17, it separates from the contaminating proteins and collects as a band in the middle of the gradient after the first round of centrifugation. On



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page 18, Iodixanol, an X-ray contrast compound for use, can be used as density gradient medium in place of CsCl; co-author Dr. Wright at CHOP has adopted instead a combinational method of column chromatography On page 19, Recently, promising results have been obtained from Phase 1 and Phase 2 clinical trials for a number of diseases On page 21, The high-dose AAV1.SERCA2a group, however, continued to do significantly better than all groups at 12 months The author needs help from a native English speaker.

ESPS PEER-REVIEW REPORT

Name of journal: World Journal of Medical Genetics

ESPS manuscript NO: 16286

Title: Adeno-associated virus vectors for human gene therapy

Reviewer's code: 00505473

Reviewer's country: India

Science editor: Yue-Li Tian

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Date reviewed: 2015-02-22 21:56

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 author has well reviewed the AAVs and its clinical usage. Following comments could be made: 1. In Fig.3, sizes of the protein markers should be mentioned. 2. A compilation of properties of AAVs and respective clinical usage should be given in a Table.