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508.BONE MARROW FAILURE: ACQUIRED

Quality of Life Analysis in Patients with AA Using the QLQ-AA/PNH-54 at Initial Diagnosis of AA - a Registry Based Analysis

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Objectives: Aplastic anemia (AA) and paroxysmal nocturnal hemoglobinuria (PNH) are ultra-rare, acquired bone marrow failure syndromes (BMFS) with need for long-term care and high morbidity despite recent therapeutic advances. There is a lack of disease-specific assessment tools of quality of life (QoL) in AA/PNH patients. So far, QoL has mostly been estimated according to surrogate parameters (e.g. blood counts, transfusion dependence or toxicity of treatment in AA patients) or has been assessed with tools specifically designed for cancer patients (e.g. EORTC Quality of Life Questionnaire QLQ-C30) or with tools focusing on fatigue (e.g. Functional Assessment of Chronic Illness Therapy, FACIT-Fatigue, PNH patients). Therefore, the QLQ-AA/PNH-54 has been developed to allow a patient tailored QoL assessment in patients with AA or PNH. Here, we present the first descriptive data of the QLQ-AA/PNH-54 of 74 AA patients at the timepoint of initial diagnosis collected between 2018 and 2020 (prior to the SARS-CoV-2 pandemic).

Methods: The QLQ-AA/PNH-54 was designed, psychometrically validated, and is currently finally validated for PNH within the IPIG (international PNH interest group)-registry. Parallel to this process, the tool was implemented within the Aachen AA/BMF registry as an instrument for real-life QoL evaluation in AA patients. In total, 54 QoL items regarding physical functioning (21 items), psychological aspects (26 items) and health care related issues (7 items) are comprised. Patients rank the occurrence of each item within the last 14 days on a scale between 0 and 3 (0 = did not occur, 1 = low, 2 = moderate, 3 = severe).

Results: 74 AA patients (37 female/ 37 male, mean/median/range age 38.42/ 35.47/ 11-73 years) were included. Patients were diagnosed with nsAA (n=11), sAA (n=23), vsAA (n=18), AA NOS (n=20) and AA/PNH-Overlap (n=2), respectively. Broken down into different functional scales, the most frequently and highest rated scales concerned psychosocial aspects (> 60 % of the patients scored 1-3; > 30% of the patients scored 3). Patients were especially burdened by worries and uncertainties with regards to their future, activity planning security and need for cautiousness in everyday life. Tiredness was the major symptom reported in relation to physical functioning. Patients often feared deterioration of their blood results as the most important health care-related issue. In comparison, low occurrence of pain, orthostatic dysregulation, susceptibility for infections, dyspnea, sleep disturbance and tendency to bleed was reported regarding physical functioning (scored 0 by over 40 to over 50 % of the patients). Similarly, low scores were reported for impairment of activities of daily life in terms of completion of household tasks and hobbies or loss of physical attraction.

Conclusion: QoL of AA patients at initial diagnosis was mainly impaired by tiredness and a high psychological burden. Interestingly, complications frequently feared and highlighted by treating physicians had a minor impact on patients' QoL. These results emphasize the importance of further QoL analysis from patient view and recommend to especially focus on the psychological burden of AA patients in patient communication at initial diagnosis.

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