

**SUBCUTANEOUS
MARZEPACOG ALFA
(ACTIVATED) IS EFFECTIVE
FOR ON-DEMAND
TREATMENT OF
SPONTANEOUS BLEEDING IN
HEMOPHILIA A RATS**

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TREATMENT OF SPONTANEOUS BLEEDING IN HEMOPHILIA A RATS WITH SQ MARZEPTACOG ALFA (ACTIVATED)



Background and Scientific Approach

Background

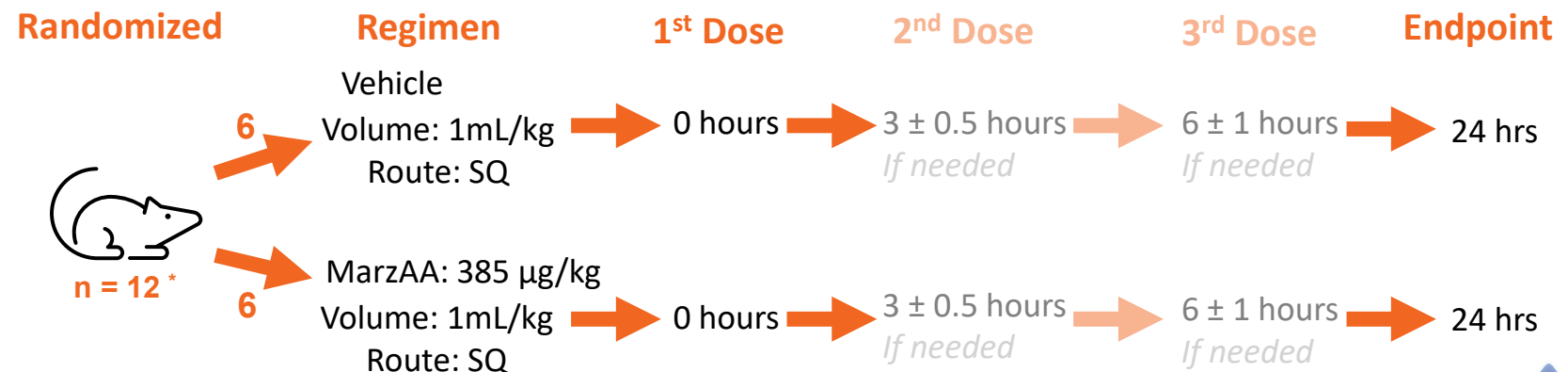
- + Individuals with hemophilia lack subcutaneous (SQ) options to treat acute bleeding
- + Marzeptacog alfa (activated) (MarzAA) is a next-generation activated recombinant coagulation factor VII (rFVIIa), with increased potency and bioavailability enabling SQ administration

Method

- + Hemophilia A (HA) rats have a bleeding phenotype comparable to humans with spontaneous unprovoked bleeding¹
- + Upon detection of spontaneous bleeding, rats were treated with SQ MarzAA (or vehicle control) to replicate the clinical Phase 3 Study (Crimson 1).

Study Protocol

1. Genotype and enroll F8^{-/-} onto protocol
2. Monitor for bleeding 2 x day x 7 days/week
3. Diagnose bleeding based on clinical signs
4. Treat with SQ MarzAA or vehicle (randomized)
1, 2, or 3 doses Q3 hour as needed based on clinical signs
5. Assess outcome at 24 hours
 - + Scored as Poor, Fair, Good, or Excellent
 - + Good or Excellent = Treatment Success
 - + Poor or Fair = Treatment Failure



* If a second bleed occurred in the same animal more than 7 days after initial treatment the animal could be treated again in a cross-over design

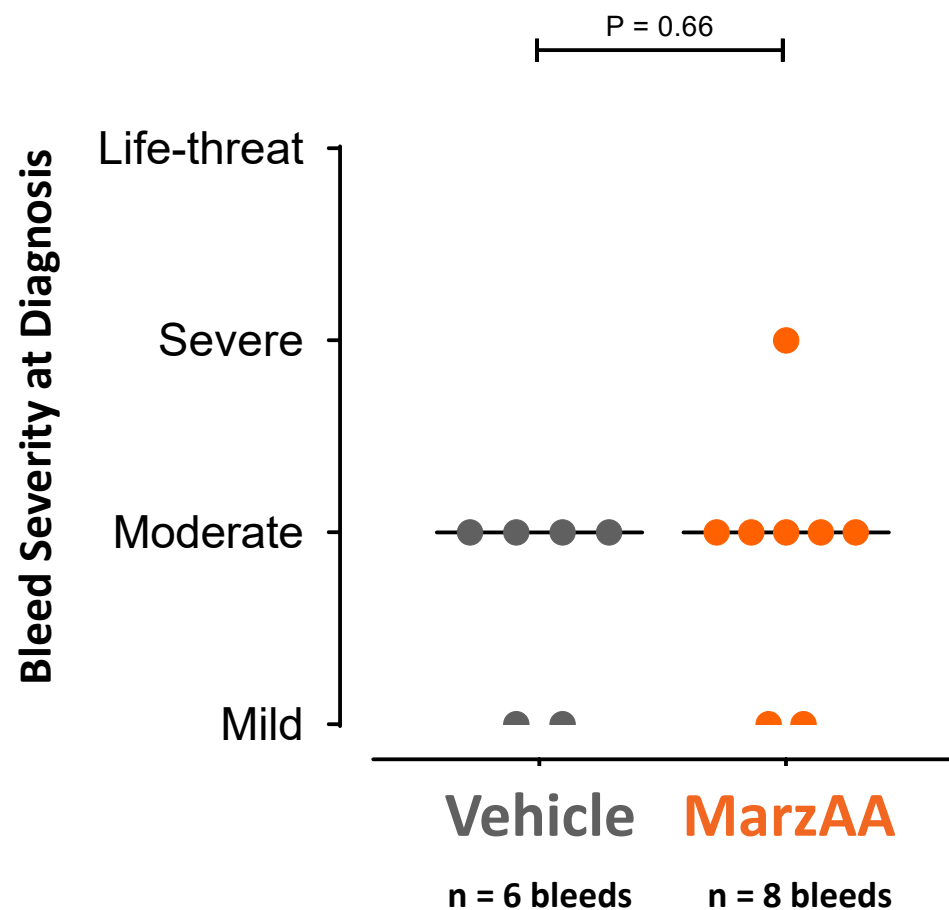


SQ MARZAA CONTROLS SPONTANEOUS BLEEDING IN HA RATS

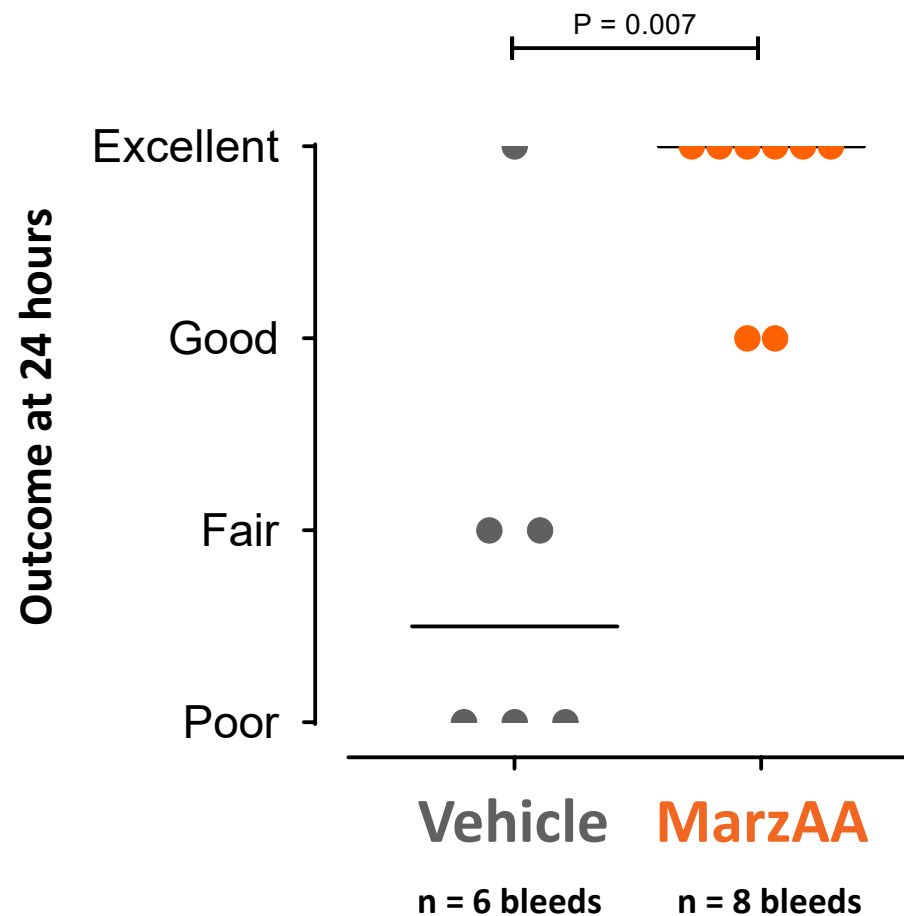


Only Two Rats Required More than One Dose of SQ MarzAA

Bleeding Severity



Clinical Outcome at 24 Hours



SQ MARZAA CONTROLS SPONTANEOUS BLEEDING IN HA RATS



Clinical Confirmation in Progress

Conclusions

- + SQ MarzAA is effective for management of spontaneous bleeding in HA rats
- + Treatment with vehicle led to either lack of improvement or significant deterioration over the 24 hours following dosing in 5 of 6 rats
- + SQ MarzAA was superior to vehicle ($p=0.007$) for treatment of mild, moderate, and severe spontaneous bleeds

Perspectives

- + Because spontaneous bleeding can occur even on prophylaxis, there is a need for a therapy that is easy, safe and fast to administer to manage and control episodic or breakthrough bleeding
- + Clinically exploring MarzAA for on-demand treatment in hemophilia is warranted





DISCLOSURES

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SCAN CODE
TO GO TO
HOMEPAGE
WITH MORE
INFO

