Specialised Healthcare Alliance
FOR EVERYONE WITH RARE AND COMPLEX CONDITIONS

Report on specialised haemophilia services and QIPP

Contents

1. Executive summary

This report forms one in a series by the Specialised Healthcare Alliance looking at services prioritised by the National Specialised Commissioning Group in relation to the delivery of quality and productivity at a time of spending constraint, otherwise known as QIPP. It was particularly informed by a stakeholder workshop on haemophilia services organised by the Alliance and the North East Specialised Commissioning Group in Newcastle on 20th October 2010. A wide range of stakeholders including patients, commissioners, clinicians, other healthcare professionals and representatives from the Haemophilia Society attended the workshop.

The report sets out some background information on QIPP and haemophilia services before seeking to distil the major themes explored during the workshop in relation to treatment, care and outcomes.

Among the most important points to emerge, attention is drawn to this report's concluding recommendations and especially:

- **The importance of moving swiftly to agree outcomes against which to measure treatment regimes, including levels of clotting factor usage, focused on helping people with haemophilia to lead a normal life;**
- **The need for greater consistency of decision-making, drawing on agreed protocols and potentially supported by the proposed NHS Commissioning Board;**
- **The role of networks in facilitating access to comprehensive care and the highest standards of care at other times.**

2. Background

2.1 What is "QIPP"?

Quality, Innovation, Productivity and Prevention (QIPP) is the flagship policy being used by the NHS to find the £15-20 billion of savings identified by Sir David Nicholson as necessary in 2011/14 as a result of rapidly rising demand for services and a challenging fiscal climate.[1]

The overall aim of the scheme is to combine improvements in quality of care with efficiency savings that can be reinvested in front-line services. Ideally, quality and productivity will go hand-in-hand, providing a better service for the patient, as well as cost savings for the NHS as a whole.[2]

The National Specialised Commissioning Group (NSCG) has prioritised ten services for taking forward the QIPP agenda, with each Specialised Commissioning Group (SCG) leading on one of the services.

In each case, the NSCG has established three main objectives in relation to QIPP as follows:

[1] For background on QIPP: *The NHS quality, innovation, productivity and prevention challenge: an introduction for clinicians* (March 2010), available from here

[2] See NHS Improvement's QIPP site for more background

- Working through the SCG Finance Network, benchmark and demonstrate value for money in the agreed services;
- Working with the SCG Public Health Network, develop common CQUIN (Commissioning for Quality and Innovation)[3] goals for the agreed services;
- Working through the SCG Public Health Network, develop common health outcomes for the agreed services.

The North East SCG was the lead for specialised haemophilia services. The Specialised Healthcare Alliance is worked with SCGs to review these services with the aim of ensuring a balanced discussion between the four strands of QIPP.

QIPP is clearly important in the context of specialised haemophilia services. Given the often high cost and pace of innovation in specialised haemophilia services, the challenge is to retain and increase quality in an environment where there is pressure to deliver significant efficiency gains and savings.

2.2 Specialised haemophilia services

Prevalence of haemophilia and treatment

Haemophilia A (factor VIII deficiency) and haemophilia B (factor IX deficiency) are the most common inherited bleeding disorders of significant clinical severity, affecting approximately 1:10,000 and 1:50,000 total births respectively[4]. The total numbers of patients with haemophilia A and B registered in the UK in 2010 were 5346 and 1125 respectively of whom 1800 and 391 were severely affected[5].

The main cost associated with haemophilia care relates to clotting factors. Usage for severely affected patients including those with inhibitors in 2009/10 shows a range of units per capita from 3.7 for factor VIII in the North West to 8.3 Units in the Pan Thames area and a range for factor IX of 0.36 in the South West to 1.76 in the East of England..
There has been a fourfold increase in use of Factor VIII since 1990[6].

Policy framework

The standard service model for haemophilia is set out in DH Health Service Guidance (HSG) (93) 30 Provision of Haemophilia Treatment and Care, issued as far back as 1993.

Haemophilia has, however, been the subject of intense policy debate as a result of contaminated blood products in the 1970s and 1980s infecting large numbers of patients with HIV/AIDS and Hepatitis C, many fatally.

In the late 1990s, this led to the introduction of a recombinant only policy for young, previously untreated patients. Funding to extend this to all patients was agreed by Ministers in 2003 and vested in Strategic Health Authorities.

In February 2009, the independent Archer Inquiry[7] reported on NHS Supplied Contaminated Blood and Blood Products. This recounted what one witness, Lord Winston,

[3] The CQUIN payment framework makes a proportion of providers' income conditional on quality and innovation. See here for more information.
[4] SSNDS Definition No.3, Specialised Services for Haemophilia (all ages) (3rd edition)
[5] UKHCDO Annual Report 2008/09
[6] ibid
[7] The Archer Inquiry, Independent Public Inquiry Report on NHS Supplied Contaminated Blood and Blood Products, Published 23 February 2009

described as the worst treatment disaster in the history of the NHS, a view with which the Inquiry agreed. In looking to the future, it observed that the involvement of patients in the evaluation of available treatments and the risks associated with their use is essential, urging that patients must be able to make informed choices about their treatment.

National definition

Definition Number 3 of the third edition of the Specialised Services National Definitions Set (SSNDS) published in 2010 covers specialised services for haemophilia and other related bleeding disorders, describing specialised activity in the following terms.

The specialist multidisciplinary team provides diagnosis and counselling, treatment, rehabilitation and follow-up of patients with inherited and acquired bleeding disorders. The team includes clinicians, specialist nurses and physiotherapists. The majority of patients are treated on an out-patient basis but there will be number of in-patient admissions, largely patients having surgery or patients with serious bleeds that are not on home treatment with clotting factor concentrates. The specialist team provides a 24 hour on-call service for patients and their families.

Out-patient and home services are an important aspect of the overall service and provide training for home treatment programmes and rehabilitation in the patients' home as well as advice, support and clinical monitoring. In contrast to many other chronic diseases, the delivery of the home based service is provided by the Comprehensive Care Centres (CCCs) and large Haemophilia Centres (HCs) rather than PCT funded community based services.

A 24-hour advice service is offered by the CCC to local hospitals and primary care teams.

A specialist surgical service is provided through the CCCs, most commonly for orthopaedic surgery and for dental surgery (including conservative dentistry and specialised oral surgery for adult and paediatric patients). As well as the surgical complications associated with bleeding, patients may have infectious diseases (such as HIV and hepatitis). Over 4000 patients with inherited bleeding disorders are at risk of vCJD for public health purposes, which complicates their surgical care as surgical instruments may require destruction or long term quarantine after use on these patients.

The care of children with haemophilia can be complex and good quality care in childhood significantly reduces morbidity in adults. Children are managed in collaboration with specialised haematological and paediatric services. Some CCCs manage all ages and some CCCs are dedicated to children; in the former case children are managed in an age appropriate environment. Where a child requires insertion of port-a-cath and / or central venous lines the responsible surgeon has paediatric expertise in such techniques. Specific arrangements are in place to manage the transfer of adolescents from paediatric care to adult care.

The specialist laboratory service supports the specialist CCC by providing definitive diagnosis of inherited and acquired bleeding disorders and by monitoring treatment effects thereafter. In particular they identify and assay specific blood clotting factors and other relevant plasma and cellular proteins/components. The specialist laboratory service also acts as a reference laboratory facility for other hospitals (other than the CCC) assisting in the diagnosis of atypical and complicated cases, supplying recommendations on assay standards and reagents, and giving advice on analytical procedures.

Specialist genetic services identify specific and relevant genetic mutations giving rise to the inherited bleeding disorders. In haemophilia, in contrast to many other genetic disorders, most of the counselling is carried out by staff in the CCC. They collaborate closely with foetal medicine and maternity services to ensure rapid prenatal diagnosis and management of inherited disorders. Where appropriate there may be further investigation of the relatives of affected patients. (Guidelines on Genetic Services have been produced by the UKHCDO Genetics Working Party). The identification of specific mutations can be useful for indicating the most beneficial treatment regimes and the likelihood of inhibitor development.

Service delivery and outcomes

The Haemophilia Alliance's national service specification for haemophilia and other bleeding disorders, as revised in 2006, sets out the essential components of high quality care[8].

More recently, the requirement to designate service providers has prompted the London Specialised Commissioning Group to develop model documentation in collaboration with all other SCGs, drawing on the Alliance's service specification and other sources. This has subsequently been endorsed by the National Specialised Commissioning Group.

The model documentation reflects the HSG by stating that 40 severely affected patients should be the minimum caseload for a specialist haemophilia centre. There is a distinction between Comprehensive Care Centres and Haemophilia Centres. UKHCDO data show that a substantial proportion of patients is being treated at centres with fewer than 40 severely affected patients.

The documentation sets out a series of core standards which should be expected of all centres while highlighting the availability of developmental standards to meet individual SCG's local needs. It later addresses clinical and patient outcomes. In relation to the former, it observes that potential clinical outcome measures in development include:

- Measurement/minimisation of the number of school days lost to children with haemophilia
- Measurement/minimisation of the number of working days lost for adults with haemophilia
- Measurement of the numbers of bleeds
- Monitoring breakthrough bleeds
- Measurement of joint scores pre/post surgery

In the first instance, it proposes that all centres should measure the number of joint immobilising bleeds.

The development of patient outcome and satisfaction measures is also advocated with pain scores as the first national patient satisfaction measure.

3. Main themes

The workshop combined scene-setting presentations and interactive sessions. The latter aimed to ensure that all those attending had an opportunity to contribute to multi-

[8] Haemophilia Alliance (2006) 'A national service specification for haemophilia and other inherited bleeding disorders, 2nd edition'

disciplinary discussion involving clinicians, commissioners, policy makers, patients and their representatives.

Overall, the workshop was broken down into the following three main sessions:

- Treatment;
- Care;
- Commissioning for outcomes.

The workgroup sessions were designed to be mutually supportive and to identify relevant points across the QIPP agenda. For example, improvements in care pathways are likely to lead to a better patient experience, enhanced health outcomes, higher quality care and improved productivity.

The following sections summarise the information presented to the workshop under each of the main headings and the workshop discussion in relation to a series of subsidiary points thereafter.

3.1 Treatment

The workshop heard about the Haemophilia Society's commitment to working in partnership with all those responsible for delivering care. The Archer Report had given powerful backing to the need for patients to be fully involved in securing access to the best treatments, with standards of care now featuring in twice yearly meetings with the Department of Health and the Society involved in key initiatives such as national procurement of clotting factors.

The benefits of high quality treatment were exemplified by personal testimony from an adult patient concerning the transformative effect of prophylaxis in reducing bleeds and delivering improved mobility and reduced pain. From a parental perspective, safety of treatment remained the foremost consideration.

As things stood, a minority of patients was on prophylaxis with the potential for a continued increase in usage of clotting factor and therefore cost with additional uptake. A proposed study to look at dosing adjusted for the half life of Factor VIII in individual adult patients held out the prospect of savings. At the same time, it was pointed out that UKHCDO data showed significant overlap in usage between patients being treated on demand and prophylactically and that changes to treatment patterns could offer savings. Work on gene therapy, initially for people with haemophilia B, was also showing real signs of progress.

In subsequent discussion, the following issues were explored:

- **Is recombinant for all still relevant?**
  There was strong support for the continuing importance of recombinant treatment in delivering the safest possible treatment with reference to inevitable unknowns in the field of viral contamination of plasma-derived product, though continued access to the latter remains important for particular patients.
- **Are patients engaged in treatment decisions?**
  This is a major recommendation of the Archer report. Most patients were reported to feel involved while some didn't want to be. Involvement in non-clinical decisions was also important.

- **What should be the outcomes of treatment?**
  A nationally agreed approach was seen as crucial in assessing the value of usage levels. Candidate measures included loss of days at school or work, reduction in bleeds, including intra-cerebral bleeds, and joint scores.
- **Are there any areas where we cannot demonstrate improved outcomes so should not be prioritised for investment?**
  A note of caution was struck about over-centralisation of treatment. There was also acknowledgement that without consistent outcome measures and monitoring it was difficult to show any improvement as a result of treatment, so this needs to be addressed urgently.
- **What should the approach to adult prophylaxis be?**
  A targeted approach was advocated starting with children with good joint scores following prophylactic treatment in transition to adult services to ensure that healthy joints were maintained. The extension of prophylaxis needed to be combined with the development of patient expertise. Prophylaxis is neither preferred nor appropriate for all patients and this should be respected.
- **Are there any gaps in consistent guidelines e.g. treating inhibitors, orthopaedic surgery, end of life care, acquired conditions?**
  The need for guidelines on orthopaedic surgery received strong support. These needed to cover issues like timing, where conservative management sometimes exacerbated joint damage and eventual costs. The length of time for increased factor usage following surgery was also a key issue in need of greater consistency. Elsewhere, guidelines on physiotherapy for local use would be beneficial; physiotherapy for people with haemophilia needs to be specialised or can inflict unwitting damage.

3.2 Care

As a prelude to developing a model of care for the Pan Thames Haemophilia Consortium, extensive qualitative and quantitative engagement had taken place with service users. This showed high levels of satisfaction, though with emergency care and out of hours access more of a concern. When asked to choose between local and specialist services for planned care, people preferred to travel for specialist treatment.

Models of care constituted a pre-requisite for designation. Audit and accreditation of services by UKHCDO was also relevant. The current arrangements sprang out of HSG 93 (30) but, along with HSG 93, were in need of updating. In particular, scrutiny of Haemophilia Centres was being tightened in line with Comprehensive Care Centres, while the role of patient auditors was being strengthened. The DH had yet to respond to UKHCDO's call for HSG 93 to be revised.

Group discussion shed additional light in a number of areas as follows:

- **What aspects of comprehensive care can be delivered locally and what needs to be centralised for greater critical mass?**
  The suggestion was made that ITI and inhibitor treatment should be centralised more nationally. Other aspects of care seen as benefiting from greater clinical mass were orthopaedic surgery, diagnosis, joint clinics for obstetric care and all children's services. It was important that the majority of local care should be delivered within a managed clinical network.
- **Is there scope to increase home delivery?**

Levels of home delivery vary significantly around the country. Home delivery is presently free of VAT, which is set to increase to 20 per cent next year. Making home delivery the norm was therefore an attractive goal but needed to be managed to ensure good outcomes around the patient's needs. Centres which did not provide home delivery should be encouraged to move towards it. Home delivery contracts with companies in Scotland were seen as quite inflexible. The new national framework agreement for England provided more flexibility with weekend and early and late deliveries making moves to home delivery easier to accommodate.

- **How can equitable access to comprehensive care services be assured?**
  Among the suggestions were the provision of transport, use of technology such as Skype, wide circulation of contact numbers and the establishment of networks enabling CCCs to support HCs.
- **Could transition to adult services be better managed?**
  Transition is a more extensive issue than just moving from paediatric care to an adult clinic setting, encompassing, for example, children moving from primary to secondary school and, arguably, the need to make children's hospitals more congenial for older children. The move to adult services itself was often as or more difficult for parents than children.
- **What access should people with bleeding disorders have to other services?**
  Reference was made to physiotherapists and dietitians, who should be locally available and supported by the tariff. Other services mentioned in the National Definition are:
  Definition No.19, Specialised Services for Liver, Biliary and Pancreatic Medicine and Surgery(adult)
  Definition No.20, Medical Genetic Services (all ages)
  Definition No.23, Specialised Services for Children; particularly sections on: specialised paediatric anaesthesia & pain management services; dentistry services; ear, nose & throatservices; gynaecology services; non-malignant haematology services; neonatal care services; oral & maxillofacial surgery services; orthopaedic surgery services; surgery services
  Definition No.26, Specialised Rheumatology Services (all ages)
  Definition No.31, Specialised Pain Management Services (adult)
  Definition No.34, Specialised Orthopaedic Services (adult)
- **Do networks provide more opportunities?**
  The view was generally held that networks could help to raise clinical standards and foster constructive collaboration between trusts and that the development of networks provided the opportunity for equitable access to comprehensive care.
- **How does audit and accreditation of centres support service improvements?**
  The relationship between designation and audit and accreditation needs to be clarified to avoid duplication. The view was expressed that audits presently tend to pick up on issues with the host hospital rather than the centre itself. Audit therefore requires outcome measures as well to be effective.

3.3 Commissioning for outcomes

The London SCG has been leading nationally on materials to support the designation of haemophilia providers. This is predicated on the whole pathway being commissioned by the responsible SCG, with all patients having access to comprehensive care when required. Of 61 centres treating severely affected patients, only 18 see more than 40 such patients as stipulated by HSG 93. Driving up quality through designation could therefore change the provider landscape.

- **What are the best outcome measures to demonstrate continuous improvement in care?**
  In essence treatment should be aiming to help people with haemophilia lead a normal life and outcome measures should focus on related issues with data collected through the National Haemophilia Database. Suggestions included days off school and college, days off work, joint scores, pain and breakthrough bleeds. Care had to be taken to avoid outcomes triggering unintended adverse results. For example, a focus on surgery under 18 might delay earlier treatment which was in the best interests of the patient. A UKHCDO working party including patient representation has been set up to look at these issues. CQUINs provide a potential mechanism for supporting delivery of well designed outcome measures.

3.4 Recommendations

In the light of the workshop, the following recommendations are made:

- Recombinant remains the treatment of choice for safety reasons, subject to the needs of individual patients;
- Patients should be involved in all decisions affecting their treatment, both clinical and non-clinical;
- A nationally agreed approach to outcome measures is an urgent priority, while taking care to avoid unintended adverse consequences;
- Adult prophylaxis should be extended on a targeted basis, starting with young patients entering adult services with good joint scores following prophylaxis as children;
- Guidance on orthopaedic surgery, including the related use of clotting factors, should be prioritised as the first of a series of protocols informing more consistent decision-making;
- Home delivery should be the norm for all patients receiving treatment in the home, built around their needs;
- Haemophilia networks should be developed across the country as a means of ensuring equitable access to comprehensive care, underpinned by robust auditing.