

# EVALUATION OF THE EARLY ACCESS PROGRAM EVOLUTION: A THREE-YEAR RETROSPECTIVE ANALYSIS

Le Mao Julie<sup>1</sup>, Gaugy Prunelle<sup>1</sup>, Tristan Theuillon<sup>1</sup>

<sup>1</sup> – CEMKA, 92 340 Bourg la Reine, France

CEMKA

Acceptance Code : HPR198

## CONTEXT

Early access program (EAP) has been a success since its implementation. Three years have passed since the introduction of the new EAP. Since the reform, data for two complete years are available: 2022 and 2023, allowing for a time-based comparison.

## OBJECTIVES

The aim of our study was to analyze the evolution of the EAP over the years and to focus on one drug of particular interest.

## METHODS

We conducted a retrospective analysis of all HAS decisions published between July 1, 2021 and June 14, 2024, by years. Our analysis concerns only first decisions and not renewal.

## RESULTS

- During the period covered by our analysis, 164 initial EAP decisions were published.
- The number of EAP decisions published by the HAS in 2023 was almost divided by two compared to 2022, 45 versus 82. This decrease could be explained by the smaller number of drugs in compassionate access (CA) or in nominative temporary utilization authorization (ATUn) that needed evaluation in 2023 compared to 2022, 27% versus 44%.
- The average time between submission of the application and publication of the EAP decision remained relatively stable between 2022 and 2023 (88 days and 96 days, respectively) but increased significantly in the first half of 2024 (120 days) (Figure 1).
- Efficacy and safety have not been demonstrated for 6 drugs without MA in the 73 AP1 appraisal.
- The severe, rare or disabling nature of the disease was recognized for almost all EAP decisions. There has been a significant drop in the percentage of products that meet the other 3 early access criteria, and the most important drop was for the presumption of innovation criteria from 87% in 2022 to 42% in 2024.

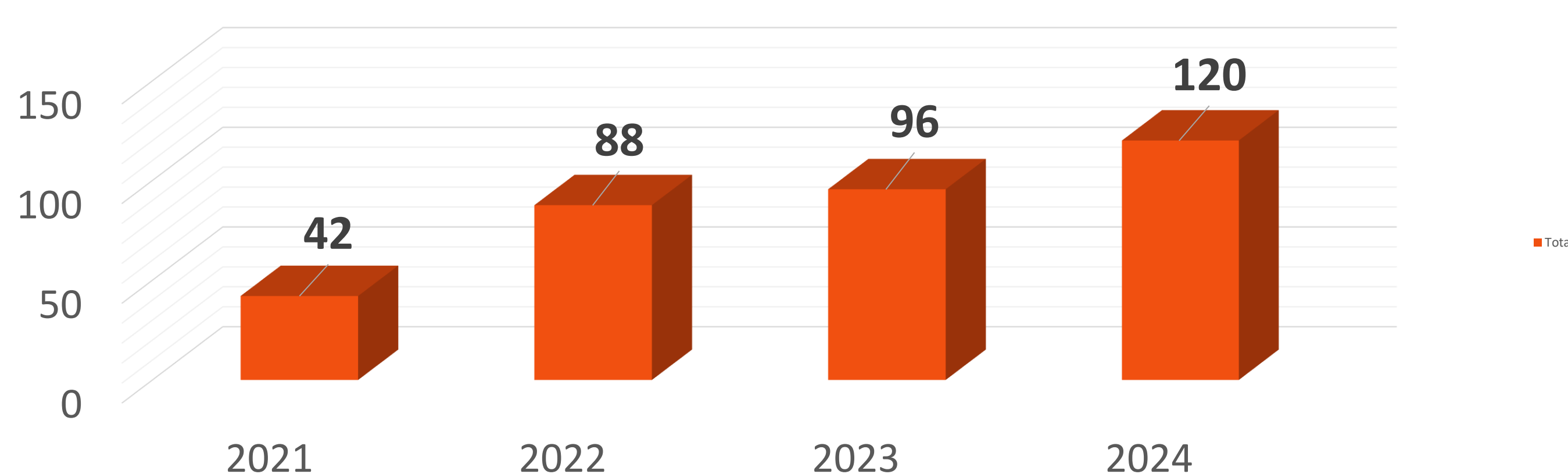


FIGURE 1. AVERAGE TIME (IN DAYS) BETWEEN DOSSIER SUBMISSION AND PUBLICATION OF EAP DECISION BY YEARS

- Over the years, oncology has remained the most represented therapeutic area.
- The number of unfavorable decisions significantly increased from 18% in 2022 to 38% in 2023, a trend that continued in 2024 with 60% of unfavorable decisions (Figure 2).

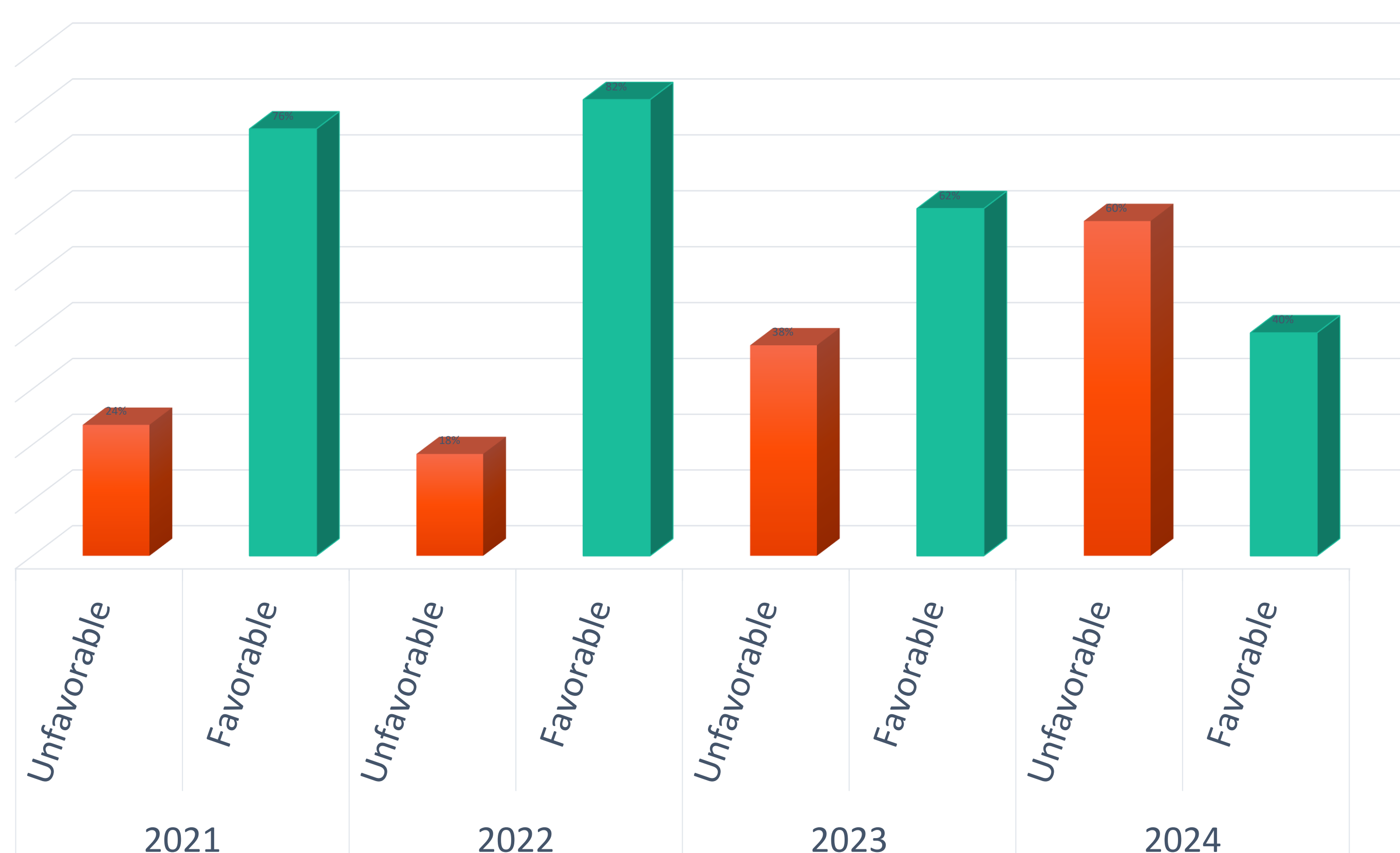


FIGURE 2. EAP DECISION

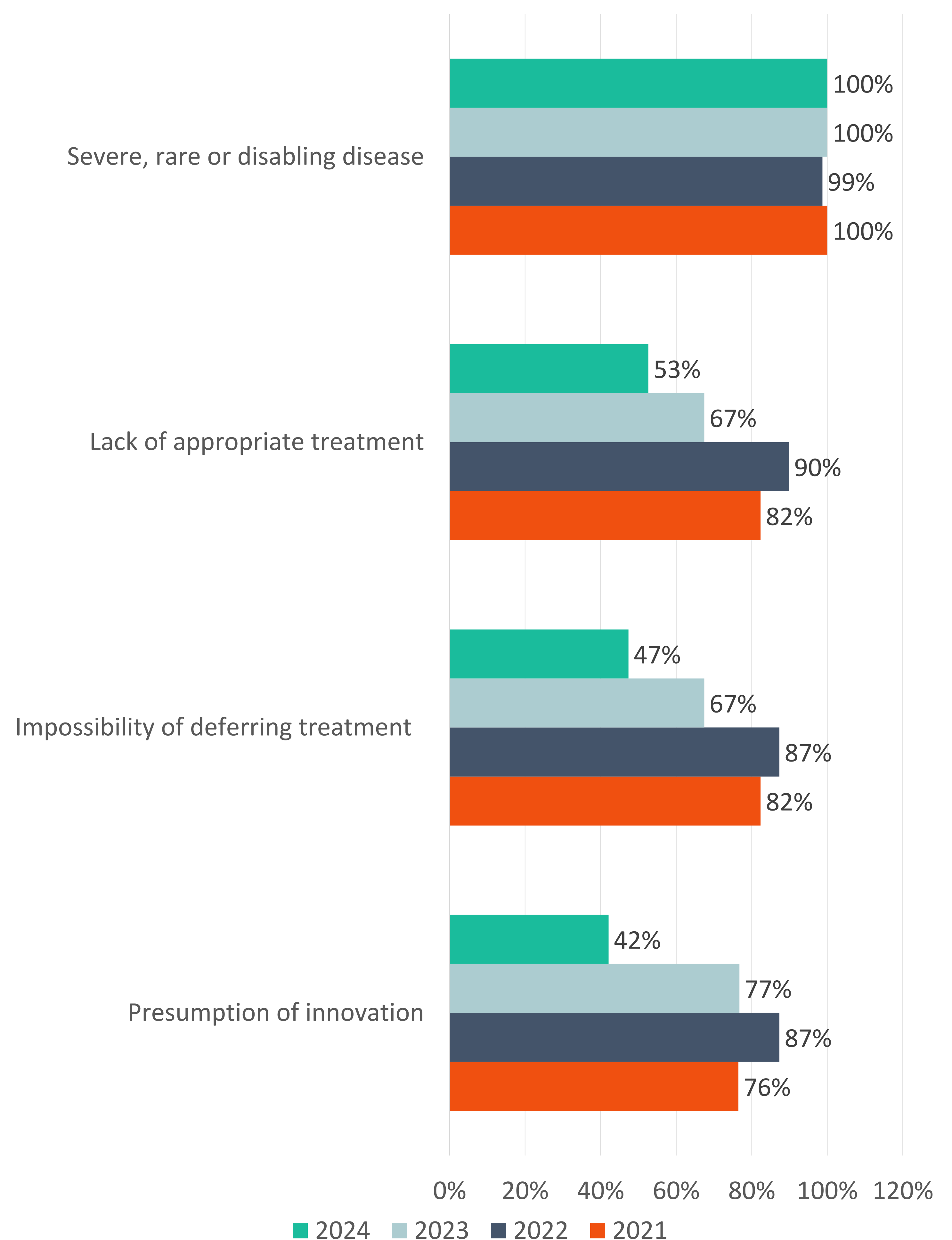


FIGURE 3. PERCENTAGE OF EARLY ACCESS CRITERIA MET PER YEAR

## CONCLUSION

After a 3-year follow-up of published EAP decisions, despite an increased number of refusals, obtaining an EAP remains a positive indicator of innovation for the Transparency Committee, with around 75% of products granting a clinical added value (ASMR) III or IV.

