New drug information

- **Azedra™ (lobenguane I 131):** The Food and Drug Administration (FDA) approved Progenics Pharmaceuticals’ Azedra for the treatment of adults and adolescents age 12 and older with rare tumors of the adrenal gland (pheochromocytoma or paraganglioma) that cannot be surgically removed (unresectable), have spread beyond the original tumor site and require systemic anticancer therapy. Azedra is a radioactive therapeutic agent that is intravenously administered as one dosimetric dose followed by 2 therapeutic doses administered 90 days apart.

- **Mulpleta™ (lusutrombopag):** Shionogi received FDA approval of Mulpleta for the treatment of thrombocytopenia in adult patients scheduled to undergo a procedure. Two months ago, Dova Pharmaceuticals’ received approval of Doptelet® for a similar indication; analysts have found that the drug profiles are also similar. Shionogi has not released its price or a launch timeline.

- **Poteligeo™ (mogamulizumab-kpkc):** The FDA approved Poteligeo for the treatment of adult patients with relapsed or refractory mycosis fungoides (MF) or Sézary syndrome (SS), the most common types of cutaneous T-cell lymphoma, after at least one prior systemic therapy. Poteligeo provides a new treatment option for MF and is the first drug FDA approved for SS.

- **Onpattro™ (patisiran):** The FDA approved Onpattro for the treatment of peripheral nerve disease (polyneuropathy) caused by hereditary transthyretin-mediated amyloidosis (hATTR) in adult patients. Onpattro is administered every 3 weeks via intravenous infusion given over 80 minutes and uses weight-based dosing. Alnylam launched Onpattro at a reported average annual wholesale acquisition cost (WAC) of $450,000.1

- **Galafold™ (migalastat):** Amicus Therapeutics received FDA accelerated approval of Galafold for the first oral treatment of adults with a confirmed diagnosis of Fabry disease and an amenable galactosidase alpha gene (GLA) variant based on in vitro assay data. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials. Galafold has launched with an annual WAC of ~$315,000. Comparatively, Sanofi’s intravenously administered Fabrazyme, the current standard of care for Fabry disease, has an annual WAC of $343,700 annually.2
New drug information continued

- **Diamomit™ (stiripentol):** The FDA approved Biocodex's Diacomit for the treatment of seizures associated with Dravet syndrome in patients 2 years of age and older taking clobazam. There is no clinical data to support the use of Diacomit as monotherapy in Dravet syndrome. Diacomit uses weight-based dosing given in 2 – 3 divided doses and will be available as capsules or powder for suspension.

New indications

- **Kalydeco® (ivacaftor):** The FDA expanded Vertex Pharmaceuticals’ Kalydeco indication to include pediatric patients age 12 months to younger than 2 years old with cystic fibrosis who have at least one mutation in the cystic fibrosis transmembrane conductance regulator gene that is responsive to the drug based on clinical and/or in vitro test data.

- **Lenvima® (lenvatinib):** The FDA approved Eisai’s Lenvima for the first-line treatment of hepatocellular carcinoma patients with unresectable disease. Lenvima is also approved in certain patients with progressive, differentiated thyroid cancer and advanced renal cell carcinoma.

- **Opdivo® (nivolumab):** The FDA granted accelerated approval to BMS’ Opdivo as the first immuno-oncology treatment option for patients with metastatic small cell lung cancer (SCLC) patients whose disease progressed after two or more prior lines of therapy including a platinum-based chemotherapy. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

- **Orkambi® (lumacaftor/ivacaftor) oral granules:** The FDA expanded the pediatric indication for the treatment of cystic fibrosis in children ages 2 – 5 who have 2 copies of the F508del-CFTR mutation and approved the new dosage form of Orkambi as oral granules.

- **Eylea® (aflibercept) intravitreal injection:** The FDA approved Regeneron Pharmaceuticals’ Eylea in patients with wet age-related macular degeneration (AMD).
August news

- “The FDA’s Antimicrobial Drugs Advisory Committee has voted 12 to 2 in favor of the safety and effectiveness of Insmed Incorporated’s amikacin liposome inhalation suspension (ALIS) for adults with nontuberculosis mycobacterial (NTM) lung disease caused by Mycobacterium avium complex (MAC) who have limited or no treatment options.”

- “Eli Lilly announced it has submitted applications to US and European regulatory agencies for nasal glucagon, which, if approved, could shave minutes and stress from the current method for delivering this hormone to people with diabetes experiencing severe hypoglycemia.”

- “Risankizumab showed superior efficacy to both placebo and ustekinumab in the treatment of moderate-to-severe plaque psoriasis. Treatment-emergent adverse event profiles were similar across treatment groups and there were no unexpected safety findings.”

- “A rolling submission of an FDA new drug application (NDA) has been completed for selinexor for the treatment of patients with penta-refractory multiple myeloma, according to Karyopharm Therapeutics, the manufacturer of the XPO1 inhibitor.”

- “The FDA issued a complete response letter to Pain Therapeutics in response to its marketing application for its abuse-deterrent extended-release oxycodone formulation Remoxy ER. The agency said the company submitted insufficient data demonstrating that the benefits of the drug outweighed the risk.”

- “The FDA Administration today approved the first generic version of EpiPen and EpiPen Jr (epinephrine) auto-injector for the emergency treatment of allergic reactions, including those that are life-threatening (anaphylaxis), in adults and pediatric patients who weigh more than 33 pounds. Teva Pharmaceuticals USA gained approval to market its generic epinephrine auto-injector in 0.3 mg and 0.15 mg strengths.”

References


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