HAUTE AUTORITÉ DE SANTÉ

ASSESS HEALTH TECHNOLOGIES

SHEET

Instruction sheet for submission of a post-registration study protocol relating to a medicinal product

10 juin 2021

Key messages

- This instruction sheet details the practical arrangements for submitting protocols for post-registration studies requested by the Transparency Committee in its opinions.
- All exchanges between the pharmaceutical company and the HAS must be via the SESAME platform.
- All PRS applications are subject to re-evaluation by the CT when the final results become available, or on the basis of interim results in certain duly specified exceptional cases. For the practical arrangements for submitting the final results, please refer to the instruction sheet concerning the submission of an application to the Transparency Committee.
- For the design of the protocol, it is recommended to consult the methodological guide on real-world studies requested by the HAS.

Request for data

When major uncertainties have been identified during the assessment of a medicinal product, the Transparency Committee identifies the essential additional data required for the re-evaluation of an indication in its opinions. This data will need to be collected in the context of one or more post-registration studies. The wording of this request, detailed in the “recommendations” section, specifies the objectives of the study and the deadline for the results expected with a view to re-evaluation.

Based on the elements brought to its attention, the Committee can identify the available data sources and specify whether the study can be conducted using data from existing studies or databases (risk management plan studies, academic cohort or registry data, database studies, etc.) or whether ad hoc data collection is necessary. The request for additional data and the timetable for re-evaluation are reiterated in the e-mail sent to the pharmaceutical company via the SESAME platform when the final opinion is sent.

As soon as the Transparency Committee opinion is published online, the study will be added to the list of ongoing post-registration studies published on the HAS website in order to promote them. In the

table published online, the HAS specifies the name of the product, its therapeutic class, the date of the opinion in which the study was requested, along with the study objectives (prescribing or use condition, efficacy, impact on morbidity and mortality, impact on the healthcare system, etc.).

Submission of a synopsis within 3 months following receipt of the final opinion

From the date of receipt of the final opinion, the pharmaceutical company holding the Marketing Authorisation has three months to submit to the HAS Medicinal Product Evaluation Department (SEM) an initial synopsis detailing the concept of the planned study, via the SESAME Post-registration study protocols form put in place for this purpose. This document should be drafted in line with the principles of the methodological guide on real-world studies assessed by the HAS.

In particular, it should specify the type of study, the data source(s), the study population, the endpoints (including measures of interest to patients), the duration of follow-up and any comparators considered. In this first document, the pharmaceutical company may specify the questions or alternative proposals that it is submitting to the HAS for its opinion.

This synopsis can be written in English and can be submitted prior to obtaining the necessary regulatory authorisations to set up the study. It must be accompanied by:

- a letter written in French justifying the methodological choices and the consistency of the proposal with the Committee's request,
- a provisional timetable indicating the dates of regulatory authorisations, the start of inclusions where applicable and the submission of the final results,
- the list of members of the Scientific Board when necessary (even if not final).

Validation of the protocol: a maximum of 2 exchanges

The SEM shall send any comments and requests for clarification to the pharmaceutical company within a target period of 2 months following submission of the synopsis.

These comments will have to be taken into account in a complete study protocol that the pharmaceutical company shall be required to submit in accordance with the deadline provided by the HAS, within a period not exceeding 2 months following receipt of the HAS comments. Based on this complete protocol, the SEM will validate the study methodology or send a final set of comments.

The HAS may, on its own initiative, organise a video conference to clarify certain elements. The exchanges take place on the SESAME platform.

Follow-up during the study

The pharmaceutical company is required to inform the SEM via the SESAME platform of any changes to the protocol, including changes to the study schedule, that occur during the course of the study.

Submission of the final results

The final results of a post-registration study must be submitted as part of a “re-evaluation” dossier at the request of the Committee via the dedicated SESAME form. The dossier must be submitted at the latest 2 months before the date announced in the opinion containing the data request. For more information, consult the application submission guide and the standard application. Once the opinion has been finalised, it will be added to the table published online on the HAS website listing medicinal products with an opinion relative to post-registration study results.

Ce document présente les points essentiels de la publication : **Instruction sheet for submission of a post-registration study protocol relating to a medicinal product**, Méthode, **1er june 2021**
Toutes nos publications sont téléchargeables sur www.has-sante.fr

HAS HAUTE AUTORITÉ DE SANTÉ
Advancing quality in the fields of health and social care services

© Haute Autorité de santé – juin 2021