

MEETING THE CHALLENGES OF TREATING PATIENTS WITH HEMOPHILIA: KEY MESSAGES

New MOAs may have more **FAVORABLE DOSING** regimens and offer treatment **OPTIONS** for **PATIENTS WITH INHIBITORS**

- Sidestep the limitations associated with factor replacement
- Target intrinsic clotting pathway components to bypass FVIII or FIX deficiencies
- Eliminate the need for venous access
- Achieve factor target levels with less frequent dosing
- Provide a treatment option for patients with inhibitors

FACTOR LEVEL is an important outcome for **gene therapies** and **improved factor replacements** trials

- Goal is to maintain factor trough levels above the 3-5% target

In clinical trials, **HEMOSTATIC REBALANCING TREATMENTS** control bleeding in several patient populations

- Appear to prevent bleeding as prophylactic treatments
- May be more effective at preventing bleeding in patients with inhibitors who are currently treated with bypassing agents

SAFETY of **hemostatic rebalancing agents**

- May require protocols for avoiding thrombotic events
- Some involve novel technologies that have not seen wide clinical application

Gene therapy SAFETY AND DURABILITY is still being studied

- Long-term safety and efficacy of gene therapies is still not known
- Trials have excluded previously untreated patients, pediatric patients, and patients with inhibitors

Emerging therapies may have **APPLICATIONS IN MANY PATIENT POPULATIONS**

- Clinical trials are testing emerging therapies as stand-alone prophylactic treatments that may reduce the burden of treatment, as well as treatment options for patients with inhibitors