

LJ

October 2011

COMM-1 Intro to Speech

Favorable Legislation for the Production of Orphan Drugs

**Topic:** Orphan Drug Legislation

**General Purpose:** To Persuade

**Specific Purpose:** Persuade the audience that there is a need to shift government policy to legislation in favor of orphan drug production.

**Central Idea:** Amending current legislation to become more favorable towards drug companies that produce orphan drugs, will lead to an increase in research towards both orphan drugs and orphan diseases.

**Attention Getter:**

- I. On January 2, 2008, a baby boy named Liam was born to Michelle and Chris Schulze of Colorado Springs, CO.
  - A. At four months old, Liam began to have cyclical fevers every few days becoming more and more frequent as time progressed.
  - B. In March of 2009, Liam was admitted to Children's Hospital of Aurora, CO. There he stopped eating, became jaundiced, and experienced pain.
  - C. Undergoing various diagnostics, Liam was diagnosed with Hemophagocytic lymphohistiocytosis (HLH) at fourteen months old.

**Introduction of Topic:**

- II. HLH is a rare disease, or orphan disease, that can be potentially fatal.
  - A. It is caused by an overactive histiocyte, which causes fever, hepatosplenomegaly, or enlargement of the spleen, and lymphadenopathy, or the enlargement of the lymph nodes.

- B. Drugs that would treat rare diseases similar to HLH are known as orphan drugs.
- C. Orphan drugs get their name from their lack of financial support for research
- D. . The development of the drug lacks of capital needed to produce a viable product which will be safe product that passes the Food and Drug Administration's market approval standards.

**Statement of Credibility/ Relevance:**

- I. According to *Pharmaceutical Executive*, as of 2010, about 7,000 rare diseases have been given the designation of an "orphan disease" by the FDA.
- II. In 1983, The Orphan Drug Act was passed, providing incentives for the development of orphan drugs.
- III. Since the law was passed, 353 orphan drugs have been approved for marketing.

**Thesis:**

By maintaining current legislation and passing laws that are more favorable to orphan drug production, we will continue to produce possible treatments and medical breakthroughs for orphan drugs.

**Let us explore this disease in the following way.**

- 1. To understand the importance of laws favorable towards orphan drugs, we must first look at what orphan drugs are as well as what is Orphan Drug Act.
- 2. We will also look at the problem to why more orphan drugs are not being researched,
- 3. How this can be solved.

**Transition:**

First, we will look at orphan drugs and the Orphan Drug Act.

**Body**

## **I Orphan drug background:**

### **A. Let's begin to explore the frequency of these problem.**

1 .According to the FDA, the Orphan Drug Act (ODA) designates an orphan disease to affect fewer than 200,000 people in the United States.

(1) An orphan drug is a drug that treats or prevents one of these orphan diseases.

### **B. Approval is a lengthy process**

According to the National Consumer Program, Generics are Powerful; patents for new drugs are for 20 years.

a. Essentially, this allows a drug company to hold the rights to sell their drug without having to worry about generics taking away market share.

b. According to the Health Law Journal, orphan drugs get added 7-year market exclusivity patent for their drug along with tax credits that equate to approximately half of the total development cost.

C. As previously stated, after the passing of the ODA, 353 drugs have been approved by the FDA for marketing.

D. In comparison, according to the Journal of Science, prior to the ODA, less than 40 orphan drugs were approved by the FDA.

E Controversy surrounding the ODA involves the incentives it provides for drug companies and whether the orphan drugs being produced actually have no expectation of producing a profit for a company.

1. According to the Journal Health Policy, some drugs have been able to produce sizable profits for companies are more frequently used for different purposes other than their original purpose, which granted the drug orphan status.
2. Examples of these drugs include Botox, Cialis, and Epogen.

**Transition:**

Second, we will look at the problems surrounding orphan drug research.

**II. Problem**

- A. Thousands of orphan diseases are known to exist, yet there are no foreseeable treatments being researched.
- B. This is due to the lack of funding placed toward orphan drugs by pharmaceutical companies looking to make a profit.
  - a. Pharmaceutical companies are not at fault, as their existence is only possible if they produce profitable drugs.
  - b. One hindrance to the production of orphan drugs is current patent legislation
  - c. Currently drug companies are only allowed to control the sole rights to their orphan drugs for up to 27 years.
  - d. Usually a drug company can easily make a profit from a drug they have spent hundreds of millions of dollars to develop
  - e. This leads to the sponsor the drug through the Food and Drug Administration's approval process.
- C. There has been an increase in orphan drugs produced since the Orphan Drug Act of 1983.

- a. The ODA has been responsible for over three hundred orphan drugs and according to *Pharmaceutical Executive* over one thousand orphan drug patents.
- b. Unfortunately, this does not really encompass extremely rare diseases.
  - i. There are diseases that affect far less than the 200,000 necessary to be considered an orphan disease and are far less likely to make a profit.
  - ii. In this case, longer exclusivity extensions should be given proportionate to the development costs and the rarity of the disease.
  - iii. There can still be more done that can be conducive to the production of orphan drugs via policy changes as well as through awareness of non-profit organizations that help fund orphan drug and disease research.

**Transition:**

Last, we will look at what everyone can do to make orphan drug research more viable to drug companies.

**III. Solution:**

- A. There are international and national non-profit organizations that provide funding towards orphan disease research along with programs that try to increase public awareness of orphan diseases and legislation that may help or hurt progress toward orphan disease research.
- B. To find more information about orphan diseases you can visit the following website
  - 1. National Organization of Rare Disorders or NORD website:

[rarediseases.org](http://rarediseases.org)

2. The European equivalent organization, Eurordis website: [eurordis.org](http://eurordis.org)
  3. You can visit NORD's advocacy page at  
<http://www.capwiz.com/rarediseases/home/>
- C. Through their advocacy page, you can find information about current bills that may help or hurt rare disease research as well as the information for the public officials that represent you. Including names, address, emails, and phone numbers.
- D. Through [rarediseaseday.org](http://rarediseaseday.org), organized by EURODIS, you can find local and international events that increase public awareness of rare diseases on Rare Disease Day this upcoming February 29, 2012.

## **Conclusion**

### **Review:**

- I. Today we have discussed orphan drugs
  - A. The Orphan Drug Act.
  - B. We have looked at the problems in producing orphan drugs an
  - C. What we all can do to try to get more orphan drugs on the market.

### **Return to Attention Getter:**

- III. Liam continued to fight HLH until it was necessary to perform a risky bone marrow transplant.
  - A. Unfortunately, Liam also had a rare HLA type, which made it difficult to gather a sufficient amount of bone marrow for the transplant.
  - B. Liam passed away in the arms of his mother on September 9, 2009.
  - C. Liam may not have survived, but there is still hope that HLH and other orphan diseases will likely have methods for treatment in the near future.



## Works Cited

- Armstrong, Walter. "Pharma's Orphans - Pharmaceutical Executive." *Pharmaceutical Executive - Pharmaceutical News and Analysis. Pharma Management, Sales, Marketing & Finance*. Pharmaceutical Executive, 1 May 2010. Web. 06 Oct. 2011.
- Cheung, Richard Y., Jillian C. Cohen, and Patricia Illingworth. "Orphan Drug Policies: Implications for the United States, Canada, and Developing Countries." *Health Law Journal* 12 (2004): 183-200. Print.
- Couzin, Jennifer. "Advocating, the Clinical Way: as Advocacy Groups in the Orphan Disease World Plunge into Clinical Trials, They're Faced with a Delicate Balancing Act." *Science* 308 (2005): 940. Print.
- Food and Drug Administration. "Definition of Disease Prevalence for Therapies Qualifying Under the Orphan Drug Act." *U S Food and Drug Administration Home Page*. Food and Drug Administration, 7 Sept. 2010. Web. 06 Oct. 2011.
- Food and Drug Administration. "Orphan Drug Act." *U S Food and Drug Administration Home Page*. Food and Drug Administration, 10 May 2011. Web. 08 Oct. 2011.
- GPM. "Why Does It Take so Long for Generic Drugs to Become Available? - Generics Are Powerful Medicine." *Generics Are Powerful Medicine*. 2010. Web. 07 Oct. 2011.  
<<http://www.genericsarepowerful.org/learn?id=0011>>.
- Wellman-Labadie, Olivier, and Youwen Zhou. "The US Orphan Drug Act: Rare Disease Research Stimulator or Commercial Opportunity?" *Health Policy* 95 (2010): 216-28. Print.