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Clinical Update

FDA Expands Approval of Drug for Treatment and Post-Exposure Prevention of Influenza

On November 23, 2020, the U.S. Food and Drug Administration (FDA) expanded the approved indication for Xofluza® (baloxavir marboxil) to include post-exposure prevention of influenza (flu) for patients 12 years and older. Xofluza® received initial approval in 2018 to treat uncomplicated flu in patients 12 years and older, who have been symptomatic for 48 hours or less. In addition to the label expansion, Xofluza®, which was previously only available in tablet form, is now also available as granules for mixing in water. The expansion for post-exposure prevention was based off safety and efficacy data from a clinical trial involving 607 people 12 years and older who were exposed to influenza by a member of their household. Participants were randomized to receive either a single dose of Xofluza® or placebo. The primary endpoint of the study identified the number of patients who were infected with the influenza virus and presented with fever and at least one respiratory symptom from day 1 to day 10. Only 1% of individuals who received Xofluza® fell into this category compared to 13% who received placebo. The most common side effects seen were diarrhea, bronchitis, nausea, sinusitis, and headache. An annual flu vaccine is still best practice for protecting against the flu, however, the expanded use of Xofluza® may serve as an additional preventative measure that can be taken by individuals who have been exposed to the influenza virus.

Safety First

Emergency Use Authorizations Play a Key Role in Addressing Public Health Crises

With the novel coronavirus presenting unprecedented challenges to public health, the FDA has been on a mission to provide access to safe and efficacious treatments in a timely manner. Several tests and treatments for COVID-19 have been brought forward under what is known as an emergency use authorization (EUA). When a public health emergency strikes, there is often an urgent need for a product to diagnose, treat, or prevent serious or life-threatening diseases or conditions. Under section 564 of the Federal Food, Drug, and Cosmetic Act (FD&C Act), the FDA may authorize emergency use of unapproved medical products in situations where there are no adequate or approved alternatives available. EUAs have been granted in the past for other declared emergencies such as anthrax, Ebola, influenza, and MERS. The key feature of an EUA is that it is not part of the standard drug development and approval pathway. When considering EUA for a drug, the FDA evaluates the benefits and risks of a treatment and decides whether an EUA is justified, provided the known benefits outweigh the known risks and there is reasonable evidence to believe the drug will be effective for the intended use. In order to ensure proper use in the clinical setting, the FDA may issue conditions of authorization, which are terms put in place to enforce the proper administration, recordkeeping and adverse event reporting needed to protect the safety of the public. The use of EUA's in the current COVID-19 pandemic, as well as many national emergencies that have come before, have shown to enhance provider's abilities to respond to medical challenges at hand and provide patient's with life-saving care.

From the Industry

FDA Approves Oxlumo™ for Rare Metabolic Disorder

On November 23, 2020, the FDA approved Oxlumo™ (lumasiran) as the first ever treatment for primary hyperoxaluria type 1 (PH1). PH1 is a rare genetic disorder that leaves affected individuals with a deficiency in an enzyme responsible for preventing accumulation of a substance called oxalate. The built-up oxalate can bind with calcium in the body and form stones throughout the urinary tract and kidneys. When untreated, chronic stone formation and accumulation of calcium oxalate in the kidneys has the potential to cause chronic kidney disease (CKD) and ultimately lead to end stage renal disease (ESRD). As kidney function continues to decline, there is a risk of oxalate reaching other organ systems in the body and causing even more severe complications. In a clinical trial involving patients 6 years and older with PH1, 26 patients were randomized to receive either Oxlumo™ monthly injection or placebo followed by a maintenance dose of Oxlumo™ or placebo every three months. When compared to placebo, it was found that the group who had received Oxlumo™ had a significantly higher reduction of oxalate levels in the urine (65% vs. 12%). At the 6-month mark, 52% of patients who had been treated with Oxlumo™ had achieved a normal 24-hour urinary oxalate level (no patients who received placebo achieved this). A second study, involving PH1 patients younger than 6 years old, showed an average decrease in urinary oxalate of 71% at the six-month mark for those receiving Oxlumo™. The most common side effects were injection site reaction and abdominal pain. Oxlumo™ received Orphan Drug designation as well as a Breakthrough Therapy by the FDA. Alynlam Pharmaceuticals was also given a Rare Pediatric Disease Priority Review Voucher, a two-part voucher designed to encourage drug development in the rare disease category, which was used by the company to expedite the review for of Oxlumo™ and may also be used to prioritize review for a future drug product.

FDA Approvals

Recent FDA Approvals

New Drug Approval: Zokinvy™ (lonafarnib)
Oral capsule approved for the treatment of a rare aging disease known as Hutchinson-Gilford Progeria Syndrome, and its associated genetic conditions, in patients 12 months and older with a body surface area of 0.39m2 or more [11/20/20 – Priority Review; Orphan Drug – EIGER BIOPHARMACEUTICALS INC]

New Drug Approval: Oxlumo™ (lumasiran)
Subcutaneous injection approved for the treatment of adult patients with a rare genetic disorder known as primary hyperoxaluria type 1, a condition which may lead to irreversible damage of the kidneys [11/23/20 – Priority Review; Orphan Drug – ALNYLAM PHARMS INC]

New Generics

New Generics Entering the Marketplace

*As of this issue, there were no new generics entering the marketplace. However, there are several projected launches we are monitoring, including:

Saphris® (asenapine maleate)
Indication: Bipolar Mania/Schizophrenia
Dosage Form/Strength: 2.5, 5, and 10 MG Sublingual Tablets
Projected Launch: 12/10/2020

Absorica® (isotretinoin)
Indication: Severe Acne
Dosage Form/Strength: 10, 20, 25, 30, 35, 40 MG Capsules
Projected Launch: 12/27/2020

Chantix® (varenicline)
Indication: Smoking Cessation
Dosage Form/Strength: 0.5 and 1 MG Tablets
Projected Launch: 4Q 2020

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