

COMPARATIVE ANALYSIS OF THE TRANSPARENCY COMMITTEE OPINIONS CONCERNING THE ADDED MEDICAL BENEFIT OBTAINED BY DRUGS AVAILABLE THROUGH THE EARLY ACCESS PROGRAM VS OTHER DRUGS IN FRANCE

Benazet F¹, Le Tohic D¹, Gurnot S¹, Andriany T¹, Berard I¹
(1.Nextep and MEDVANCE France, Paris, 75, France)

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INTRODUCTION

For each indication of a drug that has a positive reimbursement decision (sufficient medical benefit), the Transparency Committee (TC) of the HAS gives an opinion on the “Added Medical benefit” (ASMR) also called “actual clinical benefit”. The added medical benefit measures the drug’s added clinical value compared to existing therapies already reimbursed and is used to determine the price. In France, the Temporary Authorization for Use (ATU) (early access process) allows patients to be treated by drugs that may not have received a marketing authorization. These ATUs are provided in the occurrence of unmet medical needs for serious or orphan diseases in the absence of alternative treatments. During an ATU period, some data may be recorded and may impact the evaluation conducted by the HAS.

OBJECTIVES

The aim of this research was to compare the distribution of the added medical benefit levels issued by the HAS Transparency Committee depending on the early access status (Temporary Use Authorization (ATU) or not).

METHODS

All the TC opinions concerning a first reimbursement inscription adopted between January 2016 and April 17th, 2019 were analyzed. Simplified procedures and new applications following a previous application withdrawal, or a previous negative opinion were excluded.

RESULTS

In the selected time period, 188 TC opinions met the inclusion criteria which corresponded to 185 drugs: 64 medicines available through ATU were evaluated for their “Added Medical benefit” (73 ASMR assessed, one per indication) vs 98 medicines not available in early access (100 ASMR assessed). Twenty-three drugs do not have an ASMR evaluation because of a negative reimbursement decision (insufficient medical benefit).

In general, the added medical benefit measures the clinical added value of a new drug compared to existing strategies:

- By giving an ASMR I to III: the committee judges that the medicine is an innovation: it could benefit of a faster access and facial price will be consistent with the European price corridor including the UK, Germany, Italy and Spain
- By giving an ASMR IV: the new drug brings a minor improvement and could have an equal or in some rare cases, higher price than its most relevant comparators.
- By giving an ASMR V: the committee judges that there are no improvements of the clinical value compared to other alternatives. The drug can be listed only if its cost is inferior to the comparators: it has a lower price or induces cost savings.

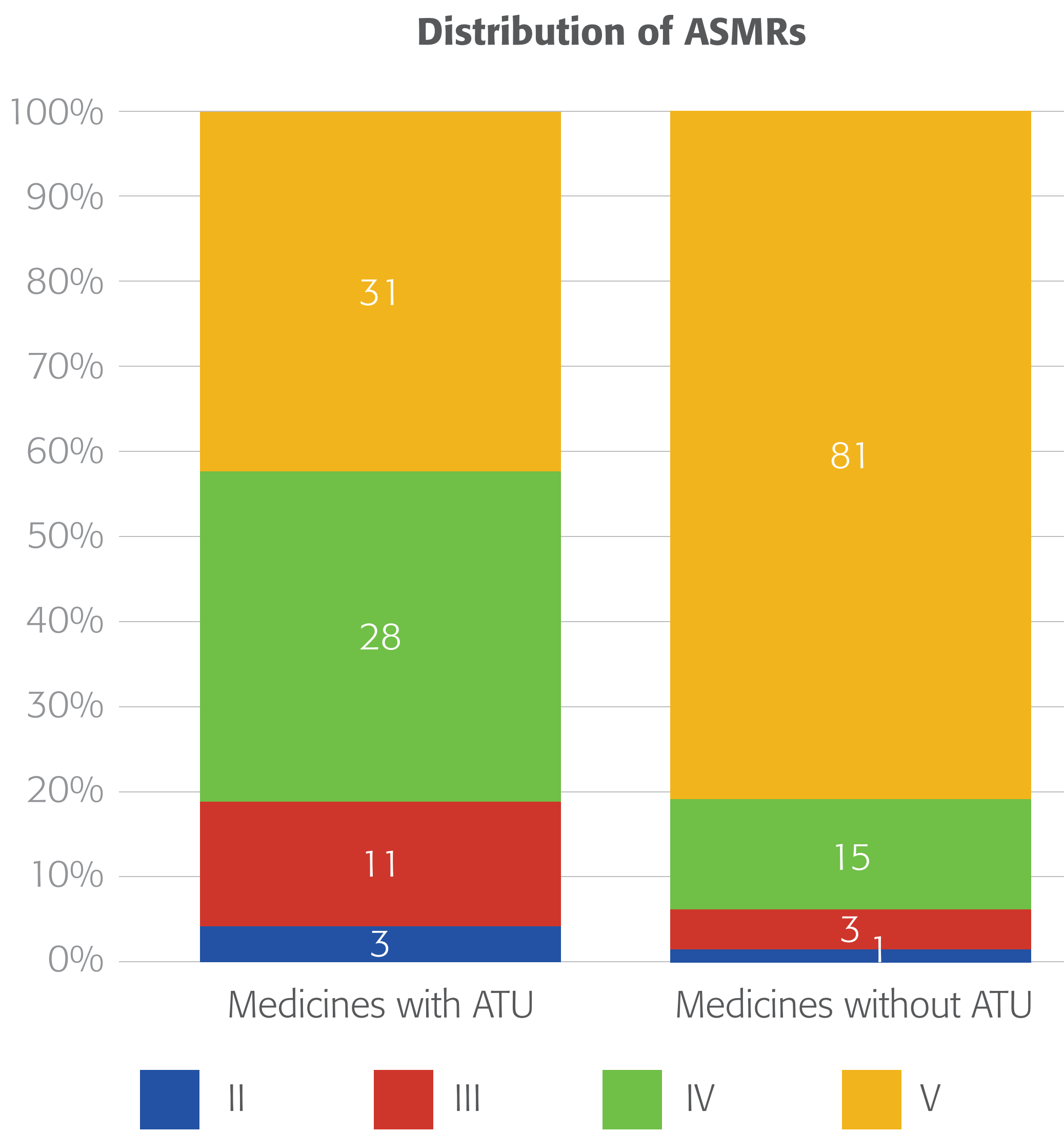
Fourteen (22%) drugs available through ATUs were granted with an ASMR considered as innovative (ASMR I-III) vs 4 without an early access.

ASMR ratings were better for drugs previously in an early access program compared those that weren’t :

- **3 important ASMR II for drugs with an ATU vs 1 for drugs without an ATU**
- **11 moderate ASMR III for drugs with an ATU vs 3 for drugs without an ATU**

For these evaluations, the most common arguments in favor of an innovative ASMR were:

- Unmet medical need and severe pathology (B type hemophilia, cecity, refractory lymphoma, spinal amyotrophy...)
- Quality of the clinical demonstration and comparative superiority to standards of care or placebo (when no alternative)
- Relevant clinical benefits (complete remission, reduction of disability...) and sometimes improvement of the quality of life



In total, **28 ASMR IV (38 %) and 31 ASMR V (42 %) were octroyed for ATU drugs vs 15 ASMR IV (15 %) and 81 ASMR V (81 %) for drugs without an ATU**. Twenty-two (34 %) drugs available in early access received a unique minor ASMR IV vs 15 (15 %) in the group without an early access. Twenty-one (33 %) drugs received no added medical benefit (ASMR V) in the group of medicines with an ATU vs 79 (80.6 %) in the group without early access. Eleven drugs were granted with two ASMRs: 9 in the ATU group vs 2 in the group without an ATU.

CONCLUSION

Drugs which benefited from an early access program were more likely to obtain an ASMR considered as innovative and a better “rating” for added medical benefit than drugs which did not benefit from an early access.