European
Reference
Network
for rare or low prevalence
complex diseases

Network
Vascular Diseases
(VASCERN)

14.00-16.00: VASCERN BOARD MEETING, chaired by Prof Guillaume Jondeau, Coordinator

Requested: 1 representative per HCP (31) & ePAG Chairs (6)
Open to all participants without voting rights

- **Terms of reference for validation by the board (Marine Hurard)**
- Priorities for Work Packages
  - RDWG
  - ePAG
  - Transversal WG
- Minimal participation to videoconferences & CPMS
- Funding
- New ERN Call for new ERN members (December 2017):
  - Criteria for each RDWG to be revised after the experience of the first year, for the new members
- Participation in interERN WG
  - Reports from Guillaume Jondeau (Monitoring), Romain Alderweireldt (Ethics), Xavier Jeunemaître (Research)
  - RD-Action work for interERNs cooperation (Victoria Hedley)
- Criteria for an event to be endorsed by VASCERN
- 2018 annual meeting: date (Doodle: 12-13-14 October 2018 (Thursday-Fri or Fri-Sat))
- Questions

16.00-16.15: coffee break
Photo booth (Nicolas Ledoux)

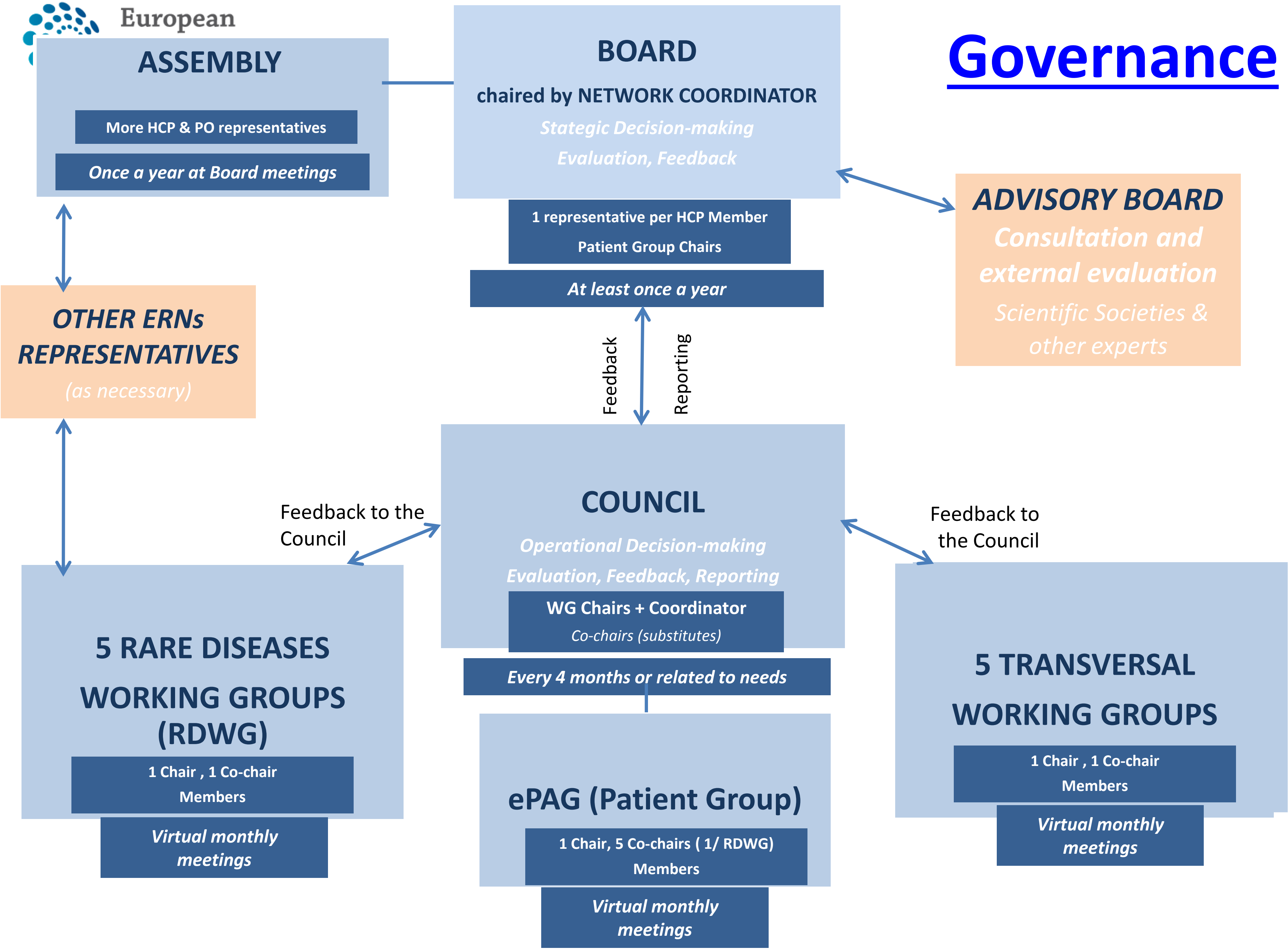
16.15-16.40**: Registry: the FAIR principle** (Marco Roos) (25’)

16.40-17.00: **Debriefing in plenary** (20’)

17.00-18.00: **Patient Group (ePAG) separate meeting** (60’)

European

Governance



ASSEMBLY

More HCP & PO representatives

Once a year at Board meetings

BOARD

chaired by NETWORK COORDINATOR

Stategic Decision-making

Evaluation, Feedback

1 representative per HCP Member

Patient Group Chairs

At least once a year

ADVISORY BOARD

Consultation and external evaluation

Scientific Societies & other experts

OTHER ERNs REPRESENTATIVES

(as necessary)

Feedback

Reporting

COUNCIL

Operational Decision-making

Evaluation, Feedback, Reporting

WG Chairs + Coordinator

Co-chairs (substitutes)

Every 4 months or related to needs

Feedback to the Council

Feedback to the Council

5 RARE DISEASES WORKING GROUPS (RDWG)

1 Chair , 1 Co-chair

Members

Virtual monthly meetings

ePAG (Patient Group)

1 Chair, 5 Co-chairs (1/ RDWG)

Members

Virtual monthly meetings

5 TRANSVERSAL WORKING GROUPS

1 Chair , 1 Co-chair

Members

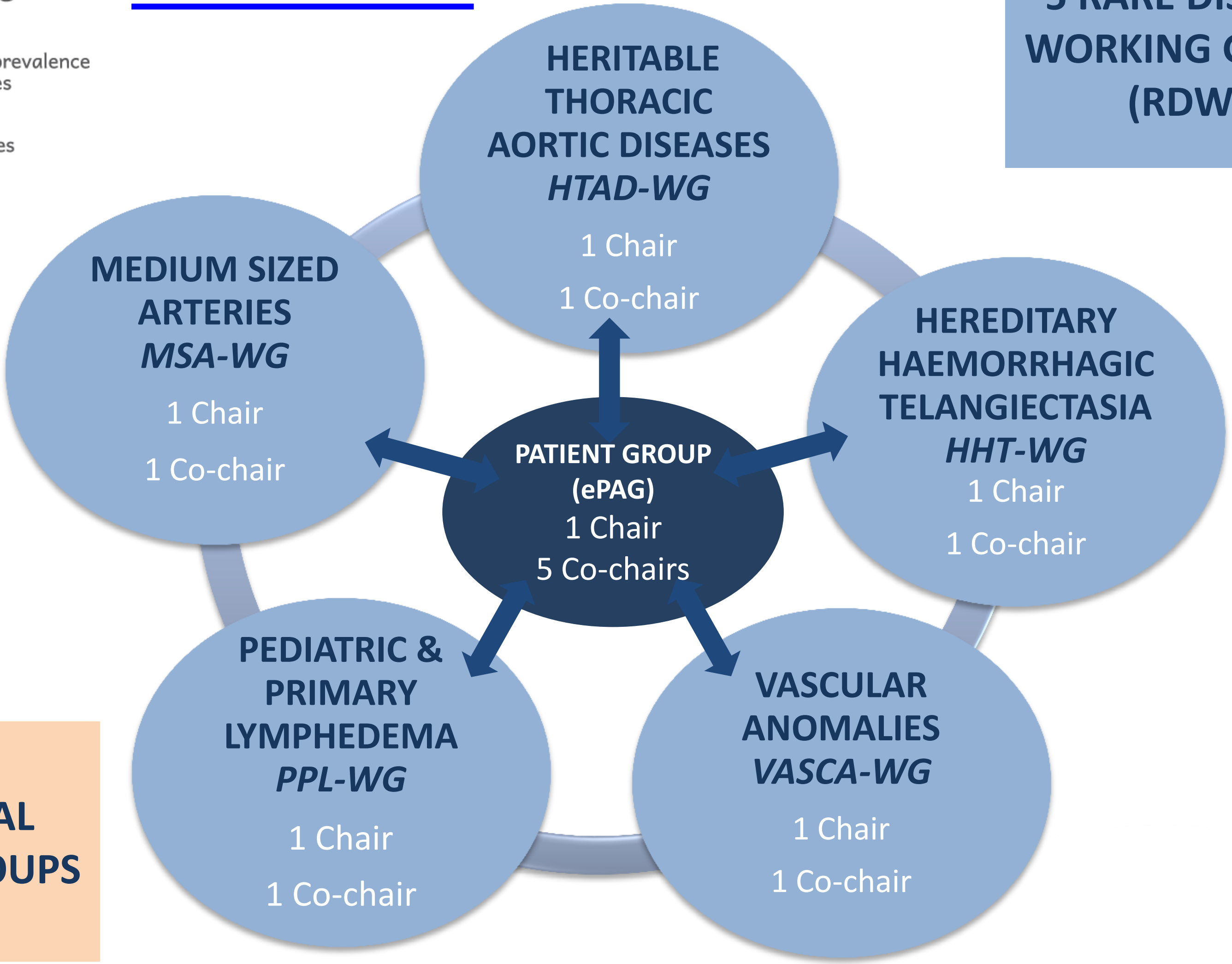
Virtual monthly meetings

European Reference Network
for rare or low prevalence complex diseases

Network Vascular Diseases (VASCERN)

Structure

5 RARE DISEASES WORKING GROUPS (RDWG)



HERITABLE THORACIC AORTIC DISEASES *HTAD-WG*
1 Chair
1 Co-chair

MEDIUM SIZED ARTERIES *MSA-WG*
1 Chair
1 Co-chair

PATIENT GROUP (ePAG)
1 Chair
5 Co-chairs

HEREDITARY HAEMORRHAGIC TELANGIECTASIA *HHT-WG*
1 Chair
1 Co-chair

PEDIATRIC & PRIMARY LYMPHEDEMA *PPL-WG*
1 Chair
1 Co-chair

VASCULAR ANOMALIES *VASCA-WG*
1 Chair
1 Co-chair

TRANSVERSAL WORKING GROUPS

Patient Registry WG
1 Chair
1 Co-chair

eHealth
1 Chair
1 Co-chair

Training & Education
1 Chair
1 Co-chair

Ethics WG
1 Chair
1 Co-chair

Communication WG
1 Chair
1 Co-chair

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Network
Vascular Diseases
(VASCERN)

Terms of Reference
Information & Transparency

- Website: www.VASCERN.eu
- Info on Members, structure, governance, WGs, activities, area of expertise, scope, contact
- Dissemination of guidelines, Pills of Knowledge, Publications, etc.
- To be updated, improved and new sections added!
- Communications tools (Newsletters, social networks, collaborative platform,…)

Work within VASCERN

- 1) Issue of internal network communication
- 2) Issue of communication: number of mails, time…

Chairs: work with Coordination team for

- Meetings organisation
- MoM review & dissemination
- Regular WG reporting to Council & Board

Solutions: eHealth collaborative platforms

- All documents in the ERN Collaborative platform
  - For minutes of meetings: sent for review and upload to the coordinating team who do the job ? Same process for all chairs?
  - Other documents go through the coordination team?
  - Start using Forum for exchanging within WGs?

- Mail to remind meetings
  - in the morning (?)
  - By the coordinating team ?
  - Shared agenda with all links for the meetings

- Monthly Videoconference
  - Strict timing
  - Systematic report
  - New system for reporting attendance in the minutes of meetings will soon be proposed

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HHT-WG

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HHT WG PROGRESS AGAINST DELIVERABLES PLANNED - 2017

Deliverables	M1 03-2017	M2 04-2017	M3 05-2017	M4 06-2017	M5 07-2017	M6 08-2017	M7 09-2017	M8 10-2017	M9 11-2017	M10 12-2017	M11 01-2018	M12 02-2018	PLANS FOR M12 DICUSSED WG#7
Clinical cases*			1. PAVM & anticoag/antiplatelets	2. PAVM and embolization vs. surgery	3. HAVM decompensated management	4. HAVM post transplant and hepatic artery thrombosis	5. GI Bleeding & antiplatelets	6. Large HAVM ? surgery	7. Diffuse PAVMs			Report	Publication?
Patient pathways	Patient Pathways v1					Patient Pathways v2 uploaded						Improved	
Mobile App									Dataset collected			Mobile APP 1st version available	. How many submitted? Deadline 15.10.17
Pills of knowledge												Video/audio lessons available for e-Learning	Building these next 6 months
Registry			Inventory of biobanks and existing registries (V1)	Agreement for prospective ONLY, not retrospective unles funding				Dataset capture tool developed for prospective				Simple dataset and inventory of biobanks and registries	CLS to share on 13th 14th October at Face to Face WG #8
Clinical trials						Discussions, publications, calls	M6 Report provided – 4 completed, in progress, funded					Annual report, completion of 1 trial	
YouTube (conferences)							Collection of conferences					Conf. available on YouTube	Record!!
Simple clinical outcome	Clinical outcomes decided					Clinical outcomes	M6 Report – provided Clinical outcomes						
Clinical outcome											M6 Report –provided Clinical outcomes	Diseases specific	
Clinical guidelines								PAVM (UK, through BTS, in Press Thorax)				Clinical guidelines	Timing for anticoagulants
Factsheets												Factsheets	Gather by Survey
Website													
Translation													
Newsletters													
Social network pages			Twitter, Facebook										

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Network Vascular Diseases (VASCERN)

HTAD-WG

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VASCERN – HTAD WG

1st Year action plan : state of play

Julie De Backer & Guillaume Jondeau

VASCERN meeting
Paris – October 13 – 14 2017

VASCERN 1st Year Action Planned

A. Sharing of experience: discussion of difficult clinical cases
B. Definition of patients pathways
C. Cross-border pathways Mobile Application
D. Pills of knowledge
E. Registries
F. Clinical trials
G. Availability of conferences on YouTube
H. Multidisciplinary school (Year 3)
I. Definition of clinical outcomes
J. Writing recommendations
   1. for optimal care of the disease (diagnosis, evaluation, treatment);
   2. for optimal care of these patients facing common problems not related to their rare diseases (e.g: how to manage a patient with vEDS who is requiring cholecystectomy?)
K. Integrating teaching & patient empowerment VASCERN Clinic Days (Year 5)
L. Communication

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VASCERN-HTAD 1st Year Action Planned

A. Sharing of experience: discussion of difficult clinical cases
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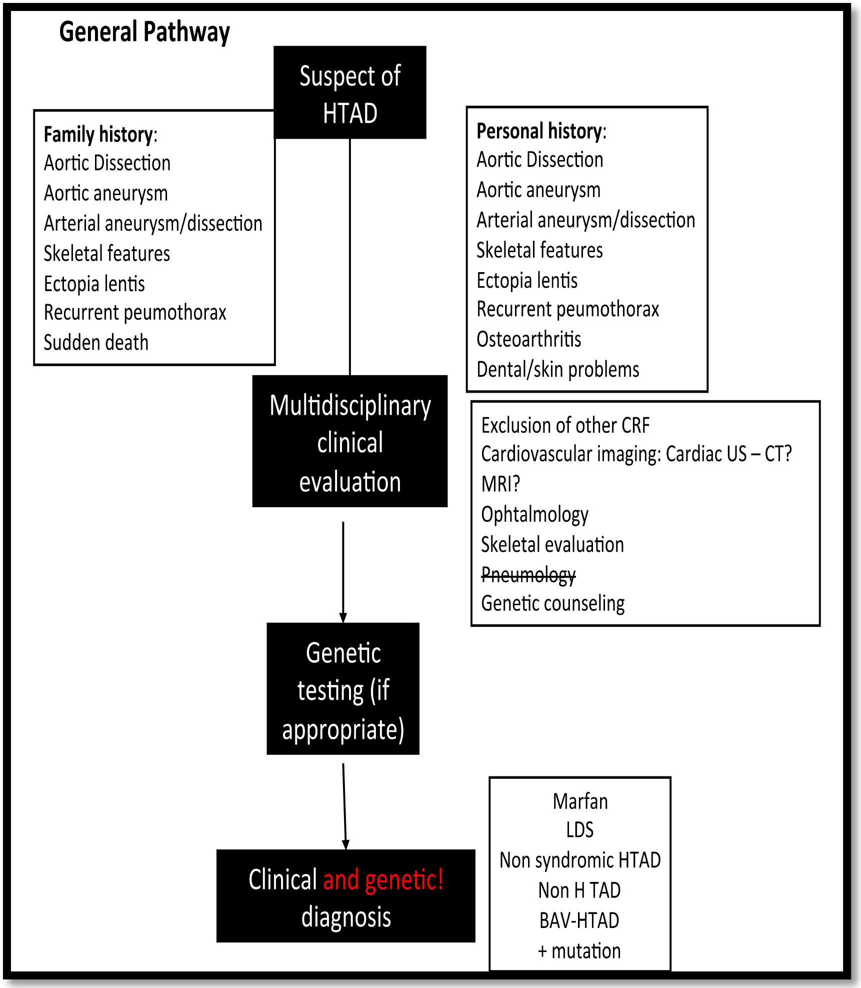
K. Integrating teaching & patient empowerment VASCERN Clinic Days (Year 5)
L. Communication

A. Sharing experience

- Monthly calls via Webex – every 4th Mon 5pm
  - 1st on March 27 2017
  - 6 in total
  - Case discussions started July 2017 (N=3)
- In person meeting @ ESC in Barcelona (Aug 28 2017)

B. Definition of patients pathways

- General chart
- Entry scenario'
- List of examina



General Pathway

Suspect of HTAD

Family history:
Aortic Dissection
Aortic aneurysm
Arterial aneurysm/dissection
Skeletal features
Ectopia lentis
Recurrent peumothorax
Sudden death

Personal history:
Aortic Dissection
Aortic aneurysm
Arterial aneurysm/dissection
Skeletal features
Ectopia lentis
Recurrent peumothorax
Osteoarthritis
Dental/skin problems

Multidisciplinary clinical evaluation

Exclusion of other CRF
Cardiovascular imaging: Cardiac US – CT? MRI?
Ophtalmology
Skeletal evaluation
~~Pneumology~~
Genetic counseling

Genetic testing (if appropriate)

Clinical and genetic! diagnosis

Marfan
LDS
Non syndromic HTAD
Non H TAD
BAV-HTAD
+ mutation

D. Pills of knowledge

- What are Heritable Thoracic Aortic Diseases (HTAD)?
- What is Marfan syndrome?
- What is Loeys Dietz syndrome?
- Open to discussion for other entities??
- How is Marfan syndrome diagnosed?
- What is the role of genetic test in HTAD?
- What is family screening for HTAD?
- How is the aorta monitored in HTAD?
- What is aortic root replacement?
- When is aortic root replacement indicated in HTAD?
- What is an aortic dissection?
- Can people with HTAD syndrome exercise?
- What are the recommendations for pregnancy in HTAD?
- Genetic counselling for the couple with HTAD.
- What is an acute aortic dissection?
- What is an acute small medium artery dissection?
- What is a chronic aortic dissection?
- Generic risk factors and HTAD

E. Registries

- MAC
- ROPAC

I. Definition of Clinical Outcomes

- HTAD Disease: Marfan syndrome (FBN1+)
- Parameters
  - number of patients screened
  - number of patients with a final diagnosis (clinical and molecular),
  - number of patients under follow-up
  - number of patients with aortic event
    - Dissection
    - Surgery
  - Number- and outcome of pregnancy

J. Writing recommendations

- ACTA2
  - Manuscript drafted
  - Discussion items defined

K. Communication

- EU IT platform

Deliverables	M1 3/17	M2 4/17	M3 5/17	M4 6/17	M5 7/17	M6 8/17	M7 9/17	M8 10/17	M9 11/17	M10 12/17	M11 1/18	M12 2/18
Clinical cases*				X	X		X					Report
Patient pathways						5 Patient pathways available						Improved
Mobile App												Mobile APP 1st version available
Pills of knowledge							List made					Video/audio lessons available for e-Learning
Registry							ROPAC & MAC					Simple dataset and inventory of biobanks and registries
Clinical trials												Annual report, completion of 1 trial
YouTube (conferences)												Conf. available on YouTube
Simple clinical outcome							Clinical outcomes defined					
Clinical outcome							ACTA2					Diseases specific
Clinical guidelines												Clinical guidelines
Factsheets												Factsheets
Website	🟥											
Translation												🟥
Newsletter	🟥	🟥	🟥	🟥	🟥	🟥	🟥	🟥	🟥	🟥	🟥	🟥
Social network pages			Twitter, Facebook									

Deliverables	M1 3/17	M2 4/17	M3 5/17	M4 6/17	M5 7/17	M6 8/17	M7 9/17	M8 10/17	M9 11/17	M10 12/17	M11 1/18	M12 2/18
*Clinical cases**				*X*	*X*		*X*					*Report*
Patient pathways						*5 Patient pathways available*						*Improved*
Mobile App												*Mobile APP 1st version available*
Pills of knowledge							*List made*					*Video/audio lessons available for e-Learning*
Registry							*ROPAC & MAC*					*Simple dataset and inventory of biobanks and registries*
Clinical trials												*Annual report, completion of 1 trial*
YouTube (conferences)												*Conf. available on YouTube*
Simple clinical outcome							*Clinical outcomes defined*					
Clinical outcome							*ACTA2*					*Diseases specific*
Clinical guidelines												*Clinical guidelines*
Factsheets												*Factsheets*
Website	🟥											
Translation												🟥
Newsletter	🟥	🟥	🟥	🟥	🟥	🟥	🟥	🟥	🟥	🟥	🟥	🟥
Social network pages			*Twitter, Facebook*									

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Network Vascular Diseases (VASCERN)

MSA-WG

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Network Vascular Diseases (VASCERN)

PPL-WG

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VASCA-WG

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Network Vascular Diseases (VASCERN)

ePAG

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eHealth / Training -WG

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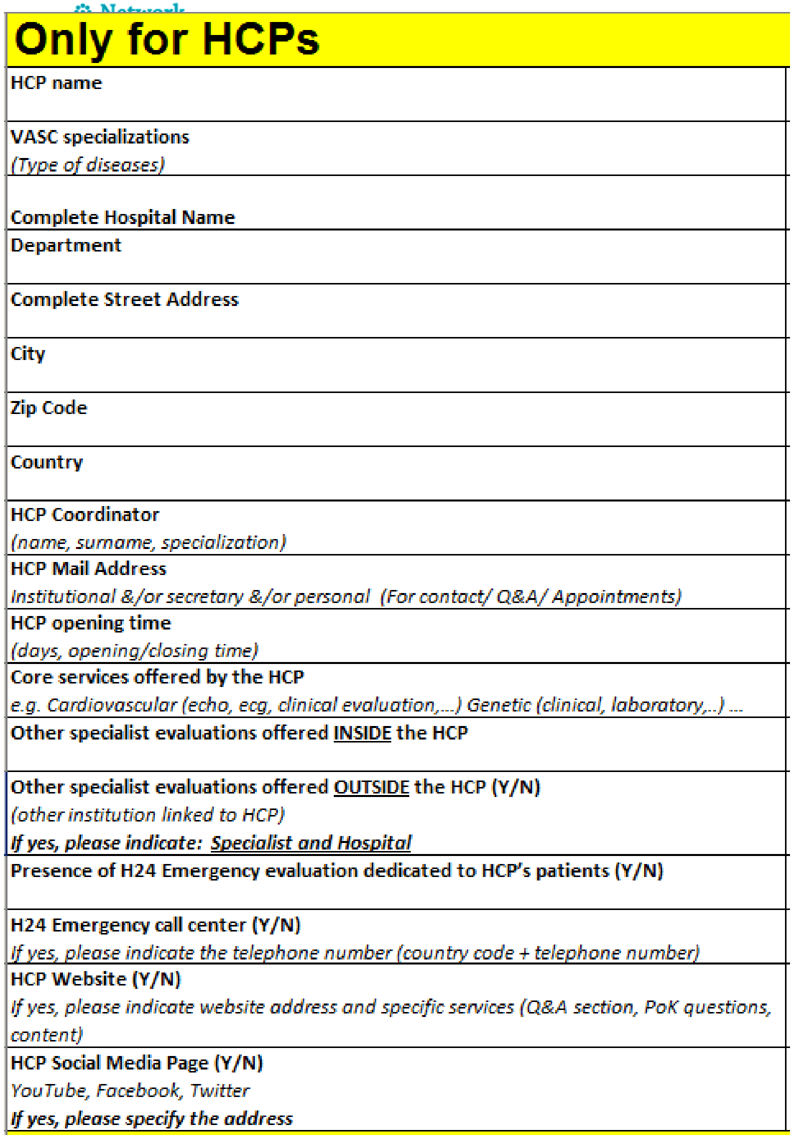
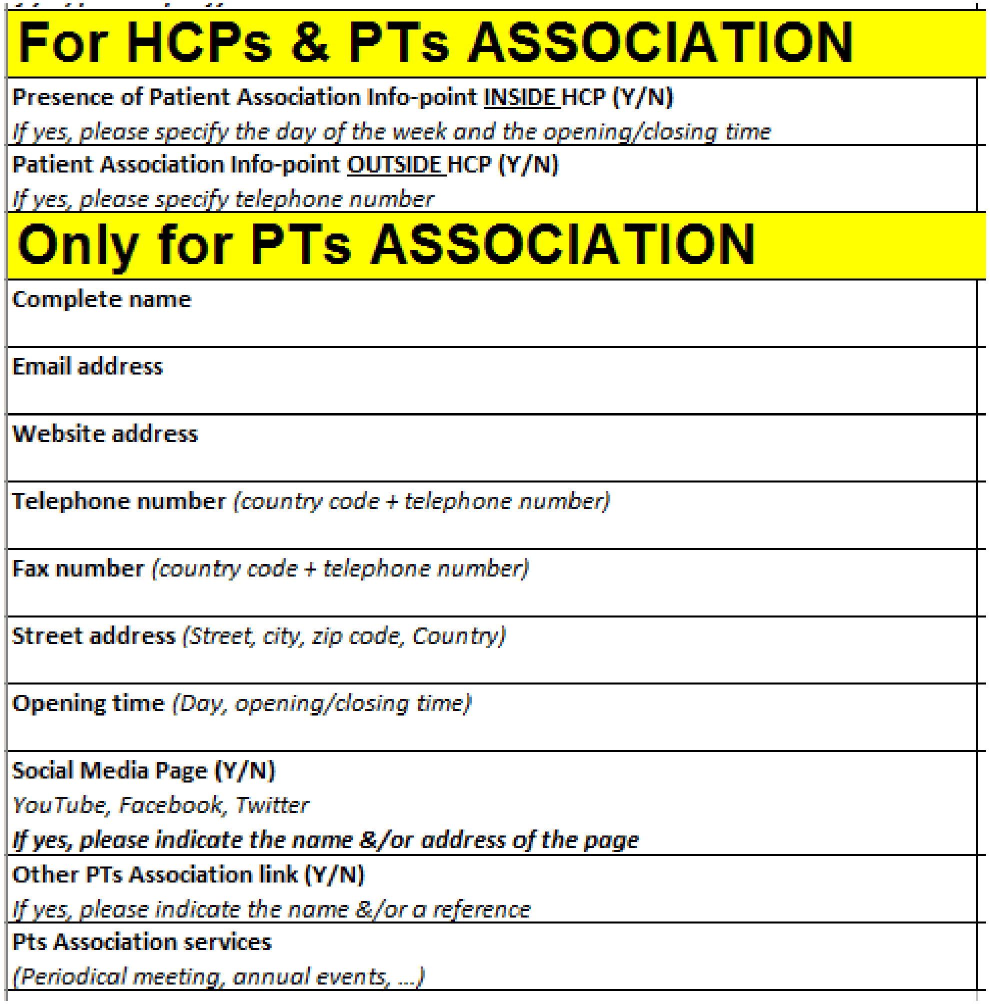
Defined dataset



Only for HCPs
HCP name
VASC specializations *(Type of diseases)*
Complete Hospital Name
Department
Complete Street Address
City
Zip Code
Country
HCP Coordinator *(name, surname, specialization)*
HCP Mail Address *Institutional &/or secretary &/or personal (For contact/ Q&A/ Appointments)*
HCP opening time *(days, opening/closing time)*
Core services offered by the HCP *e.g. Cardiovascular (echo, ecg, clinical evaluation,…) Genetic (clinical, laboratory,..) …*
Other specialist evaluations offered INSIDE the HCP
Other specialist evaluations offered OUTSIDE the HCP (Y/N) *(other institution linked to HCP)* ***If yes, please indicate: Specialist and Hospital***
Presence of H24 Emergency evaluation dedicated to HCP's patients (Y/N)
H24 Emergency call center (Y/N) *If yes, please indicate the telephone number (country code + telephone number)*
HCP Website (Y/N) *If yes, please indicate website address and specific services (Q&A section, PoK questions, content)*
HCP Social Media Page (Y/N) *YouTube, Facebook, Twitter* ***If yes, please specify the address***



For HCPs & PTs ASSOCIATION
Presence of Patient Association Info-point INSIDE HCP (Y/N) *If yes, please specify the day of the week and the opening/closing time*
Patient Association Info-point OUTSIDE HCP (Y/N) *If yes, please specify telephone number*
Only for PTs ASSOCIATION
Complete name
Email address
Website address
Telephone number *(country code + telephone number)*
Fax number *(country code + telephone number)*
Street address *(Street, city, zip code, Country)*
Opening time *(Day, opening/closing time)*
Social Media Page (Y/N) *YouTube, Facebook, Twitter* ***If yes, please indicate the name &/or address of the page***
Other PTs Association link (Y/N) *If yes, please indicate the name &/or a reference*
Pts Association services *(Periodical meeting, annual events, …)*

State of play

- Contact with developper . Development planned by the end of the year
- **Mobile APP Dataset** sent to all VASCERN HCPs & POs to be filled in by October 15th! If not already, please do it during this 2 days meeting!

Contacts:
Alessandro PINI - Alessia PAGLIALONGA - Raffaella GAETANO

State of Play : HCPs members

HCPs members: n = 32

Dataset: NO = 15
YES = 17

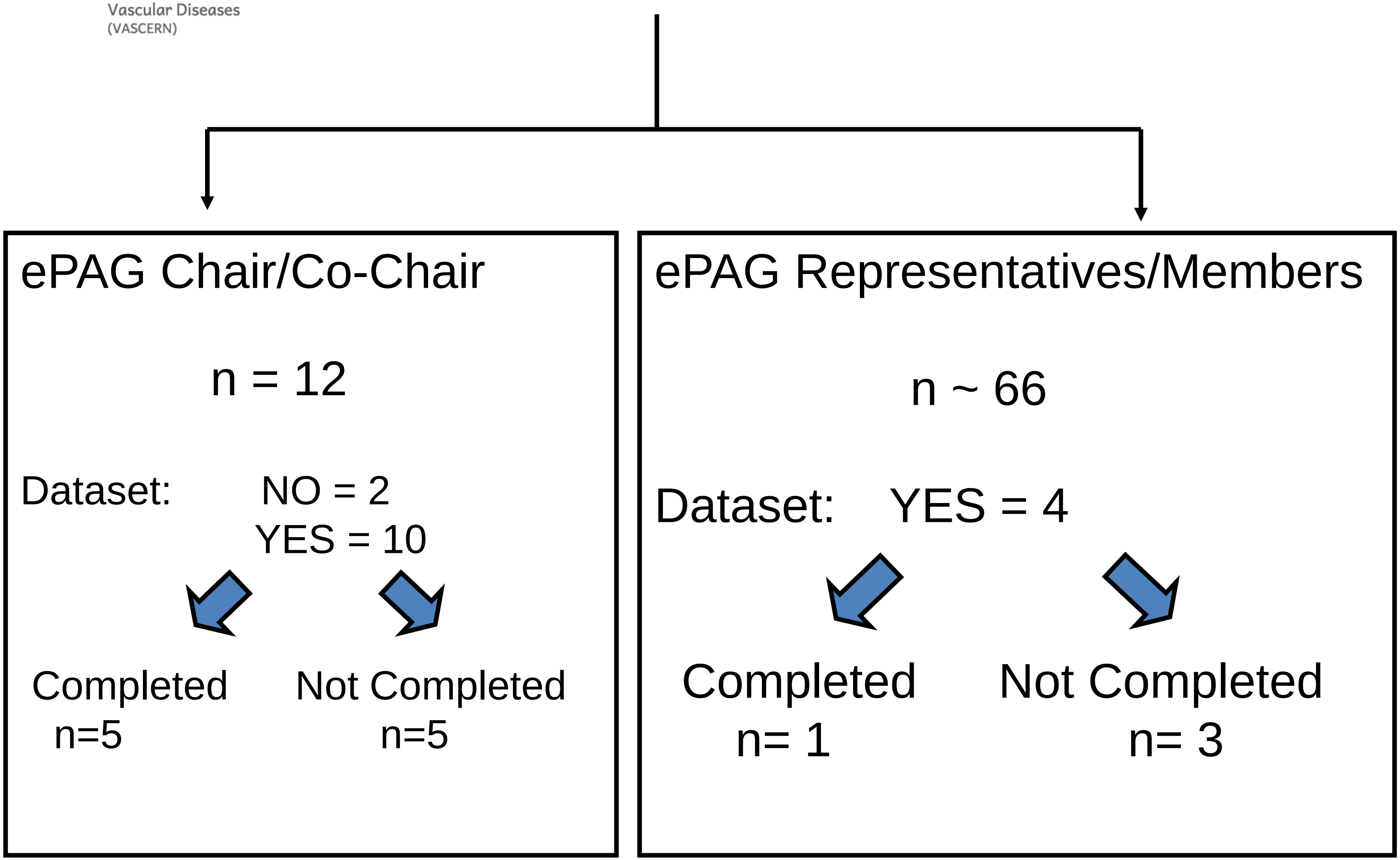
Completed
n= 8

Not Completed
n= 9

Affiliated HCPs : ??? – TDB – Next step ?

State of Play : ePAG members

ePAG members: n~ 78



ePAG Chair/Co-Chair

n = 12

Dataset: NO = 2
YES = 10

Completed
n=5

Not Completed
n=5

ePAG Representatives/Members

n ~ 66

Dataset: YES = 4

Completed
n= 1

Not Completed
n= 3

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State of Play : ePAG members

ePAG members: n~ 78

Josephine Mosset (eurordis) : Total number of Pos related to VASCERN (all ePAG - european patient advocacy group) are 78

Levels in ePAG

- Chair & Co-chair (n = 12 = present in the dataset)
- Representatives
- Members

- Few POs (rapresentatives & members) have been compiled the dataset as they are related to single HCPs

- No infos about other POs could therefore be found in the Mobile App unless related to a single HCPs or generically presented in a new dataset file for each country.

COMMON MISTAKES

- With Y/N Questions → Blank spaces
- Missing information regarding “HCPs&PTs Association”
- Regarding “VASC specializations (Type of diseases)” → vague information

HOW & WHEN TO DO IT

- Missing dataset should be completed within Saturday 14th (during Paris Meeting) – eHealth group at disposal
- Final (last call) deadline is Wednesday 18th (email it to Marine Hurard, Alessandro Pini, Raffaella Gaetano)
- We will check them all and we will contact at once those who have to correct the dataset (within Thursday 19th)
- We have to send all datasets to M4H to imput the infos within the database of the App (within Friday 20th)

Timetable for the APP – Next Step

- 1 MONTH is needed to get the Mobile App β-version 1.0 availability

- 15 DAYS are needed to check the App
  - each HCPs and each POs will check
    - Correct infos
    - Correct functionality of the App
    - Send ASAP an email to Marine and to us to report problems that have to be solved and possible solutions (if related to wrong infos)

      IMPORTANT: we should avoid just to add some more infos not previously indicated in the dataset as every correction means time loss.
    - Mobile APP should be ready to use within 25th December

LANGUAGES – Step 1

- The first Mobile APP version will be in english
- Later we have to develop a multi-lingual APP
- The APP will choose the language automatically (same language of the MobilePhone)
- How many languages do we have?
- Each HCPS and POs have to translate the infos and fulfill another dataset

LANGUAGES – Step 2

- How many languages do we have?

- All infos in the database should be available in a multi-lingual mode
- Therefore one of more HCPs/Pos in each country will have to translate the english version in the local language
- Timetable to be decided for this step

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Registry-WG

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WP5	**Registries: working towards the creation of a VASCERN registry** - Survey of existing registries and biobanks - Minimum dataset
WP6	**Clinical trials**
WP7	**Availability of conferences on YouTube**
WP8	**Definition of clinical outcomes**
WP9	**Writing recommendations** J1: Clinical recommendations J2: Do’s and Don’ts factsheets
WP10	**Communication** - Website - Social Networks - Monthly Newsletters - Collaborative Platform (internal communication) - Translation of material directed to patient care information

Registries

How to tackle the issue?

Future plans

Recommended Practices for Data Standardisation in the Context of the operation of European Reference Networks

- 2017
- RD-ACTION Output

- Collecting data in a standardised manner will allow it to become syntactically and semantically interoperable, which increases the power of that data in several ways.
- ERNs should consult rare disease FAIR data linkage specialists to discuss their specific needs and opportunities to link clinical data generated by ERNs -and ideally their constituent centres- with additional data sources.
- The most logical point of engagement is the new GO-FAIR implementation network currently being established in the rare disease domain, the main goal of which is to professionalize FAIR services for rare diseases and ERNs.
- https://www.dtls.nl/fair-data/go-fair/

”Issues”

- **Time** (who is going to fill in the database)
- **Money** (who is going to pay for it)
- Privacy issues (new EU regulations per 1-01-2018)
- “control issues”
- Exchangebility of data (FAIR)
- Local rules concerning data exchange and transfer of data
- Publication rights
- Research questions?

Actions sofar

- Forming of a technical subgroup
  - Marco Roos (RD connect), Christian Behrendt, Jerome Weinbach
  - + Derk Arts (castor) and Holger Storf (OSSE initiative)

- Part of the steering group of Go-Fair
- Grant application for FAIR based patient signal bank (hevas, castor, semantic group Leiden, RadboudUMC)
- Initiative-2 (IMI-2) European Health Data Network (EHDN)
  - UMC Utrecht, Karolinska Institutet, Oxford University and Semmelweis University) research institutes (Fraunhofer, DTL, i~HD, IHE), HTA bodies (ZIN) and an SME (P-95). **VASCERN?**

Plan

- Bottum up approach

- Start with local registries (avoid privacy and control issues)
  - Database program and structure has to be FAIR (focus at this point on interoperability
  - Same data base structure for each WG (EC minimal dataset + added WG items
  - Start with minimal datasets. Databases can be extended in a later phase

EUROPEAN COMMISSION
JOINT RESEARCH CENTRE
Directorate F – Health, Consumers and Reference Materials
Unit F.1 – Health in Society

EUROPEAN PLATFORM ON RARE DISEASES REGISTRATION (EU RD Platform)

SET OF COMMON DATA ELEMENTS FOR RARE DISEASES REGISTRATION

GROUP	ELEMENT N°	ELEMENT NAME	ELEMENT DESCRIPTION	CODING	COMMENT
1. Pseudonym	1.1.	Pseudonym	Patient's pseudonym	• String	The JRC is working on providing a pseudonymisation tool to the registries
2. Personal information	2.1.	Date of birth	Patient's date of birth	• Date (dd/mm/yyyy)	
	2.2.	Sex	Patient's sex at birth	• Female • Male • Undetermined • Foetus (Unknown)	
3. Patient Status	3.1.	Patient's status	Patient alive or dead	• Alive • Dead • Lost in follow-up • Opted-out	If dead then answer question 3.2
	3.2.	Date of death	Patient's date of death	• Date (dd/mm/yyyy)	
4. Care pathway	4.1.	First contact with specialised centre	Date of first contact with specialised centre	• Date (dd/mm/yyyy)	

5. Disease history	5.1.	Age at onset	Age at which symptoms/signs first appeared	• Antenatal • At birth • Date (dd/mm/yyyy) • Undetermined	
	5.2.	Age at diagnosis	Age at which diagnosis was made	• Antenatal • At birth • Date (dd/mm/yyyy) • Undetermined	
6 Diagnosis	6.1.	Diagnosis of the rare disease	Diagnosis retained by the specialised centre	Orpha code (strongly recommended – see link) / Alpha code/ ICD-9 code/ ICD-9-CM code / ICD-10 code	http://www.orphadata.org/cgi-bin/inc/product1.inc.php
	6.2.	Genetic diagnosis	Genetic diagnosis retained by the specialised centre	International classification of mutations (HGVS) (strongly recommended – see link) / HGNC / OMIM code	http://www.hgvs.org
	6.3	Undiagnosed case	How the undiagnosed case is defined	• Phenotype (HPO) • Genotype (HGVS)	
7. Research	7.1.	Agreement to be contacted for research purposes	Patient's permission exists for being contacted for research purposes	• YES • NO	
	7.2.	Consent to the reuse of data	Patient's consent exists for his/her data to be reused for other research purposes	• YES • NO	
	7.3.	Biological sample	Patient's biological sample available for research	• YES • NO	If YES answer question 7.4
	7.4.	Link to a biobank	Biological sample stored in a biobank	• YES (if appropriate use link) • NO	https://directory.bbmri-eric.eu
8.Disability	8.1.	Classification of functioning/disability	Patient's disability profile according to International Classification of Functioning and Disability (ICF)	• Disability profile / Score	http://www.who.int/classifications/icf/whodasii/en/

Plans for the near future

Role representative each WG in registry WG

. Approval of minimal data sets
. Data stewards in each HCP identified

Start small but whatever we built has to be robust and prepared for future developments

Financial

central IT/data steward
within vasca investments in database program (can be used for co-funding?)

European
Reference
Network
for rare or low prevalence
complex diseases

Network
Vascular Diseases
(VASCERN)

14.00-16.00: VASCERN BOARD MEETING, chaired by Prof Guillaume Jondeau, Coordinator

Requested: 1 representative per HCP (31) & ePAG Chairs (6)
Open to all participants without voting rights

- Terms of reference for validation by the board (Marine Hurard)
- Priorities for Work Packages
  - RDWGs
  - ePAG
  - Transversal WGs
- **Minimal participation to videoconferences & CPMS**
- Funding
- New ERN Call for new ERN members (December 2017):
  - Criteria for each RDWG to be revised after the experience of the first year, for the new members
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Budget

March 2017-February 2018

Previsional:

- **Personal Cost** (full time Project Manager, Project Officer, full time administrative assistant, 20% time Coordinator)
  - Total:**162 218 €**
- **Other Direct Costs**
  - Travel
  - Other goods & services (for meeting organisation)
  - Equipment
  - Total: **116 273 €**
- **Subcontracting costs**
  - Communication & Dissemination Tools
  - Translation
  - eHealth tools (Mobile APP, recording video lessons)
  - Total: **33 027 €**

Reporting expenses M8 – October 2017:

- **Personnal Cost**
  - **similar to previsional** (full time project manager, project officer, part-time (3/5) administrative assistant, 20% coordinator – *exact expenses for the 1st year to be confirmed by the HCP)*
  - delays, hiring effective M7
- **Other Direct Costs**
  - Travel (Seminar): 15 608 €
  - Meetings organisation: 45 513 €
  - Communication: 234 €
  - Total: **61 355 €**
- **Subcontacting Costs**
  - Communication (kakemono, graphism,…): 372€
  - eHealth tool (videos): 2450 €
  - eHealth tool (Mobile APP) expected cost developer
  - quote: 4.800 €
  - Total**: 7 622€**

➢ Administrative delays in Grant agreement preparation, no expenses before M4

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Position Paper on the 2018 call for new members to existing ERNs
ERN Coordinators Group (CG) – 27 September 2017

Ahead of the 2018 call for membership to existing European Reference Networks (ERNs), the ERN Coordinators Group (ERN CG) has come together to deliver joint recommendations on their role in the 2018 application process for ERN membership and share considerations on their enlargement. This Position Paper is based on the answers of twenty-two coordinators to a questionnaire that was circulated in summer. A first draft of this position paper was drawn up and discussed during a joint conference call held on 20 September and revised accordingly. The Position Paper is meant to contribute to a discussion with the European Commission and the Board of Member States on the planning and implementation of the application and selection procedure.

In particular, this paper focuses on three key aspects, namely:

- **Ensuring expansion of the networks into countries not currently represented;**
- **Active participation in the evaluation and approval process;**
- **Regular renewal of network specific criteria to be met by all potential applicants.**

With regard to the 2018 application process, ERN Coordinators would like to highlight two principles that should guide the formulation of new calls for Full Members: the **principle of excellence** and the **principle of representativeness of European Member States**. Meanwhile, the ERN Coordinators Group acknowledges the prerogative of Member States to identify Affiliated Members for approved European Reference Networks and expresses the commitment to ensure a smooth integration process of these centres.

As new full members will be joining approved networks, ERN Coordinators agree on the importance of **updating the minimum criteria** to reflect clinical and technological excellence and **prioritising countries not yet sufficiently involved in the networks**.

The ERN CG considers the future expansion of their networks as important and necessary. However, concerns persist regarding the financial and administrative manageability of the ERNs. The group therefore agrees that: the expansion process should take into consideration that the financial and administrative management of the networks needs to remain feasible; network growth should be accompanied by a sustainable strategy for future funding.

Overall, the ERN CG agrees that the selection process for additional centres needs to be based on updated criteria and designed as transparent, realistic and objective as possible. The group suggests that ERN coordinators and the Board of Member States work together in the review of applications.

In the context of ERN Membership, the ERN CG would like to point out that the approved Networks would benefit from a more comparable set of criteria for the definition of Centres of Excellence across all EU Member States.

- **Specific Criteria** for new HCP expertise center to participate in VASCERN to be revised by each RDWG after the experience of the first year.

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Continuous Monitoring of ERNs (Guillaume Jondeau)

Inter-ERNs

Questions

- What is a patient of the ERN ?
  - CPMS ?
  - Seen by HCP ?
- Patient satisfaction:
  - How to measure

- Number of
  - MS in the network
  - Working Groups set up
  - Members participating in ERN meetings

- % groups with patients
- Level of patient satisfaction

- Average time
  - from diagnosis to first treatment/surgery
  - between referral to ERN and multidisciplinary clinical advice
  - between referral to ERN and diagnosis

- Survival (i.e. after 1 year of diagnosis)
- Mortality
- Morbidity

- Number of patients
  - seen/advised by ERN (CMPS)
  - seen by the ERN HPCs
- Number of procedures (?) performed within ERN

- Adherence to clinical practice guidelines (registry)

- at least 2 HCPs from 2 countries
  - Grants
  - Number of patients in shared registries
  - Number of clinical trials

Data collection

Who will input into the system

Both ERN coordinators and HCP will be responsible for providing data (automatic !!!)

Who will monitor the system

ERN coordinators will use the system as an instrument to monitor their activities and identify areas for improvement.

It will also be a great tool to prepare for the Evaluation process and guide their Self-Assessment.

Ethical & Legal issues (data protection, conflict of interest, informed consent) (Romain Alderweireldt)

Inter-ERNs

ERN working group Ethics & legal issues & relations with stakeholders:

N. Hoogerbrugge (chair) - ERN GENTURIS
L. Giepmans (project manager) - ERN GENTURIS

J. Brunet Vidal - ERN GENTURIS
J. Clayton-Smith – ERN ITHACA
H. Dollfus – ERN EYE
B. Fonteyn – VASCERN
R. Alderweireldt - VASCERN
V. Kozich – MetabERN
C. Lampe – MetabERN
M. Scarpa - MetabERN

Next steps in Conflict of Interest paper

- Address all questions and concerns of ERN CG and BoMS
- Teaming up with Enrique's group and find a way for discussing issues with BoMS
- Finalising draft COI to be reviewed and finalised by lawyers (?DG Sante?)

RD-Action support for interERN cooperation

Victoria Hedley

VASCERN KOM 13th October 2017

This presentation is part of the project / joint action '677024 / RD-ACTION' which has received funding from the European Union's Health Programme (2014-2020).

European Commission

RD ACTION
DATA AND POLICIES
FOR RARE DISEASES

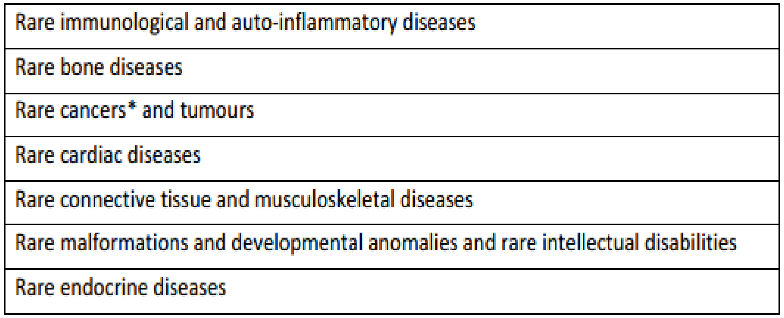
RD-ACTION & ERNs

- RD-ACTION (2015-18) WP6 (Policy & Integration) inherited focus of the EUCERD Joint Action
- 1st year of RD-ACTION: focus on providing more hands-on support to (primarily) the RD field, to implement Addendum
  - Workshops and meetings to identify leading figures
  - Matchmaker tool (2016)
- Intermediate Role: Canvassing ERN Coordinator Perspectives on various topics, to give all a 'voice' and advocate for important progress

EUCERD
European Union Committee of Experts on Rare Diseases

EUCERD RECOMMENDATIONS
on
RARE DISEASE EUROPEAN REFERENCE NETWORKS (RD ERNS)

31 January 2013



Rare immunological and auto-inflammatory diseases
Rare bone diseases
Rare cancers* and tumours
Rare cardiac diseases
Rare connective tissue and musculoskeletal diseases
Rare malformations and developmental anomalies and rare intellectual disabilities
Rare endocrine diseases

Years 2 & 3: Workshops & Policy Support

- **Once ERNs were in place, huge opportunity to unite the experience & expertise of these very different Networks, with wider field**
- **Goal: work *with* the ERNs to find shared solutions to common challenges, avoid re-inventing wheels, providing support in meeting myriad goals & responsibilities.**

RD-ACTION WPs:

The ERN 24! Now via WGs

Member States

DG SANTE & RTD

ePAGs

Topic Experts

ERN CG - WGs

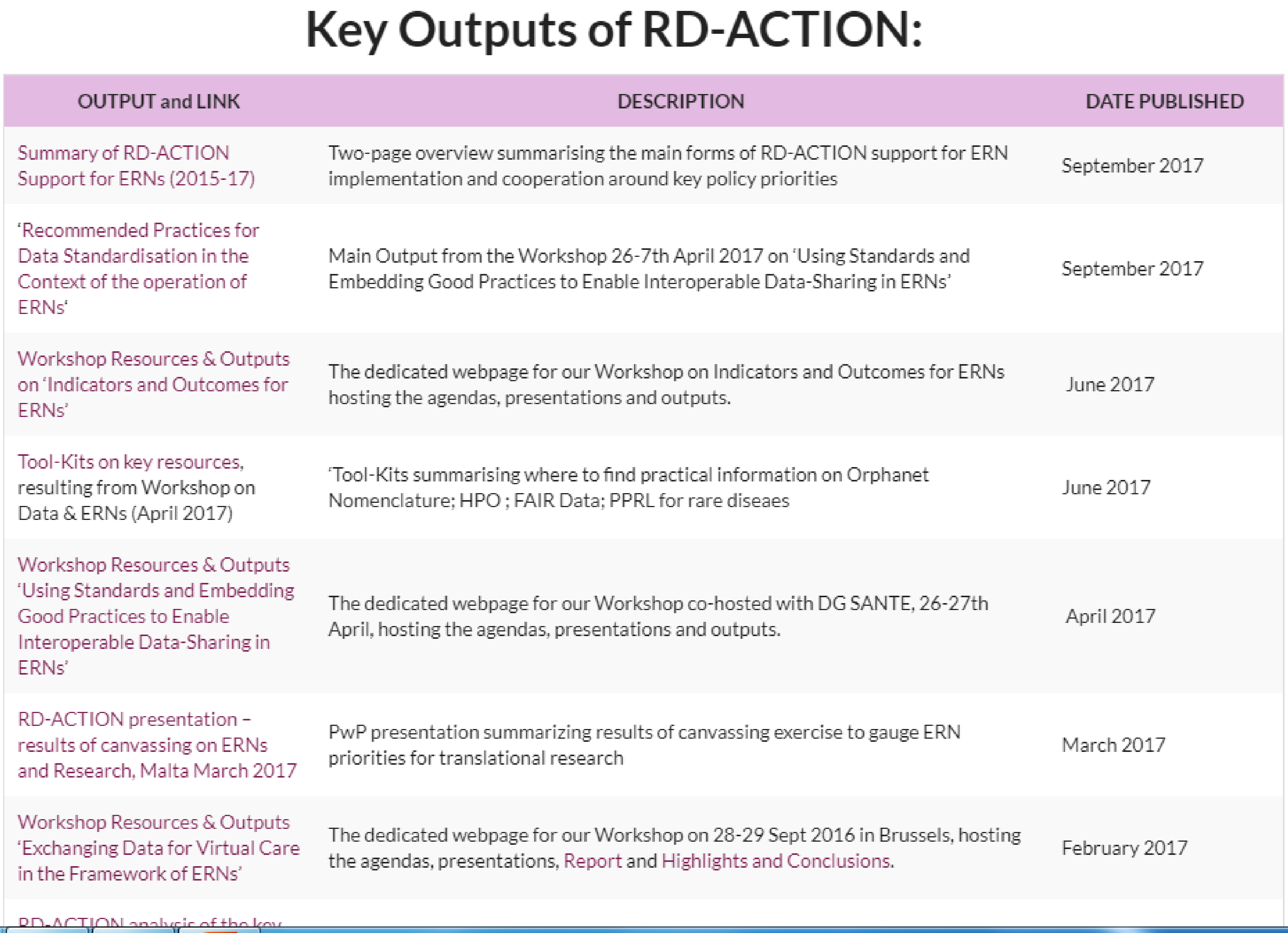
- IT and Data-Sharing
- Research
- Knowledge Generation: Training, education, capacity-building, guidelines
- Ethics and Legal issues
- Cross-Border healthcare, continuity of ERNs and communication
- Monitoring, assessment and quality improvement

Workshops To-Date:

- Sept ‘16: ***Exchanging data for virtual care in framework of ERNs*** (Brussels)
- April ‘17: ***Using standards & embedding good practices for interoperable data-sharing in ERNs*** (Brussels)
- June ‘17: ***Indicators and Outcome Measures to demonstrate the impact and added value of ERNs***

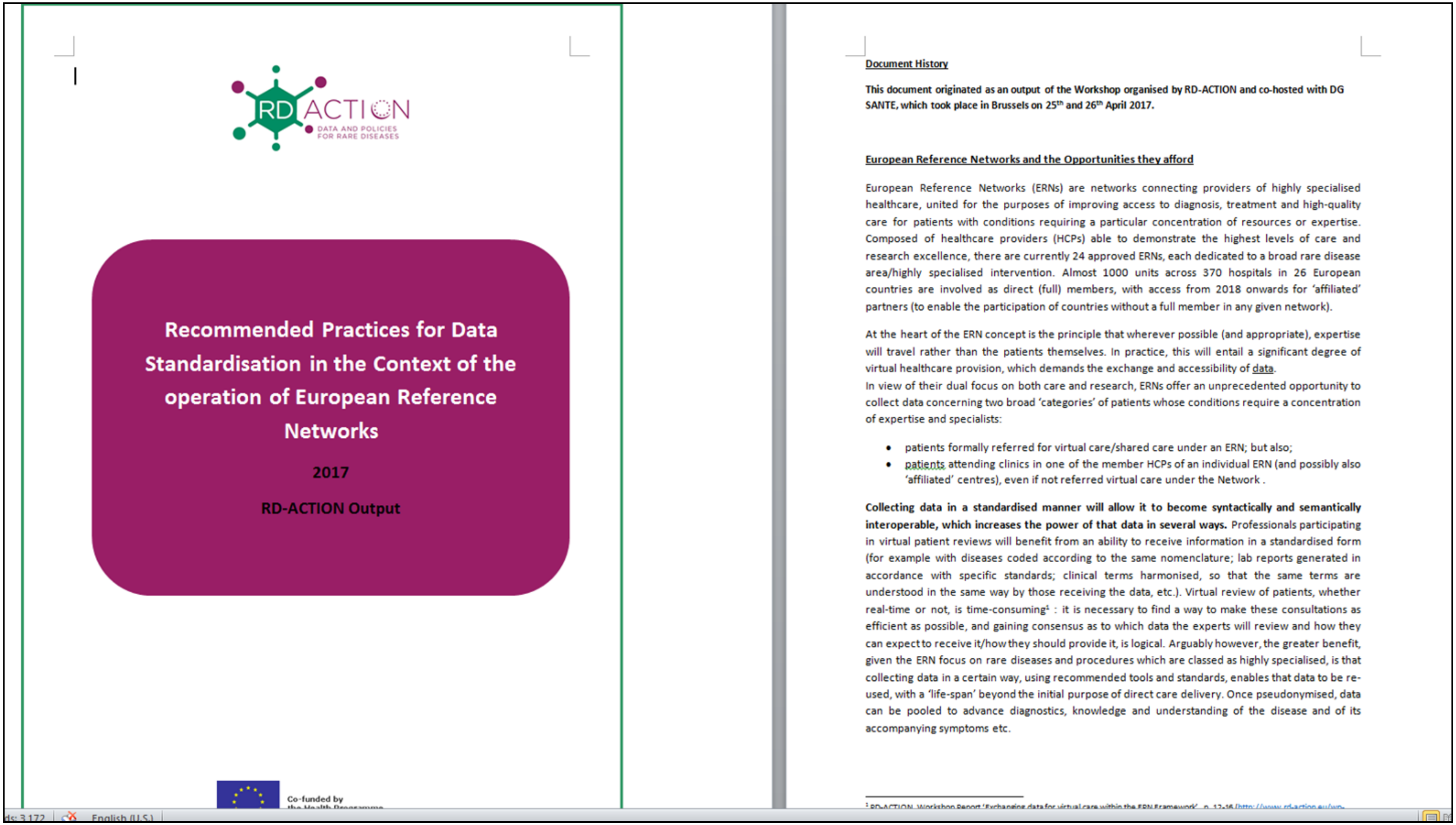
- Mantra is to be inclusive as possible – with links to outputs and webinars for all…

http://www.rd-action.eu/european-reference-networks-erns



Key Outputs of RD-ACTION:

OUTPUT and LINK	DESCRIPTION	DATE PUBLISHED
Summary of RD-ACTION Support for ERNs (2015-17)	Two-page overview summarising the main forms of RD-ACTION support for ERN implementation and cooperation around key policy priorities	September 2017
'Recommended Practices for Data Standardisation in the Context of the operation of ERNs'	Main Output from the Workshop 26-7th April 2017 on 'Using Standards and Embedding Good Practices to Enable Interoperable Data-Sharing in ERNs'	September 2017
Workshop Resources & Outputs on 'Indicators and Outcomes for ERNs'	The dedicated webpage for our Workshop on Indicators and Outcomes for ERNs hosting the agendas, presentations and outputs.	June 2017
Tool-Kits on key resources, resulting from Workshop on Data & ERNs (April 2017)	'Tool-Kits summarising where to find practical information on Orphanet Nomenclature; HPO ; FAIR Data; PPRL for rare diseaes	June 2017
Workshop Resources & Outputs 'Using Standards and Embedding Good Practices to Enable Interoperable Data-Sharing in ERNs'	The dedicated webpage for our Workshop co-hosted with DG SANTE, 26-27th April, hosting the agendas, presentations and outputs.	April 2017
RD-ACTION presentation – results of canvassing on ERNs and Research, Malta March 2017	PwP presentation summarizing results of canvassing exercise to gauge ERN priorities for translational research	March 2017
Workshop Resources & Outputs 'Exchanging Data for Virtual Care in the Framework of ERNs'	The dedicated webpage for our Workshop on 28-29 Sept 2016 in Brussels, hosting the agendas, presentations, Report and Highlights and Conclusions.	February 2017
RD-ACTION analysis of the key		



Recommended Practices for Data Standardisation in the Context of the operation of European Reference Networks

2017

RD-ACTION Output

Co-funded by
the Health Programme

Document History

This document originated as an output of the Workshop organised by RD-ACTION and co-hosted with DG SANTE, which took place in Brussels on 25th and 26th April 2017.

European Reference Networks and the Opportunities they afford

European Reference Networks (ERNs) are networks connecting providers of highly specialised healthcare, united for the purposes of improving access to diagnosis, treatment and high-quality care for patients with conditions requiring a particular concentration of resources or expertise. Composed of healthcare providers (HCPs) able to demonstrate the highest levels of care and research excellence, there are currently 24 approved ERNs, each dedicated to a broad rare disease area/highly specialised intervention. Almost 1000 units across 370 hospitals in 26 European countries are involved as direct (full) members, with access from 2018 onwards for 'affiliated' partners (to enable the participation of countries without a full member in any given network).

At the heart of the ERN concept is the principle that wherever possible (and appropriate), expertise will travel rather than the patients themselves. In practice, this will entail a significant degree of virtual healthcare provision, which demands the exchange and accessibility of data.
In view of their dual focus on both care and research, ERNs offer an unprecedented opportunity to collect data concerning two broad 'categories' of patients whose conditions require a concentration of expertise and specialists:

- patients formally referred for virtual care/shared care under an ERN; but also;
- patients attending clinics in one of the member HCPs of an individual ERN (and possibly also 'affiliated' centres), even if not referred virtual care under the Network .

Collecting data in a standardised manner will allow it to become syntactically and semantically interoperable, which increases the power of that data in several ways. Professionals participating in virtual patient reviews will benefit from an ability to receive information in a standardised form (for example with diseases coded according to the same nomenclature; lab reports generated in accordance with specific standards; clinical terms harmonised, so that the same terms are understood in the same way by those receiving the data, etc.). Virtual review of patients, whether real-time or not, is time-consuming[1] : it is necessary to find a way to make these consultations as efficient as possible, and gaining consensus as to which data the experts will review and how they can expect to receive it/how they should provide it, is logical. Arguably however, the greater benefit, given the ERN focus on rare diseases and procedures which are classed as highly specialised, is that collecting data in a certain way, using recommended tools and standards, enables that data to be re-used, with a 'life-span' beyond the initial purpose of direct care delivery. Once pseudonymised, data can be pooled to advance diagnostics, knowledge and understanding of the disease and of its accompanying symptoms etc.

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[1] RD-ACTION Workshop Report 'Exchanging data for virtual care within the ERN Framework', p. 12-16 (http://www.rd-action.eu/wp-

		One Gp pointed out that numbers of patients entered to CPMS may be affected by resource available and complexity of patient cases	Or simply **Number of patient cases entered to the CPMS**
Number of patients seen by the ERN HPCs		*As defined by the application and in order to maintain level of expertise* It is important to capture this data to demonstrate compliance with the disease-specific criteria established by each ERN on the application form – if a HCP claimed to see 300 patients with a particular group of diseases per year, it must collect data to demonstrate this.[8] One Gp proposed changing this to "total number of patients seen by all ERN HCPs – both those inside and outside of CPMS" Another Gp perceived an urgent need to define how we count these patients, particularly, how to distinguish between new patients and patients returning for a follow-up. Indeed, this breakdown is requested on the application form, and therefore HCPs will need to collect it anyway. Even the question of what one classes as patient is not so straightforward – for instance, if relatives are brought in, to assist in diagnosing the proband, are they counted too?[9]	If we accept the logic in capturing 'New' and 'Returning/Follow-up' patients here, one suggestion would be to replace this indicator with two, worded approximately as follows: **Total number of new patients seen by all ERN HCPs – both those inside and outside of CPMS** **Total number of follow-up/returning patients seen by all ERN HCPs – both those inside and outside of CPMS**

Remaining Workshops

- **How can ERNs generate, appraise and utilise clinical practice guidelines, to enhance the impact and deployment of consensus guidelines in national health systems?**
  - **(Dec 6-7th , Rome)**

- **Exploring the potential for Centres of Expertise and ERNs to provide integrated care for Rare Diseases**
  - **(April 11-13th, Frambu Norway)**

- **TBC – 1 or 2 workshops on ERNs & Research/added-value to the Therapy development & post-marketing pipeline**

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Meeting Endorsement

ERN Specific objective	Indicator name (Original Non-Paper version)	Indicator type (structure, process, outcome)
dissemination	Participation to congresses and conferences representing the ERN and presenting ERN activities and results	Process

ERN will consider reimbursment for traveling to ERN members for:

- RDWGs
- Work on guidelines
- Communication on VASCERN
- Representation in workshop for VASCERN

- Meeting program:
  - Should be discussed
    - RDWG
    - Ethics
  - Should include members of VASCERN
  - Should include presentation of VASCERN
    - Orally: 3 min
      - Set of slides available on the website
    - & poster (provided)

- Meeting should provide to VASCERN:
  - Videos (interview at least),
  - photos (website)
  - Report for dissemination (newsletter)

European Reference Network
for rare or low prevalence complex diseases

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  - RD-Action work for interERNs cooperation (Victoria Hedley)
- Criteria for an event to be endorsed by VASCERN
- **2018 annual meeting: date (Doodle: 12-13-14 October 2018 (Thursday-Fri or Fri-Sat))**
- Questions

16.00-16.15: coffee break
Photo booth (Nicolas Ledoux)

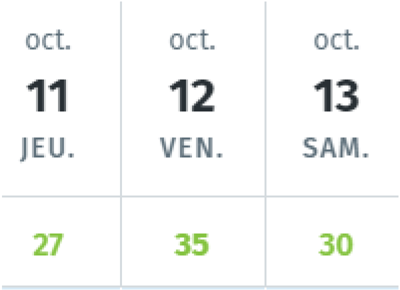
16.15-16.40**: Registry: the FAIR principle** (Marco Roos) (25’)

16.40-17.00: **Debriefing in plenary** (20’)

17.00-18.00: **Patient Group (ePAG) separate meeting** (60’)

#VASCERNdays2018

- 12-13-14 October 2018 (Thursday-Fri or Fri-Sat))?



oct. 11 JEU.	oct. 12 VEN.	oct. 13 SAM.
27	35	30

European Reference Network
for rare or low prevalence complex diseases

Network Vascular Diseases (VASCERN)

14.00-16.00: VASCERN BOARD MEETING, chaired by Prof Guillaume Jondeau, Coordinator

Requested: 1 representative per HCP (31) & ePAG Chairs (6)
Open to all participants without voting rights

- Terms of reference for validation by the board (Marine Hurard)
- Priorities for Work Packages
  - RDWGs
  - ePAG
  - Transversal WGs
- Minimal participation to videoconferences & CPMS
- Funding
- New ERN Call for new ERN members (December 2017):
  - Criteria for each RDWG to be revised after the experience of the first year, for the new members
- Participation in interERN WG
  - Reports from Guillaume Jondeau (Monitoring), Romain Alderweireldt (Ethics), Xavier Jeunemaître (Research)
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Q&A