

GUIDELINES FOR THE CLINICAL CARE OF THALASSEMIA

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What is the FDA approved treatment for thalassemia? The Food and Drug Administration on Tuesday expanded the approval for exagamglogene autotemcel (Casgevy, Vertex), a CRISPR/Cas9 gene-edited cell therapy, to include the treatment of transfusion-dependent beta-thalassemia (TDT) in patients ages 12 and older.

What is the standard treatment for thalassemia? Blood transfusions are the main way to treat moderate or severe thalassemia. This treatment gives you red blood cells with healthy hemoglobin. During a blood transfusion, a needle is used to insert an intravenous (IV) line into one of your blood vessels. You receive healthy blood through this line.

What is the health advice for thalassemia? Eat a healthy diet. Your doctor might also recommend a folic acid supplement to help your body make new red blood cells. To keep your bones healthy, make sure your diet contains enough calcium and vitamin D. Ask your doctor what the right amounts are for you and whether you need a supplement.

What is the policy of thalassemia? THE THALASSEMIA PREVENTION BILL, 2022 By SHRI RAHUL SHEWALE, M.P. to promote awareness to prevent passing of thalassemia from parents to children through mutated haemoglobin genes and for matters connected therewith. 1. (1) This Act may be called the Thalassemia Prevention Act, 2022.

What is the new treatment for thalassemia in 2024? In January 2024, the FDA approved exagamglogene autotemcel (exa-cell, brand name Casgevy™) for the

treatment of transfusion-dependent beta thalassemia in patients 12 years and older.

How do you treat thalassemia in the US? Blood transfusions are the main way to treat moderate or severe thalassemia. People receiving regular blood transfusions are at risk for certain complications such as iron overload, alloimmunization (a harmful immune reaction), and infection.

What is the gold standard for thalassemia? The gold standard for the diagnosis of β -thalassemia carriers is electrophoresis or HPLC analysis of hemoglobin (Hgb).

What is the promising treatment for thalassemia? Luspatercept enhances erythroid maturation and reduces the transfusion burden by attaching to transforming growth factor β superfamily ligands. As a result, it offers a promising treatment option for β -thalassemia-affected individuals who need RBC transfusions regularly [24].

What is the drug of choice for thalassemia? Deferasirox (Exjade) It is an oral iron-chelating agent that reduces liver iron concentration and serum ferritin levels. Deferasirox binds iron with high affinity in a 2:1 ratio. It is approved for treatment of treat chronic iron overload due to multiple blood transfusions and non-transfusion-dependent thalassemia.

What makes thalassemia worse? Symptoms can worsen with fever. They can also get worse if you are exposed to certain medicines, chemicals, or infectious agents. Blood transfusions are often needed. You have a greater risk of having a child with alpha thalassemia major.

What should not eat in thalassemia?

What is the best vitamin for thalassemia? Based on the evidence, the recommendations for vitamin D supplementation among patients with thalassemia treated for 8 weeks are 50,000 IU of vitamin D2 weekly or 2000 IU of vitamin D3 for the patients with serum level of 25-OHD 20 ng/ml (50 nmol/l).

What is the treatment plan for thalassemia? Management and Treatment Standard treatments for thalassemia major are blood transfusions and iron chelation. A blood transfusion involves receiving injections of red blood cells through a vein to restore normal levels of healthy red blood cells and hemoglobin.

How do you treat thalassemia? Thalassaemia usually requires lifelong treatment with blood transfusions and medicine. Children and adults with thalassaemia will be supported by a team of different healthcare professionals working together at a specialist thalassaemia centre.

What is the life expectancy of someone with thalassemia? Persons with thalassemia trait have a normal life expectancy. Persons with beta thalassemia major live an average of 17 years and usually die by 30 years of age. Most deaths are caused by the cardiac complications of iron overload.

Does thalassemia get worse with age? Because thalassemia is an inherited condition, we can also discuss family planning. Beginning care as an adult or resuming it: Unfortunately, the effects of thalassemia can worsen with age. If you received a diagnosis in childhood but did not need treatment at the time, we still recommend an evaluation in adulthood.

What is the new drug for thalassemia? Casgevy (exagamglogene autotemcel) is indicated for the treatment of transfusion-dependent beta thalassemia and severe sickle cell disease in patients 12 years of age and older for whom haematopoietic stem cell transplantation is appropriate and a suitable donor is not available.

What is the prognosis for thalassemia? Outlook (Prognosis) Severe thalassemia can cause early death (between ages 20 and 30) due to heart failure. Getting regular blood transfusions and therapy to remove iron from the body helps improve the outcome.

Which medicine should be avoided in thalassemia? Patients with thalassaemia should never be given iron supplements. Many baby foods, breakfast cereals and multivitamin preparations contain added iron, along with other vitamin supplements.

What happens if you don't treat thalassemia? When left untreated, this condition can lead to problems in the liver, heart, and spleen. Infections and heart failure are the most common life-threatening complications of thalassemia in children. Like adults, children with severe thalassemia need frequent blood transfusions to get rid of excess iron in the body.

How often do thalassemia patients need blood? The recommended treatment for thalassaemia major involves lifelong regular blood transfusions, usually administered every two to five weeks, to maintain the pretransfusion haemoglobin level above 9–10.5 g/dl.

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What are the new drugs for thalassemia? Given its mechanism, pyruvate kinase deficiency (PKD), sickle cell disease, and thalassemias are among the hereditary hemolytic anemias for which mitapivat has been studied in clinical trials [17]. In 2022, mitapivat became the first drug to be approved by the FDA and EHA to treat hemolytic anemia caused by PKD.

How much does thalassemia treatment cost in USA? Results: Thirty-four children aged 2-17 years with transfusion dependent thalassaemia major and their parent/guardian were included in the study. The total average cost per patient year to the hospital was \$US 2601 of which \$US 2092 were direct costs and \$US 509 were overhead costs.

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The Cartoon Introduction to Philosophy: A Q&A

What is the Cartoon Introduction to Philosophy?

The Cartoon Introduction to Philosophy is a series of graphic novels that introduces readers to major philosophical concepts and thinkers in a fun and accessible way. The series is written by Michael F. Patton and illustrated by Jim Ottaviani.

What topics do the books cover?

The books in the series cover a wide range of philosophical topics, including:

- Epistemology (the study of knowledge)
- Metaphysics (the study of reality)
- Ethics (the study of right and wrong)
- Political philosophy (the study of government and society)

Who are the books aimed at?

The books are aimed at general readers who are interested in learning about philosophy but don't know where to start. They are also a great resource for teachers and students of philosophy.

What makes the books unique?

The books are unique in their use of humor and cartoons to explain complex philosophical concepts. This makes them easier to understand and more enjoyable to read.

What are some of the benefits of reading the books?

Reading the books can help you to:

- Understand the basics of philosophy
- Develop critical thinking skills
- See the world from different perspectives
- Make more informed decisions about your life and the world around you

Sins Against Science: The Scientific Media Hoaxes of Poe, Twain, and Others

Throughout history, writers have used scientific hoaxes to entertain, challenge, and deceive audiences. Some of the most infamous examples include the works of Edgar Allan Poe, Mark Twain, and others.

Edgar Allan Poe's "The Balloon-Hoax"

In 1844, Poe published "The Balloon-Hoax" in the New York Sun, claiming to have been on board a balloon that crossed the Atlantic Ocean from England to South Carolina in just 75 hours. The story was so convincing that it caused a sensation and fooled many readers.

Mark Twain's "The Petrified Man"

In 1862, Twain published "The Petrified Man" in the New Orleans True Delta, claiming to have witnessed a man being turned into solid rock by a chemical process. The story was eventually exposed as a hoax, but it raised important questions about the gullibility of the public and the ethics of scientific journalism.

Other Notable Hoaxes

Other famous scientific hoaxes include:

- **The Cottingley Fairies (1917):** Photographs of alleged fairies that were later revealed to be manipulated.
- **The Piltdown Man (1912):** A supposed ancient human skull that was later found to be a forgery.
- **The Martian Canals (1877):** The belief that Mars was inhabited by intelligent beings based on supposed observations of canals on the planet's surface.

Implications and Impact

Scientific hoaxes have had a significant impact on society. They can damage the public's trust in science, lead to the spread of misinformation, and hinder the progress of legitimate research. They also raise important questions about the role of the media in disseminating scientific information and the need for critical thinking.

Questions and Answers

- **What are some of the motivations for scientific hoaxes?**
 - To entertain or amuse audiences

- To challenge scientific authority
- To deceive the public for financial or personal gain
- **How can scientific hoaxes be prevented or detected?**
 - By promoting critical thinking and skepticism
 - By encouraging scientific literacy
 - By relying on reputable sources of information
- **What are the potential consequences of scientific hoaxes?**
 - Damage to public trust in science
 - Spread of misinformation
 - Hinderance of legitimate scientific research

Solutions to Essential Concepts in Financial Management by Brigham

Q1: What is the Time Value of Money (TVM)?

A1: The TVM acknowledges that money has different values at different points in time due to earning power. Future cash flows are worth less than present cash flows, and the concept is crucial for evaluating and comparing investments.

Q2: Explain the Capital Budgeting Process.

A2: The capital budgeting process involves evaluating long-term investment projects. It includes identifying potential projects, estimating cash flows, assessing risk, and determining the project's profitability. The process aims to make informed decisions that enhance shareholder value.

Q3: Describe the Concept of Working Capital Management.

A3: Working capital management focuses on managing a firm's short-term assets and liabilities. It involves optimizing the use of cash, inventory, and accounts receivable to maintain liquidity, minimize costs, and support overall financial health.

Q4: Discuss the Role of Financial Leverage.

A4: Financial leverage involves using debt to finance operations. It can increase return on equity, but also carries risk. When debt levels become excessive, it can lead to financial distress and bankruptcy. Understanding the appropriate level of financial leverage is critical for sound financial management.

Q5: Explain the Importance of Dividend Policy.

A5: Dividend policy determines the distribution of a firm's earnings to shareholders. It affects shareholder returns, stock price, and the availability of funds for reinvestment. Balancing shareholder expectations, growth potential, and financial constraints is essential when setting dividend policy.

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