Inhibitor formation is a serious complication of hemophilia treatment that occurs in 30% of patients at highest risk – youth with severe hemophilia A. Inhibitors are caused by a T-cell dependent B-cell mediated immune response to exogenous clotting factor, neutralizing it and rendering it ineffective. Patients with an inhibitor require alternative treatments, which can be less effective in managing bleeds and lead to higher costs of care and increased rates of hospitalization and mortality. Further research could inform the development of methods to prevent and more effectively eradicate inhibitors.

To develop a road map for needed research in this area, working groups met in 2019 at a State of the Science Symposium at the National Heart, Lung, and Blood Institute. The meeting focused on developing scientific priorities and implementation strategies for the following types of research studies:

1. **Clinical trials to prevent and eradicate inhibitors**
2. **Specimen collection and observational cohort studies**
3. **Immunology studies of the host immune response and tolerance**
4. **Longitudinal cohort studies of FVIII immunogenicity and predictors of inhibitor risk**

In this webinar, Drs. Pipe and Ragni will discuss the progress and challenges in the design and implementation of these research studies to prevent and eradicate inhibitors.

**LEARNING OBJECTIVES:**

1. List the potential stakeholders to help implement an inhibitor research initiative.
2. Describe some of the challenges in designing inhibitor prevention and eradication trials in a rare population.
3. Explain how a longitudinal birth cohort could be used to study FVIII tolerance.
4. Describe the roles of an observational cohort and a biorepository in promoting inhibitor research.

This webinar is free and open to public health professionals, clinicians, and researchers. Advance registration is required.


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