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 - P/T Study Information

Instruction to Submitting Investigator:
* 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 13)!

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.

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Tables (in Word® or pdf format only) may be uploaded using the RSA Supplement feature. Please direct the reader to this material by referencing the uploaded table within the associated text.

1. Select a study type:

○ Pilot Study ○ Pivotal Study (main safety and/or biodistribution study that supports the IND)

2. * Study Title:

3. * Study Abstract:

4. * Rationale for Conducting the Study:

5a. Upload relevant scientific publications/investigator's brochure/other documents. *(5 uploads maximum)*

Browse... Upload

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

5b. Provide available proof of concept data and if any previous preclinical safety data. *(5 uploads maximum)*

Browse... Upload

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

6. * Select Disease Category:

☐ Heart ☐ Lung ☐ Blood ☐ Sleep ☑ Other

Please specify disease category:

7. Targeted Disease

8. Target organs, tissues, cell, etc.

9. Gene / Vector Name:

10. Animal Model(s) (e.g., knockout mouse, normal pig):

10a. Can the Animal Model be purchased from commercial vendor or other source?

○ Yes ○ No

Further comment:

10b. Will the Pharm/Tox Core need to induce disease to achieve the above model(s)?

○ Yes ○ No

Further comment:

11. General Description of Pharmacology/Toxicology/Biodistribution Study Design (doses, route of administration, study endpoint, etc.):

12. Description of the proposed clinical trial:

13. If you have submitted other RSAs that relate to this study, select them from the list below:

2007
2008
2009
2010
2011

Add
Remove

RSA ID:

Save and Continue

Section III - P/T Regulatory Information

Instruction to Submitting Investigator:

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Section I | Section II | **Section III** | Section IV | Section V - 1 | Section V - 2 | Section V - 3

1. Have you discussed pharmacology/toxicology and clinical trial design with the FDA?

(•) Yes ( ) No

Meeting Type:

Provide the following information for each contact with FDA:

Contact Date (mm/dd/yyyy): Contact Type: ( ) Phone ( ) Face-to-Face ( ) Email

Meeting/Contact Summary

Upload any FDA communications and other FDA documentation here (3 Uploads Maximum):

Browse... Upload

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

Save Meeting/Contact Info

To add information about additional meetings/contacts with FDA, please complete the fields above.

No meetings found.

2. Reason(s) for requesting Pharmacology/Toxicology testing:

Initial testing required by the FDA?

(•) Yes ( ) No

Specific Reason for Request:

Follow-up testing or supplemental testing requested by the FDA?

(•) Yes ( ) No

Specific Reason for Request:

Other?

(•) Yes ( ) No

Specific Reason for Request:

3. Provide relevant scientific literature and/or guidance documents related to follow-up or supplemental testing requested by the FDA (3 Uploads Maximum).

Browse... Upload

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

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Save and Continue

Return to RSA 2043 Home | Return to User Main Page | Return to GTRP Home Page |

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!

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Please provide information on your current and pending funding for this research study.

☑ NHLBI Funding

☑ Other NIH Funding

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

☑ No Funding Secured

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Save and Continue

Section V-1 - Vector Information

Instruction to Submitting Investigator:

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Note to Investigator: Please contact the Pharm/Tox Core regarding the amount of vector needed and the preferred vialing.

1. Has the test material been made?

○ Yes ◉ No

1a. When will it be available?

Comments:

2. Gene/Vector Name:

3. Vector Source (who is producing vector):

4. Estimated amount of material to be provided (total particles/vector genomes or transduction):

5. Vector Grade:

Note to Investigator: GMP process-comparable indicates vector prepared using similar cell culture and purification processes as GMP material, but for which some GMP requirements (such as raw materials qualification, manufacturing facility environmental monitoring, and in-process monitoring of process intermediates) are not fully implemented. In addition, characterization and release testing of this material is less extensive than for full GMP-grade material.

6. If available, provide relevant information for the vector regarding:

6a. Titer (physical and infectious):

6b. Intended characterization (e.g., identity, purity, potency):

6c. Vehicles and Excipients (indicate Lovelace to purchase or provided by vector lab):

6d. Storage conditions required:

6e. Stability; shelf life under intended storage conditions:

6f. Stability under conditions of use:

6g. Vialing: concentration of vector and volume per vial (please discuss with Lovelace for vialing appropriate for the study):

6h. Special handling procedures:

Upload special handling procedures document here (if any): *(5 uploads maximum)*

Browse... Upload

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

6i. Known toxicities (in vitro or in vivo) with transgene expression and/or vector:

Upload known toxicities here (if any): *(5 uploads maximum)*

Browse... Upload

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

7. Describe method used to make vector:

8. Vector Construct:

8a. Vector Type:

8b. Serotype:

8c. Transgene:

8d. Promoter:

8e. Transgene species specificity (e.g., human, mouse, etc.):

8f. Packaging cell line:

Upload sequence data here:

Browse... Upload

Note: To save and upload a document, select the **upload** button.*(1 upload maximum)*

Upload gene vector map here:

Browse... Upload

Note: To save and upload a document, select the **upload** button.*(1 upload maximum)*

9. Briefly describe the strategy used in construction of the vector and reasons for including viral sequences, the specific transgene sequences, and any enhancer/promoter or other regulatory regions.

10. What is the tissue specificity of the transgene expression, if any?

11. Does the investigator have an assay/method for quantifying vector?

○ Yes ◉ No

Will the Investigator develop an assay?

○ Yes ○ No

Will Lovelace need to develop an assay?

○ Yes ○ No

12. If assessment of gene expression by qPCR is an endpoint for this study, does the investigator have an assay in place?

○ Yes ◉ No

RSA ID: 2043

Save and Continue

Section V-2 - Animal Model: Testing Information Requested

Instruction to Submitting Investigator:

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Note to Investigator: In this section you will need to provide the following information:

- Required animal models
- Details for each animal model
- Tests/Assays for each animal model

***Animal model(s)**

Please only select one model at a time. You will have a chance to "Select Another Animal Model" at the end.

Mouse

***a. Age of animals to be used:** *(Check all that apply)*

☑ Neonates ☐ Young adults ☐ Sexually mature ☐ Retired breeders

Neonates Information

b-1. Further description of animal model *(Check all that apply)*

☐ Strain

☐ Genetically-modified or knockout model

☐ Disease model name

b-2. Tests/Assays

☐ Hematology

☐ Serum Chemistry

☐ Coagulation Parameters

☐ Pharmacokinetics/Biodistribution of Vector (Quantitative real time PCR)

☐ Histopathology

☐ Transgene expression

☐ Antibodies to vector and/or transgene

☐ Cell-mediated immunity to vector and/or transgene

☐ Biomarkers of Gene Function

☐ Other

b-3. Route of Administration

If unique delivery method, can you transfer dosing methods to the Pharm/Tox Core?

○ Yes ○ No ○ TBD

Further comment

If necessary, can you come to the Pharm/Tox Core to dose?

○ Yes ○ No ○ TBD

Further comment

Will specialized equipment (e.g., fluoroscope, ultrasound, i-Stat) be needed for dosing?

○ Yes ○ No

Further comment

What device(s) (e.g., inhalation system, catheter, electroporation device) will be involved in the administration/delivery?

Further comment

Will any of the devices require FDA review?

○ Yes ○ No ○ TBD

Further comment

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Save And Select Another Animal Model

Save and Continue

Section V-3 - Laboratories and Timelines: Testing Requested Information

Instruction to Submitting Investigator:

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1. Will a laboratory other than the Pharm/Tox Core perform a portion or all of the preclinical animal safety/biodistribution work?

(•) Yes ( ) No

Please provide the laboratory name and location

2. Testing Timelines

Describe any timeline constraints regarding any testing.

RSA ID: 2043

Submit Final RSA