

# ODAK—Orphan Drug for Acanthamoeba Keratitis

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## The disease

Acanthamoeba Keratitis (AK) is a rare infectious eye disease prevalent in contact lens wearers (over 85% of AK cases) resulting from exposure to Acanthamoeba Spp, a ubiquitous free living organism. Symptoms can include severe eye pain, light sensitivity, vision loss, redness and in extreme cases resulting in corneal transplant and enucleation. There is currently no licensed



## Project outline

This FP7 funded project brings together a European collaboration of industry and academia undertaking research and technological development of PHMB (Polihexanide), conducting Phase I and Phase III clinical trials, and applying for marketing authorisations aiming to provide the first approved ophthalmic intervention for the safe and effective treatment of the rare ocular disease Acanthamoeba Keratitis.

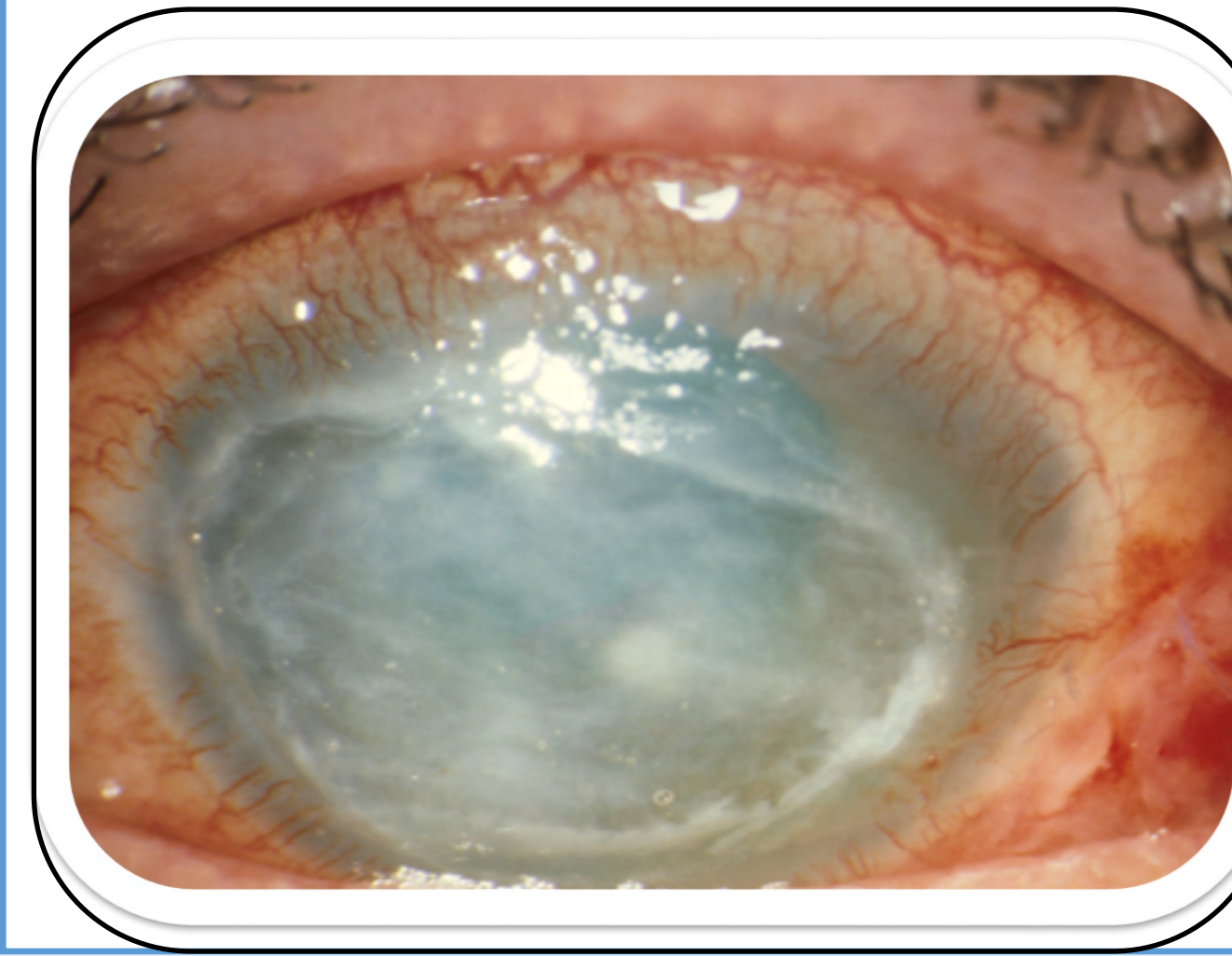


Fig.1 (left) Contact lens wearer. Fig. 2 (right) Acanthamoeba keratitis in late diagnosed patient. (from Prof John Dart)

## Results

- Retrospective study undertaken: PHMB 0.02% alone or in combination with other anti amoebic drugs has shown efficacy (Papa et al., IOVS 2015).
- Non-clinical studies (pharmacodynamics, pharmacokinetics and toxicology) on novel formulations of PHMB.
- In vitro and in vivo efficacy tests showed 0.02% PHMB as the least effective formulation against Acanthamoeba polyphaga, with respect to other tested PHMB concentrations (0.04%, 0.06%, 0.08%).
- Safety ocular profile of three PHMB formulations (0.08%, 0.25% and 0.8%) were assessed in an animal model indicating no relevant treatment-related effects of PHMB ophthalmic solutions at 0.08% and 0.25%. Only 0.8% PHMB eye drops showed moderate/severe ocular toxicity.
- Toxicological data supported the plan to investigate PHMB 0.04%, 0.06%, 0.08% (and placebo) eye drops in a Phase I clinical trial. The trial was held at three clinical centres in the Netherlands and Belgium on 90 healthy volunteers and completed in March 2016.
- A Phase III study in patients will follow to show that PHMB eye drops as a monotherapy at concentrations higher than 0.02% is effective and safe for the treatment of Acanthamoeba keratitis.

## Patient involvement in rare infectious diseases

### Challenges

- No established patient group—infection unpredictable
- Disease outcomes vastly differ patient to patient
- Difficult to engage patients once disease progression is resolved
- Lack of patient centric information
- No patient registry for patient recruitment
- Patients isolated lacking a forum to translate experience

### Benefits of patient involvement

- Patient stories providing reassurance and reducing feelings of isolation
- Contribution to preparing patient information leaflets and assisting in protocol design
- Providing information and sharing experiences for preventative care and advice
- Developing support materials for newly diagnosed