

Prognostic Factors for Improved Health-Related Quality of Life in Children and Adults With Primary Antibody Deficiencies



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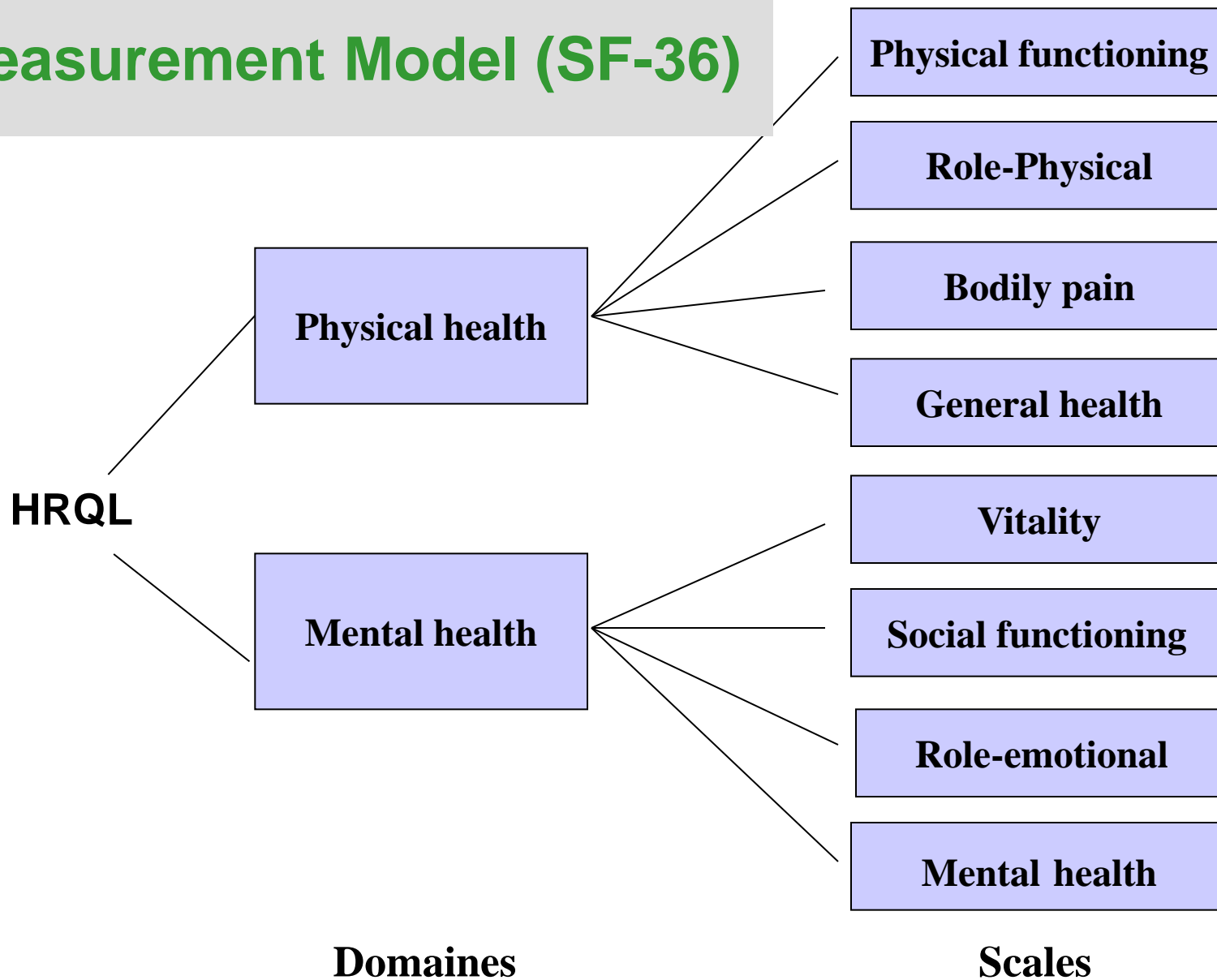
Subcutaneous Immunoglobulin (SCIG) Self-Infusions at Home

- Rapid SCIG self-infusions have an excellent safety profile, are efficacious, and can be accomplished at home.
- IVIG and SCIG replacement therapies are both efficacious to a similar extent.
- Health related quality of life (HRQL) and treatment satisfaction (TS) may be used as criteria for an evidence-based choice of treatment alternatives.

Health-related Quality of Life (HRQL)

- Subjective health status (HRQL) assessment, often not captured by conventional clinical measurements.
- Points to aspects of a person's experience which are affected only by health care interventions.
- Generic HRQL Questionnaires:
 - e.g., SF-36 (age \geq 14 years),
 - CHQ-PF50 (age $<$ 14 years)

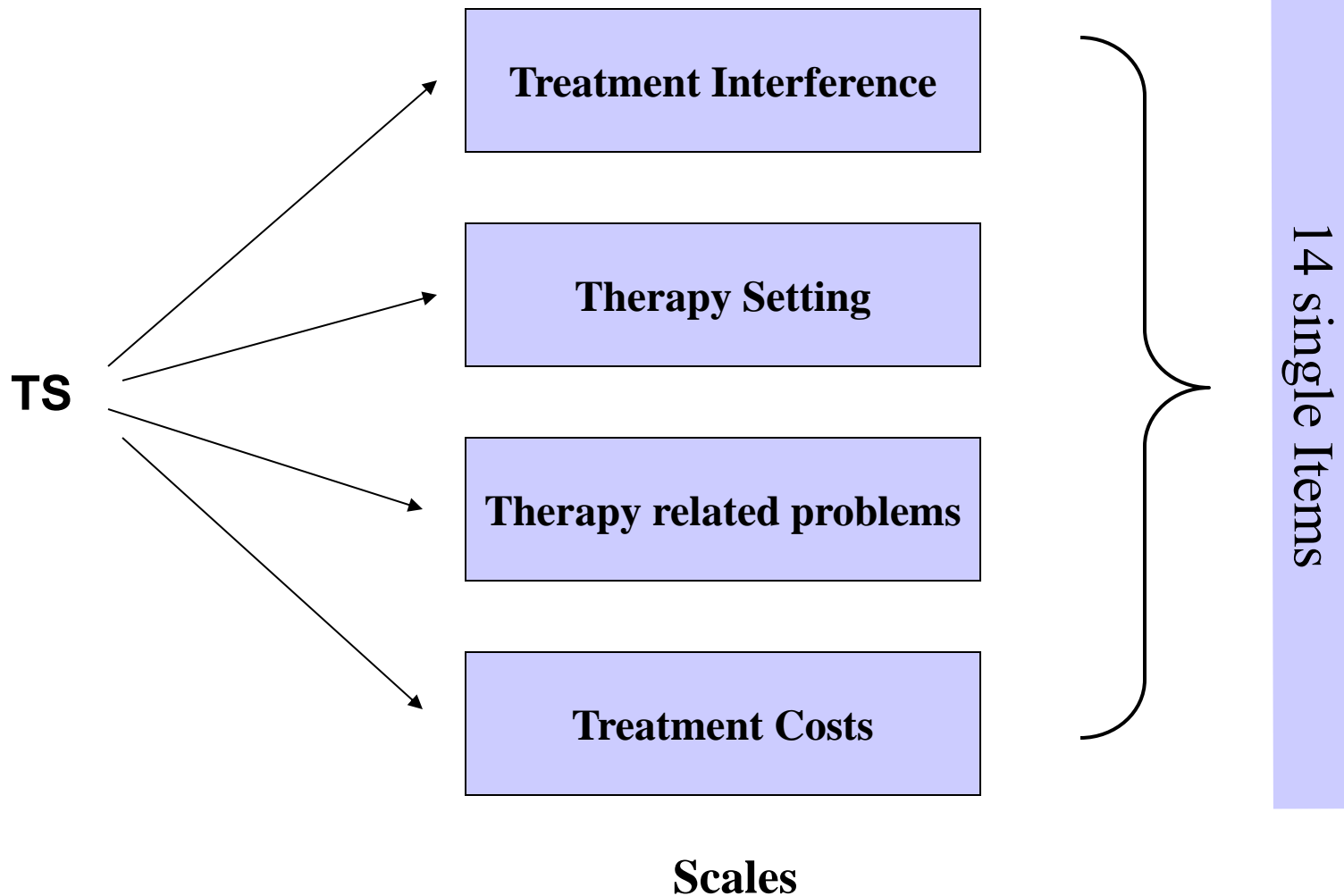
Measurement Model (SF-36)



Treatment Satisfaction (TS)

- TS is defined as the patient's evaluation of the treatment process and the outcome of treatment.
- Evaluation is a results of a comparison with subjective standards (Shikiar & Rentz 2004).
- TS as antecedent of adherence.
- Relevant TS domains encompass at least:
 - efficacy, side effects, ease & convenience of a treatment
- Additional domains possible (e.g., training process).

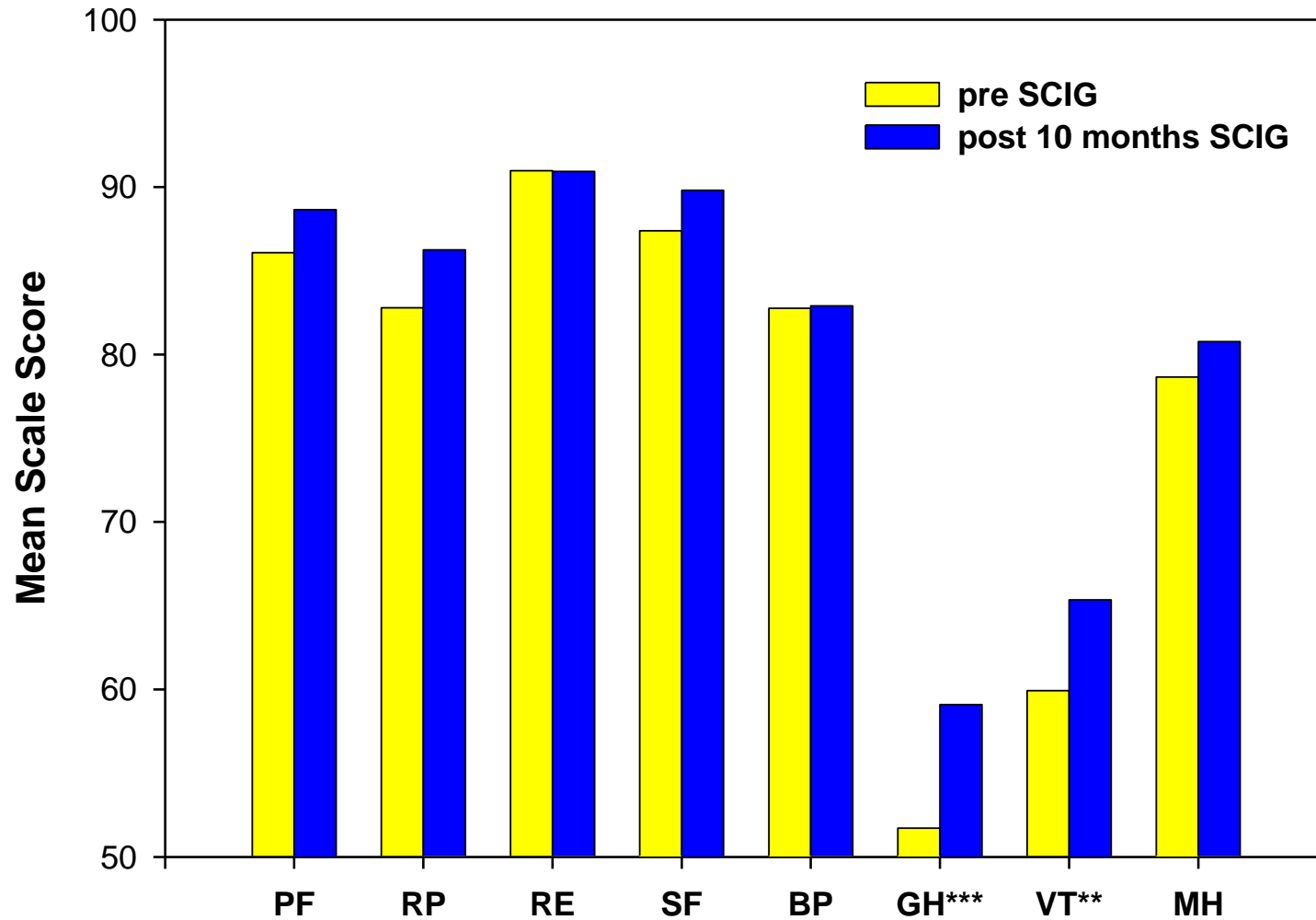
Measurement Model (LQI)



HRQL & TS in Patients Receiving SCIG

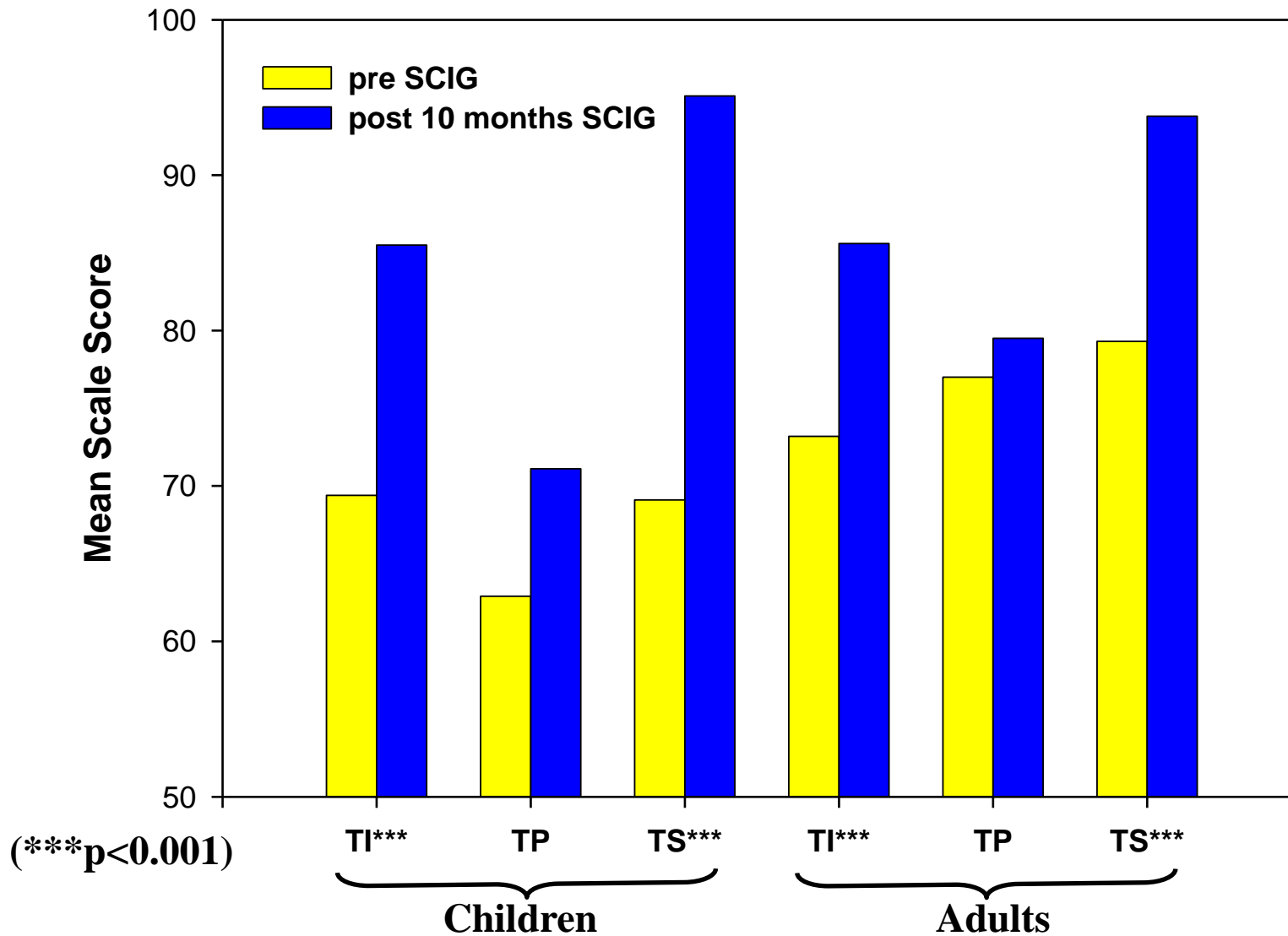
- Two multinational studies in patients with primary antibody deficiencies (PAD) switching from IVIG at the hospital/doctor's practice to SCIG self-infusions at home.
- Generic (SF-36, CHQ-PF50), TS (LQI) questionnaires, preference questions, etc. were filled in by the patient/parents before switching to SCIG and during study course.
- Various clinical measurement (e.g., IgG trough level).

SF-36 (Adults) Results After 10 Months SCIG



(**p<0.01, ***p<0.001)

LQI Results After 10 Months SCIG



Prognostic Factors for HRQL - Why?

- To learn about the relative importance of several variables that affect or are associated with patient's HRQL.
- To assess clinical landmarks during the course of the illness and to decide whether changes in treatment strategy are warranted.
- To improve the design of clinical trials, e.g., by stratified randomization.
- To improve the analysis of trials, e.g., by adjusting for imbalances.

What is Known From Literature?

Høybråten Sigstad *et al.* (2005): Survey of 55 PAD patients using SF-36.

- Unemployment
- PID-related strain
- Female gender
- IVIG administration
- Stressful events

Negative predictors for HRQL

No association between HRQL and age, diagnosis, HCV infection, and cohabitation was found.

Literature (continued...)

Howard *et al* (2005): Survey of 41 XLA patients using SF-12.

- Chronic lung disease was negative for Mental Health domain.
- No association between HRQL and age, income, insurance, marital status, IgG therapy at home or clinic.
- Concluded that it was treatment, rather than signs and symptoms of disease, that was burdensome to patients.

Current Study - Potential Background Factors

Age

Gender

Exposure to smoke

Smoker

Clinical Study

Persons in household

Education

Weight class (BMI)

Social/demographic

Duration of antibody deficiency (years)

Serum IgG level prior to HRQL assessment (g/L)

Concomitant disorders

Infection episodes within 4 weeks prior to the HRQL assessment

Local tissue reactions within 4 weeks prior to the HRQL assessment

Where did patient receive IgG prior to the study

Medical

Current Study - Prognostic Models

Relationship between background factors (social/demographic/medical) and

- SF-36 scales at 10 months for adults
- CHQ-PF50 scales at 10 months for children

For children potential prognostic variables were confined to body weight class, family size, age, duration of PAD, serum IgG level prior to the month 10 assessment, presence of local reactions, and infection episodes within 4 weeks prior to the month 10 assessment.

Current Study - Results

The following significant background factors could be identified:

Favourable for HRQL

Low age

(Ex)-Smoker

High IgG levels

Absence of joint muscle/skeletal disorders

Absence of local reactions

Low baseline scale value

SF-36 Scales

RP, RE

VT, SF, MH

VT, RP.

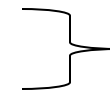
RP

GH

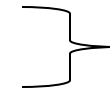
All scales



Adults



Children



All

Conclusions

- SCIG home-therapy regimen using weekly self-infusions significantly improves the HRQL and TS in adult patients and in children and their families.
- Patient- and parent-reported data should be collected in a standardised order to identify vulnerable patients/families.
- Age and concomitant joint/muscle/skeletal disorder were predicting factors, as well as IgG levels, for a poorer HRQL.
- Weekly needle sticks had no negative impact on HRQL of adult patients.
- Larger studies are needed to draw more extensive conclusions.