INTEGRATIVE METHOD FOR GENERATING INDUCED PLURIPOTENT STEM CELLS FOR GENE THERAPY

Abstract: The present invention relates to a method of generating induced pluripotent stem cells (iPSCs), for gene therapy, from a patient-derived somatic cell line, through a one-step method combining iPSC generation factors and gene therapy factors. The method for generating genetically modified iPSCs, through the one-step, can generate gene-corrected iPSCs or disease model iPSCs having undergone mutagenesis by simultaneously introducing, into host cells, a reprogramming episomal vector, which induces the reprogramming of adult somatic cells, and a carrier for gene correction or mutagenesis. The gene-corrected or mutated iPSCs exhibit features identical with and different from other kinds of normal iPSCs, and thus can be used in screening for disease treatment cellular therapeutic agents or for therapeutic substances.

Title: INTEGRATIVE METHOD FOR GENERATING INDUCED PLURIPOTENT STEM CELLS FOR GENE THERAPY

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본 발명은 유도만능줄기세포(induced pluripotent stem cell, iPSC) 제조 요소 및 유전자 치료 요소를 결합한 원-스텝(one-step) 방법을 통해 합성 유래 체세포주로부터 유전자 치료를 유도한 유도만능줄기세포를 제작하는 방법에 관한 것으로, 상기 원-스텝(one-step)에 의해 유전적 변형을 갖는 유도만능줄기세포(induced pluripotent stem cell, iPSC) 제조방법은 성체 체세포의 역분화를 유도하는 리프로그래밍 에피토모 벡터, 및 유전자 교정 또는 돌연변이 유발 전달체를 동시에 주주체포로 도입하여 유전자 교정된 iPSC 또는 돌연변이 유발된 질환모델 iPSC를 제조할 수 있고, 상기 유전자가 교정되거나, 돌연변이가 유발된 iPSC가 다른 종류의 정상형의 iPSC와 동일한 특징 및 상이한 특징을 나타냄으로써 질환치료용 세포치료제 또는 치료물질의 스크린링에 사용할 수 있다.
INTERNATIONAL SEARCH REPORT

Class: C12N 15/09; C12N 5/10; C12N 15/13; C12N 5/10; A61K 35/12

The present international search report is based on international application No. PCT/KR2015/013622.

A. CLASSIFICATION OF SUBJECT MATTER

C12N 15/09 (2006.01), C12N 5/10 (2006.01), A61K 35/12 (2006.01)

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)

C12N 15/09; C12N 15/90; C12N 15/013; C12N 5/10; A61K 35/12

Documentation searched other than minimum documentation to the extent that such documents are included in the fields searched

Japanese Utility models and applications for Utility models: IPC as above

Japanese Utility models and applications for Utility models: IPC as above

Electronic database consulted during the international search (name of database and where practicable, search terms sized)

eKOMPASS (EPPO internal) & Keywords: Induced pluripotent stem cells, reprogramming, strategy for identification, Stem Cells, November 2012, vol. 30, no. 11, pages 2437-2449

See abstract; page 2438, left column, page 2439, right column; figures 1-2.

A. DOCUMENTS CONSIDERED TO BE RELEVANT

Category* Citation of document, with indication where appropriate, of the relevant passages


See abstract; pages 2 and 4.

A WILEY, L. A. et al., "Patient-Specific induced Pluripotent Stem Cells (iPSCs) for the Study and Treatment of Retinal Degenerative Diseases", Progress in Retinal and Eye Research, Published online 04 November 2014, vol. 44, pages 15-35

See abstract; pages 17-18; figures 1-2.


See abstract, figure 1.

Further documents are listed in the continuation of Box C. See patent family annex.

* Special categories of cited documents:

“A” document defining the general state of the art which is not considered to be of particular relevance

“E” earlier application or patent but 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

“R” 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

“Q” 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

“U” 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

“X” document member of the same patent family

Date of the actual completion of the international search

30 MAY 2016 (30.05.2016)

Date of mailing of the international search report

30 MAY 2016 (30.05.2016)

Name and mailing address of the ISA/KR

Korean Intellectual Property Office
Government Complex-Daejeon, 31s Seocho-ro, Daejeon 302-701, Republic of Korea
Facsimile: 82-42-472-7140

Authorized officer

Telephone No.
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See abstract; claims 1-14.
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A. 발명이 속하는 기술분류 (국제특허분류(IPC))
C12N 15/09(2006.01)i, C12N 5/10(2006.01)i, A61K 35/12(2006.01)i

B. 조사된 분야
조사된 최소문헌 (국제특허분류 기재)
C12N 15/09 ; C12N 15/90 ; C12N 15/113 ; C12N 5/10 ; A61K 35/12

조사된 기술분야에 속하는 최소문헌, 이외의 문헌
한국특허 실질심사 안건과 및 한국특허 실질심사 신청받보에 기재된 IPC
일본특허 실질심사 안건과 및 일본특허 실질심사 신청받보에 기재된 IPC

국제특허에 이용된 전산 데이터베이스(데이터베이스의 명칭 및 검색어(해당하는 경우))
eKOMPASS(특허청 내부 검색시스템) & 키워드: 유도만, 농장기 세포, 리프트코, 레아킹, 에피 darm 백터, sgRNA, ssODN, FOP, 레스닝

C. 관련문헌
키로리학* 인용문헌명 및 관련 구절(해당하는 경우)의 기재 관련처구함

A HAMASAKI, M. 등, "Pathogenic c Mutation of ALK2 Inhibits Induced Pluripotent Stem Cell Reprogramming and Maintenance: Mechanisms of Reprogramming and Strategy for Drug Wntification", Stem Cells, 2012. 11, 30권, 11호, 페이지 2437-2449
초록; 페이지 2437, 좌표; 페이지 2439, 우표; 도면 12 참조

A KAPLAN, J. 등, "Restoration of normal BMP signaling levels and osteogenic cell differentiation in FOP mesenchymal progeni for eels by mutant allele-specific targeting", Gene Therapy, 2012. 01, 17권, 0호, NIH Public Access Author Manucript et al. Version 내부 페이지 1-9
초록; 페이지 2 및 4 참조

A WILEY, L. A. 등, "Patient-specific induced pluripotent stem cells (iPSCs) for the study and treatment of retinal degenerative diseases", Progress in Retinal and Eye Research, 온라인 공개일 2011. 11. 04, 44권, 페이지 15-35
초록; 페이지 17-18, 도면 1-2 참조

A FRANK, S. 등, "A modified TALEN-based system for robust generation of knockout human pluripotent stem cells lines and disease models", BMC Genomics, 2013. 11, 09, 14권, 논문번호 773, 페이지 1-9
초록; 도면 1 참조

% 추가 문헌이 C(계속)에 기재되어 있습니다. % 대응특허에 관한 별지를 참조하십시오.

* 인용된 문헌의 특별 키로리학
"A" 특별히 관련이 있는 것으로 보이는 일반적인 기술수준을 넘는한 문헌
"B" 국제특허원과 또 다른 출원일과 또는 출원일을 거치거나 국제출원일 이후에 출원된 출원 또는 특허 문헌
"C" 국제특허원과 또 다른 출원일과 또는 출원일을 거치거나 국제출원일 이후에 출원된 출원 또는 특허 문헌
"D" 출원일 이후에 공개되었거나 국제출원일 이전에 공개된 문헌
"E" 등일한 특허특허 키로리에 속하는 문헌

국제특허의 실제 완료일
2016년 05월 30일 (30. 05. 2016)

국제특허고고서 발송일
2016년 05월 30일 (30. 05. 2016)

S/AKR 청주 소재지 대전광역시 서구 189
4동 (문산동, 정부대전청사)

서식 PCT/ISA/210 (두 번째 용지) (2015년 1월)
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요약; 청구항 1-14 참조.
대웅 특허에 관한 정보

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