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
Patient Involvement in Clinical Research
Patient involvement in clinical registries

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I have no conflict of interest to declare
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).
<table>
<thead>
<tr>
<th></th>
<th><strong>Registry-based study</strong></th>
<th><strong>Patient registry</strong></th>
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</thead>
<tbody>
<tr>
<td><strong>1. Definition</strong></td>
<td>Investigation of a research question or hypothesis using data from an existing patient registry or from a registry newly set-up for the study.</td>
<td>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.</td>
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<tr>
<td><strong>2. Timelines</strong></td>
<td>Timelines driven by the collection/extraction and analysis of the data relevant for the specific study objective(s).</td>
<td>Generally planned to be long-term; timelines driven by schedules for routine data collection and any anticipated data analyses which prompted the registry.</td>
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<td><strong>4. Data collection</strong></td>
<td>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.</td>
<td>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.</td>
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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, Angela 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, Chandrjit P. Raut, Sylvie Bonvalot, Carol J. Swallow, Rick Haas, and Alessandro Gronchi

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 diagnosed 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

https://www.enqr.eu/
Rare cancers are not so rare: The rare cancer burden in Europe

Gemma Gatta a,*, Jan Maarten van der Zwan b, Paolo G. Casali e, Sabine Siesling b, Angelo Paolo Dei Tos d, Ian Kunkler e, Renée Otter b, Lisa Licitra f, Sandra Mallone 9, Andrea Tavilla g, Annalisa Trama a, Riccardo Capocaccia a, The RARECARE working group

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g Department of Cancer Epidemiology, Istituto Superiore di Sanità, Viale Regina Elena 299, Rome, Italy

Summary
Background Rare cancers pose challenges for diagnosis, treatment, 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, Slovakia, 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%

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

5-year Relative Survival (RS) overtime in Europe


- All rare cancers (TIERS 2)
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/institutional 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
Thank you for your attention
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

- 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?