Chapter 11
OUTCOME ASSESSMENT

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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.