ELSEVIER

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722.ALLOGENEIC TRANSPLANTATION: ACUTE AND CHRONIC GVHD, IMMUNE RECONSTITUTION

Trial in Progress: Patient-Reported Outcome Measures in the Prospective Observational Cohort Study of Patients at Risk for Chronic Graft-Versus-Host Disease in the United States (THRIVE)

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Background and Significance: Allogeneic hematopoietic cell transplantation (alloHCT) is a curative immunotherapy for patients with hematologic disorders. However, graft-versus-host-disease (GVHD) is a major limitation to the success of alloHCT, with chronic GVHD (cGVHD) as the leading cause of morbidity and mortality. The pleomorphic nature of cGVHD makes the diagnosis and patient management challenging for clinicians; thus, patient-reported symptoms often guide in diagnosing and determining the severity of cGVHD. Our current understanding of cGVHD is largely based on data from registries, single-institution prospective studies, and the cGVHD Consortium with representation from tertiary care academic centers. *Prospective studies including patient-reported outcomes and a focus on a more representative and generalizable sample of patients with cGVHD are needed to better understand cGVHD symptoms, disease burden, and practice patterns for management.* This trial in progress focuses on broad patient-centered assessment of post-alloHCT medical and psychosocial complications and patient-reported caregiver involvement in a diverse and contemporary patient population. This study uses multiple organ- or system-directed patient-reported outcome measures (PROMs) to help identify cGVHD symptoms, severity, and impact on patient quality of life including financial impact. The primary objective of THRIVE is to assess the clinical course and burden of cGVHD, including diagnosis and severity recorded in electronic case report forms (eCRFs) and PROMs. Secondary objectives include measuring healthcare resource utilization and patient economic and employment impact associated with cGVHD management, as well as patient-reported organ-specific symptoms.
Study Design and Methods: THRIVE is a prospective, longitudinal, observational study (NCT05919511) of alloHCT recipients in the United States that uses a hybrid model designed to work with HCT centers and a virtual platform. Eligible patients are ≥18 years old, received alloHCT 90-180 days before enrollment, and are able to complete patient-assessment questionnaires provided in their preferred language either alone or with minimal caregiver assistance. Up to 1500 alloHCT recipients will be recruited at HCT sites (with the expectation that ~500 will have cGVHD symptoms at enrollment; ~500 are estimated to develop cGVHD during the course of the study; ~500 are estimated not to develop cGVHD during the study; Figure). Patients have the option to participate on a virtual platform. Patients will be followed for 36 months from date of enrollment or until the earliest of study termination, withdrawal, or death. Data from 13 PROMs (Table) divided into 3 groups will be collected every 3 months using a decentralized virtual platform. To reduce survey burden, patients will be randomized into 1 of 6 sequence groups to capture 4-5 PROMs per month in year 1 and every 2 months in years 2 and 3. Clinical data, including medications and adverse events, will be collected and abstracted into eCRFs every 3 months. In general, reporting of results will be descriptive for variables of interest. Summary statistics for continuous variables and for categorical variables will be calculated for all patients as well as for subgroups of interest. Whenever applicable, 95% confidence intervals will be calculated for endpoint estimates. Univariate, multivariate, or repeated-measurement analyses will be performed to assess factors associated with evolution of clinical manifestations of and complications from cGVHD. Recruitment for this study is ongoing.

Disclosures Ponce: *Ceramedix:* Membership on an entity's Board of Directors or advisory committees; *Evive Biotechnology:* Membership on an entity's Board of Directors or advisory committees; *Incyte Corporation:* Membership on an entity's Board of Directors or advisory committees, Research Funding; *Kadmon/Sanofi Pharmaceuticals:* Membership on an entity's Board

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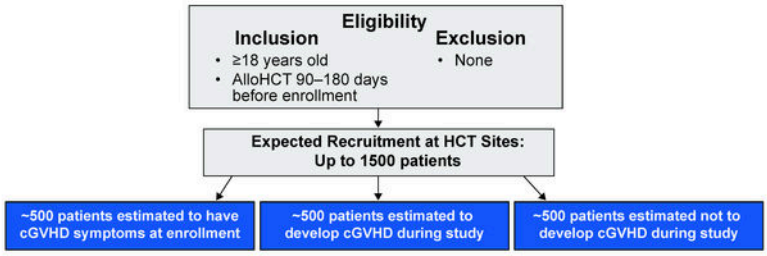
Figure. THRIVE Eligibility and Expected Enrollment



alloHCT, allogeneic hematopoietic cell transplantation; cGVHD, chronic graft-versus-host-disease; HCT, hematopoietic cell transplantation.

Table. Patient-Reported Outcome Measures Used in the THRIVE Study

Patient-Reported Outcome Measure	Information Captured
Lee Symptom Scale (LSS)	• Developed to capture the cGVHD-specific symptom burden; comprises a 30-item self-report questionnaire with 7-day recall • 8 subscale scores (energy, skin, nutrition, lung, psychological, muscle/joint, eye, and mouth) can be calculated, with an overall summary score where higher scores indicate greater symptom burden • The LSS is the most validated patient-reported measure for cGVHD symptoms
Oral Health Impact Profile (OHIP-14)	• Comprehensively assesses self-reported oral dysfunction, discomfort, and disability related to oral health conditions • Each item is scored and summed to give a total score alongside 7 subscale scores (functional limitation, physical pain, psychological discomfort, physical disability, psychological disability, and social disability) with higher scores indicating worse discomfort and disability
Ocular Surface Disease Index (OSDI-12)	• Created to assess ocular surface symptoms related to chronic dry eye disease • Questionnaire includes 12 items covering symptoms, effect on function, and discomfort scores on a scale of 0–100, with higher scores representing greater symptom severity and disability
Cutaneous GVHD Questionnaire (CuGQ)	• Designed to allow for patients to describe symptoms that follow diagnostic criteria/scoring of cutaneous GVHD • Involves reporting the extent of disease (eg, body surface area), characterization of lesions (eg, dryness, erythema, swelling, ulceration/scab, and thickening), symptoms (eg, itchiness and pain), and impact on daily life (eg, sleeping, activities of daily living, and social interactions)
Modified Patient-Generated Subjective Global Assessment (mPG-SGA)	• Used to describe nutritional status of patients after alloHCT and those with cGVHD • Provides a total score based on patient-reported weight history, dietary intake, nutritional impact of symptoms, functioning, disease state, physical evaluation, and metabolic and physical demands
Modified Frenchay Activity Index (mFAI)	• A measure of activities of daily living • Provides a broad measurement of actual activities of daily life, including home and return-to-work activities, that patients have undertaken over the last 3–6 months
Patient-Reported Outcomes Measurement Information System–Sexual Function (PROMIS-SF)	• Set of person-centered measures housed under the Healthmeasures.net platform • Measures both men/women genital symptoms and impact on sexual health
Pulmonary GVHD Questionnaire (PulmGQ)	• Measures pulmonary symptoms associated with pulmonary GVHD (NIH symptom score), which has been shown to be highly correlated with pulmonary cGVHD • Assesses impact on daily activities with functional and mental components
Patient Health Questionnaire–9 (PHQ-9)	• Self-administered depression screening tool that assesses both somatic symptoms (insomnia, loss of energy, and appetite problems) and affective-cognitive symptoms (feeling depressed, self-blame, and suicidal ideation) of depression • Helps distinguish treatment-related somatic symptoms from depression-specific symptoms
General Anxiety Disorder (GAD-7)	• A 7-item anxiety scale, with increasing scores on the scale strongly associated with multiple domains of functional impairment
Patient-Reported Caregiver Assessment (PRCA)	• Designed to assess the involvement of caregiver support and level of care they provide as reported by the patient
Patient-Reported Baseline Data (PRBD)	• Captures patient-reported demographic information (eg, age, sex, race, work, income, home address) and posttransplant clinical history of aGVHD, hospitalizations, and frequency of alloHCT clinic follow-ups
Patient-Reported Economic, Income, and Insurance Data (PREIID)	• Captures patient-reported employment status and the impact alloHCT had on employment, including disability claims • Includes questions about insurance type, insurance change, and satisfaction of insurance since alloHCT

aGVHD, acute graft-versus-host disease; alloHCT, allogeneic hematopoietic cell transplantation; cGVHD, chronic graft-versus-host disease; GVHD, graft-versus-host disease; NIH, National Institutes of Health.

Figure 1

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