# Effective Health Care
## Treatment of Retinitis Pigmentosa
## Nomination Summary Document

## Results of Topic Selection Process & Next Steps

- The topic area, *Surgical treatment of Retinitis Pigmentosa*, was found to be addressed by an AHRQ Healthcare Horizon Scanning Potential High Impact Interventions Report, one technology assessment and two recent systematic reviews. Given that these existing publications cover this nomination, no further activity will be undertaken on this topic.

***AHRQ Healthcare Horizon Scanning System - Potential High Impact Interventions Report***

- ECRI Institute. AHRQ Healthcare Horizon Scanning System Potential High Impact Interventions: Priority Area 08: Functional Limitations. (Prepared by ECRI Institute under Contract No. HHSA290201000006C.) Rockville, MD: Agency for Healthcare Research and Quality. June 2013. Available at: http://www.effectivehealthcare.ahrq.gov/reports/final.cfm.

***Technology Assessment***

- Ellery B, Mundy L, Hiller J. Horizon Scanning Technology Prioritizing Summary: Retinal implants to restore light perception in individuals blinded by retinitis pigmentosa. Australia and New Zealand Horizon Scanning Network. (Australia): Australian Government, Department of Health and Aging. June 2010. Available at: http://onlinelibrary.wiley.com/o/cochrane/clhta/articles/HTA-32010001668/frame.html.

***Systematic Reviews***

- Chuang AT, Margo CE, Greenberg PB. Retinal implants: a systematic review. Br J Ophthalmol. 2014;98(7):852-6.
- Luo YH, da Cruz L. A review and update on the current status of retinal prostheses (bionic eye). Br Med Bull. 2014;109:31-44.

## Topic Description

**Nominator(s):** Public payer

**Nomination Summary:** The nominator is interested in whether implantation with the Argus® II Retinal Prosthesis System improves the ability to perform activities of daily living in patients with severe retinitis pigmentosa.

**Staff-Generated PICO**
**Population(s):** Children and adults with mild or severe retinitis pigmentosa
**Intervention(s):** Surgical treatments involving implantable devices (e.g., Argus® II

Retinal Prosthesis System)
**Comparator(s):** A comparison of the above treatment options to each other and to other types of treatment for retinitis pigmentosa (e.g., nutritional or pharmacological treatments, stem cell implantation, and transplant procedures)
**Outcome(s):** Delayed progression of vision loss, and improvement in visual acuity and activities of daily living, including mobility and localizing obstacles in surroundings

**Key Questions from Nominator:** Does surgical treatment involving implantable devices improve the ability to perform activities of daily living in patients with severe retinitis pigmentosa?

## Considerations

- Retinitis pigmentosa is a degenerative eye disease that affects about 100,000 people in the US. It is classified as a "rare disease" by the National Institutes of Health (NIH) Office of Rare Diseases Research (ORDR).
- This disease is a form of retinal dystrophy that is caused by the breakdown of photoreceptor cells, the cells in the retina that detect light and enable vision in low light conditions. The main cause of the disease is genetic with a family history of the disease as the main risk factor.
- Symptoms often present in childhood and include a degeneration of the peripheral visual field (tunnel vision) and night blindness. Long-term effects include severe vision impairment and blindness.
- There is no cure for retinitis pigmentosa. Current treatment options include light avoidance, low vision aids (e.g., magnifying glass, walking stick, computer-based aids) and implantable retinal prostheses. The basic concept of retinal prostheses is to replace the function of the dead photoreceptor cells by eliciting neural activity in the remaining retinal cells with electric currents or potentials that detect and convert light into electrical stimuli that can then be delivered to the retina.
- The Argus® II Retinal Prosthesis System is the first implantable retinal prosthesis approved by the Food and Drug Administration (FDA) for the treatment of severe retinitis pigmentosa in the US.[1]
  - The system is intended for use in adults, age 25 years or older, with severe to profound retinitis pigmentosa who have bare light perception (can perceive light, but not the direction from which it is coming) or no light perception in both eyes, evidence of intact inner layer retina function, and a previous history of the ability to see forms. Patients must also be willing and able to receive the recommended post-implant clinical follow-up, device fitting, and visual rehabilitation.

    The system is composed of a small video camera, a transmitter mounted on a pair of eyeglasses, a video processing unit (VPU) and an implanted retinal prosthesis (artificial retina). The VPU transforms images from the video camera into electronic data that are wirelessly transmitted to the retinal prosthesis, stimulating the retina to produce images. Although the system will not restore vision to patients, it may allow patients to detect light and dark in the environment, which can aid them in identifying the location or movement of objects or people.
- This topic area was included in an AHRQ Healthcare Horizon Scanning System Potential High Impact Interventions: Priority Area 08: Functional Limitations, which was conducted by the ECRI Institute for AHRQ and published in June 2013. The report identified the Argus® II Retinal Prosthesis System as one of six topics that have high-impact potential based experts' comments and review of information

[1] US Food and Drug Administration (2013). FDA approves first retinal implant for adults with rare genetic eye disease [FDA Press Release]. Retrieved from http://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm339824.htm.

and data gathered on the topics. Overall, the experts agreed that there is significant unmet need for retinitis pigmentosa treatment options because none were available until approval of the Argus® II system. They also generally agreed that the potential to improve patient health was high because of the device's ability to restore some level of vision that improves patients' ability to function.

- This topic area was also found to be addressed by an earlier health technology assessment titled Horizon Scanning Technology Prioritizing Summary: Retinal implants to restore light perception in individuals blinded by retinitis pigmentosa published in 2010 by the Australia and New Zealand Horizon Scanning Network (ANZHSN). The report examined the three investigational retinal implants, including the Argus® II Retinal Prosthesis System, EPIRET3, and a third unnamed product and found that the evidence for the safety and effectiveness of these implants was of low quality. However, at the time this assessment was conducted, retinal prostheses to treat retinitis pigmentosa were still at the developmental stage.
- Two recent systematic reviews also covered this topic area. Chuang et al. compared the clinical availability, vision restoration potential and long-term biocompatibility of five retinal prostheses, including the Argus® II Retinal Prosthesis System, Boston Retinal Implant Project, Epi-Ret-3, Intelligent Medical Implants, and Alpha-IMS. After a review of relevant publications, the authors concluded that Alpha-IMS is the device most likely to achieve long-term success. However, only the Argus® II Retinal Prosthesis System has FDA approval. The other devices are in preclinical or clinical trials, or marketed in Europe. Luo et al. also systematically reviewed the literature on retinal prostheses, including the Argus® II Retinal Prosthesis System. The review concluded that retinal prostheses play a role in restoring vision in blind retinitis pigmentosa patients providing stable, safe and long-term retinal stimulation. However, controversy remains over the use of an external image-capturing device versus internally placed photodiode devices.