Adrenoleukodystrophy (ALD): A Case Study Using the Film "Lorenzo's Oil"

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Introduction

The purpose of this activity is to expose the students to an inherited genetic disorder by viewing the film "Lorenzo's Oil." The students will be able to learn about this rare disease and follow the progression of the disease from the initial diagnosis through the 32 month ordeal that the family endured. They will experience the frustrations and triumphs with the Odone family as they follow the challenges of finding a cure for ALD.

The students will observe the scientific method being put into practice. This is a true-life drama which depicts the social, financial, ethical and political ramifications of a little-known genetic disease that doesn't get much attention from doctors nor the research community.

This is an interdisciplinary study that could be used on several levels and with a variety of classes, for example: Biology, Chemistry, Genetics, Ethics, Health, Neurology, Nutrition, Pathology, Psychology and Anatomy.

Summary of the Disease

Adrenoleukodystrophy (ALD) is a rare inherited metabolic disorder characterized by the loss of the fatty covering (myelin sheath) on nerve fibers within the brain and progressive degeneration of the adrenal gland. The basic defect is the impaired capacity to degrade very long chain fatty acids that are found in the blood plasma and tissues of the body. These fatty acids accumulate in the brain (cerebral white matter) and the adrenal glands.

ALD is an X-linked recessive inheritance of a syndrome of Addison disease and cerebral sclerosis. It only affects males although neonatal autosomal ALD may also affect females. This neonatal form is clearly separate from the X-linked ALD. Symptoms often appear in boys between the ages of 4 and 8 and may include behavioral changes such as poor memory, loss of emotional control and dementia. Other symptoms may include muscle weakness, difficulties with hearing, speech and vision.

The symptoms of ALD may develop due to abnormal or absent peroxisomes

(microbodies that participate in the metabolism of fats) in the liver. This causes a disturbance of fatty acid metabolism and results in the abnormal accumulation of very long chain fatty acids. The exact enzyme deficiency that prevents the breakdown of VLCFAs is not known.

ALD is an allelic mutation. This disease is caused by a single mutation at the locus Xq28. There is a close linkage of ALD to the cluster of color-blindness genes. This single mutation causes deficient activity of the necessary enzyme ligase. There is a 50% chance the disease will be passed from mother to offspring. The male will express the disease and the female will be a carrier.

Diagnosis can be made from cultured skin fibroblasts or amniotic fluid cells and allows prenatal diagnosis and carrier identification of 90% of obligate heterozygotes. More recently it has been shown that the biochemical diagnosis can be performed on chorionic villi biopsy.

This disease can be prevented with a combined approach of oleic acid (Lorenzo's Oil) and a restricted diet minus very long fatty acids. Also some ALD patients have been treated experimentally with Glycerol Trioleate. More research is needed to determine the long-term safety and effectiveness of these substances.

The prognosis is excellent for those individuals who are identified prenatally or shortly after birth. There can be normal functions for those individuals who take the prescribed dose of oil and follow the restricted diet regimen.

Summary of the Film: "Lorenzo's Oil,"

MCA Universal, with Nick Nolte and Susan Sarandon, l992.

This film is based on a true story which took place from l984 to l987. The sobering news that their five year old son Lorenzo has a rare terminal disease marks the beginning of an extraordinary mission for Augusto and Michalla Odone. Despite the prognosis of death in two years because no treatment was known, the Odones set out to save their child, colliding with doctors, scientists, and support groups who are reluctant to encourage the couple in their quest for a cure. Their relentless struggle tests the strength of their marriage, the depth of their beliefs, and the boundaries of conventional medicine.

The film shows how individuals can make a difference and by using models and the scientific method the Odones discover by trial and error a treatment for ALD. The film ends on a positive note by showing several healthy children who are presently taking Lorenzo's Oil and are symptom-free of the disease. It is very heart-warming to see these normal children and know they have only the Odones to thank for their normal lives.

Another positive ending is the Myelin Project which has been funded by the national government to find a way to restore the myelin sheath which surrounds nerve cells. If the myelin can be restored, patients like Lorenzo can lead a normal life. The conclusion relates that Lorenzo can communicate by using a computer and his father, Augusto Odone, has been awarded an honorary Ph.D. for his pioneering work in researching and discovering a significant treatment for ALD.

Target Age/Ability Group:

Middle School, High School, and College Level Classes

Classtime

- 2 hours and l8 minutes for viewing the film "Lorenzo's Oil"
- 1/2 to 1 hour: Answering discussion questions (orally and written)

Materials

- Video-cassette, "Lorenzo's Oil." Can be rented or purchased.
- Hand-out sheet of summary questions

Student Activities

1. Reports: Case studies of other genetic disorders using research techniques.
2. Research on the Human Genome Project.
3. Make a Karyotype of the known genetic disorders.
4. View "Awakenings," dealing with Parkinson's Disease, and compare the films.
5. Do a pedigree chart tracing Lorenzo's disease.
6. Make an individual health-based family tree. This "genogram" can be used to determine medical risks the students have inherited. They may learn their particular risks for a disease and take preventative measures and change behavior patterns.
7. Invite a speaker from a research center on genetic disorders.
8. Have a classroom debate on bioethical issues including research time and money spent on rare genetic disorders as compared with common diseases.

References

Bruckheim, A. H., M.D., **The Family Doctor,** Educators Advantage. l994.

Lewis Ricki, **Human Genetics, Concepts and Applications,** Wm. C. Brown Publishers, Oxford, England, l994.

"Lorenzo's Oil," MCA Universal Studio, l992.

McKusick, Victor A., M.D., **Mendelian Inheritance in Man,** 9th Ed., Johns Hopkins University Press, Baltimore, Maryland, l990.

Resources

For more information on Adrenoleukodystrophy, please contact:

National Organization for Rare Disorders (NORD)
P.O. Box 8923
New Fairfield, CT 06812-1783
(203) 746-6518

ALD Project
Dr. Hugo W. Moser
John F. Kennedy Institute
707 North Brockway

Baltimore, MD 21205
(410) 522-5405

For Genetic Information and Genetic Counseling Referrals:
March of Dimes Birth Defects Foundation
1275 Mamaroneck Avenue
White Plains, NY 10605
(914) 428-7100

Film Guide For Lorenzo's Oil

Please read these questions over before you see the film!!! Then consider and write your answers on your own paper.

1. Show how Lorenzo's parents used the scientific method to solve their problem. State the problem, ask a question and use examples from the film to illustrate the steps.

2. From the description of the disease, ALD, sketch what Lorenzo's neurons most likely looked like after a year. Include a sketch of a normal neuron.

3. ALD is a recessive sex-linked or X-linked disease. What are the probable genotypes of the family members listed? Aunt #1, Aunt #2, Aunt Dee, Michalla, Augusto, Lorenzo.

4. What is Lorenzo's Oil? How does it work? Use the sink model from the film.

5. Explain Augusto's use of paper clips to represent good and bad fatty acids. What did his dream help him understand?

6. Why did the medical community resist the Odones' treatment ideas? Why did the parent support group resist? Give examples of arguments for and against.

7. How do analogies help you to understand the scientific problem? How did modeling help solve the scientific problem? Can you think of other great discoveries that employed the use of models?

8. Did the family's courage and persistence inspire you? How?

9. How did Augusto and Michalla demonstrate "life-long" learning?

10. Defend the position that diseases that affect a small % of the population should not get as much money for research as the big killers like cancer and heart disease.

11. Why did olive oil not work completely? Trace the developments in the different kinds of oils discovered.

12. What do you think is the role of support groups? How did the support group effect the Odones?

13. What was found out from the Polish biochemist? What was the purpose of the ALD Symposiums? What happens when scientists work in isolation?

14. What is erucic acid?

15. Where was the myelin research being done? What was the experiment? How could this research help Lorenzo?

16. How did the treatment for ALD really happen by accident? Can you think of any other times in medical history where an important discovery happened by mere chance?

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