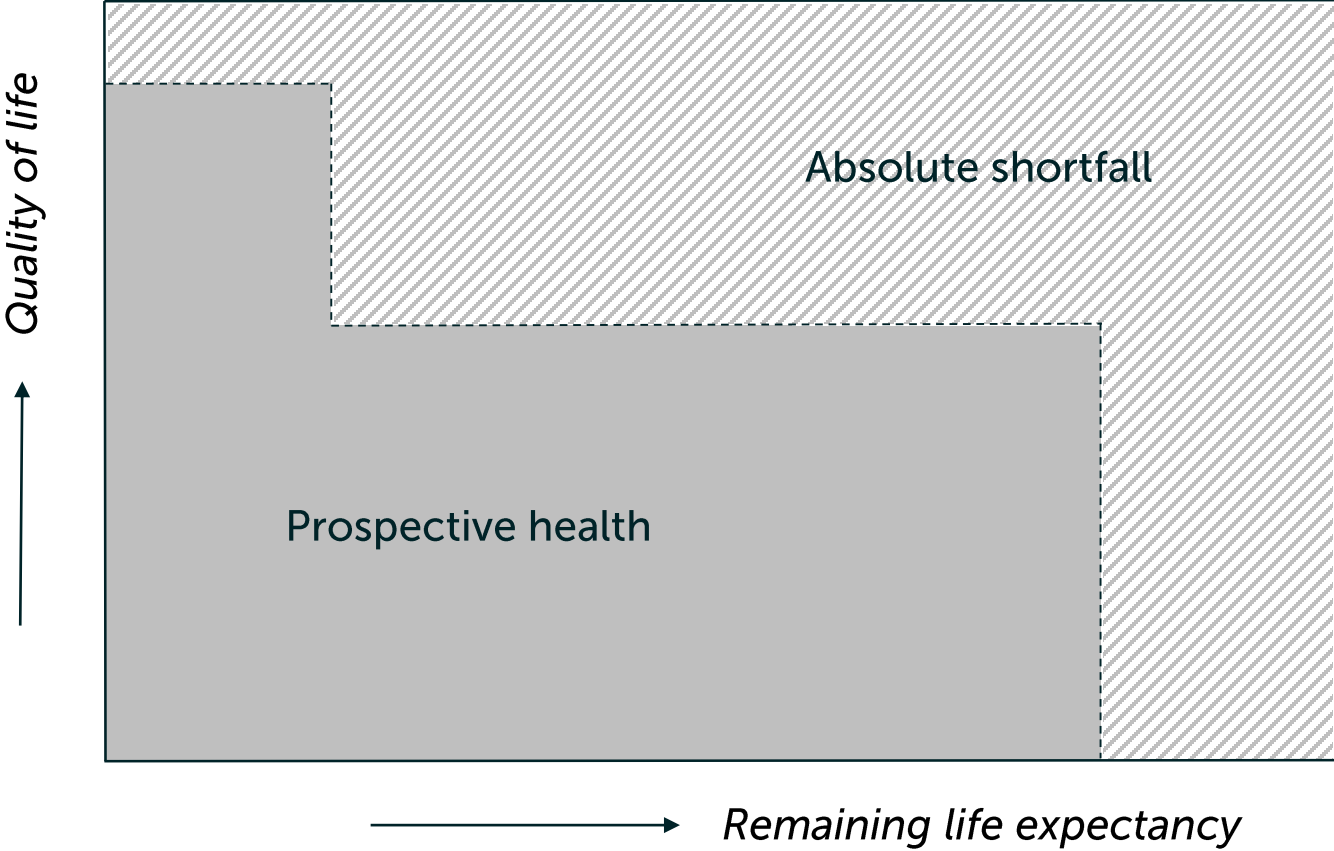


Empirical evidence and future directions for equity weighting

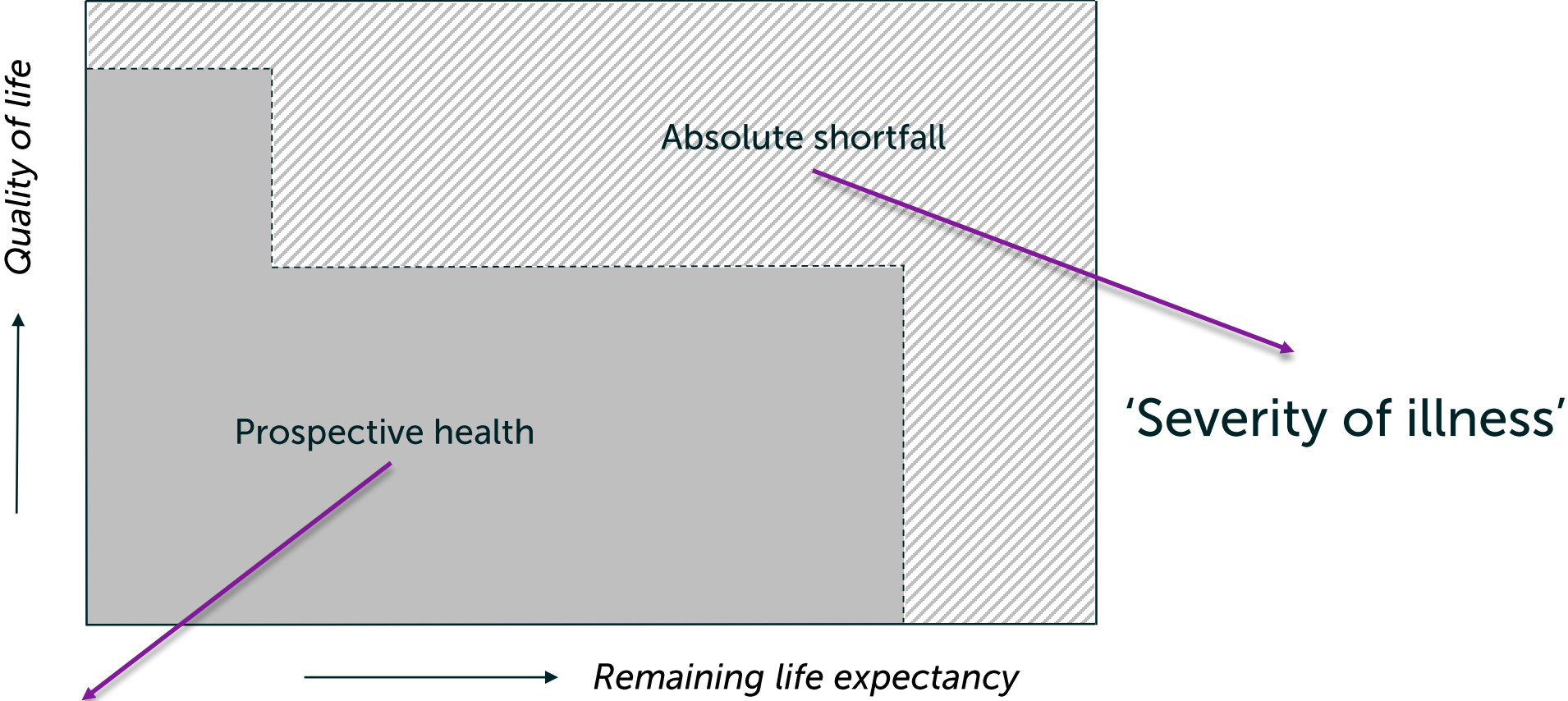
Vivian Reckers-Droog
PhD candidate, department of Health Economics, ESHPM
September 16, 2019

Proportional shortfall



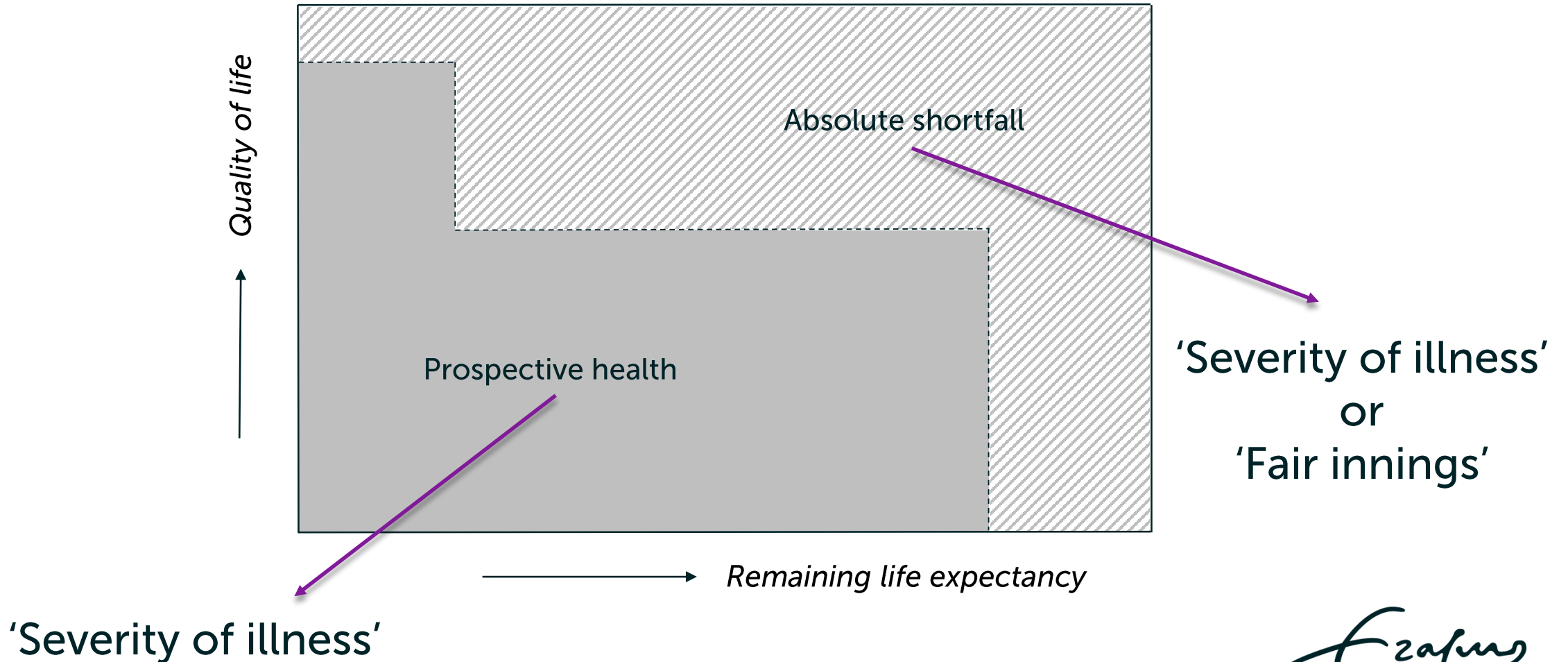
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Proportional shortfall



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Proportional shortfall



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Why proportional shortfall?

Main reasons:

1. Balances concerns for 'severity of illness' and 'fair innings'
2. Avoids ageism in reimbursement decisions (i.e. equal weight for younger and older patients)



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A brief history of..

Decision model
introduced in NL



Severity used in various ways (e.g.
qualitatively or DALYs)

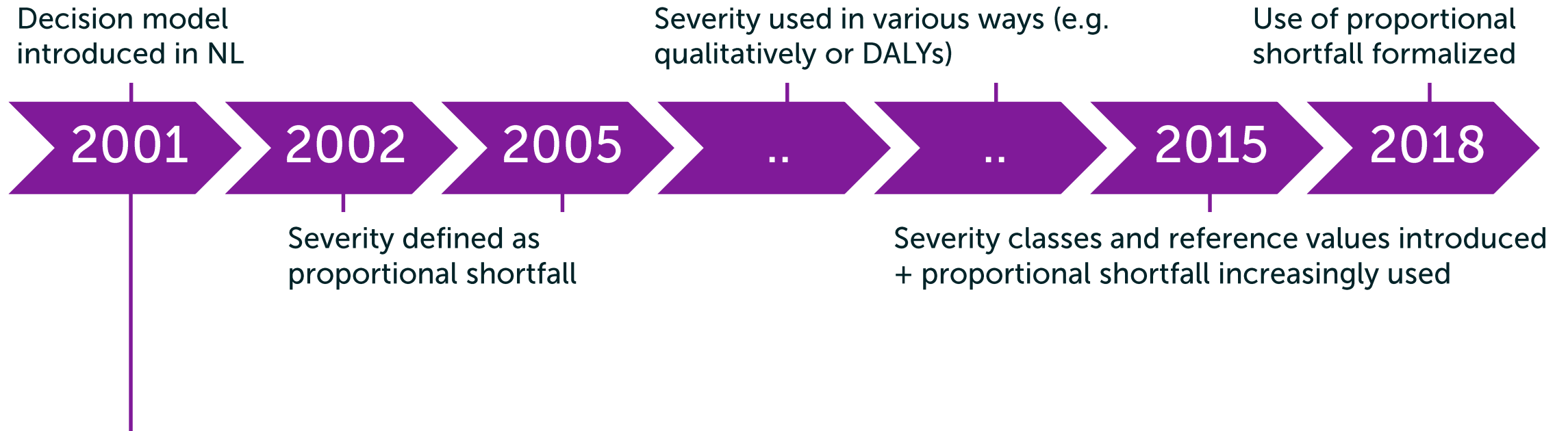
Use of proportional
shortfall formalized

Severity defined as
proportional shortfall

Severity classes and reference values introduced
+ proportional shortfall increasingly used

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A brief history of..



Since 2001, seven empirical studies examined whether proportional shortfall is aligned with societal preferences.

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Support for proportional shortfall

Study	Year	Country	Design	N	Sample	Support for PS
Stolk et al.	2005	NL	Ranking exercise	65	Convenience	++
Olsen	2013	Norway	Pairwise-choice task	503	General public	--
Brazier et al.	2013	UK	DCE	3,669	General public	--/-
Van de Wetering et al.	2015	NL	DCE	1,205	General public	--
Bobinac et al.	2015	NL	WTP	1,320	General public	-
Rowen et al.	2016	UK	DCE	3,669	General public	+
Richardson et al.	2017	Australia	Paired comparison	606	General public	+

Level of support: -- = no, - = limited, + = modest, ++ = strong.

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Support for proportional shortfall

Study	Year	Country	Design	N	Sample	Support for PS	Support for Age
Stolk et al.	2005	NL	Ranking exercise	65	Convenience	++	++
Olsen	2013	Norway	Pairwise-choice task	503	General public	--	++
Brazier et al.	2013	UK	DCE	3,669	General public	--/-	NA
Van de Wetering et al.	2015	NL	DCE	1,205	General public	--	--
Bobinac et al.	2015	NL	WTP	1,320	General public	-	++
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Richardson et al.	2017	Australia	Paired comparison	606	General public	+	++

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Support for proportional shortfall (2)

- Public generally prefers prioritising younger over older patients
- Consequence of using proportional shortfall is that older patients may more frequently be prioritised



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How to move forward?

Adjust proportional shortfall?

- To align proportional shortfall with preferences for prioritising younger patients
- To meet the objective of avoiding ageism (by giving older patients a lower weight)

Adjust monetary reference values?

- To reflect severity-related preferences within different age groups

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Societal preferences for severity and age

Two stated-preference studies conducted to examine (the strength of) societal preferences for severity and age.

Choice- and person-trade-off tasks:

- Elicit preferences for priority setting based on severity, age, and combination of both (status: in press)

Contingent-valuation tasks:

- Estimate the severity-dependent willingness to pay per QALY at different ages (status: data collection)

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Societal preferences

Difference in severity, same age:

- Preference for reimbursing treatment for more severely ill patients

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Societal preferences

Difference in severity, same age:

- Preference for reimbursing treatment for more severely ill patients

Difference in age, same severity level:

- Preference for reimbursing treatment for younger patients

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Societal preferences

Difference in severity, same age:

- Preference for reimbursing treatment for more severely ill patients

Difference in age, same severity level:

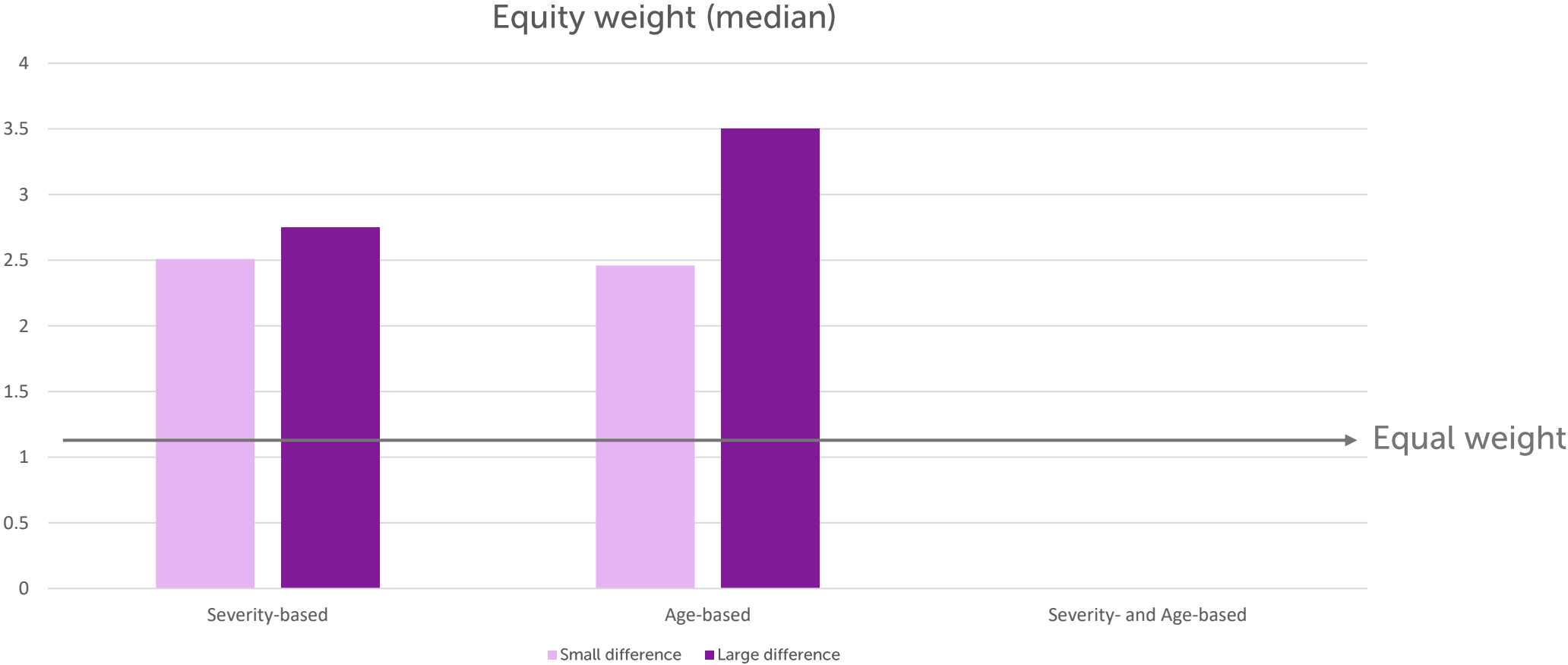
- Preference for reimbursing treatment for younger patients

Difference in severity *and* age:

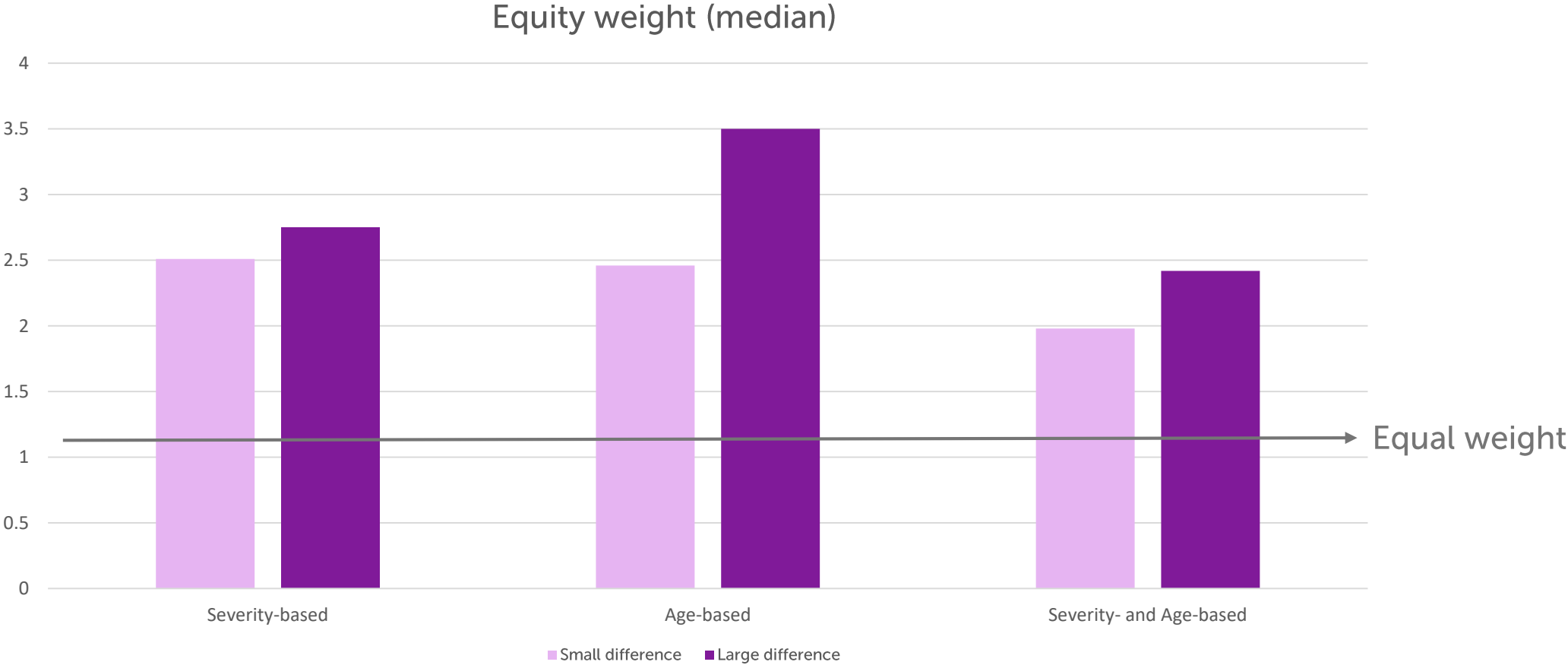
- Preference for reimbursing treatment for younger patients, regardless of patients' severity level

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Strength of preferences



Strength of preferences





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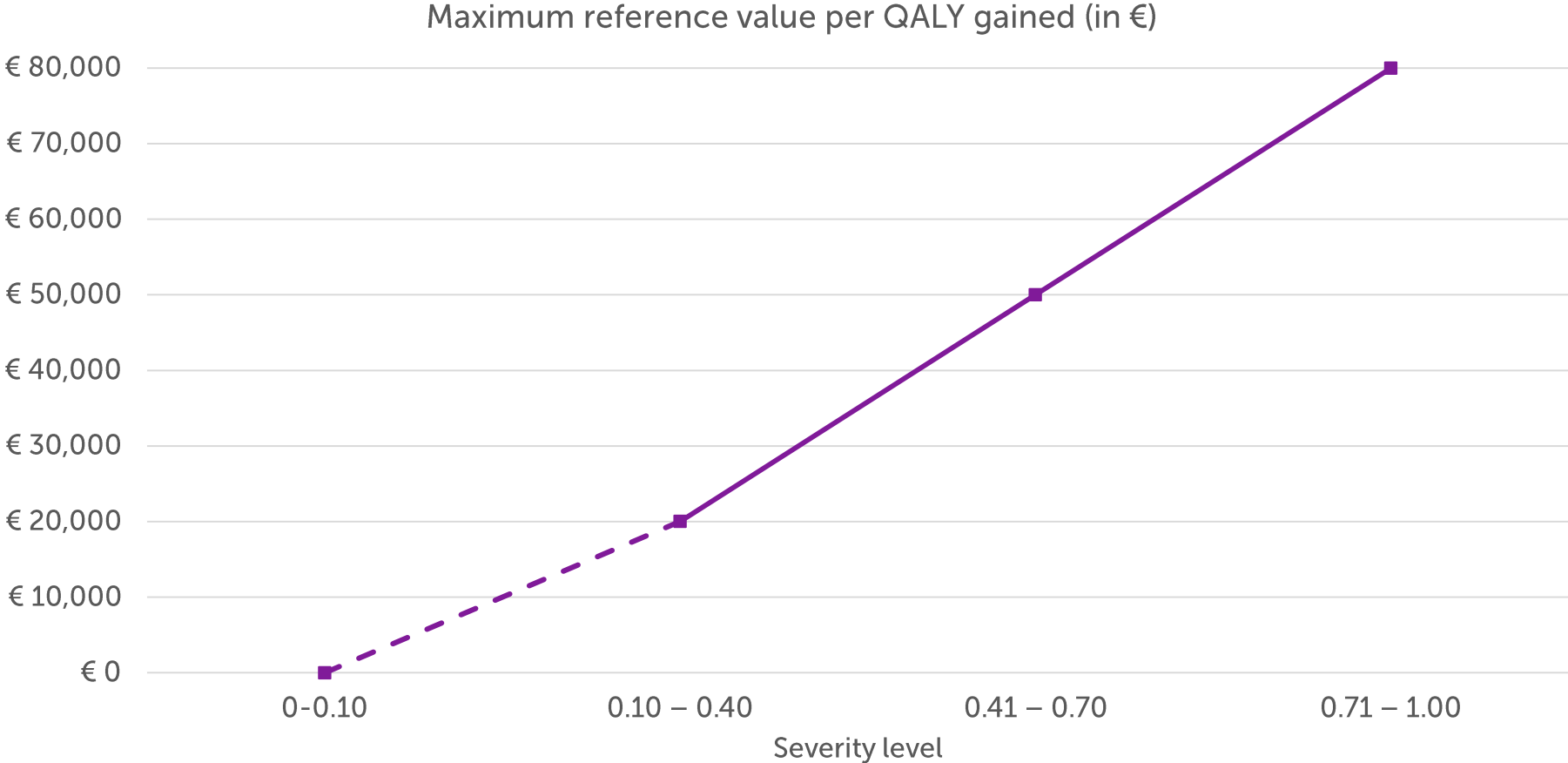


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Current decision framework



Severity-dependent WTP at different ages

		Age			
		10 years	20 years	40 years	70 years
Severity	10	€	€	€	€
	30	€	€	€	€
	50	€	€	€	€
	70	€	€	€	€
	90	€	€	€	€

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Severity-dependent WTP at different ages

		Age			
		10 years	20 years	40 years	70 years
Severity	10	€	€	€	€ LOWER
	30	€	€	€	€
	50	€	€	€	€
	70	€	€	€	€
	90	€ HIGHER	€	€	€

Hypothesis:

- Higher willingness to pay for relatively more severely ill and younger patients.



Future directions

- Severity and age may both be important, but age may be more important

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Future directions

- Severity and age may both be important, but age may be more important
- Proportional shortfall or reference values may need to be adjusted to account for age-related societal preferences in society or to avoid ageism

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Future directions

- Severity and age may both be important, but age may be more important
- Proportional shortfall or reference values may need to be adjusted to account for age-related societal preferences in society or to avoid ageism
- If severity is not 'it', what else may be relevant? Rarity of diseases?
Prioritising patients at the end of life?

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Future directions

- Severity and age may both be important, but age may be more important
- Proportional shortfall or reference values may need to be adjusted to account for age-related societal preferences in society or to avoid ageism
- If severity is not 'it', what else may be relevant? Rarity of diseases?
Prioritising patients at the end of life?
- How to account for uncertainty in severity estimates that may impact the outcomes of reimbursement decisions?

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Calculating the SAPCE

Versteegh et al. (2019) published a method and developed a tool for calculating the severity-adjusted probability of being cost effective.

By integrating:

- Uncertainty associated with patients' QALE (obtained from PSA)
- Uncertainty associated with remaining QALE in absence of disease (based on age- and sex-adjusted population QALE)

And:

- Obtaining a distribution for (absolute and/or) proportional shortfall
- Calculating the probability a new technology is cost-effective given the different reference values that may apply

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Table 1: Example calculation of the severity-adjusted probability of being cost-effectiveness

Model run	Disease burden calculation					Model results			Combined results	
	Patient QALE (Qd)	Population QALE ^a (Qn)	AS (Qn - Qd)	PS ((Qn - Qd) / Qn)	Applicable threshold in € (Vs)	Incremental costs in € (ΔC)	Incremental benefits (ΔQ)	ICER in € (ΔC/ΔQ)	INMB in € (ΔQ*Vs - ΔC)	Cost-effective ^b
1	15	25	10	0.40	20,000	20,000	0.60	33,333	-8,000.00	0
2	16	24	8	0.33	20,000	8,000	0.50	16,000	2,000.00	1
3	17	28	11	0.39	20,000	15,000	0.60	25,000	-3,000.00	0
4	15	28	13	0.46	50,000	10,000	0.50	20,000	15,000.00	1
5	14	27	13	0.48	50,000	10,000	0.40	25,000	10,000.00	1
6	13	26	13	0.50	50,000	25,000	0.30	83,333	-10,000.00	0
7	15	26	11	0.42	20,000	25,000	0.60	41,667	-13,000.00	0
8	15	32	17	0.53	50,000	15,000	0.50	30,000	10,000.00	1
9	16	25	9	0.36	20,000	25,000	0.60	41,667	-13,000.00	0
10	16	26	10	0.38	20,000	20,000	0.80	25,000	-4,000.00	0

Severity-adjusted probability of being cost-effective

40%

ICER, incremental cost-effectiveness ratio, INMB = incremental net monetary benefit; QALE, quality-adjusted life expectancy; ^a Population QALE is age and sex specific; ^b 1 = Yes.

From: Versteegh MM, Ramos IC, Buyukkaramikli NC, Ansari pour A, Reckers-Droog VT, Brouwer WBF. Severity-Adjusted Probability of Being Cost Effective. *Pharmacoeconomics* 2019;1-9.



iMTA Disease Burden Calculator

iDBC tool (R based) available for:

- The Netherlands, Norway, USA, Spain, Germany, and the UK
- (Free) download from iMTAs website: <https://imta.shinyapps.io/iDBC/>

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Want to discuss further?
Contact me

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Additional slides

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Proportional shortfall – Calculations

Different calculations in context of (strong) heterogeneity, episodic disease course, and prevention.

Heterogeneity:

- Calculated as a weighted average

Episodic course:

- Calculated and presented per subgroup *during* episode
- Representative of shortfall during episode, but total shortfall is overestimated due to exclusion of disease-free period

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Proportional shortfall – Calculations (2)

Prevention:

- Moment of treatment
- Subgroup of patients who actually fall ill

Rationale:

- Illustrates the sense of urgency/necessity of preventive treatment
- Avoids differences between patients who receive preventive or curative care for the same disease
- Avoids 'double penalty' as relatively higher costs and lower average proportional shortfall would lead to relatively less favourable ICERs for preventive treatments
- Better aligned with objective to prioritise the more severely ill

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