RARE CANCERS EUROPE

Joining forces for action

CPG
in rare cancers

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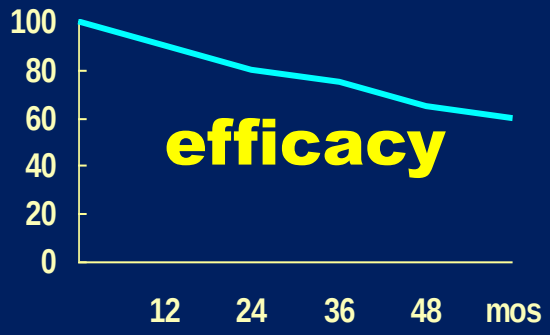
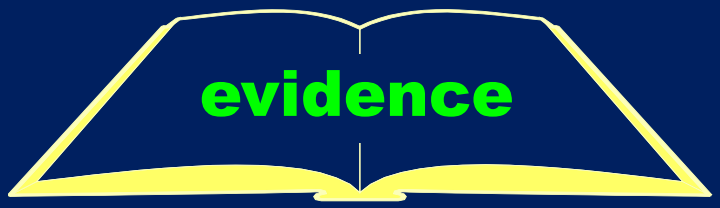
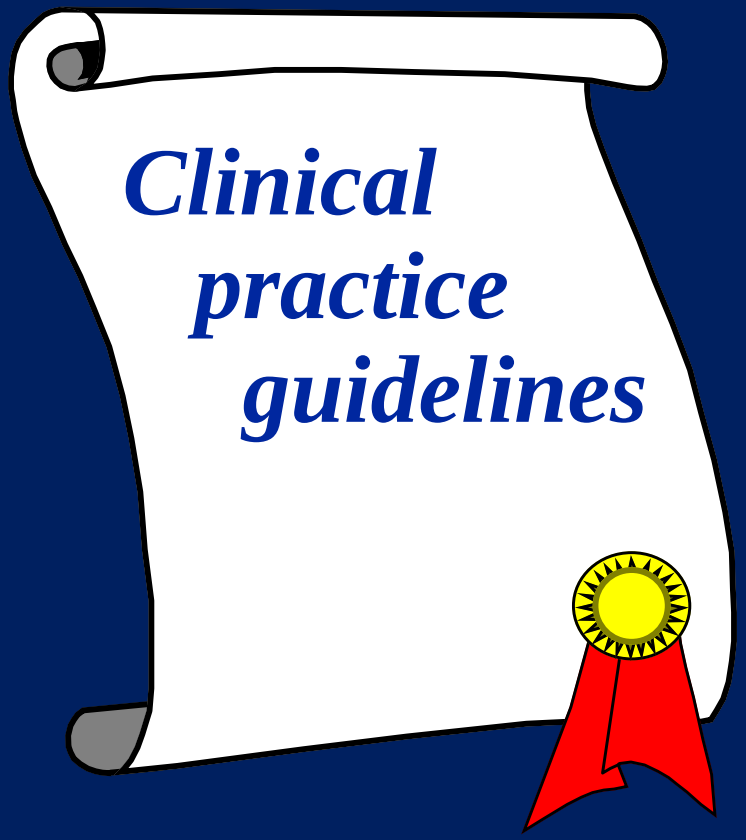
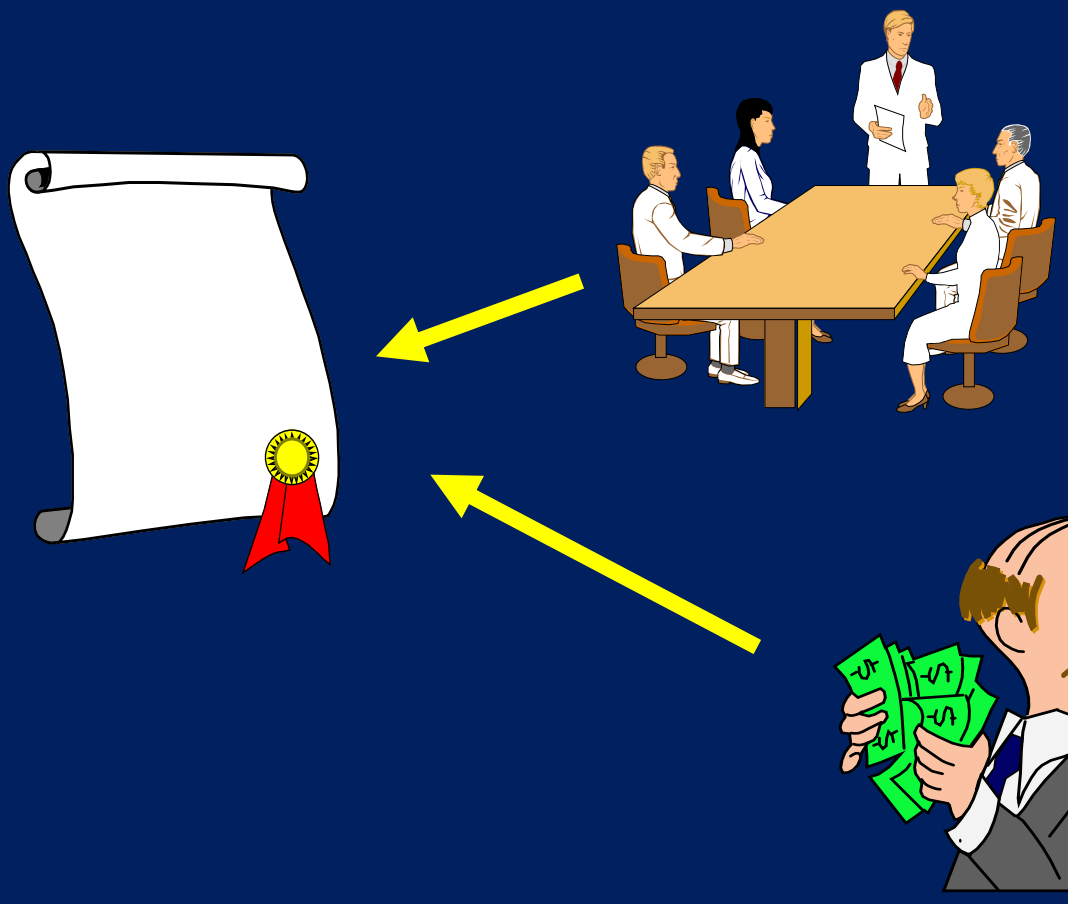




decision-making
performance
willingness to pay


Clinical
practice
guidelines

Clinical practice guidelines

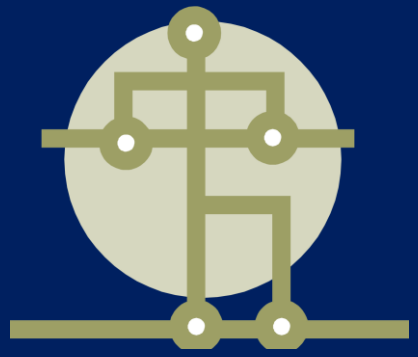


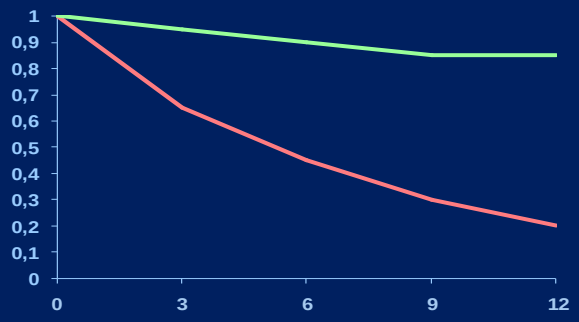
evidence-based consensus

willingness to pay

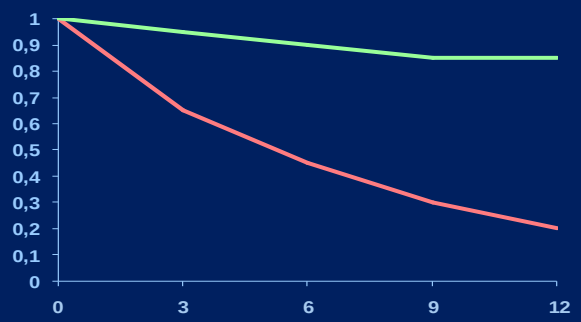
Inequalities in cancer care

EU regulator





National regulator



clinical practice guidelines

Annals of Oncology 23 (Supplement 7): vii83–vii85, 2012
doi:10.1093/annonc/mds266

Nasopharyngeal cancer: EHNS–ESMO–ESTRO Clinical Practice Guidelines for diagnosis, treatment and follow-up†

A.T.C. Chan[1], V. Grégoire[2], J.-L. Lefebvre[3], L. Licitra[4], E.P. Hui[1], S.F. Leung[1] & E. Felip[5], on behalf of the EHNS–ESMO–ESTRO Guidelines Working Group*

[1]*Department of Clinical Oncology, State Key Laboratory in Oncology in South China, Sir YK Pao Centre for Cancer, The Chinese University of Hong Kong, Hong Kong;* [2]*Department of Radiation Oncology, St-Luc University Hospital, Brussels, Belgium;* [3]*Department of Head and Neck Surgery, Centre Oscar Lambret, Lille, France;* [4]*Medical Oncology Head and Neck Unit, Istituto Nazionale dei Tumori, Milan, Italy;* [5]*Medical Oncology Service, Vall d'Hebron University Hospital, Barcelona, Spain*

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JOURNAL OF CLINICAL ONCOLOGY ASCO SPECIAL ARTICLE

American Society of Clinical Oncology Clinical Practice Guideline for the Use of Larynx-Preservation Strategies in the Treatment of Laryngeal Cancer

David G. Pfister, Scott A. Laurie, Gregory S. Weinstein, William M. Mendenhall, David J. Adelstein, K. Kian Ang, Gary L. Clayman, Susan G. Fisher, Arlene A. Forastiere, Louis B. Harrison, Jean-Louis Lefebvre, Nancy Leupold, Marcy A. List, Bernard O. O'Malley, Snehal Patel, Marshall R. Posner, Michael A. Schwartz, and Gregory T. Wolf

Type of Cancer	Organ-Preservation Strategy: Recommended	Organ-Preservation Strategy: Other Options	Basis for Recommendation	Quality of Evidence
T1 cancer of the glottis: T1—tumor limited to the vocal cord(s) (may involve anterior or posterior commissure) with normal mobility T1a—tumor limited to one vocal cord T1b—tumor involves both vocal cords	Endoscopic resection (selected patients) OR radiation therapy	Open organ-preservation surgery	High local control rates and quality of voice after endoscopic resection compared with radiation therapy; possible cost savings; ability to reserve radiation for possible second primary cancers of the upper aerodigestive tract; however, not suitable for all patients	Comparison of outcomes from case series/ prospective single-arm studies
T2 cancer of the glottis, favorable*:T2—tumor extends to supraglottis and/or subglottis, or with impaired vocal cord mobility	Open organ-preservation surgery OR radiation therapy	Endoscopic resection (selected patients)	Open organ-preservation surgery is associated with highest local control rates; however, leads to permanent hoarseness; local control rates after radiation therapy are also high, and functional outcomes may be better	Comparison of outcomes from case series/ prospective single-arm studies
T2 cancer of the glottis, unfavorable*	Open organ-preservation surgery OR concurrent chemoradiation therapy (selected patients with node-positive disease)	Radiation therapy Endoscopic resection (selected patients)	Higher local control rates after surgery compared with radiation therapy alone; quality of voice after therapy of less concern if vocal cord function is irreversibly compromised by tumor invasion; endoscopic surgery requires careful patient selection For patients with T2 N+ disease, evidence from randomized trials supports concurrent chemoradiation therapy as an organ-preservation option	Comparison of outcomes from case series/ prospective single-arm studies; randomized controlled clinical trials comparing concurrent chemoradiation therapy, and/or induction chemotherapy followed by radiation, and/or radiation therapy alone, and/or surgery followed by radiation
T1-T2 cancer of the supraglottis, favorable*:T1—tumor limited to one subsite of supraglottis with normal vocal cord mobility T2—tumor invades mucosa of more than one adjacent subsite of supraglottis or glottis or region outside the supraglottis (eg, mucosa of base of tongue, vallecula, medial wall of pyriform sinus) without fixation of the larynx	Open organ-preservation surgery OR radiation therapy	Endoscopic resection (selected patients)	Open organ-preservation surgery associated with highest local control rates; however, requires temporary tracheostomy and may lead to increased risk of aspiration after therapy; local control rates after radiation therapy are also high, and functional outcomes may be better	Comparison of outcomes from case series/ prospective single-arm studies
T2 cancer of the supraglottis, unfavorable*	Open organ-preservation surgery OR concurrent chemoradiation therapy (selected patients with node-positive disease)	Radiation therapy Endoscopic resection (selected patients)	Open organ-preservation surgery is more likely to yield higher local control rates than radiation therapy; for patients with T2 N+ disease, evidence from randomized trials supports concurrent chemoradiation therapy as an organ-preservation option	Comparison of outcomes from case series/ prospective single-arm studies; randomized controlled clinical trials comparing concurrent chemoradiation therapy, and/or induction chemo-therapy followed by radiation, and/or radiation therapy alone, and/or surgery followed by radiation
T3-T4 cancers of the glottis or supraglottis: T3 glottis—tumor limited to the larynx with vocal cord fixation, and/or invades paraglottic space, and/or minor thyroid cartilage erosion (eg, inner cortex) T3 supraglottis—tumor limited to larynx with vocal cord fixation and/or invades any of the following: postcricoid area, pre-epiglottic tissues, paraglottic space, and/or minor thyroid cartilage erosion (eg, inner cortex) T4a glottis or supraglottis—tumor invades through the thyroid cartilage and/or invades tissues beyond the larynx (eg, trachea, soft tissues of neck including deep extrinsic muscle of the tongue, strap muscles, thyroid, or esophagus) T4b glottis or supraglottis—tumor invades prevertebral space, encases carotid artery, or invades mediastinal structures	Concurrent chemoradiation therapy OR open organ-preservation surgery (in highly selected patients)	Radiation therapy	Highest rate of larynx preservation is associated with concurrent chemoradiation therapy compared with other radiation-based approaches, at the cost of higher acute toxicities but without more long-term difficulties in speech and swallowing; when salvage total laryngectomy incorporated, no difference in overall survival; organ preservation surgery is an option in highly selected patietns (eg, there are patients with T3 supraglottic cancers that have minimal or moderate pre-epiglottic invasion and are candidates for organ preserving surgery)	Randomized controlled clinical trials comparing concurrent chemoradia-tion therapy, and/or induction chemotherapy followed by radiation, and/or radiation therapy alone; and/or surgery followed by radiation; comparison of outcomes from case series/prospective single-arm studies

*A favorable T2 glottic lesion is defined as a superficial tumor, on radiographic imaging, with normal cord mobility. An unfavorable T2 glottic lesion is defined as a deeply invasive tumor on radiographic imaging, with or without subglottic extension, with impaired cord mobility (indicating deeper invasion). A favorable supraglottic lesion is defined as a T1 or T2 tumor with superficial invasion on radiographic imaging and preserved cord mobility, and/or tumor of the aryepiglottic fold with minimal involvement of the medial wall of the pyriform sinus. More locally advanced and invasive T2 suproglottic lesions are considered unfavorable.

European Journal of Cancer (2012) 48, 1392–1400

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Cancer guideline development in Europe: A survey among ECCO members

Dirk Schrijvers [a,*], Marco Rosselli Del Turco [b], Carol Maddock [c], Lorenza Marotti [b], Françoise Van Hemelryck [a]

19. Is there methodological training for members of the guideline development group before starting with the guideline development?	☐ Yes, obligatory ☐ Yes, optional ☐ No
20. Method used to collect evidence *(more than one answer possible)*	☐ Hand searches of published literature (primary and/or secondary sources) ☐ Searches of electronic databases ☐ Searches of patient registry data ☐ Searches on unpublished data
21. Methods used to analyse evidence *(more than one answer possible)*	☐ Decision analysis ☐ Meta-analysis ☐ Systematic review ☐ Non-systematic review ☐ Experience based
22. Methods used to formulate recommendations *(more than one answer possible)*	☐ Subjective review ☐ Informal expert consensus ☐ Formal expert consensus (consensus conferences, nominal group technique or Delphi technique) ☐ Evidence-linked (weighting according to a rating scheme)
23. Method of review *(more than one answer possible)*	☐ Clinical validation - pilot testing ☐ Clinical validation - trial implementation period ☐ Comparison with guidelines from other groups ☐ External peer review ☐ Internal peer review
24. Is there a process of guideline authorization?	☐ Yes, formal authorization by endorsement by professional organization of the target users ☐ Yes, authorization otherwise, please specify ..... ☐ No

Quality of evidence…


RARE
CANCERS
EUROPE


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


EURORDIS
Rare Diseases Europe


EUROPEAN
CANCER
PATIENT
COALITION


EORTC
European Organisation for Research and Treatment of Cancer


Conticanet


EuroBoNeT


ECL
Association of European Cancer Leagues


cmlsupport
chronic myeloid leukaemia support group


IBTA
INTERNATIONAL
BRAIN TUMOUR
ALLIANCE


orphanet


FONDAZIONE IRCCS
ISTITUTO NAZIONALE
DEI TUMORI


CML AdvocatesNetwork


IEO
European Institute of Oncology


SIOPE
SIOP Europe


ESO
European
School
of
Oncology
Learning to care


EUROPEAN ONCOLOGY
NURSING SOCIETY


European
Society of
Pathology


NOVARTIS
ONCOLOGY


Pfizer
Oncology


sanofi aventis
Because health matters

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.

U.S. Department of Health & Human Services

U.S. Food and Drug Administration

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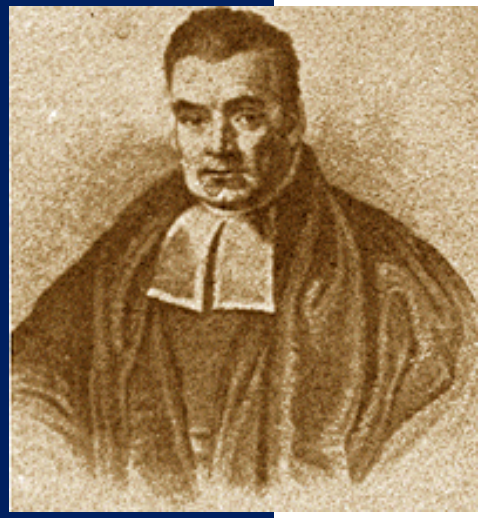
EUROPEAN MEDICINES AGENCY

SCIENCE MEDICINES HEALTH


RARE CANCERS EUROPE
Joining forces for action
Rare cancers – more common than most people think
clinical decision-making
methods to combine evidence
new study designs
surrogate end points
organization of studies
Eurocan+Platform for cross border cancer research launched
European Society for Medical Oncology
Sign the Call to Action Against Rare Cancers!



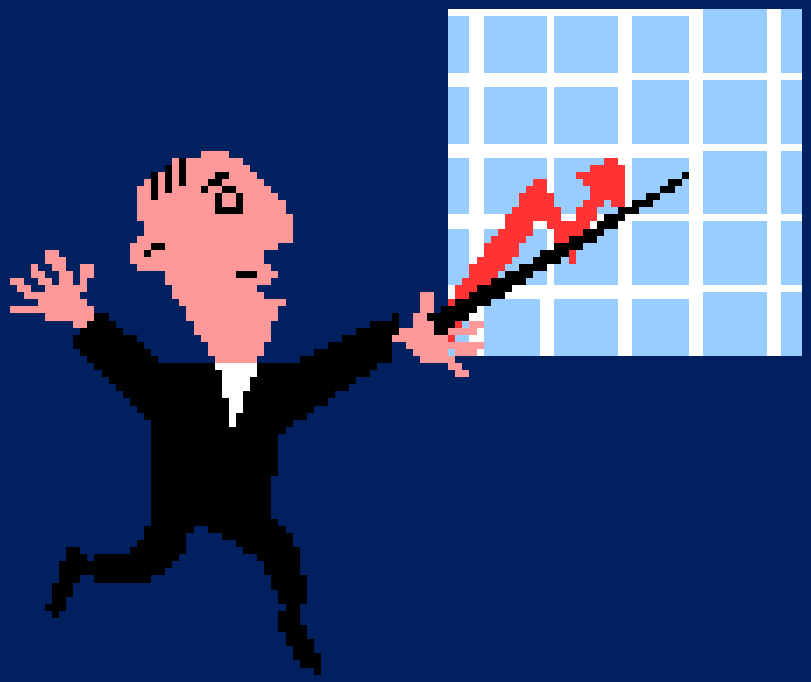
PHILOSOPHICAL TRANSACTIONS:

LII. *An Eſſay towards ſolving a Problem in the Doctrine of Chances. By the late Rev. Mr.* Bayes, *F. R. S. communicated by Mr.* Price, *in a Letter to* John Canton, *A. M. F. R. S.*

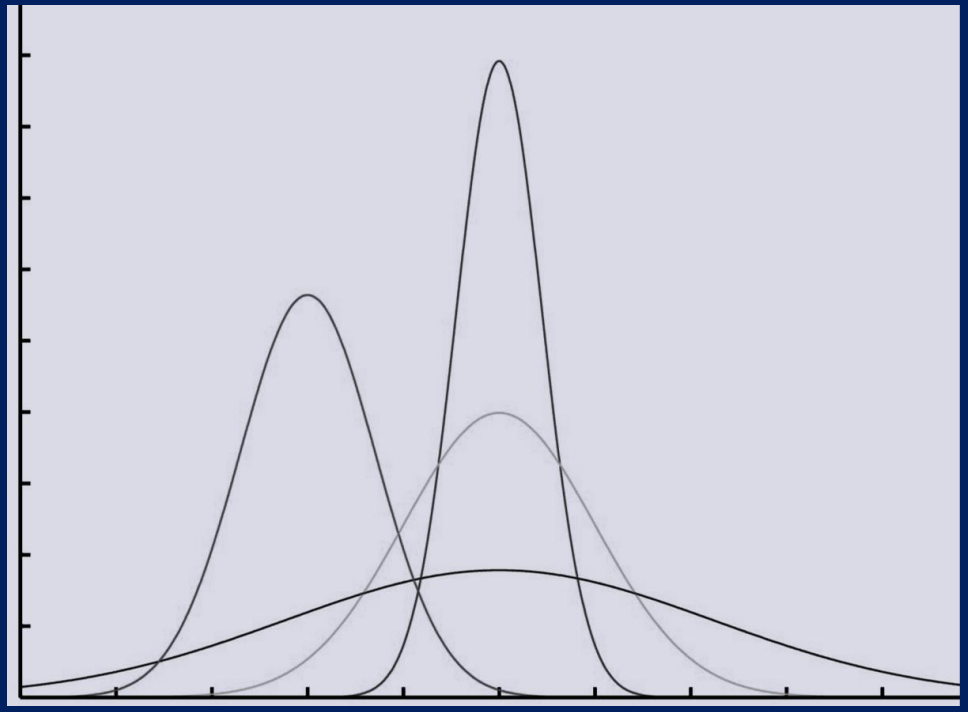
Dear Sir,

Read Dec. 23, 1763. I Now ſend you an eſſay which I have found among the papers of our deceaſed friend Mr. Bayes, and which, in my opinion, has great merit, and well deſerves to be preſerved. Experimental philoſophy, you will find, is nearly intereſted in the ſubject of it; and on this account there ſeems to be particular reaſon for thinking that a communication of it to the Royal Society cannot be improper.

Mr. Bayes & Mr. Price. Phil Trans 1763;53:370



Knowledge from all evidence…



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

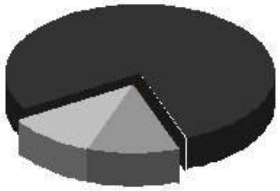
Gemma Gatta [a,*], *Jan Maarten van der Zwan* [b], *Paolo G. Casali* [c], *Sabine Siesling* [b], *Angelo Paolo Dei Tos* [d], *Ian Kunkler* [e], *Renée Otter* [b], *Lisa Licitra* [f], *Sandra Mallone* [g], *Andrea Tavilla* [g], *Annalisa Trama* [a], *Riccardo Capocaccia* [g], *The RARECARE working group*

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

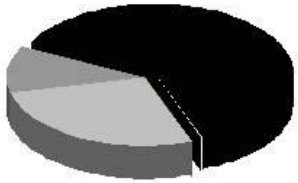
Country	Registry	Number of malignant cancers	Data quality indicators					
			Death certificate only (%)	Autopsy (%)	Microscopic verification (%)	Cases 1995-1998 censored before 5 years (%)	Morphology code NOS[a] (%)	Topography code NOS[a] (%)
Austria	Austria	304,695	8.9	0.0	85.2	5.9	10.1	0.6
Belgium	Flanders	144,715	0.0	0.2	89.8	0.0	7.3	0.5
France	Bas Rhin	13,113	0.0	0.0	95.8	3.3	3.9	0.2
	Calvados	5895	0.0	0.0	98.1	6.1	2.5	0.3
	Calvados digestive	2801	0.0	0.0	87.0	4.4	10.5	0.3
	Côte d'Or digestive	4376	0.0	0.0	82.8	0.5	17.5	0.2
	Côte d'Or haematol.	1884	0.0	0.0	100.0	7.2	0.0	0.5
	Doubs	5742	0.0	0.0	95.8	2.1	3.2	0.3
	Haut Rhin	9073	0.0	0.0	96.4	5.8	2.9	0.1
	Hérault	10,505	0.0	0.0	0.0	6.4	1.5	0.1
	Isère	12,526	0.0	0.0	94.1	4.6	4.1	0.1
	Loire Atlantique	3766	0.0	0.0	100.0	6.8	0.0	0.0
	Manche	6367	0.0	0.0	96.5	2.7	3.4	0.3
	Marne and Ardennes	168	0.0	0.0	100.0	3.6	0.0	0.0
	Somme	6481	0.0	0.0	94.2	6.6	5.5	0.8
	Tarn	4935	0.0	0.0	93.8	2.0	5.9	1.3
Germany	Saarland	54,132	3.9	0.0	91.8	5.8	8.0	0.5
Iceland	Iceland	8854	0.1	1.4	96.6	0.0	3.5	0.0
Ireland	Ireland	156,529	2.0	0.3	86.7	0.0	11.0	0.7
Italy	Alto Adige	18,676	0.7	0.0	89.5	0.0	9.2	0.5
	Biella	11,770	1.3	0.4	87.0	0.0	12.5	0.3
	Ferrara	23,760	1.1	0.0	88.1	0.4	9.7	0.6
	Firenze	66,087	0.9	0.1	80.4	0.4	17.7	0.8
	Friuli VG	78,882	0.6	1.9	91.0	0.3	9.8	2.1
	Genova	44,307	1.8	0.0	81.4	0.0	16.6	0.9
	Macerata	10,386	1.3	0.0	87.4	0.2	13.1	0.6
	Modena	34,967	0.5	0.0	88.6	0.4	11.8	0.5
	Napoli	8165	3.9	0.0	73.0	1.9	17.6	1.4
	Palermo	581	2.2	0.0	92.6	0.0	7.2	0.0
	Parma	23,836	1.0	0.0	86.0	0.3	13.1	0.7
	Ragusa	10,687	1.9	0.8	80.9	0.1	26.6	0.6
	Reggio Emilia	22,152	0.2	0.0	88.1	0.0	13.8	0.5
	Romagna	60,667	2.4	0.0	87.9	0.1	12.3	0.5
	Salerno	26,917	2.5	0.0	77.5	4.0	23.7	1.1
	Sassari	18,084	2.9	0.2	84.4	0.0	16.4	0.7
	Trento	17,788	2.0	0.0	85.0	0.3	27.8	3.8
	Umbria	45,221	0.7	0.0	84.0	0.1	12.6	0.6
	Varese	24,758	1.1	0.0	89.0	11.5	10.8	0.4
	Veneto	84,528	1.5	0.2	87.5	0.8	13.7	1.7

Eur J Cancer 2011;47:2493

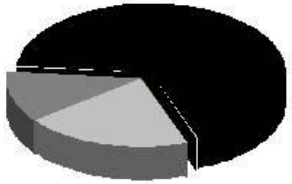
Incidence <6/100,000/y



Incidence <15/100,000/y



Prevalence <50/100,000



= *rare ("tier 1")*

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

= *frequent*

Casali PG et al, 2012, submitted

«Families» of rare cancers

- NON CUTANEOUS MELANOMA
- SKIN - Rare
- THORACIC - Rare
- UROGENITAL - Rare
- FEMALE GENITAL - Rare
- MALE GENITAL
- NEUROENDOCRINE
- ENDOCRINE ORGAN
- CNS
- SARCOMAS
- DIGESTIVE - Rare
- HEAD & NECK – Rare
- HEMATOLOGICAL - Rare
- PEDIATRIC

RARE CANCERS EUROPE

Joining forces for action

ESMO GOOD SCIENCE BETTER MEDICINE BEST PRACTICE

European Society for Medical Oncology

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