NEWSLETTER: News from the HTA Agencies

June 2025

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CADTH

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
ROZANOLIXIZUMAB	RYSTIGGO	For the treatment of adult patients with generalized myasthenia gravis who are anti-acetylcholine receptor or anti-muscle-specific tyrosine kinase antibody positive	CADTH Reimbursement Recommendation	Reimburse with conditions. Rystiggo should not be reimbursed when given during a gMG exacerbation (i.e., a moment when the patient experiences weakness in some or all muscles, without needing assistance to breath) or crisis (i.e., a moment when respiratory muscles are too weak, limiting air flow in and out of the lungs and, as a result, the patient is unable to breathe), or within 6 months of thymectomy (i.e., surgical removal of the thymus gland). Rystiggo should only be reimbursed if prescribed by or in consultation with a neurologist with expertise in managing patients with gMG, and the total cost of Rystiggo should not exceed the total drug cost with the least costly advanced treatments for gMG. Rystiggo should not be used concomitantly with rituximab, efgartigimod alfa, and/or complement inhibitors such as eculizumab.	Treatment with Rystiggo is expected to cost approximately $218,478 to $655,434 per patient per year, depending on the patient's weight.
EFANESOCTOCOG ALFA	ALTUVIIIO	Antihemophilic factor VIII (recombinant, B-domain deleted), FcVWF-XTEN fusion protein in adults, adolescents, and children with hemophilia A (congenital factor VIII [FVIII] deficiency) for: • routine prophylaxis to prevent or reduce the frequency of bleeding episodes • treatment and control of bleeding episodes • perioperative management of bleeding (surgical prophylaxis)	CADTH Reimbursement Recommendation	Reimburse with conditions. ALTUVIIIO should only be reimbursed in a similar way as other FVIII replacement therapies that are currently reimbursed by public drug plans for the treatment of patients with severe hemophilia A. The cost of ALTUVIIIO should be negotiated so that it does not exceed the annual drug program cost of treatment currently reimbursed for prophylactic use in hemophilia A.	Prophylaxis with ALTUVIIO is expected to cost approximately $345,185 (for a patient weighing 40 kg) to $690,371 (for a patient weighing 80 kg) per patient annually. For on-demand use, treatment with ALTUVIIO is expected to cost approximately $6,620 (for a patient weighing 40 kg) to $13,240 (for a patient weighing 80 kg) per bleed.

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Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
ISATUXIMAB	SARCLISA	Sarclisa (isatuximab for injection) is indicated in combination with bortezomib, lenalidomide, and dexamethasone, for the treatment of patients with newly diagnosed multiple myeloma who are not eligible for autologous stem cell transplant (ASCT).	CADTH Reimbursement Recommendation	Reimburse with conditions Sarclisa should only be reimbursed in combination with bortezomiblenalidomide-dexamethasone (VRd) and if it is prescribed by clinicians with expertise in MM. The total cost of Sarclisa in combination with VRd should be negotiated so that it does not exceed the total drug program cost associated with daratumumab-lenalidomide-dexamethasone (DRd).	Treatment with Sarclisa is expected to cost approximately $25,643 in cycle 1, $17,559 in cycles 2 to 4, $13,619 in cycles 5 to 17, and $7,555 in cycles 18 and beyond per patient per 28-day treatment cycle.
DURVALUMAB	IMFINZI	Imfinzi in combination with tremelimumab and platinum-based chemotherapy is indicated for the first-line treatment of patients with metastatic NSCLC with no sensitizing epidermal growth factor receptor (EGFR) mutations or anaplastic lymphoma kinase (ALK) genomic tumour aberrations.	CADTH Reimbursement Recommendation	Reimburse with conditions Imfinzi and Imjudo, in combination with platinum-based chemotherapy should only be reimbursed if prescribed by a clinician with expertise and experience in treating NSCLC, and if the cost of Imfinzi and Imjudo, in combination with platinum-based chemotherapy does not exceed the total cost of treatment with the least costly immune checkpoint inhibitors (ICIs) plus a platinum-based chemotherapy regimen reimbursed for the same indication.	Treatment with Imfinzi and Imjudo plus platinum-based chemotherapy is expected to cost $22,010, per 21-day cycle in the initial treatment stage (up to week 12), $15,387 per 21-day cycle in the initial maintenance phase (week 13 to 16), and $8,952 per 21-day cycle in the subsequent maintenance phase (after week 16).

CADTH

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
DOSTARLIMAB	JEMPERLI	In combination with carboplatin and paclitaxel for the treatment of adult patients with primary advanced or first recurrent endometrial cancer who are candidates for systemic therapy	CADTH Reimbursement Recommendation 	Reimburse with conditions Jemperli should only be reimbursed in combination with carboplatinpaclitaxel if prescribed by clinicians with expertise in advanced endometrial cancer. Treatment should be supervised and administered in institutions with expertise in systemic therapy delivery. The cost of Jemperli should be reduced.	Treatment with Jemperli is expected to cost approximately $10,031 per 21-day cycle.
PEMBROLIZUMAB	KEYTRUDA	Keytruda is indicated for the treatment of adult patients with primary advanced or recurrent endometrial carcinoma, in combination with carboplatin and paclitaxel and then continued as monotherapy.	CADTH Reimbursement Recommendation 	Reimburse with conditions Keytruda should only be reimbursed in combination with carboplatin and paclitaxel and then continued as monotherapy if it is prescribed by clinicians with expertise in treating endometrial cancer and the cost of Keytruda is reduced.	Treatment with Keytruda is expected to cost approximately $8,800 per 21 days. The cost per patient of 21 days of treatment with Keytruda combined with carboplatin-paclitaxel ranges from $13,915 to $14,335 in the combination period.

Generic Name	Brand Name	Indication	Type of Document	Recommendation
SODIUM THIOSULFATE	PEDMARQSI	Prevention of cisplatin (CIS)-induced ototoxicity in patients aged 1 month to 18 years with localized, non-metastatic solid tumors.	Avis de la CT	Registration. Negative opinion on reimbursement.
ELRANATAMAB	ELREXFIO	As monotherapy for the treatment of adult patients with relapsed and refractory multiple myeloma who have received at least three prior therapies including an immunomodulatory agent, a proteasome inhibitor, and an anti-CD38 antibody, and whose disease progressed during the last treatment, when all therapeutic options have been exhausted for patients who do not have access to CAR-T cell–based medicines or are ineligible for them, based on the opinion of a multidisciplinary consultation meeting (RCP).	Avis de la CT	Early access authorization renewed
SETMELANOTIDE	IMCIVREE	Registration. Positive opinion on reimbursement..	Avis de la CT	Registration. Favourable opinion on reimbursement. ASMR IV: minor: The Commission considers that IMCIVREE (setmelanotide) provides a minor improvement in medical benefit (ASMR IV) in the treatment of obesity and hunger control associated with Bardet-Biedl syndrome (BBS) or biallelic loss of pro-opiomelanocortin (POMC) function, including PCSK1 deficiency or genetically confirmed biallelic leptin receptor (LEPR) deficiency, in children aged between 2 years and under 6 years.

HAS

Generic Name	Brand Name	Indication	Type of Document	Recommendation
DURVALUMAB	IMFINZI	In combination with gemcitabine and cisplatin as neoadjuvant therapy, followed by IMFINZI monotherapy after radical cystectomy, for the treatment of adult patients with resectable muscle-invasive bladder cancer (MIBC).	Avis de la CT	Early access authorization denied
OFATUMUMAB	KESIMPTA	Treatment of adult patients with active forms of relapsing multiple sclerosis (RMS), defined by clinical or imaging features.	Avis de la CT	Reassessment at the request of the Transparency Committee (CT) Favorable opinion for continued reimbursement Comments without ASMR rating: The Commission considers that the data provided as part of this reassessment are not sufficient to change the assessment of the improvement in medical benefit stated in the previous opinion dated June 2, 2021.
OZANIMOD	ZEPOSIA	Treatment of adult patients with active relapsing-remitting multiple sclerosis (RRMS), as defined by clinical or imaging criteria.	Avis de la CT	Reassessment at the request of the Transparency Committee (CT). Favorable opinion for continued reimbursement Comments without ASMR rating: The Commission considers that the data provided as part of this reassessment are not sufficient to change the assessment of the improvement in medical benefit stated in the previous opinion dated December 2, 2021.
OCRELIZUMAB	OCREVUS	treatment of adult patients with primary progressive multiple sclerosis (PPMS) at an early stage in terms of disease duration and disability level, associated with imaging data characteristic of inflammatory activity. treatment of adult patients with active forms of relapsing multiple sclerosis (RMS) defined by clinical or imaging parameters.	Avis de la CT	First-registration. Favorable opinion for reimbursement. ASMR: V (absence): This specialty is a complementary product that does not provide any improvement in the medical benefit (ASMR V) compared to the already listed OCREVUS 300 mg, solution for dilution for infusion.

Generic Name	Brand Name	Indication	Type of Document	Recommendation
CLADRIBINE	MAVENCLAD	in adults for the treatment of highly active forms of relapsing multiple sclerosis (MS) based on clinical or imaging (MRI) parameters.	Avis de la CT	Re-evaluation at the request of the Transparency Committee Favorable opinion for the continued reimbursement Comments without ASMR rating: The Committee considers that the data provided as part of this re-evaluation are not sufficient to change the assessment of the improvement in medical benefit (ASMR) expressed in the previous opinion dated May 27, 2020.
NATALIZUMAB	TYSABRI	as monotherapy for disease-modifying treatment in adults with highly active relapsing-remitting multiple sclerosis (RRMS) for the following patient groups: 1. Patients with a highly active form of the disease despite a full and properly conducted course of at least one disease-modifying therapy, 2. Patients with severe, rapidly evolving RRMS, defined by two or more disabling relapses within one year, combined with one or more gadolinium-enhancing lesion(s) on brain MRI, or a significant increase in T2 lesion load compared to a recent previous MRI.	Avis de la CT	Re-evaluation at the request of the Transparency Committee. Favorable opinion for the continued reimbursement Comments without ASMR rating: The Committee considers that the data provided in this re-evaluation are not sufficient to change the assessment of the improvement in medical benefit (ASMR) expressed in the previous opinions dated October 3, 2018, and September 8, 2021.
FINGOLIMOD	GILENYA	As monotherapy for disease-modifying treatment of highly active relapsing-remitting multiple sclerosis (RRMS) in the following groups of adult and pediatric patients aged 10 years and older: 1. Patients with a highly active form of the disease despite a full and properly conducted course of at least one disease-modifying therapy, 2. Patients with severe, rapidly evolving RRMS, defined by two or more disabling relapses within one year, associated with one or more gadolinium-enhancing lesion(s) on brain MRI, or a significant increase in T2 lesion load compared to a recent previous MRI.	Avis de la CT	Re-evaluation at the request of the Transparency Committee Favorable opinion for the continued reimbursement Comments without ASMR rating: The Committee considers that the data provided in this re-evaluation are not sufficient to change the assessment of the improvement in medical benefit (ASMR) expressed in the previous opinions dated October 3, 2018, and February 20, 2019.

Generic Name	Brand Name	Indication	Type of Document	Recommendation
PENFLURIDOL	ACEMAP	Maintenance treatment of schizophrenia in adults	Avis de la CT	Registration. Favorable opinion for reimbursement. ASMR: V (absence): The Committee considers that ACEMAP (penfluridol) 20 mg tablets do not provide any improvement in the medical benefit (ASMR V) within the current therapeutic strategy for maintenance treatment of schizophrenia in adults.
APIXABAN	ELIQUIS	Treatment of venous thromboembolic events (VTE) and prevention of VTE recurrence in pediatric patients aged 28 days to under 18 years	Avis de la CT	Extension of indication Registration. Favorable opinion for reimbursement. ASMR: V (NONE): The Committee considers that ELIQUIS (apixaban) does not provide any improvement in medical benefit (ASMR V) in the management of venous thromboembolic events in the pediatric population after at least 5 days of initial parenteral anticoagulation.
QUININE	QUININE RENAUDIN	Treatment of malaria: pernicious attack, malarial attack, particularly in cases of resistance to amino-4-quinolines with inability to use the oral route.	Avis de la CT	Registration. Positive opinion for reimbursement. ASMR: V (ABSENCE): This product does not provide any improvement in the medical service rendered (ASMR V) compared to the injectable QUINIMAX product.
ZOLBETUXIMAB	VYLOY	VYLOY, in combination with a fluoropyrimidine- and platinum-based chemotherapy, is indicated as first-line treatment for adult patients with locally advanced unresectable or metastatic HER2-negative gastric adenocarcinoma or gastroesophageal junction (GEJ) adenocarcinoma whose tumors are Claudin (CLDN) 18.2 positive.	Avis de la CT	First registration. Positive opinion for reimbursement. ASMR: V (ABSENCE): This product is a line extension that does not provide any improvement in the medical service rendered (ASMR V) compared to the already registered presentations.

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Generic Name	Brand Name	Indication	Type of Document	Recommendation
PEGCETACOPLAN	ASPAVELI	the treatment of adult patients with paroxysmal nocturnal hemoglobinuria (PNH) presenting with symptomatic hemolytic anemia after treatment with a C5 complement inhibitor for at least 6 months	Avis de la CT	Re-evaluation at the request of the manufacturer. Positive opinion for reimbursement only in "the treatment of adult patients with paroxysmal nocturnal hemoglobinuria (PNH) presenting with symptomatic hemolytic anemia after treatment with a C5 complement inhibitor for at least 6 months." Negative opinion for reimbursement in other situations covered by the MA indication. ASMR: III (moderate): The Committee considers that ASPAVELI (pegcetacoplan) 1,080 mg, infusion solution, provides a moderate improvement in the medical service rendered (ASMR III) in the management of adult patients with PNH presenting with symptomatic hemolytic anemia after treatment with a C5 inhibitor for at least 6 months, similarly to FABHALTA (iptacopan) and VOYDEYA (danicopan).
ATEZOLIZUMAB	TECENTRIQ	first-line treatment of adult patients with advanced NSCLC who are ineligible for platinum-based chemotherapy (see section 5.1 of the Summary of Product Characteristics (SmPC) for selection criteria)	Avis de la CT	Extension of indication Registration. Negative opinion on reimbursement of TECENTRIQ as monotherapy in "first-line treatment of adult patients with advanced NSCLC who are ineligible for platinum-based chemotherapy"
DARATUMUMAB	DARZALEX	DARZALEX is indicated in combination with bortezomib, lenalidomide, and dexamethasone for the treatment of adult patients with newly diagnosed multiple myeloma who are eligible for autologous stem cell transplantation.	Avis de la CT	Extension of indication. Positive opinion for reimbursement in the indication. ASMR: IV (MINEUR): The Commission considers that DARZALEX 1,800 mg (daratumumab), injectable solution, in combination with bortezomib, lenalidomide, and dexamethasone as induction and consolidation treatment, followed by maintenance treatment with the combination of DARZALEX (daratumumab) and lenalidomide (D-VRd/D-R protocol), provides a minor improvement in the medical service rendered (ASMR IV) compared to the combination of bortezomib, lenalidomide, and dexamethasone as induction and consolidation treatment followed by maintenance treatment with lenalidomide alone (VRd/R protocol).

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Generic Name	Brand Name	Indication	Type of Document	Recommendation
ZURANOLONE	ZURZUVAE	Treatment of severe postpartum depression (PPD) following childbirth in adults	Avis de la CT	Early access authorization denied
AMIVANTAMAB/LAZERTINIB	RYBREVANT /LAZCLUZE	RYBREVANT (amivantamab) and LAZCLUZE (lazertinib) in combination as first-line treatment of adult patients with advanced non-small cell lung cancer (NSCLC) with EGFR mutations due to exon 19 deletions or L858R substitution in exon 21.	Avis de la CT	Early access authorization denied
EFGARTIGIMOD ALFA	VYVGART	as an addition to standard treatment, including first-line immunosuppressants, exclusively for adult patients with generalized autoimmune myasthenia who remain symptomatic and have anti-acetylcholine receptor (AChR) antibodies.	Avis de la CT	First-registration. Positive reimbursement opinion only as an addition to standard treatment, including first-line immunosuppressants, exclusively for adult patients with generalized autoimmune myasthenia who remain symptomatic and have anti-acetylcholine receptor (AChR) antibodies. Negative reimbursement opinion for other clinical situations covered by the approved indication (MA). ASMR: V (none): These specialties are complementary to the range and do not provide any improvement in the medical service rendered (ASMR V) compared to the already registered formulations.
AMIVANTAMAB/LAZERTINIB	RYBREVANT /LAZCLUZE	as first-line treatment of adult patients with advanced non-small cell lung cancer (NSCLC) harboring EGFR mutations due to exon 19 deletions or L858R substitution in exon 21.	Avis de la CT	Early access authorization denied

HAS

Generic Name	Brand Name	Indication	Type of Document	Recommendation
AVAPRITINIB	AYVAKYT	as monotherapy for the treatment of adult patients with unresectable or metastatic gastrointestinal stromal tumors (GIST) harboring the D842V mutation of the platelet-derived growth factor receptor alpha (PDGFRA).	Avis de la CT	Re-evaluation. Positive opinion for the continued reimbursement of AYVAKYT. ASMR: V (absence): The Commission considers that AYVAKYT (avapritinib) does not provide any improvement in the medical service rendered (ASMR V) in the management of adult patients with unresectable or metastatic gastrointestinal stromal tumors (GIST) harboring the D842V mutation of the platelet-derived growth factor receptor alpha (PDGFRA).
RUXOLITINIB	JAKAVI	JAKAVI is indicated for the treatment of acute GvHD in patients aged 28 days to less than 2 years, and chronic GvHD in patients aged 6 months to less than 2 years who have an inadequate response to corticosteroids or other systemic treatments	Avis de la CT	Early access authorization granted
PEGYLATED LIPOSOMAL IRINOTECAN	ONIVYDE PEGYLATED LIPOSOMAL	in combination with oxaliplatin, 5-fluorouracil (5-FU), and leucovorin (LV) for the first-line treatment of adult patients with metastatic pancreatic adenocarcinoma.	Avis de la CT	Extension of indication. Positive opinion for the reimbursement. ASMR: IV (MINOR): The Commission considers that ONIVYDE PEGYLATED LIPOSOMAL (pegylated liposomal irinotecan) 4.3 mg/ml, dispersion for dilution for infusion, provides a minor improvement in medical benefit (ASMR IV) compared to the combination of gemcitabine + nab-paclitaxel.
BLINATUMUMAB	BLINCYTO	BLINCYTO is indicated as monotherapy for consolidation treatment in adult patients aged 18 to 30 years with Philadelphia chromosome-negative CD19-positive B-cell precursor acute lymphoblastic leukemia (ALL) in first complete remission with negative minimal residual disease (MRD)	Avis de la CT	Early access authorization granted

HAS

Generic Name	Brand Name	Indication	Type of Document	Recommendation
CICLOSPORIN	CEQUA	treatment of moderate to severe dry eye disease (dry keratoconjunctivitis) in adult patients who do not improve despite the use of tear substitutes.	Avis de la CT	Negative opinion on reimbursement
BELZUTIFAN	WELIREG	As monotherapy for the treatment of adult patients requiring therapy for renal cell carcinoma (RCC), central nervous system (CNS) hemangioblastomas, or pancreatic neuroendocrine tumors (pNETs) that are localized and associated with von Hippel-Lindau disease, and for whom localized interventions are not appropriate.	Avis de la CT	Early access authorization granted.

IQWiG

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
GARADACIMAB	ANDEMBRY	For routine prevention of recurrent attacks of hereditary angioedema in adults and adolescents aged 12 years and older	Dossier Assessment [A25-41]	Result of dossier assessment: Indication od a considerable additional benefit	The pharmaceutical company calculates the annual therapy costs per patient for Garadacimab to be €260,672.08.
DARATUMUMAB	DARZALEX	Daratumumab is indicated in combination with Cyclophosphamide, Bortezomib, and Dexamethasone (VCd) for the treatment of adult patients with newly diagnosed systemic light chain amyloidosis (AL amyloidosis).	Dossier Assessment [A25-40]	Result of dossier assessment: Indication od a considerable additional benefit	The pharmaceutical company estimates the annual therapy costs per patient for Daratumumab + VCd to be €146,090.53 to €146,159.68 for the first treatment year and €77,369.50 for the subsequent year.
VIBEGRON	OBGEMSA	Symptomatic treatment of adult patients with overactive bladder (OAB) syndrome	Dossier Assessment [A25-39]	Result of dossier assessment: Patients ≥ 65 years: hint of a minor added benefit Patients < 65 years: added benefit not proven	The pU estimates annual therapy costs per patient for Vibegron at €605.90, which consists solely of medication costs and is considered plausible.

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
ATOGEPANT	AQUIPTA	Adults who have at least 4 migraine days per month	Dossier Assessment [A25-38]	Result of dossier assessment: - Patients who are candidates for conventional migraine prophylaxis: added benefit not proven - Patients who do not respond to any of the following drug treatments/drug classes, for whom they are unsuitable, or who do not tolerate them: metoprolol, propranolol, flunarizine, amitriptyline, clostridium botulinum toxin type A: added benefit not proven	Annual therapy costs € 3,541,28
RIBOCICLIB	KISQALI	Adjuvant treatment of patients with HR-positive, HER2-negative early breast cancer at high risk of recurrence	Dossier Assessment [A24-124]	Result of dossier assessment: 1. Premenopausal women: added benefit not proven 2. Postmenopausal women: added benefit not proven 3. Men: added benefit not proven	The pharmaceutical company (pU) calculates annual therapy costs per patient for Ribociclib + Aromatase Inhibitor + GnRH analog at €31,642.93 to €33,191.14, and for Ribociclib + Aromatase Inhibitor at €29,791.47 to €30,564.88 per patient. The figures include only drug costs, which are considered plausible.

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
RIBOCICLIB	AQUIPTA	Adjuvant treatment of patients with HR-positive, HER2-negative early breast cancer at high risk of recurrence	Dossier Assessment [A25-51] Addendum to [A24-124]	Result of dossier assessment: Premenopausal women: added benefit not proven Postmenopausal women: hint of lesser benefit Men: added benefit not proven	-
MIRIKIZUMAB	OMVOH	Adults with moderately to severely active Crohn's disease	Dossier Assessment [A25-42]	Result of dossier assessment: 1. Patients who have had an inadequate response with, lost response to, or were intolerant to conventional therapy: added benefit not proven 2. Patients who have had an inadequate response with, lost response to, or were intolerant to a biologic agent (TNFα antagonist or integrin inhibitor or interleukin inhibitor): added benefit not proven	Based on the company's information regarding treatment duration and consumption, medication costs amount to €11,720.58 per patient per year.
TISLELIZUMAB	TEVIMBRA	Adult patients with locally advanced or metastatic non-small cell lung cancer (NSCLC) after prior treatment with platinum-based chemotherapy; in addition, patients with epidermal growth factor receptor mutant or anaplastic lymphoma kinase positive NSCLC should also have received targeted therapies before receiving tislelizumab.	Dossier Assessment [A24-128]	Result of dossier assessment: 1. Patients with PD-L1 negative tumours: hint of non-quantifiable added benefit 2. Patients with PD-L1 positive tumours: added benefit not proven	The pharmaceutical company (pU) calculates annual therapy costs for Tislelizumab at €76,874.24

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
TISLELIZUMAB	TEVIMBRA	Adult patients with locally advanced or metastatic non-small cell lung cancer (NSCLC) after prior treatment with platinum-based chemotherapy; in addition, patients with epidermal growth factor receptor mutant or anaplastic lymphoma kinase positive NSCLC should also have received targeted therapies before receiving tislelizumab.	Dossier Assessment [A25-63] Addendum to [A24-128]	Result of dossier assessment: 1. Patients with PD-L1 negative tumours: hint of non-quantifiable added benefit 2. Patients with PD-L1 positive tumours: added benefit not proven	-
TISLELIZUMAB	TEVIMBRA	Adults with unresectable, locally advanced or metastatic oesophageal squamous cell carcinoma that is not treatable with curative intent, whose tumours express PD-L1 with a Tumour Area Positivity score ≥ 5%	Dossier Assessment [A24-129]	1. Patients with tumour cell PD-L1 expression ≥ 1% or a combined positive score (CPS) ≥ 10; first-line treatment: added benefit not proven 2. Patients with no tumour cell PD-L1 expression ≥ 1% and no CPS ≥ 10; first-line treatment: added benefit not proven	Therapy under assessment: Tislelizumab + cisplatin + 5-FU Annual Therapy costs: € 91,836.85-92,342.50
NIRSEVIMAB	BEYFORTUS	Prevention of respiratory syncytial virus (RSV) lower respiratory tract disease in children during their first RSV season who are not addressed in the therapeutic advice on RSV antibodies	Dossier Assessment [A25-33]	Result of dossier assessment: Indication of considerable added benefit	The pharmaceutical company estimates the annual therapy costs per patient for Nirsevimab to be €436.63 to €440.84.

NICE

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
SOMAPACITAN	SOGROYA	For the replacement of endogenous growth hormone (GH) in children aged 3 years and above, and adolescents with growth failure due to growth hormone deficiency (paediatric GHD), and in adults with growth hormone deficiency (adult GHD)	Technology appraisal [TA1066]	Somapacitan can be used, within its marketing authorisation, as an option to treat growth failure caused by growth hormone deficiency in people 3 to 17 years. Somapacitan can be used if the company provides it at the same price or lower than that agreed with the Medicines Procurement and Supply Chain, where applicable.	The list prices) are: • £285.45 per 1.5-ml vial containing 10 mg somapacitan • £428.18 per 1.5-ml vial containing 15 mg somapacitan. At the recommended dose of 0.16 mg/kg/week, the annual treatment cost for a person weighing 40 kg is £9,500
EFGARTIGIMOD	VYVGART	As add-on to standard therapy for the treatment of adult patients with generalised Myasthenia Gravis (gMG) who are anti-acetylcholine receptor (AChR) antibody positive	Technology appraisal [TA1069]	Efgartigimod is not recommended, within its marketing authorisation, as an addon to standard treatment for generalised myasthenia gravis in adults who test positive for anti-acetylcholine receptor antibodies.	The list price of efgartigimod is £6,569.73 per 400-mg solution for infusion vial and £15,307.47 per 1,000-mg solution for injection vial (excluding VAT, company submission).

NICE

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
LINZAGOLIX	YSELTY	In adult women of reproductive age for symptomatic treatment of endometriosis in women with a history of previous medical or surgical treatment for their endometriosis.	Technology appraisal [TA1067]	Linzagolix with hormonal add-back therapy can be used within its marketing authorisation as an option to treat symptoms of endometriosis in adults of reproductive age who have had medical or surgical treatment for their endometriosis.	The list price for linzagolix is £80 for a pack of 28 200-mg tablets (BNF online, accessed April 2025). The list price for hormonal add-back therapy is £13.20 for a pack of 84 estradiol 1-mg norethisterone acetate 0.5-mg tablets (BNF online, accessed April 2025). At list price, 12 months of treatment would cost £1,100.
SPESOLIMAB	SPEVIGO	For the treatment of flares in adult patients with generalised pustular psoriasis.	Technology appraisal [TA1070]	Spesolimab is recommended as an option for treating generalised pustular psoriasis (GPP) flares in adults, only if it is used to treat: • initial moderate to severe flares when: – the Generalized Pustular Psoriasis Physician Global Assessment (GPPGA) total score is 3 or more (at least moderate), and – there are fresh pustules (new appearance or worsening of existing pustules), and – the GPPGA pustulation subscore is at least 2 (at least mild), and – at least 5% of the body's surface area is covered with erythema (abnormal redness of the skin or mucous membranes) and has pustules • subsequent flares with a GPPGA pustulation subscore of 2 or more (at least mild), if the last flare was treated with spesolimab and resolved to a GPPGA pustulation subscore of 0 or 1 (clear or almost clear skin). Spesolimab can only be used if the company provides it according to the commercial arrangement. A second dose of spesolimab can be used after 8 days if a flare has not resolved to a GPPGA pustulation subscore of 0 or 1. Take into account how skin colour could affect the GPPGA score and make any adjustments needed.	The list price for spesolimab is £15,000 for 2 450-mg vials (excluding VAT; BNF, accessed December 2024).

NICE

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
TISLELIZUMAB	TEVIMBRA	For treating advanced non-small-cell lung cancer after platinum-based chemotherapy in adults.	Technology appraisal [TA1072]	NICE is unable to make a recommendation on tislelizumab (Tevimbra) for treating advanced non-small-cell lung cancer after platinum-based chemotherapy in adults. This is because BeiGene withdrew its evidence submission.	
ATEZOLIZUMAB	TECENTRIQ	Atezolizumab (Tecentriq, Roche) is indicated for 'adjuvant treatment following complete resection and platinum-based chemotherapy for adult patients with NSCLC with a high risk of recurrence whose tumours have PD-L1 expression on ≥ 50% of tumour cells and who do not have EGFR-mutant or ALK-positive NSCLC'.	Technology appraisal [TA1071]	Atezolizumab can be used, within its marketing authorisation, as an option for the adjuvant treatment of non-small-cell lung cancer (NSCLC) after complete resection and platinum-based chemotherapy in adults when: • there is a high risk of recurrence • 50% or more of tumour cells express PD-L1 • the cancer is not epidermal growth factor receptor (EGFR)-mutant or anaplastic lymphoma kinase (ALK)-positive. Atezolizumab can only be used if the company provides it according to the commercial arrangement.	The list price for atezolizumab is £2,665.38 for an 840-mg vial or £3,807.69 for a 1,200-mg vial (excluding VAT, BNF online, accessed March 2025).
MARSTACIMAB	HYMPAVZI	Marstacimab (Hympavzi, Pfizer) is indicated for 'routine prophylaxis of bleeding episodes in patients 12 years of age and older, weighing at least 35 kg, with: • severe haemophilia A (congenital factor VIII deficiency, FVIII <1%) without factor VIII inhibitors, or • severe haemophilia B (congenital factor IX deficiency, FIX <1%) without factor IX inhibitors'.	Technology appraisal [TA1073]	Marstacimab is recommended, within its marketing authorisation, as an option for preventing bleeding episodes caused by severe (factor IX [9] activity less than 1%) haemophilia B (congenital factor 9 deficiency) in people 12 years and over who: • weigh at least 35 kg and • do not have factor 9 inhibitors (anti-factor antibodies). Marstacimab is only recommended if the company provides it according to the commercial arrangement	The list price of marstacimab is confidential until published by the Department for Health and Social Care.

NICE

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
SPARSENTAN	FILSPARI	Sparsentan (Filspari, Vifor) is indicated for 'the treatment of adults with primary immunoglobulin A nephropathy (IgAN) with a urine protein excretion ≥ 1.0 g/day (or urine protein-to-creatinine ratio ≥ 0.75 g/g)'.	Technology appraisal [TA1074]	Sparsentan can be used as option to treat primary immunoglobulin A nephropathy (IgAN) in adults with a: • urine protein excretion of 1.0 g/day or more, or • urine protein-to-creatinine ratio (UPCR) of 0.75 g/g or more. Sparsentan should be stopped after 36 weeks if a person's UPCR: • is 1.76 g/g or more and • has not reduced by 20% or more since starting sparsentan.	The list price for sparsentan is £3,401.71 per 30-pack of 200 mg tablets or £3,401.71 per 30-pack of 400 mg tablets (excluding VAT; company submission).
FOSDENOPTERIN	NULIBRY	For treating molybdenum cofactor deficiency type A in people of all ages.	Technology appraisal [TA1078]	NICE is unable to make a recommendation about the use in the NHS of fosdenopterin for treating molybdenum cofactor deficiency type A in people of all ages. This is because Sentynl Therapeutics has withdrawn from the appraisal. The company's decision reflects the challenge of reaching an economically sustainable route to reimbursement.	

SMC

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
DURVALUMAB	IMFINZI	In combination with tremelimumab for the first-line treatment of adults with advanced or unresectable hepatocellular carcinoma (HCC).	Medicine Advice [SMC2735]	Following a full submission assessed under the end of life medicine process durvalumab (Imfinzi®) is not recommended for use within NHSScotland.	Durvalumab 1,500 mg intravenously every four weeks + Tremelimumab 300 mg intravenously on day 1 of cycle 1: Cost per 4-week cycle First cycle: £28,008 Subsequent cycles: £7,398
BEVACIZUMAB GAMMA	LYTENAVA	In adults for treatment of neovascular (wet) age-related macular degeneration (nAMD).	Medicine Advice [SMC2751]	Following an abbreviated submission bevacizumab gamma (Lytenava®) is accepted for use within NHSScotland.	
CLADRIBINE	MAVENCLAD	For the treatment of adult patients with relapsing forms of multiple sclerosis (RMS) with active disease as defined by clinical or imaging features.	Medicine Advice [SMC2810]	Following a full submission cladribine (Mavenclad®) is accepted for restricted use within NHSScotland.	Cladribine 10 mg tablets 3.5 mg/kg bodyweight orally over years 1 and 2. Administered as one treatment course of 1.75 mg/kg per year. Each treatment course is two treatment weeks in the first two months of each year. Course 1 (year 1): 28,661 Course 2 (year 2): 28,661

SMC

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
BEMPEDOIC ACID/ EZETIMIBE	NUSTENDI	In adults with established or at high risk for atherosclerotic cardiovascular disease to reduce cardiovascular risk by lowering LDL-C levels, as an adjunct to correction of other risk factors: • in patients on a maximum tolerated dose of a statin and not adequately controlled with additional ezetimibe treatment or, • in patients who are either statin-intolerant, or for whom a statin is contraindicated, and not adequately controlled with ezetimibe treatment or, • in patients already being treated with the combination of bempedoic acid and ezetimibe as separate tablets.	Medicine Advice [SMC2741]	In the absence of a submission from the holder of the marketing authorisation bempedoic acid / ezetimibe (Nustendi®) is not recommended for use within NHSScotland.	
BEMPEDOIC ACID	NILEMDO	In adults with established or at high risk for atherosclerotic cardiovascular disease to reduce cardiovascular risk by lowering LDL-C levels, as an adjunct to correction of other risk factors: • in patients on a maximum tolerated dose of a statin with or without ezetimibe or, • alone or in combination with ezetimibe in patients who are statin-intolerant, or for whom a statin is contraindicated	Medicine Advice [SMC2740]	In the absence of a submission from the holder of the marketing authorisation bempedoic acid (Nilemdo®) is not recommended for use within NHSScotland.	

SMC

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
RUXOLITINIB	JAKAVI	For the treatment of patients aged 12 years and older with acute graft versus host disease who have inadequate response to corticosteroids.	Medicine Advice [SMC2750]	Following a full submission assessed under the end of life and orphan equivalent medicine process ruxolitinib (Jakavi®) is accepted for use within NHSScotland.	ruxolitinib 10 mg orally twice daily Cost per 28 days (£): 2,856
SELPERCATINIB	RETSEVMO	As monotherapy for the treatment of adults and adolescents 12 years and older with advanced rearranged during transfection (RET)-mutant medullary thyroid cancer (MTC).	Medicine Advice [SMC2732]	Following a full submission selpercatinib (Retsevmo®) is accepted for restricted use within NHSScotland. SMC restriction: patients who require systemic therapy and have not previously received systemic therapy.	Selpercatinib 120 mg or 160 mg orally twice daily Cost per year (£): 85,176 to 113,568
SUMATRIPTAN/ NAPROXENE	SUVEXX	The acute treatment of the headache phase of migraine attacks with or without aura in adults where treatment with a mono-entity product has been insufficient.	Medicine Advice [SMC2756]	Following an abbreviated submission sumatriptan 85mg / naproxen 457mg (Suvexx®) is not recommended for use within NHSScotland.	
PEGYLATED LIPOSOMAL IRINOTECAN	ONIVYDE	in combination with oxaliplatin, 5-fluorouracil (5-FU) and leucovorin (LV) for the first-line treatment of adult patients with metastatic adenocarcinoma of the pancreas.	Medicine Advice [SMC2812]	In the absence of a submission from the holder of the marketing authorisation pegylated liposomal irinotecan (Onivyde®) is not recommended for use within NHSScotland.	

SMC

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
PEGZILARGINASE	LOARGYS	Treatment of arginase 1 deficiency (ARG1-D), also known as hyperargininemia, in adults, adolescents and children aged 2 years and older.	Medicine Advice [SMC2813]	In the absence of a submission from the holder of the marketing authorisation pegzilarginase (Loargys)®) is not recommended for use within NHSScotland.	