

06-06-02
PROVISIONAL APPLICATION COVER SHEET

A/PROV

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

Docket Number 65482-PRO3/JPW/GJC	Type a plus sign (+) inside this box → +
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11000 U.S. PTO

INVENTOR(S)/APPLICANT(S)			
LAST NAME	FIRST NAME	MIDDLE INITIAL	RESIDENCE (CITY AND EITHER STATE OR FOREIGN COUNTRY)
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TITLE OF THE INVENTION (280 characters max)
METHOD FOR GENETIC MODIFICATION OF HEMATOPOETIC PROGENITOR ("HP") CELLS AND USES OF THE MODIFIED CELLS

11000 U.S. PTO
60/386063
06/04/02

CORRESPONDENCE ADDRESS			
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STATE	NY	ZIP CODE	10036	COUNTRY	United States of America
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ENCLOSED APPLICATION PARTS (check all that apply)					
<input checked="" type="checkbox"/>	Specification	Number of Pages	98	<input type="checkbox"/>	Small Entity Statement
<input checked="" type="checkbox"/>	Drawing(s)	Number of Sheets	20	<input checked="" type="checkbox"/>	Express Mail Certificate of Other (specify) Mailing bearing Label No. EL 525 964 565US, dated June 4, 2002

METHOD OF PAYMENT (check one)		
<input type="checkbox"/>	A check or money order is enclosed to cover the Provisional filing fees	PROVISIONAL FILING FEE AMOUNT (\$)
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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. Gershik* **Date**

TYPED or PRINTED NAME Gary J. Gershik **REGISTRATION NO. (if appropriate)**

Additional inventors are being named on separately numbered sheets attached hereto

PROVISIONAL APPLICATION FILING ONLY

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CSHL EXHIBIT 2013
BENITEC V. CSHL
IPR2016-00014

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PAGE 2

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2025 RELEASE UNDER E.O. 14176

IN THE UNITED STATES PATENT AND TRADEMARK OFFICE

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

1185 Avenue of the Americas
New York, New York 10036
June 4, 2002

Assistant Commissioner for Patents
Washington, D.C. 20231

Box: Provisional Application

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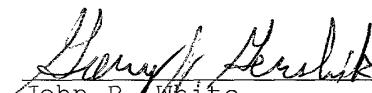
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Printed Name: John White

Respectfully submitted,


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*Application
for
United States Letters Patent*

2025 RELEASE UNDER E.O. 14176

To all whom it may concern:

Be it known that, we,

Geoffrey Symonds, Sun Lun-Quan, Rafael Amado, Greg Fanning and Janet Macpherson

have invented certain new and useful improvements in

METHOD FOR GENETIC MODIFICATION OF HEMATOPOETIC PROGENTOR ("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.

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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

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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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