

Bleeding Disorder Collaborative
March 17, 2016
Meeting Minutes

7:01 a.m. **Bleeding Disorder Collaborative for Care (BDC) Convenes**

Members Present: Rebecca Kruse-Jarres, Michael Birmingham, Dana Matthews, Donna Sullivan, Stephanie Simpson, Amanda Blair, Judy Felgenhauer

Members Absent: Lisa Humphrey, Dan Lessler, Mike Recht, Heidi Forrester

Guests: Ryan Pistoresi

7:03 a.m. **Review and Discuss Draft Research Protocol**

7:03 a.m. Rebecca suggests that meeting will cover Pharmacokinetics (PK) Protocol section of proposal. Rebecca suggests a review of the updated protocol for Dana, who was not present for the last webinar. Rebecca states that primary endpoint should be patients 12 years or older but to include younger than 12 if there are patients available. Rebecca reminds us the decision is to only study ideal body weight and actual body weight and not adjusted body weight for this study. Rebecca mentions that she has found a biostatistician who is able to help but notes they will require a fee for their service.

7:08 a.m. Members begin discussion on PK Protocol. Question raised whether to separate regular half-life product and extended half-life product. If primary endpoint is yield, they can be combined but not for half-life endpoints. Half-life can be a secondary endpoint compared to weight. Rebecca asks Michael if patients may be willing to come in for blood draws at the frequency proposed. Michael answers that it may be difficult, requiring patients to miss work or school. Using local labs may provide convenience for patients, which Michael agrees is more reasonable for patients. Dana raises question of reliability of local labs running tests. Rebecca suggests labs can “spin-down” everything for centers to run tests. Dana agrees. Michael suggests weekends may work better for some patients. Judy mentions her worries about local labs drawing samples due to history of test results reporting incorrect abnormal factor levels due to a variety of factors.

7:16 a.m. Rebecca asks whether it is appropriate to use historical PKs on people with extended half-life products who were on studies. Dana says that the protocol already specifies the requirement of a washout period, which may historical PKs may not satisfy. Dana mentions that criteria can be developed for inclusion of previous PK results for inclusion in this study. Rebecca asks if it is reasonable to use historical fall-off to estimate half-life.

7:23 a.m. Dana suggests determining bare minimum half-life time points. Rebecca agrees that bare minimum would be more reasonable, and that yield must be drawn at the same lab for ideal and actual body weights. Half-life is not reasonable to be done all in the same lab. Members agree that historical fall-off may be acceptable depending on inclusion criteria. Rebecca says 10 time points was suggested for

PK study, but is willing to reduce the number of time points to accommodate patients. Members discuss difficulties of obtaining 8-hour blood draws, and changing time points to time ranges as half-life measurements are secondary end points. Recommended time ranges were: baseline, 30–60 minutes, 5–7 hours, 20–26 hours, 44–50 hours, 69–75 hours, 93–99 hours, and 117–123 hours.

- 7: 33 a.m. Michael asks whether patients will need to end prophylaxis before enrollment in the study. It is expected that they will need to hold prophylaxis treatment to fulfill washout period requirement, but Rebecca mentions it might be not reasonable for people with significant breakthrough bleeding while off prophylaxis. Members discuss risk for these patients. Rebecca suggests a half-life that would not put patients at excessive risk for bleeding would be reasonable. Members suggest that they will develop criteria to determine if local labs are reasonable for inclusion in the study.
- 7:40 a.m. Stephanie mentions that travel for this study may be considered medical assistance and that Bleeding Disorders Foundation of Washington may help assist patients here.
- 7:42 a.m. Rebecca will bring current time ranges to the biostatistician for their input.
- 7:45 a.m. Donna suggests that the protocol be posted online for public comment via email back to Ryan for comments.
- 7:47 a.m. **Bleeding Disorder Collaborative for Care Adjourns**