







CDDF
MULTI-STAKEHOLDER
WORKSHOP

26 - 28 APRIL 2021

Endpoints in Cancer Drug Development HTA Perspective

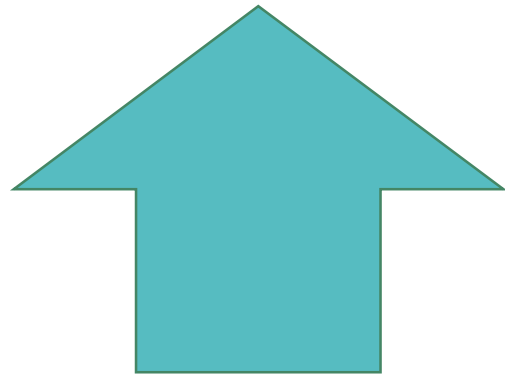
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What
outcomes
are
relevant to
patients?

Who
actually
determines
this
relevance?

Trends...



Cancer Drug
Development

Patient Relevant Outcomes

Patient Reported Outcomes

Health Related Quality of
Life (HRQoL)



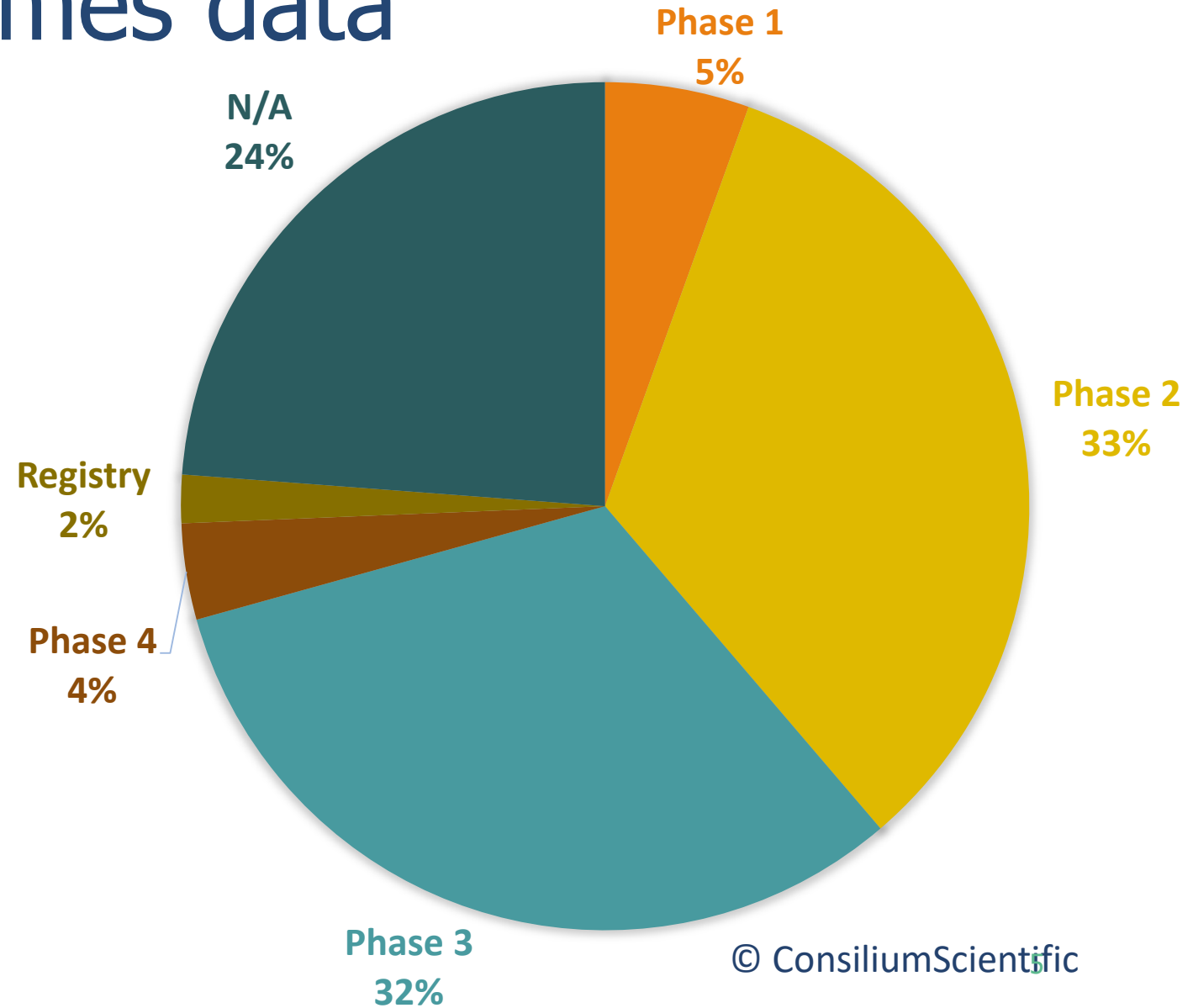


Patient Reported Outcomes data in Multiple Myeloma Trials

Only **12%** (n=382) of registered trials in Multiple Myeloma (n=3,179) collect PRO data

Phase 1	3%
Phase 2	8%
Phase 3	31%
Phase 4	22%
Registry	23%
N/A	17%

Trials in MM which collected Patient Reported Outcomes data





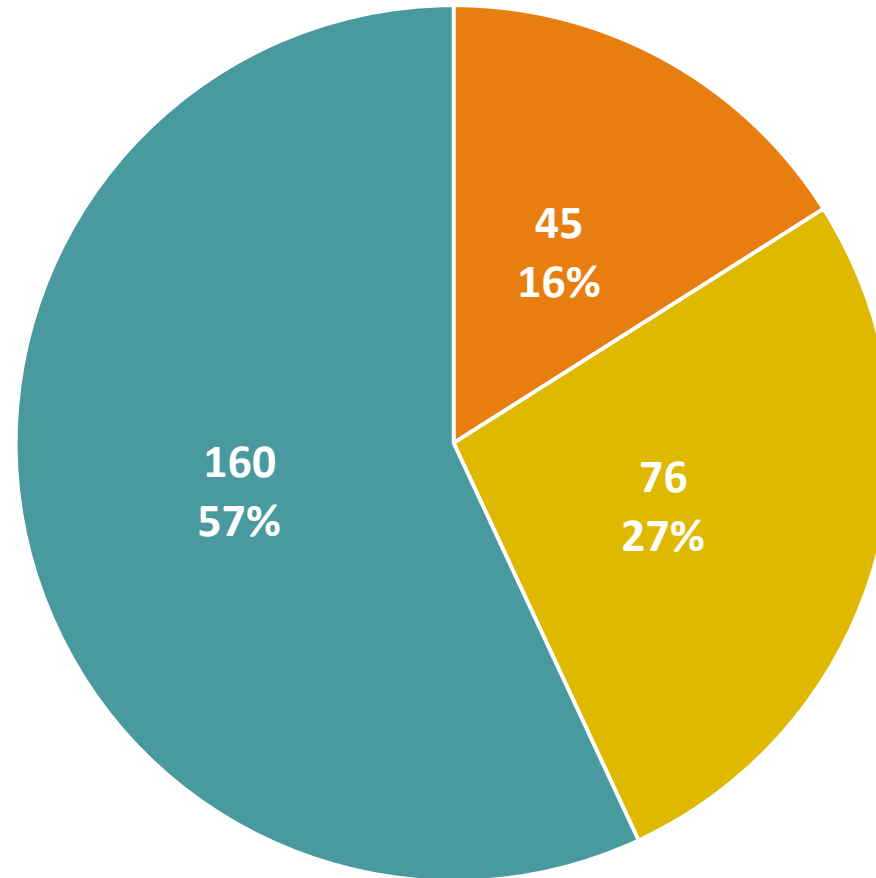
Patient Reported Outcomes as Trial Endpoints in MM

Primary	97	25%
Secondary	326	85%
Exploratory	18	5%

Based on 382 trials that collected QoL data

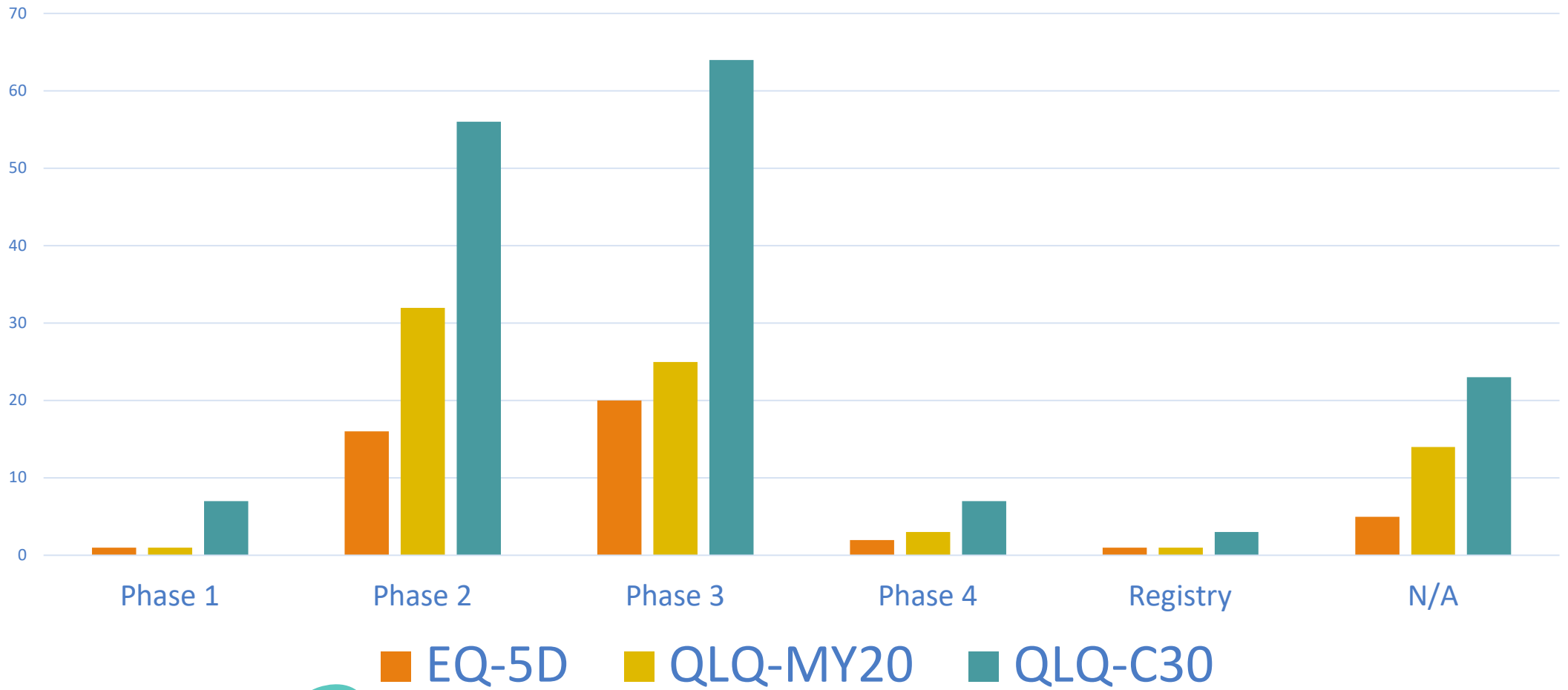


Use of QoL instruments in MM trials

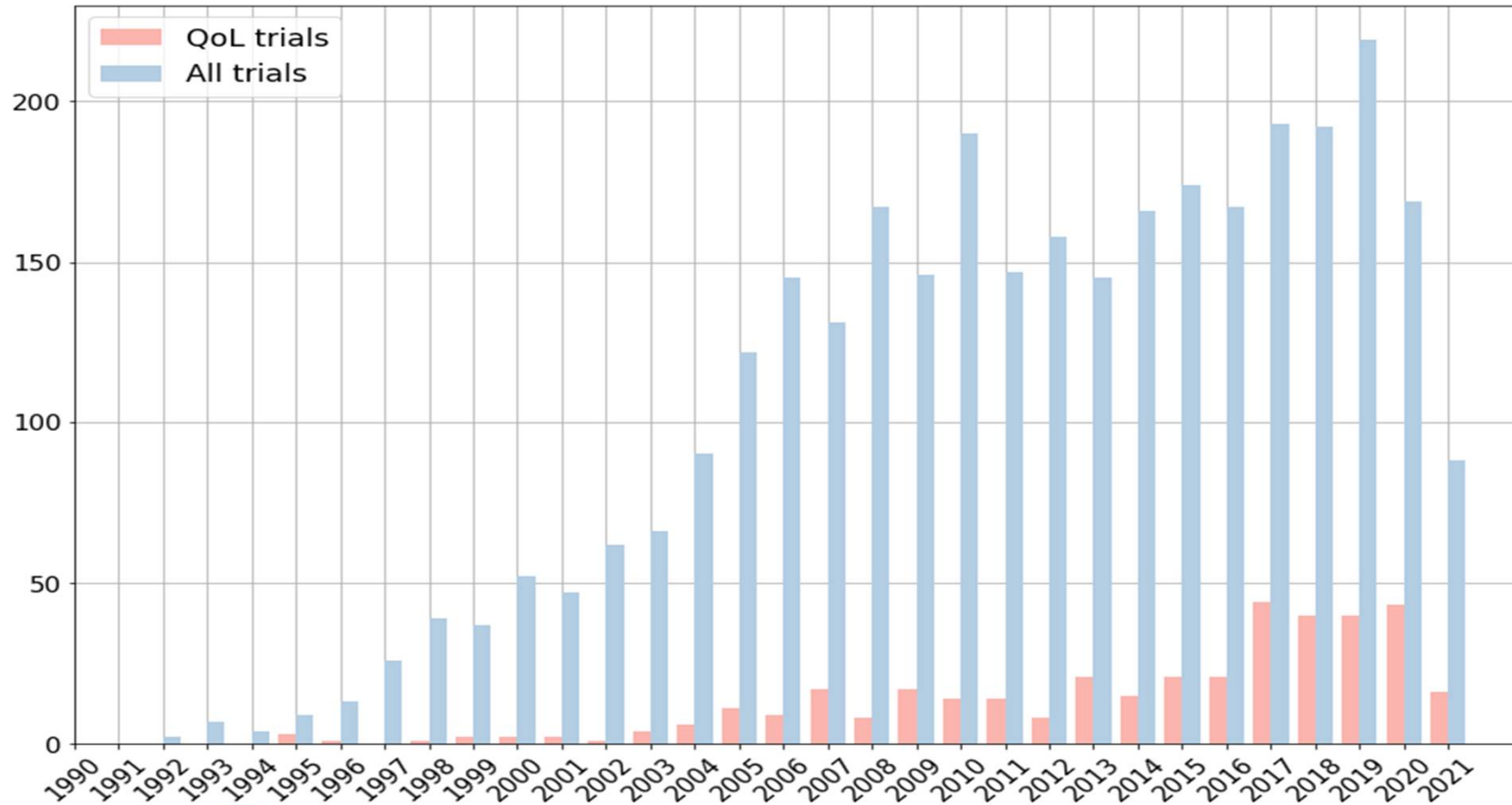


■ EQ-5D ■ QLQ-MY20 ■ QLQ-C30

QoL instruments by Phase in MM trials



Patient Reported Outcomes data collection over the years in MM trials





Problems

- PRO/QoL data collection is not standardized
- Quality of PRO/QoL data
- Timing and frequency of PRO/QoL data collection
- Representativeness of PRO/QoL data
- Lack of PRO/QoL data collection in clinical practice



HTA* perspective

(reimbursement decisions)

Patient relevant outcomes are essential for the HTA process

Patient reported outcomes must be converted into utilities for inclusion into an economic model



HTA perspective: PROs

Survival

- ✓ Overall Survival
- ✓ Progression Free Survival
- ✓ Disease Free Survival

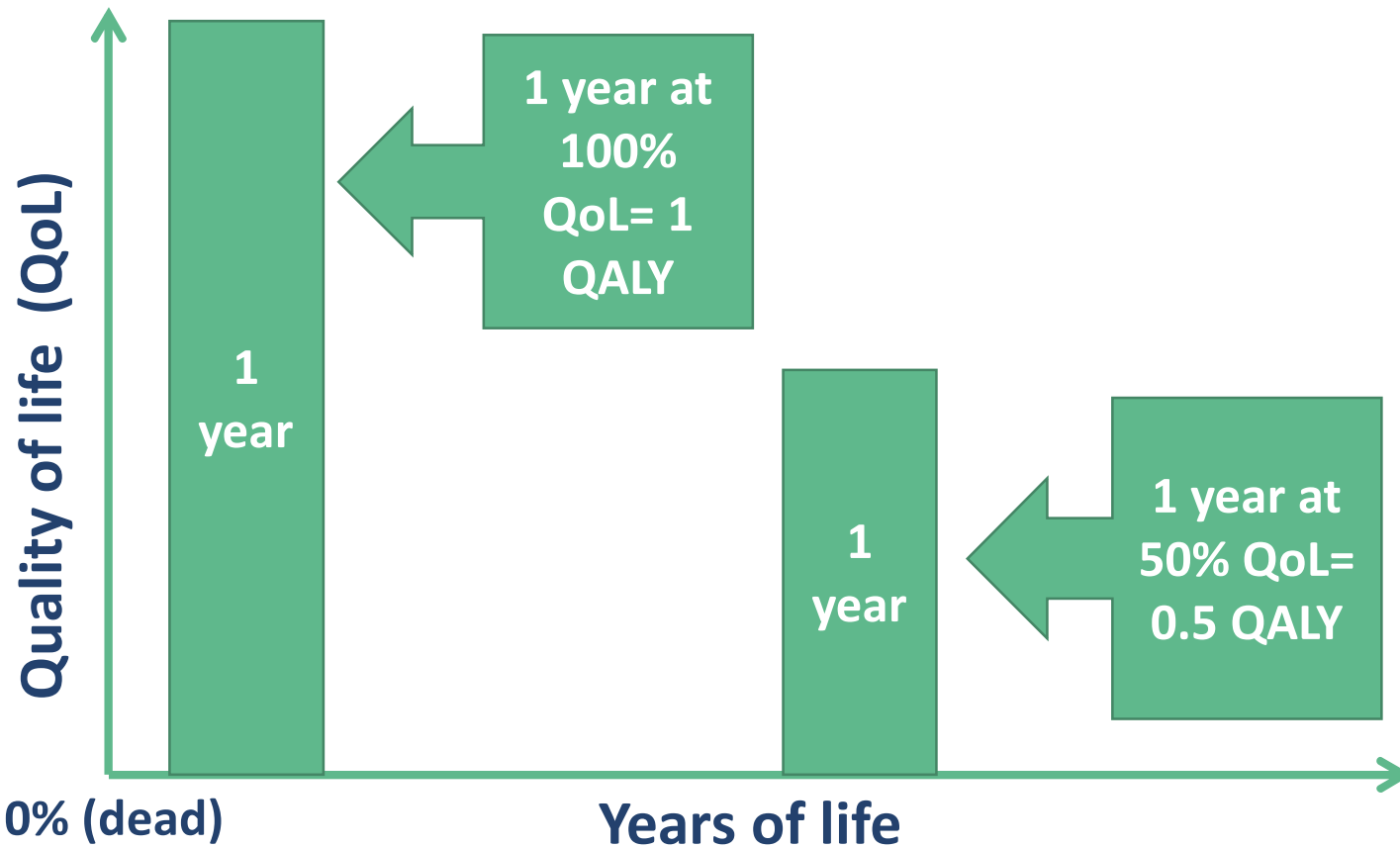
Quality of Life

- ✓ Level of Pain
- ✓ Level of Mobility
- ✓ Ability to Self—care
- ✓ Anxiety/Depression
- ✓ Usual activities



The QALY


100% (perfect health)



Measuring Quality of Life: EQ-5D

<http://www.euroqol.org/>





An EQ-5D health state may be converted to a single summary index by applying a formula that essentially attaches weights to each of the levels in each dimension



$$\text{Health State Utility} = 1 - 0.081N2 - 0.069MO2 - 0.314MO3 - 0.104SC2 - 0.214SC3 - 0.036UA2 - 0.094UA3 - 0.123PD2 - 0.386PD3 - 0.071AD2 - 0.236AD3 - 0.269N3$$

If EQ-5D is scored 11111, then the utility is 1

QoL Instruments for HTA

Generic

- ✓ EQ-5D
- ✓ SF-36

Generic Cancer

- ✓ EORTC – QLQ – C30
- ✓ FACT-G
- ✓ VAS-C

Disease Specific Instruments

- ✓ EORTC – QLQ – MY20
- ✓ FACT-MM
- ✓ FACT-L



Mapping

Algorithms for converting scores from one QoL instrument to another:

e.g. prediction of health state utility values from disease-specific instruments





What needs to be done:

Product developers **MUST** collect PROs in trials

- Collect PROs in Phase 2 as in Phase 3 + follow up
- Timing of data collection and choice of instruments are important
- Plan studies carefully not to miss patient relevant opportunities





What needs to be done

- Ensure that QoL data collection is a requirement in every trial
 - Collect QoL data during follow up
 - Incorporate QoL data collection in clinical practice/registries
- Encourage creation of QoL data sets for different population subgroups – co-morbidities, carers, etc.
- Keep QoL data current
 - Treatments change, patient profile may change





What needs to be done

- Methodological work to:
 - standardize/ compare instruments and approaches (proxies)
 - develop valuation sets/ mapping algorithms
 - validate PRO instruments



Questions





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