

Treatment outcome in young adults survey of 8 Countries – Preliminary Results

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OBJECTIVES

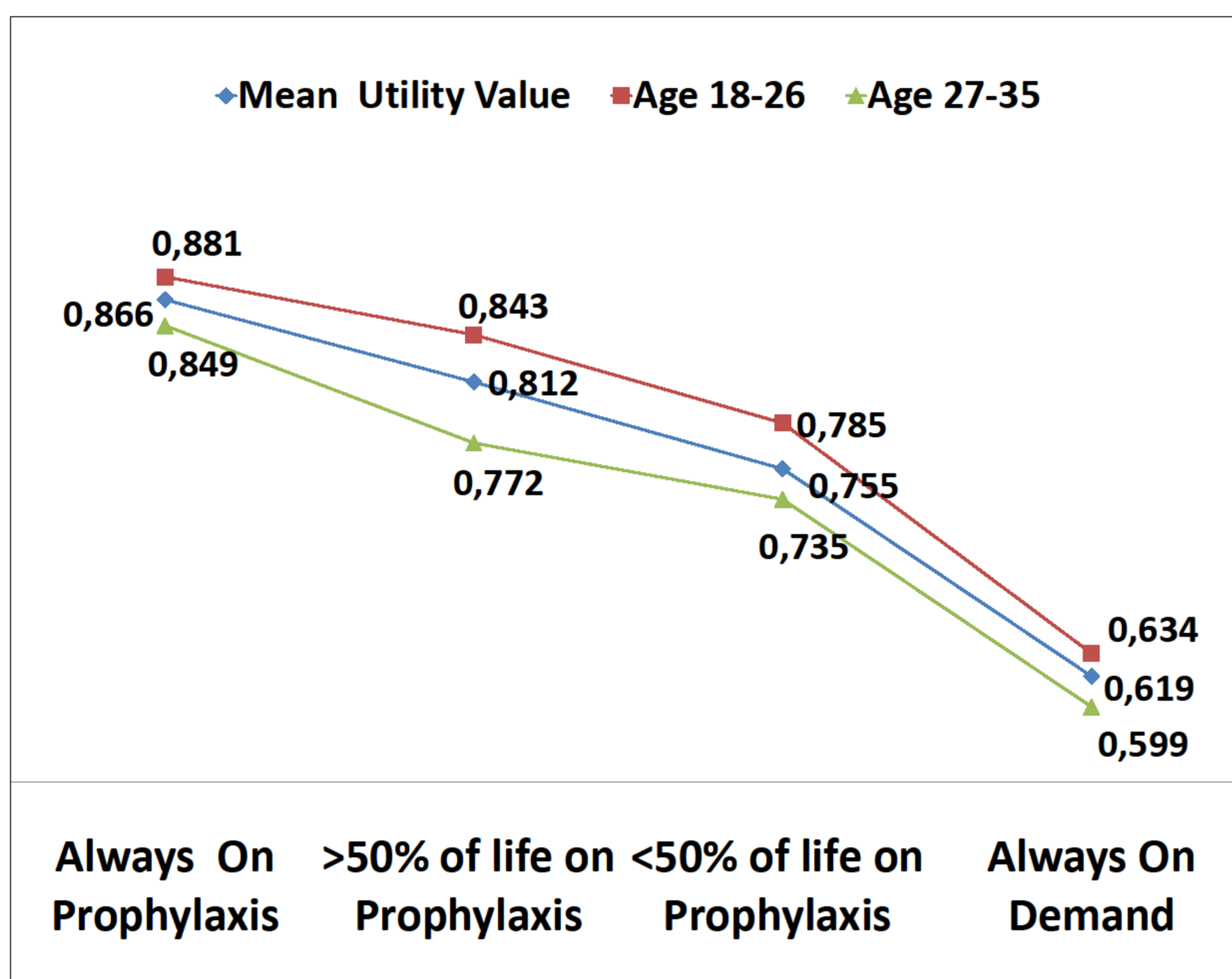
In 2009, results of a survey of prophylaxis, on-demand and combined treatment in 20-35 year old men with severe haemophilia in four European countries showed that the quality of life of patients on long-term prophylaxis is significantly better than those receiving on-demand only or individuals who have changed treatment regimens over their life time (combined group). In 2011, the survey was repeated to further examine the differences in respondents who had full access to prophylaxis and those who continued entirely with varying levels of on-demand therapy. In addition countries with distinctly different prophylaxis regimes - Canada and Netherlands - were included.

METHODS

National Haemophilia organisations in Canada, France, Ireland, The Netherlands, Poland, Romania, Sweden and the UK were asked to participate by randomly selecting 20 severe haemophilia patients aged 18 to 35 years and by asking them to complete a survey. The data collection was performed by e-mail or phone interview. 124 responses were received from 6 countries. Of these, 8 respondents were moderate and 13 had a history of inhibitors and were excluded from the analysis. The data collected was sociodemographic data, medical data and responses to the EQ-5D questionnaire. The medical data collected was the type of haemophilia, severity, treatment regime (prophylaxis vs. on-demand, length of time on each regimen), current regimen, history of inhibitors, bleeds per year, target joints, serious bleeds (head or soft tissue) bleeds, mobility, recurring bleeds, surgery, pain and use of pain medication. Data on days missed from work due to haemophilia as total number of days missed from work per year was collected. We analysed the number of bleeds related to the time spent on prophylaxis. The sample was split into four groups: *Always On-demand* (N=26), *<50% of their life on Prophylaxis* (N=26), *≥50% of their life on prophylaxis* (N=35) and *Always on Prophylaxis* (N=15). We evaluated the differences regarding the seriousness of bleeds and also the health utility value in these categories between The Netherlands (N=12), Ireland (N=16), Poland (N=20), the UK (N=12), France (N=13) and Canada (N=30). ANOVA, Correlation matrix and Chi square were used to analyse the data in PASW 18.

RESULTS

The majority of the *Always On-demand* group (61%) reported more than 30 bleeds/year. In the *Always on Prophylaxis* group, 53% of respondents reported less than 3 bleeds/year and no respondent reported more than 7 bleeds in the last year. We found significant differences regarding a greater presence of target joints, serious bleeds, recurring bleeds and surgical procedures in the *Always On-demand* group compared to the *≥50% of their life on Prophylaxis* and the *Always on Prophylaxis* group. There were significant differences in health utility value (Figure 1) among the *Always On-demand* group (0.619) having a significantly lower ($P \leq 0.01$) health utility value compared with the *≥50% of their life on Prophylaxis* (0.812) and the *Always on Prophylaxis* group (0.866). The *Always On-demand* group had significantly more mobility problems than those with *≥50% of their life on Prophylaxis* ($P \leq 0.05$) and significantly more pain and discomfort than the *≥50% of their life on Prophylaxis* ($P \leq 0.05$) and the *Always on Prophylaxis* group ($P \leq 0.001$). The Dutch cohort reported the lowest rate of target joints, serious bleeds, mobility issues, problems with recurring bleeds and lowest rate of daily pain compared to all countries, with no patients requiring invasive surgical procedures.



Poland reported the most problems with mobility and pain and also had the highest rate of early retirement due to bleeding problems with 15% of the group retiring at an average age of 32; compared to the 2.2% with an average retirement age of 30 in the rest of the group. The Netherlands had the highest health utility value with a mean of 0.915 followed by Canada (0.791), Ireland (0.786), UK (0.768), France (0.687) and Poland (0.629).

CONCLUSIONS

Prophylaxis started at an early age and continued into adulthood results in less bleeding, less damage to joints, less serious bleeds and less recurrent bleeding episodes. Prophylaxis reduces problems with mobility and reduces pain and discomfort. As a result, people with severe haemophilia who have been on prophylaxis for their entire lives to date are reporting a quality of life much closer to their peers without haemophilia.

