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The Positive Impact of CME on Healthcare Providers' Knowledge of Gene Therapy Studies in Hemophilia

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Abstract

OBJECTIVE: Gene therapy has the potential to be a dramatic paradigm shift in the care of patients with hemophilia.¹⁻⁴ To educate and prepare clinicians for this potential paradigm shift, the National Hemophilia Foundation (NHF), European Haemophilia Consortium (EHC), the World Federation of Hemophilia (WFH), and Medscape Education established a multinational collaboration to develop an online continuing medical education (CME) curriculum. The current study assessed the ability of online CME to improve healthcare providers' (HCPs) knowledge regarding how gene therapy is evolving in hemophilia. **METHODS:** A 30-minute, CME-certified, panel discussion activity was developed and launched online on 6/18/2018. Educational effectiveness was assessed with a repeated-pairs pre-/post-assessment study design, with each individual serving as his/her own control. Responses to 3 multiple-choice, knowledge questions and 1 self-efficacy confidence question were analyzed. A chi-squared test assessed changes pre- to post-assessment. P values <0.05 are statistically significant. Effect sizes were evaluated using Cramer's V (<0.05 modest; 0.06-0.15 noticeable effect; 0.16-0.26 considerable effect; >0.26 extensive effect). **SUMMARY:** To date, 3,018 HCPs (2,376 physicians) have participated in this education. This analysis comprises data from the subset of hematologists/oncologists (n=66; hem/oncs) who answered all pre-/post-assessment questions during 6/18/18-6/12/19. Significant improvements were observed overall (P<.0011; V=.164) and with respect to: Correctly identifying the nonenveloped parvovirus vector construct (adeno-associated virus; AAV) that is currently being studied in gene therapy trials (61% vs 82% [35% relative increase]; P=.0071; V=.234) Recognizing that the University College London/St Jude trial provided the first evidence that therapeutic levels of FIX could be expressed and sustained for several years using an AAV-based system (53% vs 71% [34% relative increase]; P=.03; V=.187) 21% of hem/oncs had increased confidence with regard to how gene therapy could be used to treat hemophilia A. The findings also uncovered educational needs, such as the need for additional education regarding the FVIII expression levels that have been observed within AAV gene therapy trials for hemophilia A. **CONCLUSIONS:** Participation in this online educational activity significantly improved hem/oncs' knowledge with regard to the viral vectors that are currently being studied in hemophilia trials as well as the extent and duration of factor expression that have been observed to date