SCIENTIFIC FUNDING APPLICATION

[SMALL GRANT PROGRAM – 2024]

SSC Subcommittee:	Factor XIII and Fibrinogen
Subcommittee Chair:	Dr. Sanj Raut
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RESEARCH PROJECT PLAN

Title of Project / Activity:	Determination of the minimal FXIII activity required to prevent minor and major bleeding
Objectives/Aims:	To explore the minimal factor XIII activity level required to prevent minor and major bleeding in patients with factor XIII deficiency
Target Study Population:	Sixty patients with severe congenital factor XIII deficiency. We will assay of factor XIII levels in 1 h, 24 h, 7, 14, 21, and 28 days after administration of factor XIII concentrate using amine incorporation assay. During this time, we will record the clinical pictures based on the ISTH bleeding assessment tool score (BAT).
Expected Total Duration: **Specify Phases:** **Preparation** **Recruitment** **Study** **Analysis**	Expected Total Duration: 11 months Specify Phases: Preparation: 3 months Recruitment: 3 months Study: 2 months Analysis: 3 months

BENEFIT(S) FOR SSC IF FUNDED:

SSC Specific Benefits	The objectives of the SSC are pertinent to our project, which focuses on examining the determining of minimal factor XIII activity levels required to prevent minor and major bleeding in patients with factor XIII deficiency. The research conducted has the potential to yield useful insights in the field of thrombosis and hemostasis. Specifically, it aims to provide a full understanding of the mechanisms that underlie

	spontaneous bleeding in patients belonging to this bleeding disorders, this is in line with the SSC's aim to increase scientific understanding, standardize diagnostic procedures, and improve patient care. The objective of our research is to enhance the understanding of factor XIII deficiency among the SSC (Scientific and Clinical Community) by presenting data that is based in evidence. This data has the potential to guide clinical practices and treatment approaches, ultimately leading to better results for patients.
Measurable Outcomes	Our project aims to yield a number of measurable results that contribute to a greater understanding of the relationship between factor XIII activity level and minor and major bleeding incidents in patients with factor XIII deficiency. These results include: 1- Correlation Analysis: We anticipate analyzing the correlation between the levels of factor XIII activity and the frequency and severity of bleeding episodes in patients with factor XIII deficiency. We will use appropriate statistical methods to quantify this correlation and obtain correlation coefficients that will provide us with valuable insights into the nature and strength of the relationship. 2-Risk Assessment: We intend to construct a risk assessment model that makes use of factor XIII activity levels as a predictor of minor and major bleeding events. This model will enable stratification of patients according to their risk of hemorrhage, providing clinicians with a tool for early intervention and individualized management strategies. 3- Enhancement of Clinical Guidelines: Based on the collected data, we aim to propose revisions to clinical guidelines for the treatment of factor XIII deficiency. In this context, measurable outcomes include the incorporation of factor XIII activity thresholds and the recommendation of treatment thresholds based on hemorrhage risk. 4- Publication of Findings: We intend to publish our research findings in peer-reviewed journals, contributing to the scientific literature in the field of thrombosis and hemostasis. This outcome will ensure the dissemination of knowledge to the medical community and beyond. By achieving these measurable outcomes, our project aims to advance the understanding of factor XIII deficiency-associated bleeding risks and guide evidence-based clinical decision-making
Expected Results	Our project focuses on determining the relationship between the level of factor XIII activity and the incidence of minor and major hemorrhages in patients with factor XIII deficiency. We anticipate several significant findings from this investigation: 1- Quantification of Correlation: It is expected that our study will demonstrate a measurable association between the levels of factor XIII activity and the incidence, frequency, and intensity of minor and major bleeding episodes in individuals diagnosed with FXIII deficiency. This study aims to present quantitative data that demonstrates the correlation between factor XIII activity levels and the risk of bleeding

	2- Identification of critical Thresholds: Our research aims to identify critical factor XIII activity thresholds associated with increased risk of bleeding. This outcome will enable clinicians to categorize patients into distinct risk groups and tailor treatment strategies accordingly. 3- Clinical Guidelines Enhancement: We expect that our research will contribute to the enhancement of clinical guidelines for the management of factor XIII deficiency. These guidelines may include recommendations for treatment thresholds based on factor XIII activity, thereby enhancing patient outcomes. By achieving these expected results, our project aims to provide clinicians and researchers with valuable insights into the intricate relationship between factor XIII activity and bleeding tendencies in patients with factor XIII deficiency.
Association with other SSC Priorities	Our research effort aims to examine the correlation between factor XIII activity level and the occurrence of minor and spontaneous bleeding in individuals diagnosed with FXIII deficiency. This investigation is in line with the primary objectives outlined by the Scientific and Standardization Committees (SSC) of the International Society on Thrombosis and Haemostasis (ISTH): 1- Standardization of Treatment: By exploring the impact of factor XIII activity levels on bleeding episodes, our study makes a valuable contribution towards the establishment of standardized diagnostic criteria and treatment strategies for individuals with factor XIII deficiency. This is in accordance with the purpose of the SSC, which aims to promote the use of consistent and evidence-based approaches in the diagnosis and management of bleeding diseases 2- Clinical Guidance Development: Our project's findings have the potential to inform the development of clinical guidelines specific to factor XIII deficiency. These guidelines can incorporate recommendations based on factor XIII activity thresholds, enhancing the precision and efficacy of patient care. 3- Risk Stratification: Risk stratification is an important aspect of our research, as it aligns with the objective of the SSC to enhance the development of models that assess the possibility of bleeding diseases based on specific factor XIII activity levels. This phenomenon enhances the precision of prognosis and facilitates the implementation of interventions that are specifically tailored to the individual's needs. 4- Translational Research: Our study serves to provide a connection between laboratory-based research and its practical implementation in clinical settings, therefore demonstrating an essential aspect of translational research. The application of our research findings to clinical practice is in direct accordance with the SSC's emphasis on the translation of scientific discoveries to enhance patient outcomes. 5- Collaboration and Education: Our project encourages collaboration between clinicians, researchers, and

	organizations dedicated to thrombosis and hemostasis. This collaboration fosters the exchange of knowledge and advances the SSC's mission of promoting education and interdisciplinary cooperation. Our project aims to provide valuable insights into the field of thrombosis and hemostasis by addressing the priorities set by the SSC. This research ultimately benefits patients with factor XIII deficiency and aligns with the broader goals of the SSC.

Itemized Budget

(Please provide as much detail as possible. A separate budget justification may also be submitted)

	Description	One Time Expense ✓	Recurring Expense ✓	Anticipated Cost in US$
Project Components	Sampling (tube/syringe etc)			200
Reagents/Laboratory Supplies	Amine incorporation assay kit		4	2400
Administrative Expenses (i.e. Travel, Shipping, Phone/Fax)			60	50
Staffing Support (Research Asst., Administrative Asst.)	One research asst. Two administrative ass. Secretary. Bloodsucker. Statistics personnel.	5000 US$ for one research asst. 8000 US$ for two administrative ass. 1200 US$ for secretary. 1800 US$ for bloodsucker. 1000 US$ for statistics personnel.		17000
Other expenses (categorize)				
Total (Maximum $20,000.00)				$ 19650 US

	Dollars

Additional Considerations Regarding Budget Request:

Submitted by:
E-mail:
Telephone Number:
Business Mailing Address:

To be completed by ISTH Headquarters:

Date Application Received:	
SSC Executive Committee Reviewer Comments:	
Funding Decision:	
Date of PI Notification:	

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