COMPARATIVE ANALYSIS OF THE TRANSPARENCY COMMITTEE OPINIONS CONCERNING THE ADDED MEDICAL BENEFIT LEVELS DEPENDING ON THE THERAPEUTIC AREAS: DISTRIBUTION OF INNOVATION AND TREND ASSESSMENT FOR 2016-2021

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INTRODUCTION

In France, 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 «clinical added value». The added medical benefit measures the drug's added clinical value compared to therapies already reimbursed, with regards to the medical need in the target indication. This assessment is a snapshot at a given point in time within an environment that may evolve. It may be rated major (ASMR level I), substantial (ASMR level II), moderate (ASMR level III), minor (ASMR level IV) or without improvement (ASMR level V), with the latter level corresponding to an absence of therapeutic progress. The ASMR criteria is then used to define the framework for price negotiation.

For example, the ASMR I assessment corresponds to therapeutic breakthrough situations (that saves or changes the lives of patients with a serious disease) for which all the ASMR determinants are judged to be satisfactory by the TC.

OBJECTIVES

The aim of this research was to compare the distribution, over the last 6 years, of the ASMR (added medical benefit) levels issued by the HAS Transparency Committee depending on the therapeutic area.

METHODS

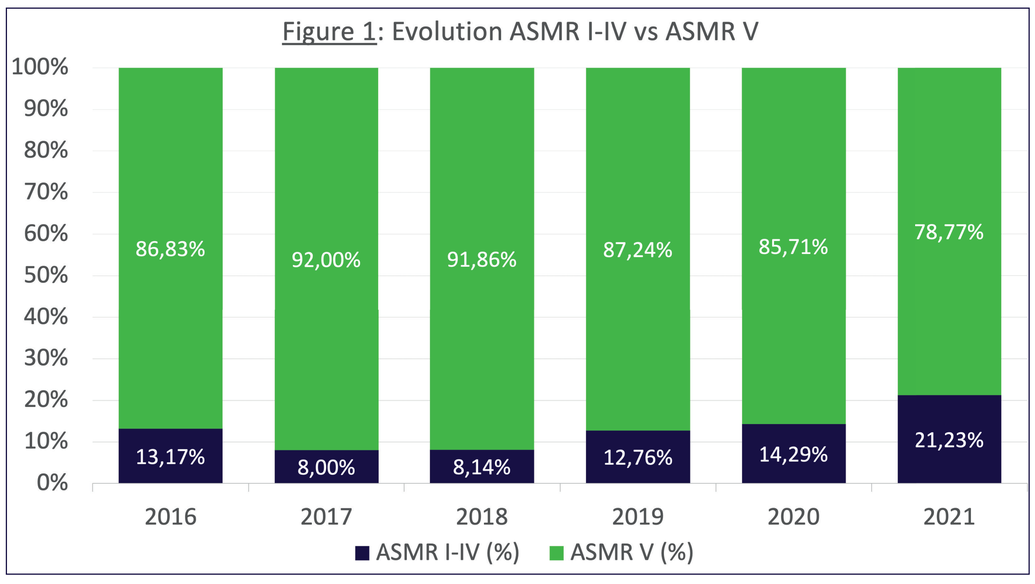
All the TC opinions concerning a first listing for reimbursement adopted between January 1st, 2016, and December 31st, 2021, were analyzed. Only primary registrations have been included, and when the TC's assessment for the medical benefit (level of reimbursement) were important (maximal level).

The data were extracted from an interministerial database called 'Data.gouv', then selected so that evaluations of the same drug with several dosages were counted only as a single first listing. The criterion for judging the innovation was the ASMR.

RESULTS

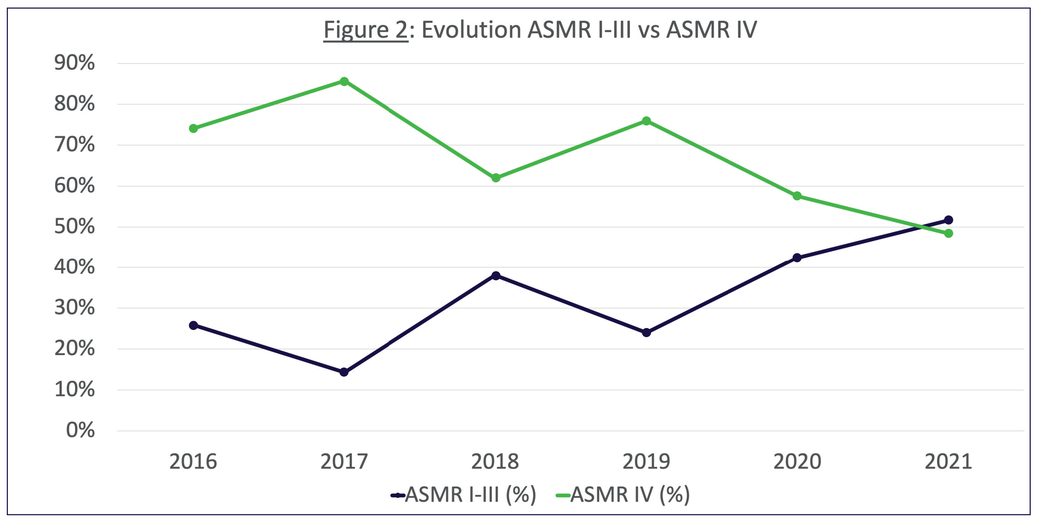
1,211 TC opinion were evaluated for the ASMR ("Added Medical Benefit") between 2016 and 2021.

Figure 1: Evolution ASMR I-IV vs ASMR V



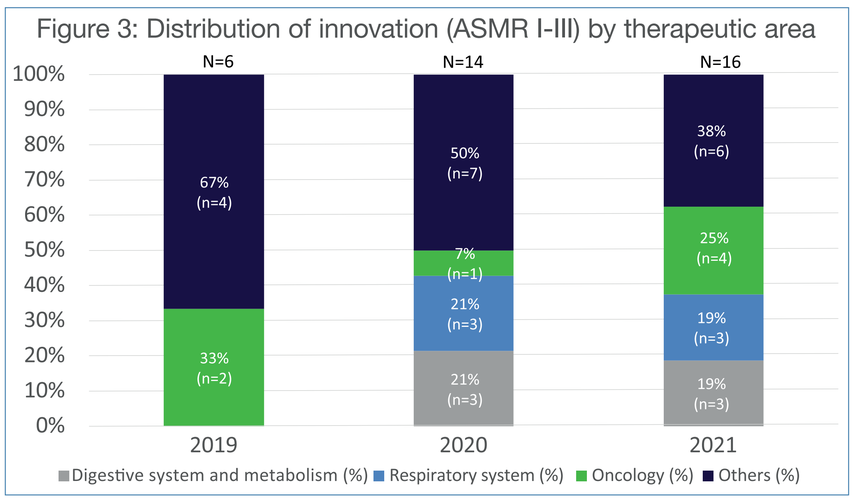
There has been a decrease of ASMR V in favor of ASMR I-IV (13% in 2016 vs 21% in 2021, for ASMR I-IV). This trend has been particularly marked since 2018, when the ASMR I-IV rate drop to 8%.

Figure 2: Evolution ASMR I-III vs ASMR IV



More specifically, when analyzing the number of ASMR I-IV, there has been an increase in ASMR I-III compared to ASMR IV since 2016 until a crossover in 2021 with a higher proportion of ASMR I-III than ASMR IV (ASMR IV: 74% in 2016 vs 48% in 2021; 52% of ASMR I-III in 2021). This ascending phase in terms of the proportion of ASMR I-III is particularly observable over the last 3 years (since 2019).

Figure 3: Distribution of innovation (ASMR I-III) by therapeutic area



By analyzing the last 3 years (2019 to 2021), we see that for oncology, which is the first therapeutics area, the proportion of ASMR I-III, which represents the assessment for the most innovative drugs, shows a decrease in the share of assessment since 2019 (33% vs 25%). In opposition, for the last 2 years, there has been a better distribution of innovation in terms of share, with 2 increased therapeutic areas -- digestive and respiratory systems-- with respectively around 20% of ASMR I-III assessment. This phenomenon is illustrated for example by the launch in 2020 of KAFTRIO® in the respiratory and GIVLAARI® in the digestive fields, both having received an ASMR II.

In addition, it should be noted that no drug in oncology has received an ASMR I or II in the last three years. Moreover, among the HAS opinions dealing with ASMR I-III in 2020 and 2021, only 20% of drugs in oncology had the status of an orphan drug. Contrariwise, 68% of all other drugs assessed over the same period and having granted with the same level of innovation received this status. Among the digestive and respiratory drugs that received an ASMR I-III in 2020 and 2021, the orphan status designation rate even rises to 83%.

DISCUSSION

In light of our analysis, the Regulation (EC) No 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products, aimed at facilitating market access for such medicinal products, may have provided incentives for pharmaceutical companies in the R&D promotion, the development and the commercialization of drug medicinal products treating orphan diseases. Among the relevant incentives we may note the credits granted for the production of orphan medicinal products, a support in the development of clinical trial protocols, and a ten-year commercial exclusivity.

CONCLUSION

There is a trend towards greater recognition of innovation in France, particularly in recent years, and a greater distribution across different therapeutic areas, especially since 2020. The growing innovation dynamic has focused mainly on rare diseases in recent years, notably enabled by a beneficial regulatory, scientific, and economic environment for the structures developing these therapies.