Research Agenda for Hematology

1) **Venous Thromboembolism**
    a. Identifying and evaluating biomarkers and incorporating such biomarkers into clinical risk assessment scoring algorithms for use in high- (patient) and low- (unprovoked) risk populations.
    b. Evaluate the effectiveness of existing antithrombotic agents in preventing VTE in high-risk populations.
    c. Identifying new therapeutic targets for the development of effective antithrombotics with the ultimate goal of discovering agents that do not cause bleeding.

2) **Precision Medicine**
    a. Understanding the interaction between genetic mutations and the epigenome in disease predisposition and response to therapy
    b. Assessing existing pre-clinical models for their ability to predict clinical outcomes, developing new models where needed, and developing evidence-based recommendations for the implementation of pre-clinical models.

3) **Epigenetics**
    a. Targeting/reversing malignant histone modifications.
    b. Development of tools and technologies for locus-specific epigenetic reprogramming e.g., CRISPR/Cas.
    c. Investigate hemoglobin biosynthesis to develop novel approaches to treat sickle cell disease, thalassemia, and other anemias.

4) **Stem Cell and Regenerative Medicine**
    a. Characterizing hematopoietic stem cell biology and improving strategies for HSC therapies.
    b. Develop an artificial and functional hematopoietic stem cell niche that allows for the expansion of repopulating hematopoietic stem cells.
    c. Develop “designer” hematopoietic cell products and facilitate large-scale production for therapeutic and diagnostic use.

5) **Immunologic Treatment for Hematologic Malignancies**
    a. Optimize use of CAR T-cell and checkpoint blockade strategies to cure hematologic malignancies and eradicate minimal residual disease.
    b. Improve efficacy and reduce toxicity for CAR T-cell and cellular therapies.
    c. Improve effectiveness of existing curative therapies, specifically allogeneic HSCT.

6) **Genome Editing and Gene Therapy**
    a. Establish strategies for determining the efficacy, safety, and toxicity of genome editing techniques
    b. Apply genome editing technology to correct HSCs derived from patients with congenital hematologic disease.