Dutch
Pituitary
Foundation

The role of patients in research

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Almere, May 5th 2025

European
Patient
Advocacy
Group

Affiliated with
European Society
of Endocrinology

Endo-ERN

European
Reference
Network

“Niets over ons, zonder ons”

‘Nothing about us, without us’

Manifest Think along and participate in decision making,
Ieder(in), MIND, Patiëntenfederatie, 2020

What I want to share today with you

- Experiential knowledge and expertise
- Usefulness and necessity of using experiential knowledge or participation
- Different approaches with the aim acchieving the same
- Practical use – example of EJPRD/ERDERA
- Renumeration of patient experts

The different roles of patients

Subject within research projects (part of the cohort)

The patient that shares experiences

- Own experiences (N=1)
- Experiences of others → Patient representative

- Patient representatives and or organisations undergo a developmental process (will come back to that at the end)

The patient and or patient expert, what can they do?

Patient advisory boards in healthcare organisations

Participants in research

Patient investigator

Sharing personal experiences of the condition with HCPs

Giving lectures for educational purposes

Developing standards of care

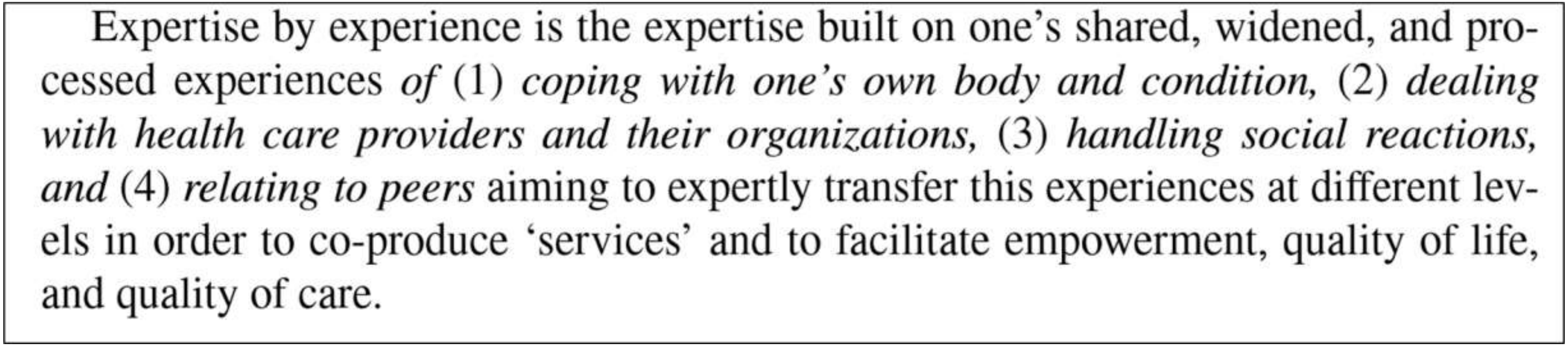
Participant in focus groups, giving interviews, etc.

Assessing scientific research proposols

Co-creating research proposals

And all of that without of course an alleged conflict of interest....

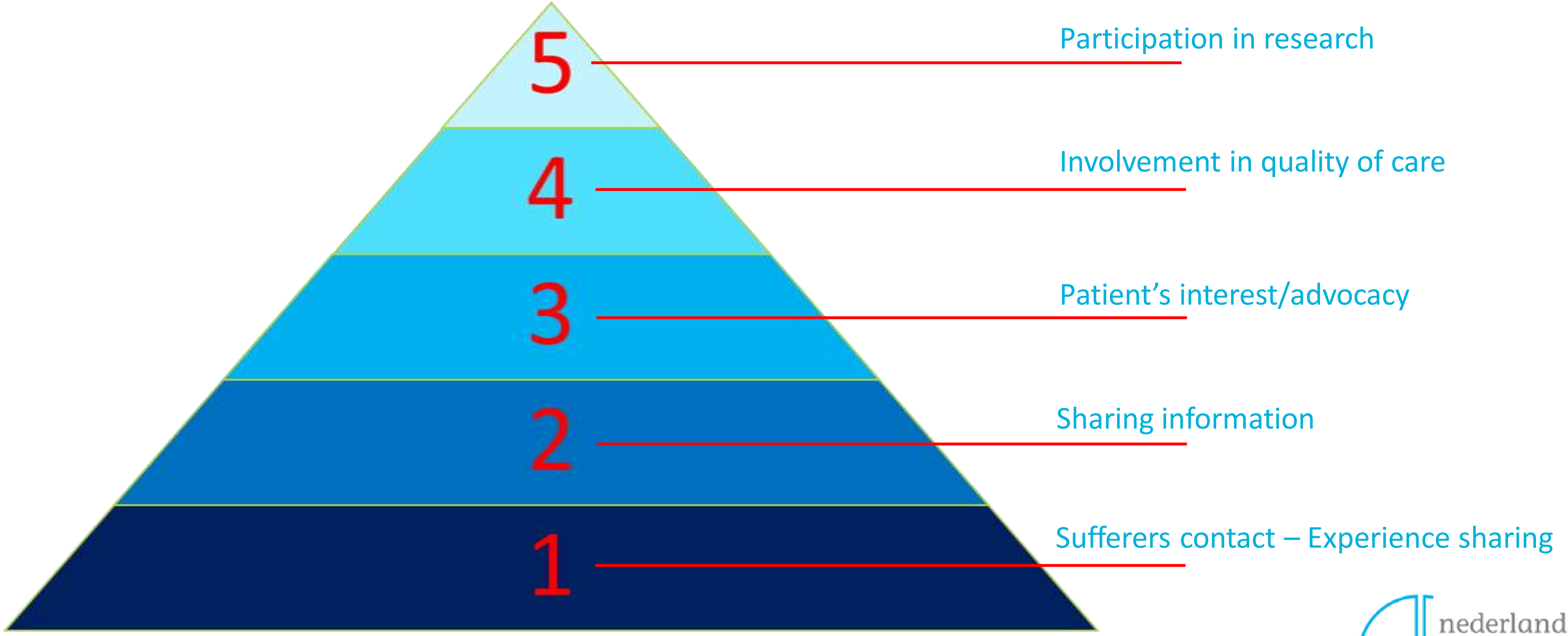
Definition of patient expertise



Expertise by experience is the expertise built on one's shared, widened, and processed experiences *of* (1) *coping with one's own body and condition,* (2) *dealing with health care providers and their organizations,* (3) *handling social reactions, and* (4) *relating to peers* aiming to expertly transfer this experiences at different levels in order to co-produce 'services' and to facilitate empowerment, quality of life, and quality of care.

Castro, E. M., Van Regenmortel, T., Sermeus, W., & Vanhaecht, K. (2018). Patients' experiential knowledge and expertise in health care: A hybrid concept analysis. *Social Theory & Health*, *17*(3), 307–330. https://doi.org/10.1057/s41285-018-0081-6

Development phases of patient organisations/ patient representatives



Participation in research

Involvement in quality of care

Patient’s interest/advocacy

Sharing information

Sufferers contact – Experience sharing

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Free after Maslow’s hierarchy model of needs

Why is patient involvement important?

- Research needs to provide an answer of the unmet needs of patients and caregivers
- Research will improve the relevance of research
- Important themes of clients and relatives come to light
- Better access to the target group and thus more involvement in projects, research and policy
- Better dissemination and exploitation of results

Involvement of patients: different visions, same goals

- The participation ladder (based on Arnstein, 1969)
- Involvement matrix of the UMCU (Smits, Van Meeteren et al, 2020)
- Patient Partnerships in rare disease research projects (Eurordis, 2020)

Participation ladder

- Step I inventory of needs and experiences
- Step II Generate ideas
- Step III Advising
- Step IV Testing and assessing
- Step V (co)-producing

Participation ladder step I

- Step I inventory of needs and experiences

Characteristics:

- Doesn’t require much time or efforts of the patient;
- No understanding of the research is required
- Can often be done digitally, in writing or at the patient’s home
- A large group can participate, also those with limited resilience or without advanced education

Points of attention:

- Patient has no say in what or how the research is performed;
- Patient is a passive source of information

Participation ladder step II

- **Step II Generate ideas**

Characteristics:

- Doesn’t require much time or efforts of the patient;
- No understanding of the research is required
- Can often be done digitally, in writing or at the patient’s home;
- Low threshold for researchers and patients
- A large group can participate;
- Limited opportunities for the patient to influence the research

Points of attention:

- Patient has no say in what or how the researuc is performed;
- Patient is a passive source of information
- No sense of ownership by the patient;
- Risk of mock participation

Participation ladder step III

- Step III Advising

Characteristics:

- Patient has an active role in the research project
- experiential knowledge has a well defined formal place within the project
- Researcher needs to justify decisions to the patient

Points of attention:

- The researcher decides
- Possible not useful for a part of the patient group, because:
  - it asks for understanding of the project
  - giving argumented advice asks for time and efforts
- Patient needs to take responsibility
- Risk of tokenism (tick the box if the patient is included)

Participation ladder step IV

- Step IV Testing and assessing

Characteristics:

- Patient has a (co)-decison making role in the research project
- Researcher takes decisions with patient's consent
- Patient isn't involced with the organisation of the research project

Points of attention:

- Possible less useful for a part of the patient group, because:
  - it asks for understanding of the project
  - giving argumented advice asks for time and efforts
  - Patients needs to have the ability to formulate clear and to the point
- Patient needs to take responsibility
- Greater likelihood that representativeness of patient input will be questioned
- Research or research programme should be designed for patient input as a (co-)decisive voice

Participation ladder step V

Step V (co)-producing

Characteristics:

- Patient is equal party or has control over the research and its results
- Patients determine (partly) what research is about and bear responsibility for the final result

Points of attention:

- Possible less useful for a part of the patient group, because:
  - patient must have a good understanding of research
  - patient must master research skills
  - patient must dare to take a responsible role in the research
- Not suitable for every type of research
- Researchers transfer part of the ownership to patients

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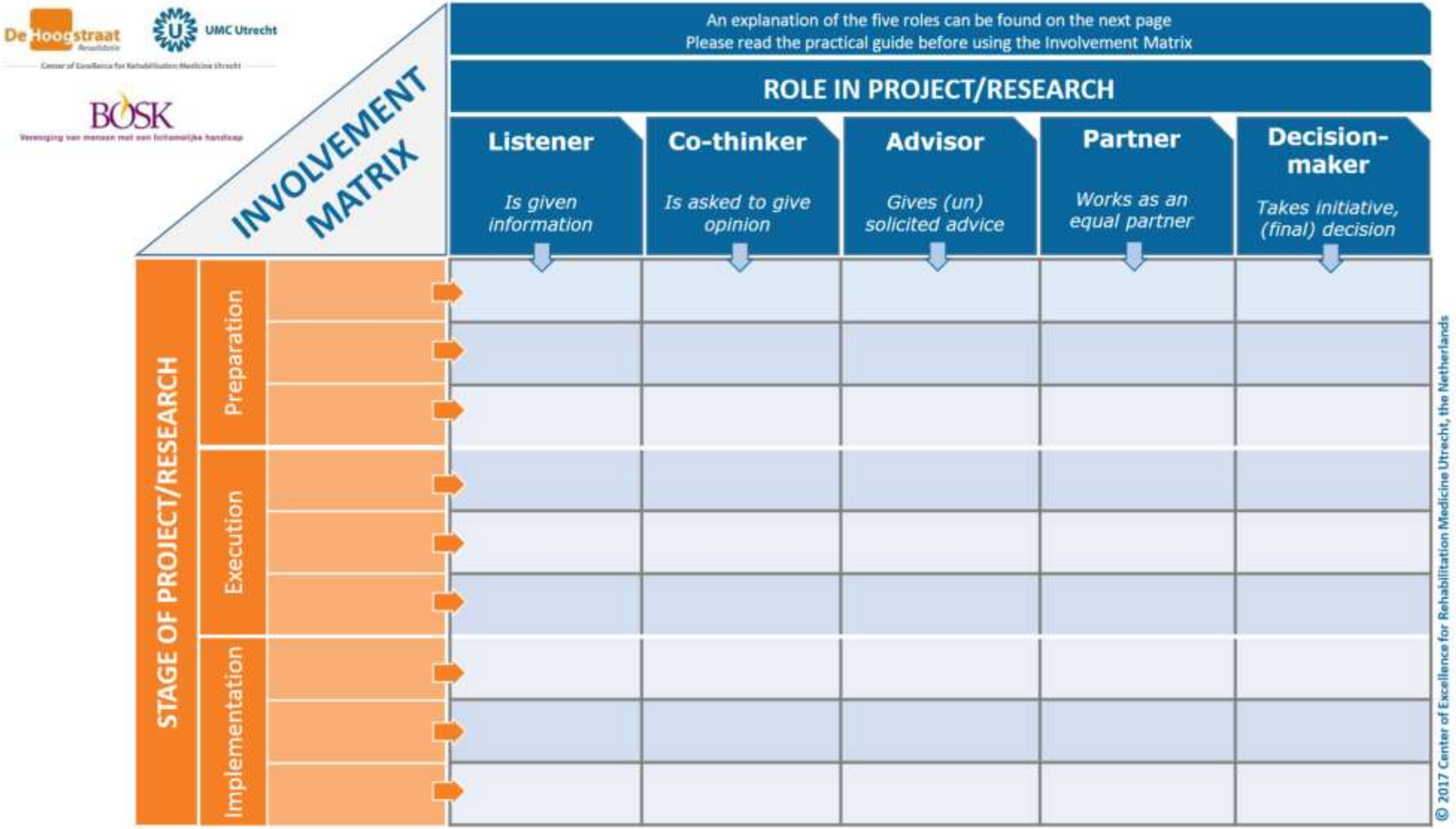

De Hoogstraat Revalidatie
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Vereniging van mensen met een lichamelijke handicap

INVOLVEMENT MATRIX

An explanation of the five roles can be found on the next page
Please read the practical guide before using the Involvement Matrix

			ROLE IN PROJECT/RESEARCH				
			Listener *Is given information*	**Co-thinker** *Is asked to give opinion*	**Advisor** *Gives (un) solicited advice*	**Partner** *Works as an equal partner*	**Decision-maker** *Takes initiative, (final) decision*
STAGE OF PROJECT/RESEARCH	Preparation						
	Execution						
	Implementation						

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https://www.kcrutrecht.nl/involvement-matrix/

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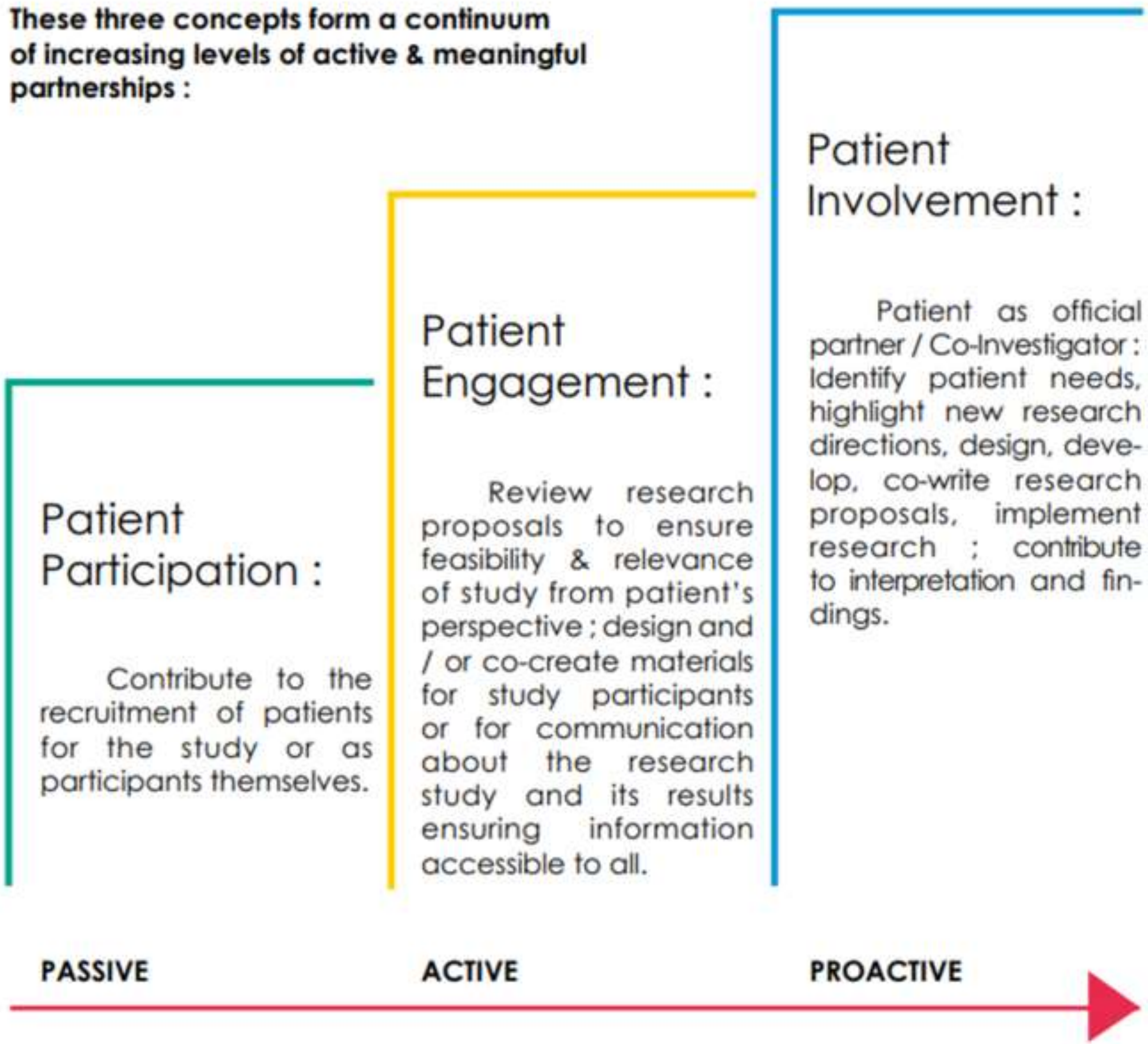
European Joint Program Rare Diseases (EJP-RD)

SHORT GUIDE
ON PATIENT PARTNERSHIPS
IN RARE DISEASE
RESEARCH PROJECTS

BASIC
PRE-CLINICAL
TRANSLATIONAL & SOCIAL

The role of patients in research



These three concepts form a continuum of increasing levels of active & meaningful partnerships :

Patient Participation :

Contribute to the recruitment of patients for the study or as participants themselves.

Patient Engagement :

Review research proposals to ensure feasibility & relevance of study from patient's perspective ; design and / or co-create materials for study participants or for communication about the research study and its results ensuring information accessible to all.

Patient Involvement :

Patient as official partner / Co-Investigator : Identify patient needs, highlight new research directions, design, develop, co-write research proposals, implement research ; contribute to interpretation and findings.

Review of projects on the patients' perspective

Part of the Scientific Evaluation Committee (SEC) since 2019 in EJP-RD

SEC consists of: scientists, bio-staticians and patient representatives

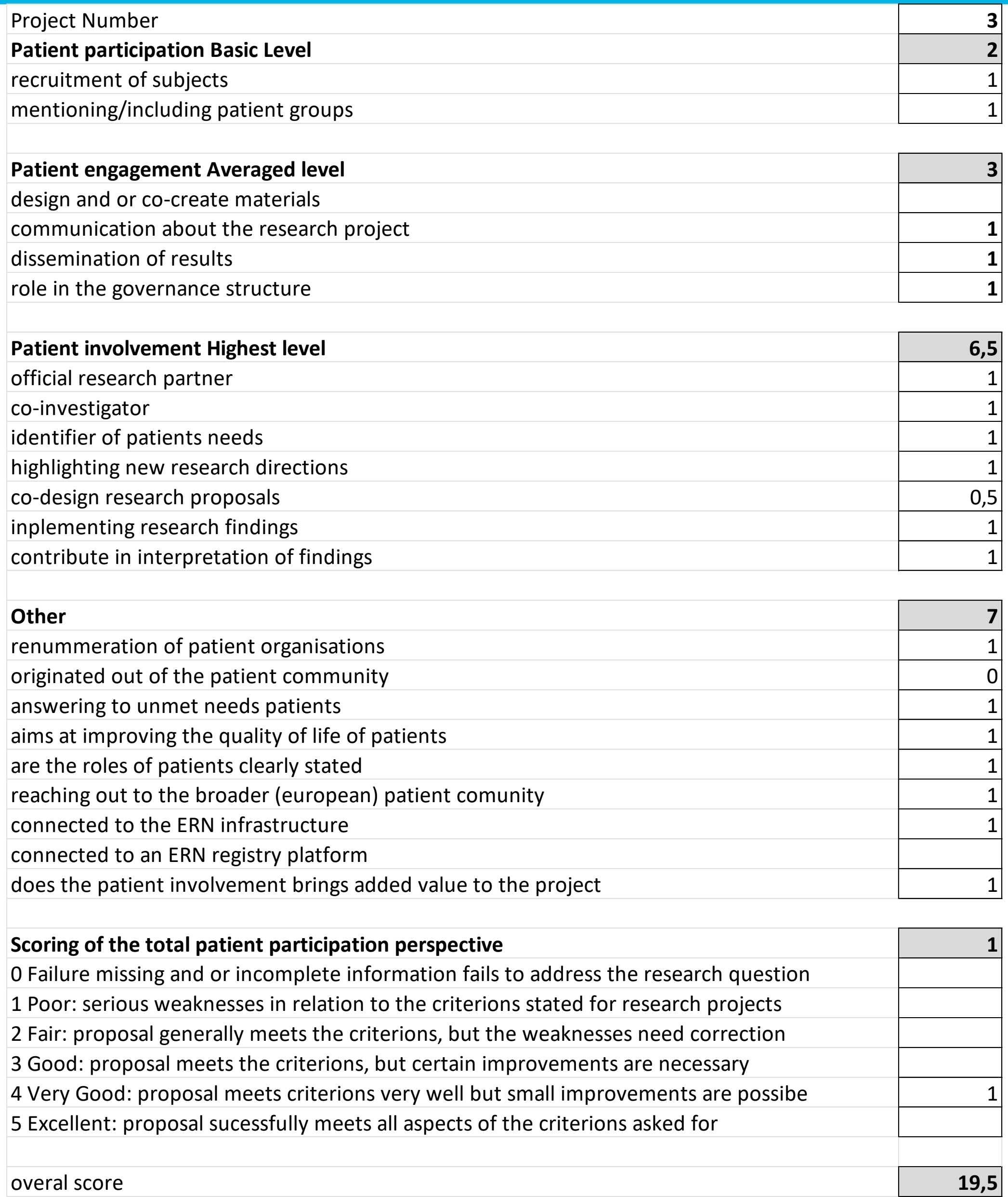
Patient involvement on 'both sides': patient organisations as part of the research proposal and patients assessing the proposals

Process needed to grow

Funding opportunities for patient involvement

How to evaluate?

- Defining categories important for patient involvement
- Scoring research proposals via a matrix
- Making research projects comparable
- Providing input for discussion



Project Number	3
Patient participation Basic Level	**2**
recruitment of subjects	1
mentioning/including patient groups	1
Patient engagement Averaged level	**3**
design and or co-create materials	
communication about the research project	**1**
dissemination of results	**1**
role in the governance structure	**1**
Patient involvement Highest level	**6,5**
official research partner	1
co-investigator	1
identifier of patients needs	1
highlighting new research directions	1
co-design research proposals	0,5
inplementing research findings	1
contribute in interpretation of findings	1
Other	**7**
renummeration of patient organisations	1
originated out of the patient community	0
answering to unmet needs patients	1
aims at improving the quality of life of patients	1
are the roles of patients clearly stated	1
reaching out to the broader (european) patient comunity	1
connected to the ERN infrastructure	1
connected to an ERN registry platform	
does the patient involvement brings added value to the project	1
Scoring of the total patient participation perspective	**1**
0 Failure missing and or incomplete information fails to address the research question	
1 Poor: serious weaknesses in relation to the criterions stated for research projects	
2 Fair: proposal generally meets the criterions, but the weaknesses need correction	
3 Good: proposal meets the criterions, but certain improvements are necessary	
4 Very Good: proposal meets criterions very well but small improvements are possibe	1
5 Excellent: proposal sucessfully meets all aspects of the criterions asked for	
overal score	**19,5**

Johan de Graaf – May 5th 2025

Three levels of patient organisations

Macro: large umbrella organisations e.g. national cancer alliances and Eurordis

Non specific conditions; paid staff; connected to important stakeholders (politics, health insurers); strong financial conditions

Meso: organisations for large common diseases: e.g. heart and lung conditions

Targeting a group of related conditions; some paid staff; specific expertises; better fund raising conditions

Micro: illness specific groups: pituitary disorders, turner syndrome, klinefelter, etc.

Targetting one specific condition; expertise based on experience; vulnerable; weak financial position; volunteers

Funding of the patient’s voice

- Too often sharing patient’s experience is considered a free service
- Especially in rare diseases resources are scarce, organisations are mostly volunteer driven
- Governmental funding of patient organisations is an exception
- Funding by pharmaceutical companies is considered inconvenient

Funding patient’s voice

- A recent example of a Dutch research funder
- Patient organisations are expected to work for free!
- Applied for a very modest amount for information materials and a webinar
- Weeks before this message I gave a presentation on patients in research to this specific funder!

Old proposal:

Name consortium partner	*Requested subsidy*	*Cash contribution*	*In kind contribution*
A **Hospital A**	*400.000*	*0*	*150.000*
B **University 1**	*150.000*	*0*	*60.000*
C **University 2**	*170.200*	*0*	*177.000*
D. **Hospital B**	*3.500*	*0*	*1.500*
E. **Hospital C**	*70.000*	*0*	*30.000*
F. **Dutch Pituitary Foundation**	*5.000*	*0*	*3.400*

New proposal:

Name consortium partner	*Requested subsidy*	*Cash contribution*	*In kind contribution*
A **Hospital A**	*400.000*	*0*	*150.000*
B **University 1**	*150.000*	*0*	*60.000*
C **University 2**	*170.000*	*0*	*170.000*
D. **Hospital B**	*3.500*	*0*	*1.500*
E. **Hospital C**	*70.000*	*0*	*30.000*
F. **Dutch Pituitary Foundation**	*0*	*0*	*3.400*

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Focussing on level 4 and 5; how to get there?

Organisations and patient representatives grow by gaining experience

- **Education** of patient representatives
- Building a network; look for mutual challenges amongst colleague patients organisations
- Become a member of the National Alliance for Rare Diseases
- Become a member of **Eurordis**
- Connect to the suitable **ERN** (European Reference Network)
- Have a look on the website of the the ERDERA Project

Questions

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