CRISPR-Cas system for gene therapy

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Invention

The invention refers to the use of an engineered 'Clustered Regularly Interspersed Short Palindromic Repeat (CRISPR)-CRISPR associated (Cas)' (CRISPR-Cas) technology targeting a sequence mutated at one or more loci in the genome of a given eukaryotic cell. The aim of this invention is to achieve a definitive and decisive treatment of four monogenic diseases resulting from mutations in a single gene, specifically Parkinson's disease, Rett syndrome, Pompe disease and Alport syndrome.

To date, there are no curative treatments available for the above diseases, only therapeutic approaches based on symptomatic treatment of patients with the aim of improving both, quality of life and life expectancy. Furthermore, the cells in which these gene mutations are relevant are notoriously difficult to modify, being either no longer dividing or protected.

In this context, the technology of the invention is specifically modelled to deliver the CRISPR-Cas9 system into such target cells and replace the mutated alleles with a copy of the non-mutated gene by homologous recombination.

Drawings
& pictures

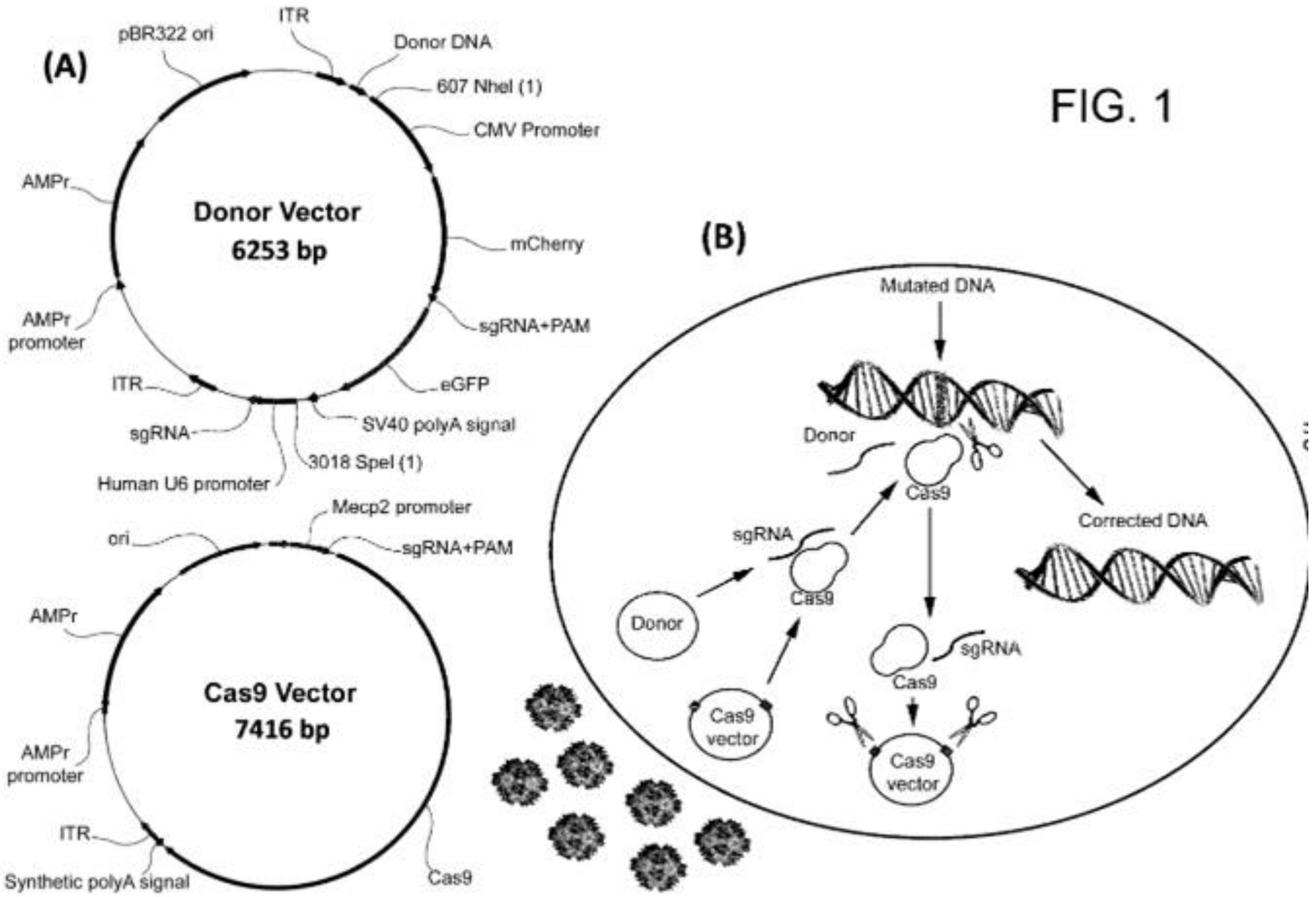
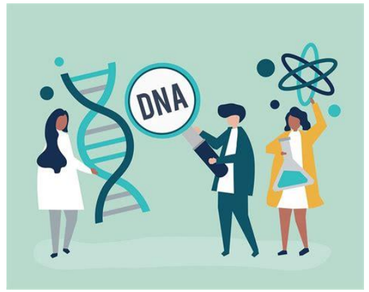


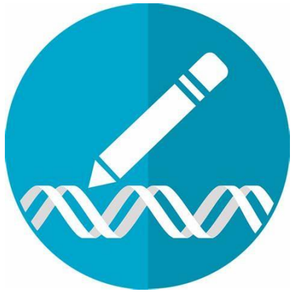
FIG. 1

Industrial applications

The technology may be of interest to biotechnology and pharmaceutical companies having (or wishing to expand to) ATMPs -Advanced Therapy Medicinal Products- in their pipeline, as well as in hospitals, health care facilities or health service structures working in personalised medicine.

In particular, the invention applies to mutations in the genome affecting organs whose cells are fully differentiated and therefore no longer dividing, such as the central nervous system (neurons, in the case of Rett syndrome) and kidneys (Alport syndrome).





Possible developments

Currently evaluated at a TRL of 3, the technology may be further developed within specific technology maturation projects aimed at raising the bar, particularly with regard to safety and hospital applicability.

The group is looking for industrial partners operating in the field of Advanced Therapy Medicinal Products interested in collaborating on the aforementioned technological maturation of the invention.

A possible project aimed at raising the TRL to 4 may be based on the large-scale study of the approach in pathology-relevant cell models (e.g. neurons for Rett and kidney cells for Alport) derived from patient cells in order to validate the safety and efficacy of the approach on a large number of cases.

The University of Siena is open to specific agreements to exploit, license or option the patented invention.

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