Trial Design for Home Dialysis Machines and Equipment Michael Aragon, MD



## **Chief Medical Officer of Outset Medical**

# **Discussion**

The Time for Home is Now The Need for Better is Real Investigational Device Trials vs Pre and Post Market Studies Device design studies for Home Patients and Care Partners

The Importance of Pilots and Early Experience Real World Data: The Need and Trial Design Challenges Unprecedented Opportunity to Help Patients Treat at Home

AAKH: Federal Focus on Home Therapies

Connectivity and Patient Health Tracking Devices

Home Dialysis Incident and Prevalence Rates Rising<sup>1</sup>

ESRD Treatment Choices (ETC) should Motivate 30% to Focus on Home Dialysis

Payor Focus on Value Based Care through Medicare Advantage Expansion There is room for improvement in technique delivery and outcomes

Overall Home population <12%

Home Dialysis improves QOL, BUT...

... burden of therapy remains a significant cause of technique failure

More frequent HHD may reduce CV admissions but increase vascular access interventions<sup>1,2</sup>

Survival rates are equivalent between ICHD and PD<sup>3</sup>

<sup>1.</sup> Weinhandl ED, Nieman KM, Gilbertson DT, Collins AJ. Hospitalization in daily home hemodialysis and matched thriceweekly in-center hemodialysis patients. *Am J Kidney Dis*. 2015;65(1):98-108. doi:10.1053/j.ajkd.2014.06.015

<sup>2.</sup> FHN trial group

<sup>3.</sup> El Sayed et al. Propensity score matched mortality comparisons of peritoneal and in-centre haemodialysis: systematic review and meta-analysis. Nephrol Dial Transplant (2020) 35: 2172–2182

Device Approval Trials Have Limited Design Flexibility

## **Regulatory Approval – FDA**

- Required for HHD Labeling
- Collaboration with FDA on Trial Design
- Pre-submission Application Strongly Recommended
- Representative Population
- Focus will be on Safety and Efficacy
- "Professional User" vs "Newly Trained" User
- Crossover of IC vs HHD

Home Device Trials: Focusing on the Patient and Care Partner

## **Clearance and Ultrafiltration from the Patient's Lens**

Need to understand "optimacy"

## **Focus on the Patient Experience**

- If the patient can't use it, they won't use it at all
- If the patient isn't sure how to use it, they won't use it safely
- If it takes too much time to use it, they won't use it as prescribed
- If it isn't reliable to use it, they won't use it enough

# Know the Patient, Before the Design

### **Patient Preference Studies**

- Qualitative or quantitative assessments of the relative desirability or acceptability to patients of specified alternatives or choices among outcomes or other attributes that differ among alternative health interventions.<sup>1</sup>
- Feature X vs Feature Y
- Top 3 Features
- Relative Value of Benefit vs.

### If you could have a home hemodialysis system with just 3 features to it and that is it, what features would you choose?



1. Patient Preference Information – Voluntary Submission, Review in Premarket Approval Applications, Humanitarian Device Exemption Applications, and De Novo Requests, and Inclusion in Decision Summaries and Device Labeling: Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders

US Department of Health and Human Services, FDA, Center for Devices and Radiological Health and Center for Biologics Evaluation and Research (2016)

# The Patient Voice is Critical to Design Trade-Offs

### **Discrete choice experiments (DCEs)**

A quantitative technique for eliciting preferences that can be used in the absence of revealed preference data

### **DCEs in Med Device**

- Value comparisons and Trade-Offs
- Device with Characteristics A vs B

Choice 2 of 12	Dialysis A	Dialysis B	No dialysis C	
Average survival time	2 years	5 years	2 years	
Number of visits <b>per week</b> to hospital for dialysis	No visits required (home dialysis)	No visits required (home dialysis)	No visits required	
My ability to travel or 'go away' on short trips	A bit restricted	A bit restricted	Not restricted	
Hours on dialysis per treatment	10 hours	4 hours	None	
Time of day my dialysis can be done	Night-time	Either day-time or evening	Not applicable	
The hospital provides a transport service to attend dialysis or doctors' appointments	Yes, at a small cost to me	Yes, at no cost to me	No, not provided	
I can change my dialysis days and times	Whenever it's needed	Whenever it's needed	Not applicable	

Morton RL, Snelling P, Webster AC, Rose J, Masterson R, Johnson DW, Howard K. Dialysis modality preference of patients with CKD and family caregivers: a discrete-choice study. Am J Kidney Dis. 2012 Jul;60(1):102-11. doi: 10.1053/j.ajkd.2011.12.030. Epub 2012 Mar 13. PMID: 22417786.

# **Actual Use Does Not Always Follow Design**

# User-centered or Human-centered design (UCD, HCD)

Design and evaluation process that pays particular attention to the intended users, what they will do with the product, where they will use it, and what features they consider essential.<sup>1</sup>

### Human factors testing

How a user actually uses a device in an environment that mimics actual use and gauges the performance in terms of the likelihood of an error or difficulty in use.<sup>2</sup>

1. Introduction	→ 2. Simulated Use	→ 3. Instructions for Use	→ 4. Interview
<ul> <li>Patient consent</li> <li>Program review</li> <li>Background questions</li> </ul>	<ul> <li>A series of tasks representing use of all basic functions of the system, including:</li> <li>Preparing the system</li> <li>Preparing the patient</li> <li>Adjusting settings</li> </ul>	Comprehension questions regarding the instructions for use.	Final questions designed to obtain subjective impressions of the system and to probe regarding observed behavior with the system.
	<ul> <li>Monitoring treatment</li> <li>Viewing and responding to system alarms</li> <li>Ending treatment</li> </ul>		

#### Table 2 Terms and descriptions

Term	Description				
Task failure	A case of performing a task incorrectly, or not completing a task, in such a way as to have a potentially significant implication for safety.				
Use error	A case of performing a task incorrectly, or not completing a task, without a potential implication for safety.				
Close call	A case of almost committing a task failure, but catching it in time to avoid making the failure (i.e., self-correcting), or in time to avoid adverse outcomes such as an injury.				
Operational difficulty	A case of struggling to some extent while completing a task, without potentially significant safety implications (e.g., experiencing confusion, taking longer than expected, experience difficulty manipulating a device's components).				

Wilcox SB, Carver M, Yau M, Sneeringer P, Prichard S, Alvarez L, Chertow GM. Results of human factors testing in a novel Hemodialysis system designed for ease of patient use. Hemodial Int. 2016 Oct;20(4):643-649. doi: 10.1111/hdi.12430. Epub 2016 May 19. PMID: 27194590.

Kim JE, Kessler L, McCauley Z, Niiyama I, Boyle LN. Human factors considerations in designing a personalized mobile dialysis device: An interview study. Appl Ergon. 2020 May;85:103003. doi: 10.1016/j.apergo.2019.103003. Epub 2020 Jan 7. PMID: 31929024.

- 1. Rodriguez, Margarita Morales; Casper, Gail; Brennan, Patricia Flatley. "Patient-centered Design: the Potential of Usercentered Design in Personal Health Records" Journal of AHIMA 78, no.4 (April 2007): 44-46.
- 2. https://www.fda.gov/medical-devices/human-factors-and-medical-devices/human-factors-considerations
- 3. Wilcox SB et al. Results of human factors testing in a novel Hemodialysis system designed for ease of patient use. Hemodial Int. 2016 Oct;20(4):643-649. doi: 10.1111/hdi.12430.

# **Real World Use Does Not Always Reflect Testing**

## **Devices out in the wild**

- Pilot Studies
- Early Experience

## **Actual Patient Population**

- Patient Response: Was PP and DC accurate?
- Performance: Did HF reflect RW?
- User Experience: Did design meet RW actual use?



# The Right Questions, The Right Population

The Right Questions for Home

- QOL
- Safety
- Retention
- Healthcare Utilization
- Labs and Clearance

## The Representative Population

- Race
- Sex
- Ethnicity
- Age
- Cause of ESKD
- Comorbidities

vol 2 Table 1.5 Number and percentage of prevalent cases of hemodialysis (HD), peritoneal dialysis (PD), and transplantation (Tx) by age, sex, race, ethnicity, and primary ESRD diagnosis, in the U.S. population, 2012

	HC	)	PI	D	Тх		
	N	%	N	%	N	%	
Age							
0-19	1,134	0.3	898	2.2	4,957	2.8	
20-44	49,843	12.4	8,187	20.2	39,965	22.7	
45-64	167,499	41.6	18,137	44.7	89,876	51.1	
65-74	95,889	23.8	8,284	20.4	32,475	18.5	
75+	88,149	21.9	5,099	12.6	8,705	4.9	
Sex							
Male	226,205	56.2	21,968	54.1	104,654	59.5	
Female	176,309	43.8	18,637	45.9	71,324	40.5	
Race							
White	221,887	55.1	26,690	65.7	128,468	73.0	
Black/ African Am	153,264	38.1	10,534	25.9	35,628	20.2	
Native American	5,839	1.5	476	1.2	1,755	1.0	
Asian	21,524	5.3	2,905	7.2	10,127	5.8	
Ethnicity							
Hispanic	68,710	17.1	5,915	14.6	35,467	20.2	
Non- Hispanic	333,804	82.9	34,690	85.4	140,511	79.8	
Primary cause	of ESRD						
Diabetes	178,012	44.2	14,120	34.8	40,688	23.1	
Hyperten- sion	116,260	28.9	10,528	25.9	27,785	15.8	
Glomerulo- nephritis	43,521	10.8	7,931	19.5	48,980	27.8	
Cystic kidney	9,543	2.4	1,895	4.7	17,463	9.9	
Other urologic	3,052	0.8	369	0.9	3,515	2.0	
Other Cause	32,513	8.1	3,660	9.0	20,306	11.5	
Unknown/ missing	19,613	4.9	2,102	5.2	17,241	9.8	
All	402,514	100.0	40,605	100.0	175,978	100.0	

Table 1 Patient baseline characteristics

Characteristic	N = 30 (%)
Age, y	$52.3 \pm 11.6$
Weight, kg	$93.8 \pm 17.0$
Men	19 (63)
Race	
White	17 (57)
Black or African American	13 (43)
Hispanic or Latino	8 (27)
Not Hispanic or Latino	21 (70)
Ethnicity not reported	1 (3)
New to home hemodialysis	17 (57)
Vascular access type	
Fistula	23 (77)
Catheter	4 (13)
Graft	3 (10)
Comorbid conditions	
Coronary artery disease	12 (40)
Congestive heart failure	1 (3)
Diabetes	18 (60)
Hypertension	29 (96)
Hypercholesterolemia	20 (66)
Carotid artery disease	6 (20)
Peripheral artery disease	5 (16)
Arrhythmia	7 (23)
Systemic inflammatory conditions	3 (10)
Tobacco use (current)	4 (13)
Tobacco use (former)	7 (23)

Data Source: Special analyses, USRDS ESRD Database. Abbreviation: African Am, African American; ESRD, end-stage renal disease.

1. 2014 USRDS Annual Report

<sup>2.</sup> Plumb, T. J., Alvarez, L., Ross, D. L., Lee, J. J., Mulhern, J. G., Bell, J. L., ... & Aragon, M. A. (2020). Safety and efficacy of the Tablo hemodialysis system for in-center and home hemodialysis. *Hemodialysis International*, 24(1), 22-28.

# **Trial Design for Meaningful Results**

## **Determining Impact (outcomes)**

Subjective vs Objective

Sample size

- Type 1 and Type 2 Error

## **Developing Controls**

Historical vs Crossover vs Self-Controlled

**Duration of Follow Up** 

**Study Monitoring** 

# **Inherent Limitations of Home Device Trials**

Blinding is not possible	Randomization is challenging	Limited historical data for comparison	Survey fatigue	"Actual" use and patient privacy
-----------------------------	---------------------------------	---	-------------------	--

# **Summary**

- Now is the time for new Home Devices and Equipment
- Patient Preference and Human Factors are key along the critical path
- Home Device Trials must be representative of the entire ESKD Population
- Patient and Care Partner QOL, decreased utilization and improved patient retention are vital endpoints
- Due to the very nature of home dialysis, prospective, crossover or self-controlled trials are likely to remain the level of evidence achievable for Home Dialysis devices



Understanding Patient Perspectives to Inform a Strengthsbased Approach to Home Dialysis

Amanda Baumgart on behalf of the SONG Investigators

Annual Dialysis Conference 5 - 7 March 2021 Missouri



# **Clinical Scenario**

You learn that a 54-year-old male patient of yours on hemodialysis often does not take all his daily medication. When you ask why, he becomes defensive and claims that he avoids taking the medication because of the negative side effects.

What is your impression of him?

He is resistant to treatment, should be trying harder to adhere to his treatment plan or does not understand the health consequences of low adherence.

OR

He is being actively involved in his treatment or is choosing not to take the medication after carefully considering the risks of non-adherence and the daily impact of side-effects.

# **Clinical Scenario**

You learn that a 54-year-old male patient of yours on hemodialysis often does not take all his daily medication. When you ask why, he becomes defensive and claims that he avoids taking the medication because of the negative side effects.

What is the best course of action?

Acknowledge his side effects but insist that he take all his medication and emphasize that serious adverse health consequences could occur if he does not.

OR

Discuss the side effects and potential culprits, and if no alternatives are found focus on side effect relief. Discuss the short- and long-term health consequences of low adherence to ensure he has made an informed decision.

## 1 | Strength-based approach

# Deficit-based vs Strength-based

- Focusing on what needs fixing
- Finding solutions *for* the patient
- Short-term solutions
- Learning from past failures
- Prioritizing the illness
- Avoiding problems
- Practitioner responsible for health
- Practitioner knows best
- Success defined by compliance

- Focusing on what is working
- Finding solutions *with* the patient
- Sustainable solutions
- Learning from past successes
- Prioritizing the person
- Building coping skills
- Patient responsible for health
- Collaborative partnership
- Success defined by the patient

Gottlieb, LN, & Laurie, NGPR 2012, Strengths-Based Nursing Care : Health and Healing for Person and Family : Health And Healing For Person And Family, Springer Publishing Company, New York.

# How? Qualitative Research!

- A strength-based approach requires in-depth understanding of the values, experiences and beliefs of patients with chronic kidney disease and their families
- This may not always be conveyed in time-constrained and power-imbalanced clinical settings
- Qualitative research can generate detailed evidence on people's priorities, goals and needs to change clinical practice and policy

Giacomini MK, Cook DJ. Users' guides to the medical literature: XXIII. Qualitative research in health care B. What are the results and how do they help me care for my patients? Evidence-Based Medicine Working Group. JAMA 2000; 284(4): 478-82.



Why is my patient not coming back for follow up? Why is my patient refusing to take their medications?

How does dialysis interfere with the daily life of my patient?

What helps my patient cope with dialysis?

## 2 Overview of qualitative research

## Definitions

"Seeks to describe and analyze the culture and behavior of humans and their groups from the point of view of those being studied" (Bryman)

"Blanket designation for all forms of social inquiry that rely primarily on qualitative data ... understanding the **meaning** of human action." (Schwandt)

#### Qualitative health research is not:

- X Survey research
- X Anecdotal does generate empiric data
- X Airy fairy follows a systematic, scientific process



	Quantitative		Qualitative
۰	To quantify relationships among variables	•	To describe, understand, explore a central phenomenon – gain a deeper understanding
		•	Explain behaviours, decisions
٠	To explore and/or test hypotheses based on predicted associations among variables	•	To generate hypotheses or theories

- To quantify relationships among variables, i.e. measure the strength of associations and the probability that the association exists in the larger population
- To describe and explain associations e.g. contextual or social reasons

**Initial design:** generate hypothesis, describe context/potential barriers for a new intervention, development of an intervention, define outcomes, inform survey design.

## Research

**Process evaluation:** assess participation in research, barriers and facilitators of uptake/implementation, responses

**Outcome evaluations:** identify reasons for trends in the results, explain differences in effectiveness, generate further hypothesis

**Shared decision-making:** decision aids, doctor-patient communication (clarify values and preferences)

## Practice

**Patient educational resources:** information about disease, treatment options, access to services that are important and relevant to patients

**Clinical quality measures:** define and measure quality indicators that concord with patient preferences

Policy

Funding priorities: inclusion of stakeholder priorities

Practice guidelines: underpin recommendations for patient-centered care

- Methodology: A way of thinking about and studying social reality (underlying theory or framework that guides the choice of methods and research process)
- Methods:A set of procedures and techniques for data<br/>collection and analysis

**Grounded theory:** Systematic and iterative data collection and constant comparative analysis to develop conceptual frameworks or theories about social processes.

**Phenomenology:** Philosophy based on reflective inquiry and description of the "lived experience" in a given context – explanations for actions.

**Ethnography:** Process of describing and interpreting the behaviors and actions of social groups or cultures usually through interviews and observations – defined population, detailed fieldwork

**Qualitative study** 



**Topic area** (population, topic)

Problem/s

Aim/purpose

Questions

Vascular access (surgical intervention) for hemodialysis

- Delayed creation of vascular access due to patient refusal Prevailing treatment-related stressor for patients on HD
- To describe patients' expectations and experiences of initiation and maintenance of vascular access for hemodialysis
  - What are patients' beliefs concerns about vascular access - why?
- What does vascular access mean to patients?
- What are the social, personal and lifestyle impacts?
- What challenges do patients face and how do they cope with it?



## Participant selection strategies

### Quantitative

- Larger sample size (to reduce sampling error, provide adequate power, and achieve statistical representativeness)
- Random
- Pre-determined
- Statistical results (precision)

### Qualitative

- Smaller sample size (select information-rich participants "key informants" to gain in-depth insight)
- Usually purposive (convenience, snowballing, theoretical)
- Depends on analysis
- Data/theoretical saturation



## Observation

Document analysis

#### Interviews

### Focus groups

#### Study events and actions within a particular social or historical context

- "Systematic method of data collection that relies on the researcher's ability to gather data through their senses within real-world contexts" (O'Leary)
- Used with other methods



Source ABC news

Communication between patients with chronic kidney disease and nurses about managing pain in the acute hospital setting MANIAS E & WILLIAMS A (2007) Journal of Chronic Illness and Healthcare in association with Journal of Clinical Nursing 16, 11c, 358–367

- 103 events of pain communication
- Competing priorities (technicalities of dialysis)
- Did not differentiate cause of pain (analgesics not tailored to individual patient needs)

Observation

## **Document analysis**

Interviews

Focus groups

**Identify, organize, evaluate, synthesize documentary data** Types of documents

- **Public:** newspapers, magazines, social media (tweets, blogs, websites, YouTube), speech transcripts, published policies
- Personal: letters, emails, journals, portfolios



Observation

Document analysis

### Interviews

Focus groups

#### Elicit an individual's perspectives

- "A specific form of conversation where knowledge is produced through the interaction between an interviewer and interviewee." (Kvale 2007)
- Partnership → meaning-making
- Flexible change questions/order
- Probes (e.g. elaboration, clarification, completion)
- In-depth information and understanding
- Usually one-to-one
- Interested in an individual-level topics

#### **Types of interviews**

- Structured interviews \* (quantitative)
- Semi-structured interviews (more common)
- In-depth / unstructured interviews (narratives, life stories)

Observation

Document analysis

Interviews

## **Focus groups**

# In-depth discussion that capitalises on group interaction

"... encourage interaction between research participants as much as possible. When group dynamics work well the coparticipants act as co-researchers taking the research into new and often unexpected directions and engaging in interaction that is both complementary and argumentative (questioning, challenging, and disagreeing with each other)." – Kitzinger 1994

- Facilitated
- Exploration and clarification of views
- Share a social/cultural experience
- 1.5 2 hours
- It is NOT:
  - a group interview
  - a support group
  - used to achieve consensus
  - used to quantify opinions



## Why run focus groups?

- To research "sensitive" populations e.g. those who feel more relaxed about talking with others who have a shared experience (ethnic minority groups, refugees)
- Brainstorm (e.g. develop an intervention)
- Explore the processes of reasoning and debating

### **Embedding activities**

- Prioritization e.g. nominal group technique, rating, ranking
- Hypothetical scenarios
- Elicit reactions to data

### **Embedded in workshops**

#### Nominal group technique







with

anything."



SONG-PD

## Analysis

- Capture the breadth and depth of the data
- Comprehensible, insightful, trustworthy, compelling, original
- Answer the research question
- Describe phenomena
- Develop a theory or explanation



Quantitative	Qualitative		
<ul><li>Coding</li><li>Starting point for most forms of qualitative data analysis</li></ul>	<ul> <li>Reading of the data, making memos, conceptualizing the data, grouping concepts into themes, identifying patterns and relationships among themes</li> </ul>		
<ul> <li>"Process of defining what the data are about" (Charmaz 2006)</li> </ul>	Coding and identifying concepts, grouping into themes		
<ul> <li>Coding is highlighting relevant segments of words and involves</li> </ul>	<ul> <li>Software used to store, label, retrieve data (facilitates but does not do analysis)</li> </ul>		
<ul> <li>Quantified estimates of effect or associations, statistics</li> </ul>	Narrative and rich description		
• Frequency	Breadth and depth		
<ul> <li>Emphasis on generalisability (involves statistical analysis to determine the extent to which the findings can be extrapolated to another population)</li> </ul>	• Emphasis on <u>transferability</u> of concepts and theories (the reader determines whether the findings "fit" or resonate in their own context or experience)		

### Thematic analysis

- Usually inductive derived from the data
- Constant comparisons within and across sources
- Output → themes (full of meaning)

### Content analysis (?)

- Deductive: code data into codes identified and defined apriori
- Used when a meaningful denominator exists for reporting proportions
- Inter-rater reliability

## Grounded theory analysis

- Open coding: generating preliminary initial concepts from the data
- Axial coding: reviewing, developing, linking, grouping codes/concepts
- Selective coding: organising and formalising relationships, developing theoretical frameworks
- Memoing

#### Coding in software

Backsburg (104 As)			old x	Senets Mort-Larn ESITETet	.0
Case Filter: Al Cases				Default Ferit and Size	A A > Related being
25 af 25 ○ 1 ○ [2] [2]bertLass	d 21 _ Henclans ↓		-] 0+	· · · · · · · · · · · · · · · · · · ·	into details just searching für Klenature any mane. I think they've really dane a very good job Facilitate: Google?
Coch Tom Server Access - presentation of person independent Perspections - technical administration (Consume 2 additional administration of the consumer personal additional administration of the consumer Access - presentation of specialism - received (Conseque particularity of person independent) (Conseque	Type m, [51113.44] Tote m, [51113.44] Tote	Paulaen 2019 1242 3168 4449 5168 5449 5168 5449 5168 5459 5500 4833 4444 7455 4444 7455 4444 7454 4594 718 5460 8167 5460 8167 5460	4 H	Decisional validation - providing	Googet here you waitly here it go with yound a will keining for special areas and talk is your peer's and see here you can yet investment and an adversarial priori. Facilitater Paper. You would go to the your investegation of the outpress and see here you can yet internetwoor. This is funding priority and your investigation or at two, thinks. Facilitater Paper. Networks and the under policy part per your may be one or two, then it. Facilitater Paper. Networks and the under policy part per your priority one or two, then it. Facilitater Paper. Networks and the set of the priority of the part of the part of the part of the paper in the part of the part
Programms - provident approver Programms - provident approver Programms - broad dimarké Provinsel válketov- ganong expensifial files Abert Jan Concorde válketov- apinong expension files (Abert Jan Concorde Válketov- apinong expension) Televá Abertatomic → Televá Searce Válketov- apinong aberta (Aberta) Válketov- apinong aberta	m, filling free m, filling free m, filling free m, filling free m, filling free m, filling free	1116/1030 1116/1030 1136/12564 11747/2035 11562/1085	- -	Confidence - reputation based t	Interview Table you's first strategy in many point order into allow a strate antice restration? First first Wess you will be approximately a strategy in many point order into allow a strate restration? First First Wess you will be approximately interview and the strategy into all be approximately interview and the strategy in the strategy interview and the strategy int
Cell Cole: • Angels: Celle Dir Colete: L. Boropp Conferce: - restruction in Initiational proof Conferce: - resculare with resperence and initiation Conferce: - resculare with resperence and initiation Conferce: - resculare with resperence and initiation Decomment variabilities: of avoing above			1	Pragmatism - perceptible appli	Facilitate: Okay. How de you feel about renewing or apparing the methodology that's being user in some of the paper? Internetive: This methodology, of course, is probably net the real is valid to it. I would hak at which they have have about the clinical internetive and everything and these (1) and any should it would have at which clinical internetive of patient they folded integrated and they are applied to the state of the source of the source of the source of the patient they folded integrate of the source
Decisional validaturo - spraiding socientità junification Decisional validaturo - rentalimang sun practice Decisional validaturo - rentalimang sun practice Gazaging information dispersion - mangaring reformation publice ad Gazaging information dispersion - mangaring advectation Essensing information dispersion - avectentistiched i navellegat ca Detrimang particulare - efficience - manareare disfando	alon gaschy			Pregnatism - sectical demand Pregnatism - techical demand	Interviews Ob, dictators, dictators, they would. Them the new techniques them, they distidance new techniques them, them are not obsolvables. In this fill work of charge your obsolvables compatible, this is a way. Interviews Obsolvable, which is the schedules, whether they as walks or in the new techniques them, there every obsolvable is the schedule schedules, whether they as walks or in the new of them, as 1 say, when they are in splicitors in a negative power that pray program schedules they are schedules. The Doppen to example a schedules on something from the journal that you have
Optiming outcomes annuing patient safety Description			•	Decisional validation - pairing r	Interview: "Like we're are taking at how the blood flows in the brain for all the scield cell warring building the proof to taking the one of the Annorance sequence more how to a because they have now block them or polarizing, to these are more scield cell instance them and they public their dual bene, them we have to use the techniques them and evaluations are started as the science of the annorance of the science of t
				Optimising outcomes - improve	Interviewee I there evidence-based medicine should be in every field in medicine, I would there. I don't there that you can unit below what accord your to say there to say the effects and the escats of in 1 thick. Utility we were actually practicine that
			-	E fundas Carlos In Cantast	

- Investigator triangulation
- Member-checking

## Themes



Translate: spell out implications



## Summary

- Qualitative research methods capture and communicate the patient voice (values, preferences, attitudes, beliefs)
- Systematic, transparent, and rigorous process
- Can be used to improve patient care and outcomes

## 3 | Strength-based research in dialysis

#### Meaning of empowerment in peritoneal dialysis: focus groups with patients and caregivers

Amanda Baumgart 록, Karine E Manera, David W Johnson, Jonathan C Craig, Jenny I Shen, Lorena Ruiz, Angela Yee-Moon Wang, Terence Yip, Samuel K S Fung, Matthew Tong ... Show more

Nephrology Dialysis Transplantation, Volume 35, Issue 11, November 2020, Pages 1949–1958, https://doi.org/10.1093/ndt/gfaa127

Published: 26 July 2020 Article history •

💪 Cite 🛛 🔎 Permissions 🛛 📢 Share 🔻

#### Abstract

#### Background

While peritoneal dialysis (PD) can offer patients more independence and flexibility compared with in-center hemodialysis, managing the ongoing and technically demanding regimen can impose a burden on patients and caregivers. Patient empowerment can strengthen capacity for selfmanagement and improve treatment outcomes. We aimed to describe patients' and caregivers' perspectives on the meaning and role of patient empowerment in PD.

#### Results

We identified six themes: lacking clarity for self-management (limited understanding of rationale behind necessary restrictions, muddled by conflicting information); PD regimen restricting flexibility and freedom (burden in budgeting time, confined to be close to home); strength with supportive relationships (gaining reassurance with practical assistance, comforted by considerate health professionals, supported by family and friends); defying constraints (reclaiming the day, undeterred by treatment, refusing to be defined by illness); regaining lost vitality (enabling physical functioning, restoring energy for life participation); and personal growth through adjustment (building resilience and enabling positive outlook, accepting the dialysis regimen).

#### Conclusions

Understanding the rationale behind lifestyle restrictions, practical assistance and family support in managing PD promoted patient empowerment, whereas being constrained in time and capacity for life participation outside the home undermined it. Education, counseling and strategies to minimize the disruption and burden of PD may enhance satisfaction and outcomes in patients requiring PD.

# Enabling good outcomes in older adults on dialysis: a qualitative study

<u>Rajesh Raj</u> <sup>⊡</sup>, <u>Bridget Brown</u>, <u>Kiran Ahuja</u>, <u>Mai Frandsen</u> & <u>Matthew Jose</u>

<u>BMC Nephrology</u> 21, Article number: 28 (2020) <u>Cite this article</u> 922 Accesses 2 Citations 7 Altmetric <u>Metrics</u>

#### Abstract

#### Background

Older patients on dialysis may not have optimal outcomes, particularly with regards to quality of life. Existing research is focused mainly on survival, with limited information about other outcomes. Such information can help in shared decision-making around dialysis initiation; it can also be used to improve outcomes in patients established on dialysis. We used qualitative research methods to explore patient perspectives regarding their experience and outcomes with dialysis.

#### Results

Seventeen interviews were analysed prior to saturation of themes. Participants (12 on haemodialysis, 5 on peritoneal dialysis) had spent an average of 4.3 years on dialysis. There were 11 males and 6 females, with mean age 76.2 years (range 70 to 83). Experiences of dialysis were described across four domains - the self, the body, effects on daily life and the influences of others; yielding themes of (i) responses to loss (of time, autonomy, previous life), (ii) responses to uncertainty (variable symptoms; unpredictable future; dependence on others), (iii) acceptance / adaptation (to life on dialysis; to ageing) and (iv) the role of relationships / support (family, friends and clinicians).

#### Conclusions

Older patients experience the effects of dialysis across multiple domains in their lives. They endure feelings of loss and persistent uncertainty, but may also adapt successfully to their new circumstances, aided by the support they receive from family, health professionals and institutions. From these insights, we have suggested practical measures to improve outcomes in older patients.

#### How do parents deal with their children's chronic kidney disease? A qualitative study for identifying factors related to parent's adaptation

Fatemeh Khorsandi, Naser Parizad, Aram Feizi & Masumeh Hemmati MaslakPak 🖂

BMC Nephrology 21, Article number: 509 (2020) Cite this article

523 Accesses | 3 Altmetric | Metrics

#### Abstract

#### Background

Parents' adaptation affects the health outcomes of children with chronic kidney diseases (CKD). Identifying factors that affect parents' adaptation is necessary to understand their adaptation status. This study aims to explore factors related to the adaptation of parents who have children with CKD.

#### Results

Two main categories extracted from the data were "adaptation facilitators" and "adaptation barriers." Adaptation facilitators were supported by three sub-categories: "social support", "family capability" and "spiritual beliefs". Four sub-categories of "adaptation barriers" were revealed as: "family-related barriers," "mental stress by others," "the chronic nature of the disease," and "unfavorable treatment conditions."

#### Conclusions

Identifying the factors influencing parental adaptation helps the medical staff to make the necessary interventions to support the parents. According to this study, increasing parent access to the required information, supporting them financially and emotionally, and helping them identify support resources can facilitate their adaptation to their child's chronic illness. Also, identifying and eliminating adaptation barriers can help parents deal better with their child's chronic disease.

## 4 | Patient involvement

"Nothing about us without us"

# **NIHR** INVOLVE

**Involvement** is research being carried out **'with'** or **'by'** members of the public rather than 'to', 'about' or 'for' them".

It is not:

- Raising awareness of research
- Disseminating research to patients and public
- Recruitment of patients as participants in research



### Examples

- Investigators on a research project
- Identifying priorities, interventions, outcomes
- Members on a Steering Committee or Advisory Group
- Developing and providing feedback on research materials (e.g. information and consent forms)
- Collecting and analyzing data



### Lancet 2014; 383: 156–65 Research: increasing value, reducing waste 1

# How to increase value and reduce waste when research priorities are set

Iain Chalmers, Michael B Bracken, Ben Djulbegovic, Silvio Garattini, Jonathan Grant, A Metin Gülmezoglu, David W Howells, John P A Ioannidis, Sandy Oliver

### US \$240 billion → 85% wasted



Mean difference in rating between patients/caregivers and health professionals

Ability to travel **Dialysis-free time** Dialysis adequacy Washed out after dialysis Anaemia Mobility **Blood** pressure Fatigue Impact on family/friends Pain Ability to work Potassium Infection/Immunity Target weight Cardiovascular disease Depression Vascular access problems Drop in blood pressure Hospitalisation Death/mortality 1.5



# Why do we need to involve patients?



Recommended by global organizations



## To get funded



To get published



To improve translation to practice and policy

# Levels of involvement





# The research cycle



Breakout Discussion

Annual Section of the section of the

and a - The same state one integer much sharrow if series to no. We make up on no. Wepd - The same same series series approxi-- where same series second

# M-FIT

Mobile exercise app to improve Fatigue In patients on dialysis: an adaptive Trial

Top patient priorities: lifestyle + fatigue Co-produced: acceptable, feasible, sustainable Population: patients on dialysis Interventions: mobile exercise app (e.g. walking, resistance) Outcome: Fatigue (primary)

> I learned that through exercise and pushing myself a little bit... it's gotten to a point now I can live a quality of life. – patient



# Core outcomes



Trials that don't address problems of relevance to end-users of research cannot inform decisions.

**362 trials**: 20% trials report mortality; 12% report CVD, 9% report QoL



Without a common outcome measure, we cannot compare effects of interventions across trials.

81 outcome domains (10713 outcome measures)



Resources are wasted when outcomes are measured and reported inconsistently.

Mortality reported in 48 different ways, CVD reported in 47 different ways)

# The SONG Initiative

To establish core outcomes for research (trials) across CKD







SONG-HD Haemodialysis



SONG-GD Glomerular Disease More than 4000 patients/caregivers & 5000 health professionals

## 100 countries

SONG-PKD olvevstic kidnev disease SONG HANDBOOK

GET INVOLVED



# PROCESS: evidence + consensus



#### References

Kirkham et al. Core Outcome Set-STAndards for Reporting: The COS-STAR Statement. PLoS Med 2016;13:e1002148 Prinsen et al. How to select outcome measurement instruments for outcomes included in a "Core Outcome Set" - a practical guideline. Trials 2016; 17:449 SONG Handbook http://songinitiative.org/reports-and-publications/



## SONG-HD

Hemodialysis



#### 1 CORE **OUTCOMES**

Critically important to all stakeholder groups Report in all trials

#### 2 MIDDLE TIER

Critically important to some stakeholder groups Report in some trials

#### **3 OUTER TIER**

Important to some or all stakeholder groups Consider for trials

Ability to travel

Ability to work Anemia

Pain

**Target weight** 

Washed out after dialysis

FATIGUE **CARDIOVASCULAR** DISEASE **VASCULAR ACCESS MORTALITY** 

Blood pressure Depression **Dialysis adequacy Dialysis-free time Drop in blood pressure** Hospitalization Impact on family/ friends Infection/immunity Mobility Potassium

3

Anxiety/stress Bone health Calcium Cognition Cramps **Financial impact** Food enjoyment Itching Nausea/vomiting **Parathyroid hormone Phosphate Restless legs syndrome Sexual function** Sleep









## 5 | Clinical implications

#### Symptoms

Fatigue Mobility Pain Stress/anxiety Depression Cognitive impairment Sleep problems Cramps Restless legs Gastrointestinal symptoms

#### Life impacts

Ability to work Ability to travel Ability to study Impact on family and friends Financial impact Dialysis-free time Dietary restrictions Lifestyle changes Social activities



## Strengths-based approach

Communication and education Build resilience Strengthen social connections Increase awareness and knowledge Access to support Build confidence and control with self-management

#### **Clinical strategies**

Preserve kidney function Patient-friendly lifestyle and diet Pharmacological management Delay dialysis start if possible Incremental transition to dialysis Patient-centered dialysis prescriptions Preserve residual kidney function

## Figure 1 | Conceptual framework of "Living Well with Kidney Disease" based on patient centeredness and empowering patient, with a focus on effective symptom management and life participation.

Kamyar Kalantar-Zadeh, Philip Kam-Tao Li, Ekamol Tantisattamo et al., Living well with kidney disease by patient and care partner empowerment: kidney health for everyone everywhere, *Nephrology Dialysis Transplantation*, Volume 36, Issue 2, February 2021, Pages 197–201, <a href="https://doi.org/10.1093/ndt/gfaa336">https://doi.org/10.1093/ndt/gfaa336</a>



#### www.songinitiative.org

Øsong\_initiative

**SONG contact:** allison.tong@sydney.edu.au