

ALLOCATING TREATMENT FOR RARE ALLERGIC DISEASES — THE RULE OF RESCUE

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ABSTRACT

The rule of rescue describes the moral imperative to save individuals whose lives are endangered without any consideration of what the rescue operation may cost. One formulation of the rule of rescue as a framework for allocating resources for rare diseases includes the following requirements: the individuals should be identifiable; their lives must be endangered by a disease that is severe, progressive, and results in a decreased life expectancy if untreated; opportunity costs are involved, and there is no existing alternative treatment. I discuss arguments for and against the rule of rescue in resource allocation; it can be used as a guide for individual decision making, but is not a good public policy framework.

INTRODUCTION

Advances in medicine and technology have increased rapidly during the past few decades. Consumers of health care have expectations regarding access to high-quality treatments and services, but costs have escalated to such an extent that no healthcare service can afford to provide everything to all patients. In a middle-income country such as South Africa state health care usually includes a basic package of primary care services that are affordable and cost-effective. How does such a system accommodate the patient with a rare disease that requires expensive treatment, but treatment that is basic to that person's medical needs?

A hypothetical case

Mrs A has hereditary angio-oedema (HAE) with numerous life-threatening episodes. A new treatment (Drug X) is available, but is very expensive. Dr P believes the medical aid fund should pay for it and writes a strong letter of motivation, but the fund's medical advisor turns down her application, citing the cost of the drug and the availability of other treatments for HAE. What framework could be used to advocate for the use of costly treatment for a patient with a rare disease?

DEFINING RARE DISEASES AND ORPHAN DRUGS

In the USA a rare disease is defined as one that affects fewer than 200 000 people (this translates to 7 per

10 000 people).^{1,2} In Europe the frequency is defined as less than 5 in 10 000; the World Health Organization defines a rare disease as having a frequency of 6.5-10 per 10 000.² These diseases have also been termed orphan diseases, implying both neglect by the medical profession and also a disease that affects very few individuals.² Orphan drugs are drugs used to treat orphan diseases. Examples of well-known rare diseases are Gaucher's disease, Huntington's chorea, and cystic fibrosis.

RESOURCE ALLOCATION FOR RARE DISEASES AND ORPHAN DRUGS

In the developed world even very expensive drugs are funded for these rare diseases, both in the public and private sectors. Generally speaking, people seem to find it acceptable that large sums of money are spent on treating individual patients, despite the fact that, if the money was spent on preventing disease in large numbers of other patients, many more people would benefit.¹ It would therefore appear that these cases are seen as being special and falling outside the normal resource allocation decisions. Why does this happen? One of the reasons is that people with rare diseases are members of an underserved healthcare community and, in accordance with the principle of justice, deserve to have their needs met. Another is the moral imperative to rescue those in dire need of care.

THE RULE OF RESCUE

The rule of rescue describes the moral imperative to save individuals whose lives are endangered without consideration of the costs incurred. 'No effort is deemed too great, and seemingly unlimited resources are expended to save these individual lives. To do less would be deemed unconscionable.'¹ Examples in everyday life are mountain and sea rescues. The 33 Chilean miners trapped underground for 69 days in 2010 were eventually rescued at a cost of \$20 million, yet no one entertained the thought that the rescue was too costly to attempt. A recent South African example is the rescue after a massive search operation of surfer Brett Archibald who fell overboard from a boat near Indonesia and survived for 27 hours in the sea. Everyone accepts the rule of rescue under these circumstances, but can we apply it to medical scenarios as well?

The rule of rescue in medicine

Albert Jonsen wrote, 'I call this ... the rule of rescue. Our moral response to the imminence of death demands that we rescue the doomed. We throw a rope to the drowning, rush into burning buildings to snatch the entrapped, dispatch teams to search for the snowbound. This rescue morality spills over into medical care, where our ropes are artificial hearts, our rush is the mobile critical care unit, our teams the transplant services. The imperative to rescue is, undoubtedly, of great moral significance; but the imperative seems to grow into a compulsion, more instinctive than rational.'³ Jonsen argued that, before the advent of renal dialysis, organ transplantation and life-saving intensive care, it

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was relatively easy to argue against medical treatments and technologies that were not cost-effective. The rule of rescue has ensured that these allocation decisions have become much more difficult. However, the reality of resource constraints must temper our impulse to save lives at any cost.

So, how can we utilise the rule of rescue as a framework for allocating resources for rare diseases? The constituents of the rule of rescue in medicine as proposed by Largent and Pearson are: the individuals should be identifiable; their lives must be endangered by a disease that is severe, progressive, and results in a decreased life expectancy if untreated; opportunity costs are involved; and there is no existing alternative treatment.¹

Identifiable individuals

The concept of *identifiability* is an essential component of the definition of the rule of rescue. In medical terms, this identifiability is related to the rarity of the disease, so that only a few individuals are affected. If the disease results in obvious physical signs it has a greater public impact, and if the affected patients are even more identifiable as a result of publicity or celebrity status, the potential effect is even greater. An example of this is the amount of publicity given to the rare debilitating neurological disease, amyotrophic lateral sclerosis, currently afflicting a well-known national sportsman.

Endangered lives

Another central component of the rule of rescue is that the person's life must be 'endangered'. According to Largent and Pearson this does not necessarily imply the condition is life-threatening but may include poor quality of life as well.¹ Orphan drugs may thus save a life or improve function and quality of life.

Opportunity costs

Generally the cost of a rescue is not factored into the decision-making process. The argument for this has been that the rarity of the disease usually implies that few patients will require the drug and that the cost to the health insurer will be bearable. However, costs are likely to increase with the development of new drugs for orphan diseases, and so cost considerations will have to be taken into account.

No existing alternative treatment

This should not make a difference from a moral point of view. All patients in immediate danger would want the best possible available treatment for their condition.

Arguments in favour of the rule of rescue as an allocation framework

One argument in favour of the rule of rescue is that the identified person has the potential of benefiting enormously with an increased chance of life, compared to others who will probably gain very little from an intervention that is applied to 'anonymous "statistical" lives'.⁵ In other words, the rule of rescue provides justification for the allocation of scarce healthcare resources to patients suffering from rare diseases and deviates from the usual utilitarian considerations. Principles of distributive justice that support the rule of rescue are: prioritising the worst off; equality in realising health; and distribution of health care according to need.⁴ Another argument in favour of the rule of rescue is the perception created among the citizenry that the state cares for those who are worst off in society.⁴

Arguments against the rule of rescue as an allocation framework

Concern for identifiable individuals in immediate danger implies that there is less concern for unknown individuals who may face danger in future.⁴ Does the fact that it is identifiable persons who benefit from the rule of rescue serve as ethical justification for using it as a framework for resource allocation? Probably not.^{1,6} However, because the beneficiaries are at significant risk of death or severe impairment, the rule of rescue does provide some ethical justification.² What it does not do is provide a guide as to *who* should benefit from the resource, for example, in the case of organ retransplantation.⁶

As a policy, using the rule of rescue to provide unlimited expensive treatment for rare disease is not sustainable.¹ The argument that professional virtues dictate that the healthcare system should offer treatment to an identifiable individual does not take into account the loss of many more lives that may occur as a result of treating the individual.⁶ Framing the rule of rescue in terms of the principles of biomedical ethics, it is analogous to beneficence towards those who are sick and needy. The problem is limiting this beneficence to prevent unfairness to those groups of patients who would have benefited from non-rescue medicine. Translating this into everyday medicine, the rule of rescue would favour emergency care, even if it has only a slight chance of success, over preventive services that would benefit large groups of people, or acute care over chronic care.

Cookson argues that fundamental to public healthcare decision making is impartiality and fairness in allocation of resources between different groups, rather than advantaging identifiable individuals.⁴

Which rescues are justifiable?

The use of enzyme replacement therapy (ERT) in Gaucher's disease is an example of an intervention that may be justified by the rule of rescue. Gaucher's disease, a lysosomal storage disorder, causes significant impairment, although it is seldom life-threatening. Cerezyme treatment may restore the patient to full function, but it is extremely expensive (\$200 000 per annum).¹ However, as there are so few patients with Gaucher's disease, the overall cost is not prohibitive. As Largent and Pearson state, the patients are identifiable, the disease has a severe impact on people's lives, and the opportunity costs are relatively low owing to the small number of people with Gaucher's disease.¹ The authors contrast this situation with that of cetuximab in non-small-cell lung cancer: the benefits are minor; the side-effects are considerable; and the potential costs are prohibitive, given that a large number of people are diagnosed with this cancer every year.¹

Consider Mrs A in the hypothetical case scenario above: HAE is potentially life-threatening and has a major impact on sufferers' lives; the patients are identifiable; a new treatment may impact both Mrs A's quality of life as well as prevent a life-threatening episode; and the opportunity costs will be affordable. According to the framework proposed by Largent and Pearson, the use of Drug X in this scenario could be justified by the rule of rescue.

CONCLUSION

The rule of rescue is used to describe the response of an individual to a person or people in immediate danger. Its application in health care is fraught with difficulty. In this article I have discussed the rule of rescue as one framework to justify allocation of scarce resources