



# Overview of Traditional Quality of Life Assessment Methodologies

**Jakob Bue Bjørner,  
Chief Science Officer,  
Optum Patient Insights**

# What is Quality of Life Assessment?

Patient and caregiver perspectives are collected through a number of techniques



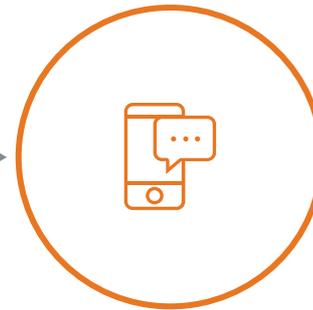
## Open Interviews

- In person
- By phone
- Focus groups
- Web discussion groups



## Structured Surveys

- Paper and pencil
- Computer, tablet, or smartphone
- Phone interviews
- Personal interviews
- Ratings by caregivers or clinicians



## Momentary Assessment

- Smartphone: "What is your level of pain right now?"



## Registers

- Uniform, clinically rich data

A combination of approaches often provides the best results

# Open interviews or structured surveys?

Both methods provide incredibly useful data in different ways.

## Open Interviews (qualitative approach)

- Provide richer information
- More flexible



## Structured Surveys (quantitative approach)

- Minimize the influence of the particular interviewer
- Often summarized as numbers
- Allows more condensed communication of results



What method best encompasses the objectives of your work?

# Standard surveys for health-related quality of life

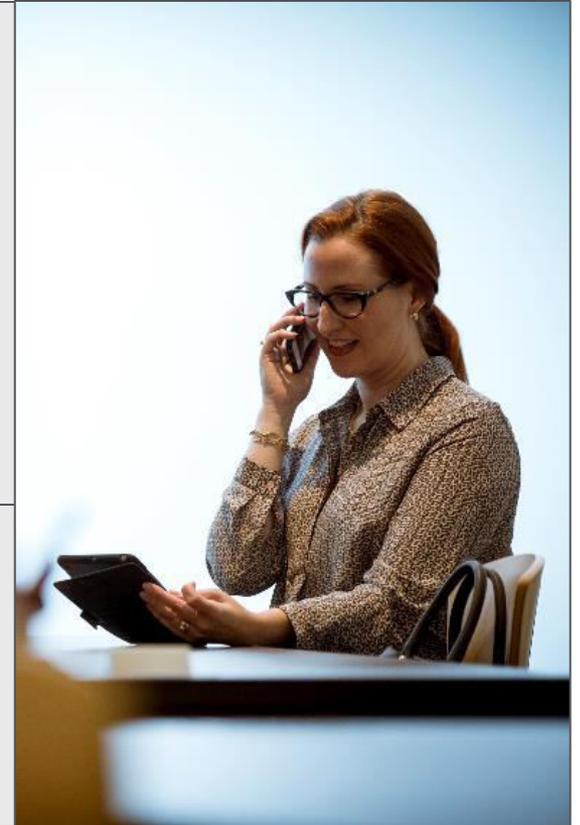
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## Generic

- Applicable across all diseases and to the general population
- Often tested and revised many times to weed out problems
- Often translated to many languages, facilitating multinational studies

## Specific

- Developed to cover the problems that are most relevant for this disease group



# Typical content of a generic survey

Most quality of life surveys address a minimal set of subject areas



## (1) Symptoms and Wellbeing

Pain, fatigue, anxiety, depression, wellbeing



## (2) Function

Physical function, role function, social function

Examples: Walk, climb stairs, open a bottle, work ability, take care of household tasks, be with friends or family.



## (3) Evaluation

Health perception, treatment satisfaction

Example: “All in all, would you say your health is...?”

# Challenges of Implementing QOL Measures in Rare Disease Clinical Trials

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## 1. Understanding the disease or condition

What is known about the condition?

How is it treated?

How does condition impact patient and caregivers?

## 2. Conceptualizing treatment benefit

What constitutes a meaningful treatment benefit?

How will the clinical study be designed, i.e., the context of use?

Which types of tools are needed?

## 3. Selecting / Developing Outcome Measure

Can any existing measure do the job?

How to develop or adapt a measure for this context?

# Steps for Using a Pre-existing Questionnaire

## 1. Qualitative testing

- Are the questions relevant?
- Are important topics left out?
- How do patients understand/interpret the questions?

## 3. Design study to get high response rates

- Avoid lengthy questionnaires
- Engage participants
- Have follow-up procedures
- Provide feedback

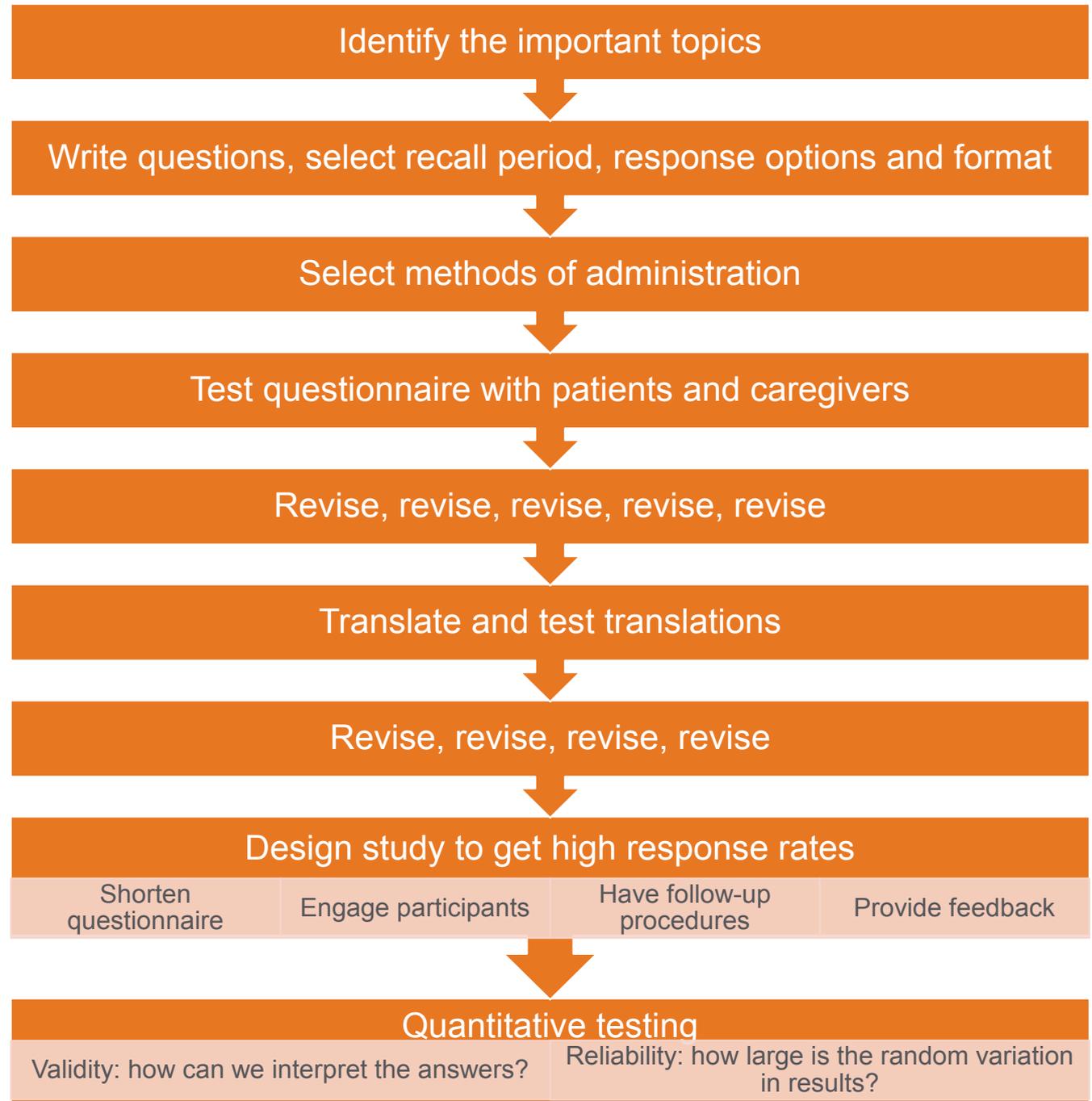
## 2. Get Permission

## 4. Quantitative testing

Validity: how can we interpret the answers?

Reliability: how large is the random variation in results?

# Steps for Developing a New Questionnaire



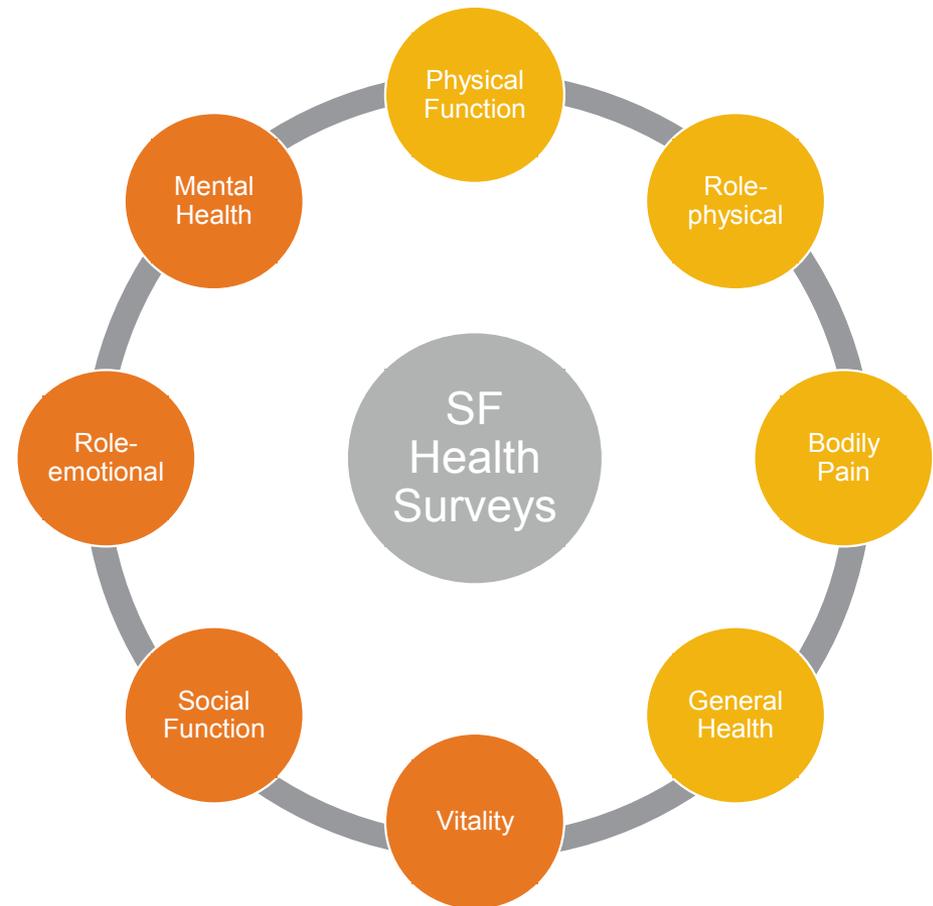
# Popular generic surveys

	SF-36v2 SF-12v2 SF-6D	EQ-5D	KIDSCREEN <sup>1</sup>	PROMIS
Target	Adults	Adults	Kids	Adults/Kids
# of items	36/12	6	52/27/10	>300 57/43/29/10
Scales	8	(5)	10/5/1	>30
Summaries	2/1	1	0	0
Translations	>180	>170	44-54	2-24
License/Costs	(Yes)	(Yes)	Commercial studies only	For translations

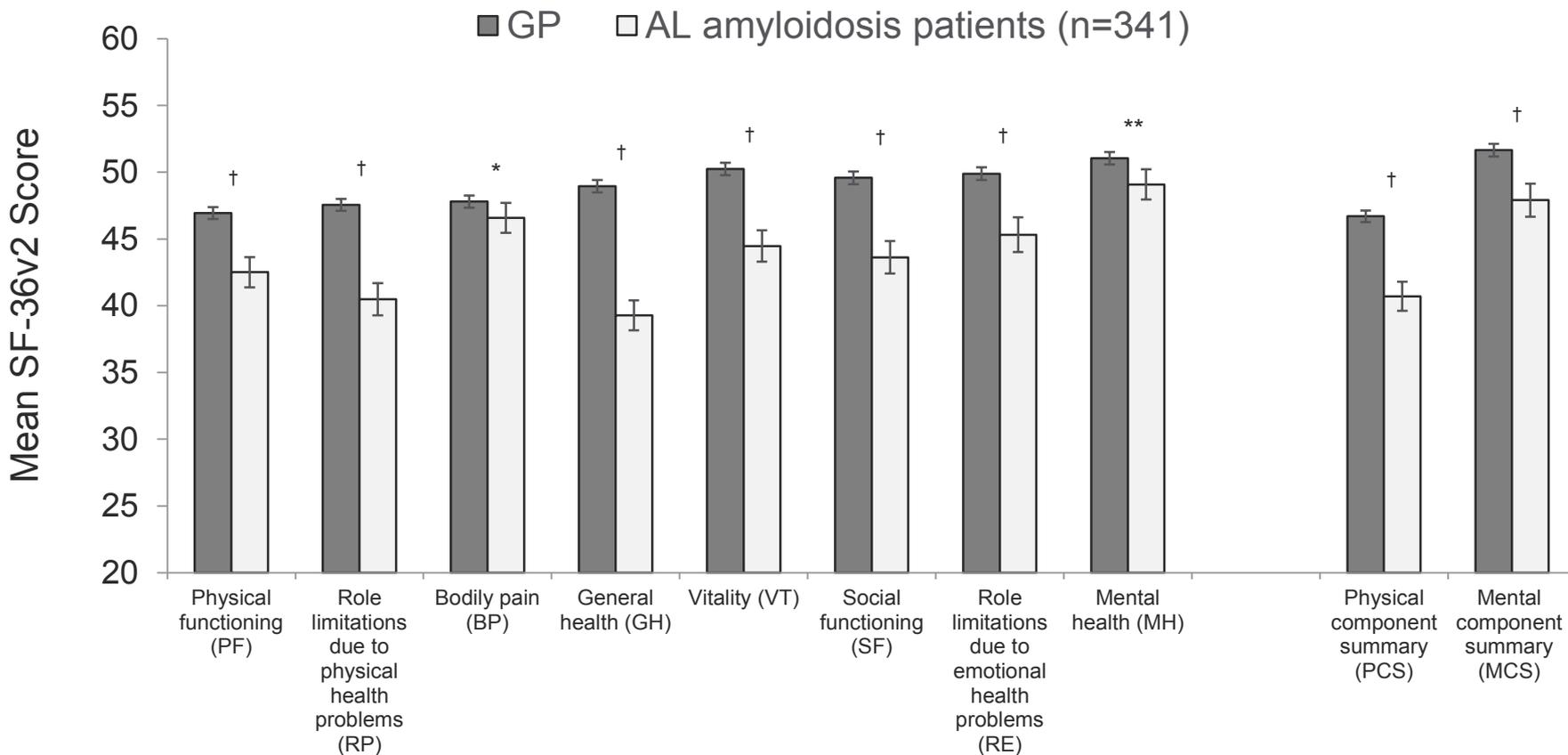
<sup>1</sup> Additional versions available for parents/primary caregivers

# How Has QOL Been Assessed Using the SF-36v2®?

- The SF-36v2® Health Survey (SF-36v2) is a widely used patient-reported outcome (PRO) measure across many healthy and disease populations.
- SF-36v2 measures QOL using eight health domains and two summary scores.
- For all scales, a score of 50 is the US population average; higher scores mean better health.
- Standardized so that outcomes are comparable across groups, times, and populations.
- Used during all phases of drug/biologic/device development.



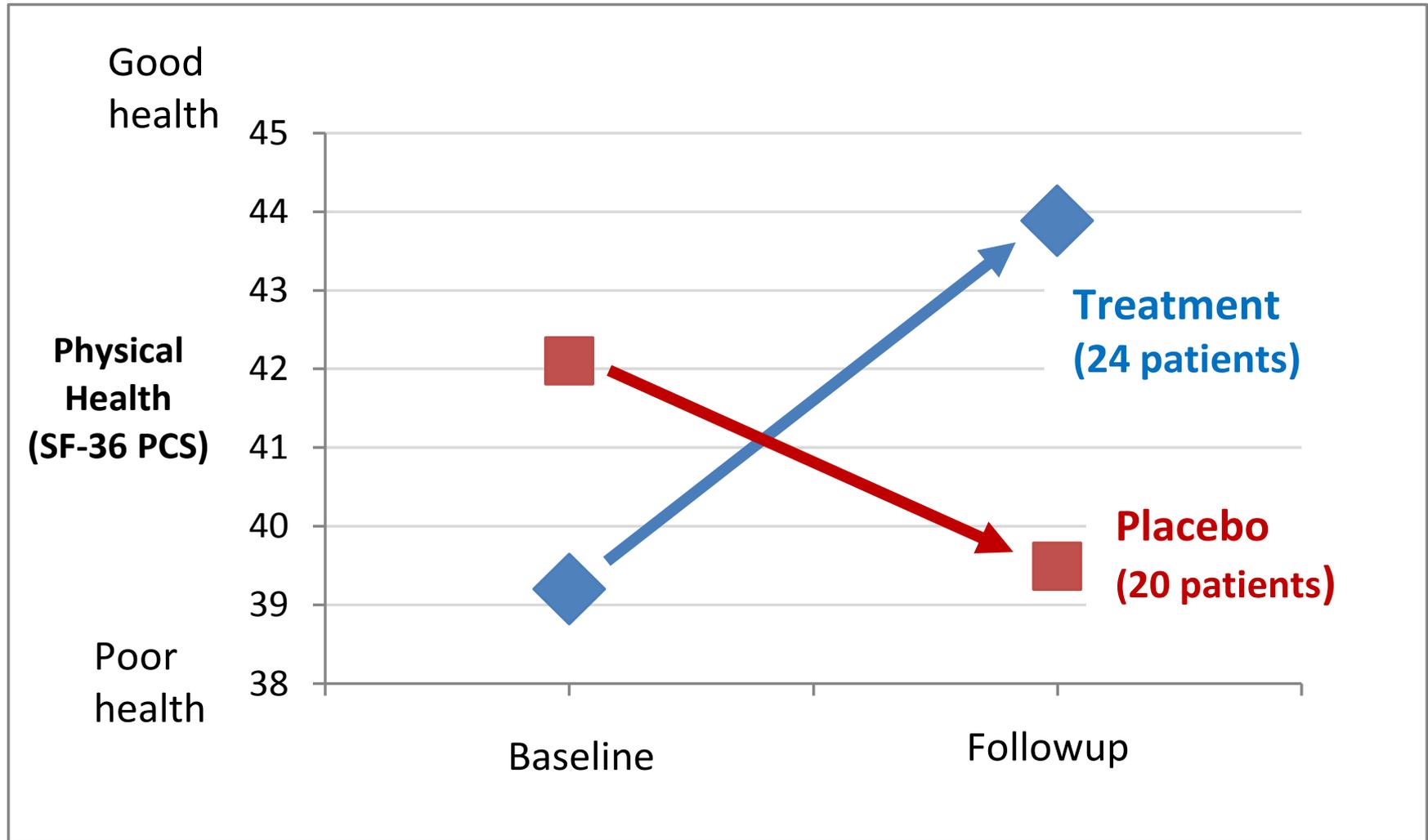
**Figure 1: Mean SF-36v2 scores of patients with AL amyloidosis and of a general population**



Notes: GP=General population; Error bars indicate 95% confidence intervals; GP adjusted to the age and gender distribution of the AL amyloidosis patient sample; Higher scores reflect better health

- \* GP > AL amyloidosis patients,  $p < 0.05$
- \*\* GP > AL amyloidosis patients,  $p < 0.01$
- † GP > AL amyloidosis patients,  $p < 0.001$

# Effect of Dichlorphenamide on Physical Health in Patients with Hypokalemic Periodic Paralysis



Adapted from Sansone et al. Randomized, placebo-controlled trials of dichlorphenamide in periodic paralysis. *Neurology*. 2016;86(15):1408-1416.

# Conclusions

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- Often best to use a mixed methods approach by combining open interviews and surveys
- The specific content of quality of life measures should be informed by patients and caregivers
- If you pick a standard measure, test that it works in this particular situation
- Developing a new survey is a huge effort, but may be necessary for some topics



Thank you.

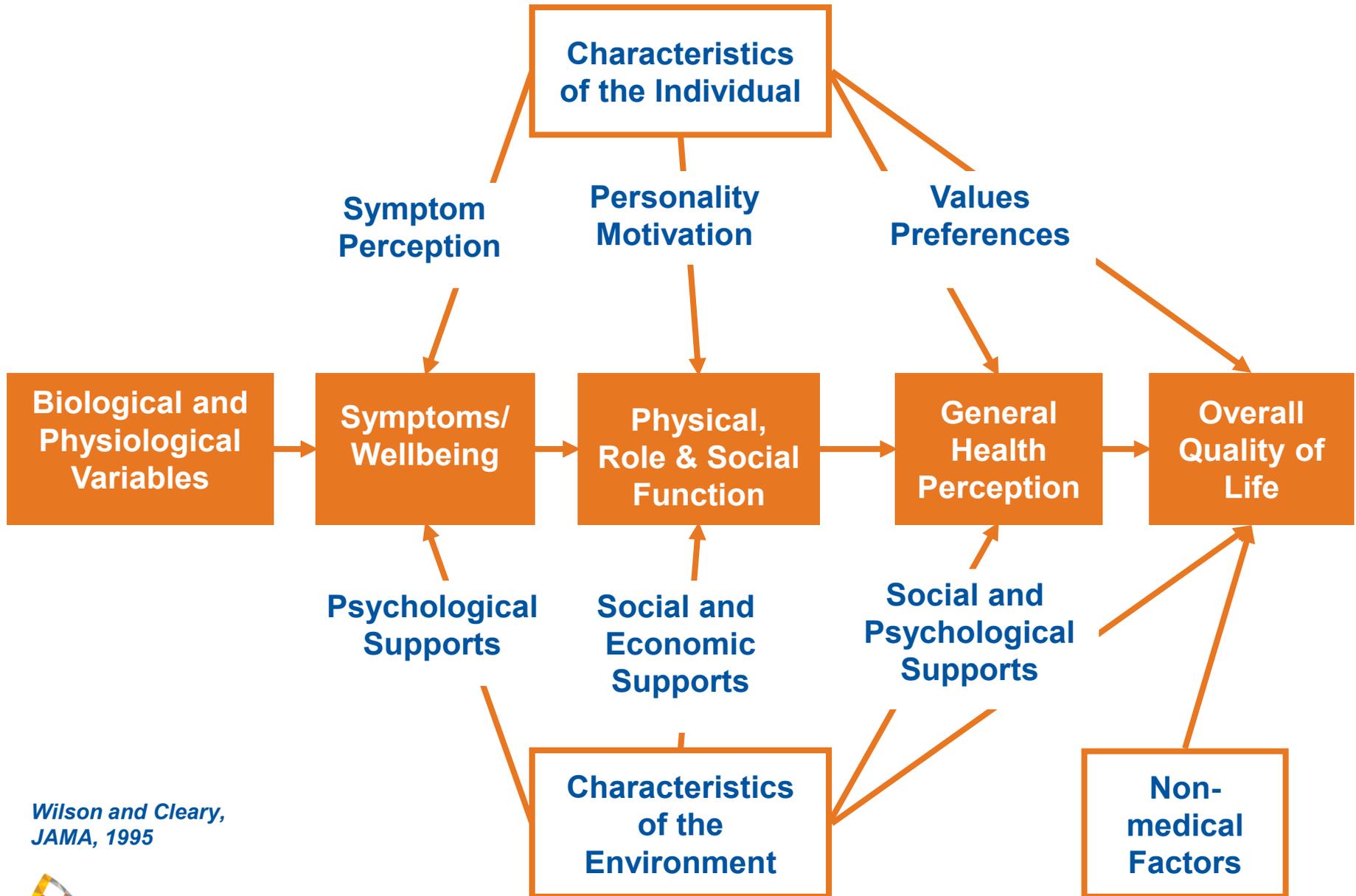


# Possible additional content for rare disorders in general?

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- Scale measuring need for information about disease
  - E.g. “It is difficult for me to find information about my disease”
- Scale concerning need to exchange experience with other patients
  - E.g. “Talking to fellow patients has helped me cope with my disease”
- Scale assessing treatment satisfaction or preference
- Scale assessing caregiver burden

# Model of Relations between Health Concepts



Wilson and Cleary,  
JAMA, 1995