

# Payer-Reported Unmet Need Burden Post Allogeneic Hematopoietic Cell Transplantation (HCT) Due to Poor Graft Function (PGF) and Characteristics of an Ideal Treatment for PGF

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## INTRODUCTION

This research leverages a unique survey approach through the PayerPro360 platform to capture critical payer insights on genetic, regenerative, and curative therapies. Focused on understanding unmet needs and current treatment paradigms for the rare indication of poor graft function (PGF), the study aims to analyze the unmet need burden and define the characteristics of an ideal treatment for PGF. Payer research is instrumental in supporting clinical development, commercialization, and product launch by reflecting real-world decision-making processes and payer perspectives. PayerPro360 addresses the common challenges in payer research- ensuring efficient, real-time engagement with high data accuracy and cost efficiency- ultimately providing a simplified, flexible solution to access actionable insights.

## OBJECTIVE

Poor graft function (PGF) that may occur after allogeneic hematopoietic cell transplantation (HCT) does not respond well to current therapies. This research elucidates the areas of unmet need in managing PGF and the expected characteristics of an ideal treatment for PGF

## METHOD

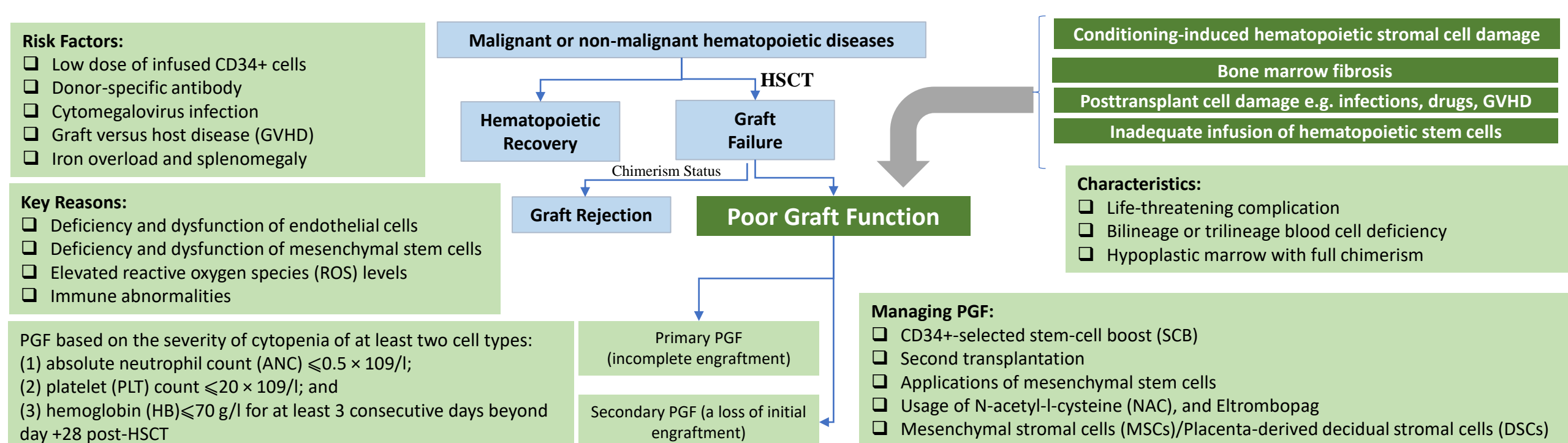
An online survey was conducted through eQuantX's PayerPro360 platform, engaging 10+ private and institutional payers in the United States in 2022, with expertise in pricing, reimbursement, patient access, policy, and advocacy programs. The payer profiles included key institutions such as Boston Medical Center (BMC), New York Blood Center, National Comprehensive Care Network (NCCN), and the Center for International Blood & Marrow Transplant Research, alongside government payers like the U.S. Department of Health and Human Services (HHS) and Centers for Medicare and Medicaid Services (CMS). Major private payers were also represented, including Cigna, Anthem, UnitedHealth Group, BlueCross BlueShield, Humana, and Aetna.

Utilizing PayerPro360's interactive features, real-time insights were gathered over a week, allowing the research team to pinpoint unmet needs in HCT patients with poor graft function and identify the most widely used treatments within this patient population. PayerPro360's agile platform facilitated follow-up inquiries for in-depth clarification, highlighting the critical challenges these payers encounter in managing HCT patients with poor graft function.

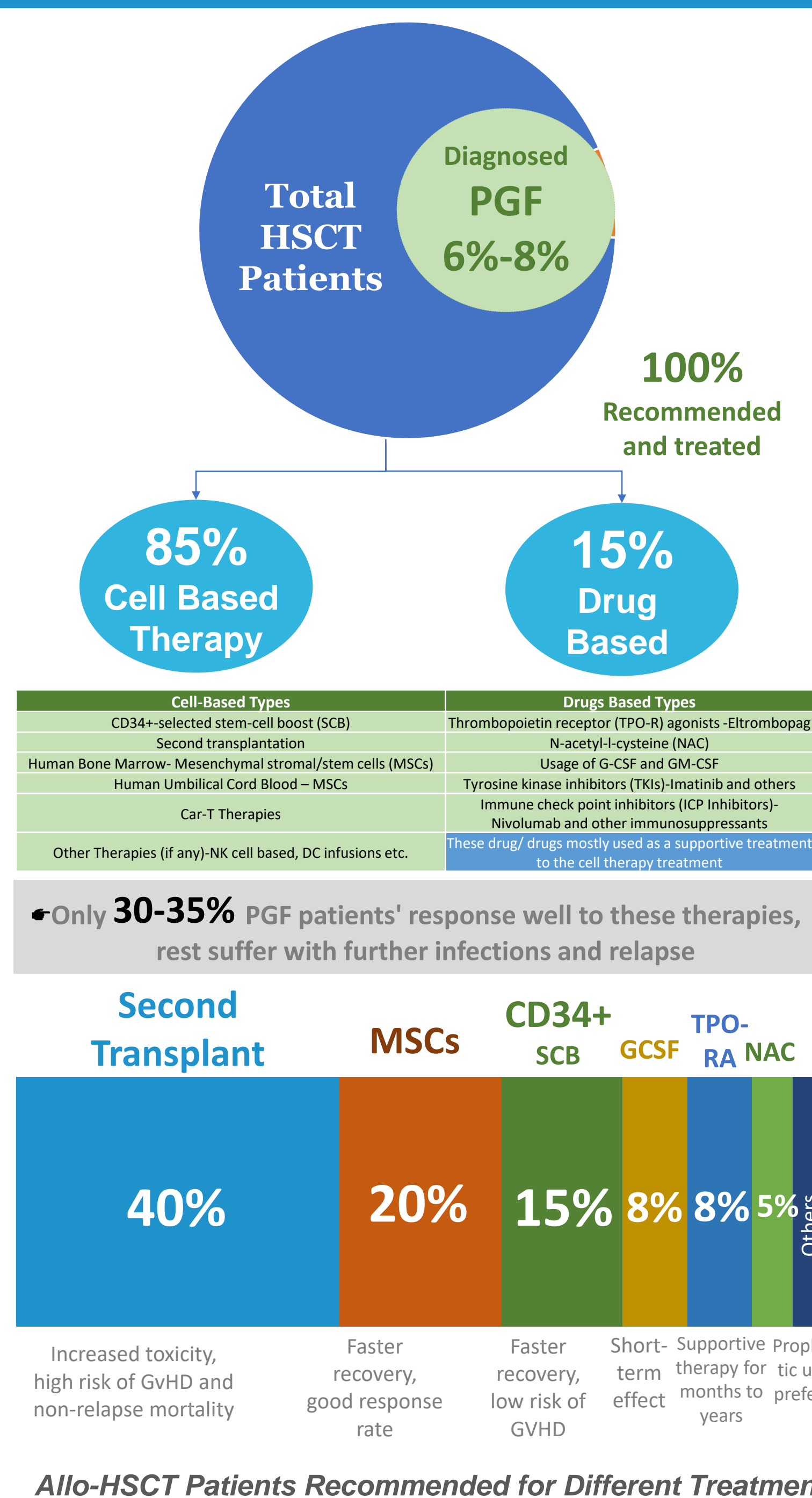
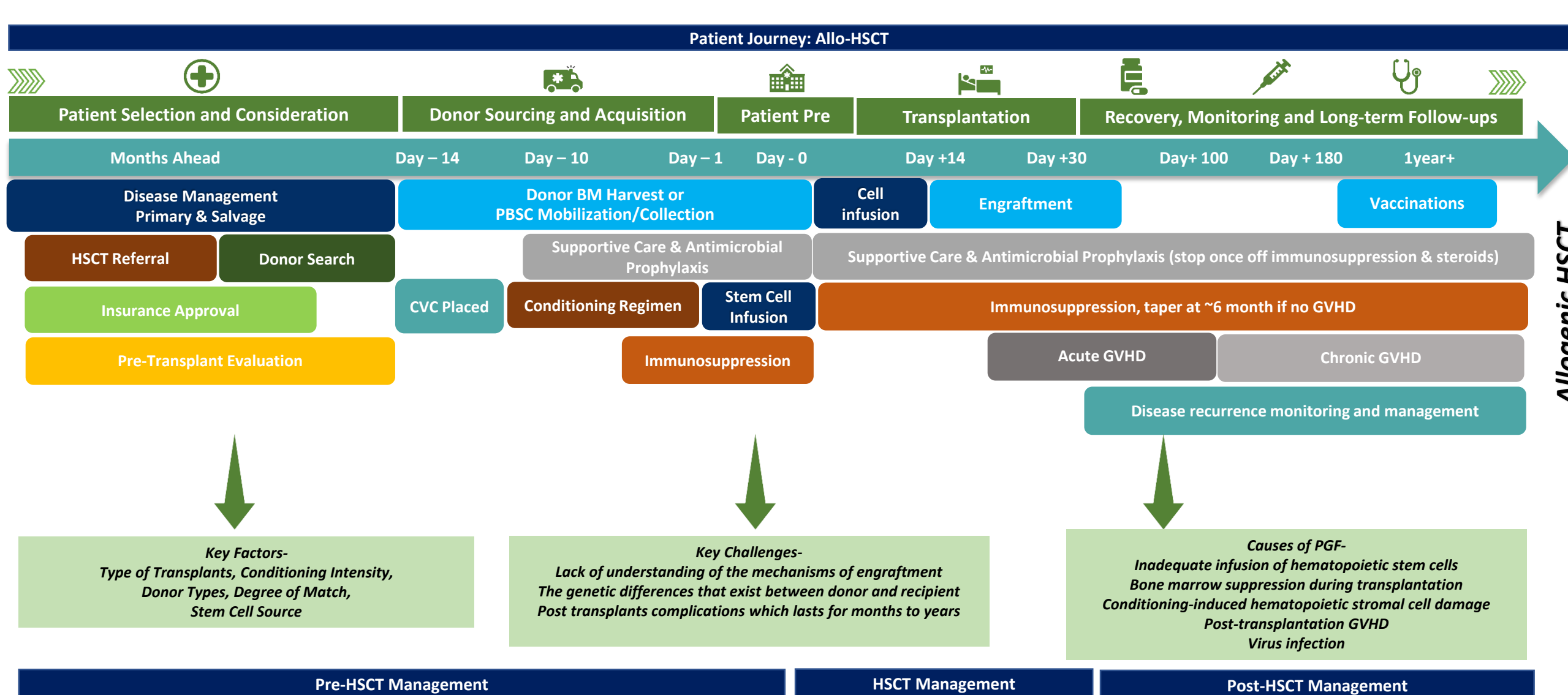


## RESULTS

### Characteristics, risk factors, causes and management of PGF



### Patient Journey: PGF following HSCT



#### Key Takeaways:

No effective and targeted therapies are available to treat PGF, there is a need of more-efficacious treatments

Establishing PGF diagnosis (causes of cytopenia)- PGF is an early and serious complication of allo-HSCT, so the treatment should start as soon as it is diagnosed

Combination therapy can give better outcomes and response rate

#### Other Unmet Needs

- In treatment of pediatric HSCT PGF patients
- Unavailability of donor at right time- long waiting time for the patient
- Segmentation of PGF into primary and secondary PGFs
- Lack of standardized diagnostic criteria for PGF
- Treatment of chemo-refractory patients after post-HSCT
- Determining the treatment sequencing of PGF- 1st-2nd-3rd line

#### What would be an Ideal Treat:

- Shorten time to recovery after HSCT
- Prevent spread of acute or chronic GVHD in PBSC patients
- Reduced possibility of viral or bacterial infection after transplants
- Reduce chances of readmission to hospitals
- Follow-up in-patient treatment would be possible without necessity of hospitalization
- Increase 1 yr. OS and 100Days OS in patients with primary cancer disease
- Need for a promising therapeutic approach to promote hematopoietic recovery post-HSCT

*"Managing PGF: There are many indications under malignant and non-malignant categories and for genetic and immunodeficiency disorders where HSCT is used for treatment, the prime objective is to treat the primary disease, however, it is highly complex and costly procedure, and PGF arises as an early complication in few of these patients, so again there are many ways by which ...your can minimize PGF severity and its further complications. The effectiveness of treatments varies according to the characteristics of the individuals, the intensity of their treatment, and the side effects experienced by the patient. -Executive Director Policy and Governance – CIBMTR"*

## CONCLUSIONS

The insights gathered from payers highlight significant challenges in the current management of poor graft function (PGF), affecting approximately 7% of patients post-HCT. Payers outlined the characteristics of an ideal treatment, which would accelerate recovery post-HCT, prevent GvHD, lower infection risks, reduce hospital readmissions, and improve survival rates for patients with primary cancers. Some payers also emphasized the need for a prophylactic treatment to prevent PGF occurrence.

Utilizing the PayerPro360 platform proved instrumental in gathering these insights, providing a dynamic interface for real-time payer engagement. By delivering accurate, actionable data on market access, pricing, and reimbursement requirements, PayerPro360 aids product development teams in refining target product profiles and aligning with payer expectations in these high-need therapeutic areas.

Payer-based primary market research for 10+ private and institutional payers in the United States

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