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** //əˈpruvd// (verb): officially allowed or given permission to use
2. **therapy** //ˈθɛrəpi// (noun): treatment to help cure or ease a condition
3. **mutation** //mjuːˈteɪʃən// (noun): a change in a gene that can cause differences
4. **cochlear** //ˈkɑkliər// (adjective): related to the inner ear for hearing
5. **transmitting** //trænsˈmɪtɪŋ// (verb): sending signals or information to another place
6. **trial** //ˈtraɪəl// (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

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