



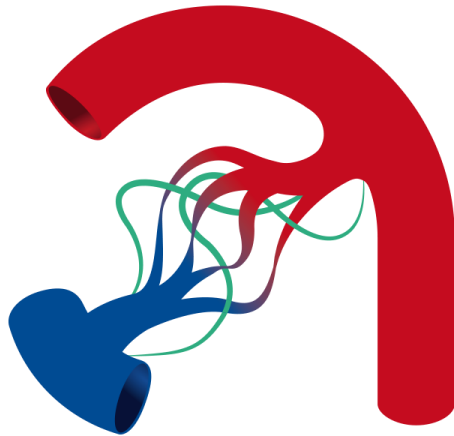
European Reference Network

for rare or low prevalence complex diseases



Network

Vascular Diseases
(VASCERN)



 **#VASCERNdays2020**

11.15-11.30 coffee break

11.30-11.45

European Patient Advocacy Group (ePAG) views, projects & new developments, including Q&A

Juergen Grunert (Chair)

Caroline van den Bosch

Claudia Crocione

Pernille Henriksen

Elena de Moya Rubio (Co-Chairs)

<https://vascern.eu/expertise/epag/>



ePAGs in the Rare Disease Working Groups

RDWG	Co-Chair	Deputy
<ul style="list-style-type: none">Hereditary Haemorrhagic Telangiectasia (HHT)	Claudia Crocione	Christina Grabowski
<ul style="list-style-type: none">Heritable Thoracic Aortic Diseases (HTAD)	Elena de Moya Rubio	Francoise Steinbach
<ul style="list-style-type: none">Medium Sized Arteries (MSA)	Juergen Grunert	Charissa Frank
<ul style="list-style-type: none">Pediatric and Primary Lymphedema (PPL)	Pernille Henriksen	Manuela Lourenco Marques
<ul style="list-style-type: none">Vascular Anomalies (VASCA)	Caroline van den Bosch	Maria Barea



Joint Activities

- Review of Terms of Reference for Patient Advocates
- Expansion of the ePAG Groups
- Participating in the first Eurordis Leadership School
- Monthly Meetings
- Exchange of best practice examples



COVID 19 related action

- We kept our monthly online meetings ongoing !!
- We assisted in developing special COVID-19 bulletins within the RDWGs
- We monitored the overall national situations as well as the disease specific limitations and restrictions



HHT

- Co-chair - Claudia Crocione - HHT Europe



- Deputy co-chair - Christina Grabowski - Morbus Osler Self-help group Germany



- The HHT ePAG is composed of 12 Advocates.
- Communication is conducted through dedicated online meetings (approx. every 2 months), email updates and surveying.



HHT - Highlights of 2020 Activities

- Patient Pathway: A team of 4 ePAGs (Germany, Sweden, Netherlands and Italy) are working on the Patient Pathway - considerable progress in structure and content of the survey and agreement on dissemination to ensure large participation. Limitations in forward steps due to COVID 19 and GDPR details that need to be defined and solved.
- New Additions: Possible enlargement of the ePAG with a delegate from the newborn Finnish organization.
- Covid 19 HHT think tank - March - June 2020- ePAG delegates invited to a weekly meeting organized by HHT Europe to bring together all front line volunteers for HHT to exchange views, best practices and coordinate solutions to major challenges of the HHT patient community.
- Dissemination of the Rare Barometer Survey on Covid and Rare diseases: the ePAG group helped disseminate this important survey to the national patient communities through national PO communication instruments.
- Involvement of the HHT ePAG in the CAVM Position Paper developed by the HHT WG.



HTAD ePAG

- Co-Chair: Elena C. DE MOYA RUBIO - Marfan Hilfe (Deutschland) e.V., Germany
- Deputy Co-Chair: Françoise STEINBACH - Association Marfans, France

Diana MAAS,

De Contactgroep Marfan Nederland, The Netherlands



Romain ALDERWEIRELDT

Association Belge du Syndrome de Marfan ABSM, Belgium





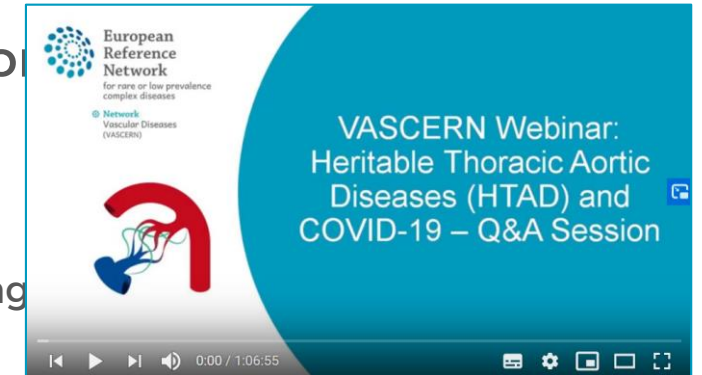
HTAD

- The HTAD ePAG is composed of 4 Advocates. Effort to enlarge the group is an ongoing task, but difficult.
- Communication is conducted through dedicated online meetings (every month) and email.
- Participation in the Ethics WGs (Romain ALDERWEIRELDT).
- Testing and corrective action for the VASCERN App.
- Teaming up with other ePAG Advocate on national level to approach National Governments to integrate ERNs in National Health Care Systems.



HTAD Education

- Participation in the E³ Summit (08/2020): 6 sessions presenting our work at VASCERN and about Lifestyle modifications and QoL in English, Spanish, French, Dutch and German:
 - Patient to Patient Support (English, Spanish, Dutch and German)
 - After Cardiac Surgery Care and Management (English)
 - Parent-to-Parent Support for Children with Neonatal Marfan Syndrome (French)
- Pills of Knowledge & Videos, including subtitles and translation
 - COVID-19 Q&A, 152 views;
 - Role of Patient Advocates in a ERN, 177 views (cc: 4 languages);
 - Le Sport et le Syndrome de Marfan, 1.732 views (cc: 7 languages);
 - 3 a week - the importance of exercise in Marfan and HTAD, 835 views (cc: 7 lang



→ The big difference in numbers reassures us, that not everybody watches videos in English.

The more languages, the more views.



HTAD Clinical Decision Support Tools

- Development of patient driven Clinical Outcome Measures for HTAD which with the Patient Journey will help to add patient needs to the Patient Pathway. (Session on Friday)
- Patient Journey on stand-by until the GDPR issue resolves (Consent Form).
- Translation of the Do's and Don'ts Factsheets for Marfan in 6 languages.
- Participation in the basic discussion on the HTAD Registry Project.





MSA

- Co-Chair Juergen Grunert Ehlers-Danlos Initiative, Germany
- Deputy Co-Chair: Charissa Frank, Bindweefsel, Belgium





MSA Highlights of Activities 2020

- MSA Patient Pathway for Vascular Ehlers-Danlos Syndrome
- Do's and Don'ts Factsheets for Rare Vascular Disease Patients
- Subtitles and Translations for our POK Video
- Bringing Patient's needs to the „Vascular EDS and Pregnancy“ topic
- Testing and corrective action for the VASCERN App
- Participation in the basic discussion on the MSA Registry Project
- Start developing the „Patient Journey“ (difficult, low response from PO's)



PPL

- Co-Chair Pernille Henriksen DALYFO, Denmark



- Deputy Co-Chair Manuela Lourenco Marques, ANDLymph, Portugal





PPL Highlights 2020

- Pills of Knowledge video validated at VASCERN days 2019 reached more than 1000 views with subtitles in 9 different languages
- Update and inclusion of additional patient associations to the VASCERN app and Orphanet website
- Extensive work on patient journey with GDPR being a major obstacle with still unsolved issues for some countries
- In the process of creating 6 new Pills of Knowledge videos based on the Dos and Don'ts with subtitles in 10 different languages



VASCA ePAG



- Co-Chair Caroline van den Bosch HEVAS, Netherlands
- Deputy co chair Maria Barea VASCAPA, Belgium



Petra Borgards

Bundesverband Angeborene Gefäßfehlbildungen e.V. (Germany)

Aaike van Oord

Lymphangiomatosis and Gorham's Disease Alliance Europe (Netherlands)



Lex van der Heijden

CMTC-OVM (Netherlands)





Cooperation with other groups and institutions

- Participation in Traversal WGs (Petra Borgards and Maria Barea)
- Participation in EJP RD NL (Aaike van Oord)
- Focus on forming National Networks of Centres of Expertise in cooperation with National Alliances for Rare diseases
- Teaming up with other ePag on national level to approach National Governments to integrate ERNs in National Health Care Systems



Projects and initiatives

- VASCA WG Covid-19 statement initiated by ePag Europe and USA
March 2020
- ePag WG poster with 3 PoKs was presented at ISSVA:
 - The lymphatic system & lymphatic malformations
 - Treatments for lymphatic malformations
 - Description of the Klippel-Trenaunay Syndrome
- New PoK on PIK3CA (HEVAS)
- Patient Journey (LGDA and CMTc)



VASCA exposure and research

- The development of the VASCA magazine by our 5 POs
- Promotion of VASCERN via (social) media and renewed websites
- VASCERN App: adaptations and efforts to enlarge the ePag community
- Cooperation on genetic research with VASCA WG by LGDA and CMTC
- Connection VASCA WG with Novartis USA with regards to the NOVARTIS European trial of BYL719

Thank you, any questions?



Contact details

- **Bundesverband Angeborene Gefäßfehlbildungen**
 - Petra Borgards (borgards@angiodysplasie.de)
 - Website: [https:// www.angiodysplasie.de](https://www.angiodysplasie.de)
- **CMTC-OVM**
 - Lex van der Heijden (president@cmtc.nl)
 - Website <https://www.cmtc.nl/en>
- **HEVAS**
 - Caroline van den Bosch (caroline@delaforet.nl)
 - Website: <https://www.hevas.eu>
- **LGDA Alliance Europe**
 - Aaike van Oord (aaike@moveout.nl)
 - Website: <https://www.lgda.eu>
- **VASCAPA**
 - Maria Barea (msbarea@yahoo.com)
 - Website: <https://www.vascapa.org>