



European
Reference
Network

for rare or low prevalence
complex diseases

 Network

Vascular Diseases

(VASCERN)

8.30-12.15: PLENARY SESSION

8.30-9.00: **Welcome by Prof Guillaume Jondeau and brief introduction by all participants (30')**

9.00-9.45: **VASCERN Structure & Organisation: state of play 1 (45')**

Presentation of VASCERN & Functioning since March (20') (Guillaume Jondeau, Marine Hurard)

Feedback, Q&A and discussion (25')

9.45-10.05: VASCERN Patient Group (ePAG) (20') including Q&A

ePAG involvement within VASCERN, co-presentation by the Patient Group Chair & EURORDIS (Paolo Federici, Matt Bolz-Johnson)

Q&A

10.05: coffee break

Photo booth

10.20-11:20: **VASCERN 1st year Action Plan: State of play 2 (60')**

Objectives & state of play of Work packages (Marine Hurard) & Feedback from the Council (Guillaume Jondeau and WGs Chairs: Julie de Backer, Claire Shovlin, Leema Robert, Miikka Vikkula, Sahar Mansour, Alessandro Pini, Leo Schultze Kool, Paolo Federici) (40')

Q&A and discussion (20')

11.20-12.15: **Achievements, Challenges & Opportunities for ERNs (55')**

The ERN Policy (Anna Carta, ERN Team, DG SANTE, European Commission) (10')

The ERN Collaborative platform and the Clinical Patient Management System (Jean-Marie Misztela)(10')

ERN Board of Member States views (Muriel Eliazewicz, French representative at the ERN Board of Member States) (10')

European Joint Programme on Rare Diseases Research: opportunities for ERNs (Daria Julkowska) (10')

Q&A (15')



VASCERN EPAG

**Matt Bolz-Johnson & Paolo
Federici**

VASCERN Kick Off Meeting, 13-14 October
2017

EURORDIS.ORG

Content

- Part 1: General ePAG framework
- Part 2: VASCERN ePAG involvement and functioning.

Part 1: ePAG General Framework

Patient Centre & Empowerment

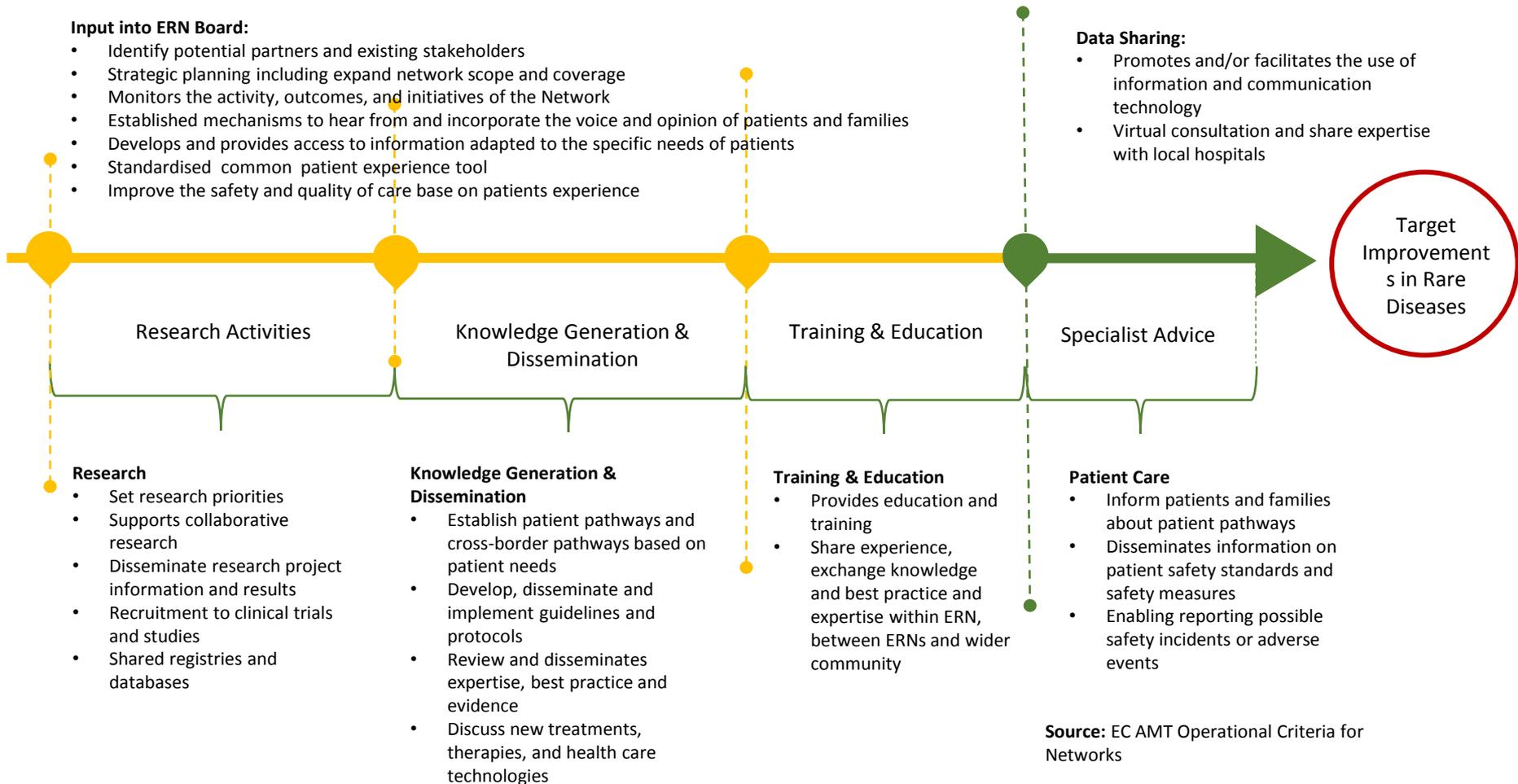
European Reference Networks (ERNs) created on founding principles of patient-centred care, patient advocate empowerment, patient engagement



European Patient Advisory Groups (ePAG):

- 24 forums for dialogue, unity & solidarity to optimise involvement of patients
- Represent patients to engage in application process & governance of RD ERNs
- Open to members & non-member patient groups in EU
- Aligned with RD ERN scope
- Composed of >150 elected ePAG reps & <1000 ePAG member organisations
- Voting Members of ERN Boards, equal voice!
- Recruitment continue for ALL disease specific clinical networks / committees and transversal working groups

ERN Blueprint for Patient Involvement



ePAG Guide : Foster Engagement & Involvement

Patient Representative Role in ERNs

Patient representatives, clinicians and researchers share a common ambition to find answers, treatments, and ultimately cures for people living with a rare disease, to enable them to lead better lives. Whilst we share this common ambition, we draw on different experiences, expertise and knowledge, and see the world from different perspectives. Understanding the patient representative's role in an ERN will enable the patient-clinician partnership to deliver on this shared ambition, successfully aligning these perspectives, experiences, expertise and knowledge and optimising the investment of resources – time, energy and funding – to fully unlock the true potential of ERNs.

The objective of patient representatives is to provide the patient perspective. The investment of their capacity, time and experiences for patient or carers representatives is at three levels:

1. Patients as a partner in their own care;
2. Patients contributing to the delivery of a common goal with each Network HCP Member, locally to their region or country;
3. Patients as a 'Patient Resource' invested at a network and European level.

This Guide focuses practice guidance to optimise the invest of patients as a 'Patient Resource' into ERNs, imparting their experiences and knowledge and to actively participate in the different groups, committees and boards. However, to do this successfully they will need to be actively involved in executive and clinical discussions and debates, and may need support to understand and digest technical, scientific, legal and medical terminology and concepts.

Patient representatives hold a privileged position in ERNs and have a valuable contribution to make into the strategic development and operational delivery of these networks. Recognising this by all – from patient representatives, clinicians and researchers - will be the deciding factor of the success of ERNs. The contribution and authority of clinicians and researchers have been honed-in and established throughout their professional lives and careers. Patient representatives' contribution draws on their real-world experience of living with a rare disease, understanding it's impact on their health and wellbeing and on their family. Due to the rarity and complexity of many multisystem rare diseases, patients ultimately become the expert in their rare disease, through their real-world experience as many clinicians may only see one case in their career.

Defining the Patient Representatives Role

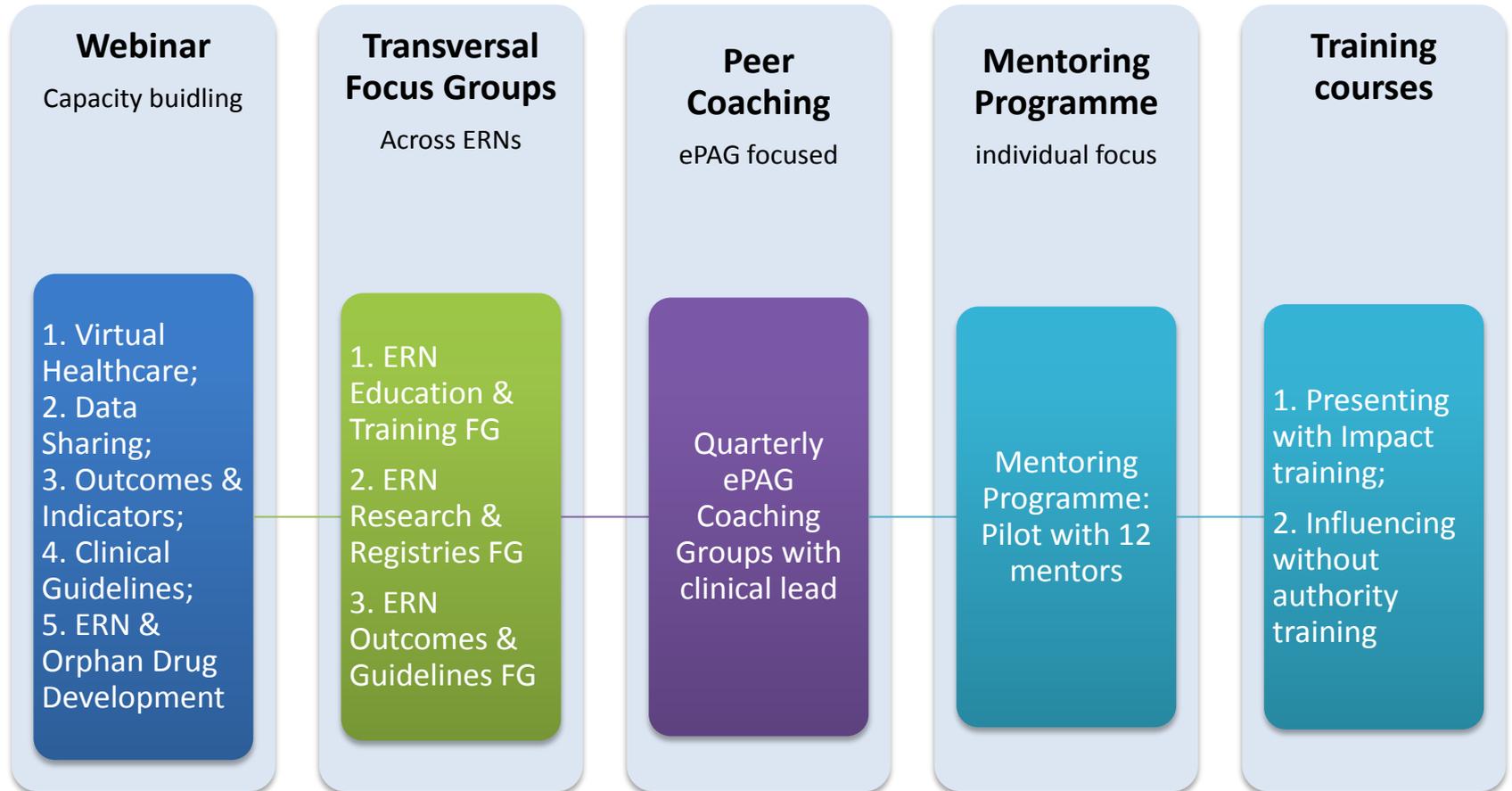
Patient representatives have a wealth of experience that can be draw on in the operational delivery of ERNs. Defining the patient representative's role in ERNs, enables the patient representatives, clinicians and researchers, to hold share expectations of their contribution into the ERN discussions, and in turn optimises their involvement.

Patient representative's role can be defined, but not limited, to four fundamental functions:

1. Patient representatives hold the unique knowledge or personal experience of a specific disease, able to convey the needs and challenges of living with this disease into network discussions, decisions and activities.
2. They are the only stakeholders who hold a holistic view of the whole process – from diagnosis, care and treatment, from early research through to orphan drug authorisation – only patients have a stake in every stage of the research and healthcare pathways.
3. Patient representative are the only ones who can ask 'the stupid questions' as these are often the most relevant and insightful questions that should be asked and are often not.
4. Hold skilled and experienced from other sectors, outside the medical/research field, that can offer a new or alternative perspectives into network discussions.



ePAG Leadership Programme



European Patient Advocacy Group Satellite Meeting



Online Communities

EURORDIS has developed online communities for ePAG representatives and rare disease patient communities to connect up.



- To discuss and exchange information within each of the 24 European Reference Network.
- Created 24 Facebook Workplace, professional network, in which we invite ePAG representative to participate.

ERN Common Patient Experience Survey

Rare Barometer Voices is a **panel of people living with a rare disease who participate regularly in EURORDIS surveys and studies.**

Exploring the possible developing this platform to be the centralised patient feedback mechanism across the ERNs will provide information in order:

- To **align strategic decisions and operational delivery of the ERNs with patients' needs and experiences**, specifically the development of healthcare pathways and treatment protocols;
- To **enable ERNs to capture the experience and feedback** of multisystem rare disease patients treated in one or multiple networks.



Avoiding duplication of efforts through **pooling of resources** to drive **economies of scale**;



Ensuring **robust, comparable and independent validated data and results** by using the same survey methodology across ERNs;



Achieving **sufficient survey sample size and coverage** of the ERNs patient population.



Covers 48 European continent countries, in 23 languages; goal of 20,000 people.

Part 2: VASCERN ePAG

VASCERN European Patient Advisory Groups

**Vascular Anomalies
(VASCA)**



**Hereditary Haemorrhagic
Telangiectasia (HHT)**



**Pediatric and Primary
Lymphedema (PPL)**



**Medium Sized
Arteries (MSA)**



**Heritable Thoracic
Aortic Diseases (HTAD)**



VASCERN Patient Group (ePAG) Chair & Co-Chairs

ePAG representative	Patient organization	Member State	Rare Disease Area
Paolo FEDERICI (Chair)	HHT Onilde Carini	Italy	HHT
Valentina FAVALLI (Co-Chair)	Magica Onlus	Italy	HTAD
Claudia CROCIONE (Co-Chair)	HHT Europe	Italy	HHT
Jurgen GRUNERT (Co-Chair)	Ehlers-Danlos-Initiative e.V.	Germany	MSA
Caroline Van Den BOSCH (Co-Chair)	HEVAS	The Netherlands	VASCA
Natascha ASSIES (Co-Chair)	NLNet	The Netherlands	PPL

ePAG Representatives

ePAG representative	Patient organization	Member State	Rare Disease Area
Patrice TOUBOULIE	MARFANS	France	Marfan
Luisa BOTELLA	HHT Espana	Spain	HHT
Christina GRABOWSKI	Morbus Osler	Germany	HHT
Ange Van Der VELDEN	LGD Alliance Europe	Netherlands	LGD
Romain ALDERWEIRELDT	ABSM Association Belge du Syndrome de Marfan	Belgium	Marfan
Franziska Haiml / Maria Barea	VASCAPA	Belgium	Vascular Anomalies

What the VASCERN ePAG wants:

- Access to qualified care for specific rare disease
- Effective therapies (that can be an ambitious task in the rare disease field)
- High levels of awareness of the disease to improve levels of diagnosis.
- Patient friendly delivery of information.



half empty
or
half full ?

The half empty was (now thanks to the ERN/ePAG it is much better)

- Few experts teams/centers
- Poor collaboration/information exchange between different medical actors
- Scarcity of sound data and guidelines for rare diseases
- Lack of local/national protocols to guide management of rare diseases

Expected outcomes of Patient rep involvement in VASCERN WGs.

- contributing to research and setting patients priority
- highlighting difficulties in access to care
- developing guidelines that are inclusive of the patient perception and preference
- disseminating **INFORMATION** and contributing to producing patient friendly guides
- linking patient advocacy groups to potential new HCPs interested in focus on our conditions

What the Vascern ePAG has achieved

- Valentina Favalli was asked to develop the first draft of the Patient Pathways for HTAD
- Transveral WG on Registries: patient rep Claudia Crocione
- Transveral WG on Ethics: Romain Alderweireldt, chair of the WG
- Paediatric Lymphedema WG: new patient rep Natascha Assies
- Training: Christina Grabowski and Karen Topaz Druckman attended the ISS Summer School on Registries
- All of us contributed to the Pills Knowledge/Mobile app (developed by Alessandro Pini)

What are our goals for 2018

- To be represented in ALL transversal groups
- Being recognized as serious stakeholders
- Activating more patient organisations to join
- Stimulating research

Thank You

