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Deliverable 6.1
Report on CPMS activity by ERN-EuroBloodNet members

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1- Introduction of the situation of the CPMS at the beginning of this year

1.1 European Reference Networks and the Clinical Patient Management System

Set-up under the 2011 Directive on Patient Rights in Cross-Border Healthcare, European Reference Networks (ERNs) are virtual networks bringing together medical specialists across Europe to tackle rare or complex diseases and conditions that require highly specialized healthcare and a concentration of knowledge and resources. For the first time, a formal structure of voluntary collaboration between healthcare providers across the EU has been created for the direct benefit of the patient.

The first 24 thematic networks include over 900 highly specialized healthcare units located in more than 300 hospitals of 25 EU countries plus Norway, and cover a wide range of disease groups that became operational in March 2017.

Healthcare providers who are members of ERNs are connected through a dedicated IT platform and, using a variety of telemedicine tools, offer access to expertise and knowledge of multidisciplinary teams, enabling patients suffering from such conditions to receive the best advice for treatment and diagnosis. A fundamental principle of the ERNs is the stipulation that knowledge should travel rather than patients (with the exception of few cases where patients may be referred for treatment in another country). Research is another key element of the ERNs providing a structured framework for joining research efforts across countries, thereby creating a knowledge hub, facilitating translational research and the development of good practice guidelines for diagnosis and care, and supporting cross-border registries. By gathering and analysing a large pool of patient cases, ERNs should contribute to observational studies and clinical trials, leading to new insights into RD and new drug therapies with potentially far-reaching benefits for patients.

In this context, the European Commission DG SANTÉ has provided ERNs with the Clinical Patient Management System (CPMS), a secure web-based application to support the networks in two core tasks:

1. Bringing expert specialised care to all patients in Europe the diagnosis and treatment of rare or low prevalence complex diseases or conditions across national borders: The system allow for virtual consultation across national borders, ensuring that the needed expertise can travel to the patient, instead of the other way around.

2. Keeping de-identified information on clinical data in a registry: to improve future knowledge on RDs, a database will be created with de-identified data of the cases introduced in the system.

In order to be included in the software application, the patient has to give explicit and unambiguous consent to his healthcare provider. This consent form has three boxes: the first concerns consent for sharing data, the second is about consent on the inclusion in the database and the third is about the possibility to be contacted for research purposes. Patients have to sign directly either in the box providing 'I consent' or in the box entitled 'I do not consent'.
First version of the CPMS was released on November 20th 2017 and a pilot phase was undertaken until March 2018. During this first phase all ERNs familiarised themselves with the system in order to assure optimal functionality. After the pilot phase, the CPMS became available for healthcare providers outside ERNs to consult the highly specialised experts within ERNs.

1.2. ERN-EuroBloodNet Operational Helpdesk

ERN-EuroBloodNet encompasses more than 450 Rare Hematological Diseases (RHD) which are very heterogenous in their nature and clinical course e.g. oncological vs non-oncological, acute vs chronic or hereditary vs acquired. Accordingly, customization of CPMS is essential to ensure the efficient use of the platform in terms of technical requirements addressing the RHD needs.
As main action funded under the call CEF TELECOM CALL FOR PROPOSALS 2017 CEF-TC-2017-2 for the project "Connecting EuroBloodNet", an Operational Helpdesk is currently being set up to monitor the functionality of ERN Collaborative Platform (ECP) and CPMS platforms according to ERN-EuroBloodNet needs and support multidisciplinary healthcare teams in the organisation of their daily work regarding cases reviewed by the ERN.
Accordingly, Fahed Ahssini has been hired as the ERN-EuroBloodNet Operational Helpdesk based in the co-coordinator centre of the network, CUB-Hôpital ERASME, Brussels. Specific objectives of Operational Helpdesk include: 1) Establishment of a Help-desk line to support HCPs members in their daily use of the platforms and 2) Identification of technical specification requirements for ECP and CPMS customization according to ERN-EuroBloodNet needs.
In this context, ERN-EuroBloodNet transversal field of action on Telemedicine and "Connecting EuroBloodNet" have establish synergies in order to allow the creation of a symbiosis between both actions for joining efforts while avoiding duplication of efforts.

2-Objectives

One of the key objectives established by ERN-EuroBloodNet is to provide inter-professional consultation by sharing of expertise and safe exchange of clinical information through the Clinical Patient Management System, as the platform supporting European Reference Networks in facilitating the decision making for the diagnosis and treatment of rare disease or low prevalence complex diseases or conditions across national borders.

Accordingly, the specific objectives for this period of network implementation are:

- To gather and report to the EC impressions and the feedbacks of the first ERN-EuroBloodNet CPMS users during the pilot phase of the platform
- To promote the CPMS use among the network members for inter-professional consultation of complex cases

3-Methods

The actions described in this section have been undertaken under the umbrella of the ERN-EuroBloodNet Transversal Field of Action (TFA) on Telemedicine, coordinated by Béatrice Gulbis, the ERN-EuroBloodNet operational helpdesk, Fahed Ahssini, and with the support from the coordination team.

3.1 Pilot testing of the CPMS

CPMS pilot testing took place from November 2017 to March 2018. In this pilot stage ERNs were requested to participate in order to detect potential improvements to be corrected in the next months.

ERN-EuroBloodNet IT and dissemination manager contacted by email ERN-EuroBloodNet members representatives and substitutes providing information on the new release of the platform, its objectives and first steps for its practical use.

Interested members who wanted to participate in the pilot phase received further details on the steps to follow either to enrol a patient, or participate in an open panel.

Once the pilot phase was closed, a questionnaire was produced by the EC in order to be circulated among the pilot users. In order to identify the main areas of improvement and first impressions on the real use of the platform, the questionnaire included the following items:

1) Do you consider that you were sufficiently well prepared in your ERN or HCP to start working with the CPMS? If not, can you please explain why?
2) Do you agree that local administration rights should be attributed to the ERN Coordinator or even on HCP level in order to streamline the access process? If not, can you please explain why?
3) Currently we grant access to CPMS for health professionals in the first place. Do you think that non-health professionals should have access to CPMS under certain conditions? Can you give examples of such non-health professionals? If so, can you briefly describe the tasks and permissions that should be attributed to them?
4) When looking for a patient, were there specific selection criteria that you applied? Can you explain why the specific patient was selected: case, urgency, complexity, other reason? If it weren't to be the pilot phase, would you still have selected the same patient?
5) Did you easily manage to put together a panel of experts to assess your patient file? If not, can you please explain what the difficulty was?
6) How many members were involved in the panel? How many HCPs and how many Member States were represented?

7) Can you inform if the outcome has been prepared and signed-off? If not, why is the assessment still on-going?
8) In case the outcome was signed-off, has the panel been closed? If not, can you please explain why?
9) Can you provide an indication on the duration of the whole process? Would you consider this being a realistic timing?
10) While a number of comments have already been received, do you think that the provided Standardised Consent Form can easily be used for the purpose of ERN patient consultations in your hospital? If not, can you please explain why?
11) If changes needed to be done, do they apply to the layout of the form or rather to the content?
12) Please provide any other comments you may have or suggestions that can help us for further improvement of the CPMS.

The questionnaire was circulated among the ERN-EuroBloodNet participants in the pilot phase. Answers were compiled by the IT dissemination manager and reported to the EC by 15 March 2018.

3.2 ERN-EuroBloodNet strategy for promoting the wide implementation of the CPMS

In order to ensure an efficient and effective implementation of the CPMS, ERN-EuroBloodNet has identified as first critical points to

1. Have a sufficient number of active users willing to provide advice in the open panels
2. Ensure the most adequate classification of RHD in the “Preferences” area as the key step for users to be selected as panel members based on their expertise

An ERN-EuroBloodNet strategy for the promotion of the use of CPMS was defined accordingly including:

<u>3.2.1 CPMS promotion and awareness among members</u>

Dedicated efforts have been focused on increasing the awareness on the CPMS and its rationale between healthcare professionals community in order to increase the number of users ensuring full disease and country coverage. Broader dissemination among ERN-EuroBloodNet members have been performed through:

- Informative bulletins via email, ie. presentation of the CPMS, procedure to get an access account…

- Dedicated sessions at ERN-EuroBloodNet face to face meetings: the Scientific and Strategic Board meetings and Board of the Network meetings.
- Dedicated slides at ERN-EuroBloodNet general presentations at European/National Congresses.

3.2.2 Upgrade of RHD categories in the "Preferences" section

Disease Categories

The first step for all users in the CPMS is the selection of their area of expertise in the "Preferences" section. This represents a crucial step since users can be filtered to participate in a panel based on the preferences selected. Accordingly, ERN-EuroBloodNet identified the upgrade of the RHD classification available in the "Preferences" section as the first action to be performed for ensuring its efficient use.

ERN-EuroBloodNet CPMS helpdesk and coordination team prepared a template including a new proposal of disease categories for each subnetwork based on ORPHA classification. A template was circulated among the Subnetworks coordinators to provide their feedback including:

- The new proposal of the Categories for each subnetwork based on ORPHA classification
- A section to score each of the Categories from 0-5 to rank the need of the CPMS for each category (0- CPMS not needed, 5- CPMS highly needed).
- A section to indicate the type of advice more required for each of the Category: clinical care/diagnosis/both
- A section to indicate if advice more required for pediatrics/adults/both for each category
- A section for the identification of one or two reference persons for each category

New category for highly specialized procedure - Bone Marrow Transplantation for oncological and non ongological disorders

The expertise required for the performance of Bone Marrow Transplantation (BMT) is highly disease-specific dependent in the RHD area. Thus, for instance, Expertise for Sickle cell disorder BMT transplantation is very different from the expertise required for Myeloid malignancies.

Accordingly, a new category will be proposed to be included in the CPMS preferences for non oncological and oncological disorders. This new proposal was included as well in the template to gather the point of view from the different experts.

4-Results

4.1 Pilot testing of the CPMS

A total of 12 ERN-EuroBloodNet participated in the pilot phase, with the enrolment of 6 patients.

A report was provided to the EC including the answers received from the questionnaires. **Annex I ERN-EuroBloodNet feedback from the CPMS pilot phase.**

4.2 ERN-EuroBloodNet strategy for promoting the wide implementation of the CPMS

4.2.1 CPMS promotion and awareness among members

As a result of the CPMS promotion, the state of the art of the CPMS is the following:

a) Numbers of panels

A total of 12 panels have been opened for RHDs since the launch of the CPMS. Table 1 summarizes the number of cases introduced per thematic area and advice required

Thematic area	Diagnosis	Treatment	Diagnosis and treatment	**Total**
Haemoglobinopathy	1		1	**2**
Hereditary erythroenzymopathies and RBC membrane defects	2			**2**
Congenital dyserythropoietic anemia, Blackfan-Diamond anemia, Acquired BMF and Inherited BMF	2	1		**3**
Myelodysplastic syndrome (MOS)		2	2	**4**
Rare lymphomas		1		**1**
Total	**5**	**4**	**3**	**12**

Table 1. Panels by thematic area and advice required

Attending to the number of cases entered in the CPMS, the higher number of panels entered have been for the advice on Myelodysplastic syndromes, representing 4 out of the 12 cases entered. Regarding the type of advice requested, there are a quite balanced number of cases entered for diagnosis and treatment, being 5 and 4 respectively, and 3 for both of them.

Nevertheless, if thematic areas are compiled according to the oncological and non-oncological hubs existing in ERN-EuroBloodNet, the following analysis is obtained:

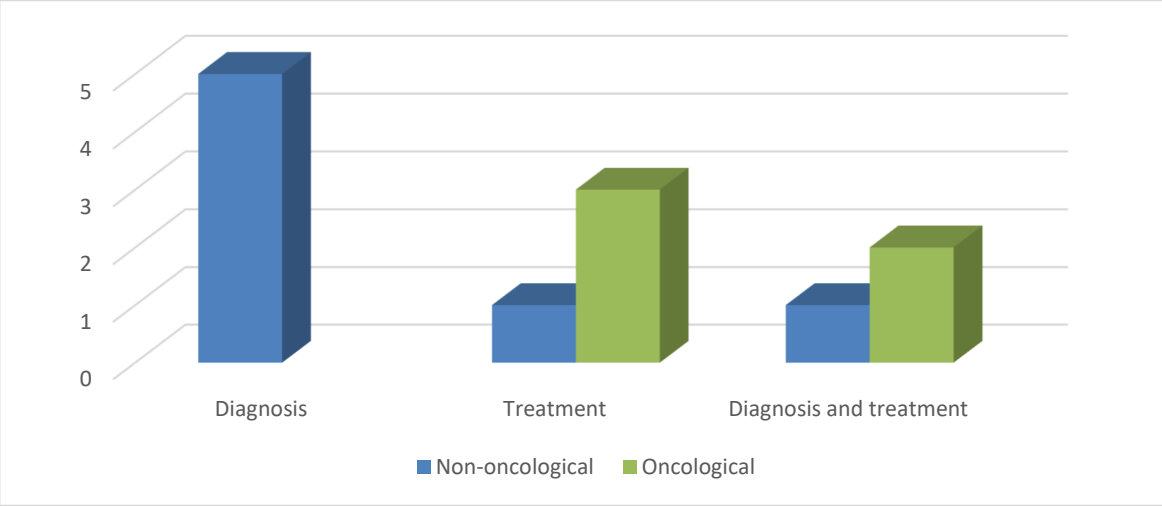

Fig 1. Number of panels by type of advice and oncological or non-oncological hub

By compiling the cases according the oncological or not-oncological diseases, a significant difference is appreciated concerning the needs of advice required. For the non-oncological disorders an important need is identified for the Diagnosis advice representing 5 of the 7 cases entered, plus one case for both diagnosis and treatment. In contrast, treatment advice is more required for oncological disorders, being 3 of the 5 cases entered, while the other 2 are for both, diagnosis and treatment. No case has been entered just for diagnosis advice on the oncological disorders.

The number of panels according to the phase in the flowchart are represented in table 2:

Panel stage	Nr.
Closed	2
Aborted	1
Open	3
Panel Selection	2
Data Completion	1
Assessment	2
Sign-off	1

Table 2. Number of cases by panel stage

According to the phase of the panels, it is important to highlight the high number of panels stuck in the Open phase, Panel selection or data completion, representing half of the panels introduced in the CPMS. In addition, considering also the time or response varying from around 1 month to

more than 6 months, the role of the panel manager to assist in the development to the case on the flowchart becomes cornerstone for the success on the provision of advice.

b) Number of CPMS account created for the EuroBloodNet ERN members

The number of haematological experts who have a CPMS account increased from 12 (March 2018) to 55 (February 2019).

4.2.2 Upgrade of RHD categories in the "Preferences" section

A total of 6 experts participated in the first analysis of the preferences upgrade. Some of the coordinators provided their view on more than one subnetworks. In these cases, the media of the answers was calculated for the analysis of the results.

All the answers and contributors in **Annex II Results from Preferences section upgrade**

CPMS need - Analysis by subnetworks

Fig. 2 illustrates the need of the CPMS by subnetwork. For those subnetworks with more than one answer, the media of the answers have been calculated for the analysis of results. RBC: Red Blood Cell, BMF: Bone Marrow Failures, Bleeding: Bleeding and coagulation disorders, HH and Iron: Hereditary Hemochromatosis and Iron metabolism disorders, Lymphoid: Lymphoid malignancies, Myeloid: Myeloid malignancies.

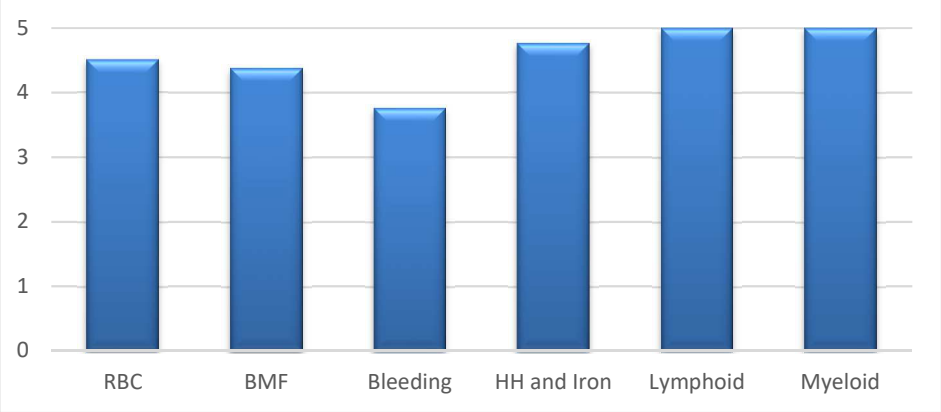

Fig. 2 CPMS need by subnetwork

According to subnetworks coordinators opinion, the CPMS is most required for the oncological disorders, where the need was total (5) for Lymphoid and Myeloid subnetworks. Regarding the non-oncological hub, the highest need was identified for the Hemochromatosis and other rare genetic disorders of iron metabolism and heme synthesis subnetwork (4,75), which encompasses

a great number of very rare disorders that in many occasions require from a second advice from different multidisciplinary team for the correct management of the patients. Also a similar result was obtained for the Red blood cell and bone marrow failure subnetworks (4,5 and 4,375 respectively). On the other hand the lowest need identified was assigned to the Bleeding and coagulation subnetwork (3,75).

The analysis of the type of advice and age of patients for which the CPMS is most required has been performed by category (see below: CPMS need – Analysis by Category).

CPMS need - Analysis by Category

The media of the answers regarding the need of the CPMS according to the categories for each subnetwork are represented in the following figures:

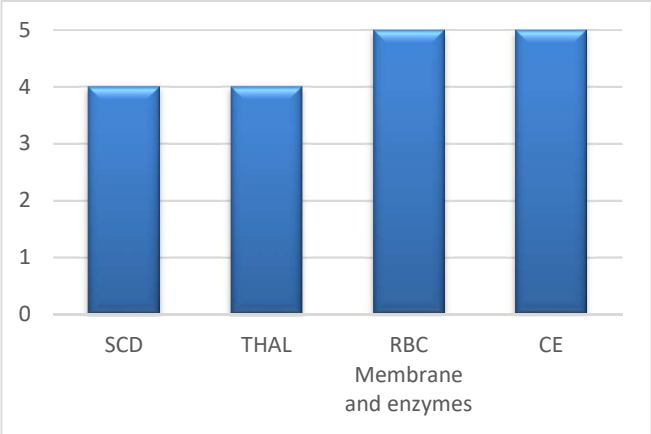

Fig. 3 CPMS Need for Red blood cell subnetwork categories. SCD: Sickle cell disorders, THAL: Thalassaemia syndromes, Red Blood Cell membrane and enzyme disorders, CE: Congenital Erythrocytosis.

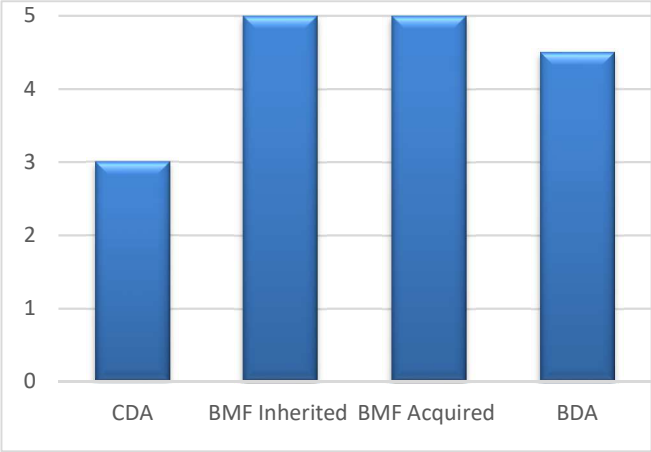

Fig. 4 CPMS Need for bone marrow failure subnetwork categories. CDA: Congenital Dyserythropoietic anaemia. BMF Inherited: Fanconi anemia, Dyskeratosis congenital, GATA2 syndrome,Congenital amegakaryocytic thrombocytopenia and others. BMF Acquired: Aplastic Anaemia and Paroxysmal Nocturnal Hemoglobinuria, BDA: Blackfand-Diamond Anaemia.

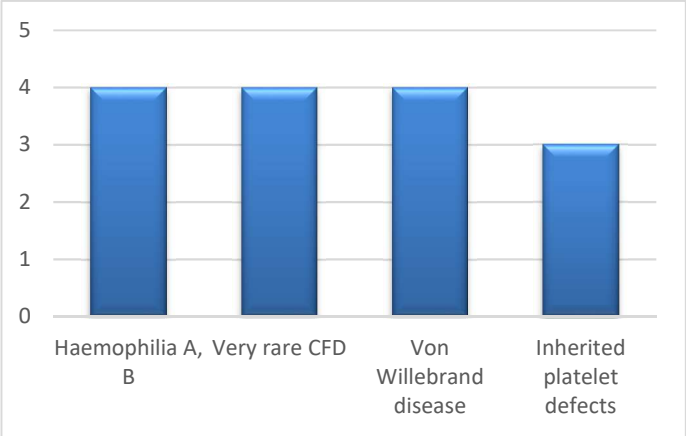

Fig. 5 CPMS Need for bleeding-coagulation subnetwork categories. Very rare CFD: The rarer congenital deficiencies of other coagulation factors (fibrinogen and factors II, V, VII, X, XI and XIII)

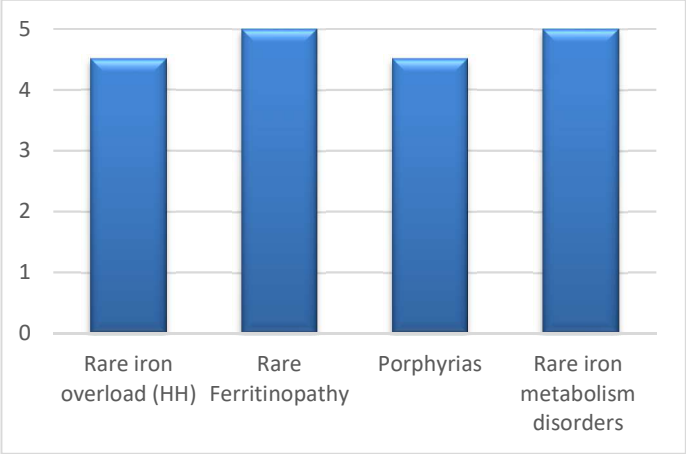

Fig. 6 CPMS Need for Haemochromatosis and rare iron metabolism and heme synthesis subnetwork categories. HH: Hereditary Hemochromatosis.

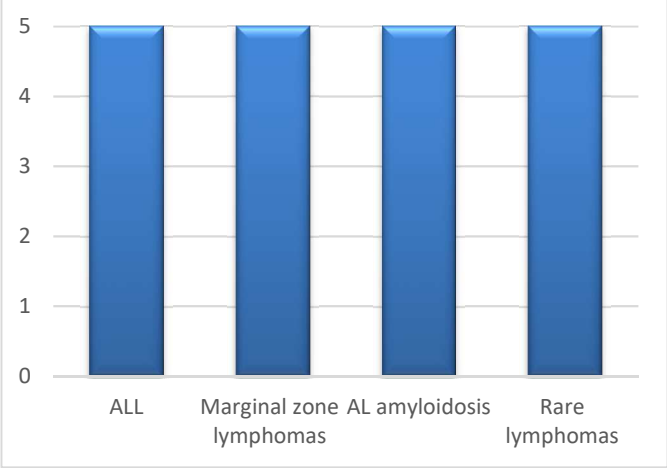

Fig. 7 CPMS Need for Lymphoid subnetwork categories. ALL: Acute lymphoblastic leukemia

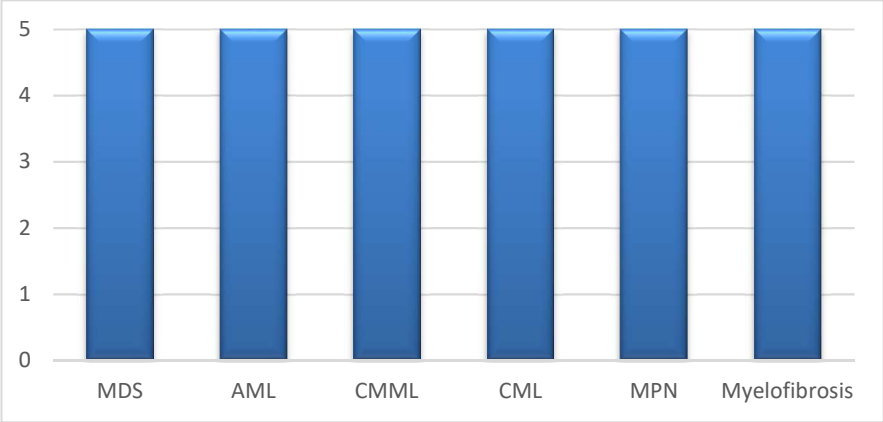

Fig. 8 CPMS Need for Myeloid subnetwork categories. MDS: Myelodysplastic syndrome, AML: Acute myeloid leukemia, CMML: Chronic myelomonocytic leukemia, CML: Chronic Myeloid Leukemia, MPN: Myeloproliferative neoplasm.

Regarding the need of the CPMS by category of disorders, it is important to highlight the difference between the answers provided by two experts on the BMF subnetworks in the Congenital dyserythropoietic anemia, being rated as essential for one expert (5) and not necessary by another (1). A similar case was also given for two answers on the Bleeding and coagulation disorders, where one expert rated the CPMS as highly necessary (5), and another expert finds it not so needed (3).

Regarding the type of advice for which CPMS is most required for each category, there is an unanimous opinion that the platform is needed for both, clinical care and diagnosis, with the only exception found by one expert on Rare Ferritinopathy, who highlighted its need for the Diagnosis.

Regarding the age of the patients for which CPMS is most required, paediatrics and adults advice have been identified the most needed for Red blood cell, bone marrow failure and bleeding and coagulation subnetworks. For the Haemochromotaosis and rare iron metabolism and heme synthesis subnetwork, the major need was identified for paediatrics and adults by one expert, while only for adults for another expert from their point of view. On the oncological hub the major need found was for adults, with the exception of Acute lymphoblastic leukemia (ALL) and Acute myeloid leukemia (AML), where the need was also highlighted for children.

New category for highly specialized procedure - Bone Marrow Transplantation for oncological and non ongological disorders

The feedback on this new category was only received from the Bone Marrow Failures subnetworks coordinators, accordingly a general analysis for the non-oncological and oncological perspective has not been possible to be performed. Nevertheless this task is ongoing so a future analysis will be undertaken in the next period of the network.

5- Challenges faced

Wide implementation of the CPMS is in itself a challenge, considering that medical community has to make a U-turn from an “easy” email exchanging with colleagues to a more “complex” and structured system. At this moment the CPMS is not well known among the experts of ERN-EuroBloodNet even if the promotion has started.

Based on the first cases introduced by ERN-EuroBloodNet, technical difficulties have been experienced by some users to success on the login and first access to the platform. Moreover, some experts refused to create a CPMS account as they considered the procedure is quite cumbersome despite of the documentation produced by the EC for guiding the process. Some of the connection issues identified were:

- The authentication step (login process) is not really well understood by the users
- PDFs and webinars about the steps to follow for connection are found too long for some users.

This impression is shared by other users that in addition consider the procedure to enrol patient and follow the flowchart a heavy procedure as well.

On the other hand, some barriers need to be contemplated and addressed on the light of European Commission and National authorities’ steps towards the wide implementation of CPMS, e.g. National legal authorizations for its use, user’s role (guest, expert), compensation of time spent by experts and legal responsibility.

6- Conclusions and next steps

CPMS represents an excellent tool for the exchange of RD complex cases, nevertheless it is a brand new platform that require a) further promotion by ERN community, b) feedback from users for its technical improvements.

In this context, ERN-EuroBloodNet have dedicated special efforts during its second year of implementation to:

a) Increase the number of users as basis for the smooth running of the cases

b) Adapt the categorization of RHDs in the Preferences area according to the RHD needs as the key step to select properly the contributors to the panel.

On this last point, the first analysis for the upgrade of RHD categories for the Preferences has provided important information on the needs from the experts' point of view and shed light to continue working on this area. Based on the experts identified for each category, their perspective on the categorisation and needs will be required in order to gather a major evidence for the performance of the categories customization.

On the other hand, based on the first cases enrolled in the platform, ERN-EuroBloodNet has also identified the main technical issues that have difficulted the access of new users to the platform. Based on this, a plan will be defined in order to ease these steps. Some of the actions contemplated will be:

- Report the EC the technical difficulties found and propose improvements in the system
- Produce of a shorter guide and FAQ questions to facilitate the access and navigation through the CPMS
- Analyse the possibility of holding webinars for short group of ERN-EuroBloodNet users
    - To explain how to create the account in "real time"
    - How to start using the platform

Annex I

ERN-EuroBloodNet feedback from the CPMS pilot phase

List of ERN-EuroBloodNet experts who participated in the CPMS Pilot phase

By alphabetic order:

Pierre Fenaux , Assistance Publique-Hôpitaux de Paris, Hôpital Saint-Louis, France

Gian Luca Forni, E.O. Ospedali Galliera, Italy

Anne Sophie Kubasch, Universitätsklinikum Carl Gustav Carus, Germany

Tabita Magalhaes Maia, Centro Hospitalar e Universitário de Coimbra, EPE, Portugal

Giacomo Marchi, AOUI Verona, Italy

Antonio Piga, AOU S.Luigi Gonzaga, Italy

Graça Porto, Centro Hospitalar do Porto, EPE, Portugal

Eduard van Beers. University Medical Center Utrecht, the Netherlands

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Unit B3 Cross border healthcare, eHealth

QUESTIONNAIRE TO ERN COORDINATORS ON THE PILOT PHASE OF THE CPMS

1. Background:

On 20 November 2017, the Clinical Patient Management System (CPMS) has been released. At the WebEx conference in November on the release of the CPMS it was highlighted that the pilot phase will be an important part of the roll-out plan. This phase will last until end of February 2018. It is an opportunity for users to gain experience in the system with real clinical data and with real consultations. During that meeting the Commission services were asked to provide a template questionnaire that can be completed by the users in the ERN.

ERN Coordinators are asked to collect and consolidate the feedback and **send them to the Commission by 15 March 2018**.Replies can be sent to SANTE-COORDINATORS-ERN@ec.europa.eu.

2. Scope:

The scope of this questionnaire does not include technical feedback or new feature requests for the clinical patient management system, this will be addressed through existing channels and an opportunity for more comprehensive feedback will be made available in quarter 2 of 2018. The scope of this questionnaire is to review and analyse your business process needs.

3. Questions:

a. Information on the ERN

1) ERN name:**ERN-EuroBloodNet (ERN on Rare Hematological Diseases)**

b. Launch of the CPMS

1) Do you consider that you were sufficiently well prepared in your ERN or HCP to start working with the CPMS? If not, can you please explain why?

"CPMS guide is clear and platform is pretty intuitive."

"I think the CPMS is completely unknown in my HCP and in the ERN. We are not prepared to use CPMS, because it is a completely new option that did not exist before. A lot of physicians are completely unknown about this option to ask peers for advise. On the other hand; HCPs in C of E, maybe think they don't need it. The question remains, DO WE REALLY NEED CPMS"

"Yes"

"Yes"

"Yes"

c. Approval touse CPMS by your ERN Coordinator

Before getting access to CPMS, your ERN Coordinator after consultation by the Commission services must approve the request.

1) Do you agree that local administration rights should be attributed to the ERN Coordinator or even on HCP level in order to streamline the access process? If not, can you please explain why?

"We think the access should be extended to healthcare professionals, other to ERN Coordinator, involved in clinical case, in order to give their contributions and allow a complete overview on the case."

"I think it could help that local administration rights are attributed to CoE HCP level. It makes them more involved and they will notice that when no HCP registers for the CPMS there is a job to do on informing about CPMS"

"Yes"

"Yes"

"Yes"

2) Currently we grant access to CPMS for health professionals[1]in the first place. Do you think that non-health professionals should have access to CPMS under certain conditions? Can you give examples of such non-health professionals? If so, can you briefly describe the tasks and permissions that should be attributed to them?

"According to our experience on CPMS until now, it is not necessary. It could be useful in the future if the platform will be provided with supplementary fields, not strictly clinical. For example, it could be useful to have a contribution by a psychologist or a biologist or a pharmacist."

"I think that supporting personnel (data managers e.g.) could help getting the patient data in the CPMS to decrease the administrative burden of the HPs. "

"For the current characteristics of the platform, I think that only health-professionals have a suitable preparation for describing or giving advice to such complex cases"

"No, I think that access to CPMS data should be given to health professionals only"

"Yes, I think they should have access to CPMS. For example, for the ERN EuroBloodNet, the test of the insertion of the first patient was done by Mariangela Pellegrini, ERN manager and Coordinator Pierre Fenaux assistant. Her tasks were: to collect the patient's consent, to insert patient data in the CPMS, to ask to Saint Louis hospital to anonymise the patient exams, to check if the viewer of radiology worked. Finally she passed the lead of the panel to Pierre Fenaux."

d. Selection of patient

For this pilot phase, ERNs were invited to enrol at least 5 patients per ERN in order to have a meaningful experience with the system.

1) Can you confirm you easily found and enrolled 5 patients? If not, can you please explain why and indicate how many patients you enrolled in the end?

[11] Health professional: a doctor of medicine, a nurse responsible for general care, a dental practitioner, a midwife or a pharmacist within the meaning of Directive 2005/36/EC, or another professional exercising activities in the healthcare sector which are restricted to a regulated profession, or a person considered to be a health professional according to the legislation of the Member State of treatment.

5 patients in total have been enrolled during the pilot phase for the ERN.

2) When looking for a patient, were there specific selection criteria that you applied? Can you explain why the specific patient was selected: case, urgency, complexity, other reason? If it weren't to be the pilot phase, would you still have selected the same patient?

"Our selection criteria are based on complexity of the case, questions not resolved and clinical interest. If it weren't to be the pilot phase, we still have selected the same patient."

"I would have selected this patient again because it was severe enough to put a lot of effort in, and the case was complex enough that I had the Idea I couldn't be solved by my CoE alone"

"I think that the major criteria is complexity and the need of an expert advice (also in a non-pilot phase)"

"I selected a patient for complexity. Yes, if it weren´t the pilot phase I would still select the same patient"

"Patients were selected according to pilot phase. First criteria was to pick patients according to certain medical exams done (e.g. PET SCAN) in order to verify the right compatibly between the hospital viewer and the CPMS viewer. In a non-pilot phase, patient would have picked according to complexity case, asking for a second advice."

e. Panel set-up

1) Did you easily manage to put together a panel of experts to assess your patient file?If not, can you please explain what the difficulty was?

"We easily manage the panel of expert selection, but at the moment they were very few."

"The strength of CPMS should be that you get advise from people you don't know. Why bother to insert a patient in CPMS if I just can call my friends in Barcelona about PK thermostability. I want advise from unexpected corner. E.G. a hemotologist from Germany I don't know of who is an expert on T-LGL and suggest to do an T-celflowcytometry analysis (which solved the case). The experts shouldn't be hand picked, by the one who asked advise in diagnostic cases. For treatment advise this is different"

"I have participated as a contributor for a patient of another center"

"No, I wanted to invite a specific colleague with whom I had already discussed the case , but she never appeared in the list of experts"

"Not applicable"

1) How many members were involved in the panel? How many HCPs and how many Member States were represented?

“For the first case we uploaded, there were 3 members, of 3 different members states.For the second one, there was one more expert, from a fourth member state.”

“3 and 3 (the HCP/MS who presented the case and two experts where invited to give advise”

“5 HCPs of 4 MS”

“Not applicable”

“Not applicable”

2) Was there a need to consult experts from non-ERN HCPs or from other ERNs?

At this pilot phase no experts out of the ERN or from other ERNs have participated in the panels.

f. Consultation

1) Once the panel was established, how many meetings were held?

No meetings have been held during this phase.

g. Outcome

After the assessment has been completed and contributions from the panel members have been recorded, the outcome of the consultation can be prepared and 'signed-off'.

1) Can you inform if the outcome has been prepared and signed-off? If not, why is the assessment still on-going?

“Our cases are at the Assessment level, so we did not arrived yet at sign-off. It would be useful to have a printable report also at temporary levels.”

“Yes, it was signed off”

“The case in which I'm involved is still ongoing because the other contributors haven't recorded their advice yet.”

“I didn´t reach this point for the reason above (I did not find the expert I wanted to invite)”

“Not applicable”

h. Panel Closure

1) In case the outcome was signed-off, has the panel been closed? If not, can you please explain why?

“Not applicable”

“It is pending to be signed off”

“The case in which I'm involved is still ongoing”

“Not applicable”

“Not applicable”

2) Can you provide an indication on the duration of the whole process? Would you consider this being a realistic timing?

“Not applicable”

“The expert panel didn’t give me any advise so, In this case CPMS really didn’t work”

“The case in which I'm involved is still ongoing”

“Not applicable”

“I cannot provide the duration of the whole process, but 2 hours per patient to insert data and medical analysis. However it was the first attempt to the use of CPMS, so slower.”

i. Standardised Consent Form

The Standardised Consent Formuploaded in CPMS aims to make the consent given legally acceptable under Directive 95/46/EC[2] and the GDPR.[3]In its Opinion, the EDPS has also considered the Standardised Consent Form as a "best practice".As some variations may exist between the Member States in their implementation or interpretation of particular requirements of Directive 95/46/EC and the GDPR, healthcare providers may need to adapt the Standardised Consent Form, adding those elements that would ensure full compliance with national law, and, in particular, with relevant data protection provisions.

2Directive 95/46/ECof the European Parliament and of the Council of 24 October 1995 on the protection of individuals with regard to the processing of personal data and on the free movement of such data.

[3]Regulation (EU) 2016/679 of the European Parliament and of the Council of 27 April 2016 on the protection of natural persons with regard to the processing of personal data and on the free movement of such data, and repealing Directive 95/46/EC (General Data Protection Regulation).Please note that the GDPR will be applicable as from 25 May 2018.

1) While a number of comments have already been received, do you think that the provided Standardised Consent Form can easily be used for the purpose of ERN patient consultations in your hospital?If not, can you please explain why?

"We think the Standardised Consent Form is easy to use in the clinical practice and understandable for patients."

"I think it is ok. However if the data will be used for research purposes instead of clinical purposes/advise it needs to be approved by at least one Dutch METC and all local quality co-ordinators."

"Yes"

"Yes"

"Yes. On the other hand the difficulty was that the patient signed a lot of consent forms in the moment he/she is hospitalized. It was difficult to make them accept to sign another additional consent for a not yet well-known project. "

1) If changes needed to be done, do they apply to the layout of the form or rather to the content?

"We did not need to do any change."

"Content"

"Not applicable"

"Not applicable"

"Both layout and content of ERN consent Form fit with French one used in the hospital/HCP."

j. Other comments or suggestions

Please provide any other comments you may have or suggestions that can help us for further improvement of the CPMS.

"We suggest to open the possibility of contribution in cases also for experts not registered on CPMS (but members of ERN)."

"I think the access to CPMS should be easier. The documentation should be less extensive. The whole process of entering data should be easier."

"No comments"

“No comments”

“ 1. CPMS Should provide a tool for anonymize the medical exams and analysis. It is indeed an overload for HCP workers and it is slow and not very easy to get.

2. Check that the RHD category are exhaustive

3. Translation of medical documents”

Annex II

Results from Preferences section upgrade

List of ERN-EuroBloodNet experts who participated in the upgrade of RHD categories in the "Preferences" section

By alphabetic order:

Pierre Fenaux, Assistance Publique-Hôpitaux de Paris, Hôpital Saint-Louis, France

Regis Peffault de la Tour, Assistance Publique-Hôpitaux de Paris, Hôpital Saint-Louis, France

Flora Peyvandi, Fondazione IRCCS CA'Granda Ospedale Maggiore Policlinico , Italy

Graça Porto, Centro Hospitalar do Porto, EPE, Portugal

Dorine Swinkels, Radboud University Medical Center Nijmegen, the Netherlands

Eduard van Beers, University Medical Center Utrecht, the Netherlands

	Score need for CPMS (0-5)	Type of advice needed: clinical care/diagnosis/Both	Advice more related to pediatrics/Adults/Both
Red Blood Cell disorders	**4,5**		
Sickle cell disorders	4	Both	Both
Thallassaemia disorders	4	Both	Both
Hereditary erythroenzymopathies and RBC membrane defects	5	Both	Both
Congenital Erythrocytosis and other rare RBC defects	5	Both	Both
Bone marrow failures and related disorders - Answer 1	**3,75**		
Congenital dyserythropoietic anemia	1	Both	Both
BMF Inherited (Fanconi anemia, Dyskeratosis congenital, GATA2 syndrome,Congenital amegakaryocytic thrombocytopenia and others)	5	Both	Both
BMF Acquired (Aplastic Anaemia and Paroxysmal Nocturnal Hemoglobinuria)	5	Both	Both
Blackfan-Diamond anemia	4	Both	Both
Bone marrow failures and related disorders - Answer 2	**5**		
Congenital dyserythropoietic anemia	5	Both	Both
BMF Inherited (Fanconi anemia, Dyskeratosis congenital, GATA2 syndrome,Congenital amegakaryocytic thrombocytopenia and others)	5	Both	Both
BMF Acquired (Aplastic Anaemia and Paroxysmal Nocturnal Hemoglobinuria)	5	Both	Both
Blackfan-Diamond anemia	5	Both	Both
Bleeding and coagulation disorders - Answer 1	**3**		
Haemophilia A, B	3	Both	Both
The rarer congenital deficiencies of other coagulation factors (such as fibrinogen and factors II, V, VII, X, XI and XIII)	3	Both	Both
Von Willebrand disease	3	Both	Both
Inherited platelet defects	3	Both	Both
Bleeding and coagulation disorders - Answer 2	**4,5**		
Haemophilia A, B	5	Both	Both
The rarer congenital deficiencies of other coagulation factors (such as fibrinogen and factors II, V, VII, X, XI and XIII)	5	Both	Both
Von Willebrand disease	5	Both	Both
Inherited platelet defects	3	Both	Both
Haemochromotaosis and rare iron metabolism and heme synthesis - Answer 1	**5**		
Rare iron overload (hereditary hemochromatosis)	5	Both	Both
Rare Ferritinopathy	5	Both	Both
Porphyrias	5	Both	Both
Rare iron metabolism disorders (sideroblastic and non-sideroblastic)	5	Both	Both
Haemochromotaosis and rare iron metabolism and heme synthesis - Answer 2	**4,5**		
Rare iron overload (hereditary hemochromatosis)	4	Both	Adults
Rare Ferritinopathy	5	Diagnosis	Adults
Porphyrias	4	Both	Adults
Rare iron metabolism disorders (sideroblastic and non-sideroblastic)	5	Both	Adults
Lymphoid malignancies	**5**		
Acute lymphoblastic leukemia (ALL)	5	Both	Both
Marginal zone lymphomas	5	Both	Adult
Light chain Amyloidosis (AL amyloidosis)	5	Both	Adult
Rare lymphomas (hairy cell leukamia…)	5	Both	Adult
Myeloid malignancies	**5**		
Myelodysplastic syndrome (MDS)	5	Both	Adult
Acute myeloid leukemia (AML)	5	Both	Both
Chronic myelomonocytic leukemia (CMML)	5	Both	Adult
Chronic Myeloid Leukemia (CML)	5	Both	Adult
Myeloproliferative neoplasm (MPN)	5	Both	Adult
Myelofibrosis	5	Both	Adult
Systemic mastocytosis			
BMT			
Non oncological disorders	5	Both	(Answer for BMF)
Oncological disorders	5	Both	(Answer for BMF)