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Exjade[®], a breakthrough once-daily oral iron chelator, now available in the UK

The only once-daily, oral, iron chelator to demonstrate continuous 24-hour chelation of excess iron, offers a new alternative to burdensome infusions¹

Frimley, UK, Thursday 21 September 2006 – Novartis today announced the UK approval of Exjade[®] (deferasirox), the first and only once-daily oral iron chelator for the treatment of transfusional haemosiderosis (iron overload) in patients who suffer from diseases such as thalassaemia, sickle cell disease, myelodysplastic syndromes and other rare anaemias.

Until the approval of Exjade, desferrioxamine (Desferal[®]) was the gold standard treatment for transfusion-related iron overload. While effective, desferrioxamine requires nightly infusions by needle and pump, often lasting eight to 12 hours per night for five to seven nights a week. As a result, many patients stop or avoid iron chelation therapy, thus risking the toxic effects of iron overload.² Left untreated, iron overload can cause damage to body tissues including liver, heart and endocrine glands.³

“Exjade will offer a welcome alternative to patients who find traditional iron chelation therapy unacceptable. Its ease of administration can only improve compliance, thus helping to reduce the health complications of iron overload,” commented Dr Farrukh Shah, Consultant Haematologist, The Whittington Hospital NHS Trust. “The approval of Exjade marks the beginning of an exciting new era for iron chelation therapy.”

Iron chelation is often necessary to prevent potentially life-threatening complications of iron overload⁴ in patients receiving frequent blood transfusions to treat certain types of rare chronic blood disorders, including myelodysplastic syndromes, thalassaemia, sickle cell disease and other rare anaemias. The need for transfusion and chelation is often life-long.⁵

Administered as a drink, Exjade is the only chelator to demonstrate continuous 24-hour chelation of excess iron with a single oral daily dose.¹

Exjade is approved in the EU for the treatment of chronic iron overload due to frequent blood transfusions in patients age six and older with beta thalassaemia major.⁶

It is also indicated in the EU for the treatment of chronic iron overload when desferrioxamine is contraindicated or considered inadequate in patients with other anaemias, children aged two to five years and patients with beta thalassaemia major with iron overload due to infrequent blood transfusions.⁶

The Exjade filings were based on the results of a clinical trials programme, including a Phase III head-to-head trial vs. desferrioxamine (Desferal®), which showed that after one year of treatment with comparable doses, Exjade produced similar reductions in liver iron concentration (LIC) compared to desferrioxamine.¹

"The approval of Exjade is fantastic news for people like me who need regular blood transfusions," said Anand Ghattaura, a thalassaemia patient from Datchet, Slough. "I've always found chelation with pump and needle difficult to keep up with and I used to worry all day about doing my infusion in the evening. Now I take Exjade in the morning with a glass of juice and can forget about it until the next day."

- ENDS -

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Notes to Editors

Iron Overload

Iron overload is a cumulative, potentially life threatening and unavoidable consequence of frequent blood transfusions used to treat certain chronic blood disorders such as thalassaemia, sickle cell disease and myelodysplastic syndromes (MDS).

As excess iron is highly toxic, the human body has a sophisticated mechanism to maintain the proper concentration of iron in the blood and meet the body's requirements.⁷ The body absorbs iron when we eat food. Transport proteins - called transferrin and ferritin - then move the absorbed iron through the body before incorporating it into haemoglobin.⁷

However, while the human body has a complex mechanism to absorb iron, it has no means to excrete excess iron.⁷ The only way to decrease iron overload is to remove it physically or by a process called chelation.⁵

Chelating agents bind iron, and the chelated iron is excreted from the body, preventing the toxic build-up of excess iron associated with transfusion therapy.⁸

The standard chelating agent is typically administered as a nightly infusion by needle and pump, often lasting eight to 12 hours per night for five to seven nights per week. As a result, many patients stop or avoid iron chelation, exposing themselves to the dangers of iron overload.⁸

Filing data

The clinical trials, which included more than 1,000 adults and children, were part of the largest prospective global clinical trials programme ever implemented for an investigational iron chelator. Liver Iron Concentration (LIC) is an indicator for body iron content in patients receiving blood transfusions. It is a measure of iron accumulation in the liver. The studies demonstrated that Exjade at 20-30 mg/kg/day led to the maintenance or reduction of iron burden in transfused patients with myelodysplastic syndromes, thalassaemia, sickle cell disease and other rare anaemias. In the clinical studies, Exjade was generally well tolerated, with the most frequently reported adverse events being nausea, vomiting, diarrhoea, abdominal pain, skin rash and increases in serum creatinine. As with desferrioxamine, cases of ocular and auditory disturbances have been reported.¹

The filing in the EU included data demonstrating the ability of Exjade to reduce total body iron. In addition to proven efficacy in reducing absolute iron burden as measured by LIC and serum ferritin, data from a clinical trial sub study also demonstrate efficacy in removing iron in the heart as measured by T2*.⁹

Mild, non-progressive increases in serum creatinine, mostly within the normal range, occur in about one-third of Exjade-treated patients. These are dose-dependent, often resolve spontaneously and can sometimes be alleviated by reducing the dose. Serum creatinine should be assessed before initiating therapy and should be monitored monthly thereafter to determine if dose modification or discontinuation is necessary. Liver function should be monitored monthly, and if there is an unexplained, persistent, or progressive increase in serum transaminase levels, Exjade should be interrupted or discontinued.¹

About Novartis

Novartis AG Corporation researches, develops, manufactures and markets leading innovative prescription drugs used to treat a number of diseases and conditions, including central nervous system disorders, organ transplantation, cardiovascular diseases, dermatological diseases, respiratory disorders, cancer and arthritis. The company's mission is to improve people's lives by pioneering novel healthcare solutions.

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