ESRD as a Preference Sensitive Condition

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Center for Devices and Radiological Health
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December 8, 2017
Outline

• End Stage Renal Disease (ESRD) Background

• What makes ESRD preference sensitive?
ESRD Background

• Pathophysiology
  – Kidney “function” < ~10% of normal
  – Accumulation of toxins and fluid normally excreted by the kidney

• Symptoms / Signs
  – Fatigue, confusion, altered sleep, nausea, vomiting, shortness of breath, fluid retention, altered taste, malnutrition, itching, etc.

• Treatments
  – Dialysis: Hemodialysis / Peritoneal Dialysis
  – Transplantation
ESRD Treatments

• Hemodialysis (HD) – 87.3%
  – Most common / default
  – Blood removed from body, filtered, and returned
  – Typically 3x / week in a clinic (in home HD ~1.8%)

• Peritoneal Dialysis (PD) – 9.6%
  – Fluid added to abdomen; fluid saturated with toxins; fluid removed and replaced; cycle repeated several times daily
  – Therapy typically delivered in the home

• Renal Transplantation (Txp) – 2.5%
  – Best available therapy / replaces native kidney function
  – Shortage of available organs
  – Surgery / Drugs required to suppress immune system
“Preference Sensitive”

- “multiple treatment options exist and there is no option that is clearly superior for all patients”
- “when the evidence supporting one option over others is considerably uncertain or variable”
- “patients’ views about the most important benefits and acceptable risks of a technology vary considerably within a population, or differ from those of healthcare professionals.”
ESRD / HD Background

• Most patients with ESRD receive hemodialysis (HD) in a clinic setting

• In-clinic HD therapy can be inconvenient
  – Requires travel to a clinic 3 times every week.
  – Much of the patient’s time is spent traveling, waiting, receiving the HD treatment, and recovering.
  – Difficult to work or travel.
  – Intense dietary and fluid restrictions.

• Strategies to increase convenience (e.g., home HD) require benefit/risk trade-offs -> preference sensitive
## Home HD Trade-off (examples)

<table>
<thead>
<tr>
<th>Benefits / Pros</th>
<th>Risks / Cons</th>
</tr>
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<tbody>
<tr>
<td>• Increased therapy options (e.g., extended, nocturnal, frequent)</td>
<td>• Increased responsibility</td>
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<tr>
<td>– Less diet/fluid restrictions</td>
<td>• Lack of trained medical personnel</td>
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<td>– Fewer medications</td>
<td>• Increased burden on family / care partners</td>
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<tr>
<td>– Shorter recovery time</td>
<td>• Need for dedicated space for treatment / supplies</td>
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<tr>
<td>• Increased flexibility with treatment schedule</td>
<td>• Social isolation from (ESRD Community)</td>
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<tr>
<td>• Increased ability to work or travel</td>
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ESRD Treatment / Preferences

Preference Sensitivity

- In Clinic HD
- Home HD
- Wearable HD
- Implant

www.merit.com
www.nxstage.com
www.nanodialysis.nl
https://pharm.ucsf.edu/kidney
Preferences / Novel Technologies

• ESRD Patient preferences data available
  – home HD, PD, and blood access for HD.
• Patient preferences around novel technologies (wearable / implantable) are largely unknown.
• Patient preferences around novel technologies will be important for regulatory approval and labeling.
• How do we obtain patent preferences for this preference sensitive area?
  – Kidney Health Initiative
    • Partner with patients and other experts to further explore preferences around novel therapies
Thank You!
Patient Partnership Perspective: Kidney Health Initiative:

Melissa West
Project Director, Kidney Health Initiative
How do we put the patient voice front and center in the context of therapeutic product development?
There is still much to do...

- Patients become true partners in the product development process
- Develop an infrastructure which allows patients to actively participate at every stage of the *product development pathway*
Kidney Health Initiative

- Public Private Partnership between the FDA and ASN
- Formed in September 2012
- Goal of promoting innovation and patient safety in kidney disease
Multi-disciplinary collaboration of over 80 organizations
KHI is a collaborative effort

- Professionals
- Government
- Research Institutes
- Providers
- Pharm/Biotech
- Patients/Family Members
- Devices

ASN/FDA
Making an impact: projects in strategic priority areas

- White papers, data standards, workshops or a roadmap
- Facilitate the passage of drugs, devices and biologics into the kidney area
- Over 15 projects to date
KHI Patient and Family Partnership Council

Current members of the KHI PFPC:

David M. White, Chair
Kevin J. Fowler, Vice Chair
Pamela M. Duquette
Denise Eilers, BSN, RN
Richard D. Fissel
Nichole M. Jefferson
Terry F. Litchfield
Roberta L. Wager, MSN, RN
Caroline Wilkie

Logos of various organizations are also included.
KHI Patient and Family Partnership Council

- Help KHI engage and network with other patients and patient organizations across the spectrum of kidney disease
- Advise industry and research partners of the needs and preferences of patients to consider as they develop new products
- Identify patients willing to participate in KHI workgroups and projects
Workshop: Patient Perspective on Medical Device Development

- Patient’s tolerance for “risk on hemodialysis” varies tremendously
- Patients on home hemodialysis may sacrifice some degree of safety for an improved (more independent) quality of life
- Organized a very successful Patient Preferences Workshop for Renal Devices in August 2015
- Strong representation from patients and their caregivers, industry partners and regulatory agencies
Patient Preferences workshop: Challenges

- Put together a multi-disciplinary committee that included patients
- Created an aggressive timeline
- Realized that the patients on the committee had no idea at all about what the FDA does or why the FDA should be interested or what the real end product of these calls would be (in terms of impacting patients)
- Retooled the entire project
- Interactive webinars (present the issues); have a two way conversation
- Provided travel support for some patients to attend
Patient Preferences workshop: Benefits

- Truly engaging patients (not just having token representation so that we can check a box in a grant application) is hard work
- We have to work hard at it (physicians, regulators, industry and patients)
- Resources
- Come out of your comfort zone
- Impact can be huge
- Right thing to do
- TRUTH!
A patient's perspective on improving ESRD care is often very different from the physicians.

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Tong et al.  
SONG Initiative
Vision Level

TPP/Patient Voice

Design Criteria
Clearance/Ultrafiltration/Weight/Portability/Endocrine

Technical/Regulatory Milestones
Take Home Message

- Real Progress in Therapeutic Product Development can only be made through a multi-disciplinary approach that includes academia, industry, federal agencies and most importantly the END USER

- Critical need for platforms to be able to do this

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