

# **Evaluation of the quality of life of severe hemophiliacs A under personalized prophylaxis guided by the pharmacokinetics of factor VIII**

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## **Introduction :**

Haemophilia, through its associated symptoms, functional limitations and treatment burden, has a direct impact on the health-related quality of life (HRQoL) of patients and their families. Important aspects of HRQoL in haemophiliacs can be assessed using appropriately developed measures, such as the Haemophilia Adult Quality of Life ( Haem -A- QoL ) questionnaire. First published in 2005, the Haem -A- QoL questionnaire was originally validated in an Italian observational study in 233 adult patients with haemophilia A and B.

The Haem -A- QoL was developed using data from focus groups of adults with haemophilia who were asked about their perceptions of their health and treatment. It consists of 46 items divided into ten dimensions: physical health, feelings, self-perception, sports and leisure, work and school, adjustment, treatment, future, family planning and relationships/partners.

Psychometric characteristics included good reliability, high convergent validity with the SF-36 questionnaire, and high discriminant validity regarding the severity of haemophilia and the presence of infections. The score calculation is performed by transforming the scores obtained in each dimension, as well as the total score, on scales ranging from zero to 100, with zero representing the best HRQoL.

**Objectives:** As clinical assessments may not be sufficient to adequately characterize hemophilia-associated morbidity, our study aims to assess the quality of life of severe hemophiliacs A without inhibitors under personalized prophylaxis guided by the pharmacokinetics (PK) of factor VIII.

## **Patients and methods:**

This is a prospective study (2021-2023) in severe and moderate haemophiliacs with severe bleeding phenotype without inhibitors, aged 17 years and older previously on standard prophylaxis or on-demand treatment who received personalised prophylaxis guided by factor VIII pharmacokinetics according to MyPkfit. Patients were asked to complete the Haem -A- QoL questionnaire before and after PK personalization.

## **Result :**

Twenty-two patients were evaluated. Median age was 24 years. Before guided PK personalization of prophylaxis: the mean total Haem -A- QoL scale score was 45.07, ranging

from 30.43 to 64.03. The dimensions “Sports and leisure” and “Physical health” had the highest means (81.13 and 58.4 respectively), indicating poorer quality of life, and the dimensions “Coping” and “Relationships and partners” were the least impaired (mean 15.15 and 10.22 respectively). Table 1 shows the mean Haem -A- QoL questionnaire scores , in total and by dimension before targeted PK prophylaxis.

**Table 1:** Evaluation of the HAEM-A-QUAL questionnaire adult version > 17 years before PK personalization .

<b>Item</b>	<b>Average score</b>	<b>Standard deviation</b>	<b>Maximum score</b>	<b>Minimum score</b>
<b>Physical health</b>	58.4	8.5	75	40
<b>Feelings</b>	49.71	18.54	75	12
<b>Self-perception</b>	49.31	15.6	100	30
<b>Sports and leisure</b>	81.13	11.11	100	55
<b>Work and school</b>	54.54	16.5	100	37.5
<b>Adaptation</b>	15.15	13.02	33.33	0
<b>Treatment</b>	56.78	12.57	71.87	12.5
<b>Future</b>	56.36	15.13	100	35
<b>Family planning</b>	23.01	16.96	56.25	0
<b>Relationship/Partner</b>	10.22	18.35	50	0
<b>Total</b>	45.07	7.91	64.03	30.43

Under personalized prophylaxis guided by factor VIII pharmacokinetics: all scores decreased, reflecting an improvement in quality of life. The mean total score decreased from 45.07 to 14.85. All items showed a significant decrease with a mean score very close to the minimum scores. Table 2 shows the mean scores of the Haem -A- QoL questionnaire , in total and by dimension under targeted PK prophylaxis.

**Table 2:** Evaluation of the HAEM-A-QUAL questionnaire adult version > 17 years under personalized PK-guided prophylaxis.

<b>Item</b>	<b>Average score</b>	<b>Standard deviation</b>	<b>Maximum score</b>	<b>Minimum score</b>
<b>Physical health</b>	16.81	17.63	65	0
<b>Feelings</b>	19.31	17.46	62.5	0
<b>Self-perception</b>	18.86	13.44	45	0
<b>Sports and leisure</b>	27.5	13.51	55	0
<b>Work and school</b>	10.78	10.41	37.5	0
<b>Adaptation</b>	11.3	12.19	25	0
<b>Treatment</b>	20.16	9.53	46.87	0

<b>Future</b>	18.63	12.06	45	0
<b>Family planning</b>	2.55	6.86	25	0
<b>Relationship/Partner</b>	4.01	10.42	33.33	0
<b>Total</b>	14.85	8.42	33.75	6.25

## Discussion

Before targeted PK personalization, the dimensions “Sports and leisure” and “Physical health” reached the highest means, a consequence of the high prevalence of arthropathy observed in these patients. With personalized prophylaxis and the availability of clotting factor concentrates, the old practice of keeping haemophiliacs away from physical activities to prevent traumatic joint injuries must be forgotten. There is a growing trend to encourage haemophiliacs to engage in sports activities, not only for the physical benefits, but also as a mechanism for social inclusion.

Under guided PK prophylaxis, all items showed a significant decrease with a mean score very close to the minimum scores reflecting the positive impact of the availability and regularity of prophylactic treatment, home treatment, therapeutic education sessions, and especially the personalization of treatment in severe hemophiliacs A.

For the dimensions "feelings", "self-perception" and "Future" we note more confidence and hope contrary to the depression initially observed.

## Conclusion and perspective

Health-related quality of life in our hemophilia patients, measured by the Haem -A- QoL instrument, showed the superiority of guided PK prophylaxis compared to previous therapies standard prophylaxis and on-demand treatment with improvement of physical health allowing healthy sports activity and good socio-professional integration.