Glaucoma is a chronic, neurodegenerative disease that is associated with damage to retinal ganglion cells and central parts of the visual pathway. Untreated glaucoma can lead to total and irreversible blindness. The World Health Organization recognizes glaucoma as a civilization disease, the major cause of irreversible blindness worldwide. Its hidden course causes that at the time of diagnosis it is usually the cause of significant visual impairment. The wiliness of glaucoma is associated primarily with the fact that in the early stages it is asymptomatic, causing a gradual loss of peripheral part of the visual field. The disease is beginning to be noticeable when the defect of the visual field is so significant that it affects the daily functioning of the patients.

It is estimated that around 70 million people worldwide suffer from glaucoma, which accounts for around 1-2% of the world population. Approximately 7.5 million new cases occur each year and the total number of glaucoma patients can reach 76 million in 2020 and even 112 million in 2040. The cause of a significant increase in the prevalence of this disease is the global aging process and civilization diseases - primarily diabetes and cardiovascular disease. In Poland, the problem of glaucoma is estimated to be about 700,000 people, of which even half might not know about their illness.

In the European population, about 70% of cases of glaucoma are associated with elevated intraocular pressure – the most important, measurable risk factor. The current glaucoma therapy is based primarily on various methods of lowering intraocular pressure. However, the progression of vision loss continues to persist in some patients despite the use of maximum doses of intraocular pressure lowering medications. Nowadays, there are no neuroprotective therapies (ie. protecting neurons, such as retinal ganglion cells, from dying) with proven efficacy for glaucoma. For this reason, the search for such therapies to inhibit retinal ganglion cell death and stimulate their regeneration (ie. renewal) is an important and current problem.

The aim of our project is to develop an innovative experimental gene therapy for the neuroprotection of retinal ganglion cells in glaucoma. Based on our previous observations as a target of our therapy we have chosen the regulatory protein present in retinal ganglion cells called HuR. This protein is crucial element in regulating the response of cells to damaging factors, including elevated intraocular pressure. Under experimental conditions, in experimental glaucoma in animals as well as in glaucoma patients we showed that the HuR protein content in the retinal neurons is significantly lowered. Therefore, we plan to experimentally increase the HuR protein content in retinal ganglion cells by gene therapy approach in experimentally induced glaucoma in rats associated with high intraocular pressure. As primary steps leading up to appropriate animal experiments, we are planning a preliminary evaluation of the efficacy and safety of our gene therapy in cell culture and tissue culture using rat retinal explants.

In our study, we expect to see a positive, protective effect of applied gene therapy expressed in slowing down the progression of glaucoma in animals irrespective of intraocular pressure values. The gene therapy we propose could became the basis for further research to create analogue therapy for humans as a complement to the current treatment of intraocular pressure lowering, thereby changing the idea of glaucoma treatment.