LAM at the European Respiratory Society Congress
Munich, September 2014

The annual congress of the European Respiratory Society (ERS) is one of the largest and most important in the respiratory calendar. This year it took place in Munich, and was attended by 21,000 delegates! Moreover, thanks to the efforts of Italian LAM patient, Iris Bassi, there were two high-profile LAM events at this year's congress. A morning patient workshop discussed the top priorities of patients living with the disease. Later the same day, a scientific symposium was held at which four leading LAM specialists - including Simon Johnson - presented an update on LAM. For a rare disease such as LAM, it was wonderful to have such well-attended, high-calibre events.

Patient Workshop & Results of the Patient Questionnaire

Many of you will be aware of an online questionnaire, distributed widely across Europe and beyond in June/July 2014, which sought to determine the top 10 priorities of women living with LAM. This survey was initiated and coordinated by Iris and staff of the ERS's patient arm, the European Lung Foundation (ELF). The results of this survey helped inform the discussions at the patient workshop.

Analysis of the 504 responses identified the following as being the top 10 priorities of LAM patients (in descending order of importance):

1. Improving methods of identifying patients with active disease
2. Availability of drugs for LAM in all EU countries
3. All patients can access a specialist LAM centre
4. Lung transplants should be available to more patients and with shorter waiting times
5. Improving non-invasive diagnosis (e.g. diagnosis by patient history, X-rays and CT scans rather than by bronchoscopy and/or open lung biopsy)
6. Patients can access treatments such as low dose sirolimus/everolimus
7. Improving care for LAM patients in general (non-specialist) hospitals/services
8. The opportunity for patients to hear about and participate in clinical trials
9. Improving diagnostic biomarkers so that diagnosis is faster and more definitive
10. The development of a European-wide organ donor service

The workshop was attended by around 45 people representing patients, patient organisations, medical professionals, researchers and industry from 12 different countries (Austria, Bulgaria, Croatia, France, Germany, Ireland, Italy, Portugal, Spain, Sweden, the US and the UK). Consequently, one of the biggest benefits of the patient workshop was the opportunity for patient representatives and LAM specialists from so many different countries to gather together and exchange ideas.

Clearly, some of the priorities identified in the survey could be acted upon more easily than others. However, this format did provide us with some good topics for small-group discussion. ELF staff led and facilitated the workshop, with a summary of the workshop appearing in the December edition of their publication, *Breathe*, and the intention to follow up the results of the questionnaire and workshop with an article in a free to access (i.e. open access) academic journal.

In our group, we identified one priority as being particularly important - a possible update to the ERS Guidelines on the Diagnosis, Treatment & Management of LAM. These guidelines are really useful for patients and their doctors, but it is now nearly 5 years since they were published. While much of the information remains the same, experience with sirolimus / rapamycin is now much greater, and the guidelines need to be updated to reflect that experience. Unfortunately, it is not possible simply to add an addendum to the existing ERS guidelines; however, it seems the clinicians discussed this issue after the workshop, and mooted the possibility of joining forces to complete guidelines currently being developed by the Americans. As patients, we should be very supportive of such a move.

One general point raised by many questionnaire respondents was the difficulty in accessing good quality information about LAM, particularly in different languages. Therefore, the ERS/ELF has set aside some funding to prepare and publish information about LAM guided by patients, and to be translated into many languages. This is very positive. As yet, I'm not aware of how this would work in practice; it may be difficult to coordinate information amongst so many different organisations. However, a considerable body of information on LAM already exists, and it may be best first to audit what material has already been prepared by national patient organisations and then choose one (or a mixture of more than one) publication to form the basis of that information, which is then translated.

Scientific symposium

It was a real coup to have four such leading LAM figures - Sergio Harari, Simon Johnson, Joel Moss and Frank McCormack - presenting on the same platform. And that they spoke to a vast auditorium, which was pretty full, is also very encouraging.

Reviewing my notes from the LAM meeting in Barcelona two years ago, it seems that the themes emerging then continue to dominate current thinking:

- There continues to be considerable progress in the understanding of the disease.

- With more patients having been on sirolimus for longer periods, there is more (and positive) evidence about the effectiveness of the drug sirolimus for LAM.

- However, we continue to face the problem of the condition being so rare, which means international collaboration remains key. (This is also why it's important for LAM patient groups to work together, whether through meetings like this or through umbrella organisations such as the European LAM Federation).

- Trials are becoming more difficult, for many reasons, including i) recruitment, as the disease is so rare; ii) the prohibitive costs and practical difficulties of trials, with such a wide-scattered patient base; and iii) - a new problem - because there is a treatment which seems to work, patients who are already on that treatment are unwilling or reluctant to come off it in order to take part in another trial.

- It is becoming ever more important to identify biomarkers for LAM. The benefits of identifying biomarkers include: i) quicker and less invasive diagnosis of the disease; ii) more accurate prognosis of disease progression for individual patients; iii) allows trials to take place with fewer numbers of participants over shorter periods of time. The US LAM Foundation is hosting a biomarker innovation summit in the US in November, which Simon is attending.

Thanks are due to Iris Bassi, for her persistence and persuasion, and the ERS and ELF for committing time, money and a platform for making these events happen. This was another important step for our rare condition, from which LAM patients will surely benefit.

Gill Hollis
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