**Online supplementary material 2. Additional study characteristics**

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| **Drug** | **Study population** | **Primary clinical outcomes** | **Instrument for assessment of HrQoL** | **Comparator in EE** |
| Cenobamate | Adult patients with epilepsy who have not been adequately controlled despite prior treatment with at least 2 antiepileptic drugs | Response rate: ≥50% reduction in frequency of crises | SF-36 | NA |
| Dapagliflozin | Adult patients with symptomatic chronic heart failure with reduced ejection fraction | CVD mortality, hospitalization or Emergency visit | Kansas City Cardiomyopathy Questionnaire (KCCQ) | NA |
| Trastuzumab deruxtecan | Adult patients with HER-2 positive, locally advanced, or metastatic breast cancer who have received two or more prior HER2-targeted regimens | Objective response rate | NS | NA |
| Exuparvovec | Patients with L-aromatic decarboxylase deficiency with severe phenotype | Key items from the Peabody scale (PDMS-2) | Carer QoL: WHOQOL-BREF | NA |
| Anti-CD19 transduced autologous CD3+ cells | Patients with relapsed or refractory mantle cell lymphoma after two or more lines of systemic therapy including a Bruton tyrosine kinase (BTK) inhibitor | Global response rate | Not stated | NA |
| Risdiplam | Patients with spinal muscular atrophy | Proportion of patients who remain seated without support for at least 5 seconds | Infant Toddler Quality of Life Questionnaire Short Form (ITQOL-SF47) | NA |
| Roxadustat | Patients with symptomatic anaemia associated with chronic kidney disease | Response rate: Proportion of patients with Hb≥11 g/dL | SF-36 | Epoetin alfa; Darbepoetin alfa; Epoetin dseta; Methoxypropylene glycol epoetin beta; epoetin beta |
| Tralokinumab | Adult patients with atopic dermatitis | Response rate: IGA 0 or 1 | NS | Dupilumab |
| Somapacitan | Adult patients with growth hormone deficiency | Change in percentage of trunk fat | SF-36 | Somatropina |
| Filgotinib | Adult patients with moderately to severely active ulcerative colitis unresponsive or intolerant to standard treatments | Endoscopy, Bleeding, Stool frequency (EBS) | SF-36, EQ-5D SF-36, Inflammatory Bowel Disease Questionnaire (IBDQ) | Infliximab, adalimumab, golimumab, ustekinumab, vedolizumab, tofacitinib |
| Lorlatinib | Adult patients with first-line ALK-positive non-small cell lung cancer | PFS | EQ-5D-5L | Crizotinib |
| Pegcetacoplan | Adult patients with anaemic paroxysmal nocturnal haemoglobinuria (PNH) anaemic after being treated with a C5 inhibitor | Response rate: Change in haemoglobin level | FACIT-Fatiga | Eculizumab |
| Tebentafusp | Adult patients with unresectable or metastatic uveal melanoma with human leukocyte antigen (HLA)-A\*02:01 positive | OS & PFS | EQ-5D-5L & EORTC QLQ-C30 | Pembrolizumab & Ipilimumab & Dacarbazina |
| Avapritinib | Adult patients with advanced systemic mastocytosis after at least one treatment  systemic treatment | Global response rate | EORTC QLQ-C30, Patient’s Global Impression of  Symptom Severity (PGIS) | Standard of care (unspecified) |
| Talazoparib | Patients with HER-2 negative breast cancer with BRCA 1/2 mutations in progression to previous treatments | PFS | EORTC QLQ-C30 & EORTC QLQ-BR23 | Capecitabina & Eribulina |
| Imipenem/cilastatin/relebactam | Adult patients with hospital-acquired pneumonia/ventilator-associated pneumonia with or without bacteraemia and for the treatment of infections due to aerobic Gram-negative organisms in patients with limited treatment options | Response rate: Survival+clinical and microbiological response | NS | Colistin+imipenem/cilastatin & Piperacillin/tazobactam |
| Nivolumab | Adult patients with unresectable advanced, recurrent, or metastatic squamous cell carcinoma of the oesophagus after prior chemotherapy | OS | EQ-5D-3L | Docetaxel o paclitaxel |
| Atezolizumab | Adult patients with metastatic non-small cell lung cancer | OS | NS | Platinum-based chemotherapy (cisplatin, carboplatin) in combination with pemetrexed or gemcitabine |
| Tucatinib | Adult patients with HER-2 positive, locally advanced or metastatic breast cancer | PFS | NS | Placebo |

Notes: EE = Economic Evaluation; NS = Not stated; NA = Not applicable; OS = Overall survival; PFS = Progression-Free survival; SF-36: 36-Item Short Form Health Survey; EQ-5D: EuroQol-5 Dimensions; EORTC QLQ: European Organisation for Research and Treatment of Cancer Core Quality of Life Questionnaire