The Challenge of Rarity – Putting the N in the NHS

England's new approach to commissioning services, products and technologies for small patient populations
The Specialised Healthcare Alliance (SHCA) is a broad coalition of patient groups supported by a smaller number of corporate members.

We were established in 2003 to campaign on behalf of people with rare diseases and other complex conditions which require specialised medical care. These conditions also tend to be both complex and expensive to treat. Examples are numerous but include certain cancers, cystic fibrosis, haemophilia, neurological conditions and a wide range of services for children. Accidents or complications of more common conditions can also trigger the need for specialised services such as burns, pain management and spinal injuries.

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Executive summary

All health systems struggle to cope with rare medical conditions. Problems take many forms but perhaps the most pervasive include:

- Diagnosis – medical practitioners may fail to identify symptoms that they come across perhaps once in a lifetime, with potential for patients to experience lengthy delay and misdiagnosis en route;
- Treatment – allied to scarce clinical expertise, the options for treatment can often be limited with areas of unmet need making for poor prognosis;
- Cost – the small patient numbers involved and nature of some rare conditions can generate high treatment costs, ranging from intensive mental health therapies through complex surgical interventions to targeted pharmaceutical products.

At the same time, the importance of patients' own knowledge is nowhere greater than in the field of rare conditions, while the scope for related research to inform developments of wider significance to society is well recognised.

For these practical and strategic reasons, the National Health Service in England has for many years commissioned (ie planned, procured and monitored) a number of services for very rare conditions at national level.

A review of these arrangements was undertaken in late 2009 and a range of changes is now being introduced. In particular, recognising the limitations of NICE’s approach to assessing cost effectiveness where very small patient populations are concerned, an ethical decision-making framework has been developed to allow assessment for national commissioning to be extended beyond services to a small number of products and technologies, usually for populations below 500.

This report explores the background to these important changes in the belief that they might have relevance to the wider NHS and other countries. It concludes with a series of recommendations including the need for:

- clarity about the criteria determining whether a product or technology will be assessed under the new arrangements or by NICE. These might be expected closely to reflect the decision-making framework;
- transparency in the process, so that all parties can see the major factors influencing decisions about which products and technologies should attract funding;
- a holistic approach, which demonstrably weighs all the complex considerations governing health gain, societal value, reasonable cost and best practice;
- robust regional arrangements for those services, products and technologies neither suitable for commissioning at national nor local level;
- ethical consistency in decision-making across the NHS drawing on the framework as a template.

Companies also need to show responsible commercial behaviour in responding to society's wish to help people with very rare conditions in great clinical need.
History

Rare diseases and specialised services

The NHS attracts such strong public support because of its ability to provide truly comprehensive medical care free at the point of delivery, from stitching a simple cut to the most complex neurosurgery.

The EU has defined a rare disease as a condition affecting not more than five people in every 10,000 of the population; a definition which also determines whether drugs are eligible to benefit from the EU’s orphan drugs regime. Similar definitions exist in the US and Japan. There is no established definition of ultra-orphan though a prevalence of one in 50,000 has gained currency in the UK.

In England, the focus for healthcare purposes has been less on the prevalence of a condition and more on the planning population needed to support a viable specialised service to treat it. Prevalence and planning population are related but, for example, expensive capital equipment might raise the planning population for one condition compared to another with identical prevalence.

NHS levels of commissioning (2009)

In England, the statutory definition of a specialised service is one involving a planning population of one million or more. The NHS levels of commissioning are often illustrated schematically using a pyramid with the commonest services, commissioned and provided locally, at the base and the rarest, commissioned nationally and provided in a few centres, at the top.
Commissioning as a concept in the NHS derives from the purchaser provider split first introduced in the early 1990s, whereby previously integrated functions were separated in the belief that this would crystallise costs more effectively, introducing greater rigour into the management of contracts and more competition amongst a range of providers.

Acknowledgement of the need for some services to be planned at national level, however, preceded the purchaser provider split with establishment of the Supra Regional Services Advisory Group in 1983. This was superseded in 1996 by the National Specialised Commissioning Advisory Group or NSCAG. Both groups were situated within the Department of Health (DH) rather than the National Health Service. This arrangement worked well until NSCAG's location within the Department excluded it from the expansion of NHS budgets beginning in 2000, leading to the accumulation of a significant deficit.

The expression of commissioning at national level has also differed from the wider NHS where, at least in principle, competition or contestability has been seen as a major advantage. By contrast, providers of national services are limited in number both as a matter of fact and policy with a view to ensuring a sufficient concentration of patients and resource to support proper levels of clinical expertise, quality and safety. The report of the Bristol Inquiry into standards of paediatric heart surgery at the Bristol Royal Infirmary (July 2001) confirmed the need to align supply with demand in specialised medicine.

The first service to be commissioned nationally was for choriocarcinoma, a rare form of aggressive uterine cancer, which is commissioned nationally to this day.

Carter Report

The need to move NSCAG out of the DH into the NHS for budgetary and other reasons was recognised well before the 2005 General Election but swept up in a wider review of specialised commissioning which followed. In particular, Lord Warner, then Minister of State for Health, recognised that local Primary Care Trusts (PCTs) were failing to band together effectively for the purpose of regional commissioning and that the links between regional and national commissioning were insufficiently strong. He therefore commissioned Sir David Carter, an ex Chief Medical Officer for Scotland, to conduct a wide-ranging review.

Although the Carter Report published in May 2006 was largely concerned with regional commissioning, it had important ramifications for national services as well, setting up the National Commissioning Group (NCG) to replace NSCAG and transferring it into the NHS. Other reasons for the transfer included strengthening the NCG’s links with wider NHS commissioning; providing for improved coherence between the different levels of commissioning from local, through regional and supra-regional, up to national level; and bringing about greater accountability and transparency.
Carter structure for the commissioning of specialised services

Moreover, while national commissioning became formally the responsibility of ten Strategic Health Authorities, the NCG was established as a standing committee of the National Specialised Commissioning Group. The voting membership comprised 10 PCT Chief Executives representing the 10 regional Specialised Commissioning Groups (SCGs) dealing with all services involving planning populations above one million but falling short of national level.

National Commissioning Group

The NCG was established in April 2007 with largely clinical membership. As with its predecessor bodies, the NCG commissioned services, accepting applications from providers and deciding on their suitability for nationally commissioned status on the basis of criteria relating to the number of patients (usually no more than 400), the number of providers (usually a maximum of four up to seven) and evidence of clinical and cost effectiveness. The NSCG and NCG are serviced by the National Specialised Commissioning Team comprising a small group of NHS managers and three expert clinical advisers.

In the following three years, the number of nationally commissioned services grew from 35 to over 50 with a budget of approaching £500 million per annum. Funding for these services has been top-sliced from PCT budgets.
Reasons for 2009 review

Accountability

Although the growth in the NCG’s portfolio reflected its popularity with providers and patient interests alike, pressure soon mounted to re-visit the Carter settlement. In particular, the clear line of accountability which had existed between NSCAG and Ministers was lost within the new, more complex NHS arrangements.

This came to a head in the assessment of an application for a service to be commissioned in relation to paroxysmal nocturnal haemoglobinuria, a form of chronic haemolysis, with the major cost component taking the form of eculizimab, a monoclonal antibody. The NCG was favourably impressed by the product’s clinical efficacy but worried about its cost (£200,000+ per patient) referring the matter to the parent National Specialised Commissioning Group. The NSCG for its part was disinclined to commission the product given its expense, though two of its 10 SCG members were already doing so at regional level. The issue was referred in turn to the chief executives of the Strategic Health Authorities but they also declined to give clear advice to the responsible Minister. The Minister was therefore left in an isolated position, ultimately deciding that the service should be approved, presumably on the basis of the severe patient need.

NICE conundrum

At the same time, rumblings about the limitations of NICE’s approach to assessing the clinical and cost effectiveness of products grew louder during 2008 and into 2009.

NICE had long maintained that its methodology could be applied to orphan drugs in the same way as other products but recognised that this approach would rule out approval for most ultra-orphan products given the disequilibrium between development costs and market size. At the same time, a different approach to ultra-orphans within the same organisation might prove difficult to sustain.

Sir Michael Rawlins, Chairman of NICE, highlighted the limitations of randomised controlled trials in his 2008 Harveian Lecture, pointing out that they were impossible for very rare diseases and arguing for a move towards greater use of judgement in place of rigid hierarchies of evidence. NICE’s prioritisation process also places greater emphasis on treatments affecting larger numbers of patients with a bigger budgetary impact.

More generally, a series of decisions turning down or limiting the use of drugs widely available in other developed markets was turning up the political heat, especially in relation to treatments for cancer. This culminated in Professor (Sir) Mike Richards, the national clinical director for cancer being asked to look at the issue of top-up payments and whether these should continue to make patients ineligible to continue receiving the balance of their treatment from the NHS. Sir Mike concluded that, without any longer precluding top-ups, the emphasis should be on making the vast majority of treatments available on the NHS. With this in mind, he invited NICE to review its approach to end of life treatments. NICE in turn asked Sir Ian Kennedy, previously chairman of the Healthcare Commission, to look at value and innovation in its appraisal process.

The end of life review and the Kennedy study led to some adjustment of NICE’s approach at the margins but turned down more fundamental change such as an increase in the QALY threshold or the incorporation of broader societal benefits in establishing cost effectiveness. Perhaps mindful of the need to support life sciences in the wake of the financial crisis, the Prime Minister announced in January 2009 the establishment of an Office for Life Sciences to explore how the sector could be better supported. A multi-pronged blueprint included an innovation pass to allow funding of orphan products for a period of up to three years pending their appraisal by NICE. The position of ultra-orphans was, however, left in abeyance.
Societal values

Limits of cost effectiveness
The most zealous proponents of cost effectiveness maintain that it represents the only fair way to allocate resources for healthcare, since issues such as rarity should not distract from maximising the health gain available to the population as a whole from a given level of expenditure. Even if this were so, the degree of confidence about the way in which cost effectiveness is calculated would need to be of a very high order for it to be the sole arbiter of what should or should not be funded.

In truth, NICE's methodology, though impressive, is the subject of considerable debate. For example, many argue that costs and benefits outside healthcare should be taken into account. NICE rejects this primarily on the grounds that these costs are beyond its statutory remit and additionally hard to define and capture. There is also concern that employment-related benefits would build in age discrimination. Experience shows as well that small changes in particular assumptions can have a large impact on the apparent cost per QALY.

Citizens' Council
Societal attitudes toward cost effectiveness have been explored in a number of reports produced by NICE's Citizens' Council. Most notably:
- Ultra-orphan drugs (November 2004);
- QALYs and the severity of illness (July 2008);
- Departing from the threshold (March 2009);
- Innovation (July 2009).

Although the questions put to the Council have varied and elicited different answers in sometimes overlapping areas, the reports cast an interesting light on societal attitudes. In particular, the consensus appears to be that:
- There are circumstances in which NICE should be prepared to depart from its standard cost effectiveness threshold;
- Formulaic considerations of health economics are important but unlikely to suffice;
- Rarity on its own is probably an insufficient reason to justify paying a premium for treatment;
- The degree of severity and the amount of health gain are the more critical factors.

The report addressing ultra-orphan drugs specifically found that 20 out of 27 members were prepared to consider paying higher treatment costs than the norm for patients with very rare diseases reflecting, in descending order of importance:
- The degree of severity;
- If the treatment will provide health gain rather than just stabilisation of the condition;
- If the disease or condition is life-threatening.

Ethical dimension
NICE has sought to address the ethical dimension to its decision-making process through a document on social value judgements. At the heart of the document, by NICE's admission, Principle 3 states that:

“Decisions about whether to recommend interventions should not be based on evidence of their relative costs and benefits alone. NICE must consider other factors when developing its guidance, including the need to distribute health resources in the fairest way within society as a whole.”
Observers would be forgiven for questioning the distinction between evidence on relative costs and benefits and distributing health resources in the fairest way within society as a whole. Furthermore, at no point does NICE give weight to the Secretary of State’s direction requiring it to take account of clinical need and innovation as well as cost effectiveness. A lack of transparency in the way social values are weighed by NICE has also tended to undermine the document’s credibility.

Parliamentary role

At some point, bureaucratic principles often come up against essentially political judgements about the kind of society in which people wish to live. In a taxpayer-funded service such as the NHS, these judgements are ultimately vested in Parliament. They have most recently been enunciated in the NHS Constitution, which is broadly supported by all the main parties. In particular, the Constitution states that:

"Everyone counts. We use our resources for the benefit of the whole community, and make sure nobody is excluded or left behind."

This is widely interpreted to lend support to people with rare conditions and is, for example, referred to on the home page of the NSCG/NCG.
New arrangements

Single advisory group
Against that background, the DH consulted upon proposals to strengthen national commissioning in the autumn of 2009, completing the exercise shortly before the General Election in May 2010 and waiting for final sign-off until the new coalition government was installed.

The nub of the proposals relates to the (re-) introduction of a single group giving direct advice to Ministers without the involvement of the NSCG or SHAs. The more innovative aspect concerns the extension of the advisory group’s remit beyond services to a small number of products and technologies and the development of an ethical decision-making framework to guide its deliberations.

The Advisory Group for National Specialised Services (AGNSS) is due to get underway in the second half of 2010 with the first services commissioned under the new arrangements from April 2011. The decision on whether or not to commission services, products or technologies nationally will formally reside with the Secretary of State for Health.

Composition
AGNSS will be composed of a broad spectrum of interests intended to give credibility to its recommendations. In addition to representation from PCTs and SHAs, membership will include clinicians, lay and patient representatives, with more specific expertise coming from a health economist, ethicist, geneticist and pharmacist. Management of the nationally commissioned portfolio will be answerable to a sub-group of the London SHA with an emphasis on finance.

Appointments to AGNSS are made by the NHS Chief Executive in the case of the Chair and the NHS Medical Director for other members. Funding for services will continue to be top-sliced from PCTs as things stand but the new government is expected to bring forward radical proposals for NHS reform (see below).

The inaugural Chair of AGNSS is Professor Michael Arthur, Vice-Chancellor of Leeds University with an academic background in cell and molecular biology especially in relation to liver fibrosis. Professor Arthur has a clinical background and was Dean of Southampton University Medical School.

Ethical framework
The truly innovative part of the new arrangements concerns the development of an ethical decision-making framework to assist AGNSS in making its decisions. This reflects the limitations of NICE’s approach to assessing clinical and cost effectiveness, especially where rare conditions are concerned.

Preparation of the framework has drawn on a number of sources, including work by the Specialised Healthcare Alliance in 2008/09 which campaigned for its creation with particular regard to:
- Urgency – how urgent is the need?
- Gravity – how severe is the need?
- Benefit – what is the impact of treatment?
- Alternatives – what options are available?

The preparatory work drew on the Alliance and a wide range of other sources in drafting materials which were then discussed at a series of four multidisciplinary workshops.
The upshot was the development of a number of fairly standard procedural principles (eg around transparency and accountability) allied to a number of entry criteria determining whether a service, product or technology should be assessed by AGNSS and 12 core criteria informing the outcome of a full application, as part of a two stage process.

The 12 core criteria, centred around patients’ need, are broken down into four clusters as follows:

1. **Health gain (does it work?)**
   - a. Severity and ability of patient to benefit
   - b. Clinical safety and risk
   - c. Clinical effectiveness and potential for improving health

2. **Societal value (does it add value to society?)**
   - a. Stimulating research and innovation
   - b. Needs of patients and society

3. **Reasonable cost (is it a reasonable cost to the public?)**
   - a. Value for money compared to alternatives
   - b. Overall cost impact and affordability, including opportunity cost
   - c. Average cost per patient

4. **Best practice**
   - a. Accessibility and balanced geographic distribution
   - b. Continuity of provision
   - c. Economic efficiency of provision
   - d. Best clinical practice in delivering the service

More than 70 stakeholders with diverse expertise were consulted over four workshops to iterate the framework.

- **Workshop 1**: Primary care and Specialised Services commissioners
- **Workshop 2**: Consultants in public health
- **Workshop 3**: Medical ethicists
- **Workshop 4**: Health economists
- **Workshop 1**: Experts in the development of decision-making frameworks
- **Workshop 2**: Experts in healthcare prioritisation
- **Workshop 3**: DH, NICE
- **Workshop 4**: Representatives of the devolved administrations
- **Workshop 1**: Representatives of patient organisations, including specialised and non-specialised conditions
- **Workshop 2**: Clinicians involved in the delivery of a diverse range of services for specialised conditions
- **Workshop 3**: National Clinical Directors
  - Transplantation
  - Cancer
  - Equality and Human Rights
- **Workshop 4**: Representatives of the Royal Societies
- **Workshop 1**: Members of the NCG
- **Workshop 2**: Members of the NSCG
A copy of the framework is reproduced as an Appendix. It will be accompanied by supporting guidance for applicants and members of the advisory group firmly anchored to the core criteria. Furthermore, the framework makes clear that criteria should not be considered in isolation but working together, as part of a holistic assessment.

A key concern of policy-makers is to ensure that the AGNSS process is seen as different to NICE but equally demanding. Accordingly, while higher costs than the norm might be countenanced for ultra-orphan treatments benefiting patients in severe clinical need, the requirement for clinical efficacy will be demanding. This is expressed in terms of number needed to treat or NNT as a measure of the number of patients whose condition improves as a result of the treatment compared to the total number of patients treated. For more expensive services, products and technologies, this number will be expected to approach 1 i.e. 100 per cent of patients treated.

Overall, the framework represents an important development in service and health technology assessment, setting out in transparent terms the range of considerations to be taken into account in making funding decisions.

**NHS reform**

The new coalition government brought forward a White Paper on health in July 2010 as a prelude to a Health Bill in the autumn. This proposes major changes to the shape of the NHS and funding flows. In particular:

- An NHS Commissioning Board will be set up with autonomy from Ministers;
- SHAs will be abolished by April 2012 and PCTs the following year;
- The majority of commissioning and related budgets will be devolved to GPs acting in consortia covering populations averaging perhaps 100,000;
- Commissioning of national and regional specialised services will, however, be vested in the Board and a number of regional offices, to be determined.

Clearly, these proposals have ramifications for specialised commissioning, not least because funding presently flows via PCTs.

Delivery of the government’s overall plans will be challenging and highly dependent on support from GPs and the British Medical Association negotiating on their behalf. The positive upshot for national commissioning could be a simplification of arrangements, including more streamlined membership of AGNSS than present structures require.
Potential problems

NICE topic selection
NICE revised its topic selection criteria in 2009 introducing new criteria and weighting by population, disease severity, resource impact and claimed therapeutic benefit. Although rare diseases would achieve a low score for population, these criteria do not preclude selection of ultra-orphan products for appraisal, which would then fall foul of normal cost effectiveness requirements. A clear protocol is therefore required to create certainty about which products and technologies fall to NICE and which will be passed on to AGNSS for consideration.

Failure to apply framework
Although the framework represents an important advance in setting out the range of considerations which should be taken into account in deciding which services, products and technologies to commission nationally, a lot will depend on the interpretation placed on it by AGNSS and especially the Chairman. It will therefore be important for the Advisory Group to demonstrate that it is applying the framework as intended and for stakeholders to hold it to account on that score.

Surge in national commissioning
The number of nationally commissioned services has grown significantly in recent years. This does not necessarily matter where treatment is otherwise being commissioned locally, poorly and inefficiently. A surge of successful applications might, however, cause concern if it fostered the view that national commissioning was a soft touch compared to NICE, especially for products and technologies. This might be allayed if stronger commissioning at regional level facilitated more de-commissioning at national level.

Inflexible budget
A wholly justifiable increase in national commissioning could still cause problems if funding was not increased to meet it. A key consideration is that costs would otherwise arise haphazardly at local level.
**Recommendations**

In conclusion, England has a strong tradition of delivering high quality services to people with very rare conditions through the medium of national commissioning. This tradition has been maintained and prospectively strengthened through the latest changes in policy, which establish a single multidisciplinary group (AGNSS) to advise Ministers, drawing on a robust ethical decision-making framework and, for the first time, explicitly encompassing products and technologies as well as services.

Recommendations intended to ensure that the maximum benefit is derived from these changes include:

- **clarity about the criteria determining whether a product or technology will be assessed under the new arrangements or by NICE**
  Even if NICE continues to act as the clearing house for technology appraisals, it is important for all concerned to know what typically falls in its bailiwick and what falls to AGNSS. Any such protocol might be expected closely to reflect the entry criteria set out in the decision-making framework;

- **transparency in the process, so that all parties can see the major factors influencing decisions about which products and technologies should attract funding**
  Practical application of the decision-making framework will be a learning process for all concerned. It is therefore doubly important that AGNSS shares its decision-making with external stakeholders;

- **a holistic approach, which demonstrably weighs all the complex considerations governing health gain, societal value, reasonable cost and best practice**
  Similarly, AGNSS needs to show that its deliberations are demonstrably holistic, which will also assist in evaluation of the new arrangements;

- **robust regional arrangements for those services, products and technologies neither suitable for commissioning at national nor local level**
  The national commissioning arrangements do not sit in isolation. Experience suggests that the relative strength of national commissioning has encouraged patients and providers to apply for it and discouraged the movement of services back to regional level. The latest improvements in national commissioning arrangements therefore need to be accompanied by equivalent strengthening at regional level to establish balance in the system. The White Paper proposals may assist in this process;

- **ethical consistency in decision-making across the NHS drawing on the framework as a template**
  The ethical considerations set out in the framework are for the most part not unique to very rare conditions but variably applied to funding decisions elsewhere in the NHS, if at all. To quote from the NICE Citizens’ Council on QALYs and the severity of illness: “When NICE is making appraisals, we would like to see more emphasis on the social value judgement element of the decision-making process and within it, attention paid to the issue of severity.”

Finally, the latest changes to national commissioning and particularly the development of the framework, represent an important opportunity for the pharmaceutical and medical technology sectors to improve market access for innovative products which deliver real clinical value, even when the small number of patients makes for higher costs than the norm. In return, industry needs to reassure customers that pricing is legitimate and avoid accusations of emotionally-leveraged profiteering.
Advisory Group for National Specialised Services

Decision-making framework for making recommendations on national commissioning

The framework has two decision steps, which correspond to the two steps of the evaluation process:

- The framework allows a full and systematic approach to decision making, centred around patients’ need and based on clearly defined evaluation criteria.
- The framework has ten procedural principles, which should be followed in every part of the evaluation process.
- The evaluation process should be based on the two decision steps of the framework:
  - **Decision step one: Outline application**
    - Initial assessment, based on nine entry criteria, related to how rare the condition is and how complex it is to care for it.
    - Detailed notes for completion of the outline application will be provided as part of the application document.
  - **Decision step two: Full application**
    - Detailed assessment based on twelve clearly defined core criteria.
    - The core criteria are organised in four groups: health gain, societal value, reasonable cost to the public, and best clinical practice in delivering the service.
    - All criteria will be considered as part of each evaluation and the outcomes (including any conditions) will be documented as part of the rationale for the decision.
    - Detailed notes for completion of the full application will be provided as part of the application document.
    - Detailed guidance for evaluation of the full application will be provided and should be consulted during the evaluation process.
    - Following evaluation of the criteria, a holistic view should be taken across all criteria to decide whether the service should be commissioned nationally.
The ten principles describe the process for using the framework

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<th>OVERALL PROCESS SHOULD:</th>
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<th>DECISIONS SHOULD:</th>
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<td>Best quality evidence which is available</td>
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<td>Realistic predictions of future need</td>
<td>Support improvements to economically efficient or clinically effective service provision</td>
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<td>Demonstrate how the evidence has been considered in a robust and documentable process, able to withstand legal challenge</td>
<td>Clear criteria, which should be used as a structure for the evaluation and decision-making process</td>
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<td>Maintain consistency but allow some flexibility in balancing the relative importance of criteria</td>
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**OVERALL PROCESS SHOULD:**

- Be transparent and accountable
- Support rational decision making
- Demonstrate how the evidence has been considered in a robust and documentable process, able to withstand legal challenge
- Maintain consistency but allow some flexibility in balancing the relative importance of criteria
- Ensure that the criteria and the approach to using them are reviewed regularly to incorporate changes in external context

**PROCESS SHOULD BE BASED ON:**

- Best quality evidence which is available
- Realistic predictions of future need
- Clear criteria, which should be used as a structure for the evaluation and decision-making process

**DECISIONS SHOULD:**

- Promote accessibility and equity and reflect societal values
- Support improvements to economically efficient or clinically effective service provision

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**Decision step one:** The entry criteria of the framework consider how rare the condition is and how complex it is to care for it

**Entry criteria for national commissioning:**

- The product, service or technology will usually consist of no more than 500 patients and/or four centres in England.
- The product, service, or technology is clearly defined.
- The clinical need for national commissioning of the product, service, or technology is significant and well defined.
- There is a clear clinical pathway for the product, service, or technology, including criteria for referring patients and a co-ordination strategy for conditions that are served by more than one clinical specialty.
- The target patient group or subset is distinct for clinical reasons.
- There will be significant benefits from national commissioning and concentrated provision, which might include improved clinical quality, focused clinical expertise, more efficient use of NHS resources.
- The product, service or technology will have a greater clinical benefit than alternative forms of care.
- The product, service or technology can be accessed by all patients who are eligible for NHS treatment.
- There is enough evidence to determine that a full review of the product, service, or technology will be useful.

Outline applications are evaluated against the entry criteria. Successful outline applications go on to the second decision step.
Decision step two: The core criteria of the framework allow a full and systematic approach to making decisions

Core criteria for national commissioning:

- The framework consists of twelve core criteria organised in four groups
- The core criteria are defined in detail on the following pages

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<tr>
<th>Criteria</th>
<th>Aims</th>
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| Clinical effectiveness and potential for improving health | 1. To evaluate the effect of the product, service, or technology on the medical condition, based on the best available clinical evidence. The evidence could include the following:  
  - Data showing that the product, service, or technology saves lives or significantly improves quality of life, including an assessment of long-term outcomes (if possible). In each case, the most appropriate way of measuring the effects of the product, service, or technology on health should be used. For example, these could include an increase in life expectancy, improvements in a patient’s quality of life, benefits for the patient and other suitable measures.  
  - Details of any improvements to quality of life from carrying out tests which are approved by the UK Genetic Testing Network (for example, improvements from identifying a patient’s condition such as connecting patients and carers with a patient group).  
  - The number of patients whose condition improves as a result of the treatment, compared to the total number of patients treated (‘number needed to treat’, NNT). For more expensive services, products and technologies, this number should be close to 100% of patients (or NNT=1).  
  - A comparison of how clinically effective the proposed product, service, or technology is, with how effective any alternatives are. Alternatives should include standard NHS care and the best supportive care. |
| Clinical safety and risk | 2. To assess the timing of the application, taking into account how urgently the service is needed and the quality of the available evidence. |
| Severity and capacity to benefit | 3. To assess applicants’ plans to collect additional data on clinical effectiveness, and how they will evaluate the benefits of the service over the next five years. |
| | 4. To assess the safety record of the product, service, or technology, based on clinical evidence. If possible, this should include the number of patients who experience an adverse reaction to the treatment, compared to the total number of patients treated (‘number needed to harm’, NNH). Where available, compare international NNH statistics from similar treatments. |
| | 5. To consider the patients’ overall health and whether their condition is life-threatening or severely disabling. |
| | 6. To assess how likely it is that patients’ health will be improved by the proposed product, service, or technology. This includes health benefits to families, carers and society. |
### Criteria in group two: Does the product, service or technology add value to society?

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| **Needs of patients and society** | 1. To consider fairly and inclusively the needs and expectations of patients, patient groups and the wider society to which they belong.  
2. To consider the effect of a positive or negative decision in light of national NHS priorities.  
3. To assess how patients, carers and members of the public have been involved in identifying the need for national commissioning of the product, service, or technology.  
4. To consider the benefit to society from stimulating research and innovation in the relevant area of medicine. This will focus on the specific benefits from developing highly effective products, services or technologies for very rare conditions where conventional rules of investment may not apply.  
5. To consider the possible benefits from introducing new products and services to the UK to:  
  • Further service development and learning;  
  • Providing a world-class service; and  
  • Collecting additional evidence (through controlled innovation) on health outcomes, cost for each patient and the natural history of the disease through disease registries.  
6. National commissioning is not expected to fund research. |

### Criteria in group three: Is the product service or technology at a reasonable cost to the public?

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<tr>
<td><strong>Average cost per patient</strong></td>
<td>1. To consider the average annual cost for each patient or, if relevant, the average cost of the course of treatment.</td>
</tr>
</tbody>
</table>
| **Overall cost impact and affordability including opportunity cost** | 2. To assess whether the NHS can afford to pay for the product, service, or technology by considering the opportunity cost and avoidable costs, as defined in point four below.  
3. To consider the overall cost impact of the proposed product, service, or technology. The overall cost impact is the total cost of the product, service, or technology, less the costs of providing any current standard services to the same patient group. The review of the total cost of the product, service, or technology will include:  
  • A breakdown of all costs, including any relevant one-off costs  
  • An estimate of the minimum funding needed for a basic provision  
  • Any expected growth or planned increases in the service over the commissioning period (five years)  
4. Given the total net cost of the proposal, to evaluate the opportunity cost of the product, service, or technology at a local and national level. Opportunity cost is the effect of investing in the product, service, or technology, rather than in another NHS service (for example, health visitor hours or a different national service).  
5. To review any estimates of the annual health-related costs to society that could be avoided by providing the product, service, or technology (this includes increases in the costs of publicly funded health and publicly funded social care). |
| **Value for money compared to alternatives** | 6. To assess fairly how the value for money of the alternative NHS provision compares with that of the proposed product, service, or technology. This assessment will be based on the difference in health gain and the difference in cost when comparing the proposed product, service, or technology to:  
  • The current standard care for these patients in the NHS;  
  • Any other reasonable alternative treatments currently provided elsewhere in the NHS; and  
  • The best supportive NHS care available to these patients.  
7. To assess value for money across the different centres, taking into account differences that are due to different locations or models of care. |
Criteria in group four: Is this the best way of delivering the product, service or technology?

<table>
<thead>
<tr>
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</thead>
<tbody>
<tr>
<td><strong>Best clinical practice in delivering the service</strong></td>
</tr>
<tr>
<td>1. To evaluate the processes that providers will use to meet standards for clinical governance and for quality.</td>
</tr>
<tr>
<td>2. To review the estimated levels of activity and numbers of patients who will be treated at each centre. Applicants should support these estimates with incidence data (the number of new cases a year) or prevalence data (the total number of patients), and should show that the centres can, between them, provide for all eligible patients.</td>
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<tr>
<td>3. To consider whether national commissioning and concentrated provision would improve:</td>
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<tr>
<td>• The patient experience;</td>
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<tr>
<td>• The safety of the service;</td>
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<tr>
<td>• The way the team of clinical specialists coordinate their work; and</td>
</tr>
<tr>
<td>• The elements of care involved in providing a comprehensive service.</td>
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<tr>
<td>4. To review how input and feedback from patients and carers has influenced planning the service. This will include any contact with relevant patient organisations.</td>
</tr>
<tr>
<td>5. To consider the applicants’ plans for ongoing research and how information will be shared to support service delivery.</td>
</tr>
<tr>
<td><strong>Economic efficiency of provision</strong></td>
</tr>
<tr>
<td>6. To make sure that the proposed providers can provide the product, service, or technology in the most efficient way. The proposed model should make full use of any existing infrastructure and its efficiency should improve if commissioned nationally.</td>
</tr>
<tr>
<td>7. To assess how providers will share information between clinical teams and with other services, to improve efficiency in the future. This should avoid any unnecessary waste of time and resources.</td>
</tr>
<tr>
<td><strong>Continuity of provision</strong></td>
</tr>
<tr>
<td>8. To make sure that the proposed providers can provide the product, service, or technology for the entire commissioning period (five years). This will include a review of their plans for keeping and training staff, making sure they continue to have clinical experts (‘succession planning’), and for maintaining equipment.</td>
</tr>
<tr>
<td><strong>Accessibility and balanced geographic distribution</strong></td>
</tr>
<tr>
<td>9. To make sure all eligible patients can access the service, regardless of where in England they live.</td>
</tr>
<tr>
<td>10. To consider how applicants plan to work with local providers to deliver the service as efficiently as possible, and offer patients the best possible experience.</td>
</tr>
<tr>
<td>11. To encourage a holistic approach to care through appropriate links with social care and other forms of care.</td>
</tr>
</tbody>
</table>

Full applications are evaluated against the core criteria and successful proposals are recommended to ministers for national commissioning

- Full applications are evaluated against the core criteria
- In addition to considering each core criterion, the advisory group will look at full applications holistically. This review will take into account how the different criteria work together, including:
  - The balance of clinical benefits and clinical risks
  - The balance of cost per patient or treatment, clinical benefits per patient, and the robustness of the evidence for clinical benefits (clinical and cost-effectiveness of the treatment)
  - The balance of overall cost impact and overall benefits from national commissioning (overall value for money of a national commissioning approach)
  - The balance of the timing of the application with the urgency of the clinical need, what clinical alternatives are available, and the need to strengthen the evidence for clinical benefits
  - The robustness and realism of the financial, activity and governance planning
- Products, services and technologies with successful full applications are recommended to Ministers as suitable for national commissioning
- Proposals, which are approved by Ministers, go on to be commissioned nationally