

## Engagement Report for Service Specifications

<b>Unique Reference Number</b>	1640
<b>Service Specification title</b>	<b>Cystinosis Service Specification</b>
<b>Lead Commissioner</b>	Sarah Watson
<b>Clinical Reference Group</b>	Specialised Renal Services CRG Paediatric Medicine CRG
Which stakeholders were contacted to be involved in specification development?	All registered stakeholders of the Specialised Renal services and Paediatric Medicine CRGs were contacted plus CRG members. UK Renal Association's Cystinosis Rare Disease Study Group and the Cystinosis Foundation UK.
Identify the relevant Royal College or Professional Society to the policy and indicate how they have been involved	British Renal Society Royal College of Physicians Renal Association The Royal College of Paediatrics and Child Health Renal Pharmacy Group
Which stakeholders have actually been involved?	University Hospitals Bristol NHS FT, Renal Pharmacy Group, Department of Paediatric Nephrology at Leeds Teaching Hospital, Nottingham Children's Hospital, Great Ormond Street, Renal services CRG. There were also responses to the consultation from eight individuals. Cystinosis UK has been involved in the specification working group.
Explain reason if there is any difference from previous question	Not all stakeholders responded to the stakeholder testing
Identify any particular stakeholder	None

<p>organisations that may be key to the policy development that you have approached that have yet to be engaged. Indicate why?</p>	
<p>How have stakeholders been involved? What engagement methods have been used?</p>	<p>The draft service specification was sent out to stakeholders via email. Stakeholders were asked to complete a response form within two weeks. A reminder email was sent out after one week.</p>
<p>What has happened or changed as a result of their input?</p>	<p>Some changes made to the service specification to clarify particular points including:  Genetic testing, with subsequent updates to clarify that testing will be provided in line with the genomics procurement and providers set out in the National Genomic Testing Directory  The role of the HSS service with local services working together to bring expertise in an appropriate way to the patient's management was highlighted as a significant consideration. This is an issue common to many HSS that needs to be handled well and dependant on the needs of patients and families. This was an issue of concern that there is balance reached between patients travelling for expertise and the expert centres working with local hubs to ensure local management of patients whenever possible.</p>
<p>How are stakeholders being kept informed of progress with policy development as a result of their input?</p>	<p>Stakeholders will be sent a copy of the specification at public consultation.  A Cystinosis Patient Study Day was held in January 2019 and patients were updated on the proposal.</p>
<p>What level of wider public consultation is recommended by the CRG for the NPOC Board to agree as a result of stakeholder involvement?</p>	<p>30 days in line with standard public consultation processes</p>

Organisation Responding	Feedback Received	PWG response
1. <b>Individual</b>	<p>The respondent supported the proposals in the service specification.</p> <p>They provided the below comment:</p> <p>This pathway seems complete, but very aspirational, and will need independent assessment throughout.</p> <p>No conflicts of interest were declared.</p>	<p>Noted</p> <p>Comprehensive outcomes as per the specification will be reported into RaDaR, national Cystinosis registry.</p>
2. Individual	<p>The respondent supported the proposals within the service specification and no further comments were given.</p>	<p>Noted</p>
3. <b>Individual</b>	<p>The respondent supported the proposals within the service specification and provided the below comments:</p> <p>As an early sense check, it might be useful to update the paragraph on Page 4 that is headlined “Links to an accredited Laboratory...”. My understanding is that since April 2018 the UK Genetic Testing Network is not responsible for accrediting Laboratories anymore, as they transitioned from CPA to ISO 15189:2012, and that each laboratory is now registered with UKAS at <a href="http://www.ukas.com">www.ukas.com</a>. It might therefore be helpful to change the link in the guidance.</p> <p>Is it cysteine or cystine measurement?</p> <p>The respondent didn't declare any conflicts of interest.</p>	<p>Service specification updated to reflect genetic testing associated with this service should be undertaken in line with the NHS England National Genomic Testing Directory for rare and inherited disease</p> <p>An error now corrected</p>
4. <b>Individual</b>	<p>The respondent didn't support the proposals within the service specification and provided the below comments:</p>	

	<p>It is unclear how this level of activity described would be sufficient to allow funding to provide the level of resources and services identified in the specification. How to identify these patients as a chargeable cohort in such a way as to commission this service and flow the activity at the relevant providers is also unclear. If you can't identify the patients how do you check on service provision and quality? ( renal registry data is only available as annual reports or specific questions not at the level required for commissioners)</p> <p>Is the expectation that activity at the shared care providers be included within the specification and paid for under specialised or would this only be at hubs.</p> <p>Is there a commissioning / procurement plan for the hubs?</p> <p>This sounds like a highly specialised service due to small numbers and expertise required.</p> <p>No conflicts of interest were declared.</p>	<p>A process for coding and counting of patients would be agreed which would be in line with other HSS. Impact Assessment work to identify current and future costs is being completed.</p> <p>Outcomes reporting would be agreed in line with the Quality Indicators as it is with other HSS. In addition we would expect an annual national meeting to take place which would include presentation of service outcomes and difficult cases</p> <p>In addition patient data will be submitted to RaDaR</p> <p>Local renal services would continue to provide day to day management of patients, this is not included in the service. Paediatric nephrology would be specialised (but not HSS)</p> <p>This will be written and agreed as part of the proposal to be taken forward to CPAG.</p> <p>Noted</p>
--	--	---

<p><b>5. Renal services CRG Chair</b></p>	<p>The respondent supported the service specification and didn't provide any further comments.</p> <p>No conflicts of interest were declared.</p>	<p>Noted</p>
<p><b>6. Individual</b></p>	<p>The respondent provided the below comments:</p> <p>This is a VERY big issue for a non-primary treatment centre. In the current state of affairs the funding of drugs can be difficult. In order to access NHSE funding for these drugs we will need to be commissioned as a specialist centre or have a mechanism in place for the funding to be charged onto the Primary treatment centre (they often refuse this). As a hub centre we will probably refuse to supply as we will not get reimbursed from NHSE for the medications (the primary treatment centres will have access to this funding)</p> <p>1) Needs to be organised as a shared care system with the primary centres and the local CCG to the patient</p> <p>2) A home delivery system provided by the primary treatment centre</p> <p>NOTE the supplies for 3 to 4 months could depending on drug take up considerable volume. Parents often have issues getting these homes. Home delivery would be needed.</p>	<p>There is a proposal with the NICE CPS programme for funding of eye drops. If agreed, we would expect prescribing to be from HSS centres. Similarly, other high cost drug prescribing would be as agreed and managed by the HSS centre. This would be according to agreed NHS England policy and ensure appropriate dispensing.</p> <p>Agreed</p>
<p><b>7. Great Ormond Street Hospital</b></p>	<p>The respondent supported the proposals within the service specification and provided the below comments:</p> <p>The specialist renal tubular disorders team at Great Ormond Street Hospital welcome this important proposal which addresses key needs for patients with this rare disease.</p> <p>We fully agree with the importance of ensuring a coordinated multi-professional service highlighted in section 2.2 of the service specification and recognise that this will require significant</p>	<p>Noted and agreed</p>

	<p>administrative and clinical coordination. We would hope that hub services will be adequately resourced to achieve this.</p> <p>We agree that all patients should have access to clinical research studies via specialist hubs, in particular anticipating the development of gene and stem cell therapies. We support the focus on seamless transition to adult care with specialist nurse and youthwork involvement and again hope that the service will be adequately resourced for this.</p> <p>In summary we support the proposed service specification to improve patient centred care for patients with this rare disorder.</p> <p>The respondent declared the below conflict of interests:</p> <p>William Van't Hoff sat on the committee which developed the service specification.</p>	
<p><b>8. Nottingham Children's Hospital</b></p>	<p>The respondent supported the service specification and provided the below comment:</p> <p>We assume that patient and parental views have been sought as well as medical teams? Happy with proposal.</p> <p>No conflicts of interest were declared.</p>	<p>Service specification has been discussed at the Cystinosis patient day.</p> <p>Cystinosis UK has been included in the specification working group and have been involved in the specification drafting.</p>
<p><b>9. Individual</b></p>	<p>The respondent supported the proposals within the service specification and provided the below comments: High cost drugs such as mercaptamine eye drops are considered in the cost of the service or there should be a blueteq form for this.</p>	<p>Use of high cost drugs excluded from tariffs would be according to NHS policy and would usually be supported by Blueteq.</p>

	<p>Clarification regarding the level of pharmacy services required to be provided as it is considered essential for overseeing access to specialised therapies and pharmacists experienced in cystinosis and its treatment can address cysteamine associated problems.</p> <p>No conflicts of interest were declared.</p>	<p>Pharmacy services to overseeing access to specialised therapies has been highlighted as an essential service.</p>
<p><b>10. Department of Paediatric Nephrology, Leeds teaching hospital</b></p>	<p>The respondent supported the proposals within the service specification and provided the below comments:</p> <p>Cystinosis is a life-limiting rare disease. Clinician exposure to managing this condition varies significantly across the UK and it is particularly challenging for young adults with cystinosis to transition to adult care particularly. Transition occurs at a time when they are more liable to develop complex extra-renal manifestations. A Hub and spoke model of care would allow local centres to still be the main provider of care to support children, young people and adults with cystinosis but allow patients of all ages to access specialist services for all aspects of their renal and extra-renal disease. It would also facilitate collection of uniform data and help improve standardisation of care. The location of the Hubs needs to take in consideration travel distance for patients as well as services provided.</p> <p>The respondent declared the below conflict of interests:</p> <p>Our department would be keen to be considered as a future Paediatric Hub for a designated cystinosis service.</p>	<p>The requirement for written, individualised, transition plan pathways to be used has been included in the service requirements. No changes to the specification agreed.</p> <p>Note the development of the transition pathway annex for this to be included in the specification if agreed.</p>
<p><b>11. Individual</b></p>	<p>The respondent supported the proposals within the policy statement and provided the below comments:</p>	

	<p>This has been discussed between the adult &amp; paediatric nephrology services here.</p> <p>We think this model is a good concept, and we look forward to supporting it. As a large trust that includes a regional paediatric nephrology service and a large adult renal service and a paediatric &amp; adult renal transplant service, with good psychology support for each, and a well-established paediatric-adult nephrology transition programme (which includes a non-clinical young adult work), and good transport links to the nearby M1, A1, A50 and A46, then I think Nottingham University Hospitals would be well placed to deliver as one of the hubs being described.</p> <p>We look forward to hearing more in due course.</p> <p>No conflicts of interest were declared.</p>	<p>Noted. The specification notes that patients with cystinosis frequently have a variety of psychological problems related to their disease and its complications and includes the requirement that providers will ensure that patients have access to appropriate psychology services and that the initial assessment will be by professional who have knowledge of cystinosis and its complications.</p> <p>No changes made.</p>
<p><b>12. Individual</b></p>	<p>The respondent supported the proposals within the service specification and provided the below comments:</p> <p>As a paediatric nephrology team we see a small number of children with cystinosis. We acknowledge that they require highly specialised, multi-speciality care. We support the idea of a co-ordinated national service to provide this care in hubs, whilst ongoing care is provided by us locally. Given the complex needs of these patients we think it is important that our patients in the North-East and North Cumbria have a choice to travel to a hub that is a manageable geographical distance away (less than 3 hours) and would support a hub in the northern region, e.g. Leeds.</p> <p>Manchester, Birmingham and London are all more than 3 hours travel distance away for our patients.</p> <p>No conflicts of interests were declared.</p>	<p>Noted. The issue of access will need to be carefully considered in and provider selection. It is also the case that where patients live at a distance from the HSS Cystinosis centre that local and HSS centres will need to work together to ensure patients access expert advice but travel to expert centres is minimised according to need and patient benefit.</p>



<p><b>13. Renal Pharmacy Group</b></p>	<p>The respondent supported the proposals within the service specification and provided the below comments:</p> <p>As this will be tertiary referral it should mention delivery of medicines which may include home delivery and the reimbursement of costs associated with this</p> <p>I do not know whether the service spec would discuss which medicines should be used but should it mention those with the lowest acquisition cost?</p> <p>The RPG should be listed as a <b>Professional body with an interest and national guidance</b></p> <p>There should also be a renal pharmacist with expertise involved in the care of adult patients as well as children.</p> <p>No conflicts of interest were declared.</p>	<p>The service specification is not changing this and not changing existing funding arrangements.</p> <p>The RPG (renal pharmacy group) added.</p> <p>Listed in specification as an essential requirement for both adults and children.</p>
<p>14. University Hospitals Bristol NHS Foundation Trust</p>	<p>The respondent supported the proposals within the service specification and provided the below comments:</p> <p>My comments relate mainly to the care of children and their families. The proposal to develop and support centres of excellence to manage this very challenging condition is welcome. With improved survival into adulthood configuring services to meet the needs of adults with cystinosis and ensuring good transition from paediatric services is particularly important. There are potential benefits of improved quality of care arising from using expert guidelines with a more structured approach and early adoption of innovative treatments. The existing service to measure white cell cystine levels is in need of improvement and some co-ordination of when samples are collected and processed may facilitate more timely reporting of results.</p>	<p>Agreed, no changes made to the specification, transition needs are included.</p> <p>Noted but no changes to the specification made</p>

	<p>We recognise that children with cystinosis require very intensive support from the multi-professional clinical teams based in the regional paediatric nephrology centres especially in infancy and adolescence. Good personal relationships and communication between the family and the professionals is needed to deliver this care successfully and should be based on a philosophy of care as close to home as possible. We would be concerned about any model that disrupts communication so in any new model there must be close working with the professional taking the lead for the care of the patient locally.</p> <p>We feel that families should be offered an opportunity to attend an annual review and funding made available to meet the travel costs which for families with children may be considerable but this attendance should not be compulsory. We do not feel that visits more than annually would be helpful. Our experience of working in a widely dispersed geographical region suggests that a single centre model may be less accessible to some disadvantaged families for example children living a long way from the supra-regional hub, single parent families, families where parents have health issues etc.</p> <p>From our experience of clinical research projects we know that children may find multiple assessments in a relatively short time with professionals with whom they are not familiar very challenging so this model may not be so successful for children. It might be more effective for assessments to be done locally with results submitted in advance for the annual review.</p> <p>Given the input needed to support children with cystinosis we do not anticipate that the development of a supra-regional hub will reduce the costs of the care delivered in the existing model but will require additional investment. We wonder whether the role of teleconferencing to deliver some aspects of this service has been considered to improve access to clinical expertise and ensure good communication?</p>	<p>Role of CNS in co-ordination to minimise visits to what is really needed. Note there may be more frequent visits for eyes.</p> <p>Working collaboratively but intention of the model is that there is central expertise offered for breathing and swallowing. Expect close collaboration and agreement centre and local services.</p> <p>Noted</p> <p>The way local and HSS services work together will need to be developed. Further discussions to be held with SWG paediatric lead and paediatric medicine CRG.</p>
--	---	---

	<p>Quality indicators 301 – Visit should be offered 303 – Results to be communicated to patients and local clinician 203 - Patient support group – safeguarding to be addressed if including children</p> <p>We are in favour of collaborative working to reduce variation, improve patient care and support the development of expertise in managing patients with cystinosis.</p> <p>The respondent declared the below conflict of interests: I am a paediatric nephrologist caring for children with cystinosis.</p>	<p>Agreed and also to GP No changes to spec but agreed for future discussion as services develop,</p>
--	---	---