

The Brussels Declaration on ADPKD

Autosomal dominant polycystic kidney disease (ADPKD) is a chronic, progressive, inherited disease in which cysts grow in the kidneys and other organs.¹ ADPKD is one of the most common life-threatening inherited diseases and is a leading cause of kidney failure. It is a complex and incurable disease that has a diverse and often profound physical and psychological impact on affected patients and families. It also confers high healthcare costs due in particular to dialysis and transplantation. The impact of ADPKD is often underestimated by healthcare professionals and other stakeholders.

ADPKD presents a unique combination of challenges warranting a specific response from healthcare policymakers and providers. The Brussels Declaration on ADPKD, issued by the European ADPKD Forum (EAF), offers the following policy-focused recommendations to help address these unmet needs and to promote access to high-quality care for all patients with ADPKD in Europe.

Recommendation 1: *Governments should support the development of a nationally co-ordinated, tiered approach to ADPKD care in collaboration with experts, patient organisations and other stakeholders.*

Patients with ADPKD need specialist diagnostic, therapeutic and preventative healthcare from various healthcare professionals throughout their lives. All patients should have access to an ADPKD specialist nephrology centre, where multidisciplinary, patient-centred care can be provided according to clinical need. Co-ordinated specialist care is likely to improve the efficiency of healthcare provision and a clear referral process will be important.

Recommendation 2: *An expanded European network of ADPKD reference centres would facilitate further research and the establishment of harmonised, integrated, patient-centred care pathways.*

Recognising existing achievements,² we support a continued dialogue between all stakeholders towards the further development of a network of European ADPKD reference centres to facilitate co-ordinated research and the development and implementation of best practice.

Recommendation 3: *The European Commission and national governments should support research to develop disease-modifying treatments for ADPKD with the potential to maintain quality of life, delay renal decline and improve life expectancy among patients, and to reduce the economic impact on healthcare systems.*

Recommendation 4: *Governments and healthcare providers should support the implementation of methods to routinely assess prognosis in patients with ADPKD to inform clinical decision-making, research and innovation.*

Recommendation 5: *All stakeholders, including the European Commission, national governments and healthcare providers, should support efforts to better inform individual patients and families affected by ADPKD, and look to involve patient organisations in policy making regarding healthcare planning and delivery related to ADPKD.*

Recommendation 6: *Health technology assessment (HTA) organisations should seek to engage patients and patient organisations in assessments to provide patients' unique knowledge about the impact of living with ADPKD, and their aspirations for new treatments, according to the HTA International Quality Standards for Patient Involvement in HTA.*

What is ADPKD?

ADPKD is a complex disease in which fluid-filled sacs, or cysts, grow and proliferate in the kidneys and other organs, notably the liver.¹ The kidney can become many times larger than in healthy people and patients experience various symptoms, including pain and bleeding and infections within cysts. Eventually kidney failure occurs in most patients. Importantly, patients with ADPKD are also prone to high blood pressure and cardiovascular disease.³ The disease can be diagnosed in both adults and children.

There is no approved treatment for ADPKD itself. Certain measures are often taken to try to slow the growth of cysts, but none has been proven to work. Patients often need treatment for pain and other disease manifestations and complications,¹ together with medicines to help prevent cardiovascular disease.³ However, there are no evidence-based, European-level guidelines. Ultimately most patients require either a kidney transplant or dialysis, on average before the age of 60 years.^{4,5}

How does ADPKD affect patients?

ADPKD has lifelong physical and psychological effects that can impair quality of life and wellbeing. Even at the early stages, most patients experience symptoms that interfere with work and physical activity or exercise.⁶ ADPKD can have a profound emotional impact, in terms of loss, uncertainty and fear,⁶ and this can be associated with anxiety or depression. ADPKD can detrimentally affect various other aspects of life, including employment, the obtainment of health or life insurance or mortgages, and family planning.

The impact of ADPKD on affected patients and families is often underestimated by healthcare professionals and other stakeholders.

What does ADPKD cost?

Patients with ADPKD incur healthcare costs throughout life due to outpatient care and hospitalisations. Costs

“ADPKD is a condition that my employers don’t seem to understand. It’s been quite frustrating trying to explain the condition and the effects it’s had on me... the tiredness and especially the amount of pain I’ve had. Even when dealing with doctors at the emergency department it can be a bit difficult... a lot of them haven’t even heard of polycystic kidney disease since medical school.”

Justin, UK

rise precipitously when patients need dialysis or transplantation.⁷ ADPKD accounts for around one in 10 patients needing these treatments Across Europe; this represents approximately 50,000 patients at a cost of €1.5 billion/year.⁸

Research on the prevention of ADPKD-related complications could offer a ‘tremendous return on investment’.² Transplantation is highly cost-effective compared with dialysis⁸ and investments to increase transplantation rates and reduce waiting times are expected to be cost-saving.

Unmet needs — why do we need the Brussels Declaration?

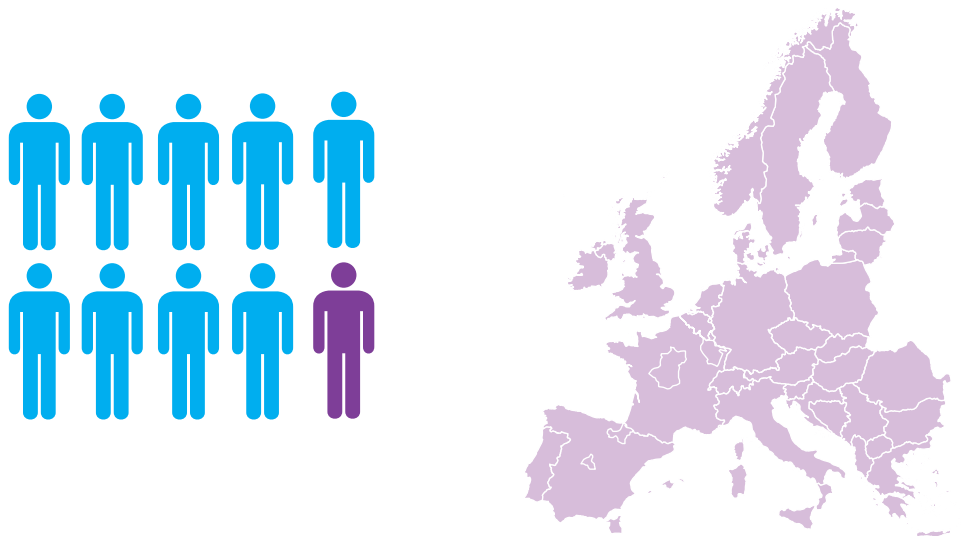
Unmet needs in ADPKD care, identified in the recent Kidney Disease: Improving Global Outcomes (KDIGO) Controversies Conference Report on ADPKD⁹ and by the EAF, include:

- All patients with ADPKD should have access to a nephrologist knowledgeable about the disease. Patterns of clinical practice for ADPKD care vary within and between European countries and so greater co-ordination of care policies and services is required.
- The optimisation and standardisation of ADPKD care in Europe is hampered by the lack of evidence-based consensus guidelines and standardised care pathways.
- There is no consensus yet on the optimal way to predict prognosis. Ongoing research aims to establish the best models to help identify patients whose disease is likely to progress rapidly and hence to allow care to be individualised.
- There is an urgent need for new medicines that delay the decline in kidney function due to ADPKD, thereby maintaining quality of life and improving life expectancy among patients and reducing the impact on European health systems.
- Further efforts to promote kidney transplantation for patients with kidney failure are necessary.

“What I fear the most is to see my daughter going on dialysis one day. Dialysis represents death for me in a way because my father died on dialysis and this is obviously a fear that everybody who has ADPKD has.”

Corinne, France

ADPKD accounts for **1 in 10** patients needing kidney dialysis or transplantation



= 50,000 in Europe → **€1.5 billion/year**

- Patients and families affected by ADPKD need specific, comprehensive, accessible information about their disease to enable to fully participate in decision-making. Furthermore, patients need to be supported to fulfil important roles in driving improvements in ADPKD diagnosis and care in partnership with healthcare professionals, researchers, healthcare system managers and health ministries.

Next steps: the Brussels Declaration on ADPKD

The Brussels Declaration offers strategies to help overcome these unmet needs. Implementing these changes will require national and international collaboration between all stakeholders in ADPKD care, including:

- Patients and their representative organisations
- Nephrologists and other specialist physicians and scientists involved in ADPKD care
- Healthcare system managers
- National government health ministries
- Bodies responsible for medicines regulation and healthcare technology assessment.

The EAF intends to facilitate dialogue and collaboration between these groups and looks forward to working with all bodies to improve and lengthen the lives of patients with ADPKD.

References

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“ADPKD is like living on a knife-edge, on the edge of a precipice, and you’re walking towards it and one day you know you will fall down it. Healthcare professionals and the wider public are not sufficiently aware of ADPKD and this must be urgently addressed.”
Tess, UK

The EAF is a multidisciplinary, international faculty of experts dedicated to improving the health and quality of life of people with ADPKD.

For further information, please refer to the EAF Report, "Translating science into policy to improve ADPKD care in Europe", available online at www.pkdinternational.org/EAF_ADPKD_Policy_Report_2015.

For printed copies of the Brussels Declaration on ADPKD and the EAF Report, please contact Laure.Sonnier@interelgroup.com.

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