

Best Practices and Interventions: Pediatric Patient Reported Outcomes

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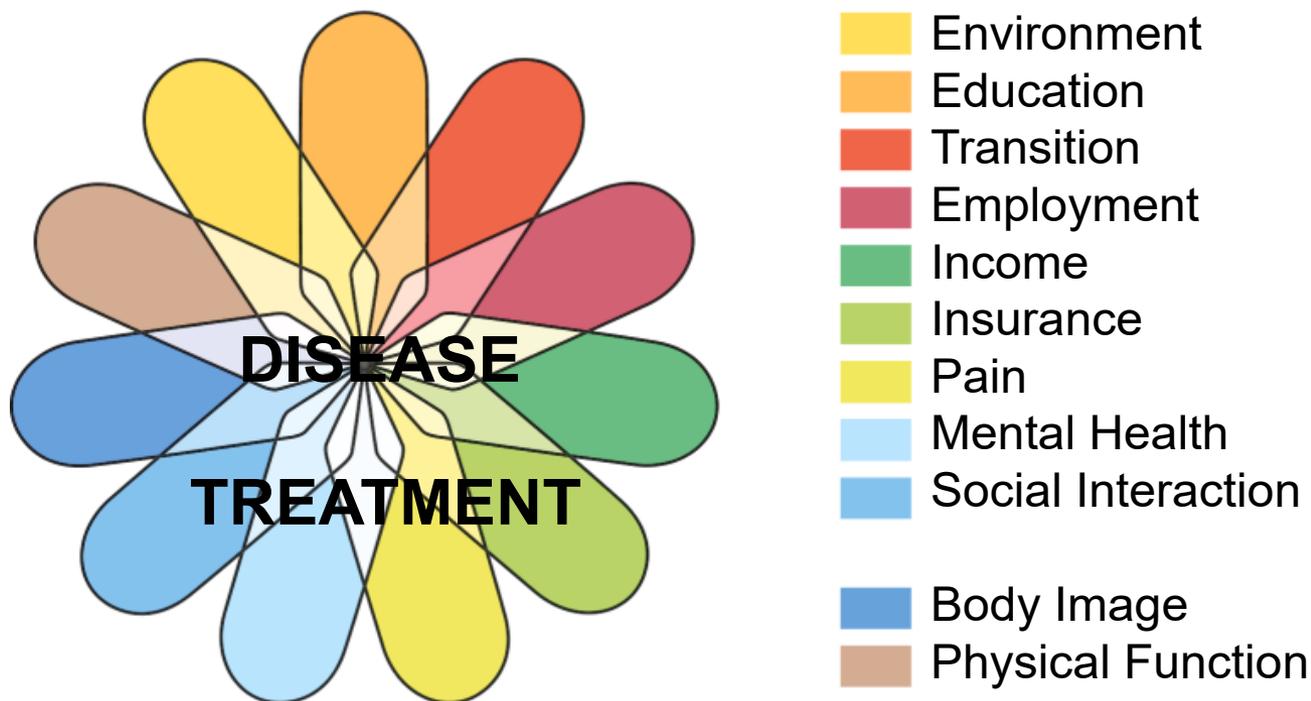
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Agenda

- Background
- Patient Engagement
- Financial Burden
- Social Determinants of Health
- Discussion

Background

- Hematopoietic cell transplantation (HCT) has largely curative intent
- Disability is not always prevented or halted by HCT



Background

- Complexity of PROs for pediatric HCT and disability
 - Development and disability
 - Inability to articulate disability may delay early Intervention services
 - Age-related variations in vocabulary and comprehension of health concepts complicate formatting and design of PROs
 - Frequent hospitalizations, medical visits, and subsequent school absenteeism negatively impact psychosocial development
 - Dependence results in a “dual responses”
 - Patient – primary and secondary
 - Caregiver - secondary



Bele, Sumedh, et al. "Patient-reported outcome measures in routine pediatric clinical care: a systematic review." *Frontiers in pediatrics* 8 (2020): 364.

Aflac Cancer and Blood Disorders Center

Background



PedsQL™



Patient Engagement

“Being in the working group gave us a voice”



Burns, Linda J., et al. "Engaging patients in setting a patient-centered outcomes research agenda in hematopoietic cell transplantation."
Biology of Blood and Marrow Transplantation 24.6 (2018): 1111-1118.

Patient Engagement

Important for the transplant community to understand what a long road transplant can be

"We thought we'd go through the transplant process and get back to our life; it's been everything but that."

If we weren't participating, transplant community wouldn't be aware of the issues that patients experience

Hope that future patients will have a better experience

Exposed us to information and opportunities that we didn't know existed

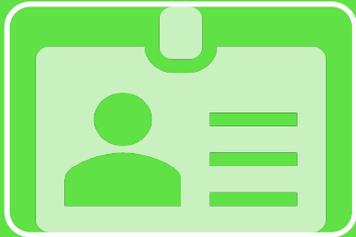
Burns, Linda J., et al. "Engaging patients in setting a patient-centered outcomes research agenda in hematopoietic cell transplantation."
Biology of Blood and Marrow Transplantation 24.6 (2018): 1111-1118.

Financial Burden



School

- Memory and attention concerns
- Restriction in social function



Employment

- Difficulty gaining employment
- Restricting to insure health insurance coverage
- Lapses in coverage or benefits



Reproduction

- Fertility preservation
- Reproductive assistance

Gifford, G., et al. *Internal medicine journal* 2014
Syrjala, K., et al. *JCO* 2005

Financial Burden

- Survey participants were able to consistently define key terms
 - Household income
 - Health
 - Financial hardship
 - Quality of life
- Determined that participants could convey the financial impact of HCT within the scope of the survey
- ***Participants were unaware of many financial resources***

Arnold, Staci, et al. "Developing a validated patient-reported outcome instrument to assess financial hardship among hematopoietic cell transplant recipients for sickle cell disease." *Quality of Life Research*. Vol. 29. No. Suppl 1. Van Godewijckstraat 30, 3311 GZ Dordrecht, Netherlands: Springer, 2020.

Social Determinants of Health

Economic Stability	Neighborhood and Physical Environment	Education	Food	Community and Social Context	Health Care System
Employment	Housing	Literacy	Hunger	Social integration	Health coverage
Income	Transportation	Language	Access to healthy options	Support systems	Provider availability
Expenses	Safety	Early childhood education		Community engagement	Provider linguistic and cultural competency
Debt	Parks	Vocational training		Discrimination	Quality of care
Medical bills	Playgrounds	Higher education		Stress	
Support	Walkability				
	Zip code / geography				

Health Outcomes

Mortality, Morbidity, Life Expectancy, Health Care Expenditures, Health Status, Functional Limitations

Social Determinants of Health

Methods

- Two pediatric cohorts who received a first, allogeneic HCT from 2006-2015 at age ≤ 18 years
- N=2053 malignant
- N=1696 non-malignant

Conclusions

Among children transplanted for malignant disease

- Neighborhood-poverty conferred an increased risk of TRM
- Medicaid insurance was associated with inferior OS and increased TRM compared to private insurance.

Social Determinants of Health

Methods

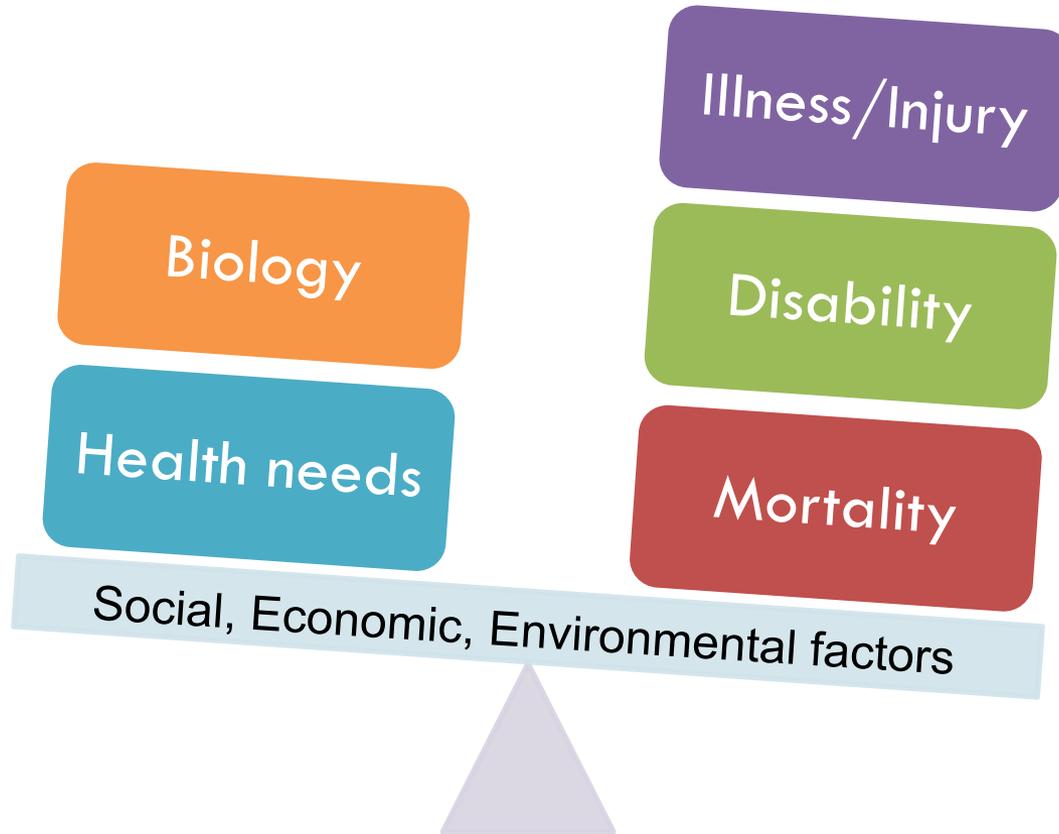
- Two insurance cohorts who received a first, allogeneic HCT from 2008-2018 for sickle cell disease
- N=225 Medicaid
- N = 174 private

Conclusions

- Compared to private insurance, Medicaid had
- Lower event-free survival
 - Higher graft failure

Mupfudze, Tatenda G., et al. "Hematopoietic Cell Transplantation Outcomes among Medicaid and Privately Insured Patients with Sickle Cell Disease." *Transplantation and Cellular Therapy* (2021).

Healthcare Disparities



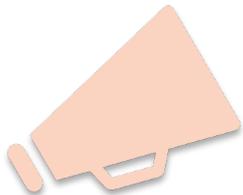
Conclusions



Healthcare needs continue
after HCT



Health-related disability and
financial burden intersect



Resources must be well
publicized and communicated

Questions



Image: One Public Education

Notes

- Highlight reliable and valuable pediatric measures
- Intersection of financial burden and disability
 - Rationale in pediatrics
- Spend time with the patient engagement for the feel
- Potential to ameliorate SES

Questions

- What are some of the elements of pediatric standard of care (best practice) to give the patients the best chance at a full recovery?
- When/How are children opposed to their parents engaged in the determination of their recovery e.g. completing measures of QOL?
- What are the most important ‘resources’ that all pediatric patients/caregivers should know about?
 - Caregiver costs
 - Lodging
 - Prescription costs
 - Childcare for patients with children