

Lorenzo's Oil

Part One.

1. Lorenzo begins to experience problems at school. What symptoms first appear?
2. What medical tests were done to assess Lorenzo's condition?
3. When Lorenzo is diagnosed with (ALD) Adrenoleukodystrophy the doctor explains the prognosis for this disorder? What does the doctor tell Mr. and Mrs. Odone?
4. The inheritance pattern for this disease is referred to as
5. Lorenzo inherits ALD from which parent? Explain why it is always this parent.
6. The biochemical pathology of this disease is related to the metabolism of which class of macromolecules?
7. What is myelin? How does ALD affect the myelin sheath of Lorenzo's neurons?
8. What trial studies did Lorenzo's parents try in order to help their son fight ALD?
9. What changes occur in the lives of the Odone's, as a result of Lorenzo's illness?
10. What decision did Lorenzo's parents make about the way they would fight the disease to save their son?

Part Two.

1. What is the general attitude of the people in the foundation towards research?
2. What is the response of the foundation leader to the ideas of the Odone's?
3. Describe the opposition the Odone's face from the medical community?
4. What is an orphan drug?

5. Augusto Odone makes a model to compare Lorenzo's condition to a kitchen sink. What is the significance of the two faucets and the drain?
6. Michaela found an interesting research article on Polish rats. What was the significance of this research?
7. During a medical conference what ideas are open to discussion on possible solutions to providing a treatment for ALD?
8. What oil offers a possible solution to Lorenzo's high level of C24 & C26 fat levels? Where did Lorenzo's father get this oil? How did Lorenzo consume the oil?
9. Explain why is this oil is only partially effective.
10. What does the paper clip model demonstrate to Augusto? What is his dream?

Part Three

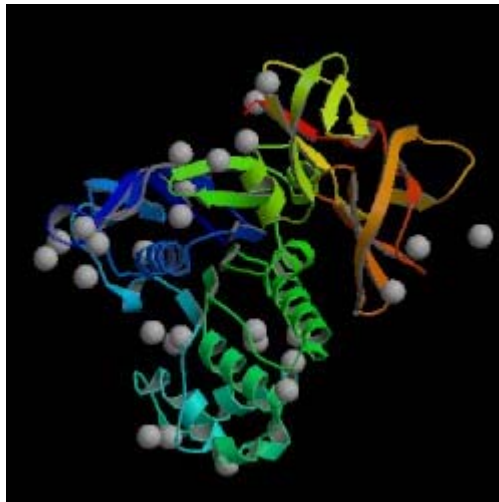
1. When Augusto explains the dream to the doctor, the paper clip model is shown to have a specific property of an enzyme reaction. Identify that property.
2. What is the name of the second oil? This oil is extracted from what plant? What is the medical basis of using this oil?
3. Who is Lorenzo's rat? Why can the therapy of Erucic acid be tested on Michaela's sister or Michaela? Why are the doctors and family concerned about toxicity?
4. After the administration of the oil, how does Lorenzo's condition begin to change?
5. When the Odone's present their findings how do the parents in the foundation respond? How is the news received in the medical community?
6. Augusto gets a new idea for a "cure" for ALD from shaking puppies. Explain how.
7. How would a cure for the ALD boys offer hope for other neurological conditions?

ALD Protein - Membrane Transporter in the Peroxisomes

Protein sequence

SEQUENCE 745 AA; 82908 MW; 13A8EFDE9EB1E7FA CRC64;

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MPVLSRPRPW RGNTLKRTAV LLALAAYGAH KVYPLVRQCL APARGLQAPA GEPTQEASGV  
AAAKAGMNRV FLQRLWLLR LLFPRVLCRE TGLLALHSAA LVSRTFLSVY VARLDGRLAR  
CIARKDPRAF GWQLLWLLI ALPATFVNSA IRYLEGQLAL SFRSRLVAHA YRLYFSQQTY  
YRVSNMDGRL RNPDQSLTED VVAFSAASVAH LYSNLTkPLL DVAVTSYTLL RAARSRGAGT  
AWPSAIAGLV VFLTANVLR A FSPKFGELVA EEARRKGELR YMHSRVVANS EEIAFYGGHE  
VELALLQRSY QDLASQINLI LLERLWYVML EQFLMKYVWS ASGLLMVAVP IITATGYSSES  
DAEAVKKAAL EKKEEELVSE RTEAFTIARN LLTAAADAIE RIMSSYKEVT ELAGYTARVH  
EMFQVFEDVQ RCHFVKRPREL EDAQAGSGTI GRSQVVRVEGP LKIRGQVVDV EQGIICENIP  
IVTPSGEVVV ASLNIRVEEG MHLITGPNQ CGKSSLFRL GGLWPTYGGV LYKPPPQRMF  
YIPQRPYMSV GSLRDQVIYP DSVEDMQRKG YSEQDLEAIL DVVHLHHILQ REGGWEAMCD  
WKDVLGGEEK QRIGMARMFY HRPKYALLDE CTSAVSIDVE GKIFQAAKDA GIALLSITHR  
PSLWKYHHTL LQFDGEGGWK FEKLSAARL SLTEEKQRLE QQLAGIPKMQ RRLQELCQIL  
GEAVAPAHVP APSPQGPGL QGAST
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Family of Molecules - ATPase - Transporters

References

Patty Sidelesky - Teacher (btang.org)

<http://carbon.cudenver.edu/~bstith/loren.htm> - Web site that explains the biochemistry

<http://www.myelin.org/aboutoil.htm> - The Myelin Project

<http://bioinfo.weizmann.ac.il/cards-bin/carddisp?ABCD1&search=Adrenoleukodystrophy&suff=txt> - Gene Card for Adrenoleukodystrophy

<http://www.ncbi.nlm.nih.gov/htbin-post/Omim/dispim?300100> - OMIM entry

<http://www.ncbi.nlm.nih.gov/disease/ALD.html> - NCBI link

http://www.ninds.nih.gov/health_and_medical/disorders/adrenolu_doc.htm - NINDs link