Our team's goal is to discover, test and develop treatments in order to prevent or limit visual impairment and to improve the autonomy and the quality of life of patients with Wolfram syndrome.

For the last 20 years, our group together with Pr Christian Hamel has made highly significant contributions concerning the clinics, genetics and pathophysiology of autosomal inherited optic neuropathies, by identifying the genes involved in these diseases, analyzing mouse models reproducing human pathologic mutations, deciphering the basic function of the uncharacterized genes and start therapy projects in this field.

Wolfram syndrome is a devastating multisystemic disorder and despite decades of intense research, no curative therapies are currently available. All aspects of this disease reinforce our commitment to elaborate a therapeutic strategy for Wolfram patients.
Our team are working on two axes:

1) Development and testing of new therapeutic drugs
2) Use gene therapy delivering WFS1 to treat Wolfram syndrome

Recently we have identified a family of molecules with the capacity to stimulate significantly the growth of retinal ganglion cells *in vitro* in a model of optic atrophy. These molecules appear to represent interesting therapeutic candidates for the disease. Our project is to test the efficacy of these molecules in a previously established mouse model of Wolfram syndrome.

Gene therapy has exciting potential. For several reasons, gene therapy will have considerable therapeutic potential in this monogenic disorder. In our first gene therapy studies in a mouse model of Wolfram syndrome, we have demonstrated that it is possible to rescue visual function using overexpression of WFS1 when it is administrated into the vitreous. To go further, we hypothesized that a gene therapy of WFS1 with an effect on the whole body could restore WFS1 expression and function in both retinal ganglion cells and other organs. We would like to evaluate functional evidence that WFS1 expression by gene transfer in all the body in a Wolfram model greatly mitigates the development of the phenotype.

Our projects represent a first step in acquiring the proof of principle necessary for carrying out clinical trial in human. Our link with the reference center for genetic sensory diseases Maolya in Montpellier will help us to improve preclinical to clinical translation.