Abstract:
For the past two decades, hematopoietic cell transplantation (HCT) has been used as effective therapy for selected inherited metabolic diseases (IMD) including Hurler (MPS IH) and Maroteaux-Lamy (MPS VI) syndromes, childhood-onset cerebral X-linked adrenoleukodystrophy (X-ALD), globoid-cell leukodystrophy (GLD), metachromatic leukodystrophy (MLD), alpha-mannosidosis, osteopetrosis, and others. Careful pre-HCT evaluation is critical and coordinated, multidisciplinary follow-up is essential in this field of transplantation. The primary goals of HCT for these disorders have been to promote long-term survival with donor-derived engraftment and to optimize the quality of life. Guidelines for HCT and monitoring are provided; a brief overview of long-term results is also presented.