Early Access to Medicines Marketing Authorisation Flexibilities Should Only Respond to Unmet Medical Needs, Must Protect Patient Safety

Health Action International (HAI), the International Society of Drug Bulletins (ISDB) and Medicines in Europe Forum (MiEF) are pleased to contribute to the EMA public consultation on the Guideline on the scientific application and the practical arrangements necessary to implement the procedure for accelerated assessment pursuant to article 14(9) of regulation (EC) No 726/2004 and the Guideline on the scientific application and the practical arrangements necessary to implement Commission Regulation (EC) No 507/2006 on the conditional marketing authorisation for medicinal products for human use falling within the scope of Regulation (EC) No 726/2004.

1. The pharmaceutical marketing authorisation procedure is a health protection measure.
EU pharmaceutical legislation provides that, as a general rule, before a medicine is authorised it has to undergo "extensive studies to ensure that it is safe, of high quality and effective for use in the target population". The importance of maintaining the requirement for solid evidence about benefits and harms before a medicine is approved as the corner stone of pharmaceutical regulation must be emphasised. Pre-market efficacy evidence is an important health protection measure as it protects patients from a potentially harmful exposure to medicines without solid scientific evidence of a benefit to health. The current marketing authorisation procedure emerged as a response to a series of drug-induced disasters and has been in place for more than 50 years. However, several attempts have been made to expand the use of premature and accelerated approvals for new drugs, particularly over the last decade.

The paucity of new medicines that offer even a modest therapeutic advantage stands in stark contrast to the large number of new products that expose patients to unjustified risks. The majority of new medicines are "me-too" drugs and not "innovative" since they do not demonstrate any added therapeutic value. A recent study found that 66% of phase III trials conducted between 2007 and 2010 were terminated for lack of efficacy. This is a failure that adaptive licensing won't address.

Rather than lowering the requirements for marketing authorisation, the EMA should favour the demonstration of a new drug's therapeutic advance when compared to the best available therapeutic option. This would act as an incentive to reorient research and development towards unmet health needs and true therapeutic progress. It is regulation for innovation.

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