Accelerating access to treatments for patients

Dr Valérie DENUX
Director Europe & Innovation

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Accelerating the access to Drugs through special authorizations

Laboratory research → Early CT → CT → Pivotal study → MA → Pricing

Individual Compassionate Use

Early access Program
- Pre-MA
- Post-MA

Off Label use framework
- Compassionate Prescription program

Special authorizations: new framework since July 2021
Individual compassionate use (AAC) assessment on a case by case basis

Assessment criteria

1. Commitment to submit an early access application is provided
2. Serious, rare or disabling illness
3. Lack of appropriate treatment
4. Presumed efficacy and safety
5. Patient not eligible on a current clinical trial in this indication
6. Clinical trial authorized but for which, the patient cannot be included
7. Treatment cannot be deferred
8. No MA in France

Criteria assessed by indication + very early access cases

Publication on the ANSM Website

- Definition of targeted population
- Protocol for therapeutic use
- Conditions of use
- Precautions for use and monitoring measures
- Summaries of periodic reports

Request by Hospital prescribers
But GPs can refer patients to their hospital correspondant physician to ensure access to these innovative treatments.

Pharmaceutical companies agree or not to give access to their product through AAC

Based on Directive 2001/83/CE – Article 5

Classified as public by the European Medicines Agency
Individual compassionate use in 2023 (AAC)

Compassionate use authorization for drugs with a commitment to early access program

12 drugs with commitments to AP are mainly used in oncology indications in 2023

More than 900 AAC have been delivered in oncology in 2023 for pediatric patients
Secured and extended access through a e-CPS connection
Easy search (brand name, INN, indication, etc.)
Communication via Programmable alerts and notifications
Rapid identification of actions to be taken (banner system)
Easier for piloting authorizations
Early access authorization (AAP) patient cohort evaluation

Based on Regulation 726/2004 – Article 83

- Treatment cannot be postponed
- Serious, rare or disabling illness
- Positive benefit/risk for patients
- Lack of appropriate treatment
- Presumed to be innovative, particularly with regard to a clinically relevant comparator

Hospital prescribers and general practitioners (particularly in certain health situations - COVID) can include their patients in these cohorts, provided they meet the conditions described in the Protocol for Therapeutic Use (PTU)

More than 100 000 patients treated in this framework since 1st of July 2021
Early access authorization

180 decisions over the past 2 years, including 125 new products

AAP decision since July 2021

- pre-MA Program
- Post-MA Program
- AP Prolongation

Favorable decision for early access program by therapeutic domain

- Oncology
- Nephro
- Dermato
- Cardio
- Hemato
- Neuro
- Endocrinology
- Infectiology
- Others

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Special authorizations: accelerating the access to treatments

Difference in access time (in months) for innovative medicines between France and other countries through the early access system

On average, patients have access 11 months earlier to drugs through the early access system with clinical benefit before a price is published in one of the corridor countries.

Source HAS

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Compassionate Prescription program (CPC) - off-label use

**Assessment criteria**

1. Presumed positive benefit/risk ratio
2. Lack of appropriate treatment
3. No clinical trials in this indication
4. No MA envisaged

**Publication on the ANSM Website**

- The decision
- The protocol for therapeutic use (PTU)

Implies off-label use to be reported by healthcare professionals, patient associations or institutions

Hospital prescribers and general practitioners can prescribe these CPC-eligible treatments (outside the AMM), provided they meet the conditions described in the PTU.

Provided reliable data collection can help for designing confirmatory clinical trial through repurposing process

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**Accelerate access to new indications**

Based on Directive 2001/83/CE – Article 5

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Compassionate Prescription program (CPC)

Clinical trial → MA → Commercial use in the MA indication

Supportive data → Consensus and RWE data

Literature → European and international recommendations

Signal → INCA, CRMR et CCMR, CNPC, patients associations

Assessment and decision granted by ANSM → CPC established

Drug repurposing

Use within the CPC program

Data collection PTU-SP

MA/MA extension

Confirmatory clinical trial

Drug repurposing
Compassionate prescription program off-label use (CPC)

- 28 CPC currently authorized, half of which in rare diseases
- 14 signals within the instruction process
- 91 signals of interest in rare diseases prioritised and to be signaled following off-label use identification
Conclusions

◆ Thousands of patients accessing treatments through special authorization programs

◆ Patients acces to innovative treatment acceleration

◆ Difficulties related to data collection (time consuming, heterogeneity, etc)

◆ Need to strengthen data analysis and utilization
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60’ Lunch break

THE SESSION WILL START AT 13.30 PM CET
or 09.30 am EST time