



# Patient-reported Outcomes- what matters to the child?

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RESPECT

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# The Subjective Perspective

- ...beyond assessments of development, growth and performance
- **what is important to kids?**
- ...what affects their wellbeing and functioning in health or disease
- ...and how do they view their health condition and its treatment....?





# Patient reported Outcomes

- Patient–reported Outcomes (PROs) is a term used to denote health data that is **provided by the patient** through a system of reporting.
- A patient–reported outcome is a patient's feedback on **their feelings or what they are able to do** as they are dealing with chronic diseases or conditions and their treatment.
- PROs can be measured before, while and after patients are **undergoing treatment** or are participating in a **clinical trial**([www.NIHPROMIS.org](http://www.NIHPROMIS.org))



## What PROs assess

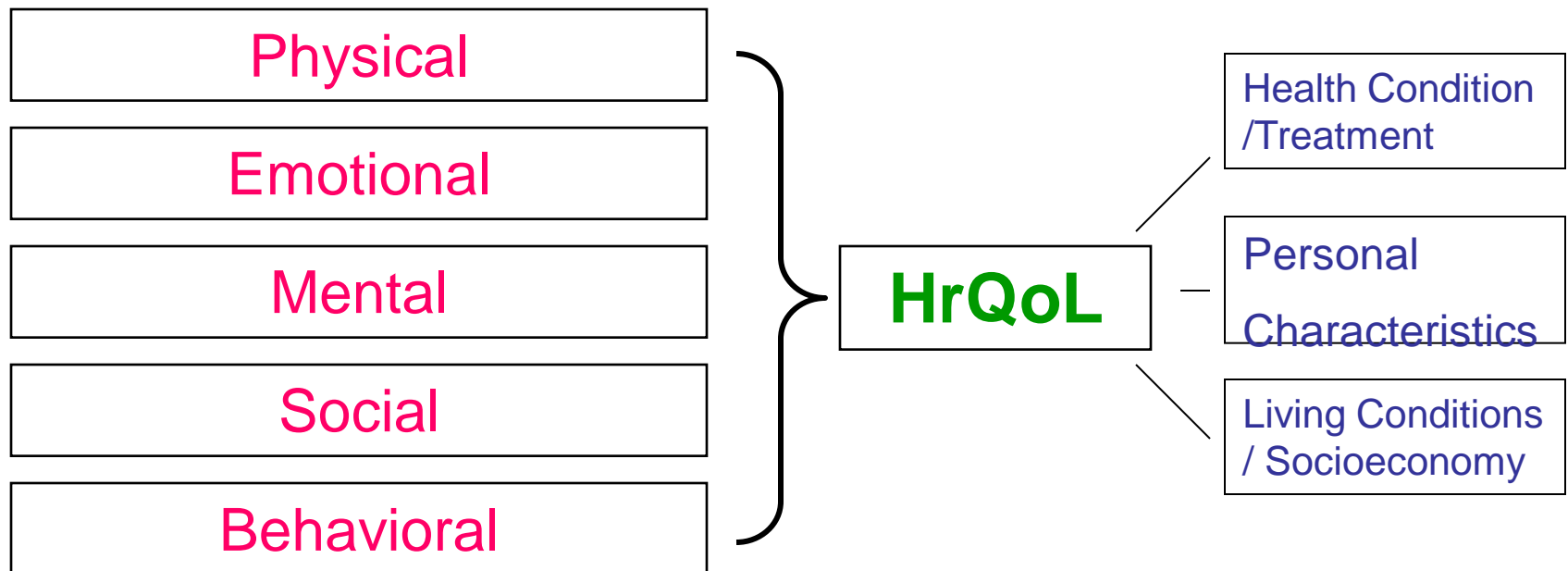
- PRO instruments may be **generic** (to be used in any disease population, covering a broad aspect of the construct) or **targeted** (developed specifically to measure outcomes of importance for persons with specific medical conditions)
- PRO instruments assess the following constructs:
  - Symptoms (impairments)
  - Patient well-being
  - Functioning (disability)
  - General health perceptions
  - Quality of life (QoL)
  - Reports and Ratings of health care
  - Treatment satisfaction
  - Patient preferences



# Model for health related Quality of life

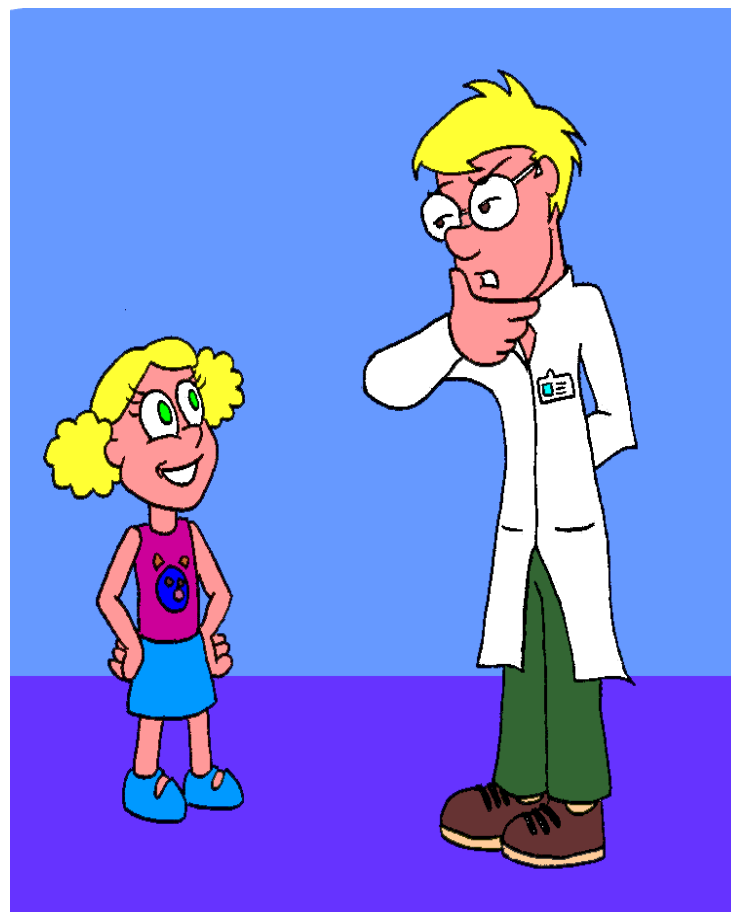
- HrQoL is a multidimensional construct pertaining to several components of well-being and function as perceived by the person and/or observers.

*(e.g. Aaronson et al. 1988)*





# QOL Assessment in Young People: how do we do it ?





# WHO Recommendations

- Instruments should be **child-centered**
- Subjective **self-report** has priority
- Instruments should be **related to age** and developmental stage
- Results should be **cross-culturally** comparable
- Instruments should have a **generic** core and **specific** modules
- Positive **health-enhancing aspects** of QOL should be stressed



# Modular System of QoL Assessment

Targeted  
(eg. Qolissy)

Chronic Generic  
(eg. Disabkids)

Generic (eg. Kidscreen)



# The KIDSCREEN-10 Index

During the past week ...

1. Have you felt fit and well?
2. Have you felt full of energy?
3. Have you felt sad?
4. Have you felt lonely?
5. Have your had enough time for yourself?
6. Have you been able to do the things that you want to do in your free time?
7. Have your parent(s) treated you fairly?
8. Have you had fun with your friends?
9. Have you got on well at school?
10. Have you been able to pay attention?

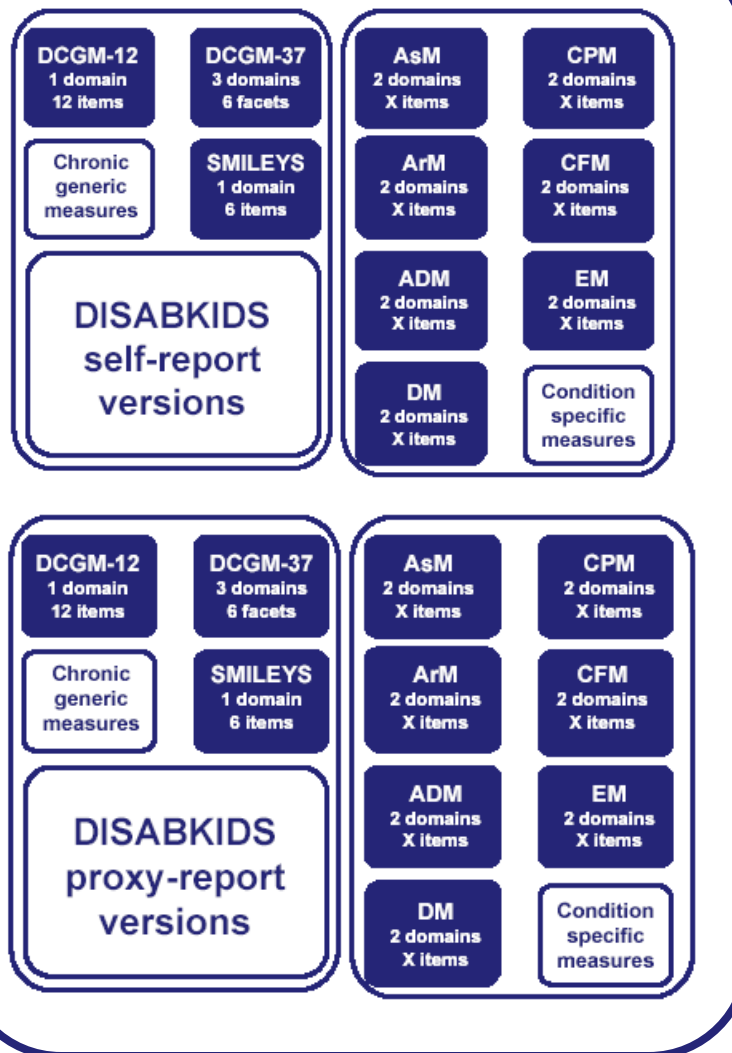
**General  
Quality of Life**



# The DISABKIDS Instrument

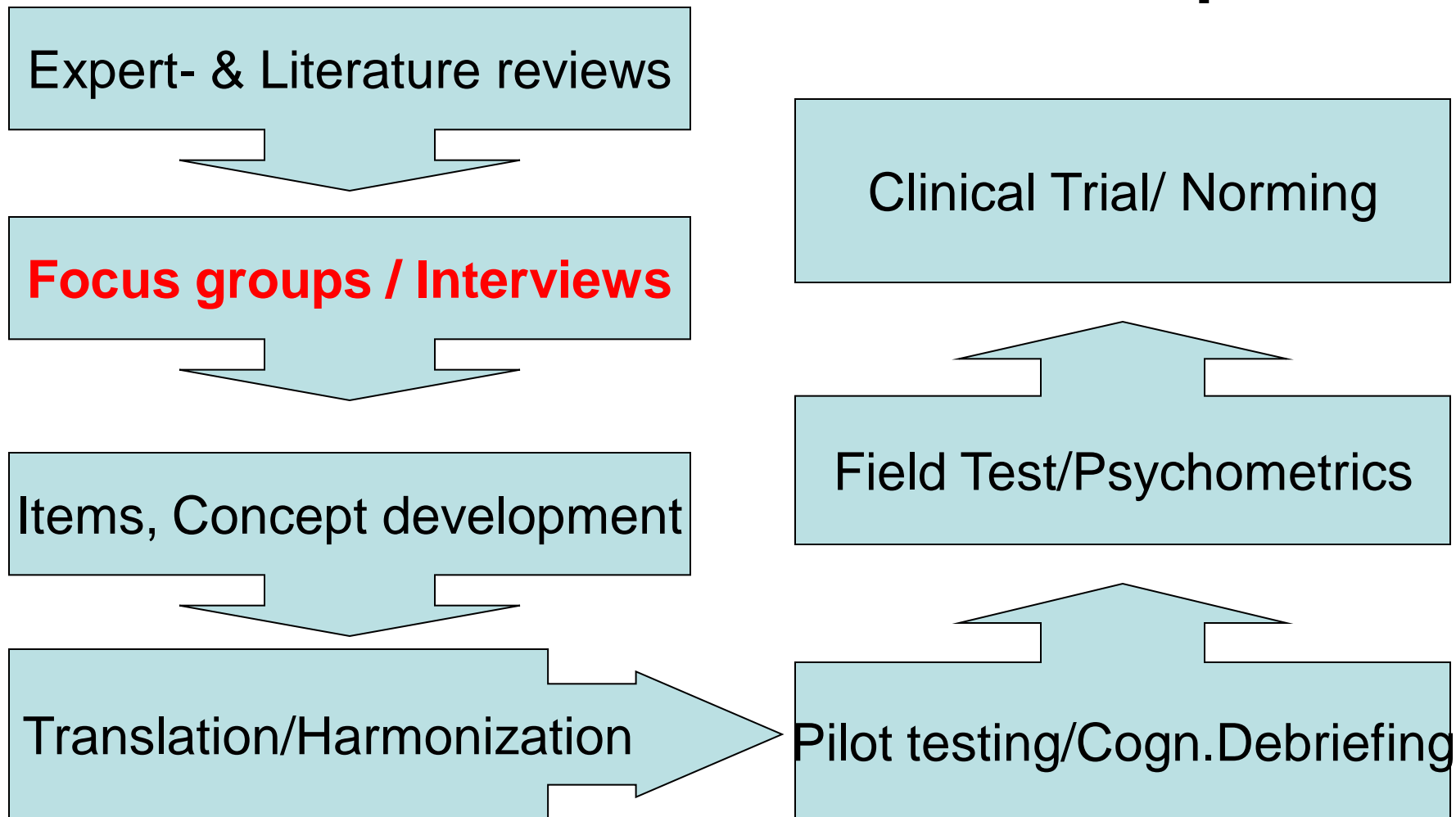
- Three age versions
- chronic generic and condition specific
- Smiley versions
- short- and long forms
- Self-report and parent-report versions
- Paper-pencil and computer versions
- Available more than 12 languages

**Tested in 1600 children**





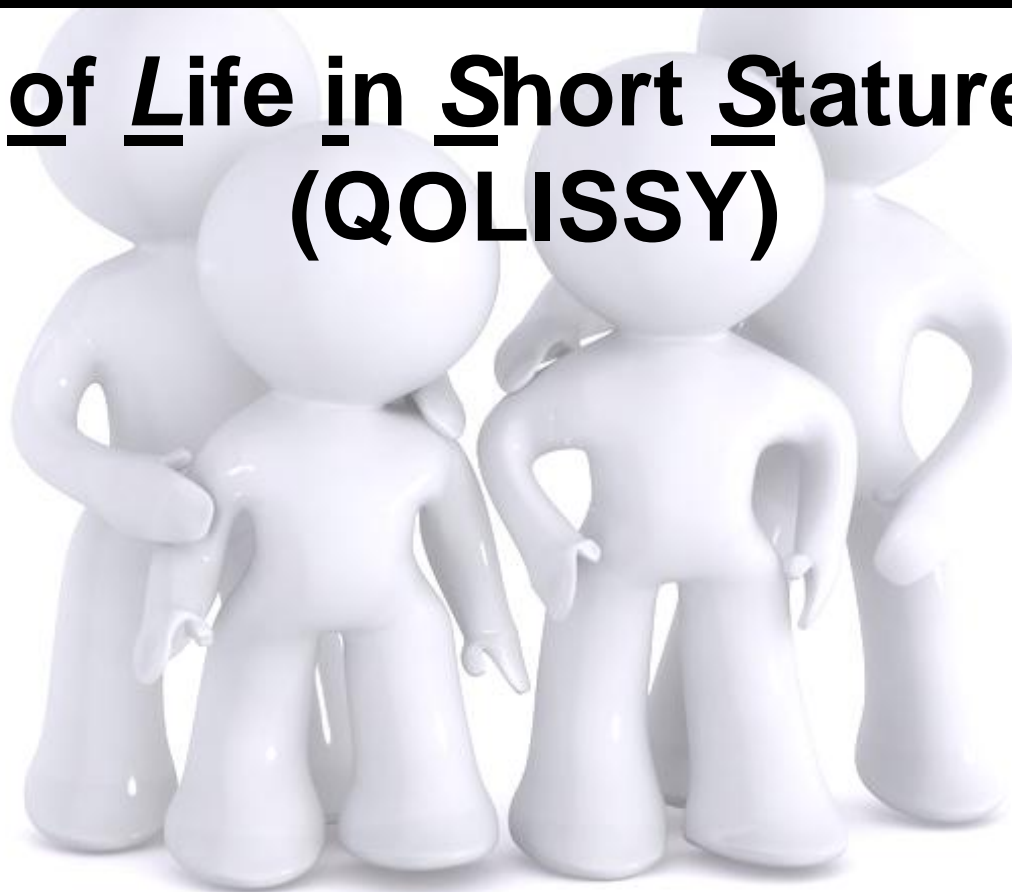
# Steps of crosscultural instrument development





**Finding out what matters:**

**Quality of Life in Short Stature Youth  
(QOLISSY)**





# Purpose of the QOLISSY Study

- **Instrument development (targeted)**

in children & adolescents (age 4-7, 8-12, 13-18 ys), diagnosed with short stature (ISS/GHD), assessing self reported quality of life and the parental perspective in five countries (S,G,F,UK,E)

- **Focus Groups, Pilot Test , Field Test**

in over 1200 children and parents

- **Future Implementation**

in clinical trials and routine care





# Focus Group Results

In meinem Leben stört mich eigentlich, dass mir die Größten aus meiner Klasse dann auch immer sagen: "Du bist so klein, du wächst ja nicht mehr"

„It bothers me that the tall children from my class always tell me: you're so small, you won't grow.“

Diskriminierung

Discrimination

..., age group 3, GHD+, Hamburg

Die tun mir andauernd weh. In der Schule hauen und schlagen die mich und sagen böse Wörter. Und ich weiß nicht warum.  
They hurt me all the time and say bad words and I don't know why

Wird geschlagen in Schule.  
Beaten at school.

Ich finds doof, wenn ich halt immer geärgert werde, so "Gartenzwerg" oder "Zwerg" oder so. Und auch du "kleine Kanalratte" und sowas. Und tun als würden sie mich nicht hören. Und sagen ich gehöre in die erste Klasse.  
I feel bad when they tease me and call me garden gnome or gnome and rat and so on or they pretend that they don't hear me and say I belong into the first grade

teasing (dwarf, grade 1)

Child, age group 2, ISS-, Erlangen

Es hänseln die anderen, ich hatte früher einen in der Klasse der hat mich immer gehänselt, zum Glück ist der durchgefallen.  
Others tease me, especially one did but fortunately he had to repeat the year

teasing

group 2, GHD+, Bonn



# QoL Domains in Short Stature

## Emotion

1. Self confidence
2. Embarrassment
3. Negative affect (angry/sad)
4. Worry about height
5. Bothered about height
6. Feeling hurt
7. Isolation
8. Satisfaction with height
9. Belief/attitudes about height

## Social

1. Teasing
2. Bullying
3. Social comparison
4. Social exclusion
5. Stigma
6. Juvenalization
7. Positive comparison
8. Social support/acceptance
9. Future

## Physical

1. Energy
2. Clothing
3. Recreational Activities
4. Limitation in daily activities

## Treatment

1. Expectations about treatment
2. External influences
3. Psychological effect
4. Treatment administration
5. Side effects
6. Benefits of treatment

## Coping

1. Acceptance
2. Ignoring/ blocking out
3. Agression /acting out
4. Denial
5. Seeking receiving support



# Domains of the QOLISSY

	Domain	Concept/ content
3 QOL Core Dom- ains	<b>Physical</b>	<b>Refers to the physical limitations that the child can experience in everyday life due to his short stature</b>
	<b>Social</b>	<b>Refers to the way short stature interferes with the child's social life: remarks, bullying, teasing, social isolation, feelings of rejection...</b>
	<b>Emotional</b>	<b>Refers to the child's feelings and emotions with regards to his short stature (being different, insecure, sad...)</b>
Pre- dictor Do- mains	<b>Coping</b>	<b>Refers to the way the child copes with negative feelings or experiences due to his short stature</b>
	<b>Beliefs</b>	<b>Refers to the child's general beliefs about stature</b>
	<b>Treatment</b>	<b>Refers to the child's experience linked to growth hormone treatment (when relevant)</b>
Parent addi- tional Do- mains	<i>Parents: Future</i>	Refers to the parent perceived child's worries about the future in relation to his short stature
	<i>Parents: Effects on parents</i>	Refers to the impact the child's growth problem has on his parents' feelings (helplessness, guilt, concern, anxiety...)



# QoL in Kids: Questions and Answers

- Is QOL a relevant concept for children? **YES**
- Are dimensions of QOL identical for children and adults? **NO**
- Do children have the ability to reflect and express own wellbeing and function? **YES**
- Are children's statements unreliable? **NO**
- Is QOL dependent on age/developmental status? **YES**
- Can QoL be measured in children? **YES**
- Can parents QOL assessments replace childrens self reports of QOL ? **NO**



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patient needs



**Please include in your trials  
outcomes that matter to us**



Thank you for your  
attention



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# Instrument Development

**Expert- & Literature reviews**



**Focus groups & Interviews**



**Conceptual Model & Item development**



**Pilot Testing & Cognitive Debriefing**



**Field Testing**





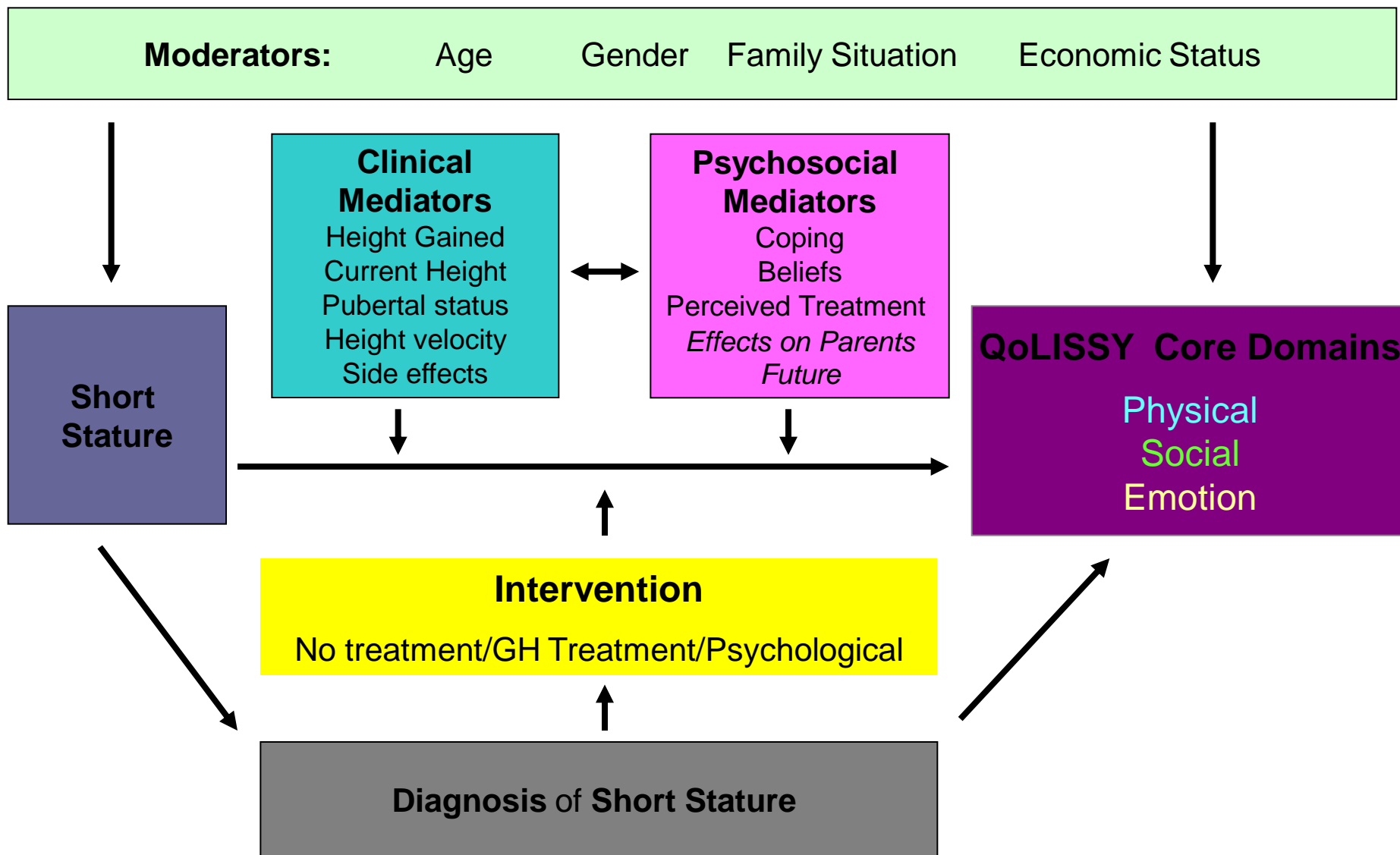
# Dimensions of QOL in Children

- Physical (mobility, vitality, pain)
- Emotional (depression, anxiety, selfregulation)
- Social (family, school, friends)
- Self-concept (coping, body image, self-esteem)
- Cognitive (concentration, learning, memory)
- Functional (role function, skills, autonomy)



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# QOLISSY: Conceptual Framework



During the past week,  
how much of the time did  
you feel happy?

never

seldom

sometimes

often

all of the time





# Objectives of HRQOL Research

- Description of well-being and function of populations, Identification of needs for care in persons with health problems  
(**epidemiological perspective**)
- Evaluation of treatment effects, Routine outcome assessment, Quality assurance of care, individual needs  
(**clinical perspective**)
- Comparison of treatment costs and benefits, Evaluation of services , Allocation of resources  
(**health economical /health systems perspective**)



## Fifth EC Framework Programme Quality of Life and Management of Living Resources

- **Public Health Research at the Population Level**
  - Screening for and Promotion of health-related Quality of Life in Children & Adolescents - A European Public Health Perspective
- **Research relating to young persons with disabilities**
  - Quality of life in Children and adolescents with disabilities and their families
  - Assessing patient views and patient needs for comprehensive care
- **KIDSCREEN**
- **DISABKIDS**



## Kindl –R Instrument

Kiddy-KINDL

Kid-KINDL

Kiddo-KINDL

Age	Assessment	
	self	parent
4-7	✓ interview	✓ proxy
8-12	✓	✓ „circular“
13-16	✓	✓ „circular“

Parent report version: During the past week my child was happy

Parent circular version: My child would say: ‘..... I was happy’.



## Examples with years of age

- **CHQ**  
(Child Health Questionnaire), USA, 1993, Landgraf et al., 5 -18
- **CHIP**  
(Child Health and Illness Profile), USA, 1993, Starfield et al., 11 -17
- **KINDL-R**  
(HrQoL in Children and Adolescents), D, 1998 Bullinger et al., 4 – 16
- **TACQOL**  
(Kids Quality of life tool, Vogels et al., NL, 1998, 6 -15
- **VSP-A**  
(Perceived Health of Adolescent), F, 1998, Siméoni et al., 11 – 17
- **PedsQL**  
(Pediatric Quality of Life Inventory), USA, 1999, Varni et al., 2 – 18



## The European QOLISSY Team

