Making the Most of your LAM Clinic Visit

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Goals Today

1) Discuss the roles of LAM clinics
2) Share strategies for how to prepare for visits
3) Provide roadmaps for common decisions regarding LAM care
4) Share resources from the TS Alliance and The LAM Foundation
Objective of The LAM Clinics

• “…to focus LAM care to medical institutions or hospitals that have the interest and expertise to deliver state of the art, coordinated, multidisciplinary LAM care, and to perform cooperative research with other LAM clinics.”

➢ To improve the care and treatment of LAM patients
LAM Clinic and Research Network

33 U.S. LAM clinics
24 International LAM clinics
Thoughts about goals of the Vanderbilt LAM Clinic

- To foster the multidisciplinary expertise needed to care for LAM patients
- To facilitate access to the latest advances in LAM care
- To facilitate the research needed to improve treatment for LAM
- To educate other providers
- To provide LAM screening for individuals with TSC
‘Hats’ of a LAM Clinic Director

- Different for different providers and institutions
- In addition to care of LAM patients, may include:
  - Care of other Rare Lung Disease patients
  - Care of other pulmonary patients (often critical care or sleep also)
  - Outpatient and inpatient responsibilities
  - Teaching
  - Administration
  - Research
  - Other service
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Scheduling Considerations

1) Do you need to see other providers?

2) What PFTs are needed?
   • Bronchodilator testing?
   • 6 minute walk test? (dress comfortably)

3) Are labs needed?
   • If so, do you need to be fasting?
   • Do these need to be timed for a sirolimus trough level?
   • If VEGF-D is being done, send-out paperwork will be needed.

4) Is other testing needed?

5) Do you need time for research participation?
Outside records

1) Don’t assume that they have been sent.

2) Your LAM clinic providers won’t know records are missing unless you tell them that you had testing.

3) NIH records require signing a NIH release form.
   - CD of imaging studies must be specifically requested in addition to paper records.

4) Bring a current medication list
   - Especially important if you are taking sirolimus or everolimus, so your provider can be aware of potential drug interactions
Setting Goals for your visit

1) Written lists of questions are appreciated by most providers. (Try to prioritize)

2) If you have had major health changes or ‘big’ new questions to discuss, consider sending a message to your provider in advance of the visit so that he/she can better prepare to answer your questions.
Communication between visits

1) What works best for you and your clinic?
2) Sick care plan
3) Lab monitoring plan
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Scenario #1: Woman with TSC, not known to have LAM
Age is a risk factor for LAM in women with TSC. The prevalence of cystic lung disease increases with age.
There is a wide spectrum of LAM disease severity in women with TSC.

MODERATE

VERY MILD

Cudzilo and Young, Chest 2013
Scenario #1: Woman with TSC, not known to have LAM

1) Discussion of symptoms, health status
2) Discussion of approach to screening and interval for repeat screening
   • PFTs
   • Chest HRCT
   • Consideration of serum VEGF-D Review of other TSC or LAM manifestations outside the lung
3) Coordination with other providers
4) Health maintenance (exercise, vaccinations, etc)
5) Patient education, questions
6) Updates about current efforts in TSC & LAM, ongoing research
VEGF-D levels discriminate the presence or absence of LAM in adult females with TSC.

TSC only = women with normal chest HRCT

Young et al, Chest 2010
Scenario #2: Woman with LAM, not on sirolimus

1) Discussion of symptoms, health status

2) PFT monitoring
   • Frequency may depend on history of stability versus progression, risk factors for progression, your preferences

3) Risk assessment
   • Consider serum VEGF-D (perhaps yearly)

4) Screening/ Review of other TSC or LAM manifestations outside the lung; coordination with other providers

5) Health maintenance (exercise, vaccinations, etc)

6) Patient education, anticipatory guidance, questions

7) Updates about current efforts in LAM (and TSC) and ongoing research
Scenario #3: Woman with LAM, on sirolimus

1) Discussion of symptoms, health status, sirolimus side effects
2) PFT monitoring, typically every 3-6 months
   • Frequency may depend on severity of lung disease, stability of lung function, duration of sirolimus use, tolerance of sirolimus, and other factors
3) Lab monitoring
4) Screening/ Review of other TSC or LAM manifestations outside the lung; coordination with other providers
5) Health maintenance (exercise, vaccinations, etc)
6) Patient education, anticipatory guidance, questions
7) Updates about current efforts in LAM (and TSC) and ongoing research
Care Models: What are your needs and how does a LAM clinic fit?

• ‘Thankfully my LAM is mild and has been stable. I want to have the monitoring I need, but…’

• ‘The LAM clinic is out of network for my insurance, and I like my lung doctor who diagnosed me with LAM. But I want to make sure that my doctor and I are up to date on the latest… What are my options?’

• ‘I live several hours from your clinic and it is hard for me to come regularly… Can you just tell my doctors what to do?’

• I go to the NIH. Do I still need to come to a LAM clinic?

• Can I go to more than one LAM clinic, either for a 2nd opinion or research studies?
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Common Questions

1) Do I have sporadic LAM or TSC?
TSC affects people differently.
Tuberous Sclerosis Complex (TSC)

• Autosomal dominant syndrome due to inherited or sporadic mutations in tumor suppressor genes \( TSC1 \) and \( TSC2 \)

• Occurs in 1 in 6000 births
  ➢ ~ 1 million people worldwide; ~ 50,000 in U.S.

• Causes a spectrum of manifestations including tumors in multiple organs
  ➢ Only some individuals have overt developmental disability.
Features of Tuberous Sclerosis Complex

- Cortical tubers
- Subependymal nodules
- Renal angiomyolipoma
- Dental pits
- Angiofibromas
- Ash leaf
- Subungual fibromas
- Shagreen patch

Courtesy of F McCormack/ The LAM Foundation
# TSC Clinical Diagnostic Criteria

<table>
<thead>
<tr>
<th>MAJOR FEATURES</th>
<th>MINOR FEATURES</th>
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<tbody>
<tr>
<td>1 Hypomelanotic macules (&gt;3, at least 5-mm diameter)</td>
<td>1 “Confetti” skin lesions</td>
</tr>
<tr>
<td>2 Angiofibromas (&gt;3) or fibrous cephalic plaque</td>
<td>2 Dental enamel pits (&gt;3)</td>
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<tr>
<td>3 Ungual fibromas (&gt;2)</td>
<td>3 Intraoral fibromas (&gt;2)</td>
</tr>
<tr>
<td>4 Shagreen patch</td>
<td>4 Retinal achromatic patch</td>
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<tr>
<td>5 Multiple retinal hamartomas</td>
<td>5 Multiple renal cysts</td>
</tr>
<tr>
<td>6 Cortical dysplasias*</td>
<td>6 Nonrenal hamartomas</td>
</tr>
<tr>
<td>7 Subependymal nodules</td>
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<tr>
<td>8 Subependymal giant cell astrocytoma</td>
<td></td>
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<tr>
<td>9 Cardiac rhabdomyoma</td>
<td></td>
</tr>
<tr>
<td>10 Lymphangioleiomyomatosis (LAM)†</td>
<td></td>
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<tr>
<td>11 Angiomyolipomas (&gt;2)†</td>
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</tbody>
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Definite diagnosis: Two major features or one major feature with >2 minor features.

Possible diagnosis: Either one major feature or >2 minor features.

*Includes tubers and cerebral white matter radial migration lines.

†A combination of the two major clinical features (LAM and angiomyolipomas) without other features does not meet criteria for a definite diagnosis.
Signs and Symptoms of TSC

Because TSC affects no two people the same, there is no sure way to predict where and how the disorder will manifest. To better understand its potential impact, click below on the organs most often targeted.
Many common questions asked at LAM clinic visits

1) What are the side effects of sirolimus?
2) What is a trough level?
3) What is new in LAM research?
4) Have you ever heard about ‘x’ in patients with LAM…
Patient Care

LAM is a very heterogeneous disease with some patients remaining stable over many years and other declining more rapidly. Many clinicians do not treat asymptomatic LAM patients with any specific therapy until it is clear that they have progressive disease. In the face of the present therapeutic uncertainty, the patient and physician should make all treatment decisions jointly, after thorough discussion of the risks and limited available data.

Renal angiomyolipoma

Pleural complications in LAM

Role of hormones and pregnancy in women with LAM

Other pulmonary management issues

Bone disease management

Cardiovascular health

LAM and air travel

Vaccinations
ER Medicine Quick Facts

The following medical information is provided as a general resource only. It is not intended to be used for patient education. It does not create any patient-physician relationship and should not be used as a substitute for professional diagnosis and treatment.

Lymphangioleiomyomatosis (LAM) is a rare lung disease in women that results in diffuse cystic changes in the lung parenchyma. It is associated with recurrent pneumothoraces, chylos pleural effusions, progressive respiratory failure and abdominal tumors, including lymphangiomyomas or angiomyolipomas, which can spontaneously hemorrhage and result in pain, anemia and hypotension.

Here are some insights for treating patients with LAM in the ER:

- **Pneumothorax** should generally be managed with chest tube drainage followed by pleurodesis on the first event.
- **Cystic changes** due to LAM are often not apparent on chest X ray. High-resolution CT scanning of the chest is the most definitive imaging test.
- **Pleural effusions** usually represent chylothorax. This may be verified by thoracentesis and assessment of pleural fluid triglycerides and cholesterol.
- **Renal angiomyolipomas** may bleed spontaneously producing flank pain, hematuria, hypotension and anemia.
- **Lymphangiomyomas** may present as low-density abdominal lesions that mimic lymphoma or other malignancies.
- If the patient is to undergo anesthesia, the anesthesiologist should be advised that the patient is at risk for a pneumothorax with positive pressure ventilation.
- **RAPAMUNE® (sirolimus)** is an FDA approved drug to treat LAM. Special precautions should be taken for patients using sirolimus as it can impair wound healing in the event of urgent surgery. Sirolimus can also produce lung injury, immunosuppression, mouth ulcers, peripheral edema, acne like lesions and elevations of cholesterol.
Supplemental Oxygen Guide

There is an increasing number of issues shared within our LAM community about access to liquid oxygen therapy. The LAM Foundation has teamed up with other rare and chronic lung disease organizations to fight for your rights to the most appropriate oxygen therapy. We are grateful to the COPD Foundation for sharing the attached educational/advocacy materials with us. If you are experiencing issues with your oxygen supplier, be sure to read through our Supplemental Oxygen Guide. Even if you have private insurance, consider making phone calls regarding the issues you’re facing. The COPD Foundation tracks trends and issues with suppliers, no matter the insurance payer. By speaking up, you will help us build our case for better access and reimbursement. The LAM community perspective is particularly important because we need to fight for the rights of active people who want to stay mobile – not tethered to a tank at home.

Click HERE (/PORTALS/0/FILES/SUPPLEMENTAL_OXYGEN_GUIDE.PDF?VER=2016-02-10-141945-657) to access the Supplemental Oxygen Guide.
Current Trials and Studies

Part of the mission of The LAM Foundation is to fund promising research. Our researchers need your help in order to conduct their research. Please see the open protocols below to see if you meet any of the requirements and find out how you can participate.

Study of the Disease Process of LAM - National Institutes of Health

Dr. Joel Moss

CLICK HERE (HTTPS://CLINICALTRIALS.GOV/CT2/SHOW/NCT00001465?TERM=STUDY+OF+DISEASE+PROCESS+OF+LAM&RANK=2) for more details.
MIDAS: Multicenter International Durability and Safety Of Sirolimus in LAM Trial

Objective: To determine if long term sirolimus is safe and effective in LAM

Approach: Registry based monitoring of long term sirolimus safety and efficacy in a cohort of patients with TSC and/or LAM

→ Being conducted across LAM clinic network through the Rare Lung Diseases Consortium

→ PI: Frank McCormack, Univ. of Cincinnati
LAM360 Community FAQ

What is LAM360 Community?

LAM360 Community is an online community for individuals affected by LAM, including patients, family members, caregivers, physicians and other healthcare providers. It provides a single online home for these many individuals. LAM360 Community provides our target audience with an engaging and interactive collaboration environment through which they can have more direct interaction with each other and with the Foundation.

LAM360 Community provides community members with a comfortable venue to share thoughts and ideas, ask questions, start discussions, read and comment on blogs and communicate with peers, thought leaders and community managers.
Thank You!

www.tsalliance.org  www.thelamfoundation.org

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