

# RSA for GMP Process-comparable and Clinical-grade Lentivirus Vector Production

## Section II

### LENTIVIRUS VECTOR STUDY INFORMATION

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

Do not use the forward/back browser buttons to navigate between RSAs; open a new browser window to access another RSA!

\* Denotes Required Fields

Select Vector Type you are requesting at this time:

- Full GMP-grade vector (required for clinical trials)  
 GMP process-comparable vector (for pharmacology/toxicology or other studies)

Note: '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.

Study Title (maximum 250 characters):\*

Study Abstract (maximum 4000 characters): \*

(If GMP process-comparable vector is requested, describe pharmacology/toxicology or other study and briefly describe planned clinical trial)

Describe the Rationale for Conducting the Study (maximum 2000 characters):\*

Summarize Study Endpoints (maximum 2000 characters):\*

Summarize Study Design (maximum 2000 characters):\*

Select Disease Category: \*  Heart  Lung  Blood  Other - Please specify disease\* \_\_\_\_\_

Targeted Disease:  ▼

Target organ(s), tissues, cell, etc.

Gene/Vector Name:

Route of administration:

Indicate the type of study in which this plasmid or vector will be used:

- Pharmacology/Toxicology Studies in animal model(s)
- Clinical Phase I
- Clinical Phase I/II
- Clinical Phase II
- Bridging Study
- Other \_\_\_\_\_

Attach clinical protocol document and informed consent here (Required if RSA is for full GMP-grade vector;  
If available, provide draft protocol when RSA is for GMP process-comparable vector):

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|  | Browse | Upload |
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Note: To save and upload a document, the upload button must be pressed.

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

**List of submitted RSAs**

**RSAs related to this study**

|  |        |   |
|--|--------|---|
| <i>This box displays the RSA numbers and titles that the investigator has already initiated. The investigator can select and add them to the far box using the blue Add button or remove them using that button.</i> | Add    | <i>RSAs that the investigator selects from the list on the left will appear here.</i> |
|  | Remove |   |

If you are the sole investigator and your institution is the only institution involved with this study, please indicate such by checking this box.

Sole Investigator and Institution

Co-investigator Collaboration:

List all investigators, companies/institutions that will be involved with this study (maximum 4000 characters):

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Have all of the investigators listed agreed to participate in this study and abide by GTRP Policies?

Yes  No

If no, please provide an explanation:

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Please provide letters of collaboration:

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Note: To save and upload a document, the upload button must be pressed.

**Save For Later Completion**

**Save and Continue**

**Section III**  
**LENTIVIRUS VECTOR REGULATORY INFORMATION**

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**Answer 1a or 1b below as applicable.**

**(1a) Have you discussed pharmacology/toxicology issues, or issues related to other non-clinical studies, with the FDA (pre-IND meeting or other)?**

**(Question applies to RSAs for GMP process-comparable vector.)**

Yes  No

If yes, provide the following information for one meeting at a time:

Meeting date: \_\_\_\_\_ [mm/dd/yyyy] Meeting Type:  Phone  Face-to-Face

Meeting Summary: (maximum 2000 characters)

**ADD Meeting**

Click on the Add Meeting button above; your meeting information will be displayed in a table and the fields above will be cleared allowing you to enter a second, third meeting with the FDA, if needed.

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**Upload any FDA communications and other FDA documentation here**

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Note: To save and upload a document, the upload button must be pressed.

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**(1b) Have you discussed the proposed clinical study with the FDA (formal pre-IND meeting or other)? (Question applies to RSAs for full GMP-grade vector.)**

Yes  No

If yes, provide the following information for one meeting at a time:

Meeting date: \_\_\_\_\_ [mm/dd/yyyy] Meeting Type:  Phone  Face-to-Face

Meeting Summary: (maximum 2000 characters)

**ADD Meeting**

Click on the Add Meeting button above; your meeting information will be displayed in a table and the fields above will be cleared allowing you to enter a second, third meeting with the FDA, if needed.

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**Upload any FDA communications and other FDA documentation here**

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Note: To save and upload a document, the upload button must be pressed.

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**(2) Has the IND been filed for use of this vector construct in the submitted study?  Yes  No**

**(Question applies to requests for full GMP-grade Vector only.)**

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**(3) Has the NIH Recombinant Advisory Committee (RAC) reviewed this study?**

**(Question applies to requests for full GMP-grade Vector only.)**

Yes; Provide date of review:[mm/dd/yyyy] \_\_\_\_\_  No

**Upload documentation:**

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Note: To save and upload a document, the upload button must be pressed.

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**(4) Has your Institutional Biosafety Committee (IBC) approved this study?**

Yes; Provide date of review:[mm/dd/yyyy] \_\_\_\_\_  No

**Upload documentation:**

Note: To save and upload a document, the upload button must be pressed.

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**(5a) Has your Institutional Review Board (IRB) provided provisional or final approval of the clinical protocol?**

Yes  No

If Yes,

Provisional approval date: [mm/dd/yyyy] \_\_\_\_\_

Final approval date: [mm/dd/yyyy] \_\_\_\_\_

(Question should be answered for requests for full GMP-grade vector.)

**Upload documentation:**

Note: To save and upload a document, the upload button must be pressed.

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**(5b) Is a review scheduled with the IRB?**

Yes; Date of Review: [mm/dd/yyyy] \_\_\_\_\_  No

This RSA's ID Number is \_\_\_\_\_.

**Section IV**

**STUDY-SPECIFIC FUNDING SUPPORT**

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**Do not exit your browser without saving your RSA information using one of the save buttons at the bottom of the page!**  
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**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 grants funds are allocated for vector production, please indicate the amount.
- If your request at this time is for GMP process-comparable vector please delineate funds available for vector production as well as funds available to you to conduct your pharmacology/toxicology testing using the vector.

NIH Funding (maximum 1000 characters):

NHLBI Funding (maximum 1000 characters):

Other (Institution, Industry, etc) (maximum 1000 characters):

No Funding Secured

This RSA's ID Number is \_\_\_\_\_.

## Section V

### LENTIVIRUS VECTOR REQUEST INFORMATION

**Do not exit your browser without saving your RSA information using one of the save buttons at the bottom of the page!**  
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1. Provide vector product name:

2. Provide preclinical or background data on the Lentiviral construct and activity if not described elsewhere in this application.

 

Note: To save and upload a document, the Upload button must be pressed. (maximum of 3 uploads)

3. Prepare a Word document to address the following questions and upload it: What packaging plasmids and packaging cell line are used; what is the method of production; what media was used in generating vector; what is the titer of unconcentrated vector; what is the method of concentration; what media is used in concentration; what is the final titer and yield (total # infectious units after concentration/total # infectious units before concentration):

 

Note: To save and upload a document, the Upload button must be pressed. (maximum of 3 uploads)

4. Provide methods of assessing gene transfer and efficacy (maximum 2000 characters):

5. Is the transgene vector being submitted for production the same as the one used in preclinical work?

Yes  No

If no, are bridging studies planned?

Yes  No

Describe (maximum 2000 characters):

6. Has transgene vector been sequenced?

Yes  No

If yes, please submit sequence data and method used.

 

Note: To save and upload a document, the Upload button must be pressed. (maximum of 3 uploads)

7. Promoter driving the transgene expression (maximum 500 characters):

8. Will you be supplying the packaging plasmids?

Yes  No

If so, which ones (maximum 500 characters):

9. Please submit sequence data and method used for packaging plasmids:

 

Note: To save and upload a document, the Upload button must be pressed. (maximum of 3 uploads)

10. Viral Envelope:

VSVG  Other \_\_\_\_\_

11. Any known toxicities (maximum 2000 characters):

12. Paper or manuscript describing the transgene/promoter, if available:

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Note: To save and upload a document, the Upload button must be pressed. (maximum of 3 uploads)

13. Identify the originator of the vector and the packaging plasmids/packing cell line (maximum 2000 characters):

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14. Are there any legal or contractual constraints on the use of the vector and the packaging plasmids/packaging cell line?

Yes  No

If yes, please explain (maximum 250 characters):

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**Pharmacology / Toxicology Testing** (question applies to RSAs for full GMP-grade vector):

15. Has Pharmacology and safety testing been completed?  Yes  No  Not Applicable

If Yes,

Provide summary of pharmacology and safety testing data

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Note: To save and upload a document, the Upload button must be pressed. (maximum of 3 uploads)

If No,

Do you plan to have the pharmacology and safety testing done by the GTRP Pharmacology-Toxicology Laboratory?

Yes  No

Note to Applicant Investigator: *You must submit a separate RSA for Pharmacology-Toxicology testing.*

**Vector Production Requirements:**

16. What formulation is required for the final product? (maximum 500 characters)

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17. Have specific release criteria been determined for the final product (e.g., Endotoxin limits, residual DNA)?

Yes  No

Describe (maximum 2000 characters):

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18. Estimated amount of vector needed for pharmacology and safety testing and other studies (total particles and infectious titer/mL):  
To denote an exponential number, use SHIFT 6 symbol. Example:  $2 \times 10^{14}$

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19. Estimate GMP process-comparable as well as full GMP vector needs, delineated separately, for preclinical development/"dry runs"  
(maximum 500 characters)

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20. Estimate vector needs based on anticipated accrual and dose schedule in the clinical study (maximum 500 characters)

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21. Considering regulatory and all other implementation issues, when do you expect the clinical study to open to enrollment?

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22. Comments (maximum 1000 characters):

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23. Describe future vector production needs for this study (2000 characters):

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**Save and Submit Preliminary RSA**

**Submit Final RSA**

This RSA's ID Number is \_\_\_\_\_