Public attitudes to rare diseases: The case for equal access

UK BioIndustry Association

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Foreword

Living with a rare or ultra-rare disease is incredibly tough for patients and their families and carers. For these patients, many of whom are children, the absence of viable treatments is a heavy burden to bear.

The innovative life sciences sector is beginning to offer some of these patients and their families real hope that access to life-altering and potentially curative treatments is a not-too-distant prospect. Though this is cause for optimism, even when these treatments are licensed, patients still face an uphill battle trying to access treatments on the NHS. This is deeply frustrating for families and patients who are so close to living a transformed life.

Therapies for rare diseases have to navigate an evaluation process in which they are severely disadvantaged and although this has been adapted better to accommodate these therapies, there is still a high level of unmet need.

The NHS is predicated on the idea that people should have equal access to healthcare and treatment, regardless of the level of need. This report, which is based on a survey of public attitudes, represents a reaffirmation of this shared value for equitable healthcare, with 78% of respondents believing that people with rare diseases should have equal access to treatments, even if this would mean additional cost to the NHS.

The appraisal of rare disease medicines needs to be reconsidered to ensure that the aspiration for equal access to treatment for people with rare diseases is borne out in reality. We must level the playing field and develop a straightforward, understandable, and rapid process that respects and puts at its heart the needs of families and people affected by rare diseases so that they can quickly and fairly benefit from innovative treatments.

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CEO, UK Bioindustry Association

Executive summary

Rare diseases affect over 3.5 million people in the UK – 1 in 17 people.[1] While only 5% of rare diseases have a treatment currently available, scientific advances are now providing medicines for the first time for many rare diseases.[2]

Key findings

- Medicines for rare diseases face significant challenges in the context of assessment and appraisal, including data paucity, small patient populations and a lack of comparator treatments.

In a survey of the public, we found:

- While the majority of people believe the NHS does a good job at providing access to medicines in general (82%), people also recognise that access to medicines for rare diseases includes other barriers – e.g., an understanding that small treated patient populations result in higher prices for individualised therapies.

- The majority of people (79%) agreed that patients living with a rare disease should be able to access medicines on the same basis as people living with more common conditions.

- The majority of people (78%) agreed that the NHS should ensure access on the basis of clinical need even if this would be more costly to the NHS because of a disease's rarity.

- A significant number of people (46%) agreed that the cost threshold for medicines for rare diseases should be raised to ensure equitable access to medicines for all.

Recommendations

- NICE should consider the value of a rarity modifier as part of the HTA process to people with rare diseases and to the general public.

- NICE should revisit its position on the public's appetite for targeted measures to support improved access to medicines for people with rare diseases.

- NICE should revisit its proposed criteria for HST to ensure that it provides a viable route to access for rare disease medicines.

- The Department for Health and Social Care should explore options for additional funding for rare disease medicines to ensure patient access in line with a comprehensive health offer within the NHS.

1 Rare Disease UK, *The Rare Reality – an insight into the patient and family experience of rare disease*, January 2016, available online via: www.raredisease.org.uk/media/1588/the-rare-reality-an-insight-into-the-patient-and-family-experience-of-rare-disease.pdf

2 Global Genes, RARE Facts, available online via: https://globalgenes.org/rare-facts (Accessed 05 February 2020)

Introduction

Rare diseases affect 1 in 17 people in the UK. Within the UK it is thought that approximately 3.5 million people will be affected by a rare disease at some point in their lifetime.[3] Despite this high prevalence, there are high levels of unmet need. Currently, only 5% of rare diseases have licensed treatments. As a result, many people living with rare diseases die prematurely or live with debilitating symptoms which place huge burdens on caregivers, families, and society.[4]

Rapid scientific advancements are facilitating the development of new life-changing and potentially curative treatments for rare and ultra-rare diseases (otherwise known as orphan and ultra-orphan medicines). Unlike treatments for common diseases, the nature of rare and ultra-rare diseases creates a unique set of considerations that need to be accounted for while evaluating the value of such treatments:

- Rare diseases often have small patient populations. Consequently, there is often a high level of uncertainty from clinical trial data due to small sample sizes.
- Many rare diseases have no current treatment alternatives. Consequently, first-in-class treatments with no suitable comparators are inherently disadvantaged due to the great difficulty in proving their clinical and cost effectiveness.

Many countries have introduced flexible mechanisms to evaluate orphan medicines that account for these challenges and provide faster access to medicines for people who need them. In England, the body that undertakes health technology assessments (HTA), The National Institute for Health and Care Excellence (NICE), introduced its Highly Specialised Technologies (HST) process in 2013 and revised it in 2017 to create a new health technology appraisal pathway for evaluating ultra-orphan medicines.[5]

Though the introduction of the HST process was a positive step forward, there remain a range of issues with the current evaluation system which have been explored by *the Genetic Alliance*,[3] the Office for Health Economics,[6] and the APPG on Access to Medicines and Medical Devices.[7]

Rapid scientific advancements are facilitating the development of new life-changing and potentially curative treatments for rare and ultra-rare diseases."

One key problem is that even though the HST process was introduced to evaluate drugs to treat very rare diseases, due to its narrow eligibility criteria, orphan and even some ultra-orphan medicines fall into the Single Technology Appraisal (STA) process.[8] As the STA process is designed for evaluating medicines for common diseases, orphan medicines are unable to demonstrate cost-effectiveness in the same way, meaning they are often not recommended.

3 Genetic Alliance, *Action for Access*, 2019. Available online via: https://actionforaccess.geneticalliance.org.uk

4 PhRMA, *A decade of innovation in rare diseases*, 2015. Available online via: http://phrma-docs.phrma.org/files/dmfile/PhRMA-Decade-of-Innovation-Rare-Diseases4.pdf

5 National Institute for Health and Care Excellence, NICE highly specialised technologies guidance, 2021. Available online via: www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-highly-specialised-technologies-guidance

6 Office of Health Economics, Appraising Ultra-Orphan Drugs: Is Cost-Per-QALY Appropriate? A Review of The Evidence, 2018. Available online via: www.ohe.org/publications/appraising-ultra-orphan-drugs-cost-qaly-appropriate-review-evidence

7 All-Party Parliamentary Group on Access to Medicines and Medical Devices, NICE Methods Review, 2019. Available online via: https://mapbiopharma.com/home/2019/09/access-to-medicines-and-medical-devices-appg-launches-report and then all subsequent references do up by one.

8 All-Party Parliamentary Group on Access to Medicines and Medical Devices, NICE Methods Review, 2019. Available online via: https://mapbiopharma.com/home/2019/09/access-to-medicines-and-medical-devices-appg-launches-report

In 2019, NICE announced that it was conducting a review of its methods and processes that will inform changes to the current evaluation process and speed up patient access to new and promising health technologies.[9] Among the proposed changes is the adoption of a severity modifier. In practice, this would alter the cost-effectiveness threshold for medicines within the STA process designed to treat patients who face the toughest burdens of illness and unmet need. Since this category includes many people living with a rare disease, the BIA welcomes this proposal.

Though a rarity modifier would not address issues associated with the narrow HST entry criteria, it would go some way to mitigate the challenges that orphan medicines face when being assessed via the STA process."

During the early stages of the review, there had also been discussion surrounding the potential adoption of a 'rarity' modifier which would alter the cost-effectiveness threshold for medicines targeted at treating rare diseases. Though a rarity modifier would not address issues associated with the narrow HST entry criteria, it would go some way to mitigate the challenges that orphan medicines face when being assessed via the STA process and would go some way to facilitating greater access to medicines for people living with rare diseases. Unfortunately, there remains disagreement over the appetite for a rarity modifier. Though a severity modifier is likely to benefit people living with rare diseases to some extent, the BIA is concerned that should NICE exclude the adoption of a rarity modifier from its proposals, people living with rare diseases may continue to fall through the gaps.

It is in this context that we have carried out a survey of public attitudes to understand their views on rare diseases, medicines access, and vitally whether people would be open to specific measures to support access to medicines for people with rare diseases.[10]

9 National Institute for Health and Care Excellence, Changes we're making to health technology evaluation, 2021. Available online via: www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/changes-to-health-technology-evaluation

10 Bioindustry Association, BIA response to consultation on NICE processes of health technology evaluation, 2021. Available online via: www.bioindustry.org/uploads/assets/ba295f2f-ca67-4bdc-a9973e1af623d0b9/BIA-response-to-consultation-on-NICE-processes-of-health-technology-evaluation-FINAL-SUBMITTED.pdf

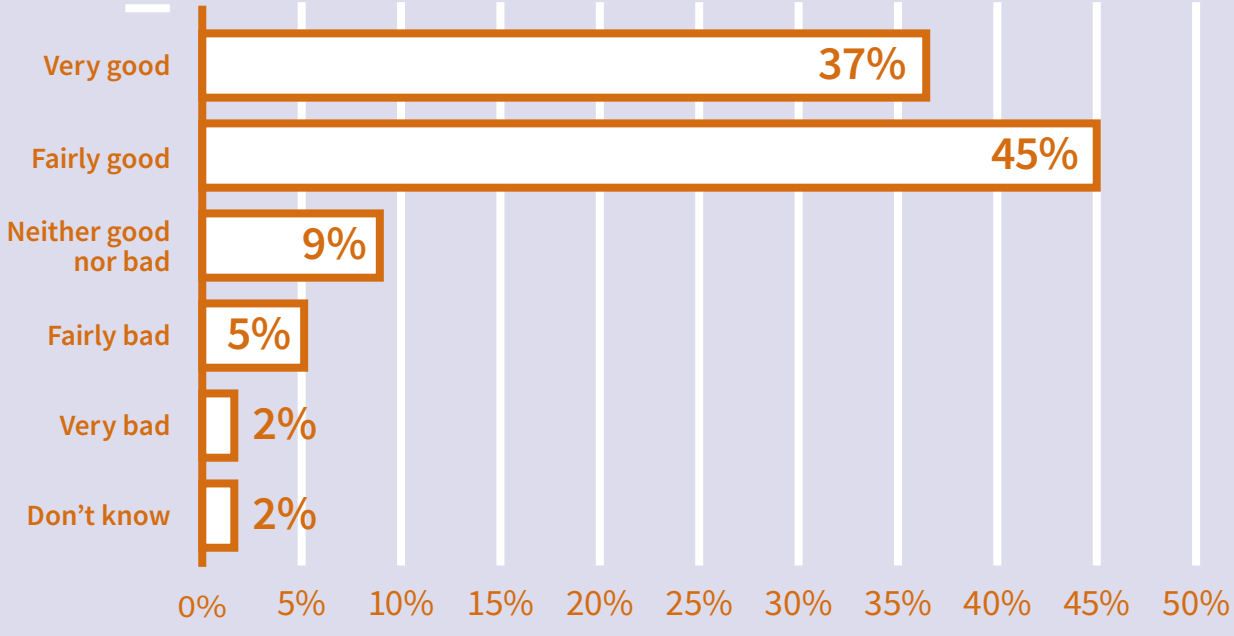
How well do people think the NHS does at ensuring access to medicines in general?

We asked how well people the NHS did at providing access to medicines to understand the public's general views on medicine access Of the 2023 respondents, the majority (82%) felt that the NHS does a 'good' job at ensuring access to medicines. Specifically, most respondents felt that the NHS did a 'fairly good' job. Though this suggests that people feel there is some room for improvement, on the whole people appear to be positive about the NHS's ability to provide access to medicines for the patients that need them.

Nevertheless, there remains a minority (7%) who felt that the NHS does a 'bad' job at providing patients with the medicines they need. Though the survey didn't provide respondents with space to give specific comments, there are a few factors to which these negative responses could be attributed. One possible factor is variability in geography or, what is often described as, the 'postcode lottery'. This refers to the reality that the quality of NHS care that patients receive can differ depending on where the patient lives. As a result, it is possible that people's perceptions of the NHS's ability to provide patients with the medicines they need are impacted by their own personal experience.

Another possible factor might be variability in patient need. Most patients will only require common over the counter or prescription medicines that are relatively cheap, safe and easy to acquire. Conversely, patients who have rare or ultra-rare diseases are likely to have very different experiences in accessing the treatment they need, and this is likely to influence their perceptions of the NHS's performance.

Fig 1: Survey question: In general, how good or bad a job do you think the National Health Service (NHS) does at providing patients with the medicines they need?



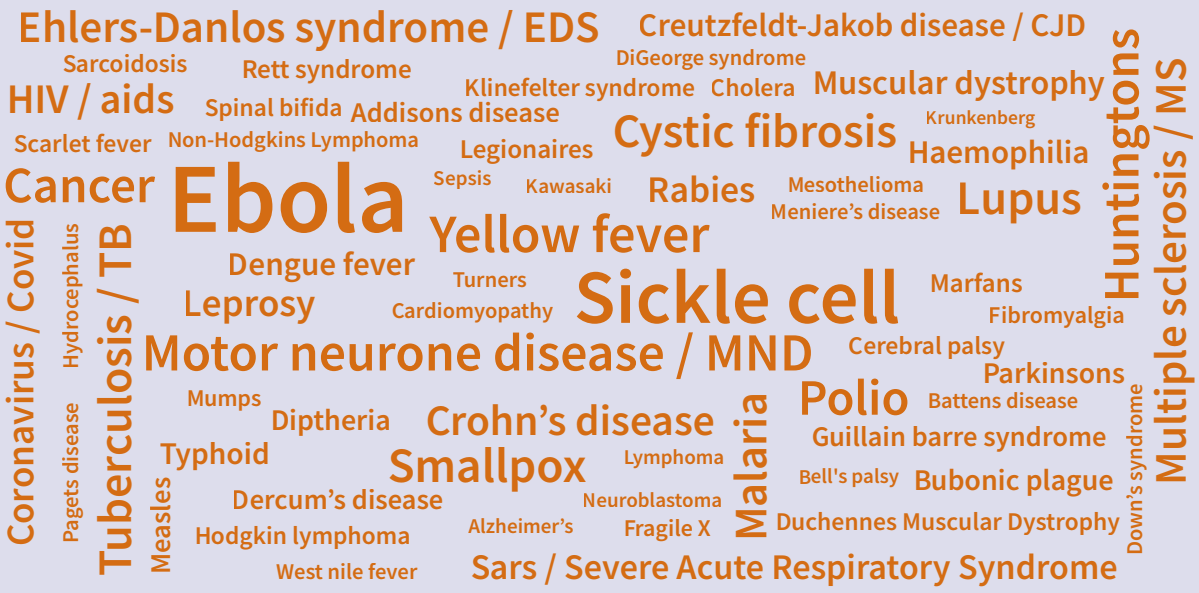
Public awareness of the challenging environment for rare diseases

Public recognition of rare diseases can be seen as a product of the attention they are given by media and political stakeholders and even then that recognition is limited."

Rare diseases by their nature are rare, and as such are less well recognised or known by the general public. We asked people to tell us what rare diseases they were aware of to understand whether any in particular have made an impact on the public consciousness. Figure 2 below shows the breadth of responses from the public when asked what rare diseases they were aware of.

None of the diseases below received more than 4% of responses from the public and the highest response was for Ebola – likely a reflection of the largest Ebola outbreak in history in 2014–16, which received global media and political attention.[11] Similarly, the higher number of responses for Motor Neuron Disease is possibly due to the 'ice bucket challenge', which became prominent online in 2014–15.[12] Public recognition of rare diseases can be seen as a product of the attention they are given by media and political stakeholders and even then that recognition is limited. That of course should not mean that they should be neglected, however that is the experience of many people living with rare diseases. Genetic Alliance undertook research in 2020, which found that 64% of people living with rare diseases feel that the 'the system is unfair on people living with rare conditions', and 66% agreed that 'the system is too slow to make decisions'.[13]

Fig 2: Before taking this survey, what, if any, rare diseases were you aware of?

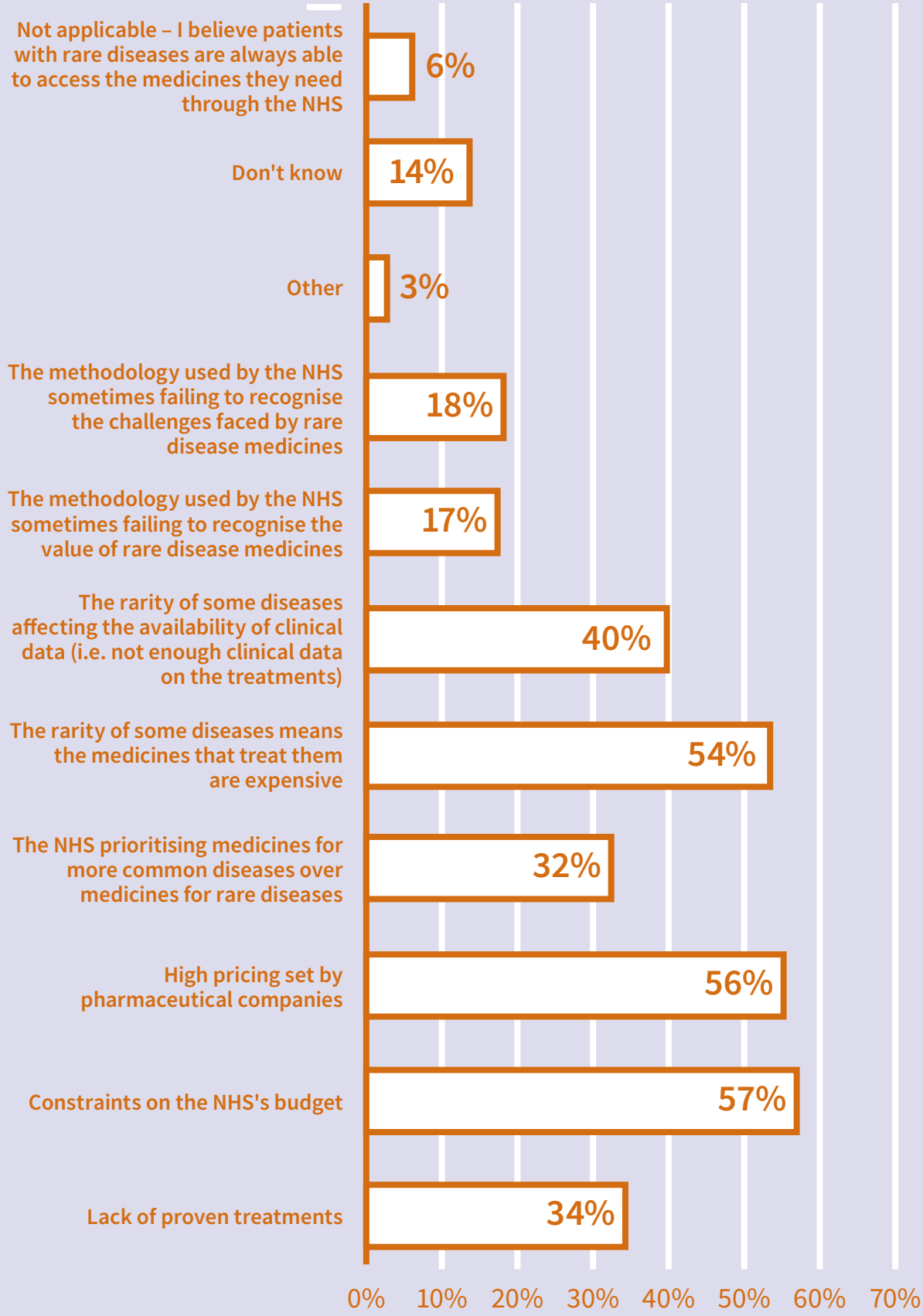


11 Centers for Disease Control and Prevention, *2014-2016 Ebola Outbreak in West Africa*, 2019. Available online via: www.cdc.gov/vhf/ebola/history/2014-2016-outbreak/index.html

12 BBC Online, *Ice bucket challenge: What's happened since?,* 2015. Available online via: www.bbc.co.uk/news/health-33640896

13 Genetic Alliance, Rare Experience 2020 report – Genetic Alliance, 2020. Available online via: https://rareexperience2020.geneticalliance.org.uk/wp-content/uploads/2020/12/Rare-Experience-2020-Report-.pdf

Fig 3: Reasons why patients with rare diseases unable to access the medicines in NHS.



Respondents recognised many of the key issues which present barriers to access to medicines for rare disease medicines."

We asked people what reasons they thought there might be for limited access to medicines for rare diseases to see how well the public understood some of the unique challenges these medicines face. In recent years, there has been a significant focus in the media and political spheres on medicines described as being 'high-cost'. This is reflected in the high proportion of respondents who highlighted the high cost of medicines and constraints on the NHS budget (56% and 57% respectively). While some individual cases have been difficult for industry/NHS relations they have ultimately led to greater openness and engagement in commercial negotiation between industry and the NHS to secure patient access to treatments. In particular, the Commercial Framework for New Medicines has provided much-needed clarity to the process and alleviated many of the issues that drove at times difficult debate.[14]

Respondents also recognised many of the key issues which present barriers to access to medicines for rare disease medicines, in particular, that the rarity of some diseases means that medicines that treat them are more expensive (54%), rarity affecting the availability of clinical data (40%) and lack of proven treatments (34%).

Very few respondents thought that people living with rare diseases faced no challenges accessing medicines for their condition (6%) and, indeed, just under a third (32%) said that they thought the NHS prioritises medicines for more common diseases over rarer ones. This is reflected in the responses of people living with rare diseases to the Genetic Alliance's patient experience survey.[15]

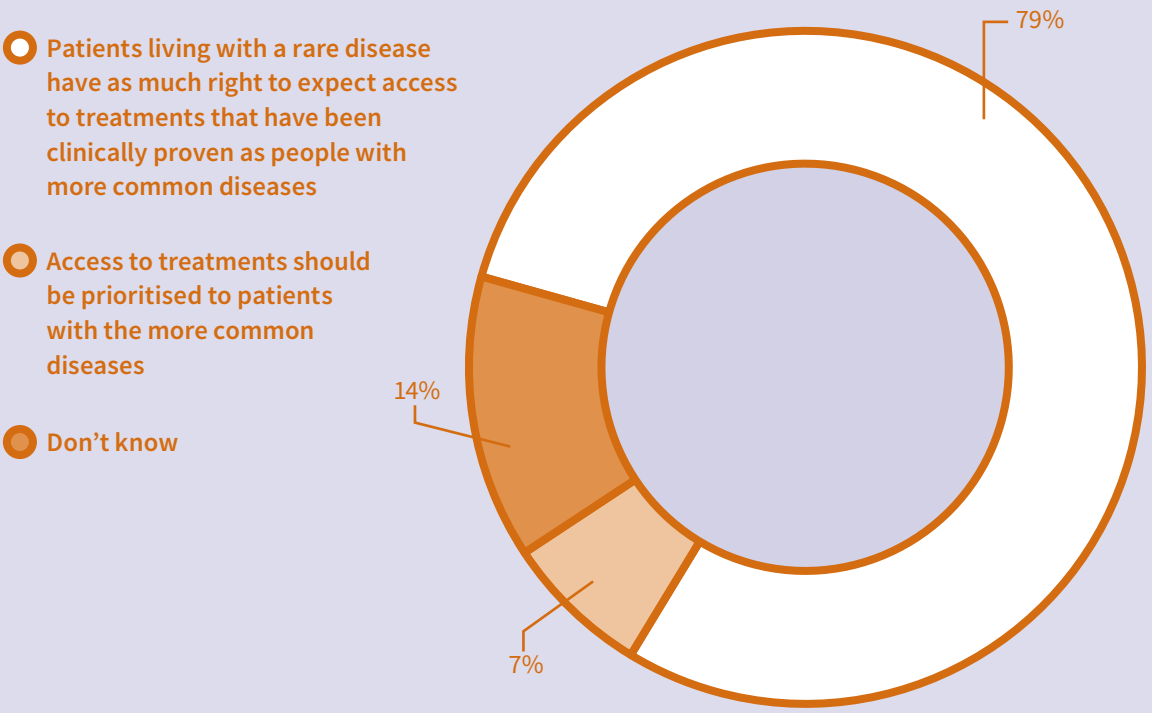
14 NHS England and Improvement, *NHS commercial framework for new medicines*, February 2021. Available online via: www.england.nhs.uk/wp-content/uploads/2021/02/B0255-nhs-commercial-framework-for-new-medicines.pdf

15 Genetic Alliance, Rare Experience 2020 report – Genetic Alliance, 2020. Available online via: https://rareexperience2020.geneticalliance.org.uk/wp-content/uploads/2020/12/Rare-Experience-2020-Report-.pdf

Public views on ensuring access to medicines for rare diseases

In its 'case for change' consultation on its Methods and Processes, NICE said "the review found limited evidence that society values health benefits for rare diseases more highly".[16] However, our research suggests the opposite. We asked respondents to say whether they agreed or disagreed with a number of statements relating to access to medicines for rare diseases and medicines for more common diseases.

Fig 4: Should patients living with a rare disease have as much right to expect access to treatments that have been clinically proven as people with more common diseases?



16 National Institute for Health and Care Excellence, The NICE methods of health technology evaluation: the case for change, December 2020. Available online via: www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/chte-methods-consultation

Fig 5: Should patients have access to a treatment based on clinical need, even if this would be costly to the NHS because of the rarity of the disease.*

- Patients should have access to a treatment based on clinical need, even if this would be costly to the NHS because of the rarity of the disease
- The NHS should not be expected to take on the cost of treating rare diseases
- Don't know

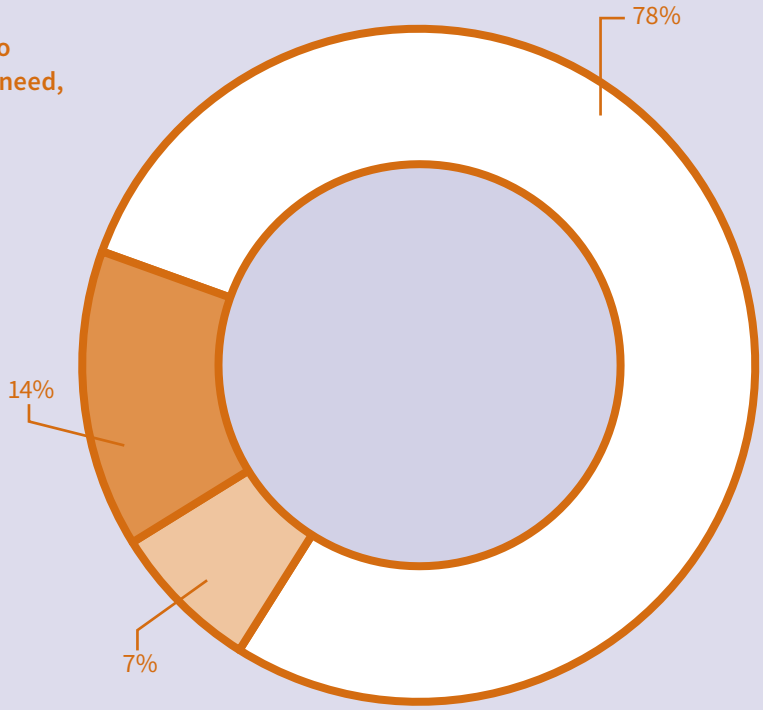
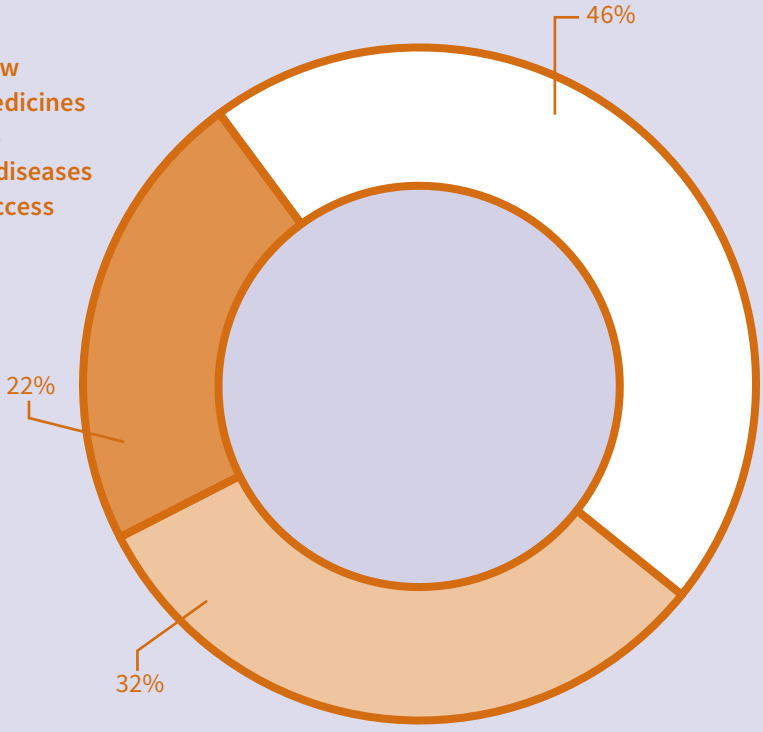


Fig 6: Should the NHS routinely allow a higher cost threshold for medicines for rare diseases compared to medicines for more common diseases if this would ensure patient access.

- The NHS should routinely allow a higher cost threshold for medicines for rare diseases compared to medicines for more common diseases if this would ensure patient access
- The NHS should allow the same cost threshold for medicines of all diseases equally, regardless of how rare the disease is
- Don't know



* The percentage figures here have been rounded and down to the nearest whole number and so may not add up to 100%

The vast majority of people agreed patients living with a rare disease should be able to access medicines on the same basis as people living with more common conditions."

Far from suggesting that society does not value measures to ensure that people living with rare diseases, in particular, are able to access medicines, these data suggest that people understand the challenges faced by medicines for rare diseases and that they would support specific measures that would ensure that patients are able to access them.

When asked if patients living with a rare disease should be able to access medicines on the same basis as people living with more common conditions, the vast majority of people agreed (79%) compared to only 7% who explicitly disagreed. This strongly suggests that people value comprehensive access to healthcare and treatments rather than a more brutally utilitarian view that the NHS should prioritise more commons conditions.

Similarly, when asked if the NHS should ensure access on the basis of clinical need even this would be more costly to the NHS because of a disease's rarity, the vast majority (78%) also agreed with this statement.

These results do not stand in isolation. In 2014, the Association of the British Pharmaceutical Industry published research which showed that 84% of the public agreed that the NHS should ensure that patients suffering from rare conditions have access to the widest possible range of medicines regardless of cost'.[17] Indeed, the same research also found that almost a third (32%) of people would be more likely to vote for a political party that promised to spend more on ensuring that people with serious or life-threatening illnesses could have the latest medicines, even if this meant savings needing to be made elsewhere.

Also in 2014, the BIA published a report which showed that 68% of people agreed that the NHS should ensure patients with very rare diseases have the same access to treatment as patients with common diseases, even if it means savings have to be made elsewhere in the NHS, with only 2% explicitly disagreeing.[18]

This shows that there is broad public interest in and support for protecting the universality of access to medicines through the NHS for rare diseases (in comparison to more common diseases) even where this would require extra spending.

We also asked people whether the cost threshold for medicines for rare diseases should be raised to ensure access. We received a large number of 'don't knows' to this question (22%), which is not unexpected given the complexity of the HTA process and some of the terminology (e.g., threshold). A little under half (46%) agreed with the statement, whereas 32% disagreed. When 'don't knows' are removed, a majority (59%) were in favour of increasing the cost threshold for rare disease medicines to ensure access.

It is clear that there is public appetite for measures that would support access to rare disease medicines if would mean a higher cost and that raising the threshold is a palatable option for a majority of the public.

17 Association of the British Pharmaceutical Industry / Savanta: ComRes, *ABPI NHS spending public poll*, 2014. Available online via: https://comresglobal.com/polls/abpi-nhs-spending-public-poll
18 Bioindustry Association, *Strong Public Support for Equal Access to Treatment for Patients with Very Rare Diseases*, 2014. Report on file

Conclusion and recommendations

Access to medicines is an important and emotive issue. For people living with rare diseases, access may also seem like an uphill battle. While rare diseases are rare individually, taken together they affect 1 in 17 people across the UK, a significant section of the population, many of whom are children. Scientific advances are now providing hope for treatments where there has been none before. For example, new treatments for Spinal Muscular Atrophy are significantly extending and enhancing the lives of the children who develop the condition.

However, small patient populations and difficulty in obtaining data often throw up barriers during the assessment of medicines for rare diseases. Recent developments as part of the NICE Methods and Process Guide review show significant promise for remedying uncertainty issues and the planned severity modifier will be of significant benefit for a number of therapy areas, including many severe and debilitating rare diseases.

However, rarity in and of itself is a specific challenge and one that is not wholly or satisfactorily resolved by the reforms currently planned by NICE. We also remain concerned that the HST process in its current form and the form proposed by NICE in its review of its process guide does not live up to its promise to promote access to medicines for rare diseases. Its strict criteria too often result in medicines that would provide significant benefit to people with rare diseases if assessed under HST falling in the gap between STA and HST.

NICE has previously stated that it does not believe that there is appetite or interest among the general public for specific measures to tackle rarity as an issue, and in particular it has discounted the need for a rarity modifier.

The data presented here suggest a different view among the general public, who recognise many of the specific challenges rare disease medicines face in the HTA process and who also value a universal and comprehensive approach to medicines access, as opposed to a utilitarian one. Respondents to our survey and previous surveys undertaken by other organisations even go so far as to agree that the NHS should accept additional costs if it will ensure access to rare disease medicines.

We recommend that:

- **NICE considers the value of a rarity modifier as part of the HTA process to people with rare diseases and to the general public.**
- **NICE revisits its proposed criteria for HST to ensure that it provides a viable route to access for rare disease medicines.**
- **NICE revisits its position on the public's appetite for targeted measures to support improved access to medicines for people with rare diseases.**
- **The Department for Health and Social Care explores options for additional funding for rare disease medicines to ensure patient access in line with a comprehensive health offer within the NHS.**

Methodology

The BIA commissioned YouGov to undertake a public attitudes survey on views on rare diseases and access to medicines.

The field work took place between 7 and 8 April 2021 with 2,023 respondents in Great Britain (no responses were received from Northern Ireland).

Alongside desk research, the BIA analysed these results to develop this report.

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RDIG Members:

- Alexion
- Alnylam
- Amicus Therapeutics
- Biogen
- Ipsen
- Pfizer
- PTC Therapeutics
- Sanofi
- Sarepta Therapeutics
- Takeda
- Vertex

RDIG represents a group of innovative bioscience companies within BIA's membership that specialise in treatments for rare and ultra-rare diseases. RDIG is committed to developing recommendations that can pragmatically inform and improve the challenge of ensuring patient access to treatments for rare and ultra-rare conditions, sometimes referred to as orphan and ultra-orphan medicines.

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This report would not have been possible without the input and expertise from BIA member companies, who we thank for their contributions.

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