



## Common Drug Review *Patient Group Input Submissions*

### **tolvaptan (Jinarc) for Autosomal dominant polycystic kidney disease (ADPKD)**

**Patient group input submissions were received from the following patient groups. Those with permission to post are included in this document.**

Polycystic Kidney Disease (PKD) Foundation of Canada — permission granted to post.

#### **CADTH received patient group input for this review on or before June 23, 2015.**

CADTH posts all patient input submissions to the Common Drug Review received on or after February 1, 2014 for which permission has been given by the submitter. This includes patient input received from individual patients and caregivers as part of that pilot project.

The views expressed in each submission are those of the submitting organization or individual; not necessarily the views of CADTH or of other organizations. While CADTH formats the patient input submissions for posting, it does not edit the content of the submissions.

CADTH does use reasonable care to prevent disclosure of personal information in posted material; however, it is ultimately the submitter's responsibility to ensure no personal information is included in the submission. The name of the submitting patient group and all conflict of interest information are included in the posted patient group submission; however, the name of the author, including the name of an individual patient or caregiver submitting the patient input, are not posted.

## Polycystic Kidney Disease (PKD) Foundation of Canada

### Section 1 — General Information

Name of the drug CADTH is reviewing and indication(s) of interest	JINARC (tolvaptan)
Name of the patient group	
Name of the primary contact for this submission:	██████████
Position or title with patient group	██████████
Email	██████████
Telephone number(s)	██████████
Name of author (if different)	
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Website	<a href="http://www.endpkd.ca">www.endpkd.ca</a>
Permission is granted to post this submission	Yes

#### 1.1 Submitting Organization

The mission of the PKD Foundation of Canada is to promote programs of research, advocacy, education, support and awareness in order to discover treatments and a cure for polycystic kidney disease (PKD), and improve the lives of all it affects.

#### 1.2 Conflict of Interest Declarations

We have the following declarations of conflict of interest in respect of project sponsorship and corporate sponsorship:

- Over the past three years, the PKD Foundation of Canada has received project sponsorship from Otsuka Canada Pharmaceutical Inc. inclusive of website translation (French), front-line initiative capacity building, and the PKD Canadian Symposium.
- Additionally, Otsuka Canada Pharmaceutical Inc. has been a corporate sponsor of the Walk for PKD campaign since 2013.

We have the following declaration(s) of conflict of interest in respect of those playing a significant role in compiling this submission:

- None

## Section 2 — Condition and Current Therapy Information

### 2.1 Information Gathering

Information was obtained through personal knowledge, patient telephone interviews and an online survey.

### 2.2 Impact of Condition on Patients

The aspects of PKD that people believed were most important to control included high blood pressure, kidney function and slowing down the progression of cyst development and growth on both the liver and kidneys. A healthy diet and exercise were also identified as ways to control these aspects of the disease.

As part of the online survey, respondents were asked to “rank each of the following in terms of how important you feel they are in determining whether or not you feel your PKD is under control”. The most frequent responses were:

- My kidney function is not declining rapidly – 63%
- I am not experiencing many PKD symptoms – 15%
- My kidneys are not growing too quickly – 13%

In terms of the effect that PKD has on day-to-day life, the people interviewed indicated that it impacts every component of their lives:

- How they get dressed and what they wear
- Wrapping bandages around their abdomens daily for pain/hernias
- Limitations on physical tasks (energy level is so low that even basic household tasks are too much)
- Social stigma (women always being asked if they are pregnant)
- Emotional toll (particularly when living in a small community) because of the way they look (large abdomen)

The survey asked respondents how often they experience a list of symptoms. The most frequent responses were:

- Fatigue – 72%
- High blood pressure – 54%
- Anxiety – 55%
- Abdominal distension – 51%
- Back pain – 48%
- Abdominal pain – 46%
- Liver cysts – 43%

The survey also asked people to “rate your level of concern about the impact of PKD on your current overall health”. Almost two-thirds (62%) of respondents said they were either extremely concerned or very concerned.

There were a number of activities that patients noted they were unable to do as a result of having PKD:

- Simple maintenance and household chores
- Riding a bicycle

- Playing sports
- Exercising
- Walking
- Activities with children
- Long distance driving (pain, bathroom breaks)

The online survey asked respondents to rate the impact PKD has had on their ability to do a list of activities. The responses that garnered the most 'significant negative impact' were:

- Engaging in sports or recreational activities – 37%
- Traveling/vacationing – 34%
- Complete everyday activities – 24 %

Lastly, the online survey provided some telling quotes about the impact of PKD on patients:

- “It becomes the centre of your existence, and early death sentence. Treatment becomes the focus of your life vs. your family. Especially having seen your parent go through the same affliction.”
- “High blood pressure, pain, infection, physical changes in your body shape and the worry about needing dialysis or a transplant can cause anxiety and uncertainty.”
- “It is a disease that totally takes over your life, from the minute you are diagnosed. It affects everything and everybody that is in your life.”

### **2.3 Patients' Experiences With Current Therapy**

Of the patients interviewed, all were currently taking JINARC, as well as medications for high blood pressure and hypertension. They were unsure of their kidney function stability, although volume scans showed slower progression of the disease.

On a related note, respondents to the survey were asked to “rate your level of agreement with each of the following”. The most frequent responses were:

- I feel that I have a good understanding of my PKD prognosis – 79%
- PKD is not a priority within the Canadian healthcare system – 75%
- My doctor or other healthcare professional is receptive/attentive to my needs – 76%
- There are no effective treatments available for PKD – 63%

The adverse effects of therapies that were most difficult to tolerate according to the patients interviewed were:

- Large amounts of fluid intake/urine output
- Advanced daily planning to manage fluid intake/urine output
- Temporary increase in liver enzymes

There were also hardships related to accessing JINARC. They included travel time and discomfort related to clinic visits. All of the interviewees noted that without a clinical trial or private drug coverage, none of them could afford JINARC.

Finally, there were definitely needs experienced by some of the patients interviewed that were not being met by JINARC. They included:

- Liver impact
- Cyst development (including existing cysts)
- Hernias
- Pain management
- High blood pressure
- Social/emotional challenges
- Impact on family life
- Ability to get life insurance
- Risk to employment (due to medication costs)
- Risk of passing PKD onto children (two-thirds of the online survey respondents have other family members who have been diagnosed with PKD)

### **2.4 Impact on Caregivers**

Caregivers conveyed a number of challenges they faced in caring for someone with PKD. There were financial hardships (costs of medications, hospital stays, insurance coverage, basic costs of living) and intimacy issues (partner may have low self-esteem due to size of abdomen, chronic pain). There were changes in lifestyle (diet, reduced travel/vacations) and physical challenges (taking on additional household responsibilities).

But the one emotion conveyed by caregivers overwhelmingly was fear. They were fearful of their loved one with PKD having liver failure, kidney failure, an aneurysm, diverticulitis, a heart attack – or passing away. They also feared the disease being passed along to their children, and were therefore very conscious of the importance of testing and monitoring their children for the signs and symptoms of PKD.

One caregiver expressed the importance of having treatments that delay the progression of the disease or, ideally, stop PKD from developing in the first place. Another felt so compelled to take action that he became a living kidney donor, doing his part to support his spouse and children who all have PKD.

The online survey asked respondents to rank the extent to which they agreed or disagreed with a series of statements. The results, as it relates to their loved ones, included the following:

- I worry about becoming a burden on close friends and family members as a result of my PKD – 67% completely or somewhat agreed
- PKD has impacted future family planning because I am fearful of passing the condition onto my children – 37% completely or somewhat agreed
- PKD has placed a strain on my relationship with my spouse/partner – 29% completely or somewhat agreed

Finally, it was noted that when you live with a PKD patient who is immune-compromised, it does not allow the caregiver to have live vaccines (i.e. to prevent shingles), and even common colds can have an impact on the family (i.e. alternative sleeping arrangements).

## Section 3 — Information about the Drug Being Reviewed

### 3.1 Information Gathering

Information was obtained through personal experience and patient telephone interviews.

### 3.2 What Are the Expectations for the New Drug or What Experiences Have Patients Had With the New Drug?

#### a) *Expectations of the New Drug*

Patients felt JINARC will delay the need for dialysis and/or transplantation, as well as prolong the quality of their lives. As affected parents, they were hopeful it would provide a healthier, more optimistic future for their children and family members by catching the disease earlier. And ideally younger generations would not suffer the full effects of PKD.

Additional expectations of JINARC included:

- Allowing individuals to work longer without taking sick leave, thereby putting them in a more sound place financially when the time comes to retire or take sick leave
- Allowing them to contribute to society more, without succumbing to the PKD lifestyle

#### b) *Patients' Experiences with the New Drug*

For those patients who have had experience with JINARC, the positive effects included:

- The feeling that they now have more time before starting dialysis and needing a transplant
- More control over their disease
- A sense of relief
- Less daily pain (slower cyst growth on kidneys means less pressure on other organs)

The negative effects included:

- Urination and frequency of times needed to go to bathroom (although most patients say they do get used to it)
- Large fluid intake, which is hard to handle at first
- Increase in thirst
- Dizziness, if patient is taking blood pressure medication (although this was resolved through medication adjustments)
- Liver enzymes – in a very small percentage of patients, liver enzymes can increase (in which case their physician simply removed them from the drug briefly to ensure their level stabilized before putting them back on JINARC)

The symptoms of PKD that JINARC managed well were:

- Depression – there was a positive emotional component of having hope and treatment
- A slowing of the decrease of the glomerular filtration rate (due to a slowing of cyst development and growth)
- Fewer major kidney pain events (i.e. resulting in fewer pain medication needed, trips to the ER, missed workdays, etc.)
- Less symptoms overall (especially for earlier stage patients)

The symptoms managed less well were:

- High blood pressure

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- Pain in other organs (liver, pancreas, back)
- For some, it does not improve their emotional outlook, having a negative impact on their day-to-day life

The adverse effects caused by JINARC that were seen as unacceptable included:

- Liver enzymes (need to be closely monitored by a physician)
- Fluid intake and urination (although some patients stated this became acceptable)
- Increased tiredness, due to getting up in the night to use the bathroom
- Dry mouth
- Dizziness (if taking both JINARC and a blood pressure medication)

Overall, patients found JINARC easy to use. They felt the oral intake was easy (tiny pills), it can be taken with/without food, it's not an injectable, and it can be taken at or away from home. The two drawbacks identified were the twice-daily regimen and urination frequency/water intake.

Lastly, patients were asked if they thought JINARC helped to better manage their PKD. The results were:

- Patients are staying on the drug, so their belief is that it is helping to slow the progression of their disease
- Patients engaged were part of the TEMPO trial that showed clear, positive data (which led to Health Canada's approval of JINARC)
- They have a positive mental attitude that they are doing something about their disease, rather than sitting around waiting for it to get worse
- They feel less pressure and pain in the kidneys since taking the drug