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**European Reference Network (ERN) on Rare Multisystemic Vascular Diseases (VASCERN), SGA Proposal 2017**

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European Reference Network (ERN) on Rare Multisystemic Vascular Diseases (VASCERN), SGA Proposal 2017

**JA2015 - GPSD [705038]**

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Project abstract

This is the VASCERN SGA Proposal for 2017, under the ERN Framework Partnership Agreement (FPA) 2017-2021. The ERN is coordinated by the ERN Network Coordinator, Pr. Guillaume JONDEAU - AP-HP, Hôpital Bichat-Claude Bernard, CRMR Marfan Diseases and related disorders (Centre de Référence Maladies Rares Syndrome de Marfan et apparentes).

VASCERN aims to facilitate and improve diagnosis, treatment and care for ALL patients suffering from Rare Multisystemic Vascular Diseases, thus enhancing access to care, and improving quantity and quality of life of these patients. Networking, sharing and spreading our expertise, promoting best practices, guidelines and clinical outcomes, patient empowerment, improving knowledge through clinical and basic research are among our objectives.

The European Reference Network on Rare Multisystemic Vascular Diseases gathers European highly specialized multidisciplinary Healthcare Providers (HCPs) in this thematic area of expertise. The Network includes 5 Rare Diseases Working Groups: Heritable Thoracic Aortic Diseases (HTAD-WG), HHT-WG, Medium Sized Arteries (vascular Ehlers Danlos) (MSA-WG), Pediatric and Primary Lymphedemas (PPL-WG) and Vascular Anomalies (VASCA-WG). The specific Patient Group enables Patient representatives to work on common issues and to be involved in all activities. In addition, several Thematic Working Groups are established to better tackle transversal issues which are planned in the Multiannual Work Programme on: eHealth, Training & Education, Patient Registry, Ethics, and Communication / Dissemination.

Summary of context, overall objectives, strategic, relevance and contribution of the action

Context
In order to implement the European Union’s Directive 2011/24/EU on the application of patients’ rights in cross-border healthcare, the European Commission (DG SANTE) has launched the first call for proposal for the creation of the European Reference Networks for rare, low-prevalence and complex diseases on March, 2016. VASCERN, a network of highly specialized experts in rare multisystemic vascular diseases, has been constituted and applied in June 2016 to this first ERN call for proposals. Simultaneously, VASCERN has applied to the call for EU co-funding from the 3rd Health Programme of the European Union, managed by CHAFEA (The Consumers, Health, Agriculture and Food Executive Agency).

European Reference Networks (ERN) are virtual networks involving healthcare providers across Europe. They aim to tackle complex or rare medical diseases...
or conditions that require highly specialised treatment and a concentration of knowledge and resources. To review a patient’s diagnosis and treatment, ERN coordinators convene a “virtual” advisory board of medical specialists across different disciplines, using a dedicated IT platform and telemedicine tools. This way it is the medical knowledge and expertise that travel rather than the patients.

The first 24 European Reference Networks (ERNs) on Rare Diseases have been approved by the ERN Board of Member States (BoMS) in December 2016, following this first EU Call and an assessment conducted by an Independent Assessment Body mandated by the European Commission. The ERNs have received the ERN label for their first 5 years of operation, and have been officially launched in March 2017.

According to Vytenis Andriukaitis, the European Commissioner for Health and Food Safety: “The value of EU collaboration is particularly clear in the case of rare and complex diseases. [...] How can ERNs improve the lives of Europeans? Patients with rare and complex conditions will be able to benefit from the best treatment and advice available for their specific condition. Their doctors will have access to a highly-specialised pool of colleagues from all over Europe. In the first phase, over 900 healthcare units will work together in 24 thematic networks, ranging from childhood cancer to immunodeficiency.”

VASCERN, coordinated at the Hospital Bichat (Assistance Publique-Hôpitaux de Paris) has signed a 5 years Framework Partnership Agreement (FPA) with the European Commission in order to be able to receive annual grant agreement to support VASCERN’s coordination and networking activities. Under this FPA, VASCERN has signed a Specific Grant Agreement (SGA) for its first year of official activities, running from March, 1st 2017 to February, 28th, 2018.

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. These rare and complex diseases require a multidisciplinary approach to patient care, which involves many different specialists belonging to the HCPs Members’ teams.

VASCERN currently consists of 31 highly specialized multidisciplinary Healthcare Providers (HCPs) from 11 EU Member States and of various European Patient Organisations (ePAG) 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
Methods and means

Overall method: Network Coordination and Conduction of various virtual and Face-to-Face VASCERN Meetings
The importance of people meeting each other’s is crucial when a network is set up; the number of meetings held during the first year illustrates the interactions within VASCERN between the different members. During this first year of operation, the first VASCERN Annual Seminar was held during two days in October 2017, Paris (the VASCERN Days 2017 Full Report (one of this year deliverable) is available at this link: https://vascern.eu/wp-content/uploads/2018/01/VASCERN-1st-Annual-Seminar-2017-Report-1-1.pdf). This event enabled our Board to meet formally once this year, and an Assembly meeting to take place with more Healthcare Providers and Patient Representatives. Our Council has met 3 times during the year (one face-to-face and 2 virtual meetings), as planned in our Terms of Reference. In addition, our Working Groups (5 Rare Disease Working Groups and 2 active Transversal Working Groups) have held in total an average of 6.75 monthly meetings during the year (a total of 81 meetings during the year).

Means: Networking & Coordination activities

1/ Virtual tools: Both the WebEx system and the ECP are provided by the European Commission to all of the 24 European Reference Networks on rare diseases.

One of the core principles of the ERNs being that the knowledge should travel rather than the patients; we are therefore also very dependent on these IT platforms as we are indeed a European “virtual” network. The ERN Collaborative Platform (ECP) is provided by the European Commission (ECP) as a Core Service in order to share our documents, a common agenda, a forum for discussion between experts, and to lower the amount of emails and ease communication between all partners. This is our internal communication tool. With more than 500 posts, VASCERN is very active and actually the most active ERN user of the ECP.

2/ Organisation of Networking activities, virtual and face-to-face meetings:
The second important mean is the organisation of the networking activities (both face-to-face and virtual) using the co-funding provided by the European Union 3rd Health Programme.

Methods and means to implement our 11 Work Packages

We have detailed in our technical periodic report the specific methods and means for our of our 11 Work Packages.

To summarize, VASCERN’s framework offers the opportunity to share expertise between medical experts, which is one of the main added value of the ERN. Conducting monthly meetings for each of the Rare Disease Working Groups (RDWGs) and for the transversal Working Group and the European Patient Advocacy Group (ePAG) is the overall method to discuss the points necessary to advance on the implementation of our action plan. In addition to our 1st Annual Seminar, these monthly meetings enable to progress and discuss our work packages, including the cases discussions (WP1) patient pathways (WP2), the Pills of Knowledge (WP4), the registries (WP5), the clinical trials and research studies (WP6), the clinical outcome measures (WP8), the clinical...
guidelines and the do’s and don’ts recommendations (WP9 and WP10). The eHealth and Training/ Education WG meetings were important milestones to implement the WP3 on the creation and development of the VASCERN App and our eLearning tools (WP4 Pills of Knowledge, WP7 Availability of conferences on YouTube), as well as to advice on the WP11 Communication, implemented by the VASCERN Coordination team. The Patient Registry WG have held meetings, allowing lots of progress with regard to our WP5 Registries. The Coordination team provides VASCERN project management, coordination, communication and the organisation of meetings, among others. In addition, using the co-funding provided by this grant agreement, we are working with service providers for the creation and development of our mobile application (developer), our website (webmaster), design of our communication t

Work performed during the reporting period

During the VASCERN Specific Grant Agreement for Y1, covering the period between March, 1st 2017 and February 28th, 2018, VASCERN’s main activities was to implement our action plan’s 11 work packages thanks to the networking and coordination activities outlined in the above part and to the very high commitment of the experts involved without funding.

WP1: Sharing of experience: discussion of difficult cases
Description of activities: VASCERN’s experts have started to share their experience, expertise and knowledge regarding complex clinical cases

WP2: Definition of patient pathways
Description of activities: Development of a patient pathway within each RDWG, indicating the optimal mode of evaluation of a patient for diagnosis, the optimal evaluation of the patient once the diagnosis is made, the optimal follow-up and the optimal therapy.

WP3: Development and implementation of Cross-border pathways Mobile Application
Description of activities: Development of a free of charge VASCERN cross-border Mobile App for smartphone IOS & Android, sharing information on HCPs and Patient Organisations (services offered, how to reach HCPs, contacts, etc.).

WP4: Pills of knowledge
Description of activities: Development of the VASCERN “Pills of Knowledge”, which are series of short single video-lessons (3 to 10mins maximum) in which experts talk about a single brief topic.

WP5: Registries
Description of activities: Important work conducted by the Registry WG and its technical task force towards the implementation of a VASCERN registry and a FAIR pilot project.

WP6: Clinical trials
Description of activities: Communication on the clinical trials and research projects, scientific publication. Research studies and clinical trials conducted
within the network.
WP7: Availability of conferences on YouTube
Description of activities: A specific VASCERN YouTube Channel has been set up in order to share scientific meetings held by our Members, VASCERN conferences, scientific congress events, patient organisation meetings and the Pills of Knowledge.
WP8: Definition of clinical outcomes
Description of activities: VASCERN RDWGs have worked on the definition of clinical outcomes (simple and disease specific).
WP9: Writing Recommendations: Clinical Guidelines
Description of activities: VASCERN five RDWGs have started to work on clinical guidelines (identification, collection, selection, assessment of the existing and writing first guidelines).
WP10: Writing Recommendations: Do’s and Don’ts Factsheets
Description of activities: Writing and validation of the Do’s and Don’ts Factsheets for Rare Vascular Disease Patients facing common situation.
WP11: Communication
Description of activities: VASCERN has created and developed several important communication tools during this first year of activities (website; monthly newsletters; social media pages: Twitter, Facebook, YouTube; communication tools with a graphic designer; translation of content for patient; internal communication on the ECP).

The main output achieved so far and their potential impact and use by target group (including benefits)

A) Structure & Functioning: a new ERN
A major result of this first year is actually the setting up of VASCERN as a network: functioning as an institutionalised ERN, with a structure, a governance, a functional coordination, a coordination team, monthly meetings, an efficient communication and dissemination strategy with newly established tools. Illustrations are the numbers of meetings held over the year and the great success of the VASCERN Days 2017 and all of the outcomes outlined in this report.
This is a major result and has enabled to set up a very strong basis for the following years under the Framework Partnership Agreement.
B) Major results linked to the Work Packages activities and outcomes
WP1: starting to share knowledge and expertise with regard to complex cases between highly specialised experts within the framework of the ERN in order to improve care. More cases proposed, CPMS tested.
WP2: defining and finalising the first VASCERN patient pathways available for testing, publication and dissemination
WP3: creating and developing a VASCERN Mobile application as a tool for
patients to easily find and reach out the expert centers they need and the patient organisations in the European Union, therefore facilitating cross-border healthcare.

WP4: Online upload of the first 5 Pills of Knowledge videos available on YouTube with one reaching already more than 600 views in 3 months, first content translated for patients (translation of patient information material).

WP5: Work towards the implementation of a VASCERN Registry complying with the FAIR principles. Decision made to support a VASCA FAIR registry pilot project. RDWG specific registry projects ongoing, including the MSA-WG European cohort project enabled by VASCERN.

WP6: Lots of research projects, studies underway within VASCERN, many collaborative. Encouraging and fostering new scientific cooperation in the field of rare vascular diseases. A Comprehensive annual report on clinical trials and research studies including collaborative and HCPs publications available for publication will be published (deliverable).

WP7: Launch of the YouTube Channel, populated with 75 videos.

WP8: Definition of the first simple and disease specific outcome measures, ongoing work. Paper submitted by the HHT-WG on how these 5 Outcome Measures provide metrics to identify healthcare providers of good care, and encourage care improvement by all healthcare providers is discussed and explained further within VASCERN HHT’s first manuscript, currently in review: Shovlin, Buscarini et al: European Reference Network for Rare Vascular Diseases (VASCERN) Outcome Measures for Hereditary Haemorrhagic Telangiectasia (HHT) in review Feb 2018.

PPL-WG Quality of Life face to face meeting, working on outcome measures for QoL.

WP9: Identification, collection, selection, appraisal of the existing national, European and international guidelines in order to anticipate the writing of new recommendations. Writing of the 2 first VASCERN expert consensus statements, work in progress for a second HHT expert consensus statement.

1/ Shovlin, Buscarini et al, European Reference Network for Rare Vascular Diseases (VASCERN) Outcome Measures for Hereditary Haemorrhagic Telangiectasia (HHT) submitted 12.02.2019. (As the paper remains in the journal review process, it is not yet available for public release.) + In progress: a second statement on NOACs (new oral anticoagulants). the first draft has been circulated and reviewed, and it will be developed to publication in Year 2.

2/ HTAD-WG expert consensus statement on ACTA2 related HTAD: work in progress.

WP10: First 3 RDWGs Do’s and Don’ts Factsheets are ready for official publications.

WP11: Establishment of an efficient communication and of new tools (website, monthly newsletters, active communication on social media: Twitter, Facebook, YouTube and now LinkedIn) with good results and outreach. The benefits and potential use are important for our target group.
Achieved outcomes compared to the expected outcomes

Although some of the targets set up in the initial plan were not fully reached, overall we have achieved most of the outcomes expected for this first year of activities as outlined in the previous section. The expected outcomes which are not fully reached are mainly due to the delay with regard to the availability of the CPMS for our WP1, and due to the lack of expert time and funding to fully reached some of the objectives (Pills of Knowledge targets have been reviewed and there are now 5 Pills of Knowledge videos on our YouTube Channel, providing eLearning to clinicians and patients). Processes to produce clinical guidelines and to define the consolidated patient pathways, the clinical outcomes and create a registry need more times. However, there are already 2 expert consensus statements generated, consolidated and draft patient pathways produced and do's and don’ts factsheets for rare disease patient care ready for publication. Several registry projects are launched, including a VASCERN Registry FAIR pilot project. VASCERN has encouraged and fostered scientific cooperation, and new scientific research projects have been launched. Not all the clinical outcomes measures were defined, nevertheless a HHT-WG paper about outcome measures in under journal review for publication. Our VASCERN App is underway to be publicly launched in the upcoming weeks, as the collection of data and development took more time than expected. Our communication activities and results are meeting our expected outcomes.

Dissemination and evaluation activities carried out so far and their major results

The dissemination activities towards our identified target groups have been very important during the course of the project and also after, as the project is running under a 5 years framework partnership agreement, the dissemination activities are not stopping.
Main dissemination activities are:
1/ VASCERN’s Website, diffusing all information about the network and its activities
2/ VASCERN’s Social Media pages (Twitter, Facebook, YouTube Channel and now the LinkedIn (established in March, 2018))
3/ VASCERN’s Monthly Newsletters, which are sent to a broad audience (large diffusion list and publication on our website) including all the patient organisations registered in the EURORDIS database for the VASCERN European Patient Advocacy Group, so that the patients and their families are widely targeted. These Newsletters include all of our results, outputs, news so it is a very important way for VASCERN to disseminate information.
4/ Internal dissemination of information on our ERN Collaborative Platform (as
underlined in the first part of this report, VASCERN is the most active ERN user of the ECP with more than 500 posts)
5/ Dissemination via our partners’ websites and social media (Healthcare Providers and Patient Organisations)
6/ Work on the diffusion list with our members to increase our communication impact
7/ Publications in in medical and paramedical journals involving VASCERN members and therefore spreading information about our network
8/ Discussions held with public policy makers
9/ Patient organisations involved: as they are fully involved within VASCERN structure and activities, patients are empowered and disseminate information, educate patients and families within the EU
10/ Translation of patient information material
11/ eHealth tools, including the mobile application under final development
12/ Common answers to research calls and other calls for proposals
13/ Dissemination of results during communication in conferences and scientific congresses, where our Members have been promoting VASCERN: our members are indeed very active in Europe and abroad, participating in scientific congresses, meetings and other events.

Overall, our communication and dissemination activity have good results for this first year (increase of users/ visits on our website and good geographical outreach, numbers of followers on our social media pages and rapid progress regarding these results, implementation of new tools and design of improved dissemination strategies in order to improve results with regard to our outcomes’ outreach).
We have received very good feedback from several stakeholders about our communication activities, including EURORDIS, the European Commission ERN policy team and other ERNs.

Evaluation activities
We have conducted several evaluation activities during the course of the project:
• At the beginning of the project, we sent out a survey to all VASCERN members, both HCP and ePAG representatives, to ascertain their motivation to join one of our transversal working groups (and if so, which one) as well as to receive their feedback on the ERN project in general (i.e. the proposal process, the proposal submitted, areas for improvement, ideas for project implementation). The majority of respondents were very happy with objectives outlined in the project and the overall process lead by the Coordination team.
• At the VASCERN days 2017, all participants (including external stakeholders) were consulted and we requested any feedback on the project during this important annual event. In addition, a satisfaction survey was sent out to all participants following our first annual seminar. The results were very positive (see our report of the Annual seminar, available online).
• As the Advisory Board was not yet constituted, the external evaluation we had during the first year is limited to the feedback received from the external stakeholders in particular those who were attending the first annual sem
Work package

Work Package 1: A- Sharing of experience: discussion of difficult cases
Start month: 1
End month: 12
Work Package Leader: APHP

VASCERN offers the opportunity for sharing experience between experts - one of the main added values of the ERN. Sharing experience at European level will be made possible using:
- Web-based platforms and teleconferencing during the monthly RDWG teleconference: difficult clinical cases will be discussed. A summary of the case, discussion and conclusions will be made available on the website and on the intranet, as decided by Chairs.
- Through e-mail via a secured mailing list including all the HCPs of the RDWG, specific question will be discussed. Similarly, a brief summary will made available on the website or on intranet, as decided by the RDWG Chairs.

Work Package 2: B- Definition of patients pathways
Start month: 1
End month: 12
Work Package Leader: APHP

It will be discussed with the respective HCPs of each WG and completed during the first 6 months of VASCERN (priority). It will enable derivation of quality of care measures and outcome measures. It will then be diffused as an educational tool, and to help new centers (in others countries). This measure is necessary before choosing relevant and consensual outcomes in the different diseases of the network. It is essential to be able to respond to the EU expectations from the ERN.
This measure is specific, measurable (number of pathways), acceptable (definition of mode of care for the rare diseases is the responsibility of VASCERN), realistic (as each HCP has already more or less
clearly defined such a pathway) and time bound (this measure is going to be implemented rapidly after VASCERN approval, because it is the necessary step to derive the first outcome measures specific for a disease).

**Work Package 3: C- Cross-border pathways Mobile Application**

*Start month: 1  
End month: 12  
Work Package Leader: APHP*

share information about the HCP from VASCERN and broader network characteristics (services offered, how to reach each HCP), an application for smart-phone IOS & Android has been developed by a Rare Disease Center in Lombardy. It is free of charge and VASCERN will adapt this program to be implemented on wider scale and in a larger set of languages so to cover the whole ERN and further national networks and HCP partners. It will allow patients throughout Europe to be able to choose the relevant HCP according to the rare disease they face (or MD to accurately choose for their patients).

This needs funding for software development (application already available, so it will only needs further development/updates), and update of data is critical for its reliability. This will be the responsibility of the ERN Project Team.

**Work Package 4: D – Pills of knowledge**

*Start month: 1  
End month: 12  
Work Package Leader: APHP*

Professional upgrade may result difficult for clinicians, in terms of time available. We plan to make it easier by the development of single video-lessons (each 7-12 minutes of duration) in which an expert talks about a single brief topic. Evaluation is included, a downloadable certificate of attendance will be available and accreditation at term. It is expected to mix specialists from different Centers/Countries and create specific modules to cover every aspect of every disease related to
VASCERN, as well every
tone of voice (i.e. levels of training/learning, from simplest to most difficult). These
“pills” of rare
disease knowledge will be available on a mobile-friendly platform and so one does
not need a PC, a
desk and can have a learning-moment virtually everywhere.
Actually, this concept will also be used without video by Cross European training of
Professionals
initially in HHT. It will be developed through a web based online course (MOOC, or
University
based), based on recent experience (2015-2016) setting up all new modules for a
new MSc in
Genomic Medicine. Modules will be part face-to-face, part online, with individual
items available as
Short Courses or standalone lectures for lesser credit. It may be feasible to expand
to a full MSc as the
Late Pr. Pierre Lasjaunias set up through ESNR.
This action will begin during the first year of VASCERN for the MOOC without video
(HHT WG
starting) and during the second year of VASCERN with the videos. The possibilities
for adaptation
and improvements are multiple

Work Package 5: E- Registries
Start month: 1
End month: 12
Work Package Leader: APHP

Registries are ongoing in numerous HCPs: local, national and international
registries. The use of
European registries faces specific problems, both technical and legal. The Registry
WG will be
responsible for these legal and technical issues (gathering the information from the
different
countries). A member of each country is expected to participate in this WG to
indicate the specificity
faced nationally. The registry WG is also expected to make the inventory of all the
existing registries
within the network.
A single VASCERN registry for all diseases covered by the network should be built,
with the capacity
of evaluation of activity and quality control (first outcome measures, to be
implemented rapidly). This
registry will progressively be implemented to analyse all the development in the
patient’s disease
(follow-up and analysis). A common minimum dataset conceivable for all RD will
initially be used in
accordance with the ERN IT platform and the JRC EU RD registration platform criteria currently under development. Links with the EU infrastructures, once set up, will be implemented. The RDWGs will have the task to determine the disease specific datasets to be implemented in this registry. A reflexion is already taking part in the framework of the 2016 Call for RD Registry for ERNs. Links with biobank data will be also studied. A reliable unique identifier must be adopted by VASCERN.

Work Package 6: F - Clinical trials
Start month: 1
End month: 12
Work Package Leader: APHP

Clinical trials are the only way to demonstrate the benefit of a new treatment, mode of follow-up and diagnostic strategy. VASCERN has therefore the ambition to promote the design and help in the realisation of international clinical trials in as many diseases included in VASCERN as possible. Therefore, VASCERN HCP Members will have to make every effort to participate in clinical trials that have been validated by the Board. All the clinical trials running through VASCERN will be listed on the website, with contact information, so that additional HCPs outside VASCERN can participate as appropriate (including cooperating and affiliated HCPs not yet included as full members). Some clinical trials are already ongoing in VASCERN. Information on publication, research and clinical trials will be regularly updated in the website. VASCERN plans include the implementation of a newsletter for calls for research collaboration or clinical trials in order to promote clinical trials. Monthly meeting of RDWG (including patient representative co-chair) will discuss new protocols, research projects and clinical trials within the area of the RDWG. Patient representatives will also have the role to promote clinical trials among the patient community. VASCERN also plan to include in its Annual Report a report on VASCERN Research Projects and Clinical Trials as requested in the ERN call, as well as publications report as appendix.
Work Package 7: G - Availability of conferences on YouTube
Start month: 1
End month: 12
Work Package Leader: APHP

A specific VASCERN YouTube Channel will be prepared in order to share scientific meetings held by HCPs of the ERN or congress events or single traditional lessons for professionals. An important part of the work will be to collect the congress events and the traditional lessons to be able to record them, or collect the recording if this is already organized. This measure is specific, measurable (number of videos available), acceptable (a public diffusion of conferences is expected), realistic (VASCERN experts are giving a lot of conferences, often recorded) and time bound (this is a continuous process, with ongoing collection of the conferences).

Work Package 8: I - Definition of clinical outcomes
Start month: 1
End month: 12
Work Package Leader: APHP

A first set of simple outcome measures will rapidly be followed in VASCERN. A specific disease will be chosen for each RDWG. This set will include simple parameters such as number of patient screened, number of patient with a given diagnosis (clinical and molecular), number of patient with a follow-up, number of patient undergoing surgery, number of patient recruited in a research study). This should be obtained rapidly (M12). A second set of outcomes will be derived after patient pathways have been implemented, and will require meetings for discussion.

Work Package 9: J.1 - Development of guidelines for optimal care of the rare disease
Start month: 1
This process implies many steps and requires organizational skills, numerous interactions between HCPs and patient representatives, meetings between experts: 1) Collect the guidelines, consensus statements, and expert position papers on the disease of interest: 3 months M1-M3; 2) Define the gap in knowledge for each disease (questions not included, guidelines not updated): M3-M4; 3) Define the area for the new guidelines to be developed (M4-M5): face to face meeting 1. Who writes what?; 4) Centralization of the manuscript by a specific person (HCP representative or another HCP member) who will be responsible for the guidelines (M5-M7); 5) Circulating the first draft among members of the disease WG and patients WG (M7-M9); 6) Making of a second draft (M9-M11); 7) Face to face meeting 2 for comments (M11); 8) Making a third draft (M11-M12); 9) Face to face meeting 3 if necessary (M12); 10) Last draft (M12-M13); 11) Circulating last draft (M13-M14); 12) Agreement on a text (M14); 13) Circulation of the text among others expert groups throughout the world (USA included) M14-M15; 14) M15: agreement (or not) on a common text on the disease studied, which will be published to appropriate journal.

The whole process should take around 1 year, including 2 face to face meetings between experts and circulation of many drafts, after definition of the scope of the guidelines which depends on the available literature. This work has to be done for each disease (e.g. Kippel Trenaunay), and sometimes independently for different aspect of a disease (e.g. treatment of HHT). Caution will be taken to not duplicate work of scientific society and to work in concert with them. This is timely bound as roughly one new document should be issued every year for each WG:

**Work Package 10: J.2- Development of guidelines for optimal care of these patients facing common problems not related to their rare diseases: Do and Don’t factsheets in common situations**

Start month: 1
End month: 12
Patients with rare diseases face frequent situations including medical situations such as abdominal surgery for any reason, dentist intervention, pregnancy. The question then arises of how to deal with frequent diseases in rare patients. The aim of this project is to propose some simple guides available to the medical community, which would indicate the particularities of care due to the fact that the patient is affected by a rare disease included in VASCERN. This has been done in France and the idea is to propose a similar service at a European level. The process should include 1) choosing the relevant situation 2) writing the simple factsheets 3) diffusion for comments 4) edition in a booklet (to be shown during meetings and disseminated through patient association), translation in several EU languages publication on the website.

Work Package 11: L- Communication

Start month: 1
End month: 12

Dissemination and awareness-raising are of major importance to share information related to health: among healthcare workers (clinicians, students, and researchers), patients, caregivers, and all target groups identified. e-Health in communication includes:

A website, mobile-friendly (L.1). A website has already been setup. It will be improved, specific pages / deliverables will be translated in different languages of EU (education material for patients) (L.2)

Internal and external communication will be set up allowing forum for professionals (reserved area). This will be used for the clinical case discussion (see above) and set up as soon as possible, depending of the ERN IT platform proposed.

Forum for patients and caregivers (L.3). This will be set up after 1 year, Newsletter and periodical minutes/deliverables/mid-term (L.4). A VASCERN monthly newsletter has already been issued, and a monthly newsletter is foreseen. Communication on news, events as well as relevant call for proposals (H2020, etc.) on the website to support circulation of knowledge, awareness-raising as well as to support European collaborative research projects, within and outside
the Network. It will also include calls for research/collaborations/patient cohorts (A specific newsletter will be issued when necessary); calls for instant pool, surveys among health operators or among patients (A specific newsletter will be issued when necessary).

Social network (L.5): Facebook/Twitter VASCERN pages (community management, Q&A for patients, promotion VASCERN and health messages (social networks) as part of health promotion campaigns...), promotion of VASCERN towards the general public and stakeholders, including public entities as well as private companies so to enhance the Network action.
## COORDINATOR

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## PARTNERS

No partners related to the current project
Newsletter
APHP
European Reference Network (ERN) on Rare Multisystemic Vascular Diseases (VASCERN), SGA Proposal 2017 (VASCERN)
Published on: 02/03/2018
Publication and dissemination of a monthly VASCERN Newsletter, development of the diffusion list

Registry
APHP
European Reference Network (ERN) on Rare Multisystemic Vascular Diseases (VASCERN), SGA Proposal 2017 (VASCERN)
Published on: 08/03/2018
Simple registry dataset (main clinical outcomes) Inventory of registries and biobanks

Simple factsheets
APHP
European Reference Network (ERN) on Rare Multisystemic Vascular Diseases (VASCERN), SGA Proposal 2017 (VASCERN)
Published on: 08/03/2018
Factsheets for RD patient facing common situation

Clinical case
APHP
European Reference Network (ERN) on Rare Multisystemic Vascular Diseases (VASCERN), SGA Proposal 2017 (VASCERN)
Published on: 02/03/2018
Monthly RDWG discussion on clinical cases Patient Case report summary available in the shared patient case management system Creation of education tool based on these summaries

Patient pathways
APHP
European Reference Network (ERN) on Rare Multisystemic Vascular Diseases
Patient pathways available for each RDWG, for dissemination and deriving clinical outcomes measures.

**Mobile application**

APHP

European Reference Network (ERN) on Rare Multisystemic Vascular Diseases (VASCERN), SGA Proposal 2017 (VASCERN)

Published on: 02/03/2018

Cross border path application available for patients, with HCPs Members and further networks.

**Pills of knowledge**

APHP

European Reference Network (ERN) on Rare Multisystemic Vascular Diseases (VASCERN), SGA Proposal 2017 (VASCERN)

Published on: 02/03/2018

Video/audio lessons available for clinician elearning.

**Clinical trial**

APHP

European Reference Network (ERN) on Rare Multisystemic Vascular Diseases (VASCERN), SGA Proposal 2017 (VASCERN)

Published on: 02/03/2018

discussion withing RDWG; publication of trials on the every month (report)

Every 6 months (M6-M12) website; annual report of ongoing and completed trials

Completion of 1 clinical trial

**Youtube**

APHP

European Reference Network (ERN) on Rare Multisystemic Vascular Diseases (VASCERN), SGA Proposal 2017 (VASCERN)

Published on: 02/03/2018

Conferences available on YouTube for training & education.

**Simple Clinical outcome**

APHP