



European Rare Blood Disorders Platform

Online Kick off meeting - Summary

2nd July 2020

AGENDA

15:15-15:30 Welcome and introduction – Maria del Mar Mañú Pereira, ENROL coordinator, Vall d’Hebron University Hospital & Research Institute

15:30- 15:45 EU-RD Platform – Simona Martin & Andri Papadopoulou, EC Joint Research Centre

15:45 - 16:15 ENROL Implementation

- Connecting EU-RHD Registries and Healthcare providers for secure sharing and re-using of data WP4 - Maria del Mar Mañú Pereira, Vall d’Hebron University Hospital & Research Institute, Barcelona.
- Patients’ Educational program WP4 – Mariangela Pellegrini, Assistance Publique - Hopitaux de Paris, Ht. St. Louis, Paris.
- Facilitating epidemiological surveillance, research and access to new treatments for RHD WP5 – Béatrice Gulbis, ERASME University Hospital / LHUB-ULB, Brussels.
- Setting-up ENROL’s Platform in line with the EU-RD Platform WP6 – Marina Kleanthous & Petros Kountouris, Cyprus Foundation for muscular dystrophy research, Nicosia.

16:15 - 16:45 Governance and Stakeholders Involvement

- ENROL Governance – Maria del Mar Mañú Pereira, Vall d’Hebron University Hospital & Research Institute, Barcelona.
- Legal and Ethical Issues - Sandra Almeida – Vall d’Hebron Research Institute, Barcelona.
- Promoting Patients involvement in ENROL – Ariane Weinman – EURORDIS
- CEOs involvement – Yolima Cossio - Vall d’Hebron University Hospital, Barcelona.

16:45 – 17:15 Open Discussion: Q&A

Summary of the meeting

The [European Rare Blood Disorders Platform \(ENROL\)](#) has officially started last 1st June 2020, conceived in the core of [ERN-EuroBloodNet](#) as an umbrella for both new and already existing registries on rare hematological disorders (RHD), ENROL aims to avoid fragmentation of data by promoting the standards for patient registries' interoperability released by the [European Platform on Rare Disease Registration \(EU RD Platform\)](#).

ENROL is coordinated by [Maria del Mar Mañú Pereira](#) ([Vall d'Hebron University Hospital \(HUVH\) - Vall d'Hebron University Hospital Foundation - Research Institute \(VHIR\)](#), Barcelona, Spain). Consortium is completed with [Béatrice Gulbis](#) ([Hôpital Erasme – Université Libre de Bruxelles \(ERASME/LHUB-ULB\)](#), Brussels, Belgium), [Pierre Fenaux](#) ([Assistance Publique - Hopitaux de Paris \(AP-HP\)](#), Paris, France) and [Marina Kleanthous](#) ([The Cyprus Foundation for muscular dystrophy research \(CING\)](#), Nicosia, Cyprus).

ENROL will map at the EU level demographics, survival rates, diagnosis methods, genetic information, main clinical manifestations and treatments in order to obtain epidemiological figures and identify trial cohorts for basic and clinical research. To this aim, ENROL will connect and facilitate upgrading of existing RHD registries, while promoting the building of new ones when / where lacking. Target-driven actions will be carried-out in collaboration with [EURORDIS](#) for educating patients and families about the benefits of enrolment in such registries.

The meeting started with a welcome and overview of ENROL at a glance provided by the platform coordinator. Introductory presentation was followed by an overview to the [EU RD Platform](#) and tools already developed by the EC - Joint Research Centre (JRC) for the promotion of interoperability standards for rare disease registries implementation in a dedicated session provided by Simona Martin and Andri Papadopoulou from the EC – JRC.

During the first block of the Kick off meeting, ENROL implementation was detailed by the different tasks leaders. In this context, a total of 6 specific working packages (WP) are defined, linked to Management, Dissemination and Evaluation of the platform together with the following three WP linked to specific objectives:

- WP4. Connecting EU-RHD Registries and Healthcare providers for secure sharing and re-using of data - linked to ENROL specific objective 1 - To connect, upgrade and build EU patient registries on RHDs, while promoting interoperability standards in line with the EU-RD Platform
- WP5. Facilitating epidemiological surveillance, research and access to new treatments for RHD – linked to ENROL specific objective 2 - To enable comparable data on RHD at the EU level for epidemiological and clinical surveillance, while promoting the engagement of basic and clinical research.
- WP6. Setting-up ENROL's Platform in line with the EU-RD Platform – linked to ENROL specific objective 3 - To create an interoperable, extendible and functional web-based platform, which will enable entering and integration of certified patient data from the available sources

During the second block of the meeting, the involvement of the different stakeholders was highlighted as fundamental pillars for the successful implementation of ENROL, including the patients, hospitals' CEOs and the legal and ethical teams. Additional collaborations as with other ERNs on rare cancer will undoubtedly be cornerstone for the alignment of common strategies to ensure the interoperability of rare disease registries.

Download here the [slides presented](#) during the meeting, available also at ENROL website section.

The following questions from the audience were addressed at the end of the meeting:

- *How will ENROL collaborate with other existing European and national registries?*

ENROL aims to support networking at the European level by upgrading and linking existing registries and, ultimately building new ones if there is no action in place. Accordingly, the interaction of existing registries with ENROL has to be analyzed case per case in order to assess how the transfer of data can be achieved in terms of:

- Legal and ethical issues: does the Informed Consent from the existing registry allow the sharing of data at the EU level? Is there any legal restriction contemplated by the national laws for the cross border sharing of data?
- Data elements: what data elements is the registry gathering? Are they part of the ENROL common data elements?
- Semantic and technical interoperability: are the data elements codified for machine interoperability following international standards? Is there a software in place interoperable with ENROL platform to allow the automatic transfer of data?

- *How is the collaboration with non-EU countries envisaged?*

Cooperation and sharing of data is contemplated with non EU members in two different ways:

- a) by the sharing of pseudonymised data, if these countries ensure the same level of security as the EU countries in terms of GDPR regulation and following the signature of a legal contract
- b) by the sharing of aggregated data (anonymous data), falling out of the GDPR scope.

- *Does ENROL envisage a blanket consent to avoid the re-contacting the patient for different research projects?*

ENROL Informed Consent includes separate statements for consenting for the sharing of data with third parties and for the possibility of being re-contacted for other projects. While patients can directly allow or deny their consent for the re-use of their data gathered in ENROL by other research initiatives with similar objectives to ENROL, re-contacting is more related to Clinical Trials or basic and translational research projects that involve data that is not gathered under the frame of ENROL, ie. Genomic data, or sampling. Accordingly, patients would need to be re-contacted by their physician for their enrolment.

- *Will data be stored centrally?*

Yes, ENROL will have the central platform for the storage and processing of pseudonymised data including all the safeguards for the secure transfer, access and processing of data.

- *How is the collaboration with industry envisaged?*

Terms of collaboration with industry has not been established yet by the EC and the ERNs coordinators working group.

Regarding access to data, industry and/or private bodies will follow the same rules as any other user of data as established by the Steering and Data Access Committee.