

LAM Treatment Alliance Global LAM Patient Summit 11-13 January 2008, Oxford, UK

Summit Report and Next Steps

How can patients facilitate research?

Fifty-seven researchers, clinicians, patients and patient family members representing seventeen countries met in Oxford recently to make concrete progress in the search for a treatment. The aim of the meeting was to establish ways in which patients can support and facilitate research.

The most limiting factor for research is not lack of interest from researchers, nor money. It is access to LAM tissue and patient data. Researchers are awaiting tissue including fluid from LAM patients in order to continue their work. Scientists are often deterred by the small number of patients in any given country, which is an obstacle for their research. During our meeting in Oxford these two major problems were addressed and the following solutions found.

1. European tissue network

A major limiting factor for LAM research is access to tissue. Tissue is anything that potentially contains LAM cells, such as samples of chyle, blood, urine, lung and kidney in addition to fluid from bronchial lavage.

Tissue is desperately needed for LAM research.

The National Disease Research Interchange (NDRI) in the US has been coordinating fresh and fixed (preserved) LAM tissue procurement and distribution since last March with a very strong track record and commitment. NDRI is willing and able to extend this service to Europe and procure, process and deliver tissue to researchers in real time.

In a session dedicated to the tissue shortage problem, it was decided that the NDRI will manage LAM tissue outside the US as well as within the US, creating an efficient global research network. Since the NDRI has the required experience and expertise, working with the NDRI is the fastest solution to providing researchers with the access to tissue they urgently need. In a vote taken at the Summit, there was overwhelming support to begin work with the NDRI immediately. The NDRI will provide an immediate, low cost, transparent and neutral solution to the tissue shortage, as well as a comprehensive understanding of the needs of researchers and a proactive approach to tissue procurement and distribution. The NDRI is not just a tissue bank. They actively solicit requests from researchers who would like to work on LAM tissue samples and they have the means to marry tissue and researcher within a matter of hours. This is vital with fresh tissue. They are also committed to helping register patient donors and ensuring quick turnaround time. Most NDRI efforts will be directed towards Europe at the outset; NDRI will investigate options for assisting tissue procurement and distribution in other parts of the world.

What can you do?

If you would like to donate your tissue and fluid to LAM researchers, the first step is to provide your contact details and fill in a consent form. The consent forms are now being updated and translated into languages other than English. Patients in countries where English is not an official language need to fill in the consent form in the official language of their country, even if they speak fluent English. Right now, you can click on the link below and leave your details with the NDRI, so they can send the forms out to you as soon as they are ready. If you live in a country where English is the official language, you can register and consent immediately.

Click on the link below and then click on 'Register to donate'
http://www.ndriresource.org/Donor_Programs/Individual_Donors/37/

If you know you are going to have a biopsy, chest drain, transplant, pleurodesis, bronchial lavage or **any other invasive procedure**, please send the NDRI an email saying what procedure is going to take place, where and when (of course those waiting for a transplant cannot provide the time of the operation, but if all the paperwork is in place, it will save time when the call does come through). For other procedures, a day's warning is better than no warning. The more advance warning we can give the NDRI, the better the chances that the procurement will work in real time. We will soon circulate a comprehensive list of tissues (fluid and solid) that are important to researchers. It is very likely that blood and urine will be increasingly valuable, so stay tuned!

This is a link to the NDRI December newsletter with news on their taking on the LAM tissue repository (on p.2):
http://www.ndriresource.org/SiteData/docs/ARareInsight12_07/0bc3e8212189efed253bd5109c43aed3/ARareInsight12_07.pdf

2. Global LAM database: LAMsight

The MIT (Massachusetts Institute of Technology) Media Lab is currently designing a database that will enable LAM patients all around the world to register their details. This is important so researchers can learn how many patients exist in each country and other general information about them. This will help in design of clinical trials and will also enable researchers and other patients (if you permit this option when registering) to contact you with news about developments in research and clinical trials.

Data will be extremely well-protected from unauthorized access and patient privacy is a high priority in the database design. As a registered user, patients will also have access to aggregated data. LAMsight will be useful for clinicians interested in disease management and new and existing LAM researchers seeking to better understand the disease based on access to a much larger population than would be the case. At the moment, a preliminary version of the database is being tested. There will be an announcement posted on the LTA homepage and as many other LAM organization websites as possible as soon as registration is open to the entire LAM community. We expect open registration to begin in 3-6 months.

The database will eventually give patients more opportunities to answer their questions systematically. Questions and answers that are today posted on local mailing lists will be effectively organized and could be accessed by many more patients.

Clinicians and researchers present at the summit are convinced that this is the best option to cope with the small numbers of LAM patients and would give researchers access to a much larger pool

of data and means to contact willing patients to obtain further information and invite them to enroll in clinical trials. We will be working closely with the research community to ensure that the site is designed in a way that is maximally useful and addresses both scientific and regulatory concerns.

3. Clinical Trials and off-label drug use

Dr Ulrich Costabel (Germany) emphasized the importance of clinical trials and of having sufficient patient numbers to give statistically significant results. Drug trials will only be possible if there are sufficient numbers of patients willing to enroll at a specific time. Without appropriately designed clinical trials and sufficient patients participating in them, it will be very difficult to find an effective treatment for LAM/ to assess the efficacy and safety of any potential therapy. The clinicians at the summit also acknowledged that fast-declining patients may not be able to wait for trials. To address this issue, the LTA will immediately start work with scientists to create an off-label use protocol.

It is only by collaboration and coordinated efforts that we will make the fastest possible progress in our quest for a treatment. By donating our tissue and participating in the initial design of the global database, and then in the final version when it is ready, we can help researchers to help us!

Yours most sincerely,

The Global Patient Summit Coordinating Committee