

Safe treatment for rare eye disease

Acanthamoeba Keratitis

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The ODAK ('Orphan Drug for Acanthamoeba Keratitis') project is working to develop a safe and effective treatment for Acanthamoeba keratitis (AK), a rare infectious eye disease which causes severe debilitation and can lead to blindness. The disease, which causes severe debilitation and can lead to blindness, affects one in 100 000 people in the EU - with 85% of cases being associated with contact lens wearing. It is caused by a common protozoan infecting the cornea and it is extremely difficult to treat because of the pathogens' resistance to antimicrobial therapy.

Aiming to fill the current gap in safe, effective and approved drugs to treat AK, the ODAK project will undertake preclinical and clinical research on the Orphan Drug Polihexanide (PHMB). 'Orphan drugs' are medicinal products intended for the diagnosis, prevention or treatment of life-threatening or debilitating rare diseases like AK. They are 'orphans' because the pharmaceutical industry has less interest under normal market conditions in developing them as they are intended for only a small number of patients suffering from very rare conditions. As such, the work of ODAK on PHMB is particularly vital - already the use of this drug

has been shown to greatly improve treatment outcomes for AK, especially when used in the early stages of the disease.

The ODAK consortium of five European companies and one university is currently engaged in preclinical trials, with clinical trials due to start in 2015. Through these, the project team hopes to identify optimal PHMB formulations and recommend the best dose-benefit treatment regimes. Professor John Dart, leading Ophthalmologist at Moorfields Eye Hospital London, notes, 'Acanthamoeba keratitis is an uncommon disease but life changing for most individuals affected. There is currently no approved, licensed treatment for this condition. The EU is to be commended for making this important investment into the development of a safe and effective medical therapy for the condition'. AK, which was extremely rare before the widespread use of contact lenses in the 1980s, produces symptoms such as severe eye pain, eye redness, blurred vision, light sensitivity, eye irritation and excessive tearing. The diagnosis of the disease is problematic as symptoms are often mistaken for bacterial or herpes virus infections, and this leads to delayed treatment. As with many conditions, early diagnosis and treatment is a major factor in achieving good treatment outcomes.

AK is one of the many existing rare diseases (RD) for which research into treatments is badly needed. Many patients with RD still lack a proper diagnosis and most of them are left without an [effective treatment](#). Research into RD is also very relevant from a scientific point of view as RD are model diseases for more common disorders and are strong drivers of innovation. The ODAK project is one of many EU-funded research projects supporting the goal of the International Rare Disease Research Consortium (IRDiRC) to provide 200 new therapies for rare diseases by 2020. SUPPORT-IRDiRC, for example, is working to directly support the IRDiRC in its mission to

coordinate and foster international collaborative research on RD. Meanwhile the harmonisation efforts of RD-CONNECT, an integrated platform connecting registries, biobanks and clinical bioinformatics for [rare disease](#) research, will be one of the primary enablers of progress in IRDiRC.

More information:

www.odak-project.eu/index.php/en/

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