COUNCIL FOR INTERNATIONAL ORGANIZATIONS OF MEDICAL SCIENCES

CIOMS

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Medicines assessment during public health emergencies needs good science, best practices and proper communication

Statement[1] of Council for International Organizations of Medical Sciences (CIOMS) International Expert Working Group[2]

Following the essential principles of evidence-based medicine and regulatory decision-making remain key also in times of public health emergencies. As has been the case with the COVID-19 pandemic, such emergencies can develop rapidly, and much-needed, robust, scientific data may not be immediately available to close the knowledge gaps. Pressures to make decisions without proper evidence have the potential to overcome sound scientific judgement and lead to unjustifiable conclusions, as well as the use of unproven therapies that may be ineffective or harmful, and have a further negative impact on public health.

One of the most complex, scientific activities during public health emergencies is to determine whether a candidate medicine intended to prevent or treat the disease is effective, and establish whether its expected benefits outweigh its potential risks to patients. This assessment is based on all available evidence about the medication and the surrounding situation including: the severity of the disease; how well patients' medical needs are addressed by alternative, available therapies; the uncertainty around how data from clinical trials or testing environments extrapolate to real-life situations; and whether specific risk management measures need to be applied to mitigate known and/or potential risks. In the case of a public health emergency, such information is often not readily available in sufficient quantity or quality to adequately support evidence-based decision-making, and the urgency of the decision context magnifies the potential consequences of action or inaction.

When decision-making in the face of high uncertainty cannot be avoided, increased focus on monitoring the safety and effectiveness of such new therapies once they are approved for use in the public domain is critical. Considerations for this expanded surveillance role should include appropriate, evidence-generating or adverse reaction monitoring strategies such as: phase IV clinical trial studies; observational studies; manufacturer-run patient registries and/or patient support programmes; patient focus groups and implementing proactive adverse reaction monitoring strategies. The monitoring of "repurposed" medicines will also be necessary under the different uses made in the pandemic, since their efficacy/effectiveness remain to be confirmed and their safety profile may well be different in a different indication. In addition, the acceptability of potential harms may be different than in other indications.

The contemporary pharmaceutical development systems benefit from the collaborative efforts of multiple stakeholders including regulators, industry, academia, patients, health-care providers and health insurers, all of whom contribute to increasing knowledge about benefit/risk relationships and the consideration of the uncertainties. When facing a public health crisis, we urge all concerned parties to maintain solid, scientific, and evidence-based principles and best practices for conducting the proper benefit/risk assessment of potential new prevention or therapy options. Among others, potential confounders and possible bias should be considered when assessing available data. All parties should uphold full transparency of the decision-making process, with a high degree of focus on the relevance of the therapy decision for the patients being treated during the emergency.

In the midst of an emergent health crisis, stakeholders should follow best practices for communication and provide information that is timely, accurate, credible, understandable, actionable, consistent, and empathetic. Poor communication, such as a lack of information; unexplained changes in key messages; or failure to communicate uncertainties can undermine credibility and disrupt risk mitigation efforts.

Members of the various CIOMS Working Groups are working to define and advance measures and approaches to improve the development and benefit/risk assessment of new therapies and enhance public health. We wish to applaud the efforts of the health-care and scientific communities, including practitioners, regulators and patients, who have come together to fight COVID-19 and hope that the CIOMS Working Groups' outcomes can also be helpful in addressing the product-related challenges and future decision-making during public health emergencies.

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[1] Disclaimer. The views and opinions expressed in the statement above are consolidated views of the participants of the CIOMS Working Group and should not be attributed to any individual expert in those or any organization with which these individuals are employed or affiliated.

[2] CIOMS Working Group WG XII: Benefit-Risk Balance for Medicinal Products – Update of CIOMS IV. More about the Working Group and the List of its members.