EARLY ACCESS IN FRANCE: REASONS FOR REFUSALS AND IMPACT ON MARKET ACCESS

Theuillon Tristan[1], Le Mao Julie[1]

[1] – CEMKA, 92 340 Bourg la Reine, France

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CONTEXT

In France, patients can benefit an early access to innovation, ahead of the marketing authorization or final reimbursement since 1992. The previous system, called Temporary Use Authorisation (TUA), had a number of limitations, in particular the lack of consistency between the evaluation for this early access program and the evaluation for reimbursement. According to the HAS, not all medicines within the framework of the TUA necessarily constitute breakthroughs in innovation. A report dated September 2017 (1) of the social security accounts commission, states that while this evaluation most often confirms the overall efficacy of treatments, their performance in relation to therapeutic alternatives, when they exist, is not always proven. This observation reveals that TUAs were granted in a very broad manner before leading to poor evaluations by the HAS for the reimbursement.

This early access program (EAP) was reformed on July 1, 2021. As of this date, the eligibility criteria, the agency responsible for EAP granting, the timelines and the process have evolved. Medicines indicated in a severe, rare or disabling disease, are eligible to EAP when there is no suitable treatment available on the market, when efficacy and safety are presumed and when the product is considered presumed innovative.

The HAS has provided manufacturers with several tools to help them understand the authorities' expectations in the context of early access. An assessment doctrine providing useful guidelines for the various stakeholders on how the HAS will make its assessments and a guide for laboratories presenting the information necessary for the preparation of an application for early access authorizations for medicinal products by the HAS and, where applicable, the ANSM, are also available online (2).

OBJECTIVES

To identify (1) the reasons for not granting EAP and (2) the impact on market access in France

METHODS

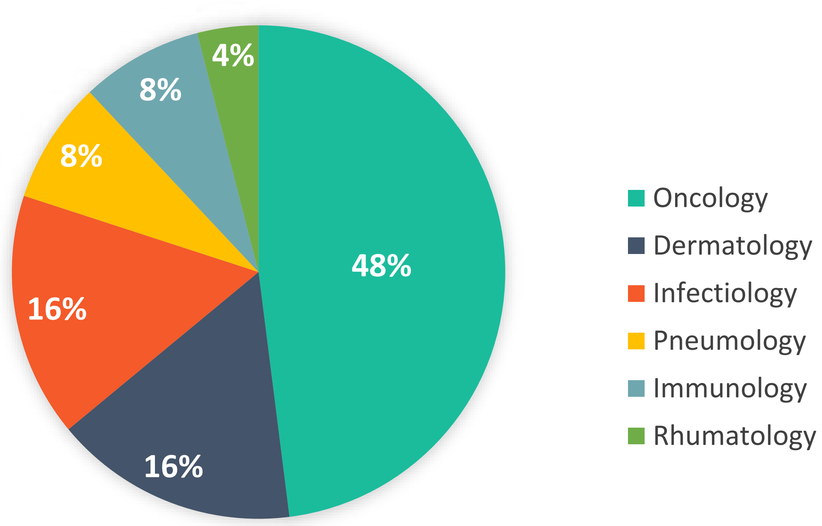
We conducted a retrospective analysis of all HAS decisions published between July 1, 2021, and May 31, 2023 that resulted in an early access denial.

RESULTS

- Among 119 EAP evaluations, 25 refusals (21%) were identified. Among the refusals, there are 11 EAP-1 (before the MA) and 14 others EAP-2 (post-MA). 11 refused drugs (n=25) had a prior TUA (assessment by the ANSM). TUA is the previous early access system, before the establishment of the EAP. It was an initial assessment by the HAS for 60% of refused treatments.

In total, the most represented therapeutic areas were oncology (48%), infectiology (16%) and dermatology (16%).

FIGURE 1. MEDICINES REFUSED IN EAP - THERAPEUTIC AREAS MOST REPRESENTED

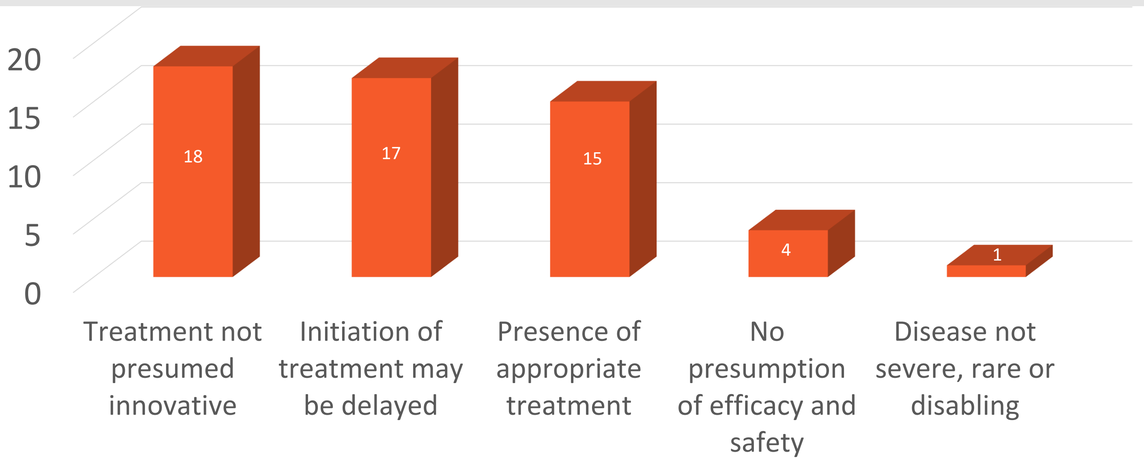


- There are various reasons for refusal. Firstly, one medicine was not indicated in severe, rare or disabling disorders. Then, appropriate comparators were identified for 60%, highlighting a partially met medical need.

Finally, efficacy and safety were not presumed for 16% and 72% were not presumed innovative by the HAS. Treatment is not presumed innovative because of:

- No minimum clinical data for 16 drugs
- no new treatment modality for 13 drugs
- Presence of uncertainties for 12 drugs

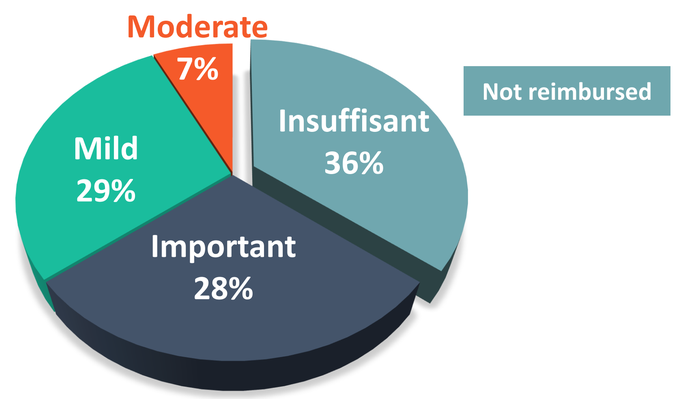
FIGURE 2. MEDICINES REFUSED IN EAP - REASONS FOR REFUSAL



- Criterion « Initiation of treatment may be delayed » is not a determining factor in the authorization of the EAP and depends on two other eligibility criteria. Among the refusals due to this criterion, 12 were due to the availability of appropriate treatments and 1 due to the severity criterion not being met.

- In fine, 14/25 medicines were evaluated for reimbursement in France. Of these, 9 were reimbursed, all in a restricted indication compared to the requested EAP indication. The TC granted an important (4/9), moderate (1/9) or low (4/9) Clinical Benefit (*Service Médical Rendu, SMR*), associated with an no Clinical Added Value (*Amélioration du Service Médical Rendu*, ASMR V) for 6 drugs or a minor Clinical Added Value (ASMR IV) for 3 drugs.

FIGURE 3. CLINICAL BENEFIT (SMR) EVALUATION FOR REIMBURSEMENT BY THE TC OF THE MEDICINES REFUSED IN EAP



CONCLUSION

The most common criteria leading to Early Access refusal was the presumption of innovation, not reached in 72% of refused medicines. In France, the denial of Early Access Program appears to be predictive of achieving no Clinical Added Value (*Amélioration du Service Médical Rendu*, ASMR V) and not important or even insufficient Clinical Benefit (*Service Médical Rendu,* SMR) resulting in no reimbursement.

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REFERENCES
(1) https://www.securite-sociale.fr/files/live/sites/SSFR/files/medias/CCSS/2017/RAPPORT/CCSS_RAPPORT-SEPTEMBRE%202017.pdf
(2) https://www.has-sante.fr/upload/docs/application/pdf/2021-06/acces_precoces_-_doctrine.pdf

COI : Tristan THEUILLON and Julie Le Mao are employees at CEMKA, one of the first French consulting firms in the field of evaluation of products, programs and organizations in Health.
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