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NEWSLETTER, July 2015, a short update on EWMnetwork news and activities.

This issue of our newsletter is written with an eye to those patient representatives who are specially interested in patient advocacy. We hope that, in reading this newsletter, you will get a clearer picture of how important it is for more patients to become interested and involved in patient advocacy and that many of you will be able to find in it some information that will be of particular use for your own organization.

Part 1: News

EMA's Advisory Committee recommends full approval of IMBRUVICA® (ibrutinib) to treat Waldenstrom's Macroglobulinemia (WM).

EWMnetwork warmly welcomes the EMA's fast progress towards accepting Imbruvica® (ibrutinib) for the treatment of Waldenström's macroglobulinemia in Europe. The positive opinion of the European Medicines Agency (EMA) Advisory Committee is to be reviewed by the EMA and a final decision is anticipated in the second half of 2015. If the final decision is positive, this would enable the use of the first EMA- approved therapy for WM patients in 28 European countries. However, the availability of ibrutinib in individual countries will be subject to local variations!
Indication for the treatment would be use with adult patients with WM who have received at least one prior therapy, or as a first-line treatment for patients unsuitable for chemo-immunotherapy.
More information can be found on the EWMn website www.ewmnetwork.eu .
Special mention must be made of the role of the USA's Dana-Farber Cancer Institute's Bing Center for WM, which helped Imbruvica gain approval for treatment of the disease in the United States and of the IWMF (International Waldenström's Macroglobulinemia Foundation) for their support and fundraising.

IWWM-9 planned for October 6-9 2016 in Amsterdam

The 9th International Workshop for Waldenström's Macroglobulinemia (IWWM-9) and Symposium on Advances in Multiple Myeloma is planned to be held October 6-9, 2016 at the Renaissance Amsterdam Hotel, in beautiful historic Amsterdam, The Netherlands.
Since the biennial IWWM began in 2000, the number of participating investigators has steadily grown, as have also the number of WM-related publications. We expect IWWM-9 will continue to reflect the ongoing research regarding biomarkers and their role as major contributors to WM.
More information about a WM patient meeting connected to the IWWM in Amsterdam will follow.

European Reference Networks (ERNs)

Action by Patient Organizations needed: *please raise the issue of ERNs with your leading clinicians and centers of excellence to alert them about this upcoming opportunity and to urge them to touch base with their member state representative about it...*
ERNs provide a unique opportunity to strengthen research and collaboration in rare diseases including rare cancers or hematological malignancies, as they will provide a platform for collaboration, spreading clinical excellence, networking and research, financially supported both by the EC and Member States.
The final version of the *Addendum* to the 2013 EUCERD Recommendations on ERNs for Rare Diseases (RDs), adopted on 10 June 2015 by the Commission Expert Group on RDs can be found on the EC's website: http://ec.europa.eu/health/rare_diseases/european_reference_networks/index_en.htm

Click under “Latest updates” on “RD ERN’s Addendum to Eucerd recommendations of Jan. 2013”. This Addendum is very important as it recommends:
1) Necessity of patient involvement in Rare Disease ERNs and
2) Grouping rare diseases in “thematic networks”
An illustrative grouping of RDs (see pg 8 of the Addendum) is the result of extensive discussions with the members of the Expert Group as well as with other stakeholders (including patient organizations) over the last two years.
Key date: December 2015 – First Call for Expression of Interest for ERNs (all areas, not only RDs) to be launched by the EC.

Call on EU National Competent Authorities for Pricing & Reimbursement to support:

1. The establishment of a table for price negotiation with a group of Member States
2. The scaling-up of the pilots on early dialog between payers and developers of medicinal products
In a context of fast scientific advances, improved dialog and emerging platforms for discussion between healthcare professionals, patients, decision-makers, industry and, progressively, EMA and HTA (Health Technology Assessment) bodies, as well as payers, EURORDIS (European Organisation for Rare Diseases) and EPF (European Patients’ Forum) call on National Competent Authorities to support the two proposals described above. *Please don’t hesitate to disseminate this to your contacts as you deem appropriate*.
More information on http://www.eurordis.org/sites/default/files/call-on-payers.pdf

Part 2: Trainings, Congresses attended by EWMnetwork board members

IWMF Ed Forum 2015, Dallas April 30-May 2

For a full report of this event in the July issue of *The Torch* click on
http://www.iwmf.com/sites/default/files/docs/Torch_16.3_July2015.pdf

EWMnetwork board member Veikko Hoikkala attended the Forum and the preceding Workshop for Support Group Leaders on April 30. He reports:
“The Workshop had circa 30 participants, but besides myself no other European was present. So, about European issues regarding support groups, co-operation etc. there is nothing new to report.
Looking back: IWMF is now 20 years old. The first Forum was held in 1996 with 75 participants; this year there were about 170 participants. Today IWMF has about 7000 members in 70 countries and 63 Support Groups worldwide. The IWMF Talk-list, operated by email, has about 1900 members. At the time being IWMF has 4 full-time and 2 part-time employees. All others are volunteers.
As usual the Workshop and Forum were excellently organized and the medical knowledge was at high level. Since the excellent London Forum, August 2014, I cannot say there were any really new or dramatic issues regarding treatments.
Clearly the discovery of gene mutation MYD 88 L 265P is opening new doors in research. Also a little more novel mutation finding, CXCR4, was mentioned . According to Dr Treon [of The Bing Center for WM] it is also an important milestone and a way to identify which patients are more likely to benefit from ibrutinib. This CXCR4 is found in ca 30 % of WM patients, MYD88 in more than 90 %.
Accordingly, regarding treatments, ibrutinib is now in the center of research.
As for other treatments, a couple of matters are perhaps worth mentioning:
* Among alkylating agents it seems bendamustine is becoming more and more popular, even if the long-term experiences/ side effects are not yet very clear.
*With cladribine the probability of second cancer is almost 10 %
*The earlier widely used CHOP combination seems to be no more recommended.
When I was diagnosed in 2002, my doctor said not to worry, because WM is not inherited. That was not correct. Research indicates now that the probability to have WM inherited is even at level 5%. It is really a high number. Anyway, I enjoyed the Forum and feeling very useful to be there. IWMF was generous and paid my hotel costs, and Finland my flights, so I did not burden very much the budget of EWMn. Last but not least, very friendly people and a lot of networking!”

Eurordis Summer School 2015

In the first week of June, EWMnetwork board member Melanie Meniar attended the annual EURORDIS Summer School in Barcelona. She reports:
”The Summer School began in 2008 as part of a commitment to empowering people living with rare diseases. It is a five-day training program that covers aspects of medicinal development, ethics in medical research and development, and the understanding of regulatory bodies and market access processes where patient representatives take on key roles. To date, more than 270 participants from over 37 countries, and representing more than 75 diseases, have received training.
The Summer School was a wonderful opportunity to learn about the processes involved in the management of rare diseases, which in the case of WM is of extra interest because of the recent developments surrounding Imbruvica® (ibrutinib).
In addition to providing a large amount of information during its five days, the Summer School was a great opportunity to share experience with others involved in the field of patient representation. This year, researchers also took part in the Summer School, which was a significant development because the processes and opportunities on a European level play such an important role in the development of medicines.
To have taken part in the Summer School strengthens the role which the European Waldenström’s Macroglobulinemia Network can play on a European level with regard to access to treatment and medication, information on clinical trials and research into further new methods of treatment.”

Lymphoma Coalition Europe (LCE) Members meeting, Vienna, June 10, 2015

EWMnetwork board member Marlies Oom attended this meeting. LCE kindly provided a grant for attending this meeting. She reports:
“Representatives from 15 European lymphoma patient organizations highlighted in short PowerPoint presentations their national organization and discussed best practices. Industry presentations, updates in therapy development were given and the brand new LCE Regional Manager presented the goals and objectives of LCE.
In the informal part of the LCE meeting and the next days at the booth for patient advocacy organizations at the EHA congress there were many opportunities for informal contacts between the lymphoma patient advocates. These contacts are important for EWMnetwork because information about WM is in some countries difficult to find. Via personal contacts EWMnetwork can help WM patient groups or lymphoma patient organizations in Europe to put information about WM on their webpages (www.waldenstrom.info) or websites”.

European Hematological Association (EHA) Congress 2015 , Vienna June 10-14

Marlies Oom also attended the EHA congress and reports: “A group of representatives from different patient organizations had prepared two patient advocacy sessions thanks to the inspiring guidance and co-ordination of Jan Geissler (European Patients’ Academy EUPATI/ CML Advocates Network).

Summary Session 1: Collaborating with patients for successful hematology research and for assessment of optimal benefits and risks
Well-informed patients and patient advocates have a key role to play in the implementation of patient-centered clinical research, access to novel treatments and in treatment optimization approaches. In an era of growing demand and emphasis on both quality and sustainability of healthcare, it is critical to involve patients in the R&D (research and development) process, as it can accelerate research and make it more effective.
In many disease areas, patients are already actively engaging in the many processes involved in the development of new treatments: from contributing to protocol design, informed consent and ethical review, to the overall medicines development process, marketing authorization and healthcare

policy. In addition, new technologies like mobile apps can help to collect better real-world data on side effects and patient-reported outcomes.

This session looked into innovative ways of involving patients in hematology clinical trial design (e.g. in trial design, but also with mobile apps for trial participants), how to educate patients in being well-informed partners in trial design, and how to strike the difficult balance between benefits and risks in trials.

Summary Session 2, topic: Partnership and evidence: key elements to improving access to treatment
There have been significant improvements in hematological treatment in the last decade. However, there are still important differences regarding how patients benefit across regions. The development of new, more expensive drugs adds pressure to these already existing differences. These access issues are a potential source of inequity, leading to suboptimal patient outcomes depending on where patients live.
Limitations to treating patients according to the best standards and treatments, lack of access to drugs and diagnostics, lack of sufficient innovative research and clinical trials and unacceptable time gaps between clinical discovery and clinical practice are some of the frustrations both patient advocates and hematologists share. Successful advocacy for access requires a collaborative approach, besides a detailed understanding of the country-specific barriers that are preventing access to the best treatment and care. However, until now, initiatives to improve patients' access to treatment have only had limited success. What is more, clinicians and patient advocates have often advocated in isolation from each other when they should have been working together.
This is why new approaches are so urgently needed if we are to resolve the issues of the last decade. Not to do so would almost certainly mean patients will not benefit from future treatments, diagnostics etc. Not taking action on this is not an option for patients and hematologists.

This session explored today's challenges, issues and the opportunities of the future. A case study "European Atlas of Access to Myeloma Treatment" was presented, as well as the perspective of a hematologist on access and two case studies from patient advocacy groups who have built new collaborative approaches between patients, clinical experts and policy makers aimed at improving access to medicines. See for the Atlas http://www.mpeurope.org/news/european-atlas-on-access-to-myeloma-treatment-survey/.

In Session 3 follow-up of the two previous sessions was discussed, summarized as follows:
- two workgroups will work on guiding patient advocates and patient organizations on how to advocate for access/ become involved in research (where to start, whom to ask for help etc.) - the idea of a doctor-patient communication training will be brought to the attention of the EHA Educational Committee
- the "official" Patient Advocacy Workgroup of EHA will hold a meeting early in 2016 to discuss the strategy and actions for collaboration between EHA and the patient community
- EHA will share their draft "Research Roadmap" for input and feedback".

Part 3: Board news

EWMnetwork's Secretary is looking for a successor.
We are looking for you if you are
- a WM patient yourself or a carer of a WM patient
- interested in patient advocacy for rare diseases like WM
- a fluent speaker and writer of English
- can spend 8-10 hours a week on this voluntary work (with peaks and quiet weeks)

If you are interested or know someone who might be, please email secretary@ewmnetwork.eu