



Effective Health Care

Polycystic Kidney and Liver Disease and Aneurysms Nomination Summary Document

Results of Topic Selection Process & Next Steps

- The Topic *Polycystic Kidney and Liver Disease and Aneurysms* was addressed by a 2015 Cochrane systematic review titled *Interventions for preventing the progression of autosomal dominant polycystic kidney disease*. Given that the review covers this nomination, no further activity will be undertaken on this topic.
 - Bolognani D, Palmer SC, Ruospo M, et al. Interventions for preventing the progression of autosomal dominant polycystic kidney disease. *Cochrane Database Syst Rev* 2015; 7:Cd010294. Available at: <http://dx.doi.org/10.1002/14651858.CD010294.pub2>.

Topic Description

Nominator(s): Individual

Nomination Summary: The nominator is interested in the effectiveness of interventions aimed at slowing the progression of polycystic kidney disease (PKD), a slowly progressive genetic disorder from which the nominator and his or her family members suffer. Currently, there is no broadly accepted treatment effective in slowing the progression of or curing the disease and treatments are limited to those that manage symptoms and complications. The nominator also appears to be interested in the risk factors for and interventions effective in reducing risk of developing a cerebral aneurysm, which is higher in patients with PKD than in patients without the disease.

The nominator also appears to be interested in increased awareness of the disease to help patients who could possibly be affected by the disease and/or are showing symptoms. Since the disease is a genetic disorder, those who have someone in their family with the disease are at risk. Low income families may also be at increased risk of inappropriate care due to inadequate health insurance and, according to the nominator, clauses regarding pre-existing conditions.

Staff-Generated PICO

Population(s): Adult men and women with autosomal dominant PKD (ADPKD)

Intervention(s): Medication or other interventions

Comparator(s): Other medication, other interventions, placebo, or usual care

Outcome(s): Disease progression (as assessed by renal function and size, complications, or patient-reported outcomes), reduction in cerebral aneurysms

Key Questions 1. In patients with polycystic kidney and liver disease, what is the risk of developing life

from Nominator: threatening cerebral aneurysms?

2. What interventions can be used to prevent the progression of the debilitating and painful cysts that develop in polycystic liver and kidney disease and to reduce the risk of developing a cerebral aneurysm?

The first key question is a question regarding prevalence of complications. It is not a comparative effectiveness question, therefore the focus of our search was on Key Question 2.

Considerations

- The topic meets EHC Program importance criteria. (For more information, see <http://effectivehealthcare.ahrq.gov/index.cfm/submit-a-suggestion-for-research/how-are-research-topics-chosen/>.)
- This topic is important because of the burden of disease and uncertainty about effective treatments for ADPKD. Current treatment targets complications, rather than aiming to reverse, stop, or slow kidney function decline and ADPKD progression. Presently, there are no drugs approved by the US Food and Drug Administration for use in slowing the progression of ADPKD.
- Topic was found to be addressed by a 2015 Cochrane systematic review titled *Interventions for preventing the progression of autosomal dominant polycystic kidney disease*. This review examined evidence for interventions aimed at preventing the progression of ADPKD in adults or children. In general, the review found limited evidence about the benefit of currently available treatment options. There seemed to be potential for harm due to adverse events based on limited evidence. The review did not report on interventions for the prevention of cerebral aneurysm. The objectives of the review were to evaluate:
 - the effects of interventions to prevent progression of ADPKD as measured by kidney function (glomerular filtration rate, serum creatinine [SCr]), doubling of SCr concentration, proteinuria or urinary albumin excretion) and clinical endpoints (end stage kidney disease, need for renal replacement treatment)
 - the effects of those interventions on kidney structure (total kidney volume, parenchymal volume, and kidney cyst volume)
 - the effects of those interventions on patient-centered endpoints such as incidence of fatal and nonfatal cardiovascular events, sudden death, all-cause mortality, hospitalizations, blood pressure control, quality of life, and kidney pain
 - general and specific adverse effects related to those interventions such as dizziness, diarrhea, (vasopressin receptor 2 antagonists); angioedema and infections (mammalian target of rapamycin inhibitors); alopecia (somatostatin agonists); and hyperkalemia (angiotensin-converting enzyme inhibitors, angiotensin receptor blockers).