

2019

# An Analysis of the Morality of the Orphan Drug Act and its Effects on the Community using Finnis' Ethical Values as Defined in Natural Law & Natural Rights

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<b>I.</b>	<b>Background.....</b>	<b>4</b>
<b>II.</b>	<b>The Origin of “Orphan” Drugs: The Kefauver-Harris Amendments to the Food, Drug, and Cosmetic Act.....</b>	<b>6</b>
<b>III.</b>	<b>The Passage of The Orphan Drug Act: Senator Kennedy’s Efforts to Encourage the Research and Development of Orphan Drugs.....</b>	<b>9</b>
<b>IV.</b>	<b>The Orphan Drug Act is Born.....</b>	<b>12</b>
<b>V.</b>	<b>The Beneficial Impact of the Enactment of Orphan Drug Act.....</b>	<b>15</b>
<b>VI.</b>	<b>Criticisms of the Orphan Drug Act.....</b>	<b>16</b>
	a. <i>A Large Number of Orphan Drugs are Primarily Limited to a Narrow Group of Therapeutic Families.....</i>	<i>16</i>
	b. <i>Pharmaceutical Companies are “only in it for the Money” .....</i>	<i>17</i>
	c. <i>Availability does not Equal Accessibility – The Unexplainably High Prices of Orphan Drugs.....</i>	<i>18</i>
<b>VII.</b>	<b>The Orphan Drug Act, while Pure in Purpose, is Flawed.....</b>	<b>20</b>
<b>VIII.</b>	<b>Introduction to Finnis’ Ethical Theory.....</b>	<b>21</b>
<b>IX.</b>	<b>Seven Goods.....</b>	<b>22</b>
	a. <i>Life.....</i>	<i>22</i>
	b. <i>Knowledge and Sociability.....</i>	<i>24</i>
	c. <i>Practical Reasonableness.....</i>	<i>25</i>
<b>X.</b>	<b>Nine Principles of Practical Reasonableness.....</b>	<b>26</b>
	a. <i>First Principle.....</i>	<i>26</i>
	b. <i>Second Principle.....</i>	<i>27</i>

c.	<i>Third Principle</i> .....	28
d.	<i>Fourth and Fifth Principles</i> .....	28
e.	<i>Sixth Principle</i> .....	29
f.	<i>Seventh Principle</i> .....	30
g.	<i>Eighth Principle</i> .....	31
h.	<i>Ninth Principle</i> .....	32
<b>XI.</b>	<b>Three Elements of Justice</b> .....	32
<b>XII.</b>	<b>Conclusion</b> .....	33

## I. Background

The Orphan Drug Act (ODA) was enacted in 1983 in order to incentivize pharmaceutical companies to engage in more research and development in order to create therapeutic molecules to treat rare diseases. Under the Orphan Drug Act, the Food and Drug Administration (FDA) defines the term “rare disease or condition” as any disease or condition that:

- (A) affects less than 200,000 persons in the United States, or
- (B) affects more than 200,000 in the United States and for which there is no reasonable expectation that the cost of developing and making available in the United States a drug for such disease or condition will be recovered from sales in the United States of such drug.<sup>1</sup>

If approved by the FDA and developed into a treatment for one of these rare diseases, these therapeutic or curative molecules are designated as “orphan drugs.” Typically, a drug will receive the designation of “an orphan drug” when the drug is “used to treat a disease whose prevalence is so low that, in absence of incentives, commercializing the drug would unlikely generate sufficient revenues to absorb the costs related to its development and marketing.”<sup>2</sup>

In a report provided by IQVIA Institute for Human Data Science that was published in October 2018, there are approximately 7,000 known rare diseases and/or conditions and only 500 orphan drug therapies that have been approved in the United States.<sup>3</sup> These diseases affect anywhere from 25 million to 30 million people in the United States, with more than half of that number being children.<sup>4</sup> When compared with the total U.S. population, this equates to right under 10% of Americans belonging to the rare disease community.<sup>5</sup> That means that almost 1 in

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<sup>1</sup> 21 U.S. Code § 360bb(a)(2).

<sup>2</sup> Andre Côté, & Bernard Keating, *What is Wrong with Orphan Drug Policies?*, 15 VALUE IN HEALTH, 1185, 1185 (Dec. 2012).

<sup>3</sup> *Orphan Drugs in the United States: Growth Trends in Rare Disease Treatments*, IQVIA INSTITUTE FOR HUMAN DATA SCIENCE, 1, 2 (Oct. 2018).

<sup>4</sup> *Orphan Drugs in the United States: Growth Trends in Rare Disease Treatments*, supra note 3.

<sup>5</sup> *Rare Diseases: Facts and Statistics*, GLOBAL GENES; ALLIES IN RARE DISEASE (last visited Jan. 31, 2019), <https://globalgenes.org/rare-diseases-facts-statistics/>.

every 10 Americans are afflicted by one or more of these rare diseases.<sup>6</sup> Despite this, the currently available treatments are only available for 5% of them.<sup>7</sup> Rare diseases are often life-threatening or at the least life-limiting, and most can only be treated or managed, but remain chronic conditions.<sup>8</sup> Recently, however, there has been an increase in curative treatments which provides hope for a better quality of life to those afflicted by these tragic rare diseases.<sup>9</sup>

Since its enactment in 1983, the Orphan Drug Act today has undergone several amendments, and currently includes the following incentives:

(1) seven years of market exclusivity for any unpatented drugs designated as treatments for rare conditions; (2) tax credits for certain research and development costs; (3) elimination or reduction of procedural fees; (4) fast-tracking of FDA review and approval of applications pertaining to orphan drugs; and (5) federal and state grants for drug development (e.g., research grants from the National Institutes of Health).<sup>10</sup>

In theory, the Orphan Drug Act has the potential to save the lives of millions of suffering people whose diseases were once completely overlooked by the pharmaceutical industry by making treatment of rare diseases a lucrative opportunity. In application, however, the way the law is written severely limits the accessibility of orphan drugs to those who need it. The primary problem revolves around the monopoly market the Orphan Drug Act creates combined with the high prices placed on such therapies. Through the Orphan Drug Act, drug manufacturers have adopted a three-step strategy to profit off the vulnerabilities of desperate people affected by tragic diseases. First, pharmaceutical companies apply for the orphan drug designation in order to obtain the substantial economic benefits while they develop the drug, work on getting it

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<sup>6</sup> *Rare Diseases: Facts and Statistics*, *supra*.

<sup>7</sup> *Orphan Drugs in the United States: Growth Trends in Rare Disease Treatments*, *supra* note 3, at 1.

<sup>8</sup> *Orphan Drugs in the United States: Growth Trends in Rare Disease Treatments*, *supra* note 3, at 1.

<sup>9</sup> *Orphan Drugs in the United States: Growth Trends in Rare Disease Treatments*, *supra* note 3, at 1.

<sup>10</sup> Taeho Greg Rhee, *Policymaking for Orphan Drugs and Its Challenges*, 17 AMERICAN MEDICAL ASSOCIATION JOURNAL OF ETHICS 776, 776 (Aug. 2015).

approved, execute marketing strategies, and subsequently sell the drug at preposterously high prices due to the low target population.<sup>11</sup> Second, once the FDA has approved the orphan designated drug, pharmaceutical companies send representatives to doctors to convince them to use it in their practice and prescribe it to their patients.<sup>12</sup> Third, they continue to profit by obtaining new treatment indications to expand sales but keeping the initially astronomical price.<sup>13</sup>

This paper will examine the history, development, enactment, applications, and criticisms of the Orphan Drug Act. Lastly, this paper will provide an in-depth ethics analysis using Finnis' theory of natural laws and natural rights to determine the morality of the act and how it can be improved to better achieve its purpose.

## **II. The Origin of “Orphan” Drugs: The Kefauver-Harris Amendments to the Food, Drug, and Cosmetic Act**

Although passed in 1983, the groundwork for the Orphan Drug Act began in the 1960s when the primary issues that fueled the act became increasingly apparent. In 1962, when Senator Estes Kefauver proposed a bill that sought to amend the Food, Drug, and Cosmetic Act.<sup>14</sup> At the core of his proposal was a desire to increase the government's control over the pharmaceutical industry while also reducing the price of prescription drugs.<sup>15</sup> After significant revisions, Congress passed the bill in October of 1962, which indeed mitigated the serious issue of an “increase price competition” among the pharmaceutical market.<sup>16</sup>

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<sup>11</sup> Côté, *supra* note 2, at 1189.

<sup>12</sup> Côté, *supra* note 2, at 1189.

<sup>13</sup> Côté, *supra* note 2, at 1189.

<sup>14</sup> Koichi Mikami, *Orphans in the Market: The History of the Orphan Drug Policy*, 0 Social History of Medicine 1, 3 (Nov. 27, 2017).

<sup>15</sup> Koichi Mikami, *supra* note 14.

<sup>16</sup> Koichi Mikami, *supra* note 14.

However, some of the amendments demanded both a rigorous and costly approach to clinical trials and authorized the FDA to be “the responsible government agency to oversee the process of drug development – the ‘gatekeeper’ of the US pharmaceutical market.”<sup>17</sup> Prior to these amendments, properly labeled drugs that were for investigational use were permitted to be freely distributed.<sup>18</sup> By stark contrast, the new amendments drastically changed this rule by issuing many new regulations. For example, the amended Food, Drug, and Cosmetic Act required drug sponsors to submit “investigational new drug” (IND) notices before starting any clinical trials and would also be required to provide the FDA with lists of all drugs already undergoing clinical trials.<sup>19</sup> After the latter occurred, those same drug sponsors had the option of either submitting an IND notice for each drug on the lists or just completely withdrawing the drug and notifying the FDA of the reasons for doing so.<sup>20</sup> This resulted in a quarter of the drugs that were initially listed being withdrawn.<sup>21</sup> With these new restrictions, the pharmaceutical industry needed to refocus their research and development towards drugs that were worth the extensive effort it would take to get them on the market and narrow the drugs they were already working on for efficiency and productivity.

In June of 1963, the Commission on Drug and Safety<sup>22</sup> held a conference addressing the issues and concerns raised after the passage of the bill.<sup>23</sup> Beyond the concerns of the now frustrated biological and chemical manufacturers, there was also the issue of a clear preference for commercially valuable drugs that was severely agitated by the 1962 amendments.

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<sup>17</sup> Koichi Mikami, *supra* note 14, at 16.

<sup>18</sup> Koichi Mikami, *supra* note 14, at 4.

<sup>19</sup> Koichi Mikami, *supra* note 14, at 4.

<sup>20</sup> Koichi Mikami, *supra* note 14, at 4.

<sup>21</sup> Koichi Mikami, *supra* note 14, at 4.

<sup>22</sup> The commission was described as “a body of experts drawn from industry and academia on pharmaceuticals, set up in 1962 by the Pharmaceutical Manufacturers Association (PMA).” Koichi Mikami, *supra* note 14, at 4.

<sup>23</sup> Koichi Mikami, *supra* note 14, at 4.



During the conference, a representative of the American Society for Clinical Investigation, Grant E. Liddle, surmised that “in most cases, the decision to withdraw was due to their ‘low commercial priority.’”<sup>24</sup> In this same conference, the chairman of the Committee on Drug Dosage, Harry C. Shirkey, used the word “orphan” in relation to these low commercial priority drugs, which was the first time on record that the term was used.<sup>25</sup> Shirkey used the phrase “pharmaceutical orphans” to describe and express a deep concern for increasing numbers of drugs approved for adults, but not for children and infants, which ultimately left children with fewer therapeutic options.<sup>26</sup> In an editorial comment written by Shirkey five years later, he deduced like Liddle that these again was solely due to the small sales potential of “pharmaceutical orphans,” especially when compared to the general cost of obtaining FDA approval and the additional cost of receiving FDA approval for pediatric use of the drug.<sup>27</sup> Later, the idea of “orphans” was again articulated by George P. Provost<sup>28</sup> in order to describe substances that were still kept in hospital pharmacies from before 1962, but that was not approved for clinical use under the new regulations.<sup>29</sup> Provost also suggested that the reasons producers stopped seeking approval of such “orphan drugs” was due to insufficient profitability<sup>30</sup>:

Shirkey and Provost both observed that in the past pharmaceutical companies had supplied some drugs at a financial loss as a service to the public—so-called “public service drugs” or “service drugs”. Assuming that it was the increased cost of obtaining marketing approval that jeopardized this practice, they argued that doctors and pharmacists should do more to help companies secure approval by collecting relevant information about unprofitable drugs.<sup>31</sup>

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<sup>24</sup> Koichi Mikami, *supra* note 14, at 4.

<sup>25</sup> Koichi Mikami, *supra* note 14, at 4.

<sup>26</sup> Koichi Mikami, *supra* note 14, at 4.

<sup>27</sup> Koichi Mikami, *supra* note 14, at 4.

<sup>28</sup> Provost was an editor of the *American Journal of Hospital Pharmacy*. Koichi Mikami, *supra* note 14, at 4.

<sup>29</sup> Koichi Mikami, *supra* note 14, at 4.

<sup>30</sup> Koichi Mikami, *supra* note 14, at 4.

<sup>31</sup> Koichi Mikami, *supra* note 14, at 4.



While the concept of “orphan drugs” was often expressed in the 1960s, it was still not heavily debated at that time. Instead, the debates focused on other amendments to the Food, Drug, and Cosmetic Act. For example, during the 1970s, more emphasis was placed on concerns expressed by the pharmaceutical industry: that the new regulations caused “drug lag,” which focused around how much longer it was taking for effective drugs to reach the market.<sup>32</sup>

However, that almost changed in 1975 when the FDA finally began to recognize drugs of limited commercial value. Specifically, the associate director for new drug evaluation at the FDA Bureau of Drugs, Marion J. Finkel, gathered a committee that “contemplated possible incentives to encourage pharmaceutical companies to produce such drugs, but concluded that the reasons they were neglected were too diverse to permit meaningful recommendations.”<sup>33</sup> Thus, no action was taken despite the knowledge and recognition that something could be done.

### **III. The Passage of The Orphan Drug Act: Senator Kennedy’s Efforts to Encourage the Research and Development of Orphan Drugs**

In 1977, the members of Congress were repeatedly and increasingly notified of pharmaceutical companies’ neglect to develop drugs that only treated a small percent of the population due to their limited commercial value.<sup>34</sup> For instance, the Congressional Commission for the Control of Huntington’s Disease and Its Consequences<sup>35</sup> elaborately discussed their concerns about this same rising trend: pharmaceutical companies were not interested in developing therapeutic and curative drugs for diseases that only affected a relatively small

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<sup>32</sup> Koichi Mikami, *supra* note 14, at 5.

<sup>33</sup> Koichi Mikami, *supra* note 14, at 5.

<sup>34</sup> Koichi Mikami, *supra* note 14, at 7.

<sup>35</sup> The Congressional Commission for the Control of Huntington’s Disease and Its Consequences was created by the Public Health Service Act of 1975 and chaired by Marjorie Guthrie. Marjorie Guthrie was a founder and the president of the Committee to Combat Huntington’s Disease, a patient support group. Koichi Mikami, *supra* note 14, at 7.

amount of the U.S. population.<sup>36</sup> After reporting their agitations to Congress, Senator Edward M. Kennedy promoted and included the research and development of what was then called “drugs of limited commercial value” in his proposal to create a National Center for Clinical Pharmacology in his Drug Regulation Reform bill.<sup>37</sup> Consequently, the FDA began to study the orphan drug problem in 1978 by convening an Interagency Task Force<sup>38</sup> in which they focused more on a solution to the problem instead of wasting time establishing definitive facts and figures.<sup>39</sup>

The Interagency Task Force briefly acknowledged the practices of pharmaceutical manufacturers before 1962 in which they would supply service drugs even at the loss of profits and used this trend as evidence of the industry’s capacity to synthesize such drugs as well as their willingness to.<sup>40</sup> Yet, that trend was now almost extinct, with the only logical factor to the shift being the increased cost of obtaining FDA approval.<sup>41</sup> Thus, in order to remedy the industry’s deterrence to manufacture service drugs, the report recommended a financial support program to aid in clinical trials until the drug gained market approval.<sup>42</sup> The task force also suggested that “[a] new FDA advisory board should also be set up ‘to encourage voluntary industry action as a matter of public interest and ... accord appropriate recognition to firms which participate on the basis of humanitarian concern.’”<sup>43</sup> These ideas are reflective of the current incentives in the Orphan Drug Act and thus laid the groundwork for it.

Subsequently, the FDA’s adopted a new approach aimed to offset both the cost of obtaining market approval and encourage pharmaceutical companies to voluntarily commit to

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<sup>36</sup> Koichi Mikami, *supra* note 14, at 7.

<sup>37</sup> Koichi Mikami, *supra* note 14, at 7.

<sup>38</sup> The Interagency Task Force was chaired by the same aforementioned Marion J. Finkel. Koichi Mikami, *supra* note 14, at 7.

<sup>39</sup> Koichi Mikami, *supra* note 14, at 7.

<sup>40</sup> Koichi Mikami, *supra* note 14, at 8.

<sup>41</sup> Koichi Mikami, *supra* note 14, at 8.

<sup>42</sup> Koichi Mikami, *supra* note 14, at 8.

<sup>43</sup> Koichi Mikami, *supra* note 14, at 8.

producing more service drugs by providing financial and organizational support, just as the Task Force proposed.<sup>44</sup> However, despite the FDA's findings and recognition regarding the obvious orphan drug issue, Kennedy's Drug Regulation Reform bill still failed in both 1978 and 1979.<sup>45</sup> Nevertheless, this was not the death of the reformation efforts. In fact, the matter continued to stay before Congress due to the increased amount of interventions from patients and practitioners alike.<sup>46</sup>

Finally, the attempted development of a treatment for myoclonus by researcher Melvin Van Woert was the final push the legislators needed to set the passage of the act in motion.<sup>47</sup>

Myoclonus is the sudden and involuntary jerking of groups of muscles, which can indicate serious underlying disorders, such as brain tumors, kidney failure, chemical or drug poisoning, head or spinal cord injury, or stroke.<sup>48</sup> More often, myoclonus is caused by a wide array of neurological disorders: multiple sclerosis, epilepsy, Parkinson's disease, Alzheimer's, and Creutzfeldt-Jakob disease.<sup>49</sup> Thus, there was a severe need in the market to take Van Woert's research and develop it to be commercially manufactured and distributed. When Van Woert approached several organizations for help, the FDA, U.S. National Institutes of Health (NIH), and the PMA, they all turned him away and were unable to offer any solutions.<sup>50</sup> This instigated myoclonus patient, Sharon Dobkin, to contact a local representative, Elizabeth Holtzman, convincing Holtzman that immediate legislative change was of the utmost importance.<sup>51</sup> Though this seems like a small anecdote in the large history of the Orphan Drug Act, it was Holtzman's

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<sup>44</sup> Koichi Mikami, *supra* note 14, at 8.

<sup>45</sup> Koichi Mikami, *supra* note 14, at 8.

<sup>46</sup> Koichi Mikami, *supra* note 14, at 8.

<sup>47</sup> Koichi Mikami, *supra* note 14, at 8.

<sup>48</sup> *Myoclonus Fact Sheet*, NATIONAL INSTITUTE OF NEUROLOGICAL DISORDERS AND STROKE, (last visited Jan. 31, 2019), <https://www.ninds.nih.gov/Disorders/Patient-Caregiver-Education/Fact-Sheets/Myoclonus-Fact-Sheet>.

<sup>49</sup> *Myoclonus Fact Sheet*, *supra* note 48.

<sup>50</sup> Koichi Mikami, *supra* note 14, at 8.

<sup>51</sup> Koichi Mikami, *supra* note 14, at 8.

bill, supported by Representative Waxman, that laid the foundation for the actual language and enactment of the Orphan Drug Act itself.

#### **IV. The Orphan Drug Act is Born**

It was in early 1980 when Elizabeth Holtzman introduced her bill that was based on recommendations from The Interagency Task Force.<sup>52</sup> The bill itself outlined measures in order to “assist the development of drugs for diseases and conditions of low incidence” by providing both administrative and economic assistance to pharmaceutical companies so that they could research and develop drugs like the one Van Woert sought to commercialize.<sup>53</sup> The bill was supported and pushed forward by Representative Waxman, who was well-known for his activism of health care reform.<sup>54</sup> Waxman had recently been appointed the chair of the House Subcommittee on Health and the Environment and, after learning of Holtzman’s bill, arranged for it to be presented to the committee in June of 1980.<sup>55</sup> Ultimately, the Holtzman bill failed when presented to the House of Representatives, but that did not stop Waxman nor should the rejection of the bill be considered a failure.<sup>56</sup> The Holtzman Bill and Waxman’s persistent advocacy raised significant awareness for the dire need of a legislative effort to address the issue

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<sup>52</sup> Koichi Mikami, *supra* note 14, at 8.

<sup>53</sup> Koichi Mikami, *supra* note 14, at 8.

<sup>54</sup> Koichi Mikami, *supra* note 14, at 8-9.

<sup>55</sup> Koichi Mikami, *supra* note 14, at 9.

<sup>56</sup> Koichi Mikami, *supra* note 14, at 9 (“The Los Angeles Times published a small article about it. This caught the eye of Maurice Klugman, who was then suffering from a rare form of cancer, and he and his brother, actor Jack Klugman, produced an episode in the television drama series *Quincy M.D.* based on the story of the family that had asked Waxman for help. The episode was effective in increasing the visibility of patients and families and building public support for legislative effort to address the problem of drugs of limited commercial value, and is remembered by many as the moment when ‘the ball began to roll’.”).

surrounding drugs of limited commercial value. Only three years later, Waxman took the matter into his own hands and submitted the bill that became the Orphan Drug Act of 1983.<sup>57</sup>

The stark difference between the bill Waxman proposed and Holtzman's proposal was the way it framed the issue of the orphan drugs, which is likely why the House was moved enough to finally resolve the orphan drug issue. Holtzman merely pushed for the FDA's support by trying to provide funding to drugs that both already existed and showed evidence of safety and effectiveness.<sup>58</sup> Holtzman's bill embodied the view of Dobkin, the myoclonic patient, who argued in front of Congress about her personal experiences with the treatment an orphan drug can provide: "the worst thing that can happen to a person is to hold a treatment in his hand, see the miracles it can bring, and then have it pulled away."<sup>59</sup> This issue is clearly an important one, but the solution Holtzman and Dobkin proposed was too narrow and very limited, which is likely the reason it failed when presented to the House of Representatives. Without a doubt, Holtzman's bill would help many lives, but what about the lives of those who never even had the blessing of holding that miracle in their hand, to begin with? By ignoring those people, Holtzman failed to provide a solution that would extend to all members of the rare disease community, leaving numerous people afflicted with diseases that had no treatment options with absolutely no hope of a better future.

When the Waxman bill was presented in front of Congress, Waxman invited the Vice President of the Tourette Syndrome Association, Abby S. Meyers, to speak Congress about her personal experience with rare diseases and conditions.<sup>60</sup> Meyers brought to light a new view of the orphan drug issue, one that encompassed those who hadn't even had that treatment, that

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<sup>57</sup> Koichi Mikami, *supra* note 14, at 9.

<sup>58</sup> Koichi Mikami, *supra* note 14, at 9.

<sup>59</sup> Koichi Mikami, *supra* note 14, at 9. (quoting House Committee, *Drug Regulation Reform*, 96th Congress, 31.)

<sup>60</sup> Koichi Mikami, *supra* note 14, at 9.



miracle, in their hands.<sup>61</sup> In a powerful testimony she gave, Meyers stated exactly how the bill, if enacted, would provide that same help, and more importantly, hope:

Millions of Americans who suffer from rare diseases live without hope. We believe that there are not enough dollars among patients who suffer from sickle cell anemia, Cooley's anemia, Huntington's disease, cystic fibrosis, Wilson's disease, Tay Sachs disease, dystonia, and many, many more, to make the manufacture of a therapeutic drug profitable.<sup>62</sup>

Meyers spoke to give a voice to those who were suffering everywhere, and it directly impacted the scope of the orphan drug problem. The goal was no longer just to streamline the development and production of present treatments for rare conditions, but instead to also promote the development of completely new drugs for those inflicted by rare diseases as well. Without the problem and solution being widened by people like Waxman and Meyers, the Orphan Drug Act would have never developed into the influential act it has become today, providing treatments and cures for those suffering from rare conditions on a global scale.

While Meyers and Waxman were on the front lines of this legislative battle, those inflicted with the disease were still very much involved in the process. In fact, they served as a major inspiration to Meyers speech in front of Congress, as Meyers spent time with a plethora of inflicted patients to gather their thoughts on this major flaw in the pharmaceutical injury. It was these patients that really gave the issue a first-person viewpoint to help expand and yet articulate the issue: “these patients indicated that the scope of the orphan drug problem needed to be expanded, to include not just the problem of securing access to existing but unapproved drugs, to the larger question of how to promote the development of new drugs for rare conditions.”<sup>63</sup>

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<sup>61</sup> Koichi Mikami, *supra* note 14, at 9-10.

<sup>62</sup> Koichi Mikami, *supra* note 14, at 9-10.

<sup>63</sup> Koichi Mikami, *supra* note 14, at 10.

This “future-oriented” view of the orphan drug problem was at the center of Waxman’s proposal during the second hearing of Holtzman’s bill, but when he brought his own bill forward, he executed his sympathies and goals by including provisions that would streamline the creation of orphan drugs and incentivize pharmaceutical companies to pursue the development of these drugs. For instance, he posited that a single, but well-controlled clinical trial when combined with post-marketing surveillance should be enough for FDA approval.<sup>64</sup> As for incentives, he propositioned that pharmaceutical companies should receive tax credits proportionate to clinical testing costs of orphan drugs and that these companies should be granted exclusive seven-year marketing rights for the drug. Lastly, Waxman framed the entire orphan drug issue as a case of market failure.<sup>65</sup> In doing so, several organizations opposed to the bill conceded, which gave Waxman the support of both consumers and potential orphan drug suppliers.<sup>66</sup> Once this support was secured, the bill passed with ease, as the House of Representative approved Waxman’s bill with only minor amendments.<sup>67</sup> In January of 1983, everything became official when U.S. President Ronald Reagan signed the Orphan Drug Act into law.<sup>68</sup>

## **V. The Beneficial Impact of the Enactment of Orphan Drug Act**

Without question, the Orphan drug act has achieved its primary purpose to a certain degree, giving hope to many people afflicted with rare diseases and has certainly increased the pharmaceutical industry’s interest in developing new therapies. Before the enactment of the

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<sup>64</sup> Koichi Mikami, *supra* note 14, at 10.

<sup>65</sup> Koichi Mikami, *supra* note 14, at 11.

<sup>66</sup> Koichi Mikami, *supra* note 14, at 13.

<sup>67</sup> Koichi Mikami, *supra* note 14, at 13.

<sup>68</sup> Koichi Mikami, *supra* note 14, at 13.



Orphan Drug Act, only 10 drugs were available to treat rare diseases.<sup>69</sup> By contrast, between January 1983 and May 2010, 353 orphan drugs were approved in the United States by the FDA.<sup>70</sup> Since then, a report titled *Orphan Drugs in the United States* released in October 2018 stated that currently there are over 500 orphan drugs on the market.<sup>71</sup> Proponents of the act severely stress this, among other things, when evaluating the true benefits and influences of the Orphan Drug Act. Essentially, they argue that the policies enacted by the Orphan Drug Act have had enormous contributions, focusing especially on "the extension of an improvement in quality of life, the acquisition of new knowledge about other types of illnesses, the considerable boon to the industry, especially in biotechnology, and the accelerated processing of drug approval applications."<sup>72</sup>

Unfortunately, these numbers are misleading for several reasons. First, the drugs fall into a narrow range a few therapeutic families, leaving many rare diseases still untreated.<sup>73</sup> Second, the few therapeutic families these orphan drugs are limited to are "those that offer a significant turnover."<sup>74</sup> For example, studies have shown that "orphan drugs used to treat rare cancer are the most profitable."<sup>75</sup> Third, while hundreds of new molecules are available, they are not accessible to patients due to unnecessarily high prices.<sup>76</sup> These three points are at the center of the debates and agitations of the Orphan Drug Act, which will be analyzed further in the next section.

## VI. Criticisms of the Orphan Drug Act

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<sup>69</sup> Taeho Greg Rhee, *supra* note 10.

<sup>70</sup> Côté, *supra* note 2, at 1186.

<sup>71</sup> *Orphan Drugs in the United States: Growth Trends in Rare Disease Treatments*, *supra* note 3.

<sup>72</sup> Côté, *supra* note 2, at 1186.

<sup>73</sup> Côté, *supra* note 2, at 1186.

<sup>74</sup> Côté, *supra* note 2, at 1186.

<sup>75</sup> Taeho Greg Rhee, *supra* note 10, at 777; *see* Côté, *supra* note 2, at 1186.

<sup>76</sup> Côté, *supra* note 2, at 1186.

a. *A Large Number of Orphan Drugs are Primarily Limited to a Narrow Group of Therapeutic Families*

On its face, the large number of orphan drugs that have been approved gives the impression that the act has allowed for new molecules to treat a wide variety of illnesses. However, only “five therapeutic classes account for 75% of the market for orphan drugs,” which will be discussed in descending order.<sup>77</sup> Out of the 353 orphan drugs that were FDA approved by 2010, 95 were directed at the oncology class. This was followed by 54 orphan drugs solely for metabolic disorders, 41 for hematology, 41 for infectious diseases, and 30 for neurological diseases. The remaining 92 were distributed among 11 other therapeutic classes: “psychiatric, musculoskeletal, gastrointestinal, dermatologic, respiratory, ophthalmologic, hepatic/biliary, immunology, cardiovascular, [ ] genitourinary disorders, and . . . treatment of intoxications/envenomations.”<sup>78</sup> Even as recent as 2015, data has shown that cancer drugs were still the predominant therapeutic class targeted by orphan drugs, making up 95 out of the 400 plus orphan drugs at that time.<sup>79</sup>

b. *Pharmaceutical Companies are “only in it for the Money”*

Many have theorized that distribution phenomenon illustrated above is evidence that pharmaceutical companies are using the Orphan Drug Act to merely turn a profit.<sup>80</sup> As evidence of this theory, many authors, analysts, and scientists alike brought attention to numerous orphan drugs on the market that “had a financial return that significantly outmatched the investments

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<sup>77</sup> Côté, *supra* note 2, at 1186.

<sup>78</sup> Côté, *supra* note 2, at 1186.

<sup>79</sup> Taeho Greg Rhee, *supra* note 10, at 777.

<sup>80</sup> Côté, *supra* note 2, at 1186.

involved.”<sup>81</sup> In two separate publications, written by Seachrist<sup>82</sup> and Casali<sup>83</sup>, the same argument is advanced: that this profitability connected to orphan drugs in the oncology realm, at least in part, can be explained by the frequent off-label use of these drugs.<sup>84</sup> Going further, in another report by Thorton titled *Opportunities in Orphan Drugs – Strategies for Developing Maximum Returns from Niche Indicators*, Thorton contended that “manufacturers have an incentive to abandon the traditional business model based on the mass sale of drugs intended for general care treatment and to turn to targeted drugs with high commercial potential.”<sup>85</sup>

These theories and observations are backed by frightening numbers from compilations that analyzed the whole population of orphan drugs and the insurmountable return of investment they provided for their manufacturers. In 2008, 43 trademarked treatments for rare diseases generated a total of one billion annual sales globally.<sup>86</sup> Another 33 trademarked drugs with orphan designations achieved annual sales that ranged from \$100 million to \$199 million:

Of these, 19 were approved for orphan applications, 7 had global annual sales of \$100 million to \$199 million, 9 had global annual sales of \$200 million to \$299 million, 5 had global annual sales of \$300 million to \$399 million, 3 had global annual sales of \$400 million to \$499 million, 5 had global annual sales of \$500 million to 599 million, and 3 had global annual sales of \$600 million to \$999 million in 2008.<sup>87</sup>

In conclusion, pharmaceutical companies are arguably are abusing the incentives contained in the Orphan Drug Act in pursuit of highly lucrative opportunities.

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<sup>81</sup> Côté, *supra* note 2, at 1185.

<sup>82</sup> Seachrist L., *Senators seek reform of Orphan Drug Act*, 85 JOURNAL OF NATIONAL CANCER INSTITUTE 1984 (Dec. 15, 1993).

<sup>83</sup> Casali, *The off-label use of drugs in oncology: a position paper by the European Society for Medical Oncology*, 18 ANNALS OF ONCOLOGY 1923 (Dec. 18, 2007).

<sup>84</sup> Côté, *supra* note 2, at 1186.

<sup>85</sup> Côté, *supra* note 2, at 1186.

<sup>86</sup> Côté, *supra* note 2, at 1186.

<sup>87</sup> Côté, *supra* note 2, at 1186.

c. *Availability does not Equal Accessibility – The Unexplainably High Prices of Orphan Drugs*

The pharmaceutical industry has contributed few treatment options for many therapeutic classes that present low commercial value, leaving many people suffering from rare diseases with little to no hope to manage the symptoms of their respective conditions. Despite this, an argument can still be made to the positive, that although the intentions of pharmaceutical companies are fueled by money, at least people in the lucrative therapeutic classes are able to receive treatment. As the saying goes, “something is better than nothing.” The availability of orphan drugs in the targeted therapeutic classes, in theory, should benefit the members of those classes, providing hope for a better quality of life. However, availability does not equal accessibility. This leads to the third major criticism of the Orphan Drug Act: such patients can’t afford the orphan designated medications due to outrageously high prices.<sup>88</sup>

For instance, one Orphan drug, Cerezyme, was developed to treat Gaucher disease, which affects about 2,000 patients in the United States.<sup>89</sup> Yet, Cerezyme “costs as much as \$400,000 every year for an adult patient.”<sup>90</sup> Another example is Fabryzyme,<sup>91</sup> a therapeutic molecule developed to treat Fabry Disease, a lysosomal storage disorder that impairs cells’ ability to function.<sup>92</sup> Fabryzyme costs each patient approximately \$300,000 annually.<sup>93</sup> The annual revenue for other drugs has only gone up in recent years. The most expensive example to date is

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<sup>88</sup> Côté, *supra* note 2, at 1186.

<sup>89</sup> Taeho Greg Rhee, *supra* note 10, at 777 (citing Côté, *supra* note 2, at 1186).

<sup>90</sup> Taeho Greg Rhee, *supra* note 10, at 777 (citing Marlene Haffner, Josep Torrent-Farnell, & Paul Maher, *Does orphan drug legislation really answer the needs of patients?*, 371 THE LANCET 1971, 1971 (June 14, 2008) and Côté, *supra* note 2, at 1186).

<sup>91</sup> Côté, *supra* note 2, at 1186.

<sup>92</sup> John Cunha, *Fabry Disease Symptoms, Cases, Symptom Treatment, and Life Expectancy*, MEDICINET, (last visited Jan. 31, 2019), [https://www.medicinenet.com/fabrys\\_disease/article.htm#what\\_is\\_fabry\\_disease](https://www.medicinenet.com/fabrys_disease/article.htm#what_is_fabry_disease).

<sup>93</sup> Côté, *supra* note 2, at 1186.

a drug marketed as Soliris,<sup>94</sup> treatment for diseases like "paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS), and anti-acetylcholine receptor (AChR) antibody-positive generalized myasthenia gravis (gMG)."<sup>95</sup> Soliris costs more than \$500,000 dollars per patient annually.<sup>96</sup> Analysts predict in 2018 the cost of Soliris will be supplanted by either a "spinal muscular atrophy drug Spinraza, which costs \$750,000 or BioMarin's Brineura for Batten disease, with an annual cost of \$702,000."<sup>97</sup>

Examples like this are not uncommon. André Côté asserts that "[t]he prices charged for these new orphan drugs frequently exceed the usual pharmacoeconomic scales and the thresholds of social acceptability."<sup>98</sup> This major consequence of the Orphan Drug Act's incentives comes down to lack of patient bargaining power and greedy pharmaceutical companies. Drug manufacturers are "free to set their own introductory prices."<sup>99</sup> This practice often leads companies to choose a price that will maximize its profits, and as of now, this is completely within their legal rights.<sup>100</sup> Additionally, payers' are precluded from any involvement in negotiating the prices due to a common practice called "disease sub-setting," "salami-slicing," or "disease stratification."<sup>101</sup> This practice occurs when a company "split[s] up a disease into

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<sup>94</sup> Alison Kanski, *Orphan drugs to grow twice as fast as rest of the industry: report*, MM&M, (May 25, 2018), <https://www.mmm-online.com/home/channel/commercial/orphan-drugs-to-grow-twice-as-fast-as-rest-of-the-industry-report/>.

<sup>95</sup> SOLIRIS® (ECULIZUMAB), ALEXION (last visited Jan. 31, 2019), <http://alexion.com/products/Soliris>.

<sup>96</sup> Alison Kanski, *Orphan drugs to grow twice as fast as rest of the industry: report*, MM&M, (May 25, 2018), <https://www.mmm-online.com/home/channel/commercial/orphan-drugs-to-grow-twice-as-fast-as-rest-of-the-industry-report/>.

<sup>97</sup> Alison Kanski, *Orphan drugs to grow twice as fast as rest of the industry: report*, MM&M, (May 25, 2018), <https://www.mmm-online.com/home/channel/commercial/orphan-drugs-to-grow-twice-as-fast-as-rest-of-the-industry-report/>.

<sup>98</sup> Côté, *supra* note 2, at 1186.

<sup>99</sup> Taeho Greg Rhee, *supra* note 10, at 777. (quoting Zhou Wellman-Labadie, *The US Orphan Drug Act: Rare disease research stimulator or commercial opportunity?*, 95 HEALTH POLICY 216, 266 (May 2010)).

<sup>100</sup> Taeho Greg Rhee, *supra* note 10, at 777 (citing Thomas Hemphill, *Extraordinary Pricing of Orphan Drugs: Is it a Socially Responsible Strategy for the U.S. Pharmaceutical Industry?*, 94 JOURNAL OF BUSINESS ETHICS 225, 225-26 (June 2010)).

<sup>101</sup> Taeho Greg Rhee, *supra* note 10, at 777 (citing Steven Simoons, *Pricing and reimbursement of orphan drugs: the need for more transparency*, 6 ORPHANET JOURNAL OF RARE DISEASES 1, 2 (June 17, 2011)).



several sub-diseases that qualify as rare diseases.”<sup>102</sup> Lastly, insurance companies often deny reimbursements for these drugs due to concerns from all payer organizations. This leads patients to tragic disappointments, as their only option after being denied reimbursements is to either pay up or say goodbye to the hope of achieving a better quality of life.

## **VII. The Orphan Drug Act, while Pure in Purpose, is Flawed**

These practices described above are the result, whether direct or indirect, of the incentives built into the act in order to promote the development of drugs therapies to treat or cure rare diseases, which raises the question, are the incentives really doing enough? In an alternative view, some researchers even suggest that the issue instead is the nature of the pharmaceutical industry itself, posing the question of whether, even if given better incentives to focus on the neglected rare diseases, the pharmaceutical industry would continue to pursue only commercially lucrative areas.<sup>103</sup> Taeho Greg Rhee suggests that if the accessibility of orphan drugs is to be improved, relevant policies in the Orphan Drug Act should be reformed to promote fairness and equity.<sup>104</sup> According to Côté:

In the medical setting, fairness is defined with respect to the aim of providing citizens with equal access to health resources, which matches their actual health. Fairness requires a positive action by the state [or government] when the market does not provide a good match between investments and health [care] needs. Finally, fairness requires that the barriers to access should be morally justifiable.”<sup>105</sup>

Thus, ideas of fairness, equity, and equality are what inspires critics who advocate for amendments to the Orphan Drug Act. The Orphan Drug Act itself was created to assist a population of people in dire need of medical treatment, and yet, the data presented clearly shows

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<sup>102</sup> Taeho Greg Rhee, *supra* note 10, at 777 (citing Steven Simoens, *supra* note 100).

<sup>103</sup> Taeho Greg Rhee, *supra* note 10, at 777.

<sup>104</sup> Taeho Greg Rhee, *supra* note 10, at 778.

<sup>105</sup> Côté, *supra* note 2, at 1185.

that it has not fully achieved that purpose. There is still a large population of people facing detrimental diseases whose needs for help are still being ignored. Can this be considered fairness? In other words, are the results of the Orphan Drug Act, as Côté puts it, “morally justifiable?” The following section will analyze such by exploring the ethical nature of both the Orphan Drug Act and its effects in the medical community.

### **VIII. Introduction to Finnis’ Ethical Theory**

In *Natural Law & Natural Rights*,<sup>106</sup> Finnis delves into a modern theory of the purpose of law and life, reminiscent of the much older works of Aquinas and Aristotle. The idea of natural justice, according to Aristotle, is a rule of equity that corrects the deficiencies of the law through reason and its universality.<sup>107</sup> This is what makes Finnis’ modern notions of goods and justice so valuable, as through the philosophies behind natural law and natural justice can we come to a superior analysis of the law that transcends conventional ideas of legal justice.<sup>108</sup> At the heart of Finnis’ theory are three fundamental concepts that will be used to come to a better ethical understanding of the Orphan Drug Act: (1) the seven basic goods, (2) the nine principles of practical reason, and (3) the three elements of justice. It will be proven through these three concepts, that at its heart, the Orphan Drug Act serves a moral purpose to correct a harmful inequity in the medical community. However, its application has caused a large and non-justifiable disparity among different subsets of people. Thus, amendments are required in order to realign the effects of the Orphan Drug Act with its just intentions.

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<sup>106</sup> John Finnis, *Natural Law & Natural Rights* 1 (2d ed. 2011).

<sup>107</sup> Michael Ambrosio, *Natural Law as a Wisdom Tradition*, in *Proceedings of the Center for Catholic Studies: Knowledge and Wisdom* 31, 31 (Seton Hall Univ.) (1998).

<sup>108</sup> See Ambrosio, *supra* note 107.



## IX. Seven Goods

Finnis asserts seven fundamental, equally important, and self-evident goods necessary for humans to have a valuable and fulfilling existence: life, knowledge, play, aesthetic experience, sociability (friendship), practical reasonableness, and “religion.”<sup>109</sup> For the purposes of this analysis, the Orphan Drug Act will be judged using four out of the seven basic goods, as these four are most applicable to the law and its implications: life, knowledge, sociability, and practical reasonableness.

### a. *Life*

Generally, the value of life is driven by the innate need in humans for self-preservation, but it’s meaning is expanded on further by Finnis.<sup>110</sup> In natural law, life signifies much more, dignifying every aspect of life such as vitality, health, freedom from pain, and the transmission of life through procreation.<sup>111</sup> These necessities drive humans into a community governed by laws because they lead to the “recognition, pursuit, and realization of this basic human purpose (or internally related group of purposes).”<sup>112</sup>

When considering the concept of life and its role in the Orphan Drug Act, the connection is clear, as the primary purpose of the Orphan Drug Act is to value life, specifically, to value the lives of those previously disregarded by the pharmaceutical industry. As aforementioned, pharmaceutical companies invest and develop commercially valuable treatments, and have for quite some time. Up until the Orphan Drug Act, only common diseases that were lucrative, such as diabetes, heart disease, and common cancers, were the focus of pharmaceutical research labs and manufacturers so that such companies could turn a profit. However today, this practice stays

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<sup>109</sup> Finnis, *supra* note 106, at 83-89.

<sup>110</sup> Finnis, *supra* note 106, at 86.

<sup>111</sup> Finnis, *supra* note 106, at 86.

<sup>112</sup> Finnis, *supra* note 106, at 86.

the same as pharmaceutical companies use their resources towards lucrative opportunities when they could easily put those resources to providing therapeutic and curative treatments to diseases that might not be as profitable. This devalues the life of anyone suffering from a rare disease. In fact, this practice completely defiles the value of life as defined by Finnis. Such practices strip vitality, health, and freedom from pain from the members of the rare disease community, a natural right all people are entitled to, not just the people who can help the makers of medicine make money.

Aside from the effects of the Orphan Drug Act, the law itself, in some populations, has helped preserve life. The Orphan Drug Act put a value on an ignored population by incentivizing otherwise greedy pharmaceutical companies into researching and developing a treatment for these people. By Finnis' standard, this boosts the argument that the act itself is in line with the ethics set forth by Finnis' understanding of natural law. While there are flaws in its application, e.g., the many non-lucrative diseases that have minimal to no treatment options whatsoever, this can be overcome through reconsideration and amendment. Thus, standing alone, the act is in line with granting the natural right of life to all people, no matter the disease, but through the furtherance of knowledge of the issue (and in some way sociability), the act's effects could also achieve this purpose. This will be discussed and expanded upon in the subsequent sections

b. *Knowledge and Sociability*

When it comes to the values of knowledge and sociability, they are deeply intertwined in all aspects of the Orphan Drug Act. Finnis explains that knowledge is rooted in curiosity and is, in its simplest terms, "getting to the truth of the matter."<sup>113</sup> As a practical matter, knowledge is a good that one should pursue in life, while ignorance should be avoided.<sup>114</sup> Going further, Finnis

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<sup>113</sup> Finnis, *supra* note 106, at 61.

<sup>114</sup> Finnis, *supra* note 106, at 63.

defines sociability as a spectrum that ranges from the value of peace and harmony among people, through the forms of human community, and to the creation of true friendship.<sup>115</sup> In regards to the Orphan Drug Act, knowledge and sociability ignited its birth, ignorance allowed its effects, and knowledge and sociability can change it so that it is restored to a just law in all aspects.

As a general matter, legislative awareness (knowledge) of an issue is the key to enacting a law. Although, when the Orphan Drug Act was enacted, both knowledge and sociability were equally important in its passing, as one flourished the other and vice versa. As discussed in the more historical section of this paper, it was the gathering of a community of people suffering from debilitating diseases that caught the attention of influential legislators. It was the friendship between those legislators and the afflicted that brought personal testimony to the floor of the house of representatives in order to humanize the issue. Hearing data objectively when compared with seeing someone suffering right in front of you is moving, memorable, and the push needed to bring significant attention to the orphan drug problem. Thus, while knowledge certainly played an important role in the Orphan Drug Act's enactment, it was sociability that facilitated a better understanding of the issue, increasing that key legislative awareness.

Conversely, the FDA's current ignorance is unjustifiable. The law was meant to provide hope for all, and yet, all of the statistics and examples provided throughout this paper are evidence that the law is being abused and segregating classes of people. It is known that therapeutic areas that are not commercially valuable are being ignored. As a result, those with rare diseases are being put into two categories: (1) individuals suffering from a rare disease whose treatments, if developed, would be highly lucrative, and (2) individuals suffering from a rare disease whose treatments project little to no commercial value. One should not be judged on

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<sup>115</sup> Finnis, *supra* note 106, at 88.

whether their condition can be capitalized on nor should one be discriminated against for that very same reason. This data has been around for centuries, and yet there has been no legislative change. This leads to only one conclusion, that the value of knowledge is being blatantly ignored by the FDA and legislators alike. Under Finnis' standards, this is unacceptable because ignorance is to be avoided, and ignorance is the inhibitor of truly fixing the unjust effects of the Orphan Drug Act.

Using Finnis' concepts of knowledge and sociability, this injustice can be remedied to align with the natural law of the world and the natural rights all humans deserve. Parallel to the action ignited in the past, there are two solutions using these basic goods: (1) the community of those afflicted with rare diseases could come together to inspire legislative change like they did when the act was first invented, or (2) the FDA will respond to the outcry of these patients and the criticisms of scientists, authors, and reports displaying the disparity by changing a flawed law. Sociability will increase knowledge, and with enough social pressure, the FDA can no longer contend that it is justifiably ignorant.

*c. Practical Reasonableness*

Practical reasonableness is the source of choosing one's actions and lifestyles by using one's own intelligence to make effective determinations and choices.<sup>116</sup> In addition to shaping one's character, practical reasonableness is a good that is applied a measuring system involving the assessment of balancing freedom to choose with reasonableness in one's actions.<sup>117</sup> Thus practical reasonableness is a complex concept "involving freedom and reason, integrity and authenticity."<sup>118</sup> Practical reasonableness, though seemingly a good that affects only the

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<sup>116</sup> Finnis, *supra* note 106, at 88.

<sup>117</sup> Finnis, *supra* note 106, at 88.

<sup>118</sup> Finnis, *supra* note 106, at 88.

individual using it, can be applied more broadly to make ethical determinations about more than one's actions. As stated before, it is a measuring system, so the algorithm of practical reasonableness can be used in all ethical conclusions, including the law. The following section will assess the Orphan Drug Act using practical reasonableness in order to provide an extensive moral analysis of the law and its effects.

## **X. Nine Principles of Practical Reasonableness**

As stated above, practical reasonableness is the mechanism in which one can make correct judgments about what is just.<sup>119</sup> This mechanism uses nine principles, all of which are "interrelated and capable of being regarded as aspects one of another."<sup>120</sup> The product of the nine principles of practical reasonableness is the morality of the matter being judged.<sup>121</sup> Therefore, at the conclusion of this section, the justness of the Orphan Drug Act and its effects will be revealed.

### *a. First Principle*

The first principle of practical reasonableness is referred to by both Finnis and Rawls as "a rational plan of life."<sup>122</sup> This involves having a harmonious set of purposes, implicitly or explicitly, which ultimately involves making commitments.<sup>123</sup> By commitments, Finnis is referring to definitive objectives that are participated in as basic aspects of human good.<sup>124</sup> In other words, one must see their life as one whole, not favoring one moment over another, and harmonizing one's commitments in order to "establish the proper perspective for choosing how to

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<sup>119</sup> Finnis, *supra* note 106, at 126.

<sup>120</sup> Finnis, *supra* note 106, at 105.

<sup>121</sup> Finnis, *supra* note 106, at 126.

<sup>122</sup> Finnis, *supra* note 106, at 103.

<sup>123</sup> Finnis, *supra* note 106, at 104-05.

<sup>124</sup> Finnis, *supra* note 106, at 104.

live one's present life."<sup>125</sup> The Orphan Drug Act satisfies the first principle because it has a harmonious and coherent set of purposes all of which are focused on the basic aspects of human good.

b. *Second Principle*

The second principle is to only prefer certain forms of good when it is rational to a commitment.<sup>126</sup> When determining if the preference is rational, one must base one's assessments on "one's capacities, circumstances, and even one's taste."<sup>127</sup> Not having the capacity for a real form of good is allowable and distinguishable from arbitrarily denying one of the seven goods as forms of excellence.<sup>128</sup> However, the Orphan Drug Act falls short when judged under both the second principle. When it was first enacted, the act seemed to align with this, but as demonstrated in the seven basic goods analysis, has since then failed. The Orphan Drug Act, through its effects, has arbitrarily denied some of the seven goods: life, knowledge, and sociability. The FDA has not taken any action, despite its knowledge of the disparities in treatment distribution, segregation of persons by the profit that can be achieved from their respective diseases, and the resulting death and suffering from those whose diseases are not lucrative. The excuse of money is not a rational reason for this preference, because it was previously shown that pharmaceutical companies do in fact have the capacity to research and develop treatments for rare diseases, even when those therapeutic molecules diseases will result in little profit. Thus, it can be deduced that the FDA and the pharmaceutical companies have arbitrarily denied more than one of the seven basic goods. While the act itself cannot control these results, amendments to the act would, leading to the conclusion that the act, as is, is unjust.

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<sup>125</sup> Finnis, *supra* note 106, at 104.

<sup>126</sup> Finnis, *supra* note 106, at 105.

<sup>127</sup> Finnis, *supra* note 106, at 105.

<sup>128</sup> Finnis, *supra* note 106, at 105-06.



c. *Third Principle*

The third principle is applying the same rule above to persons; that there should be no arbitrary preference among persons.<sup>129</sup> In short, Finnis allows for a reasonable scope for self-preference, but urges that one should still follow the “Golden Rule”: do unto others what you would have them do to you.<sup>130</sup> The Orphan Drug Act also fails under the third principle in the present day, but not in its enactment itself. In 1983, the Orphan Drug Act gave preference to some persons over others, specifically preferring those afflicted by rare diseases and conditions. However, this distinction was not arbitrary and instead was done in order to promote the goods Finnis’ theory encompasses. However, today it is apparent that the rare disease community has been sliced in half arbitrarily. As discussed above, lucrative opportunities are not a rational reason to deny some persons the rights that others are granted. It goes directly against the natural rights all persons are entitled to and directly conflicts with the Golden Rule.

d. *Fourth and Fifth Principles*

The fourth and fifth requirements are complementary to each other.<sup>131</sup> The fourth requirement, detachment, is necessary “in order to be sufficiently open all the basic forms of good in all the changing circumstances of a lifetime, and in all one’s relations, often unforeseeable, with other persons, and in all one’s opportunities of effecting their well-being.”<sup>132</sup> For example, one should not consider one’s life to be drained of meaning just because a project or objective one took up failed.<sup>133</sup> On the other hand, the fifth requirement, commitment, establishes a balance between complete and otter detachment, e.g., apathy and unreasonable

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<sup>129</sup> Finnis, *supra* note 106, at 107.

<sup>130</sup> Finnis, *supra* note 106, at 107-08.

<sup>131</sup> Finnis, *supra* note 106, at 109.

<sup>132</sup> Finnis, *supra* note 106, at 110.

<sup>133</sup> Finnis, *supra* note 106, at 110.



failure, with fanaticism.<sup>134</sup> The result of this balance is that commitments must be made, followed through, and not abandoned lightly.<sup>135</sup> One should also seek to improve their commitments by looking for new ways to carry them out.<sup>136</sup> In regards to the Orphan Drug Act, the fourth and fifth principles can be used as more of a reflective standard to see if the legislators have both followed through with their commitments and if they have continually sought to improve them. The answer is no to both issues. When the Orphan Drug Act was passed, the FDA made a commitment to achieve the particular purpose of promoting the creation of therapeutic molecules for rare diseases. However, it has since then abandoned the enforcement of the purpose, even though the Orphan Drug Act is still good law. The abandonment is evident in the clear failure of the law to have a given effect, leaving one to infer that the FDA has become detached from the failure of the objective. The FDA has also failed to improve the act through amendments to fix these failures, in violation of the fifth principle Finnis describes. The Orphan Drug Act, being governed by the FDA, becomes the victim of the FDA's inaction, resulting in a once ethical and well-intended law being applied in such a way that now currently violates natural law.

e. *Sixth Principle*

The sixth requirement is recognizing the limited relevance of consequences in order to bring good in the world through efficient actions that have a reasonable purpose.<sup>137</sup> Such actions are judged by their effectiveness, fitness for their purpose, utility, and consequences.<sup>138</sup> Ultimately, the sixth requirement relies on being efficient in pursuing certain goals while

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<sup>134</sup> Finnis, supra note 106, at 110.

<sup>135</sup> Finnis, supra note 106, at 110.

<sup>136</sup> Finnis, supra note 106, at 110.

<sup>137</sup> Finnis, supra note 106, at 111.

<sup>138</sup> Finnis, supra note 106, at 111.

avoiding harms that we regard as unacceptable.<sup>139</sup> This conduct has various applications in both moral and legal thinking.<sup>140</sup> When the House of Representatives passed the Orphan Drug Act, it was drafted in consideration of the harms present in the pharmaceutical industry. The action of its enactment, in some sense, has certainly been effective in its benefits which is intertwined by the utility it brings: it helps an even larger population of people in comparison to the number of people that were helped prior to 1983. Thus, in one sense, the Orphan Drug Act is compliant with the sixth principle. However, if its impact modern day is the standard, the emphasis remains on the consequences of the Orphan Drug Act that were discussed through the criticisms of the act. These consequences are unacceptable, even with utility considerations. Thus, the FDA's conduct, or lack thereof, in fixing the defective Orphan Drug Act causes the act to become immoral because it is ineffective in a large amount of the population encompassing those with rare and nonlucrative conditions. Conversely, the positive effects of the people who have could therapeutic and curative treatments as a result of the Orphan Drug Act cannot be ignored and tips the scales towards a moral conclusion. Therefore, the Orphan Drug Act when analyzed using the standard set forth by Finnis' sixth requirement is objectively inconclusive, but subjectively could become either moral or immoral depending on the value you place on the benefits versus the consequences.

f. *Seventh Principle*

The seventh requirement is to respect the seven basic forms of human good in every action one takes.<sup>141</sup> Additionally, one should not willingly choose "any act which of itself does nothing but damage or impede a realization or participation of any one or more of the basic

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<sup>139</sup> Finnis, *supra* note 106, at 118.

<sup>140</sup> Finnis, *supra* note 106, at 118.

<sup>141</sup> Finnis, *supra* note 106, at 120.

forms of human good.”<sup>142</sup> This principle is parallel to the seven basic goods analysis previously discussed. In sum, life, knowledge, and sociability are all satisfied in the action of the enactment of the Orphan Drug Act and the Orphan Drug Act’s text itself. However, the effects of the Orphan Drug Act and the pharmaceutical companies’ abuse of it impedes the participation in more than one of the seven basic goods. Consequently, the law is moral, but its effects are immoral. Over time, these effects strip the morality of the law which is why the need for amendments is undeniably crucial.

g. *Eighth Principle*

The eighth requirement is merely a respect for the common good, that is to say, favoring and fostering the common good in one’s community.<sup>143</sup> The Orphan Drug Act both promotes and violates the common good, depending on how its framed. In one sense, the Orphan Drug Act promotes the common good in the simple fact that more people are now being helped. Conversely, when the entire population is considered, the Orphan Drug Act incentivizes pharmaceutical companies to put their time into researching and developing treatments for a small number of people. The creation of these treatments is costly, and an argument could be made that these costs could instead focus solely on common diseases. The core of the argument lies in utilitarian ideals, that if treatments and cures were even more available for larger populations, the common good would be satisfied in that it benefits a majority of the population. However, this is not how Finnis would frame the common good. The next section will discuss this further, but Finnis’ idea of the common good in the realm of natural law and rights is rooted in justice and equality. Finnis defines equality in a way that differs from utilitarianism: equality

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<sup>142</sup> Finnis, *supra* note 106, at 118.

<sup>143</sup> Finnis, *supra* note 106, at 125.

should be proportionate to create an equilibrium.<sup>144</sup> Thus, since the Orphan Drug Act promotes an equilibrium by addressing the problems of a population in need, the Orphan Drug Act does, in fact, contribute to the common good and promotes the natural rights all humans are entitled to.

#### *h. Ninth Principle*

The final and ninth requirement is following one's conscience: "one should not do what one judges or thinks or 'feels'-all-in-all should not be done."<sup>145</sup> This principle is the principle that is least applicable to the Orphan Drug Act since it is an internal and subjective determination. Thus, an objective analysis cannot be made as to how Orphan Drug Act strikes the individual conscience. It is up to the reader to make their own determination, given the data, whether the Orphan Drug Act and its effects "feel" right. My personal inclination is that the Orphan Drug Act is theoretically moral, but changes to it would promote the seven basic goods even more in order to fully satisfy the ethical considerations Finnis proposes.

### **XI. Three Elements of Justice**

Lastly, Finnis discusses the three elements of justice as the final step in the algorithm of determining the morality of an action, or in this case, a law. The first element is called "other-directedness," which relates to one's relations with others from an "'inter-subjective' or interpersonal" standpoint.<sup>146</sup> The second element of justice is the concept of the duty to provide other persons what is due to them; their natural rights.<sup>147</sup> The third element is proportional

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<sup>144</sup> Finnis, *supra* note 106, at 165.

<sup>145</sup> Finnis, *supra* note 106, at 125.

<sup>146</sup> Finnis, *supra* note 106, at 161.

<sup>147</sup> Finnis, *supra* note 106, at 162.

equality so that a balance is reached in society.<sup>148</sup> Since the first element is an interpersonal element of justice, the following evaluation will focus on the latter two elements.

Under Finnis' theory of justice, the justness of the law can be determined by inquiring as to whether the law provides proper rights the targeted persons and whether the law promotes proportional equality. As has often been the case through the entirety of the paper, the law itself is just, but its effects contradict that conclusion, as they are immoral, unethical, and defy the purpose of the act. In the interest of non-repetition, the reasons for this conclusion will be briefly addressed. The Orphan Drug Act provided rights of life to persons who did not have such opportunities prior to its enactment. In fact, prior to 1983, the community of those afflicted by rare diseases were frankly ignored, had no hope for therapeutic or curative molecules, and were simply damned to a life of suffering and inevitable death. Thus, the Orphan Drug Act is fair in that it changed that clear disparity through distributive justice, giving the rare disease community what was due to them in a way to promotes proportional equality. Conversely, the pharmaceutical industry has displayed through their commercially valuable opportunity preference in producing new therapies that this is violating the just intentions behind the Orphan Drug Act. Pharmaceutical manufacturers are denying a population the rights due to them through natural law and are segregating the rare disease community instead of seeing all members as equal. The only solution to remedy this paradox is to redraft the law in such a way that limits the ability of pharmaceutical companies to participate in these immoral practices so that morality in all aspects can be restored.

## **XII. Conclusion**

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<sup>148</sup> Finnis, *supra* note 106, at 165.

The Orphan Drug Act will continue to be abused by the pharmaceutical industry unless it is amended to restore it to its original purpose. At its core, the policy considerations behind the amendment should above all focus on fairness as defined by Finnis. The effects of the Orphan Drug Act have allowed pharmaceutical companies to make “arbitrary” decisions as to whose diseases are more important than others. Said differently, they have decided whose lives are more deserving of treatment than others. The use of the word “arbitrary” is used in the sense that Finnis uses it, not in its literal definition. Finnis through his seven fundamental goods and three elements of justice makes it clear that lucrative opportunities, in this context, are not a rational reason to choose certain lives over others. The Orphan Drug Act aimed to achieve true fairness in the medical community: equal access to treatments, no matter the disease. Yet, as currently written, the Orphan Drug Act inadvertently allows manufacturers to directly inhibit that access through developing drugs for a narrow therapeutic set of families and by charging absurdly high prices for the therapies that do exist. What is most important is to raise awareness to elected officials and the medical community at large for the rare disease community, who are still in the same place as they were before the Orphan Drug Act was even passed. Fair access and treatment are the major policies behind the Orphan Drug Act and in order for those purposes to be properly carried out, the Orphan Drug Act should change their incentives in order to promote equal access to therapeutic molecules that can provide a better quality of life to those who never had treatment, to begin with. Unless this critical assessment and change is made, millions of members of the rare disease community will be damned to a limited life of suffering, with no hope for a better tomorrow.