



PROMs – better treatment with patient surveys

Rare Disease Symposium, May 3rd 2024

Valerie Kirchberger, HRTBT Medical Solutions GmbH



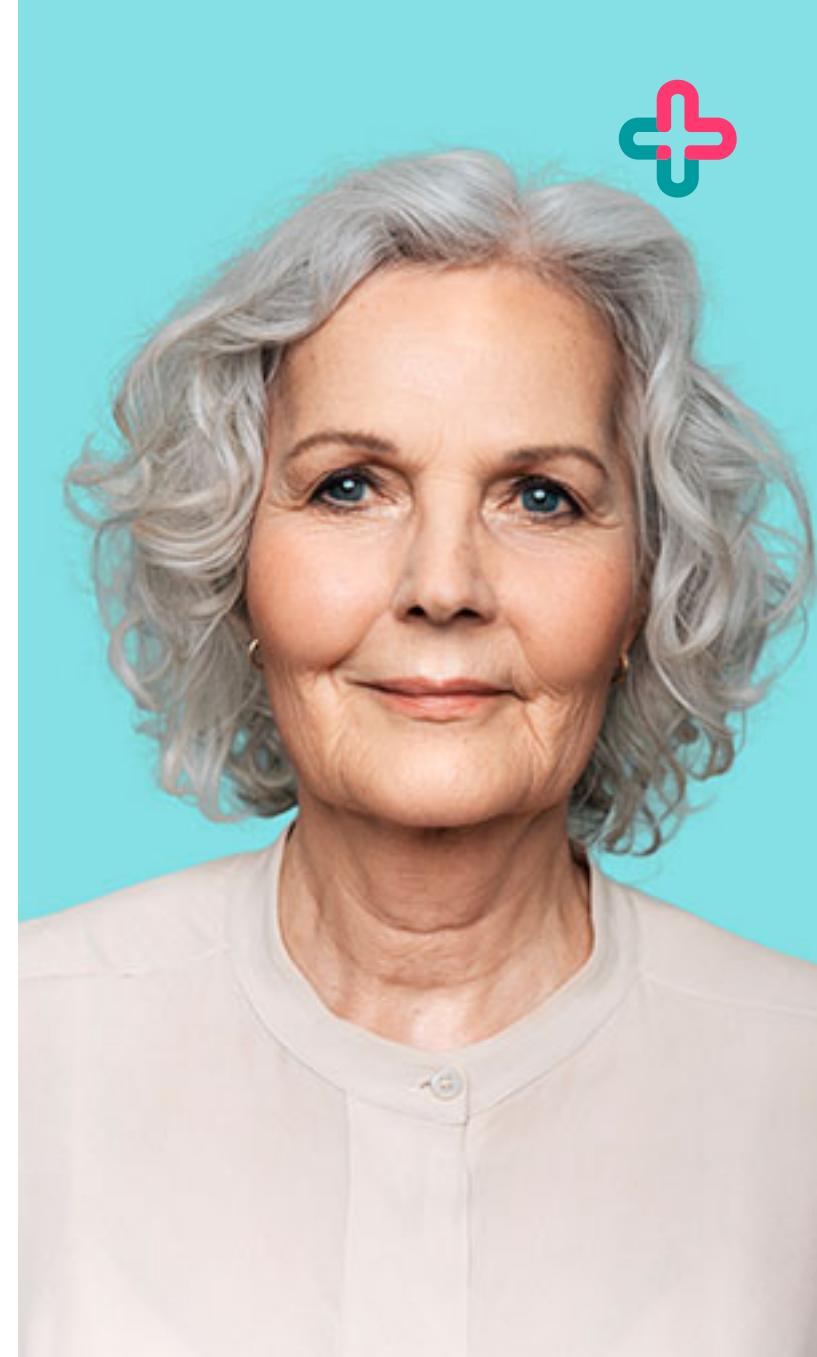
What are PROMs.

How does the individual benefit.

How does the healthcare system benefit.

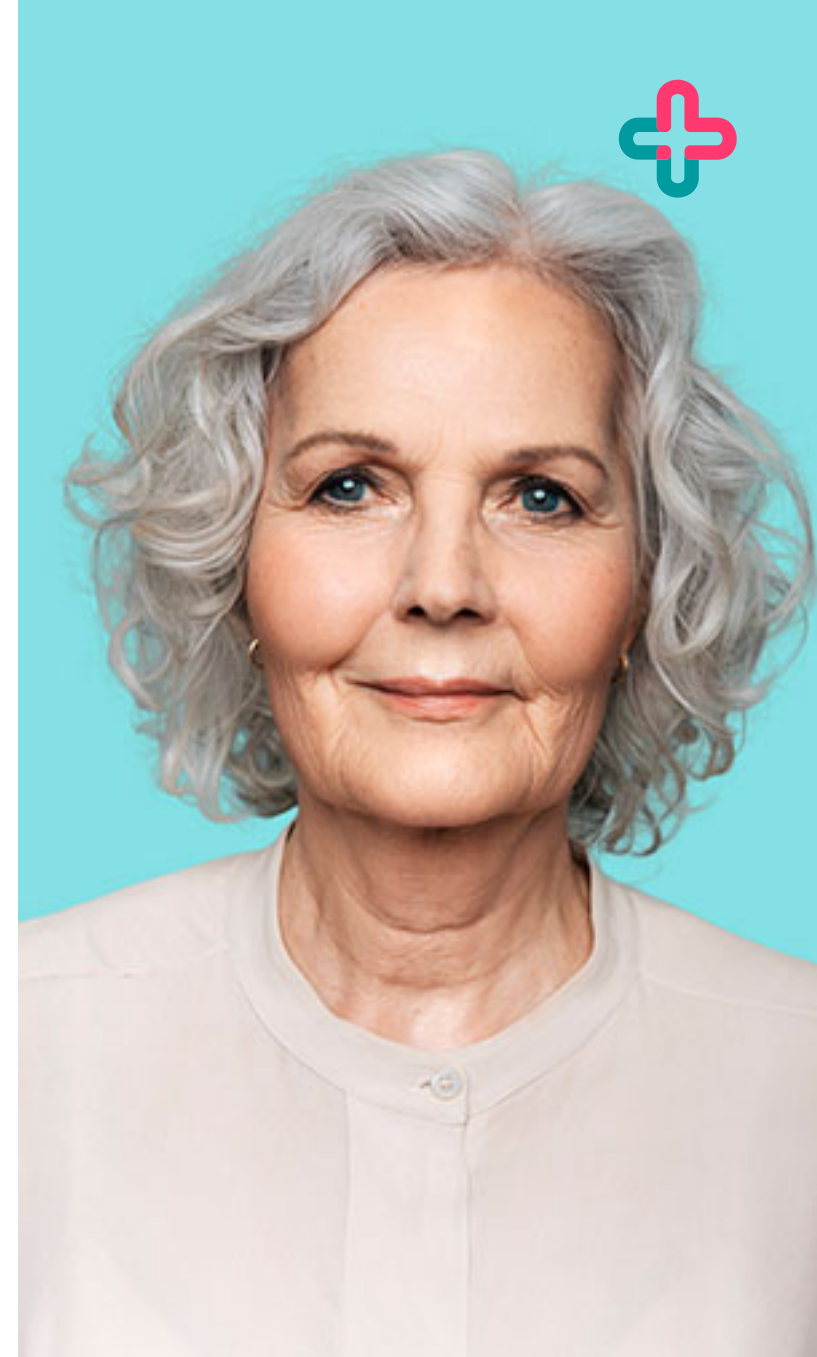
Implications for rare diseases.

PROMs= Patient-Reported Outcome Measures.

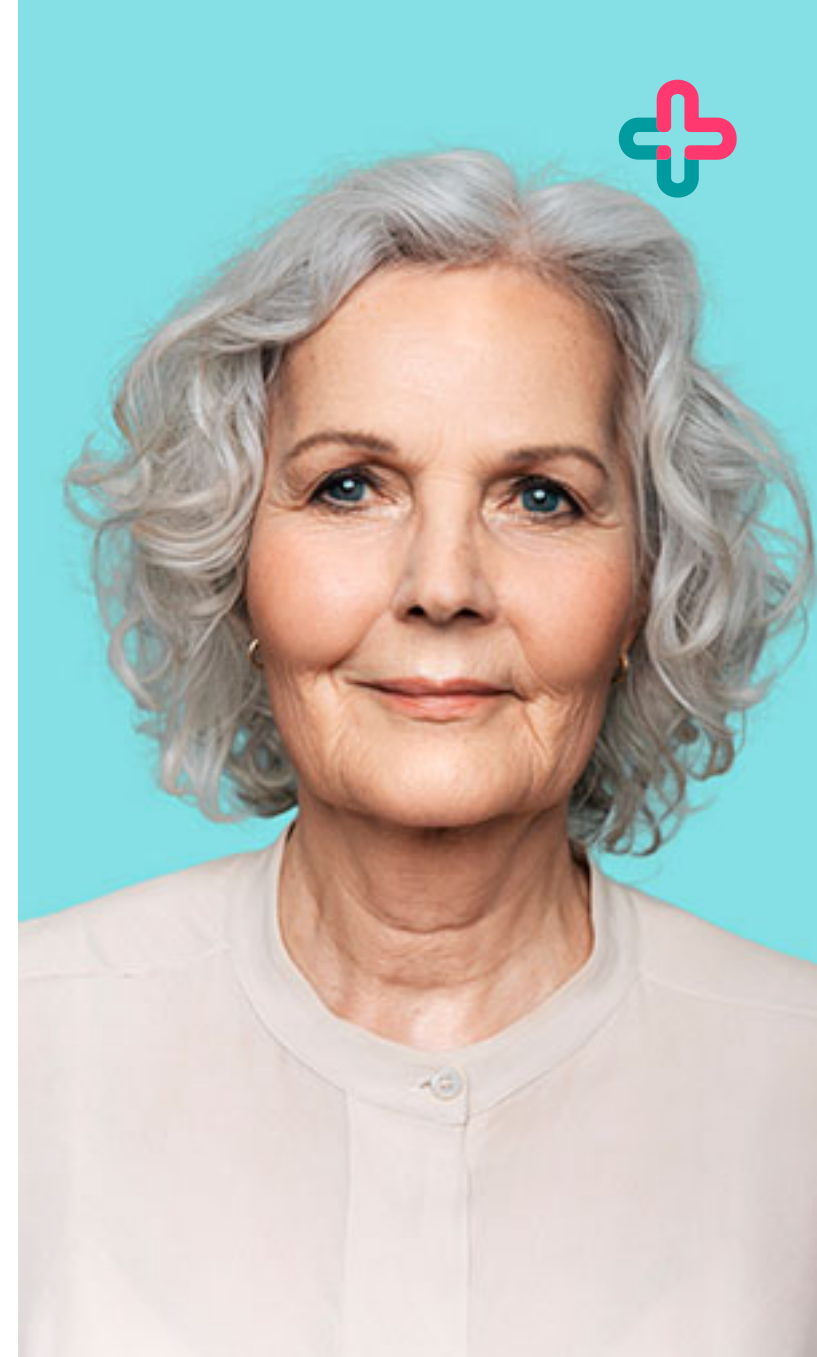


PROMs= Patient-Reported Outcome Measures.

They are questionnaires that collect health outcomes directly from the people who experience them.

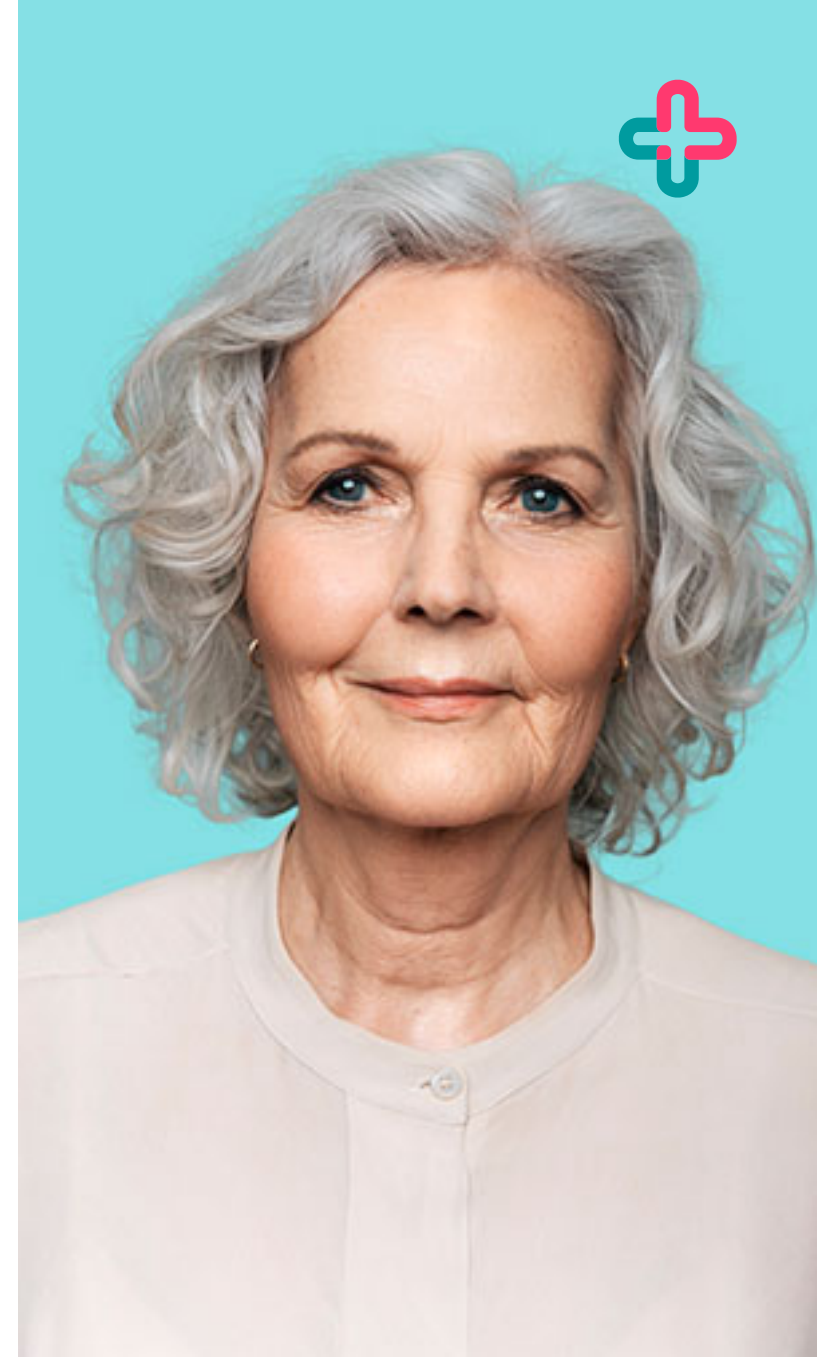


PROMs are part of the Value-based healthcare framework.



PROMs are part of the Value-based healthcare framework.

Value-based healthcare aims at restructuring the system towards an outcome-based and patient-centric one.





Why do we need PROMs ?

They add valuable information on the patient perspective.



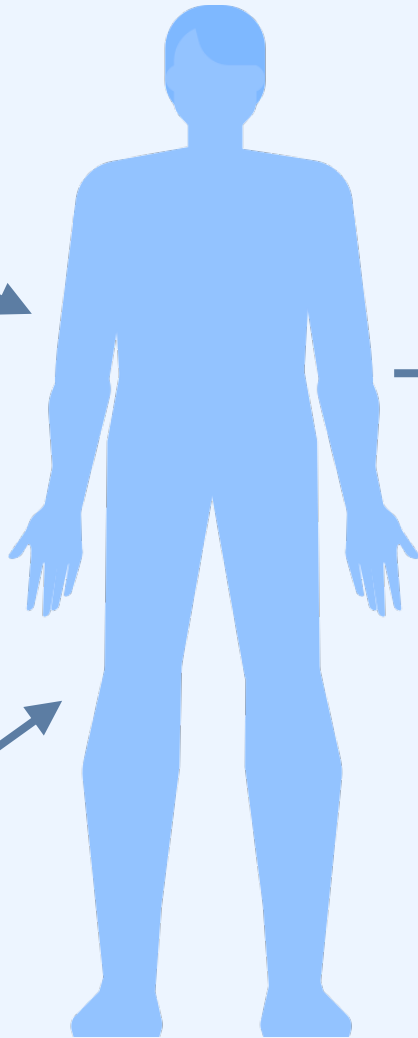
Human ecosystem

Challenges



Disposition

Biological
Psychological
Social



Functionality

Behaviour
Psychological
Neurological
Immunological
Hormonal
Cellular

Disease

Mortality
Morbidity
Quality of life





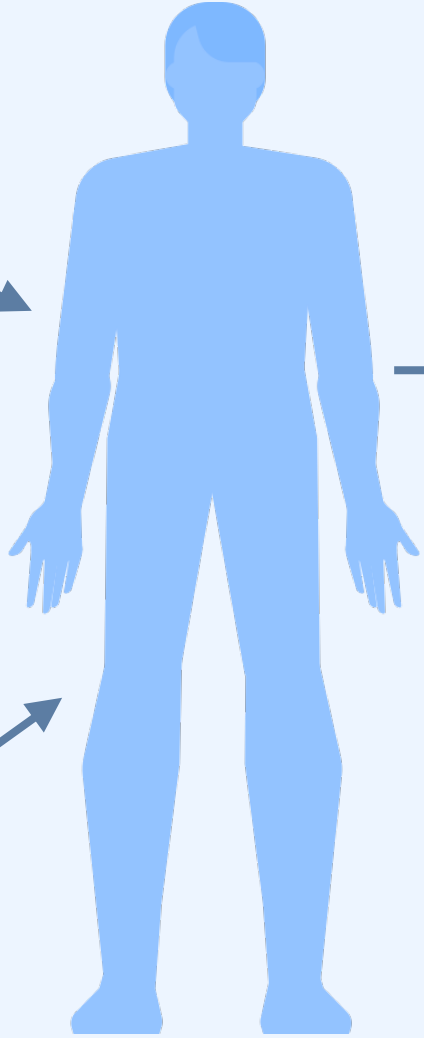
Human ecosystem

Challenges



Disposition

Biological
Psychological
Social



Functionality

Behaviour
Psychological
Neurological
Immunological
Hormonal
Cellular

Disease

Mortality
Morbidity
Quality of life

PROMs
show
Quality of
life

Benefits of using PROMs

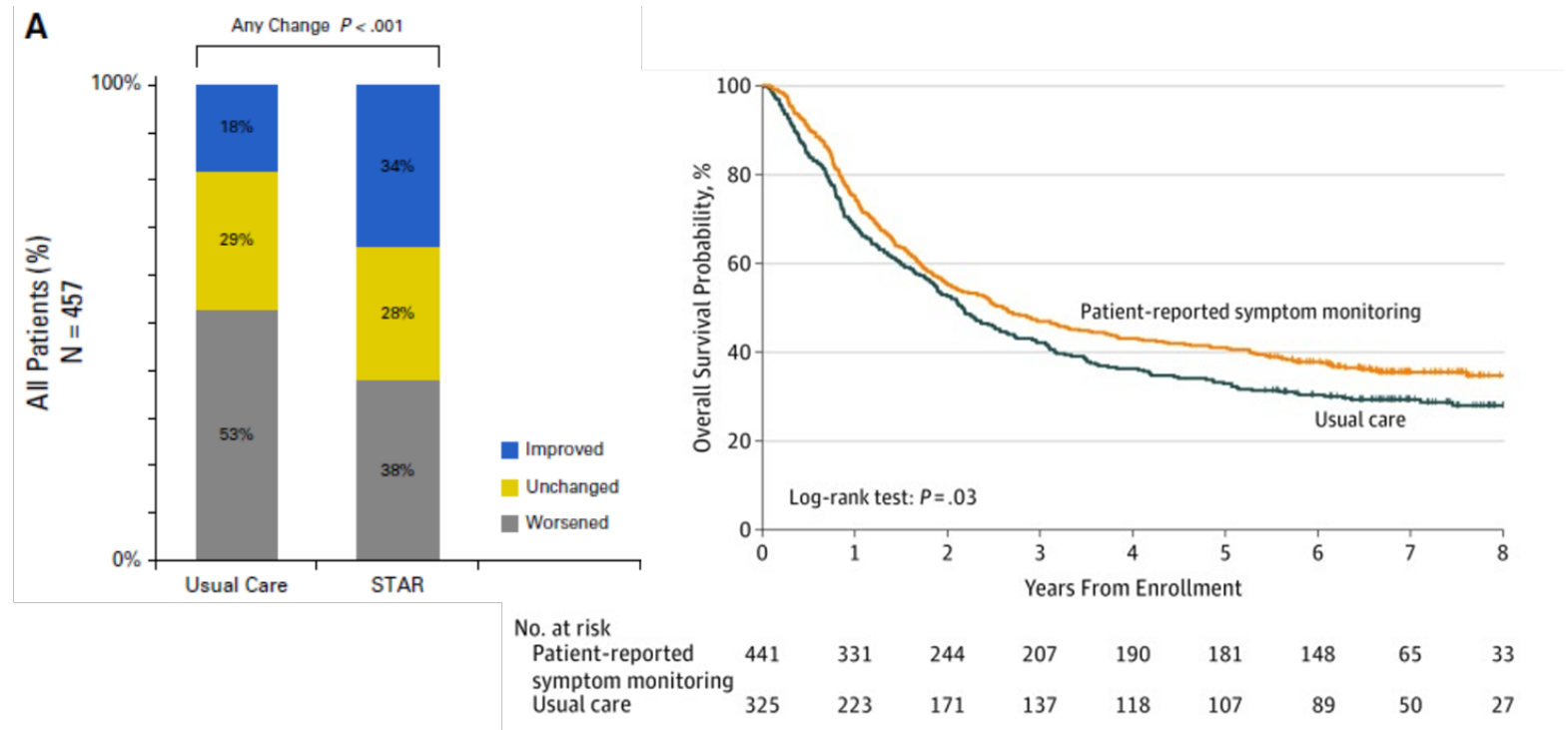
In oncology there is good evidence that PRO monitoring helps patients.





In oncology there is good evidence that PRO monitoring helps patients

Symptom control through PRO Measurement leads to **improvement in quality of life and overall survival**

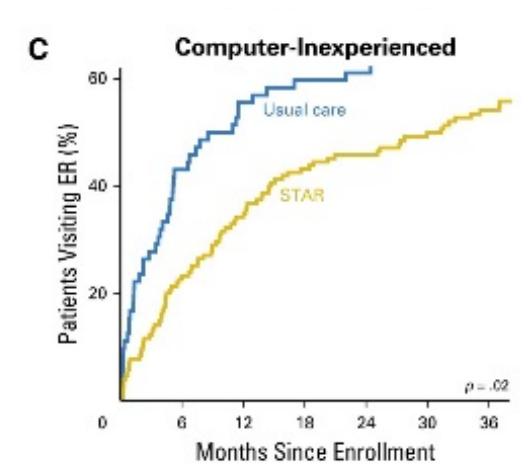
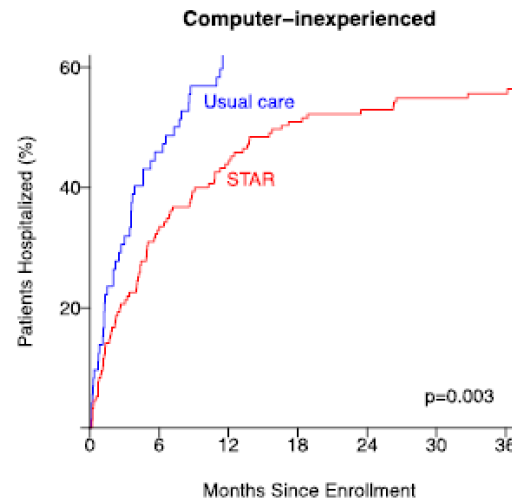


Basch, E et al J Clin Oncol. 2016 Jun 20; 34(18):2198



In oncology there is good evidence that PRO monitoring helps patients

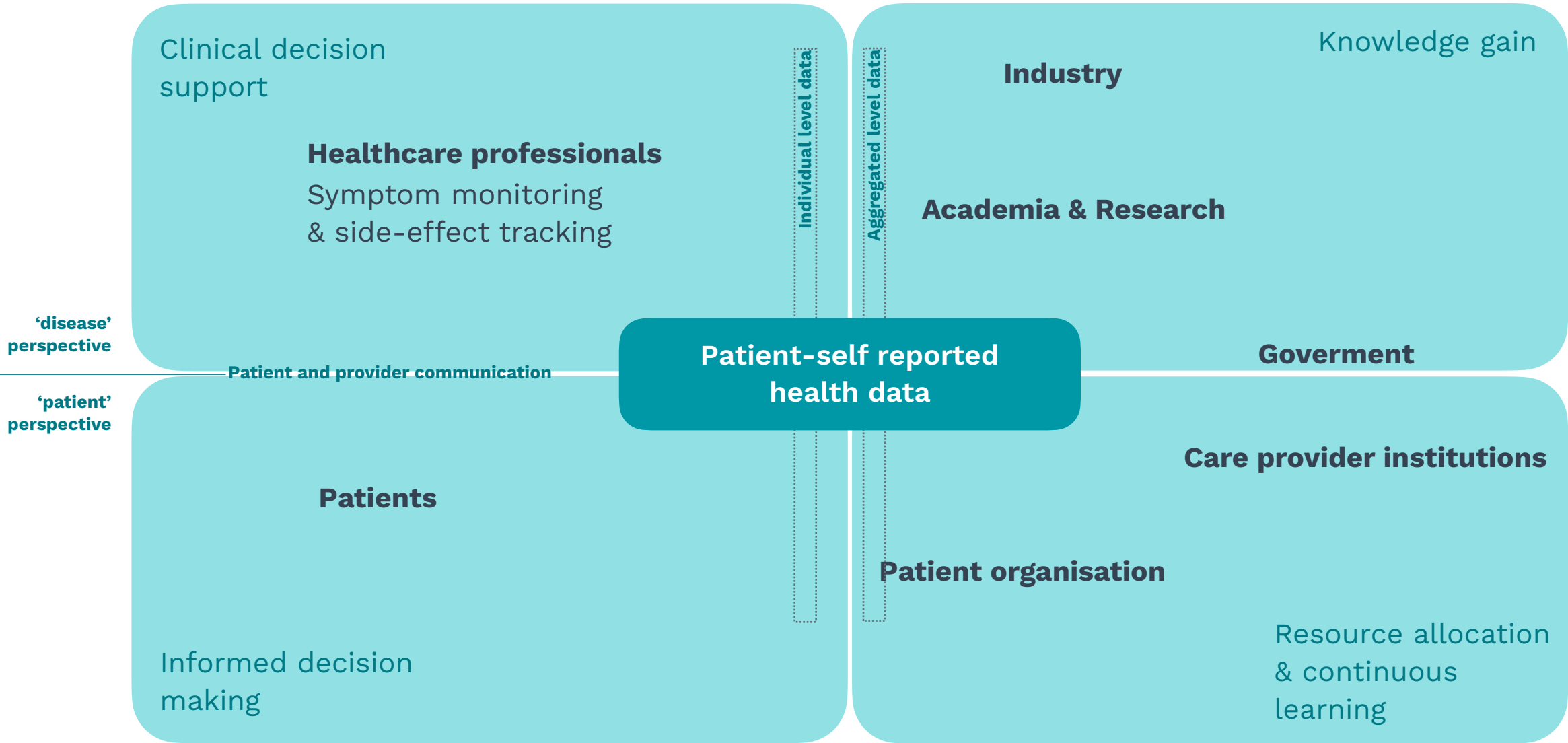
Monitoring with PRO led to a significant reduction in **unplanned emergency room visits** and hospital admissions, even in the computer inexperienced group

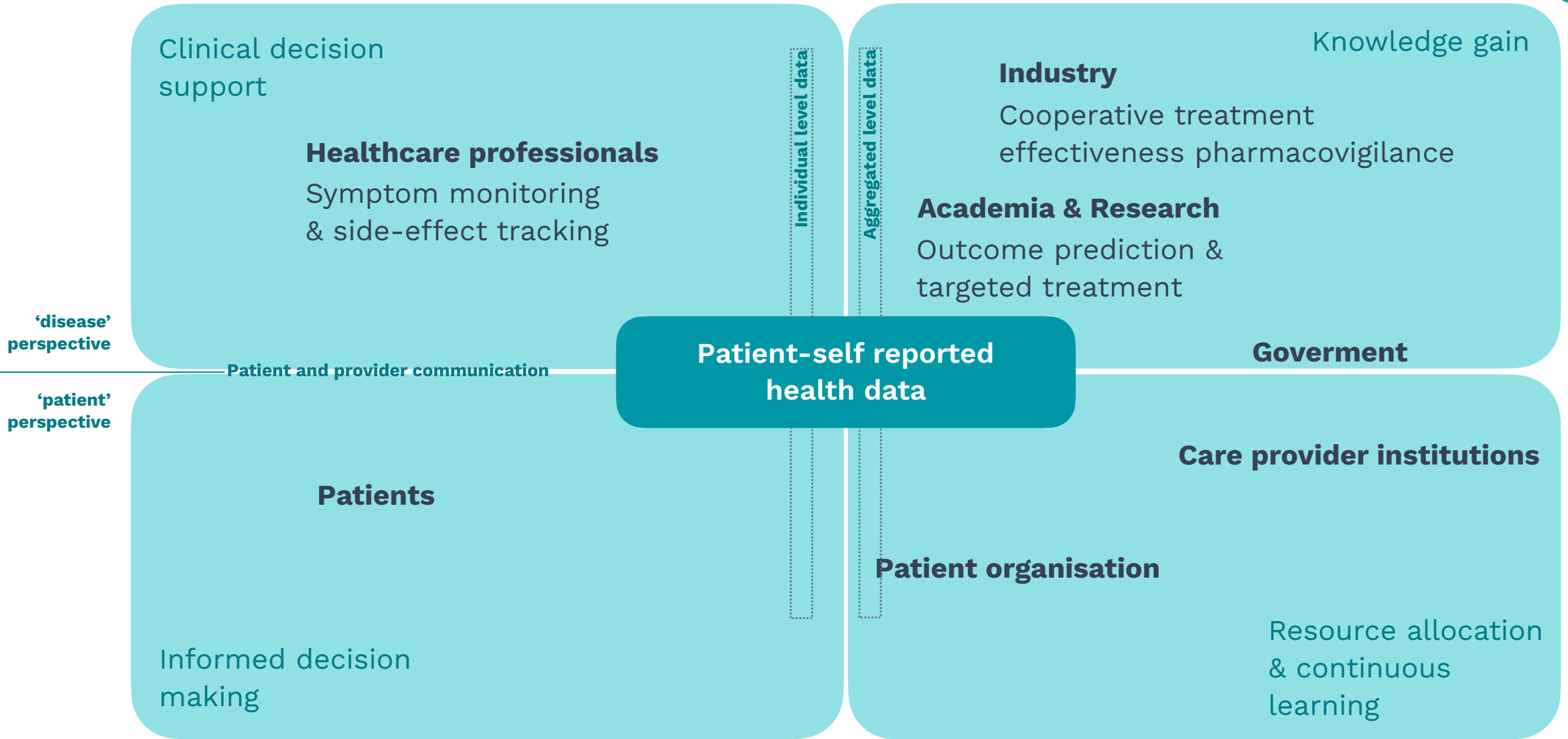


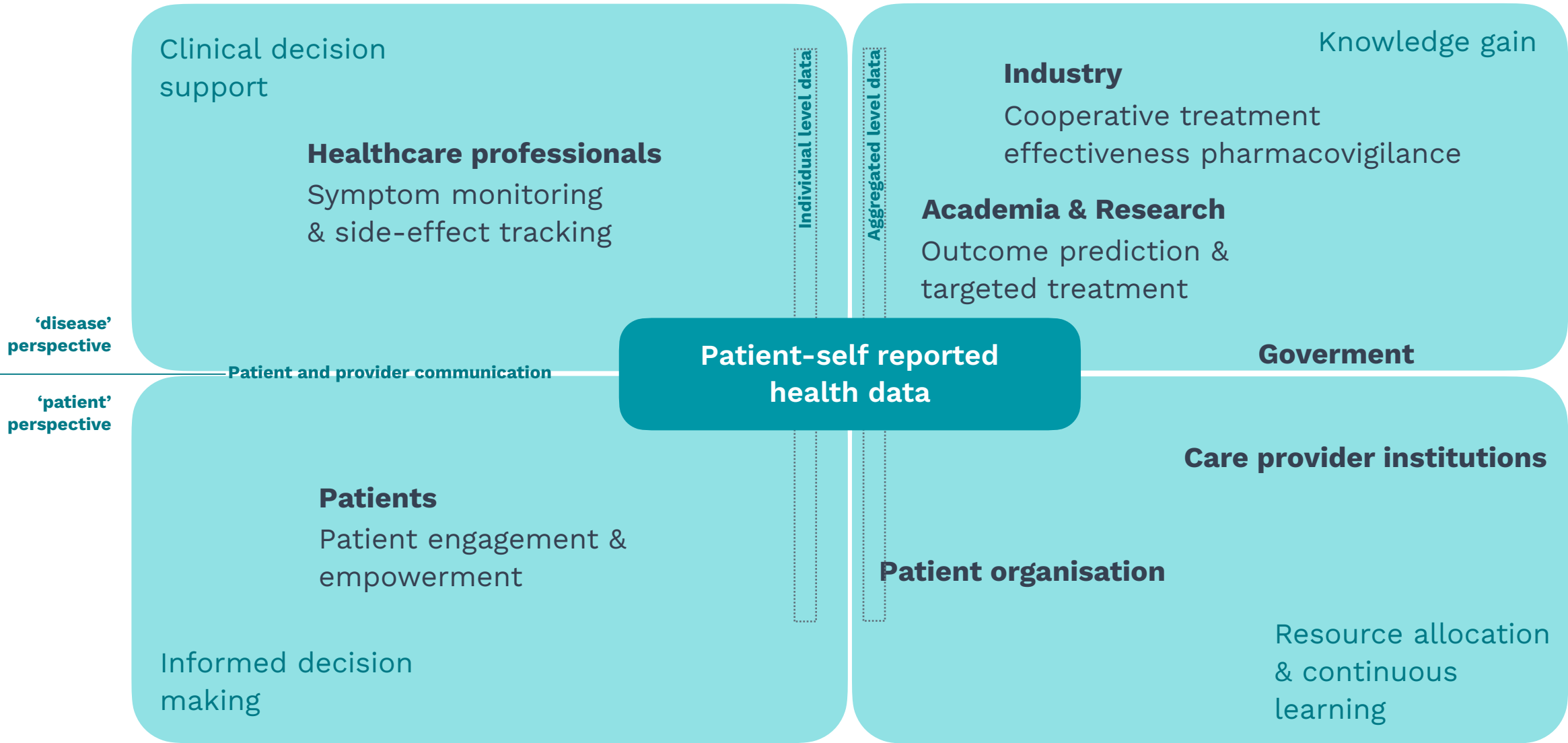
Basch, E et al J Clin Oncol. 2016 Jun 20; 34(18):2198

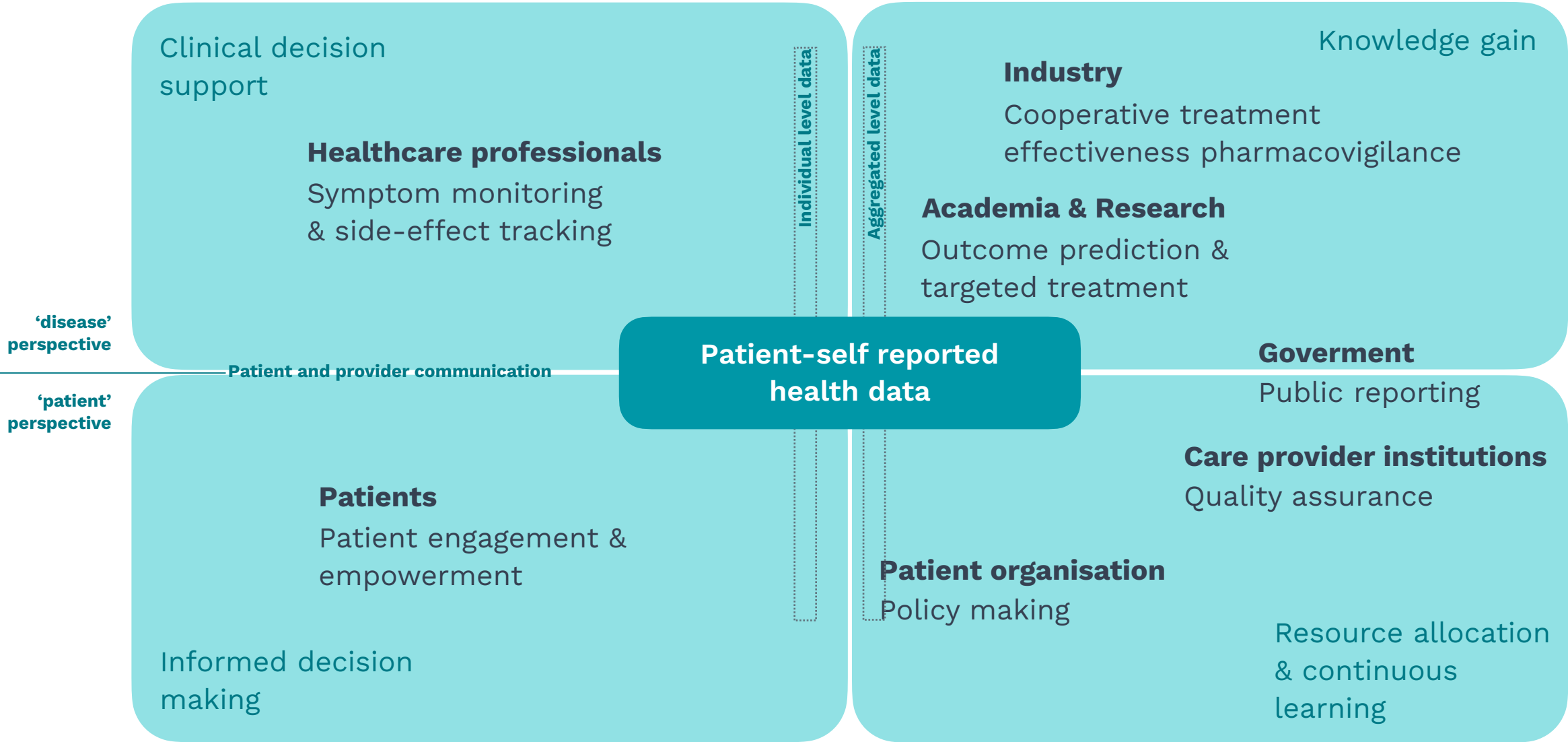


If PROMs are collected in a standardised way, there are multiple stakeholders who benefit.











Practical implications and challenges in rare diseases.



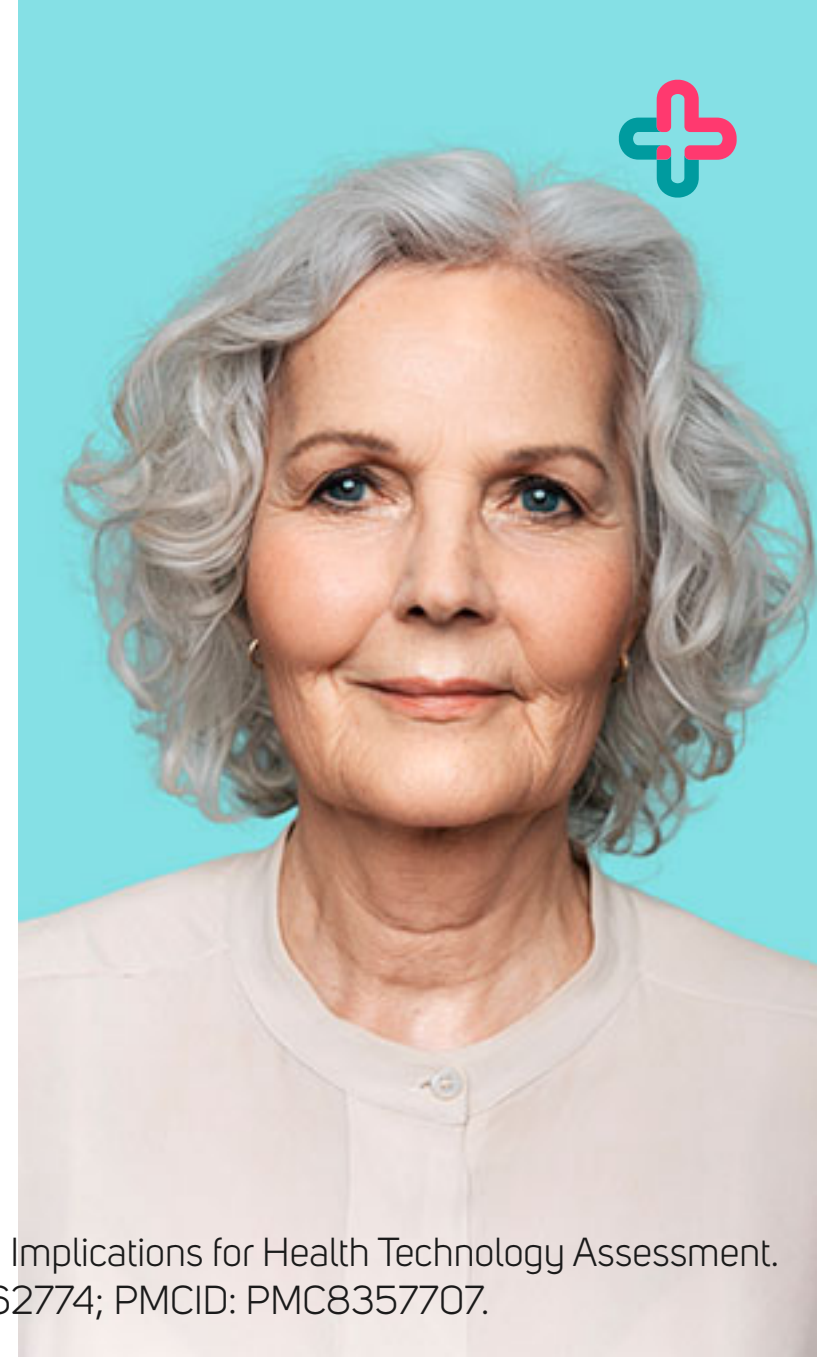
**Practical implications and
challenges in rare diseases.**

**Disease heterogeneity and smaller
patient population.**



Types of questionnaires

- **Generic, overall health**
- **Condition-specific if applicable**



Whittal A, Meregaglia M, Nicod E. The Use of Patient-Reported Outcome Measures in Rare Diseases and Implications for Health Technology Assessment. Patient. 2021 Sep;14(5):485-503. doi: 10.1007/s40271-020-00493-w. Epub 2021 Jan 19. PMID: 33462774; PMCID: PMC8357707.

Example PROMIS 29 questionnaire

Based on the domains of health that are relevant to capture life.

- Self reported (or parent reported) measures on **physical function, anxiety, depression, fatigue, sleep disturbance, social participation and pain** (interference & intensity)



Please respond to each question or statement by marking one box per row.

		Without any difficulty	With a little difficulty	With some difficulty	With much difficulty	Unable to do
Physical Function						
PFA11	Are you able to do chores such as vacuuming or yard work?.....	<input type="checkbox"/> 5	<input type="checkbox"/> 4	<input type="checkbox"/> 3	<input type="checkbox"/> 2	<input type="checkbox"/> 1
PFA21	Are you able to go up and down stairs at a normal pace?.....	<input type="checkbox"/> 5	<input type="checkbox"/> 4	<input type="checkbox"/> 3	<input type="checkbox"/> 2	<input type="checkbox"/> 1
PFA23	Are you able to go for a walk of at least 15 minutes?.....	<input type="checkbox"/> 5	<input type="checkbox"/> 4	<input type="checkbox"/> 3	<input type="checkbox"/> 2	<input type="checkbox"/> 1
PFA53	Are you able to run errands and shop?	<input type="checkbox"/> 5	<input type="checkbox"/> 4	<input type="checkbox"/> 3	<input type="checkbox"/> 2	<input type="checkbox"/> 1
Anxiety						
In the past 7 days...		Never	Rarely	Sometimes	Often	Always
EDANX01	I felt fearful.....	<input type="checkbox"/> 1	<input type="checkbox"/> 2	<input type="checkbox"/> 3	<input type="checkbox"/> 4	<input type="checkbox"/> 5
EDANX40	I found it hard to focus on anything other than my anxiety	<input type="checkbox"/> 1	<input type="checkbox"/> 2	<input type="checkbox"/> 3	<input type="checkbox"/> 4	<input type="checkbox"/> 5
EDANX41	My worries overwhelmed me	<input type="checkbox"/> 1	<input type="checkbox"/> 2	<input type="checkbox"/> 3	<input type="checkbox"/> 4	<input type="checkbox"/> 5
EDANX53	I felt uneasy	<input type="checkbox"/> 1	<input type="checkbox"/> 2	<input type="checkbox"/> 3	<input type="checkbox"/> 4	<input type="checkbox"/> 5
Depression						
In the past 7 days...		Never	Rarely	Sometimes	Often	Always
EDDEP04	I felt worthless	<input type="checkbox"/> 1	<input type="checkbox"/> 2	<input type="checkbox"/> 3	<input type="checkbox"/> 4	<input type="checkbox"/> 5
EDDEP06	I felt helpless.....	<input type="checkbox"/> 1	<input type="checkbox"/> 2	<input type="checkbox"/> 3	<input type="checkbox"/> 4	<input type="checkbox"/> 5
EDDEP29	I felt depressed.....	<input type="checkbox"/> 1	<input type="checkbox"/> 2	<input type="checkbox"/> 3	<input type="checkbox"/> 4	<input type="checkbox"/> 5
EDDEP41	I felt hopeless.....	<input type="checkbox"/> 1	<input type="checkbox"/> 2	<input type="checkbox"/> 3	<input type="checkbox"/> 4	<input type="checkbox"/> 5

PRACTICAL IMPLICATIONS

Kidsscreen

Based on the domains of health that are relevant to capture life.

- Self reported (or parent reported) measures on **physical well-being, psychological wellbeing, autonomy and parent relations, peers and social support, school environment**

Thinking about the last week...		never	seldom	quite often	very often	always
4.	Has your child felt sad?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
5.	Has your child felt so bad that he/she didn't want to do anything?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
6.	Has your child felt lonely?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
7.	Has your child been happy with the way he/she is?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

3. Family and Your Child's Free Time

Thinking about the last week...		never	seldom	quite often	very often	always
1.	Has your child had enough time for him/herself?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2.	Has your child been able to do the things that he/she wants to do in his/her free time?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
3.	Has your child felt that his/her parent(s) had enough time for him/her?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
4.	Has your child felt that his/her parent(s) treated him/her fairly?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
5.	Has your child been able to talk to his/her parent(s) when he/she wanted to?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

Condition- specific PROMs

Developed for some conditions

- **Cystic Fibrosis**
- Phenylketonuria
- Immune thrombocytopenia purpura
- Fabry Disease

Section II. Quality of Life

Please tick the box indicating your answer.

<i>During the past two weeks, to what extent have you had difficulty:</i>	A lot of difficulty	Some difficulty	A little difficulty	No difficulty
1. Performing vigorous activities such as running or playing sports.....	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
2. Walking as fast as others	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
3. Carrying or lifting heavy things such as books, shopping, or school bags	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
4. Climbing one flight of stairs	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
5. Climbing stairs as fast as others.....	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

<i>During the past two weeks, indicate how often:</i>	Always	Often	Sometimes	Never
6. You felt well	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
7. You felt worried.....	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
8. You felt useless	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
9. You felt tired.....	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
10. You felt full of energy.....	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
11. You felt exhausted	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
12. You felt sad	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

Please circle the number indicating your answer. Please choose only one answer for each question.

Thinking about the state of your health over the last two weeks:

13. To what extent do you have difficulty walking?
 1. You can walk a long time without getting tired
 2. You can walk a long time but you get tired
 3. You cannot walk a long time because you get tired quickly
 4. You avoid walking whenever possible because it's too tiring for you
14. How do you feel about eating?
 1. Just thinking about food makes you feel sick
 2. You never enjoy eating
 3. You are sometimes able to enjoy eating
 4. You are always able to enjoy eating
15. To what extent do your treatments make your daily life more difficult?
 1. Not at all
 2. A little
 3. Moderately
 4. A lot

Visualisation



- Actionable for patients and other stakeholders
- Captures the relevant dimensions of health

9:41

.PROally

BASELINE

Your results are ready!
Based on your answers:

It appears you can enhance your health further with the assistance of our insurance offers.

physical	mental
Green	Grey
Green	Orange
Green	Orange

← Back

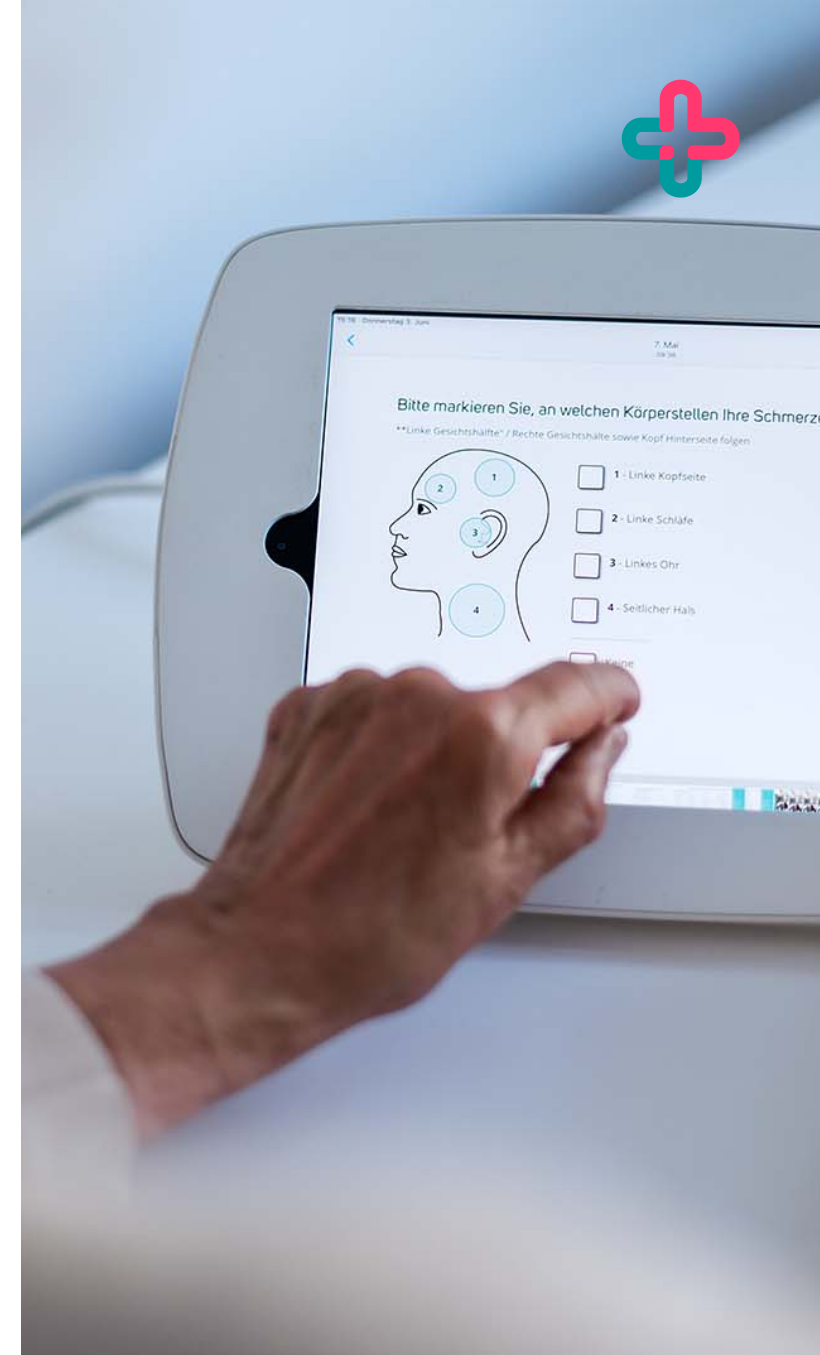
Physical Function

Measures the ability to perform everyday physical activities, such as walking, going up stairs, or doing housework.

Regular movement, a balanced diet as well as the CSS recommendations can help you improve your physical activity.

How does this work?

Recurring questionnaires,
standardised content, available
to stakeholders.





**Key message - keep it simple.
But do it.
PROMs are vital.**



Value-based Reimbursement
Patient Reported Outcomes
Real World Evidence

HRTBT Medical Solutions GmbH
Berlin - Germany

www.heartbeat-med.com