The ODC MDBR Pilot Grant Program, along with The LAM Foundation Easy Breathers Cycling Team, have provided a one-year grant to support research related to LAM in the 2022 Million Dollar Bike Ride.

Eligibility to Respond to this RFA

- Open globally to international applicants
- Individuals must hold a faculty-level appointment at an academic institution OR
- Senior scientific position at a non-profit institution or foundation

**Lymphangioleiomyomatosis (LAM):** The LAM Foundation Easy Breathers Cycling Team, led by LAM patients, family, friends, and The LAM Foundation, has raised funds for **one $75,110 pilot grant** in 2022. The focus of this grant should have a strong likelihood of future federal funding, that use LAM samples, animal models or patient data, and which have the potential to favorably impact human health will be given priority. Examples of desirable topic areas include:

- Better understanding of the molecular derangements in LAM with an aim to identify targets for future development of novel therapeutics
- Improving the existing models or creating new models to study disease pathogenesis
- Biomarker development to enable non-invasive diagnosis, better prognosticate the risk of disease progression, predict the response to treatment, or to act as end points in clinical trials. A biomarker is broadly defined as any objective modality that can measure disease activity and could include quantified biological variables (e.g., blood- or urine-based tests), novel imaging techniques, or patient-reported outcomes
- Molecular pathogenesis-guided pilot clinical trials

**Letter of Interest Instructions:**
Please visit the Orphan Disease Center website to submit your Letter of Interest (LOI), which can also be found [here](#). This one-page LOI is due no later than **Friday, September 16, 2022, by 8pm (EST)**
Full Application Instructions and Review Procedure

NOTE: Full Application is by invitation only after review of Pre-Application

Proposal Due Date: Monday, October 17, 2022 no later than 8pm (EST)
Full application documents are to be uploaded on our website, by invitation only.

FORMAT for documents:

Font and Page Margins: Use Arial typeface, a black font color, and a font size of 11 points. A symbol font may be used to insert Greek letters or special characters. Use 0.5 inch margins (top, bottom, left, and right) for all pages, including continuation pages. Print must be clear and legible; all text should be single-spaced.

Header: There should be a header at the top right on all pages of the PDF indicating the full name of the PI (e.g., PI: Smith, John D.). For your convenience, a continuation page template is included at the end of the application document.

File names: ALL files to be uploaded should start with the LAST NAME of the PI followed by the brief name of the document. Examples: SMITH CV, SMITH Cover Page, SMITH Budget. If files are not labeled properly, you will be asked to resubmit the PDFs before your application can be considered.

CONTENT to be uploaded:

☐ Cover Page/Checklist/Institutional Signature Page [PDF].

☐ NIH-style Biosketch with Other Support of PI and key personnel (5 pages max/PI, including Other Support). [PDF]
  The PI must include accurate and complete information regarding all other sources of grant support (current and pending), including title, abstract, annual and total amount of grant, inclusive funding period, and percent effort.

☐ Detailed Budget and Justification. [combined into one PDF]
  Complete Excel budget sheet (to be provided). Describe justifications in a Word document. Award will be for one year. Proposed funding period: February 1, 2023 – January 31, 2024. Total Budget depends on disease RFA:

Institutions may opt to take up to 10% IDCs from their award totals. Awarded amounts will not exceed Award Totals listed above.

Allowable direct costs
- Salary for PI*
- Salary/stipend and related benefits for graduate student/postdoctoral fellow/technical support
- Travel (up to $1500)
- Laboratory supplies and other research expenses
- IDCs of 10% are included in the total award amount

Unallowable costs
- Consultant costs
- Tuition
- Professional membership dues
- Equipment >$5,000
- General office supplies institutional administrative charges (e.g., telephone, other electronic communication, IT network, etc.)
- Pre-award charges
- Any other expenses not directly related to the project
Beginning in May 2020, PI salary on all ODC Pilot awards will be applicable to the National Institutes of Health Executive Level II Salary Cap. The current NIH Salary Cap for the year 2022 is $203,700. For background and guidance, please refer to the following link:

☐ Research Plan (5 pages max) and Bibliography (1 page max). [combined into one PDF] Include the following sections: Specific Aims, Background and Significance, Preliminary Studies/Data, Research Design and Methods. Research plan should address the following questions: 1) Do you require access to reagents, cell lines, animal models, IRB/ethical board approvals, and/or equipment necessary to complete work? If so, please describe your plan to gain access within the timeframe of this grant period. 2) Have you identified qualified personnel to complete this project within the grant period? If not, please provide your plan to do so. Text citations should use a numbered format. Include all author names in the reference list.

All previous MDBR grant awardees must include a statement of outcomes including publications, patents and additional funding granted as a result of data generated from those grants. Specific aims must be different from those in previous applications.

☐ Appendix [combined into one PDF]
Limited to 5 pages of supplemental information pertaining to proposal or preliminary data only. In addition to 5 pages of supplemental information, a maximum of 3 relevant reprints are also acceptable. Include IRB and/or IACUC approval letters if relevant.

Project Disclosures and No Cost Extensions (NCE):

• NCEs will be granted at the discretion of the ODC.
• Awardees will be limited to 1 NCE request for their award.
• Maximum NCE time awarded will be 6 months.
• NCEs will be granted after a formal request through this form found on the ODC website prior to the NCE deadline with adequate justification.
• If granted a NCE, you are still required to submit an interim scientific report 6 months into the duration of the original award period, regardless of your new project end date.
• In your letter of interest, you will be required to certify that you have identified qualified personnel to complete this project within the grant period PRIOR to the start date of the award. If you have not, you will be required to provide your plan to engage said personnel. Only under extenuating circumstances will personnel issues be considered for NCE requests.

In your letter of interest, you will also be required to state whether or not you require access to reagents, cell lines, animal models, IRB/ethical board approvals, and/or equipment necessary to complete your work. If so, you will be required to describe your plan to gain access within the timeframe of this grant period.
**Lymphangioleiomyomatosis (LAM):** One $75,110 pilot grant available focusing on proposals with strong likelihood of future federal funding, that use LAM samples, animal models or patient data, and which have the potential to favorably impact human health will be given priority. Examples of desirable topic areas include:

- Better understanding of the molecular derangements in LAM with an aim to identify targets for future development of novel therapeutics
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- Molecular pathogenesis-guided pilot clinical trials

**Grant Review Process:**

1) Grants will be reviewed for scientific content and relevance to the goals of the RFA.
2) Full applications proceed through a two-step review process. The first step includes external review and rating with an assessment of the strengths and weaknesses of each application based on the defined review criteria described below. During the second step, funding recommendations are determined based on an assessment of the reviewer scores and written comments. Final decision of funding will be made by Center Leadership.
3) Proposal Content and Review Criteria: The following criteria will be utilized in proposal review.

- **Project Proposal** - Is the proposed project of high scientific quality? Is the budget fully justified and reasonable in relation to the proposed project?
- **Background** - Is the fundamental objective of the study and hypothesis to be addressed clearly defined?
- **Scientific Approach** - Will the proposed specific aims answer the study hypothesis? Will the scientific approach effectively test and answer each specific aim? Are the study goals supported by existing data?
- **Clinical Impact** - Is the answer to the study hypothesis important to our ability to treat or reduce rare disorders/disease incidence and/or mortality? Will the proposed research lead to substantial advances and/or contribute to large leaps of understanding or knowledge that will contribute to reductions in disease incidence and/or mortality within the decade?
- **Research Significance** - Does the study address an important question that is not likely to be addressed without this funding? Does the proposed study offer a unique opportunity to explore an important issue and/or employ a novel approach to this disease research? Will the study outcomes advance our knowledge of this disease and/or contribute to changes in the focus of future research questions or the way we conduct research on this issue?
- **Investigator Qualifications** – Does the investigator hold a track record of outstanding accomplishment as evidenced by peer-reviewed publications and funding awards? Does the investigator have access to the resources and environment necessary to complete the study as outlined?
Anonymous reviewer feedback is shared upon the request of the applicant at the discretion of the Orphan Disease Center where appropriate.

Confidentiality:
The MDBR Grant Program is a confidential process and all content of the LOIs and Full Applications will be kept confidential by the ODC. In order to encourage sharing of new techniques and findings to advance science, after funding decisions are made, the ODC will share a non-confidential lay summary of the research proposals received (required with your letter of intent), including those that were not funded, with each participating funding organization. The ODC aims to respect and protect the integrity of your work, and thus will not release any proprietary information.

Fund Disbursement:
Funds will be issued through a cost reimbursement mechanism executed by purchase order from the University of Pennsylvania. Details of invoicing schedules and reporting requirements will be made available upon award. For additional information, please contact Samantha Charleston at scharle@upenn.edu or 215-573-6822.

A notice about COVID-19: ODC will continue to monitor the global pandemic and will work with awardees to accommodate extensions that allow research aims to be completed safely in a mutually agreeable timeframe.