April 28, 2025

Dear LAM Community,

I'm reaching out with an urgent request.

On April 17th, our Rare Lung Disease Consortium (RLDC) Grant proposal— *Clinical Trial Readiness for Rare Lung Diseases*— was withdrawn by the NIH over a one-page form in a 1000-page document. The form, which justified the inclusion of a single foreign site (Ireland), had been requested at the time of submission last August. However, based on longstanding NIH practice, applicants were permitted to provide such documents later in the review process — a process followed by many successful grants in previous cycles. Only in the past three weeks, under new, stricter NIH budget policies, was this flexibility quietly revoked.

Despite receiving a letter from the NIH last September confirming our proposal had advanced to study section review and assurances that any additional requested materials would be flagged, no such notice was ever given. We were blindsided, as were many other investigators. There were two other rare lung disease consortia, CEGIR (eosinophilic diseases) and FCDGC (neurological diseases) similarly caught in this abrupt shift and withdrawn. What sets the RLDC, CEGIR and FCDGC grants apart is that the Rare Disease Consortium funding mechanism is only available once every five years, whereas most other investigators can simply reapply in four months.

In past RLDC funding cycles, we have:

- Built a global network of clinics dedicated to rare lung diseases,
- Identified sirolimus as the first FDA-approved treatment for LAM,
- Developed VEGF-D, a diagnostic blood test that spares patients from invasive lung biopsies.

Today, no new LAM clinical trials are enrolling. Trials remain scarce because it is costly and logistically difficult to reach a geographically dispersed patient population.

This RLDC proposal was designed to change that, by: developing patient-driven databases, blood tests that become tools for diagnosis, management, and better clinical trial design, and home-based monitoring. This infrastructure makes LAM research faster, more affordable, and more attractive to pharmaceutical partners.

We are **not** asking for special treatment. We are simply asking for what is fair: that our Rare Disease Consortium proposals, given the unique five-year funding cycle, be granted an exception, and allowed to move forward to peer review alongside all other rare disease applications.

How you can help:

Call or write to the four Co-Chairs of the Rare Disease Congressional Caucus:

- Rep. Gus Bilirakis (R-FL): 202-225-5755
- Rep. Doris Matsui (D-CA): 202-225-7163

- Sen. Roger Wicker (R-MS): 202-224-6253
- Sen. Amy Klobuchar (D-MN): 202-224-3244

Tell them:

> *"I'm asking for your urgent help to protect rare lung disease research. We urge you to contact NIH Director Dr. Jay Bhattacharya to reinstate the RLDC grant application and other Rare Disease Clinical Research Network grants before May 5. Without reinstatement, we risk setting back rare disease research by years."*

If you are willing, a few heartfelt sentences about how you have personally benefitted from any of the RLDC initiatives mentioned above would be especially powerful.

If you don't live in FL, CA, MS, or MN:

- Find your Rare Disease Caucus representative here, or
- Contact your Member of Congress here.

Amplify the message:

- Share this email widely.
- Reshare on social media.
- Speak to local media.
- Activate your personal and professional networks.

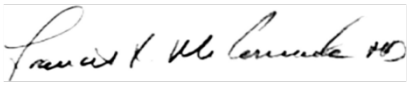
Timing is critical.

The NIH study section meets May 5th. After that, there is no path to reconsideration — and this opportunity won't come again for five years.

We know this news is heavy. But we are not giving up. We are fighting for LAM patients, for every individual touched by rare lung disease, and for the future of rare disease research itself.

Please stand with us. The clock is ticking.

In unity and urgency,



Frank McCormack, M.D.
Scientific Director Emeritus, The LAM Foundation
Gordon and Helen Hughes Taylor Professor and Director
Division of Pulmonary, Critical Care and Sleep Medicine
The University of Cincinnati School of Medicine