

## Expert testimony to inform NICE guideline development

Section A: Developer to complete	
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<b>Role:</b>	Expert testimony
<b>Institution/Organisation (where applicable):</b>	N/A
<b>Contact information:</b>	
<b>Guideline title:</b>	Renal and ureteric stones: assessment and management
<b>Guideline Committee:</b>	Guideline committee 10: April 13 <sup>th</sup> 2018
<b>Subject of expert testimony:</b>	Carers perspective on the management of children with renal and ureteric stones
<b>Evidence gaps or uncertainties:</b>	We have searched for evidence on the diagnosis and management of children and young people with renal and ureteric stones. We have found very little evidence that meets the criteria for any of our review questions. We would therefore like you to speak from your own experience as a parent of children with recurring stones.
<p>The Department of Health in England has asked NICE to develop a guideline on the assessment and management of renal and ureteric stones. This guideline will provide recommendations on the diagnosis, pharmacological and surgical management and follow-up in children and young people with symptomatic and asymptomatic renal and ureteric stones.</p> <p>Lay members are recruited onto the committee to provide a patient's perspective of treatment and care. One of the lay members providing a paediatric perspective has had to step down from the committee and we are now lacking representation in this area of the guideline. The committee are aware that is a lack of research evidence on children, and this was felt to be a gap in the evidence to not adequately represent the experiences of children and young people.</p> <p>Where the research evidence is lacking, or inconclusive, Guideline Committee members can invite expert witnesses to the group to provide their and knowledge and experience, and recommendations can be drawn from this expert witness and Guideline Committee consensus.</p> <p>The following questions have been formally reviewed by analysing the available evidence:</p> <ul style="list-style-type: none"> <li>• What is the clinical and cost effectiveness of performing imaging in patients with suspected renal and ureteric stones?</li> <li>• In people who have or have had renal or ureteric stone, what is the optimum frequency of imaging?</li> <li>• Is medical expulsive therapy clinically and cost-effective in managing people with ureteric stones?</li> </ul>	

- What is the clinical and cost-effectiveness of drugs in managing acute pain in people with symptomatic renal or ureteric stones?
- What is the most clinically and cost-effective length of time to manage people with symptomatic or asymptomatic renal or ureteric stones conservatively before active intervention (early vs delayed intervention)?
- What are the most clinically and cost-effective surgical treatment options for people with renal or ureteric stones?
- Is inserting a stent clinically and cost-effective before surgical treatment in people with renal or ureteric stones?
- Is inserting a stent clinically and cost-effective after surgical treatment in people with renal or ureteric stones?
- In people with renal or ureteric stones, what is the clinical and cost effectiveness of stone analysis, blood test and urine test compared to no test, when each is followed by the appropriate treatment for renal or ureteric stones in order to improve patient outcomes?
- What is the most clinically and cost-effective non-surgical management for preventing the recurrence of future renal and ureteric stones?
- What is the clinical and cost-effectiveness of dietary interventions to reduce the risk of future stones in people who have had renal stones?

## Section B: Expert to complete

### Summary testimony:

1. What was your experience of any procedures carried out to diagnose the stones? Were any tests or imaging carried out?

In 2012, when my son was 10 years old, we were on holiday in Jersey when he got very ill and started to pass bright red urine. We returned home from holiday right away and went to the GP immediately as my son was very lethargic and unwell. It was a Friday, and the GP's surgery assumed that this was probably just an infection, and advised that they will prescribe antibiotics before he could be referred. No tests had been done at this point. We took the antibiotics as prescribed but this made things worse. My son continued to get increasingly unwell over the weekend and had by this time passed a stone, so I sought a second opinion on Monday at the GP's surgery. On this occasion, a urine sample was taken and the results of this prompted them to send us directly to the hospital, where an X-ray was carried out.

At this point my son couldn't urinate. The x-ray showed a large mass, and so my son was referred to another hospital. After 11 days I was advised that the mass in the x-ray was too large to deal with at their hospital and my son was then referred to Great Ormond Street hospital. At this point he still couldn't urinate and was extremely unwell. I kept giving him drinks and he eventually passed another stone and then another stone seemed to get stuck - this was prior to getting a blue light to Great Ormond Street. Once there, a consultation was carried out, during which a detailed ultrasound and x-ray were done. Following these assessments, consultants requested a DMSA to check kidney function, as the mass was a 5cm staghorn calculus. (The kidney was found to be under 20% function.) Soon after this, a CT scan was also carried out.

2. What treatments your children have received, such as:
  - Drugs for managing pain. How long were these used for before the stones passed or other treatment was offered?

- Drugs to help the stone pass such as an alpha blocker or calcium channel blocker?
- Any surgical procedures performed, and if this was repeated or were different surgical techniques used?

The kidney at the stone site was damaged and surgeons suggested that we consider removing the damaged kidney. We objected to this as we had so little information about what had caused my son's condition and so we asked them to try to save the kidney during the planned procedure. Surgeons attempted a PCNL, however, the stones were too hard in consistency to remove with that procedure, they then sought our permission to carry out open surgery and advised that during the PCNL, 8 additional stones had been spotted in the urinary tract. Permission was granted, the stones were successfully removed, and the kidney saved, during the surgical procedure.

3. Were you given a choice in any treatments offered? Did you feel informed in order to make choices?

My objection to removing the kidney was based entirely on instinct, as there was no information available at this point about the stone composition or the cause of the illness. Post-surgery pain was managed using a morphine drip.

4. Was any stone analysis carried out or other tests performed after the stones were removed? If they were, do you know if the results of these were used to guide or change further treatment?

The retrieved stones were sent to the laboratory. Results came in just as my son was going into surgery to remove the stent that had been put in during open surgery. Stones were found to be composed almost entirely of cystine and doctors suspected that this may be cystinuria. This was the first hint of a diagnosis.

5. What follow-up care has been provided?

- Further scans or imaging.
- Dietary advice to prevent further formation of stones.
- Prescribed supplements to prevent a particular stone type from reforming?

My son was discharged and passed to the clinical team for further follow up. The first follow up appointment confirmed the cystinuria diagnosis. Not much information was available other than online. The pain is managed using paracetamol. I have resisted using any prescribed medication that would see my son using any drug for the rest of his life.

Through personal research, I have endeavoured to self-manage the illness through increased hydration, increasing fresh food, and limiting processed food and meat. I also monitor his Ph. levels in his urine and carry out a 24 hour urine test before every follow up appointment. I have scheduled in two bathroom visits and increased hydration during the night for my son for several years to avoid the kidneys having to deal with concentrated urine. Some of the advice I have received has been quite misleading, for example, the suggestion that lemonade would be helpful, however, this should be clarified to explain that this means fresh lemon juice diluted in water.

6. What has been your experience of follow-up care?

My son was passed from the surgical team to the clinical team, but we had very little access to information about rare stone disease and the formation of stones. Follow up care has been largely supported by my own self-management and monitoring efforts. The advice I received from clinicians was limited to increase hydration and 'eat normally' and suggestions that we use potassium citrate to support his care. We quickly noted, however, his urine Ph. levels were affected by the potassium citrate and also whenever he had eaten meat. A proactive approach to consistent diet has helped in the management of his care.

7. How has the care received impacted on the well-being of your children and their quality of life?

Following his first surgery, my son missed the beginning of the school year/term. This caused him some anxiety about returning to school, and there was a lot of fear around possibly hurting the surgical site. It's now been 6 years since my son's diagnosis and the impact on his lifestyle is undeniable. Diet and hydration are where we focus on preventative care. Additionally, 2 bathroom visits per night means he is unable to have a full night's sleep. There are hydration concerns when attending school and social events, and access to bathrooms and quick exits are always a consideration. He's been granted a pass to access the toilet at all times during classes and will, as a matter of routine carry at least 1.5 litres of water at all times. Because of these high levels of hydration he has experienced bullying at school. This has affected his confidence. He has however, learnt to take good care of himself and to be aware of how his diet affects his well-being.

The impact of this illness has resulted in a change in lifestyle for the whole family, as we have now all seen a geneticist and confirmed that both me and my other son also have cystinuria.