



EUROPEAN REFERENCE NETWORKS
FOR RARE, LOW-PREVALENCE AND COMPLEX DISEASES
Share. Care. Cure.



EUROPEAN CANCER PATIENT COALITION



“Nothing about us without us!” ECPC



Joint Action on Rare cancers (JARC)



- JARC is aimed to **integrate** and **maximize** efforts of the European Commission and EU Member States to **advance quality of care** and **research** on rare cancers.
- The public health challenge posed by rare cancers combines both the **typical problems of rare diseases** and **cancers** where the need of timely diagnosis and access to quality treatment is vital.
- JARC is shaping its efforts around the



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European
Reference
Networks



JARC^{*} which has received funding from the European Union's Health Programme (2014-2020)

Coordinated by the Fondazione IRCCS
Istituto Nazionale dei Tumori di
Milano (Italy)

Fondazione IRCCS Istituto Nazionale dei Tumori
Fondazione IRCCS Istituto Nazionale dei Tumori

Dissemination by School of Medicine,
National and Kapodistrian University
of Athens, Greece

JARC

JARC Work packages

WP number	WP name
1	Coordination
2	Dissemination
3	Evaluation
4	Epidemiology
5	Assuring Quality of Care
6	Clinical practice guidelines
7	Innovation and access to innovation
8	Medical education
9	Childhood Cancers
10	Rare Cancer Policy

Weblink: <http://www.jointactionrarecancers.eu/>



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European Reference Networks



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- The European Reference Networks (ERNs) are networks connecting **health care providers** and **centres of expertise** of highly specialised healthcare
- With the purpose of **improving access** to **diagnosis**, **treatment** and the **provision** of **high-quality healthcare** for patients with conditions requiring a **particular concentration** of resources or expertise in Europe.





> **300**
HOSPITALS



















































> **900**
HEALTHCARE UNITS



THOUSANDS OF
PATIENTS HELPED
BY 2020

- The first ERNs were launched in March 2017, involving more than **900 highly specialised healthcare units** from **over 300 hospitals** in **26 Member States**.
- JARC is instrumental to help them **evolve** by optimizing the process of ERN creation through the **provision of operational support** and **professional guidance** in the areas of: quality of care, epidemiology, research and innovation, education and state of the art definition on prevention, diagnosis and treatment of rare cancers.

ERN BOND	European Reference Network on bone disorders (Factsheet   - Website)
ERN CRANIO	European Reference Network on craniofacial anomalies and ear, nose and throat (ENT) disorders (Factsheet   - Website)
Endo-ERN	European Reference Network on endocrine conditions (Factsheet   - Website)
ERN EpiCARE	European Reference Network on epilepsies (Factsheet   - Website)
ERKNet	European Reference Network on kidney diseases (Factsheet   - Website)
ERN-RND	European Reference Network on neurological diseases (Factsheet   - Website)
ERNICA	European Reference Network on inherited and congenital anomalies (Factsheet   - Website)
ERN LUNG	European Reference Network on respiratory diseases (Factsheet   - Website)
ERN Skin	European Reference Network on skin disorders (Factsheet   - Website)
ERN EURACAN	European Reference Network on adult cancers (solid tumours) (Factsheet   - Website)
ERN EuroBloodNet	European Reference Network on haematological diseases (Factsheet   - Website)
ERN eUROGEN	European Reference Network on urogenital diseases and conditions (Factsheet   - Website)
ERN EURO-NMD	European Reference Network on neuromuscular diseases (Factsheet   - Website)
ERN EYE	European Reference Network on eye diseases (Factsheet   - Website)
ERN GENTURIS	European Reference Network on genetic tumour risk syndromes (Factsheet   - Website)
ERN GUARD-HEART	European Reference Network on diseases of the heart (Factsheet   - Website)
ERN ITHACA	European Reference Network on congenital malformations and rare intellectual disability (Factsheet   - Website)
MetabERN	European Reference Network on hereditary metabolic disorders (Factsheet   - Website)
ERN PaedCan	European Reference Network on paediatric cancer (haemato-oncology) (Factsheet   - Website)
ERN RARE-LIVER	European Reference Network on hepatological diseases (Factsheet   - Website)
ERN ReCONNET	European Reference Network on connective tissue and musculoskeletal diseases (Factsheet   - Website)
ERN RITA	European Reference Network on immunodeficiency, autoinflammatory and autoimmune diseases (Factsheet   - Website)
ERN TRANSPLANT-CHILD	European Reference Network on Transplantation in Children (Factsheet   - Website)
VASCERN	European Reference Network on Rare Multisystemic Vascular Diseases (Factsheet   - Website)

- **24 ERNs are working on a range of thematic issues** including rare neoplasm of the skin and eye melanoma: which encompass **Merkel Cell Carcinoma**.
- **CROSS BORDER HEALTH CARE DIRECTIVE:** ERNs are set up under the **2011 Directive on patients' rights in cross-border healthcare:** making it easier for patients to access information on healthcare and thus increase their treatment options.

A major step towards a Europe for Health

Directive on patients' rights in cross-border healthcare



- The ERNs are currently in their deployment phase, and expected to reach full capacity over the next five years.
- **Directive 2011/24/EU** on the application of patients' rights in **cross-border healthcare** can play a role in facilitating the implementation of the future initiative.
- ERNs provide **an opportunity** for **carrying out research** and **new treatments**, **breaking the isolation** of specialists and patients, **reducing inequalities** in care, and **fostering patient involvement**.
- ERN **future challenges** might include **reimbursement**, **interoperability**, **data confidentiality**, and **legal issues**.



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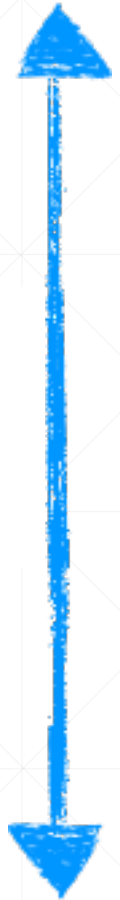


- The Joint Action on Rare Cancers and the European Reference Networks (ERNs) are **crucial game changers** for rare cancer patients in Europe bringing together scarce knowledge and fragmented resources to maximise synergies and results.
- The European Cancer Patient Coalition (ECPC) is one of the partner patient organizations representing the **needs, rights** and **hopes** of rare cancer patients within: **JARC** and **ERN-EURCAN**.

ERN Involvement



Patient



✓ General ERN level – coordinated by EURORDIS

- European Patient Advocacy Group (ePAG) per each ERN disease grouping: allowing patients to participate in the decision-making process and ensuring a democratic representation of patients.
- **ECPC is an elected ePAG representatives on rare cancer across the various ERN-EURACAN domains.**

✓ ERN level (EURACAN) – the role of ePAGs

- 1 invited ePAG representative to participate in Steering Committee meeting as observer.
- **ECPC is the co-Lead the Transversal Task Force (TTF) on Communications and Dissemination participating in Steering Committee meeting.**
- Elected ePAG representatives per domain.



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JARC

Working Group on Rare Cancers (WGRC)



ECPC counts on the **expertise** and **collaboration of rare cancer patient organisations** all over Europe to continuously represent the rare cancer patient community.

- ECPC **has established** the WGRC **operating in parallel** with the JARC, in order to guarantee that a large number of rare cancer patient organisations, representing different European countries and rare cancer types, are contributing to the activities of the Joint Action: **currently the WGRC hosts 65 member organisations.**
- ECPC is **building on experience** gained during its 3 years collaboration in **RARECAREnet** project.



Members

Organisation	Acronym	Country
Aivolisäke-potilasyhdistys Sella ry.		Finland
Asociación de Afectados por Tumores Cerebrales en España	ASATE	Spain
Asociación De Afectados Por Cáncer De Ovario	ASACO	Spain
Asociación de Pacientes con Tumores Raros de España	APTURE	Spain
Asociación Española de Afectados por Sarcomas	AEAS	Spain
Asociación Española de Cáncer de Tiroides	AECAT	Spain
Asociația Pacienților cu Neoplazii Mieloproliferative	MIELOPRO	Romania
Ass. Italiana Largectomees	ailar/FIALPO	Italy
Associação Portuguesa Contra a Leucemia	APCL	Portugal
Association contre les maladies rares du péritoine	AMARAPE	France
Association des Patients porteurs de Tumeurs Endocrines Diverses	APTED	France
Association of Cancer-Volunteers-Friends-Doctors "Smile of the Child" Athens	KEFI	Greece
Associazione Italiana GIST Onlus	A.I.G.	Italy
Bulgarian Association for Patients' Defense	BAPD	Bulgaria
Bulgarsko Sdruzhenie Limfom	BSL	Bulgaria
CARITA 'To live with Multiple Myeloma'		Poland
Slaime an Chlaire		UK
Contactgroep GIST		Netherlands
Društvo onkoloških bolnikov Slovenije		Slovenia
Sarcoma Ireland		Ireland
El Grupo Español de Pacientes con Cáncer	GEPAC	Spain
European Cancer Patient Coalition	ECPC	Belgium
European Cancer Patient Coalition, Diagnosa Leukemie	ECPC, Diagnosa CML	Czech Republic
European Lung Foundation		UK
Everything for Her		Croatia
Fondazione Istituto Nazionale dei Tumori di Milano	IRCCS	Italy
Glioblastoma Multiforme – Cancro al Cervello	Gbm Group	Italy
Group of Volunteers Against Cancer		Greece
Gynsam Sweden	GYNSAM	Sweden
Hoved hal cancer		Denmark
International Neuroendocrine Cancer Alliance		International
Italian Federation of H&N Advocacy Groups	FIALPO	Italy
Lithuanian Cancer Patient Coalition	POLA	Lithuania
Magnolia - Stowarzyszenie kobiet z problemami onkologicznymi		Poland
Mouth, Head and Neck Cancer		Ireland
Myeloma Euronet Romania	MER	Romania
Myeloma Patients Aid Association		Poland
NET Patient Foundation		UK
NET-groep		Netherlands
Netzwerk Neuroendokrine Tumoren e.V.	NeT	Germany
Patientforening for Lymfekræft og Leukæmi	LYLE	Denmark
Patients Aid Association for Sarcoma		Poland
Patients with GIST Aid Association		Poland
Pembe Hanım		Turkey

Plattform Hodenkrebs Austria		Austria
Polish Cancer Patient Coalition		Poland
imind Ajutor, Viata Este Luminoasă	PAVEL	Romania
Pseudomyxoma Survivor		UK
Pseudomyxoma Survivor (Appendix Cancer)	PMP	UK
Romanian GIST Network - Extended to sarcoma patients		Romania
Romanian Leukemia Association	ARIL	Romania
Community Health		Romania
Sarcoma Ireland		Ireland
Stichting Hematon		Netherlands
Stichting OLIJF		Netherlands
The Brain Tumour Charity		UK
The Cholangiocarcinoma Charity	AMMF	UK
The Cyprus Association of Cancer Patients and Friends	PASYKAF	Cyprus
The Nationwide Association of CML Patients Aid		Poland
Thyroid Bulgaria	VIOM	Bulgaria
Vereniging Cerebraal		Netherlands
Vivere la Speranza - Amici di Emanuele Cicio ONLUS	A.I.NET.	Italy
VulvaKarzinom Selbsthilfegruppe e.V.		Germany



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RC Online Library



- ECPC has collected information materials on most of [the rare cancers identified by the RARECARENet](#) experts. The aim was to create an [online library](#) on the [RARECARENet](#) website where patients can find information on the diagnosis, treatment and follow-up of any type of rare cancer.

RARECARENet (Information Network on Rare Cancers) is funded by the European Commission's **EU Health Programme 2008-2013** and coordinated by the Fondazione IRCCS, Istituto Nazionale dei Tumori, Italy. **RARECARENet** involves **partners** all around Europe.

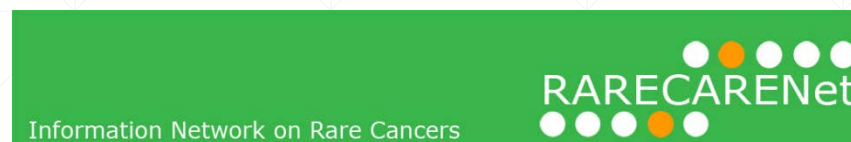
The research project was carried out between May 2011 to May 2015, closed project.



List of Rare Cancers

Rare cancers are not so rare: The rare cancer burden in Europe

Gemma Gatta ^{a,*}, Jan Maarten van der Zwan ^b, Paolo G. Casali ^c, Sabine Siesling ^b, Angelo Paolo Dei Tos ^d, Ian Kunkler ^e, Renée Otter ^b, Lisa Licitra ^f, Sandra Mallone ^g, Andrea Tavilla ^g, Annalisa Trama ^a, Riccardo Capocaccia ^g, The RARECARE working group



R=rare	Tier	Tumour	Crude incidence rate per 100,000	95% confidence interval		Number of cases collected in the RARECAREnet database from	ICD-O-3 Topography code	ICD-O-3 Morphology code
	3	Small cell endocrine carcinoma					C15-26, C48	8041-8045, 8002
R	2	Mixed endocrine-exocrine carcinoma of pancreas and digestive tract	0.01	0.01	0.01	147	C15-26	8154
R	2	Endocrine carcinoma of thyroid gland	0.24	0.23	0.25	3,796	C73.9	8002, 8013, 8041, 8240, 8246, 8249, 8345-8347, 8
	3	Medullary carcinoma					C73.9	8510, 8345, 8246
	3	Mixed medullary-follicular carcinoma					C73.9	8346
	3	Well differentiated endocrine tumours					C73.9	8240, 8249
	3	Poorly differentiated endocrine carcinoma					C73.9	8013, 8041, 8002
R	2	Neuroendocrine carcinoma of skin	0.19	0.19	0.20	3,024	C44.0-C44.9, CC51.0-C51.9, C60.0, C60.9 C63.2	8013, 8041, 8002, 8240, 8246 and 8247 in all sites
	3	Merkel cell carcinoma					C44.0-C44.9, CC51.0-C51.9, C60.0, C60.9 C63.2	8013, 8041, 8240, 8246 and 8247 in all sites
R	2	Typical and atypical carcinoid of the lung	0.39	0.38	0.40	6,160	C34	8240, 8241, 8242, 8243, 8244, 8245, 8249
R	2	Neuroendocrine carcinoma of other sites	0.90	0.89	0.92	14,120	All cancer sites except C15-C26, C34, C44, C48, C51, C60, C63.2, C73	8002, 8013, 8041-8045, 8150-8153, 8155-8157, 82
	3	Well differentiated endocrine tumours, carcinoid					All cancer sites except C15-C26, C34, C44, C48, C51, C60, C63.2, C73	8150-8153, 8155-8157, 8240-8245, 8248,
	3	Well differentiated endocrine tumours, atypical carcinoid					All cancer sites except C15-C26, C34, C44, C48, C51,	8249



Chordoma as a model within the ERNs

- **Chordomas are very rare**, occurring in **approximately 1 per million** individuals each year.
- ERNs could **contribute to the evaluation of new medicines for Chordoma**, as it is an ultra-rare disease.
- ERNs network could **facilitate access to innovative Chordoma treatments**.
- ERNs could be **involved in the adaptive licensing or conditional approval of Chordoma treatments based on new data**, thus, **reducing uncertainties** for the EMA, companies, patients and clinicians **about introducing new treatment to the market**.
- ERNs could **play an important role in generating data** and, hopefully, **following the new HTA Initiative could speed up the reimbursement decisions on orphan medicines**.

Chordoma as a model

Why?

1. Incredible variety and differences of HTA procedures across the EU

It is very hard to compare different systems to see which is more valuable

Duplications of work

Inequalities in access to innovative treatments

2. Lack of transparency of HTA

Almost impossible for patients to have access to information

Hard to check legality of the procedures

3. Unacceptable delays in the HTA process across Member States

4. Patients' involvement in HTA process is very limited

Impact is multiplied for the Chordoma, as it is a very rare cancer

There is a great need for **HTA**

harmonization across most European health systems

where establishing a

separate European HTA body to interact.

with the EMA

should be considered.

- **Upcoming ECPC-ESMO Initiative with EMA using Chordoma as Model:** push to achieve the final goal of the directive

ECPC invites patient experts, Members and non-member organisations with the shared interest in rare cancers for their **active participation** in our Working group on Rare Cancers (WGRC).

Thank you!

“Nothing about us without us!” ECPC

