



**CENTRE DU CANCER
ET D'HEMATOLOGIE**

Cliniques universitaires SAINT-LUC | UCL Bruxelles

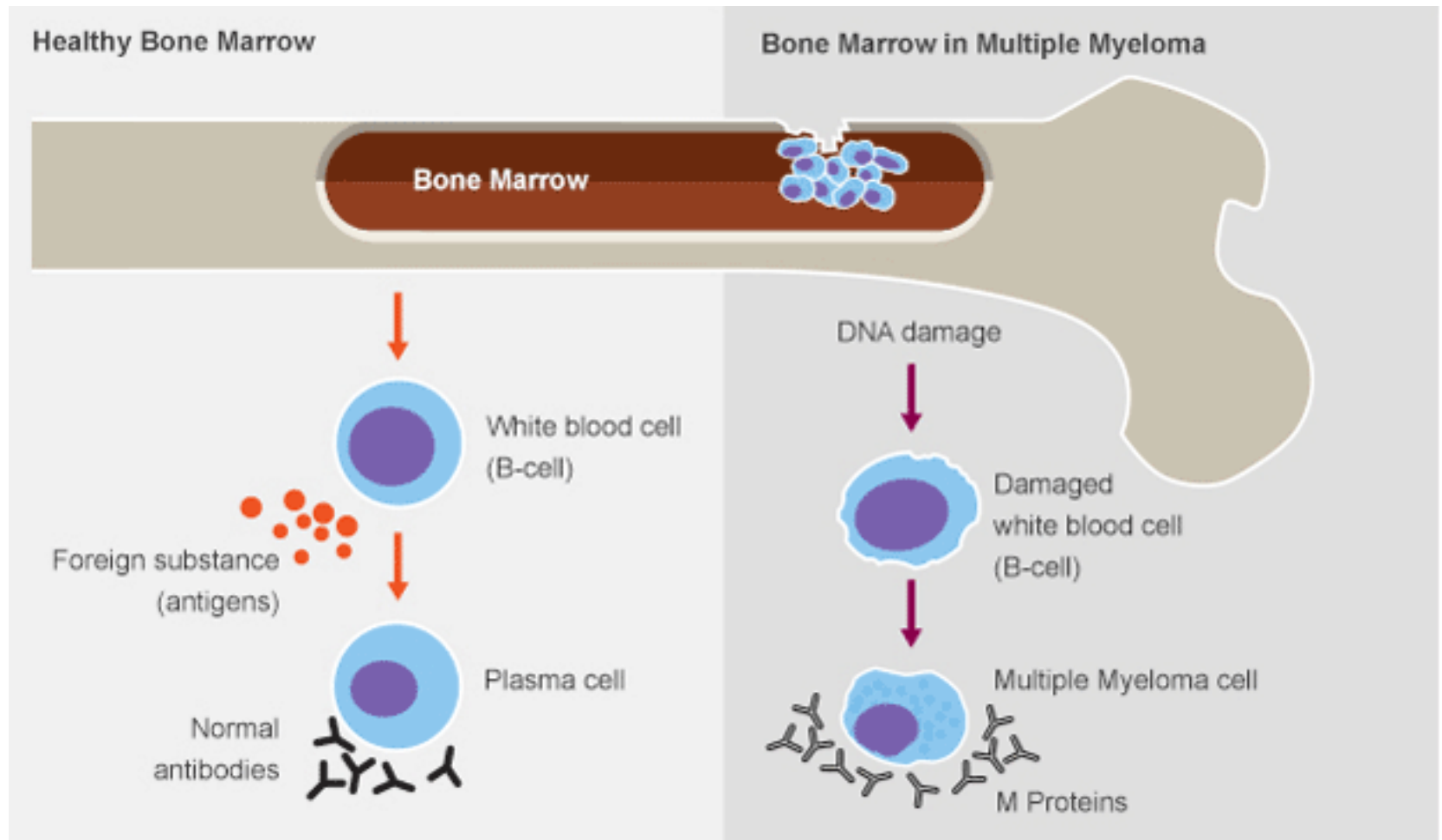
MANAGEMENT of INNOVATION in patients with MYELOMA

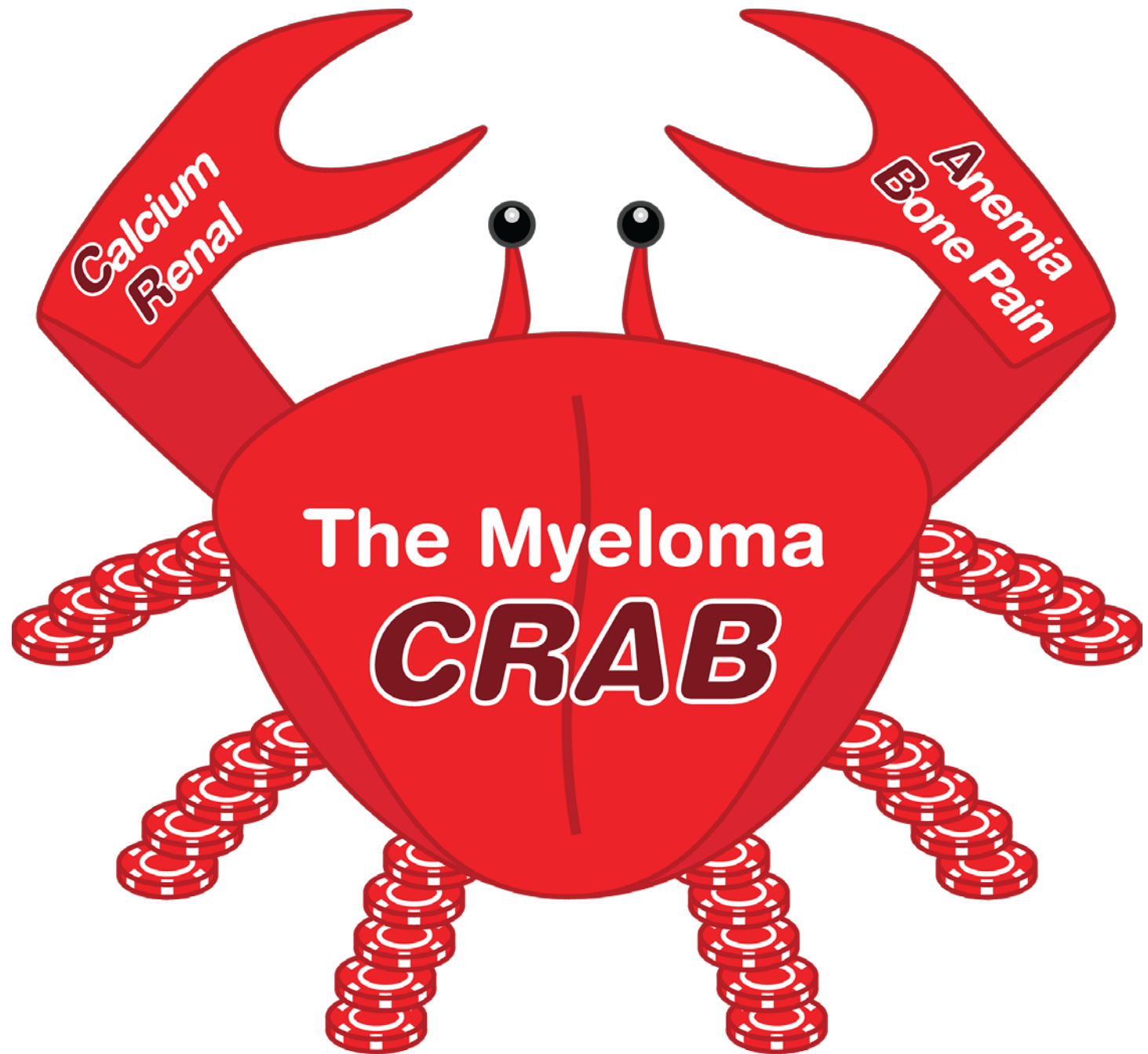
Pr. MC. VEKEMANS





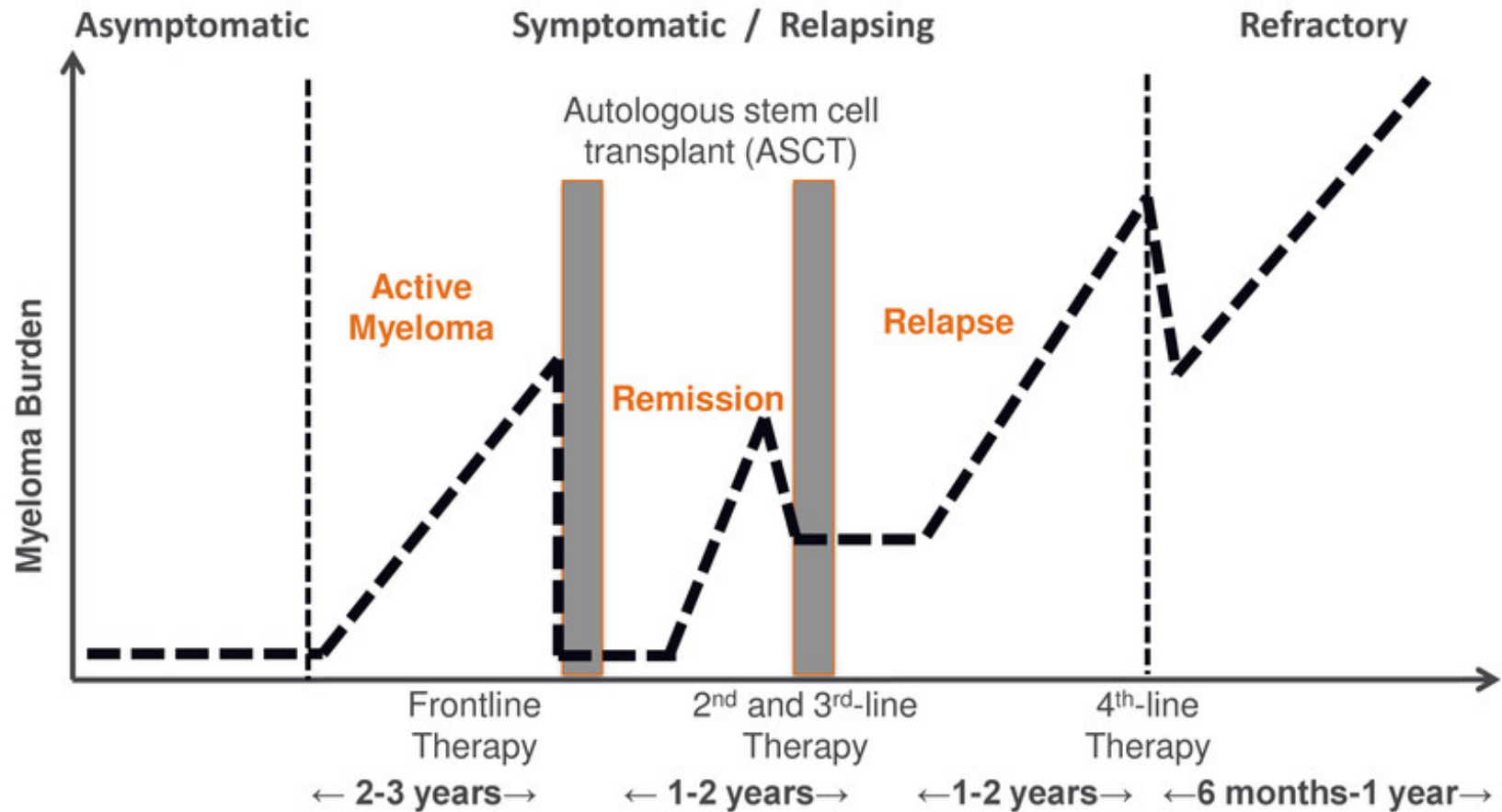
WHAT IS MYELOMA ?



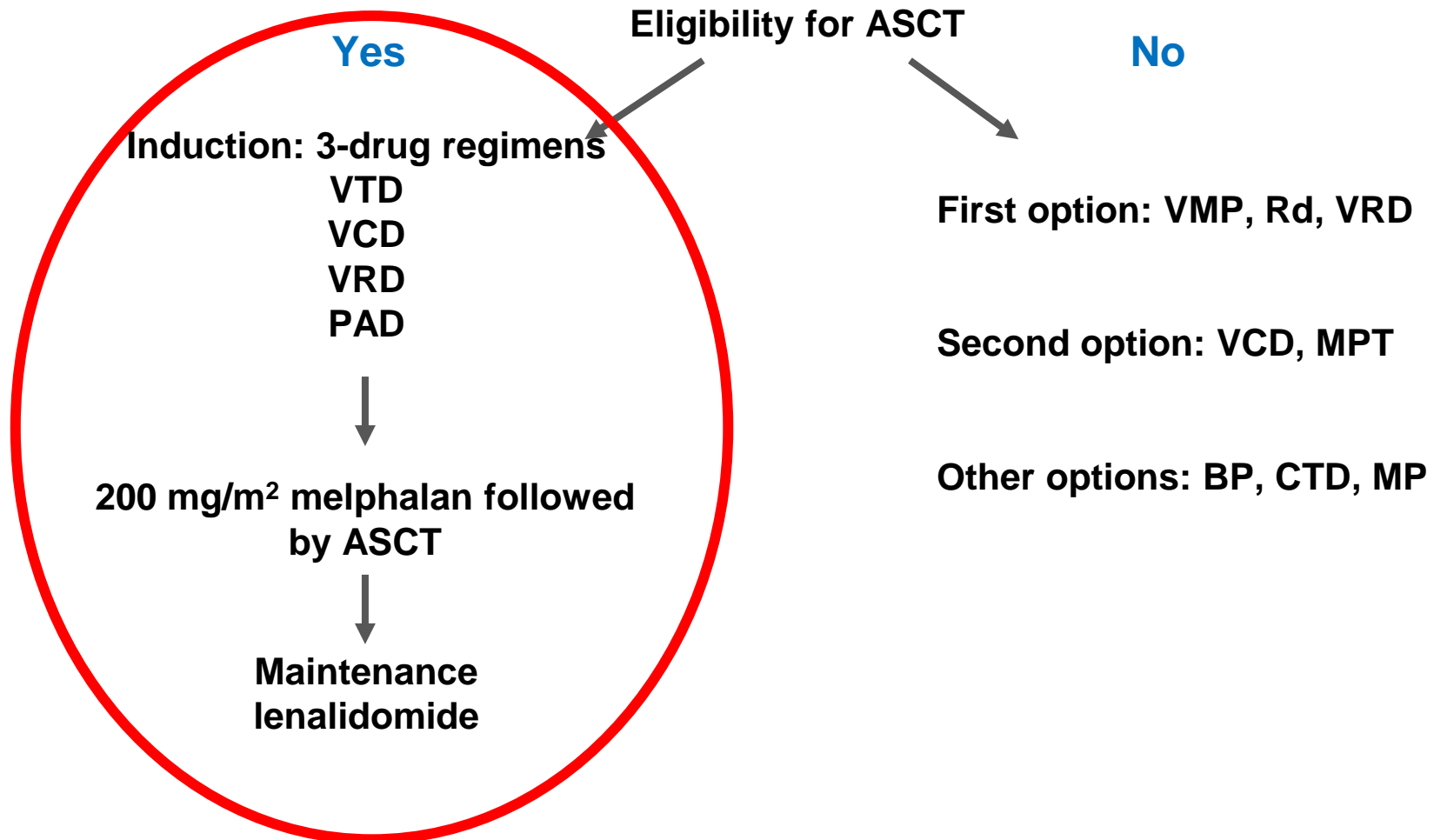




NATURAL HISTORY of MM



ESMO guidelines, FRONTLINE THERAPY



ACCORDING TO THE EUROPEAN CANCER OBSERVATORY...



38,000

NEW CASES OF MULTIPLE
MYELOMA IN EUROPE
EACH YEAR

AND AN ESTIMATED **89,000** PEOPLE ARE
CURRENTLY LIVING WITH THE DISEASE²

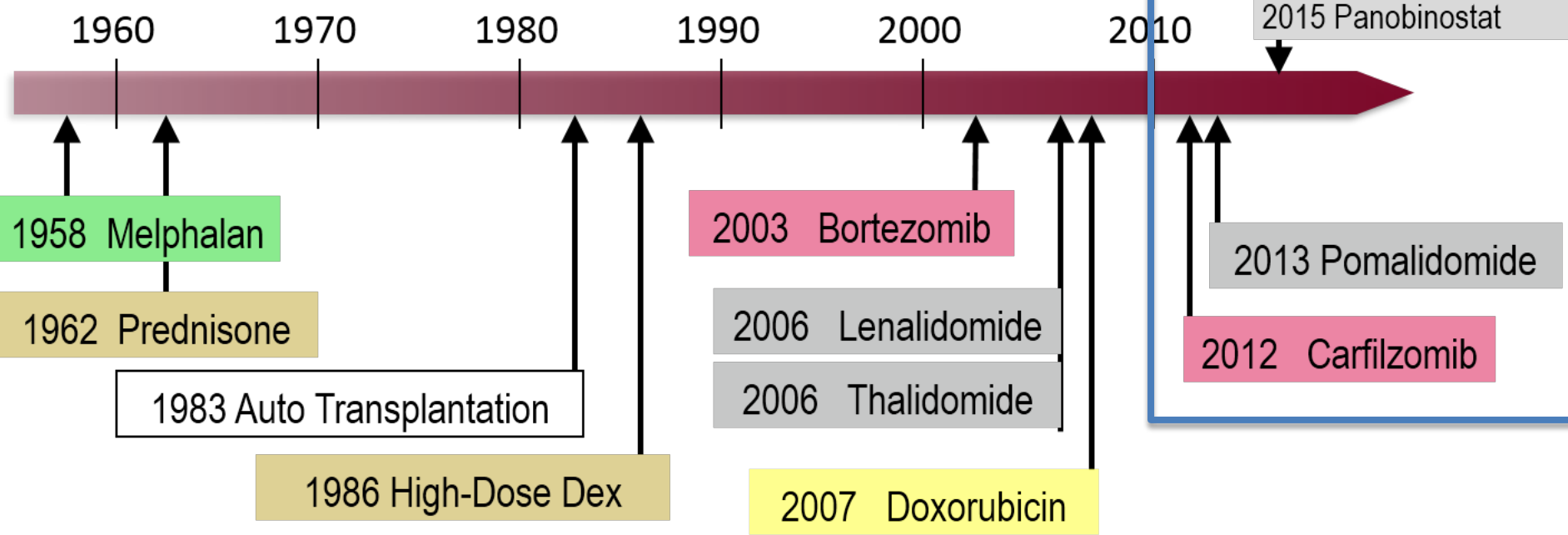
MULTIPLE MYELOMA CAN AFFECT
PEOPLE OF ALL AGES, BUT THE
MEDIAN AGE AT DIAGNOSIS IS³...

70 
YEARS

IN 2002, ESTIMATES SHOWED THAT **ONLY AROUND 1/3**
OF ALL PEOPLE DIAGNOSED WITH MULTIPLE MYELOMA
LIVED FOR LONGER THAN 5 YEARS⁴

MULTIPLE MYELOMA REMAINS INCURABLE, HOWEVER RECENT
ADVANCES IN TREATMENTS HAVE RESULTED IN A **50% INCREASE**
IN 5-YEAR SURVIVAL RATE BETWEEN 1992 AND 2008⁵

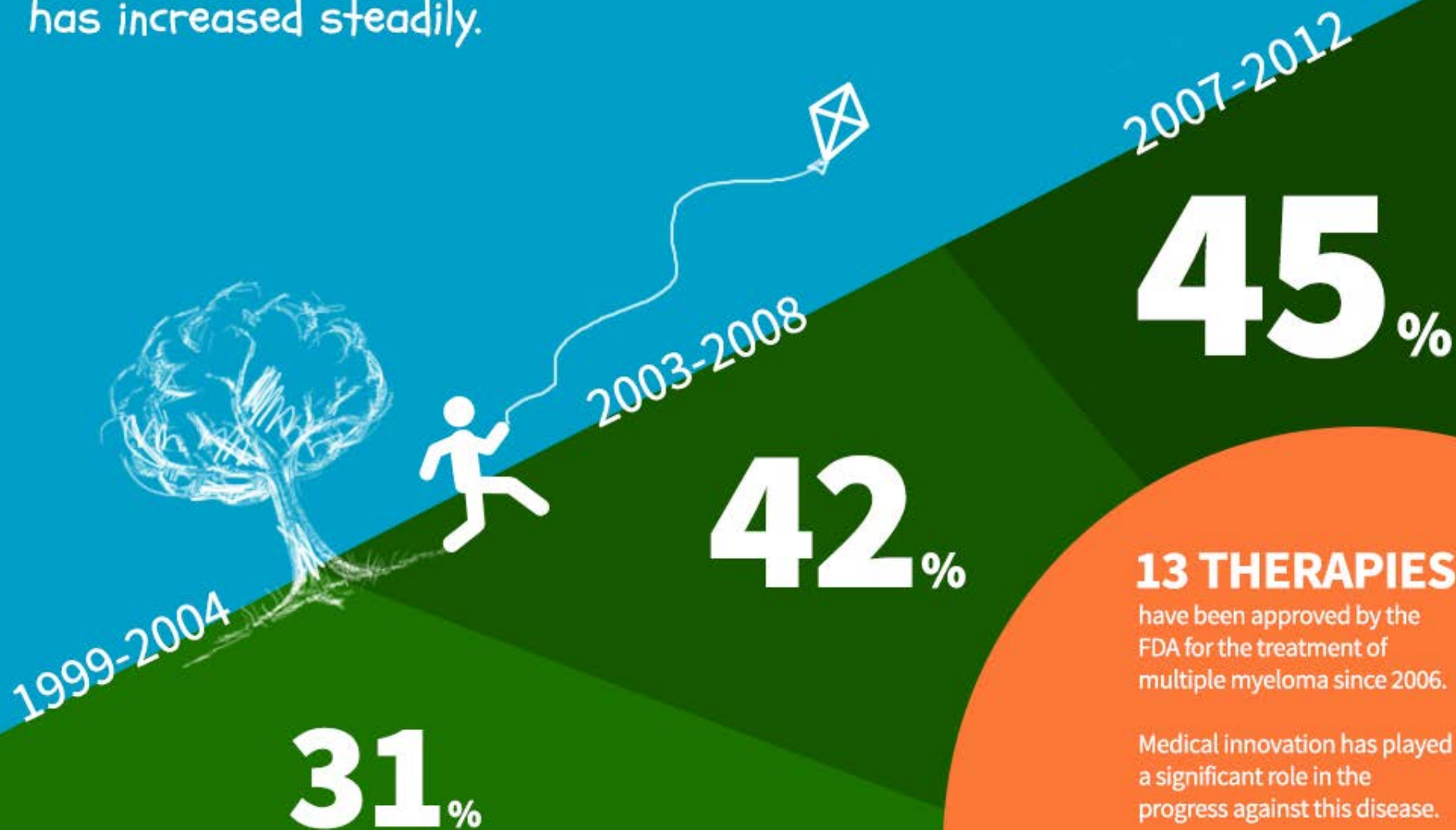
- | | | |
|---|--|---|
| Alkylator | Proteasome inhibitor ("mib") | Antibody ("mAbs") |
| Steroid | Immunomodulator ("imid") | HDAC inhibitor |
| Anthracycline | | |



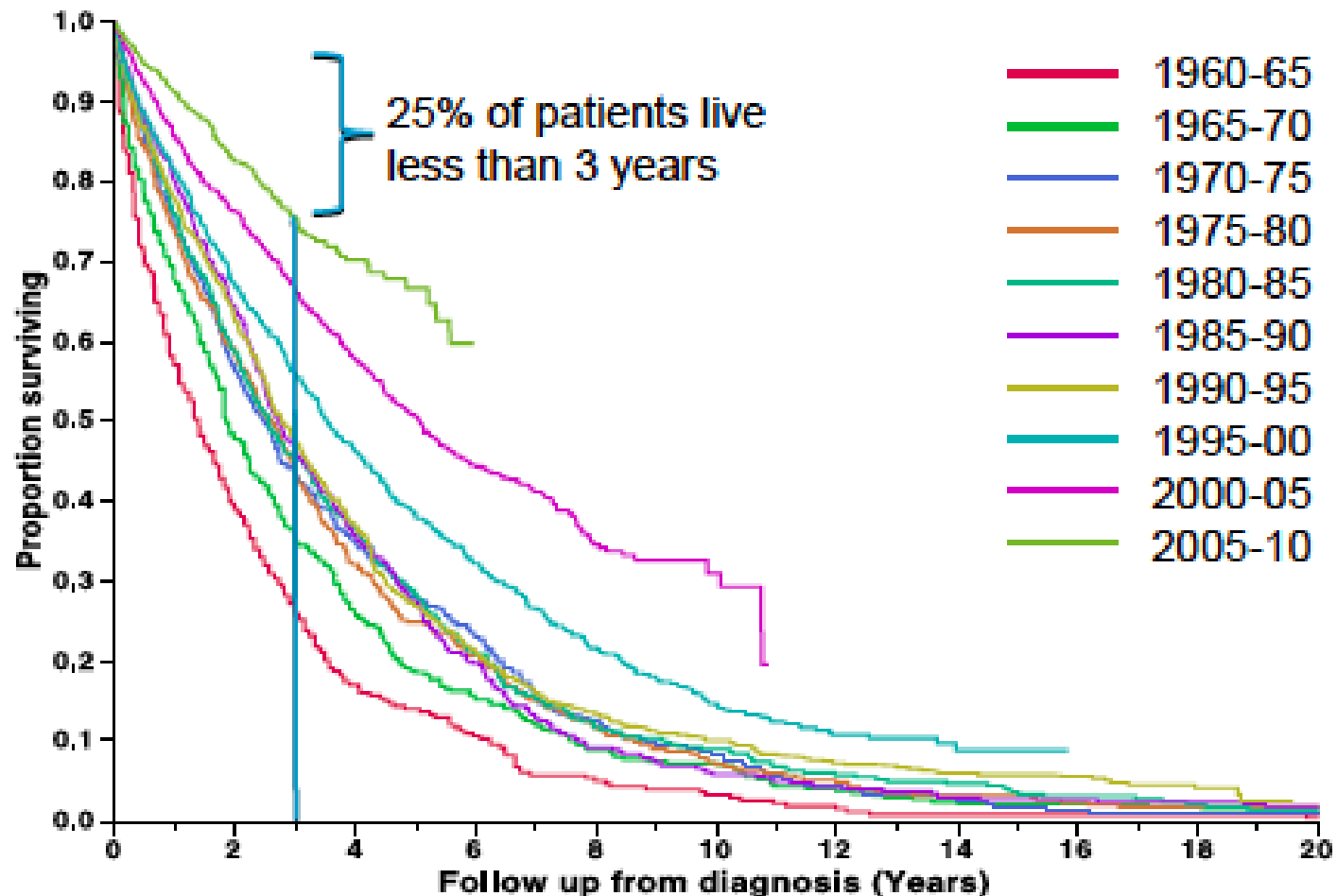
Auto = Autologous; Dex= Dexamethasone

Multiple Myeloma: A Decade of Progress

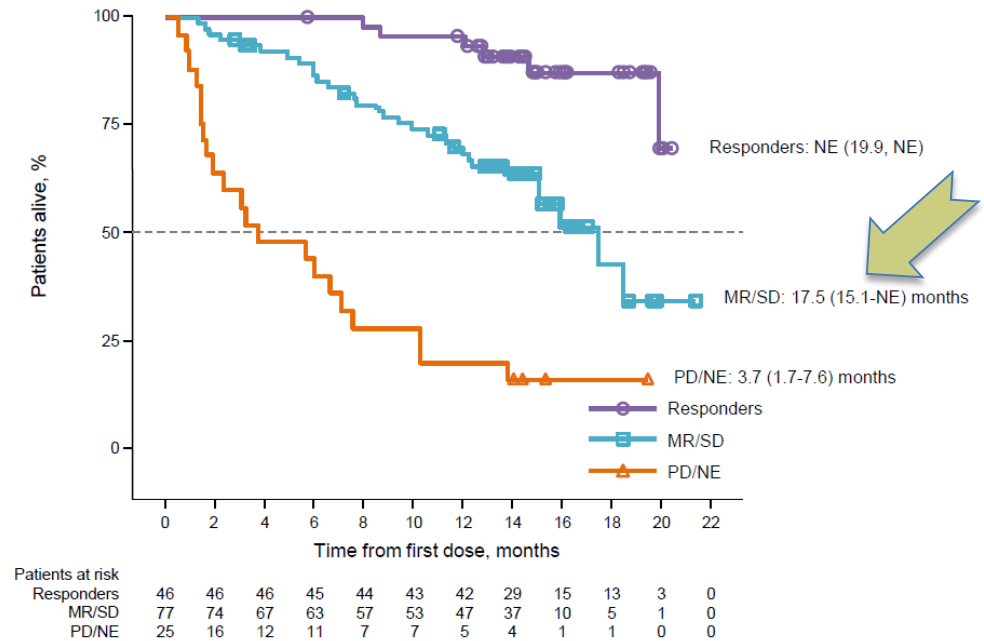
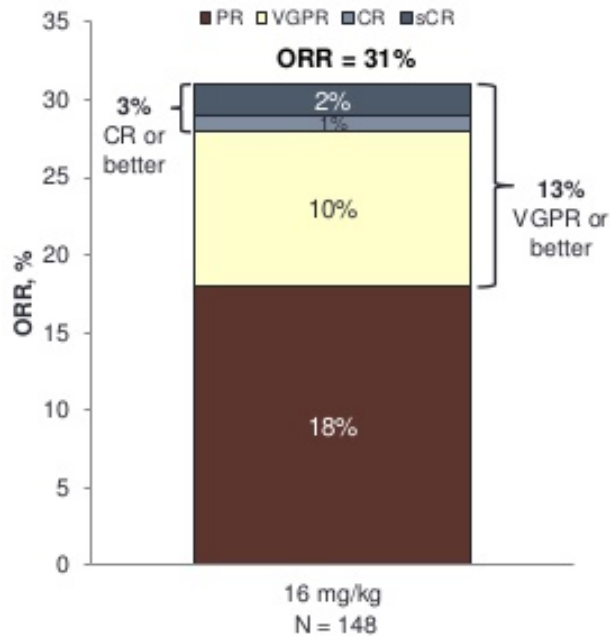
Since 1999, the five-year survival rate for myeloma patients has increased steadily.



IMPROVING SURVIVAL in MM

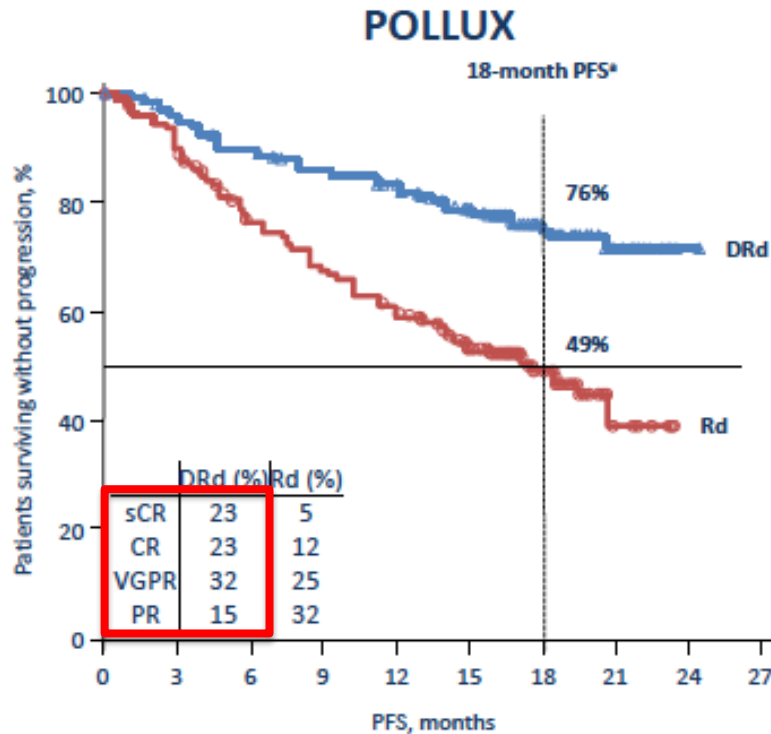


DARATUMUMAB MONOTHERAPY



- Rapid, deep, durable responses in heavily pretreated MM pts
- OS benefit even in pts who achieved a **SD or MR**

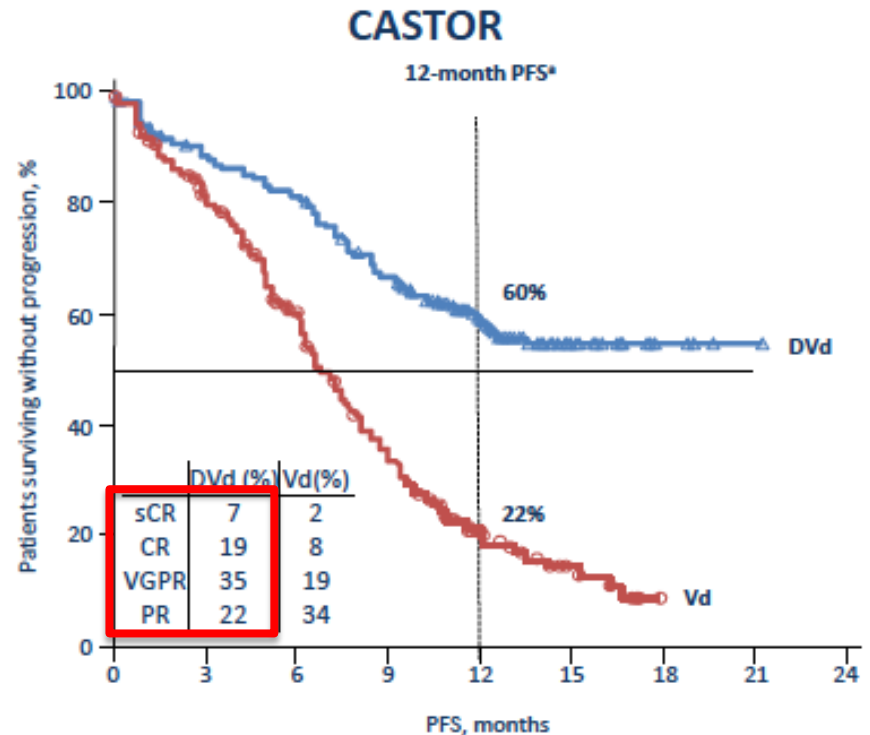
DARATUMUMAB, PFS



Median (range) follow-up:
17.3 (0-24.5) months

■ Median PFS

- DRd: not reached; Rd: 17.5 months
- HR: 0.37 (95% CI, 0.28-0.50; $P < 0.0001$)

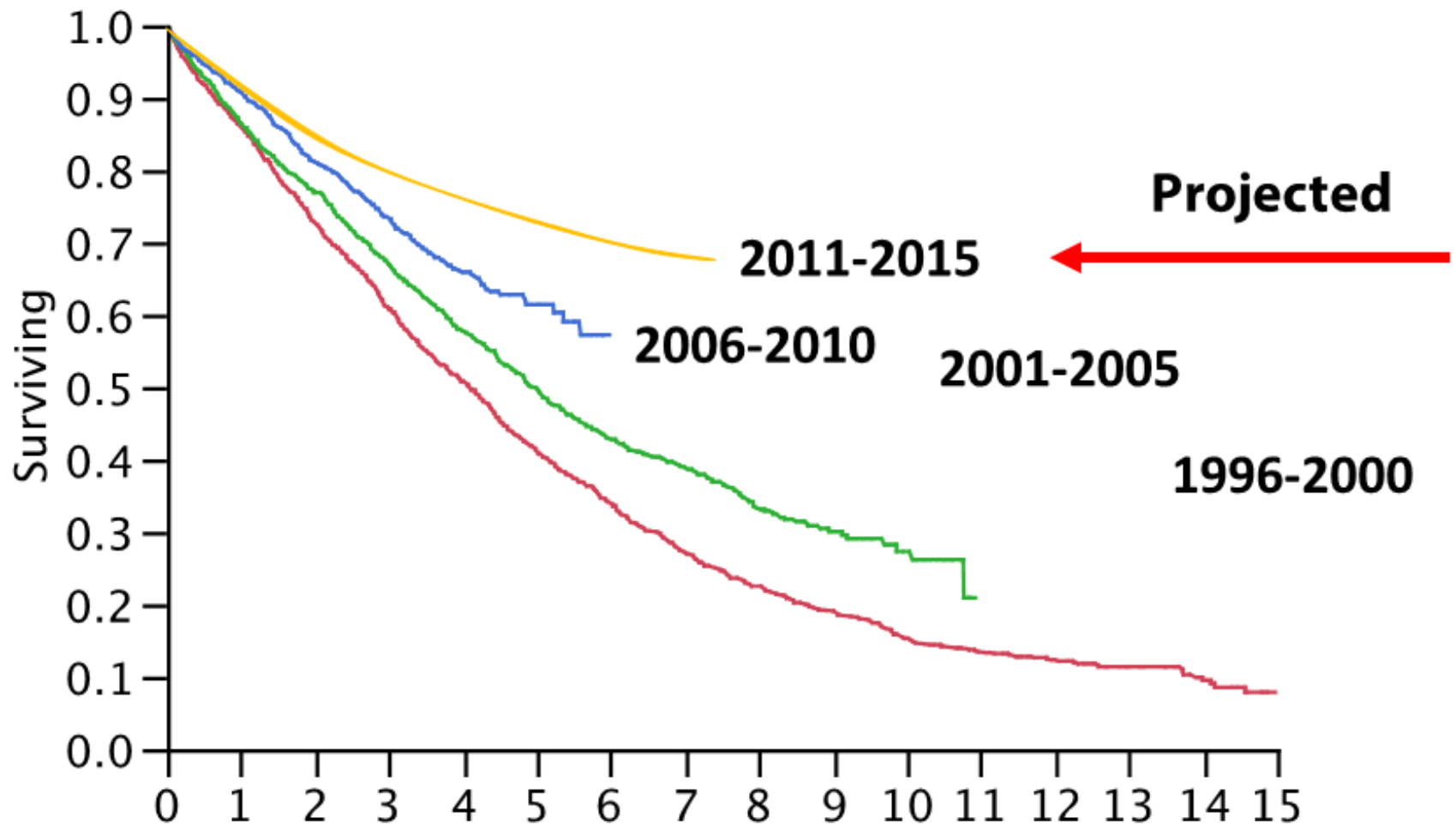


Median (range) follow-up:
13.0 (0-21.3) months

■ Median PFS

- DVd: not reached; Vd: 7.1 months
- HR: 0.33 (95% CI, 0.26-0.43; $P < 0.0001$)

SURVIVAL in MYELOMA



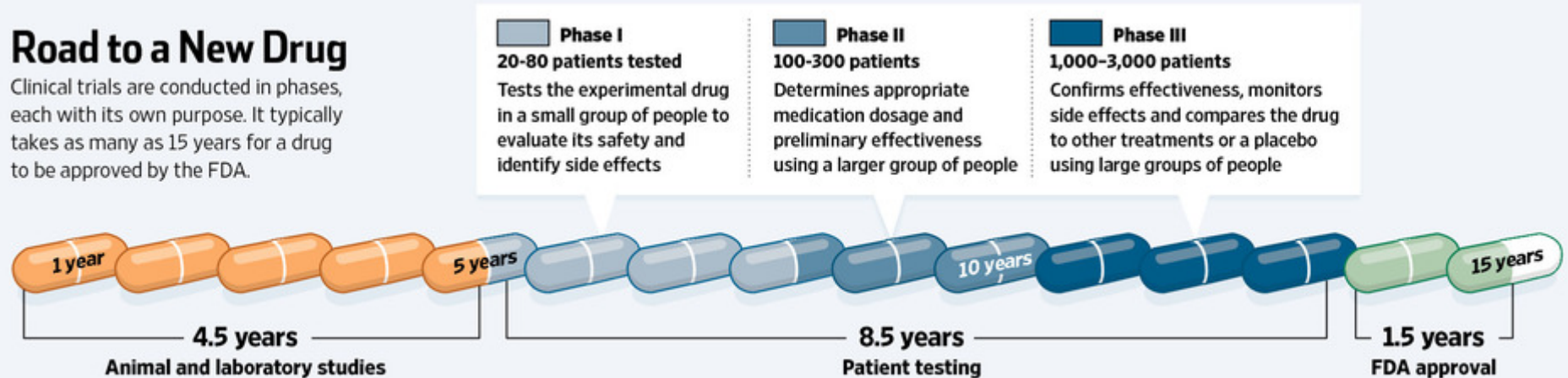


COST
OR
CARE

ROAD to a NEW DRUG

Road to a New Drug

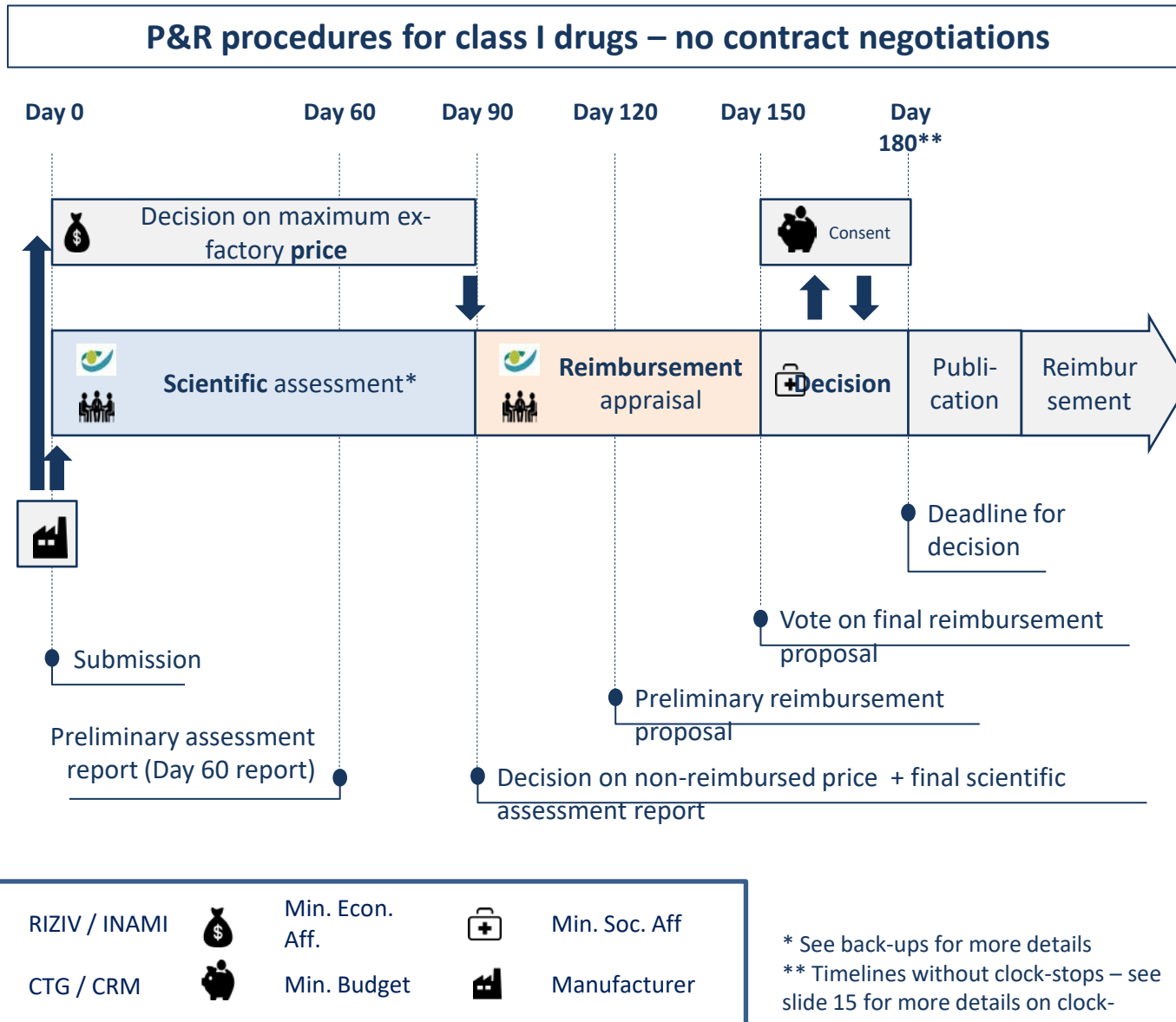
Clinical trials are conducted in phases, each with its own purpose. It typically takes as many as 15 years for a drug to be approved by the FDA.



Note: Years and number of patients are approximate. Sources: MD Anderson Cancer Center; National Institutes of Health

The Wall Street Journal

Scientific assessment and reimbursement appraisal for class I drugs



EARLY ACCESS PROGRAM

- EARLY TEMPORARY AUTHORISATION (ETA)
 - Allows patients to have a more rapid access to innovative treatments, when the registration procedure for that drug is still ongoing
 - Concerns drugs delivered for the treatment of lethal diseases, where there is no alternative
- EARLY TEMPORARY REIMBURSEMENT (ETR)

EARLY ACCESS PROGRAM

- EARLY TEMPORARY AUTHORISATION (ETA)
 - **MEDICAL NEED PROGRAM (MNP)** : on a cohorte basis :
 - group of patients
 - defined criteria
 - exemples : Kyprolis-dexamethasone,
 - Soon : ixazomib, lenalidomide maintenance
 - **COMPASSIONATE USE PROGRAM** : on an individual basis :
 - for a designated patient, unable to participate to a clinical trial
 - via AFPMS (Federal Agency for Drugs and Health Products)

Belgian PACT for INNOVATION

July 27th, 2015

Negotiations with the pharmaceutical industry

- Shorten the reimbursement procedure by 50 days
- Enhance international collaborations with NL and L, particularly on orphan drugs
- Reduction of the cost of drugs
- Reinforce Belgium's position as Europe's leader in clinical trials
- Favor more competition on the post-patent market, in order to reinvestigate 1.4 billion dollars over a 4-year period into innovative products
- Tax policy to privilege innovators and investors

DRUG SHORTAGE

- Medicines unavailable for patients because of production or stock problems
- Most of the time, alternatives can be found, but each year there is a big problem for public health in about 15 cases, mostly because a rare medicine against cancer or infections is unavailable.



ROLE of BHS



- BHS is a scientific organisation in hematology
 1. Promote scientific progress in the field of hematology
 2. Enhance collaboration with other national and international scientific organisations
- Promote quality of hematology practice in Belgium
 3. Improve education and training in the field of hematology
 4. Enhance collaboration between hematology centers
 5. Promote and facilitate peer review processes
 6. Publication of guidelines
- Inforce the position of hematology speciality in Belgium
 7. In interactions with regulatory authorities
 8. Within the field of oncology
- **Support organizations of patients with hematological disorders**
 9. In their activities
 10. In their representation

ROLE of BHS



- Support **patient organisations** in their **activities**
(also in (in collaboration with corporate partners)
 - patients meetings at the GAM
- **Support patient organisations** in their **representation**
 - in their interaction with regulatory authorities

→ **Patients committee 2016**

- Provide a forum for people to meet each other, share experiences and provide on-going support for patients and their relatives.
- Offer a platform where different patients' associations can meet
- Favor support tools to raise awareness of the diseases
- Guide us in developing new medicines, clinical trials, in seeking regulatory and reimbursement approvals, and providing patient support.



1^{er} SEMINAIRE D'INFORMATION ET DE SOUTIEN

ORGANISÉ PAR
L'INTERNATIONAL MYELOMA FOUNDATION
EN COLLABORATION AVEC LE MYMU
POUR LES PATIENTS ET LES FAMILLES

SAMEDI 6 MAI 2017 DE 10H00 À 16H30
Hôtel Dolce La Hulpe
CHAUSSÉE DE BRUXELLES 195 - 1310 LA HULPE

INSCRIPTIONS
POUR LE 28 AVRIL AU PLUS TARD
par l'envoi du carton réponse disponible dans les services
d'hématologie et auprès de votre médecin ou par mail :
b.beuwaes@skynet.be - www.myloma.be - GSM : 0474370680

avec l'appui institutionnel de



FUTURE

- Build robust clinical trials addressing specific questions
 - Based on biological characteristics of the disease,
 - Equivalent degree of survival benefit with a shorter course of therapy
 - Adjust therapy based on response
exemple : KRd
- Feed back : what are we doing?



THANK YOU for YOUR ATTENTION