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Clinical Update

FDA Approves First Oral Drug for Spinal Muscular Atrophy (SMA)

On August 7, 2020, the U.S. Food and Drug Administration (FDA) approved Evrysdi™ (risdiplam) to treat spinal muscular atrophy (SMA) in patients two months and older. SMA is a rare and often fatal genetic disorder, typically affecting infants, that is characterized by muscle weakness and degradation. SMA is caused by a lack of spinal motor neuron (SMN) protein, which is a key protein for muscle development. Evrysdi™ is the first oral drug approved for the treatment of SMA and functions by helping the body to make and maintain more SMN protein. Evrysdi™ has been shown to improve motor function as evidenced by results found in two main clinical studies. Study results in infants with Type 1 SMA showed that after taking Evrysdi™ for 12 months, 41% of infants were able to sit independently for at least 5 seconds and 90% of infants could breathe without permanent ventilation. These results indicate advancements in treatment, as untreated patients with infantile-onset SMA normally do not have the ability to sit independently and less than a quarter of this patient population is expected to survive without permanent ventilation beyond 14 months of age. Evrysdi™ was awarded a Rare Pediatric Disease Priority Review Voucher and was granted Fast Track, Orphan Drug, and Priority Review designations.

Safety First

Ferring US Issues Recall on Desmopressin Nasal Sprays

On August 5, 2020, Ferring US informed consumers of a voluntary nationwide recall of all lots of DDAVP® Nasal Spray 10 mcg/0.1mL, Stimate® Nasal Spray 1.5mg/mL, and Desmopressin Acetate Nasal Spray 10 mcg/0.1mL. DDAVP® and Desmopressin Acetate Nasal Spray are commonly used as antidiuretic replacement therapy to treat excessive thirst and or urination associated with conditions such as diabetes insipidus and head trauma. Stimate® Nasal Spray is indicated to treat hemophilia A and Von Willebrand disease, two types of bleeding disorders. The recall was initiated due to routine testing results that indicated these products contained higher amounts of desmopressin than specified, which is commonly known as superpotency. Unexpected increased amounts of desmopressin can cause low sodium levels in the blood, which can result in seizures, coma, or even death. During the time frame that the affected product was distributed, there was one non-fatal report of an adverse event potentially associated with this recall in the U.S.

From the Industry

FDA Denies Application for Peanut Allergy Patch

On August 4, 2020, DBV Technologies received a Complete Response Letter (CRL) from the FDA regarding their investigational allergy medication, Viaskin™ Peanut. A CRL may be issued when the FDA has completed its review of a new drug application and has made the decision to refrain from approving it in its present form. Viaskin™ Peanut is being studied as a non-invasive patch intended to be placed on the surface of the skin to treat peanut allergies in children 4 to 11 years old. The CRL issued to DBV Technologies details the FDA's decision not to approve the application because of concerns surrounding patch adhesion and the impact this may have on the efficacy of the product. In addition, the FDA is requiring supplementary clinical, chemistry, and manufacturing and controls data to be submitted but did not identify any specific safety issues related to the product. DBV Technologies intends to meet with the FDA to further discuss these requirements and other data that may be needed to resubmit for FDA approval.

FDA Approvals

Recent FDA Approvals

Novel Drug Approval: Lampit™ (nifurtimox)
Oral tablet for the treatment of pediatric patients weighing at least 5 pounds, 8 ounces diagnosed with a parasitic disease known as Chagas Disease [8/6/2020 – Orphan Drug; Priority Review – BAYER HEALTHCARE PHARMS]

Novel Drug Approval: Olinvyk™ (oliceridine)
Intravenous injection for the treatment of severe pain in adult patients that require opioid analgesic therapy and for whom other treatments are insufficient [8/7/2020 – TREVENA INC]

Novel Drug Approval: Evrysdi™ (risdiplam)
Oral solution for the treatment of patients 2 months and older diagnosed with spinal muscular atrophy (SMA) [8/7/2020 – Orphan Drug; Priority Review – GENENTECH INC]

New Generics

New Generics Entering the Marketplace

Demser® (metyrosine)
Indication: Pheochromocytoma (hormone-secreting tumor)
Dosage Form/Strength: 250 MG Capsule
Average Wholesale Price (AWP): Generic = $43,756 | Brand = $44,217

Chief Author: Kevin Lavery, PharmD, MBA
Contributing Author: James Pufpaff, PharmD/MBA Candidate
Editor: Dana Ranallo, PharmD