Preliminary Analysis of ATHN 16: Real-World Safety of Eptacog Beta

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Background

Eptacog beta (rFVIIa-jncw) received regulatory approval in the U.S. in 2020 for the treatment of bleeding events (BEs) in persons with hemophilia A/B with inhibitors (PwHABI), 12 years of age and older. The ATHN 16 study was designed by the American Thrombosis and Hemostasis Network (ATHN) and the investigators to collect real-world evidence on the safety of eptacog beta.

Aims

To evaluate the safety of eptacog beta when used to treat BEs in PwHABI with or without prophylactic treatment.

Methods

ATHN 16 (NCT04647227) is a phase IV, multi-center, open-label safety study enrolling PwHABI, who are either on long-term prophylaxis (e.g., emicizumab) or episodic treatment. ATHN 16 received central IRB approval.

After informed consent is obtained, each participant is provided nine (9) 75 µg/kg doses of eptacog beta to be administered as needed for bleed treatment. BEs are treated by the participant or by study staff at the time of an event. The actual dose administered (75 mcg/kg or 225 mcg/kg) is determined at the discretion of the treating physician.

The safety of eptacog beta is evaluated based on events included in the European Haemophilia Safety Surveillance (EUHASS) protocol. There are no pre-specified efficacy endpoints.

Table 1. Type and location of treated bleeds (n = 56)

BLEEDS, TREATED	n=56
Spontaneous	23 (41.07)
Traumatic	25 (44.64)
Activity/exercise, no trauma	7 (12.5)
Other, medical procedure	1 (1.79)
LOCATION, TREATED BEs	**n=56**
Ankle	9 (16.07)
Elbow	17 (30.35)
Knee	15 (26.78)
Shoulder	1 (1.79)
Hip	1 (1.79)
Joint, Finger	3 (5.35)
Muscle	4 (7.14)
Other, soft tissue, toe	1 (1.79)
Other, groin muscle	1 (1.79)
Unknown, back	1 (1.79)
Unknown, extracranial	2 (3.57)
Unknown, intracranial	1 (1.79)

Results

Between August 2021 and April 2023, thirteen participants have been enrolled, and all are on prophylactic emicizumab. The enrolled participants demographics are described below:

- Diagnosis Heme A with inhibitor, N = 100%
- Prescribed emicizumab (prophy) 92% (note as of January 2023 the single participant not on Emi started prophy)
- Sex at birth, male N = 100%
- Birth year, before 1990 N = 38.46%; 1990-1999 N = 15.38%; 2000-2009 N = 46.15%
- Ethnicity, Hispanic, Latino/a, or Spanish origin N = 15.38%; Not Hispanic, Latino/a, or Spanish origin N = 84.62%
- Race, White N = 46.15%, Black/African American N = 30.77%, Asian N = 15.38%, American Indian/Alaska Native N = 7.69%
- Education level, Primary/Secondary N = 53.85%, GED N = 7.69%, Technical school N = 15.38%, Some college N = 7.69%, 2-year college degree N = 15.38 %

Fifty-six bleeding events have been treated (41.07% spontaneous, 44.64% traumatic, 12.5% activity/exercise, and 1.79% medical procedure/surgery) in six of the thirteen (46.15%) enrolled participants. One adverse event (AE), unrelated to study product, was reported and resolved. One serious adverse event (SAE), unrelated to study product was reported and resolved.

Conclusions

To date, the safety profile of eptacog beta, in the ATHN 16 participants treated with eptacog beta, is favorable and consistent with previous reports.

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Figure 1. ATHN 16 Process Flow

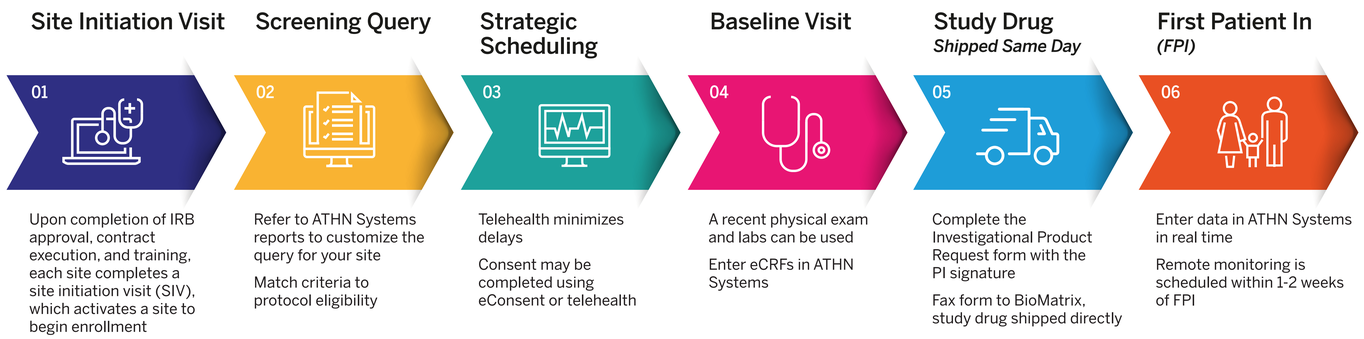

Figure 2. Schedule of Events