European Reference Networks

ERN eUROGEN 2016 -2019

developments and expectations.

Wout Feitz 2019 ERN Status: 29-11-2019.
Radboudumc, Nijmegen

EU TOGETHER WE PROTECT

Co-financed by the Connecting Europe Facility of the European Union

European Reference Network | eUROGEN Urogenital Diseases

European Reference Network
for rare or low prevalence complex diseases

Network
Urogenital Diseases
(ERN eUROGEN)

Coordinator
Radboud University
Medical Centre Nijmegen
— The Netherlands

Hospitals and Medical Professional / Experts !

COMPETITION

OR

COLLABORATION

What is the best for the future ? !

Conflict of Interest !

Innovation for Rare Diseases and Complex Conditions

The European Model:

- The creation and functioning of Centres of Expertise
- The European Reference Networks (ERNs)
- The clinical practice guidelines/clinical decision support tools
- eHealth programs, support tools, programs for new Doctors and Teams.

EU TOGETHER WE PROTECT The context

> 8,000 rare diseases affect around 30 million people in the EU.

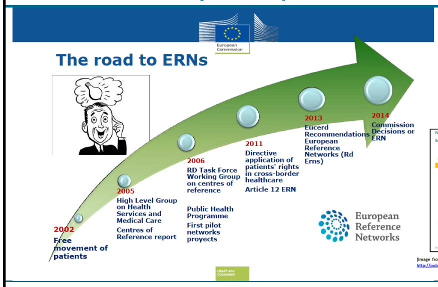
Many Patients have no access to diagnosis and high-quality treatment.

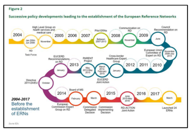
No country alone has the knowledge and capacity for all rare diseases.

European Reference Networks (ERNs) are virtual knowledge-sharing networks of expert teams of healthcare providers across Europe.

The ERN initiative receives support from several EU funding programs, including the Health Programme, the Connecting Europe Facility and Horizon 2020.

EU ERN and Policy developments





(Image from the European Court of Auditors Report on the Implementation of Directive 2011/24/EU - http://publications.europa.eu/webpub/eca/special-reports/cross-border-health-care-7-2019/en/)

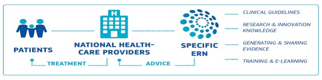
European Commission: start ERN's in 2017, Vilnius

European Reference

Networks Conference & ERN

Kick-off meeting Vilnius,

Lithuania, 9-10 March **2017**



24 ERN's started in 2017 according to operational criteria

BOND ERN	Bone Diseases	EuroBloodNet	Onco-Hematological Diseases
CRANIO	Craniofacial Anomalies and ENT disorders	**EUROGEN**	**Urogenital Diseases** ☆
Endo-ERN	Endocrine Conditions	GUARD-HEART	Diseases of the Heart
EURO-NMD	Neuromuscular Diseases	ITHACA	Congenital Malformations and Intellectual Disability
ERKNet	Kidney Diseases	MetabERN	Hereditary metabolic diseases
ERN GENTURIS	Genetic Tumour Risk Syndromes	PaedCan-ERN	Paediatric Cancer
ERN-EYE	Eye Diseases	RARE-LIVER	Hepatological Diseases
ERNICA	Inherited and Congenital Anomalies	ReCONNET	Connective Tissue and Musculoskeletal Diseases
ERN-LUNG	Respiratory Diseases	RITA	Immunodeficiency, AutoInflammatory and Auto Immune Diseases
ERN-RND	Neurological Diseases	TRANSPLANT-CHILD	Transplantation in Children
ERN-Skin	Skin Disorders	VASCERN	Multisystemic Vascular Diseases
EURACAN	Solid Adult Cancers		

ERN Governance

ERN Coordinators Group

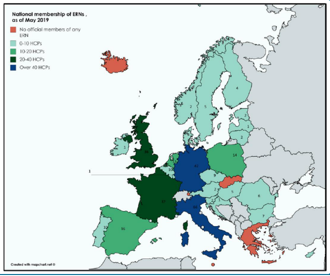
European Commission (DG Sante)

Board of Member States

Workgroups

- Knowledge Generation
- IT / CPMS
- Ethics & Data Protection
- Research
- Monitoring & Quality Improvement
- ERN Integration into National Healthcare Systems

EU Member States and ERN participation 2019



- ERN current Members include:
  - ✓ 956 highly specialised healthcare units
  - ✓ 313 hospitals
  - ✓ 25 EU Member States plus Norway
- Currently number of ERNs covered per country (including ERN members, Associated National Centers and Hubs)
  - ✓ 1264 (74.4%)

EU ERN HCP's 2019: 11-11-2019

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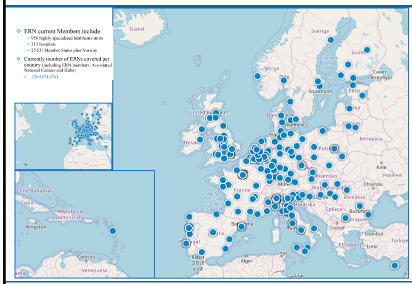
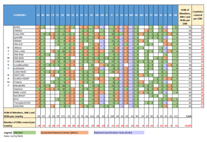


74 % Coverage of EU

24 ERN's / Countries



ERN Call for new Members 2019 > HCP Applications

2019 call for membership to the existing European Reference Networks (ERNs)

30 September - 30 November 2019

The first call for new members to join existing 24 ERNs is open until 30 November 2019. Please read carefully the content of this website before entering the application tool.

How to apply to become a member of an ERN?

The membership application process will involve several steps:

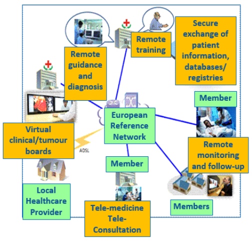
On 14-11-2019;

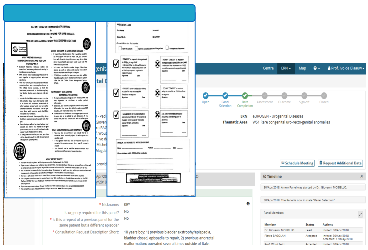
> 1000 Applications in The Online Tool

100 % EU Coverage in 2021 !

. https://ec.europa.eu/health/ern/consultations/2019_call_membership_nl

ERN IT Support & Communication: IC Form > ERN CPMS Video Consult





ERN eUROGEN - Urogenital Diseases
Thematic Area WS1 Rare congenital uro-recto-genital anomalies

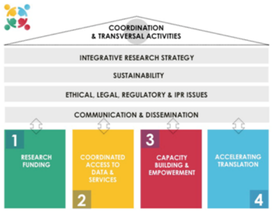
* Nickname: KEY
Is urgency required for this panel? No
* Is this a repeat of a previous panel for the same patient but a different episode? No
* Consultation Request Description Short: 10 years boy: 1) previous bladder exstrophy/epispadia, bladder closed, epispadia to repair, 2) previous anorectal malformation: operated several times outside of Italy.

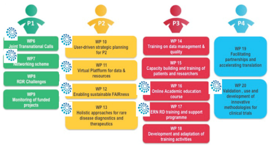
30/Apr/2018: A new Panel was started by Dr. Giovanni MOSIELLO.

30/Apr/2018: The Panel is now in state "Panel Selection"

Member	Status	Actions
Dr. Giovanni MOSIELLO	Lead	Invited: 30/Apr/2018
Pietro BAGOLAN	Accepted	Invited: 30/Apr/2018 Accepted: 17/May/2018

ERN Research Agenda and EJP - RD



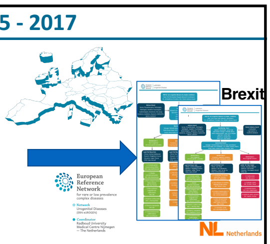


WP6 Joint Transnational Calls
WP7 Networking scheme
WP8 RDR Challenges
WP9 Monitoring of funded projects
WP 10 User-driven strategic planning for P2
WP 11 Virtual Platfform for data & resources
WP 12 Enabling sustainable FAIRness
WP 13 Holistic approaches for rare disease diagnostics and therapeutics
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 trials

Duration:	2019-2024
5-year budget:	55 mio € (EU) + 30 mio € (member states)

ERN eUROGEN Establishment 2015 - 2017

EAU Madrid / June EAU SCO 2015



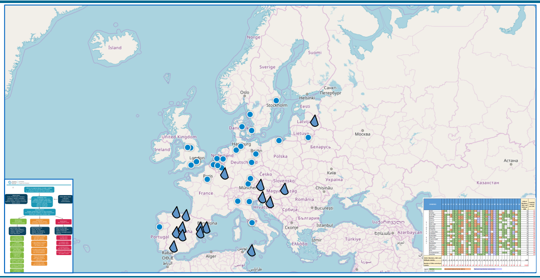
Lisbon EU 2-ERN meeting 2015

ERN eUROGEN application ***2016*** (HCP Sheffield / EAU CO)

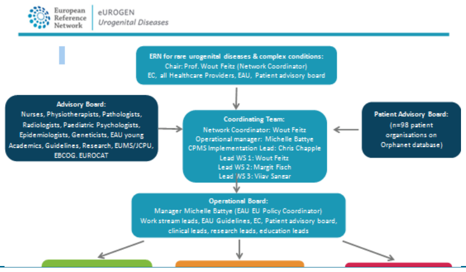
IAB process and approved by the EU Board of Member States

ERN eUROGEN 2019 SBM, Noordwijk, NL

ERN eUROGEN 2019: 45 Hospitals / 19 Member States



ERN eUROGEN Structure 2019



European Reference Network | eUROGEN Urogenital Diseases

ERN for rare urogenital diseases & complex conditions:
Chair: Prof. Wout Feitz (Network Coordinator)
EC, all Healthcare Providers, EAU, Patient advisory board

Advisory Board:
Nurses, Physiotherapists, Pathologists, Radiologists, Paediatric Psychologists, Epidemiologists, Geneticists, EAU young Academics, Guidelines, Research, EUMS/JCPU, EBCOG. EUROCAT

Coordinating Team:
Network Coordinator: Wout Feitz
Operational manager: Michelle Battye
CPMS Implementation Lead: Chris Chapple
Lead WS 1: Wout Feitz
Lead WS 2: Margit Fisch
Lead WS 3: Vijay Sangar

Patient Advisory Board:
(n=98 patient organisations on Orphanet database)

Operational Board:
Manager Michelle Battye (EAU EU Policy Coordinator)
Work stream leads, EAU Guidelines, EC, Patient advisory board, clinical leads, research leads, education leads

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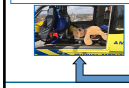
ERN eUROGEN Disease areas 2019

European collaboration / Trust
Safe exchange of Information
Life long care programs
Fact sheets for best practice
Guidelines and/or
Clinical decision support tools
Research, Training, Education



Workstream 1:
Rare Congenital Uro-recto-genital anomalies

Lead: Prof Wout Feitz
- Clinical: Andrzej Gołębiewski
- Research: Magdalena Fossum
- Education: Jorgen Thorup
- EPAG: Dalia Aminoff

1.1 Complex genital reconstructions (DSDs)
DAC: Katja Wolffenbuttel

1.2 Bladder exstrophy/epispadias
DAC: Kate Abrahamsson

1.3 Rare urological stone & kidney diseases
DAC: Kay Thomas

1.4 Non-syndromic urogenital tract malformation
DAC: Giovanni Mosiello

1.5 Posterior urethral valve
DAC: L. Henning Olsen

1.6 Posterior hypospadias
DAC: Magdalena Fossum

1.7 Urorectal/anorectal malformations
DAC: Ivo de Blaauw

Workstream 2:
Functional urogenital conditions requiring highly specialised surgery

Lead: Prof Margit Fisch
- Clinical: Dan Wood
- Research: John Heesakkers
- Education: Frank Van der Aa
- EPAG: Serena Bartezzati

2.1 Complicated & complex pelvic floor disorders
DAC: John Heesakkers

2.2 Rare diseases & conditions affecting female urethra
DAC: Richard Inman

2.3 Urethral reconstruction in rare diseases & conditions
DAC: Andrzej Gołębiewski

2.4 Rare retroperitoneal diseases & conditions
DACs: Sabine Sarnacki & Célia Cretolle

2.5 Interstitial cystitis
DAC: Mariangela Mancini

Workstream 3:
Rare urogenital tumours

Leader: Mr Vijay Sangar
- Clinical: Maarten Albersen
- Research: Asif Muneer
- Education: Mariangela Mancini
- EPAG: tbd

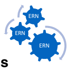
3.1 Penile cancer
DAC: Ben Ayres

3.2 Testicular cancer
DAC: Christian Meyer

3.3 Adrenal tumours
DAC: Hans Langenhuijsen

3.4 Abdomino pelvic sarcoma
DAC: Daphne Hompes

<u>Cross 24 ERN Collaboration</u>
ERNICA
ERKNet
EndoERN
ITHACA
Rare Cancers
TransplantChild



Expansion of Diseases/Conditions

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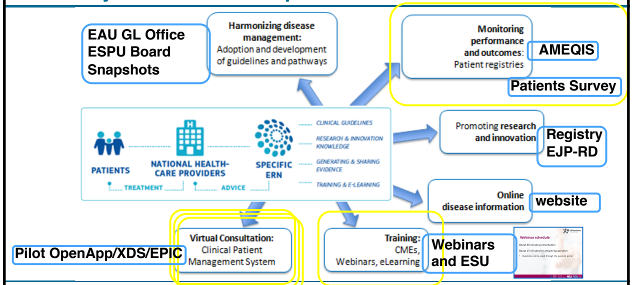
EURACAN	Solid Adult Cancers	VASCERN	Multisystemic Vascular Diseases

ERN Objectives and focus points 2019



EAU GL Office
ESPU Board
Snapshots

Harmonizing disease management: Adoption and development of guidelines and pathways

Monitoring performance and outcomes: Patient registries

AMEQIS

Patients Survey

Promoting research and innovation

Registry
EJP-RD

Online disease information

website

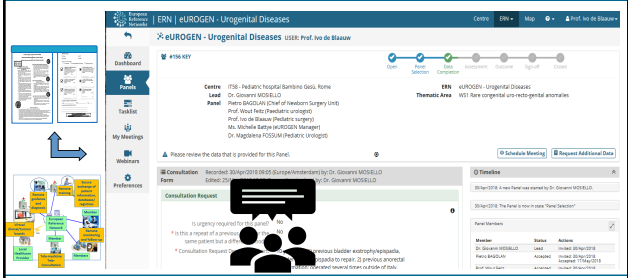
Pilot OpenApp/XDS/EPIC

Virtual Consultation: Clinical Patient Management System

Training: CMEs, Webinars, eLearning

Webinars
and ESU

ERN IT Support & Communication: IC Form > ERN CPMS Video Consult



eUROGEN - Urogenital Diseases USER: Prof. Ivo de Blaauw

#156 KEY

Centre IT58 - Pediatric hospital Bambino Gesù, Rome
Lead Dr. Giovanni MOSIELLO
Panel Pietro BAGOLAN (Chief of Newborn Surgery Unit)
Prof. Wout Feitz (Paediatric urologist)
Prof. Ivo de Blaauw (Pediatric surgery)
Ms. Michelle Battye (eUROGEN Manager)
Dr. Magdalena FOSSUM (Pediatric Urologist)

ERN eUROGEN - Urogenital Diseases
Thematic Area WS1 Rare congenital uro-recto-genital anomalies

Please review the data that is provided for this Panel.

Consultation Form Recorded: 30/Apr/2018 09:05 (Europe/Amsterdam) by: Dr. Giovanni MOSIELLO
Edited: 25/M... by: Dr. Giovanni MOSIELLO

Consultation Request

Is urgency required for this panel? No
* Is this a repeat of a previous ... for the same patient but a differ... No
* Consultation Request D... previous bladder exstrophy/epispadia, ...pispadia to repair, 2) previous anorectal ...mation: operated several times outside of Italy.

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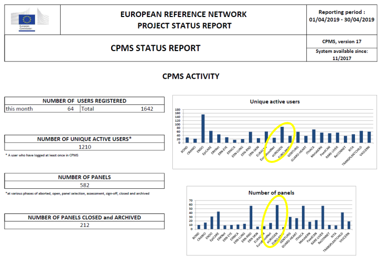
ERN AMEQIS CPMS Status Report



EUROPEAN REFERENCE NETWORK
PROJECT STATUS REPORT

Reporting period : 01/04/2019 - 30/04/2019

CPMS STATUS REPORT

CPMS, version 17

System available since: 11/2017

CPMS ACTIVITY

NUMBER OF USERS REGISTERED			
this month	64	Total	1642

NUMBER OF UNIQUE ACTIVE USERS*
1210

* A user who have logged at least once in CPMS

NUMBER OF PANELS
582

*at various phases of aborted, open, panel selection, assessment, sign-off, closed and archived

NUMBER OF PANELS CLOSED and ARCHIVED
212

eau Guidelines

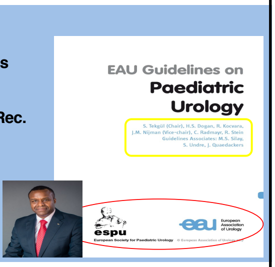
EU ERN CG workgroup and actions:

ERN Guidelines on endorsement of guidelines and / or ERN Decision Support Tools

ERN support for the developement of GL's / Rec.

EAU Guideline office and ERN collaboraton

EU Tender 2019 ! > Actions in 2020 expected



EAU Guidelines on
Paediatric Urology

S. Tekgül (Chair), H.S. Dogan, R. Kocvara,
J.M. Nijman (Vice-chair), C. Radmayr, R. Stein
Guidelines Associates: M.S. Silay,
S. Undre, J. Quaedackers

ERN training and Education 2019; new developments

HEALTH PROGRAMME 2019

Short-term exchange visit programmes for ERN healthcare professionals

1 to 4 weeks

2 mio € for 2 years
(approx. 40k €/year per ERN,
i.e. 10-15 exchange stays)

EUROPEAN JOINT PROGRAMME RARE DISEASES

PILLAR 3 – Capacity Building

Short-term exchange visit programmes for ERN researchers

Duration TBD

2.5 mio € for 5 years
(est. 6-8 exchange stays)

Webinars ERN eUROGEN and ESU Planning

UROwebinar
Learn from the experts

Title ERN eUROGEN GOTOWebinar

Presenter…… See list of SPEAKERS
Date… To be announced and coördinated with ESU

EU ERN Registry Call 2019

3rd EU Health Programme

Call for project proposals under the Annual Work Programme 2019

HP-PJ-2019

Version 1.0
14 May 2019

Open: 21 May 2019

Deadline:10 September 2019 17.00 CET

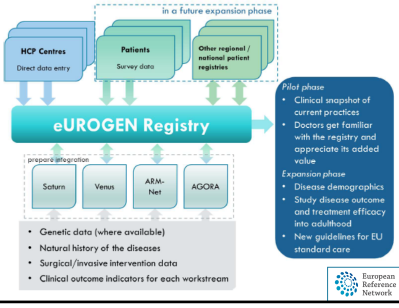
Start: March 2020

Duration:36 Month

Funding: € 200.000/ERN = max = 60 %

Mandate of all Members !

ERN eUROGEN Registry 2019 Application



- Genetic data (where available)
- Natural history of the diseases
- Surgical/invasive intervention data
- Clinical outcome indicators for each workstream

Pilot phase
- Clinical snapshot of current practices
- Doctors get familiar with the registry and appreciate its added value

Expansion phase
- Disease demographics
- Study disease outcome and treatment efficacy into adulthood
- New guidelines for EU standard care

ERN eUROGEN Registry 2019 Application

Stakeholders	Motivation	Intended effect	Means of communication
Patients & patient organisations	Better care, improve future treatments	Awareness and enrolment in the registry	Doctor, website, social media, brochures, newsletter
Health care providers / Doctors/ Specialists?	Improve training and offer better treatments	Awareness and contribution t o the registry	Website, brochures, newsletter
Public health authorities & policy makers	Efficient and affordable treatments	Awareness and support to the registry	Ad hoc meetings, brochures, conferences, website
Research community	Advance epidemiological & interventional research	Access to large database of rare urogenital diseases	Publications, conferences, newsletter
Industry	Development of new marketable products	Awareness of the impact of the registry on society	Website, social media, newsletter, brochures
ERN	Generate valuable knowledge and empower patients	Further dissemination of the registry	Reporting, meetings, events
European Union	Ensure equitable access to healthcare in a cost-effective way	Awareness of the registry and support the initiative	Reporting, events

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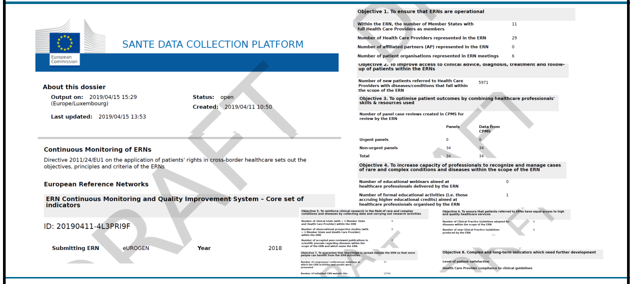
ERN AMEQIS 2019 ERN eUROGEN



SANTE DATA COLLECTION PLATFORM

About this dossier

Output on: 2019/04/15 15:29 (Europe/Luxembourg)
Status: open
Created: 2019/04/11 10:50
Last updated: 2019/04/15 13:53

Continuous Monitoring of ERNs

Directive 2011/24/EU1 on the application of patients' rights in cross-border healthcare sets out the objectives, principles and criteria of the ERNs

European Reference Networks

ERN Continuous Monitoring and Quality Improvement System – Core set of indicators

ID: 20190411-4L3PRI9F

Submitting ERN eUROGEN **Year** 2018

Objective 1. To ensure that ERNs are operational

Within the ERN, the number of Member States with full Health Care Providers as members: 11
Number of Health Care Providers represented in the ERN: 29
Number of affiliated partners (AP) represented in the ERN: 0
Number of patient organisations represented in ERN meetings: 6

Objective 2. To improve access to clinical advice, diagnosis, treatment and follow-up of patients within the ERNs

Number of new patients referred to Health Care Providers with diseases/conditions that fall within the scope of the ERN: 5971

Objective 3. To optimise patient outcomes by combining healthcare professionals' skills & resources used

Number of panel case reviews created in CPMS for review by the ERN

	Panels	Data from CPMS
Urgent panels	0	0
Non-urgent panels	34	34
Total	34	34

Objective 4. To increase capacity of professionals to recognize and manage cases of rare and complex conditions and diseases within the scope of the ERN

Number of educational webinars aimed at healthcare professionals delivered by the ERN: 0
Number of formal educational activities (i.e. those accruing higher educational credits) aimed at healthcare professionals organised by the ERN: 1

Objective 5. To reinforce clinical research in the field of rare and complex conditions and diseases by collecting data and carrying out research activities

Number of clinical trials (with > 1 Member State and Health Care Provider) within the ERN: 0
Number of observational prospective studies (with > 1 Member State and Health Care Provider) within the ERN: 0
Number of accepted peer-reviewed publications in scientific journals regarding diseases within the scope of the ERN and which name the ERN: 0

Objective 6. To ensure that patients referred to ERNs have equal access to high and quality healthcare services

Number of Clinical Practice Guidelines adopted for diseases within the scope of the ERN: 0
Number of new Clinical Practice Guidelines produced by the ERN: 0

Objective 7. To guarantee that knowledge is spread outside the ERN so that more people can benefit from the ERN activities

Number of congresses/ conferences/ meetings at which the ERN activities and results were presented: 37
Number of individual ERN website hits: 13744

Objective 8. Complex and long-term indicators which need further development

Level of patient satisfaction
Health Care Provider compliance to clinical guidelines

WS3: 23 ICD-10 codes (counting all the lower-level codes)
Total codes: 114

ERN eUROGEN: total numbers 2013- 2018 (V1; Q1-2 2019)

Total expected impact ERN eUROGEN**: 29 HCP**
on patiënt numbers: **Years: 2013 – 2019 Q 1-2**

Number of rare new patients: **43,307**

Number of rare procedures: **39,659**

Total number of Orphanet/ICD10 Codes: 114

Measure	2013	2014	2015	2016	2017	2018	2019 Q2	Total
Number of patients / year	20,542	21,574	22,344	24,335	24,796	24,290	17,591	n/a
Cumulative patient population	20,542	26,777	33,198	39,914	46,724	53,699	58,134	n/a
Number of new patients / year	5,715	6,235	6,421	6,716	6,810	6,975	4,435	43,307
Number of procedures / year	5,326	5,546	5,790	6,435	6,303	6,717	3,542	39,659

* The number of HCPs from whom we have received some response, although there are still gaps in some centres' data for particular workstreams and/or years

Rare2030

Rare2030
Foresight in Rare Disease Policy

EURORDIS
RARE DISEASES EUROPE

-EU project 2019 – 2020 (€2M)

-Foresight studies for Rare Disease Policy

-Traditional drivers and wild card factors

-Panel of Experts (ERN eUROGEN)

Rare2030

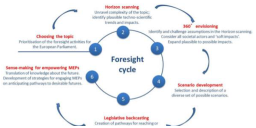
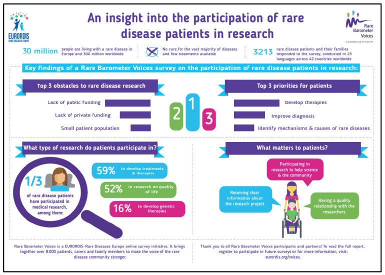


Figure 1 towards Scientific Foresight in the European Parliament

Source: JRC-IPTS

EURORDIS; How can I participate ?



EURORDIS RARE DISEASES EUROPE

Rare Barometer Voices
A EURORDIS INITIATIVE

An insight into the participation of rare disease patients in research

30 million people are living with a rare disease in Europe and 300 million worldwide

No cure for the vast majority of diseases and few treatments available

3213 rare disease patients and their families responded to the survey, conducted in 23 languages across 42 countries worldwide

Key findings of a Rare Barometer Voices survey on the participation of rare disease patients in research:

Top 3 obstacles to rare disease research

Lack of public funding
Lack of private funding
Small patient population

Top 3 priorities for patients

Develop therapies
Improve diagnosis
Identify mechanisms & causes of rare diseases

What type of research do patients participate in?

1/3 of rare disease patients have participated in medical research, among them:

59% to develop treatments & therapies
52% in research on quality of life
16% to develop genetic therapies

What matters to patients?

Participating in research to help science & the community
Receiving clear information about the research project
Having a quality relationship with the researchers

Rare Barometer Voices is a EURORDIS-Rare Diseases Europe online survey initiative. It brings together over 8,000 patients, carers and family members to make the voice of the rare disease community stronger.

Thank you to all Rare Barometer Voices participants and partners! To read the full report, register to participate in future surveys or for more information, visit: eurordis.org/voices.

(Infographic courtesy of Rare Barometer Voices)

ERN eUROGEN Supporting Partner Developments

European Reference Network | eUROGEN Urogenital Diseases

eau European Association of Urology

ESPU

ARM net

ERN's 2019 HCP Members and > 150 Affiliated Partners

› 300
HOSPITALS

› 900
HEALTHCARE UNITS

THOUSANDS OF
PATIENTS HELPED
BY 2020

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FOR RARE, LOW-PREVALENCE AND COMPLEX DISEASES

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European Reference Network

European Reference Network
for rare or low prevalence complex diseases

Network
Urogenital Diseases
(ERN eUROGEN)

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