



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 - Preclinical Vector Study Information

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 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.

1. * Study Title:

2. * Study Abstract:

3. * Rationale for Conducting the Study:

4. * Study Design:

5. Clinical Translation Statements

5a. Describe how the vectors requested in this RSA and the associated study will advance the science closer to clinical translation.

5b. Briefly describe subsequent preclinical studies you plan to conduct and how these studies will address important issues in progressing to the clinic.

5c. Identify the anticipated gene therapy clinical product(s) and their application(s) in terms of disease(s).

6. * Select Disease Category:

Heart Lung Blood Other

Please specify disease category:

7. Targeted Disease

8. Target organs, tissues, cell, etc.

9. Route of administration

10. Indicate the type of study in which this plasmid or vector will be used. (Select one)

- Proof-of-Concept Study
- IND Enabling Study (preclinical study focused on the generation of research data to include in an IND submission)

11. Describe the test system in which you are using the plasmid or vector. (Check all that apply)

In-vitro model

Animal model

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

2007	Add Remove	
2008		
2009		
2010		
2011		

13. If experiments associated with the related RSA(s) (right-hand column above) have been completed, please upload a summary of that data and any publications and presentations. (5 uploads maximum)

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

RSA ID: 0

Save and Continue

Section III - Preclinical Vector Regulatory Information

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.

[Section I](#) | [Section II](#) | **[Section III](#)** | [Section IV](#) | [Section V](#) |

1. Has your Institutional Biosafety Committee (IBC) approved this study?

Yes No

2. Has your Institutional Animal Care and Use Committee (IACUC) approved this study?

Yes No

RSA ID: 2042

Save and Continue

[Return to RSA 2042 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!

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.

[Section I](#) | [Section II](#) | [Section III](#) | **[Section IV](#)** | [Section V](#) |

Please provide information on your current and pending funding for this research study. Specify funding number(s), amount of funding, and funding period.

Note: If any of the NIH or NHLBI grant funds are allocated for vector production, please indicate the amount.

NHLBI Funding

Other NIH Funding

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

No Funding Secured

RSA ID: 2042

Save and Continue

[Return to RSA 2042 Home](#) | [Return to User Main Page](#) | [Return to GTRP Home Page](#) |

Section V - Preclinical Vector Request Information

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.

[Section I](#) | [Section II](#) | [Section III](#) | [Section IV](#) | **[Section V](#)** |

Which laboratory service are you requesting?

Note: Only one service with a maximum of 30 vectors can be selected per RSA submission

- Adenovirus Vector Production
- Adeno-Associated Viral (AAV) Vector Production
- Lentiviral/Retroviral Vector Production
- Non-Viral Vector Production

Adeno-Associated Viral (AAV) Vector Production

1. Serotype (Select one)

2. Promoter Full Name and Abbreviation

3. Transgene Full Name and Abbreviation

4. Transgene Biological Activity

5. Transgene Species

6. Polyadenylation Signal Abbreviation

7. Other elements (e.g., WPRE, P2A, chimeric intron, Flag, myc, his tag, HA tag, IRES)

8. Material Investigator will submit to Core lab (Check all that apply)

- ready-to-use pCis (pCis is the transgene plasmid containing the ITRs)
- ready-to-use pTrans (pTrans is the plasmid containing Rep and Cap)
- Plasmid for cloning or amplification
- Material will not be submitted by investigator

9. Upload manuscript(s) describing the construct. (5 uploads maximum)

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

10. Amount of vector required (To denote an exponential number, use SHIFT 6 symbol. Example 2×10^{14})

11. Target concentration of vector, if known (Default minimum is 1×10^3 gc/ml)

12. Volume of aliquots requested and number of vials at that volume (minimum volume per aliquot is 100 μ l)

Volume	Number of vials
100 μ l	<input type="text"/>
200 μ l	<input type="text"/>
300 μ l	<input type="text"/>
400 μ l	<input type="text"/>
500 μ l	<input type="text"/>
600 μ l	<input type="text"/>
700 μ l	<input type="text"/>
800 μ l	<input type="text"/>
900 μ l	<input type="text"/>
1000 μ l	<input type="text"/>

13. Time frame for start of your study

14. Shipping Address

15. Do you plan to submit additional RSAs to support this study?

Yes No

RSA ID: 2042
