Patient engagement in ADPKD and ARPKD: The role of patient organisations

Leuven, 2 December 2017
Tess Harris, President PKD International, CEO PKD Charity UK
ADPKD Patient
PKD patient organisations aim to represent the **views** and **voices of** ADPKD and ARPKD patients and families - who might not be able to speak for themselves.
What we do

Policy

Research

Activities

Peer Support

Capacity Building
We help policy-makers understand the experience and burden of living with PKD — in Health Technology Assessments for tolvaptan.

We promote the interests of PKD patients and families at every stage of health policy development.

We input to advisory groups, expert panels, European and/or national government public consultations or institutional meetings.
Autosomal dominant polycystic kidney disease (ADPKD) is a chronic, progressive, inherited disease in which cysts grow in the kidneys and other organs. 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 preventive 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, 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 and regional 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 progress 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 (IHA) 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 IHA International Quality Standards for Patient Involvement in IHA.

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 ministers
- 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


“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.”

Fesus, UK
European Reference Networks - ERNs

ERKNet
The European Rare Kidney Disease Reference Network

FEDERG

ERN RARE LIVER
We help identify research priorities that are meaningful to patients.

We rank core outcomes for clinical trials, eg SONG-PKD.

We encourage and support data collection through registries.

We engage with industry to help ensure that therapies are designed to meet the patients’ needs and priorities.

We help researchers engage with patients & provide access.
Peer support

- We deliver peer mentoring, advisory or ‘listening’ services, legal & financial support.
- We collaborate with health professionals to co-deliver self-management education.
- We connect our often-isolated PKD patient and family communities:
  - Information & support events
  - Moderated online forums (Facebook)
  - Meetups
Capacity building & education

- We promote health literacy to help patients feel more confident in making health care decisions for themselves.
- We produce patient-friendly, health-related information which is up to date, high quality and accessible.
- We educate healthcare professionals and industry about PKD.
- We try to help all PKD patient groups become more resilient, sustainable and effective.
Challenges to engagement

- Lack of resources and funding.
- Tradition of ‘tokenism’ towards patient groups.
- Ignorance about credibility, role and value of our contribution.
- Late involvement.
How can you engage with us?
Contact me!

With my background and genetics you guys should be happy I am half as normal as I am.

tess.harris@pkdinternational.org

@pkdcharity
@pkd_int
@CiliaAlliance
@Elektra