



THE EVOLVING TREATMENT LANDSCAPE OF HEMOPHILIA

REPLACEMENT THERAPY LIMITATIONS

Immunogenicity

Treatment burden and adherence

Maintaining adequate factor levels

NON-FACTOR REPLACEMENT THERAPIES ARE EVOLVING RAPIDLY

FVIII Mimetics

Antithrombin reduction

Anti-TFPIs

Anti-APC



THE POTENTIAL OF GENE THERAPY

It can achieve therapeutic factor levels in a dose dependent fashion, achieve very low annualized bleeds rates, and reduce/eliminate the need for exogenous clotting factor replacement. To date, there has been no associated inhibitors, and no therapy-associated deaths

There are still some remaining questions: can it be administered to those with active HIV, HBV and HCV? Is it safe for children with growing livers? Is there potential for insertional mutagenesis? Can it be re-administration?