GENE THERAPY

Novel Regulated Promoter for Gene Therapy

Applicant	Università degli Studi Padova,
Inventors	Alessandra Biffi, Valentina Poletti
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What it is needed for?

Neuroinflammation, characterized by microglial cell-activation, plays a major role in the pathogenesis and progression of multiple neurodegenerative diseases, such as Lysosomal Storage Disorders and Alzheimer's, severe human conditions currently lacking an effective treatment.

***Ex vivo* gene therapy** based on genetically modified hematopoietic stem cells (HSCs) approach shows great promise, yet the beneficial effect of it relies on CNS-associated microglia-like cells derived from the transplanted HSCs due to their role in neuroinflammation reduction after treatment.

To increase the efficacy of such therapeutic strategy, **we have developed a proprietary synthetic promoter** for a gene transfer vector that allows **for a controlled** –low basal levels that are significantly increased upon inflammation– **expression of therapeutic transgenes in immune-like cells**.

The inducible promoter sequence is validated both *in vitro* and *in vivo*.

Advantages

- Compact size, suitable for a lentiviral system;
- Allows for regulated expression of therapeutic protein of interest: basal levels under homeostatic conditions and increased levels upon neuroinflammation.

Applications

Cell and gene therapy approaches to treat pathological conditions of the CNS, particularly those characterized by neuroinflammation, such as Lysosomal Storage Disorders, Alzheimer's and ALS.

What we are looking for

The technology is available for licensing and/or co-development

TRL scale

Discovery | **Lead Optimization** | **Preclinical** | **Clinical Phases**

Health and Biomedical

patent@unismart.it