Implementation of the French primary long-term prophylaxis guidelines: A real-world prospective study of the FranceCoag PUPs cohort

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ABSTRACTS

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ORAL PRESENTATIONS

OR01 | Implementation of the French primary long-term prophylaxis guidelines: A real-world prospective study of the FranceCoag PUPs cohort

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Introduction: Primary long-term prophylaxis (LTP) efficiently prevents arthropathy in children with severe hemophilia. In early 2000s, the French group of hemophilia treaters (CoMETH) published their first LTP guidelines. Several issues may impair the actual LTP use in young patients. However, real-world evaluations of the implementation of such guidelines are scarce. This study aimed at prospectively analyzing the LTP guidelines implementation in France and at identifying factors modulating the LTP initiation.

Methods: The study group consisted of 395 boys from the FranceCoag PUPs cohort. All included patients had severe hemophilia A or B (FVIII/FIX <1%) and were born in 2000-2009 (i.e. after the French LTP guidelines dissemination). Sociodemographic, clinical, biological, genetic, and therapeutic data were prospectively recorded during medical visits in the hemophilia treatment center (HTC). The study group was compared to a historical group of 44 registered boys with severe hemophilia born in 1996 (retrospective data collection).

Results: In the study group, the median age at first joint bleed was 1.9 years (IQR 1.1-3.0). The cumulative incidence of primary LTP use according to age was dramatically higher in the study group compared to the historical group (e.g. cumulative incidence of LTP use 59.2% versus 8.5% at three years of age, respectively). The prevalence of

ineligibility to the guidelines, delay or absence of LTP initiation, anticipated LTP initiation, and timely LTP initiation according to guidelines was 20.8%, 9.9%, 38.5% and 30.9%, respectively. A multivariate analysis showed that two factors were significantly associated with anticipated or timely *versus* delayed or absent LTP initiation: the year of birth (OR 1.2 per each additional year, 95% CI 1.1-1.3, P < .001) and the HTC location (OR 6.4, 95% CI 1.4-29.2, P = .02 and OR 6.2, 95% CI 2.0-19.4, P = .002 for Northwest and Southeast part of France, respectively).

Discussion/Conclusion: This analysis provides pivotal data to evaluate the adherence to early LTP guidelines and to identify barriers for their implementation. CoMETH guidelines dissemination was associated with a dramatic increase in early LTP use. The study highlights the capacity of FranceCoag device to assess critical variables and evaluate therapeutic practice in one of the largest national PUPs cohort worldwide.

Disclosure of interest: None declared.

OR02 | Within-patient comparison of treatment patterns before and after switching to rFVIIIFc: A report from the UK national haemophilia database

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Introduction: Extended half-life (EHL) FVIII products may permit a reduction of infusion frequency (IF) and clotting factor consumption (CFC) whilst improving bleeding outcomes in haemophilia A (HA). Direct comparisons with standard rFVIII do not exist. We compared IF and CFC in patients using FVIII prophylaxis before and after switching to rFVIIIFc using data from the UK National Haemophilia Database.

Methods: rFVIIIFc was introduced in the UK from September 2016 using a restricted NHS England protocol. This assumed effective FVIII prophylaxis could be provided using at least one fewer infusion/wk of rFVIIIFc than previously required using rFVIII. All UK patients who used rFVIIIFc 01/09/16-30/06/2017 were identified. Patients with ≥4-weeks compliant post-switch Haemtrack (HT) home-therapy diary data were analysed. Individual IF and weekly