MDS Manifesto

A call to improve the care for patients with myelodysplastic syndromes (MDS)

Myelodysplastic Syndromes (MDS) are a group of relatively rare chronic bone marrow malignancies, which predominate in the elderly. MDS are attributed to a failure of the body's bone marrow to produce healthy blood cells. MDS are usually complicated by severe anemia (a shortage of red blood cells). Anemia is not uncommon in older people (anemia of the elderly, AoE) and it is hypothesized that 20% of all AoE cases — a total of 2 million European citizens — might be explained by as yet undiagnosed MDS. About 25% of MDS patients develop acute myeloid leukemia (AML), a blood cancer. But also many patients with MDS, who do not progress to AML, die prematurely due to the consequences of bone marrow failure.

There is still not enough knowledge available about the biology and other disease aspects of MDS. In many cases, MDS are not correctly diagnosed and related treatment recommendations are not fully applied, while symptoms and complications of the disease negatively impact the survival and quality of life of patients. Some progress has been made over the past years and several MDS treatment options are available currently. However, these treatments generally do not cure the disease and especially, in high-risk patients survival can be very short. High-risk MDS patients have a similar poor survival to locally advanced metastatic lung or pancreatic cancer. The prevalence of MDS keeps increasing due to the aging of the population and MDS under- and overtreatment is a growing financial burden on patients and healthcare systems.

The signatories of this Manifesto therefore call on European and national authorities, governments, research institutions, professional societies, patient groups, and the pharmaceutical and biotechnology industries to help increase research efforts and improve the diagnosis and care of people affected by MDS by addressing the following key needs:

1. Increased laboratory research and analysis of real-world datasets

Although detailed knowledge of acquired specific gene mutations in different types of MDS is emerging, many questions remain how these gene mutations cause MDS and how they can lead to 'targeted' treatment. Information from clinical trials alone may not be able to answer all relevant questions. Therefore, more real-world data (data generated outside clinical trials, usually during regular clinical care) on all subtypes of MDS — including comorbidities, outcomes, disease-specific health-related quality of life, and health economics — should be collected and analysed. This can be done with the help of robust and well-funded national and international patient registries, such as the European MDS Registry (EUMDS) and Big Data collaborations such as HARMONY (European Network of Excellence for Big Data in Hematology). Real-world data constitute a unique and essential resource for informing clinical, industry, regulatory and health technology assessment (HTA) decisions.

2. More international and patient-centred collaborative clinical research

Eligible MDS study populations are small and interventional study endpoints do not always fully reflect unmet patient needs. In addition, clinical trials for MDS often tend to exclude comorbidities such as cancer. Therefore, more and larger clinical trials should be conducted, recruiting MDS patients from many countries through networks, such as the European MDS Studies Coordination Office (EMSCO). MDS clinical trials should be designed and conducted with greater flexibility (e.g. by using adaptive design methods) and fully reflect the patient perspective (patient reported outcomes). We call upon industry and government funding bodies to support academic clinical investigations carried out by co-operative study groups.

3. More, and better, affordable and accessible treatment options

In light of substantially reduced survival rates (average loss of 12 years), and the growing disease burden, it is crucial that pharmaceutical and biotechnology companies invest heavily in the timely development of novel and affordable treatment options for patients with MDS. Also the European regulators, national health technology assessment (HTA) and reimbursement bodies play an important role in making these new treatment options fully accessible in all countries.

4. Improved diagnosis and professional guidance for patient management

Up to 50% of MDS patients with anemia depend on red blood cell transfusions early after diagnosis. Approximately 5% of all blood transfused is given to MDS patients. Making the right treatment decisions upfront can help avoid MDS under- and overtreatment along the way. It is therefore essential that all MDS treatment decisions are based on approved diagnosis that is obtained by using state-of-the-art MDS diagnostic tools such as, for example, genomic analyses, but also on the assessment of patient- and disease-specific parameters. In addition, real-time guidance for clinical decision-making should be provided in an easily accessible fashion and based on state-of-the-art therapeutic recommendations that include every aspect of MDS patient management. MDS-RIGHT provides an evidence-based, dynamic and interactive support platform on the MDS-Europe website for use via personal computer, tablet or smartphone.

5. Enhanced collaboration and better patient information, advocacy and involvement

No individual party involved in MDS research and care is able to make substantial progress alone, without the input and support from other MDS stakeholders, including medical specialists, nurses, patient advocates, researchers, regulatory and health technology assessment (HTA) experts and industry representatives. Therefore, more willingness is needed from all parties to enhance national and international collaboration by generating and sharing more MDS-related evidence. In addition, better patient information and advocacy as well as cross-border patient support (also for participating in clinical trials in other countries) are needed and more MDS patients should be involved in the design of clinical trials. The development of new treatment options should be accelerated by sharing and complementing clinical trials data with real-world data and more MDS patient reported outcome measures should be developed and used to assess the quality of MDS care from the patient perspective.

Addressing these key needs will lead to better MDS patient care and a more effective use of healthcare resources. All parties committed to improving the care of MDS patients are encouraged to co-sign and further disseminate this Manifesto and may also use it for their own advocacy purposes.

The manifesto is also available to co-sign and download at https://mds-europe.eu/manifesto.

Signed & endorsed by:

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MDS Patienten Interessen Gemeinschaft

The MDS-RIGHT (Providing the right care to the right patient with myelodysplastic syndrome at the right time) European research project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No. 634789. The European Commission is not responsible for any use that may be made of the information this manifesto contains.

For more information, please visit https://mds-right.org/.