NEWSLETTER: News from the HTA Agencies

August 2025

SUMMARY

HTA Agency	N°of Drugs	Drugs Name
CADTH Evidence Driven.	6	CROVALIMAB • OMAVELOXOLONE • CIPAGLUCOSIDASE ALFA + MIGLUSTAT • DUPILUMAB • DUPILUMAB • QUIZARTINIB
HAS HAUTE AUTORITÉ DE SANTÉ	19	EPCORITAMAB • AMIVANTAMAB • AMIVANTAMAB • AMIVANTAMAB • PIRTOBRUTINIB • TIRATRICOL• DOSTARLIMAB • CILTACABTAGENE AUTOLEUCEL • TEMOZOLOMIDE • ISATUXIMAB • ROPEGINTERFERON ALFA-2B • BULEVIRTIDE • TRAMADOL • PEGINTERFERON ALFA-2A • GADOTERIDOL • NATALIZUMAB • PATIROMER • PEMBROLIZUMAB • HUMAN NORMAL IMMUNOGLOBULIN
ICER INSTITUTE FOR CLINICAL AND ECONOMIC REVIEW	0	
IQWiG Institute for Quality and Efficiency in Health Care	20	IVACAFTOR/TEZACAFTOR/ELEXACAFTOR • REPOTRECTINIB • REPOTRECTINIB • BEVACIZUMAB GAMMA • CONCIZUMAB • CONCIZUMAB • TRASTUZUMAB DERUXTECAN • PEMBROLIZUMAB • EPLOTERSEN • IVACAFTOR/TEZACAFTOR/ELEXACAFTOR • MUCOPOLYCACCHARIDOSIS IVA • SARILUMAB • NIRSEVIMAB • LETERMOVIR • LETERMOVIR • UPADACITINIB • GLOFITAMAB • GLOFITAMAB • GARADACIMAB • GARADACIMAB
NICE National Institute for Health and Care Excellence	10	BETULLA VERRUCOSA • RIBOCICLIB • SACITUZUMAB GOVITECAN • RUXOLITINIB (CREAM) • DURVALUMAB + TREMELIMUMAB • TARLAMAB • PEMBROLIZUMAB • IDEBENONE • GUSELKUMAB • GUSELKUMAB
SMC Scottish Medicines Consortium	7	TRASTUZUMAB DERUXTECAN • LETERMOVIR • ZANUBRUTINIB • MIRIKIZUMAB • RIPRETINIB • DUPILUMAB • BRENTUXIMAB VEDOTIN

CADTH

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
CROVALIMAB	PIASKY	Piasky (crovalimab for injection) is indicated for the treatment of paroxysmal nocturnal hemoglobinuria (PNH) in adults and adolescents 13 years of age and older with a body weight of at least 40 kg.	CADTH Reimbursement Recommendation	Reimburse with conditions Piasky should only be prescribed at the Health Canada–recommended dosage and the cost of Piasky should not exceed the drug program cost of treatment with the least costly C5 inhibitor reimbursed for the treatment of PNH. Piasky must be prescribed by, or in consultation with, a hematologist with experience managing PNH.	Treatment with Piasky is expected to cost the public drug plans between $496,807 and $721,240 in year 1 and between $416,907 and $625,360 in subsequent years, depending on patient weight.
OMAVELOXOLONE	SKYCLARYS	For the treatment of Friedreich's ataxia in patients 16 years of age and older	CADTH Reimbursement Recommendation	Reimburse with conditions Skyclarys should only be reimbursed if the patient is under the care of a clinician experienced in treating ataxias and if the cost of Skyclarys is reduced. Treatment with Skyclarys should be stopped if the patient's mFARS score increases by more than 2 points in a year, or if their score increases to more than 80.	Treatment with Skyclarys is expected to cost approximately $399,180 per patient per year.
CIPAGLUCOSIDASE ALFA + MIGLUSTAT	POMBILITI + OPFOLDA	Cipaglucosidase alfa is indicated in combination with the enzyme stabilizer Opfolda (65 mg miglustat capsule) for the treatment of adult patients with late-onset Pompe disease (acid α-glucosidase [GAA] deficiency) weighing ≥ 40 kg. Miglustat is an enzyme stabilizer indicated in combination with Pombiliti (cipaglucosidase alfa) for the treatment of adult patients with late-onset Pompe disease (acid α-glucosidase [GAA] deficiency) weighing ≥ 40 kg. Cipaglucosidase alfa must be used in combination with 65 mg miglustat capsules.	CADTH Reimbursement Recommendation	Reimburse with conditions Pombiliti with Opfolda should only be reimbursed if prescribed by a clinician experienced in treating lysosomal storage diseases or other types of neuromuscular diseases, it is not used in combination with other enzyme replacement therapies for Pompe disease, and the cost of Pombiliti with Opfolda is reduced.	Treatment with Pombiliti with Opfolda is expected to cost approximately $683,183 per patient per year for Pombiliti and $4,788 per patient per year for Opfolda, resulting in approximately $687,971 per patient per year for the combination of Pombiliti with Opfolda.

CADTH

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
DUPILUMAB	DUPIXENT	For the treatment of adult patients with moderate-to-severe prurigo nodularis (PN) whose disease is not adequately controlled with topical prescription therapies or when those therapies are not advisable. Dupixent can be used with or without topical corticosteroids.	CADTH Reimbursement Recommendation	Reimburse with conditions Dupixent should only be reimbursed if the patient is under the care of a dermatologist who has expertise in the management of PN, and if the cost of Dupixent is reduced. When first prescribed, Dupixent should only be reimbursed for 6 months. Dupixent should not be used in combination with other systemic therapies for PN.	Treatment with Dupixent is expected to cost approximately $26,425 per patient in the first year and $25,446 per patient in subsequent years of treatment.
DUPILUMAB	DUPIXENT	Add-on maintenance treatment with intranasal corticosteroids in adult patients with severe chronic rhinosinusitis with nasal polyposis inadequately controlled by systemic corticosteroids and/or surgery	CADTH Reimbursement Recommendation	Reimburse with conditions Dupixent should only be reimbursed if the patient is under the care of a physician with expertise in managing severe CRSwNP. When Dupixent is first prescribed, the physician must submit a baseline 22-item Sino-Nasal Outcome Test (SNOT-22) score or endoscopic Nasal Polyp Score (NPS) so that response to treatment can be measured. The cost of Dupixent should be reduced.	Treatment with Dupixent is expected to cost approximately $25,534 per patient per year.
QUIZARTINIB	VANFLYTA	In combination with standard cytarabine and anthracycline induction and standard cytarabine consolidation chemotherapy, followed by quizartinib maintenance monotherapy, for the treatment of adult patients with newly diagnosed acute myeloid leukemia that is FMS-like tyrosine kinase 3 internal tandem duplication positive	CADTH Reimbursement Recommendation	Reimburse with conditions Vanflyta should only be reimbursed if treatment is initiated by clinicians with expertise in managing AML in institutions with expertise in systemic therapy delivery. Vanflyta should be negotiated so that the total treatment cost does not exceed the drug program cost of treatment with midostaurin reimbursed for the treatment of adult patients with newly diagnosed AML that is FLT3-ITD positive.	Treatment with Vanflyta is expected to cost approximately $211,617 per patient in year 1 ($237,970 with chemotherapy) and $282,675 per patient per year thereafter

HAS

Generic Name	Brand Name	Indication	Type of Document	Recommendation
EPCORITAMAB	TEPKINLY	For the treatment of adult patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL), after at least two lines of systemic treatment, who are ineligible for all available treatments or have failed CAR-T cell-based therapies	Avis de la CT	Early access authorization renewed
AMIVANTAMAB	RYBREVANT	In combination with carboplatin and pemetrexed, in the treatment of adult patients with advanced non-small cell lung cancer (NSCLC) with epidermal growth factor receptor (EGFR) mutations by deletion in exon 19 or L858R substitution in exon 21, in which a previous treatment including a third- generation EGFR tyrosine kinase inhibitor (TKI) has failed	Avis de la CT	Early access authorization renewed
AMIVANTAMAB	RYBREVANT	In combination with lazertinib, as first-line treatment for adult patients with advanced non-small cell lung cancer (NSCLC) with EGFR mutations by deletions in exon 19 or L858R substitution in exon 21.	Avis de la CT	First-time registration Favorable opinion for reimbursement ASMR: V (ABSENCE): These specialties are a range supplement which does not provide any improvement in the medical service rendered (ASMR V) compared to the presentation already listed

Generic Name	Brand Name	Indication	Type of Document	Recommendation
AMIVANTAMAB	RYBREVANT	as monotherapy in the treatment of adult patients with advanced non-small cell lung cancer (NSCLC) with EGFR mutations by insertion in exon 20, after failure of platinum-based treatment	Avis de la CT	Unsolicited extension of indication
PIRTOBRUTINIB	JAYPIRCA	As monotherapy in the treatment of adult patients with relapsed and refractory chronic lymphocytic leukemia (CLL) who have been previously treated with a BTK inhibitor and venetoclax (double-refractory)	Avis de la CT	Early access authorization denied
TIRATRICOL	EMCITATE	Treatment of peripheral thyrotoxicosis in patients with a monocarboxylate transporter 8 (MCT8) deficiency (Allan-Herndon-Dudley syndrome), from birth.	Avis de la CT	Early access authorization denied

Generic Name	Brand Name	Indication	Type of Document	Recommendation
DOSTARLIMAB	JEMPERLI	JEMPERLI is indicated in combination with carboplatin and paclitaxel for the first-line treatment of adult patients with newly diagnosed or recurrent advanced endometrial cancer (EC) who are candidates for systemic therapy.	Avis de la CT	Extension of indication Registration Favorable opinion for reimbursement in the indication ASMR: III (MODERATE): The Commission considers that JEMPERLI (dostarlimab) in combination with carboplatin and paclitaxel in the induction phase, followed by maintenance treatment with JEMPERLI (dostarlimab) monotherapy provides a moderate improvement in actual benefit (IAB III) compared to the combination of carboplatin and paclitaxel in the treatment of adult patients with newly diagnosed or recurrent advanced endometrial cancer (EC) with dMMR/MSI-H tumor status. ASMR: V (ABSENCE): The Commission considers that JEMPERLI 500 mg (dostarlimab) in combination with carboplatin and paclitaxel in the induction phase, followed by maintenance treatment with JEMPERLI (dostarlimab) monotherapy, does not provide an improvement in actual benefit (IAB V) compared to the combination of carboplatin and paclitaxel in the treatment of adult patients with newly diagnosed or recurrent advanced endometrial cancer (EC) with pMMR/MSS tumor status or whose status with regard to this deficiency is not known.
CILTACABTAGENE AUTOLEUCEL	CARVYKTI	The treatment of adult patients with relapsed and refractory multiple myeloma, having received at least one prior treatment, including an immunomodulatory agent and a proteasome inhibitor, who are refractory to lenalidomide and whose disease has progressed during the last treatment	Avis de la CT	Registration Favorable opinion for reimbursement ASMR: III (MODERATE): The Commission considers that CARVYKTI (ciltacabtagene autoleucel) provides a moderate improvement in actual benefit (IAB III) compared to the DPd [daratumumab – pomalidomide – dexamethasone] and PVd [pomalidomide – bortezomib – dexamethasone] protocols in the treatment of adult patients with relapsed and refractory multiple myeloma, who have received at least one prior treatment, including an immunomodulatory agent and a proteasome inhibitor, who are refractory to lenalidomide and whose disease has progressed during the last treatment.

Generic Name	Brand Name	Indication	Type of Document	Recommendation
TEMOZOLOMIDE	KIMOZO	as monotherapy or in combination with a specific inhibitor of DNA topoisomerase I (irinotecan or topotecan) in the treatment of pediatric patients aged 1 to 6 years and in patients aged over 6 years who are unable to swallow temozolomide in capsule form and who suffer from: - high-risk neuroblastoma that is refractory or has had an insufficient response to induction chemotherapy. - of recurrent high-risk neuroblastoma after at least a partial response to induction chemotherapy followed by myeloablative therapy and stem cell transplantation.	Avis de la CT	Refusal to renew the early access authorization
ISATUXIMAB	SARCLISA	en association avec le bortézomib, le lénalidomide et la dexaméthasone pour le traitement de première ligne du myélome multiple nouvellement diagnostiqué (MMND) de l'adulte inéligible à une greffe autologue de cellules souches	Avis de la CT	Extension of indication Modification of registration conditions ASMR: V (ABSENCE): The Commission considers that SARCLISA (isatuximab) does not provide an improvement in the medical service rendered (ASMR V) in the current strategy of first-line treatment of newly diagnosed multiple myeloma in adults ineligible for an autologous stem cell transplant.
ROPEGINTERFERON ALFA-2B	BESREMI	As monotherapy in adults for the treatment of Vaquez's disease (polycythemia vera) without symptomatic splenomegaly, intolerant or having failed hydroxyurea or when the use of hydroxyurea is not appropriate (young patients or those wishing to become pregnant)	Avis de la CT	Registration Favorable opinion for reimbursement only in the indication ASMR: V (ABSENCE): The Commission considers that BESREMI (ropeginterferon alfa-2b) does not provide an improvement in the medical service rendered (ASMR V) in the therapeutic strategy for the management of polycythemia vera, in adult patients without symptomatic splenomegaly, intolerant to or having failed hydroxyurea, or when the use of hydroxyurea is not appropriate (young patients or those wishing to become pregnant).

Generic Name	Brand Name	Indication	Type of Document	Recommendation
BULEVIRTIDE	HEPCLUDEX	treatment of chronic hepatitis delta virus (HDV) infection in adult and pediatric patients aged 3 years and older, weighing at least 10 kg, with compensated liver disease who tested positive for the presence of HDV RNA in plasma (or serum	Avis de la CT	Reassessment at the request of the CT / Extension of pediatric indication Favorable opinion for reimbursement ASMR: IV (MINOR): The Commission considers that HEPCLUDEX (bulevirtide) provides a minor improvement in medical service rendered (ASMR IV) in the strategy for managing patients infected with HDV within the framework of the recommendations issued by the HAS.
TRAMADOL	TOPALGIC LP	Treatment of moderate to severe pain" (adults and adolescents aged over 12 years)	Avis de la CT	First-time registration Favorable opinion for reimbursement ASMR: V (ABSENCE): These specialties are a range supplement which does not provide any improvement in the medical service rendered (ASMR V) compared to the presentations already listed.
PEGINTERFERON ALFA-2A	PEGASYS	Treatment of polycythemia vera or essential thrombocythemia, in adult patients intolerant to or failing hydroxyurea, or when the use of hydroxyurea is not appropriate (young patients or those wishing to become pregnant)	Avis de la CT	Extension of indication Registration Favorable opinion for reimbursement only in the treatment of polycythemia vera or essential thrombocythemia Unfavorable opinion on reimbursement in other situations covered by the MA indication. ASMR: V (ABSENCE): The Commission considers that PEGASYS (peginterferon alfa-2a) does not provide an improvement in the medical service rendered (ASMR V) in the therapeutic strategy for the management of polycythemia vera or essential thrombocythemia, in adult patients intolerant to or who have failed hydroxyurea, or when the use of hydroxyurea is not appropriate (young patients or those wishing to become pregnant).

Generic Name	Brand Name	Indication	Type of Document	Recommendation
GADOTERIDOL	PROHANCE	This medicinal product is for diagnostic use only. PROHANCE is indicated in adults and children of all ages for: - Contrast enhancement in Magnetic Resonance Imaging (MRI) of the cranial, spinal, and medullary regions. PROHANCE may also be used for whole-body MRI of pathological conditions. It enables the visualization of anatomical structures or abnormal lesions and helps differentiate between healthy and pathological tissues. PROHANCE should only be used when diagnosis is necessary and cannot be obtained by magnetic resonance imaging (MRI) without contrast enhancement	Avis de la CT	First-time registration Favorable opinion on reimbursement within the MA indications ASMR: V (ABSENCE): This specialty is a range supplement which does not provide any improvement in the medical service rendered (ASMR V) compared to the presentations already listed.
PATIROMER	VELTASSA	treatment of hyperkalemia in adults	Avis de la CT	Registration Favorable opinion for reimbursement ASMR: IV (MINOR): The Commission considers that VELTASSA 8.4 g and 16.8 g (patiromer), powder for oral suspension, provides a minor improvement in actual benefit (ASMR IV) in the current therapeutic strategy for the management of hyperkalemia in adults, in the same way as LOKELMA.

HAS

Generic Name	Brand Name	Indication	Type of Document	Recommendation
NATALIZUMAB	TYSABRI	TYSABRI is indicated as monotherapy for disease-modifying treatment in adult patients with highly active relapsing-remitting multiple sclerosis (RRMS) for the following patient groups: - Patients with highly active disease despite a full and adequate course of at least one disease-modifying therapy, or - Patients with rapidly evolving severe RRMS, defined by two or more disabling relapses within one year, and with one or more gadolinium-enhancing lesion(s) on brain MRI or a significant increase in T2 lesion load compared to a previous recent MRI	Avis de la CT	Registration Modification of registration conditions Favorable opinion on reimbursement in the community and the maintenance of reimbursement in the hospital with the new CIP code, in the MA indication.
PEMBROLIZUMAB	KEYTRUDA	In combination with carboplatin and paclitaxel, in the first-line treatment of adult patients with newly diagnosed or recurrent advanced endometrial cancer who are eligible for systemic treatment.	Avis de la CT	Extension of indication Modification of registration conditions ASMR: IV (MINOR): The Commission considers that KEYTRUDA 25 mg/ml (pembrolizumab) in combination with carboplatin and paclitaxel in the induction phase, followed by maintenance treatment with KEYTRUDA 25 mg/ml (pembrolizumab) monotherapy, provides a minor improvement in actual benefit (IAB IV) compared to the combination of carboplatin and paclitaxel in the treatment of adult patients with newly diagnosed or recurrent advanced endometrial cancer (EC).
HUMAN NORMAL IMMUNOGLOBULIN	FLEBOGAMMA DIF	pre- and post-exposure measles prophylaxis in adults, children and adolescents (aged 2 to 18 years) at risk in whom active immunization is contraindicated or not recommended.	Avis de la CT	Extension of indication Favorable opinion on reimbursement ASMR: V (ABSENCE): The specialties FLEBOGAMMA DIF 50 mg/ml and 100 mg/ml (human normal immunoglobulin) do not provide an improvement in the medical service rendered (ASMR V) in the management of pre- and post-exposure measles prophylaxis in adults, children and adolescents (aged 2 to 18 years) at risk in whom active immunization is contraindicated or is not recommended.

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
IVACAFTOR/ TEZACAFTOR/ELEXACAFTOR	KAFTRIO	Cystic fibrosis (CF) in patients aged 2 years and older who have at least one non-class I mutation, excluding an F508del and gating mutation, in the cystic fibrosis transmembrane conductance regulator gene	Dossier Assessment [A25-61]	Result of dossier assessment: Patients aged 2 to 5 years: added benefit not proven Patients aged 6 to 17 years: hint of non-quantifiable added benefit Patients from 18 years of age: hint of major added benefit	According to the information provided by the dossier, the annual therapy costs for Ivacaftor/Tezacaftor/Elexacaftor and Ivacaftor amount to €196,826.25 per patient. The annual therapy costs consist exclusively of medication costs.
REPOTRECTINIB	AUGTYRO	Monotherapy for the treatment of adult patients with ROS1-positive advanced non-small cell lung cancer	Dossier Assessment [A25-59]	Result of dossier assessment: Patients who have not previously received a ROS1 inhibitor: added benefit not proven Patients who have previously received a ROS1 inhibitor and with a programmed cell death ligand 1 expression ≥ 50%: added benefit not proven Patients who have previously received a ROS1 inhibitor and with a programmed cell death ligand 1 expression < 50%: added benefit not proven	The dossier calculates annual therapy costs per patient for Repotrectinib of €115,083.04 in the first treatment year and €117,333.27 in subsequent years.
REPOTRECTINIB	AUGTYRO	Monotherapy for the treatment of adult and paediatric patients twelve years of age and older with advanced solid tumours expressing a NTRK gene fusion	Dossier Assessment [A25-58]	Result of dossier assessment: Patients who have not previously received an NTRK inhibitor and for whom treatment options not targeting NTRK provide limited clinical benefit, or have been exhausted: added benefit not proven Patients who have previously received an NTRK inhibitor: added benefit not proven.	The dossier calculates annual therapy costs for Repotrectinib per patient at €115,083.04 for the first treatment year and €117,333.27 from the second treatment year onward.

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
BEVACIZUMAB GAMMA	LYTENAVA	Adult patients with neovascular (wet) age-related macular degeneration	Dossier Assessment [A25-57]	Result of dossier assessment: Added benefit not proven	The dossier determines the following annual therapy costs: Bevacizumab gamma: €3,095.52 to €13,799.40 for the 1st year and €0 to €13,799.40 for 1 subsequent year
CONCIZUMAB	ALHEMO	Routine prophylaxis of bleeding in patients with haemophilia B (congenital factor IX deficiency) and factor IX inhibitors and of 12 years of age or more	Dossier Assessment [A25-56]	Result of dossier assessment: Added benefit not proven	The dossier submitter (dossier) determines annual therapy costs for Concizumab per patient ranging from €369,114.94 to €859,718.80.
CONCIZUMAB	ALHEMO	Routine prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency) with FVIII inhibitors and of 12 years of age or more	Dossier Assessment [A25-55]	Result of dossier assessment: Added benefit not proven	The dossier submitter determines annual therapy costs for Concizumab per patient ranging from €369,114.94 to €859,718.80.
TRASTUZUMAB DERUXTECAN	ENHERTU	Adult patients with unresectable or metastatic HR-positive, HER2-low or HER2-ultralow breast cancer who have received at least one endocrine therapy in the metastatic setting and who are not considered suitable for endocrine therapy as the next line of treatment	Dossier Assessment [A25-54]	Result of dossier assessment: Added benefit not proven	The pharmaceutical company calculates annual therapy costs for trastuzumab deruxtecan per patient ranging from €102,906.64 to €103,271.13.

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
PEMBROLIZUMAB	KEYTRUDA	First-line treatment of adults with unresectable non-epithelioid malignant pleural mesothelioma	Dossier Assessment [A25-53]	Result of dossier assessment: Added benefit not proven	The pU estimates annual treatment costs for pembrolizumab in combination with pemetrexed and platinum-based chemotherapy to range from €99,545.19 (pembrolizumab every 6 weeks; combined with pemetrexed and cisplatin) to €102,280.86 (pembrolizumab every 3 weeks; combined with pemetrexed and carboplatin).
EPLOTERSEN	WAINZUA	Adults with hereditary transthyretin amyloidosis with stage 1 or stage 2 polyneuropathy	Dossier Assessment [A25-52]	Result of dossier assessment: Added benefit not proven	The pharmaceutical company (pU) calculates annual treatment costs per patient for Eplontersen at €361,468.08.
MUCOPOLYCCHARIDOSIS IVA	ELOSULFASE ALFA	Mucopolysaccharidosis (Morquio A Syndrome, MPS IVA) in patients of all ages	Dossier Assessment [A25-60]	Result of dossier assessment: Added benefit not proven	The pharmaceutical company (pU) calculates annual treatment costs per patient for Elosulfase alfa amounting to €419,888.30 (ranging from €211,955.90 to €627,820.70).

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
IVACAFTOR/ TEZACAFTOR/ELEXACAFTOR	KAFTRIO	Ivacaftor/Tezacaftor/Elexacaftor in combination with Ivacaftor (hereinafter referred to as Ivacaftor/Tezacaftor/Elexacaftor + Ivacaftor) compared to Ivacaftor as an appropriate comparator therapy in patients with cystic fibrosis aged 2 years and older, who have at least one non-class I mutation, including at least one gating mutation, and excluding an F508del mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene.	Dossier Assessment [A25-62]	Result of dossier assessment: Added benefit not proven	According to the information provided by the pharmaceutical company (pU), the annual therapy costs for Ivacaftor/Tezacaftor/Elexacaftor in combination with Ivacaftor amount to €196,826.25 per patient.
SARILUMAB	KEVZARA	Adults with polymyalgia rheumatica who have had an inadequate response to corticosteroids or who experience a relapse during corticosteroid taper	Dossier Assessment [A25-83] Addendum to Project [A25-83]	Result of dossier assessment: 1. Patients for whom corticosteroids are the appropriate treatment of physician's choice: hint of a non-quantifiable added benefit 2. Patients for whom the combination of corticosteroids with methotrexate is the appropriate treatment of physician's choice: added benefit not proven	The pharmaceutical company (pU) calculates annual treatment costs per patient for Eplontersen at €361,468.08.
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 G-BA (Federal Joint Committee) determines annual treatment costs per patient for Nirsevimab to be between €436.63 and €440.84.

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
LETERMOVIR	PREVYMIS	Prophylaxis of cytomegalovirus disease in cytomegalovirus-seronegative paediatric patients with a body weight of at least 40 kg who have received a kidney transplant from a cytomegalovirus-seropositive donor	Dossier Assessment [A25-68] 	Result of dossier assessment: Added benefit not proven	For Letermovir film-coated tablets, the applicant specifies the annual treatment costs separately for patients who receive ciclosporin additionally on all treatment days (€33,602.17 to €38,402.48), or receive no additional ciclosporin on any treatment day (€66,813.25 to €76,358.00).
LETERMOVIR	PREVYMIS	Prophylaxis of cytomegalovirus reactivation and disease in paediatric patients with a body weight of at least 5 kg who are cytomegalovirus-seropositive recipients of an allogeneic haematopoietic stem cell transplant	Dossier Assessment [A25-67] 	Result of dossier assessment: Added benefit not proven	For the oral formulations of Letermovir (film-coated tablets and granulate), the applicant specifies the annual treatment costs separately for patients who receive ciclosporin additionally on all treatment days (not quantifiable up to €19,201.24), or receive no additional ciclosporin on any treatment day (not quantifiable up to €38,179.00).

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
UPADACITINIB	RINVOQ	Adults with giant cell arteritis	Dossier Assessment [A25-66]	Result of dossier assessment: 1. Patients who are candidates for therapy with glucocorticoids alone: added benefit not proven 2. Patients who are not candidates for therapy with glucocorticoids alone: added benefit not proven	The applicant calculates annual treatment costs per patient for Upadacitinib monotherapy amounting to €14,166.34.
GLOFITAMAB	COLUMVI	Adults with relapsed or refractory diffuse large B-cell lymphoma, after 2 or more lines of systemic therapy	Dossier Assessment [A25-65]	Result of dossier assessment: Patients who are eligible for CAR T-cell therapy or stem cell transplantation: added benefit not proven Patients who are ineligible for CAR T-cell therapy and stem cell transplantation: added benefit not proven	The applicant (pU) calculates annual treatment costs per patient for Glofitamab at €159,102.03.
GLOFITAMAB	COLUMVI	Adults with relapsed or refractory diffuse large B-cell lymphoma not otherwise specified who are ineligible for autologous stem cell transplant	Dossier Assessment [A25-64]	Result of dossier assessment: Added benefit not proven	The applicant (pU) calculates annual treatment costs per patient for Glofitamab in combination with gemcitabine and oxaliplatin at €165,155.63.
GARADACIMAB	ANDEMBRY	For routine prevention of recurrent attacks of hereditary angioedema in adults and adolescents aged 12 years and older	Dossier Assessment [A25-94] Addendum to project [A25-41]	Result of dossier assessment: Unchanged after addendum: hint of considerable added benefit	

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: Hint of considerable added benefit	Extent and probability of the additional benefit of Garadacimab compared to berotralstat: Indication of a considerable additional benefit

NICE

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
BETULLA VERRUCOSA	ITULAZAX 12 SQ BET	Betula verrucosa (Itulazax 12 SQ-Bet, Alk-Abelló) is indicated 'in adults and children (5 years or older) for the treatment of moderate-to-severe allergic rhinitis and/or conjunctivitis induced by pollen from the birch homologous group. Itulazax is indicated in patients with a clinical history of symptoms despite use of symptom-relieving medication and a positive test of sensitisation to a member of the birch homologous group (skin prick test and/or specific IgE)'.	Technology appraisal [TA1087]	Betula verrucosa can be used as an option to treat moderate to severe allergic rhinitis or conjunctivitis caused by pollen from the birch homologous group of trees in adults with: • symptoms despite using symptom-relieving medicines • a positive sensitisation test (skin prick test or specific immunoglobulin E) to amember of the birch homologous group.	The list price of betula verrucosa is £80.12 per pack of 30 tablets (excluding VAT; company submission).
RIBOCICLIB	KISQALI	Ribociclib (Kisqali, Novartis) 'in combination with an aromatase inhibitor is indicated for the adjuvant treatment of patients with hormone receptor (HR)- positive, human epidermal growth factor receptor 2 (HER2)-negative early breast cancer at high risk of recurrence. In pre- or perimenopausal women, or in men, the aromatase inhibitor should be combined with a luteinising hormone-releasing hormone (LHRH) agonist'.	Technology appraisal [TA1086]	Ribociclib with an aromatase inhibitor can be used, within its marketing authorisation, as an option for the adjuvant treatment of hormone receptor positive, HER2-negative, early breast cancer at high risk of recurrence in adults. Combine the aromatase inhibitor with a luteinising hormone-releasing hormone agonist, unless after menopause. Ribociclib is recommended only if the company provides it according to the commercial arrangement.	The list prices of ribociclib 200-mg tablets are: • £983.33 per 21-pack • £1,966.67 per 42-pack • £2,950.00 per 63-pack (excluding VAT; BNF online, accessed March 2025).
SACITUZUMAB GOVITECAN	TRODELVY	For treating hormone receptor-positive HER2-negative metastatic breast cancer after 2 or more treatments in adults	Technology appraisal [TA1089]	NICE is unable to make a recommendation about the use in the NHS of sacituzumab govitecan for treating hormone receptor-positive HER2-negative metastatic breast cancer after 2 or more treatments in adults. This is because Gilead has confirmed that it does not intend to make an evidence submission for the appraisal. Gilead considers that the technology is unlikely to be a cost-effective use of NHS resources.	-

NICE

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
RUXOLITINIB (CREAM)	OPZELURA	Ruxolitinib cream (Opzelura, Incyte) is indicated for 'the treatment of nonsegmental vitiligo with facial involvement in adults and adolescents from 12 years of age'.	Technology appraisal [TA1088]	Ruxolitinib cream is not recommended, within its marketing authorisation, for treating non-segmental vitiligo with facial involvement in people 12 years and over.	The list price of ruxolitinib cream is £657.00 for a 100 g tube (Incyte website, accessed June 2024). The company has a commercial arrangement, which would have applied if ruxolitinib cream had been recommended.
DURVALUMAB + TREMELIMUMAB	IMFINZI + IMJUDO	Durvalumab (Imfinzi, AstraZeneca) with tremelimumab (Imjudo, AstraZeneca) is indicated 'for the first line treatment of adults with advanced or unresectable hepatocellular carcinoma (HCC)'.	Technology appraisal [TA1090]	Durvalumab with tremelimumab can be used, within its marketing authorisation, as an option for untreated advanced or unresectable hepatocellular carcinoma (HCC) in adults. Durvalumab with tremelimumab can only be used if the company provides it according to the commercial arrangement.	The list price of durvalumab is £592.00 per 2.4-ml vial and £2,466.00 per 10-ml vial (excluding VAT; BNF online, accessed April 2025). The list price of tremelimumab is £20,610.00 per 15-ml vial (excluding VAT; BNF online, accessed April 2025).

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TARLAMAB	IMDYLLTRA	Tarlatamab (IMDYLLTRA, Amgen) is indicated for 'the treatment of adult patients with extensive-stage small-cell lung cancer (ES-SCLC) with disease progression on or after at least two prior lines of therapy including platinum-based chemotherapy'	Technology appraisal [TA1091]	Tarlatamab should not be used to treat extensive-stage small-cell lung cancer in adults whose cancer has progressed after 2 or more lines of treatment, including platinum-based chemotherapy	The list price of tarlatamab is £955 per 1-mg vial or £9,550 per 10-mg vial (excluding VAT; company submission).
PEMBROLIZUMAB	KEYTRUDA	Pembrolizumab (Keytruda, MSD), in combination with carboplatin and paclitaxel, is indicated for 'the first-line treatment of primary advanced or recurrent endometrial carcinoma in adults'.	Technology appraisal [TA1092]	Pembrolizumab with carboplatin and paclitaxel can be used, within its marketing authorisation, as an option for untreated primary advanced or recurrent endometrial cancer in adults. It can only be used if the company provides it according to the commercial arrangement.	The list price is £2,630.00 for a 25 mg per 1 ml concentrate for solution for infusion 4-ml vial (excluding VAT; BNF online accessed July 2025).
IDEBENONE	RAXONE	Idebenone (Raxone, Chiesi) is indicated for the 'treatment of visual impairment in adolescent and adult patients with Leber's hereditary optic neuropathy (LHON)'.	Technology appraisal [TA1093]	Idebenone is recommended, within its marketing authorisation, as an option for treating visual impairment in Leber's hereditary optic neuropathy (LHON) in people 12 years and over. Idebenone is recommended only if the company provides it according to the commercial arrangement.	The list price for a 180 tablets pack of 150 mg idebenone is £6,364 (excluding VAT; BNF online, accessed April 2024).

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GUSELKUMAB	TREMFYA	Guselkumab (Tremfya, Janssen-Cilag) is indicated for 'the treatment of adult patients with moderately to severely active Crohn's disease who have had an inadequate response, lost response, or were intolerant to either conventional therapy or biologic treatment'	Technology appraisal [TA1095]	Guselkumab can be used as an option for previously treated moderately to severely active Crohn's disease in adults, when: • conventional or biological treatment: – has not worked (that is, the condition has not responded well enough or lost response to treatment), or – cannot be tolerated, and • a tumour necrosis factor (TNF)-alpha inhibitor has not worked, cannot be tolerated or is not suitable. Guselkumab can only be used if the company provides it according to the commercial arrangement.	The list price of guselkumab is (excluding VAT; company submission). • £4,500 for a 200 mg solution for infusion vial • £2,250 for a 200 mg pre-filled pen • £2,250 for a 100 mg pre-filled pen
GUSELKUMAB	TREMFYA	Guselkumab (Tremfya, Janssen-Cilag) is indicated 'for the treatment of adult patients with moderately to severely active ulcerative colitis who have had an inadequate response, lost response, or were intolerant to either conventional therapy, a biologic treatment, or a Janus kinase (JAK) inhibitor'.	Technology appraisal [TA1094]	Guselkumab (Tremfya, Janssen-Cilag) is indicated 'for the treatment of adult patients with moderately to severely active ulcerative colitis who have had an inadequate response, lost response, or were intolerant to either conventional therapy, a biologic treatment, or a Janus kinase (JAK) inhibitor'.	The list price of guselkumab is (excluding VAT; company submission): • £4,500 for a 200-mg solution for infusion vial • £2,250 for a 100-mg pre-filled pen • £2,250 for a 200-mg pre-filled pen.

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TRASTUZUMAB DERUXTECAN	ENHERTU	For the treatment of adult patients with unresectable or metastatic HER2-positive (IHC3+) solid tumours who have received prior treatment or who have no satisfactory alternative treatment options	Medicine Advice [SMC2854]	In the absence of a submission from the holder of the marketing authorisation trastuzumab deruxtecan (Enhertu®) is not recommended for use within NHSScotland.	-
LETERMOVIR	PREVYMIS	For prophylaxis of cytomegalovirus (CMV) disease in CMV-seronegative adults who have received a kidney transplant from a CMV-seropositive donor [D+/R-]	Medicine Advice [SMC2853]	In the absence of a submission from the holder of the marketing authorisation letermovir (Prevymis®) is not recommended for use within NHSScotland.	-
ZANUBRUTINIB	BRUKINSA	As monotherapy for the treatment of adult patients with mantle cell lymphoma (MCL) who have received at least one prior therapy	Medicine Advice [SMC2819]	Following an abbreviated submission: zanubrutinib (Brukinsa®) is accepted for use within NHSScotland.	Medicines reviewed under the abbreviated submissions process are estimated to have a limited net budget impact and resource allocation across NHSScotland.
MIRIKIZUMAB	OMVOH	For the treatment of adult patients with moderately to severely active Crohn's disease who have had an inadequate response with, lost response to, or were intolerant to either conventional therapy or a biologic treatment.	Medicine Advice [SMC2822]	Following an abbreviated submission mirikizumab (Omvoh®) is accepted for use within NHSScotland.	Medicines reviewed under the abbreviated submissions process are estimated to have a limited net budget impact and resource allocation across NHS Scotland.

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RIPRETINIB	QINLOCK	For the treatment of adult patients with advanced gastrointestinal stromal tumour (GIST) who have received prior treatment with three or more kinase inhibitors, including imatinib.	Medicine Advice [SMC2821]	Following a resubmission assessed under the end of life and orphan medicine process: ripretinib (Qinlock®) is accepted for use within NHSScotland.	Ripretinib 150 mg once daily taken orally cost per 28 days (£) 17,173
DUPILUMAB	DUPIXENT	In adults as add-on maintenance treatment for uncontrolled chronic obstructive pulmonary disease (COPD) characterised by raised blood eosinophils on a combination of an inhaled corticosteroid (ICS), a long-acting beta2-agonist (LABA), and a long-acting muscarinic antagonist (LAMA), or on a combination of a LABA and a LAMA if ICS is not appropriate.	Medicine Advice [SMC2801]	Following a full submission dupilumab (Dupixent®) is not recommended for use within NHSScotland.	Dupilumab 300 mg SC injection every 2 weeks Cost per year (£): 16,444
BRENTUXIMAB VEDOTIN	ADCENTRIS	For adult patients with previously untreated CD30+ Stage III or IV Hodgkin lymphoma (HL) in combination with doxorubicin, vinblastine and dacarbazine (AVD).	Medicine Advice [SMC2762]	Following a full submission under the orphan equivalent medicine process brentuximab vedotin (Adcetris®) is accepted for use within NHSScotland.	Brentuximab vedotin (1.2 mg/kg on Day 1 and 15 of 28-day cycle) + Doxorubicin (25mg/m2 on Day 1 and 15 of 28-day cycle) + Vinblastine (6 mg/m2 on Day 1 and 15 of 28-day cycle) + Dacarbazine (375 mg/m2 on Day 1 and 15 of 28-day cycle) Cost per six-cycle course (£) £64,233