

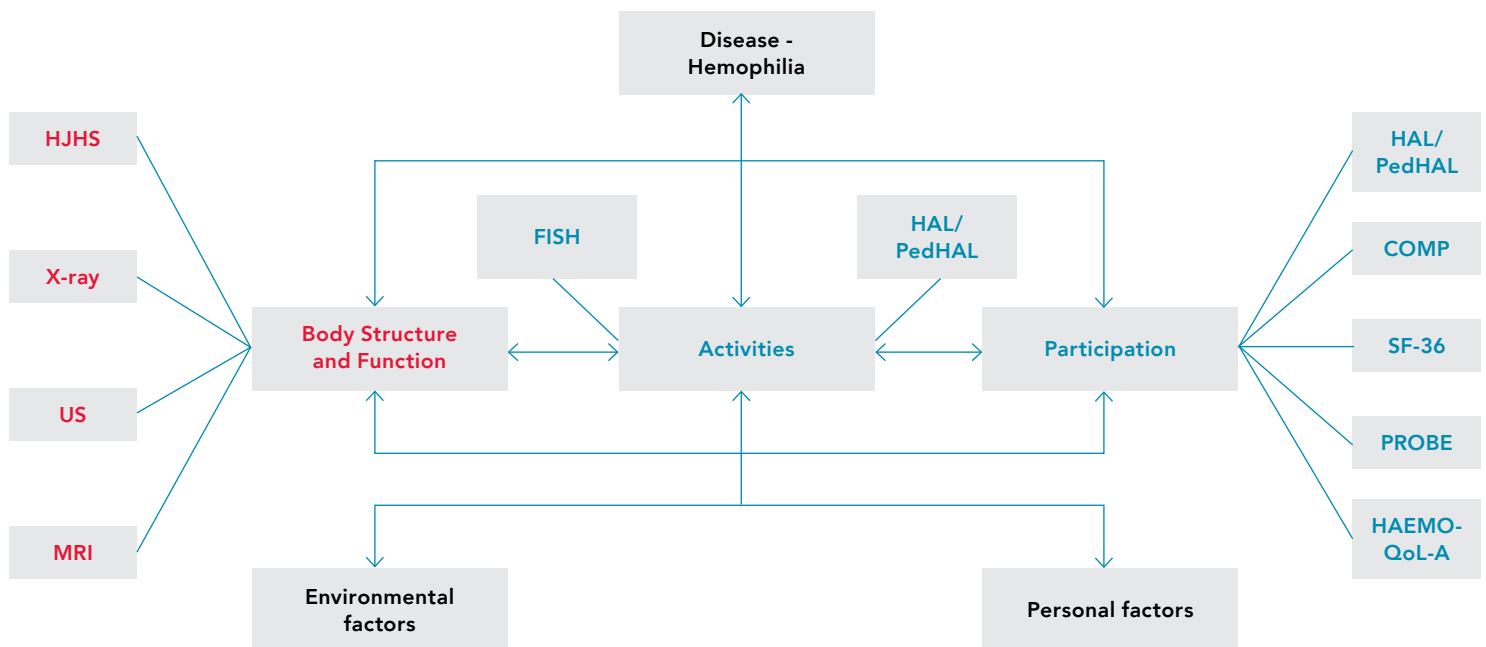
Chapter 11

OUTCOME ASSESSMENT

Pradeep M. Poonnoose, Brian M. Feldman, Piet de Kleijn, Manuel A. Baarslag, Radoslaw Kaczmarek, Johnny Mahlangu, Margaret V. Ragni, Glenn F. Pierce, Alok Srivastava

Outcome Assessment: WHAT YOU NEED TO KNOW

Outcome assessments in hemophilia measure a variety of parameters including body structure and function, activities and participation, burden of disease and health status



Body Structure and Function

Refers to the status of joints and specific muscle groups, assessed both clinically and radiologically

Activities and Participation

Refers to instrumental activities of daily living (e.g., walking, climbing steps, brushing teeth, going to the toilet) and involvement in life situations in the context of social interactions, respectively

Chapter 11 OUTCOME ASSESSMENT

Recommended measures of Body Structure and Function

HJHS

The best studied of the joint assessment instruments in both children and adults

X-Ray

The Pettersson score is the most widely used imaging measure but is not sensitive to early change

US

Ultrasound imaging can detect joint effusion, early joint disease, and subclinical joint disease

MRI

MRI is likely the most sensitive measure of joint structure but is expensive

Recommended measures of Activities and Participation

HAL & PedHAL

A disease-specific, self-reported measure of activities for adults and children

FISH

An observer assessment of functional independence for people with hemophilia

PROBE

A self-reported measurement of the impact of hemophilia on daily life for people with hemophilia

COPM & MACTAR

Generic instruments to assess perception of changes in activities and participation

Health-Related Quality of Life

The **EQ-5D** and **SF-36** are widely used generic instruments for assessing QoL in hemophilia. The Canadian Hemophilia Outcomes-Kids Life Assessment Tool (**CHO-KLAT**) was developed for children with hemophilia and the Hemophilia Well-Being Index and the hemophilia-specific QoL questionnaire for adults (**HAEMO-QoL-A**) for adults with hemophilia.