

1	<p>Treatment & Condition (Title)</p> <p>Tolvaptan (Jinarc®) for treating autosomal dominant polycystic kidney disease (ADPKD)</p>
2	<p>Associated appraisal body (NICE/SMC/Other) & Summary of ruling (to include indication, restrictions, other relevant information)</p> <p>NICE Technology Appraisal Guidance (TA358) October 2015</p> <p>Tolvaptan is recommended as an option for treating autosomal dominant polycystic kidney disease in adults to slow the progression of cyst development and renal insufficiency only if:</p> <ul style="list-style-type: none"> • they have chronic kidney disease stage 2 or 3 at the start of treatment • there is evidence of rapidly progressing disease and • the company provides it with the discount agreed in the patient access scheme.
3	<p>Number of people in Northern Ireland expected to take up service/therapy (including new cases per year)</p> <p>According to the costing template that accompanies TA358, by year 5, there will be 77 people who will be eligible for treatment with tolvaptan. Of these, 39 people are likely to take up treatment.</p> <p>Advice from local clinicians is that further work is ongoing at national level to provide clear definitions of ‘rapidly progressing disease’. A proportion of patients eligible for treatment are not currently referred to nephrologists (CKD stages 1-3) and current laboratory testing does not distinguish between stages 1 & 2. These lead to uncertainty in terms of the numbers commencing treatment. Of those patients who are eligible, a high discontinuation rate from treatment is expected.</p>
4	<p>Patient Access Scheme availability</p> <p>The company has agreed a patient access scheme. This scheme provides a simple discount to the list price of tolvaptan, with the discount applied at the point of purchase or invoice. The level of the discount is commercial in confidence.</p>
5	<p>Costs (before PAS if applicable)</p>
5.1	<p>Drug cost per patient per annum (for new and prevalent cases)</p> <p>Tolvaptan is taken orally, twice daily as a split dose. Doses can be titrated according to tolerability up to a maximum total daily dose of 120 mg. It is available as 15 mg, 30 mg, 60 mg and 90 mg tablets, in 28-day packs of split-dose tablets, at a flat net price of £1208.20, equating to £43.15 per day, regardless of dose. The annual cost of tolvaptan is estimated by the company to be £15,750 per person.</p>

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5.2	<p>Infrastructure costs per patient per annum</p> <p>Some of the eligible patients do not currently attend nephology clinics. If they are referred they will need ongoing monitoring to identify those with 'rapidly progressing disease' who would be eligible for Tolvaptan. This could include MRI scanning. These potential additional infrastructure costs are not quantified in the NICE costing template and it is not possible to provide an estimate at this stage.</p> <p>There will be costs for monitoring of patients on Tolvaptan treatment and those are set out below.</p>																		
5.3	<p>Current in year costs</p> <p>In year costs in 2015/16 will be approximately £8k.</p>																		
5.4	<p>Recurrent overall costs per annum (<i>including additional costs</i>)</p> <p>The costing template using standard assumptions estimates net costs at year 5 of £602k of which £11k is additional monitoring costs.</p> <p>The estimated trend of growth and the associated costs before the patient access scheme is applied is set out in the table below.</p> <table border="1"> <thead> <tr> <th></th> <th>Year 1</th> <th>Year 2</th> <th>Year 3</th> <th>Year 4</th> <th>Year 5</th> </tr> </thead> <tbody> <tr> <td>Number of patients</td> <td>1</td> <td>5</td> <td>10</td> <td>23</td> <td>39</td> </tr> <tr> <td>Net cost</td> <td>£8k</td> <td>£61k</td> <td>£154k</td> <td>£356k</td> <td>£602k</td> </tr> </tbody> </table>		Year 1	Year 2	Year 3	Year 4	Year 5	Number of patients	1	5	10	23	39	Net cost	£8k	£61k	£154k	£356k	£602k
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5.5	<p>Opportunities for cost savings and how these will be secured</p> <p>Tolvaptan is the first treatment to delay progression in ADPKD in terms of both total kidney volume growth and rate of kidney function decline. This delays time to kidney failure and also to when dialysis and transplant may be needed. It is anticipated that there will be increased drug costs, but this should reduce the need for hospital admission, dialysis and transplant.</p>																		
6	<p>Expected implementation period</p> <p>There is no impediment to immediate implementation for new patients.</p>																		
7	<p>Commissioning arrangements</p> <p>Given the uncertainty in terms of the numbers commencing treatment the drug will be commissioned on a cost-per-case (CPC) basis. This will allow patient numbers to be identified and the level of uptake monitored.</p>																		

8	Monitoring arrangements The cost per case applications will be reviewed by SSCT on an annual basis to allow patients numbers to be identified and the level of uptake to be monitored.
9	DHSSPS Legislative/Policy Caveats This advice does not override or replace the individual responsibility of health professionals to make appropriate decisions in the circumstances of their individual patients, in consultation with the patient and/or guardian or carer. This would, for example, include situations where individual patients have other conditions or complications that need to be taken into account in determining whether the NICE guidance is fully appropriate in their case.