RARE CANCERS EUROPE

Joining forces for action

RCE-ESMO-ESO Training Course for Rare Cancer Patient Advocates

Patient Involvement in Clinical Research

Patient involvement in clinical registries

LOGISTICS FOR THIS SESSION

- All questions should be addressed via the chat box only. We will also take questions and comments from the floor during the discussion time. Please use the ”raised hand system” in Zoom.

- We kindly request to put your camera on during the discussion time.

- For any technical issues, please send us an email at: rarecancerseurope@esmo.org and we will get back to you.

- The above information will also be posted in the chat box, so everyone is aware

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Annalisa Trama
Fondazione IRCCS Istituto Nazionale dei Tumori di Milano

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I have no conflict of interest to declare

What are clinical registries and why is patient involvement crucial?

Clinical registries are databases that systematically collect health-related information on individuals who are:

- treated with a particular surgical procedure, device or drug; or
- **diagnosed with a particular illness**, e.g. head and neck cancers; or
- managed via a specific healthcare resource (e.g. Intensive care unit).

	Registry-based study	Patient registry
1. Definition	Investigation of a research question or hypothesis using data from an existing patient registry or from a registry newly set-up for the study.	Data collection system on a group of people defined by a particular disease or condition, established for a specific purpose and used to conduct a registry-based study.
2. Timelines	Timelines driven by the collection/extraction and analysis of the data relevant for the specific study objective(s).	Generally planned to be long-term; timelines driven by schedules for routine data collection and any anticipated data analyses which prompted the registry.
4. Data collection	Restricted to what is needed by the research question including data on potential confounders and effect modifiers; additional data collection may also be required; if such additional data includes subject monitoring outside SmPC and normal clinical practice, the legislation for clinical trials apply; study may involve primary data collection or secondary use of data.	Wide range of data may be collected depending on the purpose of the registry; there should be an agreed core set of data elements to be collected with harmonised definitions, common coding system and common data entry procedures.

European Reference Network

for rare or low prevalence complex diseases

G7 Head & neck

Registry objectives

- to help describe the natural history of rare adult solid cancers;

  - how the cancer develop, progress, possible association with other diseases?

- to evaluate factors that influence prognosis and treatment response;

  -site, histology, grading impact on mortality, progression, quality of life?

- to assess treatments effectiveness (systemic, radiotherapy, surgery, target therapy, immunotherapy and possible combinations);

- to measure indicators of quality of care;

  - is treatment starting on time? Is stage properly assessed and defined? Is surgery complete?

Development and external validation of two nomograms to predict overall survival and occurrence of distant metastases in adults after surgical resection of localised soft-tissue sarcomas of the extremities: a retrospective analysis

Dario Callegaro, Rosalba Miceli, Sylvie Bonvalot, Peter Ferguson, Dirk C Strauss, Antonin Levy, Anthony Griffin, Andrew J Hayes, Silvia Stacchiotti, Cecile Le Pechoux, Myles J Smith, Marco Fiore, Angelo P Dei Tos, Henry G Smith, Luigi Mariani, Jay S Wunder, Raphael E Pollock, Paolo G Casali, Alessandro Gronchi

New research strategies in retroperitoneal sarcoma. The case of TARPSWG, STRASS and RESAR: making progress through collaboration

Winan J. van Houdt[a], Chandrajit P. Raut[b], Sylvie Bonvalot[c], Carol J. Swallow[d], Rick Haas[e], and Alessandro Gronchi[f]

Purpose of review
Retroperitoneal sarcoma (RPS) is a rare disease, and until recently, its natural history and outcome were poorly understood. Recently, collaborations between individual centers have led to an unprecedented collection of retrospective and prospective data and successful recruitment to the first randomized trial as described here.

Recent findings
A debate about the beneficial role of extended surgery in RPS triggered an initial collaboration between Europe and North America, the TransAtlantic RetroPeritoneal Sarcoma Working Group (TARPSWG). This collaboration has been instrumental in harmonizing the surgical approach among expert centers, characterizing the pattern of postresection failure of the different histological subtypes, identifying new ways to stage RPS and testing the role of preoperative radiotherapy in a randomized fashion (STRASS-1 study). The collaboration has now expanded to include centers from Asia, Australia and South America. A prospective registry has been started and a new randomized trial, STRASS-2, is in preparation to analyze the role of neoadjuvant chemotherapy for high-grade liposarcoma and leiomyosarcoma of the retroperitoneum.

Summary
Collaboration is critical to study a rare disease like RPS. Both retrospective and prospective data are useful to improve knowledge, generate hypotheses and build evidence to test, whenever possible, in clinical trials.

Where can we get the answers to the following questions?

How many cases of head and neck cancers are newly diagnosis in my country? in other countries?

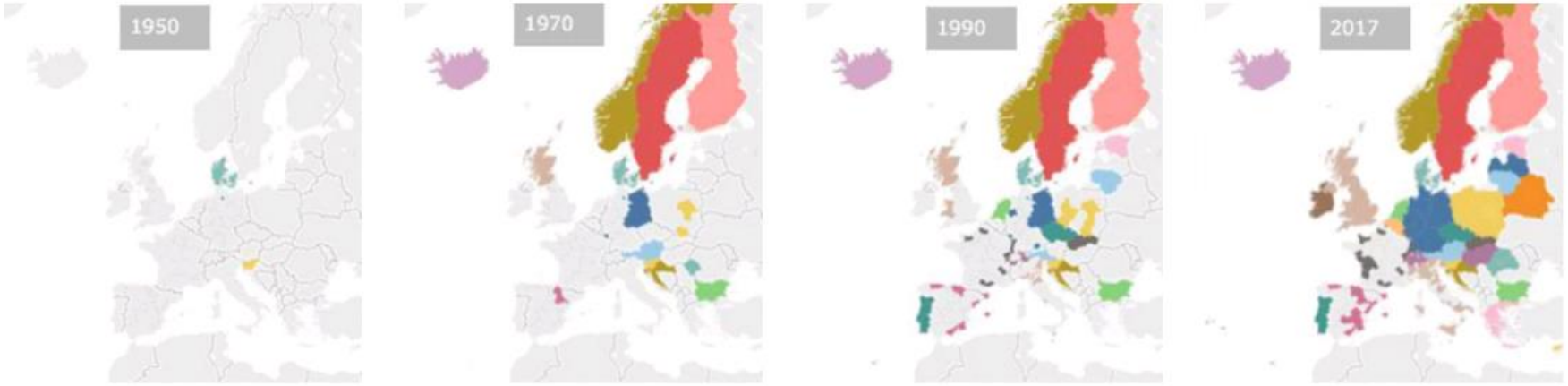
Is the number of newly diagnoses head and neck cancers increasing or decreasing?

Which is the prognosis? is it ameliorating?....

Which is the impact of cancer plan? of preventive strategies? etc.

Population-based cancer registries

Timeline of cancer registration in Europe



European Network of Cancer Registries

https://www.encr.eu/

EUROPEAN JOURNAL OF CANCER 47 (2011) 2493–2511

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journal homepage: www.ejconline.com

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*

[a] Department of Preventive and Predictive Medicine, Fondazione IRCSS, Istituto Nazionale dei Tumori, Via Venezian 1, 20133 Milan, Italy
[b] North East Netherlands Cancer Registry, Comprehensive Cancer Centre North East, P.O. Box 330, 9700 AH Groningen, The Netherlands
[c] Department of Cancer Medicine, Fondazione IRCSS, Istituto Nazionale dei Tumori, Via Venezian 1, 20133 Milan, Italy
[d] Department of Pathology, General Hospital of Treviso, Via Borgo Cavalli 42, 31100 Treviso, Italy
[e] Department of Clinical Oncology, Western General Hospital, Crewe Road South, Edinburgh EH4 2XU, UK
[f] Head and Neck Cancer Medical Oncology Unit, Fondazione IRCSS, Istituto Nazionale dei Tumori, Via Venezian 1, 20133 Milan, Italy
[g] Department of Cancer Epidemiology, Istituto Superiore di Sanità, Viale Regina Elena 299, Rome, Italy

Burden and centralised treatment in Europe of rare tumours: results of RARECAREnet—a population-based study

*Gemma Gatta, Riccardo Capocaccia, Laura Botta, Sandra Mallone, Roberta De Angelis, Eva Ardanaz, Harry Comber, Nadya Dimitrova, Maarit K Leinonen, Sabine Siesling, Jan M van der Zwan, Liesbet Van Eycken, Otto Visser, Maja P Žakelj, Lesley A Anderson, Francesca Bella, Kaire Innos, Renée Otter, Charles A Stiller, Annalisa Trama, for the RARECAREnet working group**

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See Comment page 983

Summary

Background Rare cancers pose challenges for diagnosis, treatments, and clinical decision making. Information about rare cancers is scant. The RARECARE project defined rare cancers as those with an annual incidence of less than six per 100 000 people in European Union (EU). We updated the estimates of the burden of rare cancers in Europe, their time trends in incidence and survival, and provide information about centralisation of treatments in seven European countries.

Methods We analysed data from 94 cancer registries for more than 2 million rare cancer diagnoses, to estimate European incidence and survival in 2000–07 and the corresponding time trends during 1995–2007. Incidence was calculated as the number of new cases divided by the corresponding total person-years in the population. 5-year relative survival was calculated by the Ederer-2 method. Seven registries (Belgium, Bulgaria, Finland, Ireland, the Netherlands, Slovenia, and the Navarra region in Spain) provided additional data for hospitals treating about

24% of all new cancers in Europe

600,000 new rare cancer patients

Relative survival of most rare cancers < 50%

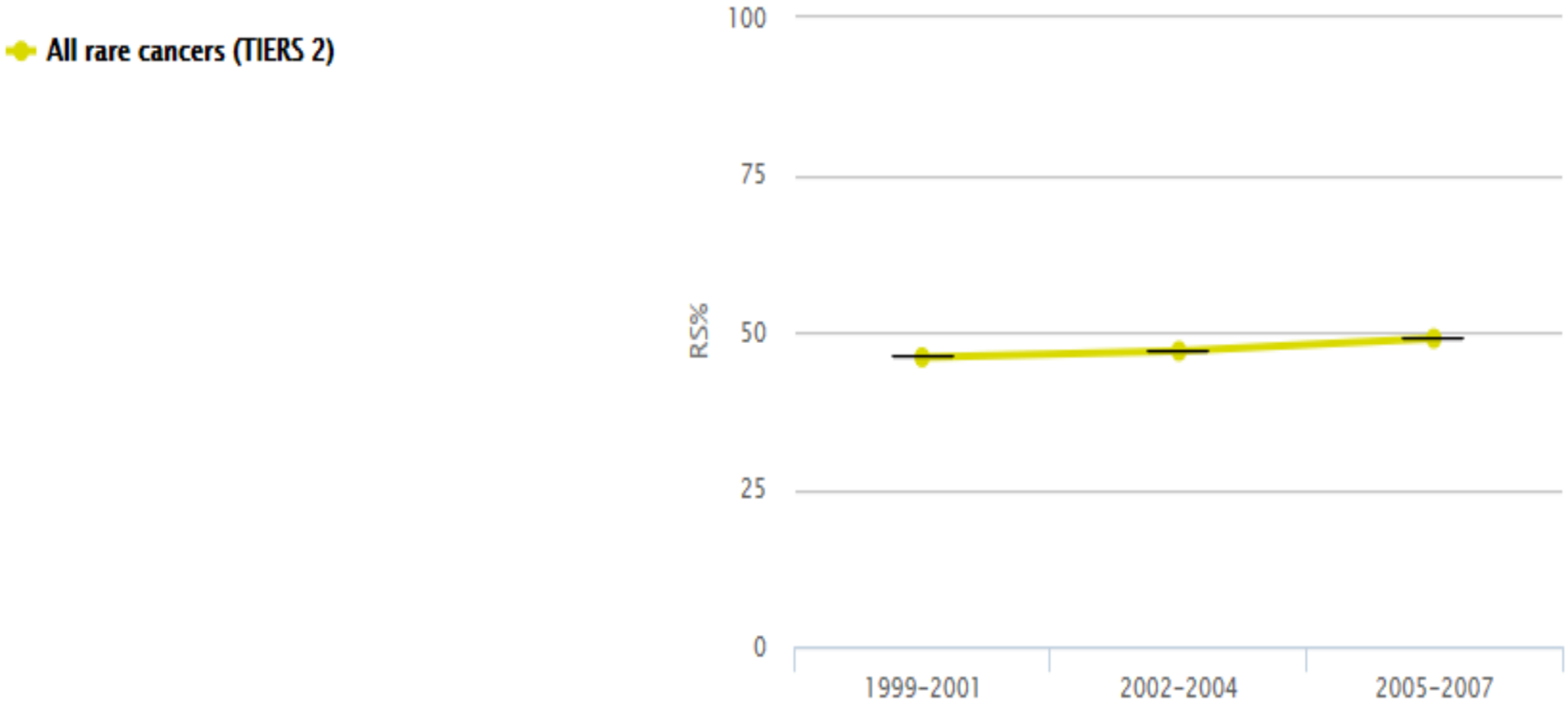
Number of cases (No.) and 5-year Relative Survival (RS%) with (95% CI) by selected rare cancers.

Family	Rare cancer entity	No.	5-year RS %
head and neck rare cancers	Epithelial tumours of nasal cavity and sinuses	6867	47 (46–49)
	Epithelial tumours of nasopharynx	7276	49 (48–50)
	Epithelial tumours of major salivary glands	14,703	61 (60–62)
	Salivary gland type tumours of head and neck	6683	67 (66–69)
	Squamous cell carcinomaof hypopharynx	19,878	25 (24–26)
	Squamous cell carcinoma of larynx	71,928	61 (60–61)
	Epithelial tumours of oropharynx	50,843	41 (40–41)
	Squamous cell carcinoma of oral cavity	54,229	48 (48–49)
rare thoracic cancers	Epithelial tumours of thymus	2729	64 (62–67)
	Mesothelioma of pleura and pericardium	27,893	5 (4–5)
rare epithelial and not epithelial ovarian cancers	Mucinous adenocarcinoma of ovary	12,010	60 (59–61)
	Clear cell adenocarcinoma of ovary	4761	56 (54–57)
	Mullerian mixed tumour of ovary	2242	21 (20–24)
	Non epithelial tumours of ovary	3970	82 (81–84)
rare male genital cancers	Testicular and paratesticular cancers	51,011	95 (95–95)
	Epithelial tumours of penis	10,210	68 (66–69)
soft tissue sarcomas	Soft tissue sarcoma (STS)	72,696	57 (56–57)
	STS of superficial trunk	7723	48 (47–50)
	STS of mediastinum	457	23 (19–28)
	STS of retroperitoneum and peritoneum	4854	39 (37–41)
gastroenteropancreatic (GEP) neuroendocrine tumours	GEP - well differentiated not functing endocrine carcinoma	15,656	72 (71–73)
	GEP - poorly differentiated endocrine carcinoma	10,456	35 (34–36)
	GEP - mixed endocrine-exocrine carcinoma	141	26 (18–37)

Gatta G, Trama A, Capocaccia R. Eur J Surg Oncol 2019;45(1):3-11.

5-year Relative Survival (RS) overtime in Europe

Period approach. Periods of diagnosis: 1999-2001, 2002-2004, 2005-2007. 94 CRs.
Error bars are 95% confidencen intervals.



Clinical registry set-up vs. patient's involvement

- Identification of objectives
- Identification of core data set
- Definition of the IT infrastructure
- Definition on the quality assurance procedures
- Definition of the protocols/guidelines for data collection
- Training
- Piloting
- Definition of legal agreements across institutions
- Ethical/institutionl board review
- Definition of the registry governance

Clinical registry maintenance vs. patient's involvement

Findible

Accessible

Interoperable

Reusable

Continuous data collection

Data quality

Data updates

Motivation

Funding

Vision

Clinical registry challenges vs. patient's involvement

- Data collection (hospital-based)
- Data quality (accuracy; timeliness)
- Data sharing
- Privacy (consensus)
- Sustainability

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Thank you for your attention

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Clinical registry: patients' perspective

Ariane Weinman

EURORDIS – Rare Diseases Europe

I have no conflict of interest to declare

Why registries matter to patients?

- Constitute key instruments for **increasing knowledge** on the diseases by pooling adequate thresholds of data for various types of research (fundamental, clinical, epimediology).

- Clinical registries are necessary to the assessment of the feasibility, planning and design of **clinical trials** and facilitate the enrolment of patients **for real-life post-marketing observational studies.**

Involvement of patient / patients' representatives

- Importance of patient representation in the **governance** – ensure a fair balance of stakeholders
- Contribute to identify patients' **unmet needs, priorities and gaps**
- Contribute to define the **informed consent**, ensuring it is understood by the patient
- Safeguard good process for patients' **data sharing**

Patient /patient representatives: additional messages

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- Ensure to map out/ use existing data/registry (especially in *rare* cancers/diseases
- Tying registry records to biobanks and having biobank buy-in early on in the process
- Retrospective data vs prospective data:
  - Need to integrate retrospective data but issue of quality of data
- Bring the translational research closer to the patients (from bench to bedside) e.g. through a tighter integration of clinical research into the routine clinical practice

DISCUSSION

Use Zoom « Chat » and/ or click on the hand “Raise Hand” in the participants list

- What is your experience with clinical registry?
- What are the major challenges you faced

  (patient involvement, collection of data, data privacy, sustainability of the registry)

- What are the lessons learned about patient involvement?
- What are the key factors for the success?