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New model for prioritised adoption and use of hospital medicine in Denmark since 2017: Challenges and perspectives★

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ABSTRACT

Technological innovation creates new treatment opportunities, while also putting healthcare budgets under strain. To deal with the rising costs of hospital medicines, the regional governments in Denmark have developed a new model for prioritising the adoption and use of hospital medicine. Marking a shift from previous policies, the new model formalises the evaluation of clinical benefit, adds an assessment of treatment costs and ensures a relatively high degree of direct stakeholder involvement. In international comparison, the new model is ambitious in terms of stakeholder involvement and adherence with principles advocated to ensure procedural justice and fair decision-making processes. However, these procedural innovations have also created new challenges. Notably, the newly formed assessment body, the Danish Medicines Council, is faced with a very high caseload and limited options to prioritise the use of its analytical resources.

1. Introduction

How should the uptake of new, costly treatments be prioritised? This question engages decision-makers as technological innovation creates new treatment opportunities while also putting healthcare budgets under strain. Whereas informal 'bedside' rationing has traditionally been a common feature of healthcare delivery, explicit priority setting has gained more political salience. Given the plurality of value judgments implied in resource allocation decisions, much political and scholarly attention centres on the need for procedural justice and fair decision-making processes [1–3].

Aiming for greater allocative efficiency of healthcare resources, fast and uniform use of new effective medications, and a stronger basis for pharmaceutical procurement [4], the regional authorities in Denmark adopted a new model for the assessment of hospital medicines in 2016 (in force since 2017). In international comparison, the new model is ambitious in terms of stakeholder involvement [5,6] and it adopts many of the principles advocated in the literature on health technology assessment (HTA) and priority setting [7,8]. Still, new challenges have emerged. The Danish experiences so far call for healthcare decision makers to balance concerns for external legitimacy with concerns for internal operation ability. Notably, the procedural innovations have provided for a situation where the Danish assessment body is confronted with a very high caseload and limited options to prioritise the use of its analytical resources.

2. Political and economic background for the prioritisation of medicines in Denmark

2.1. From national to regional-level initiatives

In Denmark, HTA has been practiced since 1997 by shifting national level agencies. However, the development of national HTA-reports for medicines has gradually been phased out and replaced by initiatives taken by the five regional authorities. In the tax-funded Danish healthcare system, the regional authorities own the hospitals and are politically and administratively responsible for organising and paying for health service delivery, including medicines. In 2009, the regions established a council that was to help reduce clinical practice variation through the development of treatment guidelines (Rådet for Anvendelse af Dyr Sygehusmedicin, RADS). In 2012, the regions established another council, which assessed the therapeutic benefit of new treatment principles and coordinated their adoption among the regions (KoordineringsRådet for Ibrugtagning af Sygehusmedicin, KRIS). The new assessment model integrates and formalises these regional-level initiatives and replaces the two former councils by one: the Danish Medicines Council. Whereas the two former councils were not politically mandated to take treatment costs into account, this is an explicit aim of the new Medicines Council.

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2.2. Political contestation of health economic evaluation

In the political debate on priority setting in healthcare in Denmark, treatment costs have been a contested issue. The regional authorities, who bear the budget responsibility, have sought to raise political debate about pharmaceutical expenses and argued for the need of priority setting [9]. The regional healthcare budget is laid down in yearly negotiations with the Ministry of Finance and is allocated to the five Regions as a block grant. Democratically elected members of the Regions have the overall responsibility for keeping the budget and allocating resources. Over the last decade, regional expenses for hospital medicines have increased markedly. Since 2012, hospital medicines have constituted the largest share of the total pharmaceutical expenditures [10]. As the growth rate of hospital medicine expenses exceeds the growth rate of total hospital expenses, pharmaceutical expenditures drive out other resources in the hospital sector, such as staff hours [9].

Members of parliament, however, have been reluctant to accept treatment costs as a basis for priority setting. In the 2015 election campaign, the prime ministerial candidates promised that patients would have access to new medicines, no matter the costs – but without conferring with the Regions [9]. When the Regions launched their new model in 2016, it therefore represented a break with the dominant political discourse at that time. Unsurprisingly, the new model met with intense debate. The medical association, which had previously sought to raise debate about the need for explicit priority setting, supported the new assessment model. The pharmaceutical industry association, whose members account for a large share of Danish exports, kept a low profile. By contrast, the major patient organisations argued strongly against the assessment model contending that it is unethical to restrict access to new treatments based on financial concerns [11]. In the end, the Parliament sanctioned the inclusion of treatment costs by the adoption of seven principles for priority setting (see Box 1) [12]. However, the political approval came without political support for the application of cost-effectiveness or cost-utility analysis. Therefore, the Medicines Council is to balance treatment costs against clinical benefit – but without using formally recognised health economic evaluation models.

Box 1: The seven principles for prioritising the use of hospital medicines in Denmark

1. *Professional expertise*: Assessment of medicines is to include a thorough and systematic assessment of the treatment benefits and the documentation of these benefits. The assessment must be carried out using the necessary and appropriate expertise.
2. *Independence*: Assessment of medicines is to be based on objective criteria and professional assessments, to ensure that the basis for decision-making is made independently and without political inference.
3. *Geographical equality:* Uniform introduction and use of medicines across the country is to be ensured. Moreover, cases where medicines are rejected as standard treatment are to be handled similarly across the various regions and hospitals.
4. *Transparency:* Assessments are to be undertaken with as much transparency as possible concerning processes, methods, criteria and the material used in the assessment process. In line with the requirements of the Transparency Directive, anyone should be able to view the basis of and the reasons given for acceptance or rejection of new medicines. Transparency also serves to facilitate public debate.
5. *Speedy introduction of new, effective medicines:* Patients are to benefit from treatment progress. Denmark should remain among the countries that are quickest to introduce new medicines with documented added treatment value.
6. *More health for money:* Healthcare resources, including resources for hospital medicines, are to be used with care, to avoid negative consequences for prevention, treatment and care in other areas of the health service. New medicines with a well-documented added clinical value must not be rejected on the grounds of economy alone. For a new medicine to become standard treatment, the price must be reasonable in relation to the added clinical value it offers compared to the existing standard treatment.
7. *Access to treatment*: Equal access is to be ensured for large and small patient groups, and individual patient needs are to be taken into account. Based on a medical assessment of individual cases, it should be possible to prescribe medicines that are not recommended as standard treatment; for instance to be able to provide high quality treatment for patients with rare diseases or to avoid functional impairment.

3. Content of the policy

Compared to the theoretical HTA models developed at European level, the Danish assessment framework for hospital medicines is more narrow in scope as it does not include specific analyses of ethical, legal and organisational implications [8,13]. In the following, the content of the new model is developed. We focus on three key innovations, that is 1) measures taken to ensure more direct stakeholder involvement, 2) formalisation of the clinical benefit assessment and 3) explicit inclusion of cost concerns in treatment recommendations.

3.1. More direct stakeholder involvement

While there is a clear trend towards stakeholder involvement and public engagement in HTA processes internationally, it varies considerably which parties are involved in which ways [5,8], and there appears to be lacking international consensus about which rationales the involvement should be based upon [6,14]. In Denmark, the new assessment model provides for stronger stakeholder involvement in at least three ways. Firstly, a broad range of stakeholders participate directly in the assessment processes. The Medicines Council consists of three units: *The Board*, which has the overall responsibility for making recommendations about the adoption of new medicines or new indications and for issuing treatment guidelines for the use of medicines within therapeutic areas. *The Secretariat*, which manages the evaluation process and drives the development of the evaluation methods. A number of *expert committees*, which define the assessment protocol (e.g. specify the patient population, the comparator and the outcome measures) and critically appraise the analyses within the framework established by the methods guidelines of the Medicines Council [15]. The expert committees consist of medical specialists, one clinical pharmacologist, one pharmacist and at least one patient representative. The two chairs of the Board and ten Board members are appointed by the regional authorities; three members are appointed by the medical societies, and – as something new – two members are appointed by the umbrella organisation for Danish patient organisations [4]. In addition, the Council has decided to supplement its expertise with a health economist. The pharmaceutical industry association, the Danish Health Authority, and the Danish Medicines Agency each has one observer in the Board. Observers participate in discussions on par with Board members but without voting right.

Secondly, while the Medicines Council can open cases at its own initiative, a new application procedure gives pharmaceutical manufacturers strong influence on which products are to undergo assessment and when. According to this procedure, manufacturers can apply for an assessment of newly marketed pharmaceuticals or new indications but are not required to do so. This provides man-

ufacturers with strategic options with regard to the timing and content of applications; e.g. whether to apply based on interim data or wait for final outcome data. The Medicines Council cannot decline any application, and once an application is filed, the Board is to make its recommendation within 12 weeks unless the applicant delays the process [15]. Applicants are responsible for identifying the evidence and conducting the analyses specified in the assessment protocol, which are then critically assessed by the expert committees.

Thirdly, transparency requirements and formalised hearing processes allow stakeholders insights and rights to comment on assessments during the process. The Medicines Council is required to send the clinical benefit assessment to the applicant for a hearing and to make the assessment protocol, the assessment report and the final recommendation publicly available immediately after approval. In addition, the Board publishes agendas and minutes from its meetings.

In sum, the model combines indirect forms of stakeholder involvement (hearing opportunities) with direct involvement in assessment processes at Board and expert committee level, providing opportunities for the Medicines Council to benefit from specific stakeholder knowledge and legitimacy gained through mechanisms of representation and more explicit articulation of reasons for specific judgments [1,6,14]. In international comparison, there is a broad representation of stakeholders at the Board level including representatives for patient and industry organisations [5,6]. In addition, manufacturers are given the main control over which and when new products and indications are assessed.

3.2. Formalisation of the clinical benefit assessment

In line with international recommendations to ensure transparency about the application of decision criteria and the possibility for stakeholders to comment on assessment reports [2,8], the Danish assessment model implies a high degree of formalisation of the assessment procedures. The methods for clinical benefit assessment draw inspiration from the framework developed by the German Institute for Quality and Efficiency in Healthcare (IQWIG). Based on the analyses made by the manufacturers, the expert committees categorise the pharmaceuticals according to seven categories of added clinical value: 'High added clinical value', 'moderate added clinical value', 'low added clinical value', 'added clinical value of unknown size', 'no added clinical value', 'negative added clinical value' and 'the added clinical value cannot be categorized' [15]. 'Added value' is defined as 'the extra value a pharmaceutical offers compared to existing treatment in terms of prolonging life, adverse effects and quality of life' [16].

Aiming to ensure explicitness about assessment methods and criteria, methods guidelines specify which outcome measures can be used as a basis for the categorisation of added clinical value, including clinical events such as 'death, disease progression or stroke' and other outcomes such as 'symptoms, quality of life and functional ability', along with detailed requirements for the judgment of effect sizes [15]. While adverse effects and quality of life measures should always be included, it is up to the expert committees and, ultimately, the Board to decide which specific measures to include in a given assessment protocol [15]. Both relative and absolute effects are included in the Danish model. The relative effect measures provides for consistent comparison of categorizations across cases. However, relative effects of similar size may have different clinical and health economic importance (e.g. the relative improvement in life expectancy from 1 week to 3 weeks vs. 1 year to 3 years). Inclusion of absolute effects provides for explicit justification of the clinical importance as the expert committees and, ultimately, the Board must define the least clinically relevant effect size for each measure. However, this also poses challenges of how to weigh absolute effects against relative effects in case of discrepancies. The reporting of clinical outcomes in natural units – as opposed to calculating QALYs – provides the Board with wide discretion in synthesising these into a statement about the added clinical value. The different outcome measures used to evaluate the same product often provide for different categories of added clinical value, and currently there is little guidance on how different categories are to be synthesised into one overall category.

3.3. Explicit inclusion of cost assessment in treatment recommendations

In HTA processes, the assessment of treatment costs is often integrated with the clinical benefit assessment through cost-effectiveness analysis [14]. By contrast, the Danish model supplements the clinical benefit assessment with a separate analysis of expected treatment costs. Based on analyses submitted by the manufacturer, the joint regional procurement organisation, Amgros, prepares an assessment of the incremental treatment costs per patient and the budget implications. According to the methods guidelines of Amgros, the estimation of the incremental costs must follow a 'restricted societal perspective', meaning that all relevant treatment-related costs should be included, regardless of who carries the costs. In addition to expenses related directly to the hospital treatment, costs borne by healthcare providers outside the hospital (e.g. GP follow-up visits) should be included, as well as costs for patients and relatives (e.g. time and transportation) [18]. Productivity gains or losses are not included in the cost analysis, due to equality concerns [17]. The time-perspective of the cost analyses was initially restricted to the follow-up time in the studies that form the basis for the clinical benefit assessment. Acknowledging that this may provide for misleading cost estimates, particularly in the case of adjuvant treatment, some modelling is now accepted by the Board introducing new needs for judgment of the relevant follow-up time. The Board's classification of the added clinical value provides a basis for Amgros to estimate an 'acceptable' price range for the given treatment and enter into confidential price negotiations with the company [15]. However, the methods guidelines do not specify how the 'acceptable' price range is to be determined. Hence, it is unclear how effects and costs are combined to form a basis for judgment of whether a new treatment is worth the price.

Following the price negotiation, it is the responsibility of the Board to settle on a final recommendation as to whether a new product should be used as a 'standard treatment' in the public hospital services [15]. The notion of 'standard treatment' has given rise to challenges of interpretation. Currently, the concept is not defined in the methods guidelines of the Medicines Council leaving it open to interpretation. In practice, the recommendation of a new therapy as standard treatment implies that hospitals are guaranteed (at least partial) reimbursement for the expenses from the Regions. Therefore, the therapy will typically be available for use at the hospitals although it may not be considered 1st line treatment. The Board can choose to recommend restricted use of a product, thereby posing more strict criteria for access than those approved during marketing authorisation. In cases with scant evidence, the Board may recommend experimental use for a limited group of patients for a limited period to allow for further data generation [16]. Since the Medicines Council is mandated only to 'recommend', not decide, the statements made by the Board are formally non-binding for the regions [4]. Following public controversy over certain products that were *not* recommended as standard treatment, the implications of the Medicines Council's recommendations for clinical discretion and patients' access to new treatments have become subject to high-level political debate. In accordance with international recommendations [8], reassessments are possible, if new evidence becomes available, the

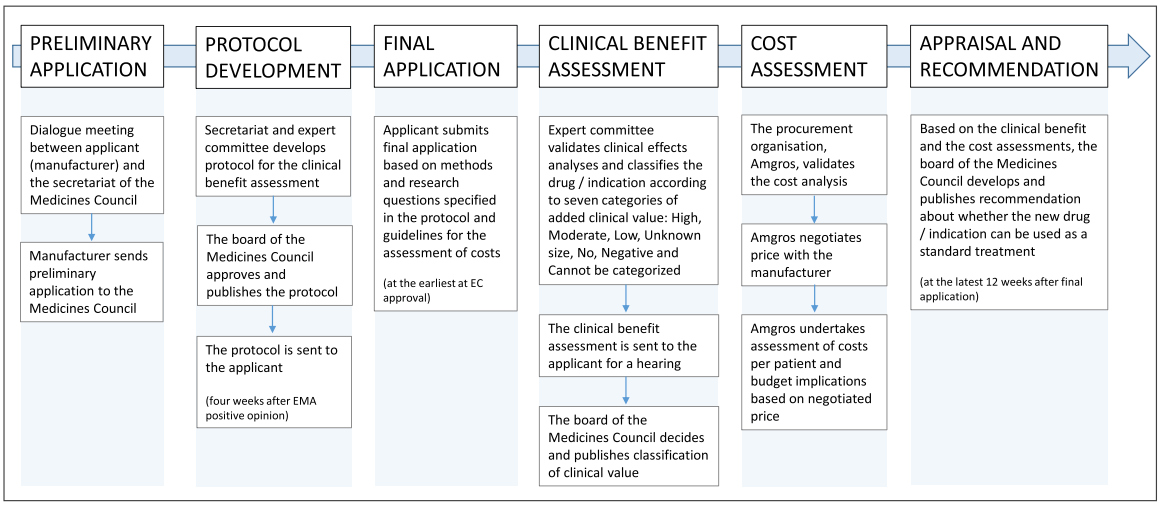

Fig. 1. Assessment process for new pharmaceuticals and indications in the Danish Medicines Council.

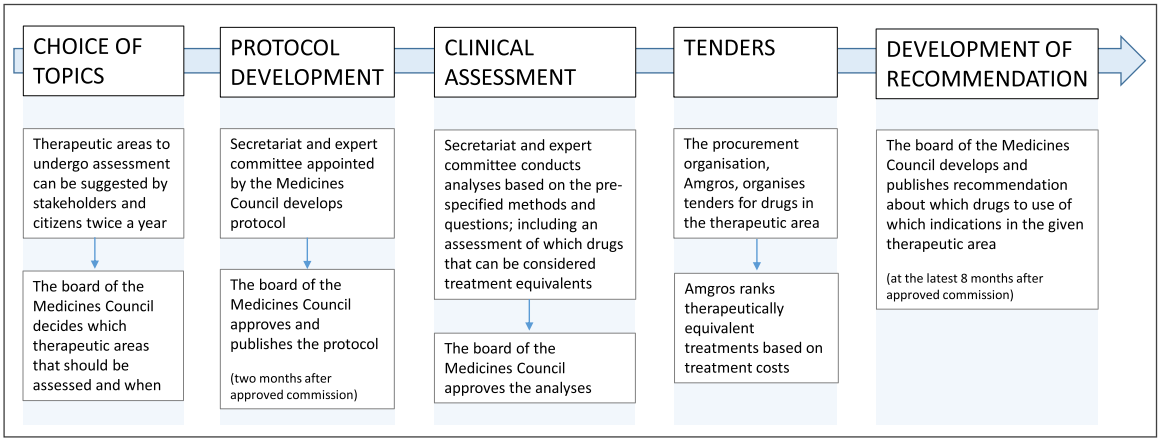

Fig. 2. Development of treatment guidelines by the Danish Medicines Council.

applying company offers to revise its price or the Board finds other relevant reasons for a re-assessment. It is not possible for stakeholders to appeal a classification or recommendation made by the Danish Medicines Council. Any cases of complaints are to be handled through the legal system.

The assessment process for new pharmaceuticals or indications is illustrated in Fig. 1.

4. Challenges and perspectives

The new assessment model is an ambitious attempt to provide an explicit and transparent basis for the prioritisation of hospital medicines, with a high degree of stakeholder involvement and a relatively short processing time. During its first two years of operation, the Medicines Council has done much to optimise its work procedures and communicate its decision rationales. Still, challenges remain.

One of the key challenges manifests itself as a vast caseload. On the input side, the Medicines Council has received many more applications than expected by the Regions [19]. During its first year of operation, the Council received 76 inquiries from manufacturers about assessments of newly marketed products and indications, which resulted in 33 preliminary and 15 final applications in 2017 [20]. As of 25th October 2018, 43 new products or indications were under assessment by the Council. In addition, nine treatment guidelines were under development [21]. This creates a considerable workload for all the involved. The Board had 13 meetings in 2018. With the agendas containing more than 20 discussion or decision items, meetings typically lasted a whole working day (up to 10 hours) [22].

Whereas the earlier councils would focus on cases they found to be of clinical and financial significance, the assessment of relevance now rests with the applicants. The application procedure ensures equal opportunities for manufacturers to have their products assessed. However, this opportunity comes at the expense of the ability of the Medicines Council to prioritise the use of its analytical resources. So far, manufacturers appear willing to apply for most new products and indications, and the Medicines Council is not mandated to reject or delay any applications. Consequently, the Medicines Council must also spend resources on assessments, which may have limited practical impact (e.g. because the budget implications are marginal or clinicians find the treatment option to be of limited clinical relevance). Furthermore, the assessments of single products tend to crowd out resources for the development of treatment guidelines [23], which are allowed a much longer processing time (6-8 months). The development of treatment guidelines serves a double goal of limiting clinical practice variation and stimulating price competition through coordination with national tenders. Consequently, the focus on single products seems to limit the ability of the Medicines Council to critically assess the treatment options within a whole therapeutic area and thereby provide a better informed ground for pharmaceutical pro-

curement. The development process for treatment guidelines is outlined in Fig. 2.

On the output side, cases can be difficult to close. There can be several reasons for this. Manufacturers sometimes apply before final outcome data are available or fail to provide the data requested by the Medicines Council. This creates a scant and uncertain evidence base, and the assessment procedure drags out when the Medicines Council has to spend time on investigating, for instance, when outcome data can be expected and whether other data than those provided by the company might exist. Adding to this challenge, the Board members have little to lean on when judging whether treatment costs appear 'fair' compared to the clinical benefit. Possibilities for benchmarks across cases are challenged by the methodological choice of not using a standardised, composite outcome measure. Furthermore, the vested interests of the stakeholders participating in the assessment and appraisal process make it challenging and time-consuming to reach consensus – particularly when decision criteria are open to interpretation. The statutes of the Medicines Council states that the Council is expected to reach consensus, though disagreements may be settled through voting [4]. Finally, the high caseload and the complexity of the cases makes it difficult for council members to keep an overview. At its height, the Council received 1321 pages of supplementary material before one meeting [23], making it virtually impossible for Council members, who typically also hold managerial positions in the healthcare services, to remember the details of each case, much less keep an overview of whether criteria are applied consistently from case to case.

5. Conclusion

The new assessment model for hospital medicines is an important step towards more explicit priority setting in the Danish healthcare services. In line with many of the principles advocated to ensure procedural justice and fair decision-making processes, the model formalises the evaluation of clinical benefit, adds an assessment of treatment costs and ensures a high degree of direct stakeholder involvement. The Danish experiences so far suggest that ambitions of ensuring external legitimacy need to be balanced with concerns for internal operation ability. First, an application-based process combined with strict time-limits needs to be balanced by internal control mechanisms that allow the assessment body to focus the use of its analytical resources; e.g. through the definition of minimum criteria for new products and indications to undergo full assessment. Such criteria could concern clinical impact, economic implications and the quality of evidence. Second, appraisal processes that hinge on consensus among multiple stakeholders call for clear decision criteria and possibilities for comparison across cases to ensure consistency in the application of criteria. Third, price negotiations based on health economic analysis are difficult if there is little consensus on what constitutes the relevant outcome measure, and how it should be quantified.

Conflicts of interest statement

As part of their employment at the Danish Center for Social Science Research, both authors undertake commissioned research for state institutions, regional governments and private institutions, including pharmaceutical companies and patient organisations.

CRediT authorship contribution statement

Sarah Wadmann: Investigation, Writing - original draft. **Jakob Kjellberg:** Writing - review & editing.

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