RARE CANCERS EUROPE

Joining forces for action

Welcome from Rare Cancers Europe

Paolo G. Casali
paolo.casali@istitutotumori.mi.it

Show your support and sign the
Call to Action Against Rare Cancers:

www.rarecancerseurope.org

RARE CANCERS

More common than you think!

Rare Cancers Europe is a joint initiative based on a partnership between the European Society for Medical Oncology (ESMO), the European Organisation for Rare Diseases (EURORDIS), the European Cancer Patient Coalition (ECPC), the European Organisation for Research and Treatment of Cancer (EORTC), Conticanet, EuroBoNeT, the World Sarcoma Network (WSN), the Association of European Cancer Leagues (ECL), the Chronic Myeloid Leukaemia Support Group, the International Brain Tumour Alliance (IBTA), Orphanet, the Chronic Myeloid Leukaemia Advocates Network, the Sarcoma Patients EuroNet Association (SPAEN), GIST Support UK & PAWS-GIST, Cancer 52, the International Kidney Cancer Coalition (IKCC), the Chordoma Foundation, the Fondazione IRCCS Istituto Nazionale dei Tumori, the European Institute of Oncology (IEO), the European Society for Paediatric Oncology (SIOP Europe), the European Society of Surgical Oncology (ESSO), the Grupo Español de Tumores Huérfanos e Infrecuentes (GETHI), the European School of Oncology (ESO), the European Oncology Nursing Society (EONS), ecancer, the European Society of Pathology (ESP), the European, Middle Eastern and African Society for Biopreservation and Biobanking (ESBB), Novartis Oncology (initiating sponsor and industry partner), Pfizer Oncology (industry partner), and Sanofi (industry partner). The campaign is moreover supported by additional corporate supporters, including Amgen (silver industry supporter) and Takeda Pharmaceuticals Europe (silver industry supporter).

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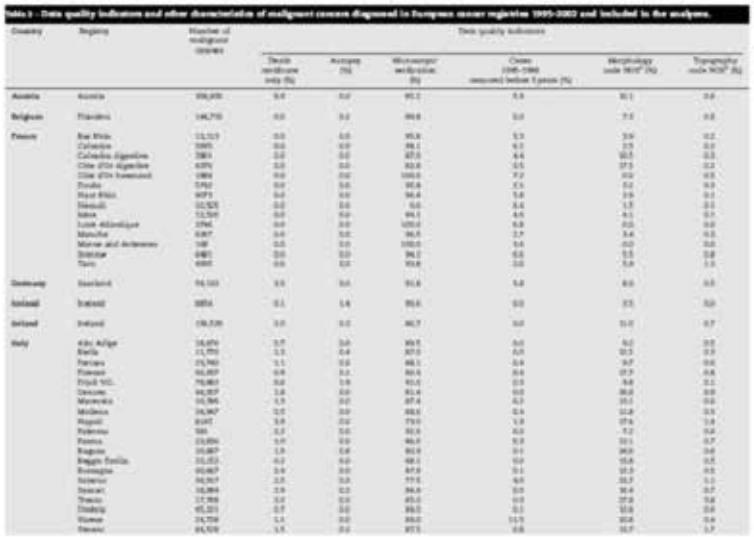
Rare cancers are not so rare: The rare cancer burden in Europe

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*



Eur J Cancer 2011;47:2493

SOFT TISSUE SARCOMA

	Incidence				Prevalence				Mortality			
	Counts	Rate	Lower CI	Upper CI	Counts	Rate	Lower CI	Upper CI	Counts	Rate	Lower CI	Upper CI
Expected cases a year in EU	23655				142509				14573			
Crude Rate - Overall	36527	4.75	4.70	4.80	16335	26.65	26.23	29.07	23756	2.93	2.90	2.96
Sex												
Men	17853	4.46	4.39	4.52	8113	26.05	25.46	26.62	10865	2.75	2.71	2.79
Women	20674	5.05	4.98	5.10	10221	31.11	30.51	31.72	12871	3.10	3.06	3.14
Age												
0-14	1537	0.92	0.87	0.97	617	5.42	5.00	5.87	370	0.25	0.24	0.27
15-24	1337	1.28	1.21	1.35	780	9.58	8.94	10.29	452	0.40	0.40	0.44
25-64	19080	4.41	4.35	4.47	9600	26.71	26.14	29.28	10106	2.34	2.30	2.37
65 +	16773	13.10	12.90	13.30	7037	69.55	67.93	71.19	9648	7.69	7.56	7.81
Region*												
North	6055	4.89	4.57	4.92	4923	36.02	35.02	37.04	3734	2.89	2.82	2.97
Centre	9230	4.49	4.39	4.58	4102	31.54	30.59	33.51	5753	2.77	2.71	2.83
East	2379	3.29	3.18	3.43	1134	18.55	17.49	19.67	1467	2.03	1.95	2.11
South	8200	4.55	4.45	4.65	1877	34.11	32.56	35.69	5130	2.81	2.74	2.87
UK-Ireland	12443	3.80	3.73	3.87	6186	24.39	23.78	25.00	7673	2.34	2.30	2.38
Overall EU	36527	4.19	4.14	4.23	16335	26.65	26.23	29.07	23756	2.68	2.66	2.71
Gross Domestic Product level*												
Low GDP 0-20000	4064	3.75	3.61	3.85	1134	18.55	17.49	19.67	2518	2.30	2.23	2.37
Middle GDP 20000-25000	25826	4.25	4.19	4.30	11394	28.03	28.30	29.37	15809	2.82	2.79	2.85
High GDP >25000	8098	4.34	4.24	4.44	5457	32.58	31.70	33.43	4982	2.88	2.82	2.74
Total National Expenditure on Health level*												
TNEH low 0-1500	4064	3.73	3.61	3.85	1134	18.55	17.49	19.67	2518	2.30	2.23	2.37
TNEH middle >1501-2250	30025	4.27	4.23	4.32	14393	29.50	29.02	29.98	18823	2.83	2.80	2.86
TNEH high >2250	3698	4.25	4.11	4.40	2547	33.08	31.74	34.43	2379	2.82	2.54	2.71

* Age adjusted Rates

N	Cohort 1995-99					
2681	Observed			Relative		
Rel Survivyear	Survival	Lower CI	Upper CI	Survival	Lower CI	Upper CI
1 yr	74.3	73.7	74.8	76.3	75.7	76.8
2 yr	62.8	62.2	63.4	66.2	65.6	66.8
3 yr	56.1	55.4	56.7	60.0	60.1	61.5
4 yr	51.8	51.3	52.4	57.0	57.1	58.5
5 yr	48.7	48.0	49.3	55.6	55.1	56.5

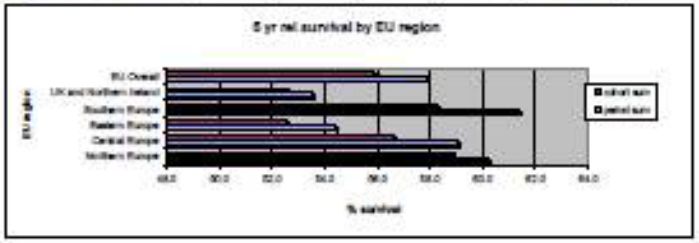
Rel Survivyear	Period 2000-2002						
	Observed			Relative			Cohort
Rel Survivyear	Survival	Lower CI	Upper CI	Survival	Lower CI	Upper CI	of Diagnosis
1 yr	75.1	74.2	75.9	77.1	76.3	77.9	2000-2002
2 yr	64.1	63.1	65.0	67.6	66.7	68.4	1999-2001
3 yr	57.9	56.9	58.9	62.0	61.0	63.7	1998-2000
4 yr	53.6	52.6	54.6	58.9	58.0	60.8	1997-1999
5 yr	50.5	49.5	51.6	57.0	57.0	58.8	1996-1998

Rel. Survival by Gross Domestic Product per capita, US$ PPP	Period 2000-2002			Cohort 1995-99		
	Relative			Relative		
	Survival	Lower CI	Upper CI	Survival	Lower CI	Upper CI
Low GDP 0-20000	54.3	50.6	58.1	53.9	51.9	55.9
Middle GDP 20000-25000	56.9	57.6	60.2	56.2	55.3	57.1
High GDP >25000	56.6	54.5	58.8	56.3	54.7	58.0

Rel. Survival by TNEH per capita, US$ PPP	Period 2000-2002			Cohort 1995-99		
	Relative			Relative		
	Survival	Lower CI	Upper CI	Survival	Lower CI	Upper CI
Low TNEH 0-1500	54.3	50.6	58.1	53.9	51.9	55.9
Middle TNEH 1501-2250	56.1	56.9	59.3	56.1	55.3	56.9
High TNEH >2250	59.8	58.1	63.4	57.2	54.9	59.5

5 yr Relative Survival by EU Region	Period 2000-2002			Cohort 1995-99		
	Relative			Relative		
Region	Survival	Lower CI	Upper CI	Survival	Lower CI	Upper CI
Northern Europe	60.2	57.6	62.8	59.0	57.0	60.9
Central Europe	58.1	57.1	61.1	56.6	55.2	58.2
Eastern Europe	54.4	50.7	58.4	52.6	49.7	55.6
Southern Europe	61.4	59.1	63.7	58.3	56.8	59.8
UK and Northern Ireland	53.5	51.5	55.6	52.6	51.3	53.9
EU Overall	57.9	56.9	59.0	55.9	55.1	56.6

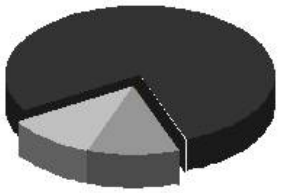
! The statistic could not be calculated



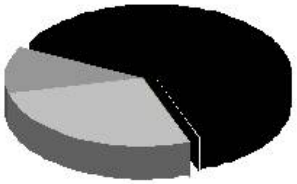
“Rare” cancers

incidence ≤ 6 /100,000/year

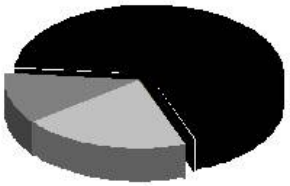
Incidence <6/100,000/y



Incidence <15/100,000/y



Prevalence <50/100,000

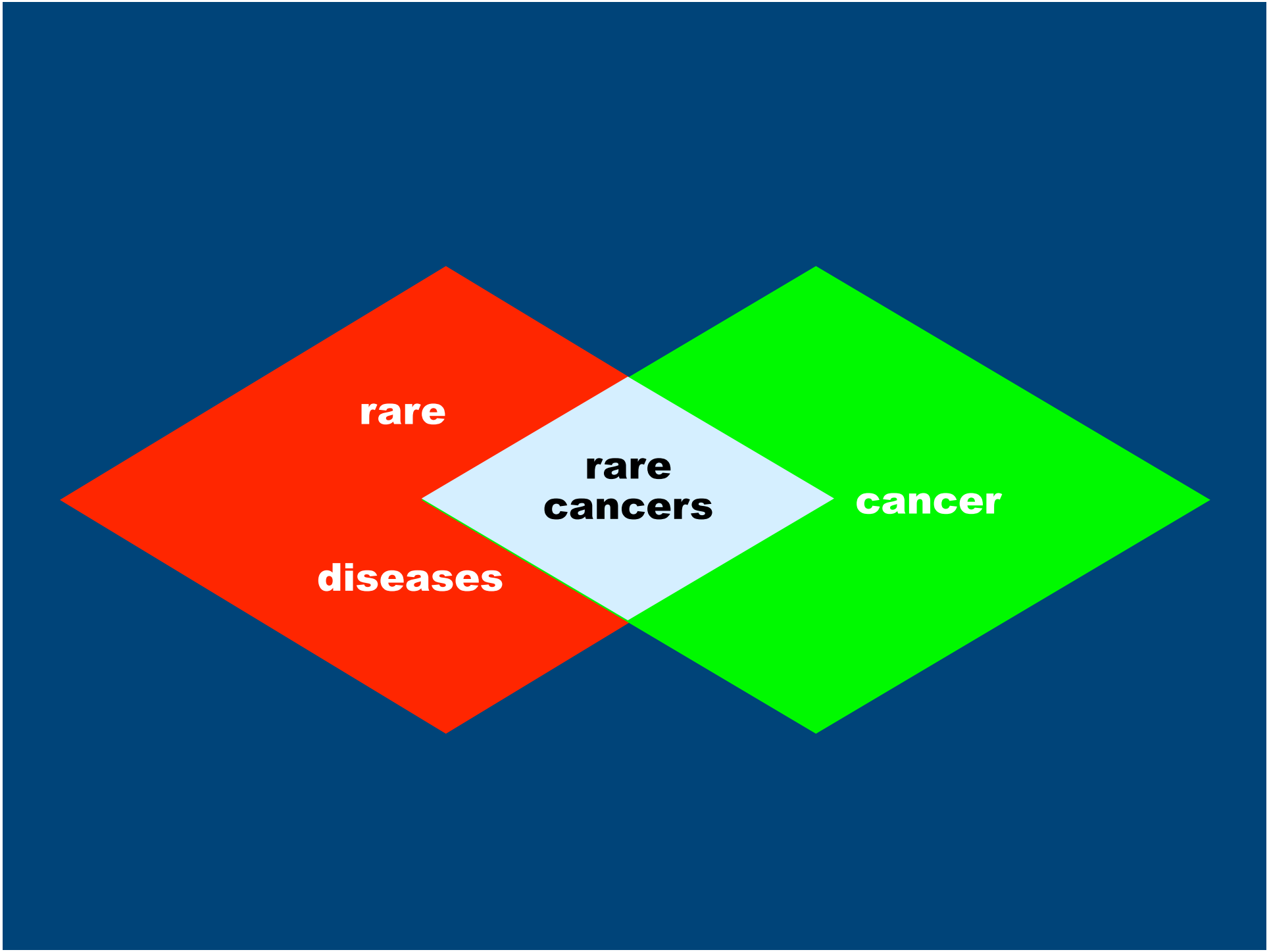


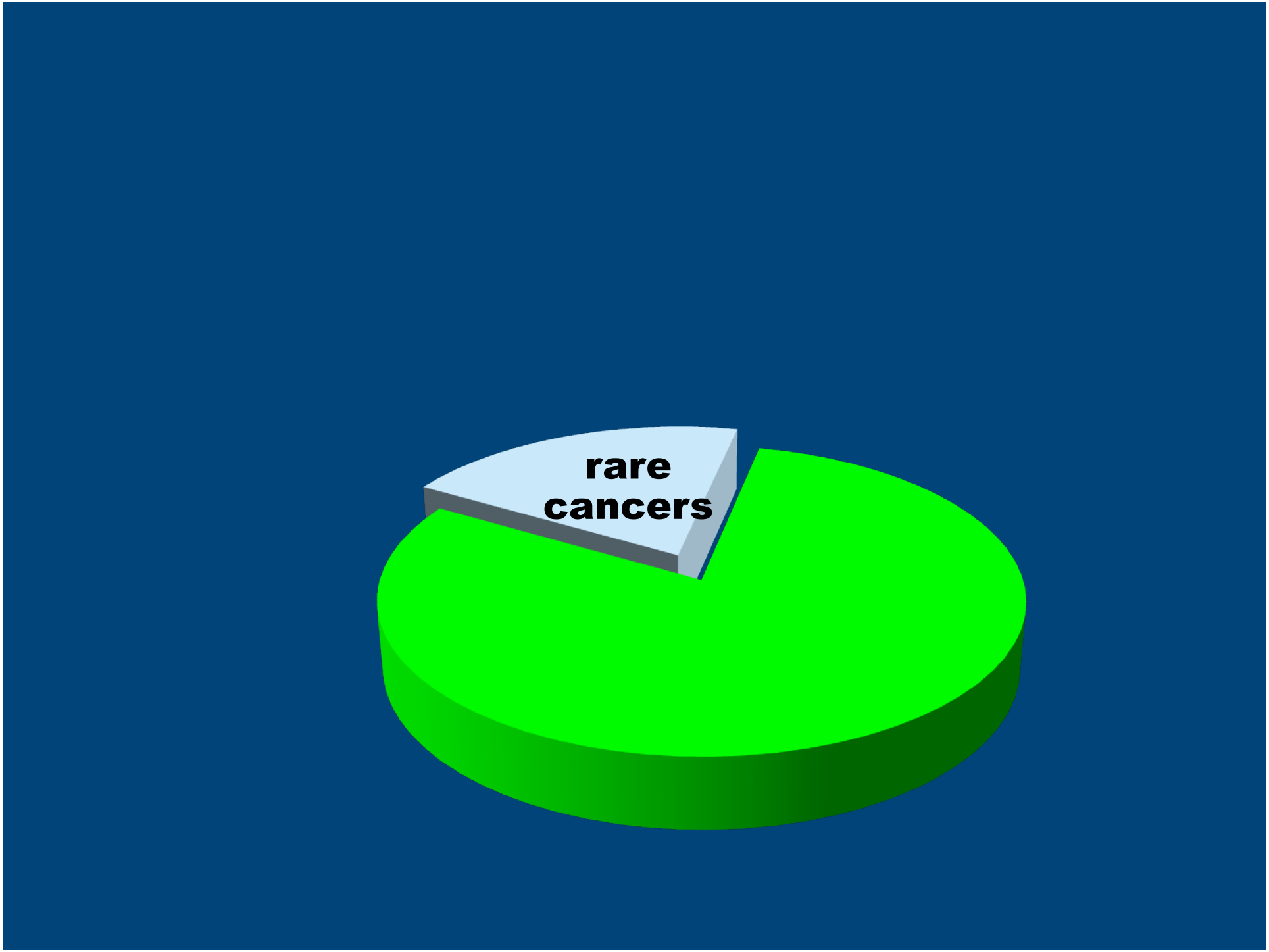
= *rare ("tier 1")*

\+ = *rare ("tier 2")*

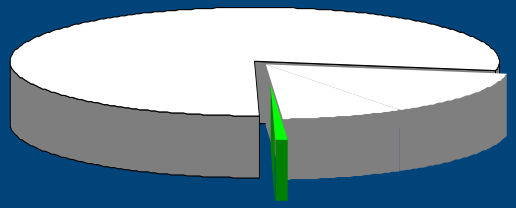
= *frequent*

Casali PG et al, 2015, to be submitted


rare
diseases
rare
cancers
cancer

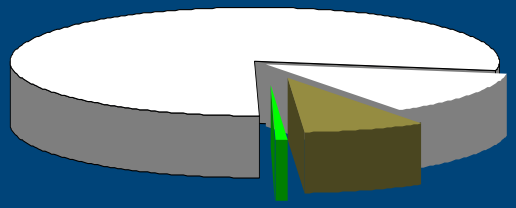

rare
cancers

Rare cancers...



childhood

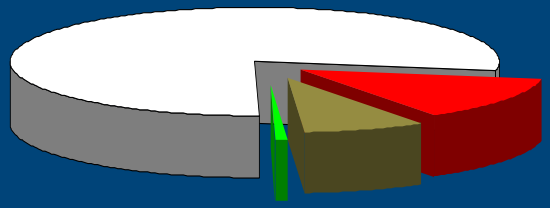
Rare cancers...



hematological

childhood

Rare cancers...



adult solid tumors

hematological

childhood

«Families» of rare cancers

1. SKIN/rare & non cutaneous MELANOMA
2. THORACIC/rare
3. MALE UROGENITAL/rare
4. FEMALE GENITAL/rare
5. DIGESTIVE/rare
6. NEUROENDOCRINE
7. ENDOCRINE ORGAN
8. CNS
9. SARCOMAS
10. HEAD & NECK

11. HEMATOLOGICAL/rare

12. PEDIATRIC

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RARE CANCERS EUROPE

review

Annals of Oncology 00: 1–7, 2014
doi:10.1093/annonc/mdu459

Rare Cancers Europe (RCE) methodological recommendations for clinical studies in rare cancers: a European consensus position paper

P. G. Casali[1]*, P. Bruzzi[2], J. Bogaerts[3] & J.-Y. Blay[4] on behalf of the Rare Cancers Europe (RCE) Consensus Panel

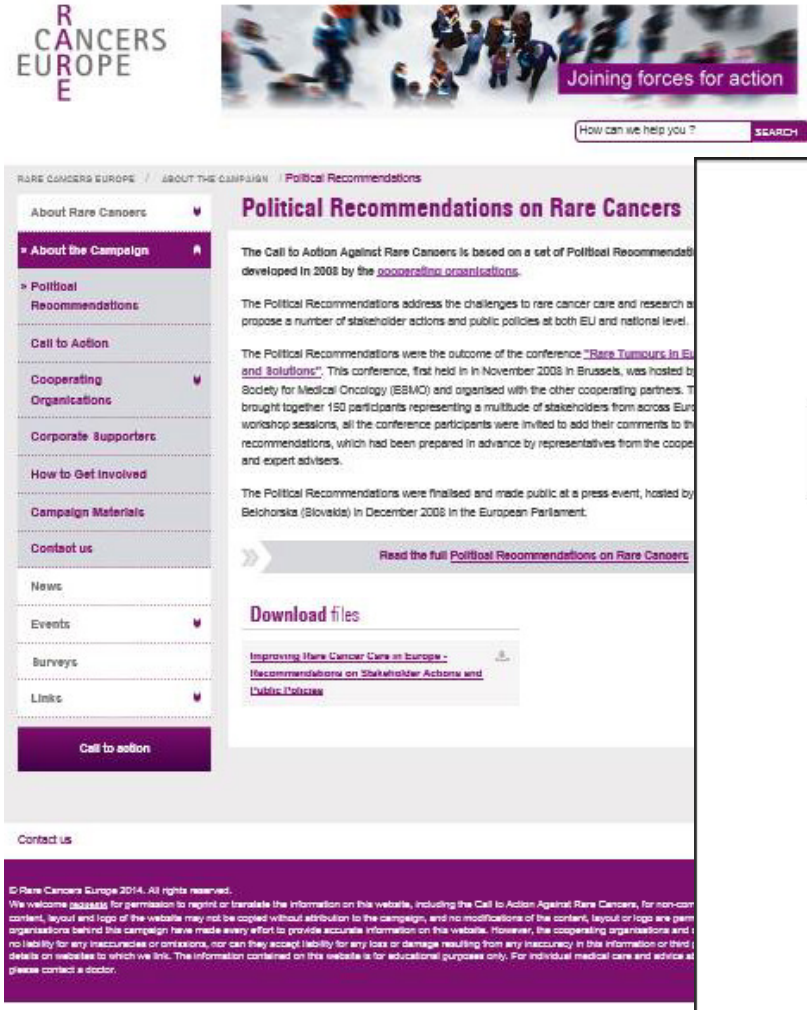
[1]*Adult Mesenchymal Tumour Medical Oncology Unit, Fondazione IRCCS Istituto Nazionale Tumori, Milan;* [2]*Clinical Epidemiology Unit, National Institute for Cancer Research, Genova, Italy;* [3]*European Organization for Research and Treatment of Cancer (EORTC), Brussels, Belgium;* [4]*Department of Medical Oncology, Centre Léon Bérard, Centre de Recherche en Cancérologie, Université de Lyon, Lyon, France*

Received 29 July 2014; revised 18 September 2014; accepted 19 September 2014

While they account for one-fifth of new cancer cases, rare cancers are difficult to study. A higher than average degree of uncertainty should be accommodated for clinical as well as for population-based decision making. Rules of rational decision making in conditions of uncertainty should be rigorously followed and would need widely informative clinical trials. In principle, any piece of new evidence would need to be exploited in rare cancers. Methodologies to explicitly weigh and combine all the available evidence should be refined, and the Bayesian logic can be instrumental to this end. Likewise, Bayesian-design trials may help optimize the low number of patients liable to be enrolled in clinical studies on rare cancers, as well as adaptive trials in general, with their inherent potential of flexibility when properly applied. While clinical studies are the mainstay to test hypotheses, the potential of electronic patient records should be exploited to generate new hypotheses, to create external controls for future studies (when internal controls are unpractical), to study effectiveness of new treatments in real conditions. Framework study protocols in specific rare cancers to sequentially test sets of new agents, as from the early post-phase I development stage, should be encouraged. Also the compassionate and the off-label settings should be exploited to generate new evidence, and flexible regulatory innovations such as adaptive licensing could convey new agents early to rare cancer patients, while generating evidence. Though validation of surrogate end points is problematic in rare cancers, the use of an updated notion of tumor response may be of great value in the single patient to optimize the use of therapies, all the more the new ones. Disease-based communities, involving clinicians and patients, should be regularly consulted by regulatory bodies when setting their policies on drug approval and reimbursement in specific rare cancers.

Key words: rare cancers, clinical trials, research methodology

Ann Oncol. 2015 Feb;26(2):300



ESMO
GOOD SCIENCE BETTER MEDICINE BEST PRACTICE
European Society for Medical Oncology

Improving Rare Cancer Care in Europe

Recommendations on Stakeholder Actions and Public Policies

Whereas

A. Rare cancers[1] belong to the group of rare diseases that are normally defined as diseases with a prevalence of less than 50 out of 100,000. Even when defined more conservatively by taking into account some peculiarities of natural history and prognosis (e.g. by selecting those cancers with an incidence rate around or lower than 5/100,000/year), rare cancers represent about 20% of all cases of malignant neoplasms, including all cancers affecting children and teenagers and many affecting young adults;

B. There are significant variations in incidence and mortality rates for different types of rare cancers. There are also significant survival differences for the same types of rare cancers between the EU member states;[2]

C. Patients' access to treatments for rare cancers varies across and within the EU member states. Information about rare cancers, their treatment options and where to obtain appropriate treatment is in many cases not readily available to patients;

D. Sub-optimal treatment outcomes are common for rare cancers due to a lack of medical expertise in the management of rare cancers, poor referral rates from general practitioners and pathologic misdiagnosis. Outcomes for a diverse range of rare cancers could be improved through the establishment of reference networks or centres of expertise. However, few reference networks or centres of expertise exist across the EU and funding is not available to cover the increased costs associated with the organisation of these networks;

E. Overall health and social costs can be far higher for patients with rare cancers because effective treatments are not always reimbursed, referrals for second

[1] This includes solid, liquid and paediatric tumours.
[2] Gatta G. Et al. Survival from rare cancer in adults: a population-based study. *Lancet Oncology*. 2006 Feb;7(2):132-40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CANCER
PATIENT
COALITION
EORTC
Conticanet
EuroBoNeT
ECL
cmlsupport
IBTA
INTERNATIONAL
BRAIN TUMOUR
ALLIANCE
orphanet
Sarcoma
Patients
EuroNet
gist
support uk
cancer52
IKCC
CHORDOMA
FOUNDATION
FONDAZIONE IRCCS
ISTITUTO NAZIONALE
DEI TUMORI
IEO
European Institute of Oncology
SIOPE
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European
Society of
Pathology
NOVARTIS
ONCOLOGY
Pfizer Oncology
SANOFI
Lilly

European Action Against Rare Cancers

Recommendations Addressing Regulatory Barriers in Rare Cancer Care

We:

1. Acknowledge that while the process for establishing the efficacy of new medicines is in principle the same for all cancers, the strength of the evidence – intended as level and quality of evidence and statistical precision – that is achievable in common cancers is difficult to achieve in rare conditions and, therefore, a higher degree of uncertainty should be accepted for regulatory as well as clinically informed decision-making.

review

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Key words: rare cancers, clinical trials, research methodology

- **Clinical decision-making**
- **Methods to combine evidence**
- **New study designs**
- **Surrogate end points**
- **Organization of studies**

How can we help you ? SEARCH

REPORTS FROM PAST EVENTS / Rare Cancers Conference 2012

Rare Cancers Conference 2012

Exploring ways to improve clinical research on rare cancers

Date : 01 Mar 2012

Organised by the European Society for Medical Oncology (ESMO) and Rare Cancers Europe, the Rare Cancers Conference, held on 10 February 2012 in Brussels, provided a multi-stakeholder platform for rare cancer and rare disease experts from across Europe to exchange views and share insights into what can be done to improve the methodology of clinical research on rare cancers.

The first two conference sessions offered an overview of rare cancers and associated challenges for clinical research and drug development and also presented a variety of (potential) solutions as well as best practice examples. Where traditional frequent clinical research approaches are not possible, due to the small numbers of patients, it is particularly challenging to make sure that rare cancer patients are not being left without appropriate clinical research and therapeutic progress.

The third session of the conference therefore also highlighted the need for reaching a broad multi-stakeholder consensus on a set of recommendations on improving the methodology of clinical research on rare cancers. These recommendations will be the product of an ongoing multidisciplinary and multi-stakeholder online consensus discussion, promoted by Rare Cancers Europe. They will focus on best methods, including innovative ones, for clinical research on rare cancers, and rare subgroups of frequent cancers, with the goal of encouraging:

- clinical researchers to exploit innovative solutions for the design and analysis of clinical studies;
- clinicians to exploit innovative solutions for the combination of all available knowledge;
- regulators to accept evidence built through these solutions;
- clinicians' and patients' communities to exploit all forms of collaboration to put together as large series as possible for prospective and retrospective clinical and translational research;
- methodologists to advance research into new methodological solutions better fitting the needs of studies on small series

All interested stakeholder groups are encouraged to actively participate in this open discussion, the result of which will be a consensus paper to be publicly presented in autumn 2012. This paper could then be used for related advocacy efforts. All parties interested in joining this discussion are invited to contact Rare Cancers Europe.

review

Annals of Oncology 00: 1–7, 2014
doi: 10.1093/annonc/mdu503

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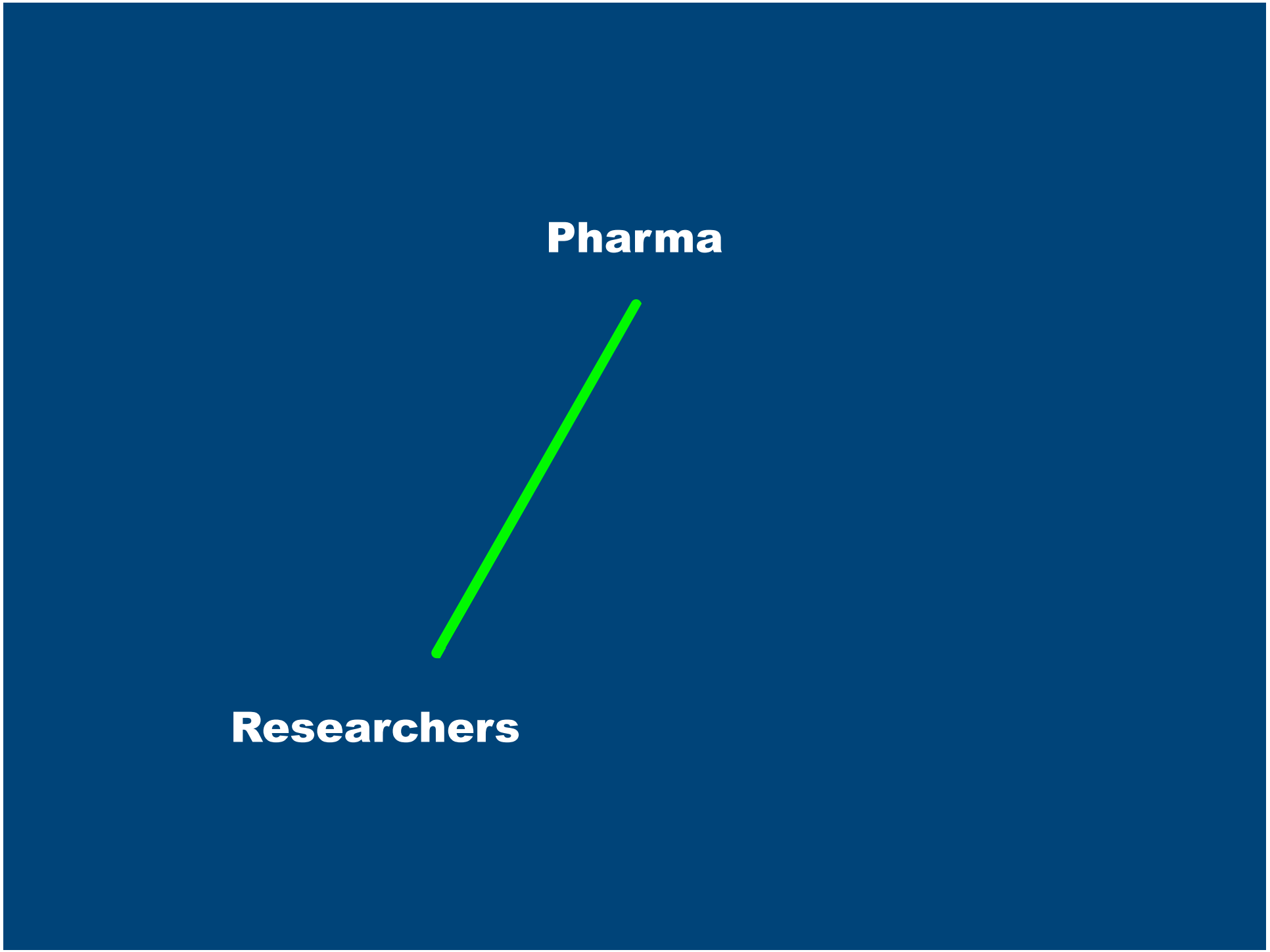
EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

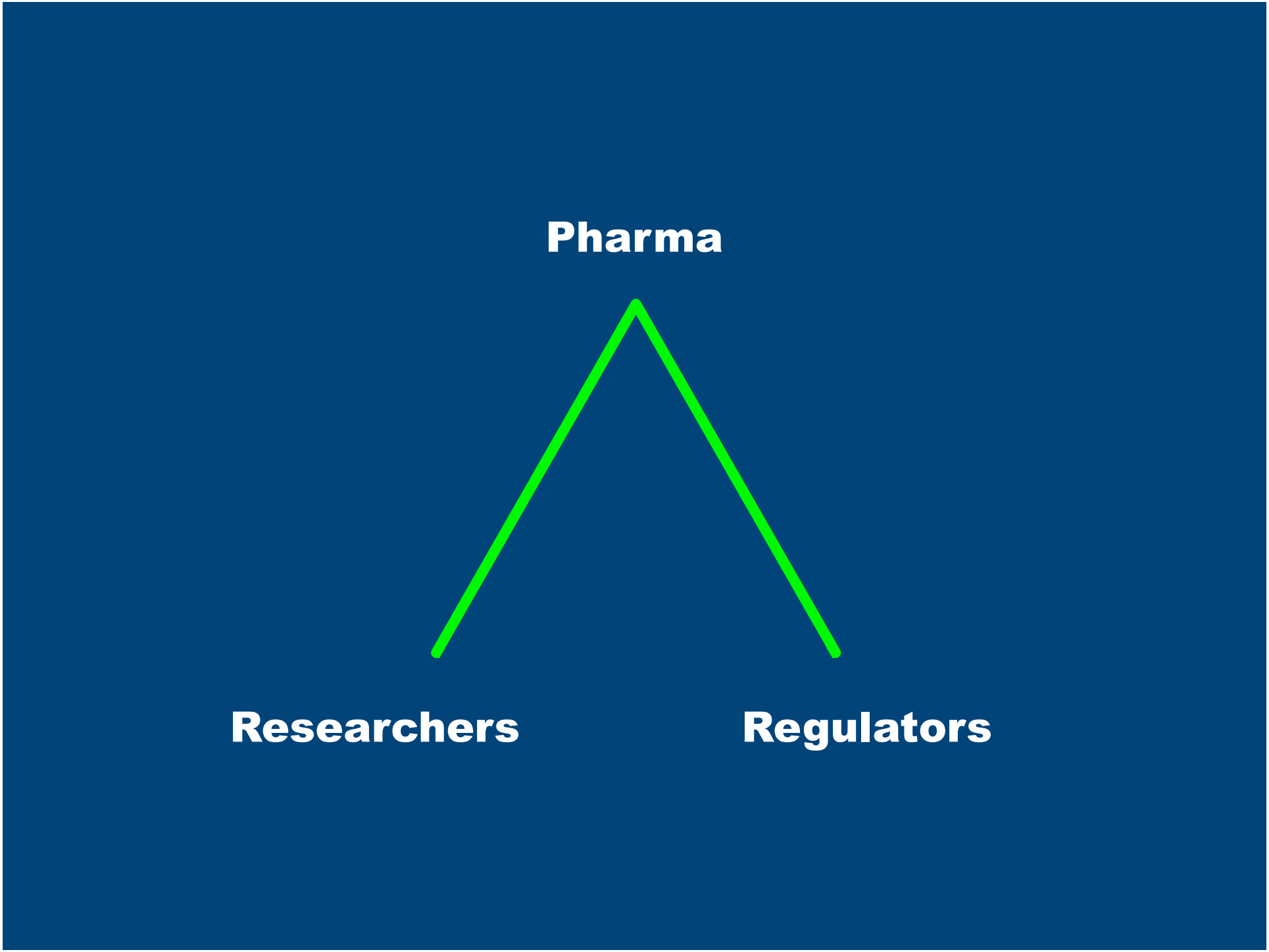
Josh Sommer

@sommerjo

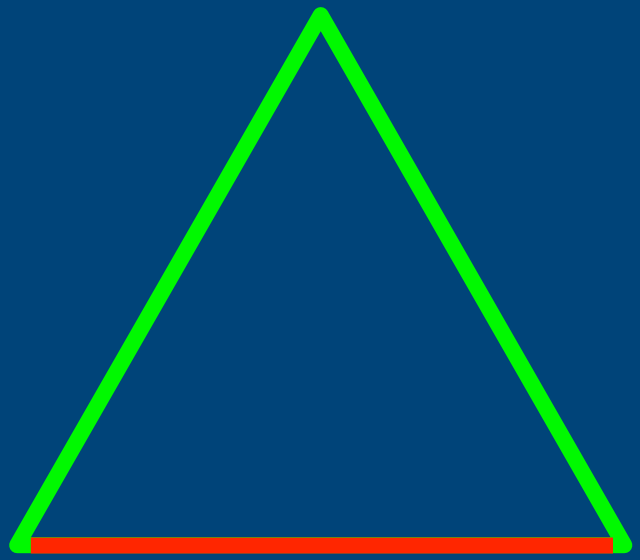
Today at EMA regulators, clinicians, @ChordomaFDN agreed to work together on guidance for chordoma trials. Progress! pic.twitter.com/ShR3SA8KXZ

30/04/2015 19:47:22


Pharma
Researchers


Pharma
Researchers
Regulators

Pharma



Researchers
Patients

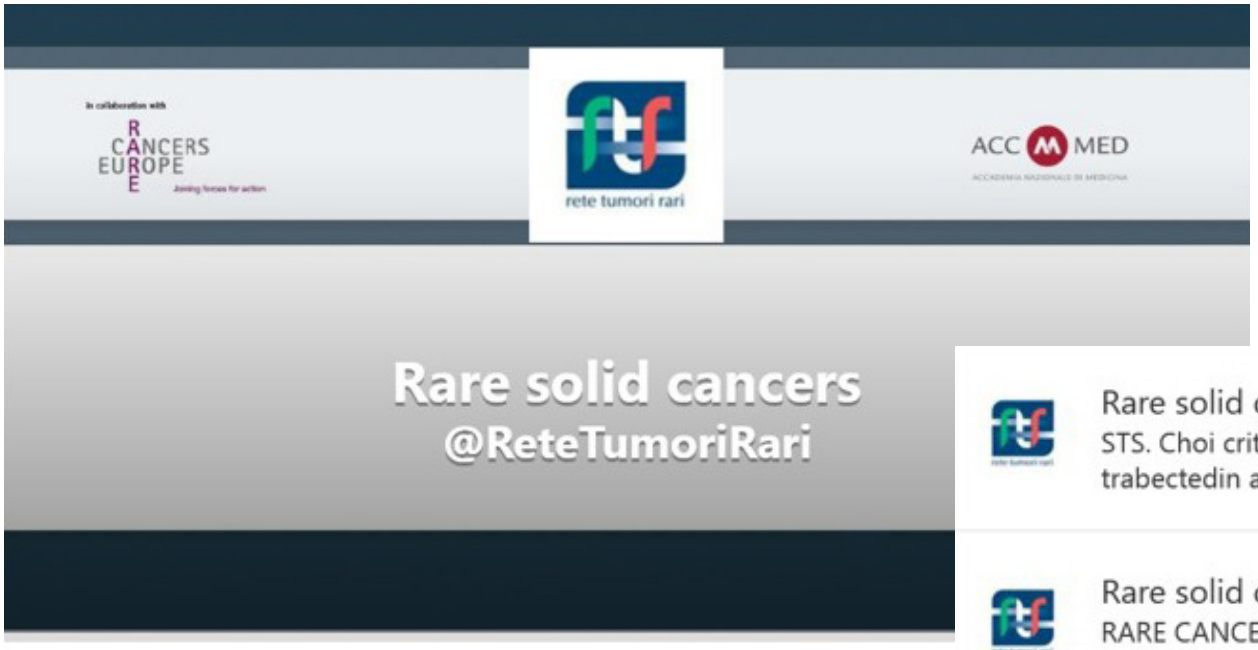
Regulators

Josh Sommer

@sommerjo

Today at EMA regulators, clinicians, @ChordomaFDN agreed to work together on guidance for chordoma trials. Progress! pic.twitter.com/ShR3SA8KXZ

30/04/2015 19:47:22



Tweets on new published papers on rare adult solid ca
for health professionals by Italian Rare Cancer Network
(see names on website)

Italy · **retetumorirari.accmed.org**

Rare solid cancers @ReteTumoriRari
STS. Choi criteria not only in GIST: they may help with trabectedin as well: pubmed.gov/25499439

Rare solid cancers @ReteTumoriRari
RARE CANCERS. A European consensus attempt to advance methodology of clinical studies: pubmed.gov/25274616

Rare solid cancers @ReteTumoriRari
STS. Continuous infusion HD-Ifosfamide is active in WD/DD-liposarcoma, even in some pts who received AI: pubmed.gov/25628856

Rare solid cancers @ReteTumoriRari
GIST. Further evidence for surgery of advanced responding disease, but again retrospective, thus only suggestive: pubmed.gov/25608769

Rare solid cancers @ReteTumoriRari
CHORDOMA. An effort by the whole disease community to build a global consensus on an ultrarare cancer: ncbi.nlm.nih.gov/pubmed/25638683

RARE CANCERS EUROPE

Rare Cancers Consensus Meeting: Pathology in Rare Cancers

10 – 11 February 2014, Brussels

- **Referral** to expert rare cancer pathologists is crucial for appropriateness
- **Networks** are the best tool for proper referral
- **Multidisciplinarity** is the best environment for rare cancer patient healthcare

RARE CANCERS EUROPE

Rare Cancers Consensus Meeting: Pathology in Rare Cancers

10 – 11 February 2014, Brussels

Conference Objective: Consensus Statement on Improving Pathological Diagnosis of Rare Cancers

Recommendations stemming from this consensus statement will be crucial in making sure that the Cross-Border Healthcare Directive has the right impact on the lives of patients suffering from rare cancers, through effective use of European Reference Networks.

17.

Call for increased integration of local, national and European centres of expertise into European **reference networks**, based on specific criteria as set out in the Commission's proposed Directive on the application of patients' rights in cross-border healthcare , in order to provide the necessary sound organisational structures for more efficient clinical research and early transfer of research data into clinical practice, thus improving the clinical management of rare cancers.

DIRECTIVES

DIRECTIVE 2011/24/EU OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL

of 9 March 2011

on the application of patients' rights in cross-border healthcare

European Reference Networks

“will provide

highly specialised healthcare

for rare or low prevalence complex diseases or conditions”

European Reference Networks

- 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**

Available at www.sciencedirect.com

SciVerse ScienceDirect

journal homepage: www.ejconline.com

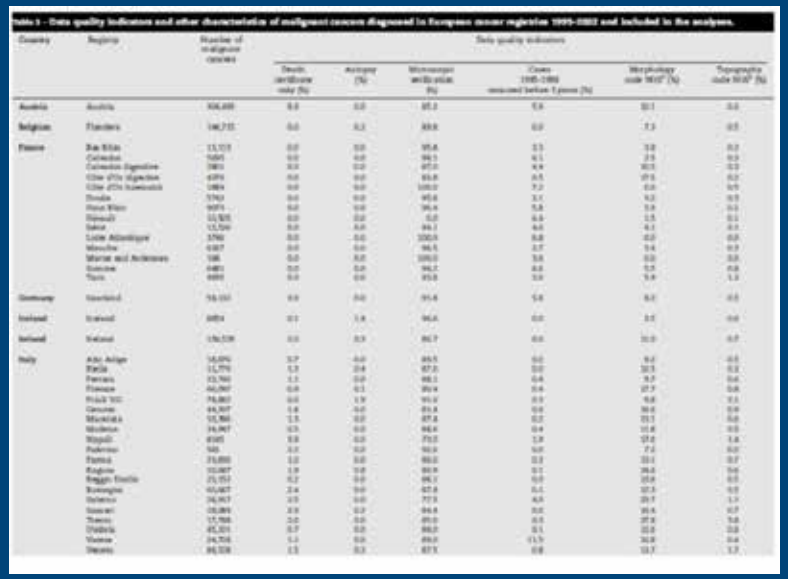
EJC

ELSEVIER

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

Eur J Cancer 2011;47:2493



RARE CANCERS

1. Pediatric cancers
2. Haematologic rare neoplasms
3. Sarcomas
4. Rare thoracic cancers
5. Neuroendocrine tumours
6. Head & neck cancers
7. Central nervous system tumours
8. Rare female genital cancers
9. Rare urological and male genital tumours
10. Endocrine gland tumours
11. Digestive rare cancers
12. Rare skin cancers & non-cutaneous melanoma

The ESMO/European Sarcoma Network Working Group*

- Paolo G. Casali, Italy (*Moderator*)
- Jean-Yves Blay, France (*Moderator*)
- Alexia Bertuzzi, Ireland
- Stefan Bielack, Germany
- Bodil Bjerkehagen, Norway
- Sylvie Bonvalot, France
- Ioannis Boukovinas, Greece
- Paolo Bruzzi, Italy
- Angelo Paolo Dei Tos, Italy
- Palma Dileo, UK
- Mikael Eriksson, Sweden
- Alexander Fedenko, Russian Federation
- Andrea Ferrari, Italy
- Stefano Ferrari, Italy
- Hans Gelderblom, Belgium
- Robert Grimer, UK
- Alessandro Gronchi, Italy
- Rick Haas, Netherlands
- Kirsten Sundby Hall, Norway
- Peter Hohenberger, Germany
- Rolf Issels, Germany
- Heikki Joensuu, Finland
- Ian Judson, UK
- Axel Le Cesne, France
- Saskia Litière, Belgium
- Javier Martin-Broto, Spain
- Ofer Merimsky, Israel
- Michael Montemurro, UK
- Carlo Morosi, Italy
- Piero Picci, Italy
- Isabelle Ray-Coquard, France
- Peter Reichardt, Germany
- Piotr Rutkowski, Poland
- Marcus Schlemmer, Germany
- Silvia Stacchiotti, Italy
- Valter Torri, Italy
- Annalisa Trama, Italy
- Frits Van Coevorden, Netherlands
- Winette Van der Graaf, Netherlands
- Daniel Vanel, Italy
- Eva Wardelmann, Germany

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

Gastrointestinal stromal tumours: ESMO Clinical Practice Guidelines for diagnosis, treatment and follow-up†

Bone sarcomas: ESMO Clinical Practice Guidelines for diagnosis, treatment and follow-up†

Ann Oncol. 2014 Sep;25 Suppl 3

http://www.esmo.org


ESMO


EORTC
The future of cancer therapy

Joint Action on Rare Cancers...

RARE CANCERS EUROPE

Joining forces for action

paolo.casali@istitutotumori.mi.it

@casali_pg