AAKP
American Association
of Kidney Patients
aakp.org

DECADE OF THE KIDNEY™
AAKP 2020-2030
AMERICAN ASSOCIATION OF KIDNEY PATIENTS

MDUFA V:
Patient & Consumer Perspective

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American Association of Kidney Patients

PERSONAL BACKGROUND

- 40-year kidney patient: 3 yrs. home dialysis; 23 yrs. transplant
- Chair of Policy & Global Affairs, American Association of Kidney Patients (AAKP)
- Chair, FDA Patient Engagement Advisory Committee (PEAC)
- Board, Kidney Health Initiative
- External Expert Panel, NIH Kidney Precision Medicine Program
- Nephrology Specialty Board, American Board of Internal Medicine (ABIM)
- Patient Editor, Clinical Journal American Society of Nephrology
- AAKP is a member of the FDA Patient & Caregiver Connection
- Policy positions under 4 U.S. presidents, 3 Virginia Governors

NATIONAL TERRAIN: BEFORE PPI/PRO/RWE

- **Random** patient preference insight/outcome data harvesting & collection.
- Driven by select individual, government or company interests & among researchers and professionals.
- Sparse infrastructure, systems or **policy frameworks** to utilize or apply data.
- **In the kidney space, patient insights & experiences were rarely tapped for discovery/useability/clinical studies**
- ***Lobbyists*** "at the table" often spoke to patient experience or industry view patient insights of "quality" and "safety"
- **Patients felt ignored, dependent & experience lacked value.**
- **Advocates FOUGHT to raise issues absent clear pathways.**

IMPACT: PATIENTS & PROFESSIONALS

- Every patient has aspirations & goals. Beyond clinical data, therapies often advance/interrupt these in unknown ways. PROs record ***why and how.***
- Validate individual patient & population experiences as ***meaningful*** data, especially those from outside clinical setting.
- Patients & their professionals ***both know*** products can be improved by formal application/incorporation of practical insights.
- Idealism: chronic & rare disease patients know their lives matter – **doctors are partnering more to include them in research to benefit others. This is changing nomenclature and engagement.**

IMPACT: GOVERNMENT & INDUSTRY

GOVERNMENT & INDUSTRY

- Anchors “patient-centered” rhetoric and intent in demonstrable tools & evidence collection – **patients are at the table.**
- **Formalizes policy & process** for applying insights to craft therapies based on substantive patient consumer engagement and insights.
- Drives pro-active patient consumer involvement at the ***front-end of research & product development lifecycle*** vs. “in reaction to.” **Creates better products.**
- Fresh source of evidence & data for regulatory and payment deliberations and decisions, ***informed by*** patients.
- Demonstrates that Federal appointed & career officials ***are responsive to and value patients as a matter of policy***, not merely “feel good” outreach.

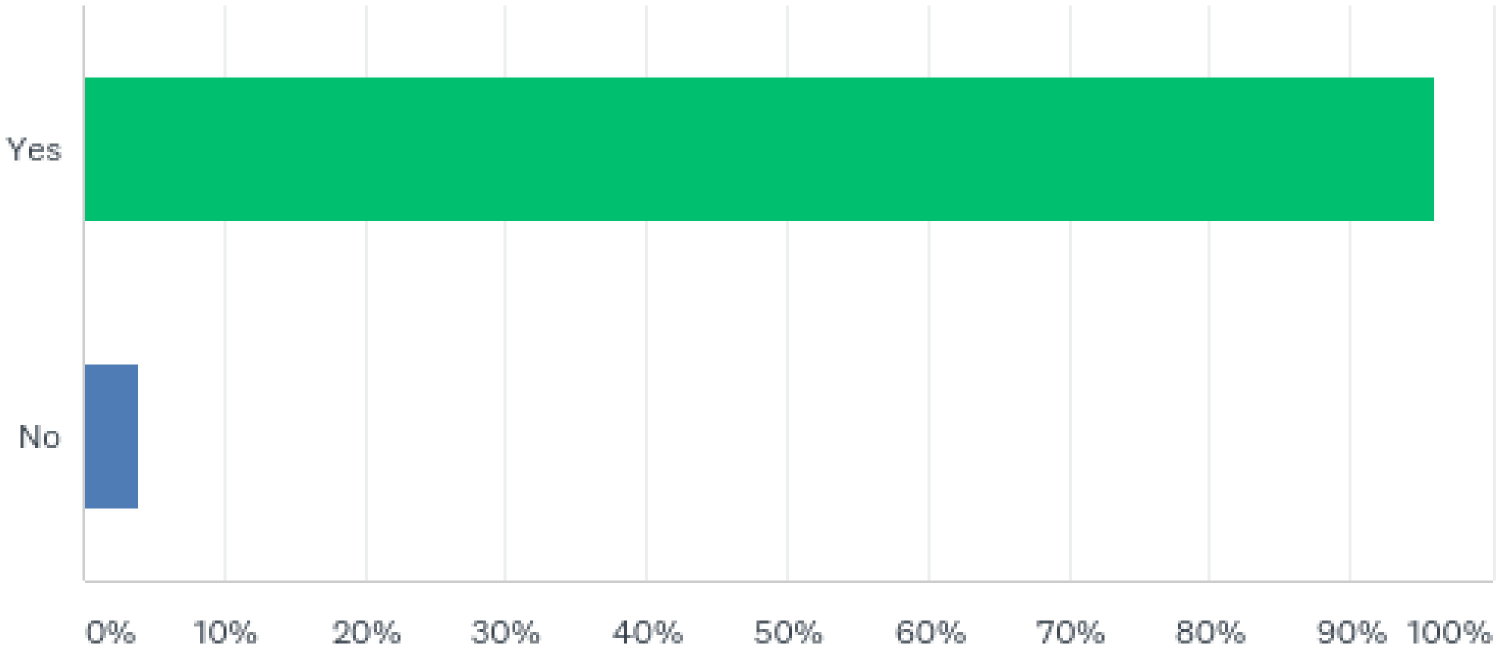
IMPACT: PATIENT ADVOCACY GROUPS

During the past 24 months, AAKP has been contacted by more than *134* companies, investment firms and major investors on questions related to patient science: research, psychoanalytics, clinical trials and product development – all seeking assistance with patient preference and insight data:

- Study design/protocols
- Participant consent forms
- Study/participant materials (i.e. patient facing items such as marketing, use of appropriate language, images and graphics)
- Trial recruitment efforts
- Patient burden assessments/useability preferences
- Targeted patient surveys
- In-person on online video focus groups
- Cognitive interviews

In the same period: AAKP had over *263* contacts with government, private sector and research institutions on other issues related patient insights related to new drug/device/diagnostic regulation, reimbursement & patient choice.

HARNESSING THE POWER OF IDEALISM



- **<u>JUNE 2020 AAKP FLASH SURVEY:</u> QUESTION: As an individual that suffers from kidney disease, if you felt your insights were valued and used to improve/advance clinical trials and drug development, would you help companies develop more products/treatments to help fellow patients and those yet to be diagnosed? *(AAKP sample @900+ patients)***

AAKP: RE-ALIGNMENT OF CAPACITIES

AAKP Center for Patient Research & Education

Research

- **AAKP Unique Patient Database (continual data collection & segmentation by disease stage)**
- **Demographic & Geo Targeting for Research & Clinical Trials**
- **Clinical Trial Design & Patient Risk & Consent**
- **Clinical Trial Awareness Campaigns**
- **Product Market Research (drug concept to interface/packaging)**
- **Direct patient interviews (one-on-one)**
- **Facilitated Patient Roundtables/Focus Groups**
- **Patient surveys (web-based/telephone)**
- **Flash surveys/questionnaires**
- **AAKP Veterans Health Initiative (VHI)**
- **AAKP Social media platforms (data analytics insights)**
  - Facebook @kidneypatient
  - Twitter @kidneypatients
  - LinkedIn
  - Instagram
  - Pinterest
- **Consults & training for Patient Advisory Panels**
- **AAKP & PCORI (all 17 major kidney projects, 10+ years)**

Education

- **Patient Training/Information on Clinical Trial Enrollment/Participation/Retention**
- **Patient Training/How to be effective in academic & industry research initiatives**
- **COVID-19 HealthLines** (webinar programs – custom tailored for audiences)
- **National Patient Meeting (largest in USA)**
- **AAKP "Understanding Kidney Disease" series** (print/online programs)
- **AAKP "Kidney Beginnings" series** (print/online/interactive programs)
- **AAKP Patient Plan series for managing disease** (print/online/interactive programs)
- **Nutrition Program/AAKP Delicious! Recipe Cards** (print/online/App programs)
- **aakpRENALIFE** (national magazine)
- **Kidney Beginnings** (e-newsletter)
- **Kidney Transplant Today** (e-newsletter)
- **aakpRENALFLASH** (e-newsletter)
- **Taking Dialysis Home** (e-newsletter)
- **AAKP Pediatric Kidney Kids** (e-newsletter)
- **Medal of Excellence Award Program**

AAKP Center for Patient Engagement & Advocacy

Engagement	Advocacy
• **Decade of the Kidney™** (AAKP initiated and led) • **Ambassador Initiative** (USA/Global) • **Ambassador Toolkit** • **Ambassador Certification Program** • **Speakers Bureau: Patient/Living Donor/Caregiver Certified Speakers** (conferences, industry meetings, town hall meetings, interviews, media) • **Social Media Training** (web-based/in-person) • **Membership Services** (patient/family/living donor; physician; healthcare professional; institutional; life; corporate) • **Flash surveys/questionnaires** • **Social Media Platforms**	• **AAKP Annual Policy Summit** (in-person/virtual) • **Washington, D.C. Capitol Hill Day Visits** • **Virtual Hill Day Visits** via Social Media/Online Platforms • **AAKP Action Center** – elected leader contacts • **I Am a Kidney Voter** (largest kidney voter registration campaign) • **Public Policy Training Workshops** (web-based/in-person) • **Patient Advocacy Training Guides** (online/mobile ready) – *in development* • **Targeted Digital Campaigns/Action Alerts** • **Press Releases/Statements** • **Patient Engagement & Advocacy Awards Program** • **Public Service & Congressional Awards Program**

FDA: TRANSFORMING THE STATUS QUO/FUTURE

- **Maintain:** MDUFA efforts that have put the patient at the center of the product development lifecycle - ***formal policy backed by guidance and process.***
- **Expand research:** encourage more academic research and help expand the next generation of patient research partners to engaged in the "science of patient insights" and PRO, PPI, RWE
- **Educate other federal agencies:** on FDA MDUFA-related successes involving patients as a policy priority.
- **Align:** federal efforts on device approval and payment through greater cross-agency collaborations, i.e. CMS, **to better inform coverage & reimbursement decisions.**
- **Educate:** non-government organizations in the national health ecosystem, like Institute for Institute for Clinical and Economic Research (ICER), whose existing evidence standards may be not reflect evolutions in patient science, patient expectations and FDA/federal patient engagement – **to help make coverage deliberations by payors better informed and balanced.**

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The oldest & largest, independent kidney patient group in the U.S.A.

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