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(54) Title: METHODS AND COMPOSITION FOR MODULATING TYPE I MUSCLE FORMATION USING PGC-1 α -

(57) Abstract: The invention provides novel methods and compositions for modulating type I muscle formation through modulation of PGC-1 α activity or expression. Also provided are methods for identifying compounds that modulate type I muscle formation through modulation of PGC-1 α activity or expression. Further provided are methods for treating disorders associated with type I and/or type II muscle formation, as well as transgenic animals expressing PGC-1 α in muscle.

INTERNATIONAL SEARCH REPORT

International application No.

PCT/US03/04792

A. CLASSIFICATION OF SUBJECT MATTER				
IPC(7) : A61K 48/00; C12N 15/63				
US CL : 514/44; 424/93.2; 435/320.1, 455				
According to International Patent Classification (IPC) or to both national classification and IPC				
B. FIELDS SEARCHED				
Minimum documentation searched (classification system followed by classification symbols) U.S. : 514/44; 424/93.2; 435/320.1, 455				
Documentation searched other than minimum documentation to the extent that such documents are included in the fields searched NONE				
Electronic data base consulted during the international search (name of data base and, where practicable, search terms used) APS, CAS ONLINE				
C. DOCUMENTS CONSIDERED TO BE RELEVANT				
Category *	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim No.		
A	ANDERSON, W. F. Human Gene Therapy. Nature. 30 April 1998, Vol. 392, Supp., pages 25-30, especially pages 25-26.	1-9, 14-20, 39-47, 49-54		
A	REKHETER et al. Gene Transfer Into Normal and Atherosclerotic Human Blood Vessels. Cir Res. 1998, Vol. 82, pages 1243-1252, especially page 1243, pages 1249-1250.	1-9, 14-20, 39-47, 49-54		
<input type="checkbox"/> Further documents are listed in the continuation of Box C. <input type="checkbox"/> See patent family annex.				
* Special categories of cited documents: <table border="0" style="width: 100%;"> <tr> <td style="width: 50%;"> "A" document defining the general state of the art which is not considered to be of particular relevance "B" earlier application or patent published on or after the international filing date "L" document which may throw doubts on priority claim(s) or which is cited to establish the publication date of another citation or other special reason (as specified) "O" document referring to an oral disclosure, use, exhibition or other means "P" document published prior to the international filing date but later than the priority date claimed </td> <td style="width: 50%;"> "T" later document published after the international filing date or priority date and not in conflict with the application but cited to understand the principle or theory underlying the invention "X" document of particular relevance; the claimed invention cannot be considered novel or cannot be considered to involve an inventive step when the document is taken alone "Y" document of particular relevance; the claimed invention cannot be considered to involve an inventive step when the document is combined with one or more other such documents, such combination being obvious to a person skilled in the art "&" document member of the same patent family </td> </tr> </table>			"A" document defining the general state of the art which is not considered to be of particular relevance "B" earlier application or patent published on or after the international filing date "L" document which may throw doubts on priority claim(s) or which is cited to establish the publication date of another citation or other special reason (as specified) "O" document referring to an oral disclosure, use, exhibition or other means "P" document published prior to the international filing date but later than the priority date claimed	"T" later document published after the international filing date or priority date and not in conflict with the application but cited to understand the principle or theory underlying the invention "X" document of particular relevance; the claimed invention cannot be considered novel or cannot be considered to involve an inventive step when the document is taken alone "Y" document of particular relevance; the claimed invention cannot be considered to involve an inventive step when the document is combined with one or more other such documents, such combination being obvious to a person skilled in the art "&" document member of the same patent family
"A" document defining the general state of the art which is not considered to be of particular relevance "B" earlier application or patent published on or after the international filing date "L" document which may throw doubts on priority claim(s) or which is cited to establish the publication date of another citation or other special reason (as specified) "O" document referring to an oral disclosure, use, exhibition or other means "P" document published prior to the international filing date but later than the priority date claimed	"T" later document published after the international filing date or priority date and not in conflict with the application but cited to understand the principle or theory underlying the invention "X" document of particular relevance; the claimed invention cannot be considered novel or cannot be considered to involve an inventive step when the document is taken alone "Y" document of particular relevance; the claimed invention cannot be considered to involve an inventive step when the document is combined with one or more other such documents, such combination being obvious to a person skilled in the art "&" document member of the same patent family			
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INTERNATIONAL SEARCH REPORT

International application No.

PCT/US03/04792

Box I Observations where certain claims were found unsearchable (Continuation of Item 1 of first sheet)

This international report has not been established in respect of certain claims under Article 17(2)(a) for the following reasons:

1. Claim Nos.:
because they relate to subject matter not required to be searched by this Authority, namely:

2. Claim Nos.:
because they relate to parts of the international application that do not comply with the prescribed requirements to such an extent that no meaningful international search can be carried out, specifically:

3. Claim Nos.:
because they are dependent claims and are not drafted in accordance with the second and third sentences of Rule 6.4(a).

Box II Observations where unity of invention is lacking (Continuation of Item 2 of first sheet)

This International Searching Authority found multiple inventions in this international application, as follows:
Please See Continuation Sheet

1. As all required additional search fees were timely paid by the applicant, this international search report covers all searchable claims.
2. As all searchable claims could be searched without effort justifying an additional fee, this Authority did not invite payment of any additional fee.
3. As only some of the required additional search fees were timely paid by the applicant, this international search report covers only those claims for which fees were paid, specifically claims Nos.:

4. No required additional search fees were timely paid by the applicant. Consequently, this international search report is restricted to the invention first mentioned in the claims; it is covered by claims Nos.: 1-9, 14-20, 39-47, 49-54
SEQ ID NO:1

Remark on Protest The additional search fees were accompanied by the applicant's protest.
 No protest accompanied the payment of additional search fees.

BOX II. OBSERVATIONS WHERE UNITY OF INVENTION IS LACKING

This application contains the following inventions or groups of inventions which are not so linked as to form a single general inventive concept under PCT Rule 13.1. In order for all inventions to be examined, the appropriate additional examination fees must be paid.

Group I, claims 1-9, 14-20, 39-47, 49-54, drawn to a method of modulating or increasing type I muscle formation comprising the use of a PGC-1 alpha nucleic acid molecule of SEQ ID NO: 1.

Group II, claims 1, 10-12, drawn to a method of modulating or increasing type I muscle formation comprising the use of a PGC-1 alpha protein sequence of SEQ ID NO: 2.

Group III, claims 1, 13, 39, 48, 49, 55, drawn to a method of modulating or increasing type I muscle formation comprising the use of an unspecified small molecule.

Group IV, claims 21-28, 30-37, drawn to a method of identifying a compound capable of modulating type I muscle formation.

Group V, claims 29 and 38, drawn to an unspecified compound that is claimed to be invented at the time the invention was made.

Group VI, claims 56-60, drawn to a non-human transgenic animal.

The inventions listed as Groups I-VI do not relate to a single general inventive concept under PCT Rule 13.1 because, under PCT Rule 13.2, they lack the same or corresponding special technical features for the following reasons:

Each of the Group I-IV is directed to a distinct process having a distinct technical feature, which would exhibit both a distinct structure and functional effect. For example, the nucleic acid sequence of SEQ ID NO: 1 is distinct from that of the polypeptide of SEQ ID NO: 2, let alone claims drawn to the use of an unspecified small molecule for therapeutic application in modulating type I muscle formation in any environment and/or animal. Also, a transgenic non-human mammal is materially distinct from the compound claims and/or any of the processes as claimed, nor the process of identifying compound has anything to do with therapeutic application of drugs in treating type I muscle associated disorders. Furthermore, 37 CFR 1.475 does not provide for multiple independent products, methods of manufacture and methods of use (37 CFR 1.473(d)).