



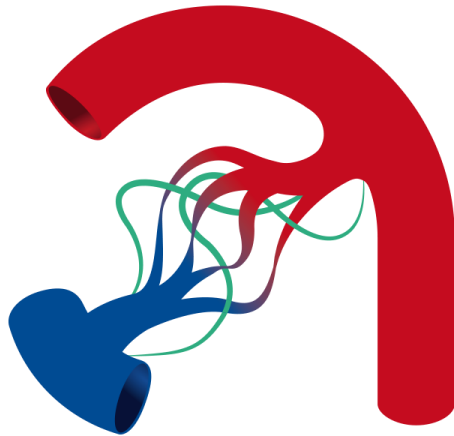
**European  
Reference  
Network**

for rare or low prevalence  
complex diseases



**Network**

Vascular Diseases  
(VASCERN)



# VASCERN DAYS 2018

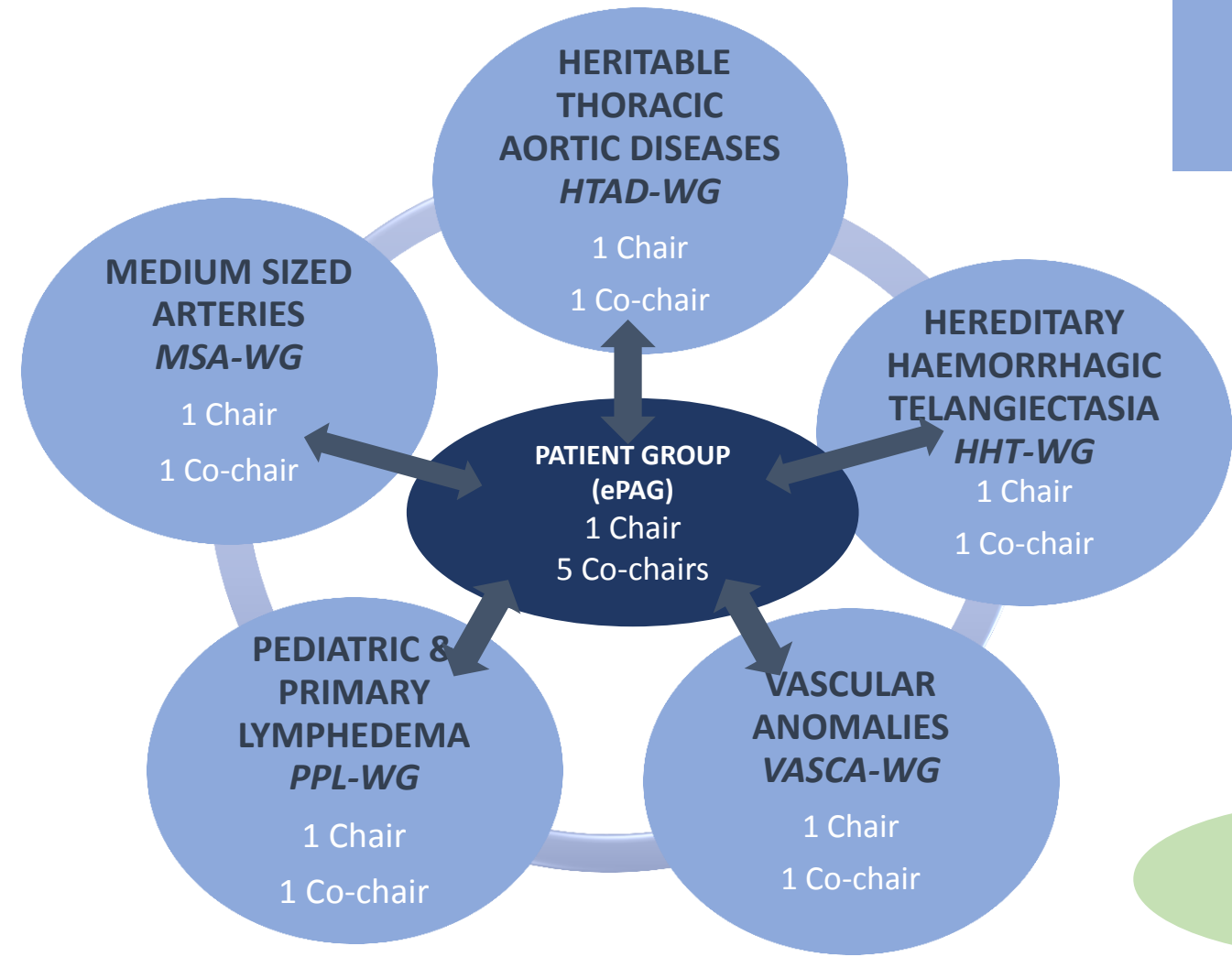
8:40-10:00: **Board session**

Chaired by Prof Guillaume Jondeau and Marine Hurard

# Structure

**5 RARE DISEASES  
WORKING GROUPS  
(RDWG)**

**+ FMD  
+ SCAD**



**TRANSVERSAL WORKING GROUPS**

**Basic sciences?**  
1 Chair  
1 Co-chair

**Patient Registry WG**  
1 Chair  
1 Co-chair

**eHealth**  
1 Chair  
1 Co-chair

**Training & Education**  
1 Chair  
1 Co-chair

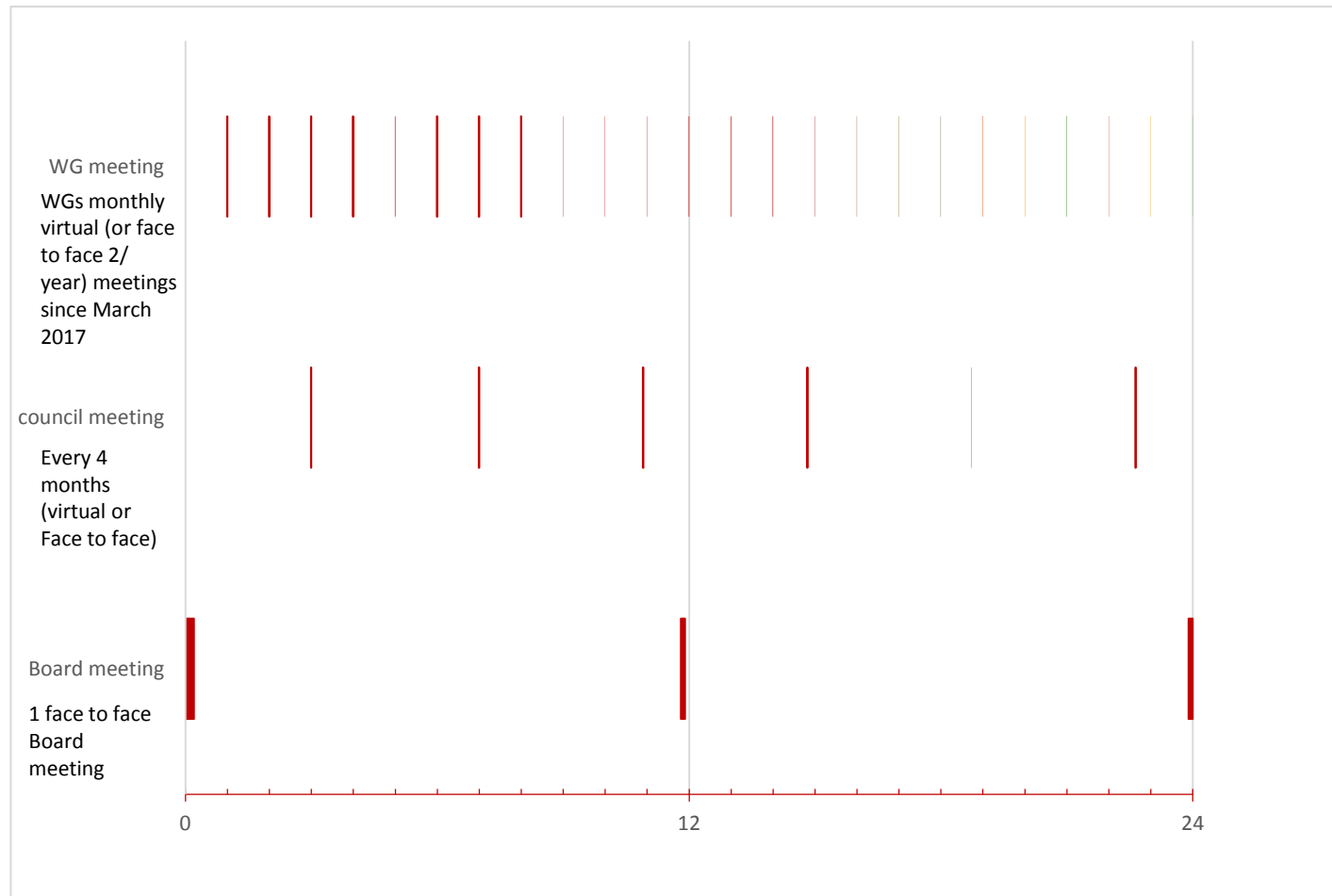
**Communication Advisory task-force**  
1 Chair  
1 Co-chair

**Ethics WG / interERNs**  
1 Chair  
1 Co-chair

# Participation in Working Groups' Meetings



## Good participation rate to RDWGs



- **HHT:** 18 meetings all but 2 participated in 15
  - 13 and 9
- **HTAD:** 16 meetings all but 4 participated in 11
  - 9, 8, 7 and 4
- **MSA:** 16 meetings all but 1 participated in 9
  - 3
- **PPL:** 16 meetings all but 2 participated in 8
  - 5 and 4
- **VASCA:** 17 meetings all but 1 participated in 13
  - 8



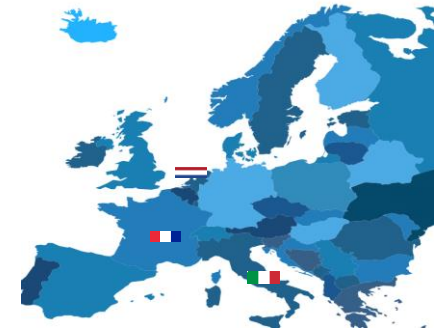
# Terms of Reference on RDWG participation

- A **Chair and a Co-chair** are responsible for the implementation of VASCERN action plan by the WG
- **HCPs representatives (Leads)** taking care of the pathologies defined are requested to participate (1 per HCP, substitute representative attends when necessary)
- The **ePAG Patient Group Co-chair** for the relevant diseases group, responsible to coordinate views of patient group and to disseminate information



# Annual Assessment of selected ERN HCPs

- Annual **assessment** of selected **ERN HCPs** by the Independent Assessment Body
- Review of technical documents (“supporting document of the ERN 2016 Application)
- **On-site audit** for selected HCPs
- Sharing 2018 experience, 2 VASCERN HCP positively assessed:
  - **HCP Antwerp**: Prof Bart Loeys, technical assessment and on-site audit
  - **HCP Bari**: Prof Carlo Sabba, technical assessment





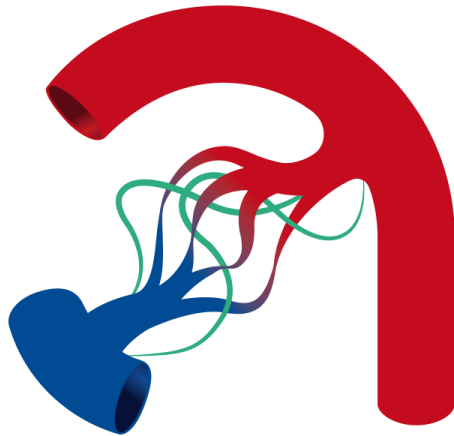
**European  
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**Network**

Vascular Diseases  
(VASCERN)



# InterERN Working Groups Progress



# InterERN Working Groups

## IT Advisory Group (ITAG) (Guillaume Jondeau)

- New Working Group merged with the European Commission IT group & ERN Coordinators
- Work on improving ERN IT services (CPMS, ECP)
- Strategic thinking regarding the Connecting Europe Facility eHealth for ERNs calls for proposals
- Meeting next week, hope to have feedback

# InterERN Working Groups



Ethical and Legal issues (Romain Alderweireldt, VASCERN's representative in the interERN WG and Chair of VASCERN transversal advisory group on Ethical and legal issues)

## Latest news

- 1st virtual meeting of the integrated WG of the ERNs and the Board of Member States representatives took place on Oct 1st
- Ideas to find new funding mechanism for ERNs
- Work on Conflict of Interest > soon finalised
- Meeting on October 19th





# InterERN Working Groups

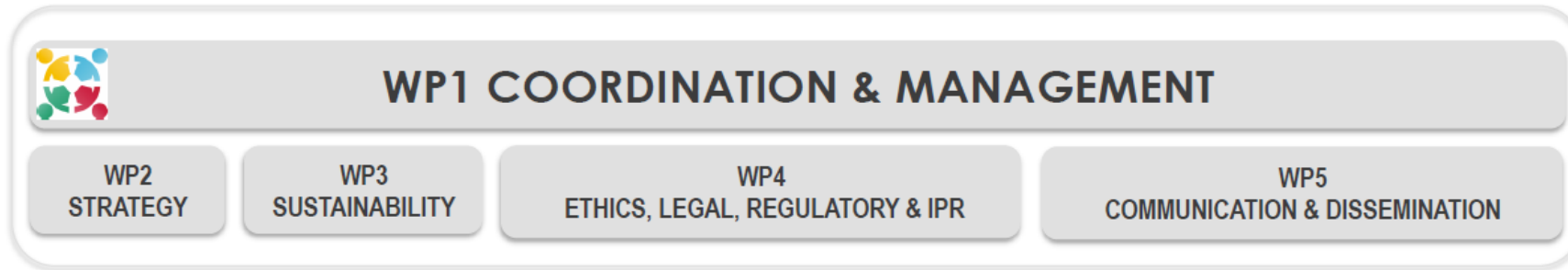
Research, including EJP-RD (Miikka Vikkula, Claire Shovlin)

- New Chair Luca Sangiorgi, ERN-BOND Coordinator
- Miikka Vikkula & Claire Shovlin
- Meeting with EMA, May 2018
  - people in the regulatory field are more aware of the existence of ERNs, very good start
  - a permanent connection with EMA
  - follow up meeting with EMA next year
  - Collection of info from ERNs on Research
- Future actions
  - State of the art started (survey)
  - More defined structure and referral persons by ERNs
  - Definition of sub-group to target specific objectives
  - Definition of a priority list of topics
  - meeting with the ERICs in the life science field (BBMRI; EATRIS; ECRIN; ELIXIR) to define relationships, invite RD Connect, EUN and HTA
  - better coordinate the ERNs participation to the different initiatives ongoing in the RD research field (EJPs RD)

# European Joint Programme on Rare Diseases (EJP-RD)



- Accepted by EC in August 2018. **Grant agreement awaiting signature.**
- Total minimum budget: **93,53 Million Euros**
- Total expected budget: > 110 Million Euros, with **55 Million from EC** and supplemented with national and in-kind contributions.
- 85 partners from 33 countries (including 25 EU member states)
- **Will run from January 2019-December 2024**



**Pillar 1: Collaborative Research Funding**



- WP6 Joint Transnational Calls
- WP7 Networking scheme
- WP8 RDR Challenges
- WP9 Monitoring of funded projects



- WP 10 User-driven strategic planning for P2
- WP 11 Virtual Platform for data & resources
- WP 12 Enabling sustainable FAIRness
- WP 13 Holistic approaches for rare disease diagnostics and therapeutics



- WP 14 Training on data management & quality
- WP 15 Capacity building and training of patients and researchers
- WP 16 Online Academic education course
- WP 17 ERN RD training and support programme
- WP 18 Development and adaptation of training activities



- WP 19 Facilitating partnerships and accelerating translation
- WP 20 Validation, use and development of innovative methodologies for clinical studies

**Pillar 4: Accelerating translation & Therapy development**



**Pillar 2: Coordinated Access to data & services**



**Pillar 3: Capacity Building and empowerment**





## IN SUMMARY:

- The EJP-RD is not a funding program for ERNs but **ERN HCPs will be able to apply for the joint transnational calls**
- There is still much to be defined!
- More information coming in January 2019 (creation of EJP-RD website )
- Coordinator: Daria Julkowska ([daria.julkowska@inserm.fr](mailto:daria.julkowska@inserm.fr))
  
- + from ERN: monitoring progress
- VASCERN should be informed of various applications

# InterERN Working Groups



Integrated Working Group on Knowledge Generation of the ERN Board of MSs and the ERN Coordinators Group (Clinical guidelines, Training, education, capacity building), Enrique Terol (European Commission DG SANTE)

- CPGs Survey conducted, Dec 2017
- RD-Action Workshop, Dec 2017,  
Outputs expected:
  - Thesaurus of Terminology
  - ‘Tool-kit’ on RD-diagnostic resources
  - Set of ‘Recommended Practices’ for ERNs and CPGs
- **Sept 2018:** creation of the new integrated WG between the ERN Coordinators & the Board of Member States representatives

- Aims
  - Towards a common ERNs methodology to develop clinical guidelines, supporting action (tender)
  - How to address the challenge of the lack of evidence in the area of rare diseases.
  - ERNs strategy on training & education
  - Taxonomy of technical documents / deliverables for ERNs (CPGs and other technical deliverables)
- Immediate Actions
  - Discussion & input on current state of play and needs for CPGs for ERNs
  - Identify possible actions/ tools related with “Knowledge Generation”

# InterERN Working Groups

Monitoring (Enrique Terol, European Commission DG SANTE, Guillaume Jondeau)



# Call for HCP Membership to existing ERNs



- Expected **Quarter 2 of 2019**
- ERN Call for HCP Membership to existing ERNs
  - Application to European Commission's ERN Call similar as 1st 2016 ERN call
  - National Endorsement Letter
  - Fullfiling specific RDWG criteria set up
- **Priority** given to:
  - Gaps in expertise (new rare diseases)
  - New EU Member States to broaden geographical coverage
  - Member States not represented in ERN or RDWGs
- **Brexit:** latest news
  - either UK HCPs will be out by the end of March 2018
  - or 2 years transition period
  - last moment
  - Any updates from the European Commission or UK HCPs?

# Revision of RDWG criteria and adding new rare diseases



- **No revision** of RDWG criteria after review by each RDWGs?
- **New rare diseases** to be added to MSA-WG
  - Fibromuscular Dysplasia: FMD
  - Spontaneous Coronary Artery Dissection : SCAD



# Affiliated Partners (Associated National Centers and Coordination Hubs)



- Nominated by national Member states by Dec 2018: official status
  - **Associated National Centres** for those ERNs where they do not have a healthcare provider established in their territory as full member;
  - **National Coordination Hub** which will establish a link with all Networks in which the Member State does not have a healthcare provider as member or as Associated National Centre and with which the Member State wishes to establish such link. ??
- Not fulfilling entirely the criteria set up by RDWGs
- 1 per EU Member States
- ERNs to develop integration objectives & strategies by end of Feb 2019

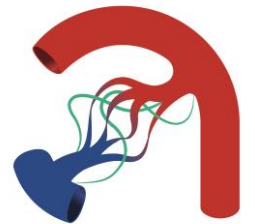
# Clarifications on official ERN Membership status



- No « Centers cooperating », not an official status
- Problem with the new call for HCP membership and confusion with interested HCPs

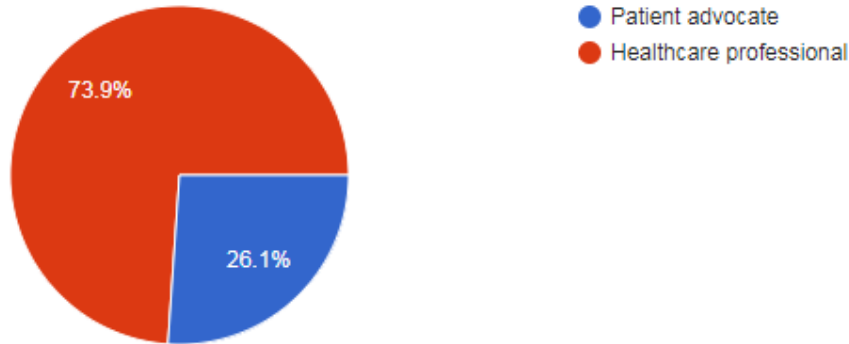
## Solutions

- Possibility of guests HCPs in CPMS
- Proposal of a new strategy for national network of expert centers (cf supra)
- Discussion also at ERN Coordinators level



# Main results of the Feedback Survey

Sent to 84 emails on 28/09/18, 23 answers



Do you feel **involved/included** and an integral part of the VASCERN network?

**20 yes**, including “I get enough contacts and feedback”

1 “I’m missing an easy to understand overview about the goals and the history behind”

1 “sort of”

1 no

Do you feel that you are receiving all necessary **information** related to VASCERN?

**21 Yes**, including “very satisfied” “sometimes more than needed”, “communication is working good” “volume of emails is too high”

1 partially

1 “I’m member of the PPL group, in the moment I do not watch the other groups”

Do you have a **specific topic/issue** that you would like to raise during the VASCERN days Board session on Thursday October 11th?

All have or will be tackled this morning

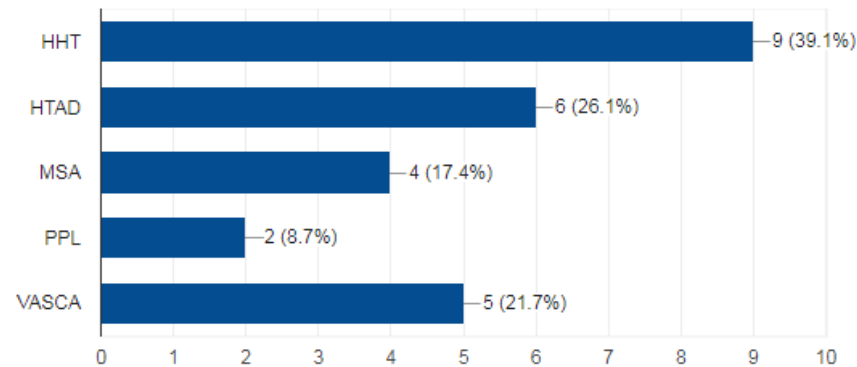
# Main results of the Feedback Survey

## Sent to 84 emails on 28/09/18, 23 answers



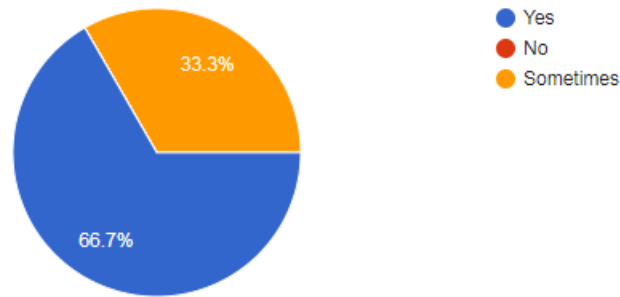
What RDWG(s) do you participate in?

23 responses



Do you read the minutes of the meetings (MoMs)?

24 responses



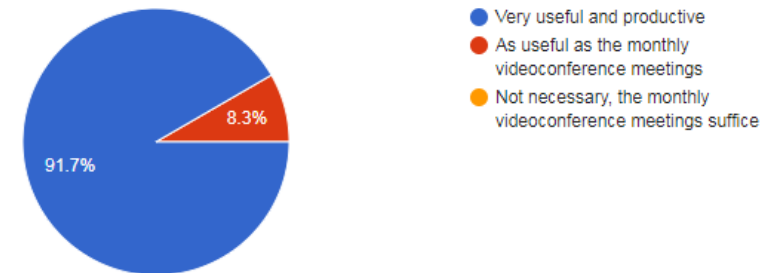
Regarding **RDWG's monthly meetings**

Overall **very good satisfaction** regarding organisation, WeBeX tool, assistance received, topics discussed, Chairs leadership, content of email received

**Suggestions** for improvement will be taken into account

How useful did you find the face-to-face meetings held by your RDWG?

24 responses



« More time » « even though less frequent I think are more productive »

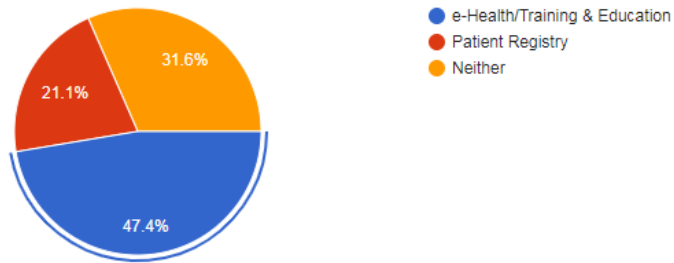
# Main results of the Feedback Survey

## Sent to 84 emails on 28/09/18, 23 answers



What transversal WG do you participate in?

19 responses



### Regarding ePAG monthly meetings

Overall **satisfied to very satisfied** regarding organisation, assistance received, topics discussed, frequency, Chairs leadership  
**Suggestions** for improvement will be taken into account

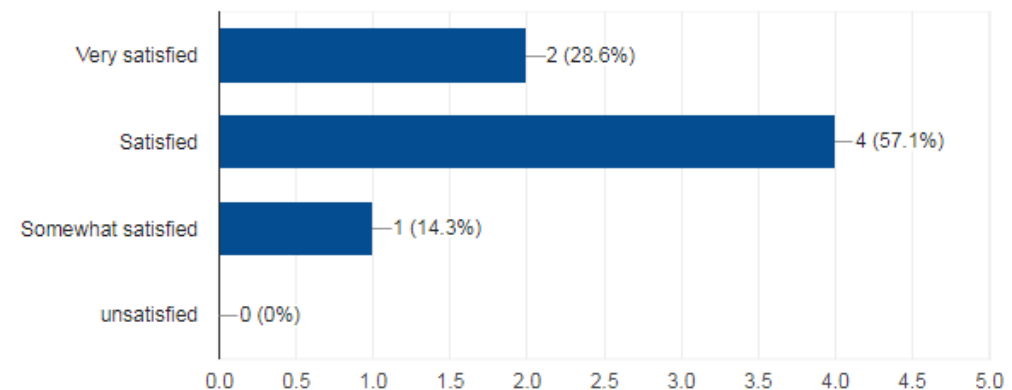
### Regarding Transversal WG meetings

Overall **satisfied to very satisfied** regarding organisation, WeBeX tool, assistance received, topics discussed, frequency, Chairs leadership, content of email received  
**Suggestions** for improvement will be taken into account

« Improve communication »

As a patient advocate are you satisfied with your involvement in VASCERN?

7 responses



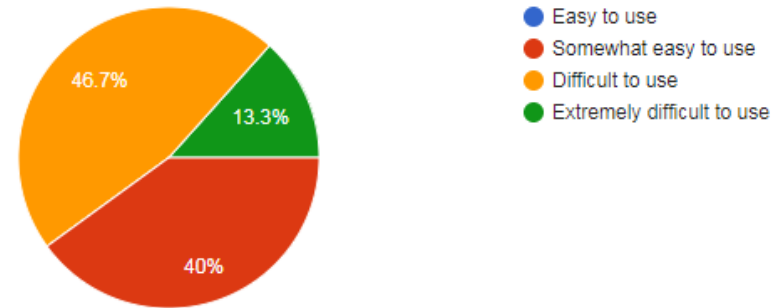
# Main results of the Feedback Survey

## Sent to 84 emails on 28/09/18, 23 answers



How would you rate the CPMS in regards to user-friendliness?

15 responses

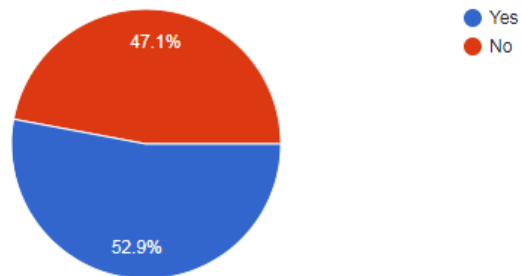


### CPMS Main difficulties

- **Time** to enter patient data. Lack of anaonymity.
- Not possible to discuss with >5 people - Firewall
- we seem to all struggle to see all the information and have a discussion - **technical difficulties** with different firewalls and operating systems etc.
- audio and video don't work with CPMS
- we could not join the panel discussion in the CPMS
- It is a big issue that we have to be **cocontrollers in the CPMS**, and I do not understand why the patients name and birthday needs to be enrolled
- no videocontact possible with other members
- Too **wieldy and difficult** to be able to discuss the number of cases we previously discussed.

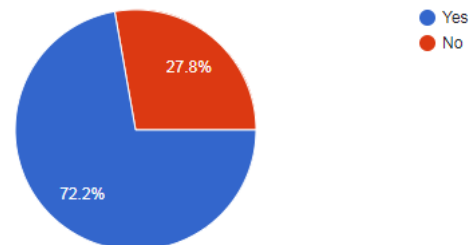
Have you participated in a panel discussion using the CPMS?

17 responses



Have you been able to receive authorisation to enrol a patient in the CPMS?

18 responses



# Main results of the Feedback Survey

## Sent to 84 emails on 28/09/18, 23 answers



### External communication

#### Website overall satisfaction (7-8/10)

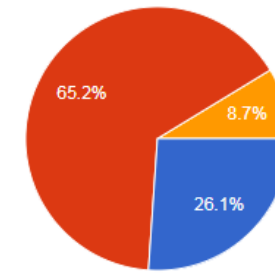
Best features “authoritative outputs from RDWGs”, “Calendar”, “Docs”, “easy to navigate”, “information”

Suggest The possibility to add/export/synchronise the Calendar with Outlook/Gmail

**Newsletters** overall satisfaction (7-8/10), info relevant, suggestion to include in email directly  
Overall very good satisfaction with **social networks** (Twitter, Facebook, YouTube, LinkedIn)

How easy is it to find the information you need on the VASCERN website?

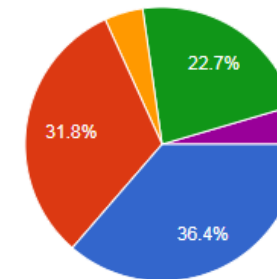
23 responses



- Very easy, I have no trouble finding information on the website
- relatively easy, I usually can find what I am looking for on the website
- not easy, I have trouble finding what I am looking for on the website

What is your principal reason for visiting the VASCERN website?

22 responses



- To find a link (e.g. to the ECP, CPMS)
- To read the latest news articles
- To refer to the calendar of events
- To visit my RDWG webpage
- To send specific links to others

# Main results of the Feedback Survey

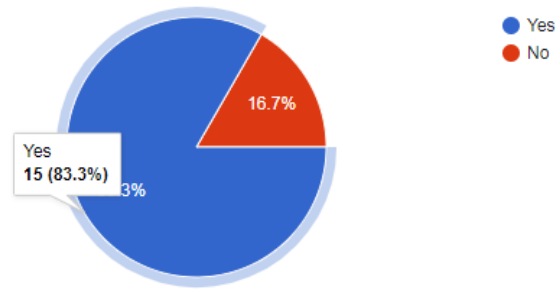
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### Governance

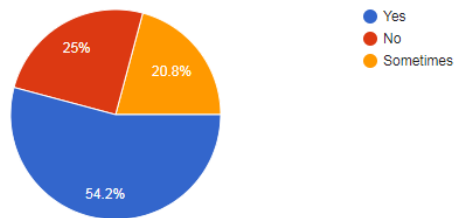
Do you feel that your feedback is relayed to the Council?

18 responses



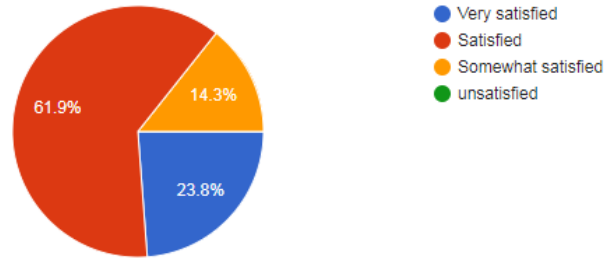
Do you read the minutes of the Council Meetings?

24 responses



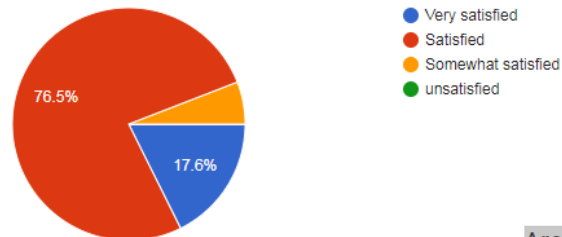
Are you satisfied with the governance structure of VASCERN?

21 responses



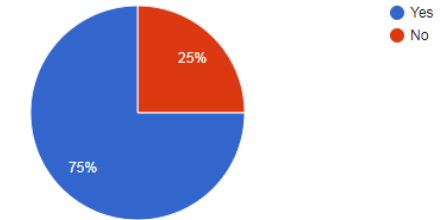
Are you satisfied with the decisions of the Council to date?

17 responses



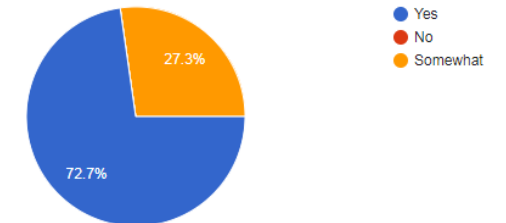
Do you think it is sufficient to meet once a year all together at the Board meeting?

24 responses



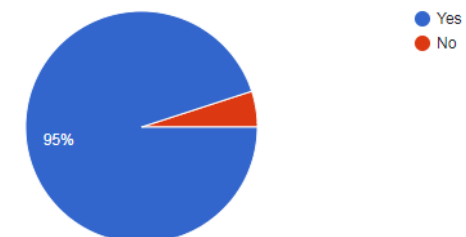
Does the Chair of your RDWG transmit the information from the Council meetings?

22 responses



Are you happy with the work packages we are working on?

20 responses







# Q&A on the Terms of References Approval

- Questions, comments on the current functioning, structure, organisation, governance
- Formal approval of current rules and projects

# Challenges and needs for the ERN sustainability and improvement - Members feedback



## Funding, HCP clinicians time

- Need of financial support for all the time researchers/clinicians put into the network
- Funding, the way I have to put time into VASCERN, looking all the issues the EU is expecting, it is NOT sustainable without funding. I keep on doing things for VASCERN, but consider withdrawing, if no funding is made available and / or EU expectations are changed.
- The principles are good and there is a lot of enthusiasm and willingness still but most of us are struggling to find enough time to devote to the project.
- Need more resources
- less bureaucracy, funding of activities - all work I put into VASCERN is free / my own time

## Improve visibility

- making the network more visible amongst doctors and researchers

## CPMS

- More time for patient discussion, whether or not in CPMS
- Platform is not working well, it is difficult
- The CPMS system has proven to be difficult to get working well for technical reasons which limit its value so far.

## New developments

- Show webinars in management, or in scientific advances, and encourage the working groups now on development (communication, research, registries, and so on)
- Standards, challenges and guidelines in genetic analysis approaches in diagnosis and research, developing new guidelines
- improve discussion on research and genetic diagnosis issues
- Need more time to discuss research projects
- To define and build work packages like research, or orphan drugs
- Need to find ways to involve HCPs more



European  
Reference  
Network

VASCERN

Gathering the best expertise in Europe  
to provide accessible cross-border healthcare  
to patients with rare vascular diseases



VASCERN, the European Reference Network on Rare Multisystemic Vascular Diseases, is dedicated to gathering the best expertise in Europe in order to provide accessible cross-border healthcare to patients with rare vascular diseases (an estimated 1.3 million concerned). These include arterial disease (affecting aorta to small arteries), arterio-venous anomalies, venous malformations, and lymphatic diseases.

VASCERN currently consists of 31 highly specialised multidisciplinary Healthcare Providers (HCPs) from 11 EU Member States and of various European Patient Organisations and is coordinated in Paris, France.

Through our 5 Rare Disease Working Groups (RDWGs) as well as several thematic WGs and the ePAG - European Patient Advocacy Group, we aim to improve care, promote best practices and guidelines, reinforce research, empower patients, provide training for healthcare professionals and realise the full potential of European cooperation for specialised healthcare by exploiting the latest innovations in medical science and health technologies.

More information available at: <https://vascern.eu>

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