Patient perspectives and priorities on NHS England’s commissioning of medicines for rare diseases

Patient Charter
Genetic Alliance UK

Genetic Alliance UK is the national charity working to improve the lives of patients and families affected by all types of genetic conditions. We are an alliance of over 180 patient organisations. Our aim is to ensure that high quality services, information and support are provided to all who need them. We actively support research and innovation across the field of genetic medicine.

Genetic Alliance UK undertakes various projects and programmes that add evidence and knowledge to improve health service provision, research and support for families. These initiatives include:

- Rare Disease UK, a stakeholder coalition brought together to work with Government to develop the UK Strategy for Rare Diseases.
  www.raredisease.org.uk
- SWAN UK (Syndromes Without A Name), a UK-wide network providing information and support to families of children without a diagnosis.
  www.undiagnosed.org.uk

Download a copy of this Patient Charter here:
www.geneticalliance.org.uk/nhsenglandcharter.htm

Published by: Genetic Alliance UK
Unit 4D, Leroy House
436 Essex Road
London, N1 3QP

Telephone: +44 (0)20 7704 3141
Fax: +44 (0)20 7359 7447
Email: contactus@geneticalliance.org.uk
Website: www.geneticalliance.org.uk

Registered Charity Numbers: 1114195 and SC039299
Registered Company Number: 05772999

Author: Louisa Petchey
louisa@geneticalliance.org.uk

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The Genetic Alliance UK team
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Executive summary

This Patient Charter makes six overarching recommendations for positive change to NHS England’s commissioning of medicines for rare diseases from the patient perspective:

Premise

NHS England evaluates and commissions more rare disease medicines than any other body and is well positioned to continue doing so while ensuring the patient voice is heard during decision making.

Recommendation

1. All seven of the evaluation and commissioning routes for rare disease medicines should be objectively considered

2. Greater patient voice is essential in decisions as to what should, and what should not be commissioned by NHS England
   - NHS England’s website should include information on the role of the Prescribed Specialised Services Advisory Group and a record of the rationale and outcomes of their decision-making
   - A simplified, streamlined and rational decision-making process should be developed for the Prescribed Specialised Services Advisory Group
   - Stakeholder engagement and public consultation should inform the development of a clear framework for determining ‘the line’ between specialised and regional commissioning

3. NHS England and the patient community need to work together to strategically address the financial sustainability of the commissioning of rare disease medicines
   - NHS England should be more transparent when describing cuts and efficiency savings
   - NHS England should engage more with the pricing of medicines and consider developing Patient Access Schemes (PAS)
   - Research that examines dosage should be encouraged, with the view to making medicines more effective and more affordable
   - NHS England should work with patients to support and improve their understanding of compliance and stoppage recommendations for medicines

For NHS England to fulfil its organisational promises to be ‘open and transparent’, ‘prioritise patients in every decision’ and ‘listen and learn’, it must optimise existing communications and engagement platforms

4. NHS England should prioritise the development of an online resource dedicated to their specialised commissioning activities
   - NHS England should ensure that all stakeholder engagement is effective, timely and accessible, and that its impact is communicated back to stakeholders
   - The voice of patients and patient groups should be recognised, valued and supported as a necessary partner in NHS England’s clinically driven model for specialised commissioning
NHS England’s Clinical Reference Groups should be granted additional resources to support their ability to give expert advice, and enable consistent decision-making and effective stakeholder engagement

- Best practice guidelines should be developed for Clinical Reference Groups in consultation with members and stakeholders
- NHS England should invest in greater administrative support for Clinical Reference Groups to manage their internal and external communications
- A technical review of the current Clinical Reference Group structure should be carried out to ensure coverage, capacity and expertise is appropriate across portfolios

NHS England’s evaluation process needs drastic streamlining and rationalisation to enable timely, patient-focused and transparent commissioning of rare disease medicines

- NHS England’s commissioning policy evaluation process should be streamlined
- NHS England’s governance structure should be rationalised with a defined role and decision-making framework for each stage
- The responsibilities of the Rare Disease Advisory Group should be redefined to take advantage of the group’s expertise and allow a greater focus on rare diseases
- NHS England should provide milestones within its evaluation timeline, with details of what action will be taken if a milestone is missed
- Horizon scanning information should be used by NHS England in collaboration with NICE to plan resource allocation with regard to future evaluations
- Medicine commissioning policies should be considered in parallel with service specifications when this will lead to speedier patient access
- Medicine commissioning policies that have been prioritised for funding should be implemented within three months
- The success of commissioning policy implementation and resultant expenditure should be measured
Introduction

The **NHS Constitution** states that we “have the right to drugs and treatments that have been recommended by the National Institute of Health and Care Excellence (NICE) for use in the NHS” and the right to expect “decisions on funding of other drugs and treatments to be made rationally following a proper consideration of the evidence”.

History has shown that medicines for rare conditions are unlikely to be appraised by NICE, with this being unlikely to change in the near future. Many patients affected by rare conditions must therefore access the medicines they need through NHS England and the routes it manages.

Each approach and pathway that can be used to determine whether or not a medicine will be made available on the NHS has its own set of criteria. The pathways can also differ in their ability to command funding, with precedence given to NICE-recommended medicines and NHS England apportioning the remaining budget.

Currently it is not clear how or why one medicine evaluation approach or access pathway is selected over another. A lack of system coherence also means that time and money is wasted because two publicly-funded bodies, NICE and NHS England, frequently evaluate the same medicine simultaneously. Moreover, with such a multiplicity of approaches and pathways, the risk of making inconsistent decisions that result in inequitable access to medicines for patients with rare conditions is high. As a result, some life-changing medicines are not being made available to the patients who need them even though less effective medicines are being funded.

The rare disease patient community want to understand how the decisions that determine access to medicines are made and ensure that the whole system is working effectively together. Patients recognise the need for rational use of resources, but rightly expect that decisions about their future health and care are made on a fair and consistent basis.

The current landscape for access to rare disease medicines

There are seven formal routes through which licensed medicines for rare conditions can be appraised and/or commissioned in order for them to be made available to patients through the NHS.

These are the **Highly Specialised Technology (HST) Evaluation Programme**, **Single Technology Appraisal (STA)** and **Multiple Technology Appraisal (MTA)** routes at NICE; evaluation through **Clinical Reference Groups (CRGs)** and the **Clinical Priorities Advisory Group (CPAG)** through NHS England, and the **Cancer Drugs Fund (CDF)**, Commissioning through Evaluation (CeE) and **Individual Funding Requests (IFRs)**, which are also managed by NHS England.

**NICE**

Historically, NICE has not been the primary route through which rare disease medicines have been considered for NHS-wide commissioning. Although the **European Medicines Agency (EMA)** introduced orphan designation for rare diseases medicines and began licensing them in 2002, peaking at 13 licenses in 2007, NICE only appraised its first orphan medicine in 2009. Since then, NICE has recommended 10 medicines, 6 of which have been for cancer indications. NICE have therefore only considered four non-cancer orphan medicines out of a possible 47, less than 10%. Various bodies within the NHS have made the commissioning decisions for all the rest.

The introduction of NICE’s **HST Evaluation Programme** in May 2013 was heralded as the route through which rare disease medicines would now be appraised. As we explored in our first **Patient Charter**, however, this route lacks the capacity or capability to effectively fulfill these aims.
NHS England
NHS England is a relatively new body, having come into being as a result of the passing of the Health and Social Care Act in 2012. Its remit in terms of determining whether medicines for rare diseases will be made available on the NHS is significant. Firstly, NHS England is the responsible commissioner for the specialised treatments and services required by those with rare conditions, meaning that it manages the available budget. It also coordinates its own evaluation procedure in order to determine which medicines should be prioritised for funding. Finally, it also is the managing body of the CDF, through which some cancer medicines can be accessed; CtE, a scheme that enables a treatment to be made available on a time-limited basis in order to determine whether it merits long-term commissioning; and the IFR process.

IFRs
The IFR process allows patients who would benefit from a medicine to access it even if they fall outside of the current commissioning arrangements for that medicine, providing that making it available is cost-effective. NHS England have stated that they do not expect this route to be used frequently as they intend for the majority of patients to be able to access appropriate medicines within existing arrangements.

Unfortunately, deficiencies within the current system for evaluating and commissioning rare disease medicines means that many patients with rare conditions are having to inappropriately go through the IFR procedure in order to access the medicine that their clinician agrees they need. This is because that medicine may not have been selected for formal appraisal and/or because no national prescribing policy exists (such as an NHS England commissioning policy).

While the IFR process is not designed to be used by a recognised cohort of patients with a clear clinical need, a lack of alternative options means this is taking place. When this occurs and the number of IFR requests made exceeds a national threshold, currently 20 applications, NHS England will consider developing a commissioning policy for that medicine (an ‘IFR trigger’). Until this proposal is evaluated and prioritised for funding, patients trying to access that medicine will be denied.
The aim of this Patient Charter
One of the key outcomes of our first Patient Charter was a clear call from the patient community for greater clarity on the entire landscape for access to rare disease medicines. Genetic Alliance UK therefore committed to developing this second Patient Charter, in consultation with our patient group members, in order to highlight and address gaps and inconsistencies within the system.

In the workshops we held with representatives from 20 patient groups, we explored all of the routes relevant to patients seeking access to medicines for rare conditions. In this Charter we outline the priorities for change identified by patient representatives related to NHS England in their capacity as the national commissioners of rare disease medicines and as the managing body of the CDF and CtE.

To complete the picture, we will be producing two reports that further explore access to medicines through the IFRs, also managed by NHS England, and the STA and MTA routes at NICE. These documents will make recommendations for the improvement of these processes from the patient perspective. The separation of these topics from the current Patient Charter reflects our belief that the importance of the issues raised in the workshop merit a separate output to ensure they receive due attention from NHS England, NICE and Government Ministers.

Specialised commissioning within NHS England
The way in which NHS England carries out its evaluations of rare disease medicines is one of the main focuses of this Patient Charter. NHS England’s governance structure for this process is summarised in the diagram overleaf. This diagram and the description of each component body that follows is based on the information available at the time of publication.

It should be noted that much of this information was not publicly available and was instead obtained through discussion with members of NHS England’s staff. In some instances, the role ascribed to each body according to NHS England literature differed from what patient representative’s had experienced of that body in action. We have tried to highlight these discrepancies where they arise. These caveats reflect the limitations of NHS England’s transparency in their methods and processes of medicine evaluation as well as the dynamic nature of NHS England at this time.

Prescribed Specialised Services Advisory Group (PSSAG)
PSSAG is a committee hosted by the Department of Health that provides advice to Ministers on what conditions, and therefore what services and medicines, fall within NHS England’s national commissioning remit. PSSAG is a multi-disciplinary committee with representation from Clinical Commissioning Groups, the Royal Colleges and NHS England as well as lay members. Ministers then consult with NHS England before making their final decision on any additions or changes to NHS England’s commissioning responsibilities.

With a proposal for NHS England to commission a medicine, this step through PSSAG is only taken if NHS England does not already commission a specialised service for that group of patients. If a service does not exist, a service specification must first be proposed to PSSAG and designated as specialised. Then the service can be evaluated by NHS England to determine whether it should be prioritised for funding and only if that is granted, can NHS England evaluate and consider the affiliated medicine and determine whether it is a priority for national commissioning.

Clinical Reference Groups (CRGs)
CRGs bring together clinicians, commissioners and public health specialists with patients and patient group representatives. There are 75 CRGs in total. CRGs have a broad role within NHS England’s specialised commissioning procedures in that they receive proposals from internal and external sources about new services or medicines that could be commissioned by NHS England and are then required to gather the relevant evidence and develop the service specification or medicine commissioning policy; the documents that are then evaluated by NHS England in order to determine what should be prioritised for funding. CRGs officially work in an advisory capacity within NHS England, although in prioritising their workload they also have a decision-making role.
**Genetic Alliance UK**

**National Programme of Care Boards (PoCs)**
CRGs for specialised services are clustered in to five Programmes of Care (PoCs). CRGs submit their service specifications and medicine commissioning policies to PoC Boards, comprised of NHS England commissioners, who decide what should be prioritised for inclusion in the PoC portfolio. The aim of PoC Boards is to provide an element of ‘peer review’, ensuring that specialised commissioning is evidence-based and cost-effective. PoC Boards are described as having an advisory role within NHS England.

**Rare Disease Advisory Group (RDAG)**
Providing advice on proposals related to ‘highly specialised’ services, RDAG is a committee comprised of commissioners, Royal College representatives and a geneticist and ethicist. It also has members to represent the interests of the devolved NHS administrations (Scotland, Wales, Northern Ireland) and four patient and public voice representatives. The names of the individuals who fill these posts are not publicly available.

**Clinical Priorities Advisory Group (CPAG)**
CPAG makes recommendations to the responsible oversight group about which services or medicines should be prioritised for funding. Although CPAG is not defined as a decision-making body but an advisory one, it can make formal recommendations, both positive and negative, on what should be prioritised for funding. A list of CPAG members is not currently available, but it is known to include four patient and public voice representatives.

**Specialised Commissioning Oversight Group (SCOG)**
Following an announcement in July 2014, SCOG has the authority to make decisions on the recommendations of CPAG about which services and medicines will receive funding. Previously their role was one of ‘operational oversight’. There remains, however, some confusion as to what proportion of commissioning proposals will be managed by SCOG alone given that ‘contentious’ issues will still require final sign-off from NHS England’s Directly Commissioned Services Committee. Membership of SCOG is not currently publicly available although it has recently recruited two patient and public voice representatives.

**Directly Commissioned Services Committee (DCSC)**
The DCSC is the ultimate decision-making body within NHS England’s structure for direct commissioning that will, according to recent governance changes, be required to adjudicate on ‘contentious’ proposals from specialised commissioning. It considers the advice and recommendations of CPAG and SCOG to reach its decisions. As with almost all of the committees within NHS England’s specialised commissioning governance structure, the membership of the DCSC is not publicly available.

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**Service Specifications**
These documents are the products of CRGs and describe what is expected of a service. They tell service providers what a service should look like and can be used by patients and NHS England to check the quality of a service and hold the provider to account.

**Commissioning Policies**
These documents define who is and who is not eligible for a particular service or treatment (a national prescribing policy). This ensures national consistency. NICE guidance can replace or be incorporated into commissioning policies, or they can be produced independently by CRGs.
The current maze-like process for evaluating medicine commissioning policies at NHS England:
This Patient Charter reasserts the right of patients affected by rare, life-limiting conditions to benefit from high cost treatments, which are free at the point of access. It recognises the broad support that exists for ensuring the NHS-wide commissioning of such medicines from the public, patients and government, particularly when the medicine is required by children.

This right is further supported by the NHS Constitution, which states not only that patients are entitled to access medicines that are deemed to be clinically effective and cost-effective, but that the NHS should provide a comprehensive service for all and promote equality by paying “particular attention to groups or sections of society where improvements in health and life expectancy are not keeping pace with the rest of the population.”

NHS England is legally mandated to promote the NHS Constitution.

There are seven formal routes through which medicines can be made available to patients affected by rare conditions. This Patient Charter recognises NICE’s limitations, both in terms of capacity and capability, to effectively appraise rare disease medicines. This Patient Charter is therefore premised by the understanding that the primary route through which patients affected by rare conditions will access the medicines they need is via those pathways managed by NHS England.

In accepting this premise, this Patient Charter highlights the importance of ensuring fair, timely and consistent approaches are taken to rare disease medicine evaluations within NHS England since comparatively few medicines for rare conditions will receive the legally mandated commissioning that a NICE recommendation affords.

This Patient Charter also seeks to ensure that NHS England fulfils its potential to have the patient voice at the heart of decision-making by ensuring that it takes advantage of the insight and expertise available from the patients and patient representatives who sit within NHS England’s governance structures; for example, as members of CRGs. This Patient Charter will aim to build on the intention already demonstrated by NHS England to fulfil this objective by identifying and addressing those areas where NHS England is currently falling short.

Within this Patient Charter, we make six overarching recommendations for positive change, from the perspective of the rare disease patient community, in order to enable NHS England to effectively and sustainably commission medicines for rare diseases.
Patient representatives agreed that ensuring the right medicine is considered for access via the most appropriate pathway, and that no medicine falls through the gaps, was a fundamentally important first step towards ensuring best use of available resources and equitable access to rare disease medicines.

They were keen to ensure that the multitude of methodologies available to evaluate, appraise or commission rare disease medicines are used appropriately and rationally, and called for NHS England and NICE to work together to achieve this.

NICE’s ability to effectively appraise medicines for rare diseases is constrained by their capacity and methodology, as well as the limits imposed by the Health and Social Care Act. As a result, patient representatives felt that NICE should ensure it only appraise those rare disease medicines where such an appraisal would add value to the NHS without duplicating the work of NHS England.

Aside from NHS England’s primary medicine evaluation procedure involving CRGs and CPAG, patient representatives were also keen to explore the benefits of utilising the other routes managed by NHS England effectively, specifically CtE and the CDF.

Patient representatives were positive about the application of CtE to medicines for rare diseases. They agreed that pilot schemes enabled the development of a higher quality evidence base than might otherwise be available for a rare disease medicine, where randomised controlled trial evidence is less prevalent. They also felt that it had the benefit of considering a treatment in a ‘real-world’ scenario, allowing patients to access medicines in a controlled way while avoiding the strict and unrealistic patient eligibility requirements associated with clinical trials.

Consequently, they were disappointed to discover that no medicine has so far been selected for CtE.
Moreover, they were concerned by the lack of information still available on CtE, such as what the eligibility requirements are or who will manage the programme, despite it being launched over a year ago in September 2013\textsuperscript{16}.

With this in mind, patient representatives called for NHS England to develop a framework for the application of CtE to rare disease medicines as a matter of priority. Their recommendations included the following:

- A commitment that the potential suitability for CtE will be considered for a rare disease medicine before an evaluation is initiated by either NHS England or NICE;
- The eligibility criteria medicines will need to meet to qualify for CtE and an indication of who will be responsible for selecting medicines for the programme should be published;
- Patient groups should be able to propose a medicine for CtE directly;
- An explanation of what data will be collected should be outlined, ensuring that the expectations of the evidence produced by CtE are in line with what can be achieved given the constraints on time, patient population size and the heterogeneity of the condition;
- The likely duration of a CtE pilot should be given;
- The continuation policy that will apply to patients accessing a medicine through CtE where the evidence from the pilot does not support national commissioning should be outlined.

Another access route discussed by patient representatives was the CDF.

**Cancer Drugs Fund (CDF)**

The CDF was introduced in 2010 as a means of enabling patients to access cancer medicines that would otherwise not be funded by the NHS. It has a ring-fenced budget of £200 million a year and will run until at least the end of 2016, with hopes that it will be continued beyond that date. NHS England took responsibility for managing the CDF on 1\textsuperscript{st} April 2014 and the Chair of the Chemotherapy CRG is responsible for making recommendations to NHS England on what drugs should be added to (or removed from) the list.

Of those licensed orphan medicines that NICE has appraised, a disproportionately greater number have been for cancer indications (45\% of licensed medicines have been appraised) compared to non-cancer (9\% of licensed medicines have been appraised).

Given that NICE has a limited capacity to effectively appraise orphan medicines, having carried out an average of only three a year since 2009, patient representatives were concerned that this capacity was not being apportioned fairly. As a result, they were concerned that patients affected by rare conditions that are not cancer are losing out on the privileged outcomes that are associated with a NICE recommendation, not least the legal mandate for commissioning that gives these medicines priority funding by NHS England.

In light of this, patient representatives considered whether NICE resources were being well spent appraising such a large number of rare cancer medicines, particularly when many of them are rejected but later appraised and commissioned through the CDF.

Patient representatives called for a system-wide approach to be taken when selecting the best access route for a medicine, ensuring due consideration is given to the appropriateness of each of the seven pathways and which represents the most effective use of time and resources.
Perhaps the most crucial decision for any patient population is that which decides whether their care is commissioned locally or nationally. The designation or removal of medicines and services from NHS England’s specialised services portfolio (which defines those services commissioned nationally) is the responsibility of Ministers, based on the advice of the Prescribed Specialised Services Advisory Group (PSSAG). This designation needs to have taken place before a commissioning policy for a medicine can be evaluated by NHS England to determine its funding priority.

PSSAG currently considers four factors to determine what qualifies as specialised and as such, whether it should be directly commissioned by NHS England:

1. The number of individuals who require the service: NHS England has chosen not to set a specific cut-off for this number, but it is expected to be similar to the EU definition of a rare disease: affecting 1 person in 2,000 or fewer.
2. The cost of providing the service or facility: This would be expected to be relatively high.
3. The number of people able to provide the service or facility: When a small number of people are affected by a condition or need a particular service, there are usually a limited number of medical professionals with the necessary knowledge and skills to provide the service or treatment.
4. The financial implications for CCGs if they were required to arrange for provision of the service or facility themselves: If the funds needed to support a specialised service meant that the CCG would be unable to allocate sufficient funds for other required services, this would support the need for nationalised commissioning.

At our workshop, patient representatives expressed their concern that PSSAG currently functions in an unnecessarily opaque and inaccessible way and lacks patient and public voice representation. Given the recent indications that NHS England intends to narrow its specialised services portfolio, patient representatives were understandably anxious that PSSAG’s role in making these decisions is currently unclear, as is the extent to which patients and patient groups will be involved in this process.

NHS England’s website should include information on the role of the Prescribed Specialised Services Advisory Group and a record of the rationale and outcomes of their decision-making.

Just as some of the other committees of NHS England whose decisions are most important, there is no clear record of the issues put before PSSAG, their decisions, their recommendations to Ministers, or the thinking behind this work. Their processes are not transparently laid out in a publicly accessible form and neither are their terms of reference. In fact, PSSAG is not mentioned at all on the NHS England website and is only described as part of the UK Government’s website.

Patient representatives called for information about PSSAG to be made accessible through the NHS England website, and its position within the NHS England governance structure demonstrated in organisational diagrams. Alongside all other committees of NHS England, meeting agendas and minutes, PSSAG membership and a clear point of contact should be available on the website.
Currently, NHS England will only consider a proposal for a medicine commissioning policy if they already commission a specialised service for the relevant cohort of patients. If such a service does not exist, one needs to be developed, designated as specialised by PSSAG and evaluated and granted funding by NHS England before the proposed medicine commissioning policy can itself go through the evaluation procedure. This process is clearly both cumbersome and time-consuming but must be followed even when there is no other component to the service than delivery of a medicine.

Patient representatives recognised that this approach may be necessary for borderline cases where it is not obvious whether the proposal is specialised and/or where the positives and negatives of local and national commissioning are difficult to balance. They agreed, however, that in most cases it is unacceptable for so much time and effort to be wasted considering obviously qualifying conditions. They called for the process of specialised designation to be made clearer and simpler, so that important, life-altering treatments can be quickly picked up by NHS England and progressed through to an evaluation, with the more rigorous hearings reserved for borderline cases only.

In addition to these broader issues, patient representatives were also keen to establish clarity on some of the details of PSSAG’s deliberations. While they were aware of the four factors used by PSSAG they felt that these could be very broadly interpreted, leaving room for a lack of transparency in PSSAG’s decision making. For example, when considering the size of a patient population, do PSSAG take into account all those that are affected by the condition or only those who are likely to take-up the offer of the service or medicine, or can this vary?

Patient representatives called for there to be improved communication of PSSAG’s decision-making processes and methodologies, and for PSSAG to clearly explain how they reach their decision to grant specialised designation or not.

Support should be made available to patient groups who have to make a case to the Prescribed Specialised Services Advisory Group

In those cases where it may be necessary for PSSAG to hear the case for why a condition, specific service or medicine should be included in NHS England’s specialised commissioning portfolio, patient representatives were keen to ensure the patient voice was heard. They agreed that to achieve this it would be essential for NHS England to offer guidance and support to the relevant patient group.

Patient representatives felt that currently they knew too little about who is responsible for gathering the evidence PSSAG uses. They were concerned that this meant that the patient voice might be absent or, conversely, that patients and patient groups may be expected to provide all of the evidence required by PSSAG themselves. Within our workshop attendees there were patient representatives who had had the responsibility of making the argument to PSSAG that their condition qualifies as specialised. This fuelled concerns that it currently does often fall to patient groups to make this case. Some patient groups are well resourced and informed enough to effectively make this representation, but not all patient communities are fortunate enough to have such a group to speak on their behalf.
Patient representatives felt it was inequitable to require patient groups to play such an important role without any help or guidance from NHS England. They pointed out that properly positioned commissioning was as much in the interest of NHS England, on the grounds of cost-effectiveness alone, as it is in the interest of the patients themselves. As a result they called for NHS England to actively support all patient communities required to present a case to PSSAG.

There was also some dissatisfaction amongst patient representatives over what factors PSSAG considers when determining whether something qualifies as specialised. They felt that, in the current climate of austerity, the idea that if local commissioners were unable to cope financially with the impact of providing medicines and services for a particular condition then it would qualify instead for national commissioning by NHS England (factor four) was laughable. From their experiences of working on commissioning policies within NHS England, patient representatives had found that a policy had to demonstrate that it was either cost-saving or cost-neutral to be considered. They called for the factors to be updated to reflect what they expect must already be the reality of PSSAG’s deliberations.

To ensure that PSSAG was taking into account all relevant factors when making their decisions, patient representatives proposed that there be further engagement between PSSAG and stakeholders regarding what those additional factors should look like.

Stakeholder engagement and public consultation should inform the development of a clear framework for determining ‘the line’ between specialised and regional commissioning.

Given the significance of the known budgetary overspend for specialised commissioning it was unsurprising that Simon Stephens’ June 2014 speech, in which he indicated that more services were going to be returned to CCG commissioning, caused significant discussion at our workshop.

Patient representatives could see that the simplest and most direct means of slimming down the portfolio and curtailing spending for NHS England would be to review which services should be designated as specialised.

There was an acceptance among patient representatives that rational discussion on this issue was a necessary task; and with such a large overspend it was felt that ‘the line’ between national specialised and local commissioning could currently be in the wrong place. Their primary concern was that ‘the line’ is now redrawn in the right place. However, there was disquiet from the attendees that this decision would not, under the current protocols, be subject to any form of public consultation: PSSAG would be free to make a recommendation to Ministers, who could then enact change.

This was felt to be one of the least democratic processes within NHS England, with PSSAG as an advisory group to Ministers able to make decisions without needing to be held to public account. Given what may be at stake for the many patients who currently have a service within specialised commissioning or are hoping to get one, patient representatives felt that such a critical decision could not be made in the absence of stakeholder input, particularly that of patients.
The NHS is currently facing an unprecedented squeeze on its finances, with the NHS budget being cut in real-terms for the last four years. This funding pressure comes at a time when a growing and aging population are requiring additional healthcare expenditure and the costs of new medicines and health technologies are rising. That being said, in the last year spending on medicines accounted for only 15% of NHS England’s specialised commissioning budget.

NHS England currently has a reported overspend on specialised commissioning of approximately £900 million. Patient representatives were understandably concerned about the impact that this overspend will have on their ability to access new medicines, or those not already routinely commissioned through the NHS.

Some patient representatives described how they have already begun to experience an effective ‘ban’ on new commissioning policies by NHS England, with the development of anything that ‘costs money’ halted at the CRG level. This has happened regardless of whether there are long-term savings to be made if the commissioning policy is implemented.

Such a curtailed uptake of new medicines comes on the back of previous delays in uptake caused by the passing of the Health and Social Care Act and the significant structural changes the Act itself produced. This left many difficult decisions without the bodies empowered to make them. With the reorganisation of the NHS unlikely to be finished for a number of years and major delays in the creation of decision-making frameworks for significant parts of NHS England’s new structure, patient representatives were concerned by the poor adoption of new medicines to date and the likely continuation of this trend into the future.

Workshop attendees agreed that having financial constraints take precedence over patient need when determining access to rare disease medicines was incompatible with the constitutional promises of the NHS. In order to shift the balance back towards the consideration of long-term cost-effectiveness and enable important factors such as quality of life, patient related outcomes and wider societal benefits to enter into discussions, patient representatives identified a number of priorities for change for how NHS England rationalises its spending on specialised services.

NHS England should be more transparent when describing cuts and efficiency savings

Patient representatives noted how the term ‘affordability’ had started to be used to explain why medicines are not approved for funding on the NHS. They found this unpalatable not only because of the relatively small proportion of NHS England’s budget that is spent on rare disease medicines, but because of the poor resource management they felt currently took place in the NHS. Many attendees were able to give anecdotal examples of wastage or inefficiencies within the NHS from their own experiences.
Patient representatives agreed that in order to justify blocking patient access to a rare disease medicine through the NHS on the grounds of ‘affordability’, NHS England had to first demonstrate that they are capable of managing their existing funds effectively. In achieving this, they recognised that NHS England will need to introduce tools and initiatives that are designed to drive savings.

Two of the tools currently used by NHS England to incentivise cost efficiencies include CQUIN: Commissioning for Quality and Innovation, which allows commissioners to withhold funds until the service provider has met certain standards; and QIPP: Quality, Innovation, Productivity and Prevention, a programme through which expenditure reduction targets can be set. While patient representatives welcomed the aims of these initiatives, they questioned their nomenclature. They felt that although it can be helpful to link savings and efficiency with ‘innovation’, too firm a link, as in the names of these two schemes, risks tarnishing the value of innovation itself.

Innovation within the NHS should be driven by improvements in the standard of care but can in some cases also bring about financial savings either in the short or longer term. The poor record of adopting innovative medicines in the NHS, particularly in the period of turmoil that has followed the announcement of the Health and Social Care Act in 2011 and the formation of NHS England, indicates an inadequate understanding of these benefits of innovation to patients and the NHS. This is despite NHS England itself being legally mandated to promote innovation.

Patient representatives felt that uptake of innovation, including new medicines, should be able to occur outside of the context of cost-cutting exercises. Importantly though, they also agreed that ‘cuts’ or ‘efficiency savings’ were necessary if NHS England is to begin affording innovative medicines in the coming years. They therefore called for NHS England to be more open about when it was introducing money-saving initiatives and to stop using ‘innovation’ as a guise.

NHS England should engage more with the pricing of medicines and consider developing Patient Access Schemes (PAS)

It became increasingly apparent during our workshop that NHS England was the primary evaluator of rare disease medicines for England. As has been previously discussed earlier in this document, NICE has performed very few appraisals or evaluations on rare disease medicines historically, with their future capacity unlikely to increase. Given the importance, therefore, of NHS England to the determination of access to rare disease medicines, patient representatives felt that NHS England should have access to some of the same mechanisms that NICE have to ensure a medicine can be cost-effective. The most important one of these tools is the ability to request the negotiation of a Patient Access Scheme (PAS).

One of the very few innovative products adopted by the NHS during the halt on progress stimulated by the Health and Social Care Act’s passage through Parliament was a treatment for a particular form of cystic fibrosis. This drug, ivacaftor (Vertex Pharmaceuticals), was the subject of a PAS.

This is the only instance of a PAS being developed without the engagement of NICE.
The power of a PAS is clear: only two of the ten orphan medicines ever recommended by NICE have been deemed cost-effective without being subject to a PAS. This indicates that if NHS England was to adopt this approach, the likely outcome would be more medicines being made available for patients with rare diseases and better value for the NHS.

If NHS England believes that it lacks the appropriate expertise and/or resource to advise the Department of Health on the negotiation of a PAS, then it was proposed that NICE be empowered to act on NHS England’s behalf, or to advise NHS England during the process.

Second, the new Pharmaceutical Price Regulation Scheme (PPRS) is often referred to as a tool by which Government expenditure of medicines can be capped. Unfortunately, patient representatives felt that there had been insufficient communication of what the implications of the PPRS will be at the level of individual medicine affordability in order to be able to understand how it may influence appraisals. They were concerned this might prevent the PPRS from stimulating the promised increase in uptake of innovation ‘so that patients had faster access to new medicines that are clinically and cost-effective’.

They called for it to be made clear what impact the PPRS agreement will have on access to rare disease medicines in England.

Correct dosing of medicines is crucial. Giving the right amount of a medicine ensures that patients receive the best possible treatment for their condition, while balancing the safety aspects of the medicine and limiting adverse events (side-effects). It is not easy to examine the correct dosage of a medicine though, and the appropriate dosage defined by a market authorisation may often be the result of a pragmatic estimate. For many rare disease treatments, clinical trials to examine treatment efficacy over a range of doses have not been carried out in the target population.

For severely disabling, rare conditions it is clear why evidence on dosage may be limited, since recruitment to a trial where there is a chance that an effective dose of the treatment may not be given would be difficult to justify. As a result, there may be uncertainty as to what the optimum dose is at the time that a medicine is being evaluated. In these circumstances, there may be scope to consider reducing the dose, under clinical supervision, as a more appropriate dosage may deliver better outcomes for some or all patients.

An additional consideration is that in many rare diseases, medicines are intended for delivery throughout life. Some of these medicines can restore life expectancy to near normal levels. This creates a scenario in which patients take an expensive medicine for many years. Just as it has been difficult to study the correct dosing of medicines is crucial: giving the right amount of a medicine ensures that patients receive the best possible treatment for their condition, while balancing the safety aspects of the medicine and limiting adverse events (side-effects). It is not easy to examine the correct dosage of a medicine though, and the appropriate dosage defined by a market authorisation may often be the result of a pragmatic estimate. For many rare disease treatments, clinical trials to examine treatment efficacy over a range of doses have not been carried out in the target population.

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dosage of rare disease medicines, there has been little work done to explore maintenance dosing, and dosing in the later stages of life.

In many cases the optimum dosage of a medicine may be less per person, or may involve it being taken over a reduced period of time. This would have a significant impact on anticipated treatment costs and therefore, on the outcomes of cost-effectiveness deliberations during medicine evaluations. Patient representatives felt that dosing was an area that could be explored collaboratively by patients and their clinicians.

Patient representatives were keen to ensure that pursuit of this approach is organised and monitored within the framework that NHS England provides. They felt that as the direct commissioner of orphan medicines, NHS England is well placed to carry out this form of post-market authorisation analysis, with the aim of improving patient care and rationalising expenditure on medicines.

Patient representatives agreed that patients need to take responsibility for their role in ensuring NHS resources are spent as effectively as possible. They felt that this recommendation should be true for all patients, but that it is perhaps most important for patients who are prescribed expensive medicines. Many representatives at our workshop felt that there was more that patients with rare diseases could do to work with the NHS in order to manage their medicines budget more effectively.

The value of adherence is best known regarding antibiotic use, where if a patient does not complete the course of treatment the infection may return. For chronic and/or progressive conditions, poor adherence can render the medicine ineffective. In some cases, the doses missed when the condition has not fully progressed may be the most important for future outcomes. With a number of rare disease medicines also commanding a high price, adherence is important simply because untaken medicines are a waste, often literally ‘money down the drain’. If patients choose to alter how much medicine they take without consultation with their clinician, they may also risk suffering adverse events while receiving little or no benefit from the medication that the NHS is still paying for them to receive.

One of the most difficult issues that a doctor and patient must tackle together is when a medicine is not working. This conversation is hardest to have when the condition is progressive, and the medicine that they are currently taking is their only option. Patient representatives at our workshop acknowledged this difficulty and recommended that more be done to inform and enable patient concordance, which involves patients and clinicians working together to reach an agreement on treatment regimens.

Genetic Alliance UK therefore undertakes to create a communication tool for clinicians and patient groups that will foster a culture in which patients are encouraged to be honest with their doctors regarding compliance and how they feel about adverse events.

This we hope will: make people realise how they can change their behaviour to have a positive impact on the NHS’s finances; create recognition that patients enter into a contract with the NHS in order to obtain medicine access; prepare patients to take cheaper generic medicines rather than branded alternatives; and provide a methodology to stop the delivery of treatments to patients when a medicine is not working. We further call on the NHS and the Government to similarly promote concordance and highlight the importance of medicine compliance with patients and the wider public.
When NHS England was established, the organisation stated their intention to achieve openness and accountability by ensuring effective communication to patients and the wider public. An example of where this has been successfully implemented is in the public accessibility of the NHS England Board meetings. These can be attended by the public or watched online, live or at a later date. The board papers for each meeting are also available on the NHS England website.

NHS England incorporates patient and public voice (PPV) representation across its decision-making governance structure. This should have two positive outcomes: first, it should ensure that patients are prioritised in every decision, as outlined in NHS England’s vision, by providing opportunities for patients to input their views; and second, it should facilitate patient and public oversight of NHS England’s activities. PPV representation includes the Specialised Services Patient Public Engagement Steering Group, replaced in June 2014 by the Patient and Public Voice Assurance Group (PPVAG); which as well as fulfilling an oversight role, provides a forum for patients and the wider public to become informed about and contribute to the improvement of specialised services. Genetic Alliance UK is represented in the PPVAG alongside four other patient organisations and individuals. There is also PPV representation on CRGs, RDAG, CPAG and, more recently, SCOG.

These structures, processes and behaviours should enable NHS England to ‘prioritise patients in every decision’, be ‘open and transparent’ and ‘listen and learn’. Unfortunately, this does not appear to be the experience of those patient representatives who engaged in our workshop.

All attendees agreed that they had insufficient access to information about NHS’s England’s governance structure, their decision-making procedures and the outcomes of those decisions. They felt that often the information they did have access to regarding NHS England’s procedures did not mirror their own experiences of being involved in or affected by the process.

As a result, patient representatives were not confident that NHS England was currently making high-quality decisions regarding access to medicines, and were concerned by the lack of internal and external quality assurance checks able to take place as a result of poor communication. Patient representatives were also concerned by the absence of a formal requirement for patients and patient groups to be included in the working groups formed to develop commissioning policies, leading to unacceptable variation between CRGs in the degree to which the patient community is engaged in the medicine evaluation process.

Patient representatives identified three broad recommendations to address these issues: the need to improve the content and structure of the website, ensure public and stakeholder engagement is of high quality and that the patient voice is valued. These themes are addressed with further specific recommendations throughout the Patient Charter.
A central issue raised by patient representatives in our workshop was the importance of NHS England’s website in delivering transparency, consistency and accountability. Patient representatives felt that the current website was inadequate and poorly maintained, with information often absent, inaccurate or out of date.

Organisational websites are the primary, and sometimes only, source of contact between an organisation and members of the public. It is therefore necessary that it contains the most up-to-date information, explained in a straightforward manner on a site that can be easily navigated.

Specialised commissioning represents a small but important component of NHS England’s portfolio of work and is of particular interest both to the patients directly affected by commissioning decisions as well as the wider public due to the proportion of the total NHS budget spent on specialised commissioning. Without an effective website, both patients and the public are left in the dark.

Patient representatives were keen for NHS England to achieve the following basic standards of organisational communication:

- Publish a clear outline of the specific role in the decision making process of each component of the specialised commissioning governance structure;
- Publish the membership of all advisory groups and committees, including, where appropriate, the name of the organisation each member is affiliated with;
- Publish in a timely manner the agenda and minutes of meetings by CRGs, PoC Boards, RDAG, CPAG, SCOG and the DCSC. Minutes should include agreed outcomes and actions.

Patient representatives further identified a number of specific measures that NHS England should implement in order to improve organisational communications and achieve transparency and accountability.

Many of these measures directly relate to online communication strategies, in particular the NHS England website. Where improvements to the website or online communication is a component of a recommendation, the recommendation will be colour-coded green.

NHS England’s website outlines their intentions to make CPAG as open and transparent as possible:

“CPAG will be open to public scrutiny and will be publicly accountable. This will be achieved by publishing meeting minutes on the NHS England website, and by having an independent patient and public voice included in the membership. It is currently under discussion as to whether CPAG meetings will be held in public.”

However, these intentions are considerably diluted in CPAG’s Terms of Reference:

“The committee will make agendas available prior to meetings on NHS England website... Once agreed by the Chair, the aim will be to publish the minutes on NHS England’s website as outlined in the procedural rules document.”

Patient representatives noted that in many cases neither the agenda nor the minutes of CPAG meetings were available online. They were keen to see this addressed and the importance of timely and accurate communication better reflected in CPAG’s Terms of Reference.
Genetic Alliance UK understands that the NHS England website is currently undergoing a review and hopes that the recommendations within this Patient Charter are recognised and reflected in the amendments made to the website’s content and structure.

NHS England should ensure that all stakeholder engagement is effective, timely and accessible, and that its impact is communicated back to stakeholders.

Good stakeholder engagement requires information to flow effectively between NHS England and its stakeholders. This requires NHS England to both provide information to and actively seek out the views of its stakeholders.

NHS England has a significant number of stakeholders ranging from large, professional organisations to individual patients and the wider public. The information and insight they can provide is valuable at all levels of NHS England’s specialised commissioning activities; from the appropriateness of specific commissioning policies to cross-cutting structural or procedural changes, such as the development of CPAG’s funding prioritisation framework. Many stakeholders, including patients and patient groups, can also provide feedback on how policies or processes are working on the ground.

NHS England’s structure aims to integrate stakeholder engagement throughout, a commitment that was welcomed by the patient community. At our workshop, however, patient representatives reflected that NHS England may have underestimated the resources required to deliver the scale of stakeholder engagement they had planned. As a result, patient representatives felt that the quantity and quality of NHS England’s stakeholder engagement currently falls short of what had been proposed, and in some cases, of what they deemed was acceptable.

Public and stakeholder consultations form the cornerstone of NHS England’s engagement strategy. To gather useful information, patient representatives felt that consultations needed to be:

- **Timely**: they should take place at a sufficiently early stage in the development of a new policy or process to enable the responses to influence and shape the proposal;
- **Accessible**: they should be visible to all those who may have an interest in responding and be open for a sufficient period of time to enable stakeholders to develop their response;
- **Open**: they should have a clear outcome, with a summary of the feedback received and NHS England’s response to that feedback made publicly available.

For stakeholders to be able to contribute effectively they need to be kept informed. As highlighted above, patient representative’s recognised the importance of a good website for achieving this. NHS England also has a considerable network of stakeholders who have registered with them to be contacted by email. In June 2014, NHS England announced that it would make use of this network by producing a Specialised Commissioning Stakeholder Newsletter, with the primary aim of updating stakeholders on the progress of the specialised commissioning Task Force. Any step towards improving channels of communication between NHS England and its stakeholders is welcome, but it remains to be seen whether this particular initiative proves to be of value to registered stakeholders.

“There is so much variability [in the degree of stakeholder engagement] and that’s just not fair”
Promoting the involvement of patients and their families, carers and representatives in decisions relevant to the diagnosis or treatment of their condition is one of the key mandated legal duties that govern NHS England. The number of patient representatives at our workshop who were members of a CRG stood as testament to NHS England’s commitment to patient and public voice representation in specialised commissioning. Involvement is not comprehensive, however, nor is it consistent. By exchanging their experiences of being involved with different CRGs, patient representatives raised the issue of disparity; both in the type of patient representation on CRGs and the degree to which patient representatives felt their views were sought and valued. Patient representatives welcomed the inclusion of PPV representation in NHS England’s governance structure for specialised services; and particularly the central role that clinical and patient experts play in the decision-making process as members of CRGs. While patient representatives agreed that in many scenarios the objectives of clinicians and patients were likely to be closely aligned, they were keen to ensure that the views of medical professionals were not allowed to become synonymous with the patient voice. They reasoned that even the best clinicians cannot fully appreciate the nature of a condition or second-guess what patients will value or tolerate. Patient representatives were also keen to ensure that the needs of patient communities were not overlooked even in the absence of a clinician with specific expertise in their condition.

Achieving a level playing field for all patient communities was a key concern of patient representatives. To achieve this, they felt it was necessary for NHS England to distinguish between those patient groups that could provide a highly-skilled and professional research, information and administrative service, and those which needed to be proactively engaged with and supported in
order to ensure the patient perspective is heard effectively. They agreed that CRGs should be more able to actively seek out proposals and/or evidence from relevant patient groups, however small.

Patient representatives also called for NHS England to ensure the PPV membership of their committees contains a consistent and appropriate balance between individual patients and patient group representatives. They felt that while all equally valuable, the insight of a patient differs from that of someone who speaks on behalf of a group of patients, as an affiliate of a patient group or as a representative of an umbrella organisation. They agreed that NHS England should recognise these differences in order to secure a consistent representation of the patient voice.

“Patient groups bring something to the table that clinicians and even individual patients can’t – but you have to get your organisation taken seriously”

Patient representatives identified a number of other specific recommendations that they felt were necessary to ensure the patient voice was heard and valued during NHS England’s decision making processes. Throughout the remaining Patient Charter, these are colour-coded pink.
Clinical Reference Groups (CRGs) are the gateway to NHS England’s medicine evaluation process. They are the first point of contact for any stakeholder wanting to propose a medicine as a candidate for evaluation. It is then also the responsibility of the CRG to decide whether or not to pursue an evaluation for that medicine, as well as produce the commissioning policy.

CRGs have a central role in determining which medicines are considered and how, as well as in collating and critiquing the evidence to support a commissioning policy. Given the importance of CRG activities and outputs, both in terms of patient need and financial impact, patient representatives felt that further investment into CRGs by NHS England was justified.

They agreed that improving the effectiveness of CRGs was an essential first-step towards NHS England achieving a consistent, equitable, accountable and transparent process for evaluating and commissioning medicines for rare diseases.

Patient representatives welcomed the principle behind CRGs and their place in the evaluation process at NHS England in that they ensure that experts in the field, including patients and clinicians, are at the centre of decision-making. But as it stands, they agreed that CRGs are insufficiently resourced to carry out the volume and breadth of work required of them at the necessary level of detail.

“There needs to be a better system at the beginning to make sure that each medicine is looked at equally”

“CRG members are all volunteers but they make very serious decisions that people’s lives hinge on”
During our workshop, patient representatives compared their experiences of working with CRGs. While many were positive, others had found their CRGs poorly organised, unhelpful or disinterested. Patient representatives were most struck by the scale of the variation between the effectiveness of CRGs and were concerned by the inequity this inevitably creates for patients trying to access medicines through NHS England’s system for specialised commissioning.

By sharing their experiences, patient representatives also began to appreciate the lack of guidance available to CRGs when they are selecting which commissioning policies should be prioritised for development. They agreed that the absence of clear criteria meant the process was opaque, unfairly inconsistent and vulnerable to the preferences of individual CRG members.

To address this, patient representatives were keen for NHS England to establish a clear and publicly available guideline outlining best practice for CRG working. They agreed its development should build on the many aspects of CRG activities that are already being done well, to produce a gold-standard. They felt that a guideline was essential if NHS England was to deliver a consistent and accountable system that ensures all patients, regardless of their condition and affiliated CRG, can expect the same high standard from the process which determines what medicines are made available to them.

Patient representatives felt it was important for any such guideline to be developed in discussion with the CRG membership and to be made available for public consultation before implementation. They felt that this would ensure the guideline was applicable to all CRGs and would immediately improve the transparency and accountability of CRGs in their commissioning policy development role.

The fundamental aspects of best practice identified by patient representatives included:

- Proactive contact with patients and patient groups in order to ensure the patient voice is heard in the development of commissioning policies;
- Effective internal and external communication of the CRG’s work-stream and its outputs to improve transparency and accessibility for stakeholders and the wider public;
- An outline of the processes required to achieve high-quality stakeholder engagement and for producing helpful consultation feedback that includes the impact of the responses received;
- Well-defined and publicly available criteria for deciding which commissioning policies should be prioritised for development.

On this last point, patient representatives were keen to ensure that CRG prioritisation decisions are made using a fair and equitable methodology and not simply on the grounds of cost.

In addition to putting in place a guideline, patient representatives were keen to introduce a regular audit for CRGs in order to measure their adherence.
The PPVAG has recently announced an initiative that will enable the PPV members of all CRGs to meet and discuss and share their experiences. This ‘PPV members’ CRG summit’ would be a useful forum within which a best practice guideline could begin to be developed, based on the first-hand experience of PPV representatives. Future summits could then also function as a regular opportunity for PPV representatives to audit their CRG’s activity against the guideline, and raise concerns if feel they their CRG is not performing to standard.

Many of the patient representatives at our workshop were either CRG members themselves or were currently working closely with their relevant CRG. One of the key barriers they identified to the effective running of CRGs was the insufficient administrative support available to enable information gathering and dissemination.

Patient representatives recounted their experiences of not having meeting dates confirmed, not receiving the papers to be discussed in advance and action points failing to be recorded or followed up on. One patient representative also described how their CRG had had their conference call facility revoked, preventing the CRG from being able to meet as frequently.

While patient representatives recognised that CRGs currently operate on a voluntary basis, they agreed that paying CRG members for their time was not the right solution. They felt that clinicians who sit on CRGs were not best placed to fill the gap in managing CRG administration, and agreed that it was also not for affiliated patient groups to provide this general administrative support. They felt that this would risk giving a single patient community undue influence over a CRG’s activities leading to inequity; particularly within those CRGs that manage a number of conditions, some of which may have no patient group to represent them.

Patient representatives instead agreed that a small, independent administrative team, employed by NHS England, could service all CRGs. They felt that the administrative team’s role should include:

- Arranging and confirming meeting dates and ensuring that each attendee has access to the required documents sufficiently in advance of the meeting;
- Developing meeting agendas, in collaboration with the Chair of the CRG, and making them available online before each meeting;
- Keeping detailed, easily understood minutes that highlight and assign tasks for completion, and making these available online after each meeting, with sign-off from the Chair.

Patient representatives felt that this would immediately address the basic difficulties that each CRG was facing with organising and managing their workload, ensuring that all members are kept up-to-date and that nothing falls through the gaps. Without this basic level of support and resource from NHS England, patient representatives could not see how CRGs would be able to deliver on the tasks they had been set to a sufficiently high standard.

Further to addressing these basic requirements, patient representatives highlighted four additional benefits a dedicated administrative team could offer that would vastly improve the ability of CRGs to engage successfully with stakeholders. These are outlined below. Patient representatives felt this further justified NHS England prioritising investment into administrative support for CRGs.
Management of a single, publicly available point-of-contact email address for each CRG

Patient representatives agreed that as the entry point for medicine commissioning policy proposals, CRGs must be easily contactable. They noted that NHS England’s website currently provides no information on how to get in touch with a CRG; and that even as a registered stakeholder you are given no contact details or even any confirmation that your registration was successful.

Patient representatives considered the absence of this information to be unacceptable. They felt that it particularly disadvantaged patients and patient groups who may struggle more than other stakeholders, such as NHS Trusts or pharmaceutical industry representatives, to identify who best to contact within NHS England and how.

To address this, patient representatives were keen to see NHS England introduce a centrally managed email account for each CRG. Correspondence could then be filtered by the administrative staff and forwarded onto the relevant CRG member and/or collated to develop the next meeting’s agenda. They felt this recognised that individual CRG members may not want to disclose their own email addresses while still providing a manageable and publicly accessible route to each CRG.

CRG PPV representatives at our workshop also expressed their willingness to act as additional points of contact specifically for patients or other patient groups looking to engage with a CRG. They felt this could be achieved by simply including the name of the patient group they are affiliated with as part of the CRG membership information; a detail that is currently not disclosed. They felt they could then act as a source of information and guidance to those patient groups less familiar with NHS England’s evaluation processes, and that it would allow PPV members to act as a route through which a wider range of patient perspective could be channelled into the discussions of each CRG. This would enable those patient groups not directly represented within a CRG to educate their PPV members about their condition and any issues related to accessing a specific medicine.

Effective use of the registered stakeholder contact email database

This second proposal stemmed from patient representative’s current disappointment with NHS England’s CRG stakeholder registration initiative. While they welcomed the principle of developing a contact database of organisations and individuals interested in each CRG, they felt it was not currently delivering on its potential to provide an effective two-way channel of communication between CRGs and stakeholders.

Genetic Alliance UK is a registered stakeholder of 44 CRGs and has received emails from less than a quarter of them. The majority of patient representatives at our workshop were also registered with one or more CRGs and their experiences varied, with some being contacted regularly, others not at all and some receiving different messages to other stakeholders.

Patient representatives agreed that this sporadic and inconsistent use of the registered stakeholder database gave the pretence of stakeholder engagement while providing very little benefit to either the stakeholders or CRGs, and represented a significant missed opportunity by NHS England. They were confident, however, that if administered effectively by a dedicated team, it would enable CRGs to access a variety of expertise and insight that would be of value to their work, would provide much needed transparency and enable stakeholders to feel involved in NHS England’s evaluation processes.
A proposed commissioning policy for a medicine needs to include details of the natural history of a condition, the size of the patient population and what the current treatment options are. Often it is only by looking through the eyes of patients, carers and their families that it is possible to appreciate the real impact that a condition and/or a new treatment can have. In recognition of this fact, patient representatives identified the inclusion of the patient voice in commissioning policy development as a key component of CRG best-practice; and were keen to see the identification of relevant patient groups and the coordination of their engagement with the CRG process within the scope of the administrative team’s role.

Patient representatives wanted to make certain that this did not just involve using the registered stakeholder mailing list as they were concerned this could overlook individual patients or patient groups who may not self-identify as stakeholders or even be aware that the registration scheme exists. They felt this would be particularly relevant for rare diseases, where often it is the patients that are the experts but where patient groups may only operate on a small scale, be poorly resourced or not exist at all. Patient representatives were keen to ensure the administrative team proactively identified all relevant patient groups or communities so that the patient voice is heard.

As outlined above, not all parties that may have something valuable to contribute to a commissioning policy will be registered as a stakeholder of the CRG doing the work. This may be because they are unaware of the system or because their condition of interest does not fall obviously into one of the 75 CRGs. This would be the case particularly for conditions with co-morbidities.

Patient representatives felt that there was sufficient risk that a patient or patient group may not be following the work of all relevant CRGs to warrant the creation of a searchable, online portal that lays out the work plans of all groups. This could then be searched for the name of a CRG, a condition or a medicine to find out whether a relevant commissioning policy is part of any CRG’s planned schedule of work.

Making information about the work being done by CRGs available online should be central to NHS England’s commitments to be ‘open and transparent’, ‘prioritise patients in every decision’ and ‘listen and learnt’ by providing another mechanism of stakeholder and public engagement. It also provides an opportunity for patients and the wider public to appreciate the scale and value of the work that is undertaken by NHS England in this area.

The information that NHS England would need to include in the online portal would be equivalent to the information NICE makes available to patients and the public when it announces a proposed technology appraisal scoping workshop. This includes details of the medicine’s manufacturer and the condition that it is being considered for use in, often before the pharmaceutical company has applied for the appropriate license. The portal could also include: who proposed the medicine as a candidate for commissioning policy development; the reasons why a medicine was prioritised or not for this process; and which stakeholders had been involved in the policy proposal and/or were currently engaged in the development of the final commissioning policy.
In the last two years over 250 medicines have been granted an orphan designation by the EMA, with a further 125 already designated in 2014. NHS England therefore has a sizeable and complex task if it is to effectively appraise all of the medicines for rare conditions that fall within the remit of specialised commissioning.

Patient representatives with experience of working with CRGs felt that having a CRG dedicated to your condition, or a clinician or PPV member of the CRG able to ‘champion’ the condition from within, was of huge benefit. While they agreed that this demonstrated the value of clinical and patient expert involvement in the decision-making process, it also highlighted the worrying difficulties faced by conditions not represented in the current CRG structure.

Patient representatives suggested that NHS England ensure the continued appropriateness of the CRG structure by carrying out a regular review. This review would look to establish whether there is sufficient coverage, capacity and expertise available within the existing 75 CRGs to continue to serve the needs of all patients affected by rare conditions. Potential outcomes of the review may include the need to create new CRGs or set up formal sub-committees that provide input on specific conditions, for example.

The review would serve as part of the CRG audit and oversight process that patient representatives were keen for NHS England to introduce. Given that CRG Chairs are re-elected every 3 years, patient representatives felt that this would be a good opportunity to ‘take stock’ and hold a review. The review would need to incorporate a period of public consultation to ensure that those not already within NHS England’s stakeholder community had the opportunity to contribute and highlight any conditions falling into the gaps between CRG specialisms.

“I feel really sorry for those conditions that have not found a ‘CRG home’ as they don’t have a route to access appropriate care and treatment... we feel very lucky that our condition is well covered”

“You have to trust that the experts in a CRG are in fact experts. It works for smaller, focused CRGs but is harder for the wider remit groups”
Once a commissioning policy has been prioritised for development by CRGs and a draft produced, it then enters into NHS England’s evaluation process. The aim of the evaluation is to determine whether the commissioning policy should be prioritised for funding; taking account of the finite specialised services budget and the need to focus investment where there is greatest patient need.

While patient representatives recognised the need for this prioritisation, they were shocked and concerned by the complex and convoluted system NHS England had created to carry out the task. After being shown an NHS England diagram of the process, patient representatives identified several steps they felt were either superfluous or asked for inequitable decisions to be made.

During our workshop, patient representatives discussed and developed an alternative system that they felt would enable evaluative decisions to be made in a more equitable, clear and timely way. Their recommendations are outlined below and range from modifying the governance structure to improving specific aspects of the evaluation process itself.

Many of the areas patient representatives identified for improvement in CRGs run as themes throughout the NHS England evaluation process and therefore, through the recommendations from patient representatives. One theme is the lack of transparency, and these recommendations are made in the context of the limited information that is currently publicly available.

Patient representatives agreed that NHS England is well placed to evaluate rare disease medicines but felt that a more efficient process needs to be put in place; describing the current system as “lengthy and cumbersome” and “over burdensome and bureaucracy heavy”. They recognised that when dealing with decisions that involve committing large amounts of resources, checks and balances need to be in place but felt that the current system only served to introduce unnecessary delays.

Their primary issue was that the process has too many steps without it being clear how one stage differs from another. Patient representatives felt that even if all the steps added value to an evaluation, which they questioned, it was still not necessary for them to work consecutively, which resulted in proposals bouncing slowly from desk to desk at risk of being overlooked or forgotten.

Patient representatives instead called for NHS England to implement a streamlined, three-point evaluation process with the development of selected commissioning policies by CRGs, prioritisation for funding by CPAG and final funding allocation by SCOG.
One of the key innovations patients proposed to streamline NHS England’s evaluation process was the removal of Programme of Care (PoC) Boards. They felt that the grouping of CRGs around five PoC Boards forced comparative prioritisation decisions to be made based on the division of CRGs into arbitrary and inequitable subgroups.

The inclusion of inherited and congenital diseases under the ‘Women and Children’ PoC, for example, means that a significant majority of rare diseases could be managed within this portfolio alone. Perversely, it also means that the ‘Women and Children’ PoC could include conditions that also affect adult men, while some congenital inherited conditions, such as some cancers or those that affect the kidneys, may have their most relevant CRGs grouped within another PoC’s remit.

Patient representatives felt that a fairer system would involve having the commissioning policy proposals of all CRGs considered for funding prioritisation by CPAG in the first instance, without prior filtration by PoC Boards. They were confident that if CRGs were given a set of criteria to

**Programmes of Care (PoCs):**
- Internal Medicine (digestion, renal, hepatobiliary and circulatory system)
- Cancer and Blood (infection, cancer, immunity and haematology)
- Mental Health
- Trauma (traumatic injury, orthopaedics, head and neck and rehabilitation)
- Women and Children (women and children, congenital and inherited diseases)
apply to their proposals in order to manage prioritisation, as outlined in the recommendation above, there would be no need for PoCs who would otherwise only duplicate this activity.

Patient representatives felt that these proposals would not only abolish the inappropriate sub-grouping of CRGs but would also significantly decrease the time taken for NHS England to carry out an evaluation, given that sign-off by PoC Boards is required at three stages within the process.

Within NHS England’s current evaluation system, a commissioning policy proposal can be rejected from further consideration at any stage. Patient representatives were concerned not only by the number of opportunities there is for this to occur, as described above, but also by the hidden and opaque manner in which these decisions are taken.

As outlined above, patient representatives have advocated for the development of decision-making criteria for CRGs and the removal of PoC Boards from the evaluation process. Currently PoC Boards are positioned to decide whether a commissioning policy proposal should be accepted into a PoC’s portfolio; a function that is described loosely on the NHS England website as the ‘development of clinical strategies’. No criteria or decision-making framework is publicly available to outline how PoC Boards decide what is in and out of their portfolio.

The remit of SCOG is equally as impenetrable. There is currently no section of the NHS England website that outlines the role of SCOG in the evaluation process or who is represented on the committee. Given that SCOG ‘has delegated authority to make decisions on the recommendations made by CPAG’ and therefore decides whether a commissioning policy gets routinely funded, it is concerning that there is so little information available. Again, it is not clear what SCOG is looking to assess in its deliberations or what criteria a commissioning policy will be measured against.

There are a number of other committees that form part of NHS England’s evaluation process whose roles are even less well defined. One example is the Clinical Effectiveness Team. No information about who makes up this team or what role they play can be readily accessed online. At our workshop, NHS England representative Fiona Marley explained to patient representatives that the team is made up of four staff members: Donna Hakes, Malcolm Qualie, Ahmed Syed and a Public Health representative, whose role it is to “look after” CPAG. Patient representatives suspected that this team are likely to play a significantly more important role in NHS England’s evaluation processes than this description suggests and were frustrated by the lack of information on the Clinical Effectiveness Team’s remit that is readily available to patients and the public.

Patient representatives called for NHS England to establish what role each component of their evaluation process fulfils, and the factors that each body should consider when making their prioritisation or funding decisions. They agreed that this information must then be made publicly available.

In light of this recommendation, patient representatives welcomed the work that NHS England is already doing to develop a transparent prioritisation framework for CPAG. There was some concern, however, that the factors NHS England planned to consider, as outlined by Fiona Marley, may not be the most appropriate. Patient representatives and Genetic Alliance UK look forward to having the opportunity to respond to NHS England’s proposals in a written consultation.
Even with clearer governance, patient representatives felt it was important to have a process for challenging NHS England's decision-making. Patient representatives called for NHS England to allow stakeholders to contest a decision if they believe NHS England to have acted unfairly, exceeded their powers or made an unreasonable decision in light of the evidence submitted. This would bring NHS England in line with NICE. Patient representatives also proposed that NHS England set a review date for medicines not prioritised for funding to ensure that patients with a continuing unmet medical need did not fall through the gaps.

One other component of NHS England’s evaluation process that was met with some confusion was RDAG. Many patient representatives were unaware of RDAG’s role, or of how and when it interacted with CPAG. In particular, patient representatives were surprised to discover that RDAG’s remit within specialised commissioning was not to consider proposals relevant to rare diseases, but those that meet the criteria for ‘highly specialised’. This is defined as those required by fewer than 500 patients. As a result, RDAG is currently only asked to consider commissioning policy proposals for patients with the rarest conditions, as well as proposals for very small populations that are not associated with a rare disease, for example hand transplant surgery.

Patient representatives felt that RDAG’s current positioning reflected a vestigial emphasis on highly specialised services. They noted the absence of a ring-fenced budget for highly specialised commissioning within NHS England’s new structure and questioned whether the expertise in RDAG would be better used during the development of commissioning policy proposals specifically for rare diseases.

It was proposed that RDAG be a source of advice and guidance to CRG’s in the process of developing commissioning policies for rare diseases. Their role could involve ensuring that the patient voice was identified and heard or that evidence outside of clinical trial data was given due consideration, for example. Patient representatives felt that in its current position, RDAG’s role was limited to returning unsuccessful proposals back to CRG’s, which might have been avoided if RDAG had inputted into the policy while it was still being developed.

Patient representatives agreed that redefining RDAG’s role was essential but were keen to ensure that it did not become “just another step in an already long process”. Patient representatives also felt that, given the number of rare diseases that primarily affect children, RDAG’s membership should include more paediatric clinicians.
NHS England should provide milestones within its evaluation timeline, with details of what action will be taken if a milestone is missed

Looking at the complexity of NHS England’s governance structure for evaluating medicine commissioning policy proposals, patient representatives were left with concerns over how long it would take. They noted that many patients affected by rare diseases have life-limiting conditions for which there is no treatment, making timely access to new medicines particularly vital. They felt that without deadlines for the completion of each stage, there would be less urgency within NHS England to progress a medicine evaluation, that it would be harder to know what will happen next and that it would be difficult to manage the expectations of patients waiting for a medicine.

There has been some reluctance from NHS England to give even an informal timeline for their evaluations; and without information about historical evaluations available online, it is impossible to infer what a realistic timeline might be. Some patient representatives reported that when they had enquired about how long it would take for their commissioning policy proposal to be considered, they had been reminded that NHS England’s default position for specialised commissioning is not to fund a medicine and so if pushed to make a hastier decision than they are more likely to reach a negative outcome.

In putting a timeline together, patient representatives were keen to ensure that NHS England outlined what procedure would be followed if, once a reasonable time had lapsed, there was still no commissioning policy decision. Some patient representatives described their experiences of going round and round the system, constantly being sent back to CRGs for further work or to the financial team for reconsideration and sign-off. They felt that it would not be fair to ask patients to wait for the outcome of a commissioning policy evaluation for longer than 12 to 18 months, and asked for NHS England to establish a clear pathway to be followed should that deadline be missed.

Horizon scanning information should be used by NHS England in collaboration with NICE to plan resource allocation with regard to future evaluations

Being able to plan for what commissioning policies will require an evaluation in the coming year is key if NHS England is to make timely decisions. NICE begins identifying the medicines to be appraised and carries out the ‘scoping’ stages as early as possible so that an evaluation can begin as soon as the medicine has been granted a license. Patient representatives were keen to ensure that NHS England was similarly aware of what medicines were in the pipeline so that sufficient time and resources could be made available within specialised commissioning to evaluate them. They agreed that to achieve this, NHS England would have to work closely with NICE to identify which medicines require an evaluation and, importantly, which route would be the most appropriate in that instance.

Based on historical information, it can be expected that approximately 13 orphan medicines will be licensed by the EMA next year. Assuming that NICE appraises three medicines through its HST Evaluation Programme, and perhaps one or two through either the STA or MTA processes, that leaves approximately eight new medicines to be evaluated by NHS England.

“It feels like you could get stuck in an endless loop of despair”

“It helps to know the [drug discovery] pipeline, that way you can predict and be prepared for what’s coming”
The actual number is likely to be higher because there are recently licensed rare disease medicines that have not been considered properly by NICE or NHS England. There is also scope for CRGs to develop commissioning policies for unlicensed medicines. This puts many more medicines within the scope of an NHS England evaluation; making it even more crucial that potential medicines are identified in advanced and prioritised effectively, in accordance with patient need.

Patient representatives were keen for NHS England to recognise the demand for medicine evaluations that they are likely to experience in the immediate future. Given the backlog of products that many CRGs inherited into their work plan, as well as the influx of stratified medicines that is anticipated as more common conditions are broken down into smaller genetically and molecularly defined subgroups, patient representatives were also keen to ensure specialised commissioning could be sustained in the longer term. They called for NHS England to develop structures and processes for specialised commissioning that will be able to cope with this demand now and in the future.

Another important issue raised by patient representatives was the efficiency of NHS England’s evaluation process. The time it can take for patients to access a medicine if a specialised service is not already commissioned. While they recognised that it was important to have a service in place through which patients can access a medicine, they did not agree with NHS England’s current process that requires a service to be approved before a medicine can even begin to be evaluated.

Being able to consider commissioning policy proposals for a service and a medicine simultaneously would streamline the process and dramatically reduce the time patients would have to wait to access a medicine. Given that patient populations who require but currently lack a dedicated specialised service are likely experiencing sub-standard care, the need to rapidly evaluate and provide both the service and the medicine is urgent. Patient representatives agreed that these proposals should therefore be a priority, rather than requiring patients to wait while the evaluation process runs twice over.

Decisions about how the following year’s specialised commissioning budget will be spent are currently made every December. This enables NHS England to set up with its providers the services and/or treatments that will be made available from the following April. While patient representatives could see how this ruling enables NHS England to regulate their expenditure on an annual basis, they felt that this was insufficient justification for delaying patient access to medicines. They felt it was both reasonable and logistically feasible to implement a prioritised medicine commissioning policy within three months of a decision from SCOG given that medicines recommended by NICE are legally mandated to be available within this time-frame.

**Medicine commissioning policies should be considered in parallel with service specifications when this will lead to speedier patient access**

**Medicine commissioning policies that have been prioritised for funding should be implemented within three months**
The success of commissioning policy implementation and resultant expenditure should be measured

Patient representatives agreed that an important final step in NHS England’s evaluation process for medicines should involve keeping track of uptake within the NHS. They felt that this was necessary for a number of reasons.

Firstly, given the history of fractured and poorly coordinated care within rare diseases, patient representatives were keen for NHS England to make certain that medicines they had approved were reaching the patients that needed them. They agreed that oversight of the implementation of commissioning policies was a critical component of quality assurance.

Medicines Optimisation CRG

This CRG works across all service-specific CRGs. Its key role is to work with experts from across the country to ensure that systems and levers are in place for selecting, buying and using medicines in secondary care (by clinical specialists or consultants). This aims to ensure that secondary care is as effective as possible; reducing harm from medicines and ensuring value-for-money.

Given that some rare diseases medicines are expensive, patient representatives felt that it was important for NHS England to have an accurate record of patient population size and total expenditure. They agreed that this would enable NHS England to plan their future budget allocations, particularly in those circumstances where the number of patients diagnosed with a condition increases following the approval of a treatment. They recognised that it was important for NHS England to be able to continue to assess the cost-effectiveness of their commissioning policies in light of this data, including the continued requirement for nationalised commissioning by NHS England.

Patient representatives proposed that this monitoring activity could be incorporated into the remit of the Medicines Optimisation Clinical Reference Group.
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Developing a prioritisation framework for directly commissioned services. NHS England, September 2014


Please see our forthcoming document on National Institute for Health and Care Excellence and rare diseases (expected publication January 2015)

