RESPONSE TO NICE APPRAISAL BY SIMONE GOREN – PATIENT EXPERT

I would like to express my deep disappointment at the NICE committee's decision not to recommend Tolvaptan (JINARC). This has caused myself and my family deep distress. As this was the only treatment currently available for treating the incurable, genetic disease ADPKD.

Whilst I understand the decision was a complex one when looking at the economic model. What I would like you to keep in mind is the human aspect to this decision. Having watched [redacted] health deteriorate as a result of this awful disease. Then watch him go through kidney transplant and all that entails both prior to the operation and seeing the ongoing struggles he faces. (Which included [redacted] having to donate her own kidney to allow him to have some quality of life). The alternative of taking medication which causes increased thirst seems an easy decision. Were it not for the fact that I am fortunate enough to be on the drug trial for Tolvaptan, then in reality I would be faced with my own health seriously declining within the next 10 years This would have not only a large impact on my own life, and family life, but also mine and my families' financial future.

I am one of the lucky ones, I have been on this medication for the past 5 years and I believe as a direct result of this my kidney function and creatine has remained stable. Although I cannot know what it would have been without it I am sure my health would have been adversely affected.

As far as I am aware there is no evidence to support the ERG's comment that "increased fluid intake …. May have an impact on decline in renal function'. Additionally I am confident that, the amount of fluid that we drink each and every day could not be replicated by someone not on this medication. For us drink is a necessity and much as someone would want to drink such large quantities I feel it would be impossible to sustain. We feel an undeniable thirst which must be quenched regardless of where or what else is happening. It wakes us in the night and means that we always have water available to us where ever we are. We will get off trains to get a drink, have to pull over to buy extra water if stuck in traffic even if this means we will be late for or miss meetings in order to quench our thirst. We panic if our water bottle goes missing or gets taken away at airports. I do not accept that someone would be able to tolerate this level of fluid intake without being on the drug. I believe I was on the placebo for the first two years of the trial and much as I wanted to drink a great deal I never drank more than 3-4 litres a day. Whereas now on the active drug I drink between 6-8 litres every day wherever and whatever I am doing.

As a result of your decision, other members of my family and the wider ADPKD community do not have the option to receive this potentially life changing drug. The future of my children and their children seems to be very uncertain, with no other possible treatments in the pipeline.

Whilst I accept that JINARC/Tolvaptan is far from perfect, I truly believe it is an excellent option for ADPKD patients and urge you to reconsider your decision.