



The 65th ASH Annual Meeting Abstracts

ORAL ABSTRACTS

901.HEALTH SERVICES AND QUALITY IMPROVEMENT - NON-MALIGNANT CONDITIONS

Assessing Psychosocial Readiness for Gene Therapy in Sickle Cell Disease: A Consensus Statement

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Introduction: Scientific evidence regarding the efficacy and safety of gene therapy for sickle cell disease (SCD) is growing and clinical trials are increasingly available to patients. As a result, clinical trial teams are now challenged with considering the ways in which psychosocial factors may affect patient decisions about pursuing gene therapy and how pre-gene therapy psychosocial functioning may influence long-term patient outcomes. Although data specific to gene therapy for SCD are currently limited, research in other chronic illness populations undergoing major medical procedures (e.g., solid organ and stem cell transplants) suggest that premorbid psychosocial functioning affects decision-making and post-procedure outcomes. Clinical observations support the relevance of considering psychosocial readiness for curative therapies in patients with SCD but there is a lack of guidance regarding how to carry out such evaluations. Therefore, the NHLBI's Cure Sickle Cell Initiative (CureSCi) formed the Patient Readiness and Resilience Working Group to develop consensus recommendations regarding the assessment of psychosocial readiness in the context of gene therapy for SCD.

Methods: The NHLBI CureSCi Patient Readiness and Resilience Working Group was formed in September 2020 and consisted of 10 members including mental health clinicians, researchers, and patients and caregivers. The working group met from 2020-2022 to develop best practice recommendations for the assessment of patient readiness and resilience prior to gene therapy for SCD. Recommendations were developed based on available scientific evidence, expert clinical guidance, review of strategic planning documents, and consultation with CureSCi subcommittees and working groups representative of patient, advocate, clinician, and researcher perspectives. A draft of the consensus recommendations was released for public comment in 2022 and final revisions were completed in early 2023.

Results: The CureSCi Patient Readiness and Resilience Working Group reached consensus regarding recommended practices for conducting pre-gene therapy assessments of patient readiness in SCD. The working group developed a recommended workflow for assessing patient readiness, including an institutional self-assessment of readiness to support patients' mental health and address psychosocial barriers to care, brief screening of cognitive functioning and health literacy, patient education about clinical trials and gene therapy, assessment of patient readiness and resilience followed by interventions to address areas of concern, and informed consent. Detailed guidance was developed across four primary domains: 1) Effective education delivery; 2) Evaluation of knowledge and understanding; 3) Evaluation of interest and motivation; and 4) Evaluation of psychosocial risk and resilience factors. The working group created a guide to support implementation of pre-gene therapy

assessments, which outlines evidence-based strategies for effective patient education, recommended assessment tools, and sample clinical interview questions.

Conclusions: As gene therapy for SCD becomes increasingly available to patients, there is a growing need to be prepared to consider readiness and resilience factors that could affect patient well-being before, during, and after treatment. This CureSCi working group developed a set of consensus recommendations regarding the assessment of psychosocial factors in the context of gene therapy for SCD. A thorough guide was developed to aid in implementation of pre-gene therapy assessments across a range of institutional settings. The presentation will review consensus recommendations, describe practical strategies for conducting assessments, and discuss areas for future research.

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