Gene Therapy Resource Program

Funded by the National Heart, Lung, and Blood Institute

To access this RSA form, please use one of the following links:

- Approved GTRP investigators - https://www.gtrp.org/Public/RSA/Default.aspx
- All other investigators - https://www.gtrp.org/Public/InvestigatorRegistration/

Section II - Trial / Protocol Information

Attention submitting investigators: The GTRP only considers providing partial funding for clinical trials. The primary focus of GTRP funding is on patient care-related expenses. The Program will not consider applications for complete funding of a trial.

This is a two-stage application submission and review process. If, after the initial review, your RSA receives provisional approval, you will need to identify other sources of funding to ensure that the trial can be fully funded. Prior to final RSA submission and review you will also need to provide the most current information concerning site participation and regulatory status.

Instructions
* Indicates a required field.

Do not exit your browser without saving your RSA information using one of the save buttons at the bottom of this Section (following item 11)!

If you need to copy information from another RSA, open a new browser window to access that RSA. To navigate between initiated RSAs use the "minimize" and "maximize" buttons. **Do not** use the forward/back browser buttons.

Protocol Documents must contain an express Data and Safety Monitoring Plan and an express Data Sharing Plan.

1. * Title of Clinical Trial:

2. * Abstract Describing Clinical Trial:

3. Upload Draft Protocol and Informed Consent - if Main Site IRB approval has not been received. (3 uploads maximum)

Browse... Upload

Note: To save and upload a document, select the *Upload* button.

4. Upload Main Site IRB approved clinical protocol and Informed Consent – if available. (3 uploads maximum)

Browse... Upload

Note: To save and upload a document, select the *Upload* button.

5. * Select Disease Category:

☐ Heart ☐ Lung ☐ Blood ☑ Other

Please specify disease category:

6. Targeted Disease

Other

Please specify targeted disease:

7. Provide specific disease description

8. * Target organs, tissues, cell, etc.

9. * Gene / Vector Name:

10. * Route of administration including device description:

11. Have you submitted other RSAs related to this trial?

○ Yes ○ No

RSA ID:

Save and Continue

Section III - Trial Enrollment - Site Participation

Instructions

Do not exit your browser without saving your RSA information using one of the two save buttons!

If you need to copy information from another RSA, open a new browser window to access that RSA. To navigate between initiated RSAs use the "minimize" and "maximize" buttons. **Do not** use the forward/back browser buttons.

Download this Word® form concerning trial enrollment and site participation. Complete the form and upload it below.

Upload completed form here. *(3 uploads maximum)*

Browse... Upload

Note: To save and upload a document, select the *Upload* button.

RSA ID: 2045

Save and Continue

Section IV - Study Specific Funding Support

Instructions

Do not exit your browser without saving your RSA information using one of the save buttons at the bottom of this Section (following item 2)!

If you need to copy information from another RSA, open a new browser window to access that RSA. To navigate between initiated RSAs use the "minimize" and "maximize" buttons. **Do not** use the forward/back browser buttons.

1. Please provide information on your current and pending funding for this clinical trial. Specify funding sources; identifying number(s), e.g., grants; amount of funding; funding designated purpose(s); and funding period.

☑ NHLBI Funding

☑ Other NIH Funding

☑ Other Funding (Institutional, Foundation, Industry, etc.)

☑ Funding Not Yet Secured

What are your plans for securing funding for your project?

2. Provide funding commitment letters for "other" funding. Letters should be on institutional or foundation letterhead and contain purpose of funding, amount of funding, period of funding, and any contingencies placed on funding.

Upload letters of funding commitment here. (3 uploads maximum)

Browse... Upload

Note: To save and upload a document, select the *Upload* button.

RSA ID: 2045

Save and Continue

Section V - Initial Budget Submission

Instructions

Do not exit your browser without saving your RSA information using one of the save buttons at the bottom of this Section!

If you need to copy information from another RSA, open a new browser window to access that RSA. To navigate between initiated RSAs use the "minimize" and "maximize" buttons. **Do not** use the forward/back browser buttons.

Please upload a readable version – printable on no larger than legal size paper - of that portion of your study budget that you are requesting from the GTRP. Remember that only patient care-related costs will be considered. This may include costs of study visits not covered under standard-of-care; costs of staff who interact directly with the patients; the cost of trial-specific equipment (list equipment and costs individually), including indirect costs if applicable. The direct and indirect costs must be clearly identified.

Upload your initial study budget(s). (3 uploads maximum)

Browse... Upload

Note: To save and upload a document, select the *Upload* button.

RSA ID: 2045

Save and Continue

Section VI - Regulatory Information Part A: Protocol

Note to Investigator: Concerning Section VI, completion of Part A is all that is required during the first phase of this submission. Following completion of the RSA through Section VI Part A, the CCC will schedule your RSA for a preliminary GTRP review. You may complete Section VI Parts B and C at a later date.

Instructions

Do not exit your browser without saving your RSA information using one of the save buttons at the bottom of this Section!

If you need to copy information from another RSA, open a new browser window to access that RSA. To navigate between initiated RSAs use the "minimize" and "maximize" buttons. **Do not** use the forward/back browser buttons.

Provide the following protocol information related to this clinical trial:

1. Download this Word® form concerning IND and FDA information. Complete the form and upload it below

Upload completed form here. (3 uploads maximum)

Browse... Upload

Note: To save and upload a document, select the *Upload* button.

2. Novel and Exceptional Technology and Research Advisory Committee (NExTRACT), date of protocol review (if applicable):

Upload communications from the OSP, NExTRACT (3 uploads maximum)

Browse... Upload

Note: To save and upload a document, select the *Upload* button.

3. Investigator and Institution Certifications and Agreement (for Clinical Trial Funding Assistance)

Instruction to RSA Applicant: Download the above document.
Please note:

- This document must be signed by you as the applicant investigator and by an official of your Institution. If you wish to make modifications to the document language, contact the CCC prior to uploading a signed form. The CCC must have a signed document before the NHLBI Gene Therapy Group can approve your RSA. You may, however, submit your RSA prior to execution of this agreement.
- If this is a multi-site trial you, as the RSA applicant, must ensure that all collaborating investigators have read and understand this document and agree to abide by the GTRP's policies. The CCC will require assurance of this from you prior to execution of a subcontract with your institution for an approved RSA. In addition, the GTRP will determine, based on individual circumstances, whether or not signed letters of collaboration are required from any collaborating investigator/institution. If required, the upload area for those letters follows in Section VI, Item 7.

Upload your signed Investigator and Institution Certifications and Agreement. (1 upload maximum)

Browse... Upload

Note: To save and upload a document, select the *Upload* button.

RSA ID: 2045

Save and Continue

Section VI - Regulatory Information Part B: Trial Management

Instruction to Submitting Investigator

- Do not exit your browser without saving your RSA information using one of the save buttons at the bottom of this Section!
- If you need to copy information from another RSA, open a new browser window to access that RSA. To navigate between initiated RSAs use the "minimize" and "maximize" buttons. **Do not** use the forward/back browser buttons.

4. Site Staffing Plan:

Please provide a clinical site staffing and management plan. Please include the Site Principal Investigator, Site Study Coordinator, and Co-investigators at the site. Include the co-investigators' roles, e.g., consenting investigator, procedural responsibility, scientific contributor-not an enrolling investigator. If you have drafted your Delegation of Responsibilities and Signature Log that document would be suitable to include. Note that GTRP only considers funding of staff directly involved in patient care. If there are multiple trial sites, each site's plans are to be included.

5. Data and Safety Monitoring Plan:

Describe your data and safety monitoring plans for this trial. Include, if applicable, your plans for clinical site monitoring by an external group; provide the name of the company or CRO that will provide this service. Include SAE reporting plans to various oversight bodies. Please describe your plans for Data Management. If your data will be stored electronically, provide documentation that the electronic Data Management System is fully validated. Please note that any trial receiving funds from the NHLBI GTRP will also be subject to review by the NHLBI Gene and Cell Therapies DSMB.

6. Letters of Site Collaboration:

Collaborating sites should provide a letter of support that includes:

- The title of the trial
- The estimated number of study participants to be recruited by this site
- Awareness of, and agreement to abide by GTRP Policies as outlined in the Investigator and Institution Certifications and Agreement document
- Any funding to be provided through the collaborating Investigator/Institution for conduct of this trial.

Letters must be signed by the site's Principal Investigator for this trial and co-signed by an Institution Official with authority regarding funding commitments.

Upload letters of collaboration here. (5 uploads maximum)

Browse... Upload

Note: To save and upload a document, select the *Upload* button.

RSA ID: 2045

Save and Continue

Section VI - Regulatory Information Part C: Site-Institution Information and Documents

Instruction to Submitting Investigator

- Do not exit your browser without saving your RSA information using one of the save buttons at the bottom of this Section!
- If you need to copy information from another RSA, open a new browser window to access that RSA. To navigate between initiated RSAs use the "minimize" and "maximize" buttons. **Do not** use the forward/back browser buttons.

Provide the following related to this clinical trial:

7. Main Site Principal Investigator as reflected on Form 1572

8. FWA number under which this trial will be conducted at main site:

9. Institutional Biosafety Committee (IBC) approval date (main site):

Upload IBC approval letter (3 uploads maximum)

Browse... Upload

Note: To save and upload a document, select the *Upload* button.

10. Institutional Review Board (IRB) approval date (main site):

Upload IRB approval letter (3 uploads maximum)

Browse... Upload

Note: To save and upload a document, select the *Upload* button.

11. If this is a multi-site trial provide information for each site.

Use the grid below to select and edit an existing site/institution.

Additional Sites

No sites found.

RSA ID: 2045

Save and Continue

Section VII - Final Budget Requested of the GTRP

Instruction to Submitting Investigator

- Do not exit your browser without saving your RSA information using one of the save buttons at the bottom of this Section!
- If you need to copy information from another RSA, open a new browser window to access that RSA. To navigate between initiated RSAs use the "minimize" and "maximize" buttons. **Do not** use the forward/back browser buttons.

This section should be completed only after you have received instruction from the CCC to upload a finalized budget in this section.

Please upload a readable – printable on no larger than legal size paper - of that portion of your study budget that you are requesting from the GTRP. Remember that only patient care-related costs will be considered. This may include costs of study visits not covered under standard-of-care; costs of staff who interact directly with the patients; the cost of trial-specific equipment (list equipment and costs individually), including indirect costs if applicable. The direct and indirect costs must be clearly identified.

Upload your final study budget. (3 uploads maximum)

Browse... Upload

Note: To save and upload a document, select the *Upload* button.

RSA ID: 2045

Save and Submit as Final