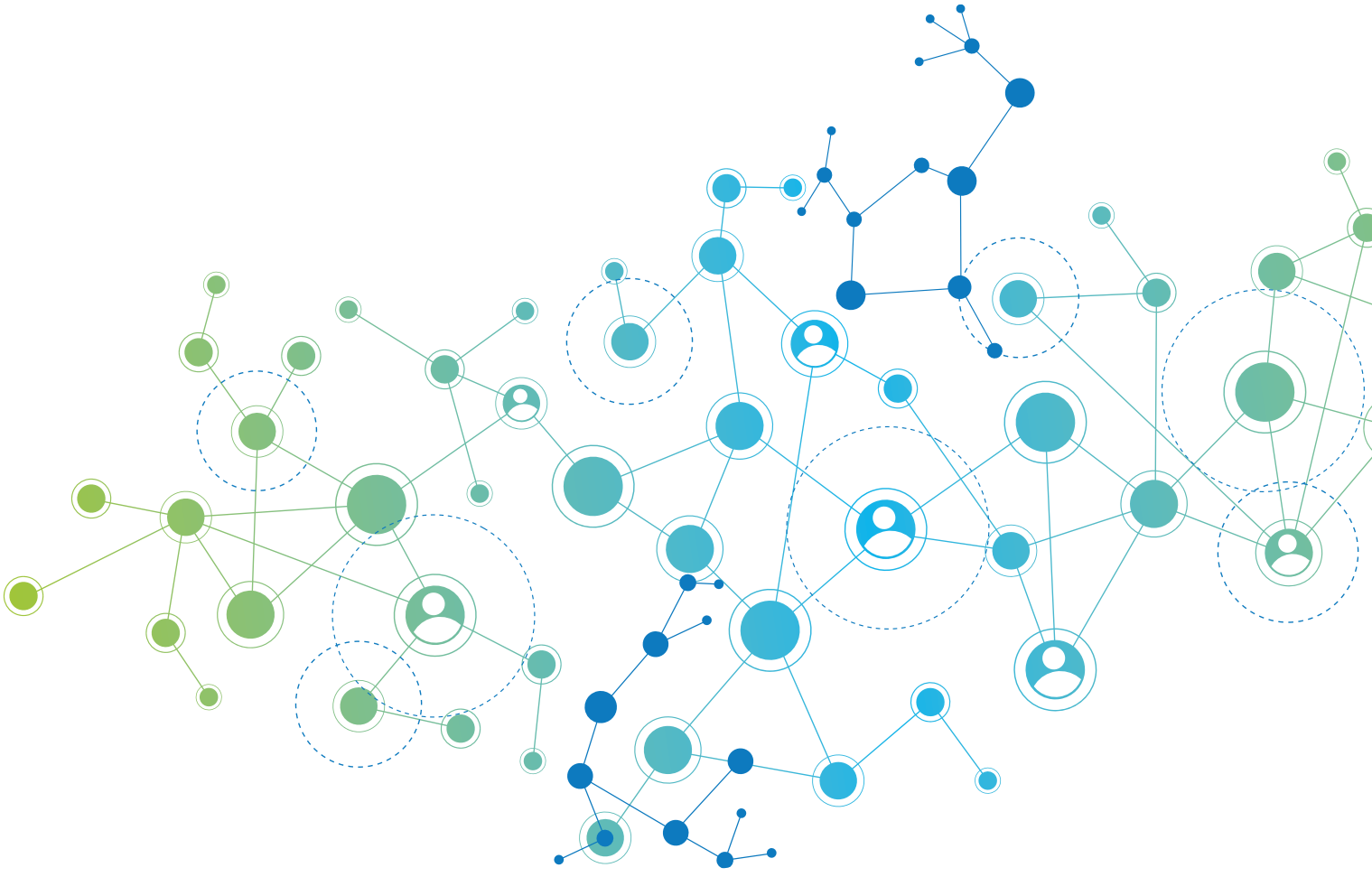
Myeloma Patients Europe

2019 Annual Report



www.mpeurope.org

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Message from the President and the Chief Executive Officer

A lot of important things happened for Myeloma Patients Europe (MPE) in 2019. MPE has maintained its key role as one of the strongest umbrella patient organisations in Europe without forgetting its main goal: providing a strong voice for myeloma and AL amyloidosis patients at a European and international level.

One of the initiatives that marked 2019 for MPE and also for the myeloma community was the launch of the Myeloma Community Advisory Boards (Myeloma CABs). For the first time, MPE organised a series of Myeloma CABs along with a working group of patient advocates with the aim of monitoring pharmaceutical developments through active and targeted interaction and long-term cooperation with pharmaceutical manufacturers, regulators, and the scientific community working in the field. Three myeloma CABs took place during 2019 with six pharmaceutical companies to discuss myeloma research, the harmonisation of good clinical practice, standard of care and access to best available myeloma therapies and diagnostic tools.

Our organisation continued playing a fundamental role in two European Commission projects funded through Horizon 2020, the biggest EU Research and Innovation programme. These projects are CARAMBA and MMPredict. The first one focuses on testing the revolutionary chimeric antigen receptor therapy or “CAR-T” cell technology to tackle myeloma. The second one is focused on clinically validating a personalised medicine tool that predicts the most effective treatment options for myeloma patients. MPE ensured that patient voice will be reflected in the project through a survey to gather information about myeloma patients’ quality of life. The main results of this survey were summarised in a poster presented at the most important European scientific congress in haematology: the European Hematology Association (EHA) Congress. Two MPE staff members were included as co-authors of this poster.

Throughout 2019, MPE has continued work on core programmes such as the Annual General Meeting (AGM), the Scholarship and Capacity Building Programme, the Advocate Development Programme and the Myeloma Access Atlas. All of these have been improved to respond to the needs of our members and myeloma and AL amyloidosis advocates in Europe. To this end, MPE started also to film educational videos in different languages with the aim of helping our members to disseminate relevant information and updates from the most important scientific congresses at a national level, always in collaboration with our members.

But our work in 2019 has not only focussed on supporting the myeloma community. MPE has continued their work around their wider commitment to the haematology and cancer communities. During 2019 the Workgroup of European Cancer Patient Advocacy Networks (WECAN) was further consolidated as a strong network in the cancer patient community. This was possible thanks to the 22 umbrella organisations involved in WECAN and under the

leadership of the MPE CEO, Ananda Plate, who has been WECAN chair for the last two years until autumn 2019. During 2019, MPE kept its key role in WECAN, not only leading its first project, “Reasonable agreements between patient advocacy and the pharmaceutical industry” (RAPP), which will be finalised at the beginning of 2020, but also having a key role in the second successful WECAN project: the WECAN Academy.

In 2020, MPE will be leading new important projects for WECAN in close collaboration with WECAN members and the recently appointed WECAN chair, Gilliosa Spurrier, from Melanoma Patient Network Europe (MPNE).

All MPE’s achievements and successes, and the progress made in our activities, programmes and projects detailed in the following pages would not have been possible without a committed and focused board, a hard-working, dedicated staff and an enthusiastic and engaged network of members and collaborators willing to work tirelessly to improve myeloma and AL amyloidosis patients’ lives.

Also, all this would not have been possible without the continued support of our sponsors and collaborators who made it possible to continue working and advocating for myeloma patients across Europe.

It has been an exciting year but the best is yet to come.

We cannot finish this letter without a very special thanks to our board member, Lisa Kotschi, who passed away last November. She never rested from trying to improve diagnosis, access and quality of life for myeloma patients in Germany in her role within her local advocacy organisation, and across Europe as part of MPE’s board. Lisa was an inspiration in her passion and commitment to advocacy and her certainty in the crucial role of both national and local advocacy organisations in driving forward improved access to best possible care, information and support. MPE and the myeloma community lost a very special, helpful person and a passionate advocate and we seek to remember Lisa and her dedication in the work we will continue in 2020

Hans Scheurer

President of Myeloma Patients Europe

Ananda Plate

CEO of Myeloma Patients Europe

MPE 2019


January
The second cohort of the Advocate Development Programme (ADP) ends its training
February
MPE attends the IMI Harmony Masterclass, a project that involved over 50 partners across Europe
March
MPE holds its Annual General Meeting (Annual Masterclass and AGM General Assembly) in Munich (Germany)
April
The third edition of the Advocate Development Programme (ADP) starts with six trainees
May
MPE holds it first Myeloma Community Advisory Board (CABs). This is the kick off of a succesful year of piloting the programme
June
Launch of the survey on myelo-ma patients information needs. MPE recruited 1.000 responses
July
The first WECAN Academy is held in Frankfurt with the MPE CEO, Ananda Plate, as WECAN chair and speaker
August
The first periodic report within H2020 projects, CARAMBA and MMPredict, was submitted to the European Comission
September
Three talks were given by MPE staff members at ESMO 2019, held in Barcelona, Spain
October
MPE Central and Eastern Europe (CEE) Workgroup on Access holds its first virtual meeting
November
MPE attends ISPOR 2019 participating in the patient roundtable and holds its last Myeloma CAB of the year.
December
MPE attended to ASH 2019 in Orlan-do, Florida, where 16 educational clips in different languages were filmed and disseminated.

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
- Helping to develop 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 46 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 who are of the same nationality or who represent the same country. It is also agreed that no public or private organisation may 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 March 2019 the Board was composed of the following members:

- 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)
- The terms of Ron Dloomy and Biljana Dodeva finished at the AGM 2019 and they stood for re-election. After the AGM 2019 elections, the board had the following composition:
  - President: Hans Scheurer (Netherlands)
  - Vice-President: Ron Dloomy (Israel)
  - Secretary: Riikka-Leena Manninen (Finland)
  - Treasurer: Johannes Brenner (United Kingdom)
  - Board members: Kristina Modic (Slovenia), Lisa Kotschi (Germany), Biljana Dodeva (Macedonia), Roman Slomkowski (Poland) and Lise-Lott Eriksson (Sweden).

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

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 Vice-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.

Ananda speaks Spanish, German, English, Catalan and French. She is currently based in Munich, Germany.

Staff

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 Head of Policy and Public Affairs 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.

Nicole Wicki

Nicole joined MPE in March 2019 as Patient Advocacy Programme Manager. She holds a Bachelor of Science with specialisation in Psychology from the University of Alberta and a Master of Science in Communication, Management and Health from the Università della Svizzera italiana. Her main fields of interest include public health, patient empowerment and equitable healthcare access.

Nicole has experience in health policy and communications research and health promotion programme evaluation in the areas of vaccination, tobacco control and addictions and mental health. Prior to working for MPE, she worked in patient advocacy in the pharmaceutical industry, driving capability building activities and initiatives to embed patient voice in pharmaceutical research and development.

Nicole is from Edmonton, Canada, and has been living in Switzerland for over eight years. She speaks English, French and German and is currently based in Zermatt, Switzerland.

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 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.

1.3 MPE members

MPE is composed of 46 myeloma patient groups and associations in 30 European countries:

- Armenia - Armenian Haematology Association (AHA)
- Austria - Multiples Myelom Selbsthilfe Oesterreich (MMSOe)
- Austria - Myelom und Lymphomhilfe österrecih
- Belgium - Contactgroep Myeloom en Waldenström Patiënten Vlaanderen vzw (CMP vzw)
- Belgium - Wallonie-Bruxelles (MyMu)
- Bosnia and Herzegovina (Associate, individual)
- Croatia - Mijelom CRO
- Croatia - HULL Croatia - Croatian leukaemia and lymphoma Society
- Czech republic - Multiple Myeloma Patient Support Group (KPMM) (Associate)
- Denmark - Dansk Myelomatose Forening
- Estonia - Eesti Müeloomiliit
- Finland - Suomen syöpäpotilaat-myelooma potilasverkosto
- France - Association Française des Malades du Myélome Multiple (AF3M)
- Germany - AMM-Online, Multiple Myeloma Online Working Group
- Germany - German Leukemia and Lymphoma Patients' Association (DLH)
- Germany - Myeloma Group Rhine Main - Leukaemia Help Rhine Main - LHRM
- Germany - Myelom Deutschland e.V.
- Hungary - MOHA– Foundation for Hungarian Oncohaematological Patients

- Israel - Amen Israeli Myeloma Patient Foundation (AMEN)
- Israel - Amyloidosis Israel
- Latvia - Latvian Cancer Patient Support Society “Dzivibas koks”
- Lithuania - Kraujas
- Macedonia - BORKA
- Macedonia - Association for help and support of patients and their caregivers with haematological diseases (HEMA)
- Netherlands - Stichting Hematon
- Netherlands - Amyloidosis Foundation Netherlands (SAN)
- Norway - Norwegian Blood Cancer Association / Blodkreftforeningenn
- Poland - Carita Fundation
- Poland - Polish Myeloma Patient Help Association
- Portugal - Associação Portuguesa Contra a Leucemia (APCL)
- Portugal - Associação Portuguesa Leucemias e Linfomas (APLL)
- Portugal - Centro de Histocompatibilidade do Norte (CHN) (Associate)
- Romania - Myeloma Euronet Romania (MER)
- Romania - SOS mielom
- Russia - Society for Assistance to Patients with Oncohaematological Diseases
- Serbia - Association of myeloma patients of Serbia (AMPS)
- Slovakia - Association of patients with haematological malignancies
- Slovakia - Slovak Myeloma Society (SMyS)
- Slovenia - Drustvo bolnikov z limfomom (DBL)
- Slovenia - Slovensko Združenje Bolnikov Z Limfomom In Levkemijo L&L
- Spain - AMILO
- Sweden - Blodcancerförbundet Sweden
- Switzerland - Foundation for the Advancement of Bone Marrow Transplantation Switzerland (SFK)
- Switzerland - Myelom Kontaktgruppe Schweiz (MKgS)
- Turkey - KANKO/BIRKAN
- United Kingdom - Myeloma UK (MUK)

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
- Helping to develop 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 2019-2024 are to:

- Provide a strong voice for myeloma patients at a European and international level.
  MPE's work relies on evidence-based advocacy. In order to represent and be recognised as a legitimate voice for the patient community, MPE must gather evidence about the needs and wants of patients to be able to represent them properly. This is not only the right thing to do but will also positively impact the perception of MPE among all key stakeholders.
- Strengthen members' and individual advocates' ability to advocate effectively.
  A skilled and well-prepared member organisation can advocate more effectively and efficiently at a national level, to overcome barriers and challenges. Also, the development of individual advocates at European and national level will help our community in achieving the desired impact.
- Secure a larger patient voice in myeloma research.
  In order to make sure myeloma research delivers to patients' unmet needs from a clinical, care and quality of life perspective, MPE needs to empower the community and its individual advocates to become equal partners in research and regulatory affairs and to contribute to discussions and solutions in a meaningful way. To justify a seat at all relevant tables and to be able to provide meaningful, qualified and evidence-based input, MPE needs to continuously educate and prepare individual patient advocates in key knowledge areas. In parallel, MPE strives to collaborate with all key stakeholders as a respected partner, especially in the areas of drug development and regulatory affairs. MPE needs to stay in dialogue with all relevant stakeholders, to ensure the needs and wants of patients across Europe are taken into account during the entire drug development process, but also needs to closely monitor and push for the new treatments to reach the patient without unnecessary delays.
- Accelerate progress in the treatment and care of myeloma patients.
  MPE needs to stay in dialogue with all relevant stakeholders, to ensure the needs and wants of patients across Europe are taken into account during the entire drug development process, but also needs to closely monitor and push for the new treatments to reach the patient without unnecessary delays.
- Increase the number of patients who have their myeloma diagnosed in a timely manner.
  Late diagnosis is still a big problem in myeloma, with poor prognosis and important quality of life issues among the main consequences for patients. MPE needs to search for effective ways of addressing this issue at a European level but also help member organisations to address this at national level. It is key for MPE not to raise awareness generally but to strictly target any initiative to ensure a meaningful impact.
- Improve access to optimal treatment and care for all myeloma patients in Europe.

Access to treatment and care is a challenge in most European countries. MPE needs to ensure it supports its members to advocate effectively and efficiently at national level, i.e. by generating and providing evidence to them, by coaching them, by helping them develop strategies to advocate. MPE will at the same time advocate and engage with stakeholders at European level whenever impact can be achieved at that level and taking into account the limited competencies in access issues that exist at European level.

- Strengthen MPE's effectiveness, sustainability and capacity to take collective action. MPE needs to continue investing, not only in growth and impact, but in strengthening the organisation from the inside. This needs to be done with proper governance rules and their effective implementation, by gathering feedback from members and staff, by having a well-functioning Board, by diversifying and securing funding sources, among other means. Towards the outside, it is crucial that MPE continues working on a communications strategy (internal and external) and its implementation, to ensure the work done and impact achieved is also well communicated to the outside and understood by both the membership and key stakeholders. This will consolidate the reputation and credibility of MPE to the outside but also increase satisfaction of members, Board and staff.

To meet its aims, MPE works directly not only with its members but also 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 Programme

MPE completes its access work under the framework of the MPE Access Atlas, a platform that MPE has developed, in collaboration with the Avedis Donabedian Research Institute (Barcelona), to help build the knowledge and capacity of members through providing the information needed to advocate effectively on national access issues. The platform is located here: http://www.mpeurope.org/atlas/

The Atlas platform includes country-specific and comparative information on 30 European health systems from a range of academic, online and organisational sources. It is also populated with the results of surveys MPE has conducted, asking myeloma patient organisations and clinical experts about myeloma treatment and care challenges they face in their country. The Atlas provides statistical information on the treatment and care access situation in a country, alongside information on the general operation of the health system and "pressure-points" to target to effect change.

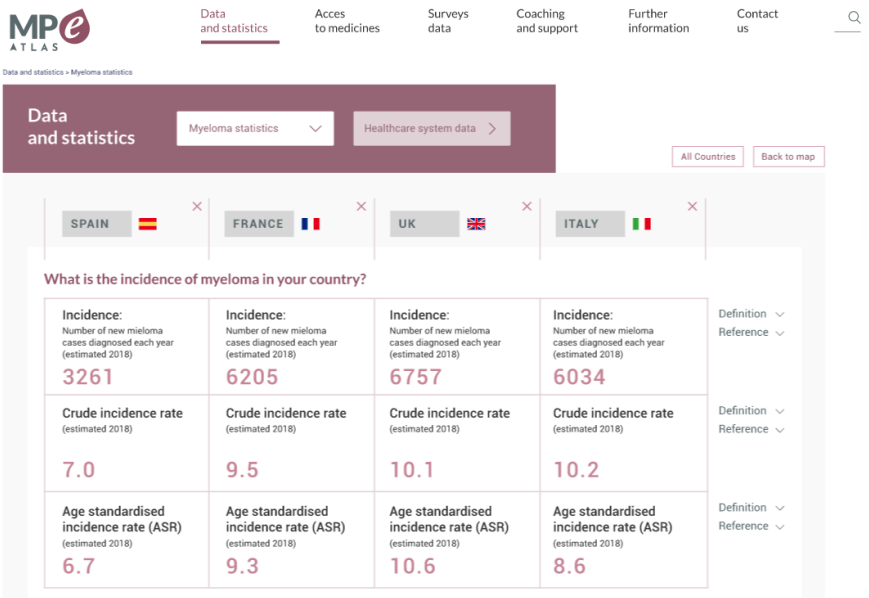
As part of the Atlas, MPE has also developed the Atlas Coaching Programme to assist members in developing an individualised strategy to overcome national access challenges. Any member of MPE can contact the team to highlight specific access challenges or reimbursement hurdles faced in their country and to collaborate to develop and implement solutions to these

challenges. Members tell MPE that this flexible method of support is very useful, given the tailored research, information and solutions MPE can provide.

Finally, to underpin the Atlas programme, MPE also engages in public affairs and policy work at a European level to advocate for fair access to high standards of treatment and care across Europe. This includes having a regular dialogue with industry on their market access programmes in Europe.

Between January and December 2019, MPE undertook the following activities:

- Analysis of the results of the access survey conducted at the end of 2018, which have been written up into a publishable report and will be published on the Atlas website
- 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 eight European countries
- The agency AR Difusión (Spain) was selected following the tender process to design and develop a new Atlas website
- Information on drug availability from all pharmaceutical companies has been collected. This information will be used to populate an online tool on the above mentioned website
- The first virtual meeting of the MPE CEE Workgroup on Access has taken place. A detailed project plan has been developed on a clinical trial position paper
- A letter was sent to the European Myeloma Network Board to develop strategic links with MPE, around issues relating to access and research programmes and a strategic meeting took place at ASH 2019 with the EMN Board
- A review and re-design of the Atlas Coaching Programme tool has been started and will be done along with the redesign of the Atlas website. A strategy is being developed for further content and evidence generation for the MPE Access Atlas, which will be implemented over 2020



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 evidence based advocacy, interaction with relevant stakeholders, drug development process and many more.

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, the European Society of Medical Oncology (ESMO) Annual Congress, the European Conference of the International Society on Pharmacoeconomics and Outcomes Research (ISPOR), among others. Congresses and meetings are chosen every year depending on dates and programmes.

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.

Six trainees were included in the ADP 2019: Elena Butler (Spain), Michael Blomqvist (Sweden), Agnes Magyar (Hungary), Chiara Zampetti (Italy), Gislinde Zimmermann (Germany) and Jürgen Martens (Germany).

The ADP 2019 kicked off in May 2019 and runs until March 2020. It includes eight webinars and three face to face meetings held at EHA, ESMO and the MPE Annual General Meeting (AGM) 2020.

The list of topics included in the ADP 2019 is the following:

Webinar Programming

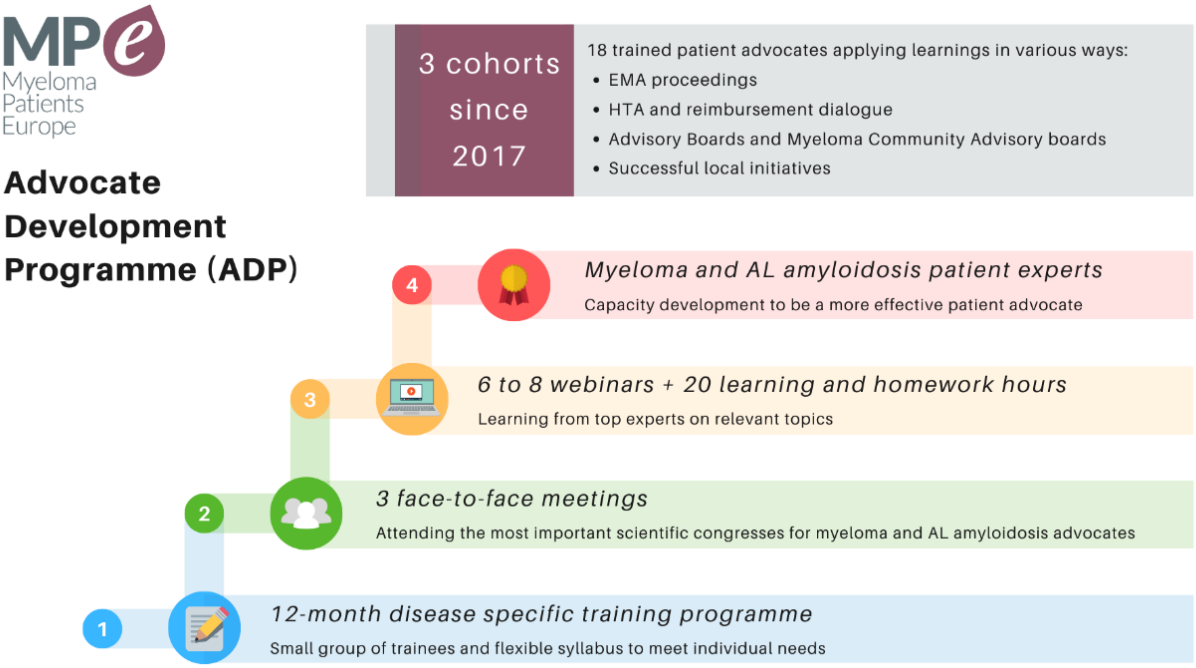
- Webinar 1 – 2019 programme kick-off
- Webinar 2 – Myeloma and AL amyloidosis basics
- Webinar 3 – The importance of quality of life
- Webinar 4 - Clinical trial protocols and study endpoints in myeloma and AL amyloidosis
- Webinar 5 – Reimbursement and market access
- Webinar 6 – Basic mechanisms of drug pricing
- Webinar 7 – ASH highlights
- Webinar 8 – Patient advocacy in action
- Recap of ADP and final debrief

Face to Face Programming

- EHA24 Face to Face
  - Welcome to the first Face to Face and EHA24
  - How drug development works
  - Key elements of a clinical trial protocol
  - Drug development and current investigational drugs in myeloma and AL amyloidosis

- Myeloma and AL amyloidosis pipeline and EHA highlights
- Statistics and why they matter to advocacy
- Reading scientific papers and posters, myeloma poster session walkthrough

- ESMO 2019 Face to Face
  - Welcome to ESMO
  - Introduction to regulatory assessment and medicines licensing
  - Evidence-based patient advocacy
- MPE Annual Meeting Face to Face
  - The big picture of patient advocacy



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. If successful, they will receive 3000 EUR in addition to optional one-to-one tutorials to help them implement and develop the activities included in their application.

In 2019, ten organisations received a scholarship within this programme:

- Amyloidosis Israel (IL)
- HEMA (MK)
- Romania SOS Myeloma (RO)
- Association for Patients with Haematologic Malignacies (SK)
- AMILO (ES)
- Swedish Blood Cancer Association (SE)
- Slovenian Lymphoma and Leukaemia Assoc, L&L (SI)
- Armenian Haematology Association (AM)
- Multiples Myelom Selbsthilfe (AT)
- Myelom und Lymphomhilfe (AT)

All the projects have been a massive success, 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 2019 can be found here: www.mpeurope.org/what-we-do/programmes/mpe-scholarship-capacity-building-programme

3.4 MPE Annual General Meeting (AGM)

Once a year MPE holds its Annual General Meeting (AGM) that includes the Annual Masterclass and the AGM General Assembly. 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, run by the most important European myeloma specialists, and advocacy sessions. The AGM also includes the AGM General Assembly, only with members, and a sponsor meeting with MPE's current and potential sponsors, to discuss present and future MPE projects and to identify potential areas of collaboration.

The MPE AGM 2019 took place in Munich, Germany, from 15 to 17 March at the Courtyard Munich City East. A specific website informing members and relevant stakeholders about the event and all logistics related, was launched half a year in advance: https://www.mpeurope.org/what-we-do/agm2019.

The meeting was attended by a total of 72 people:

- MPE Board and staff: 14
- MPE members: 35 people (23 groups)
- Guests: 7
- External Speakers: 7
- Pharma: 9

As part of MPE's educational aim, five videos were filmed with the main speakers to summarise the most important talks given at the MPE AGM. Along with the video gallery, a photo gallery

was also published on the MPE website: https://www.mpeurope.org/what-we-do/programmes/annual-masterclass-and-annual-general-meeting/annual-masterclass-video-gallery

3.5 European Commission projects: Horizon 2020 and IMI

MPE is involved in four European Commission project consortia.

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 seven 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.

The third and fourth are large-scale European Innovative Medicines Initiative (IMI) projects called HARMONY (2017-2021) and SISAQOL (four-year projects; still in final stage; kick-off planned in 2020).

3.5.1 Horizon 2020: MMPredict

Horizon2020 MMpredict, a 42-month project, approved by the European Commission, that started in November 2016 and will run until 2020. The European Commission has supported the whole project with 3,755,802 euros 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 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 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 were published in 2019 in a poster presented at the European Hematology Association (EHA) Annual Meeting along with Annemieke Van Dongen (Erasmus School of Health Policy & Management, section Health Technology Assessment, Erasmus University Rotterdam the Erasmus University Rotterdam) as the main author. Kate Morgan, Head of Policy and Access at MPE, and Ana Vallejo, Communications Manager at MPE, were also authors of this poster.

MPE would like to thank all MPE members that participated actively in this survey for their help in the review and dissemination of this activity.

Additionally, MPE has worked to inform the patient community about the progress made within this project. A wide range of communications about MMPredict were created, including filming with Annemieke Van Dongen on a range of studies presented during EHA 2019. A webinar about personalised medicine was also held on 25 June 2019.

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.

In 2020 the clinical trial will open in four cancer centres in Europe – Würzburg, Pamplona, Milan and Lille. It will only be open to around 20-30 myeloma patients, as it is an early phase clinical

trial. 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 Annual General Meeting (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.

Additionally, MPE developed in 2019 the following actions to disseminate the work done within this project:

- A talk on CAR-T cells was included in the MPE Annual General Meeting held in Munich (see point 1.4 MPE Annual General Meeting).
- The video "The role of CAR-T cells" was filmed and disseminated as a summary of this talk given by Dr. Michael Hudecek, University of Wuerzburg, Germany.
- Webinar on CAR-T treatment in myeloma. Webinar held on May 9th, 2019 to provide an overview of CAR-T cell treatment in myeloma. The talk was given by Dr Matteo Carrabba, Haematology and Bone Marrow Transplantation Unit, San Raffaele Scientific Institute, Milan (Italy).
- Video filmed during the American Society of Hematology (ASH) Annual Meeting, held in Orlando, Florida, USA. Dr Mohamad Mohty, Head of Hematology and cellular therapy department, Saint-Antoine Hospital and University Pierre & Marie Curie, Paris, France, summarised for Myeloma Patients Europe (MPE) the most important CAR-T cells updates in myeloma at ASH 2019.

More information about this project can be found also in the following links:

- https://www.mpeurope.org/what-we-do/projects/european-commission-projects-horizon-2020/caramba
- https://www.caramba-cart.eu

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 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.

3.5.4 Innovative Medicines Initiative (IMI) SISAQoL

The fourth large-scale European project is the Innovative Medicines Initiative (IMI) SISAQOL project which is still under revision and will, if accepted, prospectively run from 2020 until 2024.

Patient-reported outcomes (PROs) that assess symptoms, functioning and other health-related quality of life (HRQOL) issues are now recognised as important endpoints in the risk/benefit assessment of new cancer therapies. Although there is increased collection of PRO data in cancer clinical trials, no agreed international standards exist on the design, analysis or interpretation of these data.

One of the main challenges in developing international standards of PRO analysis is the many possible ways of designing, analysing and interpreting PRO endpoints. Individual researchers and organisations have their own set of procedures on how to analyse and interpret PRO endpoints. The diverse ways of analysing and interpreting PRO endpoints can result in conflicting and confusing conclusions being drawn from the same trial. Moreover, some commonly employed practices may be less suitable than others. The varying ways of analysing and interpreting PRO endpoints can potentially lead to erroneous and inconsistent decisions from different stakeholders, which may adversely impact patient care and outcomes.

Although recommendations on PRO design, analysis and interpretation have been published before, these were done from the point of view of individual PRO/QOL researchers, statisticians or specific academic groups or organisations; and did not take into account the needs of various decision-makers (regulators, HTA payers, clinicians and patient representatives). The joint collaboration of stakeholders and experts is needed to increase buy-in and implementation of these recommendations.

To address this need, SISAQOL proposes assembling key international stakeholders and methodological experts with a shared goal of developing a set of consensus recommendations

for PRO design, analysis and interpretation for cancer clinical trials. Specific objectives of the Consortium are:

- To achieve international consensus, across stakeholders, on the optimal use of HRQOL and PRO data in cancer clinical trials;
- To improve the quality of statistical analysis of HRQOL and PRO data in cancer clinical trials;
- To improve the standards of reporting of HRQOL and PRO data, and as such the interpretability of the data. It is hoped that this will result in more reliable interpretation, and ultimately faster dissemination, of HRQOL and PRO findings, as well as cross-referencing within and between different cancer settings, whenever this is deemed feasible.

This is a project that has not yet started. A multi-stakeholder consortium has been established by EORTC to put together a submission to the IMI for project funding.

Myeloma Patients Europe, on behalf of WECAN, has worked on the submission alongside a range of other stakeholders. Under the submission (if funding is approved), MPE would co-ordinate patient involvement in the project and co-ordinate work package 8 on communication and dissemination.

The submission has been reviewed and accepted for the second round. This was the only project to be accepted as part of this round of IMI calls, which is an achievement to highlight.

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 23 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 23 umbrella cancer patient organisations:

- Acute Leukemia Advocates Network (ALAN)

- Childhood Cancer International (CCI)
- CML Advocates Network
- Digestive Cancers Europe
- EuropaDonna
- EuropaUomo
- European Men's Health Forum
- European Organisation for Rare Diseases (EURORDIS)
- European Waldenström's Macroglobulinemia Network (EWMNetwork)
- International Brain Tumour Alliance (IBTA)
- International Kidney Cancer Coalition (IKCC)
- International MDS Alliance
- International Neuroendocrine Cancer Alliance (INCA)
- Lung Cancer Europe (LuCE)
- Lymphoma Coalition Europe
- Melanoma Patient Network Europe
- MPN Advocates Network
- Myeloma Patients Europe (MPE)
- Pancreatic Cancer Europe Network
- Sarcoma Patients Euronet (SPAEN)
- Thyroid Cancer Alliance (TCA)
- World Bladder Cancer Patient Coalition
- 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 at the end of 2018 and they were shared with MPE members, pharmaceutical companies, WECAN members and patient advocacy groups. During 2019 all contract templates were finalised. The Advisory Board Reference Agreement was published and the other three contracts templates are under review to be published in 2020. Find more information about this project in the link below:

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

3.7 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 holistically, in order to use 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 meetings will be used for training and capacity building of the advocates.

MPE executed three sittings of the Myeloma-CAB in 2019. The first took place from 11 to 13 May 2019 in Warsaw; the second from 5 to 7 October 2019 in Amsterdam and the last one from 16 to 18 November 2019 in Munich. In total six companies were involved in this programme.

3.8 Amgen Patient Preference study

During 2019 MPE and Amgen developed a survey for European myeloma patients to understand their information needs. The survey was run in 11 European countries in 2019 and will be run in one more country at the beginning of 2020, with the aim of gathering 1,000 responses across Europe. In 2019, MPE along with its members gathered 954 responses in 11 countries and is expected to meet the goal of 1,000 responses. The survey was translated into all relevant languages. The survey asks patients a range of questions designed to better understand myeloma patient information needs and preferences, particularly focusing on the types of information that are valued by patients to make informed treatment decisions.

It also aims to understand:

- Patient involvement in their last treatment decision and the factors that influenced this
- How information is associated with confidence in treatment decision-making
- Communication between healthcare professionals and patients on treatment decisions

The results of the survey will provide valuable insight into the needs of patients and will be used to inform the information provision strategies of a wide range of stakeholders, including pharmaceutical companies and patient groups. MPE, and its members, will also use the results of the survey to inform their advocacy and campaigning strategies which aim to improve the experience of patients across Europe.

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. The following materials are available in the MPE website:

- **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

- **Q&A on Health Technology Assessment (HTA).** This is the first of a series of Q&As that will continue in 2020 that analyses relevant topics for patient advocates in a patient-friendly style. This factsheets can be found in the following link along with a downloadable version of the publication: https://www.mpeurope.org/what-we-do/publications/qas/health-technology-assessment-hta

- **Webinars.** During 2019 the following webinars were held:

  - ASH highlights. Webinar held on February 11th, 2019 to review the most recent myeloma findings presented at the annual meeting of the American Society of Hematology (ASH) that took place in San Diego (California, United States) in December 2018. The talk was given by Dr María Victoria Mateos, Associate Professor of Haematology and Consultant Physician of the Haematology Department at the University Hospital of Salamanca, Spain. 262 views.
  - Webinar on CAR-T treatment in myeloma. Webinar held on May 9th, 2019 to provide an overview of CAR-T cell treatment in myeloma. The talk was given by Dr Matteo Carrabba, Haematology and Bone Marrow Transplantation Unit, San Raffaele Scientific Institute, Milan (Italy). 245 views.
  - Webinar on personalised medicines in myeloma. This webinar was held on June 25th, 2019. The talk was given by Dr Annemiek Broijl, Department of Hematology, Erasmus MC Cancer Institute, Rotterdam, Netherlands. 136 views.

- **Educational clips.** Filmed interviews and videos with top experts about a range of topics:

  - Myeloma treatment updates. Summary of the talk given by Prof. Heinz Ludwig, Wilhelminen Cancer Research Institute, Austria, about myeloma treatment updates during the MPE Annual General Meeting (AGM) held in Munich, Germany in March 2019. 173 views.

- Surrogate end-points in myeloma and AL amyloidosis. Summary of the talk given by Dr Moshe Gatt, Hadassah Hebrew University Medical Center, Jerusalem (Israel), on surrogate end-points and biomarkers in myeloma and AL amyloidosis during the MPE Annual General Meeting 2019 held in Munich, Germany. 118 views.
- Measuring and utilising quality of life. Summary of the talk given by Dr Jayne Galinsky, Health Services Research Manager at Myeloma UK, during the MPE Annual General Meeting (AGM) held in Munich, Germany in March 2019. 105 views.
- AL Amyloidosis. Summary of the Plenary Workshop on AL Amyloidosis given by Dr Moshe Gatt, Hadassah Hebrew University Medical Center, Jerusalem (Israel) and Daniel Drimer, president of Amyloidosis Israel during the MPE Annual General Meeting 2019, in Munich, Germany. 87 views.
- The role of CAR-T cells. Summary of the talk given by Dr Michael Hudecek, University of Wuerzburg, Germany, about the role of CAR-T cells in myeloma and AL amyloidosis at the MPE Annual General Meeting 2019 held in Munich, Germany. 121 views.
- EHA 2019 myeloma highlights. Interview filmed with Dr Laurent Garderet, Department of Hematology at Pitié Salpêtrière Hospital in Paris, France, to summarise the most important updates presented in the European Hematology Association (EHA) Annual Meeting held in Amsterdam, The Netherlands, from 13 to 16 June 2019. 395 views.
- Quality of life of European Myeloma Patients. Summary of the poster on quality of life of European Myeloma Patients presented at the 24th European Hematology Association (EHA) Congress which was held in Amsterdam, The Netherlands. The interview was filmed with Annemieke Van Dongen, Erasmus School of Health Policy and Management, Erasmus University Rotterdam and the main author. Two MPE staff members (Kate Morgan and Ana Vallejo) were also authors of this research developed under the Horizon 2020 project, MMPredict. 61 views.
- Cost-effectiveness of risk stratified treatment in myeloma transplant eligible patients. Summary of the poster on cost-effectiveness of risk stratified treatment in myeloma transplant eligible patients presented at the 24th European Hematology Association (EHA) Congress which was held in Amsterdam, The Netherlands. The interview was filmed with Annemieke Van Dongen, Erasmus School of Health Policy and Management, Erasmus University Rotterdam and the main author of this research developed under the Horizon 2020 project, MMPredict. 38 views.
- ESMO 2019 myeloma highlights. Dr Prof. Jesus F. San-Miguel, Director of Clinical & Translational Medicine Universidad de Navarra, Spain, summarises the main updates presented in the session “Defining optimal response in myeloma” at European Society for Medical Oncology Congress (ESMO) Annual Congress 2019. 121 views.

- ASH 2019 educational clips. MPE developed a special informative coverage at American Society of Hematology (ASH) Annual Meeting. Not only some of the videos were filmed, edited and disseminated onsite but also, for the first time, MPE filmed in different languages. Find below the educational clips filmed. In December 2019 alone, all ASH videos had 1,553 views across Europe
  - ASH 2019 preview. Video filmed with Dr Katja Weisel, University Medical Center Hamburg-Eppendorf in Germany to summarise the most important topics presented during the American Society of Hematology (ASH) Annual Meeting 2019. 77 views.
  - ASH 2019 CAR-T cell therapy in myeloma and CARTITUDE clinical trial. CAR-T cell therapy is one of the most promising treatments in a number of types of cancer. In myeloma, very promising results were presented during the American Society of Hematology (ASH) Annual Meeting. Dr Mohamad Mohty, Head of Hematology and and cellular therapy department, Saint-Antoine Hospital and University Pierre & Marie Curie, Paris, France, summarised for Myeloma Patients Europe (MPE) the most important CAR-T cells updates in myeloma. 182 views.
  - Positive results of selinexor in relapsed and refractory myeloma patients presented at ASH 2019. During the American Society of Hematology (ASH) Annual Meeting, new results on selinexor were presented. Dr Cristina Gasparetto, Duke Cancer Institute, North Carolina, USA, explains for Myeloma Patients Europe (MPE) the main results of selinexor. 52 views.
  - Results of the clinical trial CANDOR at ASH 2019. The big "late-breaking" news at the American Society of Hematology Annual Meeting were the results of the phase 3 CANDOR trial. Dr Katja Weisel, University Medical Center Hamburg-Eppendorf in Germany, explains for Myeloma Patients Europe (MPE) in this video the main results of this clinical trial. 283 views.
  - Dreamm-3 and belantamab mafodotin, a new myeloma drug. Dr Katja Weisel, University Medical Center Hamburg-Eppendorf in Germany explains for MPE what Dreamm-3 is and what we need to know about belantamab mafodotin. 99 views.
  - ASH 2019 - AL amyloidosis highlights. Interview with Dr Moshe Gatt (Israel) on AL amyloidosis highlights presented in the American Society of Hematology (ASH) Annual Meeting held from 7 to 10 December in Orlando, Florida, USA. 80 views.
  - ASH 2019 Myeloma updates. Dr Faith Davies, NYU Langone Hospital, New York, USA, summarises in this video the most important myeloma updates presented at the American Society of Hematology (ASH) Annual Meeting. 109 views.
  - ASH 2019 updates on Minimal Residual Disease (MRD). Minimal Residual Disease (MRD) is one of the big topics in myeloma. Different techniques

to measure MRD and new data about its role in predicting the risk of relapse are some of the topics discussed during the American Society of Hematology (ASH) Annual Meeting. In this video Dr Francesca Gay, University of Torino, Italy, explains for Myeloma Patients Europe (MPE) what was presented at ASH 2019 regarding MRD and the unanswered questions about its role as a surrogate endpoint. 47 views.

- ASH 2019 - Real World Evidence. Dr Faith Davies, NYU Langone Hospital, New York, USA, summarises in this video the most important myeloma updates presented at the American Society of Hematology (ASH) Annual Meeting regarding Real World Evidence. 35 views.
- Bispecific antibodies at ASH 2019. Along with CAR-T cell treatment, bispecific antibodies has been one of the most important topics discussed at the American Society (ASH) Annual Meeting. This family of drugs has shown very promising results in early phases of clinical trials in heavily pre-treated myeloma patients. Dr Mohamad Mohty, Head of Haematology and Cellular Therapy Department, Saint-Antoine Hospital and University Pierre & Marie Curie, Paris, France, summarises for Myeloma Patients Europe (MPE) the most important updates on bispecific antibodies at ASH 2019. 42 views.
- ASH 2019 Pre-clinical updates. Dr Faith Davies, NYU Langone Hospital, New York, USA, explains in this video the pre-clinical myeloma updates presented at the American Society of Hematology (ASH) Annual Meeting 2019 and why these are important to patients. 48 views.

Videos delivered in other languages:

- Myeloma and AL amyloidosis highlights. Video filmed in **Hebrew** with Dr Moshe Gatt, Hadassah Hebrew University Medical Center, Jerusalem (Israel). 261 views.
- Results of the clinical trial CANDOR at ASH 2019. Video filmed in **German** with Dr Katja Weisel, University Medical Center Hamburg-Eppendorf in Germany, to explain the big "late-breaking" news at the American Society of Hematology Annual Meeting, the phase 3 CANDOR trial. 166 views.
- Dreamm-3 and belantamab mafodotin, a new myeloma drug. Video filmed in **German** with Dr Katja Weisel, University Medical Center Hamburg-Eppendorf in Germany, to explain what Dreamm-3 is and what we need to know about belantamab mafodotin. 58 views.
- ASH 2019 updates on Minimal Residual Disease (MRD). Video filmed in Italian with Dr Francesca Gay, University of Torino, Italy, to explain what was presented at ASH 2019 regarding MRD and the unanswered questions about its role as a surrogate endpoint. 14 views.

5 Workgroups and taskforces

5.1 AL Amyloidosis Taskforce

The Annual General Meeting held in April 2018 agreed to 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 in countries where there is no patient organisation).

During 2019 a number of actions were developed by this taskforce to strengthen and improve activities focused on AL amyloidosis, not only by MPE but also but its members. This work included a survey of membership to understand their involvement with the AL amyloidosis community and how MPE can support the community. It also included a number of meetings with the Amyloidosis Alliance to discuss how to strengthen collaboration and partnership across the groups.

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.

6.1.1 Stakeholder meetings

MPE attends numerous meetings with relevant stakeholders, such as industry, clinicians, medical societies, regulators, HTA, other patient communities. Some of them are listed below:

- **CEE Celgene Summit:** Kate Morgan and Ana Vallejo attended this meeting. Kate gave a presentsation on Capacity Building & Best Practice in Access and Ana conducted a workshop about social media management and how to strengthen social media strategy and use.
- **Karyopharm advisory board:** Kate attended a meeting on selinexor in London to discuss their market access plans for the drug in the UK and Europe.
- **Takeda advisory board:** Kate and Ananda attended this advisory board in Munich to discuss unmet need in relapsed myeloma.
- **Janssen CAR-T advisory board:** Kate, Hans and Ananda attended this advisory board in Paris in February to input into their clinical development programme for CAR-T.
- **Takeda Noona steering group:** Ananda attended this meeting at EHA and remains on the steering group for this.
- **WECAN meetings:** The Workgroup of European Cancer Patient Advocacy Networks (WECAN) is an informal network of 23 cancer patient umbrella organisations in Europe, which has the aim to join forces and avoid duplication of efforts in areas and initiatives

that are cross-disease. The first face to face WECAN meeting of 2019 took place in January in Milan. This was mainly to decide an educational strategy for the cancer patient community (WECAN Academy), and to finalise the programme of the WECAN Masterclass and SmartStart.

From 6-9 July 2019, WECAN held the first WECAN Academy in Frankfurt, Germany, aiming to address common educational needs of the patient community. During the WECAN Academy, more than 100 European patient advocates were trained in advocacy tools and skills and on topics such as healthcare systems, policy and access, research and data. The WECAN Academy combined two key capacity building programmes for patient advocates:

  - WECAN Smart Start: Starting and Building a National Non-Profit Patient Group focusing on training for "beginners" in patient advocacy
  - WECAN Masterclass on Patient Advocacy focusing on training for experienced patient advocacy leaders

Ananda Plate, CEO of MPE and WECAN chair at the time, had a relevant role as a coordinator and speaker of the WECAN Academy. Several MPE members attended this event.

Two additional WECAN meetings took place in September and October 2019.

- **CDDF Multi-Stakeholder Workshop:** The Cancer Drug Development Forum multi-stakeholder workshop on involving patients in oncology drug development took place in Amsterdam, the Netherlands in June 2019. Nicole Wicki attended on behalf of MPE and presented in a session titled: "patient involvement in preference studies: a case study in multiple myeloma".
- **Takeda INSIGHT steering committee meeting:** Nicole attended the investigator meeting on behalf of MPE, held 12 July 2019 in Montreal. The meeting provided an update on the INSIGHT study progress, including review of data, and discussion of publication plans.
- **Janssen LocoMMotion study investigator meeting:** The LocoMMotion study investigator meeting took place in Amsterdam on 25 September 2019. Nicole attended on behalf of MPE and presented on patient advocacy perspectives for communication to patients about clinical trials and observational studies, how to use patient recruitment materials and to present the LocoMMotion patient information booklet and study discussion guide for investigators, for which MPE previously provided input to content.
- **Janssen advisory board on daratumumab:** Kate attended this advisory board, which focused on data around daratumumab and its subcutaneous formulation.
- **Amgen PALS:** Kate and Ananda attended this advisory board in Munich, which focused on access challenges and included presentations on BITE drugs and a tour of the BITE labs within Amgen Germany.

6.1.2 Congresses and scientific meetings

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 2019, MPE attended the following conferences:

- **ASCO 2019:** The Annual Congress of the American Society of Clinical Oncology, 55th ASCO Annual Meeting, was held in Chicago from 31 May until 4 June. 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 this important meeting to gather the most important updates for the myeloma community.
- **EHA 2019:** The European Hematology Association (EHA) Annual Congress was held in Amsterdam (The Netherlands) from 13 to 16 June. MPE also exhibited in the Patient Advocacy Hub and attended the most relevant myeloma sessions in order to inform MPE members about the latest developments in myeloma. Additionally, the MPE CEO, Ananda Plate, talked about CART-cell treatment and shared the MPE experience working in the H2020 project CARAMBA. Patient Advocacy Programme Manager, Nicole Wicki, chaired a session about access to innovation and affordability.
  In the lead up to EHA 2019, MPE held the first face-to-face meeting of the Advocate Development Programme, a three-day session covered a wide-range of topics relating to clinical development of new medicines and pipeline drugs in myeloma and AL amyloidosis.
- **HTAi 2019:** The Health Technology Assessment International (HTAi) conference took place in Cologne in June 2019. HTAi is a professional organisation promoting information sharing across the key stakeholders in HTA. During the congress, MPE Policy Manager Kate Morgan presented at, and participated in, a panel on patient involvement in health technology assessment beyond 2020.
- **IMW:** the 17th International Myeloma Workshop (IMW) will be held in Boston, USA, from 12 to 15 September. MPE Policy and Public Affairs Manager, Kate Morgan attended this scientific meeting to gather information about the latest updates in myeloma and to have a number of meetings with sponsors and stakeholders.
- **ESMO 2019:** The European Society for Medical Oncology (ESMO) Annual Congress held from 27 September to 1 October in Barcelona, Spain. Kate Morgan and Ananda Plate contributed as speakers and chairs of the Patient Advocacy track.
- **ISPOR 2019:** ISPOR 2019 was held in Copenhagen, November 2019. Kate attended the patient representative round table on quality of life and went to a wide range of presentations on reimbursement, health economics, patient preferences and health technology assessment.

- **ASH 2019:** The American Society of Haematology Annual Meeting held in Orlando, Floorida, USA, December 2019. Kate Morgan, Ananda Plate and Ana Vallejo attended the meeting, filming interviews with key doctors, listening to scientific presentation and meeting with pharma partners.

6.2 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 in 2017 and since them 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 2019, 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 2019, MPE has worked 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. This will be launched in 2020 and MPE will continue to ensure that the information is up-to-date.

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.

Workgroup of European Cancer Patient Advocacy Networks (WECAN):

WECAN is a network of 23 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 2019, MPE has had a very active role in WECAN, leading on a project entitled 'Reasonable agreements between patient advocacy and the pharmaceutical industry' and being actively involved in the most relevant WECAN activity in 2019: the WECAN Academy.

MPE's CEO Ananda Plate was the chair of this platform from September 2017 to October 2019 and has been vice-chair since then.

7 Acknowledgements

The activities and materials developed throughout 2019 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, since 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.

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