

Scope, feasibility and challenges of valuing disability in cost-effectiveness analyses submitted to the French High health authority (HAS)

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Objectives

Nearly 4.3 million people are affected by a disabling condition in France¹ leading to significant economic impacts for society, including disability-related health expenditure, benefits and social allowances to compensate lost incomes and productivity losses.

Thus, in addition to the health benefits, treatments of disability-inducing diseases are likely to have a significant impact on non-medical costs and benefits.

The High health authority (HAS) recommends that cost-effectiveness analyses (CEA) submitted to assess the efficiency of innovative treatments likely to have a significant impact on health care expenses, adopt a collective perspective including "all individuals or institutions affected by the production of cares"². In accordance with this guideline, Health Technology Assessment (HTA) in the field of disabling diseases should consider all resources involved in the production of patient's overall care with regard to their direct costs, regardless of their nature (i.e. health, domestic, or medico-social spheres) and their respective payers (i.e. patients, national health insurance, social allowance services).

In practice, many CEAs are restricted to the healthcare system due to limited evidence and data beyond direct medical costs. However, this approach is detrimental to a proper assessment of interventions in disabling diseases associated with compensation services, goods, benefits, absenteeism and productivity loss. By addressing only a part of disability-related expenditures, this approach is restrictive and excludes many costs that may be affected by an intervention from CEAs reviewed by HAS.

These restrictions may lead to a disregard of some savings allowed by disability-reducing interventions and to a biased demonstration of their medical and economic value.

We examined the challenges and feasibility of valuing disability-related social benefits (DRSB) in the French setting and attempted to quantify the impact of their inclusion on the results of CEAs in the French setting.

Methods

We first analyzed the scope of DRSB and the data available to include them in simulations in the context of HAS guidelines, modalities of disability care and published efficiency opinions in disabling diseases.

Each DRSB was then associated with the health care system, collective and Based on a modeling approach, we secondly developed a hypothetical case to assess the impact of including DRSB in CEAs.

Finally, a three-health-state Markov model was developed to quantify the impact of the choice of perspective on CEA results, for a hypothetical disabling disease case.

Results

Findings on DRSB regarding HAS guidelines and requirements

DRSB represented an annual spending of over 50Bn€ in 2019, a quarter of French social security budget³. However, few data are available to value them in CEAs, mostly consisting of aggregated data, unspecific of disability types, despite the significant heterogeneity between the types of disability, territory and interindividual recourse to welfare benefits and their impact on benefits and costs.

Thus, a comprehensive analysis of the total impact of a disability-reducing interventions would require a societal perspective, not recommended by the HAS because of the lack of robust data to quantify productivity losses.

Indeed, according to HAS guidelines, Disability Compensation Benefit (Prestation de Compensation du Handicap, PCH) and Allowances for disabled adults (Allocation aux Adultes Handicapés, AAH) should be considered as a part of collective and societal perspectives respectively (Table 1).

Table 1. Summary of French DRSB classified according to CEA perspectives.

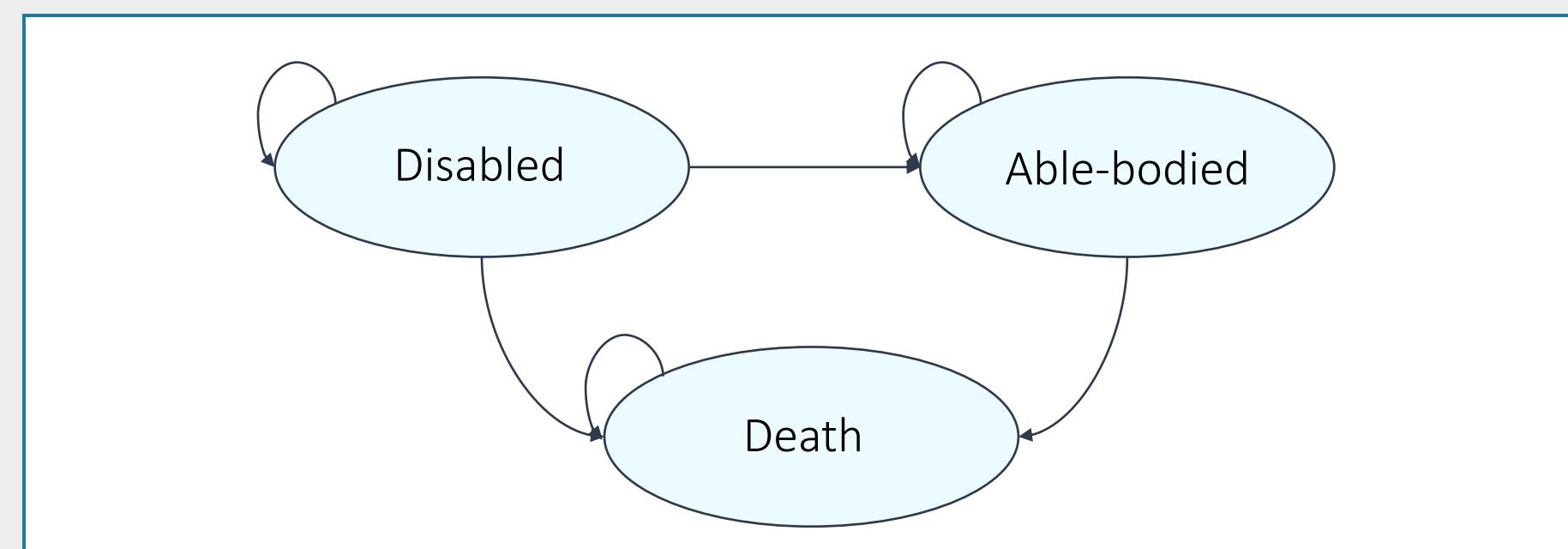
	Healthcare system	Collective	Societal
Disability compensation benefit (PCH)			
Optical assistance	No	Yes	Yes
Non-optical assistance	No	Yes	Yes
Human assistance	No	Yes	Yes
Housing arrangement	No	Yes	Yes
Transportation Assistance	No	Yes	Yes
Specific assistance	No	Yes	Yes
Animal assistance	No	Yes	Yes
Social benefits to compensate disability-induced lost incomes			
AEEH	No	No	Yes
AAH	No	No	Yes
Scholarship supplement	No	No	Yes
Invalidity pension	No	No	Yes
ASI	No	No	Yes
ASPA	No	No	Yes
AJPP	No	No	Yes
Productivity losses and community contribution losses for patients and carers			
Patient productivity losses	No	No	Yes
Informal caregivers productivity losses	No	No	Yes
Patient social participation losses	No	No	Yes
Informal caregivers social participation losses	No	No	Yes

Most efficiency opinions published in the field of disabling diseases were based on CEA restricted to the healthcare system costs. Our review notably highlighted the case of EVRYSDI® (risdiplam) in the treatment of spinal muscular atrophy, a disease associated with major motor disability. In its efficiency opinion, the HAS judged that the CEA's limitation to healthcare represented an important methodological reservation, limiting the relevance of the efficiency opinion.⁴

Application to our hypothetical case

In order to assess the potential impact of including DRSBs in CEAs for treatments in conditions associated with disabilities, we modelled a hypothetical case using a simple Markov model (Figure 1).

Figure 1. Markov model structure.



Methods

- We modeled a population of disabled adult patients. All Model parameters are reported in Table 2.
- Patients were distributed between the three mutually exclusive health states of disability, able-bodied and death. Mortality was assumed to be equivalent to the general mortality of the French population.
- Patients' quality of life and related utility were characterized using the EQ-5D-5L questionnaire⁵. It was assumed that, on average, patients had severe problems for mobility and usual activities and moderate problems for self-care, alongside slight pain/discomfort and slight anxiety/depression (health state 43422).
- A hypothetical disability-reducing intervention was introduced in this population. It was assumed that it allowed an overall 30% reduction in the disability burden of the disease by causing 30% of patients to reach an able-bodied health state consisting in slight mobility, usual activities and self-care issues, with similar pain/discomfort and anxiety/depression as in the disabled patients (health state 22222).
- Both health states were valued based on the French general population preferences⁵, determining a utility increment for able-bodied of 0.28 vs. disabled patients.
- It was assumed that direct medical costs were similar regardless of the disability: all patients were associated with the average per capita health expenditure reported for France⁶.
- 100% of patients in the Disabled health state were supposed to be DRSB recipients, whereas able-bodied patients were considered to no longer require or request DRSBs due to their improved global health status. DRSBs were valued by considering the available annual cost per patient, calculated based on the annual expenditure cost for each DRSB divided by the number of recipients. Productivity losses were valued based on the human capital approach, considering average employment rate and 100% absenteeism in the disabled health state.
- Intervention price was arbitrarily set at €250k for the whole course administered during the first year.
- Costs and outcomes were discounted at a 2,5% annual rate over a lifetime horizon.
- Four analyses were performed according to the following perspectives and including some of the corresponding relevant DRSB (Table 1):
 - A health care system perspective (accepted by HAS in many cases);
 - A collective perspective including PCH costs (recommended by HAS);
 - A societal perspective restricted to the costs supported by the social security (including both PCH and AAH costs);
 - A "full" societal perspective including PCH, AAH and productivity losses.
- Deterministic sensitivity analyses (DSA) were performed on all model parameters (0-5% discounting ; +/-25% variation for cost parameters).

Table 2. Model parameters

Parameters	Health-state	
	Disabled	Able-bodied
Mean age (year)	18	
Discount rate (%)	2,5	
Intervention cost	250 000 €	250 000 €
Transition probabilities		
No treatment - Disabled	1	0
On treatment - Disabled	0,7	0,3
Mortality	French general population mortality	
HRQoL (utility)	0,570	0,850
Disease management costs	3 102 €	
DRSB		
PCH	5 555 €	- €
AAH	11 479 €	- €
Productivity losses	47 658 €	- €

Results

In the perspective restricted to the health care system, our hypothetical intervention was associated with an ICER of 95 260 €/QALY.

Adopting the collective perspective recommended by HAS allowed an ICER reduction of 21%, at 75 412 € by the inclusion of PCH costs (Table 3).

Table 3. Impact of DRSB costs inclusion on CEA results.

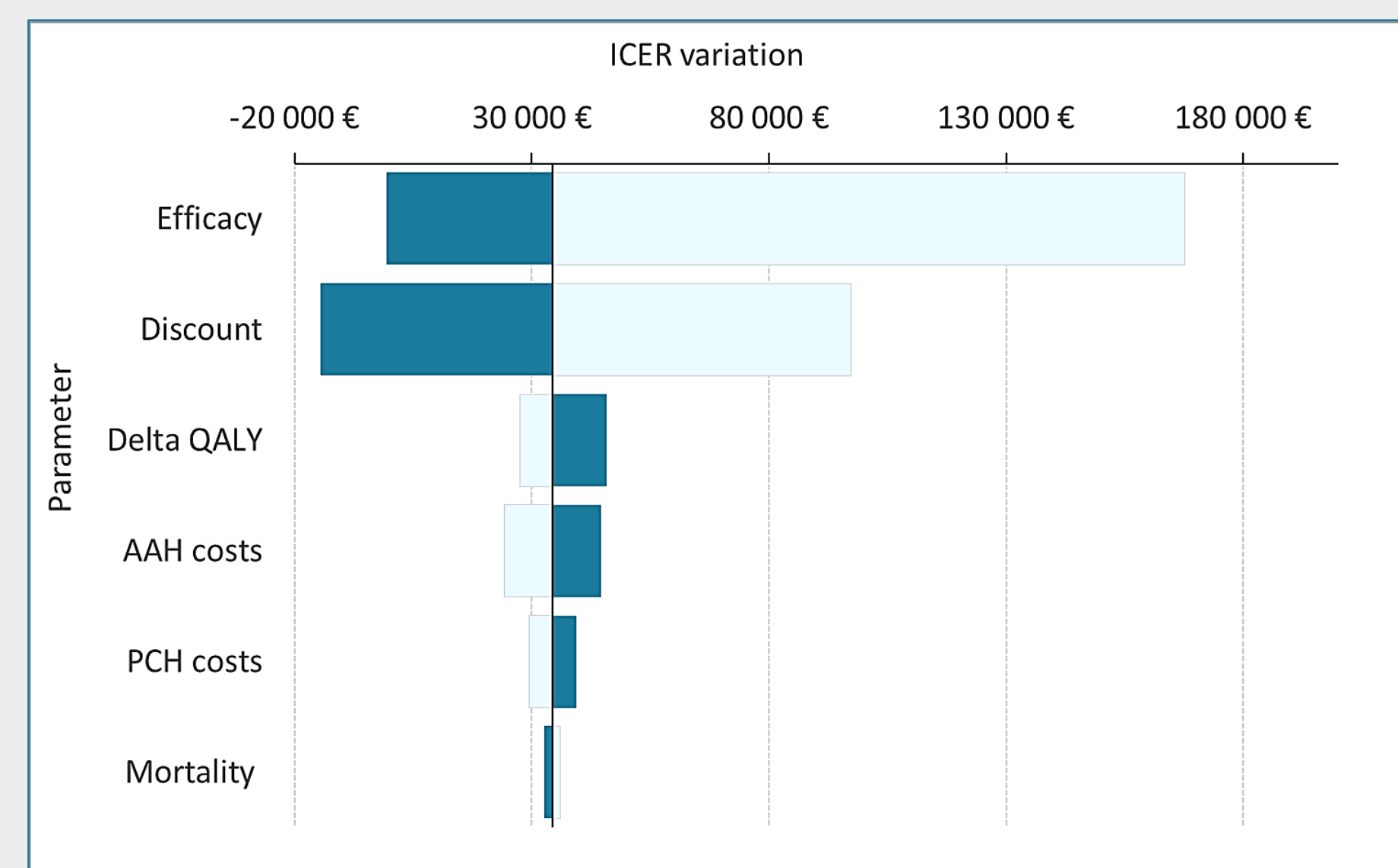
Incremental Costs Intervention	ICER (€/QALY)	ICER Variation vs. restricted health care system perspective €/QALY (%)
Health care system (restricted HAS perspective)		
250 000 €		95 260 €
Collective perspective including PCH cost (full HAS perspective)		
197 911 €	75 412 €	- 19 848 € (- 21%)
Societal perspective excluding productivity loss costs (not considered in HAS opinions)		
90 274 €	34 398 €	- 60 862 € (- 64%)
Full societal perspective (not considered in HAS opinions)		
- 356 607 €	Dominant (- 135 881 €)	- 231 141 € (- 307%)

When considering overall social security expenditures, health insurance spending and other social security service expenditures, the difference was even more significant with a 64% reduction.

Finally, considering the full societal perspective, including productivity losses was associated with the dominance of the disability-reducing intervention, suggesting the generation of savings over a lifetime time horizon.

DSA results showed that intervention efficacy was the main driver of the results, consistent with our modelling approach, where efficacy was the main determinant of DRSB recourse reduction (Figure 2).

Figure 2. DSA results.



Conclusion

Although HAS recommends adopting a collective perspective, the lack of appropriate data to accurately model DRSB in CEAs represents a challenge to adopting this perspective for disability-related treatments. This requires many assumptions to be made to transpose aggregated costs, unspecific of the modelled disease and to extrapolate disease and interventions' effects into changes in allowances eligibility and recourse.

Given the substantial impact of adding these costs to CEAs and the induced opportunities to better demonstrate and quantify the value of disability-reducing interventions in the French setting, manufacturers should acknowledge the importance of DRSB valuation and invest in time and resources in the assessment of their product impacts on DRSB recourse.

However, the lack of consideration of these costs by HAS and its high expectations in terms of methodological and data estimation robustness may set the bar too high considering the currently available data.

This highlights the need to establish a suitable framework to include DRSBs in the analysis by identifying suitable approaches and data to quantify them and innovative treatments' impact on DRSBs, as well as promoting the .

Additionally, our finding suggest that more emphasis should be put on societal perspective for treatments that reduce the burden of disability-related conditions, where the case could be made that treatment effect not only impacts the costs of producing cares relevant to the health insurance but also those related to other social security benefits, as well as the benefits that contribute to their financing that may results from improving patients' employment and labor productivity.

Main findings

Adequate assessment of interventions with high impact on DRSB following HAS' guidelines is difficult due to lacking data.

Although these methodological impediments may justify adopting a restricted healthcare system perspective, HAS has issued methodological reservations due to lacking DRSB inclusion in CEAs.

This impairs the assessed medico-economic value of disability-reducing interventions and may represent a barrier to access for these therapies in France and highlights the need to open access to qualitative DRSB-related data and establish a methodological framework to consider disability reduction in the collective perspective, allowing proper valuation of the benefits of these interventions.

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