

12-31-01

A) Ron

12/21/01
31044 U.S. PTO

PROVISIONAL APPLICATION COVER SHEET

a request for filing a PROVISIONAL APPLICATION under 37 CFR 1.53 (b)(2).

| | | |
|---------------|-------------------|------------------------------------|
| Docket Number | 65482-PRO2/JPW/GJ | Type a plus sign inside this box → |
|---------------|-------------------|------------------------------------|

| INVENTOR(s)/APPLICANT(s) | | | |
|--|--|------------------|---|
| LAST NAME | FIRST NAME | MIDDLE INITIAL | RESIDENCE (CITY AND EITHER STATE OR FOREIGN COUNTRY) |
| Symonds | Geoffrey | | 15 Hamilton Street, Rose Bay, NSW 2029 Australia |
| Lun-Quan | Sun | | 74 Fawcett Street, Ryde NSW, 2112, Australia |
| TITLE OF THE INVENTION (250 characters max) | | | |
| METHOD FOR GENETIC MODIFICATION OF HEMATOPOETIC PROGENITOR ("HP") CELLS AND USES OF THE MODIFIED CELLS | | | |
| CORRESPONDENCE ADDRESS | | | |
| John P. White, Esq. Cooper & Dunham LLP 1185 Avenue of the Americas New York, New York 10036 | | | |
| STATE | NY | ZIP CODE | 10036 |
| | | COUNTRY | United States of America |
| ENCLOSED APPLICATION PARTS (check all that apply) | | | |
| <input checked="" type="checkbox"/> | Specification | Number of Pages | 77 |
| <input checked="" type="checkbox"/> | Drawing(s) | Number of Sheets | 14 |
| <input type="checkbox"/> | | | Small Entity Statement |
| <input checked="" type="checkbox"/> | | | Express Mail Certificate of Other (specify) Mailing bearing Label No. EL 525 964 707US, dated Dec. 21, 2001 |
| METHOD OF PAYMENT (check one) | | | |
| <input checked="" type="checkbox"/> | A check or money order is enclosed to cover the Provisional filing fees | | PROVISIONAL FILING FEE AMOUNT (\$) |
| <input checked="" type="checkbox"/> | The Commissioner is hereby authorized to charge filing fees and credit Deposit Account Number: 03-3125 | | \$160.00 |

The invention was made by an agency of the United States Government or under a contract with an agency of the United States Government.

No.
 Yes, the name of the U.S. Government agency and the Government contract number are: _____

Respectfully submitted,
 SIGNATURE *Gary J. Gershek*
 TYPED or PRINTED NAME Gary J. Gershek

Date 12/21/01
 REGISTRATION NO. (if appropriate) 39,992

Additional inventors are being named on separately numbered sheets attached hereto

PROVISIONAL APPLICATION FILING ONLY

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PAGE 2

INVENTOR(S) /APPLICANT(S)

| LAST NAME | FIRST NAME | RESIDENCE (CITY AND EITHER STATE OR FOREIGN COUNTRY) |
|------------|------------|---|
| Amado | Rafael | 16050 Woodley Park Lane Encino, CA 91436 |
| Fanning | Greg | 5/22 Read Street Bronte, New South Wales 2024 Australia |
| Macpherson | Janet | 13 Day Street Leichardt, New South Wales 2040 Australia |

U.S. PATENT & TRADEMARK OFFICE

IN THE UNITED STATES PATENT AND TRADEMARK OFFICE

Applicant: Geoffrey P. Symonds et al.
Serial No.: Not Yet Known
Filed: Herewith
For: METHOD FOR GENETIC MODIFICATION OF HEMATOPOETIC
PROGENITOR ("HP") CELLS AND USES OF THE MODIFIED
CELLS

1185 Avenue of the Americas
New York, New York 10036
December 21, 2001


Assistant Commissioner For Patents
Washington, D.C. 20231

Box: Provisional Application

SIR:

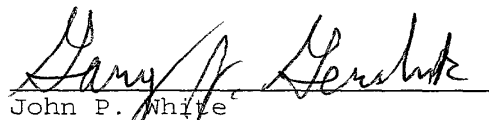
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Washington, D.C. 20231:



Printed Name: John P. White

Respectfully submitted,



John P. White
Registration No. 28,678
Gary J. Gershik
Registration No. 39,992
Attorneys for Applicant
Cooper & Dunham LLP
1185 Avenue of the Americas
New York, New York 10036
Tel (212) 278-0400

*Application
for
United States Letters Patent*

To all whom it may concern:

Be it known that, we,

Geoffrey Symonds et al.

have invented certain new and useful improvements in

METHOD FOR GENETIC MODIFICATION OF HEMATOPOETIC PROGENITOR ("HP") CELLS AND
USES OF THE MODIFIED CELLS

of which the following is a full, clear and exact description.

**METHODS FOR GENETIC MODIFICATION OF HEMATOPOETIC
PROGENITOR ("HP") CELLS AND USES OF THE MODIFIED CELLS**

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Throughout this application various publications are referenced in parenthesis. Full citations for these publications may be found listed alphabetically at the end of the specification immediately preceding the claims. The disclosures of these publications in their entireties are hereby incorporated by reference into this application in order to more fully describe the state of the art to which this invention pertains.

15 **FIELD OF THE INVENTION**

The present invention relates to gene therapy, particularly as applied to hematopoietic progenitor (HP) cells, to transduced cells and methods of obtaining them, and to methods of using them.

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BACKGROUND OF THE INVENTION

Gene therapy refers to the use of genetic sequences and their introduction into cells to alter the genetic makeup of the cells and thereby change the properties or functioning of those cells. Gene therapy may be used, for example, to correct a genetic defect by providing to the cells a good copy of a gene that functions as desired, or to provide a gene that encodes an RNA or protein that inhibits an undesired cellular or pathogen activity.

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Gene therapy may be aimed at any of a variety of diseases in which there is a genetic aspect. Of particular interest are diseases of the blood or immune systems since the hematopoietic cells are relatively easy to collect from a subject, allowing for ex vivo procedures to be used. These include hemoglobinopathies, defects of leukocyte production or function, immune deficiencies, lysosomal storage diseases

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