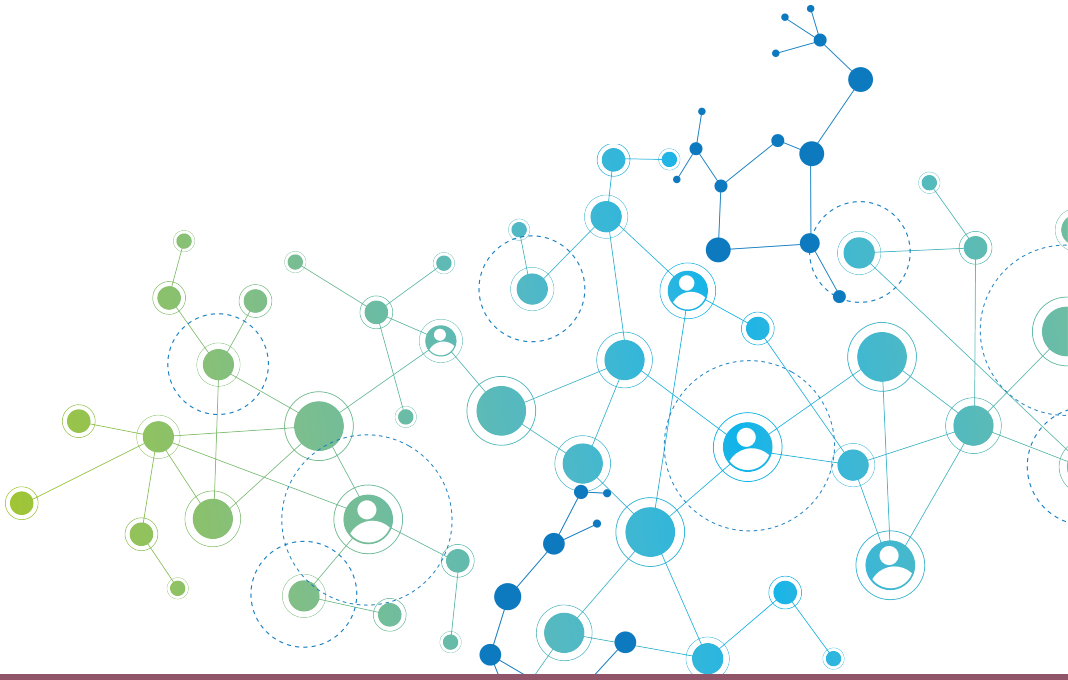
Myeloma Patients Europe

2018 Activity Report



www.mpeurope.org

Index – Activity Report 2018

Message from the President and Chief Executive Officer

2018 has been a pivotal year for Myeloma Patients Europe (MPE). Not only because our organisation has consolidated to become one of the strongest patient groups in Europe, but also because it laid the ground for key initiatives, such as the Myeloma Community Advisory Board (CAB).

MPE's membership and staff have grown consistently, as well as the outreach and influence we have, not only in the myeloma and AL amyloidosis fields but also in the haematology, cancer and rare disease communities. We have also seen an important increase in our activities and partnerships with key stakeholders at the European level.

Internally, one of the major achievements during 2018 was the amendment of our Constitution and Internal Rules at the Annual General Meeting (AGM) 2018. The new versions of these documents enables MPE to grow as a professional organisation while focusing on the needs and wants of patients, through its members. One of the highlights of the new Constitution was to open up to working in myeloma-related disease areas, such as AL amyloidosis. Shortly after, MPE welcomed Amyloidosis Israel to its membership as the first member not focusing on myeloma. The AL Amyloidosis Taskforce was then created to improve the services we provide to AL amyloidosis patients and to increase engagement of members in this disease, particularly where patient organisations are not available in their countries.

The AGM 2018 also elected Riikka-Leena Manninen (Finland), Lisa Kotschi (Germany) and Roman Slomkowski (Poland) to the Board, and we were pleased to see Hans Scheurer (Netherlands), Johannes Brenner (United Kingdom), and Kristina Modic (Slovenia), unanimously re-elected to the Board as President, Treasurer and Secretary respectively.

Last year we also finalised the Strategic Planning exercise, emerging with a clear vision for the goals and objectives of the coming five years. The Strategic Plan 2019-2024 will be discussed and voted at the AGM on 17 April 2019.

The growth of MPE is directly linked to strength of our members. For this reason, MPE continues working hard to build capacity and capabilities through professional training, targeted coaching and support, and by providing scholarships to fund key activities of our members. Two other highlights amongst our educational programmes are the Advocate Development Programme (ADP) and the Atlas Coaching Programme. Both are excellent examples of how MPE delivered tailored training and capacity building to our members, assisting them in developing advocacy knowledge and skills, identifying national access challenges and barriers, and producing individualised advocacy strategies to be implemented nationally.

In 2018 more than half of the total budget of MPE was invested in these programmes, as well as services to stimulate and enforce patient advocacy for myeloma patients through our members.

Access barriers to myeloma treatment and care across Europe were again a key priority for MPE last year. As one example, we strongly advocated for an immediate solution when our members reported issues around melphalan shortages. We are pleased to say that through the work MPE did with regulators, doctors, nurses, pharmacists and regulatory bodies – the melphalan shortage was resolved and we are now monitoring the situation to ensure the supply continues. The MPE community will closely monitor potential shortages, access barriers to essential treatments and innovations across Europe and will continue to make our voice heard at all stakeholder levels, to ensure myeloma patients have better and accessible treatments in every European country.

Throughout 2018, we continued to play a fundamental role in two European Commission projects funded through Horizon 2020, the biggest EU Research and Innovation programme. These projects are MMPredict and CARAMBA. The first one is focused on clinically validating a personalised medicine tool that predicts the most effective treatment options for myeloma patients, while the second focuses on testing the revolutionary chimeric antigen receptor therapy or "CAR-T" cell technology to tackle myeloma. These are two important projects, where MPE's

role, with the help of its members, is to ensure that myeloma patient needs and wants are taken into account and integrated from the very beginning.

Furthermore, MPE has had initial contact with the European Myeloma Network (EMN) to align and join forces in areas of common interest. Both the EMN and MPE collaborate already on the IMI project Harmony dealing with big data in myeloma.

One of the most popular projects MPE is leading on within the Workgroup of European Cancer Patient Advocacy Networks (WECAN) is the "Reasonable agreements between patient advocacy and the pharmaceutical industry" (RAPP) project. This project has been run in alignment with twelve pharmaceutical companies and Patient Focused Medicine Development (PFMD). By October 2018, the group had developed a set of guiding principles on how legal agreements between industry and patient advocates should look. These guiding principles are already changing the way these contracts are negotiated and we believe that the second step in this initiative – developing four contract templates on consultancy, collaboration, advisory board and community speaker agreement – will be a game changer in the relationship between patients and pharmaceutical companies.

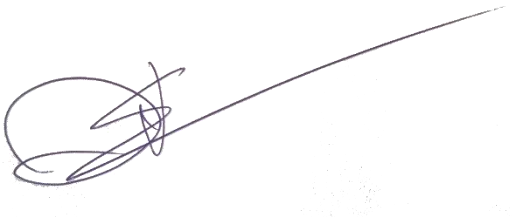
Thanks to a very committed Board, hard-working staff, and a wide network of members, volunteers and patients, restlessly working for the benefit of the myeloma community, MPE has grown to a highly effective and efficient network with the ultimate goal of improving the lives of myeloma and AL amyloidosis patients across Europe. It was an incredibly busy, fruitful and exciting year, which wouldn't have been possible without their support and commitment.

Also, this would all not have been possible without the continued support of our sponsors to drive our vision further for the benefit of European myeloma patients.

Let's continue this journey together. There is still so much to be done!

Hans Scheurer

President of Myeloma Patients Europe



Ananda Plate

CEO of Myeloma Patients Europe

1 About Myeloma Patients Europe (MPE)

MPE is an umbrella organisation of myeloma patient groups and associations from across Europe. It is registered as an international non-profit organisation under Belgian Law (AISBL) and its headquarters is located in Brussels.

MPE is dedicated to improving the treatment, care and quality of life of patients with myeloma and AL Amyloidosis. To this end, the main goals of the organisation are:

- Collaborating on projects to the benefit of the myeloma community
- Exchanging information and best practice
- Developing existing patient groups and encouraging and facilitating the setting up of new groups
- Helping to shape appropriate health-related policies and initiatives on a European and national level
- Ensuring patients across Europe receive timely access to new treatment
- Stimulating and promoting patient-centred research and clinical trials
- Developing a strong evidence base for the needs and wants of patients and their role in research
- Providing information, educational and outreach programmes to member groups

MPE acts as an umbrella organisation for 43 national myeloma patient groups from across 30 European countries.

There are three types of members:

- Full members: non-profit patient organisations that are registered in a European country.
- Associate members: Any individual person who shares Myeloma Patients Europe's goals and all other non-profit organisations that do not fulfil the criteria for full membership.
- Supporting members: Supporting members are individuals, foundations or corporations who have chosen to support the activities of the organisation through financial contributions or in-kind services. The type and amount of donation will be determined by the Board.

MPE is run by a Board comprising six to nine members, appointed for a two-year period, of which at least half are patients or carers. Board members may be consecutively re-elected twice.

The Board may not include more than two members of the same nationality or who represent the same country. It is also agreed that any public or private organisation may not be represented by more than one member of the Board.

MPE's Board members are all volunteers who are supported by the MPE staff as well as by an expert Medical Advisory Committee.

1.1 Board members

Until April 2018 the Board was composed of the following members:

- President: Hans Scheurer (Netherlands)
- Vice-President: Ibolya Kéri (Hungary)
- Secretary: Kristina Modic (Slovenia)
- Treasurer: Johannes Brenner (United Kingdom)
- Board members: Viorica Cursaru (Romania), Ron Dloomy (Israel) and Biljana Dodeva (Macedonia).
- The term of Ibolya Kéri (Vice-President) and Viorica Cursaru finished at the AGM 2018 and they didn't stand for re-election. After the AGM 2018 elections, the board had the following composition:
    - President: Hans Scheurer (Netherlands)
    - Vice-President: Ron Dloomy (Israel)
    - Secretary: Kristina Modic (Slovenia)
    - Treasurer: Johannes Brenner (United Kingdom)
    - Board members: Lisa Kotschi (Germany), Biljana Dodeva (Macedonia), Riikka-Leena Manninen (Finland), Roman Slomkowski (Poland).

1.2 Executive office

With the aim of having a wide structure to build capacity among member organisations and ensure treatment access and early diagnosis across Europe, MPE is increasingly growing.

Chief Executive Officer

Ananda Plate

Ananda is Chief Executive Officer. She joined the team in March 2013. She holds a law degree from the University of Barcelona and a master's degree in Health Policy, Planning and Financing from the London School of Economics and Political Science and the London School of Hygiene and Tropical Medicine. Her main field of interest is cross border healthcare within the EU and health data protection.

She started getting involved in patient advocacy in 2007. Since then she has collaborated with a number of patient organisations across Europe. She has worked at the leading Institute of Public Law (IDP Barcelona) and the European Commission (DG Health and Consumers). Currently she is also Chair of the Workgroup of European Cancer Patient Advocacy Networks (WECAN) and a member of the Ethics Committee at the Chamber of Physicians of Bavaria, Germany.

She speaks Spanish, German, English, Catalan and French.

Managers

Alfonso Aguarón

Alfonso joined the MPE team as Project Manager in August 2014 and left the organisation in July 2018.

He has received training in IT engineering and until 2009 most of his professional career has been developed in a high-tech R&D multinational automotive company. After being diagnosed with Hodgkin's Lymphoma in 2008 he decided to get involved in patient advocacy, first as a volunteer and later on as a professional, with the aim of deploying the use of new technologies in this area. He was involved in Spanish patient groups of haematology and oncology, first as IT Manager and since 2013 as International Affairs Manager. He has been involved in many patient networks in a national and an international level. He speaks Spanish and English.

Ana Vallejo

Ana joined the MPE team as a Communications Manager in September 2016. She holds a bachelor's degree in journalism from the Complutense University of Madrid and a Master's degree in Audiovisual Communication from the University Antonio de Nebrija in Madrid.

Ana worked as a journalist on weekly newspapers specialising in health and on consumer health monthly magazines. She has been involved in different patient advocacy groups, first as a volunteer and later on within the communication department. Since 2014, Ana worked as PR and press consultant in the health department of an international PR agency developing communication strategies for the main pharmaceutical companies.

Ana has been involved in producing two books focused on patients with cancer as a co-writer and editorial co-ordinator. She speaks Spanish and English.

Kate Morgan

Kate is Policy and Public Affairs Manager at MPE. She joined the team in September 2017. She holds a bachelor's degree in Government from the London School of Economics and Political Sciences and a Master's degree in International and European Politics from the University of Edinburgh. She also holds a Diploma in Public Relations from the Chartered Institute for Public Relations. Her main area of interest is health policy, with a specific focus on policies affecting drug development and access in European countries.

Kate previously worked for eight years in the Policy Team at Myeloma UK, where she was involved in influencing a range of policy and reimbursement decisions affecting myeloma patient access to good quality care and effective new drugs. She was involved in a number of patient and

public advisory panels in the UK healthcare system, including for the Scottish Medicines Consortium, the drug approval body in Scotland.

She been involved in a wide range of patient organisation networks, both nationally and internationally – most recently chairing the Blood Cancers Alliance, an information-sharing network of haematological cancer charities in the UK.

Business Support

Ingrid Jenisch

Ingrid joined the MPE team as Finance and Reporting Officer in August 2018. She holds a degree from the advanced technical college in Ulm, Germany, and has an apprenticeship as an electrical engineering assistant.

Ingrid has broad experience in large, medium-sized and start-up companies. She worked as referent and assistant to Executive Boards. In this role she was responsible, for board-, management- and finance-reporting and controlling. She has proficient expertise in project and programme management and implementation of administrative processes as well as in marketing and public relations.

In the health area she worked for the Innovative Medicines Initiative (IMI) project EUPATI. The European Patients' Academy. This is a pan-European project implemented as a public-private partnership by a collaborative multi-stakeholder consortium from the pharmaceutical industry, academia, not-for-profit, and patient organisations. As Finance and Reporting Officer she supported the EUPATI Director in preparing reports for IMI and the international consortium members. Financial monitoring, control and analysis were part of her responsibility. Ingrid is based in Munich, Germany.

Anna Rovira

Anna joined the MPE team as Office Assistant in May 2016 and left the organisation in April 2018.

She holds a law degree from the University of Barcelona and is currently studying a Master's Degree in Environmental Law at the University of Tarragona (CEDAT). In 2012 she participated in the Erasmus programme at Leiden University.

Working mainly as a lawyer, she has experience in social advocacy and volunteering.

1.3 MPE members

MPE is composed of 42 myeloma patient groups and associations in 30 European countries:

- Amen Israeli Myeloma Patient Foundation (AMEN) - Israel
- Association Française des Malades du Myélome Multiple (AF3M) - France

- Association of Myeloma Patients of Serbia (AMPS) - Serbia
- Association of Patients with Haematological Malignancies - Slovakia
- Associação Portuguesa Contra a Leucemia (APCL) - Portugal
- Associação Portuguesa Leucemias e Linfomas (APLL) - Portugal
- Blodkreft Foreningen - Norway
- Contactgroep Myeloom en Waldenström Patiënten Vlaanderen vzw (CMP vzw) - Belgium
- MyMu Wallonie-Bruxelles (MyMu) - Belgium
- Dansk Myelomatose Forening - Denmark
- AMM-Online - Germany
- Deutsche Leukämie- & Lymphom-Hilfe e.V. (DLH) - Germany
- Leukaemiehilfe RHEIN-MAIN e.V. (LHRM) - Germany
- Myelom Deutschland e.V. - Germany
- Drustvo bolnikov z limfomom (DBL) - Slovenia
- Slovensko Združenje Bolnikov Z Limfomom In Levkemijo L&L - Slovenia
- Eesti Müeloomiliit - Estonia
- KANKO/BIRKAN - Turkey
- Foundation for the Advancement of Bone Marrow Transplantation Switzerland (SFK) - Switzerland
- Myelom Kontaktgruppe Schweiz (MKgS) - Switzerland
- Carita Fundation - Poland
- Latvian Cancer Patient Support Society “Dzivibas koks” - Latvia
- MOHA– Foundation for Hungarian Oncohaematological Patients - Hungary
- Multiples Myelom Selbsthilfe Oesterreich (MMSOe) - Austria
- Myeloma Euronet Romania (MER) - Romania
- Myeloma UK (MUK) - United Kingdom
- Slovak Myeloma Society (SMyS-KP) - Slovakia
- Society for Assistance to Patients with Oncohaematological Diseases - Russia
- Stichting Hematon - Netherlands
- Suomen syöpäpotilaat-myelooma potilasverkosto - Finland
- Mijelom CRO - Croatia
- Asociacija “Kraujas“ - Lithuania
- BORKA - Macedonia
- Polish Myeloma Patient Help Association - Poland
- Comunidad Española de Pacientes con Mieloma Múltiple (CEPMM) - Spain
- SOS Mielom - Romania
- Blodcancerförbundet Sweden - Sweden
- Armenian Hematology Association - Armenia
- Amyloidosis Israel - Israel
- KPMM (Associate) - Czech Republic
- Individual (Associate) - Bosnia and Herzegovina
- Centro de Histocompatibilidade do Norte (CHN), (Associate) – Portugal

2 Strategic objectives

MPE has a number of broad aims including:

- Collaborating on projects to the benefit of the myeloma community
- Exchanging information and best practice
- Developing existing patient groups and encouraging and facilitating the setting up of new groups
- Helping to shape appropriate health-related policies and initiatives on a European and national level
- Ensuring patients across Europe receive timely access to new treatment
- Stimulating and promoting patient-centred research and clinical trials
- Developing a strong evidence base for the needs and wants of patients and their role in research
- Providing information, educational and outreach programmes to member groups

MPE's strategic objectives 2013-2018 have been to:

1. **Build a professional and sustainable organisation.** Patients, caregivers and sponsors deserve the best support through an effective and efficient organisation dependent on funding.
2. **Facilitate development, capacity and sustainability of members.** Provision of services to patients needs to be robust and sustainable. It must be capable of delivering quality and excellence in every respect. Also, given the number of patients involved, organisations need to have appropriate structures in place to deal with the work and to plan for succession.
3. **Encourage research and the development of new effective treatment.** Myeloma is a relapsing and remitting cancer with an evolving clone. It is also a very individual cancer. Patients therefore need constant access to more effective treatment with a tolerable side-effects profile.
4. **Ensure the timely adoption of research outcomes or results and access to state of the art diagnostic tools and treatment.** Research and clinical trials are time consuming processes. Also, approval and reimbursement systems can mean it can take many years before patients benefit from research results. Patients don't have time for this and need to benefit sooner. Critical barriers and hurdles need to be removed and advance plans put in place for an early and timely response to research findings.
5. **Encourage healthcare professionals to provide an excellent standard of care to patients and their families.** Myeloma is not just about cancer, it is about the whole person and everybody connected to them who is also impacted. Patients and therefore families deserve access to the best 'holistic care' possible, built not only around their needs as cancer patients but also their personal preferences and circumstances.
6. **Develop an evidence base for the needs and wants of patients and ensure access to high-quality information and psycho-social support.** In order to advocate effectively for patient rights within government, industry and regulators we need solid

evidence as to what the needs, wants and preferences of patients are. We need to move beyond the anecdotal, emotional and angry to an era of empirical evidence.

To meet its aims and in addition to its membership, MPE works directly with healthcare professionals, reimbursement authorities, regulators, politicians, pharmaceutical companies, the media and anyone involved in the 'myeloma community'.

3 Activity report

3.1 Myeloma Access Atlas

Access to treatment and care across Europe is extremely variable. Patient groups and associations can make a difference in improving access, but they often do not have the evidence, knowledge and tools to advocate effectively. With the aim to provide MPE members with the resources and information they may need, MPE developed the Myeloma Access Atlas, alongside the Avedis Donabedian Research Institute, Barcelona, to reflect the situation of myeloma treatment and care access in more than 30 European countries: http://www.mpeurope.org/atlas.

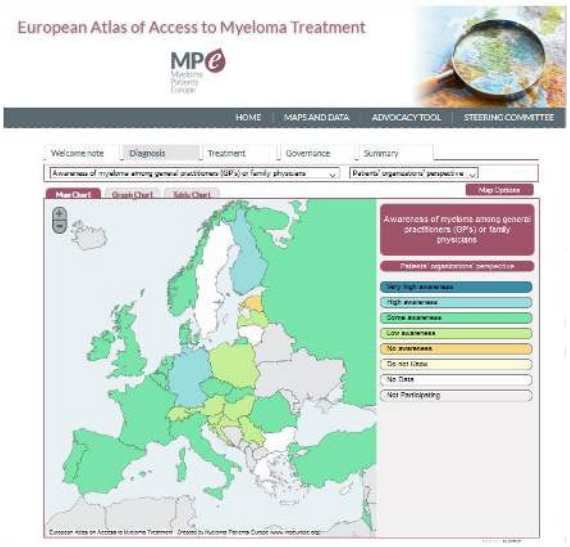
To address this, an Atlas microsite was published in 2016 based on the results of a survey conducted in 2015 to help MPE members with information and data on the healthcare situation in their country. The microsite includes country-specific and comparative information on health services from a range of academic, online and organisational references. To update this data, during 2018, a new survey was conducted by MPE asking myeloma patients and clinical experts about the myeloma treatment and care challenges they face in their country.

Between January and December 2018, MPE undertook/initiated the following activities within this programme:

- Dissemination of the MPE Access Atlas Survey to patient organisations and clinicians in all European countries
- Analysis of the results of the above-mentioned survey, to be published in 2019
- Completion of a desk review on health systems and access to treatment and care in each country
- Country-specific reimbursement profiles have been created for 8 European countries

- A review into the online functionality and layout of the Atlas is ongoing. A tender process started at the end of 2018 to identify a relevant company to design the Atlas website and make it more user-friendly. A first version to be ready for AGM 2019
- A detailed Q&A on the HTA process has been published, which can be viewed here: https://www.mpeurope.org/what-we-do/publications/qas/health-technology-assessment-hta/
- Gathering information on drug availability from all pharmaceutical companies which will be used to populate an online tool which outlines access to myeloma drugs across Europe

Atlas Coaching Programme pilot



The Atlas Coaching Programme is an opportunity for MPE members to identify priorities and build successful advocacy strategies to improve access to myeloma treatments in every country in Europe. This programme is an adapted training programme and online evidence-based advocacy tool aimed at helping member organisations to prioritise between identified access barriers and helping them to develop a comprehensive strategy to address these.

The programme provides the necessary skills and guidance for MPE members to develop tailored evidence-based advocacy strategies – based on issues they have identified as being important. A key element is the adaptability of the programme to the specific needs, context, and resources of each organisation. The programme was piloted and evaluated between October 2016 and December 2017. The approach to and content of the coaching programme was updated based on this important feedback. Additionally, one-to-one support regarding access at a national level was provided to members within this programme.

3.2 MPE Advocate Development Programme

The pioneering Advocate Development Programme (ADP) was launched in 2017, a training programme directed to myeloma advocates with the aim of providing them with the skills and knowledge that any myeloma patient advocate should have regarding key areas, such as the drug development process, when interacting with the other relevant stakeholders.

The ultimate objective is to create a pool of advocates in the myeloma community that have a deep understanding of the disease and the clinical, advocacy and policy processes involved.

The programme combines a series of theoretical sessions delivered by experts in a relevant field with practical sessions that take place during the most important scientific and regulatory

meetings in Europe that concern myeloma: the European Haematology Association (EHA) Annual Meeting and the European Society of Medical Oncology (ESMO) European Conference of the International Society on Pharmaeconomics and Outcomes Research (ISPOR).

In 2018 the programme was opened to AL amyloidosis advocates too as a result of the inclusion of this condition linked to myeloma in the MPE strategic goals and Constitution.

Significant changes were added to the ADP programme in 2018 to improve the results obtained in first cohort in 2017. This programme was coordinated by two external and experienced advocates.

The ADP 2018 kicked off in March 2018 and ran until February 2019. Six trainees were included in this year's programme: Cristina Fernández (Spain), Daniel Drimer (Amyloidosis Israel). Yervand Hakobyan (Armenia), Mirjana Babamova (HEMA - Macedonia), Lise-lott Eriksson (Sweden) and Bozhidar Kochoski (BORKA - Macedonia). The ADP included 7 webinars and 3 face to face meetings which were held at EHA, ESMO and ISPOR and an additional virtual meeting which was held in February 2019:

- The first face to face meeting, held during the European Hematology Association (EHA) Annual Congress (June 14-17, at the Stockholmsmässan - Mässvägen 1, 125 80 Älvsjö, Sweden) featured some of the some of the most respected haematologists in the myeloma community, such as Dr Sonja Zweegman, Dr Laurent Garderet and Dr Anna Sureda, along with internal speakers and ADP co-ordinators. This face to face meeting was held over two days instead of one and was followed by four days of congress with two debrief sessions and specific explanations in the poster area.
- The second face to face meeting was held during the European Society for Medical Oncology (ESMO) Annual Congress (October 19-23, at Messe Munich, Messegelände 81823 München, Germany). The two-day meeting and was addressed by important external experts such as Nicola Latino and Nathan Cherny, from ESMO. The face to face meeting was followed by six days of congress.
- The third face to face meeting was held during the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) Annual Congress (November 10-14, at Centre de Convencions Internacional de Barcelona in Barcelona, Spain). For the first time the ADP included ISPOR in the agenda taking the trainees to Barcelona to attend the third face to face meeting with top external experts such as Heidi Livingstone, from the National Institute for Health and Care Excellence. The ADP meeting was followed by two days of congress at ISPOR.

3.3 MPE Scholarship and Capacity Building Programme

The rationale behind all MPE programmes is that, by equipping members with the right tools and knowledge, they become empowered and capable of delivering essential services to patients. This will over time create strong, professional, independent and sustainable organisations that are able to advocate for the needs and wants of myeloma patients across Europe. To achieve this, MPE has developed the Scholarship and Capacity Building Programme. Members are invited

to submit applications to the programme and they will have the option to ask for one-to-one tutorials to help them to develop the activities included in their application.

In 2018, we issued two funding calls members could apply for:

- A patient and caregiver day call, supporting members to hold a patient and/or caregiver information day
- An open call for any initiative MPE members required support for

Following an application and selection process, participants are offered a grant of 3.000 € to implement their activities, which they are asked to report to MPE against.

The call for expression of interest was opened at the end of May 2018. In 2018, 13 applications from 10 organisations were received and approved, five applications for patient days and eight applications for other programmes/activities. Find below the list of organisations and projects granted 3.000 € through this programme:

- Amyloidosis Israel (Israel) - Patient day: Conference plan for amyloidosis patients and caregivers
- Blodkreft Foreningen (Norway) - Workshop on user involvement in multiple myeloma research
- Združenie pacientov s hematologickými malignitami (Slovakia) - Early diagnosis of haematological malignancies campaign and education of patients with haematological malignancies
- Blodcancerförbundet Sweden (Sweden) - Patient day: Combination therapy workshop
- Slovensko Združenje Bolnikov Z Limfomom In Levkemijo L&L (Slovenia) - Pilot programme of comprehensive rehabilitation for patients with blood cancers
- Association for Multiple Myeloma (Israel) Patient day - patients' and caregivers' day and a volunteer management programme.
- Myeloma UK (UK) Research Capacity Building Workshops
- Kraujas (Lithuania) – Patients' and caregivers' day and a project about sexuality
- BORKA (Macedonia) "Let's make changes" Myeloma campaign
- MOHA (Hungary) - Myeloma Information Day and a myeloma patient information brochure

All the projects have been a massive success, with major thanks to the hard work and efforts of our members across Europe and further afield. A report to summarise and showcase the projects MPE funded in 2018 can be found here: https://www.mpeurope.org/wp-content/uploads/2019/02/SCOLARSHIP-REPORT-MPE-2019-WEB-VERSION.pdf

3.4 MPE Masterclass and Annual General Meeting (AGM)

Once a year MPE holds its Annual General Meeting (AGM). All MPE members, sponsors and special guests (prospective members, potential sponsors, etc) are invited to this event which includes a one-and-a-half-day Masterclass to provide training to MPE members with scientific sessions, given by the most important specialists in Europe, and advocacy sessions. The AGM also includes the MPE General Assembly only with members and a sponsor meeting with MPE's current and prospective sponsors in order to explain to them present and future MPE projects and identify potential areas of collaboration.

The MPE AGM 2018 took place in Brussels, Belgium from 20 - 22 April 2018. The agenda was developed with the help of the AGM committee. The meeting was attended by a total of 74 people:

- 43 delegates
- 7 guests
- 13 industry representatives
- 6 Board Members
- 5 Staff Members

There was a total of 14 sessions:

- 4 plenary
- 6 breakout
- 4 workshops

3.5 European Commission projects: Horizon 2020 and IMI

MPE has joined three European Commission project consortia. This means, for the first time, MPE has received diversified public funding and is able to take a lead in innovative pan-European projects to further scientific and health economic understanding of therapeutic interventions in myeloma.

Two of the European Commission projects fall under the umbrella of the Horizon2020 programme. Horizon2020 is a large-scale EU Research and Innovation programme which has around €80 billion of funding available over 7 years (2014-2020). It funds a wide range of projects relating to science, innovation and tackling societal challenges. To receive funding through the programme, multi-stakeholder consortia work in partnership to develop a project proposal, which is reviewed by the European Commission. MPE is very pleased that two project proposals it was involved in, MMPredict and CARAMBA, have been accepted by the European Commission and will be implemented over the course of the next four-five years.

The third large-scale European project is the Innovative Medicines Initiative (IMI) HARMONY project which runs from 2017 until 2021.

3.5.1 Horizon 2020: MMPredict

Horizon2020 MMpredict, a 42-month project, was approved by the European Commission and started in November 2016 and will run until 2020. The European Commission has supported the whole project with €3,755,802 worth of funding distributed proportionately across project consortium members. MMpredict is working towards the development of a tool which can help myeloma doctors predict the most effective treatment for their patients. The aim is to ensure that each patient receives the most suitable treatment regimen with the highest effectiveness and the fewest side-effects, right from the start of the treatment.

SkylineDx, a member of the project consortium, has already developed and validated a diagnostic device called the MMprofiler™ which can determine the level of risk of a myeloma patient by classifying them into “high” or “standard” risk groups. Patients with “high” risk myeloma do not normally respond as well to treatment and are likely to relapse more quickly than patients who have “standard” risk myeloma. This classification is done using gene expression profiling (GEP), a technology in which the activity (or “expression”) of specific genes is measured in tissue samples – creating a patient specific picture. The GEP of an individual patient represents their biology and can give important clues on response to drugs.

The aim of the project consortium is to build upon this approach by developing a tool which can help myeloma doctors predict the most effective treatment for patients. This will be done through using predictive biomarkers, which are measurable indicators of response to treatment and disease progression. Assessing this within patient tissue samples should give researchers information on the treatment or treatment combinations which are most effective in each individual patient based on their own myeloma subtype. Correlations between predictive biomarkers and the effect of treatment or treatment combinations will then be processed into a treatment decision matrix, which should enable myeloma doctors to determine personalised medicine strategies.

The primary role of MPE in 2018 has been to ensure the patient voice is reflected in the project through conducting a survey on the quality of life of patients and patient preference on treatment. The survey has been translated into five languages (French, English, Italian, German, Dutch). MPE has worked hard on ensuring that the survey was reviewed by patients and developed a comprehensive dissemination plan to seek responses to the survey.

More than 400 responses were gathered in 2018 across these five countries. Results will be published in 2019.

Additionally, MPE has worked to inform the patient community about the progress made within this progress. The main goals of MMPredict are explained in the video below, filmed with some partners working in the project consortium. This video was recorded in English and has been translated into four additional languages: German, Spanish, Dutch and Italian: https://youtu.be/OavFGmuJJwY

3.5.2 Horizon 2020: CARAMBA

Horizon2020 CARAMBA was approved and preparation started at the end of 2017 and will run until 2022. The European Commission selected the CARAMBA project from more than 100 highly

competitive project proposals and will support it over four years with funding of €6.1 million distributed proportionately across consortium partners. Ten partners from six EU countries are collaborating through CARAMBA. The project consortium is researching an innovative immunotherapy for the treatment of multiple myeloma, known as Chimeric Antigen Receptor T-cell therapy (CAR-T). Through strategic collaboration with a wide range of stakeholders, including MPE, the consortium aims to ensure the streamlined transition of a CAR-T product from the laboratory through to myeloma patients in the clinic.

CARAMBA is specifically looking at targeting CAR-T towards a specific protein called SLAMF7 which is expressed on the surface of myeloma cells. The safety and efficacy of SLAMF7 specific CAR-T cells will be assessed through a small Phase I/II clinical trial involving around 30 myeloma patients. In the clinical trial, a type of white blood cell which makes up part of the immune system (T-cells) will be collected from patients and equipped with a chimeric antigen-receptor (CAR). When reintroduced into patients' bodies, it acts like a sensor boosting the ability of the T-cells to find and destroy myeloma cells. This innovative approach has been developed by Dr Michael Hudecek and Prof Hermann Einsele at University Hospital Würzburg.

Over the coming years, the clinical trial will be opening in four cancer centres in Europe – Würzburg, Pamplona, Milan and Lille. As well as the clinical aspects, the project consortium will collaborate on regulatory and access issues and on ensuring that patient preferences are utilised in the project.

Throughout the duration of CARAMBA, the role of MPE will be to communicate about the project to external stakeholders – most importantly MPE members, myeloma patients and their families. MPE will do this using a variety of avenues, including (but not exclusively) via its website, video and written publications and at MPE AGM. In addition, MPE will ensure that myeloma patients and carers are involved in the design of the clinical trial and in exploring the benefits and challenges of CAR-T cell therapy to healthcare systems.

To communicate the main goals of this project to patients and stakeholders, Myeloma Patients Europe (MPE) filmed an interview with Dr Michael Hudecek (University of Würzburg, Germany) The video was recorded in English and was translated into French, German, Spanish and Italian: https://youtu.be/C4eUG18BRjM

3.5.3 Innovative Medicines Initiative (IMI) HARMONY

The third, large scale, European project is the Innovative Medicines Initiative (IMI) HARMONY project which runs from 2017 until 2021. IMI works to improve health by speeding up the development of, and patient access to, innovative medicines, particularly in areas where there is an unmet medical or social need. They do this by facilitating collaboration between the key players involved in healthcare research. IMI is a large-scale public-private partnership in life sciences. It is a partnership between the European Union (represented by the European Commission) and the European pharmaceutical industry (represented by EFPIA, the European Federation of Pharmaceutical Industries and Associations). Through the IMI2 programme (the second round of funding available via IMI), there is a €3.3 billion budget for the period 2014-2020.

IMI HARMONY is a project consortium, involving over 50 partners from across the haematological community, looking at the collection and utilisation of “big data” in haematological cancers. Specifically, the project will gather together, integrate and analyse anonymous patient data from high quality sources including European clinical trials. The project consortia will also define core outcome sets for haematological diseases.

Whilst not an official project partner, MPE is involved in IMI HARMONY through Work Package 6: Stakeholder Forum. The patient involvement element of the work package is led by Leukanet, Germany. Its role is to ensure that patient stakeholders from each haematological cancer are involved in the development of the dataset, in the definition of the core outcomes set and in any additional outputs from this wide-ranging project. MPE therefore provides the myeloma patient voice into the HARMONY project, and its myeloma subgroup, ensuring that relevant patient outcomes sets are defined and that the data is utilised in a way that benefits patients.

3.6 Reasonable Agreements between Patient Advocates and Pharmaceutical Companies

The collaboration between pharmaceutical companies and patient advocates often requires both parties to sign contracts that define the terms and conditions of the collaboration, covering such matters as confidentiality, intellectual property, copyright, data protection, compensation and other responsibilities of both parties. However, the contracts provided to patient advocates are often too long, and are difficult to understand. They contain ambiguous clauses, or terms that are in conflict with the very nature of patient advocacy. They may even put the signing patient advocate at legal risk.

MPE on behalf of the Workgroup of European Cancer Patient Advocacy Networks (WECAN), a network of 21 pan-European cancer patient organisations, along with Patient Focused Medicine Development (PFMD), is leading the project “Reasonable Agreements between Patient Advocates and Pharmaceutical Companies”. This initiative analyses legal contracts between patients’ organisations or patient advocates and pharmaceutical companies, with the aim of evaluating the content of their clauses bearing in mind what a reasonable agreement should look like. The main goal is that patient advocates and patient organisations will collaborate with pharmaceutical companies to develop tools that can make the legal relationship between both parties easier and more acceptable.

This project has three goals:

1. Development of basic guiding principles serving as a basis of understanding and negotiating legal agreements between the parties.
2. Development of four contract templates in a collaborative workgroup of pharmaceutical companies and patient organisations:
    - Consultancy agreement
    - Collaboration agreement
    - Advisory board agreement
    - Community speaker agreement

3. Development of a Toolbox to guide patient organisations and industry representatives to analyse and shape their contracts for the collaboration of patient organisations with the pharmaceutical industry. The Toolbox will help to identify the most problematic clauses and to draft and negotiate them in a reasonable way.

Partners involved in this project are divided in two groups: the drafting group and the Multi-stakeholder Alignment Workgroup (MSAW).

1.- Drafting Workgroup. This is the group in charge of developing guiding principles, drafting contract templates, selecting the most problematic clauses and building the Toolbox. This group is made up of legal experts from MPE, WECAN and PFMD members, plus external experts. The Multi-Stakeholder Alignment Workgroup selected three pharmaceutical company representatives to join this group and represent all companies.

2.- Multi-Stakeholder Alignment Workgroup (MSAW). Composed of representatives of pharmaceutical companies involved in this project as well as patient advocates from WECAN who have shown interest in being involved in the process along with other stakeholder representatives.

In total, 12 pharmaceutical companies are part of this project:

- Novartis
- Pfizer
- Roche
- Novo Nordisk
- Amgen
- Celgene
- Janssen
- Takeda
- MSD
- Bristol-Myers Squibb (BMS)
- Servier
- Bayer

The Workgroup of European Cancer Patient Advocacy Networks (WECAN), is composed of 21 umbrella cancer patient organisations:

- European Men's Health Forum (EMHF)
- EuropaColon
- Europa Donna
- Europa Uomo
- European Organisation of Rare Diseases (EURORDIS)
- European Morbus Waldenström Network
- International Brain Tumour Alliance (IBTA)
- International Kidney Cancer Coalition (IKCC)
- International Neuroendocrine Cancer Alliance (INCA)
- Lymphoma Coalition Europe (LCE)

- Leukaemia Patient Advocates Foundation (LePAF)
- Lung Cancer Europe (LuCE)
- MDS Alliance
- Myeloma Patients Europe (MPE)
- Melanoma Patients Network Europe (MPNE)
- Sarcoma Patient Advocates European Network (SPAEN)
- Thyroid Cancer Alliance (TCA)
- Childhood Cancer International (CCI, formerly ICCCPO)
- Pancreatic Cancer Europe Network
- Acute Leukemia Advocates Network (ALAN)
- Youth Cancer Europe (YCE)

One of the main goals of this project was to develop a guiding principles document for reasonable legal contracts agreed by patient organisations and pharmaceutical companies. Guiding principles were finalised in 2018 and they were shared with MPE members, pharmaceutical companies, WECAN members and patient advocacy groups:

https://www.mpeurope.org/legal_agreements/wp-content/uploads/2018/11/Guiding-Principles_final-document.pdf

At the end of 2018, the first contract template on advisory board agreements was developed. The discussion and modification of this first template will be done in 2019.

3.7 HEM CAB

The Hematology Community Advisory Board (Hem-CAB) is a working group of the European haematology patient community, an informal network of (currently 11) Pan-European haematology umbrellas with more than 260 European member organisations, governed by the seven ePAG (European Patient Advocacy Groups) representatives with long-standing advocacy experience. The network is currently hosted by Myeloma Patients Europe (MPE).

The European haematology patient community works closely with researchers, academics, government, policy makers, authorities and the pharmaceutical industry, featuring a systematic, efficient and accountable network of advocates speaking from the unique perspective of the haematologic patient community. It is strongly involved in the European Reference Network EuroBloodNET and has established strong links with the European Hematology Association (EHA) through its engagement in the EHA Patient Organisations Workgroup, the EHA Fellowship Programme (with 53 patient advocate leaders at the 2017 EHA congress), the Patient Advocacy Track sessions (which have become a steady and unmissable part of the congress and through which the patient community has significantly increased the presence of patient-related topics in EHA's scientific programme), a Capacity Building Programme and a Patient Organisations' booth, among other activities. The European haematology patient community plans to hold a Hem-CAB as a platform to enable discussion between leading advocates and industry representatives in a “safe harbour environment”.

More precisely, the objectives of a Hem-CAB are:

- Patient-run community advisory boards where patient organisations set the agenda, pick the topics and invite stakeholders
- Two-way dialogue with industry (and potentially researchers, academics and authorities) to improve patients' well-being and outcomes
- Global platform where the needs and views of the patient community can be expressed
- Address challenges that patients face in accessing optimal diagnosis, monitoring, treatment and care
- Improve quality of patient information and education
- Develop patient-focused research and clinical trials
- Build capacity and knowledge in the patient community

Concretely, the main objective of the Hem-CAB meeting was to map out and document needs of the patient community, assess areas of joint thinking, and build consensus around these three topics. It aimed to achieve more effective and substantial involvement of the haematology patient community in all, even the earliest, stages of research and development conducted by the companies, to make sure the evidence generated by the patient organisations is developed for impact and is taken into account appropriately when decisions (e.g. in research and development) are being made in industry, and to find solutions to the increased burden of legal and compliance issues.

A first Hem-CAB was held in June 2018. With a preparatory meeting for all patient advocates attending, the actual CAB included the following topics: effective patient engagement in industry R&D, evidence generation by patient organisations to improve decision-making in industry, and overcoming compliance and legal hurdles in the collaboration between patient organisations and industry.

The following pan-European haematology patient organisations were represented in the first Hem-CAB meeting with one delegate each, plus the representatives on the EuroBloodNet ePAG:

- Acute Leukemia Advocates Network (ALAN)
- CML Advocates Network
- CLL Advocates Network
- EFAPH
- European Hemophilia Consortium (EHC)
- ITP Support Association
- International MDS Alliance – MDS UK
- Lymphoma Coalition Europe
- MPN Advocates Network
- Myeloma Patients Europe
- PNH European Alliance
- Thalassaemia International Federation

The meeting was organised and supported by the EuroBloodNET ePAG Project Management Office, all hosted by Myeloma Patients Europe.

The following companies participated in this first Hem-CAB with two representatives each: Alexion, Celgene, Janssen, Jazz Pharmaceuticals, Novartis, Pfizer, Servier and Takeda. In addition, the meeting was supported by AMGEN who could not send a representative on that date.

Industry was asked to present in few minutes what each company does in each of the areas covered. This presentation was followed by a moderated discussion among the group, trying to identify where things are not working properly and how it can be improved.

3.8 Myeloma CAB

MPE works closely with researchers, academics, government, policy makers, authorities and the pharmaceutical industry, featuring a systematic, efficient and accountable network of advocates speaking from the unique perspective of the myeloma patient community.

Besides providing education, information and support to its 43 member-groups across 30 European countries, MPE advocates to achieve the best possible research and equal access to the best possible treatment and care.

The first Myeloma Community Advisory Board (Myeloma-CAB) is a working group of MPE that aims to promote best-in class myeloma research as well as the harmonisation of good clinical practice, standard of care and access to best available myeloma therapies and diagnostic tools.

Myeloma-CAB meetings, based on the proven CML and ECAB model implemented by the HIV patient community and EATG for more than a decade, are community-run advisory boards where the patient community decides on the topics of highest relevance and impact for the patient community. Participants of the Myeloma-CAB meetings will have the opportunity of a two-way dialogue discussing key topics in myeloma between key leading advocates of the patient community and external stakeholders, and discussing potential follow-up action.

All agenda points of Myeloma-CAB meetings are decided by the Myeloma-CAB chairs, in consultation with the Myeloma-CAB Steering Committee. External stakeholders attending the Myeloma-CAB meeting are invited to make suggestions for agenda points, but the Myeloma-CAB makes the final decision in the interests of the community.

The CAB meetings are planned in a holistic view of using the opportunity of having the top leaders from MPE gathered, and in addition to holding discussions with the pharmaceutical industry and other stakeholders, the CAB meeting will be used for training and capacity building of the advocates.

During 2018 MPE started working on the concept and agenda of the first Myeloma CAB, to be held in May 2019.

4 MPE Educational Resources

A core aim of MPE Educational Resources is to underpin the strategic objectives and programmes of the organisation by providing a wide range of accessible and evidence-based information. MPE

wants to ensure that myeloma and AL amyloidosis patients' advocates, patients and other stakeholders across Europe are informed about the latest treatment and care developments in their respective diseases. MPE aims to ensure the dissemination of quality patient information throughout the haematology community. The core educational resources MPE provides are: factsheets, toolkits, Q&A guides, patient guides, educational clips, webinars, videos and conference reports.

4.1 Patients' materials

Providing appropriate information on diagnosis, treatment, care and support for myeloma patients and their families is one of the main objectives of MPE. Only quality information will enable patients to take better decisions and choose between the options they have in every stage of myeloma.

MPE has edited and distributed different materials adapted for patients in order to give them basic information regarding myeloma, its treatment and to help them understand the latest developments presented in international conferences:

- **A myeloma guide.** After being first published in 2016, the myeloma guide was re-edited in 2017 in a smaller format to make it handier and easier to read. The latest developments in myeloma drugs were also included in this new edition. This publication includes information about every step in the management of myeloma. The causes of myeloma, its symptoms, diagnostic tests, treatments and tips on coping with emotional and social issues are some of the topics included. The objective of this guide, written by Dr Vivienne Kendall, is giving the answers to most of the things patients want to know about myeloma: how it may affect them, what treatments they may have and how much or how little will change in what they are able to do. In addition, this guide contains new insights and future directions in myeloma treatment including the latest treatments approved and information about clinical trials and inclusion criteria. This publication is available on the MPE website (http://www.mpeurope.org/files/Patients-guide-mpe-web-version-V2.pdf) and can be requested at info@mpeurope.org.

- **Toolkits.** MPE has edited a series of toolkits for patient organisations in order to help them to build capacity in several areas of interest of their daily work. Currently, there are two toolkits available:

- **Managing volunteers**

With scientific developments and improved treatments, the number of patients that receive treatment and deal with the consequences of this illness has increased. Therefore, the creation of awareness raising campaigns, information and the setting up of support projects for all concerned require more and more people to get involved each day. Patient associations rely on fewer people and resources than they would like. Therefore, one of the main priorities must be to form a team of united and committed volunteers with whom to work and achieve the association's aims.

With the aim of offering an informative tool for the management of voluntary workers in myeloma patient associations across Europe, MPE published a "Voluntary worker management" toolkit. This guide is a support resource for patient associations, and its purpose is to give guidance and information about the process of managing volunteers. This guide offers practical proposals and introduces a voluntary worker plan that will suit the circumstances of each organisation, leading to a greater social involvement in the achievement of your aims.

This toolkit, editing by MPE, is written by Diego Villalón, social worker and co-founder of Fundación Más Que Ideas and Fátima Castaño, psycho-oncologist and programme manager at Fundación Stanpa. This publication is available on the MPE website (www.mpeurope.org) and can be requested at info@mpeurope.org.

- **Social media**

The MPE Social Media Toolkit is a support resource for MPE members and other patient organisations. The main purpose is to give guidance and information about social media management and to increase the visibility of organisational activities. Throughout the toolkit it summarises tips for patient organisations to get the greatest benefit from their social networks and how to reduce the associated risks. The toolkit is particularly important, given the increasing use of social media as a way of communicating with external audiences and managing organisational reputation.

- **Factsheets.** MPE had worked on a series of Factsheets about different myeloma drugs in a patient-friendly style. Each document includes information about how each type of drug works, what side effects the treatment has and how and when the drug is given. In

2018 MPE printed five new factsheets (Amyloidosis, Panobinostat, carfilzomib, ixazomib and Elotuzumab). This series of Factsheets includes the following treatments:

- **Stem cell transplant**
- **Thalidomide**
- **Lenalidomide**
- **Pomalidomide**
- **Bortezomib**
- **Daratumumab**
- **Amyloidosis**
- **Panobinostat**
- **Carfilzomib**
- **Ixazomib**
- **Elotuzumab**

- **Webinars.** Through this format, patients are able to attend these online meetings in which an expert explains a specific topic about myeloma supported by slides. After the talk, a time slot is reserved for a Q&A session. All webinars are recorded and are available for patients in MPE's YouTube channel and the MPE website: https://www.mpeurope.org/what-we-do/publications/webinars/.

- **Videos.** MPE developed a series of videos focusing on different areas of interest for myeloma advocates. Educational clips, summaries of MPE projects or the highlights presented at scientific meetings are some of the topics covered in our videos. During 2018 MPE filmed the following videos:

  - **CARAMBA. F**ilmed interview with Dr Michael Hudecek (University of Würzburg, Germany) on the H2020 project, CARAMBA. The video was recorded in English and was subtitled into four additional languages: French, German, Spanish and Italian.
  - **MMPRedict.** Filmed interviews with some partners working in the project consortium of the H2020 project, MMPredict. This video was recorded in English and subtitled into four additional languages: German, Spanish, Dutch and Italian.
  - **Clinical challenges in myeloma.** Educational clip with Dr Sonja Zweegman, professor of haematology, Head of department of Hematology in at the VU University Medical Centre, in Amsterdam (the Netherlands) on clinical challenges in myeloma.

5 Workgroups and taskforces

5.1 MPE Advocacy and Policy Workgroup

In 2018 MPE established a workgroup to address key EU policy initiatives. It is also designed to address additional issues such as access to medicines, treatment and care in different parts of Europe.

As part of this initiative a steering workgroup was created to focus on the specific situation of members in the Central and Eastern part of Europe (CEE). The CEE workgroup organises a CEE platform meeting in 2019 to invite MPE members of this region to exchange and identify issues and give input for the ongoing and potential new MPE collaborative actions in this area.

Direct contact with key haematologists and stakeholders was established to address the issues on access in Eastern Europe. In 2018 a draft letter was agreed between the Board of the European Myeloma Network (EMN), the largest group of myeloma researchers and doctors, and MPE, as the largest group of myeloma patient organisations in Europe, to discuss greater joint working. This has been signed off and will be sent to the EMN in 2019.

5.2 AL Amyloidosis Taskforce

The Annual General Meeting held in April 2018 agreed in include AL amyloidosis across all activities and strategy of MPE. In accordance with the decision, the AL Amyloidosis Taskforce was created.

The main objective of this group is to drive the AL amyloidosis agenda of MPE, improving the services we provide to the AL Amyloidosis Taskforce and increasing engagement of members in AL amyloidosis (particularly on countries where there is no patient organisation).

During 2019 a number of activities will be developed by this taskforce to strengthen and improve activities focused on AL amyloidosis, not only by MPE but also but its members.

6 MPE external Impact

6.1 External Meetings

Besides numerous meetings with key stakeholders, such as industry, regulators, payers, HTA bodies and European institutions, MPE has attended other key meetings and congresses. Below we highlight a few of the most relevant.

Providing appropriate information on diagnosis, treatment, care and support for myeloma patients and their families is one of the main objectives of MPE. To do that, MPE attends the most important oncology and haematology scientific meetings around the world to be updated about the latest developments regarding myeloma and AL amyloidosis. MPE also takes the opportunity to meet with key stakeholders, sponsors, pharmaceutical companies and MPE members to update them about the different programmes and initiatives that are being developed.

During 2018, MPE attended the following conferences:

- **Annual Corporate Compliance & Transparency in the Pharmaceutical Industry**
  The CEO of MPE, Ananda Plate attended the Annual Corporate Compliance & Transparency in the Pharmaceutical Industry that was held in Zurich (Switzerland) on 22 February 2018. She presented the progress of the project Reasonable agreements between patient advocacy and the pharmaceutical industry (RAPP). See point 5.6.

- **3rd ESO Masterclass in Cancer Patient Advocacy**
  The European School of Oncology (ESO) celebrated the 3rd ESO Masterclass in Cancer Patient Advocacy from 23 to 25 February in Lisbon (Portugal). This meeting brought together senior representatives of European and international cancer patient networks as well as a small number of future leaders. The CEO of MPE, Ananda Plate, presented at and attended this meeting as part of the Steering Committee. Moving from anecdotal to evidence-based advocacy, achieving sustainable access to cancer therapies or building effective and sustainable EU cancer patient networks were some of the topics discussed.
  From 2019 onwards, this Masterclass will be organised and branded by WECAN (WECAN Masterclass) and will be integrated into the WECAN Academy.

- **WECAN meetings**
  The Workgroup of European Cancer Patient Advocacy Networks (WECAN) is an informal network of 21 cancer patient umbrella organisations in Europe, which has the aim to join forces and avoid duplication of effort in areas and initiatives that are cross-disease. Some of the most popular initiatives are:
    - Reasonable agreements between pharmaceutical companies and patient advocates
    - Fair Market Value
    - WECAN Academy, which now is in charge of the ESO Masterclass and SMART START. In addition, WECAN is creating an aligned educational strategy for the cancer patient community.

  MPE's CEO Ananda Plate has been the chair of WECAN since September 2017.
  WECAN organised three meetings in 2018. The first one was held on 25 February in which MPE gave an overview of the progress made in the project "Reasonable agreements between patient advocacy and the pharmaceutical industry (RAPP)", led by MPE.
  The second one was a two-day strategy retreat held on 29 and 30 June. The attendees discussed joint action by the pan-European cancer patient umbrella organisations. Attendees had productive discussions about the training strategy for cancer patient advocates, the position paper on the European alignment on HTA (see www.wecanadvocate.eu), the project on legal agreements and fair market value, among others.
  A third face to face meeting took place at ESMO 2018, in October 2018.

- **European Patients' Academy on Therapeutic Innovations (EUPATI) training course**

  Every year the European Patients' Academy organises a training course for patients and patient representatives on the Medicines Research & Development Process which includes two face to face meetings.

  In terms of participation of MPE in EUPATI, MPE's CEO Ananda Plate is part of the Programme Committee of the current EUPATI course. MPE's former Project Manager, Alfonso Aguarón and MPE's Policy and Public Affairs Manager, Kate Morgan, participated in both face to face meetings as a speaker. Additionally, Ana Vallejo, MPE Communications Manager, was one of the trainees at EUPATI.

- **ASCO 2018**

  The Annual Congress of the American Society of Clinical Oncology, 54th ASCO Annual Meeting, was held between 1 and 5 June in Chicago. This is the most important scientific congress in oncology with hundreds of sessions presenting the most important updates in all kinds of cancers. MPE attended to gather the most important updates for the myeloma community.

- **EHA 2018**

  The European Hematology Association (EHA) Annual Congress was held in Stockholm (Sweden) from 14 to 17 June. MPE also had a booth in the Advocacy Area and attended the most relevant sessions for myeloma advocates in order to inform MPE members about the latest developments in myeloma. Additionally, Ananda Plate talked about the impact of cancer and its treatment on sex life and Alfonso Aguarón chaired a session about patient-relevant endpoints and patient-reported outcomes.

  Also at EHA 2018, MPE held the first face-to-face meeting of the Advocate Development Programme, a two-day session covering a wide range of topics relating to clinical development of new medicines and pipeline drugs in myeloma and AL amyloidosis.

- **HTAi 2018**

  The Health Technology Assessment International (HTAi) conference took place in Vancouver in June 2018. HTAi is a professional organisation promoting information sharing across the key stakeholders in HTA. During the congress, MPE Policy Manager, Kate Morgan, participated in a workshop designed to discuss patient involvement in HTA. In addition, she participated in a panel discussion on patient preference studies and how the results can be taken into account in decision-making. MPE is getting more involved in the patient involvement element of HTAi and will take this forward in 2019.

- **EMN 2018**

  The first meeting of the European Myeloma Network (EMN) took place in April 2018. This was a meeting for stakeholders involved in myeloma research to present and discuss the latest trends the field of myeloma. Kate Morgan attended the whole conference and

presented on patient experience of myeloma and the key areas of importance for myeloma patients and their caregivers/family members.

- **COMY 2018**

  This is an annual conference which took place in Paris in May 2018. Kate Morgan attended the meeting to represent MPE, participating in a pre-workshop organised by MPE member AF3M on medicines access. The rest of the conference was focused on controversies and areas of debate in myeloma research.

- **ESMO 2018**

  MPE attended the European Society of Medical Oncology (ESMO) Annual Meeting which took place from October 19-23, at Messe Munich, in Munich, Germany to be updated on key developments in oncology. MPE exhibited in the Patient Advocacy Area and also attended the most important sessions to inform our members about the areas of interest for myeloma advocates. Ananda Plate gave a talk “The Myeloma Access Atlas: Training programme” included in the session “Knowledge is power: Educating patients and advocates” within the Patient Advocacy Track.

  The Advocate Development Programme held its second two-day face-to-face meeting during this event, inviting to the ADP trainees to attend the congress (see point 5.2).

- **ISPOR 2018**

  The Pharmacoeconomics and Outcomes Research (ISPOR) Annual Congress took place in Barcelona (Spain) from November 10-14. Policy and Public Affairs Manager of MPE, Kate Morgan and the CEO of MPE, Ananda Plate participated in the ISPOR Patient Representative Roundtable which discussed topics including the evolution of patient involvement in health technology assessment.

  The Advocate Development Programme held its third face-to-face meeting during this congress. All ADP trainees were invited to ISPOR 2018 (see point 1.2).

- **EURORDIS CEF Workshop**

  The EURORDIS Council of European Federations took place in Paris (11-12 December). Every year EURORDIS invites its members to give them an update on their projects and activities. Ana Vallejo, MPE Communications Manager, attended the workshop representing MPE.

- **American Society of Haematology Annual Scientific Meeting**

  MPE attended ASH 2019 in San Diego from 1-5 December 2018. MPE team members, the CEO of MPE, Ananda Plate, and the MPE Policy and Public Affairs Manager, Kate Morgan, attended to learn about the latest scientific developments in myeloma and haematology and to network with key stakeholders in the field. It was also an opportunity to meet with MPE partners to discuss joint areas of work over the next year.

6.2 European Policy and Regulation

MPE engages with regulators such as the European Medicines Agency (EMA) on their assessment of new medicines and with policy-makers to influence ongoing initiatives that impact on myeloma treatment and care access.

During 2018, MPE aims to influence ongoing policy debates on the following topics:

- Public health
- Regulation of medicines
- Integration of health technology assessment at the European level
- Access to research and clinical trials (including EU funding on these areas)
- Life sciences and innovation

MPE works directly with a wide range of stakeholders to ensure that the right types of clinical and patient-level evidence are being collected for national decision-making on new medicines. MPE also works to overcome identified challenges with access to myeloma drugs when they arise at the regulatory level.

MPE is permanently represented at the EMA via the PCWP, through Ananda Plate (member) and Kate Morgan (alternate).

The key activities MPE has undertaken on these issues are:

- Responding to a public consultation on the EU budget (including on public health)
- Following the legislative proposal on further harmonisation of health technology assessment at the European level – creating briefings, website commentary and position papers (and working with WECAN)
- Public affairs work in Brussels, meeting with key stakeholders on the HTA legislative proposals and attending sessions in the European Parliament to discuss the debate
- Working through WECAN on the position paper on the legislative proposal on HTA harmonisation
- Presenting at the ESMO WECAN meeting on issues relating to the legislative proposal
- Participation in discussions with EFPIA on issues relating to the combination pricing of new medicines
- Following the impact that Brexit might have on medicines regulation:
- Working with two patient representatives to brief them prior to participating in the European Medicines Agency process on plitidepsin
- Designing and coordinating action on plitidepsin, writing to the EMA to inform them on the importance of the medicine to the myeloma community
- Continued work on melphalan and issues associated with medicines shortages related to the drug, meeting with relevant personnel within the European Medicines Agency to discuss progress on policy relating to medicines shortages
- Regularly attending meetings of the EMA Patient and Consumers Working Party (PCWP) to discuss issues relating to patient regulation of medicine
- Setting up regular calls with access representatives in pharmaceutical companies to discuss regulation and policy issues

- Regularly communicating via our website on issues relating to policy and regulation – such as daratumumab receiving a European licence for newly diagnosed myeloma patients

6.3 Driving advocacy in haematology

In some areas that have cross disease topics and issues, the haematology community needs to act in a united way, mainly where resources can be shared and the community shares the same objectives. This has proved very effective, especially when there are limited resources. MPE is member of the following organisations:

EuroBloodNet:

This is the European Reference Network (ERN) for rare haematological diseases. The ERN aims to provide the sharing of information, expertise and resources on these diseases across multidisciplinary healthcare teams in 15 EU member states. Within each ERN there is a European Patient Advocacy Group (ePAG) which brings together elected patient representatives and patient organisations who will ensure that the patient voice is heard in the development, programming and evaluation of ERN initiatives and activities. Ananda Plate, CEO of MPE, was elected to the EuroBloodNet ERN EPAG and throughout 2017 has ensured that views of the myeloma patients and caregivers are heard in the development of the ERN.

European Haematology Association (EHA) workgroup:

Twice a year, EHA invites patient organisations to meet and discuss current issues in haematology, to exchange information, and to develop joint projects. MPE is one of the organisations collaborating with EHA and representing myeloma patients in these discussions. As part of this workgroup, MPE was also involved in deciding the patient advocacy sessions at the prestigious EHA Annual Congress 2017, including the capacity building programme for patient advocates granted an EHA Fellowship.

European Society of Medical Oncology Patient Advocates Working Group (ESMO PAWG):

The central aim is collaborating with ESMO, patient advocates and other stakeholders to optimise cancer patient care, the continuous improvement of cancer-specific information and education, the strengthening of patient autonomy and the support of patient rights. The PAWG, of which Ananda Plate is a member, designed and co-ordinated the eight patient advocacy track sessions that took place at the ESMO annual meeting, which registered its largest ever attendance.

The European Cancer Organisation Patient Advisory Committee (ECCO PAC):

Through membership of this initiative, MPE collaborates with European professional cancer organisations and provides ECCO with direct insight into the issues and challenges faced by myeloma patients across Europe. The ECCO-PAC is the patient advisory body in ECCO. Sarper Diler represents MPE in the ECCO-PAC.

Rare Cancers Europe (RCE):

Rare Cancers Europe (RCE) is a multi-stakeholder initiative dedicated to putting rare cancers firmly on the European policy agenda and to implementing 39 political and stakeholder recommendations. These recommendations are divided into different areas of action in rare cancers: regulatory barriers, methodological barriers, the need for centres of expertise and European reference networks, barriers to patients' access to care, recommendations on education of healthcare professionals and access to information on rare cancers.

Throughout 2018, MPE has been working with RCE to create a treatment and care mobile app for myeloma patients in Europe, where they will be able to access up-to-date information on treatments and where these are approved across Europe. The first meeting took place in December 2018 and it will continue over the course of 2019.

EURORDIS

EURORDIS is a non-governmental patient-driven alliance of patient organisations representing 724 rare disease patient organisations in 64 countries. EURORDIS seeks to improve the quality of life of people living with rare diseases in Europe through advocacy at the European level, support for research and medicines development, facilitating networking among patient groups,

raising awareness, and many other actions designed to reduce the impact of rare diseases on the lives of patients and family. Kate Morgan has been appointed to the EURORDIS health technology assessment (HTA) advisory group. This is a taskforce designed to advise EURORDIS on their work programme on the topic of medicines access.

European School of Oncology (ESO):

ESO aims to improve the treatment of cancer patients across Europe through improving the skills that healthcare professionals have and improving the transfer of knowledge from research through to cancer patients in the clinical setting. ESO also runs training programmes for patient advocates to ensure they have the skills needed to advocate effectively on a wide range of issues. In 2017, MPE has been involved in steering the development of the patient advocacy masterclass to ensure it is fit-for-purpose for the cancer community and ties in with other initiatives ongoing throughout Europe.

Workgroup of European Cancer Networks (WECAN):

WECAN is a network of 21 European umbrella organisations representing cancer patients. It works to share information and establish a joint work programme on common issues across cancer and patient advocacy, including patient involvement in decision-making on treatment and care, EU proposals for further harmonising HTA and how patient advocates and industry can work together through effective collaboration. Throughout 2017, MPE has had a very active role in WECAN, leading on a project entitled ‘Reasonable agreements between patient advocacy and the pharmaceutical industry.’

7 Acknowledgements

The activities and materials developed throughout 2018 are the result of collaborative work with several organisations and stakeholders. Some of them helped us by providing funds to make our projects happen. Others worked hard along with MPE to develop initiatives and projects aimed at improving diagnosis, treatment and quality of life of myeloma patients.

A special thanks to all MPE members because they are the most important part of our work and the final objective of every activity we develop. Without their help and collaboration, MPE would have never achieved such good results this year.

MPE wishes to thank its stakeholders, sponsors, organisations and partners for their key collaboration and their help, which makes it possible for MPE to continue working for myeloma patients across Europe.

Myeloma Patients Europe AISBL

Avenue Louise 149/24
1050 Brussels, Belgium

www.mpeurope.org - info @mpeurope.org

facebook.com/mpeurope

@MyelomaEurope