Looking back and moving forward: On the application of proportional shortfall in healthcare priority setting in the Netherlands

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ABSTRACT

The increasing demand for healthcare and the resulting pressure on available budgets render priority setting inevitable. If societies aim to improve health and distribute health(care) fairly, equity-efficiency trade-offs are necessary. In the Netherlands, proportional shortfall (PS) was introduced to quantify necessity of care, allowing a direct equity-efficiency trade-off. This study describes the history and application of PS in the Netherlands and examines the theoretical and empirical support for PS as well as its current role in healthcare decision making. We reviewed the international literature on PS from 2001 onwards, along with publicly accessible meeting reports from the Dutch appraisal committee, Adviescommissie Pakket (ACP), from 2013 to 2016. Our results indicate that there is support for the decision model in which necessity is quantified and incremental cost-effectiveness ratios are evaluated against associated monetary reference values. The model enables a uniform framework for priority setting across all healthcare sectors. Although consensus about the application of PS has not yet been reached and alternative ways to quantify necessity were found in ACP reports, PS has increasingly been applied in decision making since 2015. However, empirical support for PS is limited and it may insufficiently reflect societal preferences regarding age and reducing lifetime-health inequalities. Hence, further investigation into refining PS—or exploration of another approach—appears warranted for operationalising the equity-efficiency trade-off.

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1. Introduction

The demand for healthcare is rapidly increasing for reasons that include ageing populations and the availability of increasingly advanced and expensive (new) health technologies. As healthcare resources remain scarce, the resulting pressure on available budgets renders healthcare priority setting inevitable [1,2]. Although politically and societally sensitive, the need for prioritisation is widely recognised and explicitly addressing priority setting has become indispensable for developing fairer methods for resource allocation in healthcare [3,4].

Economic evaluations of health technologies are often used to inform decision makers regarding how to allocate healthcare resources in an optimal way for society. However, the outcomes of economic evaluations only predict such decisions to a moderate extent [3,5,6]. One explanation for this disparity is that decision makers are not exclusively concerned with maximising health given available budgets, but also with distributing health(care) equitably and fairly [3,5,7,8]. Hence, an optimal allocation of resources involves setting priorities that contribute to both efficiency and equity in the distribution of health(care) [9]. Recognising that these are both important objectives of healthcare systems, it has been advocated that societal concerns for equity be explicitly and transparently incorporated into the decision-making framework [10–12].

In economic evaluations, the value of a health technology is commonly expressed in terms of an incremental cost per quality-adjusted life-year (QALY) ratio (ICER) that is evaluated against some monetary threshold value per QALY gained [3,13–15]. When the ICER is below this threshold, a health technology is considered cost-effective and eligible for reimbursement [16]. The classic approach in the economic-evaluation framework is to value QALY gains equally, i.e. to adhere to the principle that “a QALY is a QALY”, regardless of beneficiary and health technology characteristics [17]. However, this approach has been highly debated as it relies on the assumption of distributive neutrality [3]. In response to this debate, two general approaches have been suggested for operationalising the equity-efficiency trade-off [3,5]. One of these

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approaches applies equity weights to QALY gains and evaluates the adjusted ICER against a fixed monetary threshold value, and the other evaluates an unadjusted ICER against a flexible monetary threshold value [3,5,16]. Ideally, the operationalisation of the equity-efficiency trade-off is both normatively justifiable and empirically supported. However, this proves to be neither easy nor straightforward [3,18].

In relation to the operationalisation of the equity-efficiency trade-off, the severity of illness (SOI) and fair innings (FI) equity approaches have attracted much attention internationally. According to the normative theories about distributive justice that underlie these approaches, priority should be given to those who are worse off in terms of health [11,19,20]. However, the approaches are based on different normative arguments with regard to whom is considered worse off, and hence differ with regard to how they are operationalised [3,19]. A common operationalisation of SOI aims to equalise absolute health benefits in terms of current and prospective health, while FI aims to do so in terms of lifetime health [3,10,11,19]. As such, FI also considers past health [11,19]. Although both SOI and FI are to some extent normatively justifiable and empirically supported, neither of these approaches appears to satisfactorily reflect societal preferences for equity [3,5,7]. Nonetheless, different countries have either implicitly or explicitly developed normative principles or guidelines that include (aspects of) SOI or FI for informing allocation decisions in healthcare [3,5,7]. For example, in the United Kingdom (UK), the National Institute for Health and Care Excellence (NICE) formalised the SOI approach by launching guidelines for prioritising end-of-life care [21,22], while in Norway, the SOI approach is currently formalised in terms of absolute shortfall [23,24]. In an attempt to balance societal concerns regarding SOI and FI [5], proportional shortfall (PS) was introduced in the Netherlands as an equity approach that combines aspects of SOI and FI [3,5]. Although consensus about the application of PS has not yet been reached [25], the approach received considerable support from politicians and policy makers and was incorporated into the assessment phase of healthcare priority setting in the Netherlands [1,3]. As such, the Netherlands is one of the first countries to explicate the equity criterion in this context [3,5].

This study describes the history and application of PS in the Netherlands and examines the theoretical and empirical support for PS as well as its current role in healthcare decision making in the Netherlands by reviewing the international PS literature and publicly accessible meeting reports from the Dutch appraisal committee, the Adviescommissie Pakket (ACP). Although this study primarily focuses on healthcare priority setting in the Netherlands, the results of the study may also be useful for other countries seeking to operationalise the equity-efficiency trade-off for informing allocation decisions in healthcare.

2. A brief history of healthcare priority setting in the Netherlands

The report “Choices in health care” that was presented by the Dunning Committee in 1991 [26] was a landmark publication on healthcare priority setting in the Netherlands. In this report, four criteria for priority setting were formulated: necessity, effectiveness and efficiency of care, and patients’ individual responsibility for (paying for) care. In this report, the Dunning Committee used the metaphor of a funnel to describe a criteria-based decision model for evaluating the composition of the publicly funded health-insurance package. Based on this hierarchical model, technologies that (would subsequently) pass all criteria were to be included in the basic benefits package. The report was pivotal for the discussion on priority setting, and in the following years, the criteria were put into practice [26,27]. The Dutch Health Care Institute (ZIN) later reformulated these criteria as necessity of care, effectiveness, cost-effectiveness, and necessity of insurance, respectively, and supplemented these with a feasibility criterion [1,28].

Although none of the criteria were defined and operationalised without dispute, this proved to be particularly difficult for the necessity of care criterion [3,29]. The Dunning Committee defined necessity of care as care that is necessary for the prevention of premature death and/or for patients who—due to some disease or condition—cannot function normally in society [2,26]. The latter part of this definition was regarded as problematic, as it was unclear how to interpret and quantify ‘normal’ functioning. Moreover, the term ‘necessity’ implied an absolute rather than a relative cut-off point for decision making, which was amplified by the Dunning Committee’s use of a funnel metaphor [2,26]. If a technology failed to pass the ‘sieve of necessity’, the technology would not be incorporated into the public health-insurance package, and assessment of its (cost-)effectiveness and need for insurance would be superfluous [2]. However, as the degree to which health technologies are necessary varies, it was suggested that this criterion be regarded as neither absolute nor isolated from the other criteria [2,30,31].

In 2001, Stolk et al. [2] proposed a decision model in which necessity of care was defined as ‘burden of illness’ (BOI) and operationalised as a relative criterion by attaching a higher necessity score to health technologies that target diseases with a higher BOI level. Stolk et al. [2] described BOI as the average disease-related loss in quality and length of life of patients, relative to the situation in which the disease had been absent and quantified BOI in terms of QALYs on a 0–1 scale. Furthermore, they proposed connecting the necessity of care and (cost-)effectiveness criteria by attaching a higher societal willingness to pay (WTP) per QALY gained to a higher level of BOI. Specifically, the authors suggested dividing the continuous 0–1 BOI scale into seven categories and evaluating the ICER of (new) health technologies against seven associated monetary threshold values per QALY gained. The proposed cost-effectiveness threshold values per QALY gained ranged from approximately €4500 to €45,000 [32]. Deciding on the exact cut-off points for the BOI categories, the cost-effectiveness threshold range, and the shape of their reciprocal relationship were regarded as matters of political and societal concern.

The proposed model received broad support as it contributed to the development of a transparent and coherent decision model for healthcare priority setting in the Netherlands by explicitly connecting the criteria formulated by the Dunning Committee and enabling a uniform and systematic quantification of BOI across patient groups and disease areas [23,27–29]. Between 2002 and 2005, BOI was further formalised as proportional shortfall (PS) and defined as a principle that is based on the normative standpoint that priority in healthcare should be given to those who, due to some disease and if left untreated, lose the largest proportion of their QALY expectancy in absence of the disease [3,30,31]. PS is measured on a scale from 0 (no QALY loss) to 1 (complete loss of remaining QALY, i.e. immediate death), by applying:

\[
PS = \frac{\text{Remaining QALY expectation in absence of the disease}}{\text{Remaining QALY expectation in absence of the disease}}
\]

For example, a disease that results in the loss of 30 out of 60 remaining QALYs has a PS level of 0.5 (30/60), and a disease that results in the loss of 60 out of 80 remaining QALYs has a PS level of 0.75 (60/80). The remaining QALY expectation in absence of the disease can be calculated from age- and gender-specific mortality data [25]. Eq. (1) can be rewritten as:

\[
PS = 1 - \frac{\text{Expected QALYs without treatment}}{\text{Remaining QALY expectation in absence of the disease}}
\]
Applying Eq. (2) to the previous example, the PS level of 0.5 is calculated as 1 – (20/60), and the PS level of 0.75 is calculated as 1 – (20/80). PS can also be calculated by using the number of expected QALYs ‘with current treatment’ rather than ‘without treatment’ in the equations [34]. This may be a more logical calculation of PS as it arguably uses a more relevant comparator and hence agrees with the economic-evaluation methodology. However, it should be noted that calculating PS relative to the current treatment will likely lead to a different, specifically lower, PS level for the same beneficiaried and (new) health technologies. Consequently, the outcome of a reimbursement decision that is informed by a PS level that is calculated relative to the current treatment may be different for the same beneficiaries and (new) health technologies than when the decision is informed by a PS level that is calculated relative to having no treatment. The debate on the preferred comparator is likely to continue in the coming period.

While consensus concerning the definition and operationalisation of BOI gradually increased, its exact categories and the associated cost-effectiveness threshold range remained a subject of discussion for some time. In 2006, the Council for Public Health and Society (RZV) suggested a continuous, upward-sloping curve with a maximum reimbursement of €80,000/QALY [29]. This figure was substantiated by the World Health Organisation (WHO) rule of thumb that less than three times the GDP per capita per disability-adjusted life-year (DALY) averted indicated good value for money for a health technology [35], by the finding that most reimbursed health technologies in the UK had an ICER of approximately €79,000/QALY [36], and by estimations of the value of a statistical life [37,38]. Although the figure of €80,000/QALY may have been set somewhat arbitrarily, it was considered “reasonable” [29,39]. Moreover, even though €80,000/QALY was not officially adopted as the threshold value at that time, it was influential and provided the basis for ZIN to set three BOI categories with a maximum reimbursement of €80,000/QALY for the highest BOI category in 2015 [25]. Table 1 presents these three BOI categories and the associated monetary reference values and shows that a higher WTP per QALY gained, i.e. a higher necessity score, is attached to health technologies that target diseases with a higher BOI level [2,25]. For example, the ICER of a health technology that targets a disease with a PS level of 0.5 is evaluated against a reference value of €50,000/QALY, while the ICER of a health technology that targets a disease with a PS level of 0.75 is evaluated against a reference value of €80,000/QALY. A health technology that targets a disease with a PS level below 0.1, in principle, not considered for reimbursement. Hence, this category is not included in the table [25,29]. Whether it is feasible, in practice, to not reimburse a health technology that targets a disease with a low PS level remains doubtful [40]. For example, episodic illnesses like migraine may not lead to a high average PS, but do represent substantial shortfall during the episode.

Given the maximum reimbursement of €80,000/QALY for the highest BOI category and the intention to associate increasing levels of BOI with increasing monetary reference values, ZIN set the two lower thresholds at €20,000 and €50,000 per QALY. Together these may be seen as forming a logical set of values, given the endpoint of €80,000/QALY in relation to the highest BOI. In relation to the other two values, ZIN also referred to the threshold value that is applied to national immunisation and preventive care programmes in the Netherlands (€20,000/QALY threshold) and to a Dutch study on the societal WTP per QALY gained ‘in others’ (€50,000/QALY) [25,35]. ZIN advised reassessing the reference values every five to ten years [25] and to not use them as strict cut-off values, but rather as references for the Dutch government when conducting price negotiations with pharmaceutical companies and for the ACP when recommending incorporation of health technologies into the public health-insurance package.

The model, in which BOI is quantified and the ICERs of health technologies are evaluated against associated reference values, enables a transparent and coherent decision-making framework. Given that this model is increasingly applied in the Netherlands, the question arises whether there actually is sufficient support for the operationalisation of BOI in terms of PS to explicate the equity criterion. In the next two sections we will discuss the theoretical and empirical support for using PS to inform priority setting in healthcare. In the subsequent section we will review the current role of PS in healthcare decision making in the Netherlands.

3. Is there theoretical support for proportional shortfall?

In order to optimally allocate healthcare resources for society, it has been advocated that societal concerns regarding equity be incorporated in the decision-making framework [10–12]. However, what society considers to be equitable and fair for priority setting is a normative question that different people in different contexts may answer differently. Hence, when operationalising the equity-efficiency trade-off, an additional trade-off between different societal concerns regarding equity must be made. Consequently, increasing equality in the distribution of health(care) by applying one equity approach may lead to increasing inequality in the context of applying another [3,41]. It has also been argued that, when operationalising the equity-efficiency trade-off, different operationalisations are bound to face corresponding difficulties [3,16]. For example, in the context of curative healthcare, questions may arise concerning the handling of episodic diseases and the quantification of related health benefits [3,16]. In the context of preventive healthcare, questions may arise concerning the group of beneficiaries and the timeframe that is regarded as relevant for estimating health benefits [3,16,18]. For example, should PS be calculated for all treated persons or only those for whom the illness was prevented? And should PS be calculated from the time of the preventive treatment or from the time the prevented illness would otherwise have occurred? Such choices can have a profound effect on the outcomes of PS calculations [42]. Other questions may, for example, arise concerning the use of age- and gender-specific mortality data as a reference point or threshold for calculating PS [20,43,44]. The use of such different reference points for different (age and gender) groups implies that there is not one age or health expectancy that would serve as a normative reference level for all groups. Hence, this could be regarded as including some inequ(ali)ties in the calculation of PS [20]. These issues illustrate that not only is the choice of an equity approach normative, but additional normative choices must be made when applying the chosen equity approach in practice [3]. Inevitably, these choices have a large impact on PS calculations and therefore may have distributional consequences [3,18]. Although some initial choices were made when operationalising PS in the Netherlands [1,2,31], it should be noted that the discussion about how best to solve these issues is ongoing (both in the context of healthcare priority setting in the Netherlands and internationally).

SOI and FI are two renowned equity approaches that are based on different normative arguments regarding whom is considered worse off in terms of health [3,19]. As described earlier, SOI com-

Table 1
Maximum reference values (in €) per QALY gained [25].

<table>
<thead>
<tr>
<th>Burden of illness</th>
<th>Maximum reference value per QALY gained</th>
</tr>
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<tbody>
<tr>
<td>0.1–0.40</td>
<td>€20,000</td>
</tr>
<tr>
<td>0.41–0.70</td>
<td>€50,000</td>
</tr>
<tr>
<td>0.71–1.00</td>
<td>€80,000</td>
</tr>
</tbody>
</table>
monly aims to equalise health benefits in terms of current and prospective health, and FI aims to do so in terms of lifetime health [3,10,11,19]. As such, FI is consistent with the notion that, all else equal, younger people should be prioritised over older people as they have not yet enjoyed a fair share of lifetime health [5,11]. It should be noted that the role of age is merely indirect in the FI approach as it is applied as a proxy for lifetime health [20,43,44]. Indeed, in the FI approach, age itself is not regarded as a morally relevant argument for priority setting [44].

PS does not originate from a unique theory about distributive justice but was developed as an equity approach that combines aspects of SOI and FI by prioritising those who are worse off in terms of a lower amount of prospective and lifetime health [3,5]. While SOI and FI aim to equalise absolute health benefits, PS aims to equalise relative benefits between persons with respect to their potential for health [5,33]. It has been argued that PS balances societal concerns regarding SOI and FI and treats the two approaches as equally important [5]. However, PS is calculated as the fraction of disease-related QALY loss relative to the remaining QALY expectation in absence of the disease rather than to the lifetime-QALY expectation from birth. Various authors have discussed the relative nature of PS and the theoretical and empirical relevance of using a lifetime perspective for informing allocation decisions in healthcare [20,24,45–47]. Here, we would like to point out that PS may be viewed as placing more emphasis on relative prospective-health loss, i.e. the SOI component of PS, than on relative lifetime-health loss, i.e. the FI component of PS. This is illustrated by the fact that PS does not, by definition, discriminate between people with different levels of ‘enjoyed’ lifetime health as healthcare beneficiaries of all ages could potentially experience the same level of PS. For example, in the case of immediate death, healthcare beneficiaries who are 10 and 80 years old are given the same weight in the distribution of healthcare, as both will have a PS level of 1. However, when the same beneficiaries lose two of their remaining QALYs, more weight will be given to the 80-year old, as their PS level will be higher than that of the 10-year old. Indeed, in allocation decisions, PS may more frequently give a higher weight to older patients than the FI approach would. Stolk et al. [5] argued that the FI approach “discriminates against the elderly more strongly than policy makers seem to prefer” and that PS could mitigate the ageism that is implied by the FI approach. It was, therefore, hypothesised that PS might be better aligned with distributional preferences of health policy makers. Should this hypothesis not be supported by empirical evidence, the authors suggested to add age weights and adjust PS for age-related preferences.

A strength of PS, which it shares with the SOI and FI approaches, lies in its quantification of health losses in terms of QALYs. This enables the application of PS across disease areas and patient populations. However, this strength comes with a limitation as treatment benefits beyond health and health-related quality of life (QOL) that may not be captured by the QALYs are increasingly recognised as being relevant [48]. Therefore, the current application of PS, i.e. its quantification in terms of QALYs, may be regarded as appropriate for informing decisions concerning curative and preventive treatments but less so for decisions concerning treatments that focus on broader benefits, for example related to wellbeing [49]. If the aim is to generate social welfare from the public health insurance package, the application of an equity approach that is uniformly applicable and hence that models information concerning health, QOL, and broader wellbeing could be preferable for informing decisions concerning all healthcare sectors. We stress that this limitation should not be attributed to PS (or to FI or SOI) as a principle but rather to the way in which PS is currently quantified and applied in decision-making practice. In fact, PS does enable a uniform decision model for priority setting across all healthcare sectors, as the QALY in the PS equation can be replaced with—or complemented by—any other (generic) outcome measure of choice.

Table 2

<table>
<thead>
<tr>
<th>Study</th>
<th>Year</th>
<th>Country</th>
<th>Design</th>
<th>Mode of administration</th>
<th>N</th>
<th>Sample</th>
<th>Support for PS</th>
</tr>
</thead>
<tbody>
<tr>
<td>Stolk et al. [33]</td>
<td>2005</td>
<td>NL</td>
<td>Ranking exercise</td>
<td>Web-based survey</td>
<td>65</td>
<td>Convenience</td>
<td>++</td>
</tr>
<tr>
<td>Olsen [50]</td>
<td>2013</td>
<td>Norway</td>
<td>Pairwise-choice task</td>
<td>Web-based survey</td>
<td>503</td>
<td>General public (age and gender)</td>
<td>--</td>
</tr>
<tr>
<td>Brazier et al. [51]</td>
<td>2013</td>
<td>UK</td>
<td>DCE</td>
<td>Web-based survey</td>
<td>3669</td>
<td>General public (age and gender)</td>
<td>--</td>
</tr>
<tr>
<td>Van de Wetering et al. [53]</td>
<td>2015</td>
<td>NL</td>
<td>DCE</td>
<td>Web-based survey</td>
<td>1205</td>
<td>General public (age, gender, and education level)</td>
<td>--</td>
</tr>
<tr>
<td>Bobinac et al. [54]</td>
<td>2015</td>
<td>NL</td>
<td>WTP</td>
<td>Web-based survey</td>
<td>1320</td>
<td>General public (age, gender, and education level)</td>
<td>--</td>
</tr>
<tr>
<td>Rowen et al. [52]</td>
<td>2016</td>
<td>UK</td>
<td>DCE</td>
<td>Web-based survey</td>
<td>3669</td>
<td>General public (age and gender)</td>
<td>+</td>
</tr>
<tr>
<td>Richardson et al. [55]</td>
<td>2017</td>
<td>Australia</td>
<td>CSIC task</td>
<td>Web-based survey</td>
<td>606</td>
<td>General public (age)</td>
<td>+</td>
</tr>
</tbody>
</table>

Notes: CSIC = constant sum paired comparison; DCE = discrete choice experiment; WTP = willingness-to-pay study; PS = proportional shortfall.

- Level of support for PS indicated by: ++ = no; + = limited; ++ = modest; +++ = strong.
- Olsen [50] and Richardson et al. [55] examined support for PS in the context of preferences for length of life.
- Rowen et al. [52] examined support for PS relative to the current treatment.

4. Is there empirical support for proportional shortfall?

We examined empirical support for PS by reviewing the international literature on PS in the context of healthcare priority setting. We used the search terms “proportional shortfall”, “preference”, “elicitation”, “priority setting”, and “health” or “healthcare” in Google Scholar. The search was performed on October 16, 2017 and supplemented with a hand search. We restricted the search to articles written in English or Dutch, published between 2001, i.e. the year in which PS was introduced in the Netherlands, and 2017, and of which the full text was available. Articles were selected for review if the aim of the study was to elicit preferences for PS relative to either preferences for no equity weighting or for weighting on the basis of another equity approach, such as SOI and/or FI. Our search resulted in 205 studies, in seven of which preferences for PS were elicited. Table 2 presents an overview of these seven studies and their results.

Stolk et al. [33] compared support for SOI, FI, and PS by asking respondents to assign a priority rank to the treatment of ten health conditions. Stolk et al. found strong evidence for PS being consistent with social preferences for healthcare priority setting. Although preferences for PS dominated preferences for SOI, stronger support was found for FI. The authors obtained these results using a small convenience sample in the Netherlands that consisted of health policy makers, researchers, and students. Consequently, the results may be prone to bias, e.g. due to respondents sharing common opinions.

Olsen [50] examined support for PS in a sample that was representative, in terms of age and gender, of the general adult population in Norway in terms of age and gender. Olsen applied a pairwise-choice task and asked respondents to prioritise patients based on their age, remaining lifetime health without treatment, and increase in remaining lifetime health with treatment. Olsen

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found strong support for the FI approach; however, he found no support for PS.

Brazier et al. [51] examined support for BOI operationalised in terms of PS in a sample that was representative of the general population in the UK in terms of age and gender by performing a web-based discrete choice experiment (DCE). Their main results did not support PS. However, when respondents who seemingly misunderstood the DCE task were excluded, some support for PS was found.

Rowen et al. [52] used the data from Brazier et al. [51] to examine support for PS by applying the number of expected QALYs ‘with current treatment’ rather than ‘without treatment’ in the PS equation. Rowen et al. concluded that, although the results were not robust against different versions of the DCE survey, there was some modest support for BOI operationalised in terms of PS relative to the current treatment.

Van de Wetering et al. [53] examined support for PS in a sample that was representative of the general adult population in the Netherlands in terms of age, gender, and education level by conducting a web-based DCE. They found substantial preference heterogeneity and some counterintuitive results, as respondents were less likely to prioritise patients with higher levels of PS.

Bobinac et al. [54] examined societal WTP for QALY gains in patients with different levels of PS in a sample that was representative of the general adult population in the Netherlands in terms of age, gender, and education level by conducting a web-based survey. They found occasional support for PS as a predictor of the WTP for QALY gains. Some support for PS was found when QALY gains were relatively small. However, the level of support decreased when QALY gains increased in size. In addition, support for PS was generally dominated by concerns for the (younger) age of patients.

Richardson et al. [55] examined support for PS in a sample that was close to being representative of the general adult population in Australia in terms of age. They applied constant-sum paired comparison tasks and asked respondents to prioritise patients based on their gain in life years due to treatment, age, years to death without treatment, and age at death with and without treatment. Their study found some support for PS; however, found that concerns for PS were dominated by concerns for the (individual) SOI and FI approaches. Richardson et al. further found that PS insufficiently reflects respondents’ age-related preferences.

Although each of these studies examined societal support by eliciting preferences for PS, it is important to note that the studies differ with respect to the samples, methods, additionally included variables, and/or equity approaches. In addition, Olsen [50] and Richardson et al. [55] examined preferences for equity in the context of length of life, and hence did not present PS in terms of proportional QALY shortfall. Consequently, a direct comparison of the results presented in Table 2 is not possible.

Table 3
Search terms used for reviewing ACP meeting reports.

<table>
<thead>
<tr>
<th>Domain</th>
<th>Search term</th>
</tr>
</thead>
<tbody>
<tr>
<td>Priority-setting criteria</td>
<td>Necessity of care</td>
</tr>
<tr>
<td></td>
<td>Necessity of insurance</td>
</tr>
<tr>
<td></td>
<td>Effectiveness; Effect</td>
</tr>
<tr>
<td></td>
<td>Cost-effectiveness; Efficiency</td>
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<tr>
<td></td>
<td>Feasibility</td>
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<tr>
<td>Equity considerations</td>
<td>Severity of illness</td>
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<td></td>
<td>Fair incomes</td>
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<td></td>
<td>Burden of illness</td>
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<tr>
<td></td>
<td>Absolute shortfall</td>
</tr>
<tr>
<td>Treatment benefits</td>
<td>Proportional shortfall</td>
</tr>
<tr>
<td></td>
<td>Therapeutic outcome; Therapeutic value; Health-related quality of life</td>
</tr>
<tr>
<td></td>
<td>Quality-adjusted life-year; QALY</td>
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<tr>
<td></td>
<td>Wellbeing</td>
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<td></td>
<td>Capability</td>
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<td></td>
<td>Life satisfaction</td>
</tr>
<tr>
<td>Patient characteristics</td>
<td>Age</td>
</tr>
<tr>
<td></td>
<td>Socio-economic status; SES</td>
</tr>
<tr>
<td></td>
<td>Lifestyle</td>
</tr>
<tr>
<td></td>
<td>Culpability; Individual responsibility</td>
</tr>
</tbody>
</table>

Reference values

<table>
<thead>
<tr>
<th>Reference value(s)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Monetary/Threshold</td>
</tr>
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5. What is the role of proportional shortfall in healthcare decision making?

The necessity (of care and of insurance), effectiveness, cost-effectiveness, and feasibility criteria are addressed and quantified in the assessment phase of healthcare decision making in the Netherlands and subsequently assessed on social and ethical grounds in the appraisal phase. If the operationalisation of BOI in terms of PS is considered suboptimal for explicating the equity criterion, it seems reasonable to expect that this would be explicitly discussed during meetings of the ACP appraisal committee.

To examine the current role of PS in the appraisal phase of healthcare decision in the Netherlands, we conducted a review of publicly accessible ACP meeting reports that were published between January 1, 2013 and December 31, 2016. The reports include agendas, minutes, and documents, including decision reports and draft ZiN reports that were discussed by the ACP. Table 3 presents the terms (and their domains) addressing healthcare priority setting that we used for searching the reports (in the Dutch language, but translated here for clarity). Reports that did not allow a digital search, including ACP reports that were published before January 1, 2013, were excluded from the review, as were search terms that occurred in the names of health organisations and government ministries. Draft versions of minutes were included only if final minutes were not published.

Between 2013 and 2016, 179 ACP reports were published of which two were excluded for not allowing a digital search. Table 4 presents the frequency with which the search terms were identified in the remaining 177 reports. The necessity of care and of insurance, effectiveness, cost-effectiveness (including the search term efficiency), and feasibility criteria were identified 1680, 495, 8700, 4423, and 236 times, respectively. The effectiveness criterion was most frequently found, followed by the cost-effectiveness and necessity of care criteria. The necessity of insurance and feasibility criteria were identified less frequently.

PS was identified 14 times in a total of six reports, four of which discussed the operationalisation of BOI in terms of PS. In a report from 2013, ZiN described the definition and calculation of PS. In this report, ZiN stated that “PS was developed at a time when ageism was an important issue in the allocation of healthcare resources” and that “therefore BOI is calculated in proportion to life expectation, which ensures that PS does not distinguish between younger and older people”. However, ZiN also stated that “recently, there are increasing indications that people do discriminate between age groups” and that people “value health gains in younger people more than in older people”, which “argues against PS and the rule of rescue, and in favour of FI”. In the accompanying minutes, an ACP member stated that “the passage about BOI is still not in agreement with what was discussed in previous meetings” and that s/he “understand[s] that applying the capability approach is out of reach”, but that s/he “would like to see the denominator removed from the presented definition of PS”. In a report from 2015, ZiN stated that “because we have not yet reached consensus about the quantification of BOI, we will temporarily […] quantify BOI in terms of DALYs”. In this report, ZiN additionally stated that “priority will be given to solving this issue” and that “a report on the quantification of BOI will be issued this summer”. In a later
The reference of one of the ACP members to Sen’s capability approach [41] may indicate a preference for quantifying health benefits in terms of broader wellbeing, as for example is done by applying the ICECAP measure [56]. Wellbeing, including the terms capability and life satisfaction, was identified 93 times in 29 reports from 2013 onwards, among which the capability approach was identified 15 times in five reports (not in Table 4). In these reports, the capability approach was discussed as an alternative to quantifying health benefits in terms of QALYs. In a report from 2013, ZIN stated that “a recent discussion involves the question of whether the capability approach is better aligned with the social basis that underlies managing the public health-insurance package” and that “applying this approach may be more appropriate for healthcare sectors where ‘health gains’ are not the primary objective, such as long-term care and mental healthcare”. The same report stated that “changing the desired outcome of healthcare does not answer the question of when care is more necessary for one person than for another” and that “the capabilities approach can also be applied to calculate lifetime capabilities (fair innings), prospective capabilities, or the relative loss of capabilities (proportional shortfall)”, and so “applying the capability approach will not solve the issue of prioritisation in healthcare”.

The ACP members’ request to remove the denominator from the PS equation may indicate a preference for operationalising the equity criterion in terms of absolute shortfall (AS) rather than proportional shortfall, and this may in turn indicate a preference regarding FI, age, and reducing lifetime-health inequalities [23,50]. AS was not identified in any of the ACP reports and the FI approach was identified 11 times in four reports. In contrast, the SOI approach was identified 0 times. However, concerns for SOI that were expressed through concerns for prospective-health loss, the rule of rescue, and for the severity of (symptoms of) a disease or condition were identified 3 times in 1 report, 5 times in 3 reports, and 2614 in 92 reports, respectively (not in table). Age was identified 895 times in 79 reports. Regarding age and other patient characteristics, age was identified 16.6 and 2.3 times more frequently than SES and lifestyle (including the search terms culpability and individual responsibility), respectively.

Although the operationalisation of BOI in terms of PS was occasionally discussed in some reports, and in one report from 2014 an ACP member stated that “BOI cannot be quantified in numeric terms in this specific situation”, the application of BOI itself was not discussed. BOI was identified 672 times in 68 reports, and from 2015 onwards, increasingly related to the corresponding reference values (presented in Table 1), which were identified 328 times in 26 reports. In 2013, BOI was most frequently expressed in qualitative terms, e.g. in terms of “low” or “high” BOI, and only sometimes in numeric terms, by disability weights used for calculating DALYS. From 2014 onwards, BOI was less frequently expressed in qualitative terms and was mostly quantified by disability weights or the number of DALYS lost, which at times were presented alongside the mean life expectancy of patients with and without the disease. In three reports, BOI was addressed as a relative measure; however, the presented disability weights or DALYS lost were not applied as such. From 2015 onwards, BOI was most frequently quantified in terms of PS, in a total of seven reports. In four of these reports, PS calculations were presented alongside disability weights, number of DALYS lost, mean life expectancy, or years of life lost calculations.

Based on these results, it appears that the application of BOI was not publically discussed by ACP members between 2013 and 2016. The operationalisation of BOI in terms of PS, and the role of PS in healthcare decision making, was infrequently discussed.
While BOI was most frequently expressed qualitatively in 2013, it was increasingly quantified in later years, usually in terms of disability weights or the number of DALYs lost. From 2015 onwards, ICERs were increasingly related to the monetary reference values per QALY gained that were set by Zin that year [25] and BOI was most frequently quantified in terms of PS. In this context, it needs noting that there was a change in ACP members in 2015 and this may have contributed to the increased application of PS from then on. In the reports, PS calculations were frequently presented alongside disability weights, number of DALY lost, life expectancy, and years of life lost calculations. This variety may reflect that there is no consensus (yet) about the application of PS in healthcare decision making in the Netherlands.

6. Discussion

The importance of operationalising the equity-efficiency trade-off for informing priority setting in healthcare is increasingly recognised. This study described the history and application of PS in the Netherlands, examined the theoretical and empirical support for PS as an operationalisation of the equity-efficiency trade-off, and looked into the current role of PS in healthcare decision making.

Overall, our results indicate general support for the decision model in which BOI is quantified and the ICERs of health technologies are evaluated against the reference values per QALY gained set by Zin. This model connects the criteria for healthcare decision making that were previously formulated by the Dunning Committee and enables a uniform decision model for priority setting across all healthcare sectors. Consequently, the model has received broad support in research and policy circles and has been incorporated into the healthcare decision-making framework in the Netherlands.

Although the model is increasingly applied in decision-making practice, the results of our literature review suggest that theoretical support for PS is moderate at best. In applying PS, a trade-off between the SOI and FL approaches is made and, consequently, societal preferences for either of the two equity approaches may be insufficiently reflected when allocating resources in healthcare. However, this may be regarded as a general limitation that comes with applying any equity approach in practice, as improving equality in the distribution of health/care by applying one equity approach may inevitably be associated with increasing inequality in the context of applying another [3]. A limitation that is not restricted, but may be more specific, to applying PS, is that it mitigates ageism between patient groups, as beneficiaries of all ages can experience the same level of PS. The results of our study suggest that this may inadequately reflect societal preferences relating to age and reducing lifetime-health inequalities between patient groups. Although the results of our literature review suggest that empirical support for PS is limited, it should be noted that that empirical evidence regarding PS so far is scarce and inconclusive, so that a rejection of the PS approach can also not be concluded. The societal concern regarding age that is currently insufficiently reflected by PS could be incorporated by adjusting PS for age. However, there is no empirical evidence (to date) to support the hypothesis that this would better align with societal preferences and hence future research on this topic will be necessary. The results of our review of publicly accessible ACP reports suggest that the ACP did not publicly discuss the definition and operationalisation of necessity of care in terms of BOI between 2013 and 2016. In fact, BOI became increasingly quantified, and ICERs were increasingly evaluated against the reference values per QALY gained set by Zin in 2015. The operationalisation of BOI in terms of PS was publicly discussed by the ACP, although only on rare occasions. This may indicate that the ACP supports the operationalisation of BOI in terms of PS. However, the variety of BOI quantifications in ACP reports demonstrates that consensus about the operationalisation and quantification of BOI has not yet been reached.

Relatively few studies have examined the theoretical and empirical support for PS, and to our knowledge, no other study has examined the current role of PS in healthcare decision making in the Netherlands. Although this limits our ability to compare our results with those of others, we would like to compare the results of our empirical literature review to the results of a study conducted by Nord and Johansen [57] and the public consultation of NICE on the valued-based assessment of health technologies [58–60]. Nord and Johansen [57] examined support for PS, relative to preferences for no other equity approach, by conducting an empirical literature review that built on an earlier review by Shah [61] and included 20 preference studies that were conducted in nine different countries between 1991 and 2011. Under the assumption of a stable health condition and no loss in length of life for patients, Nord and Johansen found strong support for PS, although the strength of the support varied greatly between the included studies. Regarding the inclusion criteria for our literature review, five of the studies that Nord and Johansen included were conducted after the introduction of PS in the Netherlands. Of these five studies, two quantified health benefits in terms of QOL and three in terms of QALYs. However, none of the three latter studies elicited preferences for PS and, as such, were not included in our literature review. Although the results of our review seem to be discordant with Nord and Johansen’s results, and it is worth mentioning that in two of the five aforementioned studies the public was found to be less likely to prioritise patients with higher levels of SOI [45,62], a direct comparison of results is not possible for reasons that are previously described.

NICE conducted a public consultation in the UK in 2014 on the topic of value-based assessment of health technologies [58–60]. NICE asked the public, including patients, economists, academics, and members of the pharmaceutical industry, ten related questions. One of the questions concerned the extent to which the public regarded PS as an appropriate approach for quantifying BOI [58]. NICE received reactions from 121 individuals and organisations, but no general agreement emerged [59]. In summary, the public regarded PS as a measure that is feasible and suitable for calculating BOI in terms of health and QOL impact in cases where a disease affects older patients. However, as in the ACP, there were concerns about PS not being a suitable measure for capturing broader aspects of BOI that are not included in the QALY. In addition, there were concerns about PS assigning a lower weight to the BOI of younger patients than to older patients due to differences in the PS denominator, i.e. the remaining QALY expectation in absence of the disease. More generally, there were concerns about the strong reliance on the QALY in health technology assessment and in the calculation of BOI, resulting in a possible double counting of benefits when setting priorities in healthcare [58]. Because of the lack of agreement that emerged from the public consultation, NICE decided to not change their current health-technology appraisal and end-of-life guidelines [60].

Some limitations of our study must be mentioned. A first limitation concerns the lack of a systematic review of the studies that we used to examine the theoretical and empirical support for PS. However, as the number of studies examining PS is limited, we believe that our review was comprehensive and that our results were not influenced by the lack of a systematic search. A second limitation concerns the use of publicly accessible ACP reports to examine the current role of PS in healthcare decision making in the Netherlands. In addition to public meetings, the ACP held closed meetings between 2013 and 2016, and the role of PS may have been discussed more frequently in these. However, the reports of these closed meetings are not publicly accessible and therefore could not be included in our review of ACP reports. Although the role of PS...
may have been discussed more frequently during closed meetings, and the inclusion of closed meeting reports might have changed the review results, it seems reasonable to expect that any discussion of PS in a closed meeting would have been reflected in a public meeting where the decision making actually took place. A final limitation concerns the risk of double counting search terms due to a possible overlap in ACP meeting reports. This risk was reduced by excluding concept versions of minutes unless a final version was not available; however, this distinction could not always be made for meeting documents. For example, documents concerning the reimbursement of a specific treatment may have been discussed at more than one ACP meeting. As a result, a higher relative importance may have been assigned to some of the search terms. However, as the main objective of our review of ACP reports was to examine the role of PS in healthcare decision making, and PS was infrequently identified, we believe that the influence of possible double counting on conclusions drawn from the review is limited. Concerning the frequency with which the search terms were identified in the ACP meeting reports, we would like to additionally point out that these frequencies should be considered in the broader context of priority setting in the Netherlands. This broader context determines the agenda and the priorities that are set in ACP meetings and hence influences the frequency with which the search terms were identified. Apart from these limitations, we consider it a strength of our study that we have examined support for PS at three different levels, i.e. at a theoretical, empirical, and decision-making level. To our knowledge, this is the first study to examine support for PS in such an extensive manner.

Ideally, the operationalisation of the equity-efficiency trade-off are normatively justifiable and empirically supported. The various normative choices that need to be made in this context indicate that a trade-off or a compromise between different societal concerns regarding equity and fairness needs to be made. Consequently, the ‘perfect’ explication of the equity criterion may not exist, and PS, like any other explication, will have its strengths and limitations. The results of our study indicate that the decision model in which increasing levels of BOI are quantified and ICERs are related to the associated monetary reference values per QALY gained is supported and increasingly applied in decision-making practice. The operationalisation of BOI in terms of PS enables a uniform decision model for priority setting across all healthcare sectors that can be applied by replacing or complementing the QALY in the PS equation with a broader, wellbeing-related, generic outcome measure such as the ICECAP [56]. The results of our study also indicate that PS insufficiently reflects societal preferences regarding age and reducing lifetime-health inequalities between patient groups. Future research is needed to develop and examine alternative versions of PS, such as a version of PS that is adjusted for wellbeing- and age-related preferences. These could be compared to the current operationalisation of PS, also in terms of alignment with general public preferences. There are different possibilities for combining PS and age in a preference-elicitation study. For example, it may be interesting to elicit preferences for a PS version in which the denominator of the PS equation consists of patients’ lifetime-QALY expectation. It may also be interesting to elicit preferences for combinations of PS and lifetime health (or age). For this, a matrix combining different age and PS classes could be used. To increase comparability between studies, we recommend using a more standardised approach to eliciting equity weights. Related to this, we would like to mention that the variety of the ways in which BOI is quantified in ACP reports may, understandably, reflect the present lack of consensus about the application of PS. However, this variety is in itself undesirable as it hampers the transparency and comparability of BOI quantifications for different beneficiaries and, subsequently, of the relevant cost-effectiveness threshold of (new) health technologies in the Netherlands. ZIN is expected to publish a report on the standardisation of the BOI quantification later this year to solve this issue.

7. Conclusions

The results of this study suggest that there is support for the decision model in which BOI is quantified and ICERs are evaluated against increasing monetary reference values. Although consensus regarding the application of PS has not yet been reached, BOI is increasingly quantified in terms of PS in decision-making practice. As any (generic) outcome measure can be included in the PS equation, PS enables a uniform decision model for priority setting across all healthcare sectors. Empirical support for PS appears to be limited, as PS may insufficiently reflect societal preferences regarding age and reducing lifetime-health inequalities. Hence, further investigation into the refinement of PS—or exploration of another approach—appears warranted for operationalising the equity-efficiency trade-off in healthcare priority setting.

Conflict of interest

The authors have no conflict of interest to declare.

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