



Memorial Sloan Kettering  
Cancer Center

# CAR T Cell Therapy & Patient Reported Outcomes

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# Disclosures

- Representing the American Society for Blood and Marrow Transplantation and received travel funds



# What is a Patient Reported Outcome?

- Symptoms, functional status, and perceptions of health-related quality of life as reported directly by the patient without interpretation by clinicians
  - assess the **severity of symptoms** and the **interference with daily living** caused by these symptoms.
- Measured by a variety of **scales**
  - Vary by domains included, number of questions, time frame, Likert scale (1-5 vs 1-10)
  - Vary by specificity – general to disease/treatment specific
  - Validation
- How to **capture**?
  - Paper survey vs electronic questionnaire
- **Research** vs Clinical Care



# Review of PRO in Hematopoietic Cell Transplant

**Table 1.** Measures used in 114 HRQoL studies in HCT (excluding those used in only one study)

Form	No. of studies	Multidimensional vs limited domain	Minutes to complete	Access	Target population	Question type
FACT-BMT (TOI)	28	M	5–10	F, R	HCT	47 (ordinal scale)
EORTC QLQ-C30	26	M	10	L (F in academic)	Cancer	30 y/n or ordinal scale
SF-36	26	M	5–10	L (F for academic)	Healthy/patient	36 y/n or ordinal scale
HADS (Hospital Anxiety and Depression Scale)	7	LD	2–5	F	Patient	14 (ordinal scale)
SIP (Sickness Impact Profile)	6	M	20–30	F for non-funded studies	Patient	136 (ordinal scale)
Study-specific/local questionnaire	5		NA	NA	NA	NA
Chronic GvHD Symptom Scale	4	M	5	F	GvHD	30 (ordinal scale)
COH (City of Hope) -QOL (BMT)	4	M	10–20	F	HCT/cancer	41/84 items by interview
FACT-G	4	M	5–10	F, R	Cancer	28 (ordinal scale)
POMS (SF) (profile of mood state)	4	LD	3–7	P	Psychiatry/psychology	65 (30) (ordinal scale)
SLDS-C (BMT) —Satisfaction with Life Domains Scale for Cancer	4	LD	< 10		HCT	18 (faces)
SF-12	4	M	< 3	L (F for academic)	Healthy/patient	12 y/n or ordinal scale
Center for Epidemiological Studies (CES)-D (Depression)	4	LD	5–10	F	Healthy	20 (ordinal)
FACIT (-Spiritual)	3	LD	5–10	F, R	Cancer/chronic illness	12 (ordinal scale)
BDI (Beck's Depression Inventory)	3	LD	5–10	P	Psychiatry/psychology	21 (ordinal scale)
RSCL (Rotterdam Symptom Checklist)	3	M	8	F	Cancer	39 (ordinal scale)
Symptom Checklist-90 (revised)	3	M	12–15	P	Healthy > 13	90 (ordinal scale)
Spielberger State Anxiety Scale (subscales)	3	LD	10	P	Patient	40/20 (ordinal scale)
Cancer Rehabilitation Evaluation System (CARES-SF)	2	M	10–34	F	Cancer	59/139 (ordinal scale)
NHP (Nottingham Health Profile)	2	M	10–15		Healthy/patient	38 y/n
HRQoL index	2	M	10	R, F in academic		66 (ordinal scale)
EQ-5D (VAS)	2	LD	1–2	R, P	Healthy/patient	5 (Visual analog)
SDS (Symptom Distress Scale)	2	LD	5–10	Permission granted on request	Cancer	13 (ordinal scale)
HAP (Human Activity Profile)	2	LD	5–7	F in academic	COPD	94
EORTC QLQ-HDC29	2	MD		L (F in academic)	Cancer	29 (ordinal scale)
(Revised) Piper Fatigue Scale	2	LD	< 2	F	Cancer	27 (1–10, 4 open ended)
Coping Responses Inventory	2	LD	10–15	P	Healthy	48 (ordinal scale)
Interpersonal Support Evaluation List-Short Form (ISEL-SF)	2	LD		L (F in academic)	Healthy	12/40/48 (ordinal scale)

Abbreviations: F=free; GvHD=graft versus host disease; HCT=hematopoietic cell transplantation; L=requires license; LD=limited domain; M=multidimensional; n=nq; NA=not applicable; P=payment required; R=register; y=yes.



## PROMIS® Adult Self-Reported Health

Global Health

### Physical Health

Fatigue  
Pain Intensity  
Pain Interference  
Physical Function  
Sleep Disturbance

Dyspnea  
Gastrointestinal Symptoms  
Pain Behavior  
Pain Quality  
Sexual Function  
Sleep-related Impairment

### Mental Health

Anxiety  
Depression

Alcohol  
Anger  
Cognitive Function  
Life Satisfaction  
Positive Affect  
Psychosocial Illness Impact  
Self-efficacy for Managing Chronic Conditions  
Smoking  
Substance Use

### Social Health

Ability to Participate in Social Roles & Activities

Companionship  
Satisfaction with Social Roles & Activities  
Social Isolation  
Social Support

PROMIS Profile Domains

PROMIS Additional Domains

4/13/2007

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MDASI-AML/MDS

Acute Myeloid Leukemia/Myelodysplastic Syndrome Module

MDASI-BT

Brain Tumor Module

MDASI-CGVHD

Chronic Graft-versus-Host Disease Module

MDASI-CML

Chronic Myeloid Leukemia Module

MDASI-GI

Gastrointestinal Cancer Module

## FACT-BMT (Version 4)

Below is a list of statements that other people with your illness have said are important. Please circle or mark one number per line to indicate your response as it applies to the past 7 days.

### PHYSICAL WELL-BEING

		Not at all	A little bit	Some-what	Quite a bit	Very much
GP1	I have a lack of energy .....	0	1	2	3	4
GP2	I have nausea .....	0	1	2	3	4
GP3	Because of my physical condition, I have trouble meeting the needs of my family .....	0	1	2	3	4
GP4	I have pain .....	0	1	2	3	4
GP5	I am bothered by side effects of treatment .....	0	1	2	3	4
GP6	I feel ill .....	0	1	2	3	4
GP7	I am forced to spend time in bed .....	0	1	2	3	4

## PATIENT-REPORTED OUTCOMES VERSION OF THE COMMON TERMINOLOGY CRITERIA FOR ADVERSE EVENTS (PRO-CTCAE™) ITEM LIBRARY (Version 1.0)

Oral	Cardio/Circulatory	Neurological	Sleep/Wake	Sexual
Dry mouth S	Swelling FSI	Numbness & tingling SI	Insomnia SI	Achieve and maintain erection S
Difficulty swallowing S	Heart palpitations FS	Dizziness SI	Fatigue SI	Ejaculation F
Mouth/throat sores SI				Decreased libido S
Cracking at the corners of the mouth (cheilosis/cheilitis) S	Cutaneous	Visual/Perceptual	Mood	Delayed orgasm P
Voice quality changes P	Rash P	Blurred vision SI	Anxious FSI	Unable to have orgasm P
Hoarseness S	Skin dryness S	Flashing lights P	Discouraged FSI	Pain w/sexual intercourse S
	Acne S	Visual floaters P	Sad FSI	
	Hair loss P	Watery eyes SI		
	Itching S	Ring in ears S		
	Hives P			
Gastrointestinal	Hand-foot syndrome S	Attention/Memory	Gynecologic/Urinary	Miscellaneous
Taste changes S	Nail loss P	Concentration SI	Irregular periods/vaginal bleeding P	Breast swelling and tenderness S
Decreased appetite SI	Nail ridging P	Memory SI	Missed expected menstrual period P	Bruising P
Nausea FS	Nail discoloration P		Vaginal discharge P	Chills FS
Vomiting FS	Sensitivity to sunlight P	Pain	Vaginal dryness S	Increased sweating FS
Heartburn FS	Bed/pressure sores P	General pain FSI	Painful urination S	Decreased sweating P
Gas P	Radiation skin reaction S	Headache FSI	Urinary urgency FI	Hot flashes FS
Bloating FS	Skin darkening P	Muscle pain FSI	Urinary frequency PI	Nosebleed FS
Hiccups FS	Stretch marks P	Joint pain FSI	Change in usual urine color P	Pain and swelling at injection site P
Constipation S			Urinary incontinence FI	Body odor S
Diarrhea F				
Abdominal pain FSI				
Fecal incontinence FI				
Respiratory				
Shortness of breath SI				
Cough SI				
Wheezing S				

Attributes	
F: Frequency	I: Interference
S: Severity	P: Presence/Absence /Amount

\*[HealthMeasures.net/PROMIS](http://HealthMeasures.net/PROMIS)

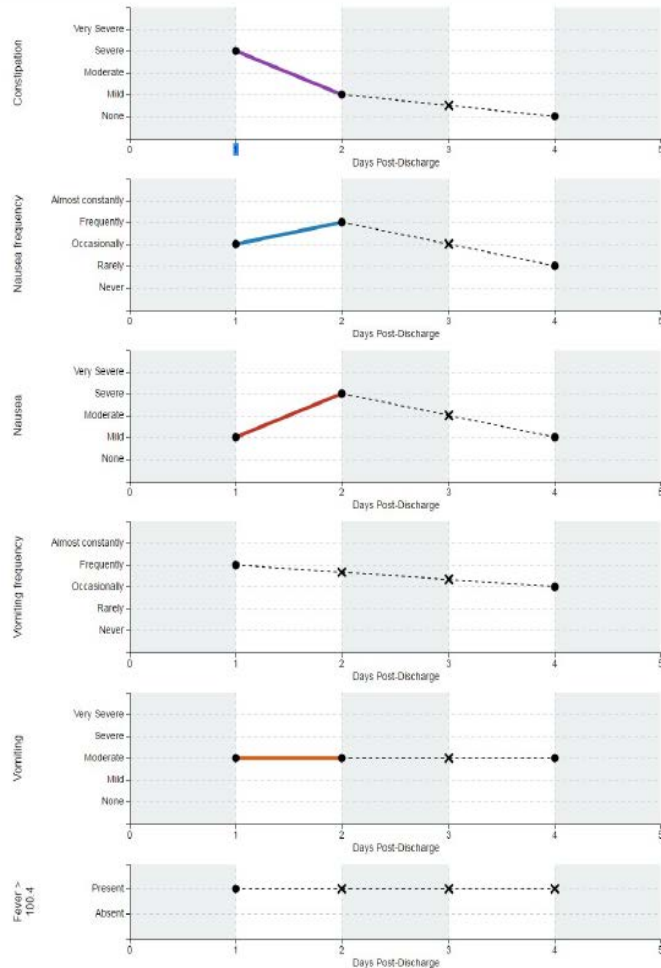
\*\*McQuellon BMT 1997

\*\*\*Cleeland Cancer 2000



# What does the data look like? -- MSK Engage

## Symptoms Over Time



## PRO-CTCAE Submission Status

Is Patient a Portal User?  
True

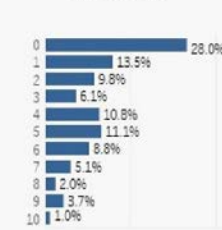
Total PRO-CTCAE Surveys Assigned	# of Unique Patients Assigned Surveys	# of Unique Patients Submitting	Avg. Completion Rate	Is Patient a Portal User?		Completion Statistics		
				False	True	Completed	Missed	To Do
3,567	356	213	30%	60	296	883	2,580	104

### PRO-CTCAE Completed by Week

Volume of assigned surveys that have been completed over time and by Post Operative Day

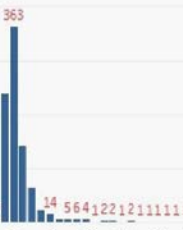


Patients Submitting by # of Surveys  
(% of patients submitting x number of surveys out of 10)



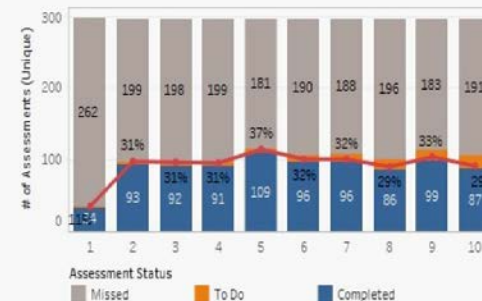
### Time Taken on Surveys (Min)

# of Surveys in Red



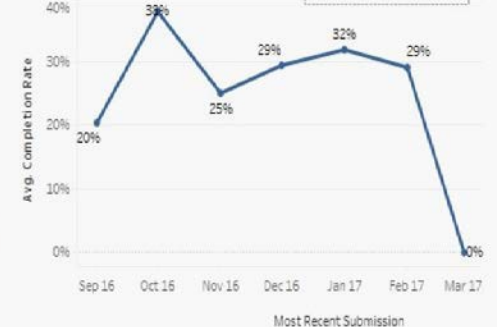
### # of Assessments by Post Op Day and Submission Status

Percent and Volume of Overall Surveys that were completed by Post Operative Day. The red line indicates the overall average completion rate by Post Op Day. Post Op Day 1= the day after discharge.



### Completion Rate by Month

Avg. Completion Rate



February 22, 2017



## Conclusions:

# CAR T-cell PRO are still in development and too early to mandate

- PROs are important to capture and study both in the clinical trial and commercial product setting
- Which measure, at what timepoints, and duration of assessments remain active questions
- How to account for bias due to inability to respond because of medical issues at the time not determined
- Interventions in real time vs collecting data only potentially limit feasibility
- Use of technology may allow for more robust collection, but limit to centers with these capabilities.