below the 70th percentile and 1 (poor adherence) if above the 70th percentile. ABR was more likely to be 0 (0 ABR), the true goal of prophylaxis, or 1 or more bleeds (1+ ABR). RESULTS: Reported ABR ranged from 0 to 5 (median 3). There was not a significant relationship between VERITAS-Pro score and ABR. There was a significantly greater percentage of patients experiencing one or more bleeds than each VERITAS-Pro group (6%) than those with the VERITAS-Pro good adherence group (62%). CONCLUSIONS: VERITAS-Pro scores reflecting adherence are significant predictors of ABR; individuals with poor adherence on the VERITAS-Pro score had ABRs of 1+ or more bleeds. However, the high percentage of patients experiencing one or more bleeds in both groups indicates that factors other than adherence may impact annualized bleed rates.

PSY40 INDIRECT COMPARISON OF THE EFFICACY OF RECOMBINANT FACTOR VIIa FUSION PROTEIN AND OTHER FACTOR VIII PRODUCTS FOR PROPHYLAXIS MODELLING THE EFFECT OF COMPLIANCE

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OBJECTIVES: For people with hemophilia A, factor VIII (FVIII) prophylaxis is burdensome, potentially leading to poor compliance. Treatment adherence and outcomes may be improved with drugs requiring less frequent infusions. In the absence of head-to-head direct comparative evidence from clinical trials, this analysis indirectly compared the prophylactic efficacy of recombinant FVIII fusion protein (rFVIIIFc) with the published results of current rFVIII products and unreported standard deviations of ABR estimated using data from clinical trials of previously treated subjects administered rFVIII. METHODS: rFVIIIFc and rFVIII were indirectly compared using data from previously treated subjects in the A-LONG phase 3 study (rFVIIIFc; identified by literature search). Efficacy was compared using reported differences in mean annualized bleed rates (ABRs) for individual and pooled results using meta-analysis with random-effects modeling. Unreported standard deviations of ABR were estimated using a Poisson distribution and adjusted for over-dispersion. A model was developed to assess the effect of compliance changes on ABR. RESULTS: This analysis included published results from the A-LONG study (severe hemophilia; rFVIIIFc; Mahalius 2013). Pooled rFVIII data (Paietta 2010, Derrick 2011, Liu 2009, Manion 2004, Shapiro 2003, and Valentinio 2012; Xynthia®; Recht 2009). Infusion frequency was 1.4-2.4 (median 2.0) times/week for rFVIIIFc and 2.3-4 times/week for rFVIII. Mean ABR for rFVIIIFc was 2.9; the pooled mean ABR estimate for rFVIII was 4.8 (2.4–4.2, λABBR=1.8, P < 0.003). Simulations showed that statistically significant improvements in mean ABR would result from improving compliance with rFVIIIFc by 2.6–12 percentage points. CONCLUSIONS: Results of this unadjusted indirect comparison suggest that routine dosing of rFVIII with rFVIIIFc may result in a lower mean ABR than that of other rFVIII products examined. Moreover, potential improvements in compliance associated with less burdensome dosing regimen as suggested by studies in other chronic diseases, may result in better effectiveness with rFVIIIFc."

PSY31 INDIRECT COMPARISON OF THE EFFICACY OF RECOMBINANT FACTOR IX FUSION PROTEIN AND OTHER FACTOR IX PRODUCTS FOR PROPHYLAXIS: SIMULATING THE EFFECT OF COMPLIANCE ON REAL-WORLD EFFECTIVENESS

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OBJECTIVES: Hemophilia B prophylaxis with factor IX (FIX) requires frequent infusions, potentially leading to poor compliance and reduced therapeutic effectiveness. In the absence of head-to-head direct comparative evidence from clinical trials, this analysis indirectly compared the prophylactic efficacy of recombinant FIX-fusion protein (rFIXFc) and other rFIX products, which require more frequent infusions. Additionally, we simulated the effects of potential differences in real-world compliance between regimens. METHODS: rFIXFc and rFIX were indirectly compared using data from clinical trials of previously treated subjects administered rFIXFc (B-LONG phase 3 study, weekly prophylaxis arm) and rFIX (published clinical studies of routine prophylaxis (rFIX; identified by literature search). Efficacy was compared using reported differences in mean annualized bleed rates (ABRs) for individual and pooled results using meta-analysis with random-effects modeling. Unreported standard deviations of ABR were estimated using a Poisson distribution and adjusted for over-dispersion. A model was developed to simulate the effects of compliance changes on ABR. RESULTS: This analysis included published results from the A-LONG study (severe hemophilia; rFVIIIFc; Mahalius 2013). Pooled rFVIII data (Paietta 2010, Derrick 2011, Liu 2009, Manion 2004, Shapiro 2003, and Valentinio 2012; Xynthia®; Recht 2009). Infusion frequency was 1.4-2.4 (median 2.0) times/week for rFVIIIFc and 2.3-4 times/week for rFVIII. Mean ABR for rFVIIIFc was 2.9; the pooled mean ABR estimate for rFVIII was 4.8 (2.4–4.2, λABBR=1.8, P < 0.003). Simulations showed that statistically significant improvements in mean ABR would result from improving compliance with rFVIIIFc by 2.6–12 percentage points. CONCLUSIONS: Results of this unadjusted indirect comparison suggest that routine dosing of rFVIII with rFVIIIFc may result in a lower mean ABR than that of other rFVIII products examined. Moreover, potential improvements in compliance associated with less burdensome dosing regimen as suggested by studies in other chronic diseases, may result in better effectiveness with rFVIIIFc.

PSY30 SYSTEMIC DISORDERS/CONDITIONS – Patient-Reported Outcomes & Patient Preference Studies

PSY39 ADHESION TO PROPHYLACTIC TREATMENT IN HEMOPHILIA AS MEASURED USING THE VERITAS-PRO AND ANNUAL BLEED RATE (ABR)

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OBJECTIVES: Few studies exist to support the hypothesis that nonadherence to prophylaxis in hemophilia leads to clinically significant outcomes. Aims of this study were to validate a measure of adherence to prophylaxis, allow objective measurement of the relationship between adherence and patient outcomes. The study objective was to compare clinical and annual bleed rates (0 ABR, the true goal of prophylaxis, or 1 or more bleeds) in 1 year of bloods a patient has in a given year. METHODS: The study sample was comprised of patients utilizing prophylaxis treatment who completed the VERITAS-Pro for the validation study published by Duncan et al in Haemophilia in 2007 (n=66). ABR was extrapolated from previous infusion logs and for, those with patients incomplete or missing logs, from electronic medical records. Pearson and Spearman correlation analyses were run between VERITAS-Pro scores and ABR. A cutoff analysis was done with the 70th percentile was chosen as a clinically useful cutoff score and VERITAS-Pro scores dichotomized and coded as 0 (good adherence) if