NEURODEGENERATIVE DISORDERS

# Therapeutic factors for the treatment of PolyQ diseases

| | |
|---|---|
| **Applicant** | Università degli Studi Padova, Fondazione Telethon, Universität Wien |
| **Inventors** | Martello Graziano, Ferlazzo Giorgia Maria, Gambetta Anna Maria, Leeb Martin |
| **Priority Date** | 11/02/2022 |
| **Protection** | IT 102022000002498<br>PCT/IB2023/051156 |

## What we are looking for

We are looking for risk capital, mentorship and partners for co-development

## What it is needed for?

The polyglutamine (polyQ) diseases are a group of **neurodegenerative disorders** caused by expanded cytosine-adenine-guanine (CAG) repeats encoding a long polyQ tract in the respective proteins. To date, at least nine polyQ disorders have been described, see bellow for a list. Among them **Huntington's disease** (HD) is the most widespread.

Currently, the standard of care of such disorders is focused on symptomatic treatments as **no effective therapeutic interventions aimed at the treatment or prevention of polyQ diseases exist**. This calls for an urgent need of innovative therapeutic strategies.

**We have identified a number of novel therapeutic factors capable of reducing the toxicity of mutated proteins causing polyQ diseases**. Their administration to both *in vitro* and *in vivo* model systems of HD via state-of-the-art gene therapy delivery systems resulted in amelioration of the detrimental effects of the mutant HTT gene, *i.e.*, in reduced cell death, oxidative stress, transcriptional alterations, and alleviated motor deficit.

## Advantages

- Patented platform technology;
- Applicable for several other diseases (versatility and scalability);
- No specific treatment for polyQ disorders exist;
- Eligible for Orphan Drug Designation.

## Applications

A treatment for polyQ diseases, namely Huntington's disease; Spinocerebellar ataxia types 1, 2, 6, 7, 17; Machado-Joseph disease; Dentatorubral pallidoluysian atrophy; and Spinal and bulbar muscular atrophy, X-linked 1

## TRL scale


Health and biomedical - Pharmaceuticals