

L-Glutamine approved for use in patients with sickle cell disease

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Madam, sickle cell disease (SCD) is group of blood disorders which are caused by the substitution of Glutamic Acid by Valine on the β globin gene. This change in the structure of haemoglobin causes red blood cells to distort into a crescent or sickle shape, especially when exposed to oxidative stress. The loss of membrane integrity results in the occlusion of blood vessels, causing numerous detrimental complications, but most characteristically varying degrees of anaemia. SCD affects an estimated 100,000 people in the United States and perhaps as many as 25 million people worldwide. In Pakistan it's incidence is estimated to be 1.92% in the general population.^{1,2}

On 7th July 2017 the FDA (Food and Drug Administration) in the United States approved the drug L-Glutamine (Trade name: Endari) for patients with sickle cell disease.³ The drug is a preparation of the amino acid L-Glutamine in oral powder form and is to be consumed after mixing in a beverage or soft food. The drug has been approved for use in patients above the age of 5 years and has proven to reduce acute complications caused by SCD.⁴ This is the first drug to be approved for use in SCD in almost 20 years, the last one being Hydroxurea which was approved in 1998. It is also the first drug to be approved for use in children with SCD.¹

L-Glutamine was approved for use in SCD after a 48-week randomized, double-blind, placebo-controlled, clinical trial in which 230 adults and children with SCD participated. The results demonstrated that L-Glutamine was effective in reducing the frequency of sickle cell crises by 25 percent and hospitalizations by 33 percent in the intervention group. The study also showed that L-Glutamine decreased cumulative hospital days by 41 percent and lowered the incidence of ACS (acute chest syndrome) by more than 60 percent. The most commonly reported side effects were nausea, headache, abdominal pain, constipation, cough, pain in extremity, back pain, and chest pain. The drug might be available to patients as early as September 2017.^{1,4,5}

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The new drugs mechanism of action remains ambiguous but it has been postulated that it may improve the reductive capabilities of NAD (Nicotinamide adenine dinucleotide). By doing so it may help reduce oxidative damage to red blood cells, thereby preventing the damage caused by red blood cell sickling.⁴

As mentioned earlier, Patients with the disease suffer from a wide variety of complications such as ACS, dactylitis and recurrent infections which can seriously hamper daily life and dramatically reduce life expectancy.^{1,6} Given that SCD causes widespread mortality and morbidity research into therapies such as L-Glutamine, should be further encouraged.

Disclaimer: This manuscript has not been previously published and is not under consideration by any other journal.

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