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Ethics in the service of the rare disease patient: Application of the thought of John Rawls and Paul Ramsey toward the increased availability of orphan drugs

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ETHICS IN THE SERVICE OF THE RARE DISEASE PATIENT:
APPLICATION OF THE THOUGHT OF JOHN RAWLS AND PAUL RAMSEY
TOWARD THE INCREASED AVAILABILITY OF ORPHAN DRUGS

by

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ABSTRACT

ETHICS IN THE SERVICE OF THE RARE DISEASE PATIENT:
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JOHN R. WILSON, JR.

Persons with rare diseases are frequently unable to obtain suitable pharmacologic treatment. Pharmaceutical companies are not apt to develop compounds for which there is a very small commercial market. Further, drugs for rare diseases are scrutinized by the Food and Drug Administration in the same manner as those for more common diseases—a contingency which, due to the enormous cost of drug research, further discourages profit-driven pharmaceutical companies from developing compounds for rare diseases.

John Rawls and Paul Ramsey offer insight into the situation and remedy of the plight of the rare disease patient. Rawls, with his notion of the basic structure of society, offers a framework in which justice dictates that the background institutions of this basic structure be constantly criticized and changed. Ramsey's thought serves the issue with his notion of agape or covenant-care—a concept which calls for the treatment of patients as suffering individuals.
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CHAPTER ONE
INTRODUCTION

1. Introduction and Statement of the Thesis

Rare diseases are defined by the Department of Health and Human Services as those affecting fewer than 200,000 persons in the United States.\(^1\) Currently, there are approximately 5,000 recognized rare diseases.\(^2\) Some of these maladies, such as Tourette's Syndrome, Huntington's Disease, and Amyotrophic Lateral Sclerosis have received media exposure to such an extent that they have become familiar to the public. Other rare afflictions, such as Rett Syndrome, Mucopolysaccharidoses, and Cornelia de Lange Syndrome are so uncommon that most physicians practice a lifetime without encountering them.

Long viewed as pathological curiosities, patients\(^3\) with rare diseases have only recently been afforded appreciable recognition and assistance within the American health care system. Among the problems which have historically distressed the rare disease patient are a lack of recognition of rare diseases as a legitimate health problem, a reluctance on the part of the pharmaceutical industry to develop compounds for which by definition there is a miniscule market, and the failure of distributional theories of health care allocation to adequately address the special needs of such patients. These problems, among others, have forced the rare
disease patient into an undesirable position within the health care marketplace.

An analysis of the plight of the rare disease patient requires an inherently interdisciplinary approach. Although the discipline of medical ethics has been primarily viewed within clinical settings, i.e., issues of the provider-patient relationship, medical ethicists are increasingly being challenged by issues of resource allocation and public policy. One may view the ethical aspects of the plight of the rare disease patient from a perspective of business ethics, as pharmaceutical companies are faced with the problem of prioritizing projects which are helpful to a group of people in need but which are simultaneously detrimental to the primary purpose of the business enterprise, i.e., profit. Further, the fact that any public policy to correct inequities in our present drug approval and marketing structures is indeed a policy of the people for the benefit of those in unfortunate circumstances necessitates the inclusion of social ethics into the equation. Also, one must consider that rare disease patients are first of all suffering persons—a consideration which suggests the inclusion of the disciplines of religion and moral philosophy as sources of enlightenment.

In view of the many academic and professional disciplines involved in a study of the rare disease patient, one may assume that a corresponding number of possible solutions are available for analysis. Laws which serve to encourage the development of drugs for the rare disease patient may be enacted. Self-help
groups may be of benefit to those in need of social support mechanisms. Philanthropy may be encouraged, by which rare disease research and the development of therapies may be facilitated. None of these contingencies, however, address the institutional structures and attitudes which impede the availability of effective therapy for the rare disease patient.

This thesis will argue that several fields of ethics offer a philosophical foundation upon which the special needs status of the rare disease patient may be addressed. Further, the thesis will contend that ethical reflection and subsequent unified action may help remedy inequities in our system of drug development and marketing—inequities which prevent or at least discourage the availability of effective drug therapy for the rare disease patient.

This thesis will examine the dominant circumstances of the rare disease patient within the spheres of business ethics, distributive justice and theological ethics. The inherently interdisciplinary nature of the resource question involved both permits and requires such a categorized approach. Business ethics enters the equation due to the fact that pharmacologic therapy in the United States is provided almost totally by profit-driven corporations. Distributional theories of justice provide the necessary framework for establishing just resource allocation schemes. One may look to theological ethics for insight into the nature of the rare disease patient as suffering individual, as well as for ascertainment of duties to one’s fellow man.
Applying theological and philosophical principles to secular institutions such as the free market, pharmaceutical companies, and legislative bodies is not without controversy or challenge. My analysis will reveal, however, that the institutions involved in such a study lend themselves to sound ethical reflection. For instance, the interrelatedness of American industry and the consumer has come under intense study by applied ethicists. Also, the very structure of health resource allocation lends itself to reflection on the ethical choices in macroallocative decision making. In addition, in order to view the rare disease patient as suffering individual rather than as a statistical deviation, one must concern oneself with issues such as human dignity and respect of persons.

Health care in the United States is largely a market-driven commodity, although the term "commodity" is used infrequently in such contexts. Nonetheless, it is clear that with the exception of the "acceptable minimum" provided to indigents and others unable to participate in the free market, health care services are largely rationed within a modified supply and demand system. "Modified" is a key description due to the extensive regulations inherent in some health care services, e.g., the marketing of pharmaceutical compounds. Thus one frequently finds markets in which demand cannot immediately be met by additional supply. A need for a particular therapy does not address the safety and efficacy issues inherent in bringing new drugs to the market. Such issues are addressed only by extensive and costly testing.
The crux of the problem faced by rare disease patients is that the primary therapy for their maladies is frequently pharmacologic, and that the marketing of pharmacologic therapy is highly regulated by the government and simultaneously controlled by profit-driven corporations. Thus the rare disease patient, by virtue of the inadequate number of persons available as either test subjects or ultimate consumers, is frequently unable to obtain viable therapy.

The rare disease patient is not the only party affected by this plight. Pharmaceutical company management is being asked by special interest groups and the federal government itself to assist the rare disease patient.\(^4\) Legislation has been enacted which encourages pharmaceutical companies, via tax incentives and marketing exclusivity rights, to develop drugs for the rare disease patient.\(^5\) Such compounds, known as orphan drugs, would not be considered for development under normal free market conditions. However, even with these incentives, the development of most orphan drugs results in a total loss to the sponsor of at least one million dollars.\(^6\) Thus pharmaceutical companies are faced with making decisions which benefit a small subgroup of consumers at the risk of compromising the furtherance of the corporation's primary business purpose, i.e., making a profit.

Although corporations have practiced philanthropy for decades, the staggering costs of developing orphan drugs suggests action grounded in a more fundamental appeal than charity. Recent scholarly work in applied ethics, for instance, indicates that
corporations may have responsibilities beyond the maximization of profit. Scholars and business leaders themselves have argued that corporations have a social responsibility which demands that sound ethical reasoning play a role in policy decisions. Others, such as economist Milton Friedman, argue that the only social responsibility of a corporation is to maximize its profits. This view holds that the benefits of profit maximization redound to society at large. Proponents of the social responsibility model would presumably support the notion of a responsibility to address the plight of the rare disease patient, whereas adherents of Friedman's profit maximization scheme would hold that no such responsibility exists.

Any ethical analysis of a particular special-needs population would be remiss without reflection on the patients themselves. One of the major problems in macroallocative decision making is the absence of consideration of the patient as individual. Paraphrasing Paul Ramsey, my analysis will look to a conceptualization of the rare disease patient as "person." Policy makers, both industrial and governmental, are no doubt prone to moral distancing. This concept, in which one finds it easier to address the needs of a familiar tangible case rather than abstract statistical cases, is well represented by the contingencies facing the rare disease patient. This thesis will advance the argument, supported by the thought of Ramsey, that the Judeo-Christian notion of love for fellow man requires that rare disease patients
be considered as individuals with particular and special neighbor-
needs rather than as pathological curiosities.

My analysis will show that the fields of business ethics, distributive justice, and theological ethics may help explicate and remedy the plight of the rare disease patient. However, none of these disciplines offer a total package of both clarification and solution. In fact, even the additive thought of the frameworks of action suggested by these three specialties does not make pharmaceutical compounds available to the rare disease patient in need. For this we must look to a cooperative approach between industry and government—an approach which recognizes government's legitimate role in intervening in the case of a market failure\textsuperscript{11} coupled with industry's recognition that pharmaceutical companies, due to their unique role in the service ethos of medicine, possess a social obligation beyond that of profit maximization.

2. Review of the literature

The literature review will consist of a description of the general thought of the authors and works cited. Subsequent chapters will afford the opportunity to further reflect upon such thought and its applicability to the rare disease patient.

The interdisciplinary character of this topic requires the utilization of an expansive range of scholarly work. For the sake of clarity and ease of transition, the literature can best be reviewed within three primary categories. First, the
responsibility of pharmaceutical companies to provide funding and expertise for orphan drug development may be viewed within the broad context of business ethics, with particular emphasis on the social responsibility of health care corporations. Second, the argument for a framework based on distributional justice may be addressed by an examination of John Rawls' theory of justice as fairness, with particular regard given to how others have come to understand the just distribution of health care from a Rawlsian perspective. Third, the notion of human dignity and respect for persons with rare diseases may be examined in light of the Judeo-Christian virtue of love. For this I shall rely on the thought of Paul Ramsey.

My first literature category will concentrate on a review of the social and moral responsibilities of corporations. Economist Milton Friedman, in an oft-quoted article entitled "The Social Responsibility of Business Is to Increase Its Profits," argues that corporations do have a moral obligation to society. As the article's title clearly suggests, however, this responsibility is embodied in the maximization of profits rather than in any "social" endeavors. Claiming that only persons, and hence not corporations, can have responsibilities, Friedman argues that the sole responsibility of business managers and executives is to further the desires of the stockholders. Assuming that the owner's desires are to increase profits, Friedman asserts that any policy which diverts corporate resources in any other direction is essentially socialistic.
Friedman views the holding of prices to prevent inflation, the establishment of pollution controls above those either required by law or in the best interest of the corporation, and the hiring of the hardcore unemployed in an effort to fight poverty to be examples of illegitimate use of corporate funds. Friedman sees such social action as the implementation of taxes on consumers via the increase such programs have on the cost of doing business. Such policies are seen by Friedman as leading to the corporate manager becoming an agent of the government, as such a policy maker is imposing taxes, albeit indirectly, and deciding that these monies are best spent on particular social projects.

Friedman's argument, however, does not actually condemn corporations for devoting resources to social causes. The qualifier is that in such a scenario the motive must remain the best interest of the corporation. Thus the donation of a park could presumably be acceptable corporate activity, provided that the park aided in the recruitment and retention of employees or performed any similar function which furthered the priority of profit maximization.\textsuperscript{12}

Closely aligned with Milton Friedman is the thought of Theodore Levitt. In defense of the classical profit-driven paradigm of business, Levitt argues that competition, furthered by concentration on increased profits, provides the pluralism which is required for a capitalistic society. Levitt sees the corporation engaged in social responsibility as posing the threat of a
"powerful economic functional group" which determines what is best for society.

Arguing that it is the role of government to look after the general and social welfare of society, Levitt calls for business to recognize its only obligation to be the maximization of profit. Thus, corporations may focus on this one primary objective and not be burdened with the social causes and needs of mankind. The result, according to Levitt, is a clear separation of business and government—a separation necessary for the advancement of a pluralistic society.\footnote{13}

Much of the debate concerning social obligations of business revolves around the moral status of corporations. In this light, one is concerned not so much with what obligations a corporation may have toward society, but rather how a corporation is to be considered by society. There are essentially two approaches to this situation. On the one hand, one can argue that corporations are moral agents with moral responsibilities. An argument along these lines will propose that corporations are separate entities which are endowed with specific rights and corresponding duties. On the other hand, one may argue that corporations are essentially no more than an aggregate of individual persons and property, and as such do not possess any more moral agency than the individual persons who comprise the organization.

Philosopher Peter French espouses a theory of corporate responsibility which provides for an acceptance of the corporation as an individual moral agent. Arguing that the "Corporate Internal
Decision (CID) Structure provides the requisite decision-making ability of personhood, French claims that such CID structures exist as a personnel and management structure which is responsible for relaying input and decisions from the lesser levels of the corporation to the policy making levels. It is at this higher echelon of the corporation that what French terms "redescriptive exposure" transfers and modifies the aggregate input into a decision which results in a policy statement. The "redescriptive exposure" insures that the decision is in the interest of the corporation and is not a transient interest of a manager of director who has input into the case. Further, this element of synthesis provides a qualitative difference in corporate decisions as opposed to the decisions made by individual executives. This latter point serves French's requirement that decision events by corporations have their basis in rationales which are different from any personal rationale of a manager or director. Thus, the corporation can be said to possess moral agency by virtue of the decisions of the CID structure which are qualitatively distinct from the aggregate personal decisions of component members of the corporation.14

Organizational theorist Michael Keeley argues against French's assertions of corporate moral agency. Contending that organizational goals and purposes are not identifiable, due to the "mystical" nature of the transfer of the action of corporate personnel into a corporate being, Keeley argues that corporations in effect have no real objectives apart from the aggregate
objectives of corporate employees. Since the identification of preconceived objectives is a major requisite in the acceptance of corporations as moral agents, at least according to French, the lack of true organizational goals leads to Keeley's assumption that corporations are indeed not separate moral agents.¹⁵

I have been considering arguments pertaining to the moral status of corporations, and therefore the obligations and responsibilities attached to them. While scholars have cogently argued both sides of the question, an affirmative stance on either position (the corporation as person or as non-person) still requires that one address the issue of what society may legitimately expect of business. If corporations are accepted as moral agents, it may be claimed that the same moral obligations and responsibilities which apply to individuals also apply to the corporation. However, one may also expect certain behavior and results from the corporation as non-person. Without sidestepping the issue of corporate moral agency, this thesis will depend more upon legitimate societal expectations of the corporation as a social system. One may view business from either of the quite different perspectives of person or non-person and nonetheless expect certain irreducible standards of behavior from any social system or construct. One may in fact argue persuasively that such normative standards themselves constitute a "moral status" even to the corporation viewed as a non-person.

The concept of social responsibility without direct allusion to moral agency is most frequently expressed in terms of the
social contract. Melvin Anshen has provided a useful historical analysis of the concept of corporate social responsibility within the social contract paradigm. Reminding us that there is nothing new about the concept of a social contract, with roots in the early Greek writings of Epictitus, Anshen describes the intellectual foundations of social contract theory by explicating the thought of Hobbes, Locke, and Rousseau. The crux of the dilemma faced by corporate managers today, according to Anshen, is that there are now non-economic contingencies exerting pressure on the system. Previously, economic growth and its classical drive for increased profits was viewed as the catalyst for the continuance of the institutionalized responsibilities and duties accepted by society. More recently, however, society has considered the fact that social progress is not necessarily only a by-product of economic growth, and that a corporation can be expected to participate at least minimally in the quest for social improvement distinct from its role as a profit-driven system.

Anshen calls for changes in the rules of our economic system without actually changing the game itself. Specifically, Anshen calls for the internalization of the social costs of doing business. Corporations may no longer purge themselves of the external costs of business, such as environmental contamination. Further, managers must adhere to the new social contract which utilizes their experience and expertise for the common good. In this way, corporations may help strike the necessary balance between ecologic and social progress--a balance which requires
recognition of the social obligations of business beyond that of a by-product of profit maximization.¹⁶

Arguments concerning the social responsibility or moral status of corporations may help establish ground rules or expectations of conduct, but do not of themselves adequately serve the issue of the underavailability of drugs for rare diseases. After all, pharmaceutical compounds, like most other health care commodities, are distributed within a free market structure. This free market approach to the distribution of health care commodities creates a prima facie injustice, as rare disease patients are unable to obtain viable therapy while such therapy is readily available for persons with more common diseases. The injustice is prima facie only, however, if one can justify these inequalities in pharmaceutical distribution by the use of general theories of justice. One such theory is that of John Rawls, who asserts that his theory of justice as fairness establishes a fair method for the distribution of goods. The thought of Rawls and his critics will form the basis for my second literature category.

Rawls is undoubtedly one of the most widely quoted contemporary philosophers. His major work, A Theory of Justice, appeals to scholars and practitioners of philosophy, social sciences, religion, and economics. Rawls describes his primary aim as presenting a theory of justice which "generalizes and carries to a higher level of abstraction the familiar theory of the social contract as found, say, in Locke, Rousseau, and Kant."¹⁷
Rawls' resulting theory, which he refers to as justice as fairness, is offered as an alternative to teleological theories of justice.\(^1^8\)

Rawls proposes that it is possible to determine an ahistorical method of choosing principles by which societal conflicts may be resolved. By utilizing Rawls' concept of the original position, one can imagine what rational persons would choose as principles of justice, provided that the contractors are indeed disinterested parties. To insure this disinterested nature of participation, Rawls requires the contractors to be blind to their own particular situations in society—a blindness referred to as the "veil of ignorance." Thus the social contractors are not aware as to what will be their race, gender, economic status, or other characteristics.\(^1^9\)

Rawls asserts that such rational disinterested contractors would choose the following two principles as a basic structure of justice:

1) Each person is to have an equal right to the most extensive total system of equal basic liberties compatible with a similar system of liberty for all.

2) Social and economic inequalities are to be arranged so that they are both:
   a) to the greatest benefit of the least advantaged, consistent with the just savings principle, and
   b) attached to offices and positions open to all under conditions of fair equality of opportunity.\(^2^0\)

Although Rawls does not specifically address health care from the standpoint of the original position or the two principles, his theory is often invoked by those seeking to develop a just
system of health care delivery.\textsuperscript{21} Norman Daniels, in \textit{Just Health Care}, argues that the best health care adaptation of Rawls is to include health care institutions and practices among those responsible for providing equality of opportunity. In this view, health is seen as necessary for fair opportunity rather than as another social good.\textsuperscript{22}

H. Tristram Engelhardt, Jr., in \textit{The Foundations of Bioethics}, asserts that an essential question in relating Rawls to health policy is whether health falls under the first or second half of the second principle. If health is viewed as a primary social good, allocational differences are justifiable provided that the differences redound to the least advantaged. If, however, health is defined as a component of equal opportunity determination, funds for health care would be allocated prior to funds for other social goods.\textsuperscript{23}

Ronald Green, in "Health Care and Justice in Contract Theory Perspective," argues that health care should be classified as a primary social good in the Rawlsian scheme, although Rawls does not categorize it as such. Health care is so important, according to Green, that contractors in the original position would "opt for a principle of equal access to health care: each member of society, whatever his position or background, would be guaranteed an equal right to the most extensive health services the society allows."\textsuperscript{24} In order to achieve this goal, Green proposes that a separate principle of justice, a right of equal access to health care, be added to the Rawlsian scheme of justice as fairness.\textsuperscript{25}
Green's proposal of a third Rawlsian principle to substantiate a right to equal health care access has not been universally received with favor. John Moskop, in an article entitled "Rawlsian Justice and a Human Right to Health Care," attacks Green's theory as requiring substantive changes in the Rawlsian scheme of justice. Specifically, Moskop argues that Rawls' doctrine of the primary social goods is not intended to cover all social goods, regardless of their relative importance. Rather, the doctrine of the primary social goods is seen as a "simplifying device" designed to (1) formulate a limited number of general principles of justice, and (2) identify relevant social positions. Moskop argues that opening the door for a third principle dealing exclusively with health care would create an urgency for other principles designed specifically for other basic needs.26

Rawls' notion of justice as fairness, while useful in formulating health care distribution schemes, does not address the needs of the rare disease patient as suffering individual, and hence does not get to the heart of our problem. The understanding and appreciation of the rare disease patient as suffering individual calls for the inclusion of theological ethics in any attempt to elucidate the plight of such patients--an inclusion which constitutes the third literature category in my analysis.

My concern with theological ethics as a contributor toward a useful construct for decision making suggests an inquiry into the thought of Paul Ramsey, who has written extensively on both
theological and medical ethics. Ramsey is not the only theological ethicist whose thought has been analyzed within the context of application to issues of medical ethics, nor is there a scarcity of other theological ethicists who have directly addressed medical ethics. Ramsey was chosen for this analysis primarily because of the potential for the application of his distinctively deontological stance to the understanding of the rare disease patient as suffering individual. A distinction must be made between Ramsey's thoughts on love and those directly addressing issues of medical ethics. The latter, although useful in our analysis, will be somewhat limited due to Ramsey's interest in clinical and microallocational issues rather than the broader issues inherent in providing therapy for the rare disease patient.

Ramsey's first major work, Basic Christian Ethics, codifies an ethics based upon Christian agape. For Ramsey, agape is derived from the concept of covenant faithfulness. Men are called to love their neighbor in the spirit of faithfulness, and not out of any sense of consideration of consequences. One of Ramsey's priorities in Basic Christian Ethics is to define the term "neighbor." Reminding us that the parable of the Good Samaritan instructs us on neighbor-love but not directly on the neighbor, Ramsey calls for the active loving of one's neighbor irrespective of whether the neighbor deserves such love.

Taking the issue of neighbor-love to a broader scale, i.e., that of superior values, Ramsey reminds us that there are two basic questions in ethics: (1) What is the Good? and, (2) Whose
Good shall prevail when a choice must be made?\textsuperscript{30} Asserting that the second question is the primary concern for ethics, Ramsey calls for the practice of agape toward the neighbor—a practice which negates the importance of erotic love or a love seeking its own reward.\textsuperscript{31}

In \textit{Basic Christian Ethics}, Ramsey expounds a model of vocations based on agape. Ramsey reminds us that the Reformation served to equalize all vocations in the sight of God. No longer was there a special religious merit attached to the clergy. Using Tolstoy’s example of love, which Ramsey compares to the Sermon on the Mount, Ramsey asserts that the vocational duties of love often call for preferential treatment of certain persons over others. Ramsey sees no inconsistency in this concept provided that the preferential love is for the good of the neighbor. However, preferential treatment is not love if it results from selfish motives.\textsuperscript{32}

Ramsey further asserts that love is always in search of a social ethic in which to express itself. Finding the best avenue for love is an example of disinterested love for neighbor. Ramsey questions whether a social order can be shaped from the norm of Christian love. Reminding us that love indeed calls for such a social policy, Ramsey asserts that the acid test for social change is to first determine the neighbor’s need. It is within this spirit that one can sway the current customs and institutions towards disinterested love for neighbor.\textsuperscript{33}
Basic Christian Ethics rather forcefully argues against the use of rules, a stance which is modified by Ramsey with the publication of Deeds and Rules in Christian Ethics. This later work raises the issue of working through rules, with Ramsey arguing that there can be no Christian social ethics without agape-based rules of practice. Our task is to seek not only our neighbor's needs but also to determine what love requires as a social practice.

One of Ramsey's central questions in Deeds and Rules in Christian Ethics is how to categorize the ideal of agape as an ethical system. Whereas other ethical systems depend upon either a deontological or teleological motif, Ramsey argues that agapism deserves a third normative category of ethics. Ramsey suggests that agapism, while distinct from deontological and teleological systems, is much closer to the former than to the latter. Christian love defines what is to be effected among men, and does not define the good in a sense of utility or desert.

There is no easy blending of Ramsey's theological ethics into his medical ethics. This has as much to do with style as with substance. For instance, The Patient as Person, Ramsey's first major treatise on medical ethics, is not so much a systematic treatment of conceptual ideas as it is a disconnected series of diverse topics. Ramsey's interest in medical ethics is clearly centered within the framework of clinical medicine. Less important for Ramsey are macroallocational issues of resource distribution.
In "The Ethics of a Cottage Industry in an Age of Community and Research Medicine," Ramsey addresses what he refers to as medicine's social requirement to do the greatest good for the greatest number of present and future patients together. Ramsey asserts that one must often determine rightness and/or wrongness of medical actions or policy irrespective of this utilitarian approach. Reminding us that there is no "unseen hand" which assures the ethical justification of policies and procedures designed to produce much aggregate good, Ramsey asserts that moral judgment must be used in determining the adequacy of any proposal. Ramsey further calls for an attitude of partnership between recipient and provider—a recognition that we are all bound by a covenant of faithfulness which allows us to be "joint adventurers" toward medically useful goals. This notion of partnership will form a cornerstone in my analysis, as I will later argue for the existence of a covenant relationship between the health care corporation and the patient.

This concludes my outlines of the three categories to be researched in this thesis. Further examination of the relevance of these categories to the rare disease patient will be found in Chapters two through five, as the thesis attempts to elucidate the predominant concerns of such patients within the American system of health care delivery.
3. Structure of the Argument

Chapter one introduces the topic and provides a general review of the relevant literature.

Chapter two examines the responsibilities of the pharmaceutical corporation to the rare disease patient. This chapter argues against economist Milton Friedman's thesis that the only social responsibility of a corporation is to increase profits, and further proposes that the role of the health care corporation in the service ethos of medicine, coupled with the special nature of health care, attaches a duty of beneficence to the pharmaceutical company.

Chapter three considers the relevance of John Rawls' theory of justice as fairness to the plight of the rare disease patient, with particular attention paid to Rawls' notion of the basic structure of society.

Chapter four argues that theological ethics, and in particular the thought of Paul Ramsey, offers a framework for viewing the rare disease patient as suffering individual—a structure based upon Ramsey's notion of agapaic covenant-care for the individual qua patient.

Chapter five offers a summary of previous arguments, as well as an analysis of the Orphan Drug Act of 1983—an analysis based upon the categories of theological ethics, distributive justice, and business ethics, and therefore consistent with the ethical disciplines examined in the thesis. Further, Chapter five offers a practical framework by which the plight of the rare
disease patient may be served—a construct based on the thought of Rawls and Ramsey.
ENDNOTES


6*Pharmaceutical Executive*, April, 1988, p. 40.


11The concept of market failure will be addressed more explicitly in Chapters three and five.

12"The Social Responsibility of Business is to Increase Its Profits."


19. Ibid., pp. 136-142.

20. Ibid., p. 302.


25. Ibid., pp. 121-123.


28 Ibid., pp. 133-152.

29 Ibid., pp. 92-103.

30 Ibid., p. 114.

31 Ibid., pp. 114-115.

32 Ibid., pp. 153-190.

33 Ibid., pp. 326-366.
CHAPTER TWO
THE PHARMACEUTICAL COMPANY AND
THE RARE DISEASE PATIENT

Central to the plight of the rare disease patient are the motives and priorities of the pharmaceutical company. In the United States, virtually all pharmaceutical compounds are marketed by profit-driven corporations. In the case of the rare disease patient, such a scenario creates a conflict between the needs of such individuals and the right of the corporation to pursue its primary business end, i.e., the generation of profits for owners and shareholders. Chapter two will present a review of corporate responsibility and moral agency, and how these concepts specifically apply to the health care corporation.

As described in Chapter one, pharmaceutical agents are marketed in the United States in a modified free market. The pharmaceutical market may be described as free in that a corporation may employ the classic market parameters of supply and demand in determining which products to develop and market. This free market is modified in the case of pharmaceuticals, given the regulatory influence of the Food and Drug Administration. A pharmaceutical company may choose to develop a product which has promise in treating an indication for which there is a viable market. However, the product will never enter the market unless proven safe and effective.
For the purposes of this section of my analysis, it is appropriate to concentrate on a consideration of pharmaceutical marketing as if it took place in a pure free market, i.e., one unencumbered by governmental controls. Government regulations, while critical in their influence over which drugs are actually approved for marketing, are best put aside when considering the ethical ramifications of the response of the corporation to special needs cases. Pharmaceutical development, in spite of governmental control, nonetheless remains essentially a supply and demand economic game—a situation which puts the rare disease patient at a decided disadvantage.

A free market requires both a private property system and a system for the voluntary exchange of goods.¹ In order for a society to successfully support a free market system of exchange of goods, it must support a system of private ownership of property which allows owners to dispose of or exchange their property as they see fit. Similarly, individuals in a free market must be able to enter voluntary exchanges with others who are willing and able to do the same.²

Until 1938, the pharmaceutical industry was similar to other emerging industries in America. The essence of pharmaceutical marketing was to find a market and fill it. Unfortunately, this often led to dangerous and ineffective medications being sold to the American consumer. 1938 saw the introduction of the Food, Drug and Cosmetic Act which introduced important safety and labeling issues. Along with the Kefauver-
Harris Amendments in 1962, which established requirements for the demonstration of efficacy of drugs, this legislation served to introduce major modifications in the pharmaceutical marketplace.³

Pharmaceutical firms, as corporations, are "organizations that the law endows with special legal rights and powers."⁴ The corporation is undoubtedly the most dominant entity in the American economic system. Only eight countries in the world (Canada, France, West Germany, Italy, Japan, United States, Soviet Union, and United Kingdom) have governmental budgets larger than the total sales revenue of the Exxon Corporation. Similarly, General Motors employs more manufacturing personnel than do 160 of the 190 countries in the world.⁵

Corporations are treated as personal entities by the law, and as such have the right to sue (and may be sued), own and control private property, enter into contractual agreements, and in general exist as "persons" in the legal sense. As such, the application of any moral standard to the corporation is quite difficult. Can one speak of "moral" or "immoral" acts of a corporation? Are corporations morally responsible for their actions? Such questions require that the notion of moral agency of corporations be addressed—a concept introduced in the literature review in Chapter one and explicated further at this juncture. At the risk of slight repetition, but toward an advantage of clarity and reference, the two positions on the moral status of corporations will be briefly restated.
On the one hand, one can argue that corporations, by virtue of their "personal entity" status granted by the law, are "moral" or "immoral" in the same sense as individuals. Philosopher Peter French argues that corporations, via their internal decision making complexes, possess the requisite objectives of personhood. This "Corporate Internal Decision" structure provides a management function which relays information from the lower levels of the corporate hierarchy all the way up to the ultimate policy making positions. At the higher echelon of management, the aggregate input from the lower and middle levels of decision making is assimilated and redefined according to the immediate and long range goals of the corporation. This "redescriptive exposure" minimizes the risk of a corporate decision being based too heavily on one person's input. In addition, the "Corporate Internal Decision" structure provides a qualitative difference in the decision apparatus of corporations vis-a-vis individuals. For French, the goals-based "Corporate Internal Decision" structure meets all reasonable requirements for personhood, and therefore allows one to state that corporations are morally responsible entities and as such are "moral" or "immoral" in their actions.\(^6\)

On the other hand, one can hold that there is no basis for holding corporations morally responsible for their actions. This view would, of course, recognize the moral agency of individuals working within the corporation. Corporations could therefore be held legally and otherwise accountable for the actions and derelictions of their employees, but the corporation qua business
entity cannot be morally responsible in the same sense as an individual. Arguing from this perspective, organizational theorist Michael Keeley claims that the requirements of personhood are lacking in the corporation. French's "Corporate Internal Decision" structure is, for Keeley, nothing but the aggregate intentions of the corporation's individual employees.\(^7\)

Business ethicist Manuel Velasquez offers a compromise solution to the issue of the existence of moral agency of corporations. Conceding that corporations indeed exist and act like persons, Velasquez argues that they are nonetheless clearly not persons. This solution hinges upon one's acceptance of the corporation's dependency on the "feelings, reasonings, and deliberations" of the human individuals who make up the corporation. This thought is expressed in the concept of primary and secondary bearers of morality. The individuals comprising a given corporation are, by nature of their personhood in the real sense, the primary agents of morality in a corporation.\(^8\) The corporation itself possesses only secondary moral agency, since it depends on the actions and thoughts of human individuals:

A corporation has a moral duty to do something only if some of its members have a moral duty to make sure it is done, and a corporation is morally responsible for something only if some of its members are morally responsible for what happened. . . .\(^9\)

It is clear that arguments for or against the notion of moral agency of corporations, as well as Velasquez's compromise, are
dependent upon the *decision* processes of the corporation, and do not define moral agency with reference to the corporation's *relationality* to individuals or to society as a whole.

Whether one agrees with Velasquez's notion of primary and secondary categories of moral agency, it is clear that some concept of moral responsibility must be accepted if corporations are to be held ethically responsible for their actions. The alternative is to view corporations as organizational behemoths who operate with machine-like oblivion to moral reasoning or ethical reflection. Although this argument is forcefully advanced by Friedman in his assertion that the only social responsibility of business is to increase profits, it nonetheless appears to rely on assumptions which, for health care corporations at any rate, are invalid. The remainder of this chapter will attempt to elucidate this point.

**The Status of Health Care**

In many respects, corporations involved in the delivery of health care goods and services are fundamentally not unlike business entities involved in other endeavors. "Finding a market and filling it" is as true of pharmaceutical firms as it is of airplane manufacturers. To fully appreciate the difference between health care corporations and other business enterprises, one must appreciate the notion of the special status of health care in the United States.
What, if anything, makes health care different from any other commodity or good? Certainly one may assume that health is a primary good, which in Rawlsian terms may be defined as a good "that every rational man is presumed to want."\textsuperscript{11} Health care helps alleviate suffering, pain and illness--contingencies which one may assume with certainty would be avoided by a rational being.

Norman Daniels presents a useful strategy for the inclusion of health care as a "special status" good. In "Health Care Needs and Distributive Justice," Daniels argues that since illness restricts the normal functioning of the human species, it correspondingly restricts an individual's opportunity to pursue offices and careers. Health care institutions therefore further the Rawlsian guarantee of equality of opportunity by successfully providing treatment of disease.\textsuperscript{12} In his later work \textit{Just Health Care}, Daniels clarifies this concept by explaining that "impairment of normal functioning through disease and disability restricts an individual's opportunity relative to that portion of the normal range his skills and talents would have made available to him were he healthy."\textsuperscript{13} This clarification is necessary in order to emphasize that

... equality of opportunity does not require opportunity to be equal for all persons. It requires only that it be equal for persons with similar skills and talents. Thus individual shares of the normal range will not in general be equal, even when they are fair to the individual. The general principle of fair equality of opportunity does not imply levelling of individual differences.\textsuperscript{14}
Thus Daniels proposes a theory which requires that health care institutions be viewed as vehicles for guaranteeing the notion of fair opportunity. Daniels does not, however, call for an unalienable right to health care. Otherwise, the expansive and expensive nature of health care needs would prevent other social goals from being addressed:

Deciding which needs are to be met and what resources are to be devoted to doing so requires careful moral judgment. The various institutions which affect opportunity must be weighed against each other.\textsuperscript{15}

Thus for Daniels any argument applying a Rawlsian scheme to a universal right to health care must be countered by the realization that to do so would seriously compromise other social goods.

\section*{Health Care Corporations as Providers}

The term "provider" is frequently used to designate the professional who exchanges his or her medical service for a fee. Similarly, institutions such as hospitals may be considered providers. One does not generally find the health care corporation, excluding those such as hospitals or health maintenance organizations, referred to as providers in the same sense as, for instance, physicians. It would appear that the absence of any personal level of service between the patient and the corporation would be the paramount reason for the exclusion of health care corporations in such categories. Pharmaceutical companies do not, for example, enter into direct relationships with consumers.
Prescription drugs are marketed directly to physicians, who then prescribe the compounds based upon the patient's particular medical needs.

Another reason for excluding health care corporations from the designation of provider would appear to be the difference between the profit motive of corporations and the fee for service concept of more direct medical exchange. Such a difference is, I believe, overstated. While one can certainly assume that the primary purpose of a corporation is to make money for its owners and stockholders, whereas the service ethos of direct medical care would presumably call for a more deontological motive, one cannot rationally conclude that the notion of corporate profit is qualitatively distinct from that of professional fees for providers. Rather, the difference is one of priority and emphasis. Medical providers must make a profit in much the same way as a corporation, even if one accepts that the fee for services rendered is secondary to the service of medical care.

Examined within this context, there appears to be no substantive difference between the concept of the corporation as provider and the physician as provider other than that of emphasis and priority of profit. If one agrees with this analysis to this point, one is faced with the issue of the ethical responsibilities of the health care corporation.

Leaving aside traditional business ethics categories such as employment practices, truth in advertising, or natural resource depletion, I shall concentrate on the relationship which the
pharmaceutical company, by virtue of its provider role, enters with patients with special needs. It is assumed that pharmaceutical companies would be subject to the same prudence and constraint as other corporations when considering the traditional categories of business ethics. My argument is that the pharmaceutical corporation's role as provider qualifies it for inclusion in the service ethos of medicine. This inclusion, coupled with the generally accepted special nature of health care in this country,17 attaches a special duty of beneficence to the health care corporation. This special duty of beneficence derives from the role of health care delivery as argued by Daniels,18 but is made operative by the acceptance of the pharmaceutical corporation as health care provider.

The notion of corporate beneficence is not inconsistent with a priority on profitability, provided one rejects Friedman's thesis. Further, since the ultimate consumer of health care services is the patient, corporate beneficence in health care invites the inclusion of a normative notion of care in policy structures.

The ethical responsibility of the pharmaceutical company to the patient with special needs is in many ways similar to the responsibility of a physician to his or her patient, although the nature of the profit motive for corporations would not allow too strong an analogy here. Both are operative within a framework of what may be described as proximate and distant goals of the respective health care provider. Specifically, these goals are profit and service. For the physician, these goals are historically
prioritized in such a way as to place a higher preference on service than on profit. In fact, the term profit is somewhat alien to the traditional physician-patient relationship. Rather, the physician's profit is contained in the fee for professional services. This does not, however, negate the fact that the physician-provider's fee for service contains a monetary consideration which will directly benefit the physician in terms of income.

The pharmaceutical company, I believe, also possesses the same proximate and distant goals. In the priorities of the corporation, however, profit considerations are prior to service. Clearly, no corporation can expect to survive without producing a profit. Even if, as a short term strategy, a pharmaceutical company diverts all of its gross profits back into research and development, leaving no net profit, the fact remains that there must be a reasonable return on investment in order to continue to introduce new drugs into the marketplace.

Such a priority on profit does not, however, negate the role of the pharmaceutical company in the service ethos of medicine. The acceptance of a pharmaceutical firm within such an ethos presupposes the acceptance of a corporation as a provider in much the same sense as one considers a physician a provider. Although one could argue against provider designation for a business whose primary purpose is to make money, I would argue that the particular health goods which are made available by the
corporation, regardless of its primary purpose, establish its role as provider.

If one looks upon the pharmaceutical company as a health provider in the same fashion as a physician provider, it would follow that ethical standards which affect the physician qua provider would likewise apply to the corporation. My contention is that pharmaceutical companies, as health care providers, have a responsibility to address the needs of special populations of patients--populations which would include the rare disease patient.

If one accepts this argument, it would appear that Milton Friedman's assertion that the only social responsibility of the corporation is to increase profits is incorrect, or at the very least does not apply to the health care corporation due to its role in the service ethos of medicine.

The rejection of Friedman's notion of profit maximization does not negate the reality of profit considerations in corporate decision making. Not only is profit prior to service, but one may also expect that the notion of service by a corporate entity could easily be influenced by motives of profit. Clearly, much of what is presented as "ethical business practice" is simply corporate egoism. Truth in advertising, affirmative action, and fair treatment of employees are examples of corporate activity which, while beneficial to others, nonetheless lend themselves to criticism of being self-serving for the corporation's long term interest. Similarly, corporate actions with a disposition toward
service, e.g., philanthropy, scholarship funds, and free or heavily discounted goods for the poor likewise serve to further the corporation's positive image and hence its overall profitability.

Corporate egoism need not be condemned too harshly. By utilizing egoistic rationale, corporations are able to consider participating in moral decision making--an exercise which would be improbable were true charity the only possibility. That is, corporate managers may more readily justify addressing issues of corporate responsibility, service to community, or sound ethical practices when such actions may be categorized as "in the best interest of the corporation."

Corporate egoism has its disadvantages, however. Any theory of ethical decision making which focuses on the "best interest of the corporation" is apt to relegate the concerns of the other party, e.g., the public, to second-order status in cases of conflict of interest. In the case of health care corporations, such teleological constructs are inconsistent with any special duty of beneficence which may be attached to the corporation--a duty consistent with the health care corporation's role in the service ethos of medicine.

I have argued that the health care corporation has a responsibility of service beyond that of profit maximization, and that corporations in general tend to develop egoistic constructs for the performance of their "ethical" duties. Applied to the plight of the rare disease patient, one can argue that such motives for action have resulted in positive steps for providing pharmacologic
therapy to these special needs cases. Drug firms are obviously aware of the need for good public relations, a positive image with physicians and others in the medical community, and cooperation with governmental agencies. However, to rely upon these ends as motives demonstrates corporate egoism, and as such does not address the duty of beneficence attached to the health care corporation.

While progress has been made in the development and marketing of orphan drugs, it is clear that much more could be done. The still large number of unsponsored compounds,¹⁹ coupled with a relatively small amount of financial assistance from governmental agencies,²⁰ demonstrates that the current system falls short of adequately addressing the needs of the rare disease patient. Change may come about within two broad categories: (1) the institutional structure which regulates prescription drug activity in the United States, and (2) the consideration of the rare disease patient as suffering individual. Chapters three and four, respectively utilizing the thought of John Rawls and Paul Ramsey, will argue that sound ethical reflection may inform the issue, and may help develop a construct in which the needs of the rare disease patient are more appropriately addressed.
ENDNOTES


5Ibid.


9Ibid., p. 22.


14 Ibid., p. 33.

15 Ibid., p. 173.

16 For an excellent treatment of these and other timely topics of business ethics, see Vincent Barry's *Moral Issues in Business* (Belmont, CA: Wadsworth, 1986).


18 *Just Health Care*, pp. 1-74.


20 Ibid.
CHAPTER THREE
JUSTICE AND THE RARE DISEASE PATIENT

As outlined in my introductory remarks, the dual problem facing rare disease patients is that the primary therapy for their diseases is usually pharmacologic, and that the marketing of pharmaceuticals is both highly regulated by the government and simultaneously controlled by profit-driven corporations. Thus the rare disease patient, by virtue of the inadequate number of persons available as either test subjects or consumers, is oftentimes unable to obtain therapy which is technologically feasible.

This lack of effective pharmaceutical compounds for the treatment of rare diseases appears prima facie to be an inequity in the system. After all, even with strict FDA regulations on the development and marketing of drugs, new agents are often approved for more common diseases. In fact, there are currently (1989) over fifty different chemical entities approved for use in hypertension.¹ The rationale for such proliferation is quite simply free market economics; there are approximately sixty million persons in the United States with hypertension.² Compared to the relatively miniscule number of persons suffering from most rare diseases, one can easily understand that managers and stockholders of pharmaceutical companies would be more inclined to pursue the market for hypertension rather than that for a given rare disease.
What is particularly disturbing to the rare disease patient is the fact that effective remedies often already exist. In such cases, the issue is not so much a matter of massive research efforts and expenditures as one of a lack of means to bring the compound to market. The only means to market a pharmaceutical compound in the United States is via a drug company—a means which requires substantial money and commitment from the corporation even though the compound may already have been proven reasonably safe and effective.

Inequalities in health care delivery are prevalent in many forms and are of interest to persons not directly involved. For instance, substandard prenatal care is both a political and a medical concern. Persons with private insurance or health maintenance contracts can generally count on receiving better quality and a larger quantity of health care services than the indigent. Examples such as these need not be entirely blamed on the free market system; "socialized medicine", while providing a more comprehensive medical support structure for all citizens, may nonetheless encourage inequalities in the delivery of services. For example, specialists are far more accessible to urban dwellers than to rural inhabitants. Further, the unpredictable nature of health care needs assures that health status itself can never be guaranteed to be equitable in distribution.

The question ethics asks is "When are inequalities unjust?" Since our concepts of injustice are generally framed within the
context of specific theories or principles of justice, one must consider whether such theories provide justification for given inequalities. If the inequalities in drug distribution which prevent the special needs of the rare disease patient from being met are justifiable within a general theory of justice, one may not then classify these inequalities as injustices. On the other hand, inequalities which violate a general theory of justice demand critical review and correction.

**Choosing a Theory**

This analysis will utilize John Rawls' theory of justice as fairness as a "test theory" for the justification of inequalities in the development and distribution of pharmaceutical compounds. This is not to negate the possible relevance of other theories, e.g., utilitarianism or libertarianism. For instance, utilitarian principles are often invoked in health policy schemes, particularly those involving resource allocation. Similarly, Nozick has cogently argued for a libertarian notion of health care delivery. However, one must appreciate that the marketing of pharmaceutical compounds takes place in a highly regulated market--perhaps regulated more than any other industry. For a theory of justice to be adaptable to the plight of the rare disease patient, it must address the nature of these regulatory mechanisms. As our discussion of Rawls unfolds, the relevance of his theory of justice as fairness will be clear.
Rawls and Health Care

Further justification for the use of Rawls' theory of justice as fairness may be found in the historical use of Rawls by health policy scholars. While this thesis is focused on the special needs of a disadvantaged subgroup of patients as opposed to health care in general, a brief analysis of Rawlsian applications in health care will provide useful background material. This background will prove helpful in further analyzing the concept of justice and the rare disease patient.

In Chapter one I outlined Rawls' theory of justice as fairness, by which he argues that it is possible to determine an ahistorical method of choosing principles by which societal conflicts may be resolved. For ease of reference, Rawls' two principles of justice are restated here:

1. Each person is to have an equal right to the most extensive total system of equal basic liberties compatible with a similar system of liberty for all.
2. Social and economic inequalities are to be arranged so that they are both:
   (a) to the greatest benefit of the least advantaged, consistent with the just savings principle, and
   (b) attached to offices and positions open to all under conditions of fair equality of opportunity.4

Rawls avoids, or at least does not directly address, the application of his theory to health care issues. Other scholars, however, have attempted to apply a Rawlsian perspective to health care delivery, usually within the context of health care rights.5
The notion of a basic right to health care is asserted in the preamble to the constitution of the World Health Organization:

The enjoyment of the highest attainable standard of health is one of the fundamental rights of every human being without distinction of race, religion, political belief, economic or social condition.6

Further, the United Nation's Universal Declaration of Human Rights declares:

Everyone has the right to a standard of living adequate for the health and well-being of himself and of his family, including, food, clothing, housing and medical care and necessary social services.7

One would be somewhat hard pressed to define these statements in workable terms. For instance, what does "the highest attainable standard of health" mean? Is one to assume that all persons are to receive the same health care allocation? Does "health" refer to perfect health or a reasonable compromise between disease and normal functioning? Should those less fortunate on the socio-economic scale share in the same dollar-for-dollar allocations for health care as the wealthy, regardless of their respective health statuses?

In addressing these and other questions, ethicists, philosophers, and others interested in health policy have looked to the applicability to health care of Rawls' concept of justice as fairness. As alluded to in Chapter one, such an application depends in large measure on how one views the priority of health care within the Rawlsian scheme of justice. An analysis of various invocations of the use of Rawls' theory in issues of
resource allocation as well as the right to health care reveals three basic categories of thought:

(1) Health care may be perceived to be a primary social good, and thus a right to health care could be argued along the same lines as a right to equal basic liberties; (2) Health care institutions may be considered as background institutions charged with providing fair equality of opportunity; and (3) Health care services may be viewed as goods available for purchase by one's individual shares of the social product.  

The first strategy is best represented by the argument forwarded by Ronald M. Green in "Health Care and Justice in Contract Theory Perspective." Green argues that health care should be considered a primary social good along with rights and liberties, powers and opportunities, and income and wealth. Due to the priority of health, Green argues, contractors in the original position would "opt for a principle of equal access to health care; each member of society, whatever his position or background, would be guaranteed an equal right to the most extensive health services the society allows." Thus Green calls for the establishment of a third principle of justice to be added to the Rawlsian scheme--a principle guaranteeing the right of equal access to health care. This principle would of necessity be ordered lexically prior to the difference principle, as "contract parties could be expected to separate access to health care from income considerations."
In a cogently argued rebuttal, John Moskop challenges Green's concept of a third principle of justice. Arguing that such a notion would require substantive changes in the Rawlsian scheme, Moskop asserts that the resultant violation of the integrity of justice as fairness would lead to a "slippery slope" effect in which principles specifically addressing other basic needs would have to be formulated as well.13

Green's theory, and to a lesser extent Moskop's criticism, eschews the fact that Rawls clearly addresses the status of health care as a good. In the same paragraph as his definition of primary social goods, Rawls assigns to health the status of "natural good," i.e., "although their possession is influenced by the basic structure, they are not so strongly under its control."14 Thus Rawls himself, while not directly addressing health care allocation, nonetheless appears to rule out the inclusion of health as a primary social good. Green's notion of a third principle of justice, while undoubtedly appealing to those with special health care needs, would appear to be a contradiction of the Rawlsian scheme rather than an addition to it.

The second strategy, which calls for the inclusion of health care institutions in the list of background institutions charged with guaranteeing fair equality of opportunity, is represented by the thought of Norman Daniels. In Chapter two I explored Daniels' assertion that health care is best viewed as a facilitator of an individual's opportunities. In summary, since illness and injury restrict what Daniels refers to as "normal species functioning,"15
it follows that acts and institutions which work to alleviate these contingencies are morally prior to acts and institutions designed for pleasure or voluntary consumption.

The third strategy of applying Rawls to health care involves the notion of health care services as goods available for purchase. Proposed by Charles Fried in *Right and Wrong*, this strategy relies heavily on the notion of the uncertainty of health care needs. Some individuals live long and complete lives unencumbered by serious accident or illness. For these persons, a basic minimum of health care would be sufficient to address their needs. There are others, however, who need constant medical attention from birth.attention that requires far greater resources than are available in any conception of a basic minimum.

Arguing that one may purchase insurance to cover any unexpected health care contingency, Fried claims that the risk of special needs would then be spread among the participants. The resulting health care rights claims would only be applicable for services for which one was prudent enough to purchase insurance protection.

Although he includes unlikely health contingencies in his theory, which would presumably include rare diseases, Fried does not address the issue of market failure—an issue central to the plight of the rare disease patient. Fried assumes a certain availability of health care goods, and constructs a distributional theory around them. In the case of the rare disease patient, however, this assumption renders his theory incomplete.
None of the three strategies which have been utilized in the application of Rawls' justice as fairness model to health care delivery provide clear insight into the problems confronting the rare disease patient. These applications, while addressing the positioning of health care within the Rawlsian scheme of justice, do not address the specific allocational issues in contingencies of medical scarcity or special needs--contingencies which must be considered if one is to address the needs of the rare disease patient.

The failure to derive a suitable Rawlsian rights-based scheme for aiding the rare disease patient's quest for pharmacologic therapy does not eliminate any usefulness of a Rawlsian construct in such cases. The crux of the problem in applying Rawls to concrete problems of health policy lies in the failure to distinguish allocative justice from distributional justice. Allocative justice is concerned with the distribution of goods and services to historical individuals with identifiable values and goals. Distributional justice includes the notion of allocative justice, but also incorporates the concept of background institutions which produce and regulate these same benefits and burdens--a concept which Rawls distinguishes as social justice.

Rare disease patients are known historical figures, and as such their needs would appear to be better addressed by distributional theories which stress allocational justice rather than by the Rawlsian scheme of social justice. Rawls himself
states that his two principles do not apply directly to allocational schemes:

If it is asked in the abstract whether one distribution of a given stock of things to definite individuals with known desires and preferences is better than another, then there is simply no answer to this question. The conception of the two principles does not interpret the primary problem of distributive justice as one of allocative justice.\textsuperscript{19}

For one to appreciate the Rawlsian notion of justice as fairness as applicable to the specific needs of known individuals, one must look toward a more basic concept than the material allocation of goods and services. Rawls can be helpful in an allocational analysis, but only within the framework of what he terms the "basic structure"\textsuperscript{20}—a contingency which has applicability to special needs cases by establishing and maintaining institutional constructs which produce and regulate the goods to be distributed.

The basic structure is the primary subject of Rawls' notion of justice, and is represented by the background institutions which perform the above-mentioned production and regulation of benefits and burdens:

For us the primary subject of justice is the basic structure of society, or more exactly, the way in which the major social institutions distribute fundamental rights and duties and determine the division of advantages from social cooperation. By major institutions I understand the political constitution and the principal economic and social arrangements. Thus the legal protection of freedom of thought and liberty of conscience, competitive markets, private property in the means of production, and the monogamous family are
examples of major social institutions. Taken together as one scheme, the major institutions define men’s rights and duties and influence their life-prospects, what they can expect to be and how well they can hope to do.\textsuperscript{21}

Rawls admits that the concept of the basic structure is vague, and that the justification for including any given institution is often unclear.\textsuperscript{22} While not overly concerning himself in \textit{A Theory of Justice} with the unclarity of the notion of the basic structure, Rawls squarely addresses the issue in a later work. "The Basic Structure as Subject" was written in an effort to further explicate Rawls' notion of the basic structure.\textsuperscript{23}

Central to Rawls' basic structure scheme is the supposition that any distribution of goods is just only if the structure of the distributional system is fair.\textsuperscript{24} Using voluntary market exchange as an example, Rawls emphasizes that it is only within the framework of the basic structure that society can expect to correct inequities. According to Rawls, restrictive rules on individual agents are insufficient to effect the necessary corrections, as individuals are either incapable or unwilling to see the significance of their failure to make the corrections. Thus the basic structure itself is seen by Rawls as the construct which "should make the corrections necessary to preserve background justice."\textsuperscript{25}

The notions of regulation and correction are critical in Rawls' conception of the basic structure. It is not enough to establish just background institutions; these same institutions must constantly be reviewed, criticized and changed. Society is
an ongoing process, and a just institution may well be unjust for the next generation.\textsuperscript{26}

My thought thus far has attempted to lead to the thesis that a Rawlsian interpretation of justice, provided that one does not concentrate only within an allocational perspective, is useful in an analysis of the special needs of the rare disease patient--an argument which is also valid when considering other disadvantaged groups, e.g., the retarded or handicapped. I will now look to a more specific examination of Rawls which will provide a framework for viewing the plight of the rare disease patient within the context of the basic structure.

Specifically, there are two components of the basic structure, market economy and government, which apply directly to this analysis. It is evident that Rawls favors a voluntary market economy, with one notable albeit predictable qualification. A voluntary market is fair only if there has been a fair antecedent distribution of goods and opportunities.\textsuperscript{27} Assuming that the requisite background institutions are just, Rawls points to two principal advantages of a market system. First, a voluntary market system is more efficient than other systems of rationing, although in reality never actually achieving strict efficiency as defined by classical economic theory. Nevertheless, a voluntary market does approach the theoretical model of efficiency.\textsuperscript{28} Second, and probably more important, a voluntary market is seen by Rawls as being consistent with his first principle (equal liberty) as well as part (b) of his second principle (fair equality of
opportunity).\textsuperscript{29} Freedom of choice in careers and occupations, as well as the decentralization of economic power, point to the conformity of a voluntary market to the Rawlsian scheme.

In additional to a voluntary market structure, Rawls also calls for an intricate scheme of government. The government is to be designed in such a way as to guarantee just distribution of goods and services--in other words, in accordance with the two principles of justice.\textsuperscript{30} Rawls proposes four distinct branches of government, each consisting of "various agencies, or activities thereof, charged with preserving certain social and economic conditions."\textsuperscript{31} The allocation branch, which has the most direct applicability to my analysis, serves to prevent "unreasonable market power"\textsuperscript{32} and to insure healthy competition. Further, this branch is charged with the task of correcting market failures which occur whenever prices do not efficiently measure social costs and benefits. In cooperation with the stabilization branch, which attempts to insure full employment, the allocation branch is also responsible for maintaining the general efficiency of the market.\textsuperscript{33}

The transfer branch considers needs and how they relate to other claims, and regulates the distribution of needs-based claims in the form of suitable minimums.\textsuperscript{34} Finally, the distribution branch functions as a means of insuring justice by a scheme of taxation and regulation of property rights.\textsuperscript{35}

Although all four branches of government proposed by Rawls work to insure the goals of the two principles of justice, the
allocation branch, with its responsibility to correct market failures, is the most helpful in this analysis. A market failure may be defined for our purposes as any contingency in which a free market either is unable to respond to traditional supply and demand factors or is unable to address the objectives of society above and beyond market efficiency. An example of the latter type of market failure is the notion of public goods, or goods and services which meet the common interests of all citizens. The first example of market failure may be represented by utility systems, which would be too expensive and cumbersome to employ if there existed several different systems marketing the same service in a given community. In the case of a market failure, there is frequently but not always justification for governmental intervention to correct the imbalance.

The difficulties faced by the rare disease patient in obtaining suitable pharmacologic therapy stem primarily from market failure. The demand for treatment of a given orphan disease is by definition too small to be commercially profitable. One could argue that the same is true for certain colors of houses, and that since there is no justification for governmental intervention in color schemes for houses, there can likewise be no justification for governmental intervention in the free market distribution of pharmaceutical compounds. This analogy breaks down, however, when one considers that the choice of color for one's house is unencumbered by government regulations, whereas the case of orphan drugs has at its core the contingency of a
federal agency requiring extensive, albeit necessary, safety and efficacy tests before authorizing the marketing of any pharmaceutical compound. One can find someone to paint one's house, but a patient with a rare disease may only turn to the heavily regulated pharmaceutical industry for assistance. In addition, the notion of suffering, a concept absent in more common examples of market failure, invites intervention in the market failure affecting the rare disease patient. Further, all this says nothing about the special nature of health and health care--issues previously addressed in Chapter two.

Rawls does not propose that his theory be used to require equal access to any goods. The basis of his thought is that background institutions comprising the basic structure of society be formulated in such a way as to insure a just process of distribution. My analysis now shifts to the question of whether the two components of the basic structure that are particularly relevant to this study, voluntary market and government satisfy the requirements of the Rawlsian notion of justice as fairness. It would appear that the American free market scheme of distributing pharmaceuticals would be in accord with Rawls' view that a voluntary market, via its efficiency and consistency with the notions of equal liberty and fair equality of opportunity, is indeed consistent with the implications of the two principles. This argument is strengthened by Rawls' inclusion of health as a primary good, and the fact that the benefits which are to redound to the least advantaged are likewise to be considered as
primary goods. One could argue that Rawls' classification of health as a natural primary good rather than as a societal primary good would preclude the consideration of health care institutions as components of the basic structure. However, beyond distinguishing between social and natural goods as a matter of classification, Rawls does not differentiate between the two regarding their applicability within his theory.

The government's involvement in the free market distribution of pharmaceuticals is primarily that of the Food and Drug Administration. Charged with insuring the safety and efficacy of drugs considered for marketing in the United States, the FDA is essentially a protective and regulatory agency. It is the extensive testing of pharmaceutical compounds that results in the high cost of drug development—a factor partly responsible for the unprofitability of orphan drugs. Thus the government's intervention in the market, which most would agree is warranted, nonetheless results in an increased burden on the rare disease patient. This burden does not, however, negate the necessary protective function of the FDA—a function that helps insure, in the words of Daniels, "normal species functioning." Chapter five will directly address government's response to the dilemma confronting the rare disease patient, including adjustments in the drug development and approval processes.

My criticism of those who propose a rights-based Rawlsian analysis of health care schemes has led to an examination of the applicability of the basic structure to the notion of health care
delivery for those with special needs. It appears evident that Rawls' philosophy does indeed provide insight into the plight of the rare disease patient, but only from the perspective of the basic structure of society. The free market distribution of pharmaceuticals, as well as the protective function of the government (FDA), appears to be consistent with Rawls' notion of just background institutions.

A caveat is in order whenever one argues for the inclusion of Rawls' theory in any analysis of a concrete situation. There is a temptation to view background institutions as rigid and static. Rawls emphasizes, however, that the basic structure is best viewed within the context of an ongoing social process—a notion that reminds us to insist on constant review, criticism and change.

The particular components of the basic structure which apply to the development and marketing of orphan drugs, i.e., government and voluntary market, must remain just in order to facilitate fair distribution of pharmaceuticals to rare disease patients. Thus the thought of John Rawls, by providing a mechanism for the development and maintenance of just background institution, informs the issue at hand.

A Rawlsian construct does not, however, address the issue of duty to one's fellow man—a duty, as argued in Chapter two, born from the role of the health care corporation as provider. Medical practitioners have defined this duty in several ways, e.g., rendering services or providing care. The notion of care, while
more frequently eliciting a vision of direct attention to the clinical needs of the patient, may also be considered in the broader sense of a warrant prescribed for the medical profession—a warrant which helps determine the ethical validity of decision and acts. Such a notion of care has been described by Paul Ramsey, and is particularly useful in medical ethics. Chapter four will explore Ramsey's notion of care and its applicability to issues of scarce medical resources.
ENDNOTES


6Constitution of the World Health Organization (Official Record--WHO 2, 100).


10Ibid., p. 117.

11Ibid., pp. 121-123.

12Ibid., p. 118.

14*A Theory of Justice*, p. 62.


19Ibid., p. 88.

20Ibid., pp. 7-11.

21Ibid., p. 7.

22Ibid., p. 9.


24Ibid., p. 160.

25Ibid.

26Ibid., p. 161.

27*A Theory of Justice*, pp. 270-274.

28Ibid., p. 272.

29Ibid.
30Ibid., pp. 274-284.

31Ibid., p. 275.

32Ibid., p. 276.

33Ibid.

34Ibid.


37Ibid., pp. 308-310.

38A Theory of Justice, p. 62.

39Ibid.


41The Basic Structure as Subject, p. 164.
CHAPTER FOUR
THE RARE DISEASE PATIENT

The rare disease patient faces many difficulties unknown to sufferers of more common diseases. The reluctance of pharmaceutical companies to develop and market orphan drugs, coupled with strict federal regulations which limit access to investigational compounds, creates a scenario which exacts a heavy toll on the humanity of the rare disease sufferer.

Chapter four will provide a description and analysis of the human needs of the rare disease patient as suffering individual. In addition, this chapter will argue for the relevance of theological ethics in helping to explicate and remedy specific disadvantages faced by the rare disease patient. Specifically, this chapter will begin with an examination of the subtopics (1) The Rare Disease Patient as Individual Sufferer and (2) The Rare Disease Patient and the American Drug Approval System. Following a description and analysis of each category will be a further study of the role of theological ethics in clarifying and solving these contingencies. For this analysis I shall depend upon the thought of Paul Ramsey, who maintains that the Christian notion of love for one's neighbor is the supreme moral principle.

The Rare Disease Patient as Suffering Individual

The plight of the rare disease patient spans two separate, but overlapping and interconnected, fields of health care, i.e.,
research and treatment. Although medical research and treatment are interrelated, including the possibility of being simultaneously operative in the same patient, the concepts represent two distinct paradigms in our health care system.

The Hippocratic model of medical treatment is based on the principle of beneficence, albeit with strong paternalistic connotations. The physician's duty to care for the sick has been historically viewed within the context of a one-to-one relationship with the patient. The Hippocratic Oath does not specifically promote service in the interest of the common good, although it must be pointed out that issues such as public health and medical experimentation were essentially unheard of at the time.¹

Although rare disease patients experience frustration in the frequent delays of their diagnoses, and at times even a failure by the treating physician to suspect a rare disease, their primary concern is not the physician-patient relationship. Rather, it is the exploration for and development of new therapies, coupled with regulatory assistance or at least a minimum of regulatory hindrance, which offers the most promise to the sufferer of a rare disease.²

Medical research presents perhaps the most useful model of utilitarianism in the health care environment. From the scientist's bench to the final testing of an agent in humans, the priority of pharmaceutical research is to develop a compound that will maximize a therapeutic benefit to an appropriate patient
population. Certainly, a pharmaceutical company must consider the commercial ramifications of any agent under consideration for development. Basic disease research, e.g., animal-model studies in a university environment, while not as directly affected by commercial considerations, nonetheless possesses a bias toward more common diseases. More federal research dollars are available for diseases for which a treatment would benefit larger rather than smaller numbers of patients. This is coupled with the desire of most scientists to pursue projects that will generate a reasonable amount of publicity for themselves and their institutions. This latter point is not totally a reflection on egotistical or personal considerations; rather, greater publicity generally leads to additional funding and other support for the researcher and his or her institution.

Similarly, a parallel may be drawn between utilitarianism and pharmaceutical marketing principles, although the latter is clearly more aligned with maximizing sales and profits than with producing a greater aggregate good for persons. A free market allows, and in fact market forces require, that the large share of a corporation's effort is directed toward achieving a maximum amount of sales for a specific market. This is of course true in any market, whether it be clothing, books, or pharmaceuticals. Thus pharmaceutical companies, by virtue of their profit-driven decision mechanisms, look to develop compounds which will first of all fill a market need and in addition provide at least a reasonable opportunity of meeting enough of that need to generate
a profit on the investment required for the development of such compounds.

Thus the rare disease patients find themselves in the quandary of living within a system which depends upon utilitarian principles in both medical research and pharmaceutical marketing. The disadvantage of such paradigms is that the needs of the individual sufferer are not addressed, or are at best relegated to a second order status behind the common good. Rare disease patients find this predicament particularly disturbing, as the utilitarian concerns of research and marketing create a scenario in which rare diseases are not only inadequately researched but also generally ignored in the marketing planning of pharmaceutical companies. It is within this context that rare disease patients and their advocates have lobbied for recognition as persons with special needs rather than as unfortunate "victims."\(^3\)

The Rare Disease Patient and the Drug Approval System

As described in my introductory remarks, the primary potential or existent therapy for most rare diseases is pharmacologic. The intricacies of the American drug approval and marketing systems, and how these relate to the special needs of the rare disease patient, are thus relevant to this analysis.

The American drug industry was essentially unregulated before 1938. Previously, federal laws designed to ensure safety and truth in advertising were either ignored or too difficult to enforce. For instance, the Pure Food and Drugs Act of 1906 was
enacted to prohibit false and misleading claims about food and drug products. Referred to as the Wiley Act, after the Agriculture Department's chief food chemist from 1883-1912, this legislation was doomed to failure after a 1911 Supreme Court ruling which allowed false or unsubstantiated claims so long as the food or drug package listed the correct ingredients present in the product. Consequently, the Sherley Amendment was passed in 1912, which specifically prohibited false and fraudulent labeling claims. However, this legislation was essentially ineffective, as fraud was difficult to prove.4

With the development of sulfanilamide in 1932, it was becoming apparent that a new age in drug discovery was dawning—an age in which science rather than hucksterism was the mainstay of drug research and development. The sulfanilamide derivatives, still known today as the "sulfa drugs," provided the first effective remedy against a wide range of infectious diseases.

In spite of the promise held by sulfanilamide, tragedy was soon to strike. Because the powder form of the drug was difficult to measure and left a bad taste, a liquid form of the drug was developed. Only after 100 people, mostly children, had died as a result of ingesting the new formulation did authorities at the American Medical Association discover that the vehicle used for the dissolution of sulfanilamide was diethylene glycol, a substance which converts to fatal oxalic acid when consumed.
Sulfanilamide elixir was never tested in animals, nor were there regulations requiring such testing.\textsuperscript{5}

The Food, Drug, and Cosmetics Act of 1938 was born out of the sulfanilamide disaster. The act empowered the FDA, then a constituent agency of the Department of the Agriculture, to require warnings on labels, ban dangerous substances, and enforce its regulations. The act also required the manufacturer to submit evidence that a drug was safe, although the FDA was charged with proving that the drug was unsafe in order to prevent its marketing.\textsuperscript{6}

The period between 1938 and 1962, while not seeing any dramatic changes in the drug approval process, did witness considerable growth in the technology necessary for new drug development. Antibiotics such as penicillin and streptomycin were discovered, and it appeared as though the United States was indeed entering a new era of medicine. It would take another tragedy to effect any substantive change in the way pharmaceutical compounds were approved for marketing.

A drug with the generic name thalidomide was developed and marketed throughout much of the world in the 1950s. Although never marketed in the United States, the drug was available to more than 1,200 American physicians as an experimental agent. Marketed as a hypnotic and sedative, the drug was frequently administered to pregnant women. Unfortunately, thalidomide was discovered to cause the teratogenic effect of phocomelia, which is characterized by absent or severely underdeveloped limbs.
The manufacturer of thalidomide, Chemie Grunenthal of West Germany, withdrew all dosage forms and strengths of the drug when faced with the alarming data about its effects. However, the experimental supplies which had been shipped to American physicians were difficult to track down. Pharmaceutical firms were not required to keep detailed records on the shipment of experimental drugs, and the physicians using such drugs essentially had only to sign a statement verifying that they were competent to dispense the drug. There were no regulations requiring that patients given thalidomide be informed as to its experimental status.\(^7\)

The thalidomide tragedy was averted in America because of the efforts of FDA staffer Frances Kelsey, who insisted on additional safety data on thalidomide before approving the drug for marketing in the United States.\(^8\)

Essentially due to the widespread concern evoked by the thalidomide tragedy, as well as the desire to prevent such an occurrence in the United States, Senator Estes Kefauver launched a campaign for drug safety and efficacy. In 1962, the Kefauver-Harris amendments to the Food, Drug, and Cosmetic Act became law. Passed unanimously, the Kefauver-Harris amendments changed the complexion of the American pharmaceutical industry. Essential features of the legislation were the requirement that the efficacy of a drug must be established by the manufacturer, and that the Food and Drug Administration was required to act on all approval applications. Additional safety parameters were
instituted by the Kefauver-Harris amendments, which served to strengthen the safety requirements of the 1938 Food, Drug and Cosmetic Act.9

The Kefauver-Harris amendments, although modernizing the drug approval process and doubtless saving thousands of lives, nonetheless had the effect of placing the responsibility for extensive safety and efficacy testing directly on the pharmaceutical industry.

The present drug approval system in the United States reflects the regulatory attitude of keeping unsafe and inefficacious drugs off the market. Few, including rare disease patients, would argue the priority of safety and efficacy. No one-patients, physicians, or industry--wants another thalidomide to be administered to humans. However, the drug approval process works against the rare disease patient in two distinct ways. First, the FDA has historically involved itself entirely in regulatory issues in a free market--a stance which prevents the agency from having any prospective input into which drugs are developed. This is neither the fault of the FDA nor within the agency's power to change, as the regulatory function of the FDA is mandated by Congress. Second, our drug approval system demands extensive testing of any pharmacologic agent in an elaborate system of basic animal research coupled with extensive human testing. This poses no problem for diseases for which large patient pools exist, beyond the aggravation of administrative processes and delays which are to be expected in so cumbersome a
process. But in the case of rare diseases, adequate numbers of available test subjects are not available. Thus, leaving aside the issue of needing a pharmaceutical company to sponsor the development of an orphan drug, there exist orphan diseases which do not provide a large enough pool of patients to scientifically determine safety and efficacy of the compound in question.

Rare disease patients acknowledge and accept the existence of drug regulations as necessary, as it is clearly in their best interest to be protected against unsafe or inefficacious drugs. Their concern is that drug regulations are inflexible and applied to orphan diseases in the same manner as to common diseases. In this context, critics have claimed that the drug approval process lacks a humanitarian component.

The Response of Theological Ethics

One must proceed with caution when attempting to apply theological insight to concrete problems, whether they be societal, medical, or otherwise. The primary source for this section of my analysis, Paul Ramsey, warns against the use of religion to prescribe specific social policy. A central aim of this thesis, however, is to demonstrate that various categories of the broad field of ethics, including those derived from theological analysis, are well suited for an understanding of the plight of the patient with a rare disease. Henceforth, this section of the analysis will proceed not with the presupposition that theological ethics may offer a definitive answer to a rather specific medical
resource allocation question, but rather with the acceptance of Shriver's notion that:

The Hippocratic oath suggests that service to any person in need is more important than payment for the service; this is a professional presupposition that links medicine at once with those traditions in religion that sanction unselfish love as the highest motive of human relationships.\(^{11}\)

The remainder of this chapter will focus on Ramsey's notions of love and service--notions derived from his distinctively Christian ethical vision.

Paul Ramsey's first major work, \textit{Basic Christian Ethics}, forwards the notion that concern for one's fellow man is best represented by the notion of agape, or unselfish love for one's neighbor. Ramsey begins his discourse with a critical look at the distinction between the righteousness and love of God and the reign of the Kingdom of God. In contrast to Brunner, Ramsey relies on the biblical conception of righteousness rather than on moral norms derived from reason.

Ramsey describes Jesus' admonition concerning anxiety to be grounded in his conviction concerning the Kingdom of God. Eschatological predictions of Jesus, as exemplified by the Beatitudes, are interpreted by Ramsey to indicate Jesus' contention that the Kingdom of God was coming regardless of man's readiness or lack thereof. This eschatological reference is stressed by Ramsey to have an enormous qualifying impact on Jesus' ethical teachings; grave doubt is expressed concerning the
validity of lifting the ethical teachings of Jesus from their original context. One may assume that it is this doubt which causes Ramsey's hesitation to directly apply Jesus' ethical teachings to concrete social problems.

Transferring Jesus' ethics from apocalyptic to non-apocalyptic terms is essential, as we live in a society in which responding to the needs of a single individual in isolation is to ignore the competing claims of others. Ramsey's thought thus appears to parallel a major concern of the rare disease patient, i.e., the recognition of individual needs in an environment in which others have needs as well.

One could well argue that Jesus' instructions for love of neighbor are invalidated because of their apocalyptic origin. However, Ramsey reminds us that "genesis has nothing to do with validity,"

and that the worth of agapaic love for neighbor is far superior to any "miscalculation of time" on the part of Jesus. Rather, the ethical teachings of Jesus are relevant precisely because of their effective focus on the immediate neighbor-needs of individuals apart from the competing claims of others.

In yet another example of addressing the needs of the individual, Ramsey states that we are not to confuse love of neighbor for love of mankind in general. The former encourages love of neighbor for his own sake, and not for the sake of a more global disposition. Love for neighbor for his own sake "insists upon a single-minded orientation of a man's primary intention toward this individual neighbor with all his concrete needs."
Love for neighbor for his own sake evolves into a universal love, since any person may be the neighbor. However, any universality is implicit rather than explicit, as would be the case for love of mankind in general.\(^\text{18}\)

Ramsey's notion of agape must be recast into more practical terms if one is to discover usefulness in its application to historical medical contingencies, whether clinical or allocational. This translation is accomplished by Ramsey's use of agape as analogous to the concept of care for the patient. Agreeing with the Barthian notion that "covenant-fidelity is the inner meaning and purpose of our creation as human beings,"\(^\text{19}\) Ramsey, in *The Patient as Person*, proposes that the field of medicine is one expression of the covenant relationship which men are called to practice with one another. Although *The Patient as Person* may be described as a rather segmented compilation of Ramsey's thoughts on several different issues, the recurrent theme of the entire volume is that of recognition of the integrity of one's fellow man.\(^\text{20}\)

Ramsey's medical ethics makes clear his supposition that agape calls for obligation to one's neighbor, and not any sense of social usefulness, to be the starting point for ethical reflection.\(^\text{21}\) Further, it is clear that Ramsey holds covenant-faithfulness, in the form of duty to care for one's fellow man, to be morally prior to any consideration of consequences:

In the language of philosophy, a deontological dimension or test holds chief place in medical ethics, beside
teleological considerations. That is to say, there must be a determination of the rightness and wrongness of the action and not only of the good to be obtained in medical care or from medical investigation."\textsuperscript{22}

Ramsey's self-description as an "ethicist of principles,"\textsuperscript{23} and therefore not a consequentialist, must not be viewed as an attitude confined to his later work within the field of medical ethics. In Basic Christian Ethics, for example, Ramsey asserts that any good which results from an act, other than the primary good for the neighbor's sake, is to be regarded as a "quite unintended by-product."\textsuperscript{24} A further, much stronger argument against the consideration of consequences is found in Deeds and Rules in Christian Ethics, in which Ramsey proclaims that "the reduction of Christian ethics to teleology is nearly the same thing as abandoning it."\textsuperscript{25}

Ramsey, responding to criticism that his notion of covenant-care is a movement away from the more basic notion of agape, and is related to his interest in medical ethics, refers his critics to the final chapter in Basic Christian Ethics in an effort to establish the priority of covenant even in this early work.\textsuperscript{26} However, a careful reading of Ramsey's notion of community reveals this concept much earlier than in his closing chapter.

Paul Ramsey directly addresses the issue of community in Basic Christian Ethics. While economists would argue that communities are essential for the exchange of services, Ramsey asserts that community is essential in the realm of spirituality as well. The primary difference between the two is that an
economic community allows men ultimately to seek only their own good, whereas spiritual community requires that the good of the neighbor be sought.

Besides Christian love, Ramsey describes two concepts which may be applied to the creation of community among persons. One may opt for either a self-centered or value-centered paradigm. Ramsey's self-centered paradigm is best represented by Jeremy Bentham, who assumed that by living under the law and its attendant threats of retributive justice one would be forced into living within the best interest of society at large. By calculating the price to be paid for violating legal and social sanctions, the individual is more apt to contribute to the good of society. That the individual's contribution is entirely self-centered does not preclude the possibility that positive acts will be taken. On the contrary, the individual will contribute positively out of the expectation of his own good being enhanced.

On the other hand, one may look to a more value-centered approach to the derivation of community duties. Using Mill's utilitarianism as an example of a value-centered approach, Ramsey argues that such an assertion is as naive as it is confusing. For such an argument as Mill's ignores Ramsey's basic question of Christian ethics, i.e., whose good it shall be when a choice must be made.

Ramsey does not allow self-centered or value-centered derivation of duties to be primary in the creation of community among men. Rather, he reaffirms his notion of concern for the
neighbor for his own sake as the primary impetus for creating such community. Spiritual community never springs from love of value or love of self.

Ramsey structures his thought on community by asserting that there are two categories of community—those that already exist and those that need creating. Education plays a major role in preserving an already existing community of men. This education is basically in the form of showing the individual his own interest in the preservation of a community of mutual interest.

On the other hand, the creation of community where none exists poses quite a different problem. Unlike the case of the already existing community, enlightened self-interest is of no value in the creation of community. Likewise, principles of distributive justice are of little value when no community of mutual interest exists.²⁷

It is at this juncture that Ramsey solidifies his thesis that it is the work of Christian love that creates community among men. Christian love enters the realm "where dwell the desperate and the despised outcasts from every human community, and brings community with them into existence."²⁸

With his insistence that the creation of community is the special role of Christian love, Ramsey does not negate the importance of such love in the preservation of community. Referring to love as the primary ingredient for the preservation of community, Ramsey allows that the ingredients of self-centeredness and mutual interest are also operative.
For Ramsey, the creation and preservation of community among men is analogous to the recognition of another human being's worth. Thus Ramsey credits love with furthering the technological revolution in the middle ages by advancing the notion of human dignity. Machinery relieved slaves of many of their most difficult duties. Here we see the most concrete example of Ramsey's notion of the interrelatedness of economic and spiritual communities.29

Ramsey asserts that agape is concerned only with the second question of Christian ethics, i.e., whose good it shall be when a choice must be made.30 His notion of agapaic care for the patient as individual, which has its origins in his earlier writings but finds its fullest expression in his treatment of medical ethics, affords support for a framework which recognizes the special-needs case. Further, Ramsey's notion of community as the result of love practiced among men serves as a useful model for cooperative relationships between fellow sufferers--relationships which are indispensable for the rare disease patient.

It would therefore appear reasonable to appeal to Ramsey's thought for guidance in allocational issues such as drugs for the rare disease patient. Ramsey himself, however, generally eschews such issues. When Ramsey does broach an allocational question, it is within the framework of the priority of life-saving techniques rather than the more common contingencies frequently faced by policy makers and providers.31 Drugs for rare disease patients, which are unavailable due to commercial considerations
affected by governmental regulations, would appear to meet any reasonable test for classification as scarce resources. Such resource questions may only be addressed within the context of social and medical priorities.

On two different occasions, however, Ramsey refers to questions of medical and social priorities as "incorrigible to moral reasoning"\textsuperscript{32} as well as to "rational determination,"\textsuperscript{33} concentrating instead on the distributional issues of scarce life-saving techniques. One may presume that Ramsey's reluctance to enter policy disputes results from his thesis that the primary concern of ethics is not what the good actually is, but rather whose good it shall be. In order to effect policy decisions, Ramsey would presumably need to concern himself more with the question, "What is the good?" This argument, I believe, gives short shrift to the question "Whose good shall it be when a choice must be made?" by neglecting the impact this question has on relationships between parties—a notion which will be addressed in Chapter five.

Ramsey's avoidance of the establishment of medical priorities fails to account for the need for care for those with special requirements—the very patients most affected by issues requiring governmental intervention in allocation schemes. Rare disease patients need to be viewed and treated as individuals. Ramsey, although not directly addressing these patients, cogently argues that this can and should be done with patients in general, and prescribes a method for agapaic care of persons in need of
medical treatment. In this respect, Ramsey's thought does indeed appear useful in macroallocational issues.

While I agree that Ramsey's thought does not allow one to formulate concrete social or medical policy in allocational issues, this does not negate any contribution which covenant-care may make to such contingencies. Any criticism at this stage is not aimed at Ramsey's ethical construct, but rather at his failure to recognize its applicability to broader issues.

Chapters three and four have argued, in general terms, for the usefulness of the thought of John Rawls and Paul Ramsey on the issue of orphan drugs. Any ethical construction which relies on Rawls and Ramsey remains abstract, however, until one examines historical contingencies which have been utilized to define and remedy the plight of the rare disease patient. Chapter five will offer such an examination, and will conclude with suggestions for the incorporation of the respective thought of Rawls and Ramsey into the problem at hand.
ENDNOTES


4Boehringer Ingelheim Pharmaceuticals, Inc. internal correspondence. Derived from a presentation by A. Segretti. Undated.


6Ibid., p. 23.

7Ibid., pp. 21, 24, 25-27.

8Ibid., pp. 25-26.

9Ibid., pp. 21-29.


11Ibid.


13Ibid., p. 35.
14 Ibid., p. 41.
15 Ibid., p. 44.
16 Ibid., p. 94.
17 Ibid., p. 95.
18 Ibid.
22 *The Patient as Person*, p. 2.
27 *Basic Christian Ethics*, pp. 234-248.
28 Ibid., p. 242.
29 Ibid., pp. 246-248.


32The Patient as Person, p. 240.

33Ibid., p. 268.
CHAPTER FIVE
THE ROLE OF ETHICS IN THE SERVICE OF THE RARE DISEASE PATIENT

Orphan drugs received little regulatory attention before Representative Elizabeth Holtzman's (D-N.Y.) introduction in 1980 of H.R. 7089, which was essentially the first orphan drug legislation. The bill provided for the establishment of an Office of Drugs of Limited Commercial Value, which would in turn be authorized to provide financial and other assistance toward the development of orphan drugs as well as to coordinate the efforts of government and industry. Several other bills followed, including the Stevenson-Wydler Technology Innovation Act (P.L. 96-480) of 1980, which provided funds for cooperative research projects between industry and academia.¹

In 1981, Congressional activity resulted in the general public becoming aware of the orphan drug problem when television actor Jack Klugman, who had recently starred in an orphan drug episode of "Quincy," appeared at a hearing on an orphan drug bill introduced by Representative Theodore Weiss (D-N.Y.). Klugman continued the argument for orphan drug legislation which had been cast in his TV program, urging committee members to consider rare disease patients as "victims." Weiss's bill never made it out of committee, but was the basis for Representative Henry Waxman's (D-N.Y.) introduction of H.R. 5238, the Orphan Drug Act.²
The Orphan Drug Act, passed in 1983, encourages the development of pharmaceutical compounds for the treatment of rare diseases. Specifically, the Act allows tax incentives and marketing exclusivity for sponsors of such compounds. Before reviewing the literature specific to the Orphan Drug Act, it is important to appreciate that such legislation is best viewed as governmental intervention in a free market. Also, the Orphan Drug Act represents cooperative behavior between government, industry, and those patients who will directly benefit from such legislation.

A careful analysis of the Orphan Drug Act shows that this legislation involves more than cooperative behavior between institutions. This is evidenced by the fact that industry sponsors for orphan drugs are actively solicited by the Food and Drug Administration, as well as the fact that grant monies are appropriated for government subsidies of orphan drug research. Thus we find that the federal government, via the FDA, has varied from its long standing position of regulation to a broader posture of facilitator and co-developer of drugs. Although the cause of orphan drugs gives an appearance of possessing a sound ethical basis, further reflection shows that the case is prima facie only. The development and marketing of pharmaceuticals in the United States is a free, albeit highly regulated, market activity. Like most other commodities, the development and marketing of drugs are subject to supply and demand factors. There must be
sufficient and adequately demonstrated rationale to justify governmental intervention in any free market.6

Edith Stokey and Richard Zeckhauser, in A Primer for Policy Analysis, argue that the government should intervene only when the free market does not operate rationally. This rather vague notion is clarified when one considers that the benefit of a free market is a better society as a whole. Still, one is faced with defining what is best for society—a task Stokey and Zeckhauser approach within the dual models of equity and efficiency. It is only within these two concepts that governmental intervention in a free market is justifiable.7

The marketplace is neither equitable nor efficient if the self-directed behavior of each individual or corporation does not lead to optimal benefits for society at large. Stokey and Zeckhauser point to "externalities," or actions by an individual, government, or business, which effect the welfare of other entities, as a major rationale for governmental intervention. For example, the priorities of a pharmaceutical company, when focused on profit structures based upon supply and demand situations, do not benefit society as a whole. This example does not mean that the entire market has failed, but rather that a certain segment of the market is not being adequately served. It is the task of policy makers to determine what corrective measures are appropriate for either whole or partial market failures.8
One solution forwarded by Stokey and Zeckhauser calls for incentives which attempt to influence market failure rather than directives which mandate certain required action. The Orphan Drug Act, as we have seen, is an incentive program. There is no requirement that pharmaceutical firms sponsor orphan drugs. Rather, the Orphan Drug Act is designed to offset the disincentives present in developing drugs for the rare disease patient.

Carolyn Asbury provides the most thorough historical analysis of the orphan drug situation to date. In *Orphan Drugs: Medical versus Market Value*, Asbury traces the history of the FDA's regulatory powers and procedures, and explicates the problems of the rare disease patient. Asbury reminds us that orphan drugs existed long before passage of the Orphan Drug Act in 1983, and in fact have for long been a topic of debate between industry and government.

Asbury relates the problem of orphan drugs to other problems of resource allocation, and asks the same questions one frequently finds in discussions on health allocations. Specifically, Asbury asks which patients should benefit from technological advances, who should pay, and who should make the decisions. For Asbury, the orphan drug issue spans the fields of economics, technology, and government.

Perhaps Asbury's major contribution is her description of the process by which rare disease patients may bring their case forward to industry and government representatives. With keen
insight into the personalities of rare disease patients and their families, as well as of the various organizations and committees that represent them, Asbury documents that the Orphan Drug Act provides a unique environment in which rare disease patients may play a major role in how their needs are met.10

As described by Asbury, the term "Orphan Drug" was not coined in reference to the Orphan Drug Act. This is further elucidated in Orphan Drugs and Orphan Diseases, a compilation of presentations delivered in Ann Arbor in 1982. The major topics of the role of the patient, governmental intervention, and scientific problems inherent in orphan drug development were as important in the year prior to enactment of the Orphan Drug Act as they are today.11

Superficial reading of the literature on the Orphan Drug Act could lead one to view this legislation as a collaborative effort between industry and government. This is partially true—yet there is another factor in the equation. It is doubtful that the Orphan Drug Act could have become law were it not for the action of consumer groups interested in the rare disease patient. In Cooperative Approaches to the Research and Development of Orphan Drugs, Abbey Meyers describes the efforts of agencies such as the National Organization for Rare Diseases, the National Orphan Drug and Device Foundation, and the Tourette's Syndrome Association. Recognizing the legitimacy of rare diseases and the widespread apathy and lack of pertinent knowledge by the medical profession, these groups have played a major role in educating the
public as well as the government and industry on the discrepancies within our drug approval system.\textsuperscript{12}

The Orphan Drug Act may be viewed as an example of cooperation between diverse interest groups. Government, industry, and rare disease patients themselves have unified in an effort to frame a construct which will afford the rare disease patient an otherwise unavailable chance at viable pharmacologic therapy. It is not unreasonable to claim that the Orphan Drug Act has been a successful venture. Over 190 compounds have been awarded orphan status by the Food and Drug Administration, leading the National Organization for Rare Disorders to proclaim that the Orphan Drug Act is "a major success and a model Federal program."\textsuperscript{13}

The success of the Orphan Drug Act may be attributed at least in part to attitudes and actions relevant to the two broad categories posited in this thesis. First, John Rawls calls for the constant review, criticism and correction of the background institutions of society, for "adjustments in the basic structure are always necessary."\textsuperscript{14} While using Rawls to settle a concrete allocational issue would indicate a misunderstanding of his intentions for the two principles of justice, I have argued that appreciating the drug regulation and marketing systems in the United States as background institutions, and as such components of the basic structure, is consistent with Rawls' scheme.

The Food and Drug Administration, functioning as the primary government agency dealing with the orphan drug situation,
has evolved from its historical role of a strictly regulatory agency charged with insuring the safety and efficacy of drugs, to an active participant in the prospective struggle to help meet the needs of the rare disease patient. The active solicitation of orphan drug sponsors, as well as the allocation of four million dollars a year for basic orphan disease research,\textsuperscript{15} indicates a willingness to implement needed changes in policy and organizational mission—a contingency required within the Rawlsian scheme.

Second, Paul Ramsey has suggested, via his agape-based ethics of care for the individual qua patient, that one has an obligation to address the needs of one's neighbor. Abbey Meyers makes reference to this disposition and its consequences:

If one examines the foundation upon which the orphan disease movement was built, you will clearly see that it is based upon an understanding that we are our brothers keeper. To do our work effectively we must have the support of our friends, as well as strangers. . . . We are very grateful to those who care, and patient enough to wait for those who do not yet recognize our suffering.\textsuperscript{16}

To this point my analysis has suggested that the respective thought of John Rawls and Paul Ramsey may inform the issue of the plight of the rare disease patient. I will now attempt to translate this abstract supposition into a more practical construct by proposing ways in which the thought of Rawls and Ramsey may support ethical action and influence behavior in this situation.
I argued in Chapter three that the usefulness of Rawls' notion of justice as fairness is limited in the case of the rare disease patient to just background institutions--constructions which would presumably include a voluntary market and governmental regulatory agencies. While the current American free market and drug regulatory systems appear to be consistent with Rawls' notion of a just basic structure, one must appreciate that these systems are, in the Rawlsian scheme, subject to review, criticism, and change. Background institutions must be basic but not static.

Change in the basic system of pharmaceutical distribution, i.e., a free market, is unlikely. The exorbitant cost of drug development can only be efficiently borne by private industry. Even if the American system of health care were to become either totally or substantially socialized, there is no reason to expect government to take over the research and development cost of prescription drugs. The United Kingdom, with its almost total system of socialized medicine, depends on the same market concept as the United States for the distribution of pharmaceutical compounds.

Any background institutional change which could efficiently increase the availability of orphan drugs must come at the level of governmental regulatory affairs. Overall, the American drug regulatory system appears to meet Rawls' test for fairness. However, according to Rawls, "even in a well-ordered society, adjustments in the basic structure are always necessary."18
Adjustments in the drug approval process could, in order to facilitate orphan drug development, be modelled on the "Compassionate Use" or "Treatment IND (Investigational New Drug)" concepts. These programs, by which drugs are made available to select patients before FDA approval is granted, are currently limited chiefly to life-threatening or debilitating diseases. In fact, the FDA in late 1988 announced an accelerated approval track for certain drugs used to treat such ailments. According to FDA Commissioner Frank Young, M.D., Ph.D., "... the FDA will review drugs for life-threatening conditions on the basis of risk-benefit analysis." Such a standard, while of great potential benefit to sufferers of life-threatening or debilitating diseases, is no less applicable to those compounds which have promise for the rare disease patient. The advantages of a "Compassionate Use" or more appropriately "Treatment IND" program is that these programs allow fewer early studies--trials which in fact may not be appropriate. FDA's serious concern for safety data is best interpreted in light of the usual availability of alternatives--a situation inconsistent with the concept of orphan drugs.

Improvements in the drug regulatory system could certainly effect greater accessibility to orphan drugs by the rare disease patient. Such policy changes are consistent with the Rawlsian notion of the constant review, criticism, and change within the basic structure of society. However, any regulatory adjustments are of themselves insufficient to address the broader problem of
motivating profit-driven health care corporations to recognize any duty beyond that of profit maximization. This motivation, I will now argue, may be served by the thought of Paul Ramsey.

In Chapter two I argued that pharmaceutical corporations, as health care providers, have an obligation beyond that of profit maximization. Further, such obligations are most frequently met by what may be termed corporate egoism, i.e., actions undertaken by a corporation which, although beneficial to some others, nonetheless are derived from self-serving motives.

How may Ramsey's rule-based deontology be applied to such a concrete secular contingency? First, it is necessary to recall Ramsey's notion of covenant-care—a concept derived from his notion of agape: For Ramsey, covenant-care is the "inner meaning and purpose of our creation as human beings" and demands a moral relationship between man, his neighbor, and institutions with which he is associated.22

Covenant-fidelity relationships are not limited to the physician-patient encounter. I argued in Chapter two that there is no substantive difference between the notion of the health care corporation as provider and that of the physician as provider, other than the difference in emphasis of profit motive. Following this line of reasoning, it is not too broad a leap toward the notion of a covenant relationship between the health care corporation and the public. This covenant between provider and recipient recognizes the special nature of health care as well as the corporation's special role as purveyor of health care services,
and is consistent with the notion of corporate responsibility beyond that of profit maximization.

The successful application of any theory of a covenant-fidelity relationship between profit-driven health care corporations and the patients they ultimately serve rests with one's interpretation of the *purpose* of the covenant. If the function of the covenant relationship is that of producing a given *good*, one is faced with the challenge of applying deontology to a concrete business issue—a task deemed inappropriate by Ramsey.\(^{23}\) Rather, one must look to Ramsey's notion that:

\[
\ldots \text{love has to do with the very *nature* of love itself, it does not simply provide love with another object--the neighbor--in whom one's *bonum* should still be sought. Christian love in its nature gives some good, it is not primarily concerned to seek good. It is deontologically, as a matter of obedience, related to the neighbor as such; it is not teleologically, as a matter of desire, related to some *bonum*.}^{24}\]

This notion of love, when viewed within the context of Ramsey's assertion that the primary concern of Christian ethics is the determination of *whose* good it shall be when a choice must be made between persons,\(^{25}\) suggests that Ramsey's fundamental concern is the *relationship* between parties.\(^{26}\)

Viewed within the notion of relationship between parties, and especially the relationship between the health care corporation and the patient, my earlier argument for the usefulness of Ramsey's thought in informing the issue of the plight of the rare disease patient appears valid. It would further
appear that Ramsey's emphasis on covenant-fidelity could be translated into a notion of an obligation of virtue on the part of the health care corporation. That is, the health care corporation is called to develop and maintain a decision making apparatus which recognizes the patient as suffering individual and simultaneously accepts the corporation's unique role as a provider within the service ethos of medicine.

Although Ramsey's ethics is clearly an ethics of duty and not one of virtue, the notion of corporate virtue is consistent with the concept of covenant-fidelity. Corporate virtue, which may be defined in terms of the willingness and ability of the corporation to make moral judgments in response to the relationality of the corporation to individuals and to society, bears ethical fruit to the extent that the concept is accepted by management. Thus Ramsey's thought provides a much needed normative element to the decision making mechanism of a given corporation.

Applying the principle of covenant-fidelity to the health care corporation does not help answer specific policy quandaries such as how much money should be allocated to orphan drug development or which specific drugs should be developed. Rather, the notion of covenant addresses the relationship between parties—a kinship which bears ethical fruit to the extent that health care corporations accept their role as providers. Such a concept, unlike that of justice, admittedly lacks the capacity to be directly applied to resource allocation issues. This does not,
however, negate the relevance of the pharmaceutical corporation's acceptance of its role as provider.

To the uninitiated, the term "rare disease" is apt to invite a vision of a pathological curiosity, or at best a disease state from which most are isolated or statistically safe. Certainly one seldom hears of Hemochromatosis or Prader-Willi Syndrome. One does, however, hear much about AIDS, which is classified as a rare disease. Perhaps the widespread publicity and increasing awareness of this calamity may provide the necessary impetus for cooperation and commitment--concepts which are enriched by sound ethical reflection.

Acceptance of a covenantal relationship points more to a change in what a health care corporation is rather than what it does. That is, the notion of agape or covenant serves the orphan drug issue by providing impetus for modification of the historical relationship between the health care corporation qua provider and the rare disease patient. Clearly, any benefit resulting from the acceptance of a covenant-based relationship would be second-hand and not direct. That is, benefits such as increased emphasis on orphan drug development would derive from the inclusion in policy decisions of the corporation's special role in the service ethos of medicine, and not from specific policy derived from a normative construct.
ENDNOTES


2Ibid., pp. 111-112, 124-129.

3Ibid., pp. 129-130.

4Ibid., p. 186.


7Ibid., pp. 292-293.

8Ibid., pp. 303-304.

9Ibid., pp. 312-315.


17Ibid.

18"The Basic Structure as Subject," p. 164.


20Ibid.

21Ibid.


25Ibid., p. 114.


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