The landscape of hemophilia care is experiencing significant changes that will impact patient treatment in the coming years. We are entering a period of unprecedented innovation in hemophilia care and treatment, with the licensure of one new non-factor replacement agent (emicizumab), and more to come in the next several years. These new drugs will add to the spectrum of options for managing hemophilia patients; however, they may raise many new questions that only additional research can answer.

In addition, the prospect of curing hemophilia with gene therapy has never looked brighter. Several phase 3 trials are ongoing, making it possible that gene therapy can be offered to patients in the near future.

During this presentation, Dr. Young will review some novel agents, including emicizumab, fitusiran, and concizumab. He will also briefly review the data from published gene therapy trials. He will also discuss some of the unique challenges that new treatments raise for clinicians and provide some potential solutions to those challenges.

**LEARNING OBJECTIVES:**

1. List two new novel therapies that are, and will be, available for the management of hemophilia.
2. State two clinical issues when prescribing emicizumab for patients.
3. Describe one strategy to consider in determining which patients would be best suited for novel therapies.
4. Describe one new avenue for the use of gene therapy in treating hemophilia.