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FONDAZIONE IRCCS
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DEI TUMORI

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

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Eur J Cancer 2011;47:2493

Table 3 - Data quality indicators and other characteristics of malignant cancers diagnosed in European cancer registries 1995-2002 and included in the analyses.

Country	Registry	Number of malignant cancers	Data quality indicators				
			Death certificate only (%)	Autopsy (%)	Microscopic verification (%)	Cases 1995-1998 detected before 5 years (%)	Morphology code NOS (%)
Austria	Austria	306,630	8.9	0.0	85.2	5.9	10.1
Belgium	Flanders	146,715	0.0	0.2	89.8	0.0	7.3
France	Bas Rhin	13,113	0.0	0.0	95.8	3.3	3.9
	Calvados	5895	0.0	0.0	98.1	6.1	2.5
	Calvados digestive	2871	0.0	0.0	87.0	4.4	10.5
	Côte d'Or digestive	4376	0.0	0.0	82.8	0.5	17.5
	Côte d'Or hematol.	1884	0.0	0.0	100.0	7.2	0.0
	Dordogne	5762	0.0	0.0	95.8	2.1	3.3
	Haut Rhin	9073	0.0	0.0	96.4	5.8	2.9
	Hérault	10,505	0.0	0.0	8.0	4.4	1.5
	Isère	12,526	0.0	0.0	94.1	4.6	4.1
	Loire Atlantique	3766	0.0	0.0	100.0	6.8	0.0
	Manche	6267	0.0	0.0	96.5	2.7	3.4
	Marne and Ardennes	168	0.0	0.0	100.0	3.6	0.0
	Somme	6481	0.0	0.0	94.2	6.6	5.5
	Tarn	4905	0.0	0.0	93.8	2.0	5.9
Germany	Saarland	54,132	3.9	0.0	91.8	5.8	8.0
Ireland	Ireland	8854	0.1	1.4	94.6	0.0	3.5
Ireland	Ireland	156,526	2.0	0.3	86.7	0.0	11.0
Italy	Alto Adige	18,476	0.7	0.0	89.5	0.0	9.2
	Brescia	11,770	1.3	0.4	87.0	0.0	12.5
	Ferrara	23,760	1.1	0.0	88.1	0.4	9.7
	Frosinone	66,087	0.1	0.9	80.4	0.4	12.7
	Friuli V.G.	78,862	0.6	1.9	91.0	0.3	9.8
	Gorizia	44,207	1.8	0.0	81.4	0.0	36.8
	Monza	10,766	1.3	0.0	87.4	0.2	11.1
	Modena	34,947	0.5	0.0	88.6	0.4	11.8
	Napoli	8165	3.9	0.0	73.0	1.9	17.6
	Palermo	581	2.3	0.0	92.6	0.0	7.2
	Parma	23,826	1.0	0.0	86.0	0.3	13.1
	Reggio Emilia	10,887	1.9	0.0	80.9	0.1	24.6
	Reggio Emilia	22,512	0.2	0.0	88.1	0.0	13.8
	Romagna	60,847	0.0	0.0	87.9	0.1	12.3
	Salsomaggiore	26,917	2.5	0.0	77.5	4.0	23.7
	Siena	19,084	2.9	0.2	84.4	0.0	36.4
	Treviso	12,788	0.0	0.0	85.0	0.0	27.8
	Udine	45,221	0.7	0.0	84.0	0.1	12.6
	Varese	24,728	1.1	0.0	89.0	11.5	10.8
	Venezia	84,518	1.5	0.3	87.5	0.8	13.7

1. Pediatric cancers
2. Haematologic rare neoplasms
3. Sarcomas
4. Rare thoracic cancers
5. Neuroendocrine tumours
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9. Rare urological and male genital tumours
10. Endocrine gland tumours
11. Digestive rare cancers
12. Rare skin cancers & non-cutaneous melanoma



Objectives

With regard to rare cancers in the EU, to improve:

- 1. Epidemiological surveillance**
- 2. Quality of care through ERNs**
- 3. Clinical practice guidelines**
- 4. Innovation**
- 5. Medical and Patient education**
- 6. Health policy measures**
- 7. Patient empowerment**

Work packages

WP

1 Coordination

2 Dissemination

3 Evaluation

4 Epidemiology

5 Assuring Quality

6 Clinical practice guidelines

7 Innovation and access to innovation

8 Patient education

Good Cancers

Rare Cancer Policy

ECPC, EURORDIS, CCI E

A, GR

CSF, FI

INT, IT

OECI

DKG, DE

WIV-ISP, BE

UP, HU

SIOPE

ICO, ES



- **18 MSs**
- **34 ass. partners**



Collaborating partners

European Cancer Patients Coalition (ECPC)

University College of London-Institute of Child Health (UCL-ICH)

European School of Oncology (ESO)

European Medicine Agency (EMA)

European Network of Cancer Registry (ENCR)

Joint Research Centre (JRC)

Belgian Cancer Registry (BCR)

Italian National Institute of Health (Istituto Superiore di Sanità)

European Society for Medical Oncology (ESMO)

Istituto Superiore di Sanità (ISS - Rare Best Practice)

European Organisation for Research and Treatment of Cancer (EORTC)

Anticancerfund

Association of European Cancer Leagues (ECL)

European Society of Surgical Oncology (ESSO)

Childhood Cancer International (CCI-Europe)

St. Anna Children's Cancer Research Institute' (CCRI)

Rare Cancer Europe (RCE)

EFPIA-EuropaBio

University of Milan

Hospital Universitario y Politécnico La Fe. GICT-Cáncer IIS La Fe, (CICT)



Deliverables...





European Reference Networks

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



European
Reference
Networks

EpiCARE . BOND
. CRANIO . ENDO .
ERKNet . EYE . ERNICA .
VASCERN . LUNG . RND . SKIN
. EURACAN . GUARD-HEART
. EuroBloodNet . eUROGEN .
GENTURIS . ITHACA . MetabERN
. PaedCan . RARE-LIVER .
ReCONNET . EURO-NMD .
TRANSPLANT-CHILD .
RITA

Share. Care. Cure.

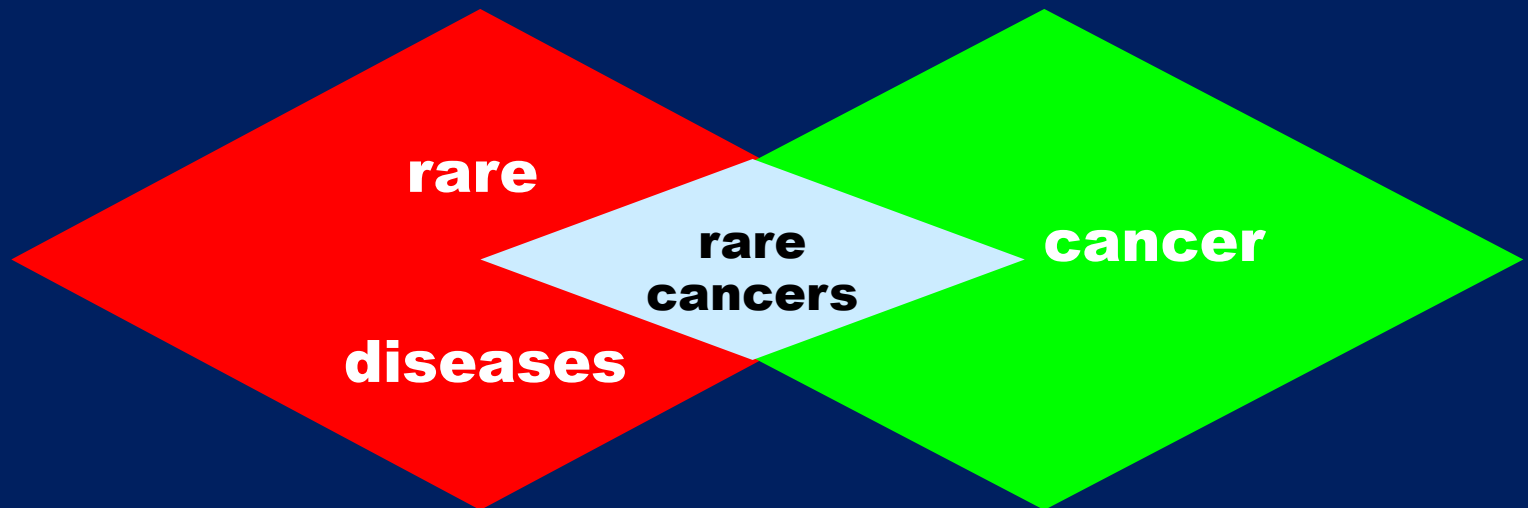


- promote good quality and safe **care** to patients by fostering proper diagnosis, treatment, follow-up and management of patients across the Network
- empower and involve **patients**
- offer and promote **multi-disciplinary advice for complex cases**
- develop and implement **clinical guidelines** and cross-border patient pathways
- exchange, gather and **disseminate knowledge**, evidence and expertise within and outside the Network
- promote collaborative **research** within the Network
- reinforce research and **epidemiological surveillance**, through setting up of shared registries
- exchange and disseminate knowledge and best practices, in particular by supporting **national centres and networks**

Definition...

1.

Rare cancers are the rare diseases of oncology and should be specifically approached by the cancer community, as well as within cancer planning by national health systems.



Epidemiological monitoring...

2.

Rare cancers should be strictly monitored epidemiologically by population-based cancer registries, in close interplay with clinical repositories, research efforts, administrative data bases.



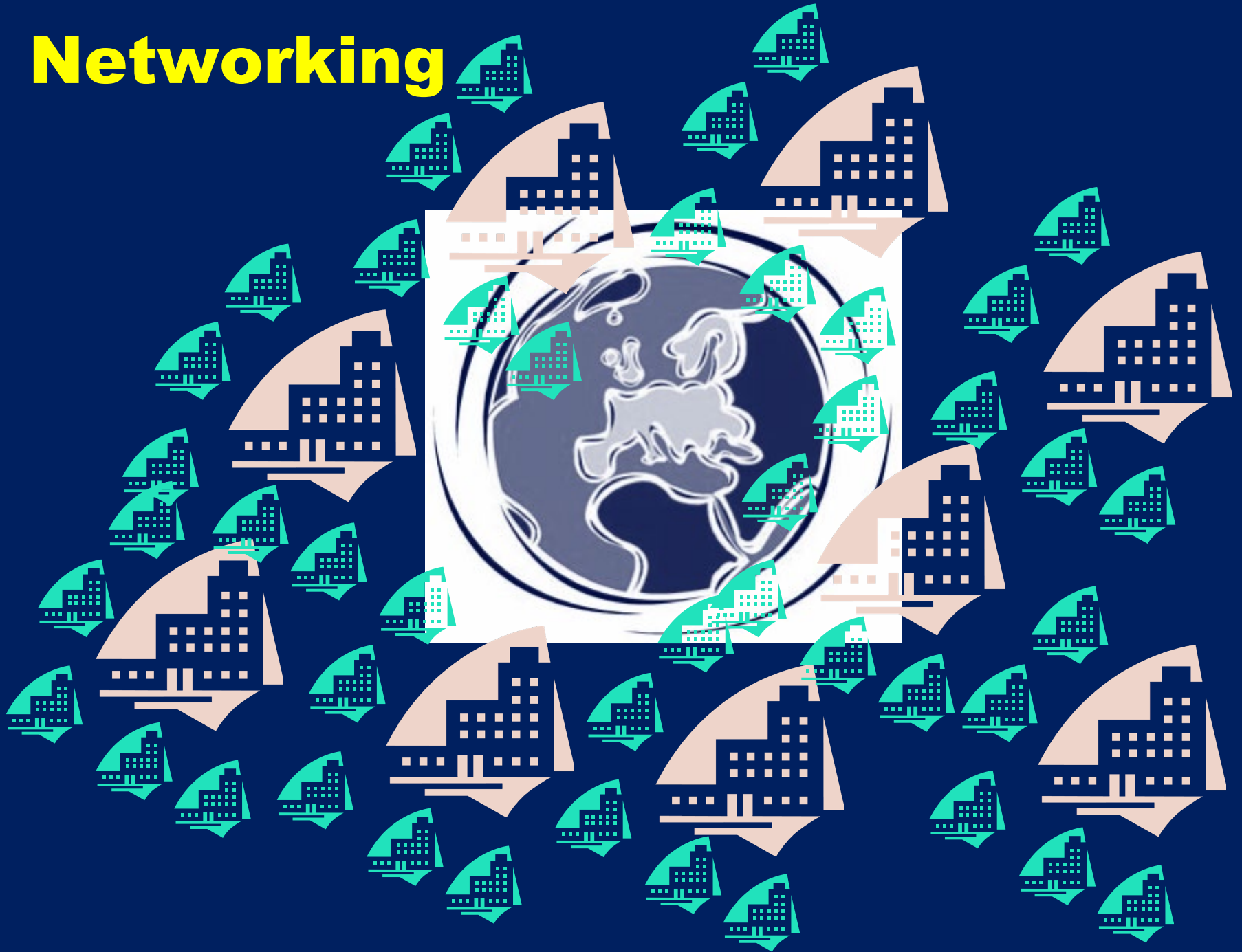
ERN Continuous Monitoring Working Group of the ERN Coordinators Group &
the Board of Member States

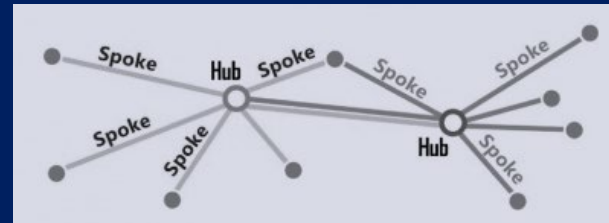
Networking...

3.

To improve quality of care in rare cancers, while diminishing/rationalizing health migration, health systems should exploit networking, built around multidisciplinary centres of reference, prioritizing pathologic diagnosis and strategic clinical decision-making.

Networking







Education & career...

4.

Medical education should exploit and serve healthcare networking, should target all families of rare cancers by proper integration with the university system and educational providers, and should be linked to dedicated career mechanisms and opportunities.

ESO-ESMO-ICE Clinical Update on Rare Adult Solid Cancers
25/11/2016 - 27/11/2016, Milan, Italy

Chair: F.G. Casali, IT - R.A. Stahel, CH

Rare cancers make up as many as one fifth of all new cancer cases. Amongst them, pediatric cancers and rare hematologic neoplasms are often covered by national educational events, while adult rare cancers are mostly grouped together and approached as a collective group of neoplasms. Indeed, they account for as many as 15% of all new cancer cases, thus being the main group within rare cancers. This Course is aimed at dealing with them as an actual entity, to provide the community of clinical oncologists operating in these tumors with a regular update of recent advances in this specific area. The ultimate goal is to strengthen the educational coverage of a group of cancers which may be neglected in spite of their collective incidence.

According to the RARECARE project, which provided a definition and a list of rare cancers, rare adult solid cancers include the following big families of tumors, each of them being thereafter covered by a corresponding educational session within the Course:

- Rare skin cancers and non-skin melanoma
- Rare breast tumors
- Rare female genital cancers
- Neuroendocrine tumors
- Endocrine gland tumors
- Sarcoma
- Digestive rare cancers
- Rare urological and male genital tumors
- Central nervous system neoplasms
- Head and neck cancers

Any educational effort in the rare cancer area makes little sense if disconnected from a networking environment. Thus, this Course will be first offered to clinical oncologists involved into the existing networks covering rare adult solid cancers, with a special attention to the new European Reference Networks. Sub to be invited within the ESO.

From the educational point of view, one cannot expect that even experienced clinical oncologists are knowledgeable about all these tumors. For this reason, the European Faculty of this Masterclass is asked to focus on the most recent advances, always trying to put them into the context of state of the art treatment of these tumors, with a special view to ESMO Clinical Practice Guidelines.

The course is an ESO-ESMO-ICE joint event





Held in collaboration with:









On the framework of:




European School of Oncology
Learning to care

**Training Courses
on Rare Adult Solid Cancers**



European
Reference
Networks

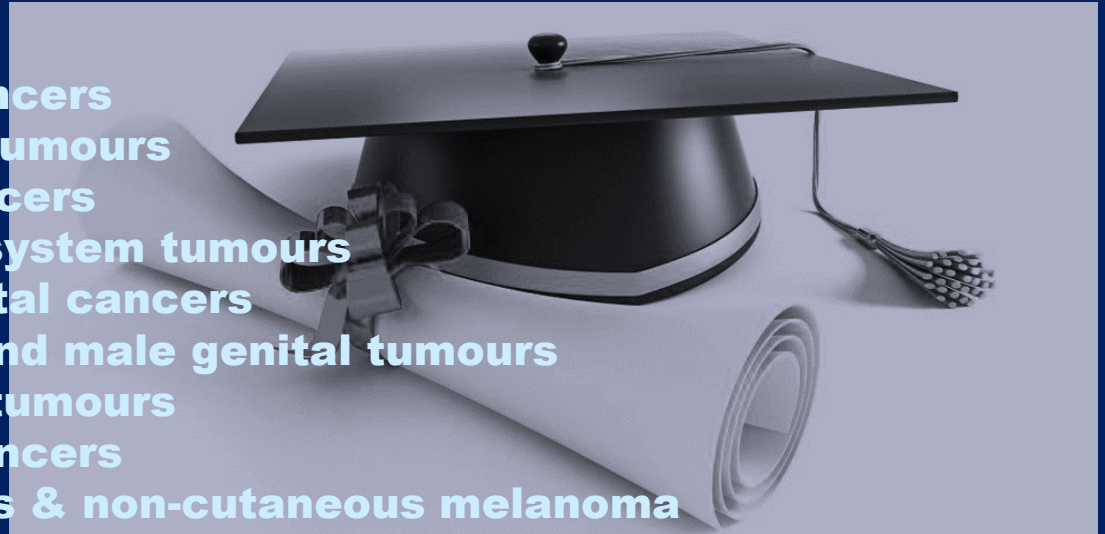


**Training Courses
on Rare Adult Solid Cancers**

R
CANCERS
EUROPE
E

European
Reference
Network
for rare or low prevalence
complex diseases
Rare
Adult Cancers
(RAC)

- 1. Pediatric cancers**
- 2. Haematologic rare neoplasms**
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UNION EUROPÉENNE DES MÉDECINS SPÉCIALISTES
EUROPEAN UNION OF MEDICAL SPECIALISTS

Association internationale sans but lucratif

International non-profit organisation

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BE- 1040 BRUSSELS
www.uems.eu

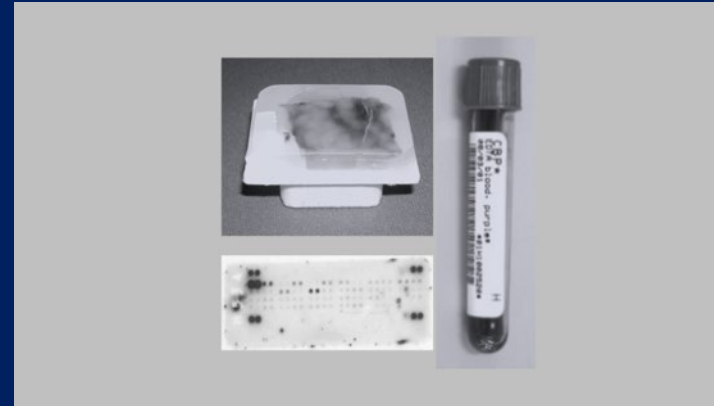
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F +32 2 640 37 30
info@uems.eu

Research...

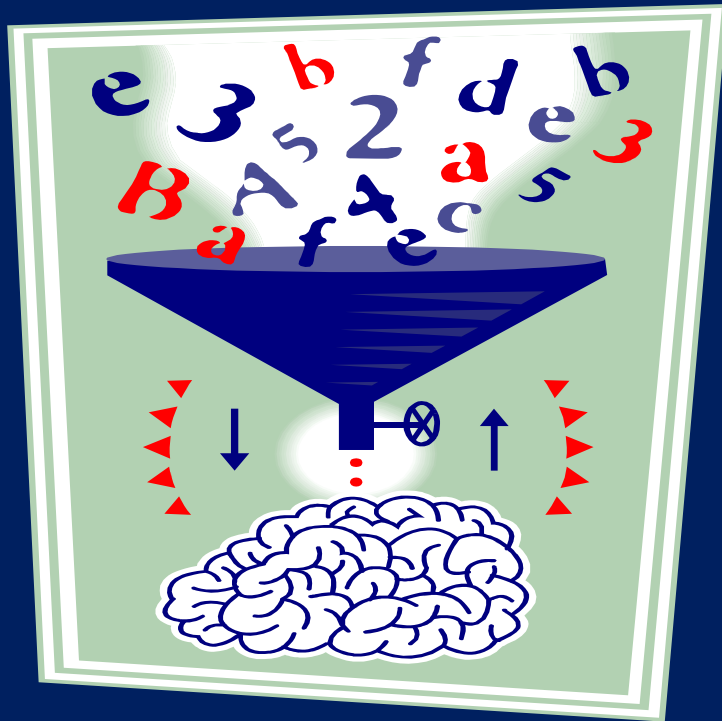
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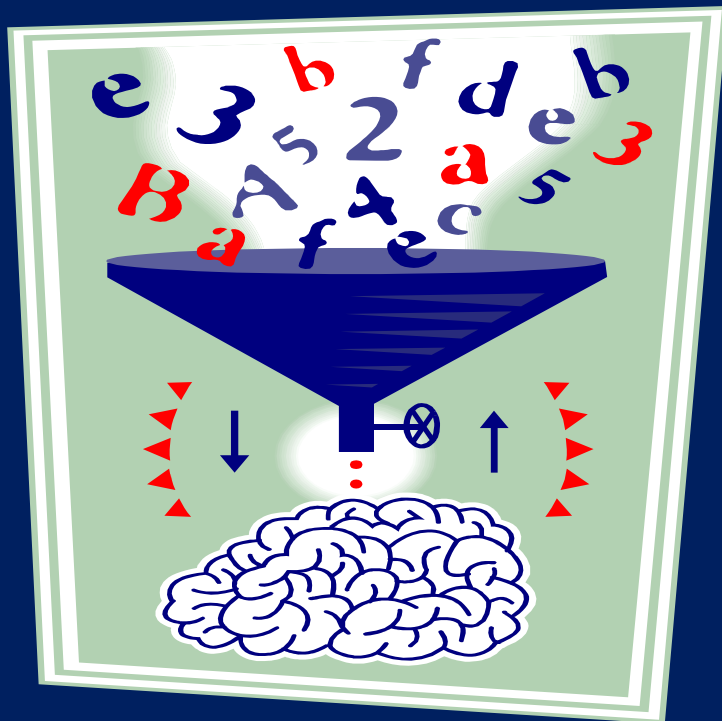
Research should be fostered by proper collaboration on clinically annotated biobanking and clinical registering, and should be able to build on healthcare and multidisciplinary collaborative networking.

Clinical registries & Biobanking



«Big data» & knowledge generation...





“BIG DATA & RCs”

Milan, Q2 2019

- **Methodological implications (vs clinical trials!)**
- **Technological windows of opportunity**
- **Added values for rare cancers**

Clinical practice guidelines...

7.

All usual state of the art instruments should be developed in rare cancers, properly accomodating the possibly high degree of uncertainty.

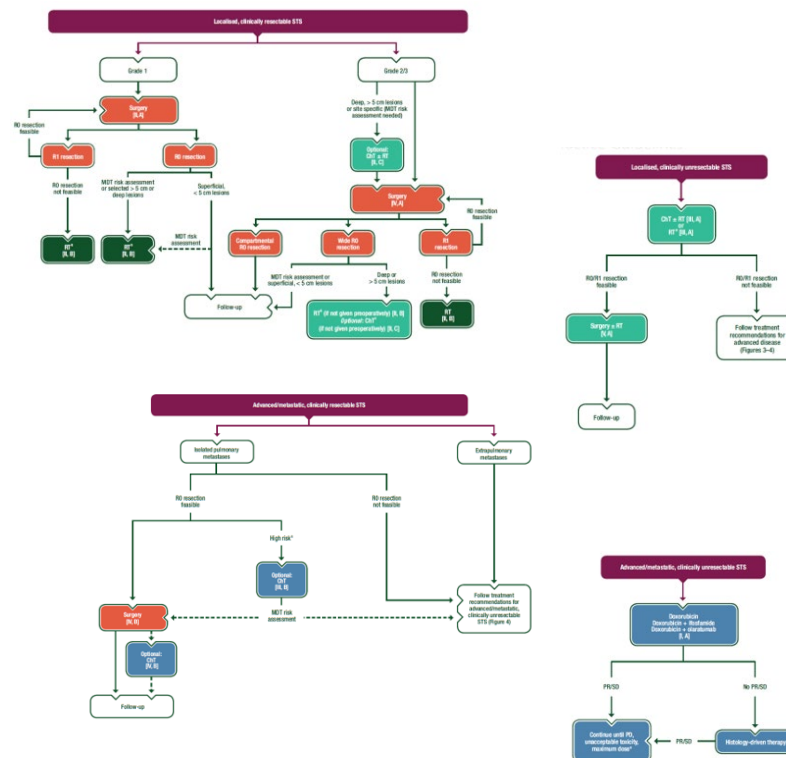


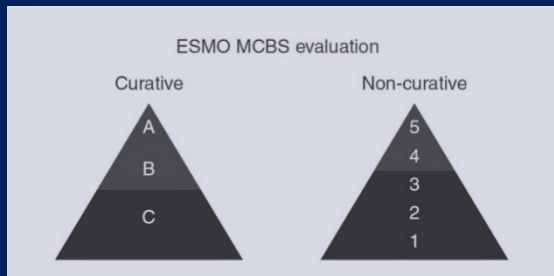
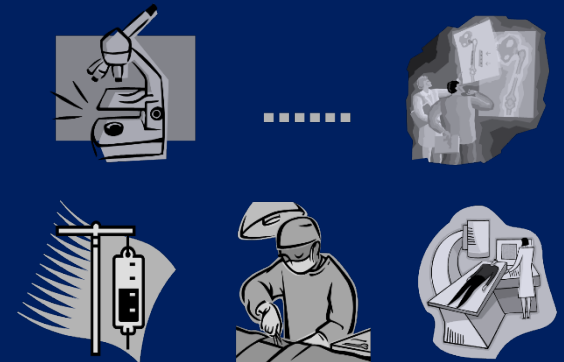
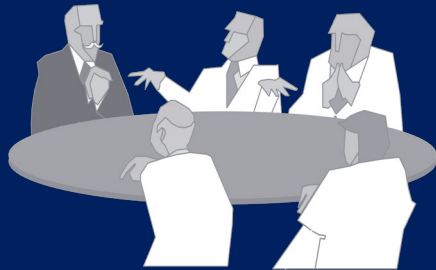
CLINICAL PRACTICE GUIDELINES

Soft tissue and visceral sarcomas: ESMO–EURACAN Clinical Practice Guidelines for diagnosis, treatment and follow-up[†]

P. G. Casali¹, N. Abecassis², S. Bauer³, R. Biagini⁴, S. Bielack⁵, S. Bonvalot⁶, I. Boukovinas⁷, J. V. M. G. Bovee⁸, T. Brodowicz⁹, J. M. Broto¹⁰, A. Buonadonna¹¹, E. De Álava¹⁰, A. P. Dei Tos¹², X. G. Del Muro¹³, P. Dileo¹⁴, M. Eriksson¹⁵, A. Fedenko¹⁶, V. Ferraresi¹⁷, A. Ferrari¹⁸, S. Ferrari¹⁹, A. M. Frezza¹, S. Gasperoni²⁰, H. Gelderblom²¹, T. Gil²², G. Grignani²³, A. Gronchi¹, A. Hannu²⁴, B. Hassan²⁵, P. Hohenberger²⁶, R. Issels²⁷, H. Joensuu²⁸, R. L. Jones²⁹, I. Judson³⁰, P. Jutte³¹, S. Kaal³², B. Kasper²⁶, K. Kopeckova³³, D. A. Krákorová³⁴, A. Le Cesne³⁵, I. Lugowska³⁶, O. Merimsky³⁷, M. Montemurro³⁸, M. A. Pantaleo³⁹, R. Piana⁴⁰, P. Picci¹⁹, S. Piperno-Neumann⁶, A. L. Pousa⁴¹, P. Reichardt⁴², M. H. Robinson⁴³, P. Rutkowski³⁶, A. A. Safwat⁴⁴, P. Schöffski⁴⁵, S. Sleijfer⁴⁶, S. Stacchiotti⁴⁷, K. Sundby⁴⁸, M. Unk⁴⁹, F. Van Coevorden⁵⁰, W. Van der Graaf²⁹, J. Whelan⁵¹, E. Wardelmann⁵², O. Zaikova⁵³ & J. Y. Blay⁵⁴, on behalf of the ESMO Guidelines Committee and EURACAN*

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(adapted from the Infectious Diseases Society of America United States Public Health Service Grading System^a)

Levels of evidence	
I	Evidence from at least one large, randomised, controlled trial of good methodological quality (low potential for bias) or meta-analyses of well-conducted, randomised trials without heterogeneity
II	Small, randomised trials or large, randomised trials with a suspicion of bias (lower methodological quality) or meta-analyses of such trials or of trials with demonstrated heterogeneity
III	Prospective cohort studies
IV	Retrospective cohort studies or case-control studies
V	Studies without the control group, case reports, experts opinions
Grades of recommendation	
A	Strong evidence for efficacy with a substantial clinical benefit, strongly recommended
B	Strong or moderate evidence for efficacy but with a limited clinical benefit, generally recommended
C	Insufficient evidence for efficacy or benefit does not outweigh the risk or the disadvantages (adverse events, costs, ...), optional
D	Moderate evidence against the efficacy or for adverse outcomes, generally not recommended
E	Strong evidence against the efficacy or for adverse outcomes, never recommended

^aBy permission of the Infectious Diseases Society of America [163].

Clinical practice guidelines...

7.

All usual state of the art instruments should be developed in rare cancers, properly accomodating the possibly high degree of uncertainty.

Regulation...

8.

Regulatory mechanisms on rare cancers, including new drug licensing, should tolerate the possibly higher degree of uncertainty, should be disease-adapted and should provide developers of innovation with certainty of rules.



- **Tolerance on quality of evidence**
- **Clinical registries within ERN**
- **Disease-based broad advice**
- **Adaptive licensing mechanisms within ERN**
- **Real world data**

London, April 16th 2018

Sustainability...

9.

Sustainability should be addressed by optimizing networking, also providing evidence of its economies, and by pursuing a value-based medicine aware of the many difficulties of rare cancers.



RARE CANCERS: AN AGENDA TOWARDS 2030



Welcome to JARC

EU Joint Action on Rare Cancers (JARC)

www.jointactionrarecancers.eu



FONDAZIONE IRCCS
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JOINT ACTION ON **RARE** CANCERS



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