

## PRESS RELEASE

### **Stakeholders agree on what is needed to improve the care of patients with myelodysplastic syndromes (MDS), a blood cell disorder**

*Nijmegen, 17 August 2017* – More than 70 leading medical specialists, nurses, patient advocates, researchers, regulatory / Health Technology Assessment (HTA) experts and industry representatives gathered on 3 May 2017 in Valencia, Spain to discuss existing challenges and what is needed to improve the situation of patients affected by myelodysplastic syndromes (MDS). More and better research data, additional and improved clinical trials and treatment options, better professional guidance and enhanced stakeholder collaboration are the keys to improving MDS patient management, the meeting found. It was hosted by MDS-RIGHT, a European Commission-funded international research consortium.

“The prevalence of MDS keeps growing and MDS under- and overtreatment are an increasing financial burden on patients and healthcare systems across Europe,” explained Prof. Theo de Witte of Radboud university medical center in Nijmegen, the Netherlands, who co-ordinates the MDS-RIGHT project. “Available treatments for MDS generally do not cure the disease and the average survival of MDS patients is just about 3-4 years,” he added. Prof. Pierre Fenaux, haematology specialist at St. Louis Hospital in Paris, France, agreed: “The current treatment situation for MDS patients is unsatisfactory and we urgently need new and better treatment options and also drug combinations for managing MDS.”

MDS are a complex group of blood cell disorders, often complicated by severe anaemia, a shortage of red blood cells. MDS predominantly affect older people who may be frail or otherwise unable to participate in clinical trials. Because eligible MDS study populations are small and interventional study endpoints do not always fully reflect unmet patient needs, the meeting stressed the importance of conducting more and larger clinical trials and observational studies. MDS patients should be recruited into clinical trials from many countries and MDS research studies should be designed and conducted with greater flexibility and truly reflect the MDS patient perspective. “Getting a potentially life-extending treatment is not always the highest priority for all MDS patients; especially when the chances of responding to such treatments are slim, some opt for quality over quantity of life” said [Sophie Wintrich](#), Chief Executive and Patient Liaison of the MDS UK Patient Support Group in London, speaking on behalf of the MDS Alliance, a global umbrella organisation of national MDS patient groups. “Non-response rates are hard to cope with for MDS patients,” she added for consideration.

Even well designed MDS trials may not answer all relevant questions, however. The MDS stakeholders argued that clinical trial data should be complemented with additional and specific real-world data on all MDS subtypes, comorbidities, treatment outcomes, health-related quality of life and also health economics. This can be done with the help of robust and well-funded patient registries, such as the European MDS Registry (EUMDS), on which the MDS-RIGHT study is based. Prof.

[David Bowen](#), consultant haematologist at St. James's University Hospital in Leeds, United Kingdom and member of a Technology Appraisal Committee of the National Institute for Health and Care Excellence, the Health Technology Assessment body for the UK, confirmed: “Real-world data collected and analysed by EUMDS and MDS-RIGHT constitute a unique resource for informing clinical, industry, regulatory and HTA decisions.”

Up to 50 percent of MDS patients with anaemia depend on red blood cell transfusions early after diagnosis. Making the right treatment decisions upfront is therefore crucial to avoid MDS over- and undertreatment along the way. All stakeholders agreed that healthcare professionals treating MDS patients must have access to state-of-the-art diagnostic tools, for example, to carry out molecular analyses, but also to tools for upfront geriatric assessment and for assessing patient- and disease-specific health-related quality of life. “These assessments may have a significant impact on treatment decisions and should be done right after diagnosis, rather than after the treatment decision is made,” said [Corien Eeltink](#), clinical nurse specialist at VU medical center in Amsterdam, the Netherlands.

The meeting also confirmed the need for [better professional guidance](#) for clinical decision-making, based on state-of-the-art therapeutic recommendations on all possible aspects of MDS patient management. Introducing a corresponding new and comprehensive online support platform currently developed by MDS-RIGHT, Prof. [Eva Hellström-Lindberg](#), haematology specialist at Karolinska Institute - Karolinska University Hospital in Stockholm, Sweden, explained: “This new dynamic and interactive set of online tools will allow healthcare providers to obtain up-to-date and evidence-based clinical practice guidance for a variety of MDS treatment pathways in real time. It will shortly be launched on the MDS-Europe website and can be accessed and used on your personal computer, tablet or smartphone.”

The stakeholders finally agreed that substantial progress can only be achieved through increased collaboration. This requires more willingness to jointly generate and share MDS-related evidence, provide cross-border patient support, for example, for participating in clinical studies in other countries, involve more MDS patients in study design and accelerate the development of new MDS treatment options by complementing clinical studies data with real-world data. The newly-established Community section of the MDS-Europe online platform can be used as a tool for stakeholders to keep each other abreast of planned and ongoing projects and to raise and discuss important MDS-related issues that need tackling. The platform will be continuously expanded and everybody interested in helping to improve the situation for MDS patients is welcome to join the online discussion at <https://mds-europe.eu/community>.

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