EARLY ACCESS IN FRANCE: DESCRIPTIVE ANALYSIS OF FIRST EAP DECISIONS

Le Mao Julie [1], Gaugy Prunelle [1]

1 – CEMKA, 92 340 Bourg la Reine, France

Acceptance Code : HTA149

CONTEXT

In France, patients can benefit an early access to innovation, ahead of the marketing authorization or final reimbursement since 1992. 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.

OBJECTIVES

Our study consisted of a descriptive analysis of EAP first evaluations.

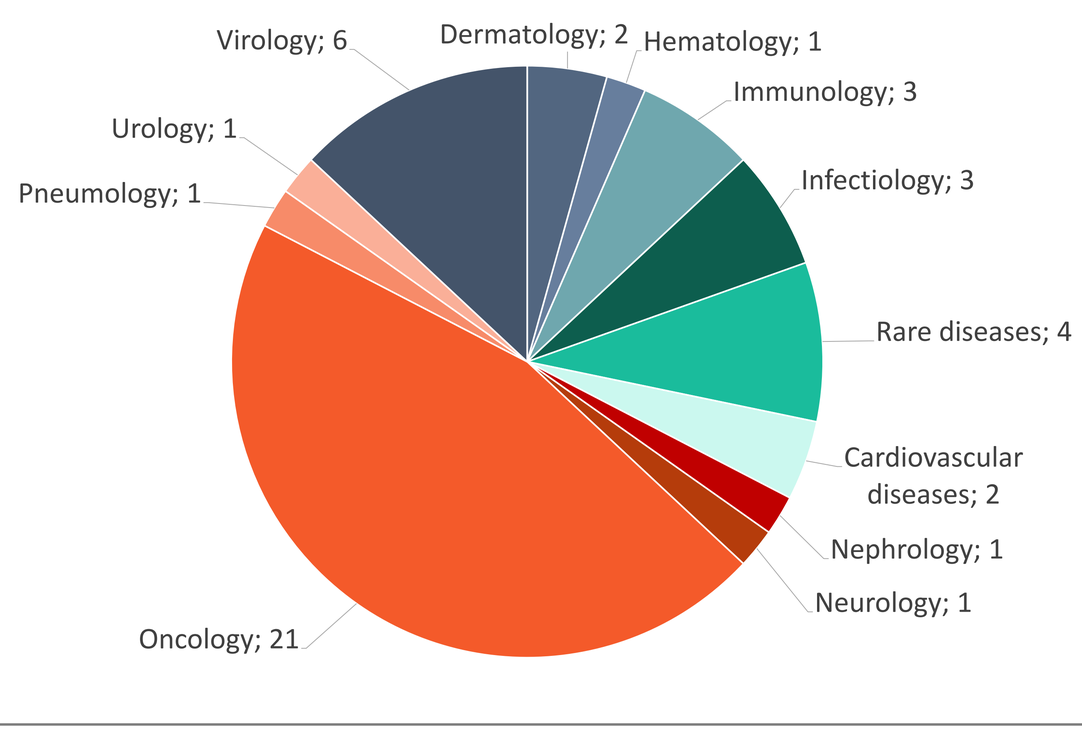
METHODS

We conducted a retrospective analysis of all HAS decisions published between July 1, 2021, and Avril 21, 2022.

RESULTS

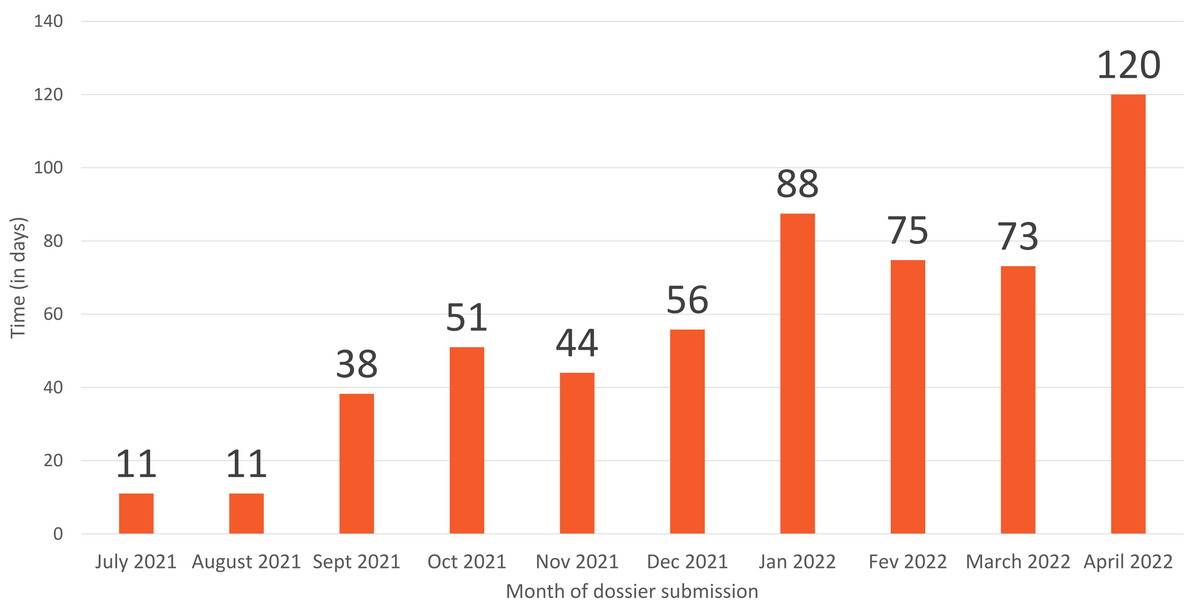
- Among 46 EAP decisions published, 37% concerned medicines without marketing authorization (MA) and 63% concerned medicines with a MA.
- The most represented therapeutic area was oncology (46%) and 17% decisions were related to COVID-19 therapies.

FIGURE 1. THERAPEUTICS AREAS OF PRODUCTS EVALUATED FOR EAP



- The average time from dossier submission to EAP decision publication was 69 days, with an increasing trend in 2022 compared to 2021 (85 vs 43 days).

FIGURE 2. AVERAGE TIME (IN DAYS) BETWEEN DOSSIER SUBMISSION AND PUBLICATION OF EAP DECISION



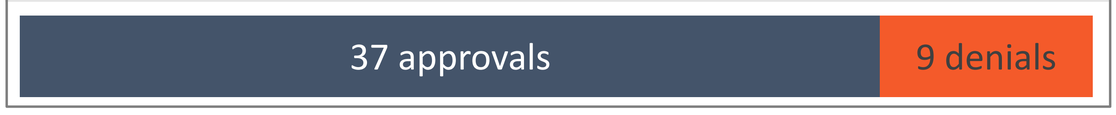
- Most evaluated medicines (67%) had comparative phase III results at the time of their evaluation.

TABLE 1. TYPES OF DATA AVAILABLE FOR THE EAP ASSESSMENT

Non-comparative study	**18%**
Phase I/II	4%
Phase II	11%
Phase III	2%
Comparative study	**78%**
Phase I	4%
Phase I/II	2%
Phase II/III	2%
Phase III	67%
Phase IIIb	2%
Other	**4%**
Data from the literature	2%
Early access data, literature review, well established use	2%

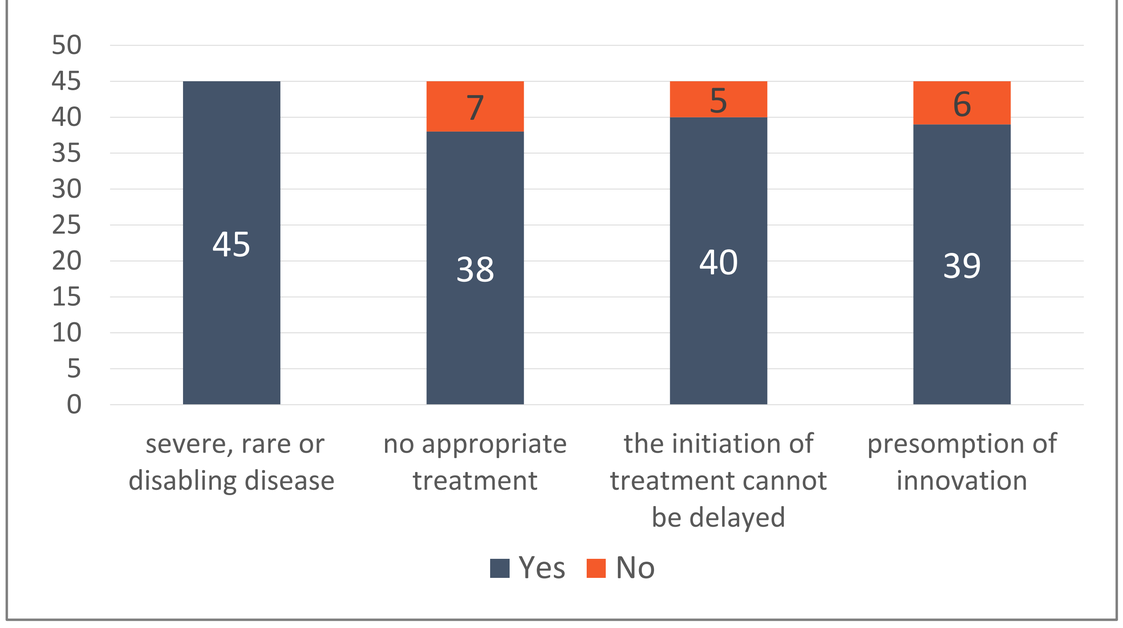
- EAP was granted for 37/46 (79%) of treatments.

FIGURE 3. EAP DECISION



- The severe, rare or disabling nature of the disease was recognized for all the EAP evaluations. The identification of appropriate comparators, the lack of a presumption of innovation, and the possibility of deferring treatment were responsible of denial for 16%, 13% and 11% of the EAP assessments, respectively.

FIGURE 4. ELIGIBILITY TO EAP CRITERIA



CONCLUSION

Since the reform, evaluation for EAP is conducted according to TC requirements and became a very important preparatory step for reimbursement. Pharmaceutical companies must ensure consistency between EAP and reimbursement dossiers and think about their market access strategy at a much earlier stage.

COI : Julie LE MAO and Prunelle GAUGY are employees at CEMKA, one of the first French consulting firms in the field of evaluation of products, programs and organizations in Health.
The study was not sponsored.