

CSL Behring – Global Program Call For Grants

Therapeutic Area: Coagulation

Disease State: Hemophilia B

Call for Grants Application Details:

- Your title must begin with ID Information **“HEMB2021”**
- Refer to Grant Submission Instructions for further information on submitting your formal grant application at CSLBehring.com/grants under Independent Medical Education.
- Additional communication on the process will be conducted exclusively through Educational.Grants@cslbehring.com or the portal grant record.

Submission Timeframe:	Oct-Nov 2021 6 weeks
Proposal:	US continuing medical education programs. Multi-support encouraged
Program Format:	Interactive US live/web programs broken up into chapter format, potential panel discussion and audience involvement with enduring materials
Program Cost:	up to \$250,000.00

CSL Behring is interested in providing grant support to a reputable and certified Continuing Medical Education (CME) provider to provide healthcare providers (HCPs) an educational, non-promotional opportunity to learn more about the role of gene therapy in Hemophilia B, how gene therapy could address the unmet needs of patients and review the clinical data of emerging therapies of gene therapy in Hemophilia B.

Needs Assessment: Role of gene therapy in Hemophilia B

Despite recent interventions that have improved the standard of care in hemophilia B, there remains a significant unmet need to address the challenges and clinical implications associated with long-term management of hemophilia B.

Hemophilia B is a rare, hereditary, X-linked recessive bleeding disorder resulting from the absence or deficiency of coagulation factor IX (FIX).¹ Approximately 70–80% of all bleeding episodes in hemophilia occur in the joints, with the ankles, knees and elbows more commonly affected.² Joint damage can occur despite prophylaxis, indicating that in some cases prophylaxis is failing to control clinical and subclinical bleeding. The CHESS II (Cost of Hemophilia in Europe: A Socioeconomic Survey II) study found that 69.9% of people with severe hemophilia B had an ABR ≥ 2 despite receiving FIX prophylaxis.³ High trough levels are important to prevent bleeds and protect joints. It has been shown that increased time spent below 5% FIX activity level correlated with an increased bleeding tendency. High factor trough levels of

between 5% and 15% improve outcomes in people with hemophilia, but higher levels may be required to eliminate all bleeds in hemophilia B.⁴⁻⁶

The key goal of gene therapy for Hemophilia B is to provide long-term benefit with sustained activity from a single administration, eliminating the need for prophylaxis and increasing the quality of life in people with Hemophilia B. Ongoing research of gene therapy treatment for Hemophilia B include AAV vector-based vehicles for gene transfer. The continued growth and success of the AAV gene therapy may replace factor replacement therapy as a transformative therapy for hemophilia.⁷

A review of the clinicaltrials.gov website in August 2020 documents 5 hemophilia B trials that are either still recruiting or remain active but no longer recruiting. Two products (AMT-061 and SPK-9001) sponsored by CSL Behring/Uniqure and Pfizer/Spark respectively are currently in Phase 3 development. Three products (FLT180a, SB-FIX, YUVA-GT-F901) sponsored by Freeline, Sangamo and Shenzhen Geno-Immune Medical Institute are currently in Phase 1/2 development.⁸

As new therapies become available and standard of care and guidelines are updated periodically, there is a need for continuing medical education for health care providers to maintain, develop, or increase their knowledge, skills, and professional performance and relationships to provide services for patients, the public, or the profession.

References:

1. Liras A, et al. Orphanet J Rare Dis 2012; 7:97.
2. Santagostino E, et al. Comprehensive Care of Hemophilia. In: WFH Guidelines for the Management of Hemophilia. 3rd ed. Haemophilia 2020; 26(Suppl 6):19–34.
3. Burke T, et al. Haemophilia 2021; 27(Suppl 2):115.
4. Shapiro A, et al. Blood 2013; 122(21):2349.
5. den Uijl IEM, et al. Haemophilia 2011; 17(1):41–44.
6. Soucie JM, et al. Blood Adv 2018; 2(16):2136–2144.
7. Pipe SW, et al. Oral presentation at 62nd ASH meeting, December 2020.
8. Batty P, et al. HemaSphere 2021; 5(3):e540.

Program Requirements:

The Program must be accredited and fully compliant with the ACCME standards for commercial support.

CSL Behring's grant in support of the Program is not subject to any condition or restriction regarding the content or execution of the Program or the selection of Program presenters or faculty members. The grant recipient will be solely responsible for the selection of the Program venue, faculty and/or educational methods, and for the quality and scientific integrity of the Program. CSL Behring will not influence the grant recipient's exercise of these responsibilities, even if asked by the recipient to do so.

The grant recipient must ensure that: (i) the Program is free of commercial bias; (ii) the Program presents objective information about any product(s) based on scientific methods generally accepted in the medical community; (iii) if CSL Behring products, or other products used to treat or being investigated to treat the same indications, are featured in the Program,

featured data is objectively selected and presented, with both favorable and unfavorable information in respect of the products fairly represented, and that there is a balanced presentation and, if applicable, interactive discussion of the prevailing body of scientific information in respect of the products and alternative treatment options; (iv) there is meaningful disclosure during the Program of any limitations on information presented in the Program; and (v) if the Program addresses unapproved (unlabeled) uses of any product, or an investigational use not yet approved for any purpose, the Program includes disclosure that the product is not approved in the United States for the use under discussion or, as may be applicable, that the product is still under investigation in respect of such unapproved use.

The grant recipient also must ensure meaningful disclosure in Program announcements and materials, and to the audience during the Program, that (i) CSL Behring is funding the Program, and (ii) a relationship exists between the grant recipient and CSL Behring and, if applicable, between the Program presenters or faculty and CSL Behring.

Additional requirements will be included in the Grant Agreement between CSL Behring and the grant recipient to be executed following award of the grant.