Supplementary Table 1– Products granted conditional marketing authorisation or authorisation under exceptional circumstances by the European Medicines Agency and evaluated by the NICE in a Technology Appraisal or Highly Specialised Technologies Evaluation

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| **Technology name** | **Type of original marketing authorisation** | **Original Marketing Authorisation wording** | **Marketing authorisation status by August 2019** | **TA/HST number** | **TA/HST title** | **TA/HST recommendation by August 2019** |
| drotrecogin alfa (activated) | exceptional | treatment of adult patients with severe sepsis with multiple organ failure when added to best standard care | Withdrawn | TA84 | Drotrecogin alfa (activated) for severe sepsis | Discontinued |
| sunitinib | conditional | treatment of advanced and/or metastatic renal cell carcinoma (MRCC) after failure of interferon alfa or interleukin-2 therapy. | Full | TA178 | Bevacizumab (first-line), sorafenib (first- and second-line), sunitinib (second-line) and temsirolimus (first-line) for the treatment of advanced and/or metastatic renal cell carcinoma | Not recommended |
| nelarabine | exceptional | treatment of patients with T-cell acute lymphoblastic leukaemia (T- ALL) and T-cell lymphoblastic lymphoma (T-LBL) whose disease has not responded to or has relapsed following treatment with at least two chemotherapy regimens | Exceptional | ID1044 | Nelarabine for treating refractory T-cell lymphoblastic non-Hodgkin’s lymphoma | Discontinued |
| nelarabine | exceptional | treatment of patients with T-cell acute lymphoblastic leukaemia (T- ALL) and T-cell lymphoblastic lymphoma (T-LBL) whose disease has not responded to or has relapsed following treatment with at least two chemotherapy regimens | Exceptional | ID1034 | Nelarabine for treating acute lymphoblastic leukaemia after two therapies | Discontinued |
| panitumumab | conditional | monotherapy for the treatment of patients with EGFR expressing metastatic colorectal carcinoma with non-mutated (wild-type) KRAS after failure of fluoropyrimidine-, oxaliplatin-, and irinotecan-containing chemotherapy regimens | Full | TA242 | Cetuximab, bevacizumab and panitumumab for the treatment of metastatic colorectal cancer after first-line chemotherapy: Cetuximab (monotherapy or combination chemotherapy), bevacizumab (in combination with non-oxaliplatin chemotherapy) and panitumumab (monotherapy) for the treatment of metastatic colorectal cancer after first-line chemotherapy | Not recommended |
| trabectedin | exceptional | treatment of adult patients with advanced soft tissue sarcoma, after failure of anthracyclines and ifosfamide, or who are unsuited to receive these agents. | Full | TA185 | treatment of advanced soft tissue sarcoma | Recommended with patient access scheme |
| lapatinib | conditional | in combination with capecitabine, is indicated for the treatment of patients with advanced or metastatic breast cancer whose tumours overexpress ErbB2 (HER2). Patients should have progressive disease following prior therapy which must include anthracyclines and taxanes and therapy with trastuzumab in the metastatic setting | Full | ID20 | Lapatinib for breast cancer (for use in women with previously treated advanced or metastatic breast cancer) | Suspended |
| canakinumab | exceptional | • Cryopyrin-Associated Periodic Syndromes (CAPS) in adults, adolescents and children aged 4 years and older with body weight above 15 kg, including: − Muckle-Wells Syndrome (MWS), - Neonatal-Onset Multisystem Inflammatory Disease (NOMID) / Chronic Infantile Neurological, Cutaneous, Articular Syndrome (CINCA), −Severe forms of Familial Cold Autoinflammatory Syndrome (FCAS) / Familial Cold Urticaria (FCU) presenting with signs and symptoms beyond cold-induced urticarial skin rash • Symptomatic treatment of adults patients with frequent gouty arthritis attacks (at least 3 attacks in the previous 12 months) in whom non-steroidal anti-inflammatory drugs (NSAIDs) and colchicine are contraindicated, are not tolerated, or do not provide an adequate response, and in whom repeated courses of corticosteroids are not appropriate • Treatment of active Systemic Juvenile Idiopathic Arthritis (SJIA) in patients aged 2 years and older who have responded inadequately to previous therapy with non-steroidal anti-inflammatory drugs (NSAIDs) and systemic corticosteroids. Ilaris can be given as monotherapy or in combination with methotrexate | Full | TA281 | Canakinumab for treating gouty arthritis attacks and reducing the frequency of subsequent attacks | Terminated |
| canakinumab | exceptional | • Cryopyrin-Associated Periodic Syndromes (CAPS) in adults, adolescents and children aged 4 years and older with body weight above 15 kg, including: − Muckle-Wells Syndrome (MWS), - Neonatal-Onset Multisystem Inflammatory Disease (NOMID) / Chronic Infantile Neurological, Cutaneous, Articular Syndrome (CINCA), −Severe forms of Familial Cold Autoinflammatory Syndrome (FCAS) / Familial Cold Urticaria (FCU) presenting with signs and symptoms beyond cold-induced urticarial skin rash • Symptomatic treatment of adults patients with frequent gouty arthritis attacks (at least 3 attacks in the previous 12 months) in whom non-steroidal anti-inflammatory drugs (NSAIDs) and colchicine are contraindicated, are not tolerated, or do not provide an adequate response, and in whom repeated courses of corticosteroids are not appropriate • Treatment of active Systemic Juvenile Idiopathic Arthritis (SJIA) in patients aged 2 years and older who have responded inadequately to previous therapy with non-steroidal anti-inflammatory drugs (NSAIDs) and systemic corticosteroids. Ilaris can be given as monotherapy or in combination with methotrexate | Full | TA302 | Canakinumab for treating systemic juvenile idiopathic arthritis | Terminated |
| ofatumumab | conditional | treatment of chronic lymphocytic leukaemia (CLL) in patients refractory to fludarabine and alemtuzumab | Withdrawn | TA202 | Ofatumumab for the treatment of chronic lymphocytic leukaemia refractory to fludarabine and alemtuzumab | Withdrawn |
| pazopanib | conditional | first line treatment of advanced Renal Cell Carcinoma (RCC) and for patients who have received prior cytokine therapy for advanced disease | Full | TA215 | Pazopanib for the first-line treatment of advanced renal cell carcinoma | Recommended with patient access scheme |
| vandetanib | conditional | treatment of aggressive and symptomatic medullary thyroid cancer (MTC) in patients with unresectable locally advanced or metastatic disease. | Conditional | TA550 | Cabozantinib and vandetanib for treating unresectable locally advanced or metastatic medullary thyroid cancer | Not recommended |
| pixantrone dimaleate | conditional | monotherapy for the treatment of adult patients with multiply relapsed or refractory aggressive Non Hodgkin B cell Lymphomas (NHL). | Conditional | TA306 | Pixantrone monotherapy for treating multiply relapsed or refractory aggressive non-Hodgkin's B‑cell lymphoma | Recommended with patient access scheme |
| crizotinib | conditional | treatment of adults with previously treated anaplastic lymphoma kinase (ALK)-positive advanced non-small cell lung cancer (NSCLC). | Full | TA296 & TA422 | Crizotinib for previously treated non-small-cell lung cancer associated with an anaplastic lymphoma kinase fusion gene | Not recommended (TA296)  Recommended with patient access scheme (TA422) |
| brentuximab vedotin | conditional | treatment of adult patients with relapsed or refractory CD30+ Hodgkin lymphoma (HL): 1. following autologous stem cell transplant (ASCT) or 2. following at least two prior therapies when ASCT or multi-agent chemotherpay are not a treatment option ADCETRIS is indicated for the treatment of adult patients with relapsed or refractory systemic anaplastic large cell lymphoma (sALCL). | Conditional | TA446 & TA524 | Brentuximab vedotin for treating CD30-positive Hodgkin lymphoma | Recommended within the Cancer Drugs Fund (TA446)  Recommended with Commercial access arrangement (TA524) |
| brentuximab vedotin | conditional | treatment of adult patients with relapsed or refractory CD30+ Hodgkin lymphoma (HL): 1. following autologous stem cell transplant (ASCT) or 2. following at least two prior therapies when ASCT or multi-agent chemotherpay are not a treatment option ADCETRIS is indicated for the treatment of adult patients with relapsed or refractory systemic anaplastic large cell lymphoma (sALCL). | Conditional | TA478 | Brentuximab vedotin for treating relapsed or refractory systemic anaplastic large cell lymphoma | Recommended with Commercial access arrangement |
| bosutinib | conditional | treatment of adult patients with chronic phase (CP), accelerated phase (AP), and blast phase (BP) Philadelphia chromosome positive chronic myelogenous leukaemia (Ph+ CML) previously treated with one or more tyrosine kinase inhibitor(s) and for whom imatinib, nilotinib and dasatinib are not considered appropriate treatment options | Conditional | TA299 & TA401 | Bosutinib for previously treated chronic myeloid leukaemia | Not recommended (T299)  Recommended with patient access scheme (TA401) |
| vismodegib | conditional | treatment of adult patients with: - Symptomatic metastatic basal cell carcinoma - Locally advanced basal cell carcinoma inappropriate for surgery or radiotherapy | Full | TA489 | Vismodegib for treating basal cell carcinoma | Not recommended |
| cabozantinib | conditional | treatment of adult patients with progressive, unresectable locally advanced or metastatic medullary thyroid carcinoma. | Conditional | TA516 | Cabozantinib and vandetanib for treating unresectable locally advanced or metastatic medullary thyroid cancer | Recommended with patient access scheme |
| ataluren | conditional | treatment of Duchenne muscular dystrophy resulting from a nonsense mutation in the dystrophin gene, in ambulatory patients aged 5 years and older. Efficacy has not been demonstrated in non-ambulatory patients. | Conditional | HST3 | Ataluren for treating Duchenne muscular dystrophy with a nonsense mutation in the dystrophin gene | Recommended with Managed Access Agreement |
| afamelanotide | exceptional | prevention of phototoxicity in adult patients with erythropoietic protoporphyria (EPP) | Exceptional | ID927 | Afamelanotide for treating erythropoietic protoporphyria | Ongoing |
| ex vivo expanded autologous human corneal epithelial cells containing stem cells | conditional | Treatment of adult patients with moderate to severe limbal stem cell deficiency (defined by the presence of superficial corneal neovascularisation in at least two corneal quadrants, with central corneal involvement, and severely impaired visual acuity), unilateral or bilateral, due to physical or chemical ocular burns. | Conditional | TA467 | Holoclar for treating limbal stem cell deficiency after eye burns | Recommended with patient access scheme |
| ceritinib | conditional | treatment of adult patients with anaplastic lymphoma kinase (ALK) positive advanced non-small cell lung cancer (NSCLC) previously treated with crizotinib. | Full | TA395 | Ceritinib for previously treated anaplastic lymphoma kinase positive non-small-cell lung cancer | Recommended with patient access scheme |
| asfotase alfa | exceptional | long-term enzyme replacement therapy in patients with paediatric-onset hypophosphatasia to treat the bone manifestations of the disease | Exceptional | HST6 | Asfotase alfa for treating paediatric-onset hypophosphatasia | Recommended with Managed Access Agreement |
| blinatumomab | conditional | adults with Philadelphia chromosome negative relapsed or refractory B-precursor acute lymphoblastic leukaemia (ALL) | Full | ID1008 | Blinatumomab for treating Philadelphia-chromosome-positive relapsed or refractory acute lymphoblastic leukaemia | Recommended with patient access scheme |
| osimertinib mesylate | conditional | treatment of adult patients with locally advanced or metastatic epidermal growth factor receptor (EGFR) T790M mutation-positive non-small-cell lung cancer (NSCLC) | Full | TA416 | Osimertinib for treating locally advanced or metastatic EGFR T790M mutation-positive non-small-cell lung cancer | Recommended within the Cancer Drugs Fund |
| daratumumab | conditional | monotherapy for the treatment of adult patients with relapsed and refractory multiple myeloma, whose prior therapy included a proteasome inhibitor and an immunomodulatory agent and who have demonstrated disease progression on the last therapy | Full | TA510 | Daratumumab for multiple myeloma | Recommended within the Cancer Drugs Fund |
| Clofarabine | exceptional | Treatment of acute lymphoblastic leukaemia (ALL) in paediatric patients who have relapsed or are refractory after receiving at least two prior regimens and where there is no other treatment option anticipated to result in a durable response | Exceptional | ID1033 | Clofarabine for treating acute lymphoblastic leukaemia in children after 2 therapies | Discontinued |