Delivering industry research in advanced therapies for rare diseases in ophthalmology

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Purpose
The overall purpose was to increase interactions with and foster trials in ophthalmology in the rare disease advanced cell & gene therapy space.

Method
• The first step was to actively engage with and build links with biotech and pharmaceutical companies scoping cell and gene therapy applications and trial readiness across a range of ocular genetic rare diseases.

• The second step was to actively bid for clinical trials in this space.

• The third step was to make use of a Cardiff University NMHII secondment to a commercial partner to initiate a two-way process of knowledge sharing, collaboration and development of opportunities and ideas.

Results
In 12 months:
Step 1- scoping and network building with 6 companies.

Step 2- 4 trials- publications arising from two studies currently under review in major neurology journal, one study recruiting to target, one study bidding for site selection complete.

Step 3- successful secondment to Cambridge based start-up developing an RNA platform for protein upregulation in ocular haplo-insufficiencies and neurodegenerative disorders.

• Generation of, and interpretation of preclinical data from disease models for its lead ocular programme.

• Mapping out the regulatory/preclinical/clinical path for its lead ocular programme, engaging with KOLs and regulators.

• Evaluating and selecting targets for the company's future indications in the ocular and neurodegenerative space.

Conclusion
Highly rewarding ongoing and increasing engagement and results on 3 levels:
1. personal learning and development,
2. increasing trials and especially entering the gene and cellular therapy space,
3. access to trials for Welsh patients increased providing equity.