

Title: The European MDS Registry; learning about the 'real' MDS patients

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What is a clinical Registry?

In order to develop new drugs to treat MDS, clinical trials are mandatory, typically testing the new drug against the treatment that is considered to be the standard at that time. However patients in clinical trials are often different to those that we see every week in our clinics because the clinical trial will try to treat a group of patients who seem quite similar to each other and that means leaving out the patients with other diseases in addition to their MDS, or those too infirm to travel to and from the trial centre for example. The best way to study a typical group of MDS patients without any restrictions is to set up a Registry. This involves obtaining consent from patients to collect information about their MDS and the treatments that they receive at their usual clinic or Day Case Unit visits. They may be asked to complete some questionnaires and sometimes to volunteer occasional extra blood samples but essentially the idea is simple; to systematically capture the usual care of all MDS patients over time into one large database.

The European MDS Registry



The European MDS Registry (EUMDS) started on April 1st 2008, recording information from patients with 'low-risk' MDS in 10 European countries. Now the Registry has expanded to include 145 individual hospitals in 17 countries. The Registry has gathered information on more than 2000 low-risk MDS patients. Patients start in the

registry within 3 months from their diagnosis and are followed for their lifetime with information recorded every 6 months. This information includes any treatment that they received, how they

perceive their quality of life and giving a small 'serum' blood sample. Patients in the registry now have information from an average of four 6-monthly visits recorded. The UK has a prominent role in the project. We have registered 327 UK patients so far, the second highest number registered per country after France. The UK hospitals that are participating in order of number of patients recruited are:

Leeds, Aberdeen, Blackpool, Worcester, Airedale, Northampton, Mid Yorkshire, Bradford, Bournemouth, Harrogate, Truro, York, Oxford, Cambridge, Nottingham, Newcastle, Glasgow, Huddersfield, Manchester Christie, Birmingham Queen Elizabeth.

We have collected half of all of the blood 'serum' samples for the entire project, which are being used for interesting research studies (701 UK samples out of a total of 1211 samples analysed in EUMDS). Our patients have completed the quality of life questionnaires with a high completion rate (298/327 patients; better than most European countries). The UK is playing a prominent role in the organisational and strategic aspects of the EUMDS programme, which is led overall by Professor Theo de Witte from The Netherlands. Professor David Bowen leads the UK arm and is also Co-Chair of the Steering Committee for the EUMDS programme. The University of York Health Sciences Unit (Dr. Alex Smith and colleagues, <https://www.york.ac.uk/healthsciences/research/ecsg/>) hosts the database, provides the informational technology support and analyses the data.

We are building a picture of how low-risk MDS is cared for generally in Europe and also in different countries with some interesting patterns emerging. As well as the general information collected and analysed, there are more detailed research projects evolving within the registry, for example looking at the patients who have received blood transfusions, studying those that have received a blood stimulating drug like erythropoietin (EPO), or the possible importance (or not) of overloading with iron for patients receiving blood transfusions.

We are also studying the bone marrow samples using modern diagnostic tests like next-generation sequencing [NGS] which gives us more detail about the different biology of each patient's type of MDS. We will then be able to look at how this new detailed information could help to predict how patients will fare generally (for example life expectancy/survival and the chances of MDS changing to more aggressive leukaemia) and maybe better predict who might respond to which treatments.

We are now moving onto the next phase of the programme, including higher-risk MDS patients in the registry and engaging new sponsors and new funding streams.

We are deeply grateful to those patients who are helping with this important research initiative.

MDS-RIGHT; a platform for research, for patients, and for other stakeholders



Although technically it maybe an 'offshoot' of EUMDS, the European Union funded 5-year MDS-RIGHT project is a wide ranging, ambitious programme with the potential to lead to an integrated European network for:

- a) MDS research,
- b) patients
- c) influencing important stakeholders such as the regulatory agencies, the payers (Department of Health in UK) and policymakers.

Almost all key opinion leaders for MDS are centrally involved in MDS-RIGHT giving this prestigious programme high credibility and huge potential. Again the University of York is an important partner with Health Economics (cost effectiveness, led by Professor Manca, <https://www.york.ac.uk/che/staff/research/andrea-manca>) now added to the goals.



Key websites

<https://mds-europe.eu>

<http://www.eumds.org>

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MDS UK Note: Interested in taking part as a patient?

If you are newly diagnosed – please ask your haematologist about it. Show them this article and state you'd like to volunteer your blood and biopsy samples – and contribute to this important research.

For any further information, please contact MDS UK:

Email: Mds-uk@mds-foundation.org or Tel: 020 733 7558