



FDA Greenlights First Gene Therapy for Hearing Loss

Description

The Food and Drug Administration (FDA) has approved Regeneron's Otarmeni, the first gene therapy for genetic hearing loss, which is significant for affected families. This groundbreaking therapy is designed to treat a rare form of hearing loss caused by a mutation in the OTOF gene, impacting about 50 newborns in the U.S. each year. The FDA granted approval under its Commissioner's National Priority Voucher program, which accelerates the review process for certain therapies.

Dr. Eliot Shearer, a pediatric otolaryngologist, described the approval as life-changing for families with hearing-impaired children. Currently, the only other treatment available for genetic deafness is cochlear implants. These devices can restore sound but do not capture the full range of sound quality. In contrast, the gene therapy offers continuous hearing that is not dependent on batteries.

Otarmeni works by correcting the faulty OTOF gene, which is crucial for making a protein called otoferlin. This protein is essential for transmitting sound signals from the ear to the brain. The FDA's approval was based on a trial involving 20 children with the OTOF mutation, where 16 showed improved hearing after a single dose.

While Regeneron will provide the drug at no cost to U.S. patients, the company does not control the administration costs, which are similar to cochlear implant surgery. The drug is currently not approved outside the U.S., and there is no price set for those living abroad. Sarah Emond, CEO of the Institute for Clinical and Economic Review, praised Regeneron for its strategy to ensure affordable access to this innovative therapy.

Vocabulary List:

1. **approved** //ə'pruvd// (verb): officially allowed or given permission to use
2. **therapy** //'θerəpi// (noun): treatment to help cure or ease a condition
3. **mutation** //mju:'teɪʃən// (noun): a change in a gene that can cause differences
4. **cochlear** //'kɔkliər// (adjective): related to the inner ear for hearing
5. **transmitting** //træns'mɪtɪŋ// (verb): sending signals or information to another place
6. **trial** //'traɪəl// (noun): a test of a new medicine or treatment

Comprehension Questions



Multiple Choice

1. What is Otarmeni designed to treat?

- Option: Genetic hearing loss
- Option: Cochlear deafness
- Option: Eardrum damage
- Option: Sound processing disorders

2. How many newborns in the U.S. are impacted by the OTOF gene mutation each year?

- Option: 20
- Option: 50
- Option: 100
- Option: 200

3. What program did the FDA use to accelerate the review of Otarmeni?

- Option: Fast Track Program
- Option: Orphan Drug Program
- Option: Breakthrough Therapy Designation
- Option: Commissioner's National Priority Voucher

4. What other treatment is currently available for genetic deafness?

- Option: Hearing aids
- Option: Cochlear implants
- Option: Gene therapy
- Option: Sound therapy

5. How many children were involved in the trial for Otarmeni?

- Option: 10
- Option: 15
- Option: 20
- Option: 25

6. What does Otarmeni correct?

- Option: Faulty OTOF gene
- Option: Hearing frequency
- Option: Eardrum vibrations
- Option: Sound amplification



True-False

7. Otarmeni is the first gene therapy approved for genetic hearing loss.
8. The FDA's approval was based on a trial with 30 children.
9. Cochlear implants can capture the full range of sound quality.
10. Regeneron will provide Otarmeni at no cost to U.S. patients.
11. The gene therapy works by fixing the OTOF gene, which is necessary for otoferlin production.
12. The drug Otarmeni is currently approved for use outside the U.S.

Gap-Fill

13. The FDA approved Regeneron's Otarmeni, the first gene therapy for genetic hearing loss, which is significant for affected _____ .
14. Otarmeni offers continuous hearing that is not dependent on _____ .
15. The gene therapy works by correcting the faulty _____ gene.
16. Regeneron's drug is currently not approved _____ the U.S.
17. The FDA's approval of Otarmeni was based on a trial involving 20 children with the _____ mutation.
18. Sarah Emond praised Regeneron for its strategy to ensure affordable access to this _____ therapy.

Answer

Multiple Choice: 1. Genetic hearing loss 2. 50 3. Commissioner's National Priority Voucher 4. Cochlear implants 5. 20 6. Faulty OTOF gene

True-False: 7. True 8. False 9. False 10. True 11. True 12. False

Gap-Fill: 13. families



14. batteries 15. OTOF 16. outside 18. innovative

Vocabulary quizzes

Multiple Choice (Select the Correct answer for each question.)

1. What does 'capacity' typically refer to in a business context?
 - Option: The maximum output a company can produce
 - Option: The amount of funding available
 - Option: The number of investors in a startup
 - Option: A regulatory requirement
2. What is usually a primary source of funding for startups?
 - Option: Grants
 - Option: Personal savings
 - Option: Investors
 - Option: All of the above
3. Which of the following best describes 'infrastructure'?
 - Option: Financial support mechanisms
 - Option: Basic physical systems of a business or nation
 - Option: Community advocacy groups
 - Option: Technological advancements
4. Who are typically considered 'investors'?
 - Option: People who support community projects
 - Option: Individuals or entities that provide capital for a business
 - Option: Participants in clinical trials
 - Option: Workers in refineries
5. What are the environmental consequences of pollution?
 - Option: Improved health
 - Option: Cleaner air and water
 - Option: Harm to wildlife and ecosystems
 - Option: Increased biodiversity
6. What does it mean when a project is 'approved'?



- Option: It has been unanimously rejected
- Option: It has received formal consent to proceed
- Option: It is still under consideration
- Option: It has been put on hold

7. In genetics, what does 'mutation' refer to?

- Option: An improvement in DNA sequences
- Option: A change in the DNA sequence
- Option: The creation of new therapeutic methods
- Option: A type of funding model

8. Which term describes the income generated from normal business operations?

- Option: Expenditure
- Option: Profit
- Option: Revenue
- Option: Assets

9. What do 'regulations' typically enforce?

- Option: Voluntary compliance in industry
- Option: Mandatory rules that govern behaviors
- Option: Market fluctuations
- Option: Increased competition

10. What does 'transition' mean in the context of organizational change?

- Option: A permanent change
- Option: A change from one state to another
- Option: An increase in staff
- Option: A decrease in revenue

Gap-Fill (Fill in the blanks with the correct word from the vocabulary list.)

- 11. The manufacturing plant is operating at full _____ to meet demand.
- 12. The startup secured additional _____ to launch its new product.
- 13. Several new _____ expressed interest in the company during the pitch.
- 14. The city upgraded its _____ to support more traffic and reduce delays.
- 15. The _____ gathered outside the building to voice their concerns about pollution.



16. The government implemented new laws to combat air _____ in urban areas.
17. The project was _____ by the board after a thorough review.
18. The _____ in the virus's genetic code made it more resilient.
19. The company reported a significant increase in its _____ this quarter.
20. New _____ for construction were enacted to enhance safety measures.

Matching Sentences (Match each definition to the correct word from the vocabulary list.)

21. The production facility operates at a maximum capacity of 10,000 units per day.
22. The project received significant funding from several venture capitalists.
23. Many investors are looking for sustainable business models in the current market.
24. Investing in infrastructure is crucial for economic growth.
25. The protesters voiced their opposition to the proposed legislation.
26. Efforts to reduce pollution are necessary for a healthy environment.
27. The new regulations were approved unanimously by the committee.
28. The scientists studied the mutation of the virus to develop a vaccine.
29. The company's revenue has increased significantly over the past year.
30. The government has established new regulations to protect wildlife.

Answer

Multiple Choice: 1. The maximum output a company can produce 2. All of the above 3. Basic physical systems of a business or nation 4. Individuals or entities that provide capital for a business 5. Harm to wildlife and ecosystems 6. It has received formal consent to proceed 7. A change in the DNA sequence 8. Revenue 9. Mandatory rules that govern behaviors 10. A change from one state to another

Gap-Fill: 11. capacity 12. funding 13. investors 14. infrastructure 15. protesters 16. pollution 17. approved 18. mutation 19. revenue 20. regulations

Matching sentence: 1. capacity 2. funding 3. investors 4. infrastructure 5. protesters 6. pollution 7. approved 8. mutation



9. revenue 10. regulations

CATEGORY

1. Business - LEVEL5

POST TAG

1. ESL learning
2. esl news
3. FDA approval
4. gene therapy
5. genetic hearing loss
6. Level 5

Tags

1. ESL learning
2. esl news
3. FDA approval
4. gene therapy
5. genetic hearing loss
6. Level 5

Date Created

2026/04/24

Author

aimeeyoung99

ESL-NEWS.COM