RAPID ASSESSMENT OF NEW HOSPITAL-ONLY MEDICINAL PRODUCTS

1 Objectives of the assessment process

The rapid assessment of hospital-only medicinal products aims to:

- add to the information required by decision-makers in adopting a medicinal product
- reduce overlapping work conducted by hospitals in connection with the adoption of a new medicinal product
- promote regional equality regarding the availability of treatments

The process is carried out in order to provide information on the therapeutic and economic value of a new medicinal product in a situation where a hospital is considering the adoption of the medicinal product. The assessment is based in part on criteria different from those used when granting marketing authorisation. In addition to clinical effects, the assessment also takes account of costs, with the intervention subject to assessment being compared as far as possible to its comparators currently in use.

The content and procedures of the approach proposed do not meet the requirements set for a full-scale Health Technology Assessment. The key objective is an appropriate assessment process that takes into consideration the needs of the various stakeholders.

2 Medicinal products assessed

The process aims to assess new hospital-only medicinal products. While no unambiguous definition exists for hospital-only medicinal products, they can be characterised as follows:

- the medicinal product is primarily intended for use in public health care hospitals
- the principal purchaser of the medicinal product in Finland is a hospital
- the administration of the medicinal product normally requires a hospital-like setting

In this context, a new medicinal product refers to a medicinal product that has recently received marketing authorisation or to a medicinal product for which a significant extension of the therapeutic indication has been granted.

The selection of the assessment topic is described in more detail in section 3.2.

3 Assessment

3.1 Parties participating in the assessment

Company (marketing authorisation holder or applicant)

- May submit material for the assessment that can be used in the compilation of the assessment report
- Checks that the assessment report does not contain any confidential information provided by the Company

University hospital districts

- Chief physicians (assessment of technologies) act as contact persons in the specific catchment areas. Furthermore, they bring forth the perspectives of the university hospital districts in the assessment process

Fimea

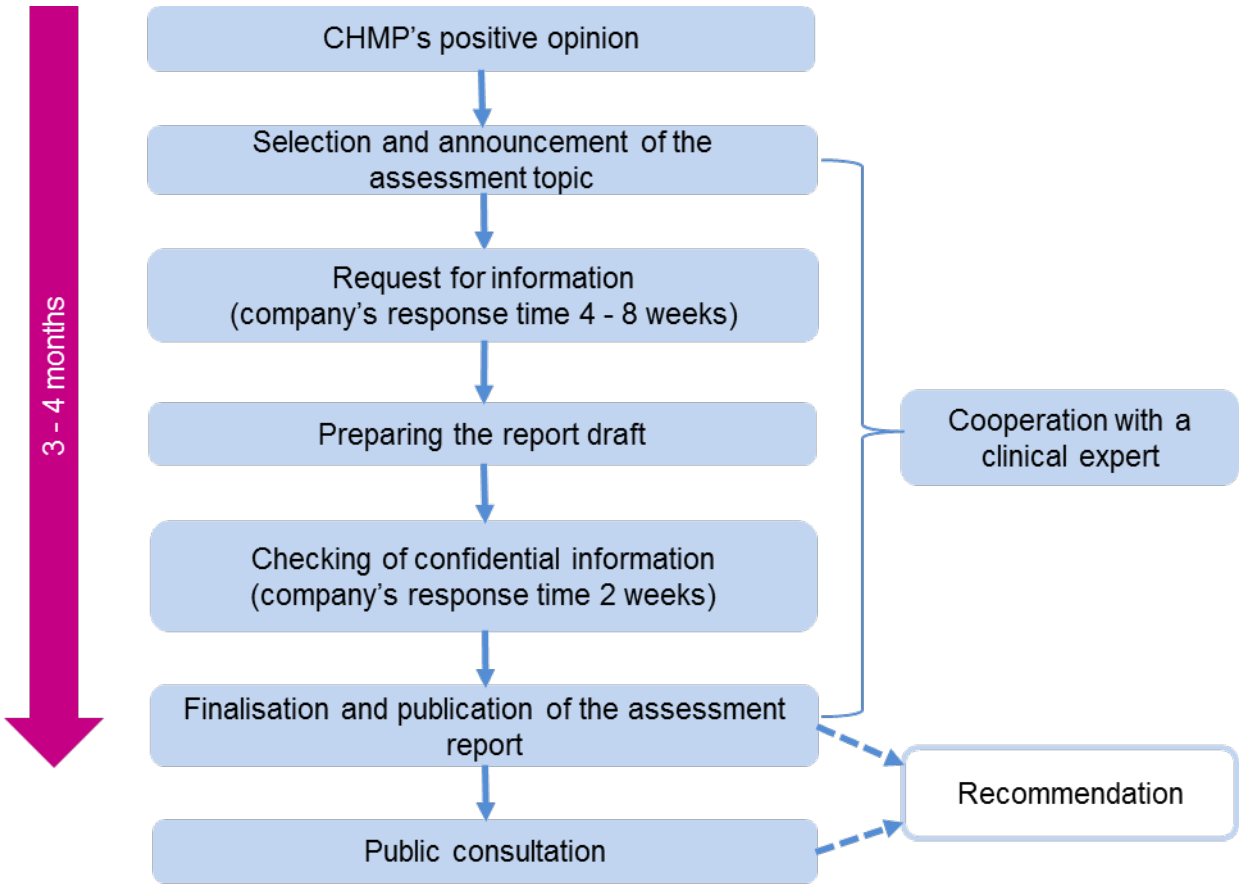
- Coordinates assessment activities
- Produces and publishes the assessment reports

Clinical experts

- Assist in the specification of the objectives of the assessment (PICO)
- Comment on the material produced by the assessment team and respond to the questions of the assessment team, in particular with regard to the current treatment practices and applicability of evidence

3.2 Assessment process

A flowchart of the progress of the assessment process is presented in the figure below (CHMP = Committee for Medicinal Products for Human Use).



Selection and announcement of the assessment topic

The assessment is initiated in such a manner that its outcomes are available as soon as possible after the marketing authorisation has been granted. Fimea follows the positive opinions issued by the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency on marketing authorisation processes concerning new medicines and the extension of therapeutic indications on a monthly basis and selects topics that are suitable for the assessment of hospital-only medicinal products. Apart from the fact that the medicinal product concerned is used in hospitals (see the criteria in section 2), factors such as the following are also considered in the prioritisation of the topics:

- Prevalence, severity and disease burden of the health problem
- Economic impact of the intervention
- Anticipated effects of the intervention on the treatment practices of the health problem concerned
- Possibility to produce assessment data on a timely basis

Other parties, such as a hospital or marketing authorisation holder/applicant, may also propose a topic for assessment. A preliminary topic proposal can be represented to Fimea prior to CHMP opinion, if possible. The decision of the initiation of assessment is taken by Fimea, with due consideration given to the resources available for assessment. Fimea schedules the progress of the assessment and informs the relevant stakeholders. The commencement of the assessment is also announced on the Fimea website.

When the assessment starts, a kick-off meeting can be agreed upon with the Company to discuss the scheduling and content of the assessment, and the request for information addressed to the Company.

Requests for information and material used in the assessment

The assessment is principally based on published research reports, on a public assessment report produced by the European Medicines Agency, and on material available from other sources. Additionally, material provided by the Company may also be utilised in the assessment. At the beginning of the assessment, Fimea sends the marketing authorisation holder a request for information where additional information about clinical studies, for example, including clinical study reports (CSR), may be requested. In the request for information, additional information about subgroup analyses, the duration of the treatment, information related to the quality of life, the costs of the treatment, the estimated number of patients in Finland and the budgetary impact of the treatment are frequently requested. Additionally, the Company may be requested to define the needs related to the collection of real world data.

The request for information will be prepared on a case-by-case basis, and the Company may also choose to submit to Fimea other material related to the assessment. Such material may include cost-effectiveness analysis and the model including related documentation (CEA material). Contacting Fimea ahead of time is recommended in order to discuss methodological details for submission, in case the Company intends to submit CEA material. In case CEA material is submitted, it will be included and assessed as part of the assessment. Otherwise cost-effectiveness analysis is not included into the assessment.

The Company will be allowed four weeks for submitting its responses. In case CEA material is submitted, eight weeks will be allowed for submitting. However, the Company is not obligated to submit such material. The assessment may also be carried out without any material submitted by the Company.

The material submitted by the Company may include information that must be kept confidential because it includes trade secrets or other sensitive information. In this case, a separate agreement will be signed with the Company on the submission of the material and the related procedures (Annex 2).

Preparing the report draft

Fimea draws up a draft for the assessment report, using all available material.

Checking of confidential information

Fimea will provide the Company with a draft of the assessment report insofar as unpublished material submitted by the Company was utilised in its preparation. The Company will be allowed two weeks to mark on the draft any previously unpublished information the Company considers confidential or declare that the draft does not contain any confidential information. In the published version of the assessment report, all information the Company has marked as confidential will be concealed. The procedure is described in greater detail in Annex 2.

Finalisation and publication of the assessment report

As a final step, Fimea finalises the assessment report, publishes it on its website and notifies the parties concerned of the assessment. A template for the structure of an assessment report and the matters to be addressed in it is provided in Annex 1.

Public consultation

The assessment report is publically available and open to comments. Comments may be submitted to Fimea's registry office. The comments and the details of the party submitting them are public and can be published.

Assessment reports will not normally be updated. Re-assessment can be conducted when, for example, there is a substantial change to therapeutic indication of the medicinal product concerned.

Preparing a recommendation

The Council for Choices in Health Care in Finland (COHERE Finland) is setting up a process that would make it possible to issue recommendations on the new hospital-only medicinal products (or extensions of therapeutic indications) assessed by Fimea. The first recommendation processes were initiated in the spring of 2017. The Council for Choices in Health Care in Finland is responsible for preparing the recommendations and for the recommendation process in general (http://palveluvalikoima.fi/en/frontpage).

4 Development of the assessment process

While the methods and content of the assessment process are largely established, they can be developed further in order to bring them into compliance with the requirements set by the various parties, especially decision-makers. The development work continues with regard to areas such as the following:

- *Managed entry agreements*. The opportunities for including managed entry agreements (MEA) as part of the range of instruments available in the assessment and decision making activities related to hospital-only medicinal products will be investigated.
- *Collection of real world data*. The opportunities for supplementing the information produced by assessment activities by systematically collecting real world data from registers and electronic health records (or for facilitating the development of such infrastructure) will be investigated.
- *Topic selection and early dialogue.* A company may confidentially inform Fimea about such future medicinal products and extensions of therapeutic indications that may be suitable for the assessment process of hospital-only medicinal products. Additionally, Fimea will investigate the opportunities for adopting a systematic procedure for improving the predictability of assessment topics.
- *Nordic collaboration.* A memorandum of understanding has been signed by the Director Generals of Fimea, NoMA and TLV, to formally start collaboration between the three authorities. The collaboration will consist of projects where the scope will be jointly produced health technology assessment reports on both relative effectiveness and applicable parts of a health economic analysis. Companies with a new, soon to be authorized, pharmaceutical product are invited to contact us, to discuss their possible participation in the collaboration.

ANNEX 1. Content of assessment and the structure of the assessment report

An assessment report is divided into the sections described below.

1 SCOPE OF THE ASSESSMENT

P	Population, patients
I	Intervention
C	Comparison, comparators
O	Outcomes
(T)	Time
(S)	Setting

2 DESCRIPTION OF THE INTERVENTION TO BE ASSESSED AND ITS COMPARATORS

This section answers the following questions:

2.1 What is the medicinal product to be assessed and for what purposes is it used?

2.2 How is the medicinal product to be assessed used?

2.3 What are the currently available treatment options?

3 CLINICAL EFFECTIVENESS AND SAFETY

This section answers the following questions:

3.1 What are the published clinical studies of the medicinal product being assessed?

3.2 What are the ongoing and unpublished clinical studies of the medicinal product being assessed?

3.3 What is the effect of the medicinal product being assessed on overall survival compared to its comparators?

3.4 What is the effect of the medicinal product being assessed on clinical endpoints compared to its comparators?

3.5 What is the effect of the medicinal product being assessed on the patient-reported outcomes compared to its comparators? (for example, the quality of life)

3.6 What is the effect of the medicinal product being assessed on surrogate outcomes compared to its comparators?

3.7 Is the effect of the intervention consistent between different patient groups (subgroups)?

3.8 How safe is the treatment compared to treatment options?

3.9 What type of uncertainty is potentially associated with the clinical effectiveness and safety?

4 COSTS

This section answers the following questions:

4.1 What is the price of the medicinal product being assessed and its comparators?

4.2 What are the total costs per patient of the intervention in relation to its comparators?

4.3 What is the budget impact of the intervention?

4.4 What type of uncertainty is associated with cost and budget impact estimates?

5 COST-EFFECTIVENESS (optional)

The section asnwers the following questions provided that the Company has submitted CEA material (model and relevant documentation) to Fimea:

5.1 What are the expected benefits and costs associated with the medicinal product being assessed and its comparators?

5.2 What is the incremental cost-effectiveness ratio (ICER) of the medicinal product being assessed in relation to its comparators?

5.3 What type of uncertainty (methodological, structural and parameter) is associated with the ICER estimate?

5.4 Is the cost-effectiveness of the intervention different between patient groups (subgroups)?

6 OTHER FACTORS (when necessary)

This section answers the following questions where necessary:

6.1 Are there any ethical, organisational, social or legal aspects specific to the intervention that should be taken into consideration in assessment?

6.2 Is the intervention associated with any specific patient perspectives that should be taken into consideration in assessment?

ANNEX 2. Agreement on the submission and use of information for the assessment of pharmacotherapies

Note! *This Agreement will be signed in Finnish. An unsigned, unofficial English translation of the Agreement is presented here. In the event of any conflict between the Finnish and English text, the Finnish version shall take precedence.*

1. Parties

xxxxxx, (hereinafter the Company)
(business ID xxx)
Address

Finnish Medicines Agency (hereinafter Fimea)
(business ID 0921536-6)
P.O. Box 55, FI-00034 FIMEA

The Finnish Medicines Agency and the Company are hereinafter collectively referred to as the Parties.

2. Contact persons

Fimea's contact person(s):

First name Last name
Position
Phone
E-mail

Company's contact person(s)

First name Last name
Position
Phone
E-mail

In matters under to this Agreement, Fimea will only deal with the Company and its contact persons.

It is the duty of the contact persons to keep track of and monitor compliance with the Agreement, to furnish information on the Agreement within their own organisations and to the other Party, as well as submitting and receiving notifications and requests for information under the Agreement. The contact person of the other Party shall be informed of any changes of contact persons or any change to a contact person's contact information without delay. The contact person shall not have the right to amend the content of this Agreement.

3. Definitions

For the purposes of this Agreement:

assessment refers to the assessment of the therapeutic and economic value of a medicine subject to assessment by Fimea;

an assessment report refers to the Fimea publication relating to the assessment;

information refers to any material provided by the Company to Fimea that can be used for the assessment or the preparation of the assessment report.

clinical expert refers to a specifically designated expert participating in the assessment who is liable to keep confidential and not to make use of any information he or she has received about the Company's trade or professional secrets or other confidential matters. The non-disclosure obligation shall survive the termination of the duty concerned.

4. Background and purpose of the Agreement

Under Section 2(1)(8) of the Act on the Finnish Medicines Agency (593/2009), the duties of Fimea include producing and compiling assessments on the therapeutic and economic value of medicines, as well as coordinating the cooperation involved. In executing its duty, Fimea performs therapeutic and economic assessments of medicines in cooperation with operators in this field. The assessments made by Fimea will be published.

This Agreement includes provisions regarding the submission of information for assessment, and regarding practical procedures and confidentiality in a situation where the Company has decided to submit information to Fimea.

The assessment may involve other companies. Fimea may conclude separate agreements with such companies regarding the submission and use of information for the assessment of pharmacotherapies..

5. Medicine subject to assessment

The present assessment concerns the therapeutic and economic effects of medicine x in the treatment of disease z. The Company is the marketing authorisation holder for medicine x.

6. Information to be submitted to Fimea

Fimea shall gain a right of use to the information. The information may include an IT application and written reports to be used in an economic evaluation. The right of use to the information includes the right to use, copy, edit and publish it.

The information shall only be used, to the extent deemed necessary by Fimea, in an assessment covered by the present Agreement. Fimea may decide to make no use of the information in the assessment.

The Company shall be responsible for ensuring that the information submitted to Fimea does not infringe the existing patent, copyright or other immaterial rights of any third party.

The Company shall, at its own cost, indemnify Fimea against any claims that may be made against Fimea regarding immaterial rights. The Company shall further indemnify Fimea against any legal costs, compensation or any other costs or liabilities towards third parties arising from claims or liabilities pertaining to immaterial rights.

7. Publicity and confidentiality

The information may include data that must be kept confidential because it includes professional and trade secrets.

Fimea shall provide the Company with a draft of the assessment report insofar as unpublished information submitted by the Company was utilised in its preparation. The Company shall be allowed two weeks to mark on the draft any information the Company considers confidential under the Act on the Openness of Government Activities or as a professional or trade secrets, or declare that the draft does not contain any confidential information. In the published version of the assessment report, all information the Company has marked as confidential will be concealed.

Notwithstanding the aforedescribed non-disclosure provisions, Fimea may, prior to the marking of confidential information, provide separately designated clinical experts participating in the assessment with access to a draft of the assessment report and data contained in the information submitted by the Company to the extent it deems necessary.

If the assessment report does not contain any unpublished information submitted by the Company, Fimea will not submit a draft to the Company for the identification of confidential information.

The Parties undertake to keep confidential any information and documents received from the other Party that may be considered confidential and are to be kept confidential pursuant to the legislation on the openness of government activities, and further undertake not to use said information and documents for any purpose other than that specified in the Agreement. Furthermore, the Parties specifically agree that the Agreement and its annexes shall be made public.

8. Costs

The Parties shall each be liable for any costs incurred by them in relation to the Agreement.

9. Validity and termination of the Agreement

This Agreement shall terminate upon the completion of the assessment and the publication of the assessment report.

The termination of the Agreement upon the publication of the assessment report shall have no effect on the provisions of the Agreement concerning the information to be submitted to Fimea, publicity and confidentiality, costs, indemnity from compensation, the transfer of the Agreement, language, governing law and settlement of disputes.

10. Transfer and amendment of the Agreement

The Parties are entitled to transfer the Agreement to a third party with whom the duties hereunder shall be vested either in full or in part. Each Party shall notify the other Party of any transfer of the Agreement.

Any amendments shall be made in writing. Any electronic amendments to the Agreement shall be deemed written amendments.

11. Language

This Agreement has been drawn up and signed in Finnish. An unsigned, unofficial English translation of the Agreement may be prepared if required. In the event of any conflict between the Finnish and English text, the Finnish version shall take precedence.

12. Governing law and settlement of disputes

This Agreement shall be governed by the laws of Finland. Any disputes arising out of this Agreement shall primarily be settled through negotiations. Failing that, the dispute shall be referred to the Helsinki District Court as the court of first instance.

13. Entry into force and signatures

This Agreement shall enter into force when signed by competent representatives of both Parties.

The Agreement has been executed in two (2) identical copies, one for each Party.

FINNISH MEDICINES AGENCY FIMEA

_____
Place and date

_____	_____
NN	NN
position	position

COMPANY

_____
Place and date

_____	_____
NN	NN
position	position