Application for Lonafarnib

The Progeria Research Foundation Lonafarnib Pre-clinical Drug Supply Program For Progeria

General Information:

Lonafarnib has shown some benefit to disease in Hutchinson-Gilford progeria syndrome (Gordon et al, PNAS, 2012; Ullrich et al, Neurology, 2013). It is essential for the research community to continue investigating the effects of lonafarnib on HGPS through preclinical studies. As new compounds emerge with potential for benefit to children with HGPS, it is also essential to perform combination drug studies using lonafarnib plus new compounds of interest. It is likely that in future, clinical trials testing new potential medications for HGPS will involve at least one combination treatment arm, administering lonafarnib plus a new drug of interest. Therefore, PRF suggests that researchers consider similar preclinical studies, with one arm testing combination treatment with lonafarnib plus new compound of interest. PRF and Merck are making lonafarnib available for these studies in order to further scientific endeavors into better treatments and cure for children with HGPS.

All applicants are required to complete this application and agreement, and have an executed Material Transfer Agreement, in order to access lonafarnib. Only applicants approved by PRF may have access to this pharmaceutical material. **These materials are not transferable to any non-approved investigator, institution or commercial enterprise.**

Please review, sign, and return a completed Application and Material Transfer Agreement

Name of Applicant (Principal Investigator):
(The Principal Investigator is the individual who is responsible for use of the materials being requested.)
Institution:

Shipping Address:

Phone, Fax:

E-mail:

Grant or contract that supports this research (include name, number and funding period if applicable):

Title of research project:

List here the quantity in milligrams (mg) of lonafarnib being requested: _____

Please note that there is a limited supply of lonafarnib, and researchers are required to order only the mg amount of lonafarnib needed for the current study.

Since one of PRF's important functions is support for individuals affected with progeria, we request written descriptions of your project and its purpose (see below a & b). When your application is approved this information may be used in PRF's newsletter, in press releases and on PRF's web site. (Please include an embargo date, if applicable.) PRF will not publish, nor make public in any form, information within or in support of your Application Package other than the layman's description without prior written consent. The public, written description need not reveal anything that would jeopardize any confidentiality associated with your project.

INSTRUCTIONS:
a: Layman's Statement for The Progeria Research Foundation. (On separate sheet)
In a brief paragraph of approximately 10 lines, describe your proposal and its significance. (This may be used for press announcements, the PRF newsletter and on PRF's web site.)

b: Provide a short scientific description of the research you will conduct using these materials. This application is reviewed for relevance to progeria research by PRF's Medical Director. An abstract from a research grant is acceptable if you have one or would like to submit one.

c: Provide a detailed justification for amount of lonafarnib requested. There is a limited supply of lonafarnib, and laboratories should request enough for current studies only.

Please note that a new PRF application is required for each future research project in which you plan to use these materials for a purpose different from what you describe in this application and from what is in the executed Material Transfer Agreement. If your research focus changes from what is in the executed MTA, PRF will require a new MTA.

The Progeria Research Foundation Lonafarnib Drug Supply Program Agreement

Application forms must be completed as set forth, with supporting documentation attached. If an application is sent in hard copy form, 2 sets are required. Scanned copies with signature will be accepted by email.

Once the application procedure has been completed and the MTA has been executed, then material ordering and transfer shall be closely coordinated with the investigator via phone or Email. Shipment methods shall be coordinated as required for domestic and/or international transfer. Notification of shipping shall be made via email.

This supply is the same used to manufacture capsules for the human clinical treatment trials. If in future, another batch of capsules is manufactured for human use, then the drug supply will be retested for stability at that time. We recommend for best practice that the researcher include an internal positive control with each experiment, to assure appropriate lonafarnib activity. All appropriate pharmaceutical regulatory and export compliance transfer requirements of the FDA, OSHA, DOT, etc., and the transit carrier shall be adhered to. The Progeria Research Foundation is not responsible for any loss, damage or mishandling once materials leave its possession. The transit carrier shall be responsible for materials during shipping.

PRF guarantees that to the best of our abilities and that of our supplier the materials are viable; correctly screened, labeled and stored. PRF provides materials without warranty to their fitness for any particular process. This provision also extends to the byproducts or derivatives. Foremost, the applicant agrees to indemnify and hold harmless PRF from any claims, costs, damages, or expenses resulting from any direct or indirect circumstances arising from use of these materials. By accepting these materials the recipient assumes full responsibility for their safe and appropriate handling. All applicable regulations and laws should be complied with in material use and disposal.

PRF requires that materials from the Lonafarnib Drug Supply Program are distributed solely for use in your laboratory and are non-transferable to other parties. PRF does not want to prevent scientific collaboration but wishes all those wanting to use this material to be applicants for use. PRF wants to avoid any and all conflicts of interest or undisclosed use of these materials. PRF's ethical standards dictate that all researchers using the material shall be approved applicants.

PRF requires recipients of lonafarnib through this program to provide a brief annual report to PRF describing the status of their research. It is a goal of PRF to be aware of the scientific progress being made that could potentially lead to human clinical trials for progeria.

I agree to the terms and conditions as set forth above:

Applicant Name:

Signature: Date:

APPROVED:

Officer of The Progeria Research Foundation:

Printed Name and Title: Leslie B. Gordon, MD, PhD

Signature: Date:

SUBMIT PDF (PREFERRED) OR 2 HARD COPIES OF THE APPLICATION AND SUPPORTING MATERIALS TO:
Dr. Leslie Gordon
The Progeria Research Foundation
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Questions: 978-535-2594