

DISTRIBUTION OF THE POSITIVE ADDED MEDICAL BENEFIT ACCORDING TO THE EARLY ACCESS STATUS AND THE POTENTIAL PUBLIC HEALTH INTEREST – ANALYSIS OF THE FRENCH HTA BODY OPINIONS (HAS TRANSPARENCY COMMITTEE)

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INTRODUCTION

In France, the Temporary **Authorization for Use (Early access program, EAP)** allows patients to be treated by drugs that may not have received a marketing authorization. These EAP are provided in the occurrence of unmet medical needs for serious or orphan diseases in the absence of alternative treatments. Among the assessed criteria, the innovative nature, particularly compared to any clinically relevant comparator is evaluated.

Then, for each indication of a drug that has a positive reimbursement decision (sufficient clinical benefit), the Transparency Committee (TC) of the HAS gives an opinion on the «**Added Medical benefit (ASMR) also called «clinical added value»**». The ASMR measures the drug's added clinical value compared to existing therapies already reimbursed and regarding the existing medical need. This criteria

is used to determine the price. Similarly, for each indication of a medicine, the TC gives an assessment of its **Public health interest (ISP)** which is part of the criteria for assessing the clinical benefit called «SMR», which impacts the reimbursement rate by the French health insurance system.

The Criteria for assessing the Public health interest (ISP):

- Medical need, seriousness of the disease and prevalence of the target population
- Potential impact on the health of the population (morbidity and/or mortality) compared to the therapeutic alternatives
- Impact on the organization of care or improvement of the care pathway and/or life course for the patient or his/her family

OBJECTIVES

The aim of this research was to compare the distribution of the positive added medical benefit levels (ASMR I-IV) issued by the HAS Transparency Committee depending on the early access status (EAP or not) and the potential public health interest (ISP or not). More specifically, the objective was to ascertain:

- Whether the innovative status of the product, as evidenced by the granting of an EAP, was subsequently reflected in the assessment of the ASMR level
- Whether or not obtaining an ISP at the time of the SMR evaluation influences the evaluation of the ASMR level

METHODS

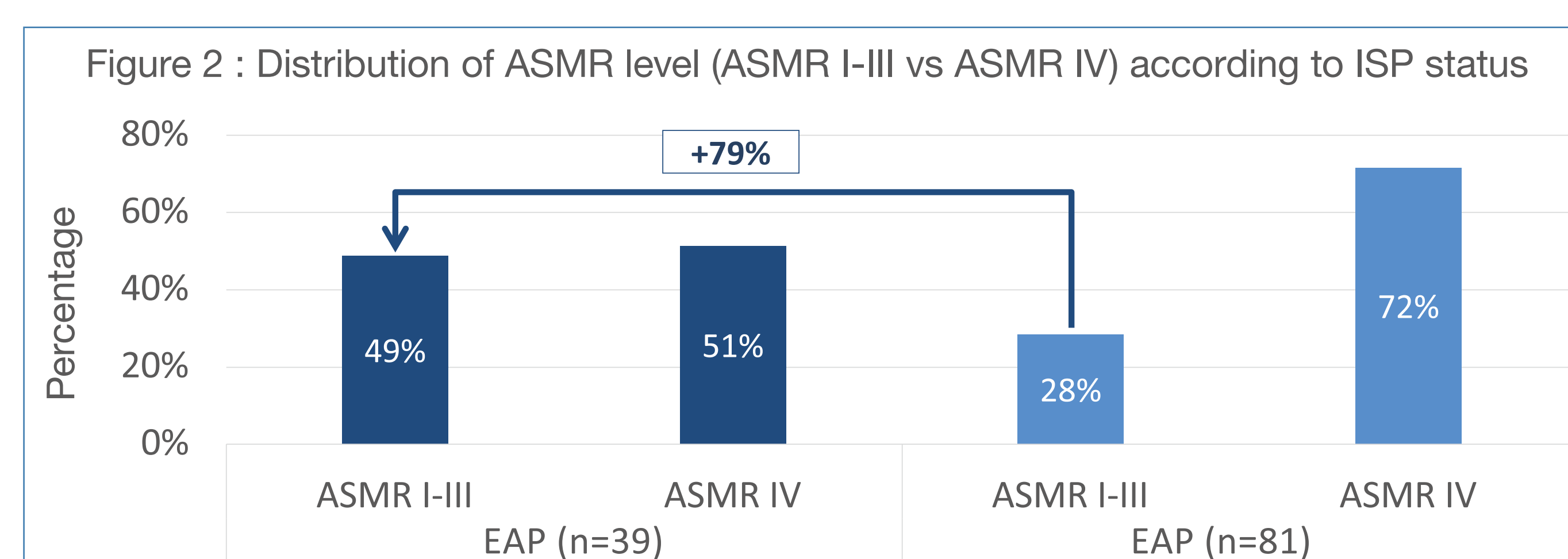
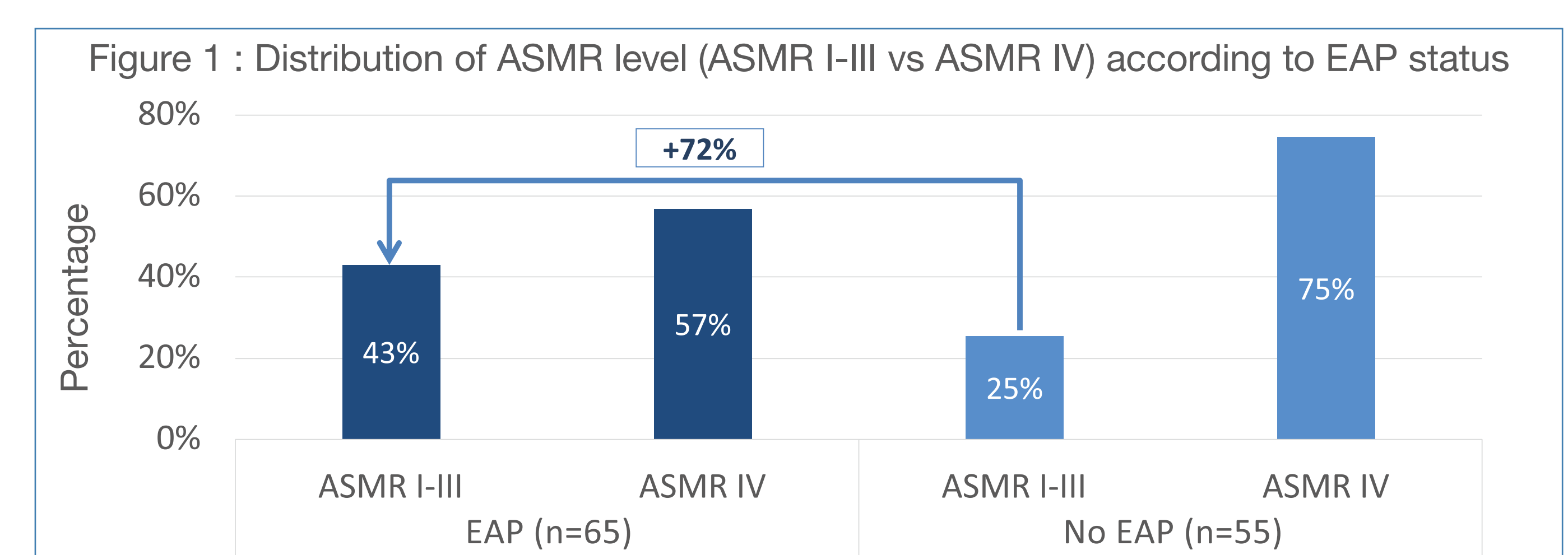
All the TC opinions concerning a first inscription for reimbursement adopted between January 1st, 2016, and December 31st, 2021, were extracted. Opinions with insufficient clinical benefit (insufficient

SMR) and no clinical added value (ASMR V) have been excluded of the analysis and opinions counting for several presentations of the specialties were counted only once.

RESULTS

120 TC opinions met the inclusion criteria, 65 (54%) had an EAP, 39 (33%) had an ISP and 23 (19%) had both an EAP and ISP.

Among the drugs whose have benefited of an EAP (65 TC opinions, 54%), the proportion whose obtained an ASMR I-III was lower than those who obtained an ASMR IV (respectively 28 TC opinions (43%) versus 37 (57%)). Among the drugs that have no EAP (55 TC opinions, 46%), the proportion that obtained an ASMR I-III was lower than those that obtained an ASMR IV (respectively 14 TC opinions (25%) versus 41 (75%)). Therefore, the number of TC opinions granting an ASMR I-III increase by 72% (25% versus 43%) when the medicine obtained an EAP. (Figure 1).



Among the medicines with an ISP, which represents a small proportion of the sample (only 39 TC opinions, 33%), the proportion that obtained an ASMR I-III was similar of those that obtained an ASMR IV (20 TC opinions (50%) for both). Among the drugs that have no ISP (81 TC opinions, 68%), the proportion that obtained an ASMR I-III was lower than those that obtained an ASMR IV (respectively 23 TC opinions (28%) versus 58 (72%)). Thus, the number of TC opinions granting an ASMR I-III increase by 79% (25% versus 43%) when an ISP has been recognized by the TC. (Figure 2)

CONCLUSION

Among several criteria, the high unmet medical need (absence of an alternative) which is considered in EAP process, ISP assessment and ASMR assessment could partly explain these results. The recognition of the innovative nature and the response to the unmet

medical need assessed during the EAP evaluation, is subsequently confirmed during the evaluation by the TC, which granted more ASMR I to III (+72%) in this sample. Similarly, it is the same with the recognition of an ISP (+79% of ASMR I-III).