Name	[redacted]
Organisation	
Role	Carer
Job title	Housewife/Carer
Location	England
Conflict	No
Disclosure	
Comments	486 General I am very disappointment in the decision to reject tolvaptan. My family have been devastated by this disease. My mother in law died at 43, my sister in law died at 52 and my husband had kidney failure at 43 followed by 2 years of dialysis and transplant. I have 2 children with the disease and this drug is the only light at the end of the tunnel for them. There are no other treatments and as they are now over 30 they are facing the inevitability of kidney failure. They have both made the decision not to have children because of this disease. To tell them that a treatment our family has awaited for years is not cost effective is heartbreaking to us. Also it surely must be better to prevent the progression of this disease and save money on dialysis, transplant etc not to mention the loss of earnings when kidney failure ensues
Submission date	05/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Retired GP and Patient
Location	England
Conflict	No
Disclosure	
Comments	487 General I am concerned that the committee has not fully understood the impact of ADPKD on patients and families in terms of daily life and effects of deteriorating renal function to the point of dialysis. There is no treatment for delaying the onset of dialysis and all that it entails, the limitations, diet restrictions, disabilities, effects on family life and reduced work capabilities. Tolvaptan has been shown to delay total kidney volume and cyst progression, where water intake and BP management have not. Tolvaptan has been licensed in Canada and Japan. In not recommending it as a treatment for ADPKD NICE has denied many patients not just hope but actual benefits of the treatment. This should not be a blanket decision, but one available to the nephrologists to make on an individual patient basis. That NICE conclude by supporting BP management and increased water intake is insulting - the majority of ADPKD patients are doing this to the best of their ability anyway.

Submission date	05/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Student
Location	Scotland
Conflict	No
Disclosure	
Comments	488 Location I can't understand how you can say this drug is beneficial to sufferes with PKD and then say it's too costly. My understanding is that this drug would stop patients being on dialsysis because it is aimed for stages 1-3 so surely the NHS would rather spend money on this drug than spend money on dialysis?
Submission date	05/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Technical Solutions Manager
Location	England
Conflict	n/a
Disclosure	Think of the fact their is no known cure which is such a mental problem
Comments	489 General This was the thing keeping me and my family going through this hell we have to endure. I live everyday seeing my two children who I have passed this life sentence to. I have gone through all the stages and have now had a transplant so have to think of all the pain and suffering I have endured which they will have to go through. I have lost my house, career and my mind over this and to think someone is stopping my children from having a treatment that will postpone their suffering is a disgrace. I am so so sorry and hurt you can't see past the cost or fact it can't work for everyone
Submission date	05/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Government Service
Location	
Conflict	No
Disclosure	

Comments	490 General Very disappointing that you have not considered the full effect of Tolvaptan on ADPKD and have looked at on kidney failure in general. This appears to be the first drug that does work for ADPKD and this should be taken into account.
Submission date	05/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Children's Nurse and ADPCD patient
Location	England
Conflict	No
Disclosure	I have worked as a registered nurse in the NHS and hospices for over 30 years. I understand the decisions NICE have to make fully as a professional. On a patient basis I am deeply troubled that the 1st drug found to work for this condition is turned down.
Comments	491 General After years of waiting and hoping for a treatment for one of the most common renal diseases Tolvaptan was developed. I am deeply disappointed that myself and every other person living with this incurable, inherited, life-threatening and debilitating kidney disease may be denied access to a licensed therapy which has the potential to improve the quality of our lives, and the lives of our family members and future generations. Regrettably, the NICE Committee have commented that aggressive blood pressure management and increased fluid intake may have an impact on declineâ€™. There is no evidence to support these statements. Whilst patients with other forms of chronic kidney disease (CKD) such as diabetes have benefited from approaches to slow the decline of kidney function, these have not worked in ADPKD[vi]. This is the first drug that has promised any relief from the unrelenting symptoms of renal failure, constant discomfort and severe pain. We are being denied the opportunity to be able to continue a productive life. On the balance of probability we face death in our later 50s early 60s if there is no intervention. I urge you to reconsider your decision and give us all a chance at a normal life.
Submission date	05/06/2015

Name	[redacted]
Organisation	
Role	Carer
Job title	

Location	
Conflict	No
Disclosure	
Comments	492 General I believe that the committee hasn't fully taken into account the affect ADPKD has on patients and carers. Everybody deserves the access to preventive and slow progression drugs. I believe that this committee haven't fully taken all relevant evidence into account, including current cost implications the NHS is experiencing with renal conditions. The active ingredient, tolvaptan, was shown to affect the rate of kidney cyst growth and modify progression; this would lead to less of a cost implication over time for the NHS.
Submission date	06/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	
Location	England
Conflict	No
Disclosure	
Comments	493 General I am deeply disappointed with this decision which does not seem to take into account the ongoing medical and psychological issues of living with PKD on a day by day basis and the prospect that kidney failure will strike before 60. I am 37 and have been on blood pressure medication every day since the age of 27. I have two children who may have inherited this. My father had to have a kidney transplant at the age of 55 and has been unemployed since then - this is what is currently facing me without Tolvaptan which, in the recent TEMPO 3:4 showed that Tolvaptan demonstrated a significant relative reduction of 49.2% on total kidney volume growth over 3 years when compared with placebo. I would urge NICE to review this for me and the thousands of others like me who without Tolvaptan will be condemned to slow and often painful decline of kidney function until transplant is the only hope.
Submission date	07/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Nutritionist

Location	England
Conflict	No
Disclosure	
Comments	General 493 As a ADPKD patient, with 2 young children with a 50/ 50 chance of inheritance, I am deeply saddened by this decision. This disease affects all of my life greatly, I am anxious, suffer depression regarding it. I also have no hope for my children that a treatment is there, and this decision is likely to thwart any interest of other drug companies to develop similar drugs. I now have no chance of . fathering more children as I do not want the heartache of potentially affecting another child.
Submission date	08/06/2015

Name	[redacted]
Organisation	
Role	
Job title	Healthcare Support Worker
Location	England
Conflict	n/a
Disclosure	
Comments	499 General I am a 26 year old female with PKD and I was diagnosed nine years ago at the age of 17. Until that point, and a couple of years after, I had suffered very few ill side effects from this condition. This changed at the age of 19. I began to suffer with pain, infections, and urate kidney stones. It was however, only a couple of times a year and I felt as though I could deal with it. It did affect my day to day life, but only for a couple of times a week, and a couple of times a year. This all changed a few years ago. I was studying a degree in BSc Midwifery. The pain and stones I experienced during this time was so severe that I struggled with the workload and fell behind. I failed my first year and had to leave the course. This was the first time having PKD and what comes with it had affected me and my life. It had ruined my dreams of a career and I felt lost. With my physical health being in such bad condition, my mental health also began to deteriorate (I have since been diagnosed with BPD.) Despite all of this, I managed to get myself a job as a Support Worker in a hospital. After beginning this job, my kidneys and condition drastically worsened. Due to the long hours and heavy work, I found that the pain which was minimal before quickly became daily. The infections which were only once or twice a year became constant. I had and still have to have a lot of days off sick and I struggled with the workload. My Bradford score was in the thousands and I couldnâ€™t reduce it because of the constant pain I was in. I have been hospitalised

	more times than I can count, and the future looks bleak. If I am in this much pain and my kidneys are still fully functional, how will the pain be when their function begins to reduce? How will I cope then, and how will I work? I am in pain every day, and the only way I can work most days is to dose up on painkillers, which cannot be good for my system. I am almost certain my job does not help my condition; however, I feel I am now stuck. Because of my condition, and it being a condition which will only get worse, not better, I have been told I am basically unemployable. A job I was offered was since withdrawn after they found out about my Kidney Disease and Bradford score. Tolvaptan gave me hope; I knew it wouldnâ€™t solve the issue of what was already there but the thought that it could prolong my kidney function for a good 5-10 years excited me. I do not know when my kidneys will begin to fail and how quickly it will progress. What I do know is that I have family members who have not suffered much in comparison and they have now been told they need a transplant while they are still in their 50â€™s. This frightens me. I donâ€™t want to have a transplant while I am still so young. The pain, and the amount I suffer with my PKD and the effect it has on my life has made me take the decision that when I am ready to have children, I am going to do it using IVF, and eggs which do not hold the gene for PKD. I would never want my children to suffer like me; in fact, I would never want even my worst enemy to suffer like this. Even carrying a child frightens me, as I am worried about how this may affect my body and my condition. I am aware that it made my nana quite ill. I find it sad that cost is a big factor in choosing to not fund Tolvaptan. It could help many people and bring hope where there was previously none. I have a granddad who has dementia and is on more tablets every day than what seems can be counted. It seems silly that a dying man of 86 who cannot even self-medicate is given more help and medication, than a young woman of 26 who still has a lot of her life ahead. I just hope your decision can be changed by mine and others stories. It is hard to know how bad it can be unless you are actually in a position like mine, and I feel the bigger picture should be looked at more than the smaller one.
Submission date	11/06/2015

Name	[redacted]
Organisation	
Role	Public
Job title	Director
Location	England
Conflict	No
Disclosure	

Comments	501 General Polycystic kidney disease has dominated four generations of our family. Tolvaptan is the first ever drug developed to reduce cysts within the kidney. Progress and hope for patients with this life threatening disease rather than waiting for the inevitable failure of the kidneys. The studies showed benefits to patients and the administrating of Tolvaptan per annum, compared to dialysis, must be cost effective to the NHS. It is not reasonable within the NHS organisation to have NO investment for kidney patients where their are benefits of cyst reduction when taking the drug. PKD impacts family, career and well being. Patients are being discriminated on the grounds of disability where no hope or drug is currently being offered. This compares with millions spent on cancer related drugs and advancement. We urge you to determine that this drug should be available within the NHS.
Submission date	11/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	
Location	England
Conflict	No
Disclosure	
Comments	503 General I think that the panel should take into account the potentially very long term psychological aspects of this disease (30/40 years), especially when at present there is NO OTHER treatment available. Hope is a very poweful medicine.
Submission date	12/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	teacher
Location	England
Conflict	No
Disclosure	
Comments	504 & 505 General I would like to say how disappointed I was to find it has not been approved. I have been on the trial for over 5 years and I have not had anywhere near the deterioration of kidney function that could have been expected. This will mean that I will be able to work for longer and pay more taxes and not be a drain on public funds. I suggest that NICE have not taken all these extra

	factors into account! It is to late for me, but the fact that there was no real treatment available with this inherited disease has meant I have had to make the difficult decision not to have any children as I did not want to pass on this dreadful disease, however this has had difficult effects on my relationship. If treatments like this drug are more available in the future then it would provide hope for people like me in the future and would completely change my family circumstances! Please, please reconsider the funding for this drug as the effects of not being treated will be huge. Surely the price of thee drug will come down in the future. There are many, many kidney patients that could benefit from this!
Submission date	15/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	
Location	England
Conflict	No
Disclosure	
Comments	527 General I'm an ADPKD patient. My maternal grandmother died from ADPKD aged 50, my mother died at the age of 48 from ADPKD. My son has inherited ADPKD and Tolvaptan represents the only viable option to delay the inevitable failure of his kidneys and the expensive progression to dialysis. Dialysis affects so many aspects of the lives of both the patient and their family. Given the progression of the disease in my family my son is likely to be unable to work when he is in the prime of his life thereby being unable to contribute to society and fulfil his talent and aspirations. My son's story will be matched by many others who are also denied access to Tolvaptan. I understand Tolvaptan may not be suitable for all ADPKD patients but to deny access to it for the many who will benefit is short sighted and completely at odds with the decisions taken by other countries in recent times to authorise the drug, most notably Canada. I urge you, please reconsider your decision.
Submission date	23/06/2015

Name	[redacted]
Organisation	
Role	Carer
Job title	Retired
Location	England

Conflict	No
Disclosure	
Comments	530 General I have cared for my husband who has ADPKD, which was diagnosed in l989, through to end stage renal failure in 2009. He then continued life on dialysis for three and a half years until he received a kidney transplant in 2013. This illness drastically restricted both our lives and those of our children. Our daughter has inherited ADPKD and is now in continuous pain from the cysts on her kidneys. As she is only 40 years of age she has many more years of pain and suffering ahead of her. I understand she is not alone in this suffering as many more people are being diagnosed with this horrible, debilitating disease. I urge the Committee to re-consider allowing the use of Tolvaptan in Britain as it is the only hope of relief for these patients.
Submission date	23/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Retired
Location	England
Conflict	No
Disclosure	
Comments	531 General I am a patient who has suffered from ADPKD for over 50 years, culminating in dialysis and eventually a kidney transplant. Sadly, my daughter who is now aged 40 years, has inherited ADPKD,and is in continuous pain from cysts on her kidneys. I am disappointed that the Committee appears to have not understood the suffering this disease has on patients and lives of family members. I urge you to reconsider permitting the use of Tolvaptan in Britain as it is the only hope of bringing some relief to patients.
Submission date	23/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	
Location	England
Conflict	No
Disclosure	
Comments	534 General I have PKD myself, my mother and father have it. My mother is

	asymptomatic but my father had 2 years of dialysis and then a successful transplant. My older sister also has it and is feeling the effects, she's currently being tested for a match with her husband and friend. I also have it, apart from reduction to my function levels I'm not noticing any effects yet but I am in a steady decline. This wouldn't just change my life, it could change my family and my world.
Submission date	23/06/2015

Name	[redacted]
Organisation	
Role	Spouse of PKD sufferer
Job title	
Location	England
Conflict	No
Disclosure	
Comments	533 General My husband has pkd and has in the past been given the hope that he could be an ideal candidate for this medication. He is young and healthy but we live in the knowledge that his health is borrowed time and the inevitable future will be years of struggle. A drug that can help him in anyway needs to be considered on more than just cost.. You need to look at the people whose lives could be changed immensely. You need to look at the families in need of some hope.
Submission date	23/06/2015

Name	[redacted]
Organisation	
Role	ADPKD patient
Job title	
Location	
Conflict	
Disclosure	
Comments	Thank you for your careful investigation of the merits of tolvpatan for delaying symptoms of autosomal dominant polycystic kidney disease. However, some aspects of the disease seem to have been given too little consideration. PKD is a disease of the poor. Not because poverty causes PKD but because PKD impoverishes families. This is a disease which affects 50% of the members of a family, debilitating many of those of working age to the extent that not only are they unable to earn money or perform any substantial share of the household chores but that they also affect the earning power of other family members by needing additional care. Families like this cannot build up resources to help smooth out the bumps of ordinary living. Famously, many young people today get help from "the Bank of Mum and Dad", but not if the parents' income disappeared prematurely and there are several other relatives

	of varying generations in need of extra help. The loss from the work force of older and more experienced workers is unfortunate for the families, and unfortunate for the country. Buying more years of a useful working life would be valuable. It is easy to find a list of the special tailored support that people with PKD receive from the NHS. For almost all of us, any blank sheet of paper will do. We receive a service designed for everybody else with chronic kidney disease, and it often seems to be given begrudgingly. Why bother about people for whom there is no effective treatment? (Unfortunately, that attitude seems to hold even when we are seeking available relief from extremely unpleasant symptoms. Our GPs often do not have the experience to be of much help, and the doctors who do have that experience appear to feel that people with an untreatable disease do not merit their time.) Many people with PKD believe it is our lack of economic clout that has left us so unsupported. Individually, we usually do not have the money, time or energy for attention-getting campaigns. That 50% chance imposes a great cost on families. Our lack of visibility is compounded by the fact that people with PKD may not be able to work long enough to reach senior decision-making positions, and even our unaffected siblings can have been hampered in their careers by an upbringing with less available money and more necessary responsibilities than their peers. We would all have preferred that people with PKD mattered enough for there to have been accurate statistics for you to have worked from. There is another pernicious effect of the age at which PKD first makes its effects felt. This is a disease of people who have children still living at home. The quality of life of those children can be badly impacted by the illness of a parent, and not just by the financial aspect. Pain and fatigue can limit the interactions between parent and child, whether it is a crying toddler who needs to be picked up and cuddled, a primary school child with energy that needs wearing off with a ball game, or a troubled teenager who might bring themselves to talk more easily if they could go for a long walk with a parent. These things need doing now -- they can't wait until another day when the parent might be feeling better. Every bad day is a day lost forever. Family evenings are less fun if one parent is too exhausted to talk and the other parent is busy seeing all the chores are completed. And all too often the strains have broken the family unit and there is just a single parent who is both exhausted and having to try and hold everything together. Of course a child in a family with PKD is likely to be making a larger-than-usual contribution to the household chores, and may explicitly be a

	carer for their parent. The children of a parent with PKD will learn that it is a hereditary disease, and one that they may have. It is very difficult to see a parent in pain and distress which you cannot alleviate. To know that it is possible that you will be in the same situation later is a terrible burden to bear. If tolvaptan can buy families a few more years of normal family life, that is hugely valuable. The children of parents with PKD deserve to have their quality of life preserved as much as possible. Many of these children were only born because their parents believed that effective medications for PKD would appear on the scene shortly -- to allow a parent to have the health to care for the child in the way that they would like to do, and to help the child live a more normal life when they are an adult. We hope that tolvaptan is only the first step along the road to major improvements for the life of people with PKD, but it is a very important symbol of that better future.
Submission date	26/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Sufferer of PKD
Location	
Conflict	
Disclosure	
Comments	I have ADPKD and have always hoped Tolvaptan would be a lifeline for sufferers. There are no other drugs available. I hoped the drug would help my daughter who has early signs of the disease and maybe my grandchildren. Surely sufferers of ADPKD should be given hope as the drug may delay kidney failure. Canada and Europe have approved the drug please give sufferers in the UK the chance to be given this drug. My late son, A PhD. Biochemist was so excited when research was being done on this drug. Please, please give further consideration to approving Tolvaptan.
Submission date	25/06/15

Name	[redacted]
Organisation	
Role	Patient
Job title	
Location	
Conflict	
Disclosure	

Comments	I would like to repond to the recent decision on the use of TOLVAPTAN, I was rencently diagnosed with ADPKD. and think that Tolvaptan may help to slow down the deterioration of my kidneys, allowing me to keep working and contributing to society eg my taxes etc. instead of me being a drain on the NHS and social security. I therefore urge you to reconsider this decision & allow me and others to lead a relatively normal life.
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	
Location	
Conflict	
Disclosure	
Comments	I am having problems trying to submit a comment for the Tolvaptan appraisal for poly cystic kidney disease. Please can this be forwarded to the correct department. I have APKD and I am currently lucky with good functioning kidneys and would hope to prolong this for as long as possible, I am fit and healthy, do not smoke, exercise regularly, I am not overweight and would like this to remain so and avoid the need for dialysis should I run into problems in the future as my disease progresses. Surely the cost of Tolvaptan would be more cost effective than dialysis 3 times a week and all the other cost any other complications will incur. I would like to support approval of Tolvaptan by NICE.
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	
Location	
Conflict	
Disclosure	
Comments	Having been unable to leave comment on your website (I am registered) I hope this email response for the Tolvaptan Appraisal Committee will be put forward for consideration. As a patient suffering from ADPKD and a suitable candidate for the new drug Tolvaptan I am extremely disappointed to hear that the NICE are not to recommend Tolvaptan to treat the condition. This drug would give hope to people like myself and their families who deal with the incurable condition on a daily basis. My kidney function is OK, but I have chronic pain which at times is acute - I recently suffered burst cysts from which the fluid went into my pancreatic cavity. This was debilitating for a number of days and took several weeks to recover from. The only 'treatment' was pain relief - most are inadequate and so to

	be pain free I used strong prescribed medication, which have its own side effects. I also have the additional complications of hypertension an increased risk of brain aneurysms (my grandfather died from one) and polycystic liver disease. I used to be very active - 6 years ago I competed in a mini triathlon - but now find it difficult to participate in any activity as I become very breathless. This concerns me on an emotional level as I have an active family and can no longer join them, but I am also worried that it will have a financial impact if I am unable to continue working (I am already denied life insurance and was unable to get a mortgage which places a great burden on my husband). Surely the cost associated with long term dialysis that I am likely to face (my mother was on dialysis for 17 years and my brother is on dialysis after having both kidneys removed) must be worth an alternative treatment being sought? I am extremely disappointed that I, and many other like myself, are to be denied access to a licensed therapy which has the potential to improve the quality of our lives, but also the lives of our family members and those of future generations.
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	
Location	
Conflict	
Disclosure	
Comments	As the partner of an ADPKD patient, I am disappointed to see that that NICE have not recommended that Tolvaptan be used to treat this condition. Over the last three years, my wife's lifestyle has been greatly affected through weight gain (approximately 15KG), breathlessness and general severe abdominal discomfort and she is no longer the active person she used to be (she competed in a triathlon 6 years ago). Until her kidney function is sufficiently reduced that she requires dialysis, there is currently little effective treatment available to ease her discomfort. Transplant, apparently, is not an option while her kidneys have sufficient function. She has read that she might be a candidate for the trialling of this drug given her current kidney function. To have this option removed before the drug has even been trialled is a grave disappointment. Her mother was on dialysis for 17 years due to the same condition. Her younger brother recently had both kidneys removed, has been on dialysis since and is awaiting a transplant with little sign of one forthcoming. Surely the costs associated with long term dialysis that both he and my wife

	potentially face must be worth an alternative treatment being sought? I trust that these thoughts will be taken into consideration before the final recommendation is made.
Submission date	26/06/2015

Name	[redacted]
Organisation	Patient
Role	Patient
Job title	Retired
Location	England
Conflict	n/a
Disclosure	
Comments	540 With polycystic kidneys the doctors plotted its development but could only control my blood pressure and diet for several years before failure and dialysis to stay alive. I have had very successful transplant and realise how ill I was before this. My daughter has inherited the disease as I did from my father and this new drug would hopefully make a better life for her as it develops.
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Carer
Job title	
Location	Wales
Conflict	No
Disclosure	
Comments	541 I am very disappointed by the initial decision to reject the use of Tolvaptan in England and Wales. My son has PKD despite the fact that there is no history of the disease in the family. The condition seriously affects his life both in terms of his physical and mental wellbeing. He will almost certainly need a kidney transplant before the age of 40 unless the progression of the disease can be slowed. Tolvaptan offers some hope to patients who at the moment have no effective treatment available. The cost to the NHS of drugs to alleviate symptoms provide dialysis and carry out transplants is considerable to say nothing of the cost to individuals in their physical and mental wellbeing . I believe Tolvaptan is authorised for use in Canana and Japan and would respectfully ask that the Committee reconsider their original decision and permit its use here.
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Musician/PKD sufferer
Location	England
Conflict	No
Disclosure	
Comments	543 Hello, Having read through much of the research to do with Tolvaptan

	- it appears to me that there is a correlation between the drug and a slowing down of this most unpleasant degenerative disease. I have known that I am a sufferer since the age of 21, I am now 38, I inherited it from my Father who was on dialysis/had a kidney transplant and lived for 17 years without his original kidneys functioning. The emotional aspect of peoples lives slowly being drained away by this disease is obviously not something that is particularly important in this study. Obviously overall cost with NHS funding as it is is supremely important. But the fact that most of the rest of the civilised world have taken this drug on as a successful slower down of PKD should surely surely be something NICE should listen to. Canada for example are NOT a silly country that wastes money. They see a drug that HUGELY INCREASES the good quality and relative value of PKD sufferes lives.. I could not even begin to tell you how much difference and extra 5 or ten years of functional kidneys would mean to me. It literally brings tears to my eyes to think about it. The stress and strain that is caused by certainty of future kidney failure is one of the bleakest feelings you can imagine. The HOPE that this drug and its successful intergration into other countries PKD treatment brings, is only equal to the despair that NICEs deeply short sighted response to Tolvaptan has caused. Why do we have to be the country that simply cannot see hope, value of life and frankly, very positive medical results over burocracy and accounting. The result saddened me deeply. I can only hope that when the board reconvene they can try to see that people living for longer without there kidneys failing IS an important thing. Even down to the simple fact that if I can work until I am 60 rather than 50 I can put at least an extra Â£100 thousand of tax into the country... I would beg the board to reconsider their verdict. As for the side effects issue.. I do not know a single human being who would prefer to be on dialysis over the side effects of a drug. And it is truly truly idiotic to think that is even a possibility. Thirsty and still functioning kidneys? I would take that EVERY SINGLE TIME and I cannot think of a human beaing who would not.
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	NHS Professional
Job title	Radiographer
Location	
Conflict	No
Disclosure	
Comments	544 My mum was diagnosed with ADPKD. She has cysts on her liver and kidneys. Sometimes the pain she suffers from the size of the cysts, or from a cyst bursting is that severe that she can hardly move. She is in constant pain in her day to day life. This drug would dramatically improve her quality of life. I think it is

	disgusting that it has been deemed as not cost effective when it will reduce the need for dialysis in the latter stages of the disease.
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	
Location	England
Conflict	
Disclosure	Yes - I found out about this through the PKD Charity website - it would obviously have been better had patients been given a chance to comment via their GP or specialist centres. I am certain that given a chance far more patients would wish to contribute about their psychological problems as a result of staring a horrible round of illness and possibly death in the face, and the cost to them and the nation of ignoring their need for a focus on the cost of the drug alone.
Comments	545 As a PKD patient, it has been 11 years since the disease was diagnosed at age 28. As well as the hypertension as a result of the disease getting progressively worse since diagnosis, I have now begun to experience discomfort when I sit, lie down or move into different positions due to the enlarged nature of my cystic kidneys. I am also exhausted continuously which means I can't work. I walk and swim to stay fit, but when I walk too much, or it is too hot outside, my ankles swell up and my feel become extremely uncomfortable, making longer walks of 10,000 steps and up unmanageable. This doesn't help in managing my weight, as you can imagine. I have been prescribed antidepressants for the feelings of hopelessness and fear of developing renal failure. I witnessed my father going through the process of dialysis and infections to his kidneys and various stays in hospital - which I do not think I could bear to endure myself. I had been given hope by the Tolvaptan trials, and by the fact that the drug has been found to help stem the progression of the disease. I could bear living as I am but the thought of the disease getting worse, and ruining my quality of life further is very distressing. I have many dreams and ideas of things I would like to achieve - but more and more I can see these slipping away, as time brings me closer to a life of dialysis, followed, if I am lucky, with the trauma of a transplant, and the rest of my life on the great number of anti-rejection drugs which my father was on after his transplant. The cost of all this to the NHS plus the cost of people becoming unable to work, with the associated costs to the exchequer and the welfare bill are surely critical in considering the whole picture of the cost of Tolvaptan. In my case, I am a housewife,

	and not in receipt of benefits - however, my husband is in the top 1% of earners, and our son is currently predicted a stellar academic career. But, we are going to have to leave the UK and move to a nation where Tolavptan is available - thus reducing tax income to the nation of Â£75k per annum as of now, with the prospect in the near future of very much greater earnings. This is a sad day for us as we love the UK, I have been contributing as a charity volunteer and we can see our son going far as he continues in education - but this is simply not workable. Our experience of hospital stays in the NHS have not been favourable - with dirt, unhelpful staff and frightening patients all helping us in our decision that without treatment to prevent my having to end up in hospital with renal failure, we cannot afford to remain in this country. The NHS has a monopoly on care in the UK, and as such private insurers do not take people like me on - unlike the system in France, where my father received first class care till his death. We will have to take away our great contributions to the UK and move to a country which will not only value us but also look after us properly. I clearly hope you will reconsider your decision to reject this important drug - and that you consider the greater cost to the economy of this disease, left untreated, but also the cost to the individual and their families of the emotional burden of getting progressively sicker, and possibly dying - since the UK has been an abject failure at ensuring people opt out of organ donation it is likely more and more people will die of this disease - this will also be on your conscience.
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	
Location	Scotland
Conflict	No
Disclosure	
Comments	546 I am saddened to hear that NICE has initially rejected Tolvaptan. I was diagnosed with ADPKD ten years ago (at 38) and I have been learning more about my disease and regularly attending renal clinic for results, ever since. My kidneys are growing steadily and significantly, as my kidney function gradually decreases. I have two young daughters and I hope that they will have an effective drug to treat them, in future, if they are found to have inherited ADPKD from me. Knowing that I have ADPKD, and that there is no drug treatment currently available, has significantly affected my outlook on life and my hope for a 'normal' middle/old age. It would make a tremendous difference to me to know that I could slow this progression to end stage renal disease by taking a

	drug on a daily basis. From what I have read and heard about Tolvaptan, I have good reason to believe that it is indeed an effective treatment, and I am aware of its availability on prescription in Canada. Therefore, I am dismayed to learn about this initial rejection in England and Wales and I wonder whether the immediate cost of introducing the drug has taken precedence over the long term benefit of having fewer patients to treat for dialysis/transplant in the future. I urge NIC to reconsider. Yours faithfully, [redacted]
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Public
Job title	Solicitor
Location	England
Conflict	n/a
Disclosure	
Comments	547 My husband has advanced polysystic kidney disease and we are in the process of donation. I do not want my 2 young daughters to have to go through this nightmare. PLEASE reconsider your position very carefully. You can and should do all you can to try help people like us. Give it a chance.
Submission date	24/06/2015

Name	[redacted]
Organisation	PKD
Role	Patient
Job title	Housewife
Location	England
Conflict	No
Disclosure	
Comments	548 I suffer from ADPKD as do other members of my family. Please think twice about your decision not to authorise tolvaptan. My consultant at [redacted] is very keen on it and has mentioned it to me as a possible ray of hope. Please reconsider even if it is only suitable for a small number of sufferers, Its a horrible thing to live with and effects not just only the patient but their friends family and work colleagues.
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Public
Job title	

Location	England
Conflict	No
Disclosure	
Comments	549 Although too late for my husband, who is already in end stage kidney failure and on dialysis, I urge you to reconsider allowing doctors to prescribe tolvaptan to patients who suffer with PKD. If a drug had been available which slowed cyst growth this would have meant a reduced number of drugs needing to be prescribed . For instance, before ESRF my husband needed numerous blood pressure medications, EPO, iron infusions, aspirin (to reduce the repeat TIA risk). He is now on dialysis aged 51 and is unable to work as effectively as he did before ESRF. If a drug had been made available sooner, then it might have reduced the need for some or all of his inpatient stays when he got a UTI or other associated complications. Finally, I do not work for Otsuka, and have no connection with them, I have, however, worked in the pharmaceutical industry so am aware of the cost of discovering new drugs and bringing them to the market. With such short patenting periods, the drug companies need to recover some of these costs in order to fund research into further innovative drugs. Please take all these other costs into account, rather than just rejecting Tolvaptan straight away. Thank you for your consideration.
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	
Location	England
Conflict	No
Disclosure	I have PKD and so does my brother, sister and at least 2 of the children.
Comments	550 Please don't reject Tolvatpan. We know it isn't a cute for all but this is a significant step forward after years of no government funding. My family has been substantially affected by polycystic kidney disease and our children deserve a chance to delay the onset. The continuous physical pain is made all the worse by the lack of any drugs to combat the disease and provide the all important hope.
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Carer
Job title	Wife of PKD patient

Location	Scotland
Conflict	n/a
Disclosure	
Comments	551 We are truly disappointed to hear that tolvaptan has been rejected. This could not only make a huge difference to my husbands life, but also the life's of my two young children.
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	
Location	England
Conflict	
Disclosure	n/a
Comments	552 For years my family has watched and waited for this drug to be approved. After a life time of being told there was no cure or treatment for this debilitating disease there was finally hope. When it was approved for use across Europe, we rejoiced. I had participated in an 18 month trial to support the drug trials and it was finally paying off! Only to be told a week later that it had been rejected due to cost! We were devastated. How can you put a coat on quality of life? I lost my grandfather, Aunty and Dad to this disease. I watched my dad die slowly over four months and sat with him the night he passed away. My sister and I are both suffering from the effects of this disease and my son was diagnosed at the age of ten. He's 13 now and I had hoped that his quality of life would not be impaired by this disease, or at least that the effects could be postponed for many years. To deny people access to the only treatment is cruel and short sighted. I cannot believe that years of medication to treat the effects of this disease, along with the cost of dialysis, transplants, associated drugs, surgery costs and staff , as well as benefits when people are unable to work,can be less expensive than a drug that has been proven to slow down the progression of this disease. Please stop thinking short term
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Patient

Job title	Senior Manager
Location	England
Conflict	
Disclosure	No
Comments	553 As a person who suffers from PKD and has seen many family members suffer and die from this dreadful disease I plead with you to please find this treatment. I have been waiting for many years for this breakthrough treatment. PKD affects my life every day both emotionally and physically. My kidneys are still functioning ok and I have been pinning my hope on something helping to prolong my kidney life. Something that can help me live a better, more productive and healthy life and ultimately those of my children who have no doubt inherited this disease as well. Please reconsider your decision, you will be saving lives.
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Public
Job title	Consultant
Location	England
Conflict	No
Disclosure	
Comments	554 PKD affects my girlfriend and several of her family. This research, and medication gave them hope that they may live longer, healthier, and avoid the difficult life relying on dialysis. Please review this decision on behalf of my family and the many other sufferers.
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Midwife
Location	England
Conflict	No
Disclosure	Long term I believe this drug offers good value for money as it will reduce the need for dialysis and transplantation, but no cost savings can ever represent the cost of human misery and suffering, both emotional and physical
Comments	555 I am health professional who also suffers from ADPKD as does my mother, aunt, cousin and my daughters are yet to be tested. ADPKD impacts on my life and that of family members . My mother is currently waiting for dialysis.

	If there is a drug available that can slow the progress of this disease then I wholeheartedly support it. I worry about my children as I am aware of the 50:50 chance that my 3 daughters have of having this disease. ADPKD is a disease that does not only affect the physical health of sufferers but their mental health also....I worry about my future and hope NICE reconsiders it's rejection and on line with Canada and the alEuropean commission approves JINARC as the first licensed drug to treat adults with ADPKD . This will not only give myself hope that the deterioration of my condition will be delayed but that there will be hope for my daughters. Please license this drug .
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Parent
Job title	
Location	Wales
Conflict	No
Disclosure	
Comments	556 As a parent of a child diagnosed with ADPKD I would like to express how deeply disappointed I am that you have not recommended the use of Jinarc for the treatment of this disease. My daughter is a 3rd year dental student at [redacted] University and her studying and daily life is very difficult due to the symptons of this disease. She is often in agony due to infections and this has an adverse effect and a massive impact on her life. People with ADPKD have been waiting for a lifeline from this deadly disease and when Tolvaptan came along it seems their prayers were answered but this hope has been taken away by the decision of NICE. I cannot understand how NICE have decided that this drug does not deliver a cost effective benefit to a person with ADPKD and I urge NICE to rethink their decision to reject this drug.
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Account Manager
Location	England
Conflict	Yes
Disclosure	

Comments	557 I have the condition ADPKD which I inherited from my father. Having seen everything that he has had to go through since his diagnosis is horrific, leading to a full Kidney transplant of which my mother donated her kidney to him! This disease has affected our whole family with the stress and strain of living with this horrendous infliction - I really feel that you should please reconsider your verdict to license this drug for use in ADPKD treatment. I personally do not want to have to go through everything I've seen my poor dad go through Living in hope [redacted]
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	GP
Location	England
Conflict	No
Disclosure	
Comments	558 I am a patient with APKD; my father had end stage renal failure from APKD and needed dialysis in his early 60s. I am also a GP working in inner city practice. I believe that this disease places a significant burden of symptoms on WORKING AGE adults,and therefore your calculations of QALY for tolvaptan should reflect this, including time off work, medications and health contacts that others of a similar age do not require. As retirement age is INCREASING, to enable the country to pay pensions for everyone, surely there is a cost benefit that has not been fully explored by enabling people, such as myself, to reduce cyst size and progression, and to end up with a longer healthy working lifespan to contribute to the UK (and in my case, disappearing species of inner city GPs!) economy? For these reasons the appraisal of cost effectiveness of Tolvaptan needs to be reviewed.
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Partner of PKD sufferer
Job title	Staff Nurse
Location	Scotland

Conflict	n/a
Disclosure	
Comments	559 I am due to be married this coming October- my partner has been diagnosed with pkd and is in good health at the moment. However his mother and his granny who also suffer both have received kidney transplants- his granny's now failing after 6 years. My partners uncle also suffers from the disease- he unfortunately has suffered bleeds in the brain. Now we are getting married we are thinking about starting a family in the near future- currently going through genetic counselling , the future is unknown for my partner and also my future children. At a recent hospital consultation we were told of this drug being on the horizon , now to be told it may not be available is heartbreaking. I look at my family's future, and as a nurse Myself see patients suffering from this and beg that you reconsider this decision. Too many generationd have suffered I pray that it won't go on. Please please see the dramatic change this could make to thousands of families.
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Public
Job title	Store Manager
Location	England
Conflict	No
Disclosure	
Comments	560 Please could you reconsider your decision. My two sisters and two nephews suffer with this. You could could prolong their lives, save their pain and let them have a better way of life!
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Patient
Location	England
Conflict	n/a
Disclosure	
Comments	561 Polycystic Kidney Disease has a big impact on the lives of patients and their families, knowing that the patient's condition may deteriorate dramatically. The uncertainty is a constant worry in our lives. Tolvapton was greeted with great excitement within the community of medical staff and patients, followed by immense disappointment by the decision not to recommend the drug for use in APKD. We feel that a lifeline that was in our reach has been snatched from us and implore that the decision

	be reconsidered.
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	
Location	England
Conflict	No
Disclosure	
Comments	562 I have pkd and this drug would have saved my family from stress and heartache whilst caring for me. It may have helped me avoid having 2 heart attacks and costing the NHS money to trear those conditions. I may also have been able to contrubute to the economy longer by delaying my need for dialysis. Please reconsider and approve this drug.
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Public
Job title	Senior personal Finance Manager
Location	England
Conflict	
Disclosure	
Comments	563 This is very upsetting and worrying. My two sisters and nephew have this disease. This treatment will prolong their lives, improve their health too. I think it is a huge mistake and the decision needs to be looked into again.
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	PhD Student
Location	England
Conflict	No
Disclosure	
Comments	564 I watched my father die of PKD related illness. I saw him endure dialysis where he would frequently bleed out. I saw his suffering. Since my diagnosis, I have been doing everything I can to stop getting end stage failure, from monitoring my blood pressure to drinking 4 litres of water a day as this was the only thing my nephrologist could recommend. I understand that the NHS, which kept my father alive for 10 years, is financially stretched but the financial cost of dialysis and all the associated

	medication that goes with PKD is surely a huge cost too, not to mention the economic cost and the emotional suffering. I chose not to have a family based purely on my PKD. I could not forgive myself if I gave it to my child too knowing there is nothing to help them. Please reconsider your appraisal. Please look at the wider costs of this ticking time-bomb of a disease. Please help me and others like me.
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	HR Manager
Location	England
Conflict	No
Disclosure	
Comments	565 This was the only hope available for my family and other pkd patients and desperately needs to be reviewed and approved to save and improve lives. This new drug could help so many people suffering this disease and I desperately urge you to reconsider tolvaptan and please accept for administration in UK
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Finance Manager
Location	England
Conflict	No
Disclosure	
Comments	566 This drug could change the lives of many patients. By postponign the need for dialysis I may be able to work long enough to enure financial security for my family. As the major wage earner I need to keep working. Whilst possible to remain at work during dialysis it is nto going to be easy, will require more time off, will affect my performance on a daily basis and all in all I will be dependent on having a considerate employer. Failing this I will be dependant on State aid. Whatever this drug costs it must pay for itself compared with the costs of treatment for kidney failure and the loss of economically productive years for patients. I understand that there could be liver consequences, but not all patients experience them and nothing is lost if you have to stop, but there is a lot ot gain. This isn't a cure but it prolongs the healthy, independant and contributing years of patients lives. Even if I stay healthy for just a few more years then that time that I can be providing for my family and not needing external support.
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Retired GP and Patient
Location	Scotland
Conflict	
Disclosure	
Comments	567 I wanted to add my support for approval of Tolvaptan. As a retired GP, I suffer from ADPKD, and have been the recipient of a kidney transplant from my wife. My daughter has now been diagnosed with the condition. I want her to have the benefit of a medication that will improve her chances of preventing chronic renal failure in the future.
Submission date	24/06/2015

Name	[redacted]
Organisation	Simply Eyes
Role	NHS Professional
Job title	Optometrist
Location	England
Conflict	No
Disclosure	this illness is genetic one . I have not self induced it. I live in fear of becoing ill and letting down my family and colleagues. I am also worried about how I will pay my bills once I become unable to work
Comments	568 SInce being made aware I have this condition I am constantly worried about my future. I have taken all the steps in my power to improve my health . I am at the stage 3A where I believe this drug could be of great help to me live a normal life. My father died at 62 . I am now 48. I constantly worry about dying so young. I employ people and worry about who will take over when I am not able to work, I feel isolated and alone with this condition. I wish in a way I had never been informed about it as the knowlesge I have this relentless condition I can do nothing about makes me feel tearful and hopeless. I was really happy when I thought there may be something that could slow down the disease process as this gave me some hope whch has now been taken away again by your decision not to fund on a not being cost effective basis
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	
Location	England
Conflict	No
Disclosure	PKD runs in my family .... I want my daughter to have a chance

	of having a more normal life with the help of this new drug
Comments	569 Why is Tolvaptan not being used ? PKD is a life long disease , my family is suffering this terminal disease ... I can't work because of this !! my father and his sister died because of this !!! my daughter is suffering now and only just 16 years old , has missed 50% of her schooling the last 2 years ... her outlook is poor as she suffers kidney pain 24/7 ... this new drug could help her enjoy life while she is young ... Cancer !!!!! well they get all the funding all the drugs .. yet PKD .. has to fundraise to help pay for the research that has bought TOLVAPTAN to this stage ... other countries have start this drug to help people who suffer with this ..... if you had this !!! wouldn't you want to be able to have this drug ???? thanks for reading this
Submission date	24/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	
Location	England
Conflict	No
Disclosure	
Comments	570 Please reconsider approving Tolvapan to slow down the process of kidney failure in APKD. For myself it is probably too late, but for my 2 sons it can make a huge difference. I have known about my condition for nearly 30years, it is like living with a time-bomb, if my sons who know they have inherited the disease, knew there was a drug that could help them live a normal life for longer, they may not live the same time-bomb life that I have, please give them the opportunity, thank you.
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Carer
Job title	Wife, Mother and Grandmother
Location	Scotland
Conflict	No
Disclosure	My husband had 8 years of dialysis two nephrectomies and then a transplant..all possibly unnecessary if the cysts had been controlled during his earlier years. Daughters now at 50-60% function and kidneys at approx 20cms each....here we go again! Far more expensive for NHS in the long run!
Comments	571 Tolvaptan is the first real hope that our family has that may delay the need for dialysis, which will cost so much more than the pills. Please do take away our hopes unless you have another more effective treatment that is proving that it will be

	better. I have a husband. Two daughters and one granddaughter so far (3 undiagnosed yet). A brother in law, a niece and 2 nephews. This is such a big deal for our family.
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Carer
Job title	Psychotherapist
Location	England
Conflict	
Disclosure	husband died of PKD and two sons are sufferers.
Comments	572 Whatever the draw backs of this new treatment it is far far preferable to Dialysis which is a really difficult treatment and which creates depression sexual dysfunction isolation and dependency issues for all its patients. The issue of thrist is paradoxical as on dialysis the patient cannot drink... or eat what he likes... and the body suffers in many ways... and with transplants the poison of the rejection drugs and steroids are far worse than the Billirubin fears... AS far as the expense is concerned... unless the drug is used it cannot be developed and refined through time - so it should be allowed to be used in specific cases to begin with. To dismiss is very short sighted.
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Carer
Job title	Environment Consultant
Location	England
Conflict	No
Disclosure	
Comments	573 My 18 year old daughter has PKD, her kidneys are enlarged and covered in cysts, she has frequent infections. Her father has the condition, her grandfather died aged 47 of the condition. Please give her some hope. The trials show some success with Tolvaptan..what other hope or help does anyone with PKD have?
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Offshore Supervisor
Location	Scotland

Conflict	No
Disclosure	
Comments	574 I believe tolvaptan should be available in the uk for all patients with PKD
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Teacher of Religious Studies
Location	England
Conflict	No
Disclosure	
Comments	575 I think Tovaptan should be available to patients in the UK as it is in Canada.
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Commercial Manager
Location	England
Conflict	No
Disclosure	
Comments	576 I am very disappointed that NICE do not believe this drug will deliver a cost-effective benefit. We as a family believe there are major benefits, particularly for our children wo are at risk of developing ADPKD. We feel they should have the best medicines available for treatment of the early stages of the disease. Also, anything that has the chance of limiting the development of this disease should be considered especially given the costs of long term care and transplant treatment. Please will you reconsider?
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	
Location	
Conflict	No
Disclosure	
Comments	577 As an ADPKD patient, the possibility of treatment, for me, for my family, for any child I may one day have, brought a lot of hope. Tolvaptan may not be the perfect treatment but it is a start. Even if it only helps in certain cases it is worth authorising.

Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	IT Manager
Location	England
Conflict	No
Disclosure	
Comments	578 As a patient I find it disgraceful that you have decided to not allow the use of tolvaptan which would increase the my amount of "active" time before I need to start worrying about kidney dialysis/transplant and delay my eventual death to PKD. Why do I pay taxes?
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	
Location	
Conflict	No
Disclosure	
Comments	579 By not recommending Tolvaptan for the treatment of ADPKD NICE has removed any hope of a delaying treatment for this disease for thousands of people currently suffering with the associated symptoms of this disease. There does not seem to be a logical medical based reason for denial of this potential delaying treatment which therefore means the decision seems to be taken for financial reasons rather than benefits to patients. This is not right and will not improve the QOL for many patients. Currently there is no SOC for ADPKD and no treatment of the underlying disease. If Tolvaptan was to delay the need for dialysis or transplant surely that would be a good thing.
Submission date	25/06/2015

Name	[redacted]
Organisation	PKD
Role	Patient
Job title	Electrician
Location	England
Conflict	No
Disclosure	

Comments	580 As a Pkd sufferer, I was extremely disappointed when I heard that nice were not going to approve tolvaptan due to financial matters. My grandad had it who had go on dialysis for the remainder of his shortened life, my mother has it who was at a similar age to when he died, I have it and it's a strong possibility my kids may have it. It would mean so much to me as I worry about all our futures. Thanking you, please reconsider!
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Public
Job title	Information Systems Executive
Location	England
Conflict	No
Disclosure	
Comments	581 I have been closely following the Tolvaptan trials over the last few years, and was hopeful that NICE would endorse the product in the UK, thereby giving hope to current and future sufferers of ADPKD. As a sufferer myself, one who is nearing the end lifespan what such drugs as Ramipril, etc can do for me, and as an imminently expectant father to a child with potential ADPKD, it is a great disappointment that NICE has turned down Tolvaptan. I urge NICE to please reconsider their decision, and help those of us afflicted to have a better chance at achieving our hopes and dreams.
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Patient & Retired GP
Location	England
Conflict	No
Disclosure	
Comments	582 I am a recently retired GP, having had to give up work on health grounds. I have ADPKD, and have considerable personal knowledge of the effects of this disease, as my father and uncle were killed by the disease, and my brother and my two sons have the condition. The TOPIC 3:4 trial would suggest that tolvaptan has a marked benefit in reducing cyst development and slowing the decline in eGFR. This could mean many who now face a prospect of renal replacement therapy in later life might now never require dialysis. I worry about the signal you are sending out if you reject tolvaptan, as it would surely act as a disincentive to the pharmaceutical industry to invest further funds in the

	development of treatment for ADPKD. When statins were first developed, they were extremely expensive and I remember reading journal articles saying that these drugs should be rejected as they could bankrupt the NHS. However, the clinical case for them rapidly became overwhelming, and far from bankrupting the NHS, they have played a significant rÃ´le in reducing NHS spending on cardiac care, and the drugs are now a fraction of their initial cost. It is too late for me to benefit from tolvaptan, but it would offer my sons the possibility of a dialysis-free future.
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Public
Job title	Retired
Location	England
Conflict	No
Disclosure	
Comments	583 The impact on family and close friends is significant as prime earner`s income stops and lifestyle changes significantly as well as partner having to be main earner plus carer and mother to teenagers .
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Carer
Job title	Logistics Co-Ordinator
Location	England
Conflict	No
Disclosure	
Comments	584 Hi, I am the parent of a 12 year old girl, who has multiple cysts on both kidneys. She is monitored every 4 months at alder hey. BP and Blood tets and urine checked. Throughout her short life she had dealt with wetting issues, water infections, headaches dry skin, itchy skin but on the whole has been ok. We know however she is a ticking bomb and there isn't a day goes by where i dont question myself through guilt, worry about her future, worry about our familys future and the strain and struggle ahead. The constant news that there is no cure, no support, no medicines to slow cyst growth or help with pain relief is heartbreaking and really makes you feel helpless. So when tolvaptan was released this was the first bit of positive news i had heard since finding out about PKD. I was hopeful that at the very least this would slow cyst

	growth down and prolong my daughters kidney health. We are aware that she will need to undergo transplant at some point so my own life and close people tend to hold back socially to hopefully day be a donar and a healthy one at that. when required. The news that you have rejected this drug is another massive Kidney blow... and although you have your reasons, expense shouldn't be one of them. I hope you revisit your decision and maybe one day that one bit of positive news PKD patients seek will actually happen.
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Public
Job title	Student
Location	England
Conflict	No
Disclosure	
Comments	585 I am aware of the severe difficulties this disease has to those who suffer it, including persistent, increasing, chronic pain, and low life-expectancy. The impact on quality of life is significant and longstanding, particularly as this can develop at a young age. Tolvaptan is the only treatment available for this disease, it is treatable and there are no alternatives, as such, it is cruel and unjust to make it unavailable on the NHS. As this disease has a high chance of being passed on, surely younger generations to come are at an increasing risk of exposure to this disease - in which case we should be encouraging investment and research in to this area, rather than removing the incentive by deeming these sufferers as unworthy of treatment. This is precisely what NICE will be doing by removing access to the only treatment available.
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Parent of a PKD Sufferer
Job title	Housewife
Location	England
Conflict	n/a
Disclosure	
Comments	586 Please reconsider recommending Tolvaptan. I lost a beautiful sister to PKD my daughter also has this dreadful disease, I want my daughter to live a full and happy life with her son and any other children she may have in the future, we need some hope, Tolvaptan could possibly bring this hope. Please reconsider.
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Public
Job title	Retired
Location	England
Conflict	No
Disclosure	
Comments	587 I have advanced stage PKD and this drug would not benefit me in an way. However, my youngest son also has the disease And I would very much like that he had the opportunity to have a drug that can slow down the growth of cysts. He would then hopefully not have to have dialysis or a transplant. Therefore, please give this serious thought and think of the potential benefits of providing a better quality of life for this with this disease. Kind regards [redacted].
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	
Location	England
Conflict	No
Disclosure	
Comments	588 I am a sufferer of PKD which was passed down to me through my mother. My son has been confirmed with it and has 3 children who may have inherited this disease. They are too young to be tested yet. It is the most common kidney disease but has no treatment whatsoever at present. Please give families like us a chance to at least slow down the progression of this disease. It is too late to benefit me but could benefit my son and my grandchildren. I do not want them to suffer as my mother did and I am now. Please listen to us - we feel forgotten. Cancer gets all the headlines and treatment but there are a significant number of us too.
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	
Location	England
Conflict	n/a
Disclosure	
Comments	589 PKD has affected me with very large kidneys, severe and chronic flank pain which has now cost me my job. This treatment was a last hope to keep my existing function for as long as possible. If it reduced the pain as well this would have been an added bonus. I'm only 35 and shouldn't be considered

	for medical retirement.
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Data Analyst
Location	England
Conflict	n/a
Disclosure	
Comments	590 As a PKD sufferer there is currently no drug for us to slow down this horrible disease which affects us in many ways and limits lifestyle. Please support it to ease the suffering
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Retired
Location	England
Conflict	No
Disclosure	
Comments	591 As an ADPKD patient, I was dismayed that NICE has rejected Tolvaptan. I watched my mother and two of my aunts die from this disease, and nothing has radically changed in the treatment of it in 40 years. Tolvaptan is the light at the end of the tunnel for many sufferers who, like me, face the prospect of life on dialysis. Waiting for your kidneys to fail completely is living with a death sentence. It is stressful and depressing. Tolvaptan offers some hope to sufferers. Surely, savings would be made on not having so many dialysis patients, fewer transplants needed, and less frequent visits to clinics. I would urge you strongly to reconsider your decision and give hope to the thousands of patients whose quality of life could be improved.
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	
Job title	Patient
Location	England
Conflict	No
Disclosure	
Comments	592 I would like to ask you to carefully reconsider the decision not to recommend tolvaptan to treat ADPKD at the second appraisal meeting on 7 July in Manchester.

Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Director
Location	England
Conflict	No
Disclosure	my father, myself and my daughter suffer from PKD and this was the first hope of treatment ever.
Comments	593 I was devastated to read that NICE had initially rejected the drug Tolvaptan. Can you imagine that having know from an early age that my kidneys were deteriorating, following in my fathers footsteps onto a life of dialysis, that because of your decision my daughter is condemned to the same life? We knew that she had PKD before she was born as we saw the cysts on the untrasound. We also knew that like my father and myself, there was no drug or treatment that could stop or slow the growth of cysts until we heard of the successful trials or Tolvaptan. For a time we believed that maybe there was something that could halt the expectation of a life of dialysis and now that hope is removed by NICE's decision. Dialysis is cruel, it is not a treatment as there is no chance of getting better, it is a slow death sentence and there is no life while on dialysis. It is just a painful existence that many more will be condemned to unless the decision is reversed. Please go and see a dialysis unit before you reconsider on Tolvaptan and keep as many people off dialysis for as long as possible. Remember that the tablets are cheaper than dialysis and all the associated drugs
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Accountant
Location	England
Conflict	No
Disclosure	
Comments	594 I come from a large family who is extensively affected by PKD. My Father is one of 12 siblings - 6 of whom are affected and have all undergone kidney transplants as a result. This genetic condition has been passed down to many more family

	members, myself included who are all preparing to undergo transplants at some point in our lives as no other treatment is currently available. From the studies and trials that have taken place, this drug could help in delaying or even removing the need for a transplant for many people, greatly decreasing the strain on the NHS for treatment such as appointments, scans, blood tests, dialysis, surgery and a lifetime of aftercare following a transplant. To not authorise the use of this drug would be a shameful waste of years of research into the causes and treatments for this disease and could prevent thousands of people living relatively healthy lives without the need for transplants which are so few and far between. I hope and wish you will reconsider your decision. Kind regards [redacted]
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Patient
Location	England
Conflict	No
Disclosure	No
Comments	595 This disease had blighted my family for several generations. Any drug that can slow down the symptoms in my eyes is essential and NICE souls approve use of this drug immediately and across the board .
Submission date	25/06/2015

Name	[redacted]
Organisation	Patient
Role	
Job title	Senior Scientist
Location	England
Conflict	No
Disclosure	
Comments	596 PKD and its treatment by dialysis often causes difficulties for people in their working lives. I feel that the opportunity to start treatment which would slow the advance of the condition and hence delay the need to start dialysis till later in life would be beneficial. I would urge you to consider this appeal sympathetically. Thank you for your attention.
Submission date	25/06/2015

Name	[redacted]
Organisation	

Role	Patient
Job title	Patient and Nurse
Location	England
Conflict	No
Disclosure	
Comments	597 As a APKD patient I have been at stage 3 for the last ten years, however generally I have seen a massive decline in my health and my ability to manage everyday life over the last three years. I am a nurse and continue to work full time but this is at a cost of things I can do at home due to tiredness. I get very tired, I feel like I am carrying twins all the time, which affects my breathing and ability to eat a meal. I have had to give up exercising and am only just managing to continue walks. If I had been given Tolvaptan ten years ago things might be different for me now. I have lost a parent and one sister to APKD, my sister being only sixty. My brother has APKD as does one of my children, the other not yet scanned. Tolvaptan gives up hope to slow down a disease that hangs like a chain around patients necks. I note , forgive me if I missed this in your calculations you do not include costs of pain relief medications, vitamin D supplements (which are costly) and other medications which increase as symptoms of kidney failure progress. The cost to society should also be considered, in treating patients with a drug that may keep them living a decent life for a while you are also keeping us at work and contributing to everyday life including paying taxes. Tolvaptan provides a hope to keep up living normally for a while longer.
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Head of Educational Support
Location	England
Conflict	No
Disclosure	
Comments	598 I am writing on behalf of myself and my two daughters, [redacted] and [redacted]. My daughters have, unfortunately inherited Polycystic Kidney Disease with combined liver cysts, which I inherited from my father, who in turn, inherited this from my grandfather. Both of the latter died in their early 60s and the prognosis for myself seems to be following a similar route. I have been on blood pressure medication for over 20 years because of the associated hypertensive condition caused by poor kidney function. One of my daughters has already started along this route. It is disheartening to hear over and over again from all the experts in the field, that there is no cure - only transplant if you are lucky and it is successful, or dialysis, which is costly, life changing

	and shortens lifespan. I am told that the cut-off for transplant is 70 years and I am half hoping that my kidneys fail before this in the hope that I might qualify for a transplant. That is all with respect to my own future but needless to say, what concerns me far more is that the future should look brighter for my children and grandchildren. We come from a long line of teachers and have much to contribute therefore to future generations. Tolvaptan offered us some hope that the growth of the cysts could be halted or slowed so that our condition would not affect our longevity. I am sure you will have heard from the bodies (charities and research organisations) of the potential for Tolvaptan to change the prognosis for PKD sufferers and to provide a more economical alternative to the interventions currently available for us. It is a not inconsiderable proportion of the population who suffer from this disease and it seems shocking that there is currently no cure available in this country. I do hope that this personal account of living with PKD will go some way to ensuring that Tolvaptan is now made available for us.
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Public
Job title	
Location	Scotland
Conflict	No
Disclosure	
Comments	599 I have watched my wife decline from full of energy, always on the go and helping others to someone who is in constant pain and incredibly tired all the time. Making these drugs available on NHS would perhaps go a great way in helping this condition.
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	
Location	England
Conflict	No
Disclosure	
Comments	600 I am a patient with ADPKD, which has caused a chronic pain condition. I wondered if it would be helpful to describe some of the symptoms I suffer from so that you are provided with a picture of the impact it has on my day to day life. And also to give you an idea of why the possibility of the success of tolvaptan is so important.

	Symptoms: - Daily back pain, constant dull ache, infrequent stabbing pain - Inability to stand or walk for more than 20 mins - Frequent bouts of insomnia - Frequent need to urinate which is uncomfortable (like cystitis symptoms) caused by burst cysts - Frequent bouts of vomiting (sometimes 7 times a day) probably caused by the size of my kidneys - For info, my kidneys have excellent function and they measure 17cm, the biggest cyst measures 6cm
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Close Friend
Job title	
Location	Scotland
Conflict	No
Disclosure	
Comments	601 Please will your reconsider your decision on the above treatment. I have a dear friend who is only 45 years of age and who suffers from this debilitating illness. It could make a huge difference to her life and let her plan for a longer and hopefully improved future.
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Public
Job title	
Location	England
Conflict	No
Disclosure	
Comments	602 My father suffered from ADPKD although it was not diagnosed until he suffered kidney failure. His health and quality of life deteriorated significantly; he had to have dialysis in a hospital 3 times a week, and he was unable to play with his grandchildren as he would have liked. The combination of renal and heart problems meant he spent a lot of his last years in and out of hospital. Fortunately I have not inherited this disease but my sister has. NICE's decision not to recommend tolvaptan to treat ADPKD means that she loses the possibility of her kidney disease not getting worse, and avoiding end stage failure. If

	there's a chance that my sister, or anyone else with AKPKD, could avoid the experience of my father, I think it should be taken. I urge NICE to reconsider its decision. I am deeply disappointed that people living with this incurable, inherited, life-threatening and debilitating kidney disease may be denied access to a licensed therapy which has the potential to improve the quality of their lives, and the lives of their family members and future generations.
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	
Job title	Marketing Executive
Location	Scotland
Conflict	n/a
Disclosure	
Comments	603 As a patient of pkd and a carer or my child who also is a pkd patient I belive tolvaptan is a break though achievement for pkd suffers. Why would this not be recognised and allowed on the NH'S? My son was diagnosed when I was pregnant and it has been a horrendous 7 years with his health and myself in my late 30s this drug would help and aid pkd suffers throughout uk and reduce the rate of cysts growing on kidneys as seen in trials. Please reconsider ur desicion u would be helping the young and old with this new drug
Submission date	25/06/2015

Name	[redacted]
Organisation	Kidney Research UK
Role	Research Charity
Job title	[redacted]
Location	England
Conflict	No
Disclosure	
Comments	604 Kidney Research UK was concerned to learn that NICE has not recommended Tolvaptan (Jinarc) within its European marketing authorisation for treating ADPKD. Kidney Research UK supported the original submission made to NICE by the PKD Charity, who went to great lengths to gather patient experiences of ADPKD. We were also fully supportive of their nomination, and subsequent NICE acceptance of, our eminent colleague, Professor Albert Ong, as the clinical expert. We have reviewed the response made by the PKD Charity to your decision. We fully endorse their view. We were pleased to read that NICE have acknowledged the

	clinical benefits of the drug and that this technology is unique and first in class; there is no other current treatment which slows the progression of the disease. However, we are deeply disappointed that NICE have not fully appreciated the wider quality of life and economic benefits which would accrue from the deployment of the treatment. This decision denies hope to the families affected now and to future generations. However, we note that Otsuka have agreed a patient access scheme (PAS) with the Department of Health. In light of the response to this decision, surely now you should embark upon a renegotiation of the discount to get this technology into routine clinical use?
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Sales Manager
Location	England
Conflict	No
Disclosure	
Comments	605 Dear NICE, I am a PKD patient and have been living with the disease for 20 years. I do not understand why Tolvaptan is not being considered for usage in the UK, when it is available in other regions such as Canada. Surely a drug which can stop the growth of kidney cysts is a better solution than dialysis and transplatation and would be more cost effective in the long run? My mother died from kidney disease after transplantation and dialysis, which she endured for over 20 years. If a pill is available to replace all those hospital stays and consultant visits, then it has to be worth a trial?
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Beauty Therapist
Location	England
Conflict	No
Disclosure	
Comments	606 As a sufferer of PKD (all family and my children have also inherited it) there is nothing that can be done except to accept it and put up with it knowing your life will be shorter than the average. Surely if there is anything that can alleviate suffering and possibly lengthen life expectancy, then we should be

	allowed it. Knowing there is nothing that can be done is one of the hardest things to accept with PKD.
Submission date	25/06/2015

Name	[redacted] [redacted]
Organisation	
Role	Patient
Job title	Patient
Location	England
Conflict	No
Disclosure	
Comments	607 As there is currently no treatment for ADPKD sufferers this drug is an important first step in finding an appropriate treatment Please re consider your descision.
Submission date	25/06/2015

Name	[redacted] [redacted]
Organisation	
Role	Patient
Job title	
Location	England
Conflict	No
Disclosure	
Comments	608 From a patients view I am so upset that the hope of treatment for this condition has gone. My father died from it 14 years ago, I then found out that I have it, and now both my children have it. My daughter is only 15 and has high blood pressure and huge cysts with poor kidney function and my son is only 11 and has high blood pressure already. This treatment would have helped them to lead a better "more normal" life and they cannot have it. It is a very disapointing result
Submission date	25/06/2015

Name	[redacted] [redacted]
Organisation	
Role	Patient
Job title	
Location	England
Conflict	No
Disclosure	
Comments	609 I am so disappointed by the rejection of the use of Tolvaptan. Disappointed for the grandmother, uncle and mother I have lost to this wretched disease, disappointed for my 3 year old daughter [redacted] and disappointed for myself. Having been diagnosed at Christmas 2014 at the age of 33, the small window of hope provided by the research that is underway and

	by trials such as those involving Tolvaptan were the things that kept me going. Please rethink. My mother spent several years on expensive dialysis and her last 2 years were predominantly as an inpatient at QA hospital Portsmouth. What life? What expense? Please reconsider the economics and the lives.
Submission date	25/06/2015

Name	[redacted]
Organisation	National Kidney Federation
Role	Kidney Patient/Carer Support Charity
Job title	[redacted]
Location	England
Conflict	No
Disclosure	
Comments	610 Official Response to NICE Appraisal consultation document This submission is made by the National Kidney Federation (NKF) in response to the consultation by NICE of â€˜Kidney disease (autosomal dominant polycystic) â€“ Tolvaptan [ID652]. The NKF is the largest kidney patient charity in the UK, forms part of Kidney Charities Together (an alliance containing Kidney Research UK, British Kidney Patient Association, Polycystic Kidney Disease (PKD) Charity and the Kids Kidney Charity), has support of the All Party Parliamentary Kidney Group and works in close partnership with various NHS organisations (including NHS England and the Clinical Reference Groups for Renal Dialysis & Transplantation) and Associated Renal Industry to improve the quality, provision and equity of access of services/treatments for kidney patients. The NKF supports the interests of all kidney patients (and their carers/family) with Chronic Kidney Disease (CKD), those being treated with renal replacement therapy (RRT) or those conservatively managed. This response directly supports the evidence presented by the PKD Charity in their submission and is intended to compliment insight from other kidney charities including the BKPA and KRUK. The NKF is saddened and deeply frustrated both with the disappointing decision by the National Institute for Health and Care Excellence (NICE) not to recommend Tolvaptan for treating Autosomal Dominant Polycystic Kidney Disease (ADPKD) but also for the many patients, carers and family members for whom this drug promises such hope of a better quality of life and potentially improved life expectancy. The ability to slow the rate of progression of ADPKD for patients and family members is priceless; keeping well for longer will enable the ability to plan, prepare, educate, gain support and

	emotionally accept future treatment. Keeping well for longer will reduce the burden on the NHS financially and enable individuals to participate and contribute fully to life being engaged citizens. Each year of dialysis is estimated to cost between Â£25-30,000, while transplantation in year one has estimates ranging from Â£16-41,000. The potential to have less dialysis, drug burden, inpatient stays, reliance on patient transport and multi-disciplinary input through Tolvaptan will bring savings to the NHS. Staying in employment is widely regarded as both beneficial for physical as well as mental health and the NKF is acutely aware from reports to our Advocacy Service and attendees at our Annual Patient Conferences that once out of work financial support and a route back into employment is minimal with an ever decreasing cuts in benefits and support and variable access to counsellors, psychologists and renal social workers within Secondary Care and delays in accessing referrals to psychological therapy in Primary Care. Working age individuals with ADPKD have the potential with Tolvaptan to sustain their time in employment and make future preparations around work. This provides the potential to stay in work after starting RRT having which gives a health benefit, but importantly offers a financial and contributory benefit to the state. Preparation allows individuals to engage and understand key educational messages which in turn enable decisions and choices around future health care to be made in a timely rather than rushed manner. For many this means treatment options that meet ones needs that have been discussed with family members and health care professionals, which importantly allow space to discuss living donation. For individuals with a potentially long pathway of many decades, time and space at the beginning of the journey is the foundation to living well and keeping well for longer. The NKF note from the PKD Charity â€˜that Tolvaptan is not suited to every ADPKD patient at CKD stages 1 to 3 (itsâ€™ indicated use). This is a very broad group but within this cohort are the faster progressing, typically younger patients, with young families, struggling to cope with their jobs and daily lives, who live with fear of early ESRD and premature death. Such patients should, we feel, be considered for early access to Tolvaptan, as soon as possible.â€™ The NKF supports in particular the above stance and we add with authority and insight that this younger cohort often faces some of the greatest challenges in managing the physical demands of RRT; inherent complications, diet restrictions, drug burden; and associated risk burden from infection, cancer and

	steroid induced diabetes. We also note the significant burden and destructive effect on families where more than one individual is identified with ADPKD and requires treatment. Kidney patients, their carers and families rightly demand the best in access to health care treatment and services from the NHS in the UK today. In terms of progressive kidney disease that results in end stage kidney disease we know there is no cure, only treatment. The potential to stave off treatment is a massive advance for any kidney disease; in the case of ADKPD this is huge - Tolvaptan is acknowledged by NICE as an â€˜innovative treatmentâ€™ and the â€˜first shown to specifically impact on ADPKD progressionâ€™, targeting the disease and not just managing the symptoms and complications. Tolvaptan not only brings the obvious health benefits but the myriad of psychological, social, financial and personal. Therefore the proposed decision not to approve Tolvaptan is of major significance for the ADPKD community and we believe will have a potentially devastating impact for their care, quality of life and length of life. Whilst the NKF is mindful that the final guidance on whether to use Tolvaptan has not been made we strongly and respectfully urge the committee to reconsider their initial considerations and recommend Tolvaptan to address a recognised high unmet need and continuing disease burden.
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Carer
Job title	Childminder
Location	England
Conflict	No
Disclosure	
Comments	611 Having watched my husband die of ADPKD dialysis (10 years) and my two adult children have inherited the disease. How can I sit back and let this happen. As you are aware it costs the national health Â£30k a year to keep a patient on dyalisis. What is the point of keeping someone alive on a machine if and when a drug comes along you are to prepared to give it a chance. Please reconsider your disicion on Toluaptan for the future of the next generation.
Submission date	25/06/2015

Name	[redacted]
Organisation	

Role	Patient
Job title	
Location	England
Conflict	No
Disclosure	
Comments	612 I am a PKD patient, female aged 38. I suffer frequently with pain but my blood tests are currently normal. The family prognosis, what limited information I have, seems reasonable with members living into their 60s without the need for medical intervention. I seem to suffer more than family members and I would have liked have been able to make the decision to use Tolvaptan on the NHS. I understand that the increased water intake patients need to take with Tolvaptan could be throwing a false positive, but even this shows Tolvaptan, by making a patient drink more, has benefits to people like me. I urge to you reconsider. Don't cut my life short. I live a full and healthy one currently and I am not a burden on the NHS. If I suffer from renal failure later, surely I will cost much more.
Submission date	25/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	
Location	England
Conflict	No
Disclosure	
Comments	615 I would urge NICE to seriously consider making Tolvaptan available to PKD sufferers. As a PKD sufferer myself, I have had to watch my grandfather's quality of life slowly deteriorate as his kidney function decreased. There is not enough research done in this area and, now that a treatment has become available (Tolvaptan) it should be made available to sufferers. It is a glimmer of hope for sufferers who face life with an untreatable disease.
Submission date	26/06/2015

Name	[redacted]
Organisation	
Role	Public
Job title	Director
Location	England
Conflict	No
Disclosure	
Comments	616 The drug could be in reach if we where to build the NHS up, as a posed to the constant erosion by cuts, I would pay more tax for a better service.
Submission date	26/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Procurement Manager
Location	England
Conflict	No
Disclosure	
Comments	618 Dear Recipient/s I am 43 year old man who was diagnosed with AKPKD around 6 years ago and have had many symptoms associated with the disease for a number of years, which were not attributed to the disease until it was finally diagnosed, these being high blood pressure (which was picked up in a free heath check at my workplace) and gastric reflux (diagnosed by my GP around 10 years ago). I was only made aware of AKPKD by a family member, who suggested that I should ask my GP to investigate my symptoms further. I have always kept myself as fit as possible and my high blood pressure was initially put down to 'stress', even though I had repeatedly told my GP that I was not under any significant stress at all. Since being diagnosed, my life has somewhat been put on hold. I am lucky enough to have a good job and salary at present, but I am reluctant to progress my career and I have expressed to my wife that I we most likely will not move house again as I am very concerned that if my AKPKD progresses further, I will leave her with a high debt burden (due to me posssibly not being able to work) and I am unwilling to put my family in this situation. Although I have a daughter, I have also put on hold the possibility of having further children, due to the reasons given above. I have also found it a challenge to get certain insurances (e.g. life insurance) due to the inhibitive cost, when I declare my condition. The physical and emotional burden of living with AKPKD is significant, as it feels like living with a 'ticking time bomb', given that the chances of end stage renal failure are high. From a purely economic standpoint, I am a higher rate tax payer and in fact, the cost of providing this drug would be far less than the tax I pay every month on my income. In other words, if the drug allows me to continue to work and lead a relatively normal life, my net contribution would significantly outweigh the cost of the treatment. I recognise that this is not the case for all AKPKD patients though. I am somewhat surprised at the initial rejection of approval for JINARC, given that EMA approval was granted and approval granted in Canada too. I am certain that the economic cost to the NHS for treatment of patients with end stage renal failure is much higher than the cost of prescribing JINARC and the impact to patients, families and lives far higher too. I would urge you to reconsider the initial appraisal outcome and grant approval for JINARC as the positive impact of such a drug is huge, with delay to kidney failure and the ability for AKPKD patients to have 'hope' in living relatively normal lives again.

	Thank you & kind regards [redacted]
Submission date	26/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Patient
Location	England
Conflict	No
Disclosure	
Comments	619 I was devastated to read that NICE has not recommended tolvaptan to treat ADPKD. For nearly 13 years, I have followed the progress of tolvaptan through the early research to trials. I was unable to take part in the clinical trial owing to low kidney function but the promise it offered of a delay to dialysis and early death has made living with ADPKD more bearable. Although the drug comes too late to delay my journey to kidney failure and possible early death, it gave hope to a better life for my niece and nephew, and 2 great-nephews, who inherited ADPKD from my affected sisters. My father died at 57 of ADPKD and one of my sisters died 3 years ago at 59 of ADPKD. A younger sister has already had a transplant. I have lost 75% of my kidney function and am almost certain to need dialysis or a transplant, which will cut short my career and, as Iâ€™m self-employed, critically affect my finances. I have actually considered opting for no dialysis or transplant, ie death, rather than trying to live on minimal money and the daily burden of needles, pills and unhappiness. I know that the NHS has budgetary constraints but there is NOTHING else that can slow the invidious progression of ADPKD. The trial showed a significant reduction in the rate of kidney function decline. In the years leading up to complete kidney failure, patients suffer considerably and cost the NHS a lot of money - in pain relief, emergency admissions and antibiotics for cyst infections, and frequent hospital appointments. Then thereâ€™s the knock on cost impact on my working life - lost earnings, career impairment, higher insurance premiums or being denied any insurance. The psychological burden can be immense - genetic guilt and a form of grief, waking up each day knowing that I will never be free of ADPKD and each day is a day nearer dialysis or transplant. 'Standard care' for ADPKD patients in the NHS is around symptom management of kidney related problems. But ADPKD is more than a kidney disease; it is not amenable to traditional CKD care and it carries with it higher risk of aneurysms, hugely cystic livers (that sometimes need transplanting as well as kidneys), acute and chronic pain from infections as well as CVD

	from hypertension. Tolvaptan is the first therapy shown to slow down this rotten disease and I urge NICE to authorise this drug.
Submission date	26/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	PKD Patient
Location	England
Conflict	No
Disclosure	
Comments	620 It is essential that Tolvaptan is given as an option for PKD patients. I have had a grandfather and father that have died of this disease. I have a brother and sister and we have all spent time on dialysis. I looked after myself as well as possible following all the guidelines and being monitored by the NHS for some 35 years and still I succumbed to the disease. It must have cost a lot to monitor me for such a period then I believe about Â£25-30k pa to dialyse plush the cost of all the drugs. I have been in intense pain for about 8 years through the cysts. I lost my business with a turnover of Â£1.5m and the jobs of 20 people because I was unfit to work. I now live off the State rather than pay in to it. I also lost my wife who couldn't cope with my illness. I have twin boys and my sister also has twins. We feel that a drug that prevents the cysts forming provides them with a greater chance of a full life and it must therefore cost the UK less in the long run. Please reconsider this application and prevent this suffering.
Submission date	26/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	
Location	Scotland
Conflict	No
Disclosure	
Comments	621 As a patient suffering from ADPKD and a suitable candidate for the new drug Tolvaptan I am extremely disappointed to hear that the NICE are not to recommend Tolvaptan to treat the condition. This drug would give hope to people like myself and their families who deal with the incurable condition on a daily basis. My kidney function is OK, but I have chronic pain which at times is acute - I recently suffered burst cysts from which the fluid went into my pancreatic cavity. This was debilitating for a number of days and took several weeks to recover from. The

	only 'treatment' was pain relief - most are inadequate and so to be pain free I used strong prescribed medication, which have its own side effects. I also have the additional complications of hypertension an increased risk of brain aneurysms (my grandfather died from one) and polycystic liver disease. I used to be very active - 6 years ago I competed in a mini triathlon - but now find it difficult to participate in any activity as I become very breathless. This concerns me on an emotional level as I have an active family and can no longer join them, but I am also worried that it will have a financial impact if I am unable to continue working (I am already denied life insurance and was unable to get a mortgage which places a great burden on my husband). Surely the cost associated with long term dialysis that I am likely to face (my mother was on dialysis for 17 years and my brother is on dialysis after having both kidneys removed) must be worth an alternative treatment being sought? I am extremely disappointed that I, and many other like myself, are to be denied access to a licensed therapy which has the potential to improve the quality of our lives, but also the lives of our family members and those of future generations. [redacted] [redacted]
Submission date	26/06/2015

Name	[redacted] [redacted]
Organisation	British Kidney Patient Association (BKPA)
Role	[redacted] of national charity and kidney patient
Job title	[redacted] [redacted]
Location	England
Conflict	No
Disclosure	
Comments	622 The British Kidney Patient Association (BKPA) notes the feedback from the NICE Evidence Review Group and kidney patients with ADPKD in making these comments to NICE for further consideration in its review of tolvaptan. While NICE has concluded that a) this is a drug with some utility for people with ADPKD but that b) the evidence it has seen has not given it sufficient confidence to justify its authorization the BKPA would like to ask it to consider approval of the drug under some well-defined conditions. This would enable the collection of further evidence in a UK population whilst also enabling people with ADPKD to choose to undergo tolvaptan treatment with full support from their healthcare professionals. There is an excellent example of

	innovative commissioning and enabling choice in the NHS decision to commission TAVI devices for aortic valve replacement http://www.england.nhs.uk/wp-content/uploads/2013/04/a09-p-a.pdf We should like to ask whether NICE would consider making such an approach. Furthermore we would like to ask whether NICE has given full consideration to the social, health and economic gains to be made by delaying development of Chronic Kidney Disease versus the quality of life, increased mortality and economic burden of dialysis? Dialysis costs are estimated as about Â£33,000 pa by NHSBT and there is evidence that people with ADPKD will live longer on dialysis, thereby increasing the lifetime costs of this treatment vs the cost of a disease-modifying drug. While transplantation is a good option it is a scarce resource with a long waiting list and about 1 kidney patient a day dies whilst they wait. For two thirds of those on dialysis their further complications will mean that they are unable to move to the transplant waiting list anyway.
Submission date	06/06/2015

Name	[redacted]
Organisation	
Role	Carer
Job title	Retired
Location	England
Conflict	No
Disclosure	
Comments	623 My wife suffers from ADPKD. Her father died of PKD related illness and was in great pain for the last 5 years of his life. I do not want to see this happen to my wife. ADPKD is not a lifestyle related disease but in my wife's case, hereditary. She is doing everything she can do keep herself healthy, from eating, exercise, keeping her blood pressure under control and drinking 4 litres of water a day. We both feel powerless over this disease. Tolvaptan is the only medication available - it's not as if patients have a choice of which to take - and NICE's decision to reject it is removing her only hope of not getting to end stage kidney failure. She is lucky that she is not in pain but she is exhausted and is unable to work. We have ruled out having a family because of this disease. If this drug were made available we may have a second chance of having children. She gets depressed and tearful, especially when she remembers how her father struggled. I strongly urge NICE to reconsider urgently - it really is a case of life or death.

Submission date	26/06/2015

Name	[redacted]
Organisation	
Role	Public
Job title	
Location	
Conflict	No
Disclosure	
Comments	624 ADPKD does not currently have any standard treatment. There are many symptoms that could be delayed if there was an approved treatment available to delay the onset of symptoms. Tolvaptan, as proposed, does not seem to have any significant medical drawbacks to prevent its approval. Therefore denial of its approval seems to be based on short-term financial reasons rather than potential improvements of QOL for thousands of patients currently suffering. In my view the use of Tolvaptan would be more cost effective, reducing the need for as much costly dialysis and transplants ultimately combined with a reduced need for costly subsequent post-transplant associated medication.
Submission date	26/06/2015

Name	[redacted]
Organisation	
Role	Local government professional
Job title	Trainee Teacher
Location	England
Conflict	No
Disclosure	
Comments	625 This is so important and should be made legal and available for PKD sufferers!!
Submission date	26/06/2015

Name	[redacted]
Organisation	
Role	Carer
Job title	
Location	Scotland
Conflict	No
Disclosure	
Comments	626 As the partner of an ADPKD patient, I am disappointed to see that that NICE have not recommended that Tolvaptan be used to treat this condition. Over the last three years, my wife's lifestyle has been greatly affected through weight gain (approximately 15KG), breathlessness and general severe abdominal discomfort and

	she is no longer the active person she used to be (she competed in a triathlon 6 years ago). Until her kidney function is sufficiently reduced that she requires dialysis, there is currently little effective treatment available to ease her discomfort. Transplant, apparently, is not an option while her kidneys have sufficient function. She has read that she might be a candidate for the trialling of this drug given her current kidney function. To have this option removed before the drug has even been trialled is a grave disappointment. Her mother was on dialysis for 17 years due to the same condition. Her younger brother recently had both kidneys removed, has been on dialysis since and is awaiting a transplant with little sign of one forthcoming. Surely the costs associated with long term dialysis that both he and my wife potentially face must be worth an alternative treatment being sought? I trust that these thoughts will be taken into consideration before the final recommendation is made.
Submission date	26/06/2015

Name	[redacted]
Organisation	
Role	Public
Job title	
Location	England
Conflict	No
Disclosure	
Comments	To whom it may concern (nice@nice.org.uk) :- I am imploring you to recommend TOLVAPTAN to treat ADPKD. My daughter age 45 has suffered for many years with this disease. She has already had a liver transplant because of it and now her kidneys are only working at 40%. She has 4 children, 3 of whom are also confirmed sufferers of ADPKD. Transplants are inevitable for all of them at some point in their lives, but surely if that process can be delayed for as long as possible, then what is a traumatic experience for them and extremely expensive procedure for the NHS, must be the sensible way to go. My point is that surely this drug could save the NHS money! I hope and pray you will listen to my concerns and the concerns of many others whose lives are blighted by this dreadful disease. Many thanks,

	[redacted]
Submission date	26/06/2015

Name	[redacted]
Organisation	
Role	Patient
Job title	Public – Patient
Location	England
Conflict	n/a
Disclosure	
Comments	628 I wish for NICE to consider approving Tolvaptan. PKD is a disease that affects whole families . I have the genetic disease, as did my father and his mother, my grandmother, before him. One of my children has also inherited the disease and it is my son for whom I hope for a cure or, at least a slower progression of cysts that cause the kidney failure. Please do not take that hope away.
Submission date	26/06/2015

Name	[redacted]
Organisation	Secondary and sixth form school
Role	
Job title	Teacher
Location	England
Conflict	No
Disclosure	
Comments	629 Sirs/Madams, I understand that your appraisal has concluded in you thinking that Tolvaptan is not costly effective in UK. Please I would like you to consider that for every person who can delay his/her dialisys treatment it is a huge saving for the tax payer. People with ADPKD start dialisy before they reach 60. When we go to dialisy we can't work full time, so we need benefits. If Tolvaptan delays the start of dialisy for patiens because the cysts are better controlled, many patients may start dialisy by the time they have to retire, so no extra benefits will be given to them. Can I also suggest to try it for, let's say 5 to 10 years and then revise again the results. Also, I would like to say that, I think, many particularindividuals give personal donations to continue investigate this terrible medical condition we have without having done anything wrong

	in our lifestyle, as it is inheritated. I think it is only fair to give us some hope after all our efforts. I have not sais anything for myself, as I am afraid it would be too late for me. I don't think they would give me the treatment at the advanced stage I am now, but please consider all those points. [redacted]
Submission date	26/06/2015

Name	[redacted]
Organisation	
Role	Public
Job title	
Location	England
Conflict	N/a
Disclosure	
Comments	630 I am deeply disappointed that this drug has not been approved. My sister is very ill with this disease and I was hoping for anything that might help in anyway towards a cure. Please can this be reconsidered?
Submission date	26/06/2015