# Newsletter European MDS Registry

June 2017, Issue No 17

**Table of contents**



## From the board

In this newsletter we look back at interactive and interesting meetings in Valencia. We are pleased that many of you participated in the investigators and operational team meetings, and provided us with quite a few relevant suggestions. We will use your suggestions to improve the performance of our Registry.

This newsletter also provides insight into the sub studies performed within the EUMDS Registry, and puts our research on impact of MDS on HRQoL in the spotlight. In case of interest or questions concerning sub studies or any other EUMDS related topic, feel free to contact me or one of the Steering Committee members. The next newsletter will be dedicated to the update of the EUMDS database.

*Theo de Witte*

## Operational team meeting

In Valencia, the Operational Team (OT) came together to discuss the details and implementation of the new protocol (V5.1), the new Case Record Forms (CRF's), and the update of database. The latter will be put online by the data management team in York, in the week of June 26th until July 3rd.

Also, the collection and logistics of patient samples was addressed.

As mentioned in previous newsletters (e.g. No.13) the collection of patient samples is of great value for the EUMDS Registry. Growing interest into the genetic background of MDS has also been expressed by participants during the investigators meeting. Therefore, we emphasize the importance of collecting blood for molecular research at least at inclusion, and preferably also at each follow-up visit.

The logistics of the sample collection will consider two levels. The project management team will first contact all OT-members in order to assess the country policy, regulations and logistics. Subsequently, individual site will be contacted to assess the most (cost-)efficient way for transfer and storage of samples. For some sites this will mean immediate shipping of samples, while in others samples should be stored locally prior to shipment when a certain number of samples are collected.

## 3rd investigators meeting

The third investigators meeting since the initiation of the EUMDS Registry was organized in Valencia, Spain. We thank Prof. Dr. Guillermo Sanz – national principle investigator (PI) of Spain – for hosting this meeting in Hospital La Fe.

Besides the Steering Committee (SC) and OT members, many local PI's, research nurses, data managers, as well as a patient representative were present.

After a word of welcome by Prof. Dr. Theo de

Witte, Sophie Wintrich – a patient representative from the MDS alliance – kicked off with a presentation on the patients perspective. She gave valuable insight in the perception of patients, and the pursuit of improving the life expectancy and quality of life of MDS patients. In response to a question by one of the medical specialists, she emphasized the importance to clearly inform the patient about the fact that MDS (even lower-risk MDS) is a blood cancer. She stressed that the shock of coming across the term 'cancer' later on, might be much greater, than when the term is introduced during the initial consultation. Cultural differences should however be considered.

Besides addressing the audience on the new protocol and database, the meeting focussed on '*How EUMDS can contribute to MDS care*'. Progress and outcomes of 6 sub studies were presented, showing the value, strength and possibilities of the Registry. The extensive amount of 'real world' data on such large numbers of patients (>2200) with extensive follow-up (>8900 visits, ranging to 8,5 years), allows us to address many observational research questions (see section on sub studies), aiming to improve MDS diagnosis, treatment and patient care.

Furthermore, the audience provided many valuable suggestions on EUMDS related topics during a feedback session. One of the suggestions was to communicate (more) about the sub studies being performed within the registry. A suggestion we will act on right away in the following section.

Interested local PI: *"very interesting, the EUMDS registry. Our hospital is not involved yet, but I would like our centre to be included"*

Input and feedback of everyone involved in the registry is of great importance to improve the performance of our Registry. All participants should feel free to contact project management or one of the SC members with suggestions.

Research nurse: *"the sub studies are very interesting and very nice to see. Definitely motivating to commit more to the EUMDS registry"*

## EUMDS sub studies

During the investigators meeting much interest was shown in the various EUMDS sub studies (listed below). So far, most of the sub studies are driven by SC members. However, we would like to increase your involvement as a larger community, to initiate new studies and to contribute to the analyses involved.

Various attendants of the investigators meeting have indicated their interest in participating in one or more of the ongoing EUMDS sub studies. If you are interested, or in case you have new ideas or suggestions, please, contact project management or one of the SC members.

| | |
|---|---|
| • Demographics | • BM fibrosis |
| • ESA | • TRIAGE-MDS (FP7) |
| • Cytomorphology | • MDS-RIGHT (H2020) |
| • HRQoL | • Auto-immune disorders |
| • Iron sub study | • Thyroid disorders |
| • Iron chelation | • Metformin |
| • Transfusions | • del20q |
| • BM metaphases | • New diagnostic algorithm |
| • Kinetics of cytopenias | • SF3B1 |
| • MDS and solid tumors | • Diabetes sub study |
| • del5q | |

All EUMDS participants may propose new sub studies, by means of submitting a proposal to the EUMDS project manager, Corine van Marrewijk. Proposals will first be critically reviewed and evaluated with regard to scientific value and feasibility by the Executive Committee, and subsequently decided upon

by the SC members. A format for sub study proposals is available at project management in Nijmegen.

A more detailed overview of the sub studies and the progress will be circulated to EUMDS participants as annex to this newsletter. ***We emphasize that all EUMDS participants are expected to protect the EUMDS research ideas, and are not allowed to distribute this document to others.*** In case you have not receive the sub study overview by e-mail yourself, but you are a EUMDS participant, you can ask Karien Croezen to sent you a copy. Updates of this overview will become available in the secure part of the EUMDS website, when a full revision of the information on the EUDMS website is completed (planned for second half of 2017).

## Contact details local PI's/site staff

In addition to more extensive communication on sub studies, improvement of communication in general was raised during the feedback session. Some local PI's indicated that they do not receive newsletters (and thus neither any other communications circulated by the central project and data management teams), while other made suggestions on the type of information they would like to receive.

Currently, the mailing list for newsletters and other communications to all EUMDS participants is limited to those EUMDS team members that have a registered login for the EUMDS website (www.eumds.org), since contact details of others are not yet centrally available in the EUMDS Registry database, only at national level.

Central storage of contact details of all local PI's and site staff involved in the EUMDS Registry is one of the major updates of the EUMDS database. Once the updated database is online (see below), we will contact each participating site to ask you to enter the requested contact details of their site as well as all from all staff members at your site actively involved in the Registry. Central storage of contact information will allow us to get relevant communication across to all participants in the near future, but also simplify for example communication by the monitor, and identification of local site PI's for acknowledgements in EUMDS publications. Contact details will only be used for internal EUMDS purposes and will not be provided to external parties.

## Sub study in the spotlight: HRQoL

MDS has considerable impact on the quality of life of patients, as is nicely described by mr. Festy in the blog '*MDS patients speak out*' *(https://mds-europe.eu/community/articles/20170411)* on the MDS Europe website (see section below). Therefore, inclusion of health-related quality of life (HRQoL) of MDS patients in daily practice as well as in clinical studies has been propagated by patient organisations and authorities. Especially the need to take into account MDS specific restrictions to optimize patient care has been expressed by patient advocates and other MDS stakeholders.

HRQoL is an important research focus within the EUMDS Registry. Since the initiation of the Registry, HRQoL has been and will continue to be monitored at initial diagnosis and at 6-monthly intervals in (almost) every EUMDS patient by means of the patient self-reported EuroQol questionnaire. This general HRQoL measure is limited to evaluation of restrictions in: pain and discomfort, mobility, usual activities, self-care, and depression (EQ-5D), and provides an overall health status (EQ-VAS) of the patient.

The first result on HRQoL impairments at diagnosis in 1690 lower-risk MDS patients from the EUMDS registry compared with an average reference population of comparable age, have been presented at the EHA-2016 and MDS-2017. The analyses showed that MDS patients specifically were more anxious and depressed, and were restricted in the ability to carry out their usual activities as compared to the non MDS population. The manuscript, initially submitted in January, is currently being revised for submission to Leukemia. Moreover, analyses plans for the second and third HRQoL manuscripts are

being prepared.

In order to allow evaluation of more MDS specific HRQoL impairments and further optimize patient care, the QUALMS (Quality of Life in Myelodysplasia Scale) – a new MDS specific questionnaire – is now introduced within the EUMDS registry (in 6 translations). QUALMS particularly addresses HRQoL restrictions frequently observed in MDS patients which are not covered by the EQ-5D, e.g. fatigue related restrictions. With 38 questions, the QUALMS is extensive, but will allow for better targeting of health care interventions. In addition to the paper versions, both HRQoL measures will also become available in an electronic version to facilitate their application.

## MDS Europe website

The MDS Europe website (www.mds-europe.eu) is an important output of the MDS-RIGHT project, and aims to be a central hub for all for all European MDS information and guidance targeting all MDS stakeholders. Since the launch of the MDS-RIGHT community section in April 2016, the website has been extended to a comprehensive online platform, which will continue to be further developed to include an interactive guideline algorithm among others.

We invite you to have a look at the website. Particularly, we would like to point out the community section, where blogs will be published regularly. Our ambition is to turn this section into a lively community, where stakeholders can engage in a dialogue (both anonymous or non-anonymous, whatever feels most comfortable) about various MDS-related topics. Please, feel free to comment and share your opinions about the posted blogs.

## Update of the online EUMDS database

The York team has been working hard to program the update of the online EUMDS database, to accommodate the changes associated with the new protocol (V5.1) and the corresponding CRF's. As indicated above, the updated EUMDS Registry database will become available online early next month. Because of the extend of the updates, a downtime of the online database is planned from ***Monday June 26th* 10 am Central Europe Time (CET) until *Monday July 3rd* 10 am CET**. All users have been informed by the data management team in York by e-mail on Wednesday June 14th. As indicated in that e-mail, the timing of the scheduled downtime is still subject to change, but users will be notified of such change in advance as much as possible. Users are also asked to ensure that the database is not in use at the scheduled down-time for the maintenance work. We apologise for any inconvenience.

This major update of the online database is necessary to allow actual registration in the online database of patients included according to the new protocol (V5.1) by the countries/site that already have all national and local ethical/regulatory approvals in place. All other countries/sites, can continue including patients according to protocol V2.2 until the approval process has been completed. The updated database will still accommodate registrations of the data of these patients as well. In case you encounter any problem with the updated online database after its launch, do not hesitate to contact the data management team (enquiries@eumds.org) and they will try to fix them as soon as possible.

The next newsletter, planned just after the summer, will be dedicated to the update of the online EUMDS Registry database and CRF's.