

Policy Issues Affecting Patient Access to Approved Therapies in Myeloma

A JOINT WORKSHOP BY
EAFPA & MIJELOM CRO
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MijelomCRO

Udruga za podršku oboljelima od multiplog mijeloma (plazmocitoma)



This workshop brought together advocates to look at policy issues affecting patient access to approved myeloma therapies. The workshop included panels of health care professionals and patient advocates as well as presentations on effective advocacy and setting up regional patient networks.

Meeting Objectives



To review policy issues affecting patient access to approved therapies in myeloma



To discuss how to elevate the discussion around blood cancer



To determine ways to overcome the challenges that are being faced by myeloma patients.

Report of the Balkans Myeloma Study Group (BMSG) Meeting

The meeting opened with an overview of the recent Balkans Myeloma Study Group (BMSG) meeting in Athens, by participants who had attended. The group was created in 2017 by hematologists to represent the collective interests of the smaller Balkan countries, with the aim of attracting clinical trials to the region.

The BMSG is currently trying to establish a registry of all myeloma patients in the region to increase interest from pharmaceutical companies and policymakers. It is estimated there are around 1500 newly diagnosed patients; with Turkey the number amounts to 3500 annually. Industry representatives highlighted that companies want to undertake studies and get drugs to patients as soon as possible, but they are incentivised to start in larger markets. Including regulatory bodies as a part of the BMSG future meetings should be beneficial so they can learn the challenges and perspectives of each country.





Key Policy Issues Affecting Patient Access

Multiple myeloma is a rare type of cancer, representing 1% of all cancer diagnoses and approximately 10% of all hematological malignancies.¹ More than 42 thousand new cases of myeloma are diagnosed each year across Europe, and 26 280 die each year. The highest incidence rate for myeloma is in Norway and the lowest rates in Bulgaria, Albania and Bosnia-Herzegovina. We can possibly explain this by low rate of diagnosis, no diagnosis or no registers in those countries.²

New drugs and treatment have been developed, with a significant improvement in progression free survival and overall survival, as well as an improvement in the quality of life of patients. However the patients across the Balkan region have had limited and varied access to the first generation of new drugs such as thalidomide, bortezomib and lenalidomide, and very limited to the second generation of new drugs.

1. Multiple Myeloma: ESMO Clinical Practice Guidelines <https://www.esmo.org/Guidelines/Haematological-Malignancies/Multiple-Myeloma> accessed on 13/5/2019

2. European Cancer Information System (ECIS) <https://ecis.jrc.ec.europa.eu> accessed on 13/5/2019

Led by **Mira Armour** of Mijelom CRO, the health care professionals panel allowed clinicians to share their perspective on access to myeloma medicine and treatments in their respective countries. Their views brought to light the very different levels of availability and accessibility in the region.

Health Care Professionals Panel

There was broad agreement that finances are a major problem to access, leading to various hardships for patients and families, and having a profound effect on myeloma patients overall survival and quality of life.

Dr. Oliver Karanfilski said that doctors' primary duty is to ask for the best for patients. There is only one science of hematology which doctors study, and it is the same in every country. However, access to medicine is largely a financial issue and patients in for example Germany have better survival than patients in Macedonia. Whilst understanding that development and research of drugs is difficult and costly, he noted that the price should not depend on the quantity of drugs purchased, but instead be tied around the standard of living in a country. In Macedonia, it was reported that the list of drugs for reimbursement has not been reviewed for 14 years, with few

exceptions. From this year, there is a limited quantity of generic bortezomib available. However in relapse, where patients fail on bortezomib, doctors have nothing else to use.

In Croatia, **Dr. Sandra Kinda Bašić** noted that things are gradually improving, but progress is slow and finances remain an issue. First and second line treatments were given at a reasonable price, but a new generation of proteasome inhibitors are needed as well as monoclonal antibody. There are ongoing negotiations with reimbursement agencies, however these are difficult and slow, and the need for pharmaceutical companies to reduce their prices is great.

Dr. Jelena Bila reported similar issues around resources in Serbia. The situation is managed by the implementation of strict guidelines for personalised treatment, evidence-based. Currently, in Serbia bortezomib is used in the treatment of relapse and in the first line treatment in high-risk patients. Standard risk patients are treated in the first line with thalidomide combinations. For this reason they use FISH. However, this has created the problem of treatment at relapse. Lenalidomide for patients in relapse is available since 2017 for those who cannot be treated with bortezomib and thalidomide. In this view, there is

a need for control risk stratification in relapse and identification of high-risk patients that would benefit the most from the new generation of PIs, IMiDs and MoAbs, which are not available yet. Limited resources require the need for having very strict guidelines.

Dr. Matjaž Sever stated that in Slovenia all three generations of inhibitors are available, as well as immunomodulators and monoclonal antibodies to some extent, but they are trying to determine the best combination for patients considering clinical data and cost. They can get drugs through studies, but this is rare. Compassionate use programmes are also a way to gain access to drugs

before they are available. In some cases doctors apply for a particular drug for a named patient and this is usually approved. Advocates cooperate with doctors and regulatory bodies to ensure that patients can receive new treatments.

The group discussed buying medicines in bulk – similar approach was adopted in Benelux for some drugs. The panel discussed whether a system could be established similar to that in Finland and Norway, considering the fact that some countries are in EU and some are not. This would require political will from all countries. The panel thought this very unlikely, as countries are at different level



of economic development. It was discussed whether regional guidelines are available or if countries are using European guidelines.

Patient Advocates Panel

A patient advocates panel followed, with a discussion among patient representatives about the challenges and lack of equity each represented country faces. The main concern was that patients do not have access to new drugs in all countries, or they get the drugs only when they become generic. There is a delay of over ten years in some countries or no access even now.

Panelists overwhelmingly agreed on the lack of funds as the main barrier. In some health care systems patients have accessed medicine through compassionate use programmes. However, there are major issues and concerns. Examples from Kosovo and Bosnia have shown that patients in majority of cases need to make private arrangements for care such as having blood transfusions, buying medicine and finding diagnostic facilities.

Many patients are going to Turkey, Croatia or any other country where

they have contacts, to buy drugs. There is no education on how to use the drugs, as pharmaceutical companies do not have representatives in some of the countries.

Panelists also discussed whether there should be a role for regulatory agencies to check the price of drugs and to initiate getting the generic drugs on the reimbursement lists. When the drug becomes generic pharmaceutical companies do not what to spend money in preparing documentation for registering the generic drug in the country.

Availability of standard cancer drugs for myeloma treatment and diagnostics seem to decrease as you move south-east of Europe. Capacity for transplantation is also limited and there are waiting lists. Funding for support groups is also a barrier to developing organisations which can make an impact on policy makers.

Necessity of informing policy makers about patient experiences has been highlighted as urgent.

Also invitation to share resources – materials, papers, information – was passed, as these are scarce.

Policy Advocacy Skills Training



Brian Kennedy from the Global Alliance for Patient Access led the group in a session about policy advocacy. He noted the value of bringing together patients and physicians to inform policymakers, raise awareness about disease burden and help further research.

The Keys to Advocacy

Create a Narrative. Patient organisations and clinician groups should not underestimate their collective expertise when talking about myeloma and related conditions. They should use poignant stories and pictures to convey their message; leverage media and social media to

highlight the problem, not the solution. Sometimes there are competing narratives, so it's important to help whoever you are advocating see your version of reality. Once they are convinced, they are more likely to act.

Build Relationships. It's important to start by identifying decision makers (i.e. elected officials, ministers, health care providers) and influencers (i.e. media, public opinion, medical societies). The goal is to make your cause their cause by building trust. We are more likely to trust information from people we know and therefore need to start developing relationships with the right decision makers – persistence is key.

It's important to set forth your narrative in a position paper. Request a meeting to explain your position and know your ask. Follow up by inviting the decision maker to address your organisation at a meeting or workshop where you can praise their support.

Importance of South East Europe Myeloma Community Working Together

Attendees agreed that they were experienced in understanding how networks work as there are already networks in the myeloma patient community including Myeloma Patients Europe. Discussion centered on why existing European networks aren't available to deliver; lack of a common agenda surfaced as one issue. The group discussed their specific needs and noted harmonising legislation is key.



Setting Up a Community Board Advisory Group

There are already some community boards in Europe and at the Pan European level and the group discussed how these can be used.

The group heard about a patient run community advisory board, which allows pharmaceutical companies and patient advocates to have a dialogue and enables capacity building. The agenda is set by the patient community yet they fight the perception that they will support only pharmaceutical interests.

All countries expressed an interest in working together, as decision makers in their countries are more likely to be receptive if calls are coming from a regional level. The scope of a collaboration was also discussed and it was agreed that it should focus broadly on hematological cancers. Robust plan would be needed to seek funding.

The group agreed to reconvene in 2019. It was suggested - to maximise attendance from clinicians - the next meeting could be attached to one of the regional meetings that clinicians are having, such as national hematologists' meetings, or EHA meeting in June.

Agora



Platform of Organisations
of people with Rheumatic Diseases
in Southern Europe

Case Study: Success of Agora, a Regional Patient Network

Attendees heard from **Elena Tsigki**, who has supported the establishment of Agora, the rheumatic and musculoskeletal disease group in southern Europe. Agora was established in 2011 after three people who met at EULAR decided there was a need to promote rights of patients who have rheumatic diseases. Despite its original focus in southern Europe, the Agora network has opened membership to eastern Europe and Israel, and now represents 19 national and regional patient organisations in over 12 countries. It maintains a relationship with other EU and global umbrella organisations.

Agora's aims are to create better conditions for people with rheumatic diseases, ensure higher quality of life for patients and promote supportive attitudes to enable patients to live independently and participate fully in society.

Agora is engaged in a wide variety of activities including hosting an annual

conference and workshops and training sessions. It supports campaigns, surveys and data collection, and collaborates on many other projects. The organisation translates educational materials so patients in each country have access to the information. Among Agora's greatest successes was in Romania in 2016/17 when it wrote a letter and created a petition against health insurer interference with the medical act, which made it difficult for rheumatoid arthritis patients to access care.

Before creating a new network, one must determine if there is a need for the organisation. Consider if a similar organisation exists already, and if creating a second network could be redundant. Consider the socio-economic and cultural context of the patients in specific regions. Once it's agreed a new network is necessary, identify the unique selling point. Then start sharing patients' experiences and common struggles. It's also necessary to identify resources, strategize advocacy efforts, fundraise, and develop communication plans.

Identifying Regional Challenges, Successes & Goals

To help focus future efforts, participants outlined challenges and successes, as well as goals of their joint activity in months and years to come. In a nutshell, it was agreed that Slovenia has gone furthest in building functional and widely available health care for myeloma patients. Moving south-east, developments and standards are seen to be less comprehensive.

Vision. Participants' shared vision is a **longer and better life for myeloma patients** - a vision that will guide their efforts and future collaboration.

Goals. To attract clinical studies to the Balkan region, and improve the availability of latest-generation medication to myeloma patients. The latter will be achieved largely through adding myeloma drugs on reimbursement lists, which requires coordinated and persistent efforts of governments, healthcare professionals, patient groups and pharmaceutical companies.

Challenges are multiple and diverse, ranging from access to basic care in some areas, to complex advocacy and procedural issues in others. Challenges can be tied to particular economical, social and political circumstances in the Balkan region.

- Lack of access to basic care in some areas. There are examples in Kosovo and Bosnia of patients making private arrangements for care such as having blood transfusions, buying medicine and finding diagnostic facilities.
- Get new generation of myeloma drugs on reimbursement lists. Remains a challenge throughout the Balkan region, although to varied degrees.
- Funding. It was identified as one of the key challenges in the Balkan region, impacting activity and progress on almost all levels.
- Weak patient advocacy. In some areas there's work to do on

strengthening patient organizations and making patients voice heard, which is seen as an important lever in improving patient rights. Organisations are in need of consistent sources of funding.

- Equal treatment and access. Outside capitals and major centers, some local hospitals in Serbia and Bosnia and Herzegovina lack resources and sometimes expertise. Treatment protocols can also differ. Croatian advocates noted in particular that they are pushing for standardization of myeloma treatment across the country. In Macedonia, there is only one hospital for hematological conditions, serving the entire country.
- Late diagnosis. This remains an issue across the region. Myeloma symptoms are not specific, and many general practitioners have never encountered this rare type of cancer. This can cause myeloma diagnosis to come rather late, which can have negative impact on overall survival and quality of life. Awareness campaigns and education are needed to help improve this.
- Pre- and post-treatment care and quality of life. This aspect of myeloma patients care is still largely underdeveloped. In

Slovenia, advocates are working towards making comprehensive rehabilitation available for all cancer patients, before and after treatment. Also, they are trying to support myeloma patients to go back to work when possible.

Successes. Slovenia has three key advocacy groups which work together. They are preparing articles for daily newspapers, and have ran a big campaign, 80% funded by the Ministry of Health. In Croatia, bortezomib-based therapy is available for all patients, and there is good cooperation with HCPs. Publications, materials and support structures are available for myeloma patients and their families. In Serbia, myeloma patients got lenalidomide after 12 years of coordinated efforts of hematologists and patient advocates. This is seen as huge progress, given that only 2 types of medication were available before. Myeloma in BiH is a newly registered organisation; they had successful educational meetings with clinicians and directors of health care, and two TV appearances. In Macedonia, improvements were made in the availability of therapies and genetics-based diagnostic methods. A newly founded patient advocacy group in Kosovo is focusing on MDS, but also other types of blood cancer.

Organizations & Individuals Participating

Dr. Josip Batinić, University Hospital Zagreb, Croatia

Neil Betteridge, EAfPA

Mijelom u BiH, Bosnia and Herzegovina

<https://www.facebook.com/mijelomubih/>

Mijelom CRO, Croatia

mijelom.hr

Mijelom Srbija

www.mijelom.rs

HEMA Macedonia

<http://www.hema.org.mk>

Društvo bolnikov z limfomom (DBL) Slovenia

www.limfom.si

Slovensko Združenje Bolnikov Z Limfomom In Levkemijo L&L

www.limfom-levkemija.org

Cancer Support Community of Kosovo CSC

<https://csckosovo.com/en>

CONCLUSION

Participants agreed that the meeting was successful and felt positive about moving forward and more empowered to help patients and improve the health care situations in their countries and the region as a whole.



About the European Alliance for Patient Access

The European Alliance for Patient Access, a division of the Global Alliance for Patient Access, is a network of physicians and patients advocating for patients' access to approved medical therapies and appropriate clinical care.

GAfPA.org

MijelomCRO

Udruga za podršku oboljelima od multiplog mijeloma (plazmocitoma)

Mijelom CRO is a non-profit organisation founded in 2011 with the purpose of giving support to multiple myeloma patients, their families and carers in Croatia and in the region.

Mijelom.hr