

# Euro-Histio-Net Meeting, Paris

Start: November 22, 12 a.m.

End: November 23, 4.30 p.m.

Participants: I Astigarraga, M Bohns Michalowski, B Boshoeven, L Debar, J Donadieu, J-F Emile, J Estrada-Veras, J Haroche, M Girschikofsky, R Haupt, G Kaltsas, P Makras, I Malinowska, B A Miller, J-L Miron, M Salamonowicz, J Salotti, M Sedky, A Tazi, C van den Bos, J Visser, E Schaefer

## 1: Welcome lunch and exchange between project partners.

Jean Donadieu, coordinator of the EHN project, opened the official part of the meeting at 1 p.m. and summarised the basic objectives of the Histio Net project, the overall budget per year, and the major achievements. He pointed out the incidence and prevalence of histiocytoses in EU and the health problems caused by these diseases. Finally he showed the problems to raise sufficient funds for the maintenance of the project and the challenges which have to be met.

Furthermore, Jean introduced the current EU policy for rare diseases, the directive for cross-border healthcare. He explained the Europlan initiative and the EUCERD recommendations on rare disease European reference networks.

## 2: Short round of introductions and approval of new Histio Net partners

Meanwhile, Histio Net has 34 partners from 15 EU countries and 7 countries outside EU. 22 partners are physicians and 12 partners are representatives of patient associations for histiocytoses. No new partners were approved.

## 3: European Reference Networks

The basic contents of the directive 2011/24/EU of the European parliament and of the council of 9 March 2011 on the application of patients' rights in cross-border healthcare were presented. It entered into force in April 2011 and the deadline for transposition in the EU Member States was 25 October 2013. The directive reinforces European Reference Networks (ERN) for rare diseases. The status quo of discussion concerning procedures for identifying and designating ERN was presented and options for new histiocytosis projects were discussed.

There remain some important open questions:

- How much funding for ERN activities will be available?
- How will "evidence of good clinical care and outcomes" be defined?
- Which concrete criteria and conditions for ERN members will be adopted?
- Will EUCERD-recommendations be integrated?
- How will sustainability of ERN be achieved?

It was decided that the project partners will observe the national and European implementing acts in order to be prepared when the next call is launched. At that time, decisions will be taken, who will contribute to any application.

#### **4. Round tables physicians' networks for LCH – situation in the different countries**

and

#### **5. Round tables patients/parents associations – situation in the different countries**

The national activities concerning the different childhood and adult histiocytoses were presented for Austria, Brazil, Egypt, France, Germany, Greece, Lebanon, Netherlands, Poland, Italy, Spain, UK, USA.

#### **6. Histio Net Project**

A five year report for the years 2009 to 2013 was presented. It included a short review of the budget, the scientific activities and the main achievements of the period, which are the following:

- LCH guidelines for childhood LCH have been published in Pediatric Blood and Cancer in 2013. They are available online in 5 languages: English, French, German, Italian, Spanish. These EHN guidelines are linked from the ORPHANET website.
- LCH recommendations for adult LCH have been published in Orphanet Journal of Rare Diseases in 2013. They are available online in English. These EHN recommendations are linked from the ORPHANET website.
- A list of frequently asked questions for rare diseases in general is available online in Arabic, English, French, German, Italian, Spanish.
- A list of frequently asked questions for LCH is available online in Arabic, Bulgarian, English, French, Italian, Spanish.
- A list of frequently asked questions for HLH is available online in English, French, German, Polish, Spanish.
- A list of frequently asked questions for ECD is available online in English.
- A map of EHN coordinators is available online.
- Maps of experts for LCH are available online for Austria, Bulgaria, France, Germany, Greece, Italy, Poland, Spain, Sweden.
- Maps of Experts for HLH are available online for Germany, Poland, Spain, Sweden.
- The EHN web portal contains lists of international and national professional networks and patient associations for histiocytoses.
- The EHN web portal contains links to news pages, web forums, newsletters and other helpful internet contents about histiocytoses.
- Internet pages for kids have been elaborated explaining basics of the disease and the examinations. They are available online in Arabic, English, French, Polish.
- A literature tool is available online. Literature is directly linked with the pubmed database.

The development of the different languages available on the web and the statistics of visits were shown. Frequentation of the web portal is continuously growing. Most new visitors in 2013 accessed the Polish or the Spanish language versions. Most of the new visitors were located in Eastern and Southern Europe or in South America.

Following the presentation of the project achievements, the project partners decided that the project is worth to be continued and that they will make some efforts in order to raise money.

## 6. Histio Net Project (cont.)

The question how to proceed with the Histio Net newsletter was discussed. Initially it was planned to send an English newsletter on a regular basis and to translate it to French, German, Italian and Spanish. This has only been done a few times in the years 2011 and 2012. The project partners decided that due to the low numbers of subscribers in the four additional languages, future newsletters will only be produced in English. The frequency should be increased. Beth Anne Miller of the Histiocytosis Association offered advice concerning the optimum contents of a newsletter which was given to her association by a professional company.

The contact forms are used in all languages, mainly by patients or relatives. Due to this fact, the project partners decided to provide contact forms in additional languages. They will all be sent to the same email address and be transmitted to the translators/coordinators for the different languages. For languages where no translations of all contents seem to be necessary (e.g. Dutch and Swedish), because many information is already available and people speak very well English, the main page of the web portal should be translated so that contact form and links to national web pages can be added.

The literature tool was presented. Due to copyright issues, the EHN web portal cannot provide access to full texts. However, there are many abstracts available. It was therefore decided to extend the literature database considerably. Michael Girschikofsky offered to provide lists of newly published literature which he receives from a professional company for literature surveys.

The partners agreed that the French Reference Centre for Histiocytosis posts national contents on one dedicated Histio Net website which is created in the French language version only.

Plans for 2014:

- Translations of recommendations for adult LCH
- Translations of FAQ list about ECD
- Maps of experts for ECD
- Additional maps of experts for LCH (e.g. Lebanon, Morocco, Egypt)
- Maps of experts: Coordinators pathology and radiology
- Establish new partners where possible (e.g. India, Russia, Turkey)

## 7: Presentation of newly published web portal contents

Michael Girschikofsky, chair of the Austrian Working Group for Histiocytic Diseases in Adults and of the EHN working group for LCH guidelines for adults, presented the recommendations for adult LCH which have been published in 2013 in the Orphanet Journal of Rare Diseases. The recommendations are available on the EHN web portal.

A new list of frequently asked questions about Erdheim-Chester disease is now available online on the EHN web portal in the section for patients. It addresses general information about ECD, diagnosis, possible symptoms, therapy and research about the disease.

The list of frequently asked questions about HLH has been updated and is also available online.

## **8: Histiocyte Society Meeting 2013**

Michael Girschikofsky presented a review of the Histiocyte Society Meeting 2013 concerning adult LCH. Physicians from several countries have formed an Adult Histiocytosis Study Group of the Histiocyte Society. The following countries are represented: Argentina, Austria, Belgium, Canada, Czech Republic, France, Germany, Greece, Italy, Japan, Netherlands, Poland, Spain, Sweden, UK, USA. The study group wants to elaborate a common parameter list for patient registries and wants to perform retrospective analyses as well as prospective analyses/registry. There will be telephone conferences every 3 months.

Johann Visser provided reflections on the annual meeting of the Histiocyte Society in October 2013. He focused on the strategic meeting of the LCH steering committee, the discussions session about rare histiocytic disorders (planning of the international rare histiocytoses registry and the HS malignant histiocytoses survey), and the education sessions about LCH and HLH. Furthermore he summarised the take home messages of a guest speaker presentation about opportunities in rare disease research. In addition, he gave an overview of the clinical trials update concerning LCH-IV, HIT-HLH, reduced-intensity conditioning for HLH and select immune deficiencies, a pediatric trial of an oral BRAF inhibitor for LCH, and a pilot study of an anti-IFN monoclonal antibody for HLH. Last but not least, Johann summarised the symposium about malignant histiocytosis.

The next annual meeting of the Histiocyte Society will take place in Toronto, Canada, October 28-30, 2014.

The project partners decided to add some information about the research on BRAF mutations to the list of frequently asked questions about LCH. The information should be taken from the respective paragraph in the medical guidelines about LCH.

### **8a: Erdheim-Chester Disease**

Juvianee Estrada-Veras, clinical investigator at the US National Institute of Health (NIH) and medical advisor of the ECD Global alliance, presented the ECD Global Alliance, an international patient association for Erdheim-Chester Disease. The ECD Global Alliance has organised the First Annual International ECD Medical Symposium in October 2013 along with an International ECD Patient and Family Gathering.

In addition, he presented the protocol and the first outcomes of a cohort of ECD patients at the NIH. According to the protocol, ECD patients undergo a pre-defined schedule of one week of examinations. They are given medical advice and therapy recommendations based on the results. Treatment is carried out in patients' hospitals.

## **9: Maps of experts**

The project partners discussed how to generate maps of experts with reference radiologists and reference pathologists for histiocytoses. It was decided that every partner should provide the address of the – to their knowledge - most experienced radiologist(s) and pathologist(s) of their countries. The maps should be called "Coordinators radiology" and "Coordinators pathology". As in the other maps, it will be mentioned that experts can recommend themselves or be recommended at any time. The decision to include them will be made on the occasion of a Histio Net meeting.