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One small step for the NHS, but one giant leap for its principles?

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Foreword

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This paper raises issues that are fundamental to health financing. The NHS faces an unprecedented period of austerity, with no clear plan to match available resources with the demand for healthcare. As Tony Hockley[1] demonstrates, past retrenchment in NHS coverage has seen big change achieved in small steps as routine items have fallen away from NHS coverage and private options developed. The unwritten contract between the NHS and taxpayers allows this to happen outside of any strategic plan or announcement. Now, it seems from the case of an ultra-rare disease highlighted in this paper that the new target for retrenchment may be at the opposite end of the care spectrum, as the treatment of serious but rare conditions are deemed unaffordable by ministers concerned more for the day-to-day NHS.

Economic austerity requires a serious discussion of the purpose of the NHS, so that taxpayers' money is targeted where it is most needed. Coverage of financially catastrophic but very rare events is one of the most common arguments for state intervention. A political approach based on gradual withdrawal from such coverage leaves everyone vulnerable. It also sends a signal of reduced support for bio-pharmaceutical innovation.

Even after the spending boom of the new millennium Britain's level of state spending on healthcare is broadly similar to those of many other developed economies. Despite the growth of discrete private markets set out in this paper, it still lags behind in total spending. The debate over "affordability" must take place within this context and a sustainable division of public-private responsibility must be found. In the meantime it seems that patients with treatable rare diseases may be the latest victim of opportunistic and covert retrenchment.

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Executive Summary

The NHS aims to provide comprehensive health care funded by general taxation rather than charges. This paper documents how the British health service has moved away from this principle during the past 30 years, as very minor initial changes in specific policy areas have led eventually to major changes towards private funding. It identifies another seemingly minor decision in 2013 that may lead to an even more dramatic shift away from comprehensive care, that is unlikely to be offset by private coverage.

The rejection of official advice strongly supporting immediate NHS adoption of the first effective treatment for a serious and very rare disease (aHUS) on overall “affordability” grounds may signal the shift of NHS retrenchment into the very core of life-saving care. The paper argues that the new approach warrants serious debate over the purpose of the tax-funded NHS. Ministers should give an honest explanation of their interpretation of "affordability" and their views on the apparent trade-off between life-saving treatments for the few and routine NHS services for the many, and explain the alternatives available to those affected by these decisions.

Introduction

The NHS was created in 1948 to remove fear of disease. A service funded mainly from general taxation could cover the parts that other systems might fail to reach. Effective care would no longer be out of the financial reach of any citizen.

Despite decades in which bits of the NHS have fallen off the edge, particularly long-term care of the elderly and adult dentistry, access to effective life-saving treatment has remained core to the principles of the NHS and a major justification for the retention of the unusual British way of funding healthcare. Amidst the NHS reforms of 2013 ministers overruled official advice that strongly supported NHS use of the first effective treatment for a very rare and very serious disease. Instead they asked that another assessment should take place in the future, with greater consideration given to the wider financial implications of treating such a rare disease.

In this paper I argue that this decision may herald the start of an extraordinary new path for the NHS. Health provision that is funded either by taxation or compulsory insurance is best placed to cover serious, rare disease. Firstly, the resources required will always be beyond the means of most households acting alone, and secondly, where very rare diseases may have a genetic component some people may find themselves uninsurable in private markets. In the absence of any serious discussion of NHS retrenchment, in which routine but insurable risks have fallen out of the NHS, the extension of retrenchment into uninsurable risks is a great cause for fear.

Redrawing the boundaries of the NHS

Unlike insurance-based systems the citizen's contract with the NHS is implicit rather than explicit. Changes to the scope of coverage are often visible only with the benefit of hindsight, as their consequences take increasing effect over time. This paper sets a recent ministerial decision over NHS treatment for a serious but very rare disease within this context of NHS retrenchment. It suggests that whilst the retrenchment of coverage for health needs is already well established, this

decision may herald a significant new front in the withdrawal of NHS coverage, pursued alongside the 2013 NHS reforms.

Past revisions to the scope of the NHS have been concentrated in the most prevalent areas of rising demand; characterised by largely predictable needs, potential quality improvements made possible with private funding, and a reasonable capacity for households to plan for the associated costs. In a decision not to follow a recommendation to make available NHS treatment for a life-threatening but "ultra-rare" disease, the Government appears to be shifting retrenchment policy onto new ground. If this proves to be the case, it represents a significant change in the contract between the state and households in England which warrants substantive debate: a decisive move away from the "rescue principle" rooted in social solidarity towards a new utilitarian approach to equity that favours the everyday over the exceptional.

Retrenchment of the tax-funded NHS has a well-established history within British health policy that dates back to the first days of the NHS[1]. After 1948 it quickly became clear that a tax-funded service could not, after all, provide the limitless cover that had been promised when Bevan decided to press ahead with a system based on nationalisation of the finance and provision of care, rather than follow the Beveridge model of social insurance adopted elsewhere in the development of the welfare state.

Although user charges were quickly legislated for in order to try to fill the funding gap, it was not until the economic crisis of the 1970s that the political consensus over the sustainability of a comprehensive NHS came under serious challenge, amidst growing discussion of public-private boundaries. During the 1980s the boundaries of the NHS were slowly redefined, as clinical advance rapidly expanded the range of possibilities for care, and rising household incomes supported increasingly consumerist solutions. Each change began with a very minor step, but with major consequences over time:

- In 1983 Supplementary Benefit regulations were amended to introduce limits on the amount by which local authorities could support the costs of private residential care, turning this little-known support into a popular right[2]. This marked the start of a programme that produced a dramatic public to private shift in the provision of long-term care[2]. This saw means-tested "social care" assume a role previously taken on by NHS-funded "health care" in hospitals and nursing homes. NHS and local authority provision of long-stay elderly beds fell from 60 percent of the total to just 20 percent[3] between 1983 and 1996. Local authority "social care" charges imposed on people placed in private care homes rose from £8m in 1985/6 to £595m in 1995/6[3].[4]
- In 1984 the government ended restrictions on the advertising of spectacles. This began a process that led to the replacement of NHS spectacles with a voucher scheme, the removal of universal free sight tests, and rapid growth in the private market for ophthalmic services. By 2005 the private ophthalmics market's annual value (sight tests and spectacles) stood at around £3bn, almost ten-times the NHS spend on these items[5, 6].
- Also in 1984 the government produced the first "black list" of items that could no longer be prescribed on the NHS. The incursion into clinical freedom led eventually to the creation of the National Institute for Health and Care Excellence (NICE) to determine access to

[2] Until then a relatively little used option under the National Assistance Act 1948
[3] At 1995/6 prices

medicines based in part on “affordability” to the NHS, as well as clinical and cost-effectiveness.

- Since 1983 Britain has also pioneered a shift towards self-medication instead of NHS prescription. By 1994 this private market had reached £1.2bn (equivalent to one-third of the NHS drugs bill). Immediately following the 2002 publication of the Wanless Report[7] on the future costs of the NHS the government took the unprecedented step of publishing a list of 50 medicines (including a statin) that it believed could be made available without prescription[8]. By 2011 private spending in the self-medication market had risen to £2.3bn[9], equivalent to more than one-quarter of the total NHS England drugs bill[10].
- After rapidly increasing NHS dental charges and the introduction of prior approval requirements for expensive work during the 1980s, a 1992 "clawback" from dentists of excess income (against an NHS target average) provoked many dentists to shift to private practice for adult dentistry, assisted by the recent creation of Denplan. Private spending on dentistry rose from a neglible base in the 1980s to almost £1bn by 1998[11], and spending on private dentistry was eventually to vie with NHS spending levels[12].

In each of these cases where a new direction towards a mixed market for care was taken, it was pursued within an environment of broadly predictable household need of the services concerned and a degree of affordability for a private alternative to NHS service. Despite heated contemporary debates at the time[4], in each of the areas of relative NHS retrenchment people have shown strong willingness and capacity to pay for the private alternative.

It is important to note that an observation that major policy change may begin with a very minor step does not imply that such change is intentional with the first step. Indeed, it is unlikely that the politicians involved in the cases mentioned above could have forecast the scale of change that would take place. The initial step may be simply opportunism or even accidental, but once new policy choices are opened in this way it is more than likely that the route of least public expenditure will be selected. Once this particular door is ajar it will be used.

Market Failure and Rare Disease

The average cost of researching and developing a new drug so that it is licensed for use on humans is estimated to have reached £1.2bn[20]. It is inevitable, therefore, that the most attractive areas for drug development will be where there is the greatest potential market. Given the scale of investment required market forces alone are unlikely to support significant levels of research into rare but serious diseases.

The US Government under President Reagan legislated in 1983 to introduce measures to offer financial and market support to these "orphan drugs", with considerable success[21]. In 1999 the European Union followed suit[22], on the basis that: *"Society cannot accept that certain individuals be denied the benefits of medical progress simply because the affliction from which they suffer affects only a small number of people"*[23].

[4] The removal of free sight tests reduced the majority of the re-elected 1987 Conservative government from 102 in 1983 to single figures in 1987.

Orphan Drugs will be very expensive per patient due to the very small patient populations involved, although it appears that prices appear to be predictable against similar classes of medicines for non-orphan diseases[24]. To qualify for designation as an orphan drug under the European Regulation the disease must be:

- either life-threatening or chronically debilitating, and prevalent in less than 5 in 10,000 people,

and

- the drug must promise significant benefit in the absence of a satisfactory alternative.

The main reward for qualification is the provision of 10 year market exclusivity[25][5], during which period a "similar" product will not be licensed for the same indication. Although this does not guarantee a monopoly, as other products can still enter the market, it has evidently provided sufficient incentive for companies of all sizes. National governments, including the US, may also support research into treatments for orphan diseases through the use of tax credits as they do in other priority areas.

The success of orphan drug legislation, with the associated costs for payers, has led some to question whether budget constrained state health systems should divert resources from mainstream to personalised care in this way. Furthermore, critics of the pharmaceutical industry balk at the high prices associated with orphan drugs, particularly where there may have been cheaper, but unlicensed, alternatives in use and an apparently simple adaptation of an existing treatment in order to obtain a valuable orphan designation[26].

The stream of treatments for otherwise poorly treated rare diseases since the European regulation, however, has also been seen by many as a cause for congratulation and encouragement to further such progress[27]. In a review of the debate over orphan drugs Kanavos and Nicod determine that problems stem mostly from limited data availability rather than from the legislation[28]. Indeed, where profits are excessive the European legislation already includes a provision to reduce the term of market exclusivity of a product, and regulators need to develop ways to assess appropriate returns and to guard against the use of orphan drug approval processes as a "trojan horse" for products that are intended for more widespread use.

NHS Reform and Ultra-Rare Disease

Some diseases and treatments are so rare that in a country the size of England their treatment might only be commissioned at the national level. Inevitably such processes can conflict with attempts to decentralise the NHS. At the time of the last such decentralisation (2003) the author was asked to survey Primary Care Trusts in England to develop an understanding of how specialised services were being commissioned within the newly devolved system. The study revealed a wide variety of arrangements, of varying rigour, and with no national oversight[13]. The Government later committed to improve the situation for this important group of diseases and treatments, and took further steps to do so following the 2006 report from the independent review led by Professor Sir David Carter. [14].

[5] The European Union will not accept another licence application during the period of exclusivity for a similar product for the same disease. This can later be reduced to six years if deemed appropriate.

One of these steps was the creation in 2010 of a group (known as AGNSS[6]) to advise ministers on which highly specialised services[7] should be commissioned nationally. At the end of 2011 in its Annual Report the new Group reported that Ministers had agreed with all of the seven recommendations for national commissioning that it had recently made[15]. Exactly a decade on from the last decentralising NHS reform, some ultra-rare disease patients once again find themselves facing a lottery of access to diagnosis and treatment as a by-product of the structural reform of the NHS and the threat of NICE as a cost-per-QALY[8] barrier to future new treatments. For ultra-rare diseases 2010 to 2012 may prove to have been a short honeymoon period following the Carter Report, when a co-ordinated national approach tailored to ultra-rare diseases was implemented.

Politicians of all parties in Britain have defended the NHS system of tax-funded care on the basis that a single national risk pool provides an efficient means of comprehensive coverage against exceptional needs. Nowhere is this more relevant than in the case of very rare and very serious, but treatable, disease.

Something akin to Jonsen's *"rescue principle" [16]* lies at the heart of most publicly-funded healthcare systems; reflecting public views of equity in the allocation of health spending. Rational, utilitarian systems for allocating resources find themselves constrained by a lack of public willingness to stand by and see individuals "doomed" when they might be rescued. It is the *"hit by a bus"* argument, and it is particularly relevant to the NHS[17] with its unmatched promise to provide a comprehensive, free-at-the-point-of-use service that eliminates the fear associated with potentially ruinous treatment costs, thereby contributing to the maintenance of a British view of civil society[18].

The question at the heart of this commentary, however, is whether the rescue principle remains central to health policy in Britain in a time of fiscal austerity. Inevitably in such circumstances the opportunity costs of effective but expensive treatments for individual patients weigh heavily upon health and finance ministers, as costs per QALY are given great importance as justification for rationing decisions, despite the many factors involved in the "value" of drugs, particularly those for serious rare disease[19].

Decisions over rare disease treatments were moved from AGNSS to NICE as part of the 2013 health reforms. Decision-making responsibility was also passed from ministers to NHS England administrators. This will have served to reinforce fears that dispassionate calculations of the opportunity costs of very specialised treatments would gain the upper hand in NHS rationing decisions. After 1st April 2013 cold utilitarianism could be better placed to win the battle against the public's affection for the rescue principle.

[6] Advisory Group for National Specialised Services
[7] With fewer than 500 patients and/or provision in four or fewer specialist centres in England
[8] Quality-Adjusted Life Year

The case of aHUS[9]

aHUS is a blood disorder that is often, but not always, inherited. Prior to the licensing of eculizumab in 2011 there was no treatment available to prevent death or organ damage, and up to 25% of patients would die following their first attack[29]. Plasma exchange and dialysis have been the mainstay of managing the disease process in those who survive. The arrival of a drug treatment has transformed patients' prospects, both in the context of the first attack and over the longer term[30, 31].

In the whole of England there are currently less than 20 reported new diagnoses each year of atypical Haemolytic Uraemic Syndrome (aHUS); many of these are children. The total number of patients in England in 2012 was 140 and at any one time in England there will be between 2.7 and 5.5 cases per million population with a confirmed diagnosis. [29]. Eculizumab therefore falls into the category of an "ultra orphan drug" for an "ultra-rare disease" (URD).

The impact of the disease on patients and their families can, of course, be devastating, particularly as it often first strikes in very early childhood and can be inherited[32]. When it is diagnosed it is commonly in response to emergency hospitalisation with kidney failure, and parents face the prospect of the situation being repeated in their children. Some parents have lost several children to aHUS[32].

In considering the balance of costs and benefits for NHS use there are, of course, complicating factors around such an assessment in the context of ultra-rare diseases. These are related mainly to the very small scale of trials, and the inevitable high costs involved in meeting the needs of such small groups of patients [30]. The Advisory Group for National Specialised Services (AGNSS) was created to assess these treatments within this unique context and to ensure that *"postcode prescribing" was avoided"* [10].

In the case of Eculizumab the decision to recommend national commissioning of the treatment appears to have been relatively straightforward, with almost universal support amongst the 17 participating members[11]. AGNSS assessed, for example, that:

> *"It seems clear from the evidence presented that the drug is effective in halting the disease process. It is close to the top end of the scale of effectiveness. Preventing the disease process, which is active throughout the body, improves quality of life in patients with long-term aHUS. In newly diagnosed patients, a particular benefit is in preventing kidney damage, so that newly diagnosed patients will not go on dialysis. The drug will also enable patients already on dialysis to receive a transplant" [29]*

With regards to the cost of treatment with Eculizumab the AGNSS assessment concluded that the cost per Quality Adjusted Life Year (QALY):

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[9] Atypical Haemolytic Uraemic Syndrome

[10] It should be noted that the EU process of qualification for orphan drug status will have already established that the treatments that would face the additional step of assessment by AGNSS in England represented significant clinical advances, worthy of special support

[11] See the Annexe to this paper for a summary of the factors AGNSS considered in support of the positive recommendation

> *"is in the lower end of the range of costs per QALY which have been estimated for the ultra orphan drugs, which is as expected if the drug is highly effective".*

The assessment noted that the number of patients would rise due to improved rates of diagnosis[12] and patient survival as a result of treatment with eculizumab. The group, therefore, recommended to ministers that as the numbers of patients rises due to the national commissioning of an effective treatment then the price of the treatment should be renegotiated with the manufacturer.[33] Within the context of very rare disease it will be normal that the number of patients will increase following the creation of a national commissioning scheme. Growth in patient numbers is foreseen in most AGNSS recommendations[15] as diagnosis of these conditions improves.

AGNSS initially assumed that as a result of its recommendations a national service for the management of patients with aHUS and commissioning of eculizumab for such patients would be available from October 2012, although this was revised to 1st January 2013 by the hospital that would act as the national centre for this service[13]. But, after several months of delay, the health minister, Earl Howe, announced in January 2013 that eculizumab would be subjected to a second assessment, under a new system for specialised services to be established within NICE sometime after the NHS reforms of April 2013. It would be the first such assessment by NICE following the abolition of AGNSS.

The Minister stated that whilst he accepted the AGNSS analysis on the clinical effectiveness of the drug he wished for:

> *" further advice on its suitability for direct commissioning taking account of its cost, benefit, and affordability"*[34].

In the meantime nationally-funded access to the treatment would be on an 'ad hoc' basis through Individual Funding Requests (IFRs). In April 2013 NHS England confirmed this approach, stating that it would fund IFR access only for newly-diagnosed patients, and for patients who already had access through their Primary Care Trusts, but not those who are already diagnosed and not receiving the drug or who are receiving it within a clinical trial or on a compassionate use basis[35]. It also meant that many aHUS cases would remain undiagnosed in the absence of a national service for the disease. The decision not to implement the AGNSS recommendation but to repeat the assessment under a new system within NICE once established, and provide selective access in the meantime, raises important issues of policy and principle. The conflict with the Coalition Agreement statement that top-down reform would not harm patient care[36] is perhaps the most obvious effect, given the severe delay in assessing this treatment caused by the transition to a new system to be created within NICE during 2013-14.

In addition to this immediate policy impact, however, the minister's decision to look again at the "affordability" of a recommended treatment for a serious, rare disease may suggest a new approach towards NHS rationing relevant to the core principles of the health system. Once again, a

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[12] One effect of national commissioning of an ultra-rare disease would be that diagnosis rates would improve across the country, rather than be concentrated around one or more centres of specialist knowledge (For example in 2010 the rate of diagnosis was twice as high in the North East of England compared to the rest of the country. The specialist centre for aHUS is in Newcastle).

[13] Newcastle upon Tyne Foundation Trust

small step may have been taken that leads eventually to a major change, whatever the initial intention. Clinicians treating rare diseases are already concerned about the transfer of assessments to NICE[37], as AGNSS was created precisely because NICE was considered inappropriate for these diseases. That the first assessment is focused on the affordability of such a drug already approved by AGNSS, and with no question over its effectiveness, these fears will be heightened.

Serious rare disease, equity and a comprehensive NHS

The Citizens Council of NICE offered advice on ultra-orphan drugs, linking their availability to the "social solidarity" and "altruism" that underpins England's tax-funded health system[38]. The Department of Health's ambition for specialised commissioning within the new NHS was that *"commissioning arrangements must be fair and consistent throughout the country"* in order to achieve equity. This would be backed by a Specialised Service Commissioning Innovation Fund (SSCIF) to more quickly deliver high impact changes[39]. Furthermore, the Department of Health's 2012 Mandate for the NHS Commissioning Board established avoidable death as its first priority[40], seemingly drawing upon the rescue principle. But a ministerial decision to question advice on a treatable, life-threatening condition on affordability grounds appears to suggest other priorities.

It may be that this latest decision, over which ministers had deliberated for several months, takes the concept of equity in the NHS in a different direction with less emphasis on the rescue of small groups of patients with serious but rare diseases and greater emphasis on overall health gain across the population. Some have already argued that this should indeed be the case, concerned by the success of legislation to encourage the development of orphan drugs[41]. It is not, however, a path that should be pursued without debate within a tax-funded health system that is justified on grounds of social solidarity. The issues of equity involved are fundamental, particularly in the context of rare diseases with a genetic component, that has such a concentrated impact on individual families.

The financial challenge presented by recent successes in treating very rare diseases is by no means unique to England[42, 43], but with unusual roots and funding the NHS must address this in a uncharacteristically transparent and open way given the special principles of the NHS that are at stake.

Of course, the minister's decision on eculizumab for aHUS may not be intended as the beginning of a new, less supportive approach to serious rare disease coverage. There may be alternative explanations, perhaps with little more intention than short-term implications for the NHS. As a delaying tactic to limit the cost pressures on the NHS in the first year after the reforms, it seems an exceptionally small financial step to be traded against the known and lasting impact of missed diagnosis and delayed treatment on people with aHUS, particularly when weighed also against the costs of two assessments instead of one; burdening a yet to be established system within NICE for the evaluation of highly specialised technologies. More worryingly, it may simply be that the patient voice is particularly weak in the case of aHUS, given the very small numbers involved. In stark contrast, a new treatment (Ivacaftor) for a rare form of Cystic Fibrosis, with similar financial

implications for the NHS and levels of uncertainty about future patient numbers[14] [44], was able to by-pass both AGNSS and NICE[45], passing from licensing to approval in just six months, and with Scotland and Wales following this English approval. In this case the Cystic Fibrosis Trust had been able to mobilise a high profile campaign for the new treatment, a course of action which is unavailable to much smaller patient groups.

Conclusion

The NHS Constitution is, perhaps, the closest thing to a health insurance contract available to English households. The first of its seven principles commits the NHS to a *"comprehensive service available to all"* but with a wider duty to promote equality with particular attention to groups *"where health and life expectancy are not keeping pace with the rest of the population"*[46]. The 2013 decision to overrule advice on aHUS treatment appears to signal a possible shift of emphasis with regard to these first principles of the NHS. Within a calculating and rational response to austerity, the focus on these particular groups may be lost.

Some will argue that the rescue principle has little place in modern systems of health funding. In doing so, however, they also undermine a core justification of a system funded from the single risk pool of general taxation. If models based on explicit insurance contracts cover small but catastrophic treatable risks, and the implicit "comprehensive" contract of the NHS does not, then public support for the current system, founded upon the elimination of fear of disease, may also legitimately be called into question. This challenge is by no means unique to aHUS, but is already being raised in the context of other rare diseases, including cancers[47] as diagnosis improves and personalised care develops. Patients, including undiagnosed patients, with aHUS are, however, the first to be subjected to a double jeopardy of two national rationing assessments following EU approval of an ultra-orphan drug to treat them, which had already passed the test of "significant benefit".

In the recent past, when overall "affordability" issues around new treatments have caused concern the NHS has resorted to payment-by-results "risk-sharing" arrangements with manufacturers to reflect uncertainties over effectiveness [48]. In the case of a treatment that available evidence shows to be consistently and highly effective, any fall back upon such arrangements would, of course, be unattractive to health ministers.

This case does seem to raise fundamental issues around the changing role of the NHS. The implicit contract with the nation has gradually changed over time. This has not been as part of the frequent, high-profile top-down structural reforms, but in small steps from small beginnings, albeit often obscured by structural reform. Where contracts are explicit, under social insurance or health savings accounts systems, options are available (or mandated) for catastrophic cover, but within the implicit "comprehensive" contract offered by the NHS there has never been such a need. If, however, the opportunity costs of preventing death and serious ill-health in very small patient groups are to be given lesser weight in the allocation of NHS resources, then a new solution must be found. In the cases of NHS retrenchment cited earlier it became clear that the NHS had been

[14] AGNSS decided in 2012 that it could not assess Ivacaftor because potential patient numbers were too high for this special assessment process for ultra-rare diseases (AGNSS Minutes, 8 December 2011)

crowding-out private alternatives. In the treatment of serious ultra-rare disease this is much less clear and there may be no alternative.

Cover for serious rare disease is a core justification for state intervention in health, as has been made consistently clear in debates over the NHS since the 1940s. Some ultra-rare diseases such as aHUS also have a genetic component, which makes private insurance coverage problematic for affected families. That politicians should now decide to give greater weight to the opportunity costs of NHS coverage for the only effective treatment for such conditions will rebuild the fear of disease that Bevan sought to eliminate with the creation of the NHS. It is important if ministers do not intend to shift the NHS away from serious rare diseases that they bring clarity to their views on affordability and the balance of priorities between treating rare disease and providing routine care. Whilst aHUS patients may be the first to face this new environment following the 2013 NHS reforms, many other breakthrough treatments for serious but very rare diseases will soon follow. The need for clarity is urgent.

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Annexe

Factors considered by AGNSS in support of a recommendation that Eculizumab should be nationally commissioned for use on the NHS

- **"Severity of disease** (25% immediate mortality, 60% are on dialysis within two years of diagnosis). AGNSS heard first-hand testimony of the very poor quality of life on dialysis
- **Lack of effective alternative treatments** (the above outcomes occur despite the use of current best available treatment, including plasma exchange)
- **Clinical effectiveness** of Eculizumab (potential reversal of kidney damage, benefits to all patients, enabling successful kidney transplantation)
- Eculizumab is life saving and patients can expect to return to a high quality of life following prescription of the Eculizumab
- Rarity, in terms of **equity of opportunity to benefit**
- The fact that aHUS is an inherited disorder that affects **whole families** with many families having lost several family members to the disease
- **Significant non-health benefits**, for example patients and carers being able to return to education or employment
- **Innovation** (the contribution to British life sciences of supporting research into and the development of treatments for rare diseases)"

Source: NHS Specialised Services, AGNSS. Minutes of Meeting held 14 June 2012
http://www.specialisedservices.nhs.uk/document/eculizumab-treatment-atypical-haemolytic-uraemic-syndrome/