Round table no. 5:

THE DEFINITION OF TARGET POPULATIONS

"HOW BEST TO ANTICIPATE THEIR DEFINITION FOR THE PURPOSES OF REIMBURSEMENT"

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The qualitative and quantitative definition of target populations for the purposes of reimbursement by the health insurance agencies is closely linked to important public health and economic issues for the various stakeholders – the pharmaceutical industry, payers, decision-makers, assessment agencies, patients. Many of those concerned expressed dissatisfaction both with the quality of target population estimates or the data used in reaching those estimates, and with the lack of transparency on how this information is used at various levels of the reimbursement decision-making process.

During the round table, much of the debate centred on the very definition of the term "target population". The term was found to encompass different populations, some of them overlapping, defined by the different actors at different moments in the process from the development of a drug to its marketing and reimbursement, and used by them for different purposes. Our group also stressed that the target population for a product was not fixed once and for all, but might evolve over time depending on factors such as advances in diagnostic resources, treatment strategies available and recommendations. A review of Transparency Commission (CT) opinions issued in 2009 shows that in 20% of cases it was not possible to determine precisely the target population for which the CT judged reimbursement justified, and that in a third of cases expert advice had to be called in to estimate the target population. Furthermore, in over 85% of cases the population defined by the marketing approval (AMM) for the drug concerned corresponded to the population for which the CT considered the medical benefit (SMR) sufficient to warrant reimbursement, and also corresponding to the population for which the CT had qualified the level of improvement in medical benefit (ASMR). It also emerged that in the vast majority of cases the "target population" for a drug was not re-evaluated by the CT when considering the application for renewal of the drug's reimbursement status.

We concluded that population estimates could be improved and proposals were made aimed at improving qualitative and

quantitative estimates of populations which have a significant impact on discussions concerning reimbursement. The group made the following proposals:

- clarify the debate and in particular abandon reference to a target population but instead define the particular population in which we are interested (e.g. population for the indication, population eligible for reimbursement, population deriving particular benefit);
- improve the dialogue between stakeholders for a better sharing of objectives, expectations and elements required for decision-making;
- produce more epidemiological details useful in estimating populations of interest, and sharing the data more effectively (e.g. support for the measures of the health industries strategic council (CSIS), a common portal for epidemiological resources, promoting research in this field through the research tax credit scheme, promoting methodological research and the use of databases maintained by the health insurance fund for salaried employees (CnamTS);
- improve estimating methods and in particular formalise the process for seeking expert opinion where necessary;
- anticipate by concentrating on situations requiring a particular action plan for evaluation of the transparency submission (e.g. drugs added to an existing therapeutic strategy when frequency (incidence or prevalence) data are lacking, when the epidemiology varies rapidly or when the risk-benefit is heterogeneous in sub-populations for the indication).