European Perspectives on MDS Patient Management

MDS-RIGHT Multi-Stakeholder Meeting

3 May 2017, 16.00-18.00 hrs CET, Auditorium 3, Palacio de Congresos de Valencia, Valencia, Spain

Meeting Report

18 May 2017

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Executive Summary

The first MDS-RIGHT multi-stakeholder meeting was held on 3 May 2017 in Valencia, Spain in conjunction with the biannual MDS 2017 International Symposium. It gathered more than 70 participants representing a wide range of MDS stakeholders, including medical specialists and nurses caring for MDS patients, MDS patient advocates, medical researchers and data managers, healthcare authorities, regulators, HTA experts, and industry representatives.

The 2-hour meeting was organised to raise awareness and generate endorsement of accepted evidence-based recommendations for MDS patient management (MDS-RIGHT Task 6.3), to obtain insights on general MDS challenges and solutions across Europe, to stimulate stakeholder collaboration and to obtain feedback on the new MDS therapeutic algorithm interactive tool and the MDS-RIGHT/MDS-Europe website. The meeting included short presentations as well as panel and audience discussions on MDS-RIGHT, MDS patient management-related challenges and solutions and MDS patient management recommendations.

MDS is a relatively rare disease that may affect as many as 2 million (predominantly elderly) people across Europe. Given that the average survival of MDS patients is only about 3-4 years and the MDS disease burden keeps growing, all stakeholders agreed that new and better drugs and drug combinations are urgently needed and they expressed their willingness to increase international stakeholder collaboration with a view to generating and exchanging more MDS-related evidence, thus helping to accelerate the development of new MDS treatment options.

This includes access to state-of-the-art diagnostic tools (including next-generation sequencing/NGS, but also tools for upfront geriatric assessment), disease-specific Quality of Life (QoL) assessment tools, up-to-date guidance for taking the right treatment decisions upfront based on therapeutic recommendations that include all aspects of MDS patient management (as foreseen by the MDS-RIGHT therapeutic algorithm interactive tool), cross-border MDS patient support, large international clinical trials that fully reflect patient needs (as well as comorbidities such as cancer) and industry collaboration and support for academic clinical trials.

As even well designed MDS trials may not be able to answer all relevant questions, it was considered vital by all stakeholders to collect and analyse more robust real-world data (RWD) on all subtypes of MDS — as well as on comorbidities, outcomes, QoL and health economics — with the help of well-funded patient registries. In fact, according to the European Medicines Agency (EMA), RWD may become a requirement for marketing authorisation applicants or holders and therefore. the EUMDS Registry (on which the MDS-RIGHT study is based) and the MDS-RIGHT project are considered a “unique resource” for informing clinical, industry, regulatory and HTA decisions.

Meeting Report

Welcome and introduction

Co-chairs: Guillermo Sanz, haematology specialist - Hospital La Fe, Valencia, Spain and
Theo de Witte, MDS-RIGHT project coordinator – Radboud university medical center, Nijmegen, The Netherlands

The meeting co-chairs, **Prof. Guillermo Sanz** and **Prof. Theo de Witte**, welcomed the more than 70 participants to this first MDS-RIGHT multi-stakeholder meeting and highlighted the progress made over the last decade in the field of myelodysplastic syndromes (MDS). They also emphasised the importance of multi-stakeholder involvement and collaboration in the management of MDS, pointing out that the following stakeholder groups were represented at this meeting:

- Medical specialists caring for MDS patients
- Nurses/social workers caring for MDS patients
- MDS and blood disorder patient advocates
- Medical researchers, data managers and MDS co-operative study groups
- Healthcare authorities, regulators, HTA experts
- Pharmaceutical companies

The goals of this stakeholder meeting were summarised by Prof. de Witte as being:

- To raise awareness and generate endorsement of accepted evidence-based recommendations for MDS patient management (Task 6.3)
- To obtain insights on general MDS challenges and solutions across Europe
- To stimulate European MDS stakeholder information exchange and involvement
- To obtain stakeholder feedback on the MDS treatment algorithm interactive tool (TAIT)
- To gain advice on how best to further improve the MDS-RIGHT/MDS-Europe website

MDS-RIGHT– Providing the right care to the right MDS patient at the right time

Theo de Witte, MDS-RIGHT project co-ordinator - Radboudumc, Nijmegen, The Netherlands

Prof. Theo de Witte gave a general introduction on the MDS-RIGHT project, which is based on the collaborative, EU-funded EUMDS Registry (**www.eumds.org**) that was started in 2007/2008. The EUMDS Registry itself started as one of the projects of the MDS-related work package of the European LeukemiaNet (ELN, **www.leukemia-net.org**), an EU-funded initiative launched in 2004.

To date, the EUMDS Registry has collected prospective, observational data on more than 2,200 lower-risk MDS patients from 16 EU countries and Israel. The non-MDS reference population analysed in the MDS-RIGHT project is a cohort of over 14,000 individuals above 65 years of age from the northern part of the Netherlands that is being investigated in the LifeLines observational follow-up study (**www.LifeLines.nl**).

Prof. de Witte introduced the main goals of MDS-RIGHT (**www.mds-right.eu**), namely to assess (epi)genetic abnormalities and to compare outcomes, costs and approaches to the diagnosis and treatment of MDS and anaemia in the elderly and to develop more effective and safer evidence-based, tailored interventions for elderly patients with anaemia and/or lower-risk MDS, leading to better treatment compliance and more cost-effective use of healthcare resources.

Started in May 2015, the 5-year MDS-RIGHT project is co-ordinated at the Radboud university medical center in Nijmegen, the Netherlands and involves 15 project partners from 8 European countries.

Presenting the rationale of MDS-RIGHT, Prof. de Witte explained that the project was based on MDS as a chronic bone marrow malignancy that is predominant in the elderly and complicated by severe anaemia. It is estimated that around 20% of elderly European citizens suffering from anaemia have MDS, meaning that as many as 2 million people could be affected by MDS.

MDS is a relatively rare disease that has a significant impact on quality-adjusted survival (with an average survival of MDS patients of about 3-4 years), leading to a loss of general life expectancy of around 8 years. The MDS disease burden keeps growing because of the ageing of the population, causing an increasing financial strain on patients and healthcare systems.

Explaining the structure of MDS-RIGHT, Prof. de Witte said that the collaborative work of the research consortium has been organised into seven highly interacting and interdependent work packages. Three work packages deal with MDS diagnostics through the collection and exchange of data, two focus on patient management and related recommendations, one is tasked with disseminating, exploiting and communicating study results and one is responsible for the overall project co-ordination and management.

Now in its second reporting period, Prof. de Witte said that the MDS-RIGHT was well on track and that a solid basis for achieving its expected impacts had been laid. In addition to the MDS-RIGHT work carried out by the project partners, several successful co-ordination and investigators' meetings were held over the past two years.

The MDS-RIGHT meetings focused on the development of interactive patient management guidelines, the development of new Case Report Forms (CRFs) for collecting data on health economics and new Core Outcome Sets (COS), project dissemination and stakeholder involvement and on the MDS-Europe online platform (**https://mds-europe.eu/**) that also serves as the home of the MDS-RIGHT project website.

MDS patient management challenges and solutions (panel presentations and discussion) – Introductory remarks

Moderator: David Bowen, Honorary Professor of Myeloid Leukaemia Studies & Consultant Haematologist - St. James's University Hospital, Leeds, United Kingdom

Prof. David Bowen briefly explained that the aims of the following panel presentations and discussion were to illustrate physician, patient, nurse, regulatory and industry perspectives on MDS patient management and related challenges and solutions. This would help to put into perspective what can be expected of the EUMDS and MDS-RIGHT projects and related outcomes and to encourage all MDS stakeholder communities to engage in these projects.

MDS patient management challenges and solutions – The medical specialist perspective

Pierre Fenaux, Haematology specialist - St. Louis Hospital, Paris, France

Prof. Pierre Fenaux started by providing examples of the improvements made in MDS diagnostics, thanks to morphology, cyto-genetics, somatic mutation analysis and flow

cytometry (FCM, a method for analysing cell surface markers). He pointed out that the International Prognostic Scoring System (IPSS) and the Revised International Prognostic Scoring System (IPSS-R, **http://ipss-r.com/**) for MDS risk assessment, but also somatic mutations, FCM and the outcome of treatment with hypomethylating agents (agents modifying DNA/molecules carrying genetic information) were now increasingly used as prognostic factors for MDS.

Prof. Fenaux continued by saying that significant improvements have also been made in the field of MDS treatment, especially with regard to allogeneic (donor) stem cell transplantation, hypomethylating agents, erythropoiesis (red blood cell formation)-stimulating agents and lenalidomide. He cautioned, however, that only a small number of MDS patients were actually eligible for transplantation, that hypomethylating agents provided only modest improvements in terms of survival and that the response duration of other treatment options was limited. Therefore, the current treatment situation for MDS patients is still unsatisfactory.

To further improve MDS diagnostics, Prof. Fenaux recommended having more trained morphologists (biologists analysing the structure of cells or molecules) and cytogeneticists (professionals analysing cellular components, particularly chromosomes) as well as increased access to next-generation sequencing (NGS, a high-throughput technique for analysing genetic information). He said that large international studies that capture, integrate, analyse and harmonise 'Big Data' on MDS (such as the Innovative Medicines Initiative (IMI) HARMONY study) would help bring about improvements in the field of prognosis.

To improve the MDS treatment situation, new drugs and companies willing to have them registered were needed, Prof. Fenaux added, as well as help from companies for academic clinical trials planned and carried out by co-operative study groups, more international co-operation to increase study populations for new treatment combinations and also the help from patient support groups to inform patients about the importance and benefits of clinical trials.

MDS patient management challenges and solutions – The MDS patient perspective

Sophie Wintrich, Chief Executive/Patient Liaison of the MDS UK Patient Support Group - King's College Hospital, London, United Kingdom

Ms. Sophie Wintrich gave her presentation on behalf of the global MDS Alliance (MDSA), an umbrella organisation of national MDS patient groups that was established in 2011 to promote collaboration, shared information, and increased awareness of MDS worldwide. The MDSA also provides a shared pool of knowledge, skills and resources as well as training and assistance opportunities for junior patient groups. The audiences of the MDSA include patients and relatives, clinicians, regulatory and HTA agencies, as well as payers.

According to Ms. Wintrich, the aims of the MDSA are to improve and promote faster strategic collaborative research carried out in a spirit of sharing information between all stakeholders — with MDS patient organisations acting as the "honest broker" between them — and more clinical trials in this field. Additional aims include better real-world patient data (i.e., data collected outside clinical trials), increased and more specific scientific evidence and also robust data on quality of life (QoL). Furthermore, the MDSA advocates for an increased access to more effective therapies, especially in low-risk MDS, where uncertainty is a major concern to patients. especially in 'Watch-and-Wait' situations. The MDSA also aims for a better dissemination of clinical practice guidelines.

Ms. Wintrich said that the MDSA was convinced that the key to overcoming current issues in the MDS arena was to obtain more and

better data with the help of robust MDS registries. She pointed out that projects like EUMDS and MDS-RIGHT, but also EuroBloodNet (a new EU-funded project to establish a European Reference Network for rare haematological diseases) and the aforementioned HARMONY study, which emphasise the use of molecular analysis, were extremely important with regard to the approval of new MDS treatment options by the European Medicines Agency (EMA) and also for Health Technology Assessment (HTA).

The MDSA also proposes the increased use of disease-specific QoL tools (QUALMS and QoL-e), which are also important for drug appraisals and HTA, more effective tissue banking and accurate databases, increased essential real-world data (including data on comorbidities), an improved cross-border supporting system for patients and biological samples and strengthened MDS stakeholder collaboration, again with MDS patient organisations as the "honest broker".

In conclusion, Ms. Wintrich explained that the above would lead to a better understanding of MDS, more refined and accurate prognostic tools based on molecular analysis, more hope for, and access to, a larger choice of treatment options (including more personalised treatments), less time wasted on receiving wrong treatments, and more flexible access to treatments with better response rates, as "non-response" rates are hard to cope with for MDS patients.

MDS patient management challenges and solutions – The nurse perspective

Corien Eeltink, Clinical Nurse Specialist - VU medical center, Amsterdam, The Netherlands

Having worked as a nurse at a multidisciplinary outpatient clinic for MDS patients for many years, **Ms. Corien Eeltink** stressed that the MDS patient population was quite heterogeneous, with some patients being frail and others still comparatively fit. She said that the challenge for geriatric care was to find the right balance between the desire for an increased life expectancy and the need for maintaining a good QoL. This is why a Comprehensive Geriatric Assessment of the needs of the patients was essential, not only for identifying frail patients but also for giving all patients the care they need.

According to Ms. Eeltink, many healthcare professionals caring for older patients are not adequately trained for using geriatric assessment and screening tools. These tools help to collect information about a patient's functional status, comorbidities, polypharmacy etc. and are then supplemented with questions about living conditions, health literacy, vision and hearing, personal distress, medication adherence, QoL, social network and caregivers, access to transportation and meaning of life.

Referring to research having shown that these assessments may have a significant impact on treatment decisions, Ms. Eeltink recommended performing the assessments right after diagnosis, rather than after the treatment decision is made. Given that most MDS patients suffer from a variety of impairments of their QoL, Ms. Eeltink said that the correct interpretation of QoL data was crucial, even though this may be complicated.

Haematology nurses often support MDS patients as a "case manager" from the time of diagnosis until the end of life. In addition to the aforementioned upfront geriatric assessment and understanding of QoL, Ms. Eeltink explained that the other roles of nurses caring for MDS patients included patient education and information and also nursing research. She said that there was a need for getting more support for the latter, also to make sure that the most appropriate interventions were identified with regard to MDS patients' QoL.

In conclusion, Ms. Eeltink argued that a full geriatric assessment may be time-consuming, but it could help detect unknown geriatric

problems in older patients. A short initial screening test, followed by geriatric evaluation for patients at risk, could help identify older patients still needing a detailed geriatric assessment. Nurses should be fully involved in MDS-related research initiatives and receive adequate training. QoL being an important outcome of MDS patient management, results should be made available for discussion with every MDS patient.

MDS patient management challenges and solutions – The regulatory/HTA perspective

David Bowen, Honorary Professor of Myeloid Leukaemia Studies & Consultant Haematologist - St. James's University Hospital, Leeds, United Kingdom

Prof. David Bowen first explained that what qualified him as an MDS clinician and researcher to talk about regulatory topics and HTA was his current work as a member of the Technology Appraisal Committee of the National Institute for Health and Care Excellence (NICE), the Heath Technology body for the UK, as well as his previous work as National Expert to the Scientific Advice Unit (Geriatric Medicines & Adaptive Pathways) of the European Medicines Agency (EMA).

He then presented the regulatory process for human medicinal products in the European Union (EU), saying that a marketing authorisation of a new medicinal product by the EMA took about one year. During this time, national early access programmes could provide 'bridging mechanisms' for reimbursement before marketing authorisation and/or in the transitional period between marketing authorisation and pricing and reimbursement decisions taken by national HTA bodies and payers.

Addressing the pharmaceutical industry, Prof. Bowen said that the EUMDS and MDS-RIGHT projects could assist trial design by looking at the prevalence of MDS sub-populations or at the treatment outcomes achieved in the real world with the current standard of care. The scientific projects could also provide supportive data for clinical trials (e.g., outcomes of a standard of care arm) for regulatory submission as well as prospective real-world data (RWD) in the post marketing setting on patient demographics (including comorbidity), outcome and QoL.

He went on to explain several mechanisms created by the EMA to improve the pace of access to new drugs, highlighting especially the Adaptive Pathways concept, where iterative programmes were developed using already existing RWD to support the generation of scientific evidence, also through the engagement with other healthcare-decision makers.

Addressing regulators in particular, Prof. Bowen explained that prospective RWD generated by EUMDS and MDS-RIGHT could also help inform early scientific advice used by the EMA and the National Competent Authorities. This could also be useful for the implementation of Adaptive Pathways concepts, through RWD collection and also in terms of informing elements of pharmacovigilance programmes (e.g., second primary malignancies or specific safety signals captured by comorbidity).

The HTA community, Prof. Bowen pointed out, could benefit from the EUMDS and MDS-RIGHT projects by obtaining country-specific outcome data (which could inform comparative effectiveness analysis and resource utilisation) and prospective outcome data with a longer follow-up period than clinical trials for a new agent, which would help reduce uncertainty associated with extrapolation by adding RWD to the cost-effectiveness analysis.

For payers, the EUMDS and MDS-RIGHT projects could assist in HTA evaluation and by providing ongoing outcome data for regular review and re-evaluation of a given drug's cost-effectiveness after several years.

Prof. Bowen concluded in highlighting the EUMDS as a "unique resource", it being the only prospective long-term registry for low-risk MDS, now amended to also include high-risk MDS, health economics as well as expanded, disease-specific QoL information.

MDS patient management challenges and solutions – The industry perspective (1)

Margaret Doyle, Global Medical Affairs Director, Haematology - Janssen, Pharmaceutical Companies of Johnson & Johnson, Dublin, Ireland

Dr. Margaret Doyle summarised the key challenges associated with drug development, saying that it could take more than 10 years and cost several hundred million dollars to get just one out of 10,000 candidate molecules registered as a novel treatment option. In addition, not all patients may have access to this new drug, depending on the situation in their country. She also stressed the importance of including the patient perspective into clinical trial protocols and of integrating study endpoints that truly reflected patient needs.

Following a brief outline of Janssen's strategic vision for the elimination of cancer, Dr. Doyle spoke about the company's focus areas in the field of haematologic malignancies, including MDS. She acknowledged that there was still a lot to be learned about complex diseases such as MDS. Therefore, collaboration across stakeholder groups is essential. She pointed out three major challenges that needed tackling, including the question of how to address the true unmet needs of MDS patients, rather than just claiming to be "patient-centred".

According to Dr. Doyle, there is a need to better understand the outcomes and deficiencies of current MDS therapies — what is working well and what is not. In her view, collaboration with EUMDS and MDS-RIGHT is a perfect opportunity for doing this. She mentioned the ongoing task of shortening the time for regulatory approval and market access, the latter often being the bigger challenge. Dr. Doyle reiterated her company's commitment to collaborating with all MDS stakeholders in order to share insights and knowledge, including the disclosure of information on company-sponsored clinical trials.

Speaking about collaborative opportunities and common goals, Dr. Doyle confirmed that the research performed by MDS-RIGHT would certainly help with the provision of timely access to innovative drugs. In addition, the collection of more data would improve understanding and help to find new ways to treat MDS. This included increasing the number of patients enrolled in clinical trials, enhancing translational programmes to accelerate clinical development and identify early benefits of new treatments, improving trial design flexibility and overcoming current deficiencies in the understanding of therapies and solutions.

MDS patient management challenges and solutions – The industry perspective (2)

Alberto Vasconcelos, Director, Medical Affairs Myeloid Disease Lead for Europe, Middle East & Africa (EMEA) - Celgene, Boudry, Switzerland

Mr. Alberto Vasconcelos started by challenging the concept of randomised clinical trials, arguing that they generally excluded groups such as patients with given comorbidities, children, pregnant women, chronic disease patients, very elderly or frail people and thus

only included a selected group of people that may not necessarily truly reflect the target population. He said that randomised, controlled clinical trials had limited power to detect rare drug adverse events and sometimes were not able to assess long-term safety or effectiveness, adding that some hypothesis may be impossible to test for ethical reasons.

Therefore, Mr. Vasconcelos reasoned, "No matter how well designed, geographically broad, long follow-up, flawless monitoring and data collection, no single trial, or cluster of trials, is ever capable of answering all the relevant questions!" He therefore stressed the need for increased stakeholder collaboration in order to complement experimental evidence with real-world evidence and to complement evidence from clinical trials with evidence generated by high quality clinical routine registries like EUMDS. This was necessary not only to inform HTA and reimbursement strategies and access to drugs, or to answer questions clinicians might have, but also to support regulatory decision-making.

Mr. Vasconcelos referred to the recent EMA Patient Registry Initiative that aims to optimise the use of existing registries (and facilitate the creation of new ones) in order to obtain post-authorisation data, hence using data on the effectiveness and safety of medicines beyond what is available from the evidence supporting a marketing authorisation.

This, Mr. Vasconcelos explained, allowed to include patients who were originally excluded from randomised clinical trials but who may still receive the medicine when the product is marketed. He concluded in quoting the EMA as saying that providing RWD may become a requirement for marketing authorisation applicants or holders, adding that registries such as EUMDS were an excellent source for obtaining this kind of information.

MDS patient management challenges and solutions – Panel discussion

All

The ensuing panel discussion was moderated by **Prof. David Bowen** and revolved around questions and comments received from the audience. Panellists included **Mr. Vasconcelos**, **Ms. Wintrich**, **Ms. Eeltink**, **Prof. de Witte**, **Dr. Doyle** and **Prof. Fenaux**.

Prof. Eva Hellström-Lindberg, haematology specialist from Sweden, asked why it was still so difficult for patients to go from one country to another to participate in a clinical trial and why MDS clinical trials often excluded the evidence of cancer that was present in many MDS patients undergoing clinical trials.

In answering the second question, **Dr. Doyle** said that the goal should always be to take as many comorbidities as possible into consideration when designing clinical trials, and that the industry was moving in this direction, but it would take time. **Mr. Vasconcelos** added that regulatory agencies often expected clinical trials to have clear exclusion criteria and clear operational

questions to measure, so it was very difficult to strike the right balance.

Responding to the first question, **Prof. Fenaux** said that this should be regulated by the EU Directive on cross-border healthcare and that European Reference Networks for Rare Diseases (including EuroBloodNet for rare haematological disorders) were being created exactly for this purpose, namely to allow patients to go to another country if a given drug is not available in their home country. Coming back to the second question, **Prof. de Witte** added that patient selection was also often seen in observational registry data, for example, selection based on age or on having received iron chelation.

Ms. Wintrich argued that it was not right to discriminate people based on age (and that it was even illegal to do so in Sweden), as sometimes elderly people could be very fit and therefore their biological age should always be considered as being more important than their calendar age. **Prof. Bowen** fully agreed and invited additional questions from the audience.

Dr. Nicolas Bonadies, haematology specialist from Switzerland, stressed the fact that extracting and transferring patient data from medical records to a registry was time consuming and expensive and recommended devising a funding strategy for data collection and for the maintenance of patient registries, including the cost for hiring data managers. **Prof. Bowen** replied that electronic patient records would help in the longer term with collecting patient data in a more cost-efficient manner and that the general focus now was on making patient registries more disease-specific.

Dr. Bonadies also raised the issue of improving the standardisation of data collection and that this should be taken into consideration early on, starting at student level. **Ms. Wintrich** replied that even though the short-term costs for setting up and maintaining registries may by high, the long-term costs of not collecting real-world evidence through patient registries would be even higher, given that lack of data already contributed to delays in the approval of new treatment options for diseases such as MDS. Registries therefore paid off in the longer term. Everybody on the panel agreed and, for the sake of time, **Prof. Bowen** thanked the panellists and closed the discussion.

MDS patient management recommendations and interactive online support

Eva Hellström-Lindberg, Haematology specialist, Karolinska Institute - Huddinge University Hospital, Huddinge, Sweden

Prof. Eva Hellström -Lindberg explained that MDS patient management recommendations were needed and widely used by MDS specialists (for establishing national guidelines), haematologists (for establishing local therapeutic routines), internists and trainees (for using them as textbooks), patients and relatives (for patient empowerment), patient organisations (for patient assistance), health care regulators, researchers, the pharmaceutical industry and others.

Given that absolute and relative survival is poor in MDS (even in lower-risk MDS) and that about 50% of patients are transfusion-dependent at diagnosis, Prof. Hellström-Lindberg said that it was crucial to make the right treatment decisions upfront. She announced that the registry was now being expanded to include all MDS subtypes as well as more details regarding treatment, outcome and health economics.

Looking at the European LeukemiaNet (ELN) MDS guidelines for the treatment of patients with IPSS intermediate-risk 2 or high-risk primary MDS (2013), Prof. Hellström-Lindberg explained future strategies for improving the outcome for patients with higher-risk MDS, for example, by including comorbidity and QoL into clinical decision-making. She also stressed the need to consider stem cell transplantation (SCT) for all newly diagnosed MDS patients; the need to decide on treatment with Azacitidine based on mutational profiles and other clinical variables; the need to improve the primary efficacy of Azacitidine; the need for developing new drugs and drug combinations; and the need to improve the outcome of allogeneic SCT, including new pre- and post-SCT strategies.

Prof. Hellström-Lindberg then introduced the ELN therapeutic algorithm for patients with IPSS low-risk or intermediate-risk 1 primary MDS (2013) and presented some conclusions with regard to improving the treatment of MDS patients with erythropoiesis (red blood cell formation)-stimulating agents (ESAs). She argued that while ESA was an effective treatment for the anaemia of lower-risk MDS patients, it would be significantly more effective if initiated before the onset of a regular transfusion need. However, there were major differences between European countries with regard to haemoglobin levels at which to start ESA treatment and also in terms of ESA reimbursement. In some countries, ESA treatment was only possible if patients regularly received transfusions. Future strategies recommended for improving the outcome for patients with low-risk MDS were similar to those presented for high-risk MDS, Prof. Hellström-Lindberg said.

Moving on to the interactive online support platform for patient management developed and provided by MDS-RIGHT, she briefly presented the outline and a format of the corresponding dynamic, therapeutic algorithm interactive tool. This new tool for healthcare providers is being developed in responsive design, for use on personal computers, tablets, smartphones and other communication devices. It will allow the continuous integration of new evidence generated by MDS-RIGHT and facilitate clinical decision-making in MDS. It is planned to further expand this interactive online support platform also for use by patients and regulatory agencies in the future.

MDS-RIGHT/MDS-Europe online platform

Alex Smith, Senior Research Fellow, Epidemiology & Cancer Statistics Group - University of York, York, United Kingdom

Dr. Alex Smith introduced the MDS-RIGHT/MDS-Europe online platform (**https://mds-europe.eu/**) as a project carried out within the MDS-RIGHT dissemination, exploitation and communication work package. She said that the aim of the website was to provide a platform for interaction with all MDS stakeholders to communicate and promote state-of-the-art MDS patient management recommendations. A dedicated multi-professional MDS-RIGHT task force was established overseeing the website structure and the (responsive) design development, content development and programming, as well as the gradual expansion, promotion and continuous updating of the online platform.

Dr. Smith explained that the website included a dedicated MDS-RIGHT project section and that it offered access to MDS-related resources, research groups, patient and professional organisations, patient information, information on clinical trials, registries, scientific publications, news and events, and a dedicated MDS community section featuring monthly discussion articles for continuous MDS stakeholder interaction. She encouraged all MDS stakeholders to contact MDS-RIGHT if they wished to contribute an article to this platform — and anybody who has an interest in MDS was invited to read and comment on any of the articles already online.

Dr. Smith also touched upon the aforementioned interactive MDS patient management recommendations section, saying that is was under construction and would be

launched shortly, allowing users to obtain clinical practice guidance for a variety of MDS treatment pathways in real time.

Since its soft launch in April 2016, visits to the online platform from around the world have been increasing, Dr. Smith explained. As the development of the website is still ongoing, she invited all stakeholders to keep visiting the website to see what is new and also to use it as a hub for European MDS information, guidance and community interaction.

Dr. Smith concluded in saying that there would also be an MDS-RIGHT workspace for all MDS stakeholders where one could find relevant documents, plans, progress reports, meeting minutes, presentations etc. Upon registration, interested stakeholders would receive a login and then have access to the material.

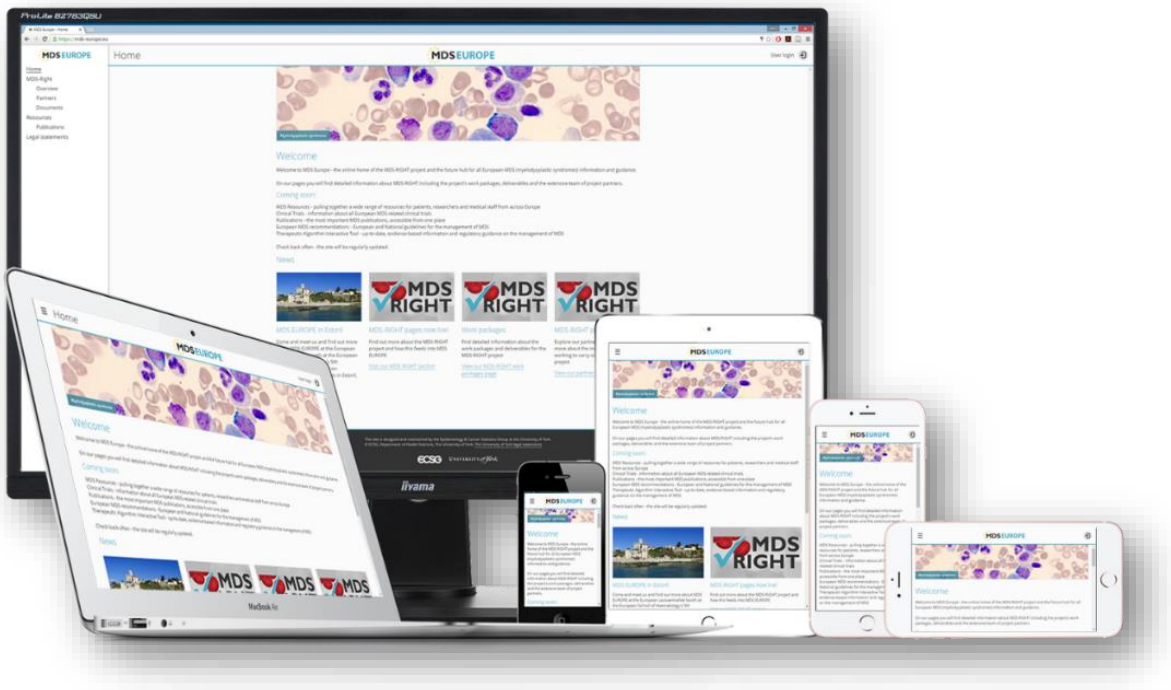
Ms. Eeltink mentioned that nurses looking after MDS patients should also be included in the development of the MDS-Europe website. Dr. Smith agreed and said that it would be very helpful to get more MDS stakeholders involved in the continuous development of the MDS-Europe online platform, also including MDS patient representatives.

Closing remarks

Theo de Witte, MDS-RIGHT project co-ordinator - Radboudumc, Nijmegen, The Netherlands

This meeting being the first MDS-RIGHT multi-multi-stakeholder meeting, **Prof. Theo de Witte** said that this meeting was a learning experience for all members of the MDS-RIGHT team. He highlighted again the continuous progress of the project, now entering its third year.

Prof. de Witte thanked all participants for attending the meeting and said that this stakeholder meeting, whose goals had been fully achieved, would hopefully prove helpful in terms of increasing future MDS stakeholder interaction and collaboration to help improve the situation for MDS patients in Europe.



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