

LEUKEMIA2020-2021

April 26-27, 2021

Coordinator: A.M. Carella

AIL President: S. Amadori

Clinical advances in hematologic malignancies: paving the way for better quality of life outcomes

Dr. Fabio Efficace

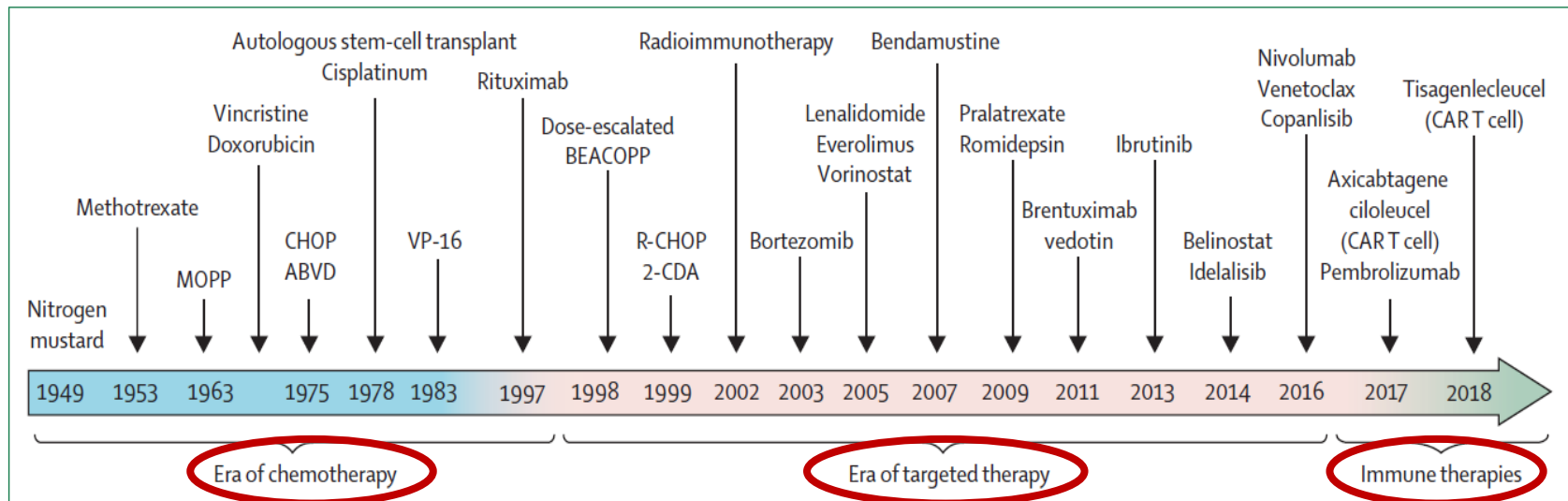
Head, Health Outcomes Research Unit, **GIMEMA Central Office**, Rome, Italy



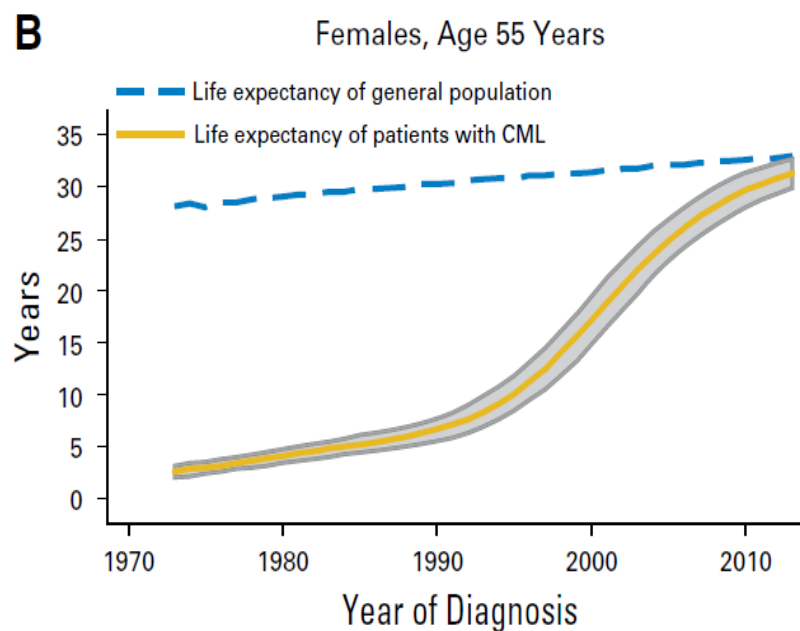
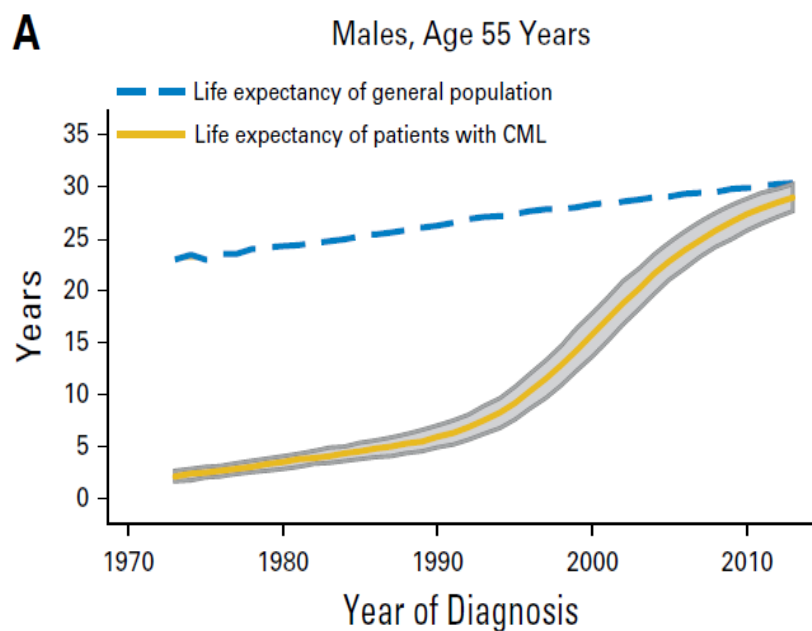
The outstanding progress of hematologic research

Novel treatments have changed the natural history of many hematological malignancies

The Evolution of **Lymphoma** treatments



Life Expectancy of patients with **chronic myeloid leukemia** now approaches that of the General Population



(Bower H, et al, J Clin Oncol. 2016, 34:2851-7, 2016)

Why should we Assess Quality of Life (QoL) in Hematology?



We need additional information to facilitate clinical-decision making



- Patients are living longer
- Several available treatment options have similar clinical efficacy
- Quality of Life is associated with Adherence to Therapy (oral drugs)
- Better define “intolerance” to a given therapy
- Regulators (eg. FDA and EMA) needs “good” QoL data

Two broad areas of applications of QoL Assessment



Clinical Research

To better understand overall treatment effectiveness



Clinical Practice

To help enhance individualized treatment decisions

Quality of Life Assessment in Clinical Research



Some examples in challenging
research settings...



Patient-reported quality of life after tisagenlecleucel infusion in children and young adults with relapsed or refractory B-cell acute lymphoblastic leukaemia: a single-arm, phase 2 trial

Theodore W Laetsch, Gary Douglas Myers, André Baruchel, Andrew C Dietz, Michael A Pulsipher, Barbara De Moerloose, Kara L Davis, Eneida Nemecek, Timothy Driscoll, Françoise Mechinaud, Christina Peters, Himalee S Sabnis, Stephan A Grupp, Gregory A Yanik, Hidefumi Hiramatsu, Jie Zhang, Sweta Shah, Andrew C Harris

Highly vulnerable population:

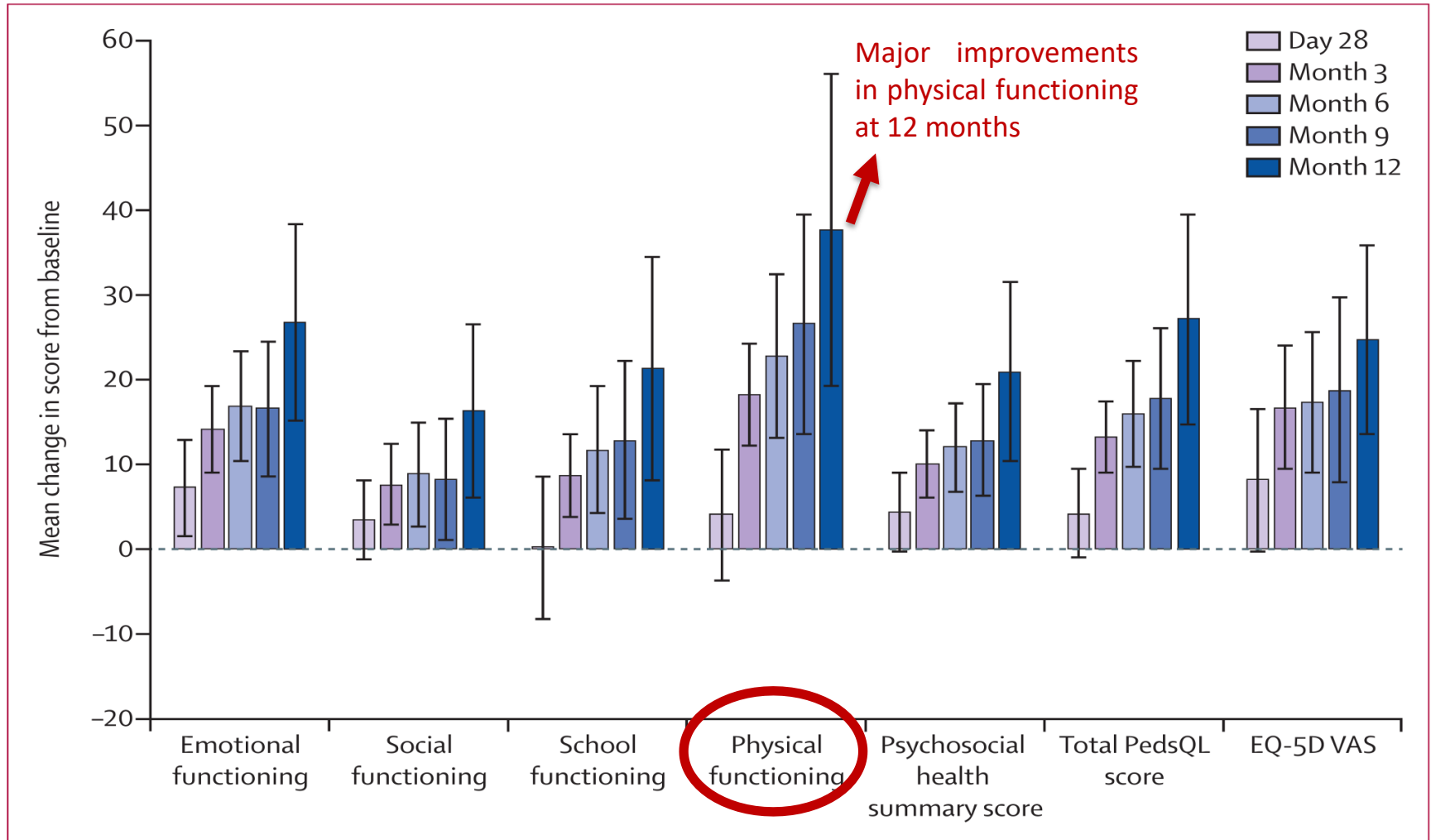
- Age: 8-23 years
- 90% relapsed disease
- 60% at least 1 HSCT
- 77% cytokine release syndrome

	Patients with quality-of-life assessment at baseline and at least one postbaseline (n=48)	Patients without quality-of-life assessment at baseline and at least one postbaseline (n=10)
Sex		
Female	19 (40%)	6 (60%)
Male	29 (60%)	4 (40%)
Age, years		
Mean (SD)	14.3 (4.5)	11.9 (3.6)
Median (IQR)	14.0 (10.0-17.5)	11.5 (9.0-14.0)
Race		
White	38 (79%)	8 (80%)
Other	10 (21%)	2 (20%)
Karnofsky or Lansky performance status		
90-100%	28 (58%)	5 (50%)
<90%	20 (42%)	5 (50%)
Previous haemopoietic stem-cell transplantation		
0	18 (38%)	5 (50%)
1 or 2	30 (62%)	5 (50%)
Disease status		
Primary refractory	5 (10%)	0
Relapsed disease	43 (90%)	10 (100%)

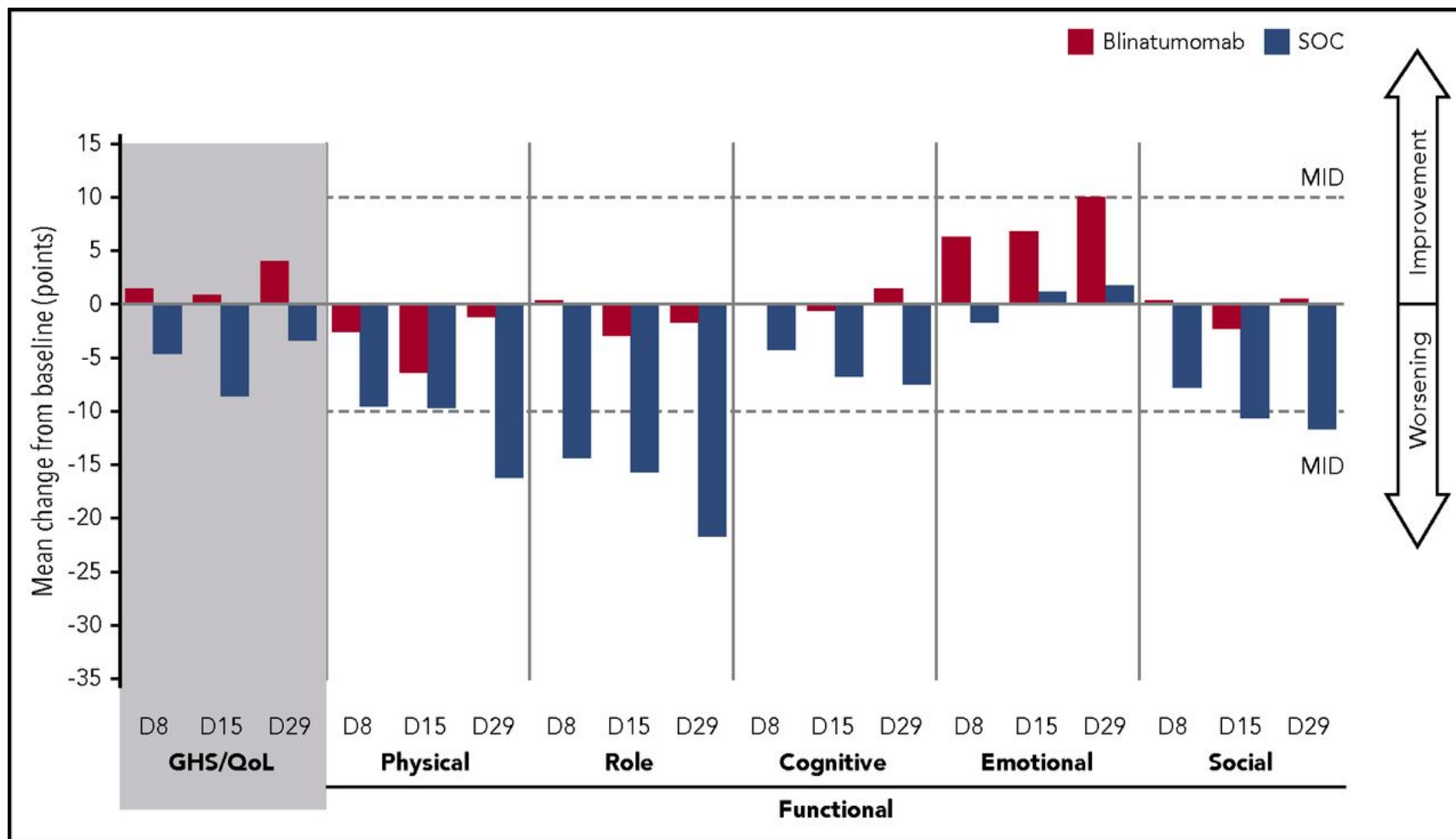
Data are n (%) unless otherwise stated. Quality-of-life assessment defined non-missing data for either the European Quality of Life-5 Dimensions visual analogue scale or Pediatric Quality of Life Inventory total score.

Table 2: Demographic and baseline characteristics according to data availability at baseline and postbaseline visits

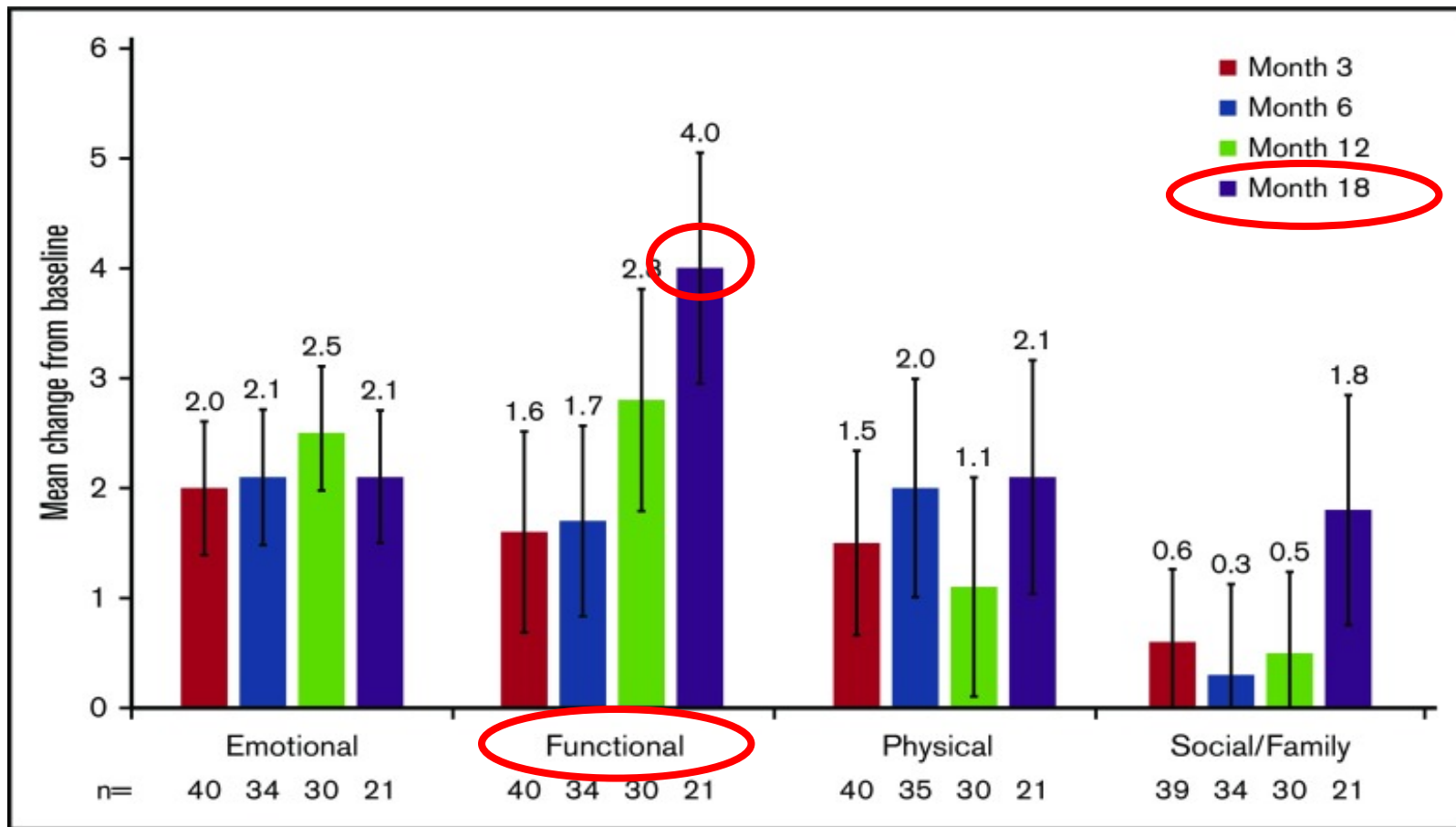
Quality of Life over time in relapsed or refractory ALL patients



QoL Outcomes: **blinatumomab** versus standard of care (SOC)



QoL in patients with relapsed/refractory diffuse large B-cell lymphoma who respond to therapy improves over time



QoL information provide independent prognostic information for Overall Survival Example in higher risk MDS Patients

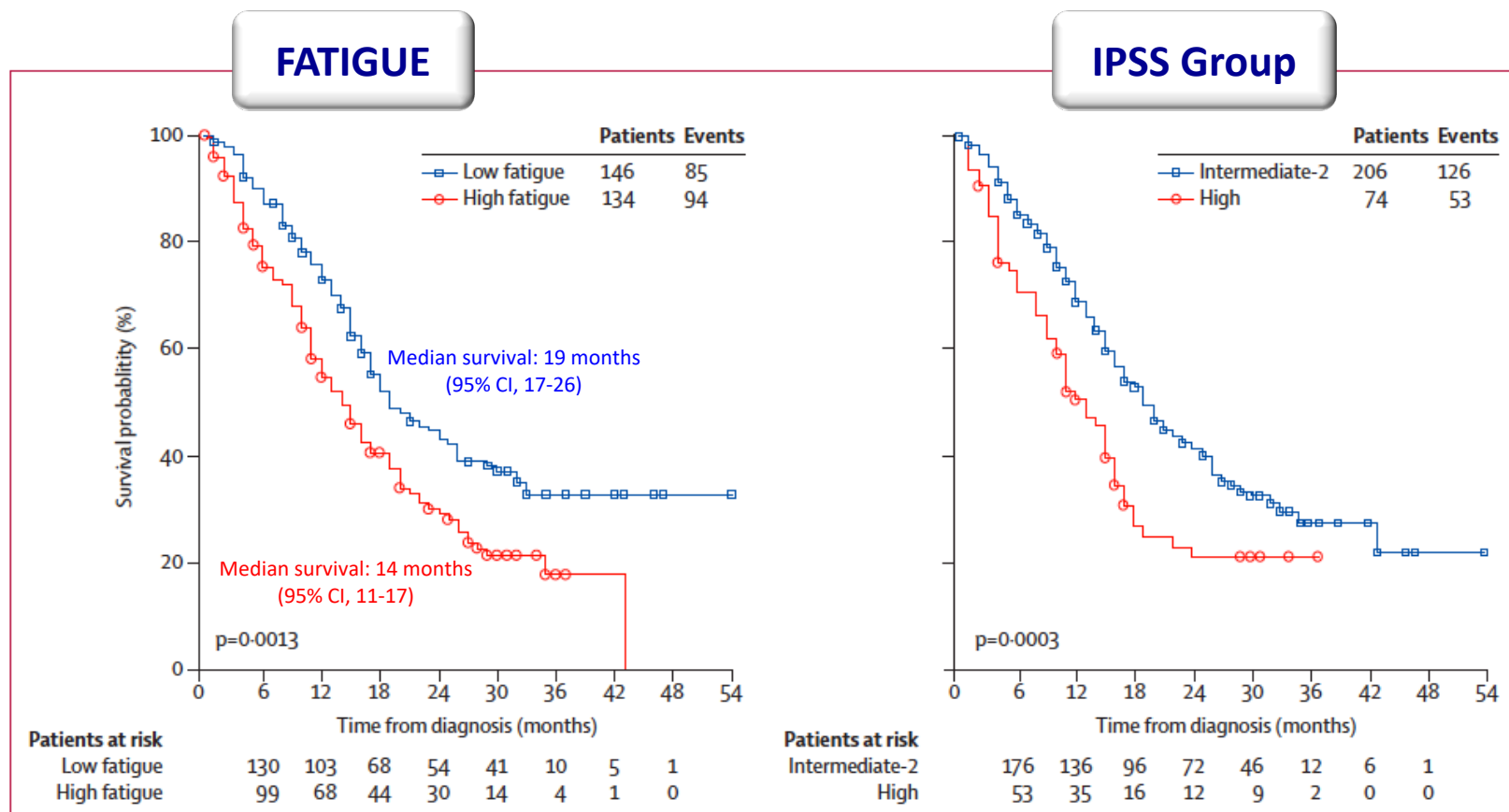


Figure 1: Overall survival by baseline patient's self-reported fatigue severity and IPSS risk group

Low fatigue denotes patients reporting a baseline EORTC QLQ-C30 fatigue score lower than median value (34 points). High fatigue denotes patients reporting a baseline EORTC QLQ-C30 fatigue score equal or higher than the median value. EORTC QLQ-C30=European Organisation for Research and Treatment of Cancer, quality of life questionnaire-core 30. IPSS=International Prognostic Scoring System.

Quality of Life Assessment in Routine Practice



What are the advantages of assessing Quality of Life in **Routine Cancer Practice**

- Facilitate and improve communication between patients and physicians
- Facilitate shared-decision making
- Enhance patient satisfaction
- Improve symptom control
- Improve Quality of Life
- **Improve Survival Outcomes**



(Basch E, et al, J Clin Oncol. 34:557-65, 2016; Bennet AV et al., CA Cancer J Clin 62:336-347, 2012; Kotronoulas G, et al, J Clin Oncol, 32:1480–1501, 2014; Snyder C, et al, Qual Life res 21:1305-1314, 2012; Frost MH, Mayo Clin Proc, 82:1214–1228, 2007; Velikova G, et al, J Clin Oncol, 22:714–724, 2004; McLachlan SA, et al, J Clin Oncol, 19:4117–4125, 2001; Detmar SB, et al, Eur J Cancer, 34:1181–1186, 1998; Denis F. et al. J Natl cancer Inst 109(9), 2017; Basch E, et al . Am Soc Clin Oncol Educ Book. 2018 May 23;38:122-134)

JOURNAL OF CLINICAL ONCOLOGY

ORIGINAL REPORT

Symptom Monitoring With Patient-Reported Outcomes During Routine Cancer Treatment: A Randomized Controlled Trial

Ethan Basch, Allison M. Deal, Mark G. Kris, Howard I. Scher, Clifford A. Hudis, Paul Sabbatini, Lauren Rogak, Antonia V. Bennett, Amylou C. Dueck, Thomas M. Atkinson, Joanne F. Chou, Dorothy Dulko, Laura Sit, Allison Barz, Paul Novotny, Michael Fruscione, Jeff A. Sloan, and Deborah Schrag

Ethan Basch, Mark G. Kris, Howard I. Scher, Clifford A. Hudis, Paul Sabbatini, Lauren Rogak, Thomas M. Atkinson, Joanne F. Chou, Dorothy Dulko, Laura Sit, Michael Fruscione, and Deborah Schrag, Memorial Sloan Kettering Cancer Center, New York, NY; Ethan Basch, Allison M. Deal, and Antonia V. Bennett, University of North Carolina, Chapel Hill, NC; Amylou C. Dueck, Mayo Clinic, Scottsdale, AZ;

A B S T R A C T

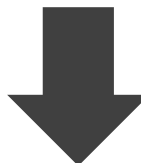
Purpose

There is growing interest to enhance symptom monitoring during routine cancer care using patient-reported outcomes, but evidence of impact on clinical outcomes is limited.

Methods

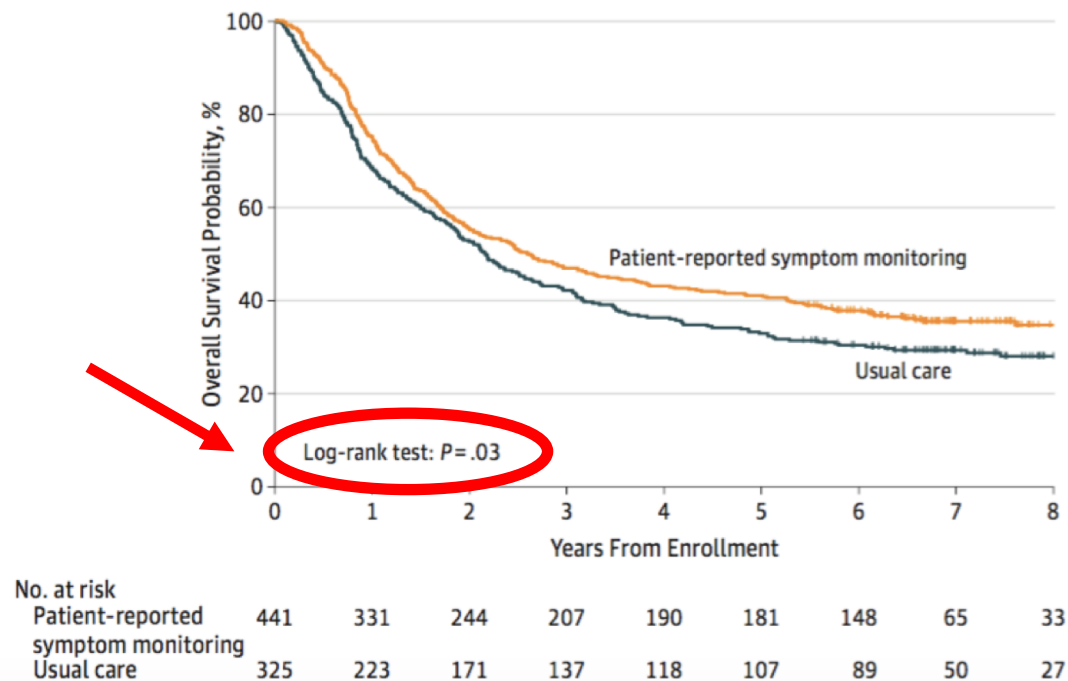
We randomly assigned patients receiving routine outpatient chemotherapy for advanced solid tumors at Memorial Sloan Kettering Cancer Center to report 12 common symptoms via tablet

Basch e, et al., J Clin Oncol. 2016 Feb 20;34(6):557-65



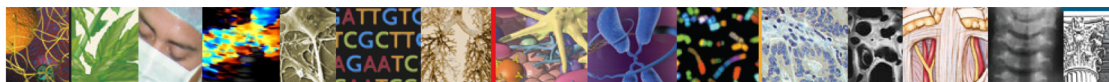
Assessing Patient-Reported Symptoms in Cancer Care Improves Overall Survival

Figure. Overall Survival Among Patients With Metastatic Cancer Assigned to Electronic Patient-Reported Symptom Monitoring During Routine Chemotherapy vs Usual Care



Basch E, et al, JAMA. 2017; doi: 10.1001/jama.2017.7156

Assessing QoL in routine care does not increase time of clinical visits



The NEW ENGLAND JOURNAL of MEDICINE

“...most surprisingly, PROs can enhance workflow efficiency and **save time** when they’re used regularly”

(Rotenstain L, et al New Engl J Med, 2017)

Perspective
OCTOBER 5, 2017

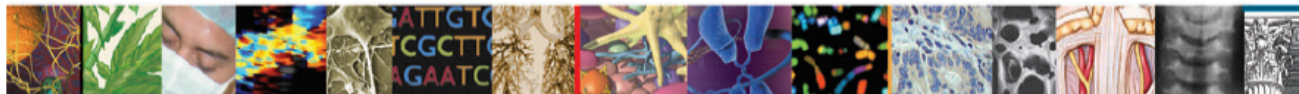
Making Patients and Doctors Happier — The Potential of Patient-Reported Outcomes

Lisa S. Rotenstein, M.D., M.B.A., Robert S. Huckman, Ph.D., and Neil W. Wagle, M.D., M.B.A.

Measuring relevant outcomes in a timely manner is a priority in a health care system increasingly focused on the delivery of high-value care. Most quality measures focus

collecting PROs for clinical and research purposes in 1998. In 2009, patients at the DHMC Spine Center were surveyed, and one third of them said that incor-

The Importance of Digital Health in the era of COVID-19



The NEW ENGLAND JOURNAL of MEDICINE

Fortunately, the world is a different place than it was in 1918. We have the technology to strengthen our health care system for our patients. It's time we put these tools into practice.

NEJM, June 2020
Perspective
JUNE 4, 2020

Covid-19 and Health Care's Digital Revolution

Sirina Keesara, M.D., Andrea Jonas, M.D., and Kevin Schulman, M.D.

In the face of the Covid-19 outbreak, Americans are waking up to the limitations of their analogue health care system. It seems clear that we need an immediate digital revolution to face this crisis.

cases, urgent action is required to transform health care delivery and to scale up our systems by unleashing the power of digital technologies.¹ Although some digital

A real-word example of a Digital Health Tool in Routine Practice: **GIMEMA ALLIANCE-Platform**



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[Comunicazioni urgenti al Medico](#)

[Le mie visite online](#)

[ESCI](#)

BENVENUTO/A IN GIMEMA ALLIANCE

Il Portale per tenere aggiornato il Suo ematologo

Il mio Questionario



Clicchi il pulsante sottostante per completare il Suo Questionario.



Comunicazioni urgenti al Medico



Qui può comunicare eventuali sintomi e/o una diagnosi da COVID-19 al Suo ematologo



Le mie visite online

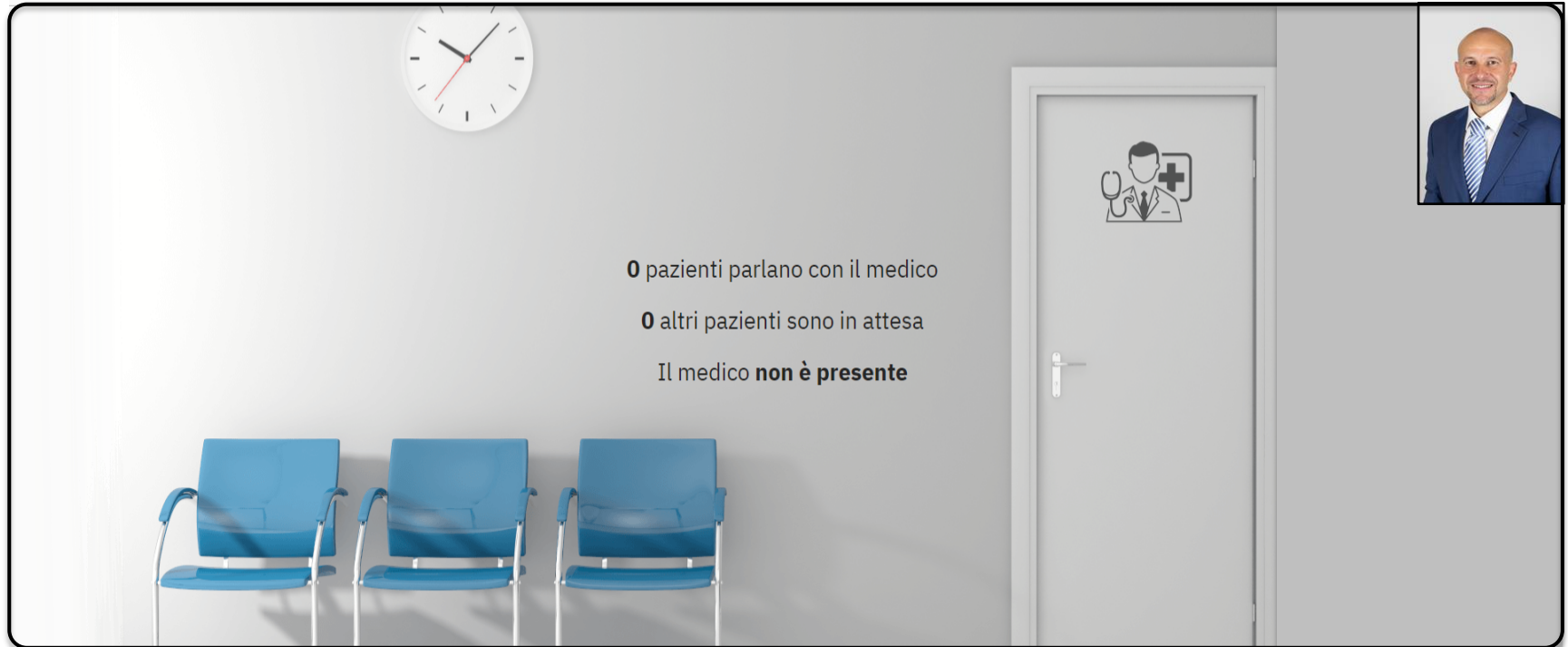


Clicca qui per visualizzare le visite online pianificate.



The Virtual Waiting Room for **Video Consultations**

 **fondazione GIMEMA** onlus
per la promozione e lo sviluppo della ricerca scientifica
sulle malattie ematologiche **FRANCO MANDELLI**



Example of Items to be completed by patients (touch-screen)



Negli ultimi 7 giorni, quanto è stato GRAVE il SENSO DI FATICA, STANCHEZZA O MANCANZA DI ENERGIA nel momento PEGGIORE?

Per nulla

Un po'

Abbastanza

Molto

Moltissimo



Physician Portal GIMEMA-ALLIANCE



GIMEMA - ALLIANCE



La Salute dei tuoi Pazienti, anche a distanza

MONITORA LO STATO DI
SALUTE E L'ADERENZA
ALLA TERAPIA

GARANTISCI LA CONTINUITÀ
DELLE CURE ANCHE A
DISTANZA

FORNISCI UN SUPPORTO
MAGGIORE AI PAZIENTI



ACCESSO

Nome utente

admin
From this website

Other Passwords for ches.pro...

Inizia

CHES - Computer-based Health Evaluation System

Per qualsiasi domanda o problema tecnico, rivolgersi all'amministratore di sistema alliance@gimema.it

Patient-Reported Symptom Profile

CHES

Gestione dei dati admin

Nome- Cognome

Dati Pazienti

CRF

QoL Paziente

- Report trasversale
- Report longitudinale
- Panoramica

Questionario

- Compila
- Aggiungi
- Mettili a disposizione per Monitor

Visite Online

Pianifica

EORTC Sintomi Specifici

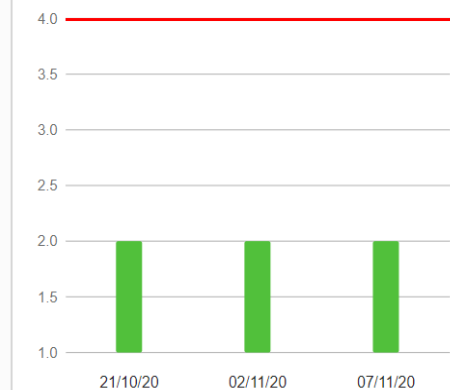
Visualizzazione: Grafico a barre con barre colorate

Valori di riferimento: Clinical Thresholds

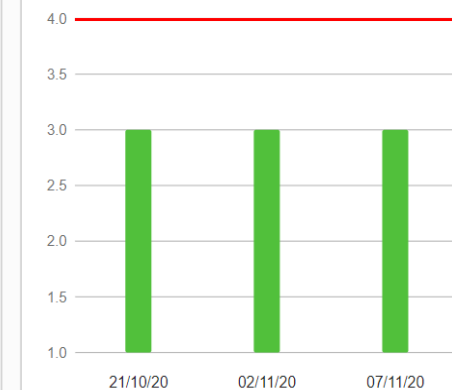
Stampa

Sintomi

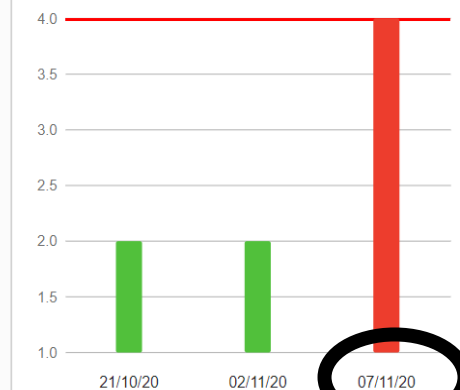
Mal di testa



Crampi muscolari



Dolore osseo



Conclusions

- ➔ **The outstanding achievements obtained in several areas of Hematology have paved the way for a more systematic use of QoL measures in clinical research and routine practice.**
- ➔ **QoL data provide unique information that are not captured with traditional biomedical indicators (e.g., Anemia, Performance status, Cytogenetics) or Physician-reported Toxicity data**
- ➔ **Assessing QoL in routine clinical practice:** can facilitate a greater physician awareness of their patients' symptom burden and my enhance patient-physician communication