

MASAC RECOMMENDATION REGARDING THE NEED FOR BLEEDING DISORDERS GENE THERAPY LEXICON DEVELOPMENT

The following recommendation was approved by the Medical and Scientific Advisory Council (MASAC) on March 8, 2024, and endorsed by the NBDF Board of Directors on April 11, 2024.

The landscape for hemophilia treatment options has greatly evolved in the last few years. Most recently is the addition of gene therapy for the treatment of bleeding disorders. {Gene therapy adds a new gene or replaces or repairs a mutated gene in a cell to prevent or treat a disease} A bleeding disorder gene therapy lexicon is currently being developed by various industry stakeholders, and the need for an objective, unbiased lexicon is vital for dissemination of the complexities of gene therapy to patients, caregivers, and healthcare providers.

MASAC recognizes the need for a harmonized terminology for healthcare providers, patients, and caregivers to use in discussions surrounding gene therapy.

MASAC recommends the development of a bleeding disorder gene therapy lexicon to serve as the agreed upon terminology. MASAC recommends that the lexicon be developed by a collaborative effort among professional organizations and representatives from the bleeding disorder community (i.e. Lived Experience Experts – [LEEs]). MASAC recommends/encourages multiple professional organizations to contribute to this effort. To that end, MASAC is willing and available to collaborate and serve in the lexicon development process.

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