Lampiran 1

CHECKLIST FOR RESEARCH ON STEM CELL AND CELL-BASED THERAPIES

Phase/Process	Key requirements	Researcher (Please tick \/)	Secretariat MREC (Please tick \/)
1. Pre-clinical studies (investigators must show their own data and not from other laboratories)	• Approval letter from animal ethics committee is recommended	☐	☐
	• Accreditation of animal research facility in institution requiring GLP compliance	☐	☐
	• Evidence that the pre-clinical studies was subjected to rigorous and independent peer review and regulatory oversight	☐	☐
	• Safety data in small animals	☐	☐
	• Safety data in large animals	☐	☐
	• Comprehensive toxicology data in small animals (including contamination, acute infusional toxicity, deleterious immune responses, unexpected behavior of the cellular product, and tumorigenesis)	☐	☐
	• Comprehensive toxicology data in large animals (including risks of contamination, acute infusional toxicity, deleterious immune responses, unexpected behavior of the cellular product, and tumorigenesis)	☐	☐
	• Proof of principle of the desired effect (that the cells have repaired the damage/disease) – unequivocal efficacy data	☐	☐
	• Show biological distribution data	☐	☐
	• Show evidence of physiologic integration and long-lived tissue reconstitution	☐	☐
	• Show that differentiation (either *in vitro* before transplantation or *in vivo* after transplantation) occur only along the desired lineages	☐	☐
	• Design based on clinical expectations	☐	☐
	• Mechanistic studies to show biology (done by the group)	☐	☐
	• GLP compliant	☐	☐

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Phase/Process	Key requirements	Researcher (Please tick \/)	Secretariat MREC (Please tick \/)
	• Evidence that the pre-clinical data has been submitted to the NPCB	☐	☐
2. Phase I trials	• Comprehensive pre-clinical studies have been done and data showed safety and efficacy in animals (performed by the group) is recommended	☐	☐
	• Procedures on how the cells be tracked in terms of homing to the target area, viability and longevity of the cells	☐	☐
	• Procedures on how the safety be monitored	☐	☐
	• Procedures to assess risks of tumorigenicity by an independent body must be implemented	☐	☐
	• Procedures to assess short, medium and long term side effects	☐	☐
	• GCP compliance	☐	☐
3. Phase II trials	• Data from Phase I trials (performed by the group themselves and if the trial is not performed by the group, explain why the data should be used for this trial)	☐	☐
	• Procedures on how the cells be tracked in terms of homing to the target area and viability of the cells	☐	☐
	• Optimisation of dose, route, regimen, patient population, endpoints, and controlled	☐	☐
	• Procedures on how the safety be monitored	☐	☐
	• Independent data safety monitoring board	☐	☐
	• Plan to assess short, medium and long term side effects	☐	☐
	• GCP compliance	☐	☐

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4. Phase III trials	• Data from Phase II trials (performed by the group themselves)	☐	☐
	• Design to show safety and efficacy	☐	☐
	• Independent data safety monitoring board	☐	☐
	• GCP compliance	☐	☐
	• Conduct 'randomised' control	☐	☐
5. Cell processing and manufacturing	• Evidence by a letter of conformance for GMP compliance and issued by relevant authority	☐	☐
	• Show evidence of relevant processes: Standard operating procedures, quality standards, environmental control, equipment qualification, analytical methods, audits, staff training, etc.	☐	☐
	• Cell processing and manufacture of any product must be conducted under scrupulous, expert, and independent review	☐	☐
	• Demonstrate that the product is safe, pure and potent	☐	☐
6. Product registration	• Show that the product has been registered with the National Pharmaceutical Control Bureau before use in human trials	☐	☐
	• License for clinical trial has been obtained	☐	☐
7. Cell characterization (pre-requisite to clinical trials)	• History of the cells in the stem cell or cell-based product	☐	☐
	• Biological characterisation of cell type	☐	☐
	• Demonstration of purity	☐	☐
	• Demonstration of potency (e.g. cells produce insulin in a physiological manner)	☐	☐
	• Manufacturing standards and independent certification, where relevant	☐	☐
	• Evidence that cells are free from contamination	☐	☐

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	• Evidence of viability and longevity of cells after transplantation (to determine the likely duration of the therapeutic effect)	☐	☐
	• Evidence that cells will home into the area of damage or repair	☐	☐
	• Evidence of genomic stability during culture	☐	☐
8. Investigators and researchers	• Is the Principal Investigator trained in cell transplantation? (Show evidence of credentialing)	☐	☐
	• Are other investigators trained in cell transplantation? (Show evidence of credentialing)	☐	☐
	• Qualifications of scientists and researchers	☐	☐
	• Registration with National Medical Research Register, Ministry of Health (MOH)	☐	☐
9. Centres performing therapy (Information for patients)	• Registration with PHCFS Act, Ministry of Health	☐	☐
	• Informing subjects about the human embryonic cell source, if applicable	☐	☐
	• The unique risks; and disclose honestly that the treatment have not been tried before	☐	☐
	• Utmost clarity on the potential benefit	☐	☐
	• Disclosing financial and non-financial conflicts of interest	☐	☐
	• Provide monitoring patients long term	☐	☐
	• Providing a clear, timely, and effective plan for adverse event reporting	☐	☐