**BMT/Cellular Therapy CRT Collaboration**

**Ver 1.0; revised 6/14/2024**

**1. Read the Protocol to Identify Support**

|  |  |  |
| --- | --- | --- |
| Population / Departments | Industry Phrases | IBC Requirement  IBCsupport@miami.edu |
| Adult  Pediatric – 18+ yo  MSOM Pipeline Trials | Autologous T-Cell Receptor Engineered T-Cells | Yes |
| Autologous Cell Therapy Product | Yes |
| Allogenic CAR-T T-Cell Therapy | Yes |
| Universal Donor NK Cell infusions | Subject to IBC |
| Neoantigen-specific effector T-cells | Subject to IBC |
| Immune Effector Cells | Subject to IBC |
| Tumor Infiltrating Lymphocytes | Subject to IBC |
| Vaccine: oncogenic retrovirus | Yes |
| Autologous Stem Cell Transplantation / BMT | No |
| Allogenic Hematopoietic Cell Transplantation | No |
| HLA Matched Unrelated Donor Bone Marrow Transplantation | No |
| HLA-Mismatched Unrelated Donor Hematopoietic Cell Transplantation | No |
| Viral: Virus-Associated Post-Transplant Lymphoproliferative Diseases | No |
| Acute GVHD | No |
| Chronic GVHD | No |
| DO NOT NEED SUPPORT | Antibody (ex. a Selective T Cell Receptor (TCR)-targeting, Bifunctional Antibody-fusion Molecule) |  |

**2. Manager requests a protocol review at the next Cellular Therapy Interest Group Meeting**

* Protocols related to transplantation will be reviewed at the next TCT CRT Meeting.
* Contact:
  + "CRS BMT Cellular Therapy Primary Investigators" [TCTPrimaryInvestigators@miamiedu.onmicrosoft.com](mailto:TCTPrimaryInvestigators@miamiedu.onmicrosoft.com)
  + Robby Friedman, Sr. Clinical Manager, BMT/Cellular Therapy at [rxf147@miami.edu](mailto:rxf147@miami.edu)
* Please include the protocol and slide deck for review

**3. A determination letter will be provided to you post-review.**

* Please forward this determination letter to your Study Intake/SSU analyst.

**Clinical Trials and Institutional Biosafety Committee Review**

(One of the fastest growing areas in clinical research is clinical trials involving recombinant DNA, or gene therapy research. Because of the risks involved, this area of research is highly regulated, involving both Institutional Review Board (IRB) and Institutional Biosafety Committee (IBC) to review the risks associated with engineered genetic material in clinical trials.

NIH Guidelines require IBC review for any genetic engineering research, including gene therapy research, that receives NIH funding or takes place at sites receiving NIH funding. The rationale behind IBC review is to ensure a thorough risk assessment is performed regarding the risks associated with the genetically modified materials and a comprehensive risk mitigation plan is in place prior to conducting the research. Gene therapy research requires safety measures to ensure that research participants and study staff as well as the community and the environment surrounding the research site are not harmed by the modified genetic material or the infectious agents that may be utilized to deliver them. An IBC can help researchers utilize the appropriate safety measures to ensure research is conducted safely and responsibly.

Certain key words can aid in determining if IBC review is likely required. The following is a list of common terms found in research protocols requiring IBC review:

**IBC Review required of Human Subject studies involving:**

• Viral vector / Virus based gene delivery vector

Common viruses include:

• Adenovirus (Ad)

• Adeno-Associated Virus (AAV)

• Retrovirus

• Lentivirus (e.g. HIV based vector)

• Herpes virus (HSV)

• Pox virus (e.g. vaccinia, canary pox or fowl pox)

• Genetically modified or reprogrammed immune cells/white blood cells (e.g. CAR T cells)

• DNA vaccines

• Plasmid

• Genetically modified

• Recombinant DNA (rDNA)

• Synthetic Nucleic Acids

• Gene editing: CRISPR-Cas9, TALEN, Zinc Finger Nuclease (ZFN)

• Gene silencing:

• Micro RNA (miRNA), RNA interference (RNAi), short hairpin RNA (shRNA), silencing RNA (siRNA)

The following list includes terms associated with types of clinical trials that increasingly utilize genetically modified materials and, in such cases, may require IBC review. The latter list includes terms that generally do not point to requiring IBC review.

**These studies may need IBC Review (need more info about the design of the investigational product)**

• Vaccines, especially for cancer and infectious agents (Ebola, SARS, MERS, Zika, etc.)

• Immunotherapy

• Cellular therapy

• Regenerative therapy

**Studies not likely to need IBC Review**

• Genetic screening or testing (e.g. 23 and me)

• Gene/genome sequencing

• Monoclonal antibody based therapy (Anything with a name ending in “Mab”)

• Tests that do not involve extraction and manipulation of genetic material (e.g. routine blood or urine tests)

**NIH GUIDELINES FOR RESEARCH INVOLVING RECOMBINANT OR SYNTHETIC NUCLEIC ACID MOLECULES**

chrome-extension://efaidnbmnnnibpcajpcglclefindmkaj/https://osp.od.nih.gov/wp-content/uploads/NIH\_Guidelines.pdf

**Section III-C. Experiments Involving Human Gene Transfer that Require Institutional Biosafety Committee Approval Prior to Initiation**

**Section III-C-1. Experiments Involving the Deliberate Transfer of Recombinant or Synthetic Nucleic Acid Molecules, or DNA or RNA Derived from Recombinant or Synthetic Nucleic Acid Molecules, into One or More Human Research Participants**

Human gene transfer is the deliberate transfer into human research participants of either:

1) Recombinant nucleic acid molecules, or DNA or RNA derived from recombinant nucleic acid molecules, or

2) Synthetic nucleic acid molecules, or DNA or RNA derived from synthetic nucleic acid molecules, that meet any one of the following criteria: a) Contain more than 100 nucleotides; or b) Possess biological properties that enable introduction of stable genetic modifications into the genome (e.g., cis elements involved in integration, gene editing); or c) Have the potential to replicate in a cell; or d) Can be translated or transcribed.

Research cannot be initiated until Institutional Biosafety Committee and all other applicable institutional and regulatory authorization(s) and approvals have been obtained.

The deliberate transfer of recombinant or synthetic nucleic acids into one human research participant, conducted under a Food and Drug Administration (FDA) regulated individual patient expanded access Investigational New Drug (IND) or protocol, including for emergency use, is not research subject to the NIH Guidelines and thus does not need to be submitted to an IBC for review and approval.