

Bleeding Disorder Collaborative
March 23, 2016
Meeting Minutes

- 7:08 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, Mike Recht, Dan Lessler, Heidi Forrester
- Members Absent: Lisa Humphrey
- Guests: Ryan Pistoresi, Marla Feinstein (National Hemophilia Foundation)
- 7:09 a.m. **Review and Discuss Medicaid Evidence-based Decisions Project (MED) Reports and Medicaid Clinical Brief**
- 7:10 a.m. Decision made to review only one MED report during this webinar and to review other reports individually offline and provide comments and questions to Ryan. Ryan will provide comments and questions back to MED.
- 7:15 a.m. Members discuss current Phase II clinical trial occurring at University of Pittsburg on dosing patients by different body weight calculations. Rebecca will call investigators at the University of Pittsburg affiliated with this clinical trial for more information. Members express interest in continuing with current proposed research protocol and wish to remain complementary to and not to overlap with the ongoing Phase II clinical trial.
- 7:24 a.m. Rebecca provides information from her discussion with the biostatistician about measurable outcomes for this clinical trial. It was determined that a feasible outcome for this clinical trial to measure is to determine what percentage of patients fall below 66% recovery as a primary outcome. Patients failing to achieve at least 66% recovery will be noted as inadequately dosed. Some members express concern that the number is far too low, but members reply that this is what is consistent and standard with the literature. A secondary outcome would be to measure what percentage of patients fall below other levels. A secondary outcome to measure is to determine what percentage of patients rise above 120% recovery.
- 7:38 a.m. Members clarified that the primary outcome for this trial will be to measure what percentage of hemophilia A patients, age 12 years and older, with a BMI of 25 and over fall below 66% recovery between dosing of the same product by ideal body weight and actual body weight. Any additional analysis will be a sub-analysis (hemophilia B, patients under 12 years of age, patients of BMI of 30 and over, recovery below other percentages) and may not have an adequate sample size to determine statistical significance.
- 7:43 a.m. Public comments for the research protocol to date were reviewed. A comment from Department of Health regarding the lack of calculations and criteria for

determining Body Mass Index (BMI) in the pediatric population was discussed. Mike R will reach out to a Pediatric Nutritionist at Oregon Health & Science University for input on this matter. Rebecca will update research protocol to address changes and discussion from today's webinar.

- 7:48 a.m. Members request more information on the processes for public comments. Members expressed interest in receiving more feedback from stakeholders. As chair, Rebecca can decide when to open discussion to the public for stakeholder engagement. These public comment sections may occur toward the end of webinar sessions due to structure and format of online meetings. Stephanie raises question of whether public comment should be input only or dialogue.
- 7:50 a.m. Marla Feinstein of the National Hemophilia Foundation (NHF) raises a question regarding the initial research documents listing key questions and whether they were still being pursued by the collaborative. Donna replies that they were pursued as literature reviews to see what evidence was available and to inform what clinical research to pursue. Donna continues that other questions not submitted to MED have been altered due to budget constraints. Members expressed interest in new MED research proposal. Members and public will be able to provide feedback on MED proposal to Ryan. Marla mentions that new guidelines from NHF will be posted to guidelines.gov and should be considered by the collaborative.
- 8:01 a.m. **Bleeding Disorder Collaborative for Care Adjourns**