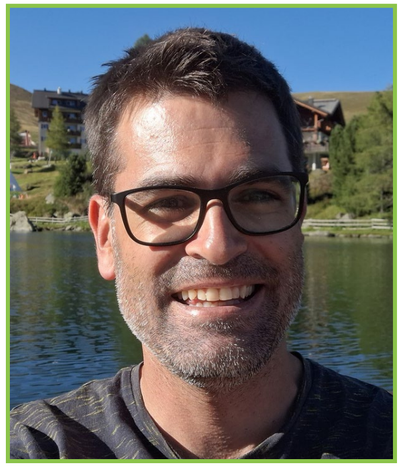
ePAGs

Empowerment und Mitgestaltung

European Patient Advocacy Group

Vorstellung

- **Michael Pölzl**
- **Rheumalis:** Kompetenz, Rat und Hilfe für an Rheuma erkrankte Kinder, Jugendliche, junge Erwachsene und Angehörige
- **Patientenvertreter:** ERN RITA, ENCA, EULAR (European Alliance of Associations for Rheumatology), PReS (Pediatric Rheumatology European Society)
- **Beruflich:** Pilot / Software Developer / MINT Lehrer / Vater

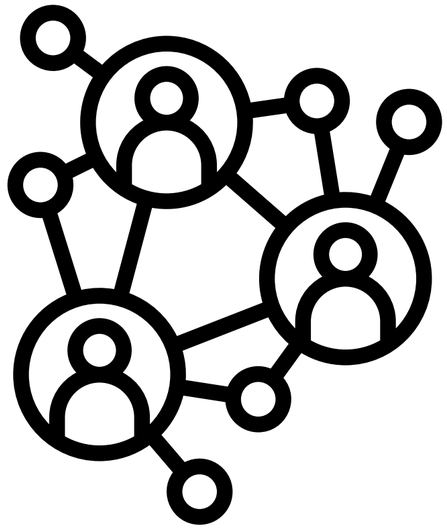


Offenlegungserklärung

- Keine Interessenkonflikte

Was sind Europäische Referenznetzwerke (ERNs)?

European Reference Networks



- Zusammenschlüsse von medizinischen Experten und Patientenvertretern für definierte Gruppen von seltenen Erkrankungen auf europäischer Ebene
- Europäische Kommission 2017
- ca 8.000 seltenen Erkrankungen in 24 Gruppen

Endo-ERN, ERKNet, ERN BOND, ERN CRANIO, ERN EpiCARE, ERN EURACAN, ERN eUROGEN, ERN EURO-NMD, ERN GENTURIS, ERN GUARD-Heart, ERN PaedCan, ERN RARE-LIVER, ERN ReCONNET, ERN RITA, ERN TRANSPLANT-CHILD, ERN-EuroBloodNet, ERN-EYE, ERN-ITHACA, ERN-LUNG, ERN-RND, ERN-Skin, ERNICA, MetabERN, VASCERN

ERN Aufgaben

- Zugang zu Diagnose, Behandlung und Fachwissen von seltenen Erkrankungen zu verbessern.
- Das Bewusstsein für seltene Krankheiten zu schärfen.
- Entwicklung von Leitlinien für die klinische Praxis
- Wissen europaweit austauschen – das Wissen reist, nicht der Patient.
- Virtuelle Beratung (Clinical Patients Management System)
- Anlegen von Registern

European Patient Advocacy Group

ePAGs

EURORDIS RARE DISEASES EUROPE

- ePAGs sind Patientenvertretungsgruppen, die jeweils einem ERN zugeordnet sind.

Ziele:

- Patientenbedürfnisse in die Arbeit der ERNs integrieren.
- Den Dialog zwischen Patientengemeinschaft und Fachleuten ermöglichen.

Wie können Patientenvertreter*innen in ERNs mitwirken?

- Brückenfunktion: Kommunikation zwischen ERN und nationaler Patientenlandschaft.
- Mitarbeit in Arbeitsgruppen (z. B. Forschung, Kommunikation, Leitlinien)
- Mitgestaltung von Patienteninformationen, Schulungen, Studien
- Rückmeldung zu Versorgungserfahrungen
- Ethik, Transparenz, Patientenrechte

Meine Erfahrungen als ePAG Member
ERN-RITA

rita

European Reference Networks

European Reference Network for Rare Immunological Disorders

ERN-RITA ePAG: RIPAG

Anforderungen Patientenvertreter:

- Erfahrung mit einer seltenen Erkrankung und Fähigkeit, die Interessen anderer Betroffener innerhalb des ERN- Wirkungsbereiches zu vertreten.
- Offizielle Unterstützung durch eine nationale Patientenorganisation
- Motivation, Kommunikationsfähigkeit, Teamgeist.
- Vertraulichkeit und professionelles Verhalten
- Englischkenntnisse und digitale Kompetenzen
- Freiwilliges Engagement / Verfügbare Zeit

Zeitlicher Aufwand - Beispiel

Task	Periodicity	Time commitment
Regular ePAG meeting (within your ERN)	Approx. every two months	1h to 1h 30 min
ERN working groups calls	Variable, depending on the groups you'd like to be involved in	Time to prepare: 30 min-1h Duration of calls: 1h to 1h 30 min
ERN annual meeting* and ePAG annual meeting	Once a year	1-2 days meeting
Document writing, review and reading	Highly variable	Typically, 2 days per month but can increase to 5 days per month

Konkrete Mitwirkung

- Online Meetings, Informationsaustausch
- Mitarbeit in Arbeitsgruppen
- Teilnahme am General Assembly
- Erstellen von Patient Journeys
- Mitwirken beim erstellen von Clinical Guidelines und Care Pathways
- Mitwirken bei Webinaren, etc.

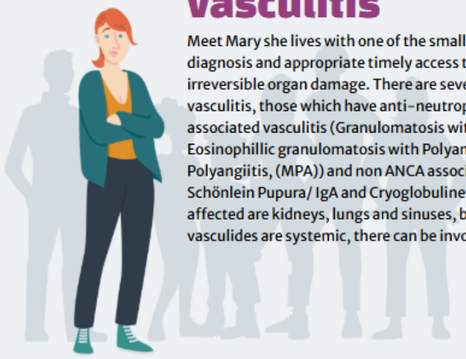
ERN RITA working groups:

Education, Clinical Guidelines, Communication, IT e-Health, Molecular Testing, Patient Organisations, Pharmacovigilance / Biological Therapies, Registries/Biobanks, Research, Stem Cell & Gene Therapy, Transition of Care.

Patient Journeys

(Patienten Reise/Wege)

The journey of a patient with small vessel vasculitis



Meet Mary she lives with one of the small vessel vasculitis conditions. Early diagnosis and appropriate timely access to treatment is essential to avoid irreversible organ damage. There are several different types of small vessel vasculitis, those which have anti-neutrophil cytoplasm antibodies – ANCA associated vasculitis (Granulomatosis with Polyangiitis, (GPA), Eosinophillic granulomatosis with Polyangiitis, (EGPA), Microscopic Polyangiitis, (MPA)) and non ANCA associated vasculitis (Henoch Schönlein Pupura/ IgA and Cryoglobulinemic Vasculitis). The main organs affected are kidneys, lungs and sinuses, but as these small vessel vasculides are systemic, there can be involvement of any organ.

Symptoms
Diagnostic
Treatment
Follow-up & aging

Symptoms
Diagnostic
Treatment
Follow up & Ageing

Symptoms description

ENT
Sinusitis
Nose bleeds
Crusting
Repeated infections
Loss of hearing
Hoarseness
Saddle Nose

ORAL HEALTH
Bleeding gums
Ulcers

CARDIOLOGY
Arrythmia
Spasm of blood vessels
Heart failure
Heart Oedema

GASTRO-ENTEROLOGY
Diarrhoea
Nausea
Bleeding
Pain
Perforated bowel (rare)

NEPHROLOGY
Haematurea
Frequent kidney infections
Urine foaming
Increased Blood pressure

NEUROLOGY
Loss or alteration of sensation Difficulty with co ordination Difficulty moving around

OPHTHALMOLOGY
Bloodshot eyes
Sensitivity to light
Inflammation of eye and eye structures
Conjunctivitis Changes to vision

RESPIRATORY
Wheeze
Difficulty breathing Cough
Coughing up blood Asthma
Repeated chest infections

DERMATOLOGY
Rash
Ulcers
Necrosis

NUTRITION
Loss of appetite
Loss of weight

INFECTIONS
Swollen glands
Feeling unwell Fever
Recurrent infections
not responding to antibiotic treatment

RHEUMATOLOGY
Joint pain
Joint swelling
Fatigue
Muscle pain

Symptoms
Diagnostic
Treatment
Follow up & Ageing

Symptoms Challenges

HEALTHCARE PROFESSIONALS AWARENESS & EDUCATION

These are rare conditions so there is poor awareness of Vasculitis within the healthcare system especially primary care.

Symptoms come and go and a lot of symptoms are dismissed – regarded as psychosomatic.

Vasculitis mimics other illnesses.

ACCESS TO EARLY, ACCURATE DIAGNOSIS

Symptoms come and go and a lot of symptoms are dismissed – regarded as psychosomatic.

Risk of permanent organ damage without timely intervention.

No definitive diagnostic tool.

COMMUNICATION AND COORDINATION

These are systemic conditions resulting in many HCPs being involved in care. These may not always be aware of each other's intervention.

HEALTH IMPACT

A wide range of very diverse physical symptoms.

Wide range of variation between individuals.

Symptoms can come and go.

Appears in childhood, adolescence as well as adulthood (peak incidence is age 50-60 years)

PSYCHOLOGICAL & SOCIAL IMPACT

Impact on both patient and carer

Anxiety, Depression Isolation and loss of self-confidence as symptoms increase and interfere with independence.

Financial challenges.

Absences from work or from school.

Costly health interventions.

QUALITY OF LIFE

Uncertainty re what is happening and what the future holds

Reduced independence

Symptoms Needs

HEALTHCARE PROFESSIONALS AWARENESS & EDUCATION

Increase awareness of small vessel vasculitis amongst the healthcare professionals.

Embedding Vasculitis in HCP and Medical student education modules.

Increase awareness and encourage attendance of existing specialist Vasculitis courses.

COMMUNICATION AND COORDINATION

Improve onward referral to other disciplines and improve communication between the same.

Collaboration between healthcare professionals involved in care to achieve earlier diagnosis and access to treatment.

Encourage holistic view of the patient, not the individual symptoms.

Symptoms can come and go.

DIGITAL HEALTH

All involved healthcare professionals to have access to health care records to ensure continuity of care.

SUPPORT & ADVOCACY

Support patient and family at this difficult time as they await a diagnosis .

Refer to local agencies who may be able to help with support and financial challenges.

Forschung

(Co-Authorship)

European Reference Networks | rita

The European Reference Network that aims at improving the care of patients with Rare Immunological Disorders

New Publication: Recommendations for Transitioning Young Patients with PID and AID to Adult Care

JAN 15, 2025

We are delighted to announce the publication of a new research paper written by members of ERN RITA, including the Transition Working Group, chaired by Siobhan Burns during the past years.

The paper addresses the critical need for optimising the transition of young patients with Primary Immunodeficiency Diseases (PID) and Autoinflammatory Disorders (AID) to adult services. Using the Delphi method, the study establishes expert consensus recommendations for effective transitional care.

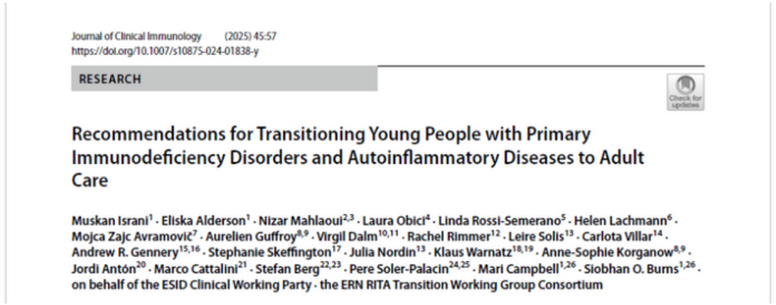
Key highlights include guidelines on programme structure, collaboration with social systems, and safe care transfer. These recommendations aim to standardise and improve health outcomes for patients across Europe.

This publication underscores ERN RITA's commitment to advancing care for rare diseases.

📖 **Read the full publication here.**

Categories

- PID (29)
- AID (24)
- AI (29)
- PR (38)



Journal of Clinical Immunology (2025) 45:57
https://doi.org/10.1007/s10875-024-01838-y

RESEARCH

Recommendations for Transitioning Young People with Primary Immunodeficiency Disorders and Autoinflammatory Diseases to Adult Care

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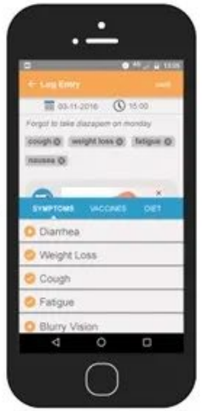
Forschung Beispiel Rheumatologie:
PARADISE Project / Mitwirkung als PRP

Novel biomarkers of immune activation

Trinity College Dublin
The University of Dublin

Traditional biomarkers and EHR data



Patient reported outcomes

Integration Engine

ADAPT

Multi-modal AI assessment

Relapse risk

Induction therapy

Maintenance therapy

Continue therapy?

Wean therapy?

Relapse

Off drugs

6 months

12 months

Co-funded by the European Union

This project was supported by national funding bodies: ANR, BMG, DS-CAT, HRB, MSE, VINNOVA, under the frame of ERA PerMed.

Persönliche Eindrücke:

- Man muss kein Experte sein (kann aber einer werden).
- Man wächst mit den Aufgaben, Möglichkeiten der Weiterbildung.
- Kein „Tokenism“, Patientenmeinungen werden ernstgenommen.
- Erstaunlich wie oft eine Patientenmeinungen neue Perspektiven aufzeigen.
- Freundliches, informelles aber sehr professionelles Arbeitsklima. Gespräche auf Augenhöhe.

Bonus Vorteile:

- Netzwerken, neue Kontakte knüpfen
- Wissen erweitern
- Persönliche Weiterentwicklung
- Erfüllung und Sinnhaftigkeit

Für Fragen stehe ich gerne zur Verfügung!


Rheumalis
Kompetenz, Rat und Hilfe für an Rheuma
erkrankte Kinder, Jugendliche,
junge Erwachsene und Angehörige
RHEUMALIS

Vielen Dank!