SANOFI GENZYME
Rare Blood Disorders
Medical Affairs
Request for Proposals

Date: September 16, 2021	
Disease State: Hemophilia	
Therapeutic Area: Rare Blood Disorders	
Area of Interest: Hemophilia A and B	
Geographic Scope: Global	
Internal Requestor Information:	
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Due Date: October 13, 2021 no later than 5:00 PM ET	
Submission Portal: https://sgrants.envisionpharma.com/vt_sgrants/	
RFP Title: Hemophilia 2021	

BACKGROUND

- Hemophilia is a genetic disease resulting in a deficiency or dysfunction in clotting factor VIII or clotting factor IX leading to insufficient thrombin potential, compromising the formation of a stable clot[1]. The inability of people with hemophilia to form stable clots is associated with prolonged bleeding, characterized by excess bruising and spontaneous bleeding into joints, muscles, internal organs and the brain[2]. Lower levels of thrombin generation correlate with a more severe bleeding phenotype in people with hemophilia[1]. Prophylaxis is the standard of care for people with severe hemophilia, and for some people with moderate hemophilia[3], but questions around treatment modalities remain. Whilst the treatment landscape is evolving and new therapies with improved PK/PD parameters, novel technologies and mechanisms of action are being developed with this goal[4,5] . Today, a "normalized hemostasis and lifestyle is becoming the goal of hemophilia care[6]. Indeed, time at high to normalized factor levels could "normalize" hemostasis and likely lead to absence of bleeding, long-term preservation of joint function and increased capacity for enjoying an active life [6,7].
- It is thus becoming increasingly important for hematologists and other health care professionals caring for patients with hemophilia to deepen their understanding of the intrinsic mechanisms of this disease: primary hemostasis occurs in parallel of secondary hemostasis, in which thrombin plays a central role in the cycle of coagulation factor activation, fibrinogen conversion into fibrin and clot stabilization[8-12].
- Also, the impact of extravascular distribution on hemostasis is an emerging topic in hemophilia B as Factor VIII and factor IX have unique pharmacokinetic profiles and behave differently in the body: Factor VIII primarily remains in the plasma, whereas Factor IX rapidly distributes in the extravascular space, reducing its plasma concentration[13-16]. Studies suggest that in hemophilia B mice, extravascular Factor IX is at least as important for coagulation as is circulating Factor IX[17]. The majority of Factor IX resides extravascularly

where it is important for haemostasis, although the mechanisms at play are not fully elucidated[18].

REQUEST FOR ITP IME GRANT PROPOSALS

SANOFI GENZYME is seeking proposals for an enduring program to close these independently defined healthcare gaps to improve healthcare professional knowledge of differential diagnosis and treatment strategies in Hemophilia A &B. *Proposals can target one or multiple audiences.*

Specifically, Sanofi Genzyme will consider Hemophilia A and B IME programs including, but not limited to, the following:

- Enduring with/without Live Program component
- Accredited or Non-accredited IME activities
- Single supported and multi-supported activities
- Relevant to a Global audience
- Maximum request not to exceed $200,000

Preference will be given to proposals that recommend appropriately designed interventions that are likely to enhance a learner's knowledge of the unmet needs and employ proven strategies to overcome knowledge and performance gaps and barriers.

PROPOSALS

Proposal should include the following information

- Target Audience and Audience Generation: describe methods for reaching the target audience including description of recruitment and placement strategies to maximize participation.
- Learning Objectives and Content Accuracy: Provide clearly defined and measurable learning objectives framed as expected practice improvements in relation to the identified gaps and barriers.
- Include an overview of program content and explanation of criteria that will guide content selection, considering level of evidence and other variables. Sanofi Genzyme is committed to the highest standards in ensuring patient safety; the applicant should describe methods to ensure complete, accurate, evidence-based review of key safety data for any therapeutic entities discussed in the activity. Explain how content will be updated, if necessary, throughout the program period, and how accuracy will be ensured.
- Educational Methods: Sanofi Genzyme supports the ACCME guidance for educational methods to be clearly designed to address the knowledge, competence and/or performance gaps that may underlie an identified healthcare gap. Your proposal should demonstrate an understanding of instructional design as it relates to the gaps in the knowledge, competence, or performance of the targeted audience. Educational methods and design should be based on current literature in CME best practice and consistent with ACCME accreditation criteria, as applicable. Preference will be given to applications that utilize methods that have been shown to result in practice improvements, and/or with data on the effectiveness of other programs of the same type.
- Faculty Recruitment and Development: Provide Information on the expected qualifications of contributors and description of methods to ensure recruitment of course directors and faculty who meet the qualifications. Explain any methods that will be used to ensure that faculty are fully trained in the program expectations and any skills that may be needed to ensure effective delivery of intended education.

- Program Evaluation and Outcomes: Provide a description of the approach to evaluate the reach and quality of program delivery; methods for monitoring individual activities and for ensuring ongoing quality improvements.
- Preference will be given to programs with Objectives and Outcomes Plans with objective measures of changes in knowledge, and/or additional measures of improvements in competence, performance, patient health, population health, and/or system improvements, as aligned with the design of the intervention.
- Budget: Include a detailed budget with rationale and breakdown of costs, per unit, and description of each budget line item. In addition, please include any registrations fees paid by the learner, and how the fees will be applied.
- Accreditation: If proposal involves an accredited program, the accreditation must be provided by an appropriate accrediting body and fully compliant with the accrediting body's criteria and applicable government guidelines and regulations.
- Fair Balance: The proposal should briefly describe methods for ensuring fair and balanced content, identification and resolution of conflict of interest, in alignment with applicable ACCME criteria.
- Communication and Publication Plan: Provide a description of how the provider will keep Sanofi Genzyme informed of progress. If applicable, include description of how the results of this educational intervention will be presented, published or disseminated.

REFERENCES

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