

Title (en)

TARGETED TRANSFER RNAS FOR TREATMENT OF DISEASES

Title (de)

GEZIELTE TRANSFER-RNAS ZUR BEHANDLUNG VON KRANKHEITEN

Title (fr)

ARN DE TRANSFERT CIBLÉS POUR LE TRAITEMENT DE MALADIES

Publication

**EP 4069841 A1 20221012 (EN)**

Application

**EP 20829427 A 20201201**

Priority

- US 201962942690 P 20191202
- US 201962942667 P 20191202
- US 202063010856 P 20200416
- US 202063111856 P 20201110
- US 2020062671 W 20201201

Abstract (en)

[origin: WO2021113218A1] Provided are engineered tRNA molecules and vectors encoding engineered suppressor tRNA molecules that recognize and readthrough of disease-causing premature stop codons. Pharmaceutical formulations of the engineered tRNA or vectors encoding the engineered tRNA are provided for use in treating diseases associated with truncation of a protein caused by premature stop codons present in the nucleic acid sequence encoding the protein.

IPC 8 full level

**A61K 31/7088** (2006.01); **A61K 48/00** (2006.01); **C12N 15/11** (2006.01)

CPC (source: EP)

**A61K 31/7088** (2013.01); **A61K 31/712** (2013.01); **C12N 15/11** (2013.01); **C12N 15/85** (2013.01); **C12N 15/86** (2013.01); **C12N 2310/51** (2013.01); **C12N 2320/51** (2013.01); **C12N 2750/14143** (2013.01)

Citation (search report)

See references of WO 2021113218A1

Designated contracting state (EPC)

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Designated extension state (EPC)

BA ME

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