Title (en)

ENGINEERING OF A MINIMAL SACAS9 CRISPR/CAS SYSTEM FOR GENE EDITING AND TRANSCRIPTIONAL REGULATION OPTIMIZED BY ENHANCED GUIDE RNA

Title (de)

KONSTRUKTION EINES DURCH ERWEITERTE GUIDE-RNA OPTIMIERTEN MINIMALEN SACAS9-CRISPR/CAS-SYSTEMS ZUR GENEDITIERUNG UND TRANSKRIPTIONSREGULIERUNG

Title (fr)

INGÉNIERIE D'UN SYSTÈME CRISPR/CAS MINIMAL SACAS9 POUR L'ÉDITION DE GÈNES ET LA RÉGULATION TRANSCRIPTIONNELLE OPTIMISÉE PAR UN ARN GUIDE AMÉLIORÉ

Publication

EP 3625338 A1 20200325 (EN)

Application

EP 17910146 A 20170519

Priority

CN 2017085202 W 20170519

Abstract (en)

[origin: WO2018209712A1] Programmable and precise regulation of Cas9 functions by utilizing a set of compact Cas9 derivatives created by deleting conserved HNH and/or REC-C domains based on the structural information across variant class 2 CRISPR effectors is provided. A novel strategy for engineering the dimeric gRNA-guided nuclease by splitting the mini-dSaCas9 and fusing the FokI domain right after the split point to increase the on-target DNA cleavage efficiency and potentially reduce the off-target effect because of a closer proximity of dimeric FokI nuclease to the target sequence is also provided. By combining the optimized and compact gRNA expression cassette and the downsized SaCas9 derivatives, the entire CRISPR/Cas system with different effector domains for transactivation, DNA cleavage and base editing is loaded into a single AAV virus. Such an all-in-one AAV-CRISPR/Cas9 system will be particularly appealing in biomedical applications that require safe and efficient delivery in vivo.

IPC 8 full level

C12N 9/22 (2006.01); **C12N 15/115** (2010.01)

CPC (source: EP)

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