

Oral Solution of Known Compound for Treatment of Diseases, Including Sick Cell Disease

Published date: March 12, 2012

Technology description

Technology Summary:

A novel formulation of a known compound for treating diseases including cardiovascular, inflammatory, sickle cell and other diseases. The novel formulation is designed to safely administer therapeutic levels of the known compound in a liquid form.

Background:

The selected compound exhibits medical properties and has shown promise in treating a variety of diseases. Given its potential toxicity and narrow range between efficacious and toxic doses, the primary challenge in using this compound as a therapeutic agent is to deliver known levels that do not cause harm. In addition, delivery must be easy for the patient.

Patients suffering from inflammatory diseases (Crohn's disease, ulcerative colitis, rheumatoid arthritis, multiple sclerosis, systemic inflammation), ischemic diseases (coronary, organ, neural or shock-induced ischemia), cardiovascular diseases (ischemia/reperfusion, vascular disease), sepsis and others will potentially benefit from the use of the compound as a therapeutic agent. It also will be of great benefit to manage and prevent crises in patients with sickle cell disease, which affects an estimated 70,000 to 100,000 subjects in the United States. The life expectancy of these patients is only about 40 years and a large percentage experience sickle cell crises several times a year, resulting in strokes, organ damage and poor health. Currently, only chronic transfusions have been shown to reduce the number of crises and prevent strokes, although they don't prevent silent strokes that result in cognitive impairment. Transfusion programs are cumbersome, costly and put patients at risk for iron-overload. Hydroxyurea treatment reduces the number of crises but does not prevent strokes, may have secondary effects in some patients, and poor compliance with the medication occurs in the adult sickle cell population. While bone marrow transplantation can cure the disease, best results are obtained when the donor is a sibling. High-cost and co-morbidities also limit the use of this curative procedure.

Technology Description:

The invention delivers a known compound in a liquid formulation in an amount that is sufficient to reduce or eliminate at least one clinical symptom in the affected patient. The new formulation will

reduce the number of sickle cell crises experienced by the patient. The treatment may be repeated one or more times during a prescribed time period to provide a multi-dose treatment regimen and/or to provide a chronic treatment regimen.

Application area

Sickle cell disease

Inflammatory, ischemic, cardiovascular diseases

Sepsis and others

Advantages

Can be accurately delivered orally

Palatable formulation to encourage compliance

Institution

[Children's Hospital Los Angeles](#)

联系我们



叶先生

电话 : 021-65679356

手机：13414935137

邮箱：yeyingsheng@zf-ym.com