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Regulatory Affairs, Quality Systems, Policy, and Ethics

STANDARD REGULATION FOR STEM CELL PRODUCTS IN INDONESIA

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Keywords: GMP , Stem Cell, Regulation.

Background & Aim: Advanced therapy medicinal products (ATMPs) are medicines for human use that are based on genes, tissues, or cells. Currently, there has been a constant increase in the number of clinical trials evaluating ATMPs, with a focus on CBMPs, including stem cell–based medicinal products for regenerative medicine. However, the scientific assessment of stem cells product is still challenging from a regulatory perspective. It is important to clearly define the manufacturing process because cells can be impacted by various factors during their isolation and processing. Regarding this, we would like to describe the validation process in manufacturing Umbilical Cord-Mesenchymal Stem Cells (UC-MSC) comply with local GMP regulation in Indonesia.

Methods, Results & Conclusion: In complying with standard regulation for stem cell manufacturing for clinical application in Indonesia, a laboratory should establish an operational permit according to Indonesia's Ministry of Health Regulation, PERMENKES No 50 tahun 2013 about Operation of Stem Cell Processing Laboratory for Clinical Application. Along with it, for public manufacturing, the laboratory should be GMP certified by the local FDA. Upon the certification process, the qualification of laboratory facilities and equipment should be completed with process validation of the manufacturing. The validation process includes the donor qualification, isolation and expansion of stem cells, cryopreservation and thawing, packaging, and delivery process. All the processes should comply with the local standard to ensure the quality of the product and the patient's safety.

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KEY ASPECTS TO CONSIDER FOR THE DEVELOPMENT OF A REGULATORY FRAMEWORK FOR THE USE OF CELL THERAPIES

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Keywords: Regenerative medicine, Regulation, Consensus.

Background & Aim: Regenerative medicine aims to reestablish cell and tissue functionality after overuse, accidents, environmental factors, aging, and by studying how our body regains its health and improves tissue recovery. Therapies based on cells and their byproducts have shown promising results in clinical trials and practice for the treatment of several diseases, including arthritis, ischemic events, cancer, wounds, and tissue damage. These therapies implement the use of cells, from one type or a mix, intact and alive, artificially generated, modified or conditioned, as well as secreted factors, vesicles, and subcellular components to treat illnesses or lesions.

Methods, Results & Conclusion: The development of successful therapies, which will fill the gap between expectations and reality, needs to follow strict scientific and medical protocols, and apply ethical standards to offer safe and beneficial therapeutic options. Many countries lack a consensus regarding their regulatory framework, and how to promote clinical research towards the application of effective therapies. The interaction between academic institutions, regulatory entities, industry, investors, and non-governmental organizations is critical to move research forward and promote the advancement of regenerative therapies. Proper clinical investigation has difficulties in moving forward due to excessive regulation. In contrast, unproven treatments flourish in an unregulated context. Basic and key aspects need to be considered to promote and regulate the development of cell therapies. It is our thought that more evidence and a consensus among researchers, medical experts and regulation representatives could be applied to develop a consistent regulatory framework that could benefit the development and application of cell therapy products.

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A COMPENDIAL AUTOMATED AND RAPID STERILITY TESTING TECHNOLOGY FOR CELL AND GENE THERAPY PRODUCTS THROUGH REGULATORY PERSPECTIVE AND INDUSTRIAL APPLICATION

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Keywords: Compendial sterility.

Background & Aim: Cell & Gene Therapies have very short shelf lives compared to traditional drugs. Besides, they cannot be sterilized, therefore the historical compendial sterility test methods based on filtration and that takes 14 days to complete as defined in USP <71>: "Sterility Tests" is not compatible.

Methods, Results & Conclusion: In order to assess the sterility of these therapies prior to transfusion, the BacT/ALERT Dual-T (BTA), an automated and more rapid technology, has been developed to reduce the time to result while keeping the highest level of performance and compliance. This method is now recognized as compendial and can be used to replace the traditional 14 day sterility test to assess the sterility of cell-based products as defined in the EP Chapter 2.6.27 "Microbiological Examination Of Cell-Based Preparations". And a USP Chapter USP <72> Respiration-Based Rapid Microbial Methods for the Release of Short Shelf Life Products, is being developped to adress this technology as well.

The objective of this work is to present the pathway to making an alternative test compendial for the specific cellular products. The BTA will be presented and discussed as a compendial test replacing the traditional compendial sterility test, and other alternative technologies will also be discussed. A review of all major literature, scientific articles, validation studies will be presented that demonstrates the use and applicability of the BTA to assess the sterility of cell-based products in comparison to the compendial sterility test method. Different implementations of the BTA within the Cell and Gene Therapies field and the biotechnology industry will be discussed, with recently adopted implementation strategies for different type of therapies.

Finally, a regulatory update will be given on the recent advancements in EP and USP enabling the use of more Modern, Rapid and Automated Sterility Testing solutions to replace the historical sterility test, resulting in greater patient safety.

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HOW INSTITUTIONAL BIOSAFETY COMMITTEES CONTRIBUTE TO SAFETY, CAPACITY AND REGULATORY APPROVALS IN CELL AND GENE THERAPY TRIALS

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