Regulating Early or Compassionate Access to Health Products

Consultez la version française

Several types of procedures regulate and facilitate the early or compassionate use of certain health products. These procedures enable patients to gain rapid and safe access to health products that represent a major therapeutic advance or address an unmet medical need.

Additionally, the Innovation and Guidance Desk (Guichet Innovation et Orientation, GIO) is a support service for the development of innovative health products.

Compassionate Use and Early Access Authorizations

When a drug does not yet have—or does not have at all—marketing authorization (MA) in France, its use may be exceptionally authorized if the presumed benefit outweighs the risk and only in the absence of an appropriate therapeutic alternative.

Authorizations may be granted for **compassionate use** (Compassionate Use Authorization – Autorisation d'accès compassionnel (AAC)) or **early access** (Early Access Authorization – Autorisation d'accès précoce (AAP)).

AAC are issued by ANSM upon request from a physician or healthcare facility for a specifically named patient. They primarily concern drugs not intended for commercialization for the relevant indication, which are neither under development nor subject to an MA application. AAC are valid for one year and may be renewed. For drugs under development for commercial purposes, the provision of AAC is linked to a commitment from the laboratories to apply for early access authorization.

AAP are issued by the French National Authority for Health (Haute Autorité de Santé, HAS) upon request from a laboratory. In this case, the drug in question is prescribed to a group of patients treated and monitored according to criteria defined in a therapeutic use protocol (protocole d'utilisation thérapeutique - PUT) and data collection protocol (recueil des données - RD).

- If the AAP application is submitted **before obtaining MA**, it can only be granted after evaluation and a favorable opinion from ANSM.
- If the AAP application is submitted **after obtaining MA**, HAS alone conducts the evaluation and decision-making process.

Compassionate Prescription Framework

When a drug already has MA for one or more indications but is used in practice for other situations, ANSM may establish a **compassionate prescription framework** (cadre de prescription compassionnelle - CPC) for a period of three renewable years to ensure safe prescribing.

A CPC can only be granted if the therapeutic need is not already met by another treatment and if scientific data suggest a favorable benefit/risk balance for the patients concerned.

Exemptions for Medical Devices

An exemption system exists for medical devices. It allows for the exceptional use of these devices, in the absence of CE marking, in very specific situations and always in the best interest of patients.

Promoting Research and Therapeutic Innovation in France for the Benefit of Patients

The Innovation and Guidance Desk (guichet innovation et orientation - GIO) is an ANSM service that supports the development of innovative health products. It facilitates exchanges between ANSM departments and innovation stakeholders (academics, start-ups, and industry) involved in the development of a health product. It is a digital exchange platform through which project leaders can request opinions or meetings on scientific, technical, or regulatory issues, all within a harmonized framework and in compliance with ethical standards.

This service raises awareness and supports project leaders from the earliest stages of development, even before applications or requests are submitted to the Agency. The advice provided is based on the specific characteristics of the product under development or the research being conducted, as well as the latest knowledge in terms of diseases, target populations, and existing treatments.

By providing guidance on required data, procedures to follow, and regulatory expectations, this service accelerates the availability of innovative health products within the framework of clinical research, particularly for unmet medical needs, rare diseases, or pediatric projects..

Access the guichet innovation et orientation (GIO) (in French)

Furthermore, as part of its participation in the**Scientific Advice Working Party (SAWP)** of the European Medicines Agency (EMA), ANSM is responsible for providing scientific opinions on drug developments or investigation techniques useful for these developments. These opinions are submitted to the Committee for Medicinal Products for Human Use (CHMP) or the Committee for Orphan Medicinal Products (COMP)..