STATEMENT

IFPMA’s comment on off-label use in the context of the WHO’s Model List of Essential Medicines

07 APRIL 2023, GENEVA – IFPMA recognizes the importance of the WHO EML in the global health context. The increasing number of products placed on the EML demonstrates the critical role played by the biopharmaceutical industry in creating therapies that are safe, efficacious and essential in reaching the UN Sustainable Development Goals, chief amongst them achieving universal health coverage.

However, we would like to express our concern with what we consider to be a misuse of the WHO’s Square Box Symbol (SqB). While IFPMA supports the revised methodology for a clearer and consistent use of the SqB, extra caution is needed when using this tool to list medicines for off label use. In particular, the use of products that have been reviewed and approved by regulatory authorities for a certain indication should be prioritized over the use of off-label products for the same indication. Whilst marketing authorization holders do not recommend off-label use when a regulatory approved treatment for that same indication exists, if a treatment is listed on the EML for an off-label indication, this caveat should be clearly indicated in the EML to enable countries, patients and healthcare professionals to make an informed decision about their use.

IFPMA strongly believes that regulatory approval(s) for a given indication is the best indicator (and a requirement at national level) that a medicine has enough data on quality, efficacy and safety to support its clinical use. For innovative medicines in particular, data submitted to national regulatory authorities for the purpose of granting marketing authorization and life cycle management is often the most detailed source of information regarding these products’ safety and efficacy. By listing medicines that have not been approved by a regulatory authority for a given indication, decision-makers may be encouraged to choose medicines that may not be adapted to local healthcare systems’ capabilities or that may not be supported by adequate regulatory oversight (e.g. insufficient establishment of safety and effectiveness, adverse events management and traceability…). This practice could lead to negative outcomes for patients and health systems in general.

The square box system must be used adequately to ensure patients receive the most appropriate and effective medicines wherever they live. We urge the WHO Expert Committee to prevent any precedent-setting whereby the square box system in the WHO EML is misused and inconsistent with the organization’s values, principles and guidelines, which ultimately bears the risk of undermining
the WHO’s efforts to promote global access to quality care and reduce health inequalities. Patients worldwide rely on the WHO EML, and it is imperative that these lists continue to be based on science and the highest possible level of evidence to remain trustworthy and credible.

About IFPMA

The International Federation of Pharmaceutical Manufacturers and Associations (IFPMA) represents over 90 innovative pharmaceutical companies and associations across the globe. Based in Geneva, IFPMA has official relations with the United Nations and contributes industry expertise to help the global health community improve the lives of people everywhere. The industry’s two million employees discover, develop, and deliver medicines and vaccines that advance global health.