21 February 2023

Piloting the application procedure in the assessment of new hospital-only medicines

Introduction

One of the goals of the reform of pharmaceutical matters launched by the Ministry of Social Affairs and Health is to standardise the principles for evaluating the introduction of medicines used in outpatient care and in hospitals.[1] Currently, these systems have differences, which may result in the way the medicine is administered and, consequently, the division of medicines into outpatient and hospital-only medicines having an inappropriate effect on the decision to uptake a medicine. In addition, procedures related to the uptake of hospital-only medicines have been considered difficult to predict in some respects.

The goal of the pilots now being launched is to find a new way of organising Fimea's assessment activities for hospital-only medicines and, in particular, procedures related to the launching of assessments. The goal is not to change the content of the assessment or to influence the subsequent recommendation and procurement procedure.

At present, the way that assessments are launched (application procedure for outpatient medicines and a model focused on initiatives by officials for hospital-only medicines) is a principled key difference in the procedures for introducing outpatient and hospital-only medicines. The pilots will introduce a partial application procedure for launching Fimea's assessments of hospital-only medicines. At a later stage, a potential transition to the application model could make it possible to standardise the procedures for assessing outpatient and hospital-only medicines, for example, with regard to application requirements or fees. This would also increase the coverage, predictability and timeliness of assessments of hospital-only medicines.

Pilots and schedule

The piloting of the application procedure will be launched in 2023. Products entering the market for the first time that are intended for hospital use and for which there are no plans to seek reimbursement status in outpatient care may be included in the piloting of the application procedure. However, a hospital-only medicine that has not been previously assessed and is not in use may also be considered for inclusion in the piloting of the application procedure. Extensions of the therapeutic indications of products in use are not covered by the procedure to be piloted at this stage. Regarding them, the operating model corresponds to the procedure of the current process, and an opportunity for an application-based procedure is not applied or offered at this stage. Vaccines are also not included in the piloting. If necessary, Fimea may also carry out assessments of hospital-only medicines according to the current assessment model for hospital-only medicines during the piloting stage.

[1] Ministry of Social Affairs and Health: Points of views on Need for Changes in Medication and Distribution system of Medicines. Reports and Memorandums of the Ministry of Social Affairs and Health 2019:5.

Operating model in application procedure pilots

The operating method in the application procedure pilots mainly corresponds to the processes and practices of assessing hospital-only medicines. However, the assessments are launched in another way (see Figure 1).

In Fimea's regular hospital-only medicine assessments, Fimea selects the assessment topics monthly from among the CHMP's positive opinions and sends an information request to a company, which launches the assessment. In the application procedure pilots, the initiative comes from the company. The company informs Fimea of the new hospital-only medicine and the anticipated date for submitting the application and proposes the scope of the assessment (the PICO process). Fimea comments on the scope of the assessment and the suitability of the topic for the application procedure pilots. The assessment is primarily carried out according to the therapeutic indication specified in the marketing authorisation. If desired, the company may propose a scope that has a narrower target group than the therapeutic indication. After defining the scope of the assessment, Fimea will wait for the application submitted by the company. During the waiting period, a non-disclosure agreement related to the disclosure and use of data will be signed. After the application has arrived, the actual assessment and the subsequent stages are carried out as usually in the assessment of hospital-only medicines. The assessment report corresponds to the current report for hospital-only medicine assessments in terms of structure and content. The purpose of the piloting of the application procedure is not to change the stages related to the preparation and procurement procedure of the COHERE Finland recommendation.

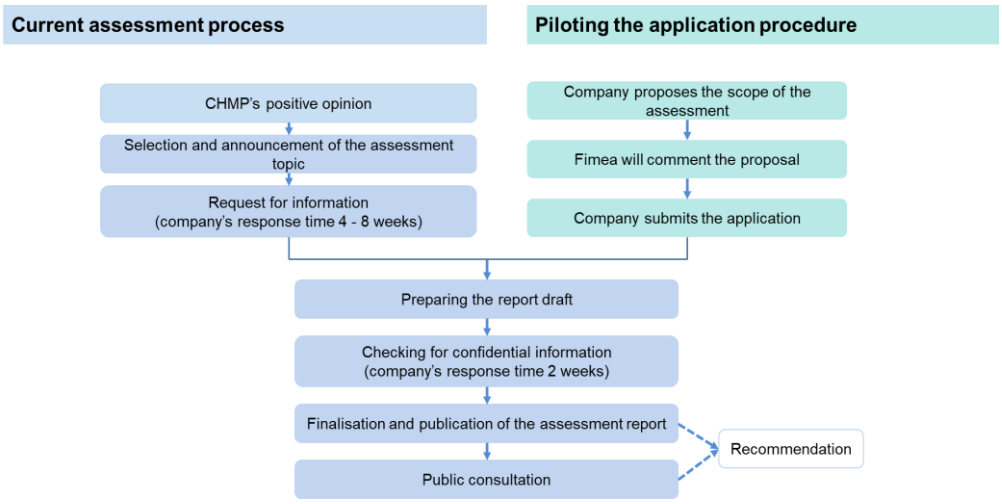

Figure 1. Current process of hospital-only medicine assessment and the piloting of the application procedure

Launching assessments in the pilot stage

The company will contact Fimea and propose the scope of the assessment (PICO). The companies are asked to contact Fimea at hta@fimea.fi. At the same time, the company will give its estimation of the date on which the application will be submitted. At the time of contact, the company must

have a good understanding of the expected date and therapeutic indication of the CHMP's opinion.

Fimea will comment on the scope of the assessment and approve or reject the topic as an application procedure pilot.

The company submits the application, and the assessment is launched. The application may be submitted at the time the CHMP issues its positive opinion at the earliest. Before submitting the application to Fimea, the company and Fimea will draw up a standard agreement related to the disclosure and use of data[2].

If the submission of the application has not been agreed upon before the CHMP's positive opinion (see stages 1 and 2 above), Fimea will contact the marketing authorisation holder during its normal procedures related to the selection of topics. In connection with this, the topic's suitability and prerequisites for an application-based procedure will be examined, and an enquiry concerning the possible date of the assessment will be made. After the examination, Fimea will decide whether to launch the assessment on the initiative of officials in accordance with the current operating model or whether to wait for the application material.

Fimea publishes a monthly list of topic selections based on the CHMP's positive opinions. The topics included in the application procedure are included in the same list, but they are not specified separately.

Application guidelines

Where applicable, the Pharmaceuticals Pricing Board's application instructions for the reimbursement status of a new medicinal product[3] and the instructions for preparing a health economic evaluation[4]are used in the application material guidelines. Separate guidelines on the application of the application instructions have been prepared for applications concerning the assessment of hospital-only medicines sent to Fimea (attached). The application guidelines will be updated based on the experiences gained in the pilots.

Pilot assessment and process development

The pilots are open for applications from 2023 onwards, and experiences in the first stage will be gathered for approximately a year. During the piloting, the experiences gained from the procedure with regard to Fimea and key stakeholders will be assessed, and the procedure descriptions and guidelines as well as other operating methods will be specified. Based on the experiences gained in the pilots, a possible transition to an application-based procedure instead of a procedure based on initiatives by officials will be considered. In the future, procedures related to updating assessments and collecting additional evidence after introduction will also be examined.

[2] Process description of the assessment of hospital-only medicinal products, Annex 2
[3] https://www.hila.fi/content/uploads/2021/05/Uusi_valmiste_ohje_250521.pdf (in Finnish)
[4] https://www.hila.fi/content/uploads/2020/01/Instructions_TTS_2019.pdf

With the EU's HTA Regulation, the clinical part of the assessments will in the future be produced as an EU joint assessment, and the EU Member States will comment on the scope of the assessment at the initiation stage of the joint assessment. This stage could replace the stage of the process in which Fimea takes a position on the scope of the assessment if Fimea's hospital-only medicine assessment would shift more permanently to the application procedure. EU joint assessments are also taken into account in the application guidelines. The EU's permanent HTA mechanism will be introduced in stages and is only applied on a full scale in 2030. Regardless of whether the official-initiated or application-based procedure is used, some of the assessments do not currently provide a clinical assessment produced by the EU mechanism.

Annex 1. Guidelines on the application of the Pharmaceuticals Pricing Board's application instructions

General

This guideline is applied when the marketing authorisation holder applies for an HTA assessment of a new hospital-only medicine from Fimea as part of the piloting of the application procedure. The contents of the application material follow the Pharmaceuticals Pricing Board's application instructions for the reimbursement status of a new medicinal product[5] and the instructions for preparing a health economic evaluation[6], the application of which in Fimea's hospital-only medicine assessments is described in these guidelines. Guidelines concerning the use of the Pharmaceuticals Pricing Board's e-services do not apply to material submitted to Fimea. The companies are asked to contact Fimea at hta@fimea.fi. The actual application material is submitted to the designated contact persons through Fimea's Secure Mail service[7].

Application of the application instructions for the reimbursement status of a new medicinal product

At least the following attachments listed in the application instructions are sent to Fimea:

- a valid marketing authorisation decision or the CHMP's positive opinion concerning the granting of a marketing authorisation
- a valid SPC (a summary of product characteristics) or a draft SPC
- a clinical assessment report of the marketing authorisation official or its draft
- a report on the therapeutic value, including a list of references
- a report on costs and economic efficiency
- a health economic evaluation, including a list of references
- references

In the report on the therapeutic value, the guidelines for the application of a special reimbursement status are followed. The market forecast and the information on sales and patient numbers can be reported in a manner that deviates from the Pharmaceuticals Pricing Board's application instructions, but in a manner that the information presented contains sufficient grounds for the assessment of the budget impact.
To assess costs and economic efficiency, the company has the opportunity to present a proposal for a confidential price or pricing model. However, the reports must also always present the information on the basis of the public wholesale price of the product.

In addition, the company is requested to provide references (links) to ongoing HTA assessments in other EEA countries and Canada as well as information on the results of completed recommendations or the stage of their processing.

Application of the instructions for preparing a health economic evaluation

Comparators according to the PICO procedure should be used as treatment options.

All information in the health economic evaluation must be primarily based on the information presented in the therapeutic value report. The results can also be presented using a confidential contract price or pricing model.

[5] https://www.hila.fi/content/uploads/2021/05/Uusi_valmiste_ohje_250521.pdf (in Finnish)
[6] https://www.hila.fi/content/uploads/2020/01/Instructions_TTS_2019.pdf
[7] https://www.fimea.fi/web/en/about_us/contact_information/secure-mail