### **Gyrate atrophy: Development of a new treatment to prevent blindness in children**

Gyrate atrophy (GA) is a metabolic disorder in which a certain substance (ornithine) cannot be converted into another substance (proline) in the body. The accumulation of ornithine is poisonous, especially for the nerve cells of the eye, and leads to gradual loss of vision and eventually complete blindness. The current treatment with an ornithine restricted diet is very difficult to sustain and also not sufficiently effective. In a number of steps we will develop a treatment that can really prevent this blindness and that is also well tolerated. Our multidisciplinary team uses the latest imaging, (stem)cellular and molecular techniques to gain more insight into the cause and course of gyrate atrophy (GA), and how this disease can be treated. In GA, the nerves are affected, and in particular, and in the first instance, the so-called RPE layer. The RPE nerve layer works together with other nerve cell layers from the retina, such as rods and cones, which together cause blindness in GA. We will mimic the cultured nerve cell layer from the retina (RPE) of GA patients and of healthy control persons and then analyze and compare them. We will unravel disease mechanisms and thereby develop rational therapy on cultured (GA) retinas to test them in a GA mouse model: proof-of concept. This research model and its outcomes are important for therapy development for other metabolic diseases and genetic eye disorders with retinal abnormalities. Our vision is to help as many affected families as possible, by preventing blindness through state-of-the art research.