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جامعة مصر الدولية

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DRUG INFORMATION CENTER

Drug Information Newsletter

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INSIDE THIS ISSUE:

FDA Approves a New treatment for Patients with Myelofibrosis

Drug Name: Inrebic® (fedratinib)

Pharmacological Class: Kinase inhibitor

Company: Celgene

Approval Status: Approved August 2019

Treatments: Adults with Myelofibrosis

What is Myelofibrosis?

Myelofibrosis can be considered a chronic type of Leukemia. It is a rare type of bone marrow disorder where scar tissue is formed in the bone marrow. Thus, unhealthy production of the blood cells moves from the bone marrow to the liver and spleen causing organ enlargement and total organ damage.

Background Information about Inrebic® (fedratinib):

Inrebic® (fedratinib) is considered a kinase inhibitor. Kinase inhibitor has activity against (JAK2) which is abnormally activated with myeloproliferative cancer. Inrebic® is the second drug approved for Myelofibrosis after the release of Jakafi®(ruxolitinib) in 2011.[1] The JAKARTA clinical study showed more than or equal to a 50% reduction in Myelofibrosis-related symptoms. Inrebic is indicated for the treatment of adult patients with intermediate-2 or high-risk primary or secondary (Post-Polycythemia Vera or post-essential Thrombocythemia) Myelofibrosis. The recommended dose of Inrebic® is 400 mg capsule orally given once daily. The patients receiving Inrebic must have at least baseline platelet count of greater than or equal to 50 x 109/L. This drug can be given with a high fat meal to reduce the incidence of nausea and vomiting.[2]

References:

1. *"About INREBIC® (Fedratinib) and How It Works: Patient." Inrebic.com, https://www.inrebic.com/about-inrebic.*
2. *INREBIC (Fedratinib) Dosing, Indications, Interactions, Adverse Effects, and More." Inrebic (Fedratinib) Dosing, Indications, Interactions, Adverse Effects, and More, 30 Nov. 2021, https://reference.medscape.com/drug/inrebic-fedratinib-1000346.*

FDA Approves First Treatment for Patients with Beta Thalassemia

What is Beta Thalassemia?

As Beta Thalassemia is a rare blood disorder, the FDA has approved Reblozyl® (luspatercept–aamt). Beta Thalassemia is an inherited blood disorder that causes anemia by reducing the hemoglobin level in the blood. The hemoglobin is responsible for carrying oxygen to the cells in all the body. Low level of hemoglobin leads to a decrease of oxygen that results in anemia. The anemia symptoms are generalized fatigue, weakness and pale skin. So patients with Thalassemia need lifelong regimens of blood transfusion, and treatment of iron overload that results due to the continuous blood transfusion. Iron overload can cause serious organ damages. Thus, researchers try to find new treatment approaches for this disease.[1]

Background Information about Reblozyl® (Luspatercept):

Reblozyl® (Luspatercept) is an erythroid (RBCS) maturation agent. It is the first drug indicated for adult patients having anemia due to beta thalassemia and need continuous RBCS transfusion. The recommended starting dose is 1 mg/kg once every 3 weeks given in the form of subcutaneous injection. According to BELIEVE trial (NCT02604433), the efficacy was tested. The primary efficacy outcome measure was the proportion of patients achieving RBC transfusion burden reduction. The most obvious side effects for patients receiving Reblozyl® were headache, arthralgia, fatigue, dizziness, and abdominal pain. It is not indicated neither for pregnant nor breastfeeding women.[2]

References:

1. *"Sickle Cell Disease Clinical Trials." Centerwatch Iconnect, https://www.centerwatch.com/clinical-trials/listings/condition/627/sickle-cell- disease/?phase=2&page=4.*
2. *Annex I Summary of Product Characteristics - Europa. https://www.ema.europa.eu/en/documents/product-information/reblozyl-epar-product- information_en.pdf.*

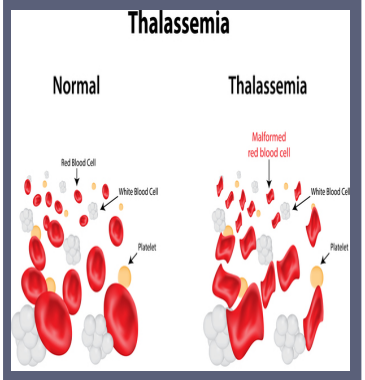
Drug Name: Reblozyl® (luspatercept- aamt)

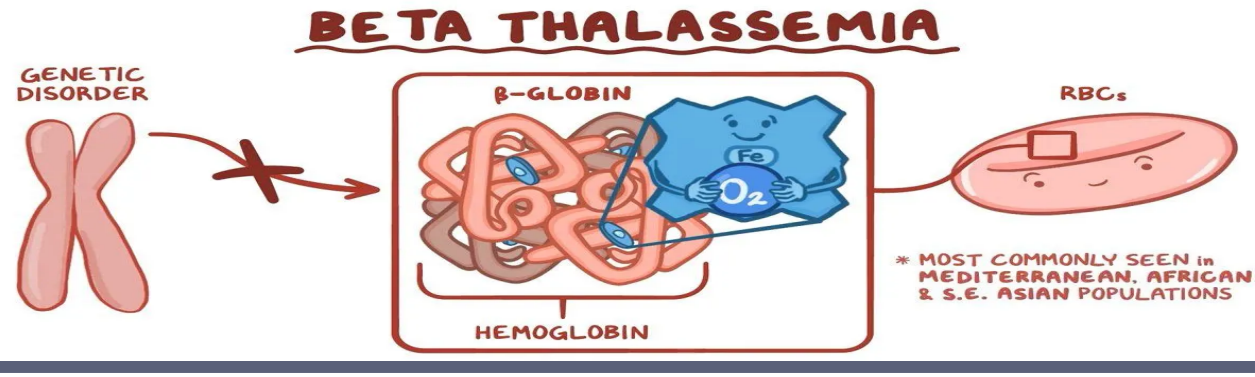
Pharmacological Class: Hematopoietic agent

Company: Celgene and Acceleron Pharma

Approval Status: Approved Novamber 2019

Treatments: Anemia in adults with beta thalassemia who need continuous RBCS transfusion





FDA Approves First Treatment for Acute Hepatic Porphyria

What is Hepatic Porphyria?

Hepatic Porphyria is a rare hepatic disorder. During the production of heme in this disorder, the toxic porphyrin molecules build up in the liver. This build up causes acute porphyria attacks, which can lead to severe pain, respiratory failure, seizures, and mental status changes that can lead to serious complications like paralysis. The porphyria attacks occur suddenly and can lead to permanent neurological damage and death. The diagnosis of this disease is based on detecting specific Heme precursors in urine or feces.[1]

References:

1. *Ernst, D. (2019, November 20). Givlaari Approved for Acute Hepatic Porphyria. MPR. Retrieved February 8, 2022, from https://www.empr.com/home/news/givlaari-approved-for-acute-hepatic-porphyria/*
2. *Givlaari (givosiran) Dosing, Indications, Interactions, Adverse Effects, and More. (2021, November 1). Retrieved February 8, 2022, from https://reference.medscape.com/drug/givlaari-givosiran-4000009*

Background Information about Givlaari® (Givosiran)

Before the approval of Givlaari® (Givosiran), the treatment options for Porphyria have only provided partial relief from the pain that characterizes these attacks. Symptomatic treatment and liver transplantation were the only known therapy. The new approved drugs can treat this disease by reducing the number of attacks instead of only reliving the pain. The mechanism of action of this drug is interfering with RNA to reduce the levels of ALAS1 and mRNA. This leads to reduced circulating levels of neurotoxic intermediates Aminolevulinic acid (ALA) and Porphobilinogen. Clinical trials done on patients with acute hepatic porphyria the patients received the new drug Givlaari® and other patients received placebos treatment. Those who received Givlaari experienced 70% lower number of porphyria attacks compared to those receiving a placebo. The dose recommended for patients is 2.5 mg/kg SC every month. The dose is based on actual body weight. The most common side effects the patients experienced with Givlaari® were nausea and injection site reactions. Healthcare providers have to measure the kidney and liver functions before giving this new drug and periodically during the treatment course.[2]

Black Box Warning for Fedratinib

An important black box warning for fedratinib is stated in the label of the drug **"Serious and Fatal Encephalopathy"**, including Wernicke's (a condition caused by decreased level of thiamine vitamin B1). Thus it is a must to measure the thiamine levels in all patients before initiating Inrebic® and periodically during the treatmet. Also if encephalopathy is suspected the patient will start thiamine parenterally till the symptoms resolve and the patient thiamine level is normalized[1]

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