

# AKU Society

Compilation of sample patient costs



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Final

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- This presentation provides no assurance over the range of values explicitly stated as all costing information used was from guidance provided in the public domain and was not derived from patient level costing.
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# Purpose and process

## Background

AKU is an ultra rare genetic disease affecting between one in 250,000 and one in 1m people in the UK. It is due to a missing enzyme leading to the accumulation of a substance called homogentisic acid at 2,000 times the normal rate. This acid binds to cartilage and bone, turning it black and brittle in a process called ochronosis. This causes severe disability as life progresses. There is no licensed treatment. The AKU Society is in touch with 80 patients in the UK from children to adults.

## Purpose

The purpose of this presentation is to document the general findings resulting from a high level investigation of estimated AKU related costs that the NHS currently incurs for a sample of patients living with AKU.

## Process

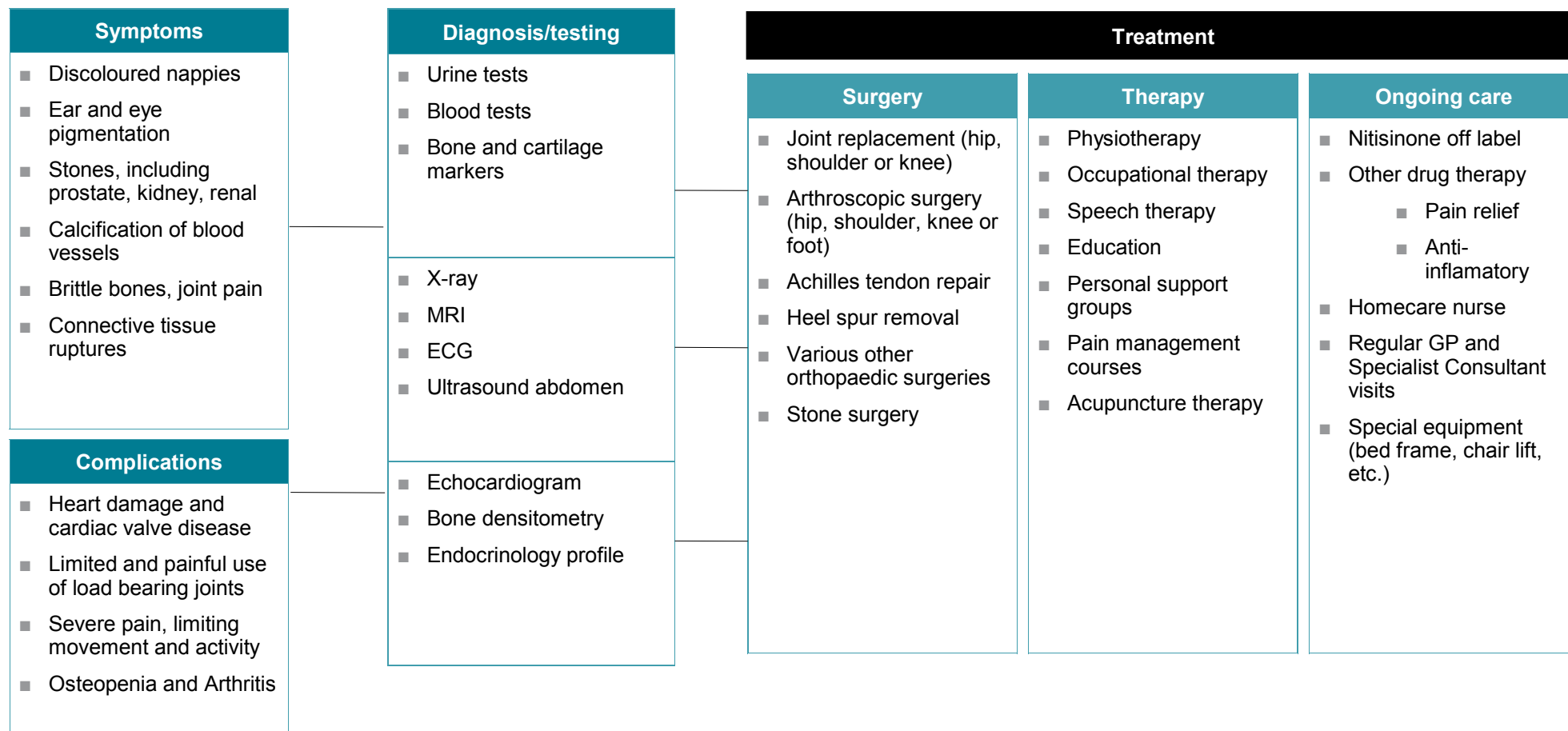
1. Interview non-patient members of the AKU society to gain an understanding of the disease and a potential costs that the NHS is incurring related to AKU patients.
2. Perform external research to gain an understanding over potential health care events, resulting cost triggers and potential sources of cost information.
3. Interview five AKU patients. Gain an understanding of each unique case, tracking all health care evenings which could trigger a cost. Determine which events are specifically AKU related and those that could be readily attributed to other conditions or regular care.
4. Perform research to determine a range of estimated costs for data captured.
5. Review and interpret relevant findings.
6. Confirm findings with those within the AKU Society and any appropriate/ available experts to identify any significant abnormalities within the process, assumptions and findings. Amend process and assumptions where appropriate.

## Key findings

- Individual patient costs vary greatly depending on the stage of the disease they are in and the resulting number of surgeries and patient care required. **Direct costs from one patient for one year can be in excess of £90,000** without taking into account drug therapy and physical aids required to live as normal a life as possible. These **additional costs bring the single possible direct patient total to over £100,000**, and possibly even larger given the limit to which direct costs were captured for each patient.
- Data from this research could be extrapolated to the current known AKU UK population. When extrapolated, calculations show that **yearly direct health care cost attributed to AKU range from £0.2 million to £3.0 million** depending on the number of surgeries patients incur in any one year.
- A **weighted average of all possible scenarios shows that total direct health care cost costs of approximately £1.0 million** may be a more reasonable conservative estimate.
- A conservative approximation of the **total costs of AKU in the UK including indirect costs (i.e. - lost wage and production) is approximately £1.4 million to £2.0 million per year with the upper limit possibly being as high as £7.0 million.**
- According to the AKU Society: ‘These results show that the costs of AKU patients to the NHS are tiny when compared to the overall budget of the NHS of £100bn. However, the costs are high when assessed per individual AKU patient. Providing better treatment, access to a currently unlicensed drug called Nitisinone, and better coordinated care could lead to reductions in costs per patient and significant improvements in their health and wellbeing.’

# General findings

AKU patient costs vary significantly by stage of disease, access to appropriate care, as well as medical professional and patient understanding of the disease.



## High level view of tangible direct patient costs

Band	General Description	Range of costs	Comments
<b>High</b>	<p>Age: Generally &gt;60</p> <p>Significant symptoms: &gt;20 years</p> <p>Major AKU surgeries: &gt;=4</p> <p>No longer employable</p>	<p>2010: £8,000 to £61,000 per patient</p> <p>2009: £8,900 to £94,000 per patient</p> <p><b>Last 5 years: £38,000 to £284,000 per patient</b></p>	<p>significant costs include: Organ failure/ fatigue, organ surgery, multiple joint replacements, multiple tendon ruptures, arthritic conditions, long hospital stays, extensive testing and consultations, home care and long hospital stays. Some patients have experienced 10+ surgeries.</p>
<b>Medium</b>	<p>Age: Generally &gt;50</p> <p>Significant symptoms: &gt;10years</p> <p>Major AKU surgeries: &gt;=3</p> <p>Able to seek some employment</p>	<p>2010: £1,000 to £5,000 per patient</p> <p>2009: £3,500 to £8,900 per patient</p> <p><b>Last 5 years: £5,100 to £18,900 per patient</b></p>	<p>significant costs include: Joint replacements and related on-costs (eg physiotherapy), hospital stays, extensive testing and consultation with a variety of specialists.</p>
<b>Low</b>	<p>Age: Generally &gt;40</p> <p>Significant symptoms: &lt;10 years</p> <p>Major AKU surgeries: &gt;=1</p> <p>Able to seek part time employment</p>	<p>2010: £500 to £1,200 per patient</p> <p>2009: £500 to £1,200 per patient</p> <p><b>Last 5 years: £3,900 to £8,100 per patient</b></p>	<p>significant costs include: Joint surgery, testing, consultations with specialists, and numerous GP visits.</p>

# High level view of intangible patient costs

Band	Direct economic impact	Unquantifiable costs
<p style="text-align: center;"><b>High</b></p>	<p>not able to hold meaningful employment and pay personal taxes.</p> <p>relying on disability benefits for a significant length of time.</p> <p>significant requirement for home care funding (up to two people four times per day).</p>	<p>significant travel costs due to ongoing required attendance at hospitals for testing, therapy and surgery pre-op/ post-op sessions.</p> <p>family costs of related care and taking time off work to care for or transport patient.</p> <p>significant personal impact on patient and family members as disease impacts daily tasks in a significant way.</p> <p>side effects from significant level of drug therapies.</p>
<p style="text-align: center;"><b>Medium</b></p>	<p>unable to continue with previous employment/ career and generally not able to sustain income resulting tax payments at previous levels.</p> <p>possibly drawing on disability benefits.</p>	<p>requires retraining for employment where requirement is not to sit at a desk or significant physical activity.</p> <p>alternative therapies in pain management sought out due to limited access to assistance.</p> <p>personal impact of not being able to contribute to society in a similar fashion that they were used to.</p> <p>side effects from drug therapy.</p>
<p style="text-align: center;"><b>Low</b></p>	<p>Drain on health care system pre-diagnosis due to significant level of testing, along with possible false diagnosis and related therapies.</p> <p>significant amount of sick time off or short term leave of absence.</p>	<p>significant level of alternative therapies sought out.</p> <p>personal impact of the unknown future complications due to having a rare disease with no current cure.</p> <p>side effects from drug therapy.</p>

## Other findings

1. For a majority of the patients interviewed, there was a significant level of testing completed before an appropriate diagnosis was made, generally taking place over a number of years.
  - A) These costs generally took place prior to the period of investigation (pre 2001) and thus were not included in this analysis.
  - B) There are likely a number of AKU patients not currently diagnosed who are going through the same process and thus a cost to the system not specifically identified here.
1. There are significant individually unquantifiable costs incurred related to drug therapy (specifically pain management), health care devices, and psychological distress which were found to occur across the majority of patients interviewed.
2. There are rare but significant health issues which appear to have a correlation to AKU including stroke, cardiac failure, kidney failure and Parkinson's disease.
3. The longer a patient has been living with significant complications resulting from AKU, the longer and more costly a recovery from necessary surgeries will take. Patients who are having their first or second surgery generally required less than three days recovery in hospital, while those who had been living with significant symptoms for longer and were having their third or more surgery can require more than a week to recover in hospital along with a few days in special or intensive care.
4. For those patients who are not diagnosed at a young age when possible discoloration is evident in their nappies it was found that the time from first additional major symptoms to actual appropriate diagnosis can take a significant amount of time. Over this period of time patients endure a significant amount of testing, consultant time and therapy resulting in significant unnecessary health care costs, which were not included in this study.
5. Recorded patient events and resulting costs dropped significantly when looking out past five years due to patients not remembering exact treatments that long ago and thus not recording them as events. A drop was also seen for four and five years out, but not as significant and not for all patients so this data was captured and reported.
6. A drug called Nitisinone, currently licensed for the treatment of Hereditary Tyrosinemia Type 1 but not for AKU, has shown strong potential for AKU. However, the AKU Society is only aware of two AKU patients who have access to Nitisinone off-label as Primary Care Trusts generally refuse to reimburse it. Nitisinone prescribed off-label for AKU currently costs between £3,000-£6,000 per patient per year. If prescribed early enough in life, it could significantly reduce or prevent the debilitating clinical symptoms of AKU, wiping out any need for joint surgery and other interventions. Further studies are being carried out on Nitisinone for AKU.

# Key assumptions and limitations of findings

## Key assumptions

1. Costs included in the sources used for cost research are reasonably in line with actual costs incurred by the NHS for the patient events recorded.
2. All costs included are costs incurred by the NHS as a direct result of the patient living with AKU. Appropriate consideration for this assumption was taken throughout the research and not all patient events discussed during interviews were included in costing due to event being unrelated to AKU (e.g. – regular yearly GP check-up).
3. Where not enough appropriate information was available for costs, a conscious effort was made to take the average or more conservative estimate from similar health care events.
4. Where not enough information was available to determine if event was AKU related or not, a conscious effort was made to take a conservative approach.

## Key limitations

1. The sample of AKU patients was self selected. Preliminary discussions with the AKU society suggests that there are a higher percentage of individual living with AKU in the Moderate to Low categories
2. No AKU patients under the age of 40 were interviewed. These individuals are still a part of the AKU patient population and due to the nature of the disease would likely have to be considered as additional lower or no cost category if this information is extrapolated to the population of individuals living with AKU.
3. Due to the conservative approach taken, there is a significant risk that the costs highlighted in this report are significantly lower than actual costs incurred by the NHS.
4. Findings, other than extrapolations on the “Key Findings” page do not include costs of drug therapy or medical devices (home stair lift, special beds, toilet adaptors, etc.) as these costs were not readily determinable despite being identified in patient interviews.

# Sources

1. NHS reference costs 2009-2010, [http://www.dh.gov.uk/en/Publicationsandstatistics/Publications/PublicationsPolicyAndGuidance/DH\\_123459](http://www.dh.gov.uk/en/Publicationsandstatistics/Publications/PublicationsPolicyAndGuidance/DH_123459)
  - (a) Reference Costs 2009-10 overview and summary (PDF)
  - (b) Appendix NSRC04: NHS trust and PCT combined reference cost schedules (XLS)
  - (c) Appendix RC1: Reference costs index (RCI) (XLS)
  - (d) Appendix NSRC01: NHS trust reference cost schedules (XLS)
  - (e) Appendix NSRC02: Primary care trust reference cost schedules (XLS)
  - (f) Appendix NSRC03: PMS and sites reference cost schedules (XLS)
  - (g) Appendix NSRC05: Non-NHS provider schedules (XLS)
  - (h) DBRC organisation-specific reference cost data
2. NHS Payment by Results 2010-11 National Tariff Information, <http://data.gov.uk/dataset/payment-by-results-2010-11-national-tariff-information>
3. AKU Business Case Financials v3.xls (Obtained from Nick Sureau of the AKU Society)
4. AKU Society interviews: Nick Sireau, Chairman; Dr. Lakshminarayan Ranganath, co-founder and Medical Director; Oliver Timmsi, Communications Project Manager; and Duncan Batty, Patient Support.
5. Royal Liverpool and Broadgreen University Hospitals, Blood Sciences Department: Craig Evans, Blood Sciences Directorate Manager; and Paul McNulty, Divisional Finance Manager
6. Patient interviews: Five AKU patients, all names are confidential
7. *Cost-of-Illness Studies – A Primer*, Joel E. Segel, January 2006, RTI International, RTI-UNC Center of Excellence in Health Promotion Economics
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9. Consumer Price Index (CPI) data: [http://www.statistics.gov.uk/downloads/theme\\_economy/CPI.pdf](http://www.statistics.gov.uk/downloads/theme_economy/CPI.pdf)