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

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INTRODUCTION

For each indication of a medicine, the Transparency Committee (HAS) gives an opinion on its "Medical benefit" (SMR). This opinion determines whether the medicine is reimbursed (three ratings: important, moderate, low) or not (insufficient medical benefit) and the rate of its reimbursement by the French national health insurance (important: 65%; moderate: 30%; low: 15%). 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.

The aim of this research was to compare the distribution of the medical benefit levels (SMR) issued by the HAS Transparency Committee (TC) depending on the early access status (ATU or not) in France.

OBJECTIVES

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

METHODS

In the selected time period, 188 TC opinions met the inclusion criteria, concerning 185 drugs. Ninety-seven percent (64/67) of the medicines available in the early access program had a favorable reimbursement decision with sufficient medical benefits compared to 83% (98/118) of the medicines not available through the early access program with sufficient medical benefits.

RESULTS

In the early access group, only 3 drugs had a negative reimbursement decision (Figure 1) for all of their indications (vs 20 drugs without an ATU – Figure 2):
- Mepsevii® because its clinical benefit wasn’t established (biologic criteria used in the clinical trial not linked with clinical benefit),
- Raxone® because the superiority on the primary criteria endpoint vs placebo wasn’t demonstrated,
- Uptravi® in 2016 because its efficacy/safety data (primary criteria endpoint was a composite endpoint with no impact demonstrated on mortality) and transposability.

Among the 67 medicines available through the ATU program, 83 “Medical benefits” (SMR) were evaluated vs 134 for the 118 medicines without an ATU (64/67) of the medicines available in the early access program had a favorable reimbursement decision in proportion, medicines available through early access programs had a better rating for medical benefits than medicines without an early access (Figure 3):
- Important: 62/83 (78%) for drugs with an ATU vs 82/134 (61%) for drugs without an ATU
- Moderate: 4/83 (5%) for drugs with an ATU vs 7/134 (5%) for drugs without an ATU
- Low: 6/83 (5%) for drugs with an ATU vs 12/134 (5%) for drugs without an ATU
- Insufficient: 11/83 (14%) for drugs with an ATU vs 33/134 for drugs without an ATU (25%). Some drugs have multiple indications and can have an important medical benefit for one indication and an insufficient one for another which explains the difference between the number of drugs not reimbursed and the number of insufficient medical benefit.

CONCLUSION

Medicines which benefited from an early access program were more likely to obtain a favorable reimbursement decision and a better "rating" for medical benefits than the products which were not available through early access. A high unmet medical need (absence of an alternative) which is a common criterion to obtain an early temporary authorization and a sufficient medical benefit, could explained the difference observed between the two groups. This conclusion is also consistent with the fact that drugs without a Marketing Authorization need to have a presumption of relevant clinical efficacy in order to obtain a temporary authorization for use (ATU).