

The EGA members meeting took place on Tuesday 28th & Wednesday 29th June in Zaragoza, Spain and was – as ever - an amazing event with a great atmosphere.

Friends, old and new, gathered to share stories and experiences and there was much laughter and fun, and of course a lot of hard work by all...



Biennial meeting

There were 46 participants representing 32 countries: Albania, Austria, Belgium, Bosnia & Herzegovina, Bulgaria, Canada, China, Croatia, Czech, Denmark, Estonia, Finland, France, Germany, Greece, Guatemala, India, Israel, Italy, Jordan, Latvia, Macedonia, Moldova, Paraguay, Poland, Romania, Russia, Serbia, Slovenia, South Africa, Spain and the UK.

The programme was designed by Anne-Grethe Lauridsen (Denmark) with input from the EGA board members. On Tuesday morning, the board presented key work programme areas, focusing on what has been achieved and where the EGA has made a difference: International Gaucher Day; Advocacy training; Communication; Strengthening relationships; The EGA volunteer charter; Go With Gaucher; Humanitarian Aid; European Working Group on Gaucher Disease; Best practice sharing project.



In the afternoon, presentations were made by five EGA representatives to share experiences of support from the EGA in South Africa; Serbia; Albania; China and Canada in the areas of access to treatment; forming new patient groups and raising awareness.

We would like to thank Dr Hylton Sevitz, Biljana Jovanovic, Fation Dragoshi, Yifan Deng (pictured left) and Christine White for sharing their stories with us.

On Wednesday small workshop groups were formed to discuss key topical areas: access to treatment; creating awareness; beyond Gaucher; interest and training of clinicians/doctors; running the patient association. The feedback from these groups will be discussed by the board when they next meet in September and used to take forward the work of the EGA.

In the afternoon, participants also had the opportunity to meet representatives from industry (Genzyme, Orphazyme, Pfizer and Shire) who gave short presentations and took questions from the audience.

Minutes from the meeting will be sent out separately after the summer but in the meantime you can view all the presentations, **including the EGA video**, here: https://www.dropbox.com/sh/pynbwwn41te8nmt/AAQjywLmj_P-w6ndmAT90Zda?dl=0

Change to the constitution

During Tuesday's programme, Pascal Niemeyer and Tanya Collin-Histed gave a presentation on behalf of the EGA board to recommend that the EGA's constitution be amended to allow all members to have equal rights. Participants were split into small groups and asked to discuss the plans for the EGA to eventually become a global organisation.

Two board members facilitated each group and, as impartial moderators, were not able to express their own or the board's opinion.

One participant took notes and one participant presented the results of the workgroup: what is the groups answer to "How would you decide?"; did you find a consensus?; summary of key discussion points; pros and cons.

Some very interesting discussions were had as a result of the workgroups and on Wednesday morning, during the biennial general meeting, all full members were asked to vote to adopt the amended Articles of Association set out in the Special Resolution.

The votes, including those posted by proxy, were counted by an external person from the organising agency and verified by Jo Higgs.

More than two thirds of the participants entitled to vote agreed with the board's recommendation and changes will now be implemented **gradually** for the EGA to become a global organisation.

New board

After 8 years of being chairman, Jeremy Manuel stood down and announced that the board had elected Pascal Niemeyer to take over leadership of the EGA.

The new board was formally announced as: Vesna Aleksovska (Macedonia); Biljana Jovanovic (Serbia); Anne-Grethe Lauridsen (Denmark); Jeremy Manuel (UK); Pascal Niemeyer, Chair (Germany); Johanna Parkkinen (Finland); Jasenka Wagner (Croatia); Sandra Zariņa (Latvia) and Irena Žnidar (Slovenia).



EGA film

The EGA has made an image film that was presented at the biennial meeting and at the EWGGD. The film is now available to view on our website and Facebook page and will shortly be available on YouTube.

Ethical awareness

Since the biennial meeting in Haifa, the EGA board has been working to strengthen the importance of the Code of Practice and in Zaragoza Sandra Zariņa gave a presentation to encourage members to adopt a Code. Sandra was very pleased to be approached by several members asking for further support in adopting a Code of Practice. It has proved that members evaluate the importance of having the Code in building credible, accountable and transparent relationship with the commercial organisations, including pharmaceutical companies.

Humanitarian Aid

At this year's EWGGD the EGA made a request to the EWGGD Board to include a session on 'Humanitarian Aid' which was agreed and took place on Friday 1st July. The session was chaired by the EGA's new chairman Pascal Niemeyer and Prof Carla Hollak. By including this session in the EWGGD programme we wanted to raise awareness of the ongoing huge unmet needs of patients globally without treatment and also to highlight the challenges faced by the treating physicians in countries where the Government does not support reimbursement for enzyme replacement therapy for its citizens.

We had four invited speakers including the EGA's CEO Tanya Collin-Histed;

- The EGA's role in supporting the growing unmet need of Gaucher patients globally that cannot access treatment in their own country - Tanya Collin-Histed (EGA)
- Working within the Governments capabilities to support Gaucher patients in Pakistan; an alternative strategy - Prof Huma Cheema (Pakistan)
- Managing the symptoms of Gaucher patients without access to ERT in India – by video; Dr Sujatha Jagadeesh (India)
- A practical experience of dealing with two siblings from Jordan & the German experience of managing refugees with Gaucher disease in Germany - Dr Jörg Reinke (Germany)

In summary

It was clear to see that in many of the countries where patients do not have access to ERT there are dedicated doctors working hard to create sustainable clinical infrastructure, registries, networks within their countries to share good practice and constant pressure working with the patient organisations and Pharma to put pressure on their Governments to provide reimbursement for their patients. The Pharma Companies are playing their part by providing differing levels of charitable access to a limited number of patients and working to raise awareness of the disease and improve diagnosis. However it was recognised that there is still plenty of room for improvement by all of the companies involved in the Gaucher community.

However we as a patient community must recognise that there cannot be just one solution to this unmet need. No longer is it enough to say that all patients should have ERT. What is gold standard of ERT/SRT in many countries is not possible today, tomorrow or even in the next few years to come. Therefore we must support these countries and work with them to look at how best this growing unmet need can be met. Yes, it is a challenge! There are alternative solutions to be explored to help patients with treatment, for them to have a chance at a future. For example; The use of Ambroxol; The role of BMT; encouraging and navigating pharmaceutical companies running clinical trials to work with experts who have untreated patients. We recognise that the use of BMT may be controversial and for many countries unthinkable but in some countries they are faced with no choice, simply BMT or death.

And finally...

The session was well received, created a lot of conversation and opinions which went beyond the conference room. We believe this session brought this issue to the forefront and will result in more collaboration, more action by the companies and ultimately hope for many untreated Gaucher patients globally.

We will send out a newsletter highlighting some of the important outcomes from the EWGGD meeting after the summer.

Austria, Belgium, Bosnia & Herzegovina, Bulgaria, Canada, China, Croatia, Czech Republic, Denmark, Estonia, Finland, France, Guatemala, Germany, Greece, India, Ireland, Israel, Italy, Japan, Jordan, Latvia, Lithuania, Luxembourg, Macedonia, Mexico, Moldova, Netherlands, Norway, Paraguay, Poland, Romania, Russia, Serbia, Slovakia, Slovenia, South Africa, Spain, Sweden, Switzerland, UK, Ukraine, USA

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