Supplemental Material

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Hematology/Oncology (cancer) Approvals & Safety Notifications: Previous News Items

2009

- FDA granted approval to romidepsin for injection (ISTODAX, Gloucester Pharmaceuticals Inc.) for the treatment of cutaneous T-cell lymphoma (CTCL) in patients who have received at least one prior systemic therapy. More Information (November 6, 2009)
- FDA granted accelerated approval to ofatumumab (Arzerra, GlaxoSmithKline) for the treatment of patients with chronic lymphocytic leukemia (CLL) refractory to fludarabine and alemtuzumab. More Information (October 26, 2009)
- FDA granted approval to pazopanib tablets (VOTRIENTTM, GlaxoSmithKline) for the treatment of patients with advanced renal cell carcinoma. More Information (October 19, 2009)
- FDA granted accelerated approval to pralatrexate injection (FOLOTYN, Allos Therapeutics, Inc.) for the treatment of patients with relapsed or refractory peripheral T-cell lymphoma (PTCL).More Information (September 24, 2009)
- FDA granted approval for the use of bevacizumab (Avastin, Genentech, Inc.) in combination with interferon alfa for the treatment of patients with metastatic renal cell carcinoma. More Information (July 31, 2009)
- FDA implemented Class Labeling Changes to anti-EGFR monoclonal antibodies, cetuximab (Erbitux) and panitumumab (Vectibix): *KRAS* Mutations changes were made to the product labels of cetuximab (Erbitux ImClone Systems, Branchburg, NJ) and panitumumab (Vectibix Amgen, Thousand Oaks, CA). More Information (July 17, 2009)
- FDA approved pemetrexed (Alimta) for maintenance treatment of locally advanced or metastatic nonsquamous non-small cell lung cancer patients with no disease progression after four cycles of platinum-based first-line chemotherapy. More Information (July 2, 2009)
- FDA approves ferumoxytol (Feraheme Injection, AMAG Pharmaceuticals, Inc.) for the treatment of iron deficiency anemia in adult patients with chronic kidney disease (CKD). Ferumoxytol is an iron-containing product for intravenous (IV) administration.More Information (June 30, 2009)
- FDA granted accelerated approval to bevacizumab injection (Avastin, Genentech, Inc.) as a single agent for patients with glioblastoma, with progressive disease following prior therapy.More Information (May 5, 2009)
- FDA approves everolimus tablets (AFINITOR, Novartis) for treatment of patients with advanced renal cell carcinoma after failure of treatment with sunitinib or sorafenib More Information(March 31, 2009)

2010 Notifications

- FDA granted approval for denosumab (Xgeva, Amgen Inc.) for the prevention of skeletal-related events (SREs) in patients with bone metastases from solid tumors. Denosumab is not indicated for the prevention of SREs in patients with multiple myeloma. More Information (November 18, 2010)
- FDA granted approval for eribulin mesylate (Halaven Injection, Eisai Inc.) for the treatment of patients with metastatic breast cancer who have previously received an anthracycline and a taxane in either the adjuvant or metastatic setting, and at least two chemotherapeutic regimens for the treatment of metastatic disease. More Information (November 15, 2010)
- This notice is to inform you of important new safety information regarding the already labeled adverse reaction of oligohydramnios (decreased amniotic fluid). Based on post-marketing adverse event reports, cases of oligohydramnios sequence manifesting as pulmonary hypoplasia, skeletal abnormalities, and neonatal death have been reported in the offspring of mothers exposed to trastuzumab (Herceptin, Genentech Incorporated) during pregnancy. More Information (November 1, 2010)
- FDA granted accelerated approval to everolimus (Afinitor, Novartis), an mTOR inhibitor, for patients with subependymal giant cell astrocytoma (SEGA) associated with tuberous sclerosis (TS) who require therapy but are not candidates for surgical resection. Afinitor was originally approved in March 2009 for the treatment of adult patients with advanced renal cell carcinoma (RCC) whose disease was resistant to sunitinib or sorafenib. More Information (October 29, 2010)
- FDA granted accelerated approval to dasatinib (Sprycel, Bristol-Myers Squibb), an orally administered kinase inhibitor, for the treatment of newly diagnosed adult patients with Philadelphia chromosome positive chronic myeloid leukemia in chronic phase (CP-CML). The recommended dasatinib dose for this indication is 100 mg orally once daily. Sprycel was originally approved in June 2006 for the treatment of adult patients with CP-CML resistant or intolerant to prior therapy that included imatinib. More Information (October 28, 2010)
- FDA granted approval for trastuzumab (Herceptin), Genentech, Inc.), in combination with cisplatin and a fluoropyrimidine (capecitabine or 5â€fluorouracil), for the treatment of patients with HER2 overexpressing metastatic gastric or gastroesophageal (GE) junction adenocarcinoma, who have not received prior treatment for metastatic disease. More Information. (October 20, 2010)
- This communication is to inform members of the oncology community of recent changes in the measurement of serum creatinine which may have an impact on carboplatin dosing. Based on preliminary communications with the National Cancer Institute/Cancer Therapy Evaluation Program, a potential safety issue with carboplatin dosing has been identified. More Information (October 10, 2010).
- Pfizer Inc., in agreement with the FDA, announced that the commercial marketing of Mylotarg (gemtuzumab ozogamicin) will be voluntarily discontinued, and the new drug application (NDA) for Mylotarg will be withdrawn as of October 15, 2010. More Information (June 21, 2010)
- FDA granted accelerated approval to nilotinib (Tasigna Capsules, Novartis Pharmaceuticals Corporation), an orally administered kinase inhibitor, for the treatment of adult patients with newly diagnosed Philadelphia chromosome positive chronic myeloid leukemia (Ph+ CML) in chronic phase (CP-CML). More information(June 17, 2010)
- FDA approved cabazitaxel (Jevtana Injection, sanofi-aventis) for use in combination with prednisone for treatment of patients with metastatic hormone-refractory prostate cancer (mHRPC) previously treated with a docetaxel-containing regimen. More Information (June 17, 2010)
- FDA approved hexaminolevulinate hydrochloride (Cysview for Intravesical Solution, Photocure ASA), as an optical imaging agent for use in combination with the Karl Storz Photodynamic Diagnostic D-Light C (PDD) System for cystoscopic detection of non-muscle invasive papillary cancer of the bladder for patients suspected or known to have lesion(s) on the basis of a prior cystoscopy. More Information (May 28, 2010)
- FDA approved erlotinib (Tarceva) tablets for maintenance treatment of patients with locally advanced or metastatic non-small cell lung cancer (NSCLC) whose disease has not progressed after four cycles of platinum-based first-line chemotherapy. More Information (April 16, 2010)
- FDA has been informed by Lundbeck, Inc., the sole worldwide manufacturer of Mustargen (mechlorethamine HCl for injection), that there will be an extended supply shortage of this drug. More Information (April 9, 2010)
- FDA granted approval to rituximab (Rituxan, Genentech), in combination with fludarabine and cyclophosphamide (FC), for the treatment of previously untreated and previously treated patients with chronic lymphocytic leukemia (CLL). More Information (February 18, 2010)
- FDA approved a risk evaluation and mitigation strategy (REMS) to ensure the safe use of Erythropoiesis-Stimulating Agents (ESAs). The medications included in the program are marketed by Amgen under the names Aranesp (darbepoetin alfa) and Epogen (epoetin alfa) and by Centocor Ortho Biotech Products under the name Procrit (epoetin alfa). FDA required Amgen, the manufacturer of these products, to develop the REMS based on studies demonstrating that use of ESAs can increase the risk of tumor growth and shorten survival in patients with cancer. More Information (February 16, 2010)
- FDA granted accelerated approval to Tykerb (lapatinib) tablets for use in combination with letrozole tablets for the treatment of postmenopausal women with hormone receptor positive metastatic breast cancer that overexpresses the HER2 receptor for whom hormonal therapy is indicated. More Information (January 29, 2010)

2011 Notifications

- FDA has approved changes to the package inserts for methotrexate products for intravenous administration. Additional information has been added to the WARNING section of the Label. More Information (December 21, 2011)
- FDA approved changes to the Risk Evaluation and Mitigation Strategies (REMS) for romiplostim (Nplate for subcutaneous injection, Amgen Inc.) and eltrombopag (Promacta tablets, GlaxoSmithKline LLC) to remove the restricted distribution programs and the safety reporting requirements that were part of the REMS. Prescriber, institution, pharmacy, and patient enrollment and these data collection requirements are no longer necessary. More Information (December 6, 2011)
- FDA approved asparaginase Erwinia chrysanthemi [Erwinaze, injection, EUSA Pharma (USA), Inc.] as a component of a multi-agent chemotherapeutic regimen for the treatment of patients with acute lymphoblastic leukemia (ALL) who have developed hypersensitivity to E. coli-derived asparaginase. More Information(November 18, 2011)
- FDA approved ruxolitinib (Jakafi oral tablets, Incyte Corporation) for the treatment of intermediate and high risk myelofibrosis, including primary myelofibrosis, post-polycythemia vera myelofibrosis and post-essential thrombocythemia myelofibrosis. Results of two randomized controlled trials in patients with intermediate or high risk myelofibrosis comparing ruxolitinib to placebo (Study 1) or to best available therapy (Study 2) were the basis of approval. More Information (November 16, 2011)
- FDA approved cetuximab (Erbitux, ImClone LLC, a wholly-owned subsidiary of Eli-Lilly and Company) in combination with platinum-based therapy plus 5-florouracil (5-FU) for the first-line treatment of patients with recurrent locoregional disease and/or metastatic squamous cell carcinoma of the head and neck (SCCHN). More Information (November 7, 2011)
- FDA granted accelerated approval to deferiprone (Ferriprox Tablets, ApoPharma, Inc.), an oral iron chelator for the treatment of patients with transfusional iron overload due to thalassemia syndromes when current chelation therapy is inadequate. More Information (October 14, 2011)
- Changes in bevacizumab (Avastin, Genentech, Inc.) package insert regarding: risk of ovarian failure, osteonecrosis of the jaw, risk of venous thromboembolic event (VTE) and bleeding in patients receiving anticoagulation therapy after first VTE event. More Information (September 30, 2011)
- FDA granted accelerated approval for the use of eculizumab (Soliris, Alexion, Inc.) for the treatment of pediatric and adult patients with atypical hemolytic uremic syndrome (aHUS). More Information (September 23, 2011)
- FDA granted approval for denosumab (Prolia, Amgen Inc.) as a treatment to increase bone mass in patients at high risk for fracture receiving androgen deprivation therapy (ADT) for nonmetastatic prostate cancer or adjuvant aromatase inhibitor (AI) therapy for breast cancer. In men with nonmetastatic prostate cancer, denosumab also reduced the incidence of vertebral fracture. More Information (September 16, 2011)
- FDA granted accelerated approval to granted accelerated approval to crizotinib (XALKORI Capsules, Pfizer Inc.) for the treatment of patients with locally advanced or metastatic non-small cell lung cancer (NSCLC) that is anaplastic lymphoma kinase (ALK)-positive as detected by an FDA-approved test. The FDA approved the Vysis ALK Break-Apart FISH Probe Kit (Abbott Molecular, Inc.) concurrently with the crizotinib approval. This companion diagnostic test is designed to detect rearrangements of the anaplastic lymphoma kinase (ALK) gene in NSCLC. More Information (August 26, 2011)
- FDA granted accelerated approval to brentuximab vedotin (Adcetris for Injection, Seattle Genetics, Inc.) for two indications: (1) treatment of patients with Hodgkin lymphoma after failure of autologous stem cell transplant (ASCT) or after failure of at least two prior multi-agent chemotherapy regimens in patients who are not ASCT candidates and (2) treatment of patients with systemic anaplastic large cell lymphoma (ALCL) after failure of at least one prior multi-agent chemotherapy regimen. More Information (August 19, 2011)
- FDA approved vemurafenib tablets (ZELBORAF, Hoffmann-La Roche Inc.) for the treatment of patients with unresectable or metastatic melanoma with the $BRAF^{V600E}$ mutation as detected by an FDA-approved test. More Information (August 17, 2011)
- FDA approved sunitinib (Sutent Capsules, Pfizer, Inc.) for the treatment of progressive, well-differentiated pancreatic neuroendocrine tumors (pNET) in patients with unresectable, locally advanced, or metastatic disease. More Information (May 20, 2011)
- FDA approved everolimus (Afinitor Tablets, Novartis Pharmaceuticals Corporation), for the treatment of progressive neuroendocrine tumors of pancreatic origin (PNET) in patients with unresectable, locally advanced or metastatic disease. The safety and effectiveness of everolimus in the treatment of patients with carcinoid tumors have not been established. More Information (May 5, 2011)
- FDA approved abiraterone acetate (Zytiga Tablets, Centocor Ortho Biotech, Inc.) for use in combination with prednisone for the treatment of patients with metastatic castration-resistant prostate cancer (mCRPC) who have received prior chemotherapy containing docetaxel. More Information (April 28, 2011)
- FDA approved vandetanib tablets (Vandetanib Tablets, AstraZeneca Pharmaceuticals LP), a kinase inhibitor, for the treatment of symptomatic or progressive medullary thyroid cancer in patients with unresectable, locally advanced, or metastatic disease. The use of vandetanib in patients with indolent, asymptomatic or slowly progressing disease should be carefully considered because of the treatment-related risks of vandetanib. More Information (April 6, 2011)
- FDA approved peginterferon alfa-2b (Sylatron, Schering Corporation, Kenilworth, NJ 07033), for the treatment of patients with melanoma with microscopic or gross nodal involvement within 84 days of definitive surgical resection including complete lymphadenectomy. More Information (March 29, 2011)
- FDA approved ipilimumab injection (YERVOY, Bristol-Myers Squibb Company) for the treatment of unresectable or metastatic melanoma. More Information. (March 25, 2011)
- FDA approved rituximab (Rituxan, Genentech, Inc) for maintenance therapy for patients with previously untreated follicular, CD-20 positive, B-cell non-Hodgkin lymphoma who achieve a response to rituximab in combination with chemotherapy. More Information (January 28, 2011)

2012 Notifications

- FDA granted accelerated approval to ponatinib (Iclusig tablets, ARIAD Pharmaceuticals, Inc.) for the treatment of adult patients with chronic phase, accelerated phase, or blast phase chronic myeloid leukemia (CML) that is resistant or intolerant to prior tyrosine kinase inhibitor therapy or Philadelphia chromosome positive acute lymphoblastic leukemia (Ph+ALL) that is resistant or intolerant to prior tyrosine kinase inhibitor therapy. More Information. December 17, 2012
- FDA approved an expanded indication for abiraterone acetate (Zytiga Tablets, Janssen Biotech, Inc.) in combination with prednisone for the treatment of patients with metastatic castration-resistant prostate cancer.More Information. December 10, 2012
- FDA approved cabozantinib (COMETRIQ capsules, Exelixis, Inc), for the treatment of patients with progressive metastatic medullary thyroid cancer (MTC). Cabozantinib is a small molecule that inhibits the activity of multiple tyrosine kinases, including RET, MET, and VEGF receptor 2. More Information. November 29, 2012
- FDA granted accelerated approval to omacetaxine mepesuccinate, (SYNRIBO for Injection, for subcutaneous use, Teva Pharmaceutical Industries Ltd.), for the treatment of adult patients with chronic or accelerated phase chronic myeloid leukemia (CML) with resistance and/or intolerance to two or more tyrosine kinase inhibitors (TKIs). More Information. October 26, 2012
- FDA approved a 90-minute infusion for rituximab (Rituxan Injection, Genentech, Inc.) starting at Cycle 2 for patients with non-Hodgkin's lymphoma (NHL) who did not experience a grade 3 or 4 infusion-related adverse reaction during Cycle 1. Patients with clinically significant cardiovascular disease and high circulating lymphocyte counts (≥5000/mcL) are not recommended to receive the faster infusion. More Information. October 19, 2012
- FDA expanded labeling to include the results of an additional trial evaluating the safety and efficacy of pemetrexed (ALIMTA, Eli Lilly and Company) for the initial treatment of patients with locally advanced or metastatic, non-squamous, non-small cell lung cancer followed by pemetrexed maintenance in patients whose disease has not progressed after four cycles of platinum and pemetrexed as first-line chemotherapy. More Information. October 17, 2012
- FDA approved paclitaxel protein-bound particles for injectable suspension, albumin-bound (ABRAXANE for Injectable Suspension; Abraxis Bioscience a wholly owned subsidiary of Celgene Corporation) for use in combination with carboplatin for the initial treatment of patients with locally advanced or metastatic non-small cell lung cancer (NSCLC) who are not candidates for curative surgery or radiation therapy. More Information. October 11, 2012
- FDA approved regorafenib (Stivarga tablets, Bayer HealthCare Pharmaceuticals, Inc.), for the treatment of patients with metastatic colorectal cancer (mCRC) who have been previously treated with fluoropyrimidine-, oxaliplatin-, and irinotecan-based chemotherapy, an anti-VEGF therapy, and, if KRAS wild type, an anti-EGFR therapy. More Information. September 27, 2012
- FDA approved bosutinib tablets (Bosulif, Pfizer, Inc.) for the treatment of chronic, accelerated, or blast phase Philadelphia chromosome positive (Ph+) chronic myelogenous leukemia (CML) in adult patients with resistance or intolerance to prior therapy. More Information. September 4, 2012
- FDA approved enzalutamide (XTANDI Capsules, Medivation, Inc. and Astellas Pharma US, Inc.), for the treatment of patients with metastatic castration-resistant prostate cancer who have previously received docetaxel. More Information. August 31, 2012
- FDA granted accelerated approval for everolimus tablets for oral suspension (Afinitor Disperz, Novartis Pharmaceuticals Corp.) for the treatment of pediatric and adult patients with tuberous sclerosis complex (TSC) who have subependymal giant cell astrocytoma (SEGA) that requires therapeutic intervention but cannot be curatively resected. More Information. August 30, 2012
- FDA granted accelerated approval for vinCRIStine sulfate LIPOSOME injection (Marqibo, Talon Therapeutics, Inc.) for the treatment of adult patients with Philadelphia chromosome-negative (Ph-) acute lymphoblastic leukemia (ALL) in second or greater relapse or whose disease has progressed following two or more anti-leukemia therapies. More Information. August 9, 2012
- FDA approved ziv-aflibercept injection (ZALTRAP, Sanofi U.S., Inc.) for use in combination with 5-fluorouracil, leucovorin, irinotecan (FOLFIRI) for the treatment of patients with metastatic colorectal cancer (mCRC) that is resistant to or has progressed following an oxaliplatin containing regimen. More Information. August 3, 2012
- FDA approved everolimus tablets (Afinitor®, Novartis Pharmaceuticals Corporation) for the treatment of postmenopausal women with advanced hormone receptor-positive, HER2-negative breast cancer in combination with exemestane, after failure of treatment with letrozole or anastrozole. More Information. July 20, 2012
- FDA granted accelerated approval to carfilzomib injection (Kyprolis, Onyx Pharmaceuticals), for the treatment of patients with multiple myeloma who have received at least two prior therapies, including bortezomib and an immunomodulatory agent, and have demonstrated disease progression on or within 60 days of the completion of the last therapy. More Information. July 20, 2012
- FDA granted approval to cetuximab (Erbitux, ImClone LLC, a wholly owned subsidiary of Eli Lilly and Co) for use in combination with FOLFIRI (irinotecan, 5-fluorouracil, leucovorin) for first-line treatment of patients with K-ras mutation-negative (wild-type), EGFR-expressing metastatic colorectal cancer (mCRC) as determined by FDA-approved tests for this use.
  FDA also approved the Therascreen KRAS RGQ PCR Kit (QIAGEN Manchester, Ltd) concurrent with this cetuximab approval. More Information. July 9, 2012
- FDA approved pertuzumab injection (PERJETA, Genentech, Inc.) for use in combination with trastuzumab and docetaxel for the treatment of patients with HER2-positive metastatic breast cancer who have not received prior anti-HER2 therapy or chemotherapy for metastatic disease. Pertuzumab is a recombinant humanized monoclonal antibody that targets the

extracellular dimerization domain (Subdomain II) of HER2, and thereby blocks ligand-dependent heterodimerization of HER2 with other HER family members, including EGFR, HER3 and HER4. More Information. June 8, 2012

- FDA approved pazopanib tablets (VOTRIENT, a registered Trademark of GlaxoSmithKline) for the treatment of patients with advanced soft tissue sarcoma (STS) who have received prior chemotherapy. The efficacy of pazopanib for the treatment of patients with adipocytic STS or gastrointestinal stromal tumors (GIST) has not been demonstrated. More Information. April 26, 2012
- FDA granted accelerated approval to everolimus (Afinitor tablets, Novartis) for the treatment of adults with renal angiomyolipoma, associated with tuberous sclerosis complex (TSC), who do not require immediate surgery. More Information. April 26, 2012
- FDA announced major actions taken to bolster the supply of Doxil (Janssen Research and Development LLC) and preservative-free formulations of methotrexate. In response to the critical shortage of Doxil (doxorubicin hydrochloride liposome injection) effective immediately FDA is exercising its enforcement discretion for the temporary importation and distribution of Sun Pharma Global's Lipodox. More Information February 21, 2012
- FDA granted regular approval for imatinib mesylate tablets (Gleevec, Novartis Pharmaceuticals) for the adjuvant treatment of adult patients following complete gross resection of Kit (CD117) positive Gastrointestinal Stromal Tumors (GIST). Accelerated approval for this indication was granted in December 2008. Labeling is also revised to include the results of a randomized trial demonstrating that recurrence-free survival (RFS) and overall survival (OS) were improved by continuing adjuvant imatinib therapy to 36 months. More Information(January 31, 2012)
- FDA approved vismodegib (ERIVEDGE Capsule, Genentech, Inc.) for the treatment of adults with metastatic basal cell carcinoma or with locally advanced basal cell carcinoma that has recurred following surgery or who are not candidates for surgery, and who are not candidates for radiation. More Information (January 30, 2012)
- FDA approved axitinib tablets (Inlyta, Pfizer, Inc.) for the treatment of advanced renal cell carcinoma after failure of one prior systemic therapy. More Information (January 27, 2012)
- FDA approved glucarpidase injection (Voraxaze, BTG International Inc.) for the treatment of toxic plasma methotrexate concentrations (> 1 µmol/L) in patients with delayed methotrexate clearance due to impaired renal function. More Information (January 17, 2012)

Hematology/Oncology (Cancer) Approvals & Safety Notifications

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2015

- FDA approved approved ramucirumab (CYRAMZA, Eli Lilly and Company) for use in combination with FOLFIRI for the treatment of patients with metastatic colorectal cancer (mCRC) whose disease has progressed on a first line bevacizumab-, oxaliplatin- and fluoropyrimidine-containing regimen. Ramucirumab is a recombinant human monoclonal IgG1 antibody that binds to the human vascular endothelial growth factor- receptor 2 (VEGF-R2), preventing the interaction of VEGF-R2 to its ligands. More Information. April 24, 2015
- FDA approved dinutuximab (Unituxin, United Therapeutics Corporation), in combination with granulocyte-macrophage colony-stimulating factor (GM-CSF), interleukin-2 (IL-2), and 13-cis-retinoic acid (RA), for the treatment of pediatric patients with high-risk neuroblastoma who achieve at least a partial response to prior first-line multiagent, multimodality therapy.More Information. March 10, 2015
- FDA approved filgrastim-sndz (ZARXIO Injection, Sandoz Inc.), as a biosimilar to US-licensed Neupogen for the five indications for which US-licensed Neupogen is approved. The formulation of ZARXIO differs from that of US-licensed Neupogen in one inactive component. More Information. March 6, 2015
- FDA granted approval to nivolumab (OPDIVO, Bristol-Myers Squibb Company) for the treatment of patients with metastatic squamous non-small cell lung cancer (NSCLC) with progression on or after platinum-based chemotherapy. More Information. March 4, 2015
- FDA granted accelerated approval to panobinostat (FARYDAK capsules, Novartis Pharmaceuticals) in combination with bortezomib and dexamethasone for the treatment of patients with multiple myeloma who have received at least two prior regimens, including bortezomib and an immunomodulatory agent. As a condition of this accelerated approval, FDA requires the sponsor to conduct a trial to verify and describe the clinical benefit of panobinostat for patients with multiple myeloma. More Information. February 23, 2015
- FDA approved lenvatinib (Lenvima) for the treatment of patients with locally recurrent or metastatic, progressive, radioactive iodine-refractory differentiated thyroid cancer. More Information. February 13, 2015
- FDA granted accelerated approval to palbociclib (IBRANCE, Pfizer, Inc.) for use in combination

with letrozole for the treatment of postmenopausal women with estrogen receptor (ER)-positive, human epidermal growth factor receptor 2 (HER2)-negative advanced breast cancer as initial endocrine-based therapy for their metastatic disease. More Information. February 3, 2015

- FDA approved ibrutinib (Imbruvica Capsules, Pharmacyclics, Inc.) for the treatment of patients with Waldenstrom's macroglobulinemia (WM). Ibrutinib was initially approved in November 2013 for the treatment of patients with mantle cell lymphoma (MCL) who have received at least one prior therapy. Ibrutinib also received approval in February 2014 for the treatment of chronic lymphocytic leukemia (CLL) in patients who received at least one prior therapy and in July 2014 for the treatment of CLL with 17p deletion. More Information. January 29, 2015

2014

- FDA granted accelerated approval to nivolumab (OPDIVO, Bristol-Myers Squibb Company) for the treatment of patients with unresectable or metastatic melanoma and disease progression following ipilimumab and, if BRAF V600 mutation positive, a BRAF inhibitor. More Information. December 22, 2014
- FDA approved olaparib capsules (Lynparza, AstraZeneca Pharmaceuticals LP) as monotherapy for the treatment of patients with deleterious or suspected deleterious germline BRCA mutated (gBRCAm) (as detected by an FDA-approved test) advanced ovarian cancer who have been treated with three or more prior lines of chemotherapy. More Information. December 19, 2014
- FDA approved lanreotide (Somatuline Depot Injection, Ipsen Pharma) for the treatment of patients with unresectable, well or moderately differentiated, locally advanced or metastatic gastroenteropancreatic neuroendocrine tumors (GEP-NETs) to improve progression-free survival. More Information. December 16, 2014
- FDA approved ramucirumab (Cyramza Injection, Eli Lilly and Company) for use in combination with docetaxel for the treatment of patients with metastatic non-small cell lung cancer (NSCLC) with disease progression on or after platinum-based chemotherapy. More Information. December 12, 2014
- FDA approved ruxolitinib (Jakafi, Incyte Corporation) for the treatment of patients with polycythemia vera (PV) who have had an inadequate response to or are intolerant of hydroxyurea (HU).More Information. December 4, 2014
- FDA granted accelerated approval for blinatumomab (BLINCYTO, Amgen Inc.) for the treatment of Philadelphia chromosome-negative relapsed or refractory B-cell precursor acute lymphoblastic leukemia (R/R ALL). More Information. December 3, 2014
- FDA approved bevacizumab solution for intravenous infusion (Avastin, Genentech, Inc.) in combination with paclitaxel, pegylated liposomal doxorubicin, or topotecan for the treatment of patients with platinum-resistant, recurrent epithelial ovarian, fallopian tube, or primary peritoneal cancer. More Information. November 14, 2014

- FDA approved ramucirumab (Cyramza, Eli Lilly and Company) for use in combination with paclitaxel for the treatment of patients with advanced gastric or gastroesophageal junction (GEJ) adenocarcinoma. Ramucirumab was approved in April, 2014 as a single agent for the treatment of patients with advanced gastric or GEJ adenocarcinoma refractory to or progressive following first-line therapy with platinum or fluoropyrimidine chemotherapy.More Information. November 5, 2014
- FDA granted accelerated approval to pembrolizumab (KEYTRUDA, Merck Sharp & Dohme Corp.) for the treatment of patients with unresectable or metastatic melanoma and disease progression following ipilimumab and, if BRAF V600 mutation positive, a BRAF inhibitor.More Information. September 4, 2014
- FDA approved bevacizumab solution for intravenous infusion (Avastin, Genentech, Inc.) for the treatment of persistent, recurrent or metastatic cervical cancer, in combination with paclitaxel and cisplatin or paclitaxel and topotecan. More Information. August 14, 2014
- FDA approved idelalisib (Zydelig tablets, GileadSciences, Inc.) for the treatment of patients with relapsed chronic lymphocytic leukemia (CLL), in combination with rituximab, for whom rituximab alone would be considered appropriate therapy due to other co-morbidities. More Information. July 23, 2014
- FDA granted accelerated approval to belinostat (BELEODAQ, Spectrum Pharmaceuticals, Inc.) for the treatment of patients with relapsed or refractory peripheral T-cell lymphoma (PTCL).More Information. July 3, 2014
- FDA granted accelerated approval to ceritinib (ZYKADIA, Novartis Pharmaceuticals Corporation) for the treatment of patients with anaplastic lymphoma kinase (ALK)-positive, metastatic non-small cell lung cancer (NSCLC) with disease progression on or who are intolerant to crizotinib. More Information. April 29, 2014
- FDA approved an oral suspension of mercaptopurine (Purixan,NOVA Laboratories Limited). Mercaptopurine is a 20 mg/ml oral suspension. Purixan is indicated for the treatment of patients with acute lymphoblastic leukemia (ALL) as part of a combination regimen. More Information. April 28, 2014
- FDA approved siltuximab (Sylvant Injection Janssen Biotech, Inc.), for the treatment of patients with multicentric Castleman's disease (MCD) who are human immunodeficiency virus (HIV-) - negative and human herpes virus -8 (HHV-8) -negative. More Information. April 23, 2014.
- FDA approved ramucirumab (Cyramza, Eli Lilly and Company) for use as a single agent for the treatment of patients with advanced or metastatic, gastric or gastroesophageal junction (GEJ) adenocarcinoma with disease progression on or after prior treatment with fluoropyrimidine- or platinum-containing chemotherapy. Ramucirumab is a recombinant monoclonal antibody of the IgG1 class that binds to vascular endothelial growth factor receptor-2 (VEGFR-2) and blocks the activation of the receptor. More Information. April 21, 2014
- FDA approved ofatumumab (Arzerra Injection, for intravenous infusion; GlaxoSmithKline) in

combination with chlorambucil, for the treatment of previously untreated patients with chronic lymphocytic leukemia (CLL), for whom fludarabine-based therapy is considered inappropriate. More Information. April 17, 2014

- FDA granted accelerated approval to ibrutinib (IMBRUVICA, Pharmacyclics, Inc.) for the treatment of patients with chronic lymphocytic leukemia (CLL) who have received at least one prior therapy. Ibrutinib previously received accelerated approval on November 13, 2013 for the treatment of patients with mantle cell lymphoma who have received at least one prior therapy. More Information. February 12, 2014

- FDA granted accelerated approval to trametinib (Mekinist tablets, GlaxoSmithKline, LLC) and dabrafenib (Tafinlar capsules, GlaxoSmithKline, LLC) for use in combination in the treatment of patients with unresectable or metastatic melanoma with a BRAF V600E or V600K mutation as detected by an FDA-approved test.More Information. January 10, 2014

2013

- FDA approved sorafenib (NEXAVAR tablets, Bayer Healthcare Pharmaceuticals Inc.) for the treatment of locally recurrent or metastatic, progressive, differentiated thyroid carcinoma (DTC) refractory to radioactive iodine treatment. Sorafenib was previously approved for treatment of renal cell carcinoma (2005) and hepatocellular carcinoma (2007) More Information. November 22, 2013
- FDA granted regular approval for crizotinib (Xalkori, Pfizer, Inc.) capsules for the treatment of patients with metastatic non-small cell lung cancer (NSCLC) whose tumors are anaplastic lymphoma kinase (ALK)-positive as detected by an FDA-approved test. More Information. November 20, 2013
- FDA granted accelerated approval to Ibrutinib (IMBRUVICA, Pharmacyclics, Inc.) for the treatment of patients with mantle cell lymphoma (MCL) who have received at least one prior therapy. More Information. November 13, 2013
- FDA approved obinutuzumab (GAZYVA injection, for intravenous use, Genentech, Inc.; previously known as GA101) for use in combination with chlorambucil for the treatment of patients with previously untreated chronic lymphocytic leukemia (CLL). More Information. November 1, 2013
- FDA has asked the manufacturer of the leukemia chemotherapy drug Iclusig (ponatinib) to suspend marketing and sales of Iclusig because of the risk of life-threatening blood clots and severe narrowing of blood vessels. More Information. October 31, 2013
- FDA provided information on the availability of doxorubicin hydrochloride liposome injection to supply the U.S. market. More Information. October 8, 2013
- FDA granted accelerated approval to pertuzumab injection (PERJETA, Genentech, Inc.) for use in combination with trastuzumab and docetaxel for the neoadjuvant treatment of patients with HER2-positive, locally advanced, inflammatory, or early stage breast cancer (either greater than 2 cm in diameter or node positive) as part of a complete treatment regimen for early breast cancer. More Information. September 30, 2013

- FDA approved paclitaxel protein-bound particles (albumin-bound) (Abraxane for injectable suspension, Abraxis BioScience, LLC, a wholly owned subsidiary of Celgene Corporation), in combination with gemcitabine for the first-line treatment of patients with metastatic adenocarcinoma of the pancreas. More Information. September 6, 2013
- FDA approved afatinib (Gilotrif tablets, Boehringer Ingelheim Pharmaceuticals, Inc.), for the first-line treatment of patients with metastatic non-small cell lung cancer (NSCLC) whose tumors have epidermal growth factor receptor (EGFR) exon 19 deletions or exon 21 (L858R) substitution mutations as detected by an FDA-approved test. The safety and efficacy of afatinib have not been established in patients whose tumors have other EGFR mutations. Concurrent with this action, FDA approved the therascreen EGFR RGQ PCR Kit (QIAGEN) for detection of EGFR exon 19 deletions or exon 21 (L858R) substitution mutations.More Information. July 12, 2013
- FDA approved denosumab (Xgeva injection, for subcutaneous use, Amgen Inc.) for the treatment of adults and skeletally mature adolescents with giant cell tumor of bone that is unresectable or where surgical resection is likely to result in severe morbidity. More Information. June 13, 2013
- FDA approved lenalidomide capsules (REVLIMID, Celgene Corporation), for the treatment of patients with mantle cell lymphoma (MCL) whose disease has relapsed or progressed after two prior therapies, one of which included bortezomib. More Information. June 5, 2013
- FDA approved trametinib (MEKINIST tablet, GlaxoSmithKline, LLC), for the treatment of patients with unresectable or metastatic melanoma with BRAF V600E or V600K mutation as detected by an FDA-approved test. More Information. May 29, 2013
- FDA approved dabrafenib (TAFINLAR capsule, GlaxoSmithKline, LLC), for the treatment of patients with unresectable or metastatic melanoma with BRAF V600E mutation as detected by an FDA-approved test. More Information. May 29, 2013
- FDA approved radium Ra 223 dichloride (Xofigo Injection, Bayer HealthCare Pharmaceuticals Inc.) for the treatment of patients with castration-resistant prostate cancer, symptomatic bone metastases and no known visceral metastatic disease. Xofigo is an alpha-particle emitting radiotherapeutic drug which mimics calcium and forms complexes with hydroxyapatite at areas of increased bone turnover, such as bone metastases. More Information. May 15, 2013
- FDA approved erlotinib (Tarceva, Astellas Pharma Inc.) for the first-line treatment of metastatic non-small cell lung cancer (NSCLC) patients whose tumors have epidermal growth factor receptor (EGFR) exon 19 deletions or exon 21 (L858R) substitution mutations. This indication for erlotinib is being approved concurrently with the cobas EGFR Mutation Test, a companion diagnostic test for patient selection. More Information. May 14, 2013
- FDA approved ado-trastuzumab emtansine (KADCYLA for injection, Genentech, Inc.), for use as a single agent for the treatment of patients with HER2-positive, metastatic breast cancer who previously received trastuzumab and a taxane, separately or in combination. Patients should have either received prior therapy for metastatic disease or developed disease recurrence during or within six months of completing adjuvant therapy. More Information. February 22,2013

- FDA granted accelerated approval to pomalidomide (POMALYST capsules, Celgene Corporation) for the treatment of patients with multiple myeloma who have received at least two prior therapies, including lenalidomide and bortezomib, and have demonstrated disease progression on or within 60 days of completion of the last therapy. More Information. February 8, 2013
- FDA approved doxorubicin hydrochloride liposome injection (Sun Pharma Global FZE), a generic version of DOXIL Injection (doxorubicin hydrochloride liposome; Janssen Products, L.P.) for the treatment of ovarian cancer in patients whose disease has progressed or recurred after platinum-based chemotherapy and for AIDS-related Kaposi's sarcoma after failure of prior systemic chemotherapy or intolerance to such therapy. More Information. February 4, 2013
- FDA approved bevacizumab (Avastin, Genentech U.S., Inc.) for use in combination with fluoropyrimidine-irinotecan or fluoropyrimidine-oxaliplatin based chemotherapy for the treatment of patients with metastatic colorectal cancer (mCRC) whose disease has progressed on a first-line bevacizumab-containing regimen. More Information. January 23, 2013

Search terms for literature search for accelerated approvals

1. Melanoma

Surrogate: ORR

Pubmed

(melanoma[tiab]) AND (objective response[tiab] OR response rate[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

Google scholar

allintitle: melanoma (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

2. Follicular or SLL

Surrogate: ORR

Pubmed

(follicular[tiab] OR small lymphocytic[tiab]) AND (objective response[tiab] OR response rate[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

Google scholar

allintitle: Follicular (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

allintitle: “Small lymphocytic” (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

3. PTCL

Surrogate: ORR

Pubmed

(T cell lymphoma[tiab]) AND (objective response[tiab] OR response rate[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

Google scholar

allintitle: “T cell lymphoma” (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

4. NSCLC

Surrogate: ORR

Pubmed

(non small cell lung[tiab])AND (objective response[tiab] OR response rate[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

Google scholar
allintitle: non small cell (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

5. CLL
Surrogate: ORR

Pubmed
(CLL[tiab] or chronic lymphocytic leukemia[tiab])AND (objective response[tiab] OR response rate[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

Google scholar
allintitle: chronic lymphocytic (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

6. Mantle cell lymphoma
Surrogate: ORR

Pubmed
(Mantle cell[tiab])AND (objective response[tiab] OR response rate[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

Google scholar
allintitle: mantle (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

7. Breast cancer
Surrogate: pCR, PFS

Pubmed
(Breast cancer[tiab]) AND (objective response[tiab] OR response rate[tiab] OR pathologic complete response[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

Google scholar

allintitle: Breast cancer (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “pathologic complete” OR “meta-analysis”)

8. Multiple myeloma
Surrogate: ORR

Pubmed
(Myeloma[tiab]) AND (objective response[tiab] OR response rate[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

Google scholar
allintitle: myeloma (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

9. CML
Surrogate: Major molecular response (MMR), Major Cytogenetic Response (MCyR) and Major Hematologic Response (MaHR), Confirmed complete cytogenetic response (CCyR)

Pubmed
(CML[tiab] OR chronic myeloid leukemia[tiab]) AND (objective response[tiab] OR response rate[tiab] OR cytogenetic response[tiab] OR molecular response[tiab] OR hematologic response[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

Google scholar
allintitle: CML (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "response rate" OR “molecular response” OR “cytogenetic response” OR “hematologic response” OR “meta-analysis”)

10. Tuberous sclerosis with subependymal giant cell astrocytoma
Surrogate: ORR

Pubmed
(astrocytoma [tiab]) AND (objective response[tiab] OR response rate[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

Google scholar
allintitle: astrocytoma (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR “SEGA” OR "response rate" OR “meta-analysis”)

11. Ph- ALL
Surrogate: CR, CRi

Pubmed
(ALL[tiab] OR acute lymphoblastic leukemia[tiab]) AND (objective response[tiab] OR response rate[tiab] OR cytogenetic response[tiab] OR molecular response[tiab] OR hematologic response[tiab] OR complete remission[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

Google scholar
allintitle: lymphoblastic leukemia (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR “complete remission” OR "response rate" OR “meta-analysis”)

12. Tuberous sclerosis with renal angiomyolipoma
Surrogate: ORR

Pubmed
(angiomyolipoma [tiab]) AND (objective response[tiab] OR response rate[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

Google scholar
allintitle: angiomyolipoma (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

13. Hodgkin’s lymphoma and anaplastic large B cell lymphoma
Surrogate: ORR

Pubmed
(Hodgkin[tiab] OR Hodgkin's[tiab] OR anaplatic large[tiab])AND (objective response[tiab] OR response rate[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

Google scholar
allintitle: Hodgkin (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

allintitle: Hodgkin’s (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

allintitle: Anaplastic (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

14. Glioblastoma
Surrogate: ORR

Pubmed
(Glioblastoma[tiab])AND (objective response[tiab] OR response rate[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

Google scholar
allintitle: Glioblastoma (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

Search terms for literature search for regular approvals

1. Renal cell carcinoma

Surrogate: PFS

Pubmed

(Renal cell[tiab]) AND (objective response[tiab] OR response rate[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

Google scholar

allintitle: “Renal cell” (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

2. CTCL

Surrogate: ORR

Pubmed

(T cell lymphoma[tiab]) AND (objective response[tiab] OR response rate[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

Google scholar

allintitle: “T cell lymphoma” (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

3. Medullary thyroid cancer

Surrogate: PFS

Pubmed

(Thyroid cancer[tiab]) AND (objective response[tiab] OR response rate[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

Google scholar

allintitle: “Thyroid cancer” (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

4. GEP-NET

Surrogate: PFS

Pubmed

(Neuroendocrine[tiab]) AND (objective response[tiab] OR response rate[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

Google scholar

allintitle: “Neuroendocrine” (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

5. Basal cell carcinoma

Surrogate: ORR

Pubmed

(Basal cell[tiab]) AND (objective response[tiab] OR response rate[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR stud*[tiab])

Google scholar

allintitle: “Basal cell” (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

6. Soft tissue sarcoma

Surrogate: PFS

Pubmed

(sarcoma[tiab]) AND (objective response[tiab] OR response rate[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

Google scholar

allintitle: “sarcoma” (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

7. Ovarian cancer

Surrogate: ORR

Pubmed

(Ovarian cancer[tiab]) AND (objective response[tiab] OR response rate[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

Google scholar

allintitle: “Ovarian” (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

8. Ovarian/fallopian/primary peritoneal

Surrogate: PFS

(Fallopian[tiab]) AND (objective response[tiab] OR response rate[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

(Peritoneal[tiab]) AND (objective response[tiab] OR response rate[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

allintitle: “Fallopian” (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

allintitle: “Peritoneal” (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

9. Giant cell

Surrogate: ORR and duration of response

Pubmed

(Giant cell[tiab]) AND (objective response[tiab] OR response rate[tiab] OR overall survival[tiab] OR progression free[tiab] OR disease free[tiab] OR endpoint*[tiab] OR surroga*[tiab]) AND (review[tiab] OR search[tiab] OR meta-analys*[tiab] OR systematic[tiab] OR validation[tiab] OR correlation[tiab] OR association[tiab] OR relationship[tiab] OR pooled[tiab]) AND (trial[tiab] OR studies[tiab])

Google scholar

allintitle: “Giant cell” (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response rate" OR “meta-analysis”)

10. Multicentric Castleman’s disease

Surrogate: ORR and duration of response

Pubmed

(Castleman's[tiab] OR Castleman[tiab]) AND (response[tiab])

Google scholar

allintitle: “Castleman’s” (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response" OR “meta-analysis”)

allintitle: “Castleman” (endpoint OR endpoints OR surrogate OR surrogacy OR "overall survival" OR "progression-free" OR "disease-free" OR "time to progression" OR "response" OR “meta-analysis”)

Search terms for subsequent publication

Google scholar was searched using 'drug name, 'cancer type', and 'survival'. Search was limited to articles published after January 1, 2009.

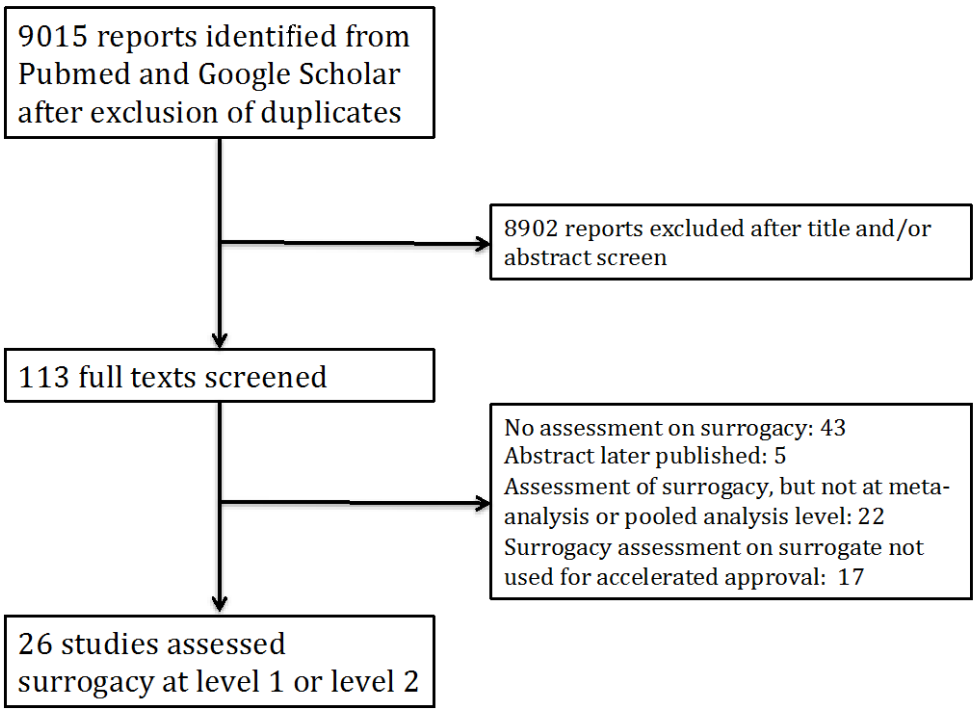
Search terms for accelerated approvals

1. Belinostat peripheral lymphoma
2. Bevacizumab glioblastoma
3. Blinatumomab acute lymphocytic leukemia
4. Brentuximab Hodgkin lymphoma; Brentuximab anaplastic lymphoma
5. Carfilzomib multiple myeloma
6. Ceritinib lung cancer survival
7. Crizotinib lung cancer survival
8. Dasatinib chronic myeloid leukemia survival
9. Everolimus giant cell astrocytoma survival
10. Everolimus angiomyolipoma
11. Ibrutinib chronic lymphocytic leukemia survival
12. Ibrutinib mantle cell lymphoma survival
13. Idelalisib follicular lymphoma survival; Idelalisib small follicular lymphoma survival
14. Lapatinib letrozole breast cancer survival
15. Liposomal vincristine acute lymphocytic leukemia
16. Nilotinib chronic myeloid leukemia survival
17. Nivolumab melanoma survival
18. Ofatumumab chronic lymphocytic leukemia survival
19. Omecetaxine chronic myeloid leukemia survival
20. Pembrolizumab melanoma survival
21. Pertuzumab breast cancer survival
22. Pomalidomide multiple myeloma survival
23. Ponatinib chronic myeloid leukemia survival
24. Pralatrexate peripheral lymphoma
25. Trametinib and dabrafenib melanoma survival

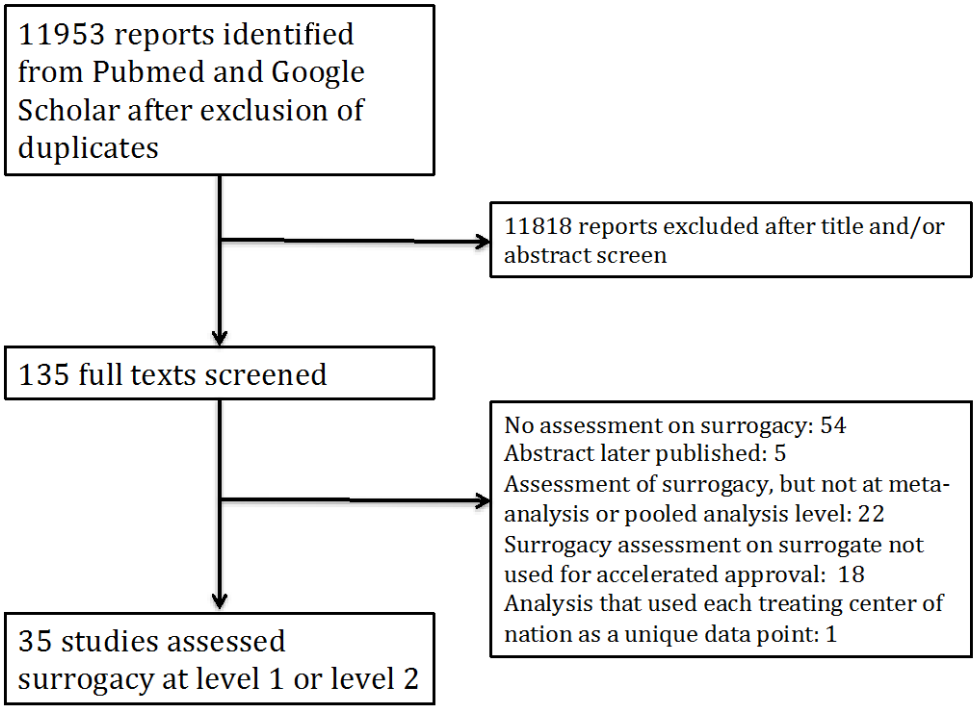
Search terms for regular approvals

1. Bosutinib chronic myeloid leukemia survival
2. Nab-paclitaxel lung cancer survival
3. Olaparib ovarian cancer survival
4. Romidepsin cutaneous lymphoma
5. Vismodegib basal cell carcinoma survival
6. Denosumab giant cell tumor
7. Lenalidomide mantle cell lymphoma survival
8. Afatinib lung cancer survival
9. Axitinib renal cell carcinoma survival
10. Bevacizumab ovarian cancer survival; Bevacizumab fallopian; Bevacizumab peritoneal
11. Bevacizumab renal cell carcinoma survival
12. Cabozantinib thyroid cancer survival
13. Crizotinib lung cancer survival
14. Dabrafenib melanoma survival
15. Erlotinib lung cancer survival
16. Everolimus neuroendocrine tumor survival
17. Everolimus renal cell carcinoma survival
18. Everolimus breast cancer survival
19. Lanreotide neuroendocrine tumor survival
20. Obinutuzumab chronic lymphocytic leukemia survival
21. Ofatumumab chronic lymphocytic leukemia survival
22. Pazopanib sarcoma survival
23. Pazopanib renal cell carcinoma survival
24. Interferon renal cell carcinoma survival
25. Pertuzumab breast cancer survival
26. Rituximab follicular lymphoma survival
27. Rituximab chronic myeloid leukemia survival
28. Sorafenib thyroid cancer survival
29. Sunitinib neuroendocrine tumor survival
30. Vandetanib thyroid cancer survival

Consort diagrams



Accelerated approval



Accelerated and Regular approval

Flow Diagram of Overall Study Strategy