



The National Institute for Innovation in Manufacturing Biopharmaceuticals

Next-Generation Sequencing (NGS)
Method Development Study
Request for Information 2023.1
(RFI 2023.1)

RFI Release Date: August 10th, 2023

Submission due: September 05, 2023 (5pm EST)



1. Overview

The mission of the National Institute for Innovation in Manufacturing Biopharmaceuticals (NIIMBL) is to accelerate biopharmaceutical manufacturing innovation, support the development of standards that enable more efficient and rapid manufacturing capabilities, and educate and train a world-leading biopharmaceutical manufacturing workforce. NIIMBL is excited to announce the Request for Information (RFI) for participation in a Method Development Study for Characterization of AAV vectors by Next Generation Sequencing. This document provides comprehensive submission details for interested parties.

2. Request for Information

- **Background**

NIIMBL invites interested parties to submit a response to this RFI intended to lead to a method development study aimed at developing and optimizing Next Generation Sequencing (NGS) methods for the comprehensive characterization of Adeno-Associated Virus (AAV) gene therapy vectors. By fostering collaboration among various laboratories and industry partners, NIIMBL aims to enhance the reliability, consistency, and understanding of AAV gene therapy vector properties using NGS-based analysis methods in future studies.

Through this RFI, NIIMBL aims to gauge the interest and willingness of the community to participate in a planned future NGS study. Responses will play a pivotal role in helping NIIMBL shape the future of AAV vector characterization and revolutionize biopharmaceutical manufacturing. We encourage interested and capable parties to join this critical endeavor and become an integral part of driving innovation forward.

NIIMBL expects to release a Request for Application (RFA) for funding to a subset of organizations that have responded to the RFI based on capability assessments from the RFI responses. **Note** NIIMBL may choose not to issue an RFA on this topic, RFI2023.1 is not a solicitation for funding.

- **Description**

There is a pressing need for reproducible quality control assays for AAV-based gene therapy vectors. Next Generation Sequencing (NGS) holds significant promise for confirming viral genome integrity and detecting contaminating DNA. The overarching objective of this NGS investigation is to develop a robust and consistent NGS-based AAV characterization method for implementation within the Viral Vector (VV) Program.

- **Submission Details**

Interested parties are requested to submit a brief description (3 pages or less) detailing the following:

1. Technical Approach: Describe your technical approach to develop an NGS-based method for AAV vector characterization. Include pre-treatments, extraction, library preparation, sequencing, and analysis details. Specify the expected readout of the method and how it will be used to characterize AAV vectors. Link the technical approach to the tasks and milestones outlined in a Gantt chart. Include success criteria and evaluation metrics for measuring project success, ensuring deliverables are specific and quantitative where possible.



2. Description of Capabilities: Highlight the capabilities of your organization that enable the execution of the technical approach, including personnel, instrumentation, and facilities. Identify a technical lead and any other senior/key personnel playing leadership roles in a future possible project. Discuss how the team will maintain timelines, stay within budget, and make decisions.
3. Description of Outputs: Provide brief answers to the following questions:
 - a) What would the team willing and able to share or make available to the NIIMBL Viral Vector Program, including government, industry, and academic stakeholders?
 - b) Would the team consider participation in a future interlaboratory study using a common protocol and in-process materials?
 - c) What concerns or constraints does the team have about sharing protocols, data, results, methods, approaches, etc.?
4. Gantt Chart: Present a Gantt chart (monthly interval) outlining the proposed timelines for completing the project's key milestones and tasks. The Gantt chart is not included in the 3-page limit.
5. Price: Provide a price estimate for the proposed technical approach, including a breakdown of expenses. In developing a price estimate, assume that NIIMBL will provide vectors required for the study. Price estimate is not included in the 3-page limit.

- **Submission Process**

RFI submission packages must be submitted via the NIIMBL submission hub. Submissions received after the deadline will not be considered. RFI submissions should be marked as "NIIMBL Confidential," in accordance with the NIIMBL Bylaws, limiting access to NIIMBL members or Federal representatives.

- **Summary**

NIIMBL encourages enthusiastic participation in this RFI for the Method Development Study for Characterization of AAV vectors by Next Generation Sequencing. Your contributions will be invaluable in advancing biopharmaceutical manufacturing and furthering the understanding of AAV gene therapy vectors. Let us collaborate to drive innovation and transform the future of biopharmaceuticals.

3. Abbreviated List of Acronyms

1. RFI: Request for Information
2. RFA: Request for Application
3. VV: Viral Vector Program
4. ST: Steering Team
5. IP: Intellectual Property
6. NIIMBL: National Institute for Innovation in Manufacturing Biopharmaceuticals
7. PI: Principal Investigator
8. NGS: Next Generation Sequencing
9. AAV: Adeno-Associated Virus