

Cor-Edit-P

Cardiac open reading frame edition to study cardiomyopathies in pigs

Call: ERC-2020-AdG

Project Reference: 101021043

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Host Institution: Klinikum rechts der Isar, Technical University Munich

Description:

Heart failure represents a common cause of death in European societies and is frequently based on dilated cardiomyopathy (DCM) which might be caused by mutations in cardiomyocyte genes. While no specific treatment exists, new therapeutic options are a major unmet clinical need. As attractive novel key approach, Cor-Edit-P will use Crispr-Cas9 based gene editing for distinct gene therapy of genetic cardiomyopathy, using pigs as a unique, clinic-related large animal model system. Highly cardiotropic adeno-associated viral (AAV) vectors will be used in pigs *in vivo*, applying the precise, reliable and versatile Cas9 technology. Pioneering this approach, we were able to restore significant dystrophin expression in muscles and hearts of pigs suffering from Duchenne muscle dystrophy. Exploiting unique and cutting-edge technology, **Cor-edit-P aims at specifically eliminating the underlying cause of genetic DCM to improve cardiac function, reduce the risk of deadly arrhythmias and increase span and quality of life.**

Cor-edit-P will

- generate currently lacking porcine models of genetic cardiomyopathy, using AAV-Cas9 to induce mutations in sarcomere genes, e.g. titin (TTN) and β -myosin heavy chain (MYH7);
- exercise curative Crispr-Cas9 mediated gene editing of DCM in pigs *in vivo*, using the PLNR14del mutation in the phospholamban (PLN) gene as prominent example;
- use human patient-derived PLN-R14del ventricular progenitor cells for gene correction *ex vivo* followed by transplantation of corrected cells into PLN-R14del pigs.

More information

[The project in CORDIS](https://cordis.europa.eu/project/id/101021043)

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Project details

Start Date: 2022-01-01

End Date: 2026-12-31

EU Contribution: EUR 2 499 375

Total Costs: EUR 2 499 375

Funding Scheme:

ERC-ADG - Advanced Grant

Topic:

ERC-2020-ADG - ERC ADVANCED GRANT

Funding :

H2020-EU.1.1. EXCELLENT SCIENCE



European Research Council
Established by the European Commission