NEWSLETTER: News from the HTA Agencies

January 2025

SUMMARY

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CADTH Evidence Driven.	7	EXAGAMGLOGENE AUTOTEMCEL • EXAGAMGLOGENE AUTOTEMCEL• FERRIC CARBOXYMALTOSE • SPESOLIMAB • ERDAFITINIB • AMIVANTAMAB • RAVALIZUMAB
HAS HAUTE AUTORITÉ DE SANTÉ	20	RUXOLITINIB • DURVALUMAB + OLAPARIB • ELAFIBRANOR • ENFORTUMAB VEDOTIN • APADAMTASE ALFA • ELECTROLYTE SOLUTION • SUMATRIPTAN/NAPROXENE • ABALOPARATIDE • F(ab')2 fragment of equine antivenom immunoglobulins from European vipers • SPESOLIMAB • NIROGACESTAT • ZOLBETUXIMAB • IVOSIDENIB • ADAGRASIB • BIFONAZOLE • ODEVIXIBAT • POVIDONE IODINE/ISOPROPYL ALCOHOL • DORZOLAMIDE/TIMOLOL • UBLITUXIMAB • ODEVIXIBAT
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CADTH

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
EXAGAMGLOGENE AUTOTEMCEL	CASGEVY	For the treatment of patients 12 years of age and older with sickle cell disease with recurrent vaso-occlusive crises	CADTH Reimbursement Recommendation	Reimburse with conditions. Casgevy should only be reimbursed if prescribed by a hematologist with expertise in SCD, if it is not a re-treatment (Casgevy is a one-time treatment), and if the cost of Casgevy is reduced.	Casgevy is expected to cost approximately $2,800,000 per administration per patient
EXAGAMGLOGENE AUTOTEMCEL	CASGEVY	For the treatment of patients 12 years of age and older with transfusion-dependent beta-thalassemia	CADTH Reimbursement Recommendation	Reimburse with conditions. Casgevy should only be reimbursed if prescribed by a hematologist with expertise in TDT, if it is not a re-treatment (Casgevy is a 1-time treatment), and the cost of Casgevy is reduced.	Treatment with Casgevy is expected to cost approximately $2,800,000 per administration per patient, regardless of the number of vials required.
FERRIC CARBOXYMALTOSE	FERINJECT	For the treatment of iron deficiency anemia (IDA) in adult and pediatric patients 1 year of age and older when oral iron preparations are not tolerated or are ineffective	CADTH Reimbursement Recommendation	Reimburse with conditions. Ferinject should only be reimbursed if it is prescribed by a clinician with expertise in managing iron deficiency anemia in adult and pediatric patients aged 1 year and older and the cost of Ferinject is reduced.	Treatment with Ferinject is expected to cost approximately $225 to $900 per adult (aged 18 years and older) and $90 to $675 per pediatric patient (aged 1 to 17 years) per the treatment course.
SPESOLIMAB	SPEVIGO	For the treatment of generalized pustular psoriasis (GPP) in adults and pediatric patients 12 years of age and older and weighing at least 40 kg	CADTH Reimbursement Recommendation	Reimburse with conditions. Spevigo should only be reimbursed if prescribed by clinicians (dermatologists or rheumatologists) with expertise in managing GPP and other types of psoriasis. For the prevention of flares, the initial authorization should be for 6 months of treatment. For continued renewal of Spevigo for the prevention of flares, the severity of GPP as measured by the generalized pustular psoriasis physician global assessment (GPPGA) total score at initiation should be maintained (not worsen), patient should experience fewer flares compared to baseline, and the reduction in the number of flares should be sustained. Spevigo should only be reimbursed ifthere is a reduction in drug price.	For the treatment of acute flares, Spevigo is expected to cost approximately $21,900 or $43,800 (if 2 doses are required) per flare. As preventive therapy, Spevigo is expected to cost approximately $102,000 in the first year and $95,000 in subsequent years of treatment per patient.

CADTH

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ERDAFITINIB	BALVERSA	For the treatment of adult patients with locally advanced unresectable or metastatic urothelial carcinoma harbouring susceptible FGFR3 genetic alterations who have disease progression during at least 1 line of prior therapy.	CADTH Reimbursement Recommendation	Reimburse with conditions. Balversa should only be reimbursed if it is prescribed by a clinician with expertise in treating patients with UC with susceptible FGFR3 genetic alteration confirmed using a validated test. The price of Balversa should be reduced. It must also be feasible to test patients for FGFR3 genetic alterations.	Treatment with Balversa is expected to cost approximately $6,706 per patient per 21-day treatment cycle.
AMIVANTAMAB	RYBREVANT	In combination with carboplatin and pemetrexed for the first-line treatment of adult patients with locally advanced (not amenable to curative therapy) or metastatic non-small cell lung cancer (NSCLC) with activating epidermal-growth factor receptor (EGFR) Exon 20 insertion mutations.	CADTH Reimbursement Recommendation	Reimburse with conditions. Rybrevant in combination with carboplatin and pemetrexed should only be reimbursed when started in combination with platinum-based chemotherapy (i.e., carboplatin and pemetrexed), and the cost of Rybrevant is reduced. It should not be reimbursed for patients with untreated brain metastases or those who have had previous systemic therapy, adjuvant treatment (given after surgery), or neoadjuvant treatment (given before surgery) if those treatments were completed less than 6 months before the cancer worsened. Rybrevant in combination with carboplatin and pemetrexed must be prescribed by specialists with experience managing NSCLC.	Treatment with Rybrevant in combination with carboplatin and pemetrexed is expected to cost approximately $16,083 per patient per 21-day cycle for cycles 1 and 2, $10,076 for cycles 3 and 4, and $9,091 for cycles 5 and beyond, assuming an average patient weight of 66 kg.
RAVALIZUMAB	ULTOMIRIS	For the treatment of adult patients with anti-acetylcholine receptor antibody-positive generalized Myasthenia Gravis	CADTH Reimbursement Recommendation	Reimburse with conditions. Ultomiris 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 lungs, and as a result the patient is unable to breathe), or within 12 months of thymectomy (i.e., surgical removal of thymus gland). Ultomiris should only be reimbursed if prescribed by or in consultation with a neurologist with expertise in managing patients with gMG and the cost of Ultomiris is reduced. Ultomiris should not be used concomitantly with rituximab, efgartigimod alfa, or complement inhibitors, such as eculizumab.	Treatment with Ultomiris is expected to vary in cost due to weight-based dosing and cost differences between the first and subsequent years. Ultomiris costs between $495,186 and $597,136 in year 1 and between $473,340 and $568,008 in subsequent years.

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RUXOLITINIB	JAKAVI	Treatment of patients aged 2 years to less than 12 years with acute or chronic graft-versus-host disease (GvHD) who have had an inadequate response to corticosteroids or other systemic treatments	Avis de la CT	Early access authorization granted.
DURVALUMAB + OLAPARIB	IMFINZI + LYNPARZA	IMFINZI, in combination with carboplatin and paclitaxel is indicated for use as a first-line treatment in adult patients with advanced or recurrent endometrial cancer who are candidates for systemic treatment, Follow-up maintenance treatment with IMFINZI in combination with olaparib for endometrial cancer that does not have MMR system impairment (pMMR). LYNPARZA in combination with durvalumab for maintenance treatment of adult patients with advanced or recurrent endometrial cancer that has a tumor without impairment of the MMR system (pMMR), and whose disease did not progress during the first line of treatment with durvalumab in combination with carboplatin and paclitaxel	Avis de la CT	Early access authorization denied. There is appropriate treatment in the indication considered as long as the JEMPERLI (dostarlimab) specialty: - is indicated at the same level of therapeutic strategy: in the treatment of patients with advanced endometrial cancer newly diagnosed or relapsing and candidates for systemic treatment, regardless of their MMR/MS tumor status; - is currently available in France as part of an early access authorization; - has satisfactory efficacy and tolerance data that do not suggest a loss of patient luck, in light of the anticipated intake of the treatment being requested for early access.
ELAFIBRANOR	IQIRVO	Treatment of primary biliary cholangitis (PBC) in combination with ursodeoxycholic acid (AUDC) in adults with an inadequate response to AUDC, or as monotherapy in patients who do not tolerate AUDC, and for which the available 2nd line treatments are not suitable according to the judgement of the prescriber	Avis de la CT	Early access authorization denied. IQIRVO (elafibranor) is not likely to bring about a substantial change in patient management based on the available data, given the lack of efficacy established so far on pruritus or fatigue, and the use of liver transplantation. The efficacy results compared to placebo are based on an intermediate composite endpoint (alkaline phosphatase and bilirubinemia), whose effect on bilirubinemia is not established and that on normalization of alkaline phosphatase, which is very relevant, is low.

HAS

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ENFORTUMAB VEDOTIN	PADCEV	In monotherapy for the treatment of adult patients with locally advanced or metastatic urothelial carcinoma who have previously received platinum salt chemotherapy and a programmed death receptor inhibitor-1 or a programmed death receptor ligand inhibitor -1	Avis de la CT	Réévaluation. Favourable opinion for reimbursement. Opinion in favour of renewal of PADCEV's early access authorization
APADAMTASE ALFA	ADZYNMA	Enzyme-linked substitution therapy (TES) in children under 12 years of age with congenital thrombotic thrombocytopenic purpura (cTPT) due to a deficit in ADAMTS13	Avis de la CT	Early access authorization granted.
Electrolyte solution	SODIUM GLYCEROPHOSPHATE KABI	in adults and children from birth to 18 years of age (newborns, infants, children and adolescents) for the correction of moderate to severe hypophosphatemia and the provision of phosphorus during parenteral nutrition.	Avis de la CT	First-time registration. Approval of reimbursement. ASMR: V (absence): the Commission considers that KABI SODIUM GLYCEROPHOSPHATE 216 mg/mL (1 mmol/mL), dilutable solution for infusion, provides no improvement in medical service rendered (ASMR V) compared with other phosphorus-based products already available in France.
SUMATRIPTAN/NAPROXENE	NOMANESIT	Acute treatment of the cephalalgic phase of migraine attacks with or without aura in adults for whom a triptan alone is ineffective and/or in cases of intense recurrence.	Avis de la CT	Registration Approval for reimbursement only in the acute treatment of the cephalalgic phase of migraine attacks with or without aura in adults for whom a triptan alone is ineffective and/or in cases of intense recurrence. Refusal of reimbursement in other situations covered by the AMM indication. ASMR: V (absence): the Commission considers that NOMANESIT (sumatriptan 85 mg/naproxen sodium 500 mg) does not provide an improvement in medical service rendered (ASMR V) in the therapeutic strategy for acute treatment of the cephalalgic phase of migraine attacks with or without aura in adults for whom triptan alone is ineffective and/or in cases of intense recurrence.

Generic Name	Brand Name	Indication	Type of Document	Recommendation
ABALOPARATIDE	ELADYNOS	Treatment of osteoporosis in post-menopausal women with either a history of at least one vertebral fracture or a history of at least two fractures.	Avis de la CT	First-time registration. Approval for reimbursement only in the treatment of osteoporosis in postmenopausal women with either a history of at least one vertebral fracture or a history of at least two fractures. Refusal of reimbursement in other situations covered by the AMM indication. ASMR: V (absence): the Commission considers that ELADYNOS (abaloparatide) does not provide an improvement in medical service rendered (ASMR V) in the management of osteoporosis in postmenopausal women with either a history of at least one vertebral fracture or a history of at least two fractures.
F(ab')2 fragment of equine antivenom immunoglobulins from European vipers (Vipera aspis, Vipera berus, Vipera ammodytes)	VIPERFAV	Treatment of envenomations (grade II or III) by European vipers (Vipera aspis, Vipera berus, Vipera ammodytes), in patients with rapidly expanding oedema and/or the appearance of systemic signs: vomiting, diarrhoea, abdominal pain, hypotension.	Avis de la CT	First-time registration. Approval for reimbursement. ASMR: V (absence): This product is a range extension which does not provide any improvement in medical service rendered (ASMR V) compared with the presentation already registered.
SPESOLIMAB	SPEVIGO	Prevention of flare-ups of generalized pustular psoriasis (GPP) in adults and adolescents aged 12 and over with refractory disease, i.e. non-responders, ineligible or intolerant to currently available treatments	Avis de la CT	Early access authorization granted.
NIROGACESTAT	OGSIVEO	Treatment of adult patients with desmoid tumors (DT) that have progressed after at least one prior line of therapy, including tyrosine kinase inhibitors (TKIs)	Avis de la CT	Early access authorization granted.

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ZOLBETUXIMAB	VYLOY	In combination with fluoropyrimidine and platinum-based chemotherapy, is indicated in the first-line treatment of adult patients with HER-2-negative, unresectable or metastatic locally advanced gastric or esogastric junction adenocarcinoma whose tumors are CLDN 18. 2 positive and who do not express PD-L1 or who express PD-L1 with a Combined Positive Score (CPS) < 5 or who are not eligible for treatment with a PD-1/PD-L1 inhibitor	Avis de la CT	Early access authorization granted.
IVOSIDENIB	TIBSOVO	In monotherapy for treatment of adult patients with locally advanced or metastatic cholangiocarcinoma with an IDH1 R132 mutation, ECOG score 0 or 1, who have progressed after one line of systemic therapy and are ineligible for FOLFOX chemotherapy.	Avis de la CT	Early access authorization granted.
ADAGRASIB	KRAZATI	As monotherapy for the treatment of adult patients with KRAS G12C-mutant advanced non-small-cell lung cancer (NSCLC) whose disease has progressed after at least one prior systemic therapy	Avis de la CT	Early access authorization denied. There is appropriate treatment in the indication considered as long as the JEMPERLI (dostarlimab) specialty: - is indicated at the same level of therapeutic strategy: in the treatment of patients with advanced endometrial cancer newly diagnosed or relapsing and candidates for systemic treatment, regardless of their MMR/MS tumor status; - is currently available in France as part of an early access authorization; - has satisfactory efficacy and tolerance data that do not suggest a loss of patient luck, in light of the anticipated intake of the treatment being requested for early access.

Generic Name	Brand Name	Indication	Type of Document	Recommendation
BIFONAZOLE	BIFONAZOLE SUBSTIPHARM	Local treatment of mucocutaneous mycoses caused by dermatophytes and/or Pityriasis versicolor and/or Candida	Avis de la CT	First-time registration. Approval for reimbursement. ASMR: V (absence): This speciality is a hybrid which does not provide any improvement in medical service rendered (ASMR V) compared to the reference speciality already registered.
ODEVIXIBAT	KAYFANDA	Treatment of cholestatic pruritus associated with Alagille syndrome (AGS) in patients aged 6 months or older	Avis de la CT	Registration. Approval of reimbursement. ASMR: III (moderate): the Commission considers that KAYFANDA (odevixibat) provides a moderate improvement in medical service rendered (ASMR III) in the current therapeutic strategy.
POVIDONE IODINE/ ISOPROPYL ALCOHOL	PERPRUP	This medicine is to be used for antisepsis of healthy skin prior to invasive medical procedures (including surgery) and has bactericidal and yeast-cidal activity. PERPRUP is indicated for adults, adolescents and children aged 1 year and over.	Avis de la CT	Registration. Approval of reimbursement. ASMR: V (absence): the Commission considers that PERPRUP (povidone-iodine, isopropyl alcohol) 7.25 mg/ml + 633 mg/ml, solution for cutaneous application, does not provide an improvement in medical service rendered (ASMR V) compared with other available povidone-iodine-based alcoholic antiseptic solutions.
DORZOLAMIDE/ TIMOLOL	COSIDIME	Treatment of increased intraocular pressure (IOP) in patients with open-angle glaucoma or pseudoexfoliative glaucoma, when topical beta-blocker monotherapy is insufficient	Avis de la CT	Registration. Favourable opinion for inclusion in the lists of medicines reimbursable by the social security system and approved for community use

Generic Name	Brand Name	Indication	Type of Document	Recommendation
UBLITUXIMAB	**BRIUMVI**	Treatment of adult patients with active forms of relapsing-remitting multiple sclerosis (RRMS) defined by clinical or imaging parameters.	Avis de la CT	First-time registration. Approval of reimbursement. ASMR: V (absence): the Commission de la transparence considers that BRIUMVI (ublituximab) 150 mg, solution to be diluted for infusion does not provide an improvement in medical service rendered (ASMR V) in the management strategy for patients with early-stage RRMS in terms of disease duration and inflammatory activity, and does not provide an improvement in medical service rendered (ASMR V) in the management strategy for patients with very active or severe RRMS.
ODEVIXIBAT	**BYLVAY**	Treatment of cholestatic pruritus associated with Alagille syndrome (AGS) in patients aged 6 months or older	Avis de la CT	Early access authorization renewed

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
ATEZOLIZUMAB	TECENTRIQ	First-line treatment of advanced non-small cell lung cancer in adult patients for whom platinum-based chemotherapy is not an option and whose tumours have no EGFR mutations or ALK translocations.	Dossier Assessment [A24-97]	Patients with PD-L1 expressions ≥ 50% on tumour cells: added benefit not proven. Patients with PD-L1 expressions < 50% on tumour cells: added benefit not proven.	The pU determines annual therapy costs per patient for atezolizumab of 67 767.78 € or in the amount of 69 507,78 €
ENCORAFENIB	BRAFTOVI	Adult patients with advanced non-small cell lung cancer with a rapidly accelerated fibrosarcoma isoform B V600E mutation	Dossier Assessment [A24-101]	Patients with PD-L1 expression ≥ 50%, first-line treatment: added benefit not proven. Patients with PD-L1 expression < 50%, first-line treatment: added benefit not proven.	The pU determines annual therapy costs per patient or per year for binimetinib + encorafenib. Patient in the amount of 118 898,75 €
ENFORTUMAB VEDOTIN	PADCEV	First-line treatment of adult patients with unresectable or metastatic urothelial carcinoma who are eligible for platinum-containing chemotherapy	Dossier Assessment [A24-98]	Patients for whom cisplatin-based therapy is a suitable treatment option: hint of non-quantifiable added benefit. Patients for whom cisplatin-based therapy is not a suitable treatment option: hint of major added benefit.	The pU determines for Enfortumab Vedotin in combination with Pembrolizumab annual therapy costs per patient of 191 822.47 €
PEMBROLIZUMAB	KEYTRUDA	First-line treatment of adult patients with unresectable or metastatic urothelial carcinoma	Dossier Assessment [A24-99]	Patients for whom cisplatin-based therapy is a suitable treatment option: hint of non-quantifiable added benefit. Patients for whom cisplatin-based therapy is not a suitable treatment option: hint of major added benefit. Patients for whom cisplatin-based and carboplatin-based therapy is not a suitable treatment option: added benefit not proven.	The pU for pembrolizumab in combination with enfortumab vedotin determines annual therapy costs per patient in the amount of 192 080,77 € to 193 052,48 €

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
BINIMETINIB+ ENCORAFENIB	MEKTOVI	Adult patients with advanced non-small cell lung cancer with a rapidly accelerated fibrosarcoma isoform B V600E mutation	Dossier Assessment [A24-100]	Patients with PD-L1 expression ≥ 50%, first-line treatment: added benefit not proven. Patients with PD-L1 expression < 50%, first-line treatment: added benefit not proven. Patients after first-line treatment: added benefit not proven.	The company calculated annual treatment costs per patient of €118,898.75 for binimetinib + encorafenib.
ATEZOLIZUMAB	**TECENTRIQ**	Adult patients with completely resected non-small cell lung cancer at high risk of recurrence after platinum-based chemotherapy whose tumours express PD-L1 in ≥ 50% of the tumour cells and who do not have EGFR mutations or ALK-positive NSCLC; adjuvant treatment	Dossier Assessment [A24-102]	Result of dossier assessment: Hint of minor added benefit.	The company calculated annual treatment costs per patient for atezolizumab of €67,767.78 (s. c. administration) or €69,507.78 (i. v. administration, once every 3 weeks).
PEMBROLIZUMAB	KEYTRUDA	Adult patients with locally advanced, or early-stage triple-negative breast cancer at high risk of recurrence, in combination with chemotherapy as neoadjuvant treatment, and then continued as monotherapy as adjuvant treatment after surgery	Dossier Assessment [A24-104]	Patients for whom paclitaxel + carboplatin followed by doxorubicin or epirubicin + cyclophosphamide is the suitable neoadjuvant chemotherapy of physician's choice: hint of minor added benefit 2. Patients for whom paclitaxel + carboplatin followed by doxorubicin or epirubicin + cyclophosphamide is not the suitable neoadjuvant chemotherapy of physician's choice: added benefit not proven	For the pembrolizumab combination therapies specified in Section II 2.2, the company calculated residual therapy costs per patient of €52,351.34 to €53,595.26 for the neoadjuvant phase and €47,550.60 to €52,294.00 for the adjuvant phase.

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
CAPIVASERTIB	TRUQP	Women and men with PIK3CA/AKT1/PTEN-mutated, ER-positive, HER2-negative, locally advanced or metastatic breast cancer	Dossier Assessment [A24-105]	Women after recurrence of the disease during or after (neo-adjuvant endocrine therapy, no treatment to date in locally advanced or metastatic stage: added benefit not proven Men after recurrence of the disease during or after (neo-)adjuvant endocrine therapy, no treatment to date in locally advanced or metastatic stage: added benefit not proven Women with disease progression during or after endocrine therapy that occurred in the locally advanced or metastatic stage: added benefit not proven Men with disease progression during or after endocrine therapy that occurred in the locally advanced or metastatic stage: added benefit not proven	The company determined annual treatment costs per patient for capivasertib + fulvestrant + GnRH analog in the amount of €98,545.75 to €99,276.05 and for capivasertib + fulvestrant Annual treatment costs per patient in the amount of € 96,694.29.
DELGOCITINIB	ANZUPGO	Adult patients with moderate to severe chronic hand eczema for whom topical corticosteroids are inadequate or inappropriate	Dossier Assessment [A24-107]	Added benefit not proven	For delgocitinib, the company calculated annual therapy costs of €1,798.20 to €6,563.43.
ALECTINIB	ALECENSA	Adjuvant treatment following complete tumour resection for adult patients with anaplastic lymphoma kinase-positive non-small cell lung cancer at high risk of recurrence	Dossier Assessment [A24-73]	Patients for whom adjuvant platinum-based chemotherapy is suitable: added benefit not proven; Patients after prior platinum-based chemotherapy or patients for whom this therapy is not suitable: added benefit not proven	The company calculated annual treatment costs per patient for alectinib in the amount of €73,480.50.
ALECTINIB	ALECENSA	Adjuvant treatment following complete tumour resection for adult patients with anaplastic lymphoma kinase-positive non-small cell lung cancer at high risk of recurrence	Dossier Assessment [A23-115] - Addendum to [A24-73]	1. Patients for whom adjuvant platinum-based chemotherapy is suitable: Unchanged after addendum: added benefit not proven 2. Patients after prior platinum-based chemotherapy or patients for whom this therapy is not suitable: Unchanged after addendum: added benefit not proven	

NICE

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TEBENTAFUSP	KIMMTRAK	As monotherapy for the treatment of human leukocyte antigen (HLA)-A*02:01-positive adult patients with unresectable or metastatic uveal melanoma.	Technology appraisal [TA1027]	Tebentafusp is recommended, within its marketing authorisation, for treating HLA-A*02:01-positive unresectable or metastatic uveal melanoma in adults. Tebentafusp is only recommended if the company provides it according to the commercial arrangement.	The list price for tebentafusp (200 micrograms per 1-ml vial) is £10,114.
ANDEXANET ALFA	ONDEXANET	Adult patients treated with a direct factor Xa (FXa) inhibitor (apixaban or rivaroxaban) when reversal of anticoagulation is needed due to lifethreatening or uncontrolled bleeding	Technology appraisal [TA697]	Andexanet alfa is recommended as an option for reversing anticoagulation from apixaban or rivaroxaban in adults with life-threatening or uncontrolled bleeding, only if the bleed is in the gastrointestinal tract, and the company provides andexanet alfa according to the commercial arrangement.	The list price for andexanet alfa is £11,100 per 4-vial pack of 200 mg of powder for solution for infusion. The average cost of a course of treatment at list price is £15,000 per patient.
DURVALUMAB	IMFINZI	Durvalumab (Imfinzi, AstraZeneca) with platinum-based chemotherapy as neoadjuvant treatment, and then as monotherapy after surgery, is indicated for 'the treatment of adults with resectable (tumours ≥ 4 cm and/or node positive) NSCLC and no known EGFR mutations or ALK rearrangements.	Technology appraisal [TA1030]	Durvalumab is recommended, within its marketing authorisation, as neoadjuvant treatment with platinum-based chemotherapy, then continued alone as adjuvant treatment, for treating non-small-cell lung cancer (NSCLC) in adults whose cancer: is resectable (tumours 4 cm or over, or node positive) and has no epidermal growth factor receptor (EGFR) mutations or anaplastic lymphoma kinase (ALK) rearrangements.	The list price is £2,466 per 500-mg vial. The cost of a course of perioperative treatment of durvalumab is approximately £69,779.

NICE

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ANDEXANET ALFA	ONDEXANET	For reversing anticoagulation in adults with intracranial haemorrhage	Technology appraisal [TA1029]	NICE is unable to make a recommendation about the use in the NHS of andexanet alfa for reversing anticoagulation from apixaban or rivaroxaban in adults with life-threatening or uncontrolled bleeding in the skull (intracranial haemorrhage). This is because AstraZeneca has confirmed that it does not intend to make an evidence submission for the appraisal. AstraZeneca considers that currently there is not enough evidence that the technology is a cost-effective use of NHS resources in this population.	
BIMEKIZUMAB	BIMZELX	For treating moderate to severe hidradenitis suppurativa in adults.	Technology appraisal [TA1028]	NICE is unable to make a recommendation about the use in the NHS of bimekizumab for treating moderate to severe hidradenitis suppurativa in adults. This is because UCB Pharma withdrew from the appraisal. UCB Pharma could not agree an economically sustainable route to reimbursement for this additional indication.	
VAMOROLONE	AGAMREE	For the treatment of Duchenne muscular dystrophy (DMD) in patients aged 4 years and older.	Technology appraisal [TA1031]	Vamorolone is recommended, within its marketing authorisation, as an option for treating Duchenne muscular dystrophy (DMD) in people 4 years and over.	The anticipated list price of vamorolone is £4,585.87 per 100 ml of a 40 mg/ml oral suspension.
ANHYDROUS SODIUM THIOSULFATE	PEDMARQSI	The prevention of ototoxicity induced by cisplatin chemotherapy in patients 1 month to <18 years of age with localised, non-metastatic, solid tumours.	Technology appraisal [TA1034]	Anhydrous sodium thiosulfate is recommended, within its marketing authorisation, for preventing hearing loss caused by cisplatin chemotherapy in people 1 month to 17 years with localised, non-metastatic solid tumours.	The list price for anhydrous sodium thiosulfate is £8,277.71 per 8-g vial.

NICE

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NIRAPARIB	ZEJULA	**Niraparib with abiraterone acetate and prednisone for untreated hormone-relapsed metastatic prostate cancer in adults.**	**Technology appraisal [TA1032]**	**NICE is unable to make a recommendation about the use in the NHS of niraparib with abiraterone acetate and prednisone for untreated hormone-relapsed metastatic prostate cancer in adults. This is because Johnson & Johnson Innovative Medicine has confirmed that it does not intend to make an evidence submission for the appraisal. Johnson & Johnson Innovative Medicine considers that the technology is unlikely to be used at this point in the treatment pathway.**	
VADADUSTAT	**VAFSEO**	**The treatment of symptomatic anaemia associated with chronic kidney disease (CKD) in adults on chronic maintenance dialysis'.**	**Technology appraisal [TA1035]**	**Vadadustat is recommended, within its marketing authorisation, as an option for treating symptomatic anaemia caused by chronic kidney disease in adults having maintenance dialysis.**	**The list prices of vadadustat are: • £148.59 per 28-tablet pack of 150-mg tablets; • £520.08 per 98-tablet pack of 150-mg tablets.**

SMC

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
VAMOROLONE	**AGAMREE**	Treatment of Duchenne muscular dystrophy (DMD) in patients aged 4 years and older.	Medicine Advice [SMC2721]	Following a full submission assessed under the orphan medicine process vamorolone (Agamree®) is accepted for use within NHSScotland.	Administered orally: • If < 40 kg: 6 mg/kg once daily. • If > 40 kg: 240 mg once daily. If > 40 kg and assuming no down-titration: £100,155 If < 40 kg and assuming no down-titration: £32,101 to £98,653 (ranging from 12 kg to 39 kg) The UK list price (excluding VAT) for 100ml of 40mg/ml of vamorolone is £4,585.87
CROVALIMAB	**PIASKY**	As monotherapy for the treatment of adult and paediatric patients 12 years of age or older with a weight of 40 kg and above with paroxysmal nocturnal haemoglobinuria (PNH): - In patients with haemolysis with clinical symptom(s) indicative of high disease activity. - In patients who are clinically stable after having been treated with a complement component 5 (C5) inhibitor for at least the past 6 months.	Medicine Advice [SMC2728]	Following an abbreviated submission crovalimab (Piasky®) is accepted for restricted use within NHSScotland.	
UBLITUXIMAB	**BRIUMVI**	Treatment of adult patients with relapsing forms of multiple sclerosis (RMS) with active disease defined by clinical or imaging features.	Medicine Advice [SMC2731]	Following an abbreviated submission ublituximab (Briumvi®) is accepted for restricted use within NHSScotland.	

SMC

Generic Name	Brand Name	Indication	Type of Document	Recommendation	Info on Costs
RISANKIZUMAB	**SKYRIZI**	For the treatment of adult patients with moderately to severely active ulcerative colitis who have had an inadequate response to, lost response to, or were intolerant to conventional therapy or a biologic therapy.	Medicine Advice [SMC2686]	Following an abbreviated submission risankizumab (Skyrizi®) is accepted for use within NHSScotland.	
CICLOSPORIN	**CEQUA**	Treatment of moderate-to-severe Dry Eye Disease (keratoconjunctivitis sicca) in adult patients who have not responded adequately to artificial tears.	Medicine Advice [SMC2739]	Following an abbreviated submission ciclosporin (Cequa®) is accepted for restricted use within NHSScotland.	
IPTACOPAN	**FABHALTA**	As monotherapy in the treatment of adult patients with paroxysmal nocturnal haemoglobinuria (PNH) who have haemolytic anaemia.	Medicine Advice [SMC2676]	Following a full submission iptacopan (Fabhalta®) is accepted for restricted use within NHSScotland.	Dose regimen: 200 mg orally twice daily Cost per year: £344,500
DANICOPAN	**VOYDEYA**	As an add-on to ravulizumab or eculizumab for the treatment of adult patients with paroxysmal nocturnal haemoglobinuria (PNH) who have residual haemolytic anaemia.	Medicine Advice [SMC2675]	Following a full submission danicopan (Voydeya®) is accepted for restricted use within NHSScotland.	The recommended starting dose of danicopan is 150 mg three times a day administered orally, increased to 200 mg three times a day after a minimum of 4 weeks of treatment depending on clinical response. Cost per year: 150 mg three times a day: £49,861; 200 mg three times a day: £66,481

SMC

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RELUGOLIX, ESTRADIOL, NORETHISTERONE	**RYEQO**	**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.**	**Medicine Advice [SMC2666]**	**Following a full submission relugolix, estradiol, norethisterone acetate film-coated tablets (Ryeqo®) is accepted for use within NHSScotland.**	**Dose regimen: One tablet daily Cost per year (£): 936**
BICTEGRAVIR, EMTRICITABINE, TENOFOVIR ALAFENAMIDE	**BIKTARVY**	**Treatment of human immunodeficiency virus-1 (HIV-1) infection in paediatric patients at least 2 years of age and weighing at least 14 kg to less than 25 kg without present or past evidence of viral resistance to the integrase inhibitor class, emtricitabine or tenofovir.**	**Medicine Advice [SMC2760]**	**In the absence of a submission from the holder of the marketing authorisation bictegravir/emtricitabine/tenofovir alafenamide (Biktarvy®) is not recommended for use within NHSScotland.**	
ROZANOLIXIZUMAB	**RYSTIGGO**	**As an add-on to standard therapy for the treatment of generalised myasthenia gravis (gMG) in adult patients who are anti-acetylcholine receptor (AChR) or anti-muscle-specific tyrosine kinase (MuSK) antibody positive.**	**Medicine Advice [SMC2761]**	**In the absence of a submission from the holder of the marketing authorisation rozanolixizumab (Rystiggo®) is not recommended for use within NHSScotland.**	**Dose regimen: 200 mg orally twice daily Cost per year: £344,500**
FOSDENOPTERIN	**NULIBRY**	**For the treatment of patients with molybdenum cofactor deficiency (MoCD) Type A**	**Medicine Advice [SMC2624]**	**The Scottish Medicines Consortium (SMC) has completed its initial assessment of the evidence for the above product using the ultra-orphan framework**	**The recommended dose for patients aged from 1 to less than 18 years of age is 0.90 mg/kg once daily. For patients aged less than 1 year, see SPC for details. Cost per 28 days: Body weight up to approx. 10.5 kg = £33,754 Body weight up to approx. 21 kg = £67,509 Body weight up to approx. 31 kg = £101,263**

SMC

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SIROLIMUS	HYFTOR	For the treatment of facial angiofibroma associated with tuberous sclerosis complex in adults and paediatric patients aged 6 years and older.	Medicine Advice [SMC2710]	Following a full submission assessed under the orphan medicine process sirolimus (Hyftor®) is accepted for use within NHSScotland.	A dose of 125 mg gel should be administered per 50 cm2 lesion in the face, twice daily. Maximum recommended daily dose, 600mg gel in patients aged 6-11 years; or, 800mg gel in patients aged ≥ 12 years - Patients aged 6-11 years, up to £8,800 - Patients aged ≥12 years, up to £12,000