in the tail vein transection model and saphenous vein model. As a result, the sensitivity of these models is as low as a few percent of FVIII, a level relevant to prophylaxis in humans and better suited for testing the new generation of long-acting coagulation molecules (Mei et al. 2010; Pastoft et al. 2012). With these specific efficacy models, the hemophilia mouse models have proven to be a predictive model with high clinical translatability for patients.

5 Conclusion

In vivo target validation with predictive animal models is a crucial part of the drug development process. A successful preclinical target validation study can significantly de-risk a clinical development program. Therefore, the selection of an animal model and tools to be used for in vivo validation must be performed with careful deliberation on the experimental design and a full understanding of the pharmacology of the tool molecules used in the experiments in order to have a model system that closely resembles the human disease condition.

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