New drug information

- **Elzonris™ (tagraxofusp-erzs):** The Food and Drug Administration (FDA) approved Stemline Therapeutics’ Elzonris for the treatment of blastic plasmacytoid dendritic cell neoplasm (BPDCN) in adults and in pediatric patients, 2 years of age and older. BPDCN is a rare, rapidly progressing cancer of the bone marrow and blood that can affect multiple organs, including the skin, lymph nodes, spleen and liver. Prior to the approval of Elzonris, the standard treatment for BPDCN has been intensive chemotherapy followed by bone marrow transplantation. Elzonris contains a Boxed Warning to alert health care professionals and patients about the increased risk of capillary leak syndrome which may be life-threatening or fatal to patients in treatment. Elzonris uses weight-based dosing and is administered intravenously over 15 minutes once daily on days 1 to 5 of a 21-day cycle. Stemline anticipates launching Elzonris in early 2019.

- **Ultomiris™ (ravulizumab):** The FDA approved Alexion Pharmaceuticals’ Ultomiris for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH). PNH is a rare, life-threatening disease in which the bone marrow does not produce enough blood cells and red blood cells break apart prematurely. Two weeks after the first dose, Ultomiris is administered intravenously every 8 weeks. Alexion’s Soliris® is also indicated for the treatment of PNH but is administered every two weeks. Soliris is also FDA approved for the treatment of atypical hemolytic uremic syndrome (aHUS) and generalized myasthenia gravis (gMG). Soliris loses exclusivity in March 2021 at which time a generic may become available on the market. Alexion launched Ultomiris at a 10% discount to Soliris in an effort to move patients from Soliris to Ultomiris. Alexion is studying Ultomiris in aHUS and is developing Ultomiris for gMG; as well as developing a subcutaneous formulation of Ultomiris. Analysts anticipate approval of additional indications for Ultomiris may further reduce the cost of Ultomiris.
New drug information (continued)

- **Inbrija™ (levodopa inhalation powder):** The FDA approved Acorda Therapeutics’ oral inhalation medication Inbrija for the intermittent treatment of off episodes in patients with Parkinson’s disease treated with carbidopa/levodopa. Each Inbrija capsule contains 42 mg of levodopa inhalation powder that must not be swallowed. The content of two capsules are inhaled via inhaler as needed for off symptoms up to 5 times a day. US WorldMeds’ Apokyn® is also FDA approved as a Parkinson’s off-episode rescue medication. Apokyn is a subcutaneous injectable for the treatment of acute intermittent treatment of hypomobility off episodes associated with advanced Parkinson’s disease. Inbrija is expected to be available by prescription in the United States in the first quarter of 2019. The price has not been disclosed.⁴

- **Asparlas™ (calaspargase pegol-mkl):** The FDA approved Shire’s Asparlas as a component of a multi-agent chemotherapeutic regimen for acute lymphoblastic leukemia (ALL) in pediatric and young adult patients age 1 month to 21 years. Shire developed Asparlas based on Sigma’s Oncaspar® (pegaspargase), which is approved as a first-line treatment for patients with ALL and for ALL patients with hypersensitivity to asparaginase. Oncaspar is administered intramuscularly or intravenously no more frequently than every 14 days. Compared with Oncaspar, Asparlas can only be dosed intravenously but allows for a longer interval of 21 days between doses.⁵

- **Cutaquig™ (immune globulin subcutaneous [human]-hipp) 16.5%:** The FDA approved Octapharma's subcutaneous immune globulin (SCIG) Cutaquig for the treatment of primary humoral immunodeficiency (PIDD) in adults. It is estimated that 1 in 1,200 people in the United States are diagnosed with PIDD, or approximately 150,000 to 360,000 people.⁶ Cutaquig is manufactured by a process nearly identical to Octapharma’s intravenous Octagam® 5% and 10%.⁷ Multiple SCIG products are available in 10% and 20% concentrations. Cutaquig launch plans and price have not been released.

- **Bafiertam™ (monomethyl fumarate delayed release):** The FDA gave tentative approval to Banner Life Sciences’ oral Bafiertam for the treatment of adults with relapsing forms of multiple sclerosis (MS). Relapsing remitting MS affects approximately 85 percent of patients diagnosed with MS, or an estimated 2 million people worldwide.⁸ Bafiertam is a bioequivalent drug to Biogen’s Tecfidera® (dimethyl fumarate).⁸ The full FDA approval is expected following a patent expiration on June 20, 2020 just months before Tecfidera generics are expected in September 2020.⁸

- **Ontruzant® (trastuzumab-dttb):** Samsung Bioepsis received FDA approval for Ontruzant, its biosimilar to Genentech’s Herceptin®, for the treatment of HER2-overexpressing breast cancer or metastatic gastric or gastroesophageal junction adenocarcinoma. Ontruzant will be commercialized by Merck. This is the third Herceptin biosimilar approved by the FDA including Mylan/Biocon’s Ogivri® and Celltrion/Teva’s Herzuma®. None of the launch plans have been released for these biosimilar products.
New indications

- **Lynparza® (olaparib):** The FDA granted approval to AstraZeneca and Merck’s Lynparza for the first-line maintenance treatment of adult patients with deleterious or suspected deleterious germline or somatic BRCA-mutated (gBRCAm or sBRCAm) advanced epithelial ovarian, fallopian tube or primary peritoneal cancer who are in complete or partial response to first-line platinum-based chemotherapy. Lynparza is also approved for the maintenance treatment of recurrent ovarian cancer, advanced gBRCA-mutated ovarian cancer after three or more lines of chemotherapy and for germline BRCA-mutated HER2-negative metastatic breast cancer.

- **Keytruda® (pembrolizumab):** The FDA granted accelerated approval to Merck’s Keytruda to treat adult and pediatric patients with recurrent locally advanced or metastatic Merkel cell carcinoma. The FDA also approved Keytruda for the treatment of patients with advanced hepatocellular carcinoma who have been previously treated with sorafenib.

- **Coagadex® (Coagulation Factor X, Human):** The FDA expanded Bio Product Laboratory’s human-derived coagulation factor Coagadex to include prophylactic treatment for patients with hereditary factor X deficiency who are younger than 12 years of age. Coagadex is the only treatment specifically for hereditary factor X deficiency.

- **Egrifta® (tesamorelin F4 formulation):** Theratechnologies received approval for its single-vial formulation of Egrifta for the same indication as the original Egrita two-vial formulation for the reduction of excess abdominal fat in HIV-infected patients with lipodystrophy. This new formulation is four times more concentrated than the original Egrifta formulation, reducing the volume of administration to 0.35 mL instead of 2.0 mL, and is stable at room temperature.

- **Envarsus XR® (tacrolimus extended release):** The FDA extended the indication of Veloxis’s Envarsus XR for the prophylaxis of organ rejection in kidney transplant patients in combination with other immunosuppressants. Envarsus XR was initially approved for organ rejection prophylaxis in kidney transplant patients converted from tacrolimus immediate-release formulations, in combination with other immunosuppressants.

- **Sprycel® (dasatinib):** The FDA expanded Bristol-Myers Squibb’s Sprycel in combination with chemotherapy for the first-line treatment of pediatric patients with newly diagnosed Philadelphia chromosome-positive (Ph+) acute lymphoblastic leukemia (ALL). Sprycel was previously approved for:
  - Pediatric patients with Ph + chronic myeloid leukemia (CML) in chronic phase
  - Adults with newly diagnosed Ph + CML in chronic phase
  - Adults with Ph + CML that is chronic, accelerated, or myeloid or lymphoid blast phase with resistance or intolerance to prior therapy including imatinib
  - Adults with Ph + ALL with resistance or intolerance to prior therapy

- **Cabometyx® (cabozantinib):** The FDA approved a new indication for Exelixis’ Cabometyx for the treatment of patients with hepatocellular carcinoma (HCC) who have been previously treated with sorafenib. Cabometyx was initially approved for the patients with advanced renal cell carcinoma (RCC).
January news

- “U.S. regulators approved AstraZeneca and Merck’s Lynparza, the fast-growing poly ADP ribose polymerase (PARP) protein inhibitor in certain ovarian cancer patients who’ve responded partially or completely to an initial round of chemo. Regulators based the decision on data showing the drug could cut the risk of disease progression or death by 70 percent in the population, helping 60 percent of women in the trial log three years without a relapse. The approval is the first for the PARP class of drugs.”

- “A pair of new medicines designed to treat spinal muscular atrophy, a rare and often fatal genetic disease affecting muscle strength and movement, may be beneficial but appear to be priced too high to be considered cost effective, according to the Institute for Clinical and Economic Review (ICER). Spinraza®, from Biogen, costs $750,000 during the first year of treatment and $375,000 thereafter. A forthcoming gene therapy from Novartis, which is awaiting FDA approval, is expected to cost between $4 million to $5 million. Both drugs do not meet ICER’s quality-of-life years (QALY) criteria.”

- “New phase 3 data showed positive results for caplacizumab (Cablivi™) in the treatment of acquired thrombotic thrombocytopenic purpura (aTTP) in adults, according to a study published in the New England Journal of Medicine. Currently, the standard treatment for aTTP includes a daily plasma exchange and immunosuppression. Even with treatment, patients are still at risk of developing acute blood clotting conditions and recurrence of disease. Caplacizumab, an anti-von Willebrand Factor (vWF) nanobody, inhibits the interaction between ultra large vWF multimers and platelets, which stops the formation and accumulation of micro-clots that cause thrombocytopenia, tissue ischemia, and organ dysfunction in aTTP.”

- “An advisory panel to the FDA voted 16 to 1 in favor of approval for Evenity™, Amgen’s osteoporosis treatment for postmenopausal women at high risk for fracture, Reuters reported. According to the article, Evenity helps reduce the risk of fracture by increasing bone formation and inhibiting breakdown of bone minerals. However, the panel did raise concerns regarding cardiovascular safety risks linked to the drug, but ultimately voted that the drug’s benefits outweighed its risks, the article reported.”

- “Officials with the FDA have accepted Genentech’s supplemental Biologics License Application (sBLA) for atezolizumab (Tecentriq®) in combination with chemotherapy as first-line treatment for certain patients with non-small cell lung cancer (NSCLC), according to a press release. If approved, atezolizumab will be available in combination with nab-paclitaxel (Abraxane®) and carboplatin for patients with metastatic non-squamous disease who do not have EGFR or ALK genomic tumor aberrations. According to Genentech, the FDA is expected to make a decision on the application by September 2, 2019.”

References