Seeking novel applications for our lung-selective gene therapy vector (AAV2-L1)

Overview

Boehringer Ingelheim invites scientists to submit proposals for novel gene therapy concepts in pulmonary diseases and especially those that could be enabled by our unique lung-specific vector (AAV2-L1).*

Partners would gain privileged access to our unique lung-specific gene therapy vector and collaborate with Boehringer Ingelheim scientists who have cutting-edge gene therapy and drug discovery expertise. Moreover, funding requests up to €200.000 can be submitted as part of the proposal and will be considered for selected projects.

The opportunity bears the potential for presentation at research conferences and high impact publications in refereed journals.

Please share initially only non-confidential data with your proposal. All incoming proposals will at first be evaluated by representatives who are independent from Boehringer Ingelheim scientists engaged in ongoing internal projects. Successful proposals will only be approved if they do not conflict with internal projects.

Boehringer Ingelheim believes that this unique gene therapy vector has the potential to enable many gene therapy concepts for pulmonary diseases and, therefore, seeks to work collaboratively with scientists who are motivated to bring new gene therapy treatments to patients.

Submissions for collaborations can only be considered if they arrive no later than April 12, 2019, 23.59 pm PST.

*It is the proposer's responsibility to confirm that it is possible to import AAV constructs from Germany prior to submitting their proposal.

Summary

Boehringer Ingelheim invites proposals for novel applications of its lung-specific gene therapy vector in pulmonary diseases. We are looking for innovative and testable hypotheses, combining therapeutic gene constructs with pulmonary indications of high medical need and the AAV2-L1 vector. Successful applicants will be provided with their gene constructs packaged in the AAV2-L1 vector; moreover, they will receive appropriate funding and collaborate with our gene therapy experts.

Background

Achieving efficient, targeted and durable gene delivery is a major hurdle to realizing the full therapeutic potential of gene therapy. Adeno-associated viral (AAV) vectors have become an important platform for in vivo gene therapy based on their safety profile and ability to transduce a broad range of target tissues[1]. There are an increasing number of clinical trials utilizing AAVs and the first AAV-based gene therapy has recently received FDA approval[2].

Boehringer Ingelheim has the exclusive rights for the therapeutic application of the lung-specific AAV2-L1 gene therapy vector for pulmonary diseases. We invite applications to collaborate with us on new therapeutic concepts and thereby bring innovative medicines to patients.

AAV2-L1 was identified by a NGS-guided *in vivo* selection of AAV2-dervied capsid variants with a lung tropism and high transduction efficiency[3]. This tropism is conferred by displaying a hepta-peptide (ESGHGYF) on the AAV2 capsid surface. Specifically, AAV2-ESGHGYF (AAV2-L1) is selectively taken-up by the lung where it transduces mainly pulmonary vascular endothelial cells and pneumocytes by a currently undefined receptor. There is a »100-fold tropism for lung when compared to the wild-type AAV2 14 days after intravenous administration to mice; thereafter, transduction is maintained for at least 244 days. This vector is suited to the *in vivo* delivery of a gene construct, up to 4.7 kb (incl. promoter and regulatory elements), to the lungs of mice to test therapeutic hypotheses.

Your gene construct will be packaged in the AAV2-L1 vector and provided free of charge in the amount required for the experiments (in rodents).

Boehringer Ingelheim believes that this unique gene therapy vector has the potential to enable many gene therapy concepts for pulmonary diseases and, therefore, seeks to work collaboratively with scientists who are motivated to bring new gene therapy treatments to patients.

References

1. Colella P, Ronzitti G, Mingozzi F Emerging Issues in AAV-Mediated In Vivo Gene Therapy. Mol Ther Methods Clin Dev. 2018 Mar 16; 8: 87–104..doi: 10.1016/j.omtm.2017.11.007
2. https://www.fda.gov/newsevents/newsroom/pressannouncements/ucm589467.htm

3. Körbelin J, Sieber T, Michelfelder S, Lunding L, Spies E, Hunger A, Alawi M, Rapti K, Indenbirken D, Müller OJ, Pasqualini R, Arap W, Kleinschmidt JA, Trepel M. Pulmonary Targeting of Adeno-associated Viral Vectors by Next-generation Sequencing-guided Screening of Random Capsid Displayed Peptide Libraries. Mol Ther. 2016 Jun;24(6):1050-1061. doi: 10.1038/mt.2016.62.

Key Success Criteria

Boehringer Ingelheim is seeking proposals that have:

- Innovative ideas, backed with a compelling scientific rationale, for the use of a lung-specific gene therapy vector (AAV2-L1) for pulmonary diseases with a high unmet medical need
- A therapeutic gene construct (up to 4.7 kb incl. promoter and regulatory elements) and a target pulmonary indication
- A novel, testable working hypothesis distinct from those previously published
- Research plan detailing an in vivo proof of concept (PoC) study in rodents.

Additional key success criteria are:

- The quality and feasibility of the existing data and/or the experimental plan that will be used to test the hypothesis
- The experimental endpoints and how well these can be translated to human disease

If confidential data exists that would strengthen the proposal, the solution provider may indicate that confidential information is available to share under a Confidential Disclosure Agreement (CDA). If Boehringer Ingelheim finds the non-confidential concept proposal sufficiently interesting, they will execute a CDA for confidential discussions.

Possible Approaches

Our Boehringer Ingelheim team is open to all proposals that can fully or partially meet their requirements. Funding of up to €200.000 may be available for selected projects upon request and support requirements should be outlined in the submitted proposal. Collaborating scientists will benefit from direct access to Boehringer Ingelheim's drug discovery and validation capabilities.

Anticipated Project Phases or Project Plan

Phase 1 – Review of Proposals by beginning of May 2019
Phase 2 – Collaborations starting Q3/4 2019

Submitting a collaboration proposal

- Click the “Collaborate” button at the top of the page.
- Log in, or register for *opnMe.com* (you will be prompted).
- Follow the easy online three-step-process to generate and upload your submission document
    - Download proposal template for collaboration (recommended)
- Upload your proposal and attach additional files of information if you want to.
- Click “Continue to next step”