

An assessment of current approaches to
health technology assessment in Norway and abroad

Beyond severity

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Table of contents

Executive summary	4
1. The changing landscape of health technology assessment	6
1.1 Small patient groups	6
1.2 This report	6
2. Value assessment in an international perspective	7
2.1 Assessing the value of novel medical interventions	7
2.2 Challenges to conventional cost-effectiveness analysis	8
2.3 Value elements considered by Nordic and other HTA bodies	8
2.4 A changing landscape	9
3. Measuring value	11
3.1 Prevalence	11
3.2 Severity	13
3.3 Impact on caregivers	15
3.4 Productivity	15
3.5 Uncertainty	16
3.6 Can we measure value better?	17
4. Aggregating multiple value elements	19
4.1 Modified cost-effectiveness analysis (mCEA)	19
4.2 Multiple-criteria Decision Analysis (MCDA)	20
4.3 Case-by-case decisions	21
4.4 What works best?	21
5. What constitutes fair and equal access to healthcare?	23
5.1 Objectives	23
5.2 Survey respondents	23
5.3 Results	23
5.4 Key findings	25
6. Conclusions and recommendations	28
7. References	30

Executive summary

The assessment of new pharmaceuticals poses an increasing challenge to national health technology assessment (HTA) bodies, both in terms of quantity and complexity. Approaches and methodologies have consistently evolved but a mature consensus across countries has still not been reached. This leads to challenges in access for patients, who may see that promising treatments are available in other countries, but not in theirs. It also creates unclear incentives for pharmaceutical developers, who navigate an inconsistent HTA landscape.

Treatments for small patient groups pose a particularly tough challenge for national HTA bodies. These treatments may involve more limited clinical trial evidence, or a significant degree of uncertainty regarding their long-term costs and benefits. However, there is still no consensus across HTA bodies on what the best approach to assess these treatments is.

We conducted a study on behalf of Sanofi AS on the state of healthcare technology assessment, with a focus on the evaluation of new medicinal products. The purpose of this report is to provide a picture of the key consensus and remaining disagreements across HTA bodies, and explore whether these approaches align with societal views. We compared the perspectives and methods applied by nine national HTA bodies, and explored the remaining challenges being faced. We then conducted a survey among Norwegian adults to investigate their preferences regarding healthcare prioritisation decisions.

Our review of current HTA guidelines for the assessment of new medicines revealed that most agencies incorporate additional factors beyond standard cost-effectiveness into their decisions. Disease severity and uncertainty stand out as common elements currently considered, while the impact on caregivers, productivity and disease prevalence have also garnered attention in recent years. Nevertheless, there is a lack of consensus regarding the inclusion and measurement of each of these elements in practice. Further research and methods development are needed to address unresolved challenges and harmonise approaches across borders.

Several initiatives have been undertaken to establish stronger ties across national HTA bodies and collaborate on the development and harmonization of HTA practice. The European Network for HTA (EUnetHTA) has proposed a methodology and process for conducting joint clinical assessments at the European level, while HTA agencies from Australia, Canada, New Zealand and the UK established their own group, which includes collaboration in methods development as one of their three key priorities. Collaboration in terms of methodological development is not currently an objective of the Joint Nordic HTA Bodies (JNHB) initiative but should be carefully considered over the coming years. Such platforms for collaboration can contribute to harmonised approaches across borders as well as provide HTA bodies with needed support in the development of methods and guidelines.

We next address the question of how to best aggregate value into a single, comparable measure. We focus on three different approaches that have been suggested to compare two or more alternatives across multiple dimensions in health economic analysis. Modified cost-effectiveness analysis (mCEA), multiple-criteria decision analysis (MCDA) and case-by-case decisions all have advantages and disadvantages. Their role in the healthcare assessment landscape should be considered carefully to protect the integrity and transparency of the system. Structured deliberation processes, not unlike MCDA, are already being applied by agencies when reviewing their methods, while their use remains rare in individual HTA evaluations. HTA bodies should strive to achieve the highest possible degree of transparency and predictability in their assessments, to provide clarity to both patients and producers.

How value is assessed and understood is an ethical question and the principles applied in practice ultimately a political decision. We conducted a survey among a sample of Norwegian adults to understand their preferences around healthcare prioritisation. **A significant share of respondents was willing to sacrifice overall health gains in favour of treatments for severe diseases, treatments that allow patients to return to work, treatments for diseases that affect caregivers and diseases that affect a small number of people.** Societal views in Norway thus seem to align with a more comprehensive approach to value assessment than what is currently being implemented.

Key findings and recommendations



What is value?	Broad social support	Measurement challenges persist	Foster transparency and predictability	International collaboration
There is significant variation across HTA bodies regarding which value elements to include in their assessments of new treatments. This creates uncertainty among patients and producers.	It is important that healthcare systems reflect social preferences. Our survey of Norwegian adults suggest there is support for a broader, more comprehensive approach to value in healthcare assessments.	Value elements other than cost-effectiveness are often incorporated in a qualitative manner. Even when they are measured (e.g., severity) there are disagreements across agencies on how best to do this. Further methods development and harmonisation are needed.	Uneven treatment access across countries can create mistrust among developers, regulators and patients. There is a need for increased transparency and predictability in decisions to strengthen trust among stakeholders.	Collaborative platforms, such as the European Network for HTA can harmonise approaches and aid HTA bodies in developing methods and guidelines. The Joint Nordic HTA Bodies initiative may be a suitable forum for this.

1. The changing landscape of health technology assessment

The assessment of new pharmaceuticals poses an increasing challenge for health technology agencies, both in terms of quantity and complexity. Methodologies have consistently evolved but a mature consensus across countries has still not been reached. Treatments for small patient groups pose a particularly tough challenge for national payers.

The development landscape for new pharmaceuticals has experienced steady change over the past decades. The persistent development of new treatments for hard-to-treat conditions, as well as the advent of personalised medicine have led to an increasing number of promising new treatments arriving to the market each year. This meant that health technology assessment (HTA) bodies faced the challenging task of evaluating an ever-increasing number of new products each year (Figure 1-1).

The challenge is not only one of quantity, but also of complexity. The methods used for the assessment of new pharmaceuticals have had to evolve in step with both the characteristics of the new treatments, the types of evidence available for them and each health authorities' needs and priorities. National HTA guidelines are frequently reviewed and updated as a result. While a consensus exists around the basic building blocks of this methodology, there are significant areas in which further research and international collaboration are needed to arrive at a consistent approach across countries with otherwise similar healthcare systems.

1.1 Small patient groups

Treatments for small patient groups can pose a particularly tough challenge for HTA bodies and healthcare decision-makers interested in ensuring equal access to healthcare. These treatments may involve limited clinical trial evidence due to the difficulty of recruiting enough patients. They may also involve significant degree of uncertainty regarding their long-term costs and benefits associated with the new treatment.

Several HTA agencies have thus established principles that incentivise innovation in the

Figure 1-1: Methods assessments completed by the Norwegian Medicines Agency (NOMA) by year (as per May 2nd, 2024)

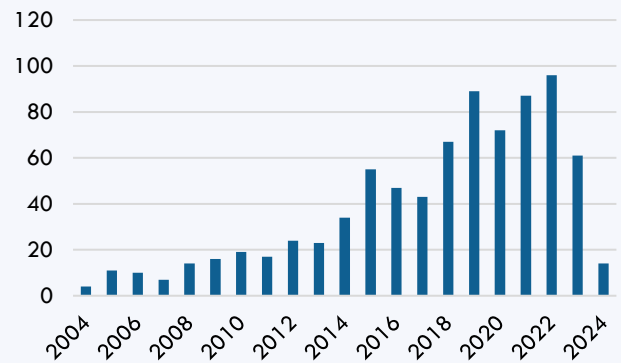


Illustration: Oslo Economics based on data from NOMA

treatment of small patient groups, either through R&D support schemes, increasing their willingness-to-pay (WTP) for new treatments or entering into risk-sharing agreements with companies developing them. However, there is still no consensus across European countries on what the best approach is to achieve this objective.

1.2 This report

In this report we focus on the current state of health technology assessment in Norway and abroad, and highlight the key challenges faced by HTA bodies in the process of evaluating new pharmaceuticals for reimbursement. We discuss what value means in this context, and whether a more comprehensive approach to value assessment is needed, before describing the key value elements currently considered by a selection of HTA bodies. We then discuss some key remaining issues regarding how we measure the value provided by new medical treatments along a series of different dimensions, as well as how one can aggregate this information to provide clear answers for decision-makers.

Finally, we conduct a survey of Norwegian adults to assess their preferences regarding healthcare prioritisation, to understand whether current approaches align with social preferences.

This work was supported by Sanofi AS.

2. Value assessment in an international perspective

Standard cost-effectiveness analysis has been criticised for ignoring important elements of value provided by new medicines. We reviewed healthcare assessment guidelines from Norway and eight other countries and found that most agencies incorporate additional factors into their decisions. Disease severity and uncertainty stand out as common elements currently considered, while the impact on caregivers, productivity and disease prevalence have also garnered attention in recent years.

2.1 Assessing the value of novel medical interventions

The basic building block of health technology assessment is cost-effectiveness analysis, which compares the health gains and costs that would follow from the introduction of a new proposed treatment. Health gains are commonly measured in quality-adjusted life years (QALY), a measure that summarises both expected life years and health related quality of life (HRQoL). QALYs are measured

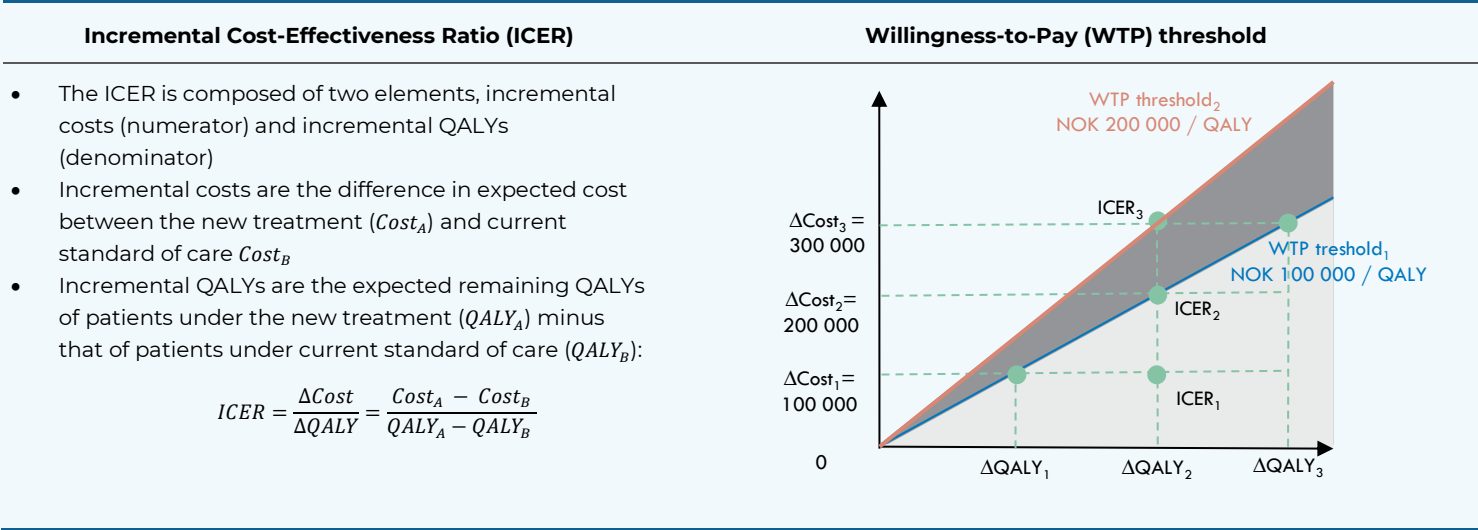
on a scale from 0 to 1, where 1 implies a year of “perfect health”. QALYs are additive, meaning that two life years with a HRQoL of 0.5 is equal to one year of life with perfect health.

The cost associated with the introduction of a new treatment are also compared to those under current standard of care for the given patient population. Relevant costs could for example include an increase in drug costs and/or a reduction in hospitalisations.

The aspects of health and the type of costs included in the analysis depend on the chosen perspective and are a matter of constant debate. Most European HTA-bodies, for example, take a healthcare sector perspective, considering costs related to use of healthcare services and drug costs, but ignore wider societal impacts such as the value of innovation or the productivity gains stemming from patients returning to work.

Cost-effectiveness analysis consists in the estimation of an Incremental Cost-Effectiveness Ratio (ICER) – the additional cost per QALY gained (Table 2-1). This ICER can then be compared to pre-defined WTP thresholds, that determine whether the proposed new treatments are introduced or not. Some HTA bodies, such as National Institute for Health and Care Excellence (NICE) in the UK have explicit WTP thresholds, while others, such as the Norwegian Medicines Agency (NOMA) do not.

Table 2-1: Incremental Cost-Effectiveness Ratio (ICER) and Cost-Effectiveness Threshold



Source: Oslo Economics based on Claxton et al., 2008 [12], [19].

2.2 Challenges to conventional cost-effectiveness analysis

Cost-effectiveness analysis has been acknowledged as a useful starting point for discussions on value. It secures a consistent evaluation of health gains and costs and makes it possible to compare novel technologies up against each other.

Over recent years however, a series of limitations to standard cost-effectiveness analyses have been discussed [9]. In 2018 an ISPOR Special Task Force on US Value Assessments published the “ISPOR value flower” (Figure 2-1). Each petal of the flower represents a possible source of value delivered by new pharmaceuticals. The petal illustrates how QALY gains and net costs (i.e., the ICER) may only capture a subset of the benefits delivered by new treatments.

Several HTA bodies now incorporate additional value elements to their assessments, either within the calculation of the ICER or in the form of “decision modifiers” that shift the WTP threshold [23]. Additional elements of value, as we describe later in this chapter, include disease severity, prevalence (or rarity), the availability of treatment alternatives, and innovation, for example [23].

2.2.1 A matter of perspective

HTA bodies have also adopted different perspectives for their assessments of new

treatments. This has important implications regarding which aspects of value can and should be considered when assessing the cost-effectiveness of new treatments, and which treatments end up being prioritised and made available for patients.

Cost-effectiveness analysis can for example take the perspective of the healthcare sector, or a broader societal perspective. From a healthcare system perspective, only relevant costs in the healthcare system are included when calculating the ICER. Similarly, only direct health gains are included. Alternatively, a societal perspective could include productivity losses and other costs (or savings) faced by actors other than the patient or the healthcare sector.

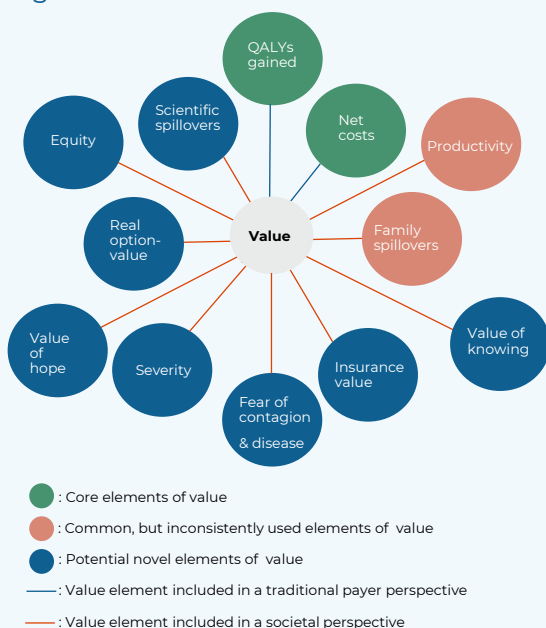
There is still significant debate regarding what the most appropriate perspective is for an HTA setting, with HTA bodies opting for different approaches, based on the mandates they are given, and the priorities set by the health authorities. Currently an extended healthcare system perspective is applied in Norway. However, a Norwegian Expert Committee recently recommended taking a strict healthcare perspective instead, while allowing for supplementary analyses from broader perspectives [12].

2.3 Value elements considered by Nordic and other HTA bodies

HTA authorities across countries have different approaches to valuing health technologies (Table 2-2). In practice, we see that across the HTA bodies reviewed, agencies have taken steps towards more comprehensive value assessment. Table 2-2 presents the main method for assessing new technologies in a selection of countries. Whenever possible, we include the perspective of the assessment, as well as the qualitative and quantitative modifiers that are taken into consideration by each agency. The table is based on publicly available guidelines, communication with some of the agencies and other similar, recent reviews [13].

We find that several countries, including Norway, take a broader perspective than the strict healthcare system perspective. Sweden formerly allowed for the inclusion of productivity losses but have in more recent years changed this practice [12]. The Netherlands takes a broader societal perspective, including productivity and unpaid work, informal care (time spent and QALYs), unrelated healthcare costs and costs for the family of the patient. Some agencies explicitly allow for

Figure 2-1: The ISPOR value flower



Source: [9]. Illustration: Oslo Economics.

submission of additional analysis including a broader perspective with for example non-health gains and impact on caregivers, such as Canada and Australia.

Most HTA agencies consider disease severity as part of their assessment. NOMA (Norway), the National Health Care Institute (ZIN) in the Netherlands and NICE (UK) calculate severity quantitatively while the rest, including Pharmaceutical Benefits Advisory Committee (PBAC) in Australia, the Canadian Agency for Drugs and Technologies in Health (CADTH) in Canada and the Haute Autorité de Santé (HAS) in France, consider severity qualitatively

Some agencies account for rarity, mainly either qualitative or through designated tracks. In Norway, a simplified HTA requiring less documentation is accepted for the treatment of extremely severe conditions and a higher WTP threshold is applied [24]. NICE (UK) have for example established explicit recommendations for highly specialised technologies for very rare conditions. For these medicines, a higher WTP threshold applies (£ 100,000) than for other technologies (£ 20,000). Qualitative modifiers may justify recommendations for technologies with an ICER exceeding these thresholds [7]. In France, an HTA is not required for treatments for rare diseases with a budget impact of less than € 50 million a year [13].

Most HTA agencies have also noted that uncertainty should be explored with either deterministic or probabilistic scenario analyses. Many also state that methodological uncertainty should be discussed. However, they have no quantitative guidance on how these results explicitly are taken into consideration in terms of, for example, a different WTP threshold. This applies to NOMA, as well as several other HTA agencies.

Some HTA agencies qualitatively consider equity in their assessment. Most ask for analysis of subgroups of the patient population, whilst other also explicitly ask for expected equity-related implications of a treatment, for example CADTH (Canada) [16].

In general, there is a consensus around cost-effectiveness analysis as a standard approach for value assessment of new health technology among the HTA bodies reviewed. In addition, there are commonalities across multiple agencies, including NOMA (Norway), agreeing on inclusion of modifiers for severity and uncertainty. Finally, there are elements that are less clear, but nonetheless often discussed, such as prevalence, impact on caregivers and productivity.

2.4 A changing landscape

A **clear consensus around the strengths of cost-effectiveness analysis** is reflected in most HTA-bodies' current practice, mainly relying on ICERs calculated from a healthcare sector perspective.

Disease severity and uncertainty appear regularly as additional factors that may influence decisions in either a qualitative or quantitative way. There appears to be an **increasing acceptance of the need to expand the value assessment of novel medical interventions**, including elements such as disease rarity, equity or the impact of the disease on the patient's ability to work and their caregivers.

Table 2-2: Modifiers in HTA guidelines in different countries

Country	Track	Value elements included (besides cost-effectiveness)	Reference
Norwegian Medicines Agency (NOMA) Norway	General	Perspective: Extended healthcare perspective Qualitative: Uncertainty Quantitative: Severity	[10]
Dental and Pharmaceutical Benefits Agency (TLV) Sweden	General	Perspective: Limited societal perspective Qualitative: Severity, rarity	[5, 12, 13], Communication with TLV
Danish Medicines Council (DMC) Denmark	General	Perspective: Limited societal perspective Qualitative: Uncertainty, severity	[12-14]
Finnish Medicines Agency (FIMEA) Finland	General	Qualitative: Prevalence, disease severity and burden, rarity, budget impact	[17], communication with FIMEA
National Institute for Health and Care Excellence (NICE) England and Wales	General	Perspective: Healthcare perspective Qualitative: Innovation, non-health objectives of the NHS, uncertainty, uncaptured benefits Quantitative: Severity	[7, 12, 13, 18]
	HST	Criteria: Highly specialised technologies for very rare conditions Qualitative: Impact of the technology on the overall delivery of the specialised service, additional staffing and infrastructure requirements, including training and planning for expertise	[7]
Haute Autorité de Santé (HAS) France	General	Perspective: Extended healthcare perspective Qualitative: Uncertainty, severity Quantitative: Rarity	[13, 20]
National Health Care Institute (ZIN) Netherlands	General	Perspective: Societal Qualitative: Uncertainty Quantitative: Severity	[13, 21]
Canadian Agency for Drugs and Technologies in Health (CADTH) Canada	General	Perspective: Healthcare perspective. A broader perspective can be submitted in an additional non-reference analysis (including impact on caregiver and non-health effects of patients) Qualitative: Uncertainty, equity, severity	[12, 13, 16]
Pharmaceutical Benefits Advisory Committee (PBAC) Australia	General	Perspective: Healthcare perspective. A broader perspective can be provided as supplementary analyses (for example non-health related outcomes) Qualitative: Availability of substitutes, severity, rarity and rescue*, uncertainty, equity, public health issues (for example development of resistance), patient affordability, budget impact	[13, 22]

*Rescue entails that the clinical improvement is sufficient to qualify as a rescue from the medical condition.

3. Measuring value

Disease severity, prevalence, impact on caregivers, impact on productivity and uncertainty are often discussed as potential value elements to consider in addition to cost-effectiveness. We delve into their rationale, the approaches taken in practice to measure and quantify them, as well as the unresolved methodological challenges behind each element.

In the previous chapter we have described the limitations of cost-effectiveness on its own as a measure of the value delivered by new interventions. We have also identified a series of additional criteria besides cost-effectiveness which are commonly used in HTAs in Europe and elsewhere. These include the degree of severity of the disease, the prevalence of the disease, the disease's impact on productivity and on the patient's caregivers, and the degree of uncertainty involved in the decision.

3.1 Prevalence

When patient groups are small, incentives to invest in researching and developing new interventions may be weakened. Low R&D levels lead to fewer and less effective treatment alternatives. Collecting evidence for particularly rare diseases is also

challenging. Patient numbers are small, long-term effects are often uncertain and clinical studies are often single-armed [25]. Perhaps not surprisingly, a recent mapping of the state of access to gene therapies in Norway found that uncertainty and price were the two most common reasons for rejection [25]. Thus, it is argued, the WTP for treatments destined to small patient groups should be higher, to ensure equitable access to high quality healthcare to all patients [23].

Arguments have also been raised against the inclusion of prevalence as a decision modifier in cost-effectiveness assessment. One such argument states that its inclusion might lead to double counting. Diseases with low prevalence may have fewer treatment options as the resources going into researching and developing new treatments for them are likely limited. Fewer treatment options result in worse outcomes for patients, thereby increasing the measured severity of the disease. The current cost-effectiveness assessment framework, which as we have seen often incorporates disease severity, may thus already address the concern of equitable access to healthcare [26, 27].

Secondly, it is paramount that healthcare prioritisation criteria are aligned with social preferences, and it is still unclear whether there is a general preference towards prioritising low prevalence diseases. A 2010 survey of 1,547 Norwegians aged 40-67 found strong general

Table 3-1: TLV's proposed prevalence decision modifier

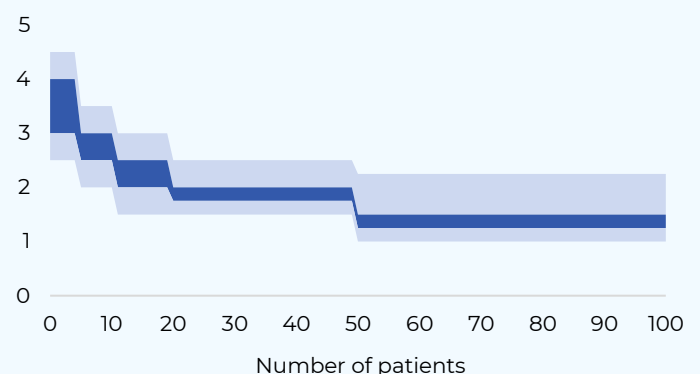
How is the staircase model applied?

The Swedish Medicines Agency has in recent years proposed a series of changes to its value assessment framework and methods [3-5]. One of their proposed changes is a stepwise model, a "ladder", which increases the WTP for treatments destined at particularly small groups of patients (see Figure).

For treatments directed at fewer than 100 patients (all indications), the ladder indicates a steep increase in WTP along a range of values depending on other characteristics such as the degree of uncertainty surrounding the treatment's efficacy. This adjustment to WTP would only be applied if the severity of the condition is deemed very high. This proposal is currently under evaluation by the Swedish government.

WTP by disease prevalence

Willingness-to-pay per QALY (million SEK)



Source: Oslo Economics adapted from TLV (2003) [5, 16].

support for statements aligning with a preference for equal treatment rights for patients with rare diseases. However, the study did not find a societal preference for rarity in cases where the treatment of patients with rare diseases would come at the detriment of patients with common diseases [28]. A recent survey of US respondents found broad support for prioritising *common* diseases over less prevalent ones [29], in line with another similar study conducted in the UK [30], while a survey in the Netherlands found a degree of support for the prioritisation of rare diseases [31]. These diverging results may indicate the importance of the preference elicitation method chosen and the precise phrasing used in surveys.

3.1.1 Current practice

Several countries have adopted measures to increase access to new interventions for small patient groups. One common approach has been to establish separate tracks for orphan or ultra-orphan drugs [32]. NOMA (Norway) accepts a lower level of documentation and a higher level of resource use for assessment of diseases with small patient groups and severely severe conditions. Another example of this is NICE's (UK) assessment of highly specialised technologies, which allows a subset of drugs fulfilling a set strict of criteria to be assessed using a substantially higher WTP threshold than the more general single health technology assessment track.

This "separate track" approach has been criticised for not appropriately addressing the challenges that are faced by interventions directed at small patient groups, while at the same time disrupting the equity of the overall health technology assessment system [32].

Alternative approaches have thus been proposed that do not require separate assessment frameworks. Several countries have introduced severity modifiers which effectively prioritise treatments for diseases with a high unmet need. The Dental and Pharmaceutical Benefits Agency (TLV) in Sweden has in turn developed a quantitative decision modifier based on disease prevalence, which increased the WTP threshold for treatments aimed at very small patient groups. This is for the moment only a proposal and has not been implemented, but it reflects the ongoing efforts being made to address the challenge of prioritising treatment to small patient groups.

3.1.2 Moving forward

The introduction of rarity as a standalone value element in health technology assessments still faces important practical challenges. Some of the most concrete challenges are described by TLV

(Sweden) in their proposal of the "prevalence ladder" approach [5]. For example, HTA bodies aiming to roll out a similar decision modifier should be careful to design the WTP steps in such a way that maintains the incentive for drug developers to deliver their products to as wide a population as possible. In addition, care should be taken to ensure that value elements considered do not overlap with each other, to ensure that the proposed value assessment framework remains consistent.

Given these challenges, it is worth considering whether access to new interventions for small patient groups can be increased by means other than a decision modifier. The challenges related to the collection of clinical evidence that affect treatments for small patient populations may for example be addressed by a more flexible approach to addressing uncertainty in HTA applications as currently done in Norway, or by the introduction of risk-sharing contracts between producers and payers.

3.2 Severity

A key assumption behind the estimation of the ICER, as described in the previous chapter, is that *all QALYs are equal*. This means in practice that a similar improvement in health is worth the same regardless of who is experiencing it. However, it is reasonable to assume that a similar improvement in health is more valuable for patients with very severe diseases than for patients who are in almost perfect health.

This idea that greater value should be assigned to health gains to patients in more need has been tested in public preference surveys conducted in multiple countries. Generally, these studies show that people are willing to pay more for treatments for patients with severe conditions, even if they come at the cost of fewer treatments for healthier patients, something often referred to as a “severity premium” [33].

As previously mentioned, NOMA (Norway) applies a quantitative modifier for disease severity. HTA bodies around the world have included disease severity as a common modifier for their reimbursement decisions as well, increasing their WTP for treatments for more severe conditions. ZIN (Netherlands) and NICE (UK) have both introduced quantitative modifiers to their general WTP for new medicines based on disease severity measures. Finland, Denmark, Canada, and Australia, among others, include disease severity as a qualitative factor affecting reimbursement decisions (Table 2-2).

3.2.1 Current practice

Several ways to implement severity in an economic evaluation have been proposed, and this remains an active area of development for health economics practitioners. The first point of disagreement among the HTA bodies that have introduced a quantitative measure of disease severity in their assessments is what to measure. While NOMA (Norway) includes severity as measured by absolute shortfall (AS), ZIN (Netherlands) applies it using proportional shortfall (PS) instead (Table 3-2). NICE (UK), applies both AS and PS, perhaps reflecting the degree of uncertainty that remains about which of these two measures most aligns with social preferences.

NOMA (Norway), as well as ZIN (Netherlands) and NICE (UK), have established a stepwise approach to adjusting the WTP threshold for more severe conditions, with discrete increases in WTP as severity increases (Figure 3-1).

Table 3-2 Quantitative measure of severity: Absolute and proportional shortfall

Absolute shortfall (AS)
<ul style="list-style-type: none"> The number of healthy life years lost because of premature death and/or reduced quality of life during the period of illness, compared to the general population. Absolute shortfall is equivalent to future loss of healthy life years between individuals with a condition and those without it. It is derived by subtracting the expected total QALYs of individuals with the condition under current treatment (P_A) from the total QALYs expected by the general population at the same age ($QALY_A$): $AS = QALY_A - P_A$
Proportional shortfall (PS)
<ul style="list-style-type: none"> The proportion of future healthy life years lost by individuals living with a condition, compared to the general population. It is computed by dividing the absolute shortfall in QALY expected by this patient population (AS) by the expected remaining QALYs for people in the same age group in the general population ($QALY_A$): $PS = \frac{QALY_A - P_A}{QALY_A} = \frac{AS}{QALY_A}$

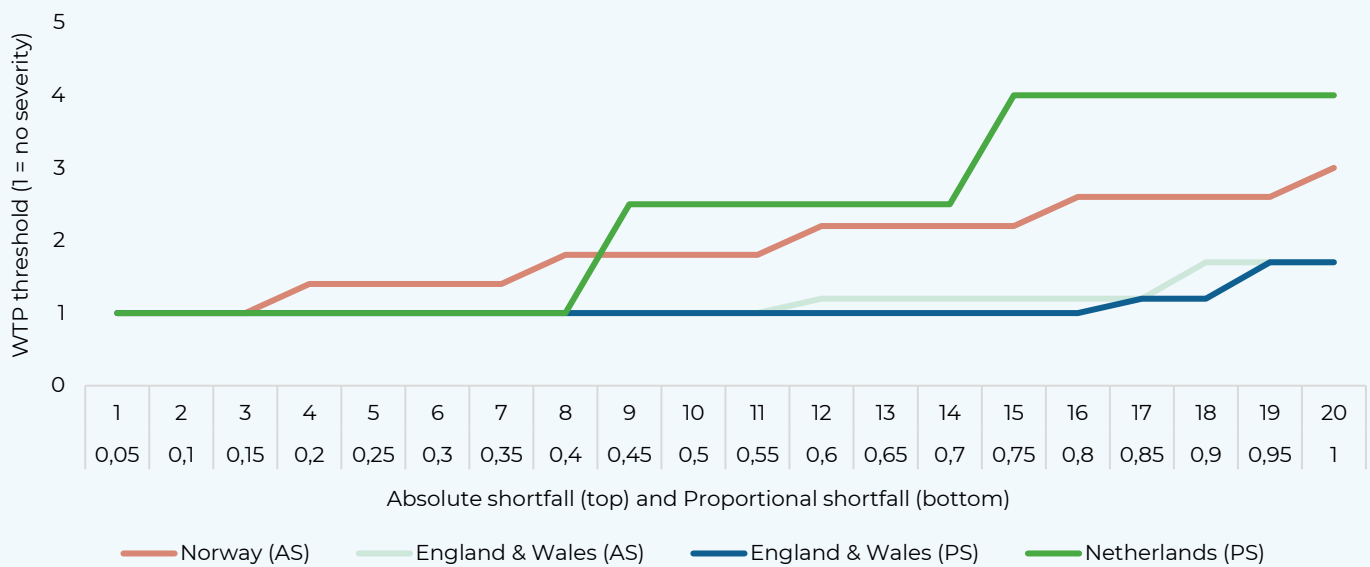
Source: Oslo Economics

Norway implements AS in an undiscounted way, while both the Netherlands and NICE in the UK apply a discount rate to PS/AS measures.

3.2.2 Moving forward

As seen above, there is still a lack of consensus regarding which of the two commonly used severity measures (AS or PS) best captures the social preference towards prioritising treatment for more severe illnesses. On the one hand, AS measures the years of healthy life lost by people living with the disease. Since younger people have longer life expectancies than older people, they will generally have more to lose to disease. So chronic diseases that affect younger patients will often have higher AS scores than diseases that affect the

Figure 3-1: Willingness-to-pay (WTP) thresholds by disease severity measure and country



Note: WTP thresholds for all three countries have been normalised to 1 (= to WTP for low severity conditions) for clarity. Source: Oslo Economics, based on Magnussen et al (2015), NICE UK (2022) and Reckers-Droog et al, (2018) [6-8].

elderly. The opposite is true for PS. Elderly patients that are nearer to the end of their lives have relatively fewer healthy life years ahead of them, and thus severe diseases affecting them will lead to high PS scores.

Further disagreement persists regarding whether AS should be calculated directly or whether these expected future QALYs should be discounted using a reference yearly rate, with countries taking different approaches to this.

Perhaps more fundamentally, there is still significant disagreement regarding *what constitutes a severe disease*, and *how much higher WTP should be for them*. All three countries have discrete disease severity groups, but the cut-offs determining when WTP should increase, and by how much, varies significantly (Figure 3-1). The highest WTP category in the Netherlands starts with diseases with a PS score of 0.75, while this threshold is set at 0.95 in the UK. In Norway the highest severity group starts at an AS score of 20, which consists of only the most severe, lifelong conditions. The highest WTP thresholds for the Netherlands, Norway and the UK are 4, 3 and 1.7 times their general levels, respectively.

Finally, disease severity is measured based on the average age at the time of diagnosis or start of treatment. This approach is reasonable for diseases that affect people of similar ages. However, for diseases that may affect people of very different ages, this may lead to young people suffering from

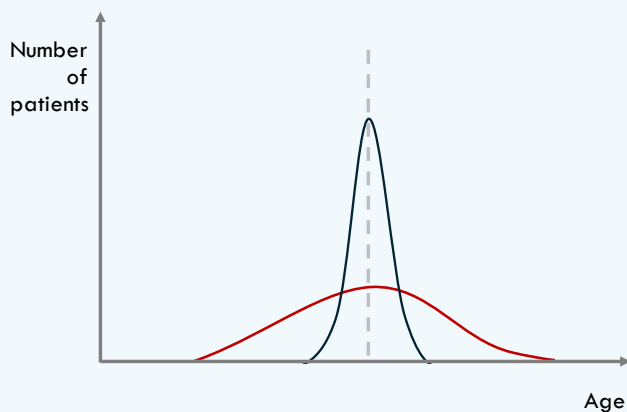
diseases deemed of low severity, because they are in a group of unlucky few being relatively young compared to the average age of the patient population. This may be a condition they might have to live with their whole lives and thus the severity of this disease for young patients could be very high.

This problem can be reduced by including not only the mean, but also the distribution, of ages at diagnosis in the calculation of the absolute shortfall implied by a disease (Figure 3-2). Under the current method for the shortfall is the difference between the remaining expected QALYs of individuals with the condition under current treatment and the remaining QALYs expected in the general population. The remaining QALYs for the diseased population is based on the average age of the patient population. However, this assumes that a patient above and below the average have the same quality of life per life year. Younger patients are expected, on average, to have a higher quality of life each year than their older counterparts. Thus, including the age distribution of the patient population (as seen in the red line in Figure 3-2) will increase the estimated shortfall, the broader the age distribution of the disease, thereby prioritising these diseases over those where the distribution is centred around the mean.

3.3 Impact on caregivers

Disease may affect the patient caregivers' physical and mental health as well as their capacity to work. The effects of new treatments on caregivers could thus be substantial. However, there is still a significant distinction made by HTA bodies between impacts on patients and impacts on caregivers. This practice may reflect a value judgement (deeming the impacts on patients as more relevant than those on caregivers) as well as a practical challenge (impacts on caregivers are often more challenging to document).

Figure 3-2: Example: two diseases of patients with similar average age, but different age distributions



Source: Oslo Economics. Disease A (blue) and B (red) affect patients with the same average age. However, Disease B affects a larger number of young patients than Disease A.

3.3.1 Current practice

Norwegian HTA guidelines allow for the inclusion of health impacts (measured in QALYs) on both the patient and the caregiver(s). However, the impact on the caregivers is a separate analysis and not a part of the base case [10]. Denmark also considers the impact on caregivers, while Sweden is currently assessing whether and how to consider it in its HTA process [12]. The ICER institute considers impacts on caregivers separately to their conventional cost-effectiveness analysis, as part of a series of factors that quantitatively or qualitatively affect their ultimate recommendations on cost-effectiveness [34].

3.3.2 Moving forward

Impacts on caregivers will likely vary in significance depending on the disease. It is reasonable to assume that caregivers of patients with chronic, severe, childhood illnesses are very affected, for

example. Partners of patients with mild food poisoning, on the other hand, are more likely to be unaffected by the disease. It is therefore not surprising that the approaches taken by HTA bodies and other stakeholders to incorporating these impacts vary and that these impacts are generally considered only on a case-by-case basis.

In the Norwegian guidelines, the same requirements are made for documentation of changes in quality of life for patients as for caregivers, which raises challenges for producers who often do not have this degree of evidence. Impacts on caregivers are also likely to vary significantly by country, depending on the structure of the healthcare system and the degree of support offered to families of patients (compared to formal care), making the collection of relevant evidence harder. Nevertheless, the lack of appropriate evidence does not preclude decision-makers from taking important, but undocumented impacts into account, and this is reflected in both the Norwegian guidelines and the ICER institute's value assessment framework [10, 34].

3.4 Productivity

Some novel treatments provide a double benefit to society: they improve the health of patients and increase production. This increase in production may come from patients feeling better and being able to get back to work, or by patients avoiding an early death thanks to the treatment. A recently published study estimated the production lost to disease each year in Norway to approximately 372 billion Norwegian kroner (31 billion Euro per 2024) [35]. There is potential for novel treatments to increase the overall productivity of a country.

The inclusion of these production effects on health technology assessments is, however, contested. Since younger individuals and those in the working age population are more likely to experience larger production gains than the elderly or the unemployed, incorporating production losses may lead to prioritising diseases affecting the former groups over those impacting the latter [12, 35]. One could partly address these concerns by assigning median national wages and labour market participation rates to all patients in each age group and assign non-market "wages" to the time gained by retired or unemployed persons. However, concerns about the equity implications of taking a societal perspective in HTA settings, and thus including production as an additional element of value for new novel treatments, remain.

3.4.1 Current practice

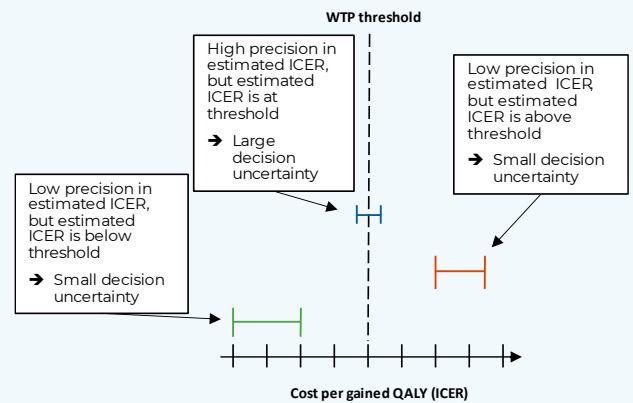
Production losses are currently not assessed in the Norwegian HTA process. A recent report commissioned by Norwegian Ministry of Health recommended the inclusion of production losses in cost-effectiveness assessments in the healthcare sector only as part of a separate (i.e., not the base case) analysis, when appropriately documented and deemed relevant [12].

Unlike most other countries, the Netherlands has incorporated production effects in its base case cost-effectiveness analyses of new treatments. This includes both paid and unpaid time. Paid work time lost to disease is composed of both being less productive, but still at work (so-called “presenteeism”), and work absenteeism [21]. The Swedish HTA agency has previously considered the production gains of an intervention associated with people’s ability to return to work. However, they now believe these effects need to be considered with more caution in light of ethical concerns of discriminating against people far from the labour market [4].

3.4.2 Moving forward

Most HTA bodies still recommend against the inclusion of productivity losses in the base case analysis (Table 2-2). However, this is based on practical and ethical concerns. New treatments can potentially lead to large productivity gains, especially in disease areas in which production losses are the largest [35]. The challenge for policymakers is thus to develop a framework that allows for this value to be realised while maintaining the core principle of equal access to

Figure 3-3: Not all uncertainty is equal



Source: Oslo Economics, adapted from TLV (2021).

healthcare. Efforts aimed at valuing time lost to disease equally, regardless of whether the patients are employed or not, may help address these concerns.

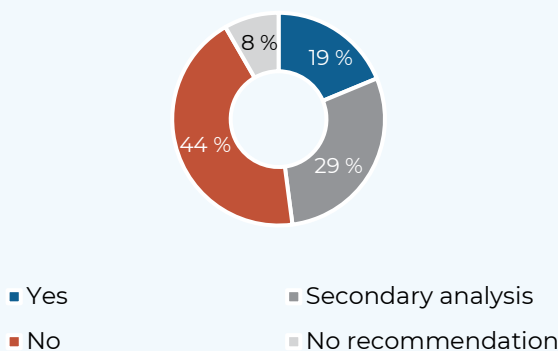
Alternatively, a case-by-case approach, in which the relevance of productivity effects is discussed, and their inclusion/exclusion is justified, may be more appropriate. This seems in line with the recommendations made by an expert group to the Norwegian Health Ministry in early 2024 [12]. In a similar vein, the US Institute for Clinical and Economic Review (ICER) performs its assessments taking both a healthcare system and a societal perspective, and includes an “impact inventory” listing clearly which elements are considered in each case [34, 36]. Identifying disease areas in which productivity losses are most likely to be included in national HTA processes may be a valuable first step for increasing the transparency and predictability of such an approach [35].

An additional practical challenge to the inclusion of productivity effects is the lack of available evidence. In these cases, the ICER institute conducts analyses by use of indirect estimates from official statistics and published literature [37]. This is in response to the concern that the lack of evidence does not necessarily mean that impacts are zero (dubbing this the “non-zero” approach).

3.5 Uncertainty

There is a broad consensus among the HTA bodies reviewed that treatments for which costs and benefits are well understood should be prioritised over others with more uncertain outcomes. This reflects the key challenge faced by HTA bodies and reimbursement agencies: they make decisions with

Figure 3-4: Guidelines recommending the inclusion of productivity losses in the economic evaluation of medical treatments



Source: Oslo Economics. The figure reflects the results of a review of 48 guidelines for economic evaluation of new medical treatments from 46 countries, conducted in 2021 by Jiang et al., (2022) [11].

financial implications today, on treatments with both costs and benefits that will happen in the future.

In practice, of course, all assessments involve a degree of uncertainty, and this is not always relevant to the decisions made by regulatory agencies. TLV (Sweden) helpfully distinguishes between uncertainty regarding a treatment's cost-effectiveness and uncertainty related to their decision (Figure 3-3). It is this decision making uncertainty that primarily poses an issue for HTA bodies [3]. ZIN (Netherlands), defines decision uncertainty more precisely as a situation in which the probability that the intervention is cost-effective is between zero and 100 percent at the reference WTP threshold [21].

Uncertainty is often especially large for precision medicines and advanced therapy medicinal products (ATMPs) such as gene therapies. These treatments have the potential to provide large benefits, accruing during a whole lifetime. They also entail large costs. Given that these treatments are often only studied in small clinical trials and over a few years before being assessed, there is usually a significant amount of uncertainty regarding the duration of these long-term health gains [3].

3.5.1 Current practice

The impact of the different assumptions made in a cost-effectiveness analysis is often calculated using deterministic sensitivity analyses (DSA) or scenario analyses. The joint uncertainty of the cost-effectiveness model is in turn assessed using probabilistic sensitivity analyses (PSA) that change all relevant parameter values at the same time based on probability distributions for each parameter. Most HTA bodies require the inclusion of both DSA and PSA in their assessments, although some important changes have recently been made and proposed by the HTA agencies in Norway, Sweden and the Netherlands that are worth noting.

In their latest update to their guidelines, NOMA (Norway) advises against the use of broad sensitivity analyses in which all parameters in the model vary within a discretionary range [10]. This change reflects the understanding that not all parameters are always relevant for such an analysis. Only scenarios that are deemed relevant should be assessed to provide an accurate picture of the underlying uncertainty of the case. TLV (Sweden), in turn, has recently proposed changes to its approach for assessing uncertainty in cost-effectiveness analyses for ATMPs. They propose the analysis to include a probability weighted average of different ICERs, considering different outcomes such as the duration of the outcome [5]. TLV (Sweden) also

suggested to include best- and worst-case scenarios to complement their base case assessment, in line with what is done by the ICER institute [37].

Conversely, ZIN (Netherlands) estimates the ICER by dividing the mean costs by the mean impact over all PSA iterations, building in the analysis of uncertainty into their base case analysis. [21]. A value of information (VOI) analysis should also be conducted based on the outcome from the PSA to help assess whether to delay the approval of the treatment, awaiting further evidence [21].

3.5.2 Moving forward

HTA bodies and decision-makers involved in reimbursement decisions are faced with the challenge of making decisions with large financial implications today, based on uncertain outcomes in the future. In this sense, their role is not unlike that of other public agencies deciding on large investment projects. It is therefore understandable that they take decision uncertainty seriously and prioritise certain over uncertain outcomes.

Nevertheless, the degree of acceptable uncertainty at any point in time depends on what is deemed feasible given resources and methods available. Interventions that are developed for very small populations such as gene therapies challenge the existing frameworks of many HTA bodies. Faced with this new challenge, alternative approaches may need to be developed to address uncertainty in novel ways.

HTA bodies may, for example, decide to accept a higher amount of decision uncertainty for specific disease areas. This would allow them to maintain a unified value assessment framework for all new medical treatments proposed, while accounting for the special circumstances involved in the development of these treatments. Alternatively, scenario analyses showing the implications of different risk-sharing or outcomes-based financing agreements may be helpful to inform decision making, as it could provide a better understanding of the financial implications of the case's uncertainty.

3.6 Can we measure value better?

The rationale for the inclusion of each of the value elements discussed in this chapter is clear, and rooted in the fact that conventional cost-effectiveness may lead to a too restrictive view of what the value of new treatments can be. However, healthcare prioritisation decisions are ultimately

political decisions that must be made in alignment with society's preferences. **A first step in assessing whether to incorporate further value elements into healthcare technology assessments is thus to assess what the societal view on these elements is.** We conducted an exploratory survey as part of this project and present its results in Chapter 5.

Once a set of new value elements is agreed upon, the question of how to measure them becomes more relevant. As discussed in this chapter, **there are still a lack of consensus in the field regarding how best to capture these concepts** in a consistent and comparable way.

Perhaps more importantly, there is also the question of **whether these are distinct elements of value or not.** Introducing a novel value element into the assessment of new treatments should ensure that there is a meaningful distinction between treatments or diseases worth making. For example, if all diseases classified as severe also

affect the patient's caregivers, then the inclusion of impacts on caregivers as a separate value element will have little effect on which treatments get approved and which do not. But if only some severe diseases affect caregivers, its introduction will serve to prioritise treatments for those diseases that do affect caregivers.

From the standpoint of the current Norwegian practice, the recommendations by the recent Expert Report on Healthcare Prioritisation seem particularly relevant to the findings in this chapter. Said Expert Group advised to replace the current extended healthcare sector perspective with a strict healthcare sector perspective in health technology assessments. This would exclude considerations such as productivity impacts, since these do not fall under the budget of the health authorities. It is worth thus considering **whether budget allocation decisions across government agencies should constrain the types of value that can be realised from new medical technologies.**

4. Aggregating multiple value elements

Three different approaches have been suggested to compare two or more alternatives across multiple dimensions in health economic analysis. Modified CEA, MCDA and case-by-case decisions all have advantages and disadvantages. Their role in the healthcare assessment landscape should be considered carefully to protect the integrity and transparency of the system.

Once a decision has been made regarding which value elements should be included in the analysis, and how these will be accounted for, the question that remains is how to compare treatments along these different dimensions. This is a challenging problem as HTA bodies require a systematic process through which they can gather the necessary evidence and create a clear rank between alternative treatment options that allows them to make transparent decisions.

Three main approaches for this have been applied in practice or proposed by the health economics literature:

1. Modified cost-effectiveness analysis (mCEA)
2. Multiple-criteria Decision Analysis (MCDA)
3. Case-by-case decisions

Each of these approaches, as well as their advantages and disadvantages, are described briefly in the coming sections.

4.1 Modified cost-effectiveness analysis (mCEA)

As discussed in earlier chapters in this report, standard cost-effectiveness analysis involves estimating an ICER (incremental QALYs divided by incremental costs) and comparing it to pre-established WTP thresholds. This approach incorporates two characteristics of the treatments being compared, namely their health benefits (measured in QALYs) and their costs, and summarises them into a single measure, the ICER.

Modified CEA (mCEA) tweaks this approach by quantitatively incorporating additional criteria to the same framework, either by modifying the ICER or by shifting WTP thresholds. These criteria could be disease severity or disease prevalence, for

example. Norway, the UK and the Netherlands currently implement versions of mCEA in their HTA processes. In Norway, the WTP threshold is increased according to disease severity, while in the UK QALY gains are multiplied, thereby reducing the ICER of treatments for severe diseases. Both approaches achieve the same objective: to incorporate value elements not included in the standard ICER calculation into the decision.

In principle, CEA can be modified to include multiple additional value elements if reliable ways are designed to quantify them or to modify decisions based on them. However, in practice, challenges remain to the design of mCEA value assessment frameworks that incorporate multiple value elements.

For instance, consider the following hypothetical problem faced an HTA body that wants to prioritise treatments for severe diseases and for rare diseases. If a treatment is proposed for a disease that has both high severity (i.e., high priority), and high prevalence (i.e., low priority), how should the HTA body weight each element against each other? This is a good example of the challenge of incorporating additional value criteria systematically to the standard CEA framework.

One solution to this problem could be to establish a clear ranking across these criteria. This is, for example, the approach taken by NICE UK when dealing with their two quantitative decision modifiers: disease severity and highly specialised technologies (HST). In principle, both modifiers could apply simultaneously for a given new treatment and thus potentially lead to the problem described above. However, the NICE UK guidelines state:

For highly specialized technologies, the severity of the condition is already implicitly captured in the selection of technologies for evaluations. No additional QALY weighting for the severity of the disease is applied. [7]

This pragmatic approach is also the one proposed by TLV when discussing the possibility of introducing a prevalence decision modifier. Their

suggestion is only to assess whether a disease fulfils the criteria for an increased WTP based on its prevalence once it is determined that it is also a very severe disease [5].

If, on the other hand, a strict ranking of the importance of the different value elements being assessed is not possible, or deemed inappropriate, HTA bodies have generally opted for a case-by-case approach, leaving a degree of discretion in the hands of decision makers to use their own judgment when weighting factors against each other (see Section 4.3).

4.2 Multiple-criteria Decision Analysis (MCDA)

Multiple-criteria Decision Analysis (MCDA) is an approach especially designed for conducting comparative analysis of two or more competing alternatives along multiple dimensions.

MCDA consists of a structured deliberation process in which a range of stakeholders are invited to participate in both the selection of the relevant elements of value to include in the assessment, as well as in the choice of the weights assigned to

each element. Alternatives are then compared based on an overall score, calculated based on the agreed upon weights. Quantitative MCDA, in which numerical scores are assigned to each of the alternatives compared, often consists of seven distinct steps as shown in Figure 4-1.

4.2.1 Example of an MCDA process

Once a decision problem has been selected and the relevant elements of value to be assessed have been chosen, a performance matrix is developed in which the results for each alternative are presented. In the example below, two treatments, A and B, directed at different diseases are being compared against each other (Table 4-1).

Table 4-1: MCDA Performance matrix

Treatment	Cost (NOK)	Severity	Rare?
A	1,000	3	No
B	3,000	1	Yes

Source: Oslo Economics

As one can see, the scores that each alternative receives for each value element are challenging to aggregate directly. In this case, costs are expressed in monetary terms, disease severity is expressed as a score and rarity is a binary variable.

The next steps of the analysis are therefore to discuss with stakeholders how to convert these values into comparable scores for each value element, and how to weight each value element's score in the construction of an overall score. Suppose that these discussions lead to the outcome presented the Results matrix presented below (Table 4-2).

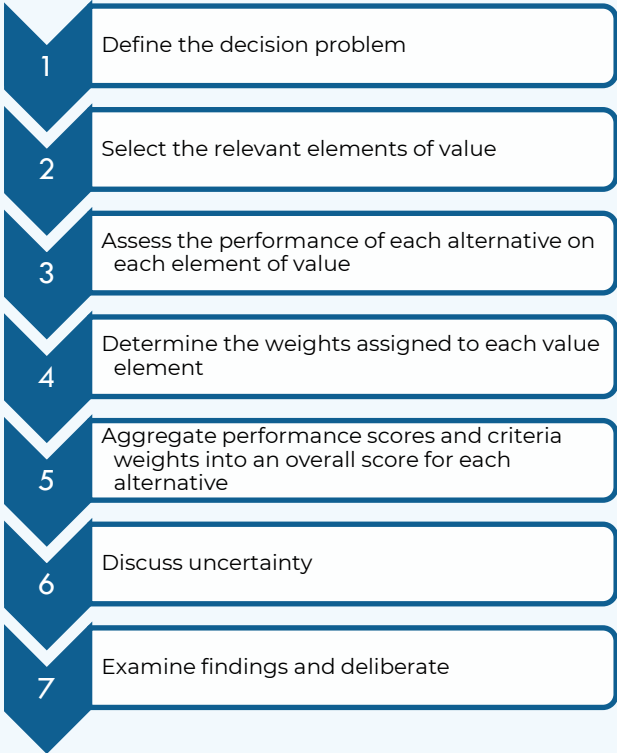
Table 4-2: MCDA Results matrix

Treatment	Cost (NOK)	Severity	Rare?	Score
Weight	50%	25%	25%	-
A	33	100	0	42
B	100	33	100	83

Source: Oslo Economics

In this example, stakeholders have agreed to give treatment cost a 50% weight, disease severity a 25% weight and disease rarity a 25% weight. As a result, the overall score for alternatives A and B was 42 and 83, respectively, concluding that Treatment B is best candidate for introduction.

Figure 4-1: Steps in the application of quantitative MCDA



Source: Oslo Economics based on [1, 2].

4.2.2 Remaining challenges

MCDA can be an effective way to assess alternatives across dimensions that are challenging to evaluate together, either because each value element is measured in different ways or because the relative importance of each element in the overall picture is unclear. It provides a structure for deliberation and assessment that allows a wide range of stakeholders to participate in these discussions and provide input at different stages, giving decision makers a wide range of perspectives on which to base their choices. There are, however, a few important limitations to the widespread use of MCDA in HTA processes for new medical treatments.

First, MCDA can be both time and resource intensive, requiring the input of multiple stakeholders during several rounds for each decision. As of April 2024, the Norwegian Medicines Agency was actively working on the assessment of 42 different treatments simultaneously. Introducing an MCDA structure to these assessments may lead to longer processing times.

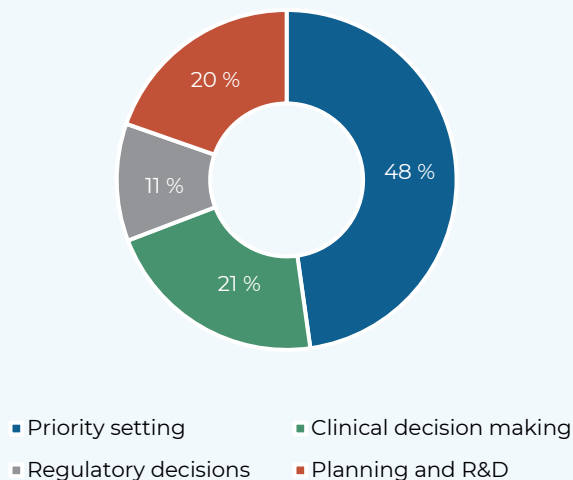
Perhaps more importantly, the nature of the MCDA approach in which stakeholders define the relevant value elements to assess, as well as the weights assigned to them in each individual assessment opens the door for inconsistencies across different decisions. This may lead to lower predictability at the system level. It is perhaps for this reason that the use of MCDA for HTA or reimbursement decisions of pharmaceuticals remains rare (Figure 4-2).

4.3 Case-by-case decisions

Additional value elements are currently mostly considered as qualitative decision modifiers by the HTA bodies reviewed in this report and the ICER institute. This means that any evidence supporting said value may be considered as a relevant factor by the evaluating bodies, but without established guidelines regarding how (or how much) this may ultimately affect their decisions.

This eliminates the practical challenge of having to document and quantify each of the different value elements in a comparable way, giving decision makers a degree of flexibility in their decisions. This flexibility might be warranted, when important elements of value are challenging to measure or when documenting them in a quantitative way is not realistic. However, unless decisions are clearly explained and justified, this may lead to a real or perceived lack of transparency and predictability.

Figure 4-2: Decision context for MCDA studies (1990-2020)



Source: Oslo Economics, adapted from [3, 15]. Of the studies included in the Regulatory decisions class, only a small subset refer to assessments for new treatments in a setting similar to that of a formal HTA process for reimbursement purposes.

4.4 What works best?

In decision-making and prioritising scarce resources between patient groups, it is crucial that HTA bodies employ a method that enables them to compare novel interventions up against each other, and against already established treatment options. **Cost-effectiveness analysis has some clear advantages in this context.** At the same time, the method has weaknesses in terms of including multiple value elements, as discussed in the previous chapter. As such, new methods for assessing novel interventions have been proposed in health economics literature.

Both the modified CEA and MCDA have advantages over ad-hoc approaches; they are systematic and transparent. Modified CEA has some advantages compared to MCDA in this context, as it allows for comparable results across individual assessments and thus increases predictability. **Cost-effectiveness is thus likely to remain the key criteria in the context of decision-making for new pharmaceutical products,** regardless of method applied [38]. As opposed to MCDA, CEA was, however, not designed to encompass multiple criteria at once, and **challenges remain in designing modified CEA algorithms that account for multiple value elements** simultaneously.

On the other hand, **structured deliberation processes such as MCDA are currently being used** by both the ICER institute and HTA bodies, such as NICE, to update their methods and processes [38]. Given that these are not processes that happen very frequently, the time and resource intensity of such an approach is not such a large hindrance. And the resulting structured, documented and transparent deliberation aims to increase the overall trust and predictability of the system as a whole.

Finally, the fact that some elements of value may prove challenging to quantify or might be only relevant in a subset of assessments, highlights the **value of leaving some flexibility on the hands of HTA bodies themselves**, to assess relevant criteria qualitatively on a case-by-case basis, as is done among several today. However, the shortcomings of this approach should be carefully avoided by documenting clearly and transparently the rationale behind each decision.

5. What constitutes fair and equal access to healthcare?

How value is assessed and understood is an ethical question and the principles applied in practice ultimately a political decision. We conducted a survey among a sample of Norwegian adults to understand their preferences around healthcare prioritisation. Societal views appear to align with a comprehensive approach to value assessment. The effect of treatments on the patient's ability to work appears to be a key concern.

Given that the general public is both the payer and user of the public healthcare system in Norway, it is important that their priorities align with those of the Norwegian healthcare authorities. To explore this question in the Norwegian context, we ran an online survey on a sample of the Norwegian adult population. The survey's objective was to gather the public's preferences regarding different value elements related to hypothetical treatments being introduced into the Norwegian healthcare system and understand what societal views regarding these are.

5.1 Objectives

The survey was meant as a preliminary scoping exercise and not as an academic study. It did not include burdensome calculations or precise trade-offs for respondents, to ensure a high level of participation. Results should thus be interpreted with care. The survey was also kept short to maintain completion rates as high as possible. A more robust data collection exercise would be necessary for the purpose of informing policy, in which respondents are faced more complex choices, and particularly, where they face choices between multiple different dimensions simultaneously such as is done in discrete choice experiments.

Ideally, such surveys should also be conducted multiple times to ensure that observed results replicate across studies. The present survey is however among the first of its kind including multiple prioritisation criteria conducted in Norway,

since a 2010 study on preferences regarding orphan drugs [28].

The exercise consisted of a brief introduction to the topic followed by five questions (see Appendix A for full details on the survey materials). The survey was conducted from April 9, 2024, to April 17, 2024 by Respons Analyse.

5.2 Survey respondents

A total of 1,010 Norwegians aged 18 and above completed the survey. Respondent's characteristics were relatively well aligned with the general Norwegian population in terms of gender, age and region of residence (Table A 1). Sampling weights were applied to each individual respondent to further match the distribution of these traits among the general adult Norwegian population. All results presented in the following sections will therefore be presented after weighting factors have been applied.

5.3 Results

5.3.1 Priorities within the allocation of the health budget

First, we asked the respondents whether they agreed or disagreed with six statements regarding which kind of diseases or treatments should be prioritised by the Norwegian healthcare sector. The first statement was meant to capture the perceived importance of cost-effectiveness of new treatments. The following five statements delved into the value elements discussed in the previous chapter: disease severity, prevalence, impact on caregivers, productivity and uncertainty.

There was little disagreement with the statement that the health budget should be allocated in such a way that it provides the best possible health for the population; 94 percent of respondents either agreed or strongly agreed with this (Figure 5-1).

When it came to disease severity, the third key prioritisation criteria (besides cost and benefit) included in Norwegian healthcare assessments, however, there was significantly less agreement. Fewer than half (42%) of the respondents agreed or strongly agreed that patients with the most severe illnesses should receive priority, despite other groups with less severe illnesses could achieve greater health benefits with the same resources. One in three respondents neither agreed nor

disagreed, while 16 percent disagreed or strongly disagreed with the statement. Similar results were found for statements referring to prioritisation of uncommon diseases, and diseases that have large impacts on caregivers (36% and 39% agreement, respectively).

Almost 60 percent of respondents agreed that treatments which enable patients to return to work should be given a higher priority than treatments that do not impact patients' ability to work. Furthermore, 74 percent of respondents agree or strongly agree that treatments known to be effective are prioritised over treatments with more uncertain outcomes.

5.3.2 Scenario questions

Questions 2 through 5 involved more specific scenarios in which respondents were asked to choose an allocation of funds between treatments for two different diseases. The objective with this set of questions was to provide respondents with an

explicit trade-off to consider, between two concrete options. One of these treatment options (the second in each set) was intentionally designed to reflect a standard cost-effectiveness solution.

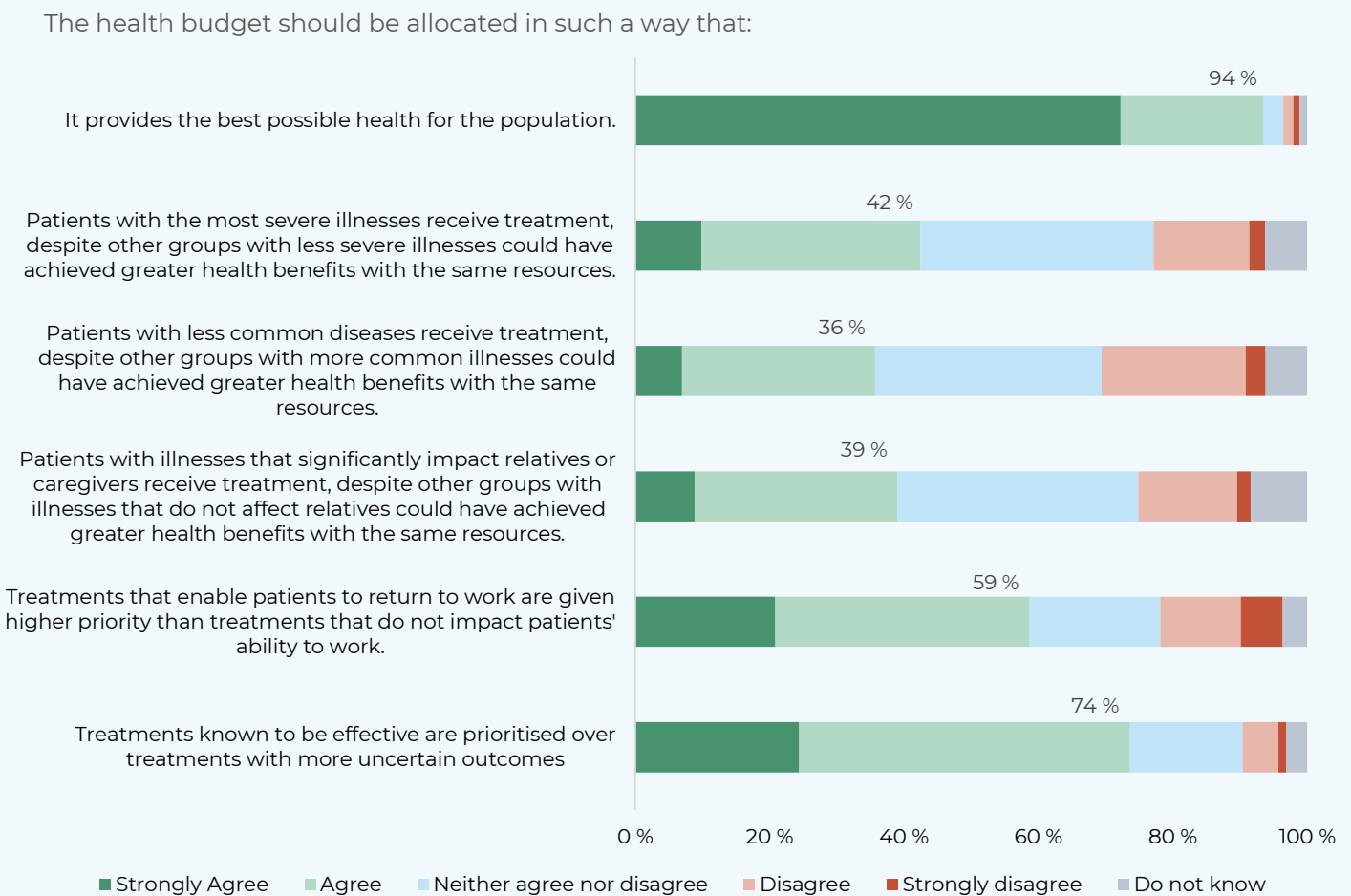
Respondents were then given five possible ways to allocate these hypothetical funds, ranging from fully prioritising the criteria being explored to fully maintaining a cost-effectiveness approach.

5.3.3 Scenario 1: Disease prevalence

In this scenario, respondents were asked to decide between allocating funds to the treatment of either disease A, a rare disease that affects 200 people in Norway a year, or Disease B, a more common disease that affects 10,000 people a year.

Respondents were informed that both diseases have similar effects on patients, and that the treatments are likely to be just as effective for both diseases. However, the treatment for disease A (rare) was four times as expensive as the treatment for disease B (common). Therefore, with the available funds, it was possible to treat a maximum

Figure 5-1: To what extent do you agree or disagree with the following statements?



Source: Oslo Economics based on survey response data provided by Respons Analyse. Data labels indicate the share of respondents who agreed or strongly agreed in each case.

of 100 patients with disease A or 400 patients with disease B. Given that both treatments provide the same health benefits to patients, the treatment for disease B would be superior from a fully utilitarian cost-effectiveness perspective.

Most respondents (46%) would spend most of the funds on treating the common disease and a small portion on the rare disease (Figure 5-2). Interestingly, 38 percent of respondents opted for spending at least half of the resources on treating the more uncommon disease, despite it being four times more expensive than the alternative (Table 5-1).

5.3.4 Scenario 2: Disease severity

In this scenario, disease C is a severe disease that leads to patients being bed-ridden for the rest of their lives, while disease D is a milder disease that leads to some pain and discomfort for the rest of the patients' lives. Both treatments lead to the same health benefits for the patients; to feel moderately better than in the absence of treatment. However, it is four times more expensive to treat the more severe disease (C).

In this case, responses were more evenly distributed than for the case of prevalence, with no alternative receiving more than 27 percent of the votes (Figure 5-2). Perhaps surprisingly given the responses to our first question, 58 percent of respondents opted for spending at least half of the funds on the treatment for the more severe disease, despite its cost (Table 5-1).

5.3.5 Scenario 3: Impact on caregivers

Respondents were next asked to decide between treating a disease which affects both the patients and their caregivers and a disease that only affects the patients. Diseases were assumed to be equally severe, and treatments were assumed to be just as effective for the patients, but for disease E the treatment would allow caregivers to be free from their caregiving duties. Most respondents believed the funds should either be allocated equally across the two diseases (35%) or spent primarily on treating the disease affecting caregivers (25%) (Figure 5-2).

5.3.6 Scenario 4: Productivity

Finally, respondents were asked to allocate the same funds between a disease that makes the patients unable to work (G) and one that does not affect the patient's ability to work (H). Thus, while the treatments for each disease were assumed to have the same impact on the patients' health, in the case of disease G, getting treatment would also affect the patients' ability to work. For additional clarity, respondents were told that patients of

disease H were not able to work and would continue to be so despite receiving treatment. In line with the previous scenarios, it was finally assumed that was four times as expensive to treat disease G.

In this scenario, 18 percent of respondents opted for treating the disease that would allow patients to get back to work, despite this being the most expensive of the two alternatives. A total of 78 percent opted for spending at least half of the funds on this disease, the highest share observed from any of the scenarios presented (Table 5-1).

Table 5-1: Share of respondents willing to spend at least 50 percent of the funds on the less cost-effective treatment

Criteria	Share of responders (%)
Prevalence	38
Severity	58
Impact on caregivers	66
Productivity	78

Source: Oslo Economics

5.4 Key findings

There is value beyond cost-effectiveness

In the first question in our survey, 94 percent of respondents stated that they agreed or strongly agreed with the statement that the health budget should be allocated so that it provides the best possible health for the population. This aligns with the strict utilitarian perspective of achieving the most good with the resources available, regardless of how these benefits are distributed. **In line with this, only 36 percent said they agreed (or strongly agreed) with prioritising treatment for patients with less common diseases at the expense of treatment for more common illnesses.**

However, in scenario questions, when faced with the choice between two treatments providing the same health benefit, very few respondents opted for the alternative that maximised health (e.g., in scenario 1 to "Use all funds on Disease B"). Most respondents chose instead to spend at least part of these funds on the more expensive treatments, thereby reducing the total number of patients treated. **This suggests that people do**

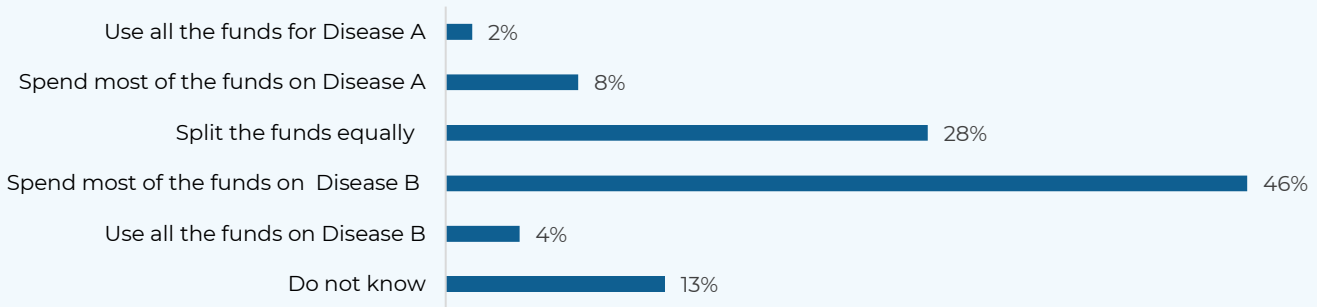
take other factors into consideration besides cost-effectiveness.

Being able to work is something worth paying for

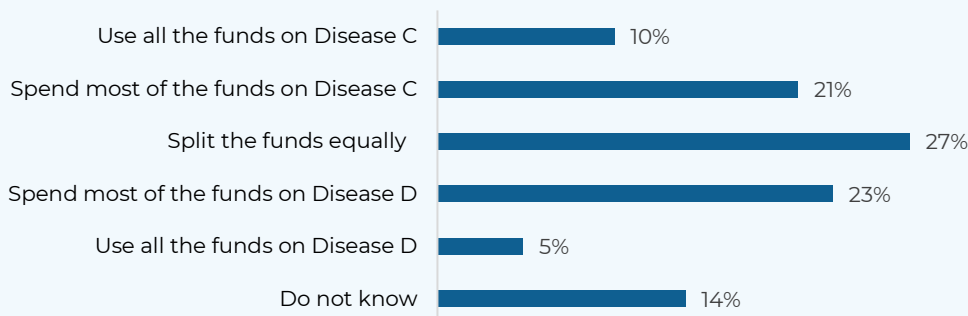
A total of 78 percent of respondents stated that they would spend at least half of the funds on an expensive treatment allowing patients to return to work in Scenario 4, by far the highest share across all four scenarios. **The productivity effects of healthcare treatments appear to be more valuable for respondents in our sample than other characteristics such as whether treatments are directed at severe, rare or diseases that affect the patient's caregivers.** This is particularly interesting in light of the recent recommendations to the Norwegian Health Ministry drafted by an Expert Panel recommending against the inclusion of such effects in health economic assessments [12].

Figure 5-2: How should the healthcare sector allocate extra funds between two different treatments?

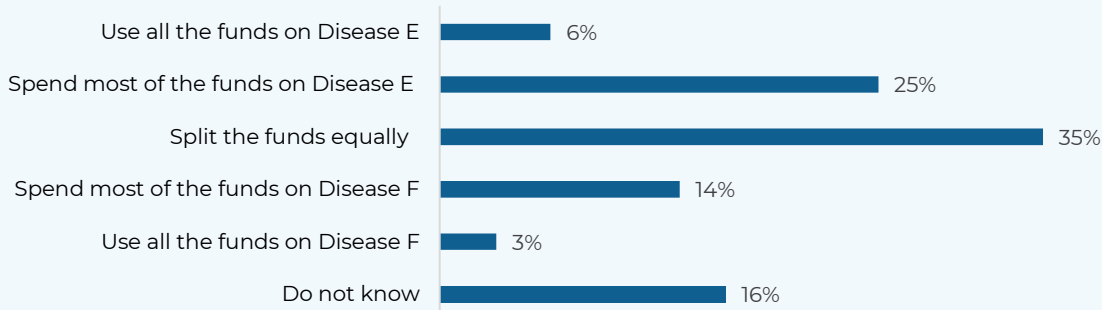
Scenario 1: Between Disease A (rare) and B (common)



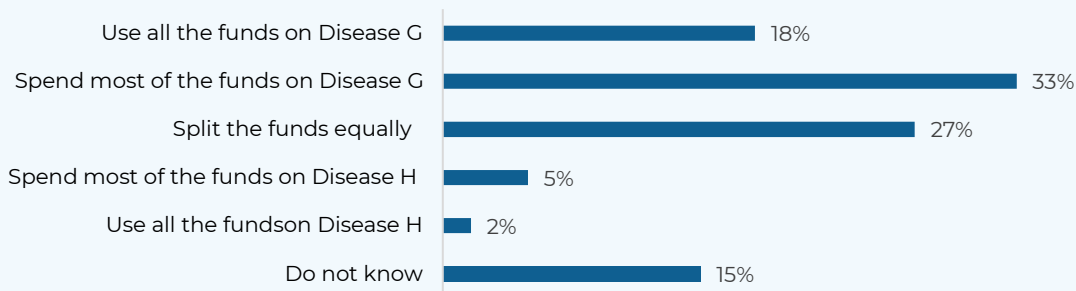
Scenario 2: Between Disease C (severe) and D (mild)



Scenario 3: Between Disease E (affects patient and caregivers) and F (affects patient only)



Scenario 4: Between Disease G (if treated, can go back to work) and H (no impact of treatment on work ability)



Source: Oslo Economics based on survey response data provided by Respons Analyse. Note: In each scenario, respondents were asked to choose how they think additional healthcare funds should be spent between two alternatives. In each scenario, the treatment for the first disease (i.e., the one aimed at the rare diseases, the severe disease, the disease affecting both patients and caregivers or the treatment that would allow patients to get back to work) was four times as expensive as the treatment for the second disease.

6. Conclusions and recommendations

The rapidly evolving landscape of the development of new pharmaceuticals puts pressure on national HTA bodies to tackle both the increasing quantity and complexity of the decision problems they face. This report provides an overview of the approaches taken and value elements considered by different HTA bodies, as well as the key methodological challenges that remain. A survey of 1,000 Norwegian adults was conducted to incorporate their views on healthcare prioritisation decisions. This chapter summarises our key findings.

There is still a lack of consensus on what constitutes value

Across the nine national HTA bodies assessed (Chapter 2), we found that most incorporate additional value elements into their decisions besides cost-effectiveness (i.e., net costs and QALYs gained). Disease severity and uncertainty are currently the most prevalent decision modifiers considered either quantitatively or qualitatively, with a number of agencies also considering disease prevalence, impact on caregivers, productivity losses and equity as relevant for their decisions.

There is nevertheless a significant amount of heterogeneity across HTA bodies from countries with otherwise comparable healthcare systems and priorities. This may partly be explained by HTA bodies being instructed to adopt slightly different analysis perspectives by their national health authorities, and thus be beyond each individual HTA body's control. However, inconsistencies remain between agencies operating under very similar mandates (such as Norway and Sweden). This leads to a lack of clarity from the perspective of patients and producers.

Societal views support a more comprehensive approach to value

The results of our survey revealed that Norwegians are often willing to trade-off health gains in favour of other criteria they deem just as important as cost-effectiveness. These criteria include disease severity, whether a treatment allows patients to return to work, the impact of the disease on caregivers or disease prevalence. The degree of

value they place on each of these elements, and therefore how willing they are to deviate from a standard cost-effectiveness approach in each case, varies.

Interestingly, most respondents were willing to invest in treatments allowing patients to return to work, an interesting finding in light of the recent report by an Expert Committee in Norway that recommended taking a strict healthcare sector perspective and thus ignore these impacts in health technology assessments.

Our findings from the survey reflect the challenge of defining what fair and equal access to healthcare means in practice. They also suggest that the inclusion of multiple value elements into health technology aligns with societal views on the matter.

Challenges remain regarding how to measure the value provided by new treatments

Other than cost-effectiveness, most value elements considered in HTA processes in the countries reviewed in this report are incorporated into assessments in a qualitative way. This lack of clear guidance on the methods used and the weights applied to each value element make it hard to compare practices across borders.

Even among those elements for which explicit guidance exists, there is significant variation in approaches across countries and a series of unresolved issues remain. For example, Norway, the UK and the Netherlands currently define disease severity very differently. The three countries also disagree quite strongly on how much higher their WTP threshold should be for severe versus non-severe conditions. The Netherlands also stands out among our sample as the single country in which productivity losses are considered, while others have implicitly or explicitly decided to exclude them from their main analyses.

Further research and continuous development of HTA methodologies is needed to address these remaining challenges.

Transparency and predictability can be improved

The current lack of a harmonised approach to HTA evaluations across countries leads to a situation in which some patients have access to promising treatments in some countries but not in others. There may be good reasons for this, for example, different prioritisation criteria being used in each

country. This highlights the importance of communicating the rationale for each decision clearly and transparently.

Transparency and predictability are key for maintaining trust between pharmaceutical developers, regulatory agencies and the public. Transparency is also the most effective tool that HTA bodies have, to communicate the right incentives to pharmaceutical producers. Clear guidance on the methodologies applied and the criteria used for assessing value contribute to this transparency.

A degree of discretion may nevertheless be warranted in HTA processes. Allowing HTA bodies to assess specific criteria qualitatively on a case-by-case basis, for example, may be a pragmatic solution to data availability and/or measurement challenges. Qualitative assessments are thus a common approach across the countries surveyed for this report

Regardless of the approach used by HTA agencies, there are strong arguments towards pushing for increased transparency in the rationale and methodologies being used, the evidence and the assumptions underlying each reimbursement decision. At the system level, transparent processes of structured deliberation regarding potential changes to existing methodologies, in which multiple perspectives are invited to participate, help build trust between stakeholders.

International collaboration can unlock further progress

The current landscape of increased quantity and complexity of health technology assessments puts national HTA bodies under significant pressure. While countries may have slightly different priorities and objectives, international collaboration may contribute to increased harmonisation of methods across borders.

Nordic HTA bodies have recognised this opportunity. Norway, Finland and Sweden established a joint assessment process (FINOSE) in 2017, which was extended for another three years in 2023 with the incorporation of Denmark and Iceland and renamed Joint Nordic HTA Bodies (JNHB). HTA agencies from Australia, Canada, New Zealand and the UK established their own group, which includes collaboration in methods development as one of their three key priorities [39]. Collaboration in terms of methodological development is not currently an objective of the JNHB but should be carefully considered over the coming years.

The European Network for HTA (EUnetHTA) has proposed a methodology and process for conducting joint clinical assessments at the European level and is another international forum for the advancement of HTA methods.

Such platforms for collaboration can contribute to harmonise approaches across borders as well as provide HTA bodies with needed support in the development of methods and guidelines.

7. References

1. Baltussen, R., et al., *Multicriteria decision analysis to support health technology assessment agencies: benefits, limitations, and the way forward*. Value in Health, 2019. **22**(11): p. 1283-1288.
2. Thokala, P., et al., *Multiple criteria decision analysis for health care decision making—an introduction: report 1 of the ISPOR MCDA Emerging Good Practices Task Force*. Value in health, 2016. **19**(1): p. 1-13.
3. Lundin, D. and A. Alassaad, *How should we assess and pay? Health-economic assessments and payment models for precision medicines and ATMPs*. 2021, Tandvårds- och läkemedelsförmånsverket: Stockholm. p. 1-161.
4. Tandvårds- och Läkemedelsverket, *Introduktion till hälsoekonomisk utvärdering*. 2023, TLV: Stockholm.
5. TLV, *Stärkt tillgång till läkemedel vid sällsynta hälsotillstånd - till långsiktigt hållbara läkemedelskostnader*. 2023, Tandvårds- och läkemedelsförmånsverket: Stockholm.
6. Helse- og omsorgsdepartementet, *På ramme alvor - Alvorlighet og prioritering*, in *Rapport fra arbeidsgruppe nedsatt av Helse- og omsorgsdepartementet*. 2015.
7. Health, N.I.f. and C. Excellence, *NICE health technology evaluations: the manual*. 2022.
8. Reckers-Droog, V., J. van Exel, and W. Brouwer, *Willingness to Pay for Health-Related Quality of Life Gains in Relation to Disease Severity and the Age of Patients*. Value Health, 2021. **24**(8): p. 1182-1192.
9. Neumann, P.J., L.P. Garrison, and R.J. Willke, *The history and future of the “ISPOR value flower”: addressing limitations of conventional cost-effectiveness analysis*. Value in Health, 2022. **25**(4): p. 558-565.
10. Norwegian Medicines Agency, *Submission Guidelines - For single technology assessment of medicinal products*. 2023, Norwegian Medical Products Agency. p. 1-65.
11. Jiang, S., et al., *Incorporating productivity loss in health economic evaluations: a review of guidelines and practices worldwide for research agenda in China*. BMJ Global Health, 2022. **7**(8): p. e009777.
12. Melberg, H.O., et al., *Perspektiv og prioriteringer. Rapport fra ekspertgruppen perspektiv i prioritering nedsatt av Helse- og omsorgdepartementet*. 2024, Helse- og omsorgdepartementet: Oslo. p. 1-137.
13. Radu, P., et al., *Navigating change: a comparative analysis across health technology assessment agencies on their positions on five key topics*, in *OHE Contract Research Report*. 2024, Office of Health Economics: London.
14. Council, T.D.M., *The Danish Medicines Council methods guide for assessing new pharmaceuticals*. 2020.
15. Gongora-Salazar, P., et al., *The use of multicriteria decision analysis to support decision making in healthcare: an updated systematic literature review*. Value in Health, 2023. **26**(5): p. 780-790.
16. CADTH, *Guidelines for the Economic Evaluation of Health Technologies: Canada*. 2017.
17. Fimea, *Rapid assessment of new hospital-only medicinal products*. 2018, Finnish Medicines Agency
18. Excellence, N.I.f.H.a.C., *CHTE methods review Modifiers Task and finish group report*. 2020: p. 1-85.
19. Claxton, K., et al., *Value based pricing for NHS drugs: an opportunity not to be missed?* Bmj, 2008. **336**(7638): p. 251-4.
20. HAS, *Choices in methods for economic evaluation - HAS*, in *Health technology*. 2020, Haute autorite de sante.
21. Nederland, Z., *Guideline for Economic Evaluations in Healthcare*. Zorginstituut Nederland. 2016, Diemen, The Netherlands.
22. PBAC, *Guidelines for preparing a submission to the Pharmaceutical Benefits Advisory Committee*. 2016.
23. Zhang, K.G., M., *International Cost-Effectiveness Thresholds and Modifiers for HTA Decision Making*. OHE Consulting Report, 2020.
24. DMP. *Særskilt små pasientgrupper med svært alvorlig tilstand*. 2017 14.12.2023 [cited 2024 07.08.2024]; Available from: <https://www.dmp.no/offentlig-finansiering/metodevurdering-av-legemidler/dokumentasjon-for-metodevurdering/hvordan-sikre-tilgang-til-legemidler-for-serskilt-sma-pasientgrupper-med-svert-alvorlig-tilstand>.
25. Oslo Economics, *Genterapi i Norge: Status, utfordringer og muligheter*. 2021, Oslo Economics: Oslo.
26. McCabe, C., et al., *Orphan drugs revisited*. Journal of the Association of Physicians, 2006. **99**(5): p. 341-345.
27. McCabe, C., K. Claxton, and A. Tsuchiya, *Orphan drugs and the NHS: should we value rarity?* Bmj, 2005. **331**(7523): p. 1016-1019.
28. Desser, A.S., et al., *Societal views on orphan drugs: cross sectional survey of Norwegians aged 40 to 67*. BMj, 2010. **341**.
29. Johnston, K.M., et al., *Comparing Preferences for Disease Profiles: A Discrete Choice Experiment from a US Societal Perspective*. Applied Health Economics and Health Policy, 2024. **22**(3): p. 343-352.

30. Bourke, S.M., C.O. Plumpton, and D.A. Hughes, *Societal Preferences for Funding Orphan Drugs in the United Kingdom: An Application of Person Trade-Off and Discrete Choice Experiment Methods*. *Value Health*, 2018. **21**(5): p. 538-546.
31. van de Wetering, E.J., et al., *Are some QALYs more equal than others?* *Eur J Health Econ*, 2016. **17**(2): p. 117-27.
32. Blonda, A., et al., *How to value orphan drugs? A review of European value assessment frameworks*. *Frontiers in Pharmacology*, 2021. **12**: p. 631527.
33. Skedgel, C., et al., *Considering severity in health technology assessment: can we do better?* *Value in Health*, 2022. **25**(8): p. 1399-1403.
34. ICER, *Value Assessment Framework*. 2023, Institute for Clinical and Economic Review.
35. Kinge, J.M., et al., *Production losses from morbidity and mortality by disease, age and sex in Norway*. *Scandinavian Journal of Public Health*, 2023: p. 14034948231188237.
36. Sanders, G.D., et al., *Recommendations for Conduct, Methodological Practices, and Reporting of Cost-effectiveness Analyses: Second Panel on Cost-Effectiveness in Health and Medicine*. *Jama*, 2016. **316**(10): p. 1093-103.
37. ICER, *ICER's Reference Case for Economic Evaluations: Elements and Rationale*. 2023, Institute for Clinical and Economic Review.
38. Garrison, L.P., *Multicriteria Decision Analysis and Value Assessment Frameworks: Where Do We Stand? What Next?* *Value in Health*, 2024. **27**(1): p. 3-6.
39. NICE. *International health technology assessment collaboration expands* [Accessed: May 18th, 2024]. 2023; Available from: <https://www.nice.org.uk/news/article/international-health-technology-assessment-collaboration-expands>.

Appendix A Social preference survey materials and additional information

A.1 Survey materials

What follows below is the full text (in Norwegian) of the survey conducted to elicit social preferences regarding healthcare prioritisation from a representative sample of Norwegian adults.

Før nye behandlinger (f.eks. et legemiddel) blir tatt i bruk i den norske helsetjenesten gjør helsemyndighetene en vurdering av behandlingen basert på tre overordnede kriterier. Disse kriteriene inkluderer helseeffekten av behandlingen, hvor mye behandlingen koster, og hvor alvorlig sykdommen som behandlingen virker for anses å være. Når for eksempel et nytt legemiddel innføres i helsetjenesten innebærer dette at det er mindre ressurser som kan benyttes på behandlingsalternativer for andre pasienter.

I de påfølgende spørsmålene ønsker vi å få ditt syn på hvilke behandlinger som bør dekkes av den offentlige helsetjenesten. Spørsmålene er formulert for å kunne gi oss en bedre forståelse av hvordan den norske befolkningen verdsetter og prioriterer behandlinger av ulike typer sykdommer.

Q1 – Hvor enig/uenig er du i følgende utsagn knyttet til prioriteringer innenfor helsebudsjettet?

	Svært uenig	Uenig	Verken enig eller uenig	Enig	Svært enig	Vet ikke
Helsebudsjettet bør brukes på en måte som gir best mulig helse i befolkningen.						
Helsebudsjettet bør brukes slik at pasienter med de mest alvorlige sykdommene får behandling, selv om det er andre grupper med mindre alvorlige sykdommer som kunne oppnådd en større helsegevinst med de samme ressursene.						
Helsebudsjettet bør brukes slik at pasienter med sjeldne sykdommer får behandling, selv om det er andre grupper med mer vanlige sykdommer som kunne oppnådd en større helsegevinst med de samme ressursene.						
Helsebudsjettet bør brukes slik at pasienter med sykdommer som har store negative virkninger for pårørende/omsorgspersoner får behandling, selv om det er andre grupper med sykdommer som ikke rammer pårørende som kunne oppnådd en større helsegevinst med de samme ressursene.						
Helsebudsjettet bør brukes slik at behandlinger som gjør det mulig for pasienter å komme tilbake i arbeid blir prioritert høyere enn behandlinger som ikke påvirker pasienters arbeidsevne.						
Helsebudsjettet bør brukes slik at behandlinger vi vet har god effekt prioriteres høyere enn behandlinger der effekten er mer usikker.						

I det følgende vil vi be deg vurdere noen eksempler på valg en gjerne må ta i prioriteringen av behandlinger i helsevesenet

Q2 – Forestill deg at helsebudsjettet øker med 1 million kroner, og at helsemyndighetene må fordele pengene mellom behandlinger av to forskjellige sykdommer;

- Sykdom A: en sjelden sykdom som rammer 200 personer i Norge hvert år
- Sykdom B: en mer vanlig sykdom som rammer 10 000 personer i Norge hvert år (med samme kjennetegn (alder/kjønn) som sykdom A)

Videre antar vi at sykdommene påvirker pasientens helse på nokså lik måte, men at det er fire ganger så dyrt å behandle sykdom A som sykdom B. Grunnet prisen på behandlingen er det kun mulig å behandle 100 pasienter med sykdom A eller alternativt 400 pasienter med sykdom B innenfor det økte budsjettet på 1 million kroner.

Hvordan mener du at helsemyndighetene bør benytte den økte budsjettbevilgningen?

1: Bruke alle midlene til å behandle sykdom A. (Sykdom A = 100 pasienter; Sykdom B = 0 pasienter; Totalt behandlede pasienter = 100)

2: Bruke mesteparten av midlene til å behandle sykdom A, men også bruke et lite beløp på å behandle sykdom B (Sykdom A = 75 pasienter; Sykdom B = 100 pasienter; Totalt behandlede pasienter = 175)

3: Dele midlene likt (50-50) mellom behandling av sykdom A og B (Sykdom A = 50 pasienter; Sykdom B = 200 pasienter; Totalt behandlede pasienter = 250)

4: Bruke mesteparten av midlene til å behandle sykdom B, men også bruke et lite beløp på å behandle sykdom A (Sykdom A = 25 pasienter; Sykdom B = 300 pasienter; Totalt behandlede pasienter = 325)

5: Bruke alle midlene til å behandle sykdom B (Sykdom A = 0 pasienter; Sykdom B = 400 pasienter; Totalt behandlede pasienter = 400)

6: Vet ikke

Q3 – Forestill deg nå at helsemyndighetene må fordele de samme midlene mellom behandling av to andre sykdommer:

- Sykdom C: en alvorlig sykdom (pasienter er sengeliggende resten av livet), og
- Sykdom D: en mildere sykdom (noe smerte eller ubehag hver dag resten av pasientens liv).

Begge behandlingene gir den samme helsegevinsten for pasientene – å føle seg moderat bedre enn de ville gjort ved fravær av behandling. Det er fire ganger så dyrt å behandle sykdom C som sykdom D, noe som betyr at med disse midlene er det mulig å maksimalt behandle enten 100 pasienter med sykdom C eller alternativt 400 pasienter med sykdom D.

Hvordan bør myndighetene benytte disse midlene?

1: Bruke alle midlene til å behandle sykdom C. (Sykdom C = 100 pasienter; Sykdom D = 0 pasienter; Totalt behandlede pasienter = 100)

2: Bruke mesteparten av midlene til å behandle sykdom C, men også bruke et lite beløp på å behandle sykdom D (Sykdom C = 75 pasienter; Sykdom D = 100 pasienter; Totalt behandlede pasienter = 175)

3: Dele midlene likt (50-50) mellom behandling av sykdom C og D (Sykdom C = 50 pasienter; Sykdom D = 200 pasienter; Totalt behandlede pasienter = 250)

4: Bruke mesteparten av midlene til å behandle sykdom D, men også bruke et lite beløp på å behandle sykdom C (Sykdom C = 25 pasienter; Sykdom D = 300 pasienter; Totalt behandlede pasienter = 325)

5: Bruke alle midlene til å behandle sykdom D (Sykdom C = 0 pasienter; Sykdom D = 400 pasienter; Totalt behandlede pasienter = 400)

6: Vet ikke

Q4 – Forestill deg nå at helsemyndighetene må fordele de samme midlene mellom behandling av to andre sykdommer:

- Sykdom E som rammer pasienter og deres pårørende/omsorgspersoner (de må bruke mye av sin tid på å pleie pasienten), og
- Sykdom F som kun rammer pasienter.

Begge sykdommene er like alvorlige for pasientene. Begge behandlingene gir den samme helsegevinsten til pasientene, men ved sykdom E vil behandlingen også innebære at pårørende/omsorgspersoner ikke lenger behøver å bruke sin tid på å yte omsorg. Det er fire ganger så dyrt å behandle sykdom E som sykdom F, noe som betyr at med disse midlene er det mulig å maksimalt behandle enten 100 pasienter med sykdom E eller alternativt 400 pasienter med sykdom F.

Hvordan bør myndighetene benytte disse midlene?

1: Bruke alle midlene til å behandle sykdom E. (Sykdom E = 100 pasienter; Sykdom F = 0 pasienter; Totalt behandlede pasienter = 100)

2: Bruke mesteparten av midlene til å behandle sykdom E, men også bruke et lite beløp på å behandle sykdom F (Sykdom E = 75 pasienter; Sykdom F = 100 pasienter; Totalt behandlede pasienter = 175)

3: Dele midlene likt (50-50) mellom behandling av sykdom E og F (Sykdom E = 50 pasienter; Sykdom F = 200 pasienter; Totalt behandlede pasienter = 250)

4: Bruke mesteparten av midlene til å behandle sykdom F, men også bruke et lite beløp på å behandle sykdom E (Sykdom E = 25 pasienter; Sykdom F = 300 pasienter; Totalt behandlede pasienter = 325)

5: Bruke alle midlene til å behandle sykdom F (Sykdom E = 0 pasienter; Sykdom F = 400 pasienter; Totalt behandlede pasienter = 400)

6: Vet ikke

Q5 – Til slutt, forestill deg nå at helsemyndighetene må prioritere mellom behandling av to andre sykdommer:

- Sykdom G: Sykdommen gjør at pasienten ikke har mulighet til å jobbe, men om pasienten mottar behandling vil en kunne komme tilbake i jobb.
- Sykdom H: Sykdommen påvirker ikke pasientens evne til å jobbe, og tilgang til behandling vil ikke ha konsekvenser for pasientens arbeidsevne.

Behandling av de to sykdommene har samme nytten for pasientene om man kun ser på hvordan behandlingen påvirker pasientens helse. Behandlingene er ulike ved at den for sykdom G gjør at pasienten kan komme tilbake i arbeid, noe som ikke er tilfellet for sykdom H der pasienten kun oppnår en helseeffekt (som er like stor som for sykdom G). Pasientene av sykdom H er ikke i arbeid og kommer ikke til å være det selv om de blir behandlet. Det er fire ganger så dyrt å behandle sykdom G som sykdom H, noe som betyr at med disse midlene er det mulig å maksimalt behandle enten 100 pasienter med sykdom G eller alternativt 400 pasienter med sykdom H.

Hvordan bør myndighetene benytte disse midlene?

1: Bruke alle midlene til å behandle sykdom G. (Sykdom G = 100 pasienter; Sykdom H = 0 pasienter; Totalt behandlede pasienter = 100)

2: Bruke mesteparten av midlene til å behandle sykdom G, men også bruke et lite beløp på å behandle sykdom H (Sykdom G = 75 pasienter; Sykdom H = 100 pasienter; Totalt behandlede pasienter = 175)

3: Dele midlene likt (50-50) mellom behandling av sykdom G og H (Sykdom G = 50 pasienter; Sykdom H = 200 pasienter; Totalt behandlede pasienter = 250)

4: Bruke mesteparten av midlene til å behandle sykdom H, men også bruke et lite beløp på å behandle sykdom G (Sykdom G = 25 pasienter; Sykdom H = 300 pasienter; Totalt behandlede pasienter = 325)

5: Bruke alle midlene til å behandle sykdom G (Sykdom G = 0 pasienter; Sykdom H = 400 pasienter; Totalt behandlede pasienter = 400)

6: Vet ikke

A.2 Additional results

Table A 1: Characteristics of survey respondents

Characteristic	Number of respondents, unweighted (N=1 010)	Share	Number of respondents, weighted (N=1 010)	Share	Share in Norwegian population
Gender					
Male	512	51 %	508	50,3 %	50,3 %
Female	498	49 %	502	49,7 %	49,7 %
Age group					
18-24	91	9,0 %	109	10,8 %	13,3 %
25-34	163	16,1 %	177	17,5 %	17,0 %
35-44	176	17,4 %	169	16,7 %	16,3 %
45-54	191	18,9 %	172	17,0 %	16,6 %
55-64	165	16,3 %	156	15,4 %	15,0 %
65+	224	22,2 %	227	22,5 %	21,8 %
Region					
Oslo og Akershus	264	26,1 %	262	25,9 %	25,9 %
Sør-Østlandet	185	18,3 %	187	18,5 %	18,5 %
Innlandet	69	6,8 %	70	6,9 %	7,0 %
Agder og Rogaland	152	15,0 %	145	14,4 %	14,4 %
Vestlandet	163	16,1 %	167	16,5 %	16,5 %
Trøndelag	88	8,7 %	88	8,7 %	8,7 %
Nord-Norge	89	8,8 %	91	9,0 %	9,0 %
Highest degree of education					
Primary/Secondary	279	27,6 %	274	27,1 %	62,8 %
University degree (max 3 years)	285	28,2 %	289	28,6 %	25,1 %
University degree (4 or more years)	438	43,4 %	440	43,6 %	11,5 %
Not reported	8	0,8 %	7	0,7 %	0,6 %
Household income (pre-tax, NOK)					
< 400.000	79	7,8 %	86	8,5 %	
400.000-599.999	117	11,6 %	116	11,5 %	
600.000-799.999	136	13,5 %	137	13,6 %	

800.000-1.000.000	119	11,8 %	117	11,6 %
1.000.000-1.200.000	111	11,0 %	113	11,2 %
1.200.000-1.400.000	136	13,5 %	137	13,6 %
> 1.400.000	170	16,8 %	162	16,1 %
Not reported	142	14,1 %	141	14,0 %

Source: Oslo Economics. Note: Responses are weighted based on gender, age-group and region of residence to match the national population distribution from Statistics Norway (SSB).

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