



# Leaving No One Behind

Delivering High Quality, Efficient Care  
for People with Rare and  
Complex Conditions

**Specialised Healthcare Alliance**  
FOR EVERYONE WITH RARE AND COMPLEX CONDITIONS

# Foreword

## **Baroness Pitkeathley OBE, Chair**

Over the last two years, the Alliance has burgeoned, with membership now standing at 61 patient-related organisations supported by nine corporate members. More importantly, we are delighted by the growing strength of our relationships with the NHS, borne out by the recent multidisciplinary workshops looking at a range of services prioritised in relation to QIPP – Quality, Innovation, Productivity and Prevention. We are grateful to the regional Specialised Commissioning Groups for their invaluable help in making the workshops happen and in ensuring such excellent discussion and debate. We have also been delighted by participation from the other home nations.

Along with its membership, the Alliance's influence has continued to grow over the last two years. This was seen most vividly in the House of Commons' Health Select Committee's report on commissioning in March 2010, which gave such prominence to our analysis of specialised commissioning arrangements. We now see real potential in the Health and Social Care Bill's proposal to place responsibility for specialised services with the NHS Commissioning Board, providing we can get the all important detail right.

The truth is that healthcare is becoming ever-more specialised as our understanding of rare and complex conditions and their treatment becomes more sophisticated. A key task for the Alliance in these straitened times is to identify how we can harness the benefits of specialism with the needs of individuals and their families.

In his remarks to our conference last November, Earl Howe, Minister for Quality at the Department of Health, endorsed the commitment in the NHS Constitution that “no one should be left behind” simply because their condition is rare and therefore more expensive to treat.

The Alliance publishes this report as a contribution to the development of a strategy for delivering this commitment.



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# 1. Executive Summary

- 1.1 The Specialised Healthcare Alliance is a coalition of 61 patient-related organisations supported by nine corporate members which campaigns on behalf of people with rare and complex medical conditions.
- 1.2 The Carter Review of Commissioning Arrangements for Specialised Services in 2005/06 marked a watershed in the development of associated policy and has yielded real benefits for patients in the years that followed. As the House of Commons' Health Select Committee concluded last year, however, implementation has been far from complete, reflecting structural weaknesses which Carter sought to address but could not eradicate.
- 1.3 The coalition government's proposals for reform of the NHS in England therefore mark a major opportunity to consolidate and build upon the gains of Carter. These changes also coincide with the EU Council's Recommendation on action in the field of rare diseases. A coherent approach to specialised services, drawing on the legacy of Carter and the opportunities of reform, should serve to meet the EU Recommendation's call for member states to establish and implement strategies for rare diseases by the end of 2013.
- 1.4 Accordingly, this report takes stock of recent developments in specialised commissioning and the legacy of Carter before going on to identify a series of key drivers in delivering improved care and value for people with rare and complex conditions. In doing so, it draws on a series of workshops the Alliance arranged with regional Specialised Commissioning Groups (SCGs) during the last quarter of 2010 looking at a number of specific high cost services. It is also informed by discussion and debate at the Alliance's national conference held in November.

- 1.5 Times have moved on, but many of the principles set out in the Carter Report remain relevant for high quality specialised care across the UK, regardless of commissioning arrangements. Thus a significant conclusion of these deliberations is that the baby should not be thrown out with the bath water and that many of the principles set out in Carter remain valuable in a reorganised NHS.
- 1.6 Of equal significance, the Alliance's workshops and conference have underlined the importance of involving patients, families and their representative organisations right, left and centre in the design and delivery of care. Despite the rhetoric, this is still often lacking. Yet people understandably have valuable insights about their symptoms at the time of diagnosis and are exceptionally well placed to comment on the design and delivery of care, often in the home. This extends to efficiencies, where patients involved in decisions about their care are more likely to lend support.
- 1.7 The Alliance looks forward to working in partnership to advance the main recommendations of this report, focusing on:
- The need for the doctor/patient relationship to evolve in a way which speeds diagnosis through better referral pathways and the ability of many patients to help inform diagnosis, predicated on well signposted sources of reliable information;
  - The role of patient organisations in protecting the vulnerable and driving up standards, sometimes through the provision of more innovative services;
  - The need to ensure integrated pathways, especially through the development of multidisciplinary networks;
  - The contribution of NICE quality standards or their equivalent to high quality specialised care and outcomes;
  - The desirability of outcome measures which capture both clinical and patient perspectives with a view to maximising effectiveness and efficiency;
  - The central importance of patient registries, with more sharply focused datasets helping to keep down costs.

Attention is also drawn to the service specific recommendations arising from the workshops.

## 2. Health Select Committee Report

- 2.1 In a survey of NHS management in 2003, the Specialised Healthcare Alliance found that specialised services were often referred to as an afterthought in government policy. The decision of the House of Commons' Health Select Committee to look at specialised commissioning as a major component of its 2009/10 inquiry into commissioning therefore represented a rare opportunity to bring the issue centre-stage.
- 2.2 The Committee took as its starting point the Carter Review of specialised commissioning in 2006. The genesis of the review was the recognition that PCTs were not collaborating effectively in the specialised arena, to the potential detriment of people in need of specialised care. The need for collaboration hinges on optimising the development and use of clinical resources for relatively smaller patient populations, for whom the NHS is also uniquely well placed to share financial risk.
- 2.3 Carter set out the components of first class commissioning arrangements for people with rare and complex conditions, along with proposed governance arrangements to deliver them, couched in the NHS structures of the time. The Health Select Committee noted that implementation of Carter had been widely seen as positive for specialised services but that progress had been patchy, meaning that there was significant inconsistency in care between different parts of the country.

### The patient's role in diagnosis

**Sandy Yarrow (64) from Henley on Thames contracted polio when she was 11**

"I was diagnosed with polio when I was 11 and had to stay in hospital in isolation for five weeks.

Polio left me with a very weak arm and I became quite breathless doing activities but I was determined to carry on pursuing my greatest passion - dancing. However not being able to fully lift and control my arm meant I had to give it up, which broke my heart as I had just been offered a solo part in the West End.

When I was 44 I was diagnosed with ME, but after reading an article on the illness I found that many of the symptoms are similar to that for Post Polio Syndrome (PPS). It was a flash of realisation for me. I contacted the consultant who wrote the article and he provided me with information on PPS which I took to my GP and this led to my illness eventually being confirmed.

My life could have been very different but I have a wonderful husband, and two lovely daughters and now three grandchildren and I wouldn't swap them for the world."

# The importance of patient choice and integrated care

**Robert Hodgkins has been living with haemophilia from a very early age.**

"I was diagnosed with haemophilia almost from birth. The development of treatments in this era such as clotting treatment, factor VIII and recombinant manmade factors enabled me to be treated quickly, preventing the loss of my life through injury and stopping joint damage. These were the early highpoints, although spending one or two days a week in hospital as a child was normal.

But, frustratingly, not having been treated with regularly administered prophylaxis, joint damage still occurred. This has caused me a variety of problems and has added to the limitations I already had. Although learning to treat myself gave me more independence, as a child, there was no home delivery system in place and getting the treatment from the hospital was problematic, with both of my parents having to take time off work to collect it. The situation is now much improved with home delivery and a more integrated approach leading to significant improvements in my care. With prophylaxis and good advice from an orthopaedic surgeon, I was able to decide not to have surgery which would have resulted in fixed ankle joints and permanent loss of movement. I am now able to exercise in a gym regularly and do not need a walking stick."

## 3. National Commissioning

- 3.1 While the Health Select Committee's deliberations on regional specialised commissioning unfolded, the Department of Health consulted on significant changes to national commissioning. These have been more extensively explored in the Alliance's previous report "The challenge of rarity" but focused on:
- Strengthening the governance surrounding decisions on the national commissioning of services, products and technologies; and
  - Development of an ethical framework to help inform such decisions.
- 3.2 In relation to governance, the main proposal concerned the introduction of a single advisory group giving direct advice to Ministers. The Advisory Group on National Specialised Services (AGNSS) is composed of a broad spectrum of interests from commissioners, through clinicians to lay and patient representatives, a health economist, ethicist, geneticist and pharmacist.
- 3.3 The ethical decision-making framework is broken down into two steps, the first setting out nine entry criteria and the second holistic assessment based on twelve clearly defined core criteria. Entry criteria include a clinically distinct patient population for the product, service or technology usually of no more than 500 and/or four centres in England. The 12 core criteria, centred on patients' needs, are broken down into four clusters as follows:

1. Health gain (does it work?)
  - a. Severity and ability of patient to benefit
  - b. Clinical safety and risk
  - c. Clinical effectiveness and potential for improving health
2. Societal value (does it add value to society?)
  - a. Stimulating research and innovation
  - b. Needs of patients and society
3. Reasonable cost (is it a reasonable cost to the public?)
  - a. Value for money compared to alternatives
  - b. Overall cost impact and affordability, including opportunity cost
  - c. Average cost per patient
4. Best practice
  - a. Accessibility and balanced geographic distribution
  - b. Continuity of provision
  - c. Economic efficiency of provision
  - d. Best clinical practice in delivering the service

The new arrangements were signed off by the Secretary of State for Health following the General Election in May 2010 with Professor Michael Arthur, Vice-Chancellor of Leeds University, becoming the inaugural Chair prior to AGNSS's first meeting in September.

#### 3.4 The SHCA is now working to promote:

- Clarity about the criteria determining whether a product or technology will be assessed under the new arrangements or by NICE. These might be expected closely to reflect the decision-making framework;
- Transparency in the process, so that all parties can see the major factors influencing decisions about which products and technologies should attract funding;
- A holistic approach, which demonstrably weighs all the complex considerations governing health gain, societal value, reasonable cost and best practice;
- Ethical consistency in decision-making across the NHS drawing on the framework as a template.

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

## 4. The White Paper

- 4.1 In the meantime, the coalition government published its White Paper “Equity and Excellence: Liberating the NHS”. Although, on the commissioning front, most attention has understandably been given to the replacement of Primary Care Trusts by GP commissioning consortia, the White Paper also proposes sweeping changes to the way in which specialised services are commissioned. In particular, responsibility and funding for both national and regional specialised services are set to rest with the NHS Commissioning Board (NHSCB).
- 4.2 Accordingly, the White Paper reforms have exciting potential to address the shortcomings identified by the Health Select Committee prior to the General Election. The Alliance has, however, highlighted a number of issues, including:
- The scope of the NHS Commissioning Board's remit for specialised services needs to be clearly defined. In the Alliance's view, the only credible basis for doing so is the recently revised third edition of the Specialised Services National Definitions Set. This should be in its entirety, though subject to regular revision;
  - Similarly, the Board's regional presence needs to be clarified and sufficiently extensive to sustain effective engagement with the NHS at local level, eg to ensure that referrals management is consistent with more timely diagnosis of rare and complex conditions. The Alliance recommends a minimum of eight offices. This would also make it easier to retain the best of the important expertise in the current SCGs;
  - Urgent priority should be attached to costing patient pathways covered by the National Definitions Set to inform the budget required by the Board for specialised commissioning purposes from April 2012. A buffer fund may be required to accommodate likely inaccuracies in the sums retained by the Board or devolved to GP commissioning consortia, especially in the first years of the new arrangements. This might also act as a deterrent to cost shifting;

- In the meantime, PCTs are likely to become increasingly distracted by the move towards GP commissioning consortia. The NHS Commissioning Board should therefore assume early oversight of specialised services from April 2011, initially working through the existing SCGs;
- The accountability of the Board for commissioning specialised services needs to be clearly defined, with recourse for individuals and groups who feel they have been poorly served. These patients must also be enabled to play a role in the new HealthWatch arrangements.

## The need for tailored provision of specialised services

**Phillippa Farrant is the mum of 18 year old Daniel, who has Duchenne muscular dystrophy.**

Duchenne is a rare, genetic condition which is life-limiting, progressive and severely disabling. Life expectancy for boys affected can be only the late teens to early twenties without the right support provided by a multi disciplinary team of neuromuscular specialists. The team can aid a timely diagnosis, and provide care and support to the family throughout the lifetime of this condition, which is especially important at the time of transition into adult services when many patients fall through the net.

Phillippa has sought out expert care at every stage for her son. However, she has constantly come up against barriers – a lack of understanding of neuromuscular conditions, poor communication between health and social care professionals and insufficient services. Ongoing physiotherapy and hydrotherapy is essential to keep Dan as mobile as possible in his wheelchair – preventing muscle contractures, reducing pain and aiding his breathing, preventing chest infection, which could seriously endanger his health. However, such services are not available. The lack of provision means that young men like Dan may have unnecessary stays in hospital, costly to their health and quality of life but also to the NHS.

When Dan was diagnosed, Phillippa was told that he would only live to 13. He's now 18 – officially an adult. With specialist care Dan could live for many more years. They are now planning his 21<sup>st</sup> – and hope that the NHS can get him there.

## 5. Carter's Legacy

- 5.1 Clearly, the prospective arrival of the NHS Commissioning Board renders the Carter Report largely redundant in terms of the structures and governance necessary to support specialised commissioning. In terms of the aspirations of the Board, however, many of the principles enunciated in the report hold good, as set out below.

### **Finance**

- 5.2 Carter recommended that all specialised services covered by the Specialised Services National Definitions Set should have been defined, quantified and costed by 2008/09. This has simply not happened and should be an urgent priority for the Board, not least because it will help inform the sums retained by the Board and devolved to GP commissioning consortia.
- 5.3 Furthermore, SCGs were invoked to have an annual process for debating priorities and explicit mechanisms for eliciting and documenting the views of all interested parties, including providers and patient and public representatives. A similar process is essential for the NHSCB.

### **Accountability**

- 5.4 Carter recommended that SCGs should produce an annual commissioning plan, an annual work programme and an annual report, which should be disseminated widely. The NHSCB should be in a strong position to build on this recommendation, including a periodic independent survey of stakeholders' views.

### **Research**

- 5.5 One of the few recommendations not adopted by the then government concerned funding mechanisms for pre-trial and post-trial treatment costs that are outside national tariff in order to safeguard future research. This, however, remains a key issue, as picked up in the key drivers section of this report.

### **Specialised Services National Definitions Set (SSNDS)**

- 5.6 As recommended by Carter, the National Definitions Set has been reviewed and a third edition published. In the first instance, this provides the most sensible basis for scoping the NHSCB's commissioning responsibilities. Other than a minimum planning population of one million, however, the criteria for services to be included in the SSNDS remain opaque. A more thorough review of the future purpose and structure of the SSNDS would therefore make sense once the new commissioning arrangements for specialised services have been established.

# Redesigning services to improve patient care

## Stroke service provision in London

A new strategy to tackle stroke in the capital has been developed in response to the underperformance found by the Sentinel Stroke Audit in 2004 and 2006.

In February 2009 eight hyper acute stroke units (HASU) were opened in London. Each HASU brings together stroke experts and equipment to provide 24 hour care in centres that are reachable by a 30 minute London ambulance journey. The units provide immediate response with specialist assessment on arrival, a CT scan within 30 minutes if necessary and, where appropriate, thrombolysis within three hours of the onset of symptoms. Patients spend less than 72 hours in an HASU before being transferred to a Stroke Unit (SU) which are based in local hospitals and provide ongoing multi-therapy rehabilitation.

The 2010 National Sentinel Stroke Audit has shown huge improvements in stroke care with five of the six top services being in London. All the London HASUs were in the top quartile of national performance and thrombolysis rates are higher than that reported for any other large city in the world.

The development of acute stroke services in London and elsewhere has lessons for specialised commissioning.

## Designation

5.7 Designation of providers to deliver specific specialised services was a keystone of Carter, based in each case on a nationally agreed set of patient-centred, clinical, service, quality and financial criteria. Crucially, the purpose of designation is to secure an appropriate concentration of clinical expertise and activity, located to maximise geographical access to high quality, safe and cost effective services. Although this might be seen as reflecting one of the major lessons of the Bristol Inquiry, progress has been painfully slow. The Alliance will therefore be looking to the NHSCB to inject an early and sustained sense of urgency, maybe working through networks.

## Integrated care

5.8 Integration of tertiary with local care remains one of the abiding concerns of those involved in specialised commissioning. Carter saw strong relationships with clinical networks as a major component in bringing this about, with PCTs helping to ensure a good fit between the commissioning plans of GP practices and SCGs. In practice, this has happened patchily at best.

- 5.9 The greater quality and consistency which the NHSCB should afford specialised commissioning carries with it the theoretical risk of disconnection from local care. In practice, however, the Board will be in a powerful position to bring about the engagement of GPs, with networks set to play an even more important role in bringing together responsible parties along patient pathways.

### **Choice**

- 5.10 Carter recommended that opportunities for patients to make choices regarding specific aspects of their care and treatment should be maximised with the proviso that choice of specialised service providers will be limited to designated providers. The coalition government's commitment to "nothing about me without me" and extension of choice from choice of providers to choice of treatment powerfully reinforce Carter's original ambition.

### **Payment by Results**

- 5.11 As recommended by Carter, there has been significant work aiming to progress the development of PbR for specialised services. The danger with tariffs is that they fail to capture the true costs of specialised care, for example by including data from other providers. The Alliance has therefore always favoured developing PbR in conjunction with the designation of providers for particular services, whose costs can then be captured. The emergence of the NHSCB as the sole commissioner for specialised services may, however, make PbR less of a priority.

### **National clinical databases**

- 5.12 Progress has also been limited in establishing national clinical databases for specific specialised services to enable commissioners and providers to monitor clinical outcomes and performance against standards. This has probably stemmed in part from disagreement as to who should pay. The value of databases or registries is such that withholding funds may prove a false economy.

### **Information and patient and public involvement**

- 5.13 The provision of information about specialised commissioning has improved enormously as a result of Carter both via national and regional websites. Contact details for lead commissioners for services have, however, remained hard to find and this needs to change. Patient and public involvement also needs to be embedded to an even greater extent in the new arrangements led by the Board eg in relation to prioritisation and designation.

### **Overview and scrutiny**

- 5.14 Carter recommended that in the interests of timely, well-managed service change, the DH should advise setting up joint Overview and Scrutiny Committee (OSC) standing committees based on SHA boundaries. Furthermore, it was suggested that where an SCG had applied to an individual/joint OSC and not received a decision within six months, it should be free to proceed.

- 5.15 In practice, the arrangements relating to changes in specialised services remain convoluted and problematic as the needs of larger populations are considered many times over at local level. In its response to the White Paper, the Alliance therefore proposed that the NHS Commissioning Board should be able to consult on such proposals once only, with the Secretary of State acting as final arbiter.

### **Status of commissioning**

- 5.16 Last but far from least, Carter expressed concern about the need to raise the profile of specialised commissioning, in part to address a shortage of capacity and skills. The creation of the NHSCB with its explicit focus on commissioning and direct responsibility for specialised commissioning presents a golden opportunity to remedy this longstanding problem.

## The critical importance of early care plans

### **Freddie Patenall was born four weeks early to parents Becky Jeffery and Darren Patenall and diagnosed with a rare genetic condition called ichthyosis**

Ichthyosis predominantly affects the skin but also carries other physical complications such as developmental issues, failure to thrive and often difficulty in maintaining a healthy weight in infants. Despite his diagnosis and Freddie weighing less than 5lb, he was sent home from hospital after two days with a pot of emollient cream and no formal care plan or aftercare arrangements. Becky and Darren were given no practical advice on how to care for their son and received only routine health visits.

Over the following weeks Becky and Darren raised numerous concerns such as his weight loss but health professionals had very little knowledge of the condition or how it should be managed. The lack of communication between health and social care professionals was also apparent when referrals were not made and photos went astray, never reaching the specialists who were meant to be reviewing Freddie.

Sadly Freddie passed away at 12 weeks having never seen a specialist. It was only discovered at this point that Freddie had one kidney and was blind. It was acknowledged by the hospital where Freddie died that he had not received proper care and treatment and that his blindness may have been prevented and his quality of life could have been better.

## 6. QIPP And Specialised Services

- 6.1 Quality, Innovation, Productivity and Prevention (QIPP) is the flagship policy being used by the NHS in England 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.<sup>1</sup>
- 6.2 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.<sup>2</sup>
- 6.3 The National Specialised Commissioning Group (NSCG) prioritised ten services for taking forward the QIPP agenda, with each Specialised Commissioning Group (SCG) leading on one of the services. The Specialised Healthcare Alliance proposed a collaborative approach involving multidisciplinary workshops and reports on each of the services.
- 6.4 Eight of these workshops have now occurred with nearly 400 people attending, including approximately a third commissioners, a third healthcare professionals, a sixth patient representatives and a sixth others, such as DH officials, industry personnel and very welcome representation from the devolved administrations. A workshop on medium secure mental health services has not been progressed to avoid duplication with a DH-led project, while a workshop on rarer cancers is planned for the spring of 2011, now that the government's refreshed cancer strategy has been published.
- 6.5 The individual service reports are available on the SHCA website but, for ease of reference, the major points from each are summarised below. In addition, the major outcomes of an equivalent event run by the UK Genetic Testing Network (UKGTN) are included, given the relevance of genetic testing to specialised services and the QIPP agenda.

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<sup>1</sup> For background on QIPP: The NHS quality, innovation, productivity and prevention challenge: an introduction for clinicians (March 2010), available from here

<sup>2</sup> See NHS Improvement's QIPP site for more background

## **Blood and marrow transplantation**

6.6 Among the most important points to emerge, attention is drawn to:

- The need to develop a consensus as to what constitutes 'robust evidence';
- The possibility of developing a national board/expanding the use of the BSBMT adjudication Panel to reduce inconsistencies in commissioning and clinical practice;
- The potential for using the patient survey developed by Lymphoma Association, Myeloma UK and Leukaemia care to develop relevant patient outcome measures and ensure that the service is patient centred;
- The need to develop designation for Hematopoietic stem cell transplantation (HSCT) to improve quality and efficiency;
- The potential for using CQUINs to improve data collection where necessary.

## **Cardiology**

6.7 Among the most important points to emerge, attention is drawn to:

- The advantages of a directory of services to assist accurate, timely referral;
- The need to keep central funding to support networks as a key pillar of integrated care;
- The potential benefits of collaborative benchmarking between tertiary centres to ensure that patients are tested, treated and set on the road to recovery expeditiously;
- The importance of improved procurement alongside better understanding of outcomes and value, maybe through a device register;
- Scope for better monitoring and management of outcomes, including greater use of CQUINs and PROMs, subject to improved patient involvement and risk adjustment of results.

# Matching provision to need

## **Michaela's son Nathan was born at 24 weeks gestation and required specialised care.**

Nathan was born on 1 May 2009 at 24 weeks gestation, weighing just 620g. Michaela's waters broke after complications with her pregnancy and immediately went with her partner to their nearest tertiary hospital. However there were no intensive care cots available leading to her being transferred by ambulance to a hospital more than 50 miles away where she gave birth.

Over the first few days Nathan struggled to survive. Slowly though, he got better and after two and a half weeks Michaela was finally allowed to hold him for just 10 minutes. However, at around three weeks old Nathan suffered a shocking incident which Michaela believed was due to a shortage of nurses: Michaela went into the ward to find his oxygen saturation was dangerously low. Nathan's own nurse had not noticed as she had been assisting another nurse care for a baby three cots away. Michaela immediately called over a nurse to increase his oxygen but it is impossible to tell yet whether Nathan has suffered long-term brain damage from this incident.

Nathan was eventually transferred to Great Ormond Street for surgery before finally being transported back to their local hospital. Over time things slowly improved until, nearly five months later, Nathan was finally allowed home. Nathan is now a happy one year old doing well.

## **Children's services**

6.8 Definition 23 of the NDS on specialised children's services is unusually broad in scope. The workshop and report therefore focused on neonatal care and the first two years of life, which account for about half of total expenditure. Among the most important points to emerge, attention is drawn to:

- Better matching of provision to need across the various levels of neonatal care;
- The importance of more effective team working;
- Greater empowerment of parents, including support for parental care at home;
- An openness to new models of care, such as the ambulatory care model pioneered in East & North Hertfordshire.

## **Haemophilia**

6.9 Along with neurosciences, the haemophilia workshop precipitated a series of firmer recommendations as follows:

- 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.

## **HIV**

6.10 Among the most important points to emerge, attention is drawn to:

- The importance of involving individual patients in discussion at an early stage about potential efficiency savings, for example in relation to home delivery of drugs or the use of generic medicines;
- The need to look at clinical and patient outcomes in tandem and to use CQUIN as a means of securing delivery;
- The potential opportunity arising from GP commissioning to involve GPs more actively in non-specialised care for people with HIV and to normalise important aspects of prevention, notably testing;
- The continuing need for improved IT to support all aspects of care.

## **Neurosciences**

6.11 Along with haemophilia, the neurosciences workshop precipitated a series of firmer recommendations, as follows:

- The contribution patients can make in helping inform diagnosis and subsequent decisions about treatment and care should be recognised and enlisted;
- Referral direct to an expert centre needs to be available for appropriate patients to speed diagnosis and spare patients from prolonged limbo;
- Personal care plans should be universally introduced as an essential requirement of integrated care, supported by a nominated keyworker;

- Multidisciplinary neuroscience networks should be developed across the country to shape services and ensure proper integration within healthcare and between health and social care;
- The hub and spokes model has the potential to expedite key decisions about diagnosis and care, consistent with most delivery at a local level;
- Registries should be more widely used to benchmark care and support research.

## Renal services

6.12 Among the most important points to emerge, attention is drawn to:

- The importance of better identification of chronic kidney disease (CKD) in primary care given the level of late presentations;
- The need to clarify the relationship between GP consortia and specialist providers of renal services once the White Paper changes are implemented;
- The wide variations nationally in home dialysis, and need to provide better support to home dialysis patients;
- The implication of the inclusion of renal services within the national tariff from April 2011 onwards.

## The value of home care

### William Withers struggled to get a dialysis regime that suited him

"I received dialysis at a satellite unit for more than four years. Satellite dialysis seemed to be my only option as I was unaware of home dialysis. I did, however, speak with staff about becoming self caring at the unit and they were supportive. A year of self care convinced me that I wanted to dialyse at home, but no training was available because of staff shortages and there was no access to my method of choice, nocturnal dialysis.

Out of frustration, I wrote to my MP, who took the matter up with the SHA and PCT. Although the reply months later bore little relationship to my situation, it did seem to initiate some change at the hospital and I was finally offered training. This was only available three times per week and not after work hours or at home. My partner, who also works full time, had to attend, with each session taking eight hours including the dialysis and a round trip of about 70 miles.

When I was eventually allowed a home machine the hospital was unable to support nocturnal dialysis and daily treatments left me exhausted. In the end I decided to do nocturnal dialysis without the help of my hospital and sought advice from overseas specialists. After 18 months, I am now very happy with much improved health compared to my old regime."

## Spinal cord injuries

6.13 Among the most important points to emerge, attention is drawn to:

- The need to ensure effective communication, both between a referring trust and a SCIC and, more generally, between the various individuals and organisations providing care to a person with a SCI;
- The importance of exploring the possibility of greater involvement of GPs and other primary and community healthcare staff and of various initiatives, such as telerehabilitation and SCIC satellite clinics, in providing effective life-long care and treatment to patients;
- The potential of PROMs to capture vital data on what is most important to patients and the quality of the care provided to them.

## Genetic testing

6.14 Among the most important points to emerge from the UKGTN conference<sup>3</sup>, attention is drawn to:

- The need to ensure that clinicians who are non-genetic specialists order the correct tests for the right reasons. UKGTN testing criteria can provide important guidance to help clinicians order the most appropriate tests and provide commissioners with evidence that the tests are beneficial;
- The increasing role of genetic testing where a condition is strongly suspected, to avoid invasive testing or hospital admissions;
- The importance of developing collaborative systems to support the interpretation of data to connect observed mutations to physical expressions of those mutations;
- The need for genetic laboratories to scale-up or consolidate resources to provide a service which will meet growing demand in the NHS and to compete with private companies;
- The need to increase awareness and knowledge of genetic testing in the medical community at large;
- The important preventative role that genetic testing can play in enabling patients to make the best choices about their long term health and plan for the future.

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<sup>3</sup> The second report of the UKGTN was launched at the conference and is available under 'What's New' on <http://www.ukgtn.nhs.uk/gtn/Home>

## 7. Key Drivers For Improved Specialised Services

- 7.1 In the wake of the workshops looking at specific specialised services, the Alliance ran a national conference on delivering quality in specialised care on 16<sup>th</sup> November 2010. This included a series of presentations looking at quality from different perspectives as a prelude to audience discussion, which helped to crystallise a number of key drivers relevant to the delivery of cost-effective quality. These are summarised below.

### **Diagnosis**

- 7.2 Establishing an accurate diagnosis often proves a cause of lengthy delay where rare conditions are concerned, adversely affecting quality and sometimes length of life in the process.
- 7.3 The default position is that GPs should be better trained to identify rare conditions but this seems likely to prove fruitless when few, if any, cases will be seen by an individual practitioner during their career. The Alliance therefore favours a change in the doctor/patient relationship, which is more focused on referral pathways than diagnosis per se and more open to the help patients with rare conditions may be able to provide in discussing their symptoms, predicated on well signposted sources of reliable information. Such an approach is also consistent with "nothing about me without me" and maximising the potential for subsequent self-care.

### **Networks**

- 7.4 There is a natural tension in specialised care between the need to share clinical expertise and financial risk across bigger populations and the desirability of delivering the majority of care at a local level, including the home.

# The vital role of GPs in the referral pathway

## Norman Starling's late wife Molly had Motor Neurone Disease.

Molly had fallen over for no discernible reason and lost control of her left foot. Although Norman had questioned whether there might be a neurological cause such as MS, the GP had referred Molly to a cardiologist. Having been sent back to the GP, Molly had been referred to a podiatrist and then on to a rheumatologist. The rheumatologist ordered an MRI scan prior to her being seen by a spinal surgeon, who referred her to a neurologist, where a diagnosis of Motor Neurone Disease was made – a year after the onset of symptoms and only three months before Molly's death.

Following her diagnosis, Molly did not see another doctor until she lost the ability to swallow. Meanwhile, social services had assessed her position only over the phone and shown very little understanding.

Norman questioned whether Molly's GP had shied away from making a diagnosis because of its gravity. Above all, he regretted that they had not made more of her remaining months of quality life because of the late diagnosis.

- 7.5 The Alliance supports the development of networks as a crucial element in reconciling these demands. Such networks have already proved successful in fields such as cancer and cardiovascular disease and need to be replicated in specialised areas of sufficient size, such as neuroscience. For the majority of specialised services, generic networks should be established. These would bring together all the parties necessary to apply the principles of integrated care and would have the ability to intervene constructively where specific problems emerge.

### Quality standards

- 7.6 NICE quality standards are set to play an important role in underpinning the outcomes framework being introduced by the coalition government. As with the framework itself, however, there will be limited scope for detailed coverage of specialised services with the risk that what is not covered is overlooked. The Alliance therefore recommends that NICE should develop some generic standards for specialised care (eg making care plans and nominated keyworkers universally available) alongside specific exemplar services. The majority of specialised commissioning should be covered by a library of standards developed under the auspices of the NHSCB as a corollary of designation.

## **Registries**

7.7 The QIPP project has shown that registries are a keystone of good care, helping to ensure that patient populations are accurately identified, underpinning clinical research and supporting the dissemination of results. The existence of disease registries is, however, patchy at best with the risk that funding constraints will make matters worse. This would be a false economy and set back the cause of better specialised care. The NHSCB should ensure that registries are set up across the National Definitions Set with data entry a requirement for designated providers. This should be matched by a rigorous approach to the scope of data being collected, focusing on those things which will deliver most value, which will in turn contain costs.

## **Patient organisations**

7.8 People with rare conditions are especially vulnerable because of the isolation which rarity can bring. Patient organisations therefore have a vital role to play in lending support to patients and their families and providing a knowledgeable interface with commissioners and providers. As well as helping to drive up standards, good patient organisations will help in service development and sometimes in service delivery and have a crucial role to play in supporting integrated care, which needs to be recognised and respected. It follows that clinicians should be encouraged to provide information about relevant patient support at the time of diagnosis.

## **Unitary outcomes**

7.9 There is a tendency to look at clinical and patient outcomes separately and to consider related initiatives such as CQUINs in similar fashion. The best outcomes will, however, be mutually reinforcing. Patient experience also has a vital part to play in delivering successful outcomes. For example, it is increasingly recognised that poor hospital food makes patients miserable but also impedes their recovery and drives up costs. Every effort should therefore be made to look at outcomes in a holistic way which brings the clinical, patient and financial perspectives together.

## 8. Recommendations

- 8.1 The overarching recommendation of this report is that the NHSCB should build on the legacy of Carter by ensuring implementation of the main principles with any necessary adjustments for changed circumstances. For the most part, these principles also have application in the other home countries and the Specialised Healthcare Alliance would commend them to the devolved administrations accordingly. We would also like to see this consideration extended to research throughout the UK, given its vital importance to people with rare conditions and the future development of the life sciences sector.
- 8.2 In taking this agenda forward, the Alliance will be paying particular attention to what it sees as the main drivers in delivering high quality care and specifically:
- The need for the doctor/patient relationship to evolve in a way which speeds diagnosis through better referral pathways and the ability of many patients to help inform diagnosis, predicated on well signposted sources of reliable information;
  - The role of patient organisations in protecting the vulnerable and driving up standards, sometimes through the provision of more innovative services;
  - The need to ensure integrated pathways, especially through the development of multidisciplinary networks;
  - The contribution of NICE quality standards or their equivalent to high quality specialised care and outcomes;
  - The desirability of outcome measures which capture both clinical and patient perspectives with a view to maximising effectiveness and efficiency;
  - The central importance of patient registries, with more sharply focused datasets helping to keep down costs.

## 9. Appendix

### Membership of the Specialised Healthcare Alliance (as at January 2011)

#### Patient groups

AKU Society

AMEND (the Association for Multiple Endocrine Neoplasia Disorders)

Anthony Nolan

Arthritis and Musculoskeletal Alliance (ARMA)

Arthritis Care

ASBAH (Association for Spina Bifida and Hydrocephalus)

BAPEN (British Association for Parenteral & Enteral Nutrition)

Behcet's Syndrome Society

Bliss (premature baby charity)

Bowel Cancer UK

British Liver Trust

British Paediatric Rheumatology Group

British Polio Fellowship

British Society for Rheumatology

Cancer of Unknown Primary

Child's Liver Disease Foundation

Children Living with Inherited Metabolic Disorders (CLIMB)

Children's Heart Federation

(The) Children's Trust, Tadworth

CLIC Sargent

Contact a Family

Cystic Fibrosis Trust

Ehlers Danlos Support Group

For Dementia

GBSSG (Guillain-Barre Syndrome)

Grown Up Congenital Heart Patients' Association

Haemophilia Society

Hepatitis B Foundation UK

Hepatitis C Trust

Ichthyosis Support Group

International Brain Tumour Alliance

Kidney Alliance

Limbless Association  
Lymphoma Association  
Macmillan Cancer Support  
Motor Neurone Disease Association  
MS Society  
MS Trust  
Muscular Dystrophy Campaign  
Myeloma UK  
National AIDS Trust  
National Brain Injury Centre, St Andrew's Healthcare  
National Kidney Federation  
National Voices  
Neurofibromatosis Association  
National Rheumatoid Arthritis Society  
Niemann Pick Association  
Parkinson's UK  
PINNT (Patient on Intravenous and Naso Gastric Nutrition Therapy)  
Primary Immunodeficiency Association  
Progressive Supranuclear Palsy Association  
Pulmonary Hypertension Association  
Rarer Cancers Foundation  
Sarcoma Trust  
Scleroderma Society  
Sickle Cell Society  
Sign (mental health and deafness)  
Speakability  
Spinal Injuries Association  
Teenage Cancer Trust  
Terrence Higgins Trust

## **Corporate**

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# Specialised Healthcare Alliance

FOR EVERYONE WITH RARE AND COMPLEX CONDITIONS

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