Supplementary Table 1

**Literature review search strategy-** **MEDLINE and EMBASE (via the Ovid platform) and EconLit (via the EBSCO platform)**

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| **Topic** | **Search** | **Keywords** |
| Inherited retinal disease and Voretigene neparvovec | S1 | #1 leber congenital amaurosis'/exp  #2 retina dystrophy'/exp  #3 vitelliform macular degeneration'/exp OR 'vitelliform macular dystrophy'/exp  #4 inherited retinal disease'/exp OR 'inherited retina disease' OR 'inherited retinal dystrophy'/exp OR 'inherited retina dystrophy'  #5 retinitis pigmentosa'/exp  #6 early onset severe retinal dystrophy' OR eosrd  #7 early childhood retinitis pigmentosa' OR ecrp  #8: #1 OR #2 OR #3 OR #4 OR #5 OR #6 OR #7  #9 rpe65 mutation'  #10 rpe65-mediated'  #11 biallelic rpe65'  #12 rpe65' OR 'rpe 65' OR 'rpe65 gene' OR 'rpe65 protein' OR 'rpe65 protein'/exp OR 'rpe65 gene'/exp  #13 rpe65 AND gene  #14 #9 OR #10 OR #11 OR #12 OR #13  #15 #8 AND #14  #16 aav2-hrpe65v2'/exp OR 'aav2-hrpe65v2':ti,ab OR 'aav2'  #17 voretigene neparvovec'/exp OR 'voretigene neparvovec':ti,ab  #18 #16 OR #17  #19 #15 AND #18 |
| Economic evaluations | S2 | #1 economic evaluation  #2 cost-effectiveness  #3 cost-utility  #4 #1 OR #2 OR #3 |
| Combined searches | S3 | S1 AND S2 |
| Limits | Exclude not in English, conference abstracts and systematic reviews. | |
| Result | 11 citations | |

**Manual search of HTA websites**

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| Terms: Inherited retinal disease   Retinal dystrophy  Leber congenital amaurosis  Retinitis pigmentosa  Voretigene neparvovec  Luxturna  Database: CEA Registry, Canadian Agency for Drugs and Technologies in Health (CADTH), Institute for Clinical and Economic Review (I.C.E.R.), National Centre for Pharmacoeconomics Ireland (NCPE), National Institute for Health and Care Excellence (NICE), Swedish Dental and Pharmaceutical Benefits Agency (TLV), Norwegian medicines agency (NoMA), Netherlands National healthcare institute (ZIN), Scottish Medicines Consortium (SMC), and the Australian Medical Services Advisory Committee (MSAC) websites |

**Inclusion/Exclusion criteria**

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| **Included** | Full economic evaluation reported. |
| **Excluded** | Reports not in English, conference abstracts and systematic reviews. |

**List of data extracted for review**

* Title
* Abstract
* background and objectives
* target population
* setting and location
* study perspective
* comparators
* time horizon
* discount rate
* choice of health outcomes
* measurement of effectiveness
* measurement and valuation of preference based outcomes
* estimating resources and costs
* currency, price, date and conversion
* choice of model
* assumptions
* analytical methods
* study parameters
* incremental costs and outcomes
* characterising uncertainty
* characterising heterogeneity
* Discussion- study findings, limitations, generalisability and current knowledge
* Source of funding
* Conflicts of interest