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| |  |  | | --- | --- | | ***Administrative Use Only*** | | | **Registration Number** | **Approval Date** | | IBC# |  |         **Biosafety Registration**  **for Clinical Research Involving Recombinant or Synthetic Nucleic Acid Molecules** | | | | | | | | |
| * The **Institutional Biosafety Committee (IBC)** will review this registration form as per the   [*NIH Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules (NIH Guidelines; April 2019*](https://www.luriechildrens.org/globalassets/documents/luriechildrens.org/research/research-management--support/researcher-toolkit/irb-resources/nih-guidelines-2019.pdf)*)***.**   * Please review the [*NIH Guidelines* Appendix M](https://www.luriechildrens.org/globalassets/documents/luriechildrens.org/research/research-management--support/researcher-toolkit/irb-resources/nih-guidelines-2019.pdf) prior to filling out this registration form. * **Further information can be found** on the [Institutional Biosafety Committee Resources Page](https://www.luriechildrens.org/en/research/toolkit/ibc-resources/). * **Submit this registration form and supporting documents via e-mail to** [*IBC@luriechildrens.org*](mailto:IBC@luriechildrens.org)**.** | | | | | | | | |
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| 1) | **Full Protocol Title:** *Should match titles of IRB Protocol and of Protocol registered with the NIH.* | | | | | | | |
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| 2) | | **Principal Investigator:** | |  | | | | |
|  | | **Division/Department:** | |  | | | | |
|  | | **e-Mail:** | |  | | | | |
|  | | **Phone:** | |  | | | | |
|  | | **Emergency Phone:** | |  | | | | |
|  | |  | |  | | | | |
| 3) | | **Other Key Contact** *(i.e.., research coordinator)***:** | |  | | | | |
|  | | **e-Mail:** | |  | | | | |
|  | | **Phone:** | |  | | | | |
|  | |  | |  | | | | |
| 4) | | **Emergency Contact:** | |  | | | | |
|  | | **Phone:** | |  | | | | |
|  | |  | |  | | | | |
| 5) | | **Study Personnel who will handle the biological agent:**  *List all personnel who will be trained on the study-specific biosafety practices. Indicate their roles as they relate to the agent: i.e., receipt, preparation, handling, transport, or administration of study biological agent/drug.**There is no need to include personnel who provide standard of care treatment or do not have exposure to the agent.* ***Note:*** *The PI is responsible for ensuring proper training of all authorized study personnel.* | | | | | | |
|  | | **Name, Credentials (Note:** *“Research Pharmacy” or “Clinical Research Unit” may be listed collectively, but attach a letter of support)* | | | | Describe contact with biological agent *(receipt, preparation, handling, administration, transport, disposal, specimen collection, etc.)* | | |
|  | | **Example**: Research Pharmacy | | | | Receives and prepares agent | | |
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|  | | ***Note:***  *All clinical staff receive Bloodborne Pathogen Training upon hire and annually thereafter and are offered Hepatitis B vaccine upon hire or required to sign a declination.* | | | | | | |
|  | |  | | | | | | |
| 6) | | **Study Abstract:** | | | | | | |
|  | | **(a)** Provide the Purpose or Specific Aims or Hypothesis of the proposed Clinical Study.    **(b)** Explain the rationale for use of recombinant or synthetic nucleic acids in human participants particularly in children e.g., explain benefits of proposed biological or investigational agent over current standard-of-care treatment. (200 words or less)    **(c)** Provide a concise summary of preclinical and, as applicable, clinical studies conducted in support of the proposed study. Additionally, indicate specific section(s) of the Clinical Protocol/Investigation Brochure that provides details of this information. (200 words or less)    ***NOTE:*** *Define all acronyms at first usage. Use non-technical language to allow all IBC members including community members with non-scientific backgrounds to understand the study and assess the risks.* ***Do not*** *include details of clinical procedures in this section.* | | | | | | |
|  | |  | | | | | | |
| 7) | | **Description of transgene or nucleic acid sequence to be delivered:** | | | | | | |
|  | | Name of transgene or nucleic acid sequence *(e.g. Globin cDNA, Globin Gene, Globin siRNA)* | | | | |  | |
|  | | Species source of transgene or nucleic acid sequence *(e.g. human, mouse, synthetic)* | | | | |  | |
|  | | Function *(e.g. transcriptional factor, oncogene, tumor suppressor, suppressor of gene expression: Identify gene, toxin)* | | | | |  | |
|  | | Modifications *(e.g., mutations, deletions, truncations)* | | | | |  | |
|  | | Regulatory elements in the construct *(e.g., promoters)* | | | | |  | |
|  | | Will this be a drug resistance gene?  *Select “No” if only using drug resistance as a selection marker* | | | | | Yes  No | |
|  | | Will the gene product potentially elicit an adverse immune response? | | | | | Yes  No | |
|  | |  | | | | | | |
| 8a) | | **Description of vector delivery system:** | | | | | | |
|  | | Will a viral vector be used?  If *“No”*, proceed to 8b. | | | Yes  No | | | |
|  | | Type of Virus *(e.g., AAV, adenovirus , lentivirus, herpes virus)* | | |  | | | |
|  | | Modifications to the virus *(e.g., deletions to attenuate or self-inactivate, encapsulation in any synthetic complex, number of plasmids used to generate virus, changes to tropisms)* | | |  | | | |
|  | | Was helper virus used to produce the viral vector?  Identify the helper virus if used *(e.g., adenovirus)* | | | Yes  No | | | |
|  | | Was the helper virus removed from the final drug product preparation? | | | Yes  No  N/A | | | |
|  | | Briefly describe how the viral vector(s) was derived *(i.e., the number of plasmids, types of cells used, etc.)*. | | |  | | | |
|  | | Is the virus replication competent i.e., virus is able to replicate or make more virus particles? | | | Yes  No | | | |
|  | | Describe method(s) for replication-competent virus testing. Write *“None”* if testing was not done. | | |  | | | |
|  | | Will the viral vector potentially elicit an adverse immune response? | | | Yes  No | | | |
|  | | If gene product elicits an immune response, briefly describe how this will be mitigated, | | |  | | | |
|  | | Provide an assessment of potential for insertional mutagenesis of viral vector. | | |  | | | |
|  | | Intended *ex vivo* or *in vivo* target cells. | | |  | | | |
|  | | Is a viral vector map included in a separate attachment? | | | Yes  No | | | |
|  | |  | | |  | | | |
| 8b) | | Will other delivery system(s) be used *(e.g., plasmid, bacteria)*? | | | Yes  No | | | |
|  | | Identify the other delivery system. | | |  | | | |
|  | | Describe the biosafety features of the other delivery system *(e.g., non-infectious)*. | | |  | | | |
|  | |  | | | | | | |  | |
| 9) | | **Description of study biological agent/drug:** | | | | | | |  | |
|  | | Name of agent/drug (both long and, if applicable, short name). | | |  | | | |  |
|  | | Other material(s) used in preparation of study agent/drug (aside from vector and nucleic acids) that will be administered to the human research subject *(e.g., helper virus, carrier particles)*. | | |  | | | |
|  | | Source of study agent/drug *(e.g., Sponsor name, name of commercial vendor, PI’s lab, collaborator’s name)* | | |  | | | |
|  | | Delivery method to human participants: route (e.g., intravenous), concentration, frequency | | |  | | | |
|  | | | | | | | | |
| 10) | | **Collection of Study Participant Specimens** | | | | | | |
|  | | Will specimens be collected from human participants?  Yes  No  If “*No”*, proceed to section 11. | | | | | | |
|  | | What specimens will be collected from study participants who received the biological agent or drug *(e.g., blood, urine, tissue samples)*? | | | | | | |
|  | | Confirm that body fluids and tissues obtained from participants  Yes  N/A in the study will be handled using Universal Precautions under  Biosafety Level 2. | | | | | | |
|  | | Which laboratory or research facility will receive the collected specimens? | | | | | | |
|  | | If participant specimens will be shipped, provide the name and training date of the designated study personnel responsible for transport and/or shipment.  **Study Personnel:**      **Date of Training:** | | | | | | |
|  | | | | | | | | |
| 11) | | **Study Location(s)** | | | | | | |
|  | | Where is the study biological agent/drug stored and prepared for patient administration? *If at the Research Pharmacy, attach a Letter of Support.* | | | | | | |
|  | | Where will study biological agent/drug be administered? *List specific hospital room, unit, or clinic.* | | | | | | |
|  | |  | | | | | | |
| 12) | | **Risk Group Classification:** *(See Appendix B of NIH Guidelines for appropriate Risk Group assignment.)* | | | | | | |
|  | |  | **Risk Group One (RG-1):** Biologicalagents that are not associated with disease in healthy humans *(e.g., adeno- associated virus (AAV – all serotypes) and recombinant or synthetic AAV constructs, in which the transgene does not encode either a potentially tumorigenic gene product or a toxin molecule and are produced in the absence of a helper virus, etc.).* For other examples, consult the *NIH Guidelines*, *Appendix B-I. Risk Group 1 (RG1) Agents***.**  List all RG-1 biological agents to be used: | | | | | |
|  | |  | **Risk Group Two (RG-2):** Biologicalagents that are associated with human disease which is rarely serious and for which preventive or therapeutic interventions are often available. For examples, consult the *NIH Guidelines*, *Appendix B-II. Risk Group 2 (RG2) Agents.*  List all RG-2 biological agents to be used: | | | | | |
| **Note:** Currently, research with biological agents falling into RG3 or RG4 categories is not permitted at the Institution due to the absence of BSL-3&4 facilities necessary to conduct this type of research:   * *RG-3: Biological agents that are associated with serious or lethal human disease for which preventive or therapeutic interventions may be available (high individual risk but low community risk).* * *RG-4: Biological agents that are likely to cause serious or lethal human disease for which preventive or therapeutic interventions are not usually available (high individual risk and high community risk).* | | | | | | | | |
|  | | | | | | | | |
| 13) | | **Biosafety Containment Level:** *(Biosafety Levels are described in Appendix G-II. Physical Containment Levels**of NIH Guidelines.)* | | | | | | |
|  | |  | **Biological Safety Level One (BSL-1):** Work with agents not known to consistently cause disease in healthy adults, animals and/or the environment. | | | | | |
|  | |  | **Biological Safety Level Two (BSL-2):** Work with agents associated with disease in humans, animals and/or the environment. The route of exposure into the host is generally through ingestion, injection, absorption and/or mucous membrane exposure. | | | | | |
| **Note:** As above, the Institution does not have facilities that comply with BSL3 or BSL4 requirements.   * *BSL-3: Work with indigenous and/or exotic agents capable of causing serious or potentially lethal disease and present the potential of aerosol transmission. The most common route of exposure is via the inhalation route, although exposure may be possible through ingestion, injection, absorption and/or mucous membrane exposure.* * *BSL-4: Work with dangerous or exotic agents which pose high risk of life-threatening disease. The route of exposure may be unknown.* | | | | | | | | |
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| 14) | | **NIH Category:** *(Refer to* ***Section III*** *of the NIH Guidelines)* | | | | | | |
|  | |  | **III-A:** Require IBC approval, and NIH Director approval before initiation. | | | | | |
|  | |  | **III-B:** Require IBC approval and NIH OSP approval before initiation. | | | | | |
|  | |  | **III-C:** Requires IBC and Institutional Review Board approval (if applicable) before research participant enrollment. *(e.g. 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* | | | | | |
|  | |  | **III-F:** Exempt experiments *(To be determined by IBC. Biosafety Registration still required)* | | | | | |
|  | | | | | | | | |  |
| **Attachments** *– Include the following with the submission* | | | | | | | | |  |
|  | | **Letter of Support from Infection Prevention and Control –** Should describe any special precautions such as PPE for staff, staff or family members that should avoid contact (immunocompromised, pregnant, elderly, etc.) | | | | | | |
|  | | **Letters of Support** - Should be specific to the IBC to ensure the Research Support Services are aware of the biohazardous risks (i.e. Research Pharmacy, Clinical Research Unit, etc.) | | | | | | |
|  | | **Clinical Protocol and/or Investigational Brochure –** Should containdetailed description of investigational drug product and preclinical (and clinical if available) studies conducted in support of the proposed clinical trial. | | | | | | |
|  | | **Vector/Transgene Map** | | | | | | |
|  | | **Material Safety Data Sheet** (if available for biological agent/drug) | | | | | | |
|  | | **IRB Consent Forms** *–* Explaining study and potential risks to participants. | | | | | | |
|  | | **Safety Protocols**: Should include study-specific biosafety procedures including but not limited to: (a) minimizing risks to study personnel involved in the preparation and administration of biological agent and address accidental exposure to biohazardous materials; (b) minimizing risks to study participants and other persons with whom the participant comes in contact and address potential horizontal transmission of infection e.g., viral; and 3) minimizing risks to the environment and address disposal of biohazardous materials.  Examples of Safety Protocols:   1. Safety Protocol for Human Research with Investigational Drug Product containing Lentivirus Vector 2. Safety Protocol for Human Research with Investigational Drug Product containing Adeno-Associated Virus Vector | | | | | | |
| **Assurances** | | | | | | | | |
|  | | I assure the information provided is accurate and complete. I agree to conduct this research using the appropriate biosafety level of containment. | | | | | | |
|  | | I accept responsibility for ensuring all study personnel are properly trained and understand the potential biohazards and relevant biosafety practices, protective equipment and techniques, and emergency procedures. | | | | | | |
|  | | I assure that all involved personnel will have access to the Biosafety Registration and all subsequent addenda to the Registration, the Safety Protocol(s), the Clinical Protocol, and all study-related IBC documents and Lurie Children’s Standard Operating Procedures (SOPs). | | | | | | |
|  | | I agree to abide by all institutional policies and procedures as well as those contained in the most current *NIH Guidelines*. | | | | | | |
|  | | I confirm that the IBC [Safe Handling, Administration, and Disposal of Study Biological Agents Policy(8/14/20)](https://www.luriechildrens.org/globalassets/documents/luriechildrens.org/research/research-management--support/researcher-toolkit/irb-resources/safe-handling_administration-and-disposal-of-study-biological-agents_8.14.20.pdf) SOP, the [Pharmaceutical Waste Management SOP](https://www.luriechildrens.org/globalassets/documents/luriechildrens.org/research/research-management--support/researcher-toolkit/irb-resources/pharmaceutical-waste-management.pdf), and have submitted with the IBC [Safety Protocol](https://www.luriechildrens.org/globalassets/documents/luriechildrens.org/research/research-management--support/researcher-toolkit/irb-resources/safety-protocol.docx) any additional measure that are required per the study agent/protocol. | | | | | | |
|  | | I have outlined any additional safety measures needed or planned (e.g., issuing a “wallet card” to the participant with information regarding viral shedding, etc.). | | | | | | |
| Principal Investigator Signature Date | | | | | | | |  |
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