NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Patient/carer expert statement (STA)

Tolvaptan for treating autosomal dominant polycystic kidney disease [ID652]

Thank you for agreeing to give us your views on this treatment that is being appraised by NICE and how it could be used in the NHS. Patients, carers and patient organisations can provide a unique perspective on conditions and their treatment that is not typically available from other sources. We are interested in hearing about:

- the experience of having the condition or caring for someone with the condition
- the experience of receiving NHS care for the condition
- the experience of having specific treatments for the condition
- the outcomes of treatment that are important to patients or carers (which might differ from those measured in clinical studies, including health-related quality of life)
- preferences for different treatments and how they are given
- expectations about the risks and benefits of the treatment.

We have already asked your nominating organisation to provide an organisation's view. We are asking you to give your views as an individual whether you are:

- a patient
- a carer (who may be voicing views for a patient who is unable to) or
- somebody who works or volunteers for a patient organisation.

To help you give your views, we have provided a questionnaire. You do not have to answer every question — the questions are there as prompts to guide you. The response area will expand as you type. The length of your response should not normally exceed 10 pages.

1. About you

Your name: Simone Goren
Name of your nominating organisation: PKD Charity
Do you know if your nominating organisation has submitted a statement?

☑ Yes ☐ No

Do you wish to agree with your nominating organisation's statement?

☑ Yes ☐ No

(We would encourage you to complete this form even if you agree with your nominating organisation's statement.)

Are you:

- a patient with the condition?

☑ Yes ☐ No

- a carer of a patient with the condition?

☐ Yes ☑ No

- a patient organisation employee or volunteer?
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☐ Yes ☑ No

Do you have experience of the treatment being appraised?

☑ Yes ☐ No

If you wrote the organisation submission and do not have anything to add, tick here ☐ (If you tick this box, the rest of this form will be deleted after submission.)

2. Living with the condition

What is your experience of living with the condition as a patient or carer?

I was diaganosed with PKD about 20 years ago after discovering that my father had the condition. My father went on to receive a transplant. When I was diagnosed I had already had two of my children and had not had any symptoms. I have since had raised blood pressure and high cholesterol. Both of which are being treated with medication. I was made aware of the Tolvaptan trial and was assessed and accepted on it. I do not currently experience pain as a result of my condition.

3. Current practice in treating the condition

Which treatment outcomes are important to you? (That is, what would you like treatment to achieve?) Which of these are most important? If possible, please explain why.

The outcomes that are important to me are the slowing down of the growth of the cysts and a stable kidney function which will allow me to continue to have a full and active work and personal life. I would like to avoid having dialysis or the need for a kidney transplant which given my family history is a very high possibility.

What is your experience of currently available NHS care and of specific treatments? How acceptable are these treatments – which did you prefer and why?

Currently there is no treatment available for PKD, so I am very grateful to be on the Tolvaptan trial. The symptoms caused by the growth of the cysts (high blood pressure, cholesterol) can be controlled by medication, But there is nothing available to stop the cysts from continuing to multiply and grow.

4. What do you consider to be the advantages of the treatment being appraised?

Benefits of a treatment might include its effect on:

- the course and/or outcome of the condition
- physical symptoms
- pain
- level of disability
- mental health
- quality of life (such as lifestyle and work)
- other people (for example, family, friends and employers)

- ease of use (for example, tablets rather than injection)
- where the treatment has to be used (for example, at home rather than in hospital)
- any other issues not listed above

Please list the benefits that you expect to gain from using the treatment being appraised.

The advantages I expect to gain from this treatment are many they include: :
Not having my life dependent on dialysis and all that this entails
Not having to wait for a kidney donor or ask or expect a family member to donate theirs.
Not have the physical symptoms associated with kidney failure
Have an increased quality of life both within work and home
The above outcomes would have a hugely negative effect on my family including my 3 children who all know they have a 50% chance of having this disease.

Please explain any advantages that you think this treatment has over other NHS treatments in England.

There are no other treatments available for PKD. The future can appear very bleak when faced with no option for treating this condition

If you know of any differences in opinion between you and other patients or carers about the benefits of the treatment being appraised, please tell us about them.

5. What do you consider to be the disadvantages of the treatment being appraised?

Disadvantages of a treatment might include:

- aspects of the condition that the treatment cannot help with or might make worse
- difficulties in taking or using the treatment (for example, injection rather than tablets)
- side effects (for example, type or number of problems, how often, for how long, how severe. Please describe which side effects patients might be willing to accept or tolerate and which would be difficult to accept or tolerate)
- where the treatment has to be used (for example, in hospital rather than at home)
- impact on others (for example, family, friends and employers)
- financial impact on the patient and/or their family (for example, the cost of travel to hospital or paying a carer)

- any other issues not listed above

Please list any concerns you have about current NHS treatments in England.

The NHS is just treating the effects of PKD and is many ways providing nothing for people with this condition. All that we are given is routine check ups until we are suffering kidney failure when the only option becomes dialysis or transplant.

Please list any concerns you have about the treatment being appraised.

I take and tolerate the highest dosage of Tolvaptan. The side effects experienced with the drug Tolvaptan are increased thirst and urination. Initially these took a little getting used to. You tailor your life to include both of these two elements. But I have found them completely manageable. Over time they have become part of my life and my routines. I ensure that I have drinks with me and at all times and am able to access a toilet. Additionally as the time that the drug can be taken is able to be slightly adjusted as long as there is nine hours between doses. This can be useful on long train journeys etc.

If you know of any differences in opinion between you and other patients or carers about the disadvantages of the treatment being appraised, please tell us about them.

6. Patient population

Do you think some patients might benefit more from the treatment than others? If so, please describe them and explain why.

Do you think some patients might benefit less from the treatment than others? If so, please describe them and explain why.

I believe all patients should be given the opportunity to access this treatment. It can be life changing and life enhancing and help to change the outcomes for this awful genetic condition for them and future generations

7. Research evidence on patient or carer views of the treatment

Are you familiar with the published research literature for the treatment?

☐ Yes ☑ No

If you answered 'no', please skip the rest of section 7 and move on to section 8.

Please comment on whether your experience of using the treatment as

part of routine NHS care reflects the experience of patients in the clinical trials.

Do you think the clinical trials have captured outcomes that are important to patients? Are you aware of any limitations in how the treatment has been assessed in clinical trials?

If the treatment being appraised is already available in the NHS, are there any side effects that were not apparent in the clinical trials but have emerged during routine NHS care?

Are you aware of any relevant research on patient or carer views of the condition or existing treatments?

☐ Yes ☐ No

If yes, please provide references to the relevant studies.

8. Equality

NICE is committed to promoting equality of opportunity and eliminating discrimination. Please let us know if you think that recommendations from this appraisal could have an adverse impact on any particular groups of people, who they are and why.

9. Other issues

Do you consider the treatment to be innovative?

☑ Yes ☐ No

If yes, please explain what makes it significantly different from other treatments for the condition.

There is currently absolutely no treatments available for PKD patients. Patients can do nothing to stop the growth of the cysts on their kidneys and liver. They are faced with an uncertain future as regards the timescale but do know that their health and kidney function will deteriorate in time. Tolvaptan gives suffers an option to do something rather than nothing. Whilst this drug is not without its challenges to those taking it, they are manageable.

Is there anything else that you would like the Appraisal Committee to consider?

This drug is essential for patients of PKD and their families. This genetic condition has a huge impact on patients and their families. Each child of an affected parent knowing they have a 50% chance of inheriting this disease. This is a worrying statistic for both parents and children to have hanging over them. The fact that there is no known treatment makes this even worse. Having a drug available which can slow down the growth of cysts can make a significant difference to not only the physical wellbeing of these people but also their mental health

10. Key messages

In no more than 5 bullet points, please summarise the key messages of your submission.

- Currently no treatment for PKD
- Future of dialysis, transplant and numerous health conditions relating to PKD
- Side effects are manageable
- Increased quality of life for patients and their families
- Drug should be licenced for all qualifying sufferers

Simone Goren

January 2015