

The Orphan Drug Act: How Debate and Diplomacy Improved Healthcare for Orphan Disease Patients

Jasper Gu

Senior Division

Individual Exhibit

Student-Composed Words: 492

Process Paper: 498

Process Paper

Being fascinated by science, I explored the broad subject and came upon orphan diseases. Further studying orphan diseases, I discovered the Orphan Drug Act (ODA) was created to help pharmaceutical companies produce orphan drugs and decided to make this the topic for my exhibit. The ODA is related to the theme as there were many debates between Congress, the FDA, pharmaceutical companies and the public throughout the creation of it. Additionally, the ODA itself is an act of diplomacy since the different parties created it through negotiations and compromises. While forming the ODA there were successes and failures from multiple sides in the process of debate, however in the future unexpected consequences occurred.

To study this topic, I used many resources including a journal written about past medical events, a publication by Henry Waxman about events in Congress and online articles containing history of the ODA. Additionally, I had a personal interview with Henry Waxman on his role in the ODA while he was Chairman of the Health and Environment Subcommittee. This interview was very helpful, I was able to see his perspective and compare it to sources I had used which helped me form my own judgment on the topic. These sources helped me understand the history of orphan drugs and identify successes, failures and consequences that led to and followed the creation of the Orphan Drug Act.

I made my exhibit into four main sections, background, success, failures and consequences. I decided to have background as a part of the board so I can inform the viewers of necessary content that led up to the issue. Additionally, success, failures and consequences are components of the exhibit because they are minor elements of the theme. However, I believe debate and diplomacy are major elements of this year's theme so I incorporated them throughout the exhibit and chose to highlight the ideas.

Orphan drug development came to a halt when the Kefauver-Harris Amendment was legislated. Patients with various orphan diseases were unable to access medication since pharmaceutical companies were refusing to develop orphan drugs. This sparked debates among different affiliations in the 1980's. The result was an act of diplomacy, the creation of the Orphan Drug Act (ODA). Although the Act created a dramatic increase in orphan drug development helping orphan disease patients receive treatment, it contained loopholes that pharmaceutical companies abused for their own gain.

The Orphan Drug Act was a significant moment in history because it had encouraged pharmaceutical companies to create orphan drugs who were unwilling to at the time. This resulted in a countless number of people with orphan diseases being helped. Before the ODA, there had been only ten drugs on the market for orphan diseases, but as a result of the bill passing there have now been over 400 orphan drugs passed by the FDA. This had a major impact since there are over 20 million people with orphan diseases, meaning roughly 1 in 10 United States citizens have an orphan disease.

Annotated Bibliography

Primary Sources:

Duricka, John. "Jack Klugman Testifying in Congress." *7 Fascinating Facts about Jack Klugman*, 1981, <https://www.metv.com/lists/7-fascinating-facts-about-jack-klugman>. Accessed 2021.

Henry Waxman decided to invite Jack Klugman to testify before Congress to convince that orphan drugs were a big problem. This picture taken by John Duricka showed the seriousness of the situation in Congress and the face of determination for Jack Klugman to convince the officials. This was also helpful as it visualized the image of debate in Congress not just for me but also those who view my exhibit.

Gu, Jasper. "Interview with Henry Waxman." 19 Dec. 2021.

Henry Waxman was the chief sponsor of the ODA and as the U.S. Representative of California from 1975 to 2015, he was a key figure in the creation of it. I was lucky enough to reach out to him and receive a response. With the interview with him I discussed his part in the ODA and his personal thoughts which would be unattainable through other sources. It was very helpful since I could get in touch with such a big figure in my topic.

Henry Waxman. 2006, <http://www.democrats.reform.house.gov/photo.jpg>. Accessed 5 Apr. 2022.

This image of Henry Waxman helps viewers visualize him as he was a key figure in the ODA. As the chief sponsor of the act he worked with a group from the orphan disease community and spread awareness about the topic. This led him to proposing the problem to Congress, which resulted in the ODA.

"Letter to Ronald Reagan to Sign the ODA." *Orphan Drugs*, 2018, <https://99percentinvisible.org/episode/orphan-drugs/>. Accessed 17 Oct. 2021.

The picture of the letter sent to Ronald Reagan helped me understand how many people wanted the Orphan Drug Act to be passed and how many people are now grateful for what he had done. It also showed how useful the publicity from Jack Klugman's was, since without him not as many people would have gathered together to create the letter.

"President Kennedy Signs Kefauver-Harris Amendments of 1962, Food and Drug Review." *Oscar Hopefuls Contending with Drug War History*, 2015, <https://pointshistory.com/2015/10/13/oscar-hopefuls-contending-with-drug-war-history/>. Accessed 2021.

This image shows President John F. Kennedy signing the Kefauver-Harris Amendments, which caused drug production to increase in price. This was helpful as it showed a major event, the Kefauver-Harris Amendments caused drug production prices to dramatically increase, inclining drug manufacturers to focus on mainstream drugs instead of orphan drugs.

“Protest Scene from Quincy M.E. Episode ‘Give Me Your Weak.’” *Orphan Drugs*, 2018, <https://99percentinvisible.org/episode/orphan-drugs/>. Accessed 2021.

This picture from the show, *Quincy M.E.* that had starred Jack Klugman and was produced by Maurice, gave me an understanding of the impact the public can make as it convinced a senator to let the Orphan Drug Act pass through the Senate. This image was helpful as it showed the amount of support that Jack Klugman and Maurice Klugman had for the orphan drug issue.

“Richard Crout.” *Aspet*, 2020, <https://www.aspet.org/aspet/news/news/2020/07/09/obituary-j-richard-crout>. Accessed 2021.

This picture showed what Richard Crout looked like since he was a big figure in the FDA at the time of the Congressional hearings, playing a big role in the outcome of the Orphan Drug Act.

Waxman, Henry A. *The Waxman Report: How Congress Really Works*. Twelve, 2010.

The Waxman Report provided information on events that took place in Congress. Additionally, it gave the story of how he and his group worked on the ODA and brought the orphan drug issue to the public eye. It was helpful as it provided a self account of what happened in Congress by a key contributor of the ODA.

Witt, Karen. “HOUSE TOLD OF NEED FOR 'ORPHAN DRUGS'.” *New York Times*, 10 Mar. 1981, p. 16.

This newspaper from the New York Times helped me visualize what it was like in Congress while the ODA was being passed. From the images it showed me how determined each party was to debate for their side. It was helpful as it was not just one photo, but three photos consisting of Jack Klugman, J. Richard Crout from the FDA and Ted Weiss, who is a representative of Manhattan.

Secondary Sources:

Armstrong, Walter. "Pharma's Orphans." 1 May 2010.

From this story it talks about orphan drugs and his personal account of events such as other peoples experiences with orphan diseases. It additionally gives evidence of the increase of orphan drugs after the ODA had been put in place.

Buck, Jerry. "TV Actor Goes to Congress to Plead for 'Orphan Drugs'." *The Lawrence Daily - Journal World*, 1981.

This newspaper article helped me understand how much Jack Klugman cared about orphan drugs and his love for his brother as Maurice Klugman had a rare disease too. It was also helpful since it was written by someone in 1981 which showed a point of view from the news while the event was happening.

Cheung, Richard, et al. "Orphan Drug Policies: Implications for the United States ..." *Orphan Drug Policies: Implications for the United States, Canada, and Developing Countries*, 2004,
https://www.researchgate.net/publication/7239721_Orphan_drug_policies_implications_for_the_United_States_Canada_and_developing_countries.

The text was about the history of the ODA and describes how different events had impacted the result of it. Other than orphan drugs in the United States it additionally provides details on the status of the drugs in other countries such as Canada and Singapore. This study helped me analyze the background of how the act started and the consequences that it had in the future.

Commissioner, Office of the. "John Swann Comments on Rare Disease Day 2018." U.S. Food and Drug Administration, FDA, 2018,
<https://www.fda.gov/industry/orphan-products-development-events/story-behind-orphan-drug-act>.

Written by a FDA historian it talks about the history of the ODA. It helped give an overall summary and communications between different parties. The report discussed events such as the Department of Health and Humans services promoting orphan drugs and the role that Abbey Meyers, the head of the National Organization for Rare Disorders, played in the ODA.

"Drug Prices for Rare Diseases Skyrocket While Big Pharma Makes Record..." *Drug Prices for Rare Diseases Skyrocket While Big Pharma Makes Record Profits*, AHIP, 10 Sept. 2019,

<https://www.ahip.org/news/press-releases/drug-prices-for-rare-diseases-skyrocket-while-big-pharma-makes-record-profits>.

The press release from AHIP describes the absurd prices that orphan drugs are at. It describes drugs being increased 26-fold over the past 2 decades and being extremely more expensive than mainstream drugs. This is helpful because it helps show the consequences of the ODA.

Hillinger, Suzanne, director. *The Six Million Dollar Claim. Episode 11: 'The Six Million Dollar Claim,'* 2019,
<https://www.nytimes.com/2019/08/23/the-weekly/life-changing-drugs-almost-nobody-can-afford.html>. Accessed 5 Apr. 2022.

This online video called *The Six Million Dollar Claim* talks about orphan drugs and explains how orphan drugmakers are placing absurd prices on orphan drugs. This is helpful as it shows the consequences of the ODA and how the producers are abusing the incentives provided.

Kilpatrick, James. "Orphan Drug' Law Makes Sense." *The Deseret News*, 13 Nov. 1984, pp. 9–9.

This newspaper gives a point of view on the ODA from the news network in 1984 which is after it passed and talked about whether the act was a good decision or not. This is an useful source since it gave an opinion from the times that was new to the ODA rather than sources now which have the capability of seeing the consequences/successes that it had.

Kim, Sumin. Issue 6 ed., Volume 11, NORTH WESTERN JOURNAL OF TECHNOLOGY AND INTELLECTUAL PROPERTY, 2013, *The Orphan Drug Act: How the FDA Unlawfully Usurped Market Exclusivity*.

Issue 6 of Volume 6 talks about market exclusivity in the Orphan Drug Act. It describes events such as Henry Waxman trying to not give pharmaceutical companies uncontrolled market exclusivity. This was helpful because it showed Henry Waxman's attempt and how he was right since in the future the pharmaceutical companies took advantage of that incentive.

"Number of Orphan Drugs Approved from 1983-2018." *Orphan Drugs in the United States*, IQVIA, 2018,
<https://www.iqvia.com/insights/the-iqvia-institute/reports/orphan-drugs-in-the-united-states-growth-trends-in-rare-disease-treatments>. Accessed 11 Nov. 2021.

This graph helps show in a visual representation how the Orphan Drug Act had greatly impacted the number of orphan drugs created. From this graph you can see that from 2 orphan

drugs approved in 1983 which is before the orphan drug passed compared to 80 which was in 2017, there was a major increase in orphan drug production.

“Orphan' Drugs: A Struggle for Relief.” *The New York Times*, 31 Oct. 1982.

The article from ‘The New York Times’ helped me know the opinions from both sides of the argument, the pharmaceutical companies and doctors/patients that have been impacted. It was helpful as it partially morphed my perspective on the ODA. In the text it talks about a lady that suffers from a rare disorder and her doctor was able to create the treatment for it. However, due to the FDA, she wasn’t able to take it without the drug getting approval.

“Orphans in the Market: The History of Orphan Drug Policy .” *Social History of Medicine*, 3rd ed., vol. 32, Oxford University, 2017, pp. 609–630.

The section from the journal describes what had happened inside of the Congress hearing and the arguments each side had made. It was helpful as it contains many personal accounts from different parties in debate such as the FDA and pharmaceutical company representatives. Additionally, this helped me shape my opinions on the ODA. It also included many quotes from the debates that occurred in Congress.

Sarah Jane Tribble, Sydney Lupkin. “Drugmakers Manipulate Orphan Drug Rules to Create Prized Monopolies.” *Kaiser Health News*, KHN, 30 Nov. 2017, <https://khn.org/news/drugmakers-manipulate-orphan-drug-rules-to-create-prized-monopolies/>.

This article talks about how drugmakers are abusing the ODA and making patients pay absurd prices for the drugs. This is shown through statistics such as graphs and infographics. These statistics show the absurd prices that orphan drugmakers are raising prices to and how the majority of the top 10 mainstream market drugs are approved as an orphan drug.

Trufelman, Avery. “Orphan Drugs.” *99% Invisible*, episode 329, 13 Nov. 2018.

This podcast helped me understand the problems the orphan disease patients had to go through when there were no orphan drugs being created. It was helpful because one of the main speakers for this episode was Abbey Phillips who is the head of the National Organization for Rare Disorders which played an important role in the ODA.

