



HAUTE AUTORITÉ DE SANTÉ

GUIDE

Authorisation for early access to medicinal products: HAS assessment doctrine

This English version is published for information, however only the French version is deemed authentic.

17 June 2021

Contents

| | |
|---|-----------|
| Introduction | 3 |
| 1. Context | 4 |
| 1.1. Definition | 4 |
| 1.2. Early access authorisations | 4 |
| 1.3. Assessment process | 5 |
| 2. Eligibility criteria | 6 |
| 2.1. Severe, rare or debilitating disease | 6 |
| 2.2. Lack of appropriate treatment | 6 |
| 2.2.1. Prerequisites | 6 |
| 2.2.2. Definition | 6 |
| 2.2.3. Specific cases of concurrent developments and second entrant in the context of early access authorisations | 8 |
| 2.3. Impossibility to defer treatment initiation | 8 |
| 2.4. Presumptively innovative nature, particularly compared to any clinically relevant comparator | 8 |
| 2.4.1. Prerequisites | 8 |
| 2.4.2. General definition | 9 |
| 2.4.3. Novel treatment regimen | 9 |
| 2.4.4. Suitable development plan and basis of data required | 9 |
| 2.4.5. Specific cases | 11 |
| 2.4.6. Regulatory status of medicinal products | 11 |
| 3. Protocol for temporary use and data collection (PUT-RD) | 12 |
| 3.1. Value of observational/real-world data in the context of early access | 12 |
| 3.2. Guidelines for simplified and high-quality data collection | 13 |
| 3.3. Specific case of post-MA early access authorisation | 13 |
| Abbreviations and acronyms | 15 |

Introduction

The Transparency Committee (TC) of the French National Authority for Health (HAS) is responsible for the scientific and medical assessment of medicinal products when pharmaceuticals companies submit applications to the French Minister of Health for registration on the lists of medicinal products reimbursed by National Health Insurance.

The remit of the HAS was extended with the publication of the French Social Security Financing Act for 2021¹ which completely reformed the authorising procedure for early and derogatory access to medicinal products by creating:

- Pre-marketing authorisation (formerly cohort Temporary Authorisation for Use - ATUc) and post-marketing authorisation (formerly Temporary Funding Scheme - PECT) early access (AP, *accès précoce*)
- Compassionate access (AC, *accès compassionnel*) incorporating compassionate use authorisations (AAC, formerly ATUn) and compassionate prescription schemes (formerly RTU).

The HAS is now responsible for early access authorisation decisions. These decisions must be issued within short and regulated timeframes, allowing prompt access for patients to presumptively innovative medicinal products for indications with an unmet medical need. Authorisations for compassionate access are under the scope of competency of the ANSM.

The reform did not change the key role of the ANSM in establishing the presumption of efficacy and safety of an indication for which a marketing authorisation (MA) has not been granted. However, the reform gives the HAS the decision-making role in respect of early access authorisations and their public funding cover. This structure ensures that assessments and decisions are consistent by creating a continuum of access between derogation-based schemes and the common law funding scheme. The reform allows both institutions to collaborate within their respective area of expertise (registration in the list of treatments eligible for reimbursement as regards the HAS).

The doctrine enables the HAS to describe its methods in close collaboration with the ANSM: transparency, patient involvement, and observational/real-world data collection reinforcement.

By adopting an assessment doctrine that is specific to early access, the HAS has sought to provide useful information for stakeholders on how it will issue its authorisations because, beyond its transparency obligation, the HAS is very committed to giving visibility to its assessment methods and thus ensuring consistency in its decisions.

¹ [Article 78 of the French Social Security Financing Act for 2021 No. 2020-1576 of 14 December 2020.](#)

1. Context

1.1. Definition

The doctrine is a work tool designed to provide benchmarks and visibility with respect to the main criteria for the assessment of medicinal products and, consequently, the expectations concerning submissions by companies.

The doctrine explains the basic principles of the scientific and methodological rationale adopted by the HAS when it analyses data and incorporates it in its assessments, in view of the medical context. This doctrine forms a general framework to be applied to early access decisions. It is designed to be updated notably to consider methodological, regulatory or contextual changes.

The explanation of early access authorisation application assessment methods aims to:

- outline the fundamental principles of its assessments and in particular the similarities and differences between the early access authorisation decision and the assessment by the Transparency Committee for public funding cover (see Transparency Committee’s doctrine)
- to address request for clarifications from public authorities, patient and consumer associations and manufacturers.

The objective is to guarantee transparent, reproducible, fair, and consistent decisions.

1.2. Early access authorisations

Early access authorisation is an exceptional derogation-based scheme enabling the early availability and reimbursement for one or more indication(s) of a medicinal product indicated **for a severe, rare or incapacitating disease**, when **all the following conditions** stipulated in article L.5121-12 of the French Public Health Code (CSP) are met:

- *There is no appropriate treatment,*
- *The initiation of the treatment cannot be deferred,*
- *The efficacy and safety of the medicinal product are strongly presumed based on the results of clinical trials;*
- *This medicinal product is presumed to be innovative, notably compared with a clinically relevant comparator.*

The HAS shall make a decision in respect of these eligibility criteria when a manufacturer applies for an early access authorisation for a specific indication, after a favourable review by the ANSM on the presumption of efficacy and safety of the indication if an MA has not yet been granted for this indication. For added clarity, these criteria are explained hereinafter in chapter 2 of this document.

The HAS points out that this early access scheme is no substitute for a clinical trial. The inclusion of eligible patients in an ongoing clinical trial with the medicinal product in question must be prioritised.

Early access authorisation is applicable to medicinal products for:

- **An indication for which an MA has not yet been granted** and for which the pharmaceutical company has submitted or undertakes to submit an MA application once the early access authorisation has been granted. This early access is referred to as “Pre-MA early access”.

The decision of the HAS is taken following a favourable opinion from the ANSM, confirming the strong presumption of efficacy and safety of the medicinal product for the indication in question.

The pre-MA early access authorisation is subject to compliance to a protocol for therapeutic use and data collection from treated patients (PUT-RD, *protocole d'utilisation thérapeutique et de recueil de données*) and to the periodic submission of a summary report of these data (see [section 3](#) of this document).

- **An indication for which an MA has been granted and which is not yet reimbursed within the common law framework**, and for which the pharmaceutical company has submitted, or undertakes to submit within one month following the granting of the MA, an application for registration in one of the two lists of medicinal products eligible for reimbursement. This early access is referred to as “Post-MA early access”.

Post-MA early access can apply to:

- an indication previously authorised for “pre-MA” early access under the first scheme;
- or an indication that has never been approved for “pre-MA” early access funding (first post-MA early access application).

Only the HAS is involved in post-MA early access decisions, including following pre-MA early access, insofar as an MA has been granted for the indication in question, confirming the efficacy and safety of the medicinal product.

As for pre-MA early access, the post-MA early access authorisation is subject to adherence to a protocol for therapeutic use and data collection from treated patients (PUT-RD), which may be simplified compared to that applied for the “pre-MA” scheme, and to the periodic submission of a summary report of these data.

1.3. Assessment process

Prior to the decision of the HAS, the Transparency Committee issues an opinion as to whether the eligibility criteria stipulated in article L. 5121-12 of the French Public Health Code have been met, and in relation to the PUT-RD.

The early access authorisation decision may be accompanied by a greater degree of risk for the patient than that generally accepted within the scope of the assessment for reimbursement, in particular for pre-MA early access authorisations. Indeed, the maturity of the application submitted and the level of evidence of the data available at the time of authorisation are more limited.

In order to ensure satisfactory early access conditions, and in view of the potential uncertainties pending the availability of the findings of clinical trials, the eligibility criteria for the early access authorization are continuously assessed.

As for all medical and scientific assessments, the HAS assessment is based on an analysis of all the clinical data available at a given time for the medicinal product in question and in the indication assessed. The assessment is, by definition, temporary; it corresponds to a snapshot at a given point in time and is liable to evolve on the basis of new data concerning the product's efficacy and safety.

Before issuing its opinion, the Committee may decide to hear patients, stakeholders and/or the manufacturer having submitted the application.

2. Eligibility criteria

2.1. Severe, rare or debilitating disease

The severity of a disease or its debilitating nature is assessed in view of the medical context based on the description of the symptoms and organ involvement, the mortality rate, and the impact of the disease on patients' quality of life.

The prevalence and incidence of the disease are used to support its rarity (particularly in accordance with article 4 of the Council Recommendation of 8 June 2009 on an action in the field of rare diseases).

2.2. Lack of appropriate treatment

2.2.1. Prerequisites

Identifying an appropriate treatment (AT) is an important step in the early access decision, insofar as the existence of an AT in the treatment pathway represents a criterion for the denial of early access to a medicinal product.

The lack of AT aims to ensure that no satisfactory therapeutic option other than the candidate medicinal product for early access is available for the patient in routine practice.

This process should be differentiated from the identification of clinically relevant comparators (CRCs) carried out by the Transparency Committee, when assessing the medicinal product for registration in the list(s) of products eligible for reimbursement, with a regulatory (price setting) and scientific (assessment of the quality of the evidence) objectives.

Given the current definition of clinically relevant comparators (see [Transparency Committee's doctrine](#)), the HAS considers that an appropriate treatment must be a clinically relevant comparator, but that a clinically relevant comparator is not necessarily an appropriate treatment.

The HAS highlights that it can identify ATs according to the subpopulations included for the indication with a presumption of efficacy and safety by the ANSM (pre-MA) or for the indication covered by the post-MA early access application.

2.2.2. Definition

An appropriate treatment is a pharmacological or non-pharmacological therapeutic alternative:

- recommended at the same level of the treatment pathway on the date of the assessment,
- AND accessible in routine practice in France on the date of the assessment,
- AND reimbursement by public funding on the date of the assessment,
- AND with satisfactory efficacy and safety data to suggest that the patient would lose no potential benefit compared with the predictable benefit of the drug in the early access application.

A medicinal product alternative is considered recommended if it is:

- An authorised treatment, i.e. approved for marketing authorisation (MA) and/or early access,
- Or a treatment used off-label, on a case-by-case basis, in the light of the guidelines of national and international learned societies or public bodies, and particularly if data documenting its efficacy are available.

A medicinal product alternative is considered accessible in routine practice if it is available in France at the time of the early access assessment, without stock shortages or significant supply pressure.

A medicinal therapeutic alternative is considered reimbursed by public funding if:

- It is used in accordance with its MA and:
 - included in one of the lists of products eligible for reimbursement (retail and/or hospital formulary list) on the date of the assessment, documented by publication in the Official Journal
 - Or authorised under post-MA derogation-based access;
- It is used off-label and:
 - used in hospitals,
 - or authorised under pre-MA derogation-based access.

A non-medicinal therapeutic alternative should be recommended, accessible and covered by funding.

Supportive care treatments, which can be clinically relevant comparators, will not be considered as sufficient appropriate treatments compared to the potential contribution of the medicinal product covered by the early access application.

Where the appropriate alternative is a medicinal product included only in the hospital formulary list but not included in the supplementary list, in particular indications with a minor Clinical Added Value score (CAV IV – ASMR IV), the HAS will recommend effective cover for all patients subject to this assessed indication rather than authorise early access to a medicinal product with a higher level of uncertainty.

Consequently, any medicinal product, whether authorised or off-label, or any non-pharmacological therapeutic alternative, recommended in routine practice, covered by public funding, and which has a similar benefit compared to the medicinal product subject to early access can be considered as an appropriate alternative. The benefit, defined in terms of efficacy, safety or care pathway, accounts for the data available and uncertainties in respect of the medicinal product subject to early access.

The HAS specifies that, at an equivalent level of presumptive efficacy, a therapeutic alternative will not be considered as an appropriate treatment once the purpose of the medicinal product covered by the early access application is to:

- Simplify the care pathway or have a positive organisational impact (e.g. hospital or non-hospital care pathway);
- Improve patients' quality of life (e.g. change from injectable administration to oral administration);
- Improve the purpose of the treatment (e.g. from palliative purpose to curative purpose).

2.2.3. Specific cases of concurrent developments and second entrant in the context of early access authorisations

In the case of concomitant developments of medicinal products for a similar indication and with efficacy and tolerance levels deemed to be similar, several early access authorisation applications may be requested within a short period of time. Should this occur, the HAS had defined the situations in which the first medicinal product applied for will be considered as an AT for the later medicinal products applied for.

In the case of early access for the later medicinal product (and based on equivalent benefit compared to the first medicinal product for which no AT had been identified), the first medicinal product will be considered as an AT once it is available and reimbursed on the date of the early access application in respect of the second medicinal product.

As a result:

- If two medicinal product applied for simultaneously, the first will not be considered an appropriate treatment for the second and vice versa;
- If early access to the second medicinal product is applied for more than two months after a positive decision for the first medicinal product, the first medicinal product may be considered an AT provided it is available;
- If early access to the second medicinal product is applied for while the first medicinal product is being assessed or within two months of a positive decision if the first medicinal products is available, the first medicinal product will not be considered an AT.

2.3. Impossibility to defer treatment initiation

The assessment of the option to defer a treatment without involving a serious and immediate risk for the patient's health, is particularly based on whether an appropriate treatment exists or not.

2.4. Presumptively innovative nature, particularly compared to any clinically relevant comparator

2.4.1. Prerequisites

The presumption of innovation is assessed with regard to the development plan of the medicinal product compared to its clinically relevant comparator(s) if they exist, i.e. in relation to the resources available in the care strategy.

The classification of presumptive innovation does not prejudice the subsequent conclusions of the Transparency Committee within the scope of the assessment for inclusion in the lists of products eligible for reimbursement.

Within the candidate indication for early access authorisation, the HAS may need to identify subpopulations for whom presumptive innovation can be recognised. This segmentation may particularly be carried out based on the development plan.

2.4.2. General definition

Pending the assessment for inclusion in the lists of products eligible for reimbursement:

A potentially innovative medicinal product within the scope of an early access authorisation is required to fulfil the three following conditions:

- It is a novel treatment regimen offering patients a substantial change,
- The medicinal product has a suitable development plan and clinical findings supporting a presumptive benefit for the patient
- The medicinal product must not have any significant unknowns in relation to safety or other important data.

The HAS emphasises that early access authorisation must not hamper inclusions in an ongoing clinical trial.

2.4.3. Novel treatment regimen

The medicinal product must represent a novel treatment regimen in respect of the disease, capable of contributing major progress or a substantial change in treatment, regardless of the mode of action of the medicinal product (novel or not), and either in terms of efficacy (including quality of life), safety, practicality or convenience of use or care pathway (organisational impact).

The HAS considers a novel mode of action on its own without any evidence of an effect not to be sufficient to define presumptive innovation. For this reason, the HAS uses the concept of novel treatment regimen, which is broader than merely a novel mode of action.

2.4.4. Suitable development plan and basis of data required

Time-frame of early access application

The HAS considers the time-frame of the early access application with respect to the envisaged MA date, i.e. the date of the early access application in relation to the date of the MA for the indication in question.

For this reason, the data available **shall vary according to the timing of the early access authorisation application** in relation to the estimated MA granting date or the actual granting date.

The HAS has therefore sought to **adapt its requirements** to account for applications prior to the availability of data from pivotal study/studies for the medicinal products under development. As such:

- **For early access authorisation applications submitted well before the estimated MA granting date (pre-MA application prior to filing the MA application or distant estimated MA date):**
 - preliminary findings (suggesting a significant benefit) from proof-of-concept clinical trial(s) (comparative or not, optionally on an intermediate endpoint, if justified)
 - and with a suitable development plan, including:
 - a comparative pivotal trial in the final recruitment phase, wherein the trial design enables the demonstration of clinical added value
 - a trial with simplified methodology but deemed acceptable by the Committee given the severity or rare nature of the disease

- **For early access authorisation applications near the estimated MA granting date**
 - intermediate or final findings of pivotal study/studies submitted with the MA application with a suitable design and findings indicating a major benefit in respect of efficacy and/or safety and/or practicality and/or quality of life
 - **For early access applications for an indication approved by the MA (post-MA)**
 - conclusive findings on the primary endpoint of the pivotal superiority study/studies enabling the granting of the MA
- or
- findings from a trial with simplified methodology but deemed acceptable and enabling the granting of the MA given of the severity or rare nature of the disease,
 - and with an expected benefit for the patient.

Suitable development plan

The HAS will assess whether the manufacturer’s development plan enables the demonstration of a potential clinical benefit, and thus helps minimise the risk associated with the lack of availability of findings or their partial availability or in the case of immature findings.

In order to deem a development plan suitable, the HAS shall particularly consider the following factors described below (non-exhaustive list).

Table 1: Summary of the methodological factors determining whether a study design is suitable for recognition of presumptive innovation or not

| Study methodology | Suitable | Unsuitable | Specific scenarios for which a study methodology conventionally deemed “unsuitable” might be suitable |
|--------------------------|---|--|---|
| Development phase | II or III | I with no other ongoing study | Rare diseases |
| Objective | Superiority | Non-inferiority | Antibiotic therapy, antivirals |
| Study type | Comparative Non-comparative if justified, e.g.: - Effect size deemed significant by the TC - Comparison vs external control | Non-justified non-comparative | Rare diseases |
| Comparator | Clinically relevant comparator (CRC) Placebo or supportive care if justified (rare diseases, treatment of last resort) External control | Unjustified placebo Numerous CRCs available with possible comparison Previous entrant provided comparison to a CRC Non-clinically relevant active comparator while CRCs are available | Rare diseases |

| | | | |
|------------------|---------------------|---|--|
| Primary endpoint | Clinically relevant | Not clinically relevant and with no suitable development plan | Paediatric pharmacokinetics (if scope of extrapolation of data available for adults) Rare disease: inclusion of secondary EPs in the case of biological CRC |
|------------------|---------------------|---|--|

CRC: clinical relevant comparator, EP: endpoint

Results

The presumption of significant therapeutic improvement compared to the existing situation shall be assessed by the Committee with regard to the data available, including preliminary data in the case of early access applications submitted well before the envisaged MA granting date for the indication in question.

It should be noted that where the findings are available, the positive impact, major progress or substantial change in respect of care should be documented to confirm the innovative nature of the medicinal product for the indication in question. For this purpose and unless duly justified, an assessment of the endpoint(s) should be envisaged in the study protocol with a view to being able to demonstrate this benefit.

For example, an assessment criterion envisaged in the pivotal study to assess the positive impact of the delivery form should make it possible to demonstrate this impact once the data are available, subject to exceptions.

2.4.5. Specific cases

In view of the wide range of clinical scenarios encountered, it is not possible to envisage a single definition of presumptive innovation. In this context, the HAS assesses the different components of the definition accounting for specific aspects of certain therapeutic fields such as infectious disease and paediatrics. Exceptions are considered on a case-by-case basis. The weight of the components taken into account in the definition will be dependent on the scenarios encountered. It will not be possible to establish thresholds given the diversity of the scenarios encountered.

2.4.6. Regulatory status of medicinal products

In the indication concerned by early access, medicinal products designated orphan status or awarded PRIME status by the EMA (or *breakthrough therapy* status by the FDA) are likely to fulfil the conditions to be considered presumptively innovative insofar as the criteria to be met to be admissible for the designation of orphan status or for PRIME status concern the same aspects, particularly the fact that the medicinal products are likely to provide major clinical added value compared to existing treatments, or to benefit patients with no treatment options or with an unsatisfactory treatment option.

This regulatory status does not exempt the HAS from ruling on the eligibility criteria.

3. Protocol for temporary use and data collection (PUT-RD)

Early access authorisations are subject to the pharmaceutical company complying with a protocol for temporary use and data collection (PUT-RD), set out by the HAS, in collaboration with the ANSM where applicable, and appended to the authorisation decision.

This PUT-RD allows the collection of observational/real-life data from patients receiving a medicinal product under an early access authorisation. These data are collected under care routine conditions and not collected as part of a research study.

The data collected as part of the early access authorisation procedure are not intended to replace clinical trials and do not change the Transparency Committee's expectations in terms of clinical development set out in the [Transparency Committee's doctrine](#) for inclusion in the list of medicinal products eligible for reimbursement (stemming from common law). On the other hand, these data are additional and therefore provide input for the assessment of the medicinal product by the HAS for early access authorisation renewal and, eventually, for the assessment for reimbursement.

Information collection as part of the early access authorisation procedure must be the subject of a declaration of conformity with the specific French data protection authority (CNIL) framework for this data processing in the context of early access.

As a reminder, early access authorisations are no substitute for clinical trials: inclusion of patients eligible for an ongoing clinical trial for the indication in question in such a trial must be prioritised.

3.1. Value of observational/real-world data in the context of early access

For a new medicinal product or a new indication, early access schemes represent the **first opportunity to collect real-life observational data/in France to document its use and contribute to the future Transparent Committee assessment for registration in lists of medicinal products eligible for reimbursement.**

For medicinal products available early before an MA is granted, the PUT-RD should enable the collection of a restricted number of variables regarding:

- **Patient characteristics**, including those relating to the disease and to compliance with the indication along with prescriber characteristics;
- **Conditions of use**;
- **Efficacy, including quality of life** using a patient reported outcome measure (PROMs);
- **Safety**.

All of the data and variables expected by the HAS are detailed in the manufacturers' [guidance document on submitting an early access application](#). It is recommended, in the absence of previously developed and validated tools, to consult with patient associations and learned societies to identify the variables of interest, particularly those pertaining to efficacy and quality of life. PROM validity and interpretation in the investigated disease should be supported with a literature review. It is also advised to refer to the [HAS methodology guide on real-word studies](#).

The HAS points out that these outcomes must be clinically relevant. The data collection should be possible in routine clinical practice without requiring supplementary or additional check-ups or examinations.

The pharmaceutical company should submit to the HAS, and to the ANSM where applicable, a draft PUT-RD based on the [template available on the HAS website](#).

The results of the data collection will be presented in a summary report drafted by the pharmaceutical company, according to a frequency set out in the PUT-RD. Insofar as possible, the HAS shall define the frequency to align it with the early access renewal application and with the estimated reimbursement application filing date. It is forwarded to the HAS and to the ANSM and a summary of this report shall be published online.

3.2. Guidelines for simplified and high-quality data collection

Early access to a medicinal product *de facto* involves the set-up of data collection. The HAS shall particularly focus on:

- **Data quality and exhaustivity:** the manufacturer is expected to play an active role in data collection input and monitoring by providing the necessary resources to the medical teams concerned. These data must be systematically collected and processed. The proportion of missing data should be limited (<10%) to enable an exhaustive assessment of all the patients treated with the medicinal product concerned by early access. A high number of missing data items and/or failure to adhere to the PUT-RD will be considered by the HAS in its decisions and assessments.
- **Patient involvement in the scheme,** with the inclusion of a PROM where patient feedback is essential, particularly in incapacitating and severe diseases. The manufacturer is expected to provide a validated French-language self-reported questionnaire, that is interpretable and specific to the investigated disease, after consulting with a patient association. Failing a validated specific self-reported questionnaire for the disease, a “Patient Global Impression Change” type question can be envisaged.
- **Simplification of data collection by clinicians, pharmacists, and patients:** the HAS recommends prioritising the use of digital platforms to facilitate data input, ensure traceability, and prevent missing data. Standardisation of the variables to be collected and the PUT-RD template published online by the HAS will also help the stakeholders concerned in the field to have better knowledge of the data expected and become familiarised with data input.
- **Potential reuse of data for research purposes particularly in the context of post-inclusion studies and after obtaining the regulatory authorisations required.** To this end, it is recommended to design the data collection **to facilitate linkage with data from the SNDS** (National Health Data System) upon request of the HAS. Database storage on the Health Data Hub is also encouraged.

3.3. Specific case of post-MA early access authorisation

If the early access authorisation application for a medicinal product is made after the MA has been awarded, the requirements in relation to data collection within the scope of the PUT-RD may be reduced where applicable.

If data collection is required, pending the assessment by the Transparency Committee, for common law reimbursement, the HAS may limit its request under the PUT-RD solely to information pertaining to the number of patients treated accompanied by a description of patient and prescriber characteristics and of the conditions of use. Safety data should be collected in the conventional pharmacovigilance circuit.

Abbreviations and acronyms

| | |
|---------------|---|
| AAC | <i>Autorisation d'accès compassionnel</i> (Compassionate access authorisation) |
| AC | <i>Accès compassionnel</i> (Compassionate access) |
| ANSM | <i>Agence Nationale de Sécurité des Médicaments et des produits de santé</i> (French National Agency for Medicines and Health Products Safety) |
| AAP | <i>Autorisation d'accès précoce</i> (Early access authorisation) |
| AP | <i>Accès précoce</i> (Early access) |
| AT | Appropriate treatment |
| ATU | <i>Autorisation temporaire d'utilisation</i> (Temporary authorisation for use) |
| ATUc | <i>Autorisation temporaire d'utilisation de cohorte</i> (Cohort Temporary Authorisation for Use) |
| ATUn | <i>Autorisation temporaire d'utilisation nominative</i> (Named-patient Temporary Authorisation for Use) |
| CAV | Clinical Added Value |
| CRC | Clinically relevant comparator |
| EP | Endpoint |
| HAS | <i>Haute Autorité de santé</i> (French National Authority for Health) |
| MA | Marketing authorisation |
| PECT | <i>Prise en charge temporaire</i> (Temporary funding scheme) |
| PUT-RD | Protocol for temporary use and data collection |
| RTU | <i>Recommandation temporaire d'utilisation</i> (Temporary recommendation for use) |
| TC | Transparency Committee |

Find all our publications at
www.has-sante.fr

