

**CLINICAL PRIORITIES ADVISORY GROUP  
XX XX XX 2016**

<b>Agenda Item No</b>	
<b>National Programme</b>	Internal Medicine
<b>Clinical Reference Group</b>	Renal Services
<b>URN</b>	1640

**Title Insert service specification title in full**

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<b>Actions Requested</b>	1. Agree the service specification proposition
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**Proposition**

Cystinosis is a very rare inherited lysosomal storage disease presenting in infancy (95% of cases) with severe kidney disease but subsequently involving multiple organs (eyes, thyroid, liver, diabetes, muscle deterioration). It occurs when the mechanism removing excess cystine, an amino acid, breaks down. It then accumulates within body cells preventing these cells from functioning correctly. Patients often present to paediatric nephrology services although multi organ disease requires multi-speciality input. Effective drug therapy and renal transplantation now allows survival into the fifth decade.

There is currently no dedicated specialist service for Cystinosis in England. Local care services focussed in paediatric nephrology units. Current care is fragmented with major geographical variability in care quality. Early initiation of cysteamine therapy can significantly postpone, or even prevent complications of the disease. Poorly coordinated care results in patients attending multiple clinics on different days at different sites. Since the use of cysteamine and the introduction of renal replacement therapy in paediatric populations, patients with cystinosis are increasingly surviving into adulthood. However, there are few transition services from childhood to adult clinics and limited adult services. Clinical inexperience compromises care especially within adult services. Better recognition and treatment of late-term complications in adulthood will improve long-term outcomes in patients.

This proposal for the development of services to provide an integrated multi-disciplinary one stop service for both paediatric and adult patients to ensure consistent and quality service is delivered. The specification describes a hub and spoke model of care.

Given the known patient population it is suggested 3 adult and 3 paediatric hubs be commissioned. 3 geographically opportune services in England is likely to be best in terms of patient travel time and access to multidisciplinary resources.

**The committee is asked to receive the following assurance:**

1.	The Head of Clinical Effectiveness confirms the proposal has completed the appropriate sequence of governance steps and includes where necessary an: Evidence Review; Clinical Panel Report
2.	The Head of Acute Programmes / Head of Mental Health Programme confirms the proposal is supported by an: Impact Assessment; Stakeholder Engagement Report; Consultation Report; Equality Impact and Assessment Report; Service specification Proposition. The relevant National Programme of Care has approved these reports.
3.	The Director of Finance (Specialised Commissioning) confirms that the impact assessment has reasonably estimated a) the incremental cost and b) the budget impact of the proposal.
4.	The Clinical Programmes Director (Specialised Commissioning) confirms that the service and operational impacts have been completed.
5.	The Director of Nursing (Specialised Commissioning) confirms that the proposed quality indicators have been adequately defined.

**The following documents are included (others available on request):**

1.	Service specification proposition
2.	Consultation Report
3.	Evidence Summary (where completed)
4.	Clinical Panel Report (where completed)
5.	Equality Impact and Assessment Report

No	Metric	Summary of benefit (where applicable)
1.	Survival	Where an evidence review has been completed, please include metric of survival (e.g., 30 days benefit, 50 years benefit)
2.	Progression free survival	

3.	Mobility	
4.	Self-care	
5.	Usual activities	
6.	Pain	
7.	Anxiety / Depression	
8.	Replacement of more toxic treatment	
9.	Dependency on care giver / supporting independence	
10.	Safety	
11.	Delivery of intervention	

No	Metric	Summary from evidence review
		[AS ABOVE]

### Considerations from review by Rare Disease Advisory Group

**To be completed**

### Pharmaceutical considerations

The high cost drug related to the management of Cystinosis is Mercaptamine Hydrochloride which is subject to a separate commissioning policy proposition (1832 Mercaptamine hydrochloride for corneal cystine deposits in people aged older than 2 years).

### Considerations from review by National Programme of Care

#### POC Board support

Select appropriate option:

1) The proposal received the full support of the <insert PoC name> Board on the <insert date>

- 2) The proposal received the support of the <insert PoC name> PoC Board on the <insert date>, subject to the following comments <insert comments>
- 3) The proposal received the support of the <insert PoC name> PoC Board on the <insert date> but CPAG is asked to note that the proposal did not have the full support of the Working Group, who have raised the following concerns: <insert reasons>
- 4) Other – free text (only for minority of cases not fitting into the above)

**Benefit of Service Specification:**

The proposed service model will benefit this patient group by:

1. Improve diagnosis of patients
2. Early initiation of cysteamine therapy can significantly postpone, or even prevent complications of the disease.
3. Better recognition and treatment of late-term complications in adulthood will improve long-term outcomes in patients.
4. Supporting transition from paediatric services to adult services

**Implementation timescale:**

3) Service reconfiguration / procurement required. Expected implementation date January 2021.

