

Impact of Drug Benefit Assessment on Medical Oncology

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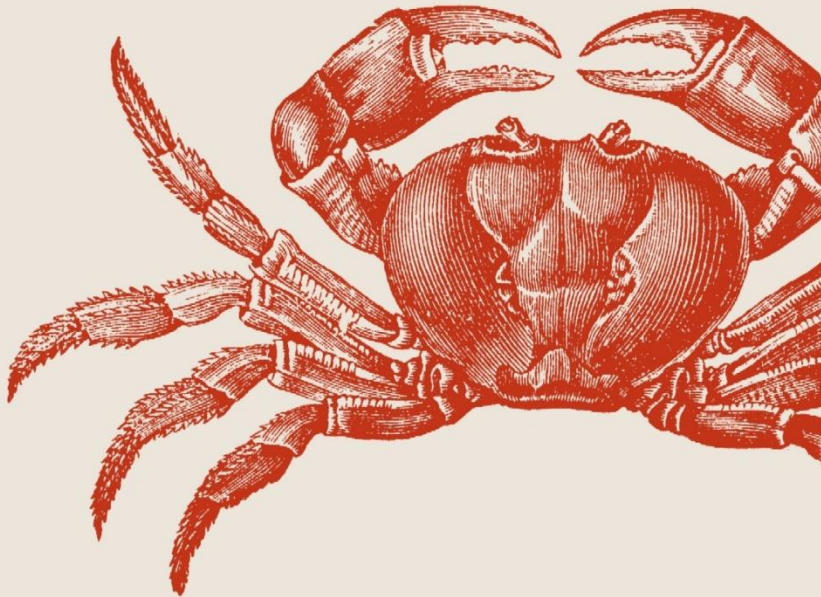
Madrid, February 13th, 2013



SIDDARTHA MUKHERJEE

EL EMPERADOR
DE TODOS LOS MALES

UNA BIOGRAFÍA DEL
CÁNCER



taurus
T

Oncology is

- most innovative discipline
- high level of evidence
- expensive
- emotional

Pharmaceutical Industry

Gemeinsamer
Nutzen für alle

IQWiG

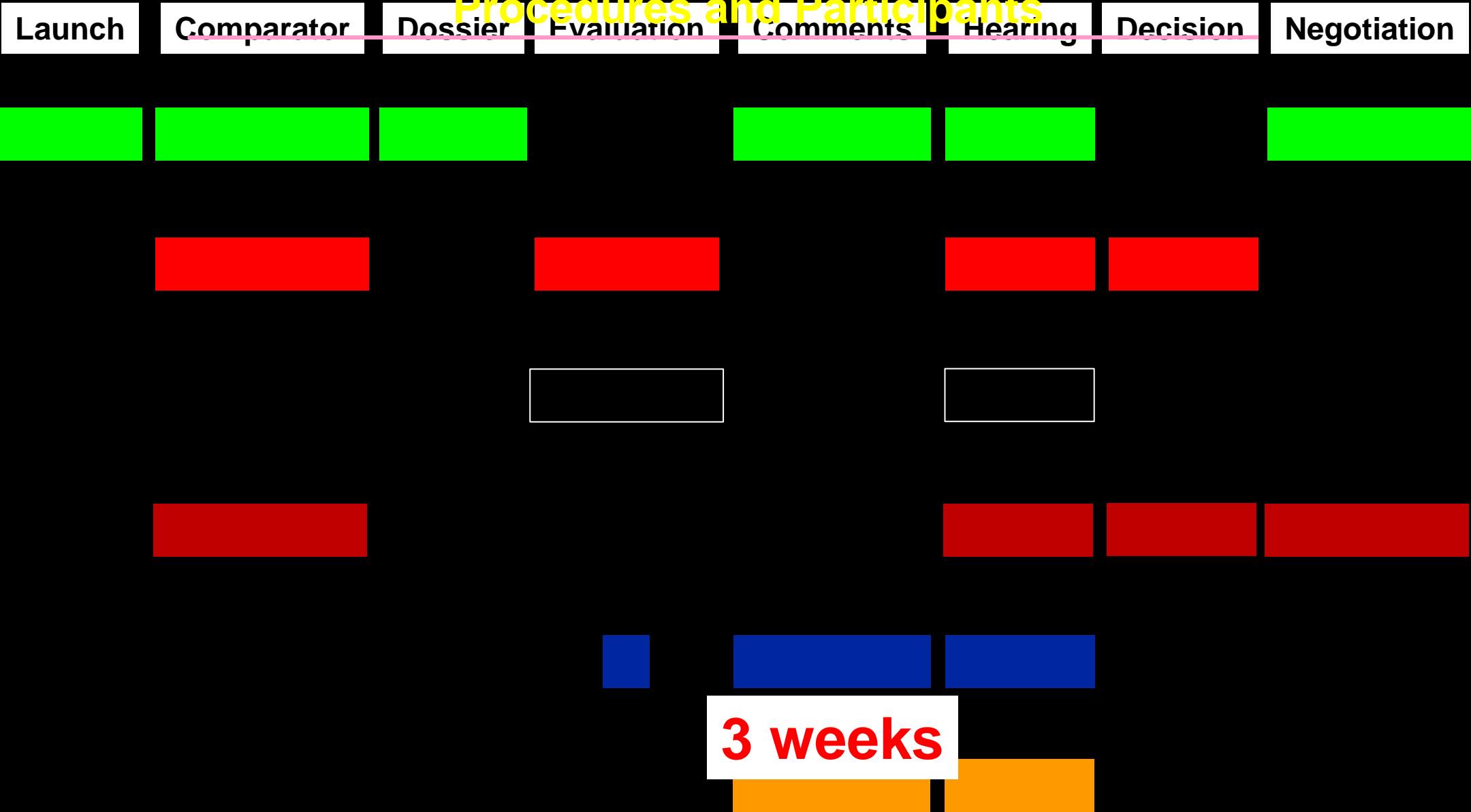
Insurance

Medical
Societies

Patients

Benefit of New Drugs in Oncology

Procedures and Participants



3 weeks

Substance	Drug	Indication	Cost / 4 weeks	Endpoint	pU	IQWiG	Discussion	G-BA
Abiraterone	Zytiga®	Prostate	5450*	OS	1	2 subgroup	• Comparator	2
Cabazitaxel	Jevtana®	Prostate	5444*	OS	1	2 subgroup	• Comparator (</≥ 65 yrs) • Adverse Events	3
Eribulin	Halaven®	Prostate	3463*	OS	2	none subgroup	• Comparator • Endpoint	3
Ipilimumab	Yervoy®	Melanoma	31341	OS	1	2	• Comparator • Adverse Events	2
Vemurafenib	Zelboraf®	Melanoma	6189*	OS	1	2	• Adverse Events	2
Vandetanib	Caprelsa®	Thyroid, medullary	6185*	PFS	1	none	• Patient selection	Resub- mission
Ruxolitinib	Jakafi®	MPS	4743*	Spleen size QoL	2	- (orphan)	• Adverse Events	pending
Axitinib	Inlyta®	Kidney (RCC)	5597*	PFS Side effects	2	2 subgroup	• Comparator • Endpoint	pending
Decitabine	Dacogen®	AML ≥65 yrs	7085*	PFS, OS QoL	2	- (orphan)	• Comparator	pending

Patient – related Outcome

Endpoints

- Mortality

 - Overall Survival

 - Cancer Specific Survival

- Morbidity

 - Progression-Free Survival

 - Remission Rate

 - Symptom Control

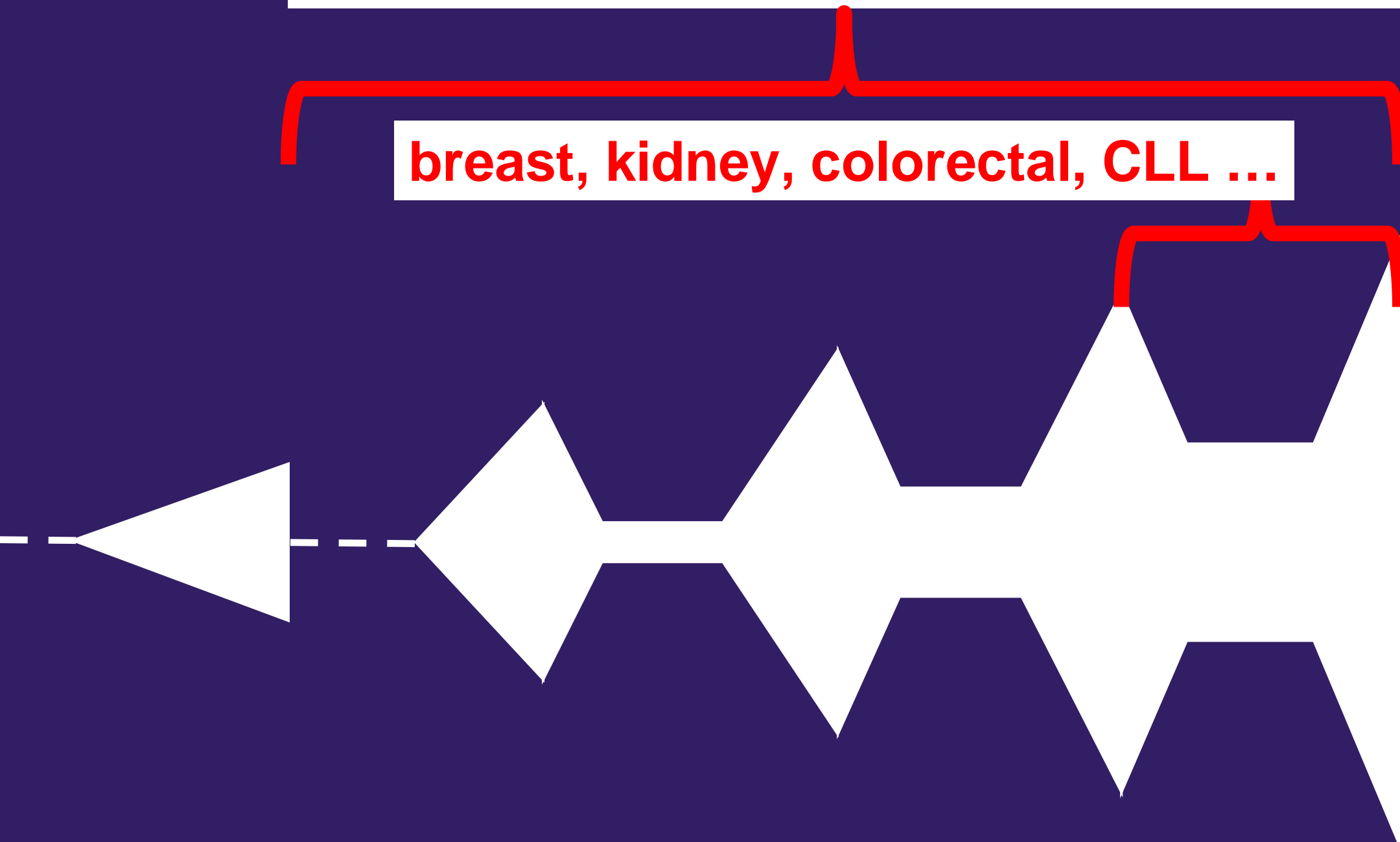
**Morbidity is Not a
Surrogate Parameter**

- Quality of Life

 - Adverse Events

OS: Overall Survival

breast, kidney, colorectal, CLL ...



Years

Overall Survival

Vandetanib in Medullary Thyroid Cancer

Placebo



Progressive Disease



**Overall
Survival?**

Ⓜ



Vandetanib

Overall Survival

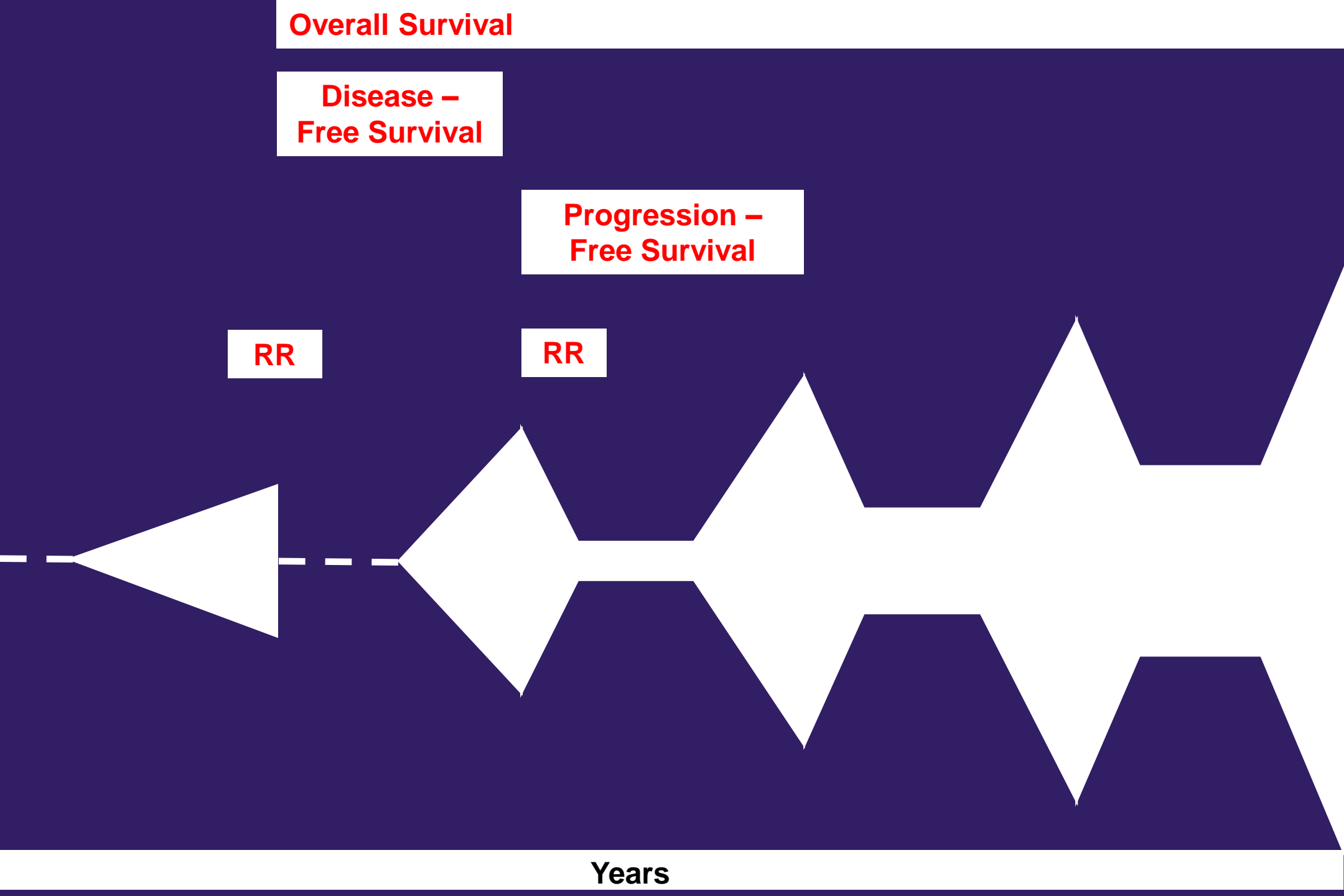
**Disease –
Free Survival**

**Progression –
Free Survival**

RR

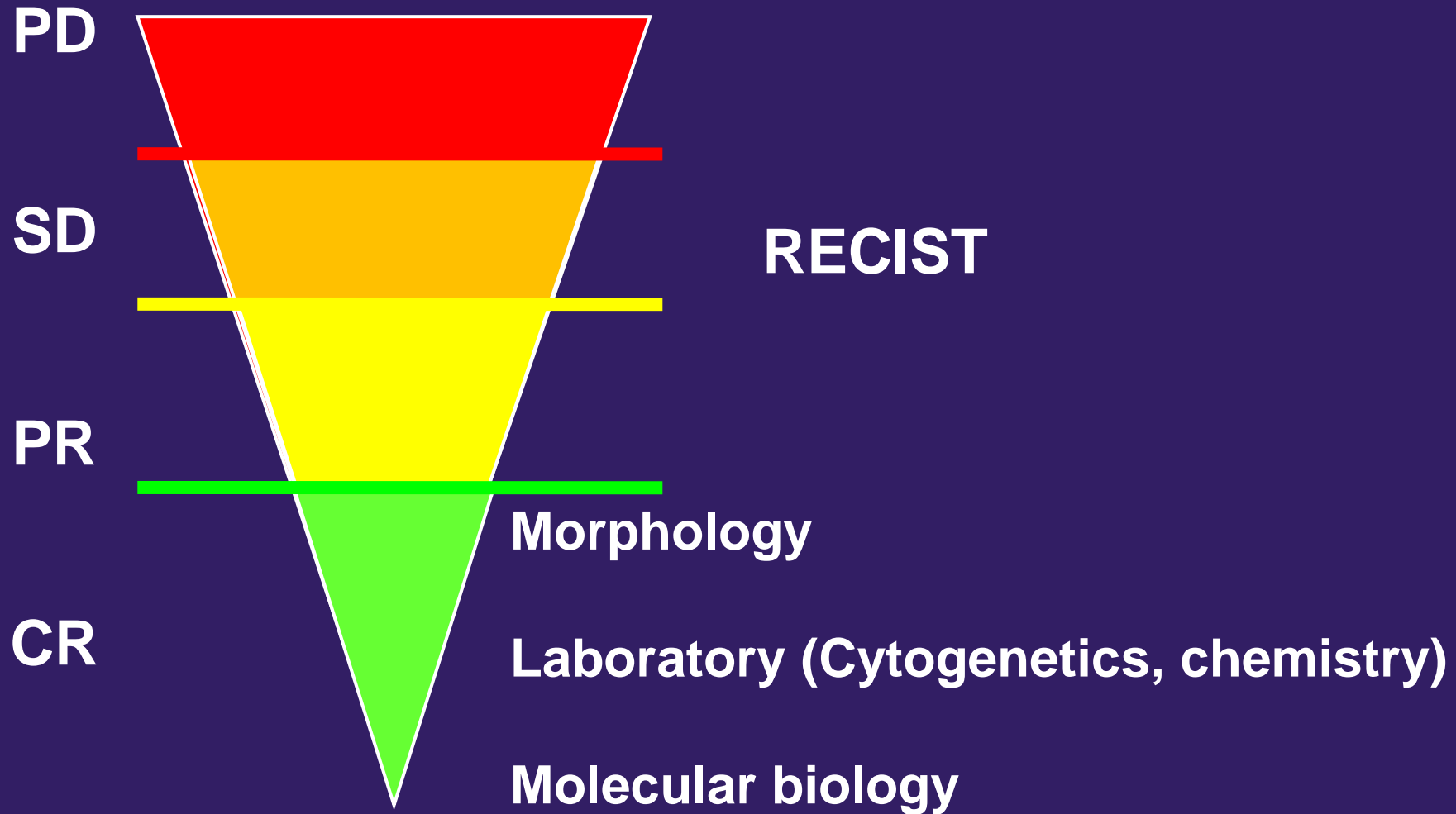
RR

Years



Patient – Related Outcome

Criteria of Disease Status



Patient – Related Outcome

Quality of Life

Time Period	n	EORTC OLQ	FACT FACIT	HADS	others
1993 – 2001	46	26 %	0	13 %	13 %
2001 – 2009	103	41 %	25 %	10 %	23 %

Benefit of New Drugs in Oncology

Adverse Events

Current Standard

CTCAE Scale

Classification

**Liver enzymes Grad 3 =
polyneuropathy Grad 3?**

Need

Clinical Grading

relevant

manageable

reversible

Benefit of New Drugs in Oncology

Comparator

New Drug

**Pivotal Trial
Control Arm**

**Other
Comparator**

Benefit of New Drugs in Oncology

Comparator

Axitinib

Sorafenib

**Medical
Societies**

Everolimus

**Gemeinsamer
Bundesausschuss**

Cancer



The NEW ENGLAND JOURNAL of MEDICINE

THE LANCET

Guidelines

ASCO



CLINICAL REVIEW ARTICLES at

medclinics.com



JNCI JOURNAL OF THE NATIONAL CANCER INSTITUTE

THE LANCET

Leading the hematology

JOURNAL OF

ONCOLOGY

Official Journal of the American Society of Clinical Oncology

British Journal of Haematology

Annals of Oncology

Benefit of New Drugs in Oncology

Comparator - Guidelines

AWMF

since 1995

Process

2-3 years

Update

every 5 years

ESMO

since 2001

heterogeneous

not adapted to national standard

ASCO

selected subjects

NCCN

no open access

not adapted to national standard

onkopedia guidelines

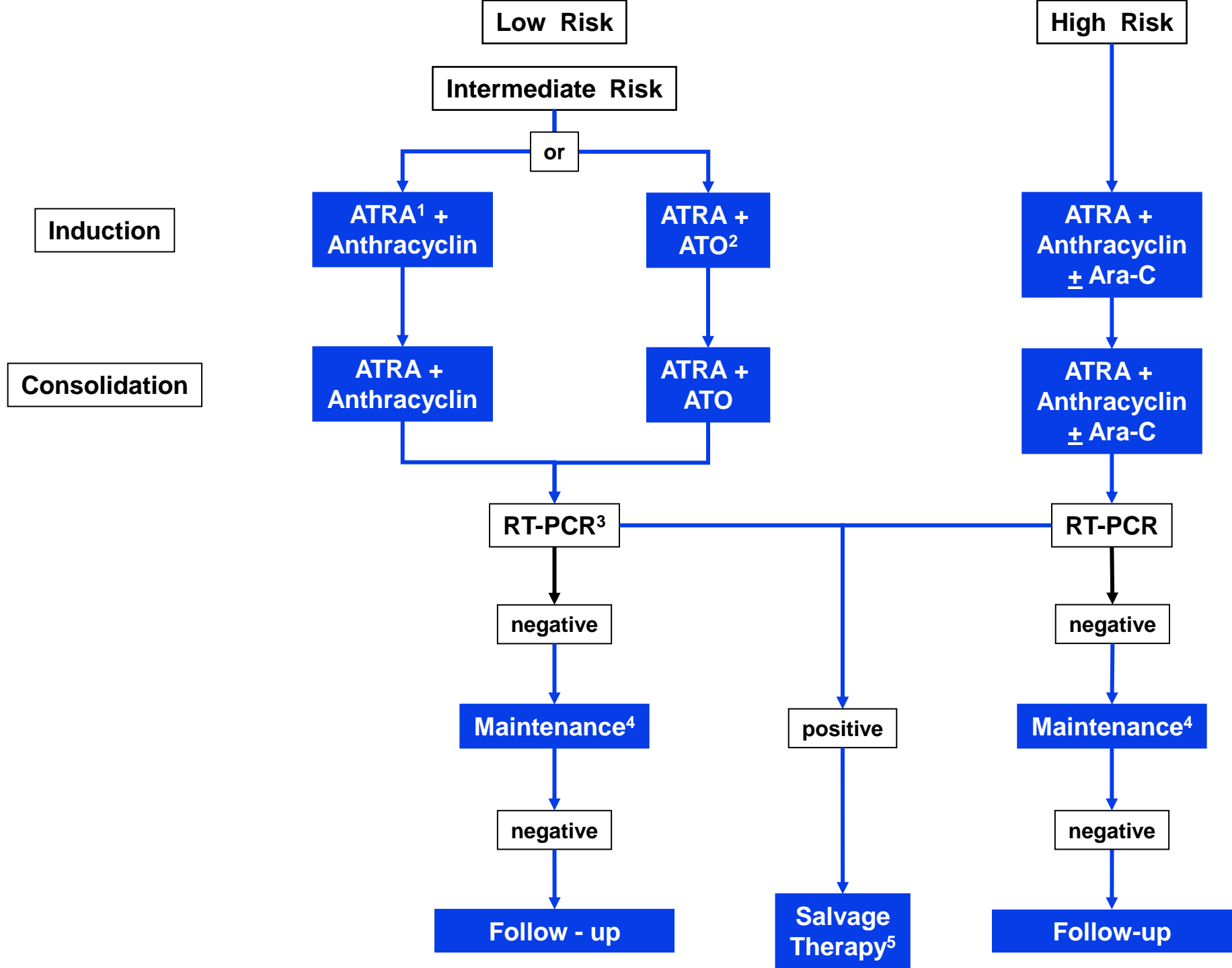
www.onkopedia-guidelines.info

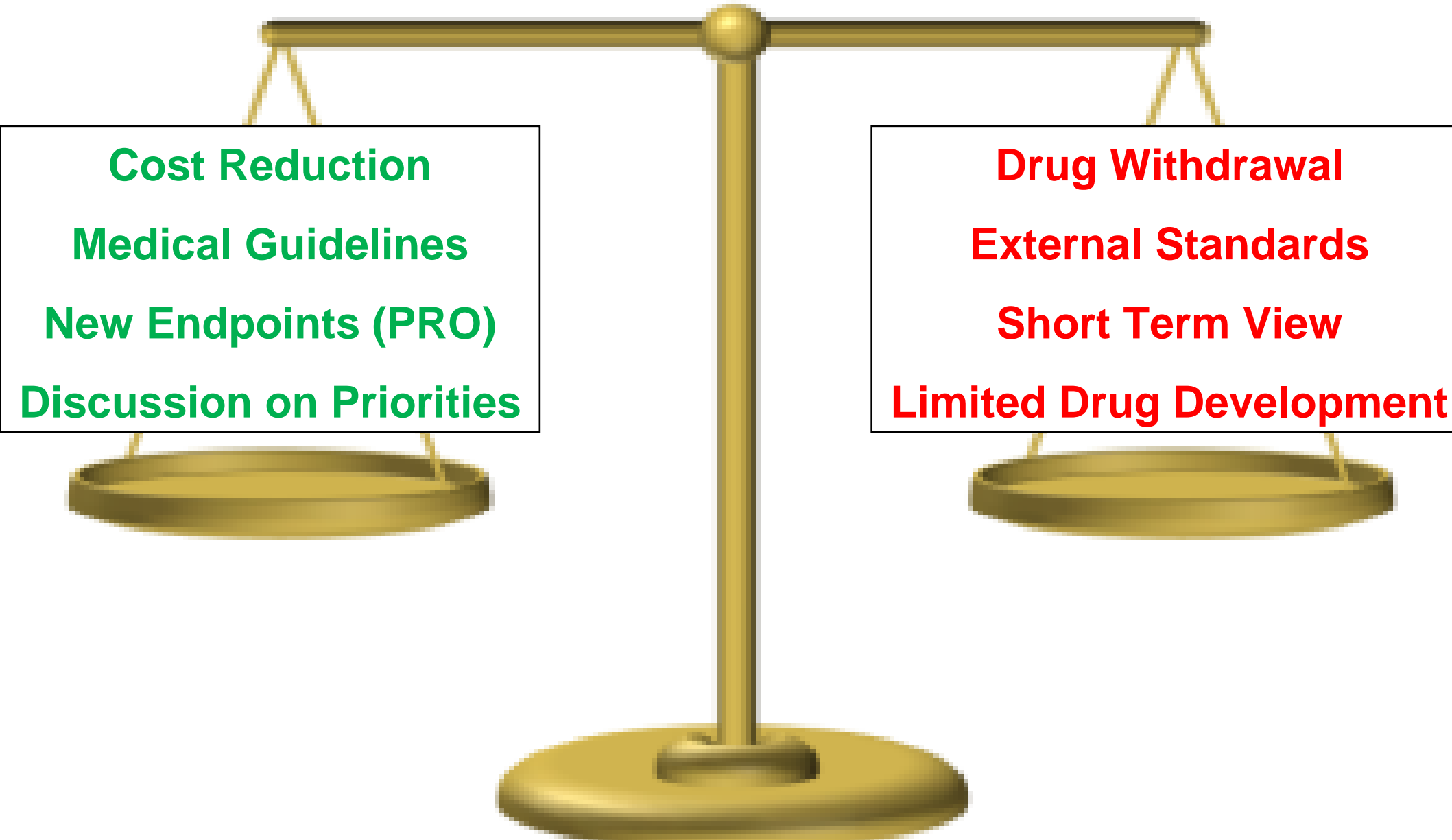


Benefit of New Drugs in Oncology

Guidelines

General Subjects	5
Non-malignant Hematological Disorders	10
Malignant Hematological Disorders	23
Solid Tumors	14
Supportive Care	10
Pediatric Oncology and Hematology	5





Cost Reduction
Medical Guidelines
New Endpoints (PRO)
Discussion on Priorities

Drug Withdrawal
External Standards
Short Term View
Limited Drug Development

