Application and Agreement for Lonafarnib

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

General Information:

It is essential for the research community to continue investigating the effects of lonafarnib on HGPS through preclinical studies. Lonafarnib has shown some benefit to disease in Hutchinson-Gilford progeria syndrome (Gordon et al, PNAS, 2012; Gordon et al, Circulation, 2014). 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 must complete this Application and Agreement, have an executed Material Transfer Agreement ("MTA"), and be approved by PRF in advance in order to access lonafarnib (hereinafter, "Compound"). Only applicants approved by PRF may have access to this Compound. **Compound is not transferable to any non-approved investigator, institution or commercial enterprise.**

Please review, sign, and return a completed Application/Agreement and Material Transfer Agreement ("MTA")

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

Institution:

Shipping Address:

Billing Address: ☐ (Check here if same as 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). The description described in (a) should not exceed one page 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. Donors do need, however, some indication of your project. It is very important that donors understand both the importance and the usefulness of their donations.

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 the Compound. 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 the Compound for a purpose different from what you describe in this application and from what is in the executed MTA. 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 (preferred method of delivery) will be accepted by email.

Once the application procedure has been completed and the MTA has been executed, then Compound 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.

As set forth in the MTA, the Compound will be supplied "As Is." For example, Certificate of Analysis may be included with each shipment and may indicate that the retest date is past-due. We note, however, the Compound is from the same supply used to manufacture capsules for the human clinical treatment trials and the compound has been shown to be chemically stable.

Therefore, we recommend for best practice that the researcher include an internal positive control with each experiment, to assure appropriate Compound 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 the Compound leaves its possession. The transit carrier shall be responsible for the Compound during shipping.

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

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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, Inc:

Printed Name and Title: Audrey Gordon, President and Executive Director

Signature: Date:

SUBMIT 2 HARD COPIES OR ONE SCANNED COPY (Preferred) OF THE APPLICATION AND SUPPORTING MATERIALS TO:

Dr. Leslie Gordon
The Progeria Research Foundation
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