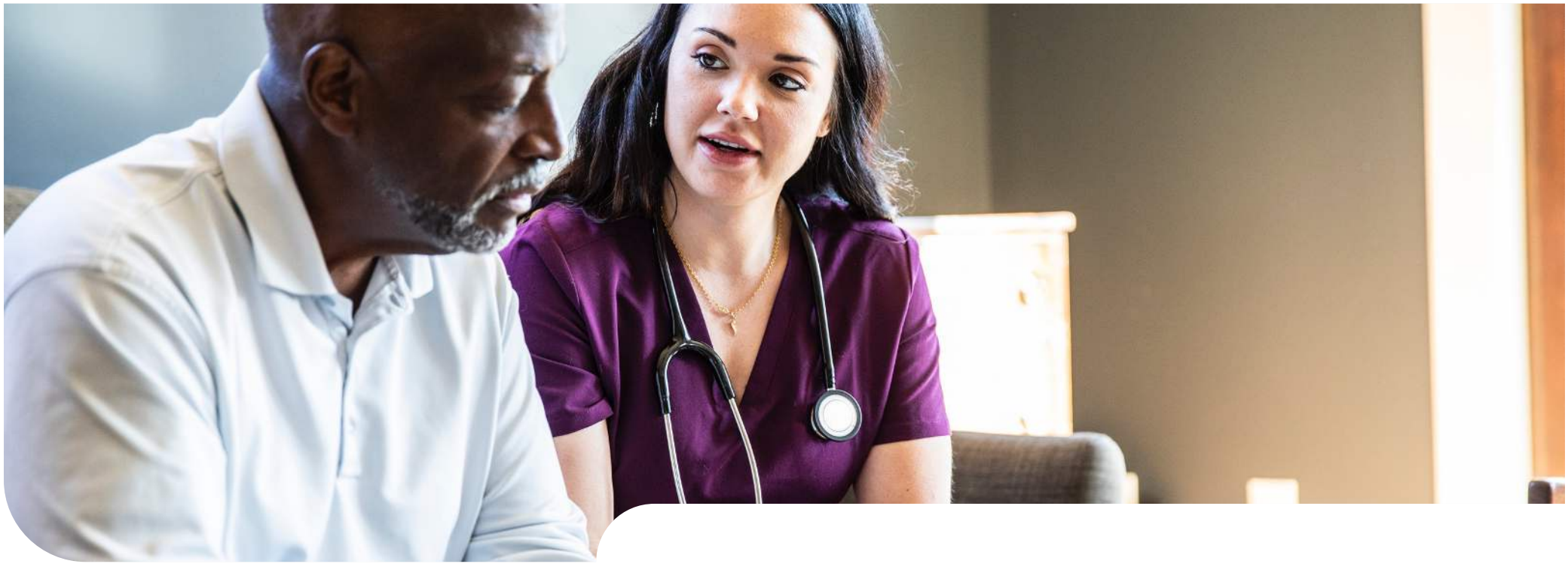

Participatief onderzoek in de huidige kennismaatschappij

For the times they are a-changing

UMC Utrecht

Astrid Janssens

Julius Center voor Gezondheidswetenschappen en Eerstelijnsgeneeskunde, Bioethics & Health Humanities

Patiëntenparticipatie is *hot*

Korte geschiedenis van een aangekondigd momentum

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Increasing value, reducing waste. (Lancet, 2014)

The Lancet
https://www.thelancet.com

The Lancet | The best science for better lives

The best science for better lives. Explore the latest high-quality research from **The Lancet** Group.

Patiëntenparticipatie als *oplossing*

Biomedical research: increasing value, reducing waste

Malcolm R Macleod, Susan Michie, Ian Roberts, Ulrich Dirnagl, Iain Chalmers, John P A Ioannidis, Rustam Al-Shahi Salman, An-Wen Chan, Paul Glasziou

Full-Text HTML | PDF

Series Papers

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

Full-Text HTML | PDF

Increasing value and reducing waste in research design, conduct, and analysis

John P A Ioannidis, Sander Greenland, Mark A Hlatky, Muin J Khoury, Malcolm R Macleod, David Moher, Kenneth F Schulz, Robert Tibshirani

Full-Text HTML | PDF

Increasing value and reducing waste in biomedical research regulation and management

Rustam Al-Shahi Salman, Elaine Beller, Jonathan Kagan, Elina Hemminki, Robert S Phillips, Julian Savulescu, Malcolm Macleod, Janet Wisely, Iain Chalmers

Full-Text HTML | PDF

Increasing value and reducing waste: addressing inaccessible research

An-Wen Chan, Fujian Song, Andrew Vickers, Tom Jefferson, Kay Dickersin, Peter C Gøtzsche, Harlan M Krumholz, Davina Ghersi, H Bart van der Worp

Full-Text HTML | PDF

Reducing waste from incomplete or unusable reports of biomedical research

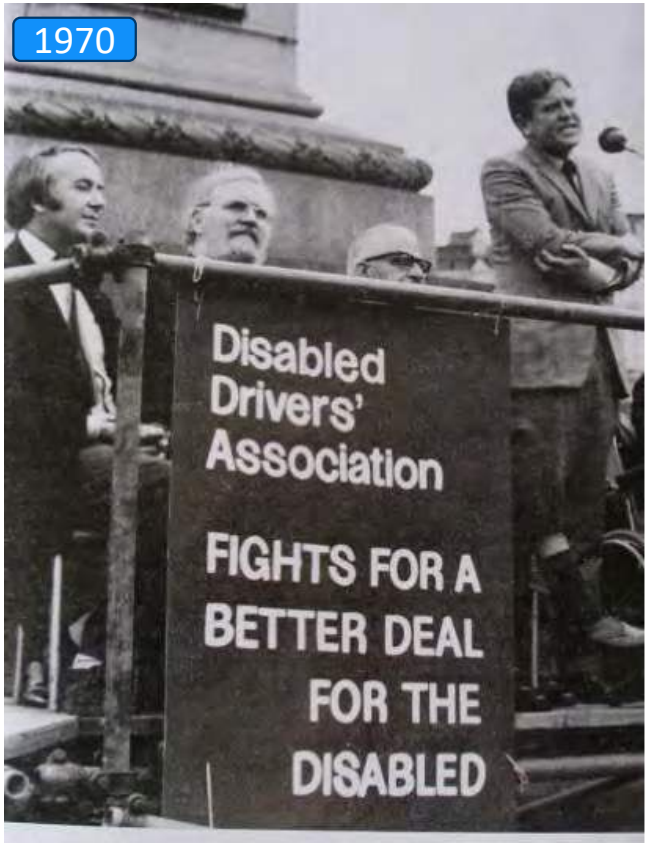
Paul Glasziou, Douglas G Altman, Patrick Bossuyt, Isabelle Boutron, Mike Clarke, Steven Julious, Susan Michie, David Moher, Elizabeth Wager

Full-Text HTML | PDF

Universiteit Utrecht

UMC Utrecht

Patiëntenparticipatie: Grassroots movement



For the times they are achanging, B Dylan
Come gather 'round people
Wherever you roam
And admit that the waters
Around you have grown
And accept it that soon
You'll be drenched to the bone
If your time to you is worth savin'
And you better start swimmin'
Or you'll sink like a stone
For the times they are a-changin'

Afbeeldingen: People's History Museum; The Disability movement; Mario Suriani / Associated Press

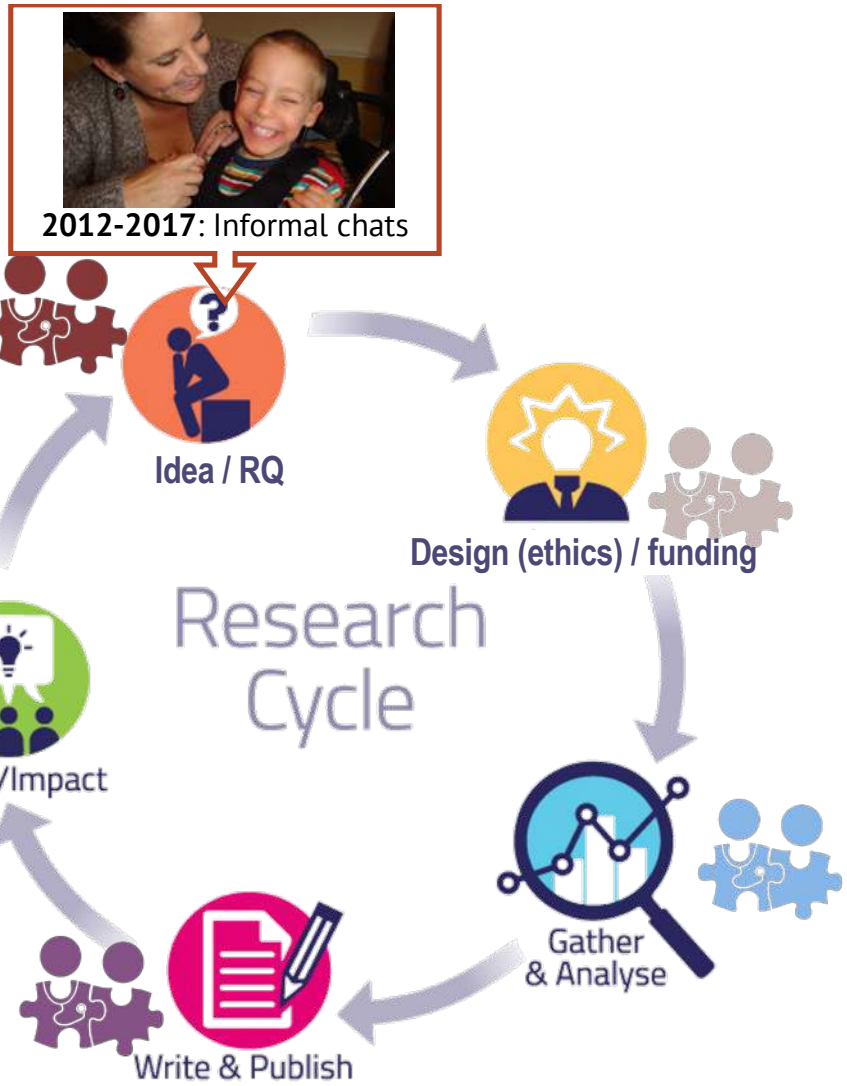
UMC Utrecht

Patiëntenparticipatie (in onderzoek)

Waar hebben we het over?

Parent Carer Wellbeing project: Julia

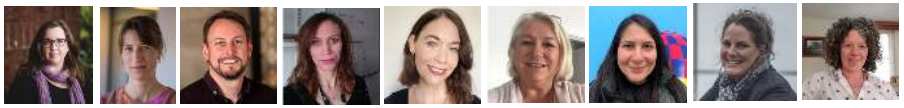
Parent Carer Wellbeing project - Julia



Parent Carer Wellbeing project

2017-19: Create team

Experts by profession ⟷ Experts by experience



Nov 2019-May 2020:
1) Study Design
2) Two rounds NIHR funding

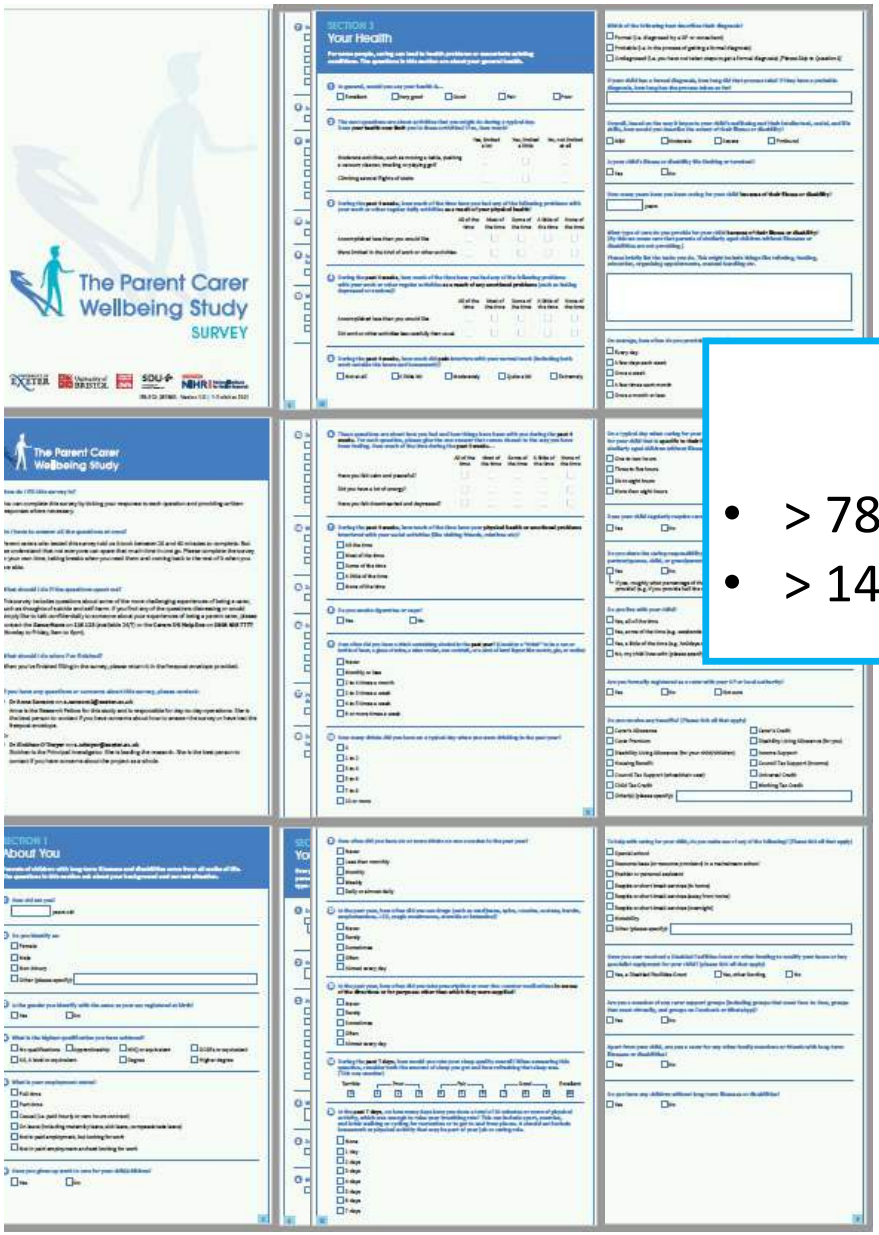
Aug 2020: NIHR grant awarded - £240,871.00

Summer 2021
- Ethics approval
- Draft survey

Parent Carer Wellbeing project

Nov 2021
- Survey open
- Recruitment video

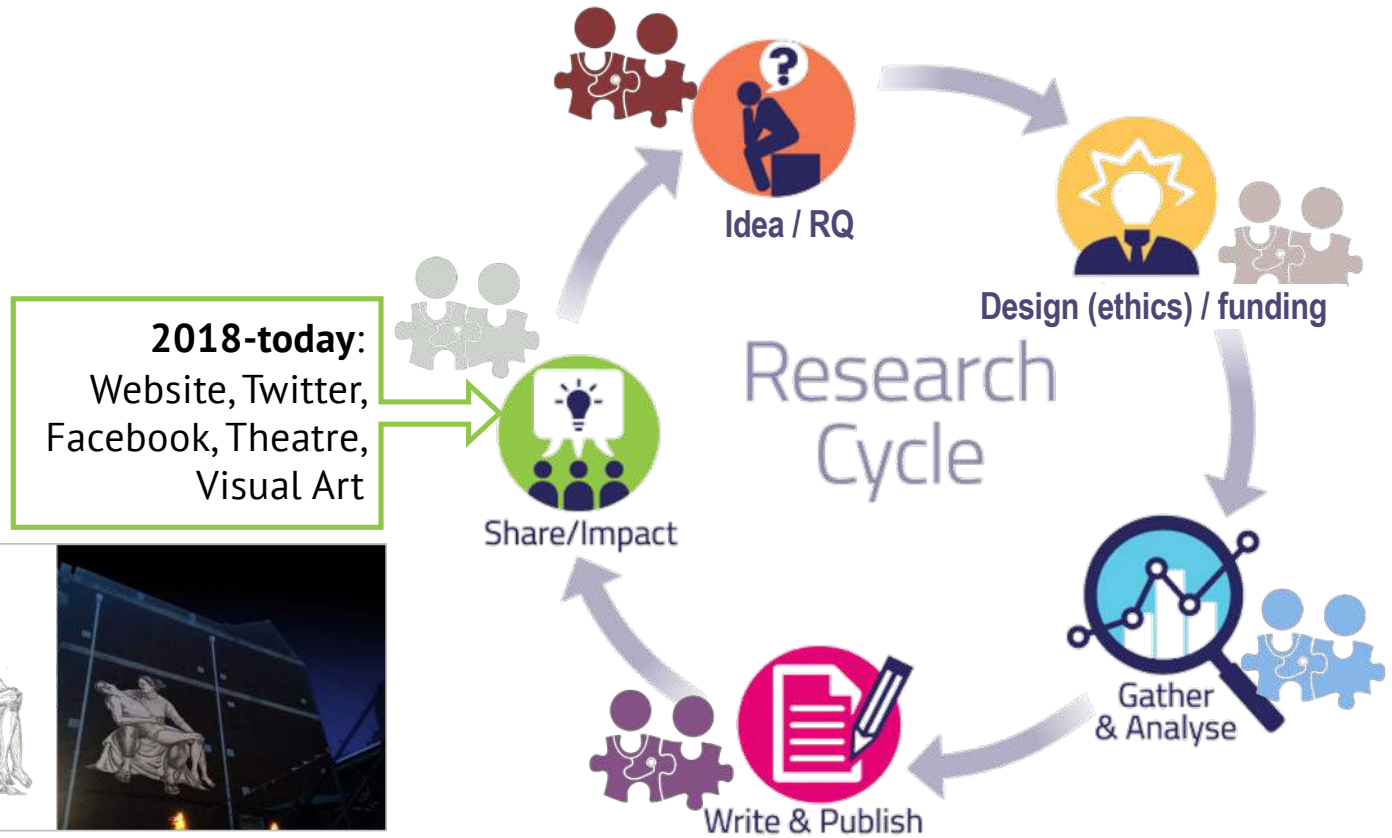
Jan-Mar 2022
- Interview guide & consent forms
- Trial Interviews
- Safety protocol



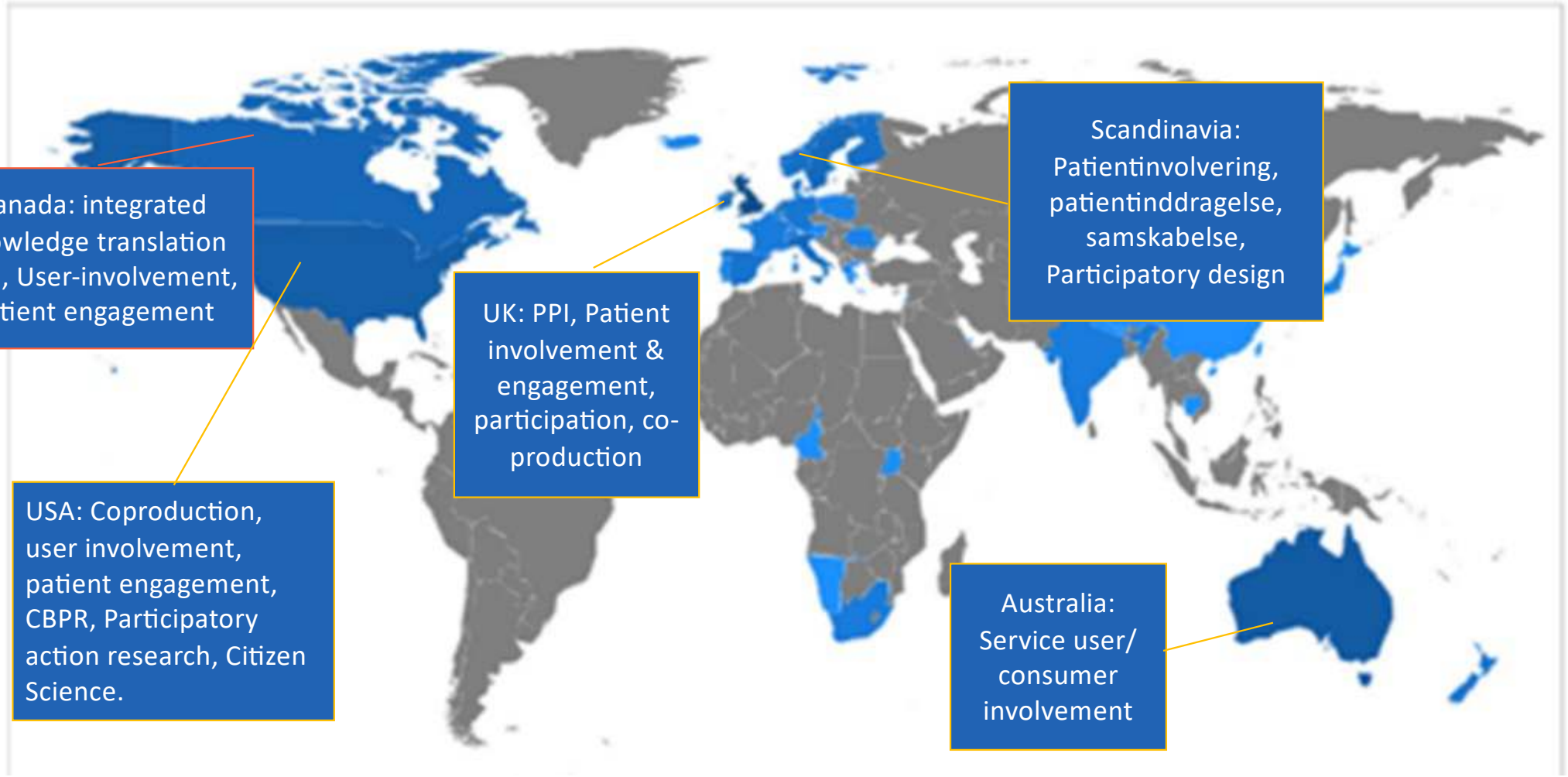
maart 2022 (survey -32pg)

- > 780 ouders vulden de vragenlijst in
- > 140 registreerden zich voor interview

Parent Carer Wellbeing project



Patiëntenparticipatie



Patiëntenparticipatie (in onderzoek)

Review uitgevoerd door Patient Engagement in Research Working Group of the ISPOR Patient-Centered Special Interest Group (PCSIG)

- 169 articles
- 244 distinct definitions

The active, meaningful, and **collaborative interaction** between patients and researchers across all stages of the research process, where research decision making is *guided** by patients' contributions as partners, recognizing their specific experiences, values, and expertise.

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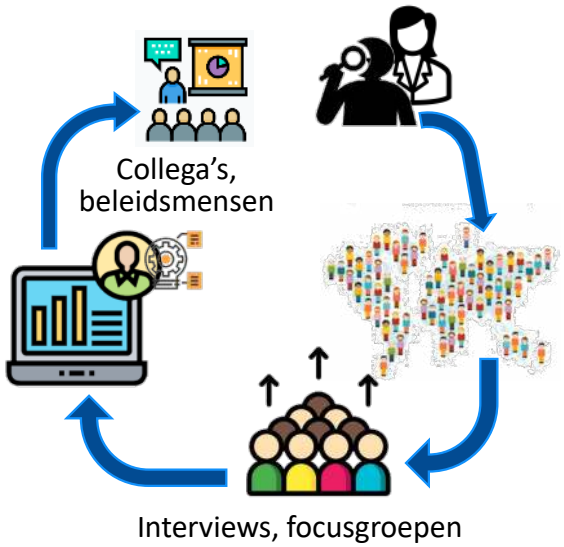
Harrington, R. L., et al. (2020). Defining Patient Engagement in Research: Results of a Systematic Review and Analysis: Report of the ISPOR Patient-Centered Special Interest Group. *Value Health*, *23*(6), 677-688. https://doi.org/10.1016/j.jval.2020.01.019

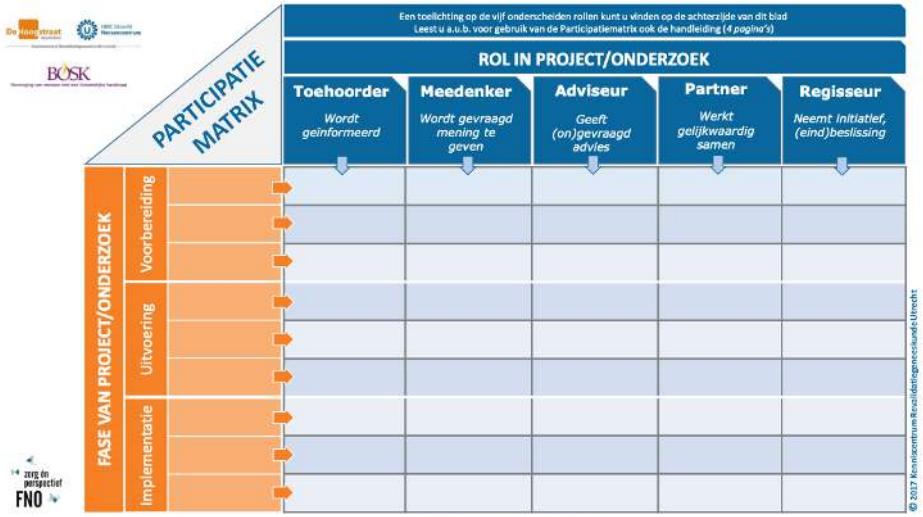
Patiëntenparticipatie en kwalitatief onderzoek: same same?

Traditioneel onderzoek: Patiënt = SUBJECT	Participatief onderzoek: Patiënt = deel van het proces (PARTNER)
STUDY DESIGN	GEEN STUDY DESIGN – Manier van onderzoek doen

- - Stem van de patiënt - -

Indirect & geen macht — (in)direct & inspraak / macht





- Toehoorder: wordt geïnformeerd
- Meedenker: wordt om mening gevraagd
- Adviseur: geeft (on)gevraagd advies
- Partner: werkt gelijkwaardig samen
- Regisseur: neemt initiatief, (eind)beslissing

gaat over samenwerken met patiënten en burgers om onderzoek / onderwijs / zorg vorm te geven. De mate van betrokkenheid kan variëren, er is bijna altijd sprake van tweerichtingscommunicatie.

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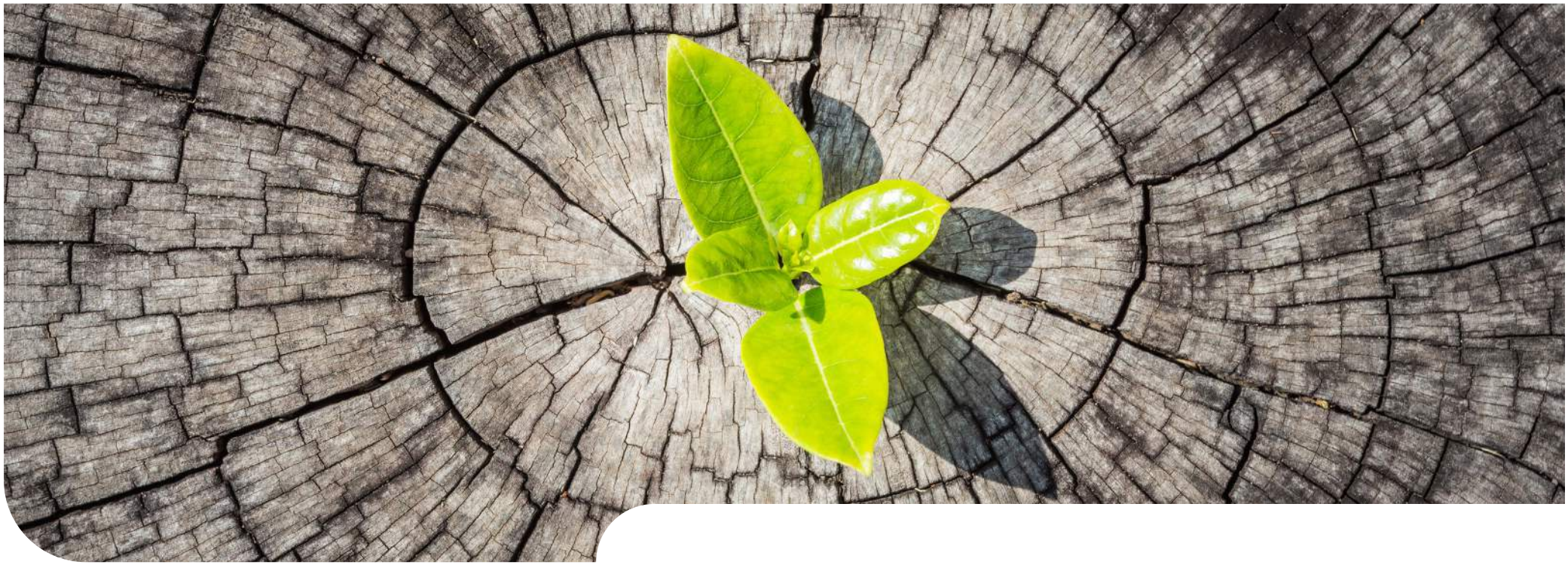
Smits, D.W., et al., *Designing a tool to support patient and public involvement in research projects: the Involvement Matrix.* Res Involv Engagem, 2020. **6**: 30.



Het momentum is nu

Waar staan we?

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Wat weten we? Trials

1. Beter trial design:
    a) relevant outcomes & study endpoints (COMET Initiative 2016)
    b) patiënt-vriendelijke protocols
2. Ethische overwegingen en patiëntveiligheid (Staley 2015)
3. Werving, retentie ↗ (Crocker 2018)
4. Verhoogde relevantie en impact van onderzoeken: ↗ implementatie
5. Kostenbesparingen (tijdsbesparing bij het op de markt brengen van medicatie; Levitan, 2018)

UMC Utrecht

Staley, K. (2015). 'Is it worth doing?' Measuring the impact of patient and public involvement in research. *Res Inv Eng*, *1*(1), 6. https://doi.org/10.1186/s40900-015-0008-5
Crocker, J. C., et al. (2018). Impact of patient and public involvement on enrolment and retention in clinical trials: systematic review and meta-analysis. *BMJ*, k4738. https://doi.org/10.1136/bmj.k4738
Levitan, B., et al. (2018). Assessing the Financial Value of Patient Engagement: A Quantitative Approach from CTTI's Patient Groups and Clinical Trials Project. *Therapeutic Innovation & Regulatory Science*, *52*(2), 220-229. https://doi.org/10.1177/2168479017716715

Enkele cijfers: PP in (medisch) onderzoek

- BMJ: papers die PP activiteit rapporteren (2014 – PP richtlijn)
  - juni 2013-mei 2014: 1/189 papers (0.5%)
  - Juni 2015-mei 2016: 16/152 papers (11%)
- BMJ Open, PY: 2020 papers bestudeerd voor patiëntenparticipatie
- PERCENTAGE papers met PP in Nederland?

A. 10% < x < 20%

B. 20% < x < 30%

C. > 30%

Price, A., et al., *Frequency of reporting on patient and public involvement (PPI) in research studies published in a general medical journal: a descriptive study.* BMJ Open, 2018. **8**(3): p. e020452.

Enkele cijfers: PP in (medisch) onderzoek

- BMJ: papers die PP activiteit rapporteren (2014 – PP r
  - juni 2013-mei 2014: 1/189 pap
  - Juni 2015-mei 2016: 16/152 pap

- BMJ Open PY: 2020, 3000 papers
  - Overall: [redacted]
  - UK: [redacted]
  - Nederland: [redacted]

Table 1 Number of papers published in BMJ Open in 2020 by location, and proportion of these with PPI

Location	n	% of total	Mean % PPI (95% CI)
UK	591	19.7	44.5 (40.5 to 48.5)
Canada	195	6.5	30.8 (24.3 to 37.3)
Australia/New Zealand	269	9.0	21.9 (17.0 to 26.9)
France	118	3.9	21.2 (13.8 to 28.6)
Netherlands	120	4.0	20.8 (13.5 to 28.1)
Germany	101	3.4	16.8 (9.5 to 24.2)
USA	227	7.6	16.3 (11.5 to 21.1)
Europe (other)	501	16.7	15.5 (12.4 to 18.7)
Central and South America	59	2.0	11.3 (3.9 to 18.7)
Central and South Asia	72	2.4	10.2 (2.4 to 18.0)
Africa and West Asia	163	5.4	8.0 (3.8 to 12.1)
East and Southeast Asia	232	7.7	6.4 (3.3 to 9.6)
China	352	11.7	3.4 (1.5 to 5.3)
	3000	100.0	20.6 (19.1 to 22.0)

Note that location here means each country with ≥100 papers and otherwise means region.
PPI, patient and public involvement.

Table 3 Number of papers published in BMJ Open in 202 in each research topic with ≥50 papers, and proportion of these with PPI

Research topic	n	% of total	Mean % PPI (95% C
Mental health	149	5.0	36.9 (29.1 to 44.7)
Qualitative research	51	1.7	35.3 (22.0 to 48.5)
Health services research	168	5.6	31.0 (23.9 to 38.0)
Neurology	82	2.7	29.3 (19.4 to 39.2)
Surgery	102	3.4	28.4 (19.6 to 37.2)
Rehabilitation medicine	65	2.2	26.2 (15.4 to 36.9)
Oncology	88	2.9	25.0 (15.9 to 34.1)
Cardiovascular medicine	120	4.0	24.2 (16.5 to 31.9)
Paediatrics	91	3.0	23.1 (14.4 to 31.8)
Diabetes and endocrinology	85	2.8	22.4 (13.4 to 31.3)
Obstetrics and gynaecology	98	3.3	21.4 (13.3 to 29.6)
Emergency medicine	66	2.2	21.2 (11.3 to 31.2)
General practice/ family practice	94	3.1	20.2 (12.0 to 28.4)
Public health	366	12.2	16.1 (12.3 to 19.9)
Infectious diseases	79	2.6	15.2 (7.2 to 23.2)
Global health	97	3.2	13.4 (6.6 to 20.2)
Health economics	61	2.0	6.6 (0.3 to 12.8)
Epidemiology	241	8.0	6.2 (3.2 to 9.3)
Medical education and training	58	1.9	3.4 (–1.3 to 8.2)
	1261	71.8	

PPI, patient and public involvement.

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Lang, I., et al., *How common is patient and public involvement (PPI)? Cross-sectional analysis of frequency of PPI reporting in health research papers and associations with methods, funding sources and other factors.* BMJ Open, 2022. **12**(5): p. e063356.

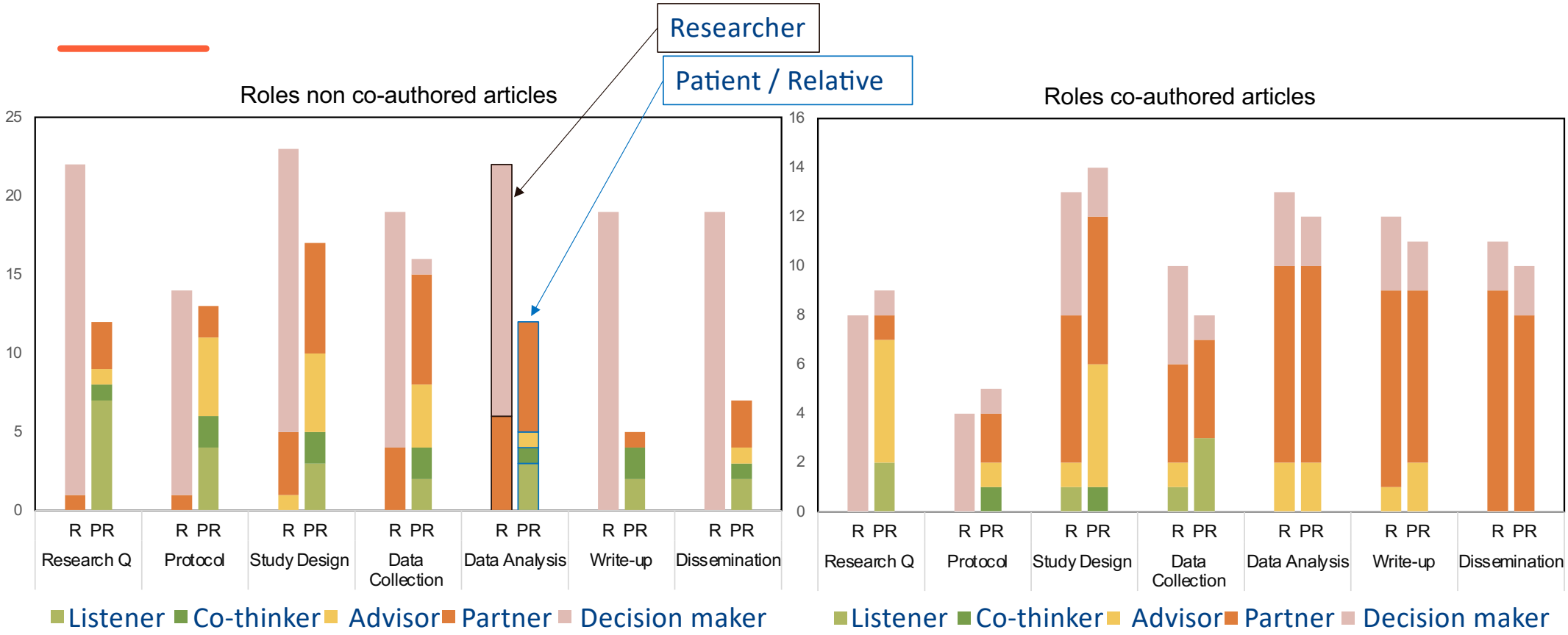
Enkele cijfers: PP in (medisch) onderzoek

- Studie 2021:
  - 27 general nursing journals
  - 89 randomised controlled clinical trials
  - Aantal studies die PP rapporteren: 

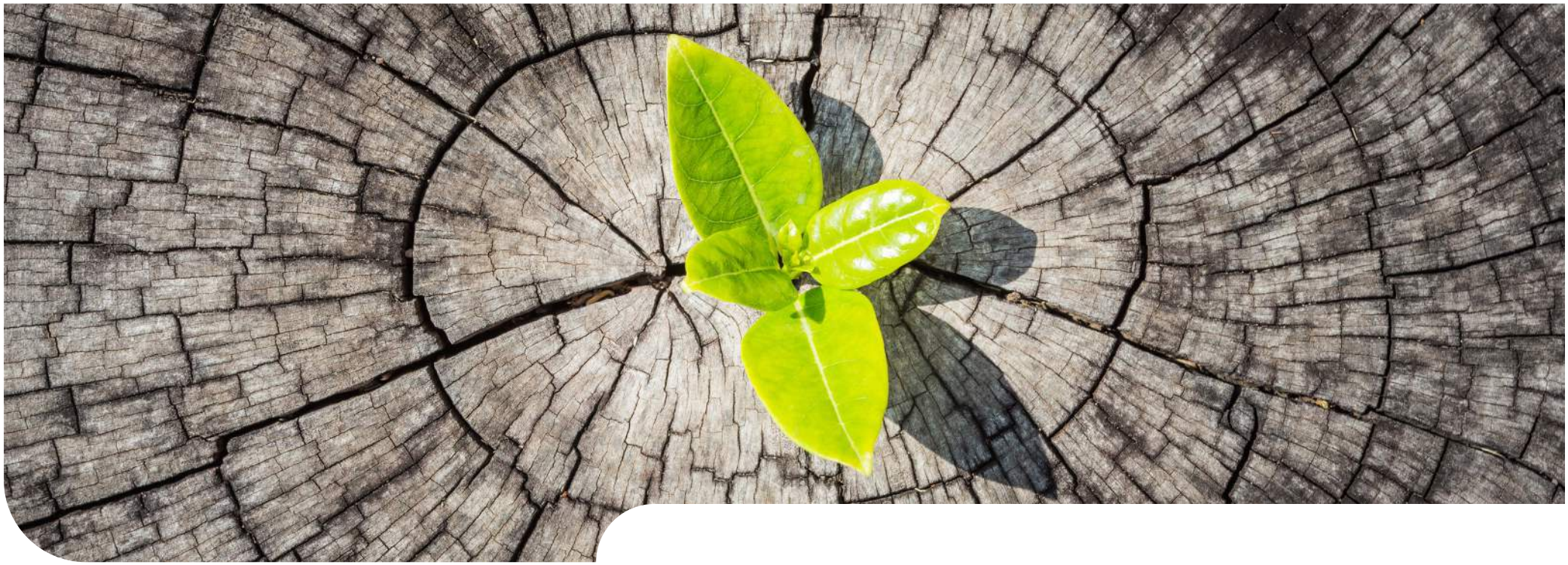
A. 0-10%
B. 10-40%
C. >40%

Gray, R., et al., *Reporting of patient and public involvement and engagement (PPIE) in clinical trials published in nursing science journals: a descriptive study.* Res Involv Engagem, 2021. **7**(1): p. 88.

Rol van patiënten in onderzoek



Karlsson, A.W., et al., *Roles, outcomes, and enablers within research partnerships: A rapid review of the literature on patient and public involvement and engagement in health research.* Research Involvement and Engagement, 2023. **9**(1).



Van momentum naar verandering

De *kansen* en *gevaren* van top-down patiëntenparticipatie

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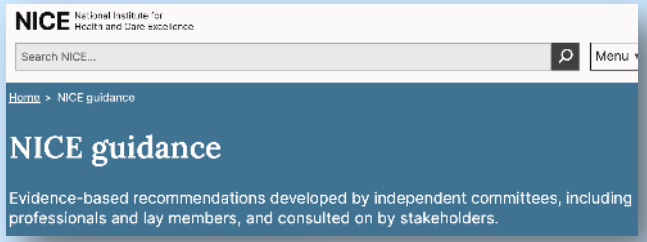
Bottum-up → Top-down

- Richtlijnen
- Onderzoeksprioriteiten



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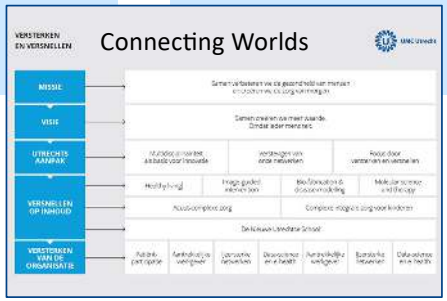
PP verwacht / verplicht:

- BMJ (British Medical Journal)
- EMA
- EU – Funding
- CCMO (Centrale Commissie Mensgebonden Onderzoek)
- UMC Utrecht / UU, OUH



Verandering

Patiëntenparticipatie. Top-down versus bottum-up.

patiëntenparticipatie = ‘technical matter’

PP = a way of being / doing

omdat ik moet (bv. CCMO, UMC Utrecht)

het levert me wat op

mensenrechten & methodische aanpak

mensenrechten, democratisering

Instrumentalist / consumerist
PP = means to an end

(pragmatic) ideologist
PPI = has value in itself

Professioneel/helikopter perspectief Diverse groep Al wie wil bijdragen

- Diversiteit in patiënten
- Bijdrage patiënten ≠
- PP ervaring patiënten ≠
- Motivatie onderzoeker: intrinstiek / extrinsiek
- Schade?

UMC Utrecht

Karlsson, A.T., et al., Wider institutional research cultures and their influence on patient and public involvement and engagement in health research – An institutional ethnography. Social Science & Medicine, 2023, under review.

De huidige kennismaatschappij

It’s a knowledge world and it’s ruled by knowers

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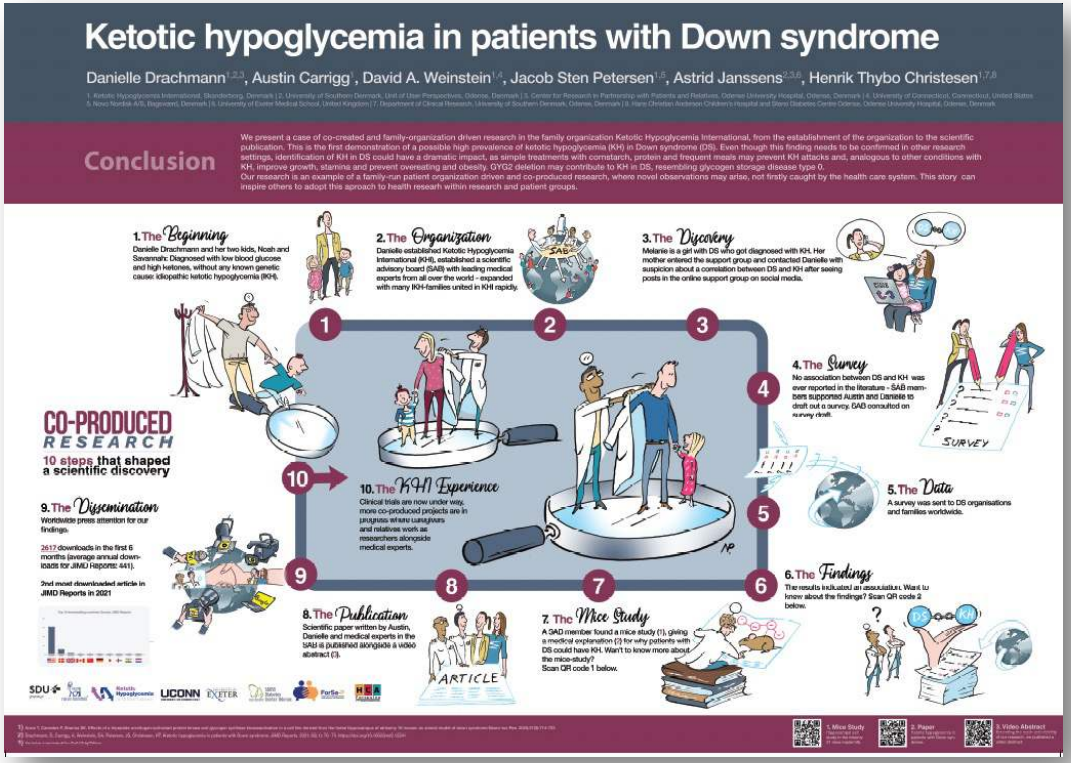
Kennisbijdrage van patiënten

1. Unknown unknows



Received: 29 June 2020 | Revised: 30 June 2021 | Accepted: 2 July 2021
DOI: 10.1002/jmd2.12241

RESEARCH REPORT

JIMD REPORTS SSIEM WILEY

Ketotic hypoglycemia in patients with Down syndrome

Danielle Drachmann[1,2] | Austin Carrigg[1] | David A. Weinstein[3] | Jacob Sten Petersen[4] | Henrik Thybo Christesen[5,6]

[1]Ketotic Hypoglycemia International (KHI), Skanderborg, Denmark
[2]The Danish Committee for Health Education, Copenhagen, Denmark
[3]Glycogen Storage Disease Program, Department of Pediatrics, University of Connecticut, Farmington, Connecticut, USA

ABSTRACT

Background: Ketotic hypoglycemia (KH) without an identifiable underlying metabolic or hormonal disease is historically named idiopathic KH. The prevalence is unknown, but idiopathic KH is considered the most frequent cause of hypoglycemia beyond the neonatal period. KH in Down syndrome (DS) has not been reported.

Hyper-nursing in children later found to have ketotic hypoglycemia

Project description

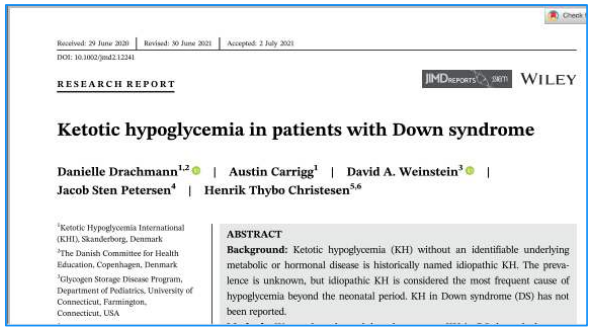
Background

Ketotic hypoglycemia (KH) is the most frequent form of hypoglycemia after the neonatal period. KH can be divided into physiological KH and pathological KH (1). The first-line treatment is dietary with frequent meals and complex carbohydrates. Pathological KH is often an overlooked condition with delayed diagnosis, which may prompt mothers to extend the frequency and duration of breastfeeding to relieve undiagnosed hypoglycemia in their children. This so-called hyper-nursing has been identified among mothers in the global patient organization Ketotic Hypoglycemia International (KHI; www.ketotichypoglycemia.org).

Many factors affect breastfeeding patterns and duration (2,3). The term hyper-nursing has not been described or defined in the literature. The World Health Organization (WHO) recommends that infants are exclusively breastfed for six months (4). The knowledge on the benefits and adverse effects of breastfeeding for longer than 12 months is sparse (5).

Kennisbijdrage van patiënten

1. Unknown unknows



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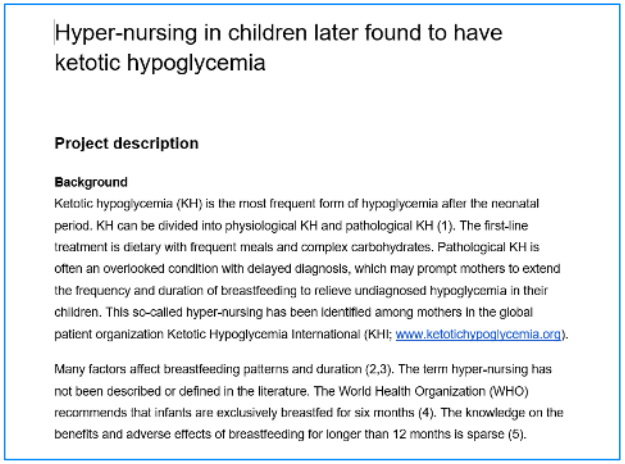

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Many factors affect breastfeeding patterns and duration (2,3). The term hyper-nursing has not been described or defined in the literature. The World Health Organization (WHO) recommends that infants are exclusively breastfed for six months (4). The knowledge on the benefits and adverse effects of breastfeeding for longer than 12 months is sparse (5).

2. Inzicht in relevantie van (aspecten) van een aandoening / behandeling
3. Data verzameling ‘op maat’

UNIVERSITY OF EXETER | University of BRISTOL | CARDIFF UNIVERSITY PRIFYSGOL CAERDYDD | SDU University of Southern Denmark | FUNDED BY NIHR | National Institute for Health Research

The Parent Carer Wellbeing Study

April 2021
Start of the Parent Carer Wellbeing project

De *machts*verhoudingen in de huidige kenniswereld

Wat is KENNIS en wie is DRAGER van kennis

De geprivilegeerde kennisstatus van de onderzoeker / hulpverlener:

⇒Epistemische autoriteit & geloofwaardigheid HVL

⇒Selectieve getuigenis van patiënt

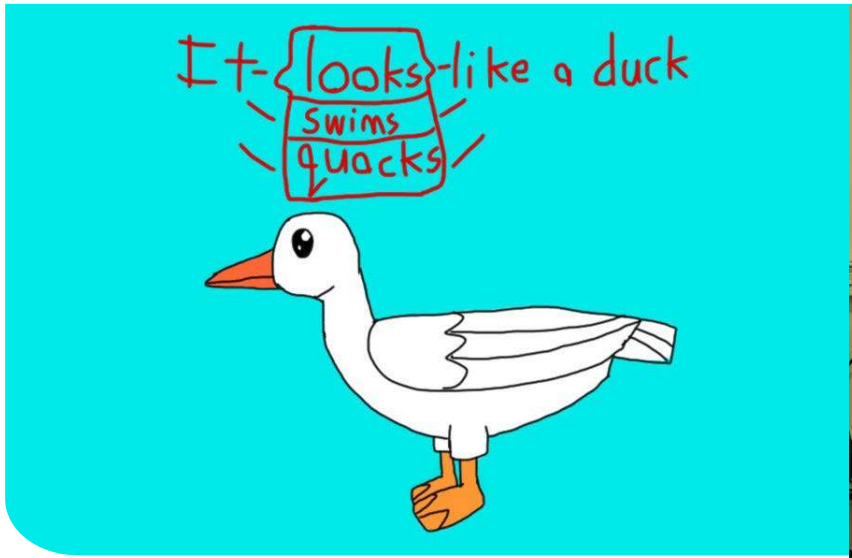
⇒Interpretatieve onrechtvaardigheid (Hermeneutical injustice)

Fricker, M., *Epistemic injustice. Power & ethics of knowing*. 2007, Oxford Scholarship Online: Oxford.

Epistemische uitdagingen

- (Epistemische) autoriteit
- Epistemische scheiding
- Geloofwaardheid

- Hermeneutische assimilatie



Huidige gezondheidszorg / onderzoekswereld ≠ epistemisch onrechtvaardig,
MAAR faciliteert epistemische onrechtvaardigheid.

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De toekomst ~~van~~ is patiëntenparticipatie

Moment van reflectie

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Tol van het succes van (top-down) patiëntenparticipatie?

- Rol van patiëntenorganisaties
- Over je grenzen gaan
- Schade berokkenen (ethisch kader?)
- Diversiteit (health inequality?)
- Vergoeding (transparantie)
- Opleiding, vorming (wat, wie)

Call for action

Actief werken aan een epistemisch rechtvaardige setting

Patiënten moeten kunnen deelnemen onder voorwaarden die zowel gelijkwaardig zijn als recht doen de unieke aard van de ervaringen en kennis die zij – evenals anderen die bijdragen – inbrengen.