Rare diseases in healthcare priority-setting: Should rarity matter?

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Abstract

Rare diseases pose a particular priority-setting problem. The United Kingdom gives rare diseases special priority in healthcare priority-setting. Effectively, the NHS is willing to pay much more to gain a Quality-Adjusted Life year related to a very rare disease than one related to a more common condition. But should rare diseases receive priority in the allocation of scarce healthcare resources? This article develops and evaluates four arguments in favor of such a priority. These pertain to public values, luck egalitarian distributive justice, the epistemic difficulties of obtaining knowledge about rare diseases, and the incentives created by a higher willingness to pay. The first is at odds with our knowledge regarding popular opinion. The three other arguments may provide a reason to fund rare diseases generously. However, they are either over-inclusive because they would also justify funding for many non-rare diseases or under-inclusive in the sense of justifying priority for only some rare diseases. The arguments thus fail to provide a justification that tracks rareness as such.

Introduction

Rare diseases pose a unique challenge for contemporary priority-setting in healthcare. Specifically, a rare disease prompts us to consider whether its rarity constitutes an independent reason to aid those who suffer from it. There is no universally accepted definition of a rare disease.[1][1] In the United Kingdom and the EU, a disease affecting five or fewer in 10,000 people is considered rare.[2] The NHS estimates that there are between 5000 and 8000 rare diseases in the United Kingdom. While each affects only small groups, combined, they affect 3 million people's lives.[2] Worldwide rare diseases are estimated to affect 3.5–5.9% of the population.[3]

This article addresses the role of rarity in priority-setting,[2] specifically

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[1] What follows applies to all definitions based on how common a disease is.
[2] This sets aside other important aspects, including measures like prevention, detection, diagnosis, coordination of care, and research.[2] Others stress the need for registers and knowledge sharing.[4] For a further list of policy initiatives see [5]

whether societies should have a higher willingness to pay for pharmaceutical drugs for rare diseases.[3] This question is pertinent to any healthcare system explicitly or implicitly conducting priority-setting with references to cost-effectiveness. Such rationing is often based on the QALYs provided by a drug. This creates a ranking of how much health the drug produces relative to its costs. Nothing in the bare order tells us which drugs (if any) society should fund or how much we should be willing to pay for each QALY gained.[6] Across countries and academia, there has been considerable debate over the usefulness of QALYs as a health measure and the shortcomings of cost-effectiveness analyses.[7,8] Such systems frequently include measures and practices that depart from what cost-effective analysis would recommend. The current NHS practice in the United Kingdom regarding rare diseases is a clear example of this.[4]

In 2017, NICE set a maximum additional QALY (quality-adjusted life-year) threshold of up to £300,000 for highly specialized treatments.[10] Drugs for rare diseases that cost less per QALY are approved for routine commissioning. Willingness to pay varies from £100,000 to a maximum of £300,000 per QALY, depending on the additional QALYs gained by the patient.[10] Finally, such assessments are subject to a standard budget impact test.[5] Thus, the threshold for rare diseases is up to ten times higher than the standard NICE threshold of £30,000 applied to non-specialized treatments.

This higher threshold effectively exempts rare diseases from the cost-effectiveness framework applied to common diseases. A likely reason for this is that many drugs

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[3] While what follows applies to both treatments and drugs the latter term is used for brevity.

[4] Rosenberg-Younger et al. examine how Israel, Canada, and Australia approach rare diseases.[9] The recommendation committees making funding decisions consider primarily clinical evidence regarding cost-effectiveness. They also invoke other values, specifically a rule of rescue and equity of access,[9] and committee members are concerned that the effects of orphan drugs are difficult to assess.[9] None of the countries surveyed had a separate system for evaluating drugs for rare diseases at that point. However, it was not an uncommon thought that we should hold these to a different standard than common diseases.

[5] This implies that if the costs for the approved drug would exceed £20 mio pounds in one of it's first three years the price must be renegotiated.[10,11] This test is applied to all drugs, both rare and common.

for rare diseases are so-called orphan drugs, i.e., they are not commercially viable because of the low demand.[12][6] Orphan drugs are often expensive to develop, and their potential market is small. Furthermore, the complexity and rarity of these diseases make research and controlled trials more difficult.[13,14,12,15] Thus, when standard procedures for health technology assessment are employed, drugs for rare diseases do not fare well compared to drugs for more common diseases.[16]

Does the rarity of a disease constitute a reason for special treatment? This is controversial and has spurred considerable debate.[17–21] This article assesses several possible justifications for such prioritization. Specifically, it is evaluated whether the arguments justify prioritizing rare diseases as such (as opposed to just some rare diseases) and whether the arguments are over-inclusive in the sense that they justify paying more for some common diseases as well. The following sections develop and assess arguments from public opinion, luck egalitarian justice, epistemic reasons, and the incentives created by giving priority to rare diseases.

Priority to rare diseases because this reflects public opinion

One possible justification would be to submit that prioritizing rare diseases reflects a deep conviction held in society. If the general population strongly prefers special priority to those who suffer from a rare disease, it might constitute a reason to provide it. But such a justification is, of course, only viable if the available knowledge about people's beliefs corroborates it.

To assess this, we must look at studies that evaluate the general population's views about rare diseases. Dekker et al. surveyed Norwegians' preferences regarding funding for rare disease treatment. They conclude that there is no specific preference for giving priority to those who have a rare disease. [22] When presented as if rare and common diseases are equally expensive, most people prefer to treat common diseases. Once the assumption about equal costs is relaxed to reflect that rare diseases are often

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[6] The definition of orphan drugs varies, but rarity of disease is one condition in both the US and UK for considering something an orphan disease and the drugs aimed at this an orphan drug.[12]

more costly to treat, the willingness to fund rare diseases.[22] Studies elsewhere corroborate the findings from Norway. A survey of over 4000 adults in the United Kingdom found no preference for prioritizing rare diseases.[23] A list-choice experiment also from the United Kingdom concluded in a similar vein that the general public "does not value rarity as a sufficient reason to justify special consideration for additional NHS funding of orphan drugs."[24] Another list-choice experiment reached a similar conclusion.[25] In several surveys and experiments, Wiss did not identify a willingness to pay more for rare diseases.[26,27] In general, the available surveys of public attitudes towards rare diseases do not indicate that public opinion justifies a higher willingness to pay to treat rare diseases.[28] Therefore, public opinion cannot justify a higher willingness to pay for rare diseases.

Rare disease as bad luck: Equality of resources

Some argue that the current NHS priority to rare diseases is unfairly unequal [20] or that fairness considerations cannot justify such a priority.[19] Such concerns reflect the notion that if the plight of people with a rare disease is relevantly similar to that of people with common diseases, then the higher priority to the former is not justified. But perhaps, if we apply a luck egalitarian perspective, we may reach a plausible conception of the relevant difference. Luck egalitarianism is a prominent theory in contemporary debates about distributive justice [29–33] and has recently been applied to health and healthcare.[34–36][7] Luck egalitarians believe that it is unjust if people are worse off than others through no fault or choice of their own (i.e., through bad luck).[8]

One luck egalitarian argument springs from Ronald Dworkin's idea of equality of resources.[9] According to Dworkin, a just society provides each individual an equal

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[7] The theory remains controversial in health [37–48].

[8] In his discussion of rare diseases and fairness, Juth sensibly dismisses that luck egalitarians could support priority for rare diseases based on the thought that these reflect option luck to a lesser degree than common diseases [19].

[9] Dworkin did not consider himself a luck egalitarian [49], but his contribution to the literature is undisputed [33]

share of resources to realize a distributive ideal, sensitive to differences in our ambitions but not in our endowments.[50][10] The basic idea is that each receives an equal amount of resources to pursue these ambitions and preferences.[53] However, as inborn resources differ, a mechanism is required to counteract this. To this end, Dworkin develops the device of a hypothetical insurance market where people insure themselves against a lack of productive talent, handicaps, and illnesses.[54] The degree to which people would have insured against something constitutes a measure of what compensation equality of resources requires.[50,54] For equality of resources to provide an argument for why rare diseases should receive higher priority, it would need to be the case that people would, under such circumstances, insure against suffering from a rare disease. If they would do so in the hypothetical insurance situation, it provides a justice-based reason to compensate those who suffer from rare diseases in the real world.

However, there are several problems with the ability of Dworkin's approach to provide such a justification. Such an argument would have to present reasons why rational agents, with adequate knowledge about risk and rarity, would pay more to be insured against losing a QALY to a rare than to a common disease. Roemer points out in his critique of Dworkin, rational people would underinsure against things that are very unlikely to occur.[55] If people would underinsure against rare diseases in a hypothetical insurance market or insure against them only to a similar extent as against common diseases, equality of resources does not provide a reason to have a higher willingness to pay for rare diseases.

Equality of resources is not in itself a reason society should be willing to do so. Dworkin's approach to distributive justice is explicitly market-based. This works against its ability to provide an argument for covering rare diseases through the already described mechanisms of supply and demand. It would then seem that

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[10] For recent applications to health, see [51,52]. For a luck egalitarian critique, see [31]

equality of resources and the hypothetical insurance measure do not favor allowing special priority to rare diseases.

Rare disease as bad price luck

Another luck egalitarian justification could be developed using the work of G.A. Cohen.[11] He is a luck egalitarian who disagrees with Dworkin about several issues, including the market's role in distributive justice.[57,58] According to Cohen, a more straightforward interpretation of what it means for people to suffer through bad luck entails that people can suffer from what Cohen calls "bad price luck".[57,58] That is when the things people want or need are expensive through no fault of their own.[57,58] It is reasonable to suggest that people who suffer from rare diseases suffer from bad price luck. Their efforts to have full health are hampered because achieving this is much more expensive for them than for others. Thus, there is a bad price luck argument that people who suffer from rare diseases, which are costly to treat, are unlucky in the relevant luck egalitarian sense. Their bad luck pertains to the laws of supply and demand working against what they need and want.

However, on further consideration, the bad price luck argument is over-inclusive. It justifies much more than an exemption for rare diseases. We could similarly argue that everyone who suffers from a disease (rare or common), which is expensive to treat, suffers from bad price luck. The luck egalitarian reasoning just presented mitigates against not treating people who suffer from diseases that are costly to treat. This is true for rare and common diseases alike. Thus, luck egalitarianism cannot justify an exemption for rare diseases. Instead, it provides a fairness-based reason to be critical of cost-effectiveness priority-setting as such. The price luck argument is over-inclusive.

Rare disease priority to counteract epistemological difficulties

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[11] Miller argues that Cohen is not a luck egalitarian,[56] but that is a minority position.[33]

Another kind of argument takes its starting point in the already mentioned difficulties with assessing rare diseases.[5,17] Could these difficulties give us a reason to treat rare diseases differently than common diseases? Perhaps when we subject rare diseases to the same test as common diseases, those suffering from rare diseases are unfairly disadvantaged due to the epistemological difficulties of attaining precise knowledge about what we can do for them. We might then understand the higher willingness to pay as a better-safe-than-sorry policy towards those who suffer from rare diseases.

There has been considerable empirical debate over this matter. Some argue that funding decisions already consider uncertainty about effects and that existing assessment methods are not as inadequate for analyzing drugs for rare diseases as the above suggests.[17] Others point out that these epistemological difficulties do not track a rare/common distinction.[17] However, we deem these empirical disagreements, this kind of argument offers insufficient justification for giving rare diseases a special priority. The argument would, if successful, justify priority for some but not all rare diseases. This is the case because even if we accept the empirical claims on which it rests, it would only be valid for some rare diseases. Specifically, those with a lot of uncertainty about effects. The argument would not apply for rare diseases as a group. The argument is thus under-inclusive.

Furthermore, the argument may also apply to some common diseases where there is uncertainty about effects. This suggests that the argument is also over-inclusive.

The incentive-based argument for priority to rare diseases

A final set of arguments refer to the instrumental value of providing priority to rare diseases. Increasing society's willingness to pay for rare diseases alters the structure of incentives. Specifically, it strengthens the incentive to produce drugs for rare diseases.[17][12] This increases the possible profitability of such research and, therefore, the likelihood that companies will develop these drugs. The incentives-based

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[12] EU has explicitly incentivized companies to produce orphan drugs.[59]

argument stresses that nobody would develop effective and affordable drugs without a higher willingness to pay for rare diseases.

This argument has some similarity to the epistemology argument just assessed. Both arguments rely on the idea that there is something particular about rare diseases that justifies priority. In the preceding argument, this pertained to uncertainty; here, it pertains to unprofitability. And again, the thought is that if we provide incentives, we will get a solution that is otherwise not forthcoming. Unless we are willing to pay more for a rare disease drug now, we will never achieve a future state of affairs where the price is lower. On the incentive-based argument, our increased willingness to pay is an investment in the availability of cheaper drugs in the future.

The good thing about this argument is that it proposes a story about how paying more now will save us from doing so in the future and ensure the availability of affordable care to (some of) those suffering from rare diseases. But the argument does not work for reasons similar to the argument about uncertainty. It is over-inclusive. The problem is that the described mechanism would also apply to many common diseases. If we increase our willingness to pay, say ten-fold, for drugs for a common disease, we would create incentives of a similar kind and (perhaps) cheaper drugs in the future. Consider suffering from a common disease who would benefit from drugs currently deemed too expensive when assessed by the standard threshold. They would also benefit from an increased willingness to pay. Both now, when access to drugs would be forthcoming, and in the future, because a higher willingness to pay might create better or cheaper drugs for them. For these reasons, the incentive-based argument is, as stated, over-inclusive.

One particular version of the incentive-based argument might avoid the over-inclusive problem.[13] Suppose that for some drugs for rare diseases, the unfavourable cost-effective ratio is driven entirely by the high cost and not by weak or uncertain effects. Suppose further that the high costs are due to the rarity of the diseases that

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[13] I am grateful to an anonymous reviewer for suggesting this discussion.

affect economies of scale in producing or developing drugs. If we do not have a higher willingness to pay for these drugs, they are unlikely to be made available. This version of the incentives-based argument highlights the lack of economies of scale as the important difference between rare and common diseases.

Would this argument be over-inclusive as the version discussed above? That is, would it entail we should be willing to pay more for some common diseases? Perhaps not. Common diseases benefit from economies of scale. When common diseases are not cost-effective, it is for reasons unrelated to economies of scale. There might then be an egalitarian reason for favouring the aforementioned rare diseases, as the burden of not doing so is unequally put on those, who suffer from these diseases. While some common diseases are also not cost-effective, they are so for reasons unrelated to economies of scale. Refusing to pay more for these common diseases has fewer unequal effects because and the costs are perhaps more likely to decrease over time. Is there then an egalitarian argument for providing an incentive for rare diseases?

Not quite. The argument just presented is too narrow to justify current NHS practices of paying more for all rare diseases. The argument highlights a subset of rare diseases as eligible for a higher willingness to pay, specifically, those which are effective but expensive due to lack of economies of scale. This contrasts with a general higher willingness to pay for rare diseases because that also includes those, which are not cost-effective for other reasons, such as providing little benefit. The argument is thus under-inclusive. The presented version of the incentive-based argument is therefore not a successful argument for a higher willingness to pay for rare diseases as such, but it does provide a reason to pay more for some specific rare diseases.

A final consideration is whether the argument is also over-inclusive. That is whether it would also require a higher willingness to pay for some common diseases. Its affinity with the bad price luck argument discussed earlier suggests that we must consider this problem seriously. The suggested reason against over-inclusiveness on behalf of the adjusted incentive-based argument was that common diseases do not, by definition,

lack economies of scale.[14] But of course, it could be that a group of drugs for common diseases, which are effective, does not suffer from a lack of economies of scale but which are still so expensive that they are above the regular threshold. This would force us to consider whether the mere presence or absence of economies of scale is in itself of moral significance or whether pragmatic or practical considerations would support the introduction of such a policy for rare diseases only. One possible practical consideration would be that to pay more for the subset of common diseases just described would be prohibitively expensive. Given the number of rare diseases, this suggested difference is at least not crystal-clear. However, we deem the question of over-inclusiveness, the point already made about under-inclusiveness means that the adjusted incentives-based argument cannot justify the current priority for rare diseases.

Conclusion

The article has developed and addressed potential arguments to justify a higher willingness to pay for drugs for rare diseases compared to common diseases. The arguments considered pertain to public opinion, luck egalitarian distributive justice, the epistemological difficulties of assessing rare diseases, and the incentives provided by special priority for rare diseases. None of these were deemed successful. They do not justify considering rarity of specific moral relevance. However, the article does not refute that some and perhaps many rare diseases are, for reasons unrelated to their rarity, candidates for diseases for which society should be willing to pay more. In fact, several of the arguments put forward cut also imply that there are be common diseases, which should be given a treatment similar to that currently provided to rare diseases.

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[14] Or at least that they do not lack a demand. As this article focusses on the specific NHS policy I set aside cases where common diseases lack effective demand because of a lack ability to pay.

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