Aspetti economico-sanitari

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Mobilizzazione di cellule staminali emopoietiche "chemo-free" nel Mieloma Multiplo: è tempo di prime time?

Bologna, 16 Marzo 2017

Conflitto di interessi

- Nel corso del biennio 2014-2016, CL ha ricevuto finanziamenti non condizionati per attività di ricerca, consulenza e presentazioni congressuali da parte delle seguenti aziende: AbbVie S.r.l.; Boehringer Ingelheim Italia S.P.A.; Boston Scientific S.p.A.; Celgene Italia Srl; CSL Behring SpA; Ferring S.p.A.; Helsinn Healthcare SA; Roche S.p.A; UCB Pharma S.p.A.

Descrizione del costo sociale del MM in Italia - I

Tumori, 99: e193-e202, 2013

Cost of illness in patients with multiple myeloma in Italy: the CoMiM study

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ABSTRACT

Aims and background. Multiple myeloma is the second most common hematological cancer. Although it accounts for only a relatively small percentage of all cancer types, the costs associated with managing multiple myeloma are considerable. Available studies are mainly focused on health care costs. The *Costo del Mieloma Multiplo* (Cost of MM, CoMiM) study investigated the cost of illness of multiple myeloma in Italy during one year of disease management.

Methods. CoMiM is a retrospective, prevalence-based, multi-center, cross-sectional study based on a stratified sample of patients seen during normal clinical practice (asymptomatic; symptomatic on drugs; symptomatic receiving autologous stem cell transplantation; plateau/remission). Demographics, clinical history, health care and non-health care resource consumption data were collected. Costs were evaluated from the societal viewpoint and expressed in Euro 2008.

Results. Data on 236 patients were analyzed (39 asymptomatic, 17%; 29 symptomatic receiving autologous stem-cell transplantation, 12%; 105 symptomatic receiving drugs, 44%; 63 plateau/remission, 27%). The total cost of illness reached € 19,267.1 ± 25,078.6 (asymptomatic, € 959.3 ± 1091.6; symptomatic receiving drugs, € 21,707.8 ± 21,785.3; symptomatic receiving autologous stem-cell transplantation, € 59,243.7 ± 24,214.0; plateau/remission, € 8130.7 ± 15,092.5). The main cost drivers of total cost of illness were drugs and hospital admissions (46.1% and 29.4%, respectively). Antineoplastics and immunomodulators drove the cost of drugs (21.6% and 21.1% of the total cost of illness). Cost of antineoplastics was led by bortezomib (97.4%), whereas the cost driver for immunomodulators was lenalidomide (99.4%). Cost of hospitalization funded by the Italian National Health Service was strongly influenced by transplantation (94.6%), whereas chemotherapy and skeletal fractures did not exceed 1% and 2%, respectively.

Conclusions. Despite some limitations, the CoMiM study provides Italian health care decision-makers with an insight into the stratified cost of illness of multiple myeloma patients.

Key words: cost of illness, hematological cancer, Italy, multiple myeloma.

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Petrucci et al., Tumori 2013; 99(4): 193e-202e

Descrizione del costo sociale del MM in Italia - II

Table 1 - Sample characteristics

	Asymptomatic	Symptomatic		Plateau/remission	Total
		Drugs	ASCT		
Disease situation, n (%)	39 (16.5)	105 (44.5)	29 (12.3)	63 (26.7)	236 (100)
Gender: Female, n (%)	14 (35.9)	53 (50.5)	14 (48.3)	33 (52.4)	114 (48.3)
Age[a]					
Mean ± SD	67.8±9.7	69.3±8.8	58.7±7.4	64.3±9.7	66.4±9.6
Stage of disease, n (%)					
0	2 (5.1)	6 (5.7)	0 (0.0)	6 (9.5)	14 (5.9)
1	31 (79.5)	44 (41.9)	12 (41.4)	39 (61.9)	126 (53.4)
2	5 (12.8)	32 (30.5)	12 (41.4)	9 (14.3)	58 (24.6)
3	1 (2.6)	23 (21.9)	5 (17.2)	9 (14.3)	38 (16.1)
Years from diagnosis ≤5, n (%)	23 (59.0)	77 (73.3)	27 (93.1)	37 (58.7)	164 (69.5)
Complications[b], n (%)					
0	35 (89.7)	59 (56.2)	10 (34.5)	32 (50.8)	136 (57.6)
1	3 (7.7)	32 (30.5)	15 (51.7)	25 (39.7)	75 (31.8)
2	1 (2.6)	12 (11.4)	3 (10.3)	4 (6.4)	20 (8.5)
3	0 (0.0)	2 (1.9)	1 (3.5)	2 (3.2)	5 (2.1)
4	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Comorbidities[c], n (%)					
0	17 (43.6)	56 (53.3)	13 (44.8)	35 (55.6)	121 (51.3)
1	13 (33.3)	34 (32.4)	14 (48.3)	22 (34.9)	83 (35.2)
2	8 (20.5)	13 (12.4)	2 (6.9)	6 (9.5)	29 (12.3)
≥3	1 (2.6)	2 (1.9)	0 (0.0)	0 (0.0)	3 (1.3)
Employment status, n (%)					
Employed	5 (12.8)	9 (8.6)	9 (31.0)	10 (15.9)	33 (14.0)
Other	1 (2.6)	5 (4.7)	1 (3.5)	0 (0.0)	7 (3.0)
Housekeeper	6 (15.4)	13 (12.4)	3 (10.3)	5 (7.9)	27 (11.4)
Retired	27 (69.2)	76 (72.4)	16 (55.2)	48 (76.2)	167 (70.8)
Unemployed	0 (0.0)	2 (1.9)	0 (0.0)	0 (0.0)	2 (0.9)
Hospital location, n (%)					
North	8 (20.5)	29 (27.6)	9 (31.0)	19 (30.2)	65 (27.5)
Center	26 (66.7)	58 (55.2)	16 (55.2)	30 (47.6)	130 (55.1)
South	5 (12.8)	18 (17.1)	4 (13.8)	14 (22.2)	41 (17.4)

ASCT, autologous stem-cell transplantation.
[a]Difference in mean age among the four disease situations (bootstrap ANOVA): $P < .0001$.
[b]Includes cardiovascular, ophthalmologic, neurological and thrombotic-coagulative complications.
[c]Includes diabetes, hypertension, non-hematological malignancies and other comorbidities related to the following specialist areas: cardiology, clotting, endocrinology, gastroenterology, gynecology, nephrology, neurology, orthopedics, pneumology, psychiatry.

Petrucci et al., Tumori 2013; 99(4): 193e-202e

Descrizione del costo sociale del MM in Italia - III

Table 5 - Annual INHS health care costs and COI[a] (€ 2008 per patient)

	Asymptomatic		Symptomatic				Plateau/ Remission		Total	
			Drugs		ASCT					
	(n=39)		(n=105)		(n=29)		(n=63)		(n=236)	
INHS health care costs	Mean ± SD	(%)	Mean ± SD	(%)	Mean ± SD	(%)	Mean ± SD	(%)	Mean ± SD	(%)
Hospital admissions	-	(0.0)	499.9±1206.0	(2.3)	43,957.4±14,164.9	(74.2)	147.4±662.5	(1.8)	5663.3±15,199.4	(29.4)
Emergency room treatments	6.2±398.0	(0.7)	83.5±286.1	(0.4)	84.0±187.9	(0.1)	7.7±43.0	(0.01)	50.6±206.4	(0.3)
General practitioner visits	38.1±79.9	(4.0)	203.6±212.0	(0.9)	164.5±254.5	(0.3)	108.2±138.1	(1.3)	146.0±193.7	(0.8)
Specialist visits	86.7±88.2	(9.0)	405.1±292.7	(1.9)	253.8±175.6	(0.4)	156.6±155.3	(1.9)	267.5±257.7	(1.4)
Clinical & diagnostic tests	512.7±634.4	(53.4)	1406.0 ±1910.2	(6.5)	1526.0±3602.4	(2.6)	756.8±1407.4	(9.3)	1099.8±1975.3	(5.7)
Drugs										
Systemic corticosteroids	-	(0.0)	65.9±93.9	(0.3)	21.3±52.6	(0.04)	12.0±39.9	(0.2)	35.2±73.8	(0.2)
Biphosphonates	30.6±93.6	(3.2)	81.0±346.0	(0.4)	317.6±1474.8	(0.5)	752.2±4349.4	(9.3)	280.9±2322.5	(1.5)
Antineoplastics	-	(0.0)	8963.4±15,252.2	(41.3)	1410.5 ±3310.5	(2.4)	2.3±10.1	(0.03)	4161.9±11,090.8	(21.6)
Immunomodulators	-	(0.0)	6425.0±14,745.2	(29.6)	299.0±546.3	(0.1)	4412.2±13,627.7	(54.3)	4073.2±12,339.6	(21.1)
Antianemics	-	(0.0)	429.6±1439.4	(2.0)	-	(0.0)	131.6±1026.4	(1.6)	226.3±1109.3	(1.2)
Antibacterics & antivirals	-	(0.0)	33.1±130.0	(0.2)	13.3±38.5	(0.02)	1.2±9.5	(0.01)	16.7±88.9	(0.1)
Other[a]	64.71±404.09	(6.8)	142.2±616.0	(0.7)	27.3±60.3	(0.05)	18.0±61.5	(0.2)	82.1±446.0	(0.4)
Radiotherapy	-	(0.0)	38.5±318.7	(0.2)	24.0±129.2	(0.04)	17.9±101.2	(0.2)	24.8±223.2	(0.1)
Transfusions	-	(0.0)	12.1±36.9	(0.1)	3.6±11.4	(0.01)	2.1±13.4	(0.03)	6.4±26.3	(0.03)
Disability aids	-	(0.0)	13.6±41.9	(0.1)	10.3±36.6	(0.02)	25.9±87.4	(0.3)	14.3±55.0	(0.1)
Total INHS health care costs	739.0±992.2	(77.0)	18,802.4±19628.1	(86.6)	48,112.5±15,533.6	(81.2)	6551.9±14,350.8	(80.6)	16,148.8±21144.0	(83.8)
Patients and their family costs										
Out-of-pocket										
Hospital admission	-	(0.0)	-	(0.0)	-	(0.0)	47.6±378.0	(0.6)	12.7±195.3	(0.1)
Specialist visits	13.3±58.3	(1.4)	65.1±194.6	(0.3)	31.7±119.7	(0.1)	180.6±1085.7	(2.2)	83.3±577.8	(0.4)
Clinical & diagnostic tests	12.9±51.2	(1.3)	14.2±61.0	(0.1)	2.1±11.1	(0.003)	8.4±33.6	(0.1)	10.9±49.0	(0.1)
Disability aids	-	(0.0)	37.3±102.3	(0.2	50.8±99.4	(0.1)	21.3±97.2	(0.3)	28.5±92.4	(0.2)
Transport by ambulance	-	(0.0)	10.9±63.8	(0.1)	109.5±500.8	(0.2)	18.1±144.0	(0.2)	23.1±195.6	(0.1)
Other transport	116.3±198.5	(12.1)	582.7±1042.9	(2.7)	383.5±577.6	(0.7)	197.7±447.4	(2.4)	378.4±786.8	(2.0)
Overnight stay	2.1±12.8	(0.2)	-	(0.0)	-	(0.0)	7.6±51.2	(0.1)	2.4±27.0	(0.01)
Paid care[b]	2.6±16.0	(0.3)	94.6±370.1	(0.4)	-	(0.0)	87.5±306.4	(1.1)	65.9±295.2	(0.3)
Productivity losses										
Working days lost by patient	21.9±95.3	(2.3)	1394.2±7905.8	(6.4)	9538.3±17,612.4	(16.1)	762.6±4230.9	(9.4)	1999.6±8808.0	(10.4)
Working hours lost by caregiver for informal care	51.3±147.7	(5.4)	706.4±1804.2	(3.3)	1015.4±2100.1	(1.7)	247.2±572.9	(3.0)	513.5±1469.1	(2.7)
Total patients and their family costs	220.3±319.2	(23.0)	2905.4± 8176.6	(13.4)	11,121.2±18809.2	(18.8)	1578.8±5443.2	(19.4)	3118.2±9462.9	(16.2)
Total COI*	959.3±1091.6	(100.0)	21,707.8±21,785.3	(100.0)	59,243.7±24,214.0	(100.0)	8130.7±15,092.5	(100.0)	19,267.1 ±25,078.6	(100.0)

ASCT, autologous stem-cell transplantation; COI, cost of illness; INHS, Italian National Health Service.
[a]Includes allopurinole, analgesics, antihemorrhagics, antihistamines, antihyperthensives, antiulcer agents, fluconazole, phenobarbital, psychotonics, statins, vitamin D3.
[b]Includes home help, nurse at home, patient's companion.
*Difference in mean COI among the four disease situations (bootstrap ANOVA), $P < 0.0001$.

Petrucci et al., Tumori 2013; 99(4): 193e-202e

Descrizione del costo sociale del MM in Italia - IV

Table 6 - Multiple regression models

Dependent variable Log (costs)	Model 1 Total INHS health care costs			Model 2 Total COI		
Predictors	Coefficient	SE	*P*	Coefficient	SE	*P*
Demographic						
Gender (female=1)	0.050	0.167	0.766	0.120	0.154	.434
Age ≥65 yr	-0.278	0.179	0.122	-0.420	0.165	.011
Clinical						
Years from diagnosis ≤5	0.320	0.185	0.085	0.353	0.170	.039
Disease situation[a]						
Symptomatic drugs	1.686	0.339	<0.0001	1.538	0.311	<.0001
Symptomatic ASCT	2.763	0.451	<0.0001	2.484	0.415	<.0001
Plateau/remission	0.619	0.292	0.035	0.694	0.268	.010
Drugs (≥1)	1.352	0.260	<0.0001	1.362	0.239	<.0001
Hospitalization (≥1)	0.848	0.278	0.003	0.851	0.255	.001
Constant	5.886	0.261	<0.0001	6.294	0.240	<.0001
Number of observations	236			236		
R-squared	0.615			0.633		
Adjusted R-squared	0.602			0.620		
F	(8, 227) = 45.33			(8, 227) = 48.92		
P > F	<.0001			<.0001		
Heteroskedasticity chi2	1.35			3.60		
P > chi2	.245			.058		
Omitted variable bias F	(3, 224) = 1.52			(3, 224) = 1.99		
P > F	.209			.116		

ASCT, autologous stem-cell transplantation; COI, cost of illness; INHS, Italian National Health Service; SE, standard error.
[a]Reference disease situation, asymptomatic.

Petrucci et al., Tumori 2013;99(4):193e-202e

Analisi costo-efficacia - I

- "Cost effectiveness analysis [CEA] is a method for evaluating the outcomes and costs of interventions designed to improve health. The results are usually summarized in [incremental] cost-effectiveness ratios [ICER] that demonstrate the cost of achieving a unit of health effects."

Gold et al., 1996

Analisi costo-efficacia - II

Analisi incrementale: comparazione della differenza nei costi e nell'efficacia dei programmi sanitari confrontati.[1,2]

- **Costo incrementale** (ΔC) = ($Costo_A$- $Costo_B$).
- **Efficacia incrementale** (ΔE) = ($Efficacia_A$- $Efficacia_B$).
- **Rapporto costo/efficacia incrementale (*Incremental Cost-Effectiveness Ratio* — ICER)** = $\Delta C/\Delta E$.

1.Gold et al., 1996
2.Drummond et al., 2015

Analisi costo-efficacia - III

- **Indicatori di efficacia incrementale** più diffusi in letteratura:
  - anni di vita salvati (*Life-year saved* – LYS);
  - anni di vita salvati corretti per la qualità di vita sperimentata dal paziente relativamente al proprio stato di salute (*Quality-adjusted life years* – QALYs).
- Qualità di vita sperimentata dal paziente = utilità.

1.Gold et al., 1996
2.Drummond et al., 2015

Analisi costo-efficacia - IV

- Il QALY si ottiene moltiplicando il periodo di tempo in cui si manifesta l'effetto di un programma sanitario (es.: 1 anno di sopravvivenza libero da progressione di malattia) per il valore dell'utilità sperimentata dal paziente.

- L'utilità è una variabile casuale continua, di norma compresa tra 0 ed 1 (estremi inclusi), dove:
  - 0 = stato di salute percepito come pari a o peggiore della morte;
  - 1 = perfette condizioni di salute.

1.Gold et al., 1996
2.Drummond et al., 2015

CEA Plerixafor plus GCSF (PG) vs Cyclophosphamide plus GCSF (CG) in MM - I

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COMPARATIVE COST UTILITY ANALYSIS OF PLERIXAFOR PLUS GCSF VERSUS CYCLOPHOSPHAMIDE PLUS GCSF AS SALVAGE MOBILIZATION REGIMENS IN MULTIPLE MYELOMA PATIENTS

Tuffaha, H.W.[1], Hussein, A.A.[2], Abdel-Rahman, F.A.[2] [1]King Hussein Cancer Center, Amman, Jordan; [2]King Hussein Cancer Center, Amman, Jordan

Introduction: Plerixafor is a novel agent that enhances the mobilization of peripheral blood stem cells (PBSCs) in lymphoma and mul-

Results: The model showed that PG was associated with higher probability of achieving successful re-mobilization and subsequent transplant. The average total costs associated with CG and PG were $41,500 and $58,400 respectively. The estimated ICER was $52,813/QALY. (Table 1) The sensitivity analysis revealed that the ICERs ranged from $86,500 to $40,488 per QALY gained when the probability of PG success ranged from 60% to 95%.

Conclusion: This analysis showed that the use of Plerixafor plus GCSF as salvage mobilization regimen in MM patients was not cost effective compared to Cyclophosphamide plus GCSF from the perspective of our health care system. To our knowledge, this is the first study to describe a cost utility analysis of Plerixafor use in this indication.

Tuffaha et al., Biol Blood Marrow Transplant 2012;18(2):S248

CEA Plerixafor plus GCSF (PG) vs Cyclophosphamide plus GCSF (CG) in MM - II

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Table I. Analysis of Plerixafor plus GCSF (PG) versus Cyclophosphamide plus GCSF (CG)

Regimen	PG	CG	Incremental results
Probability of successful mobilization	0.80	0.27	0.53
Average total costs	$58,400	$41,500	$16,900
Life year gained	4.3	3.8	0.50
QALY	2.58	2.26	0.32
ICER ($/QALY gained) for PG			$52,813

Tuffaha et al., Biol Blood Marrow Transplant 2012;18(2):S248

CEA Plerixafor plus GCSF (PG) vs Cyclophosphamide plus GCSF (CG) in MM - III

- **Analisi di base**

Programma sanitario	Costi (US$2010)	QALYs	Δ Costi	Δ QALYs	ICER
CG	41.500	2,26	-	-	
PG	58.400	2,58	16.900	0,32	52.813*

ICER=*Incremental Cost-Effectiveness Ratio*; QALYs=*Quality-adjusted life years*.

***Ogni QALY incrementale ottenuto con PG vs CG costa al finanziatore US$ 52.2813.**

Adattato da Tuffaha et al., Biol Blood Marrow Transplant 2012;18(2):S248

Come giudicare l'accettabilità del costo per anno di vita o QALY salvato incrementale? - I

- **Proposta USA_1**:[1] US$50.000.
- **Proposta USA_2**:[2] US$50.000-US$100.000.
- **Proposta Svezia per Europa**:[3] €50.000.
- **Proposta Italia_1**:[4] €25.000-€40.000.
- **Proposta Italia_2 (farmaci oncologici):**[5] €87.330 (IC 95%: €37.024; €137.636).

1. Mark et al., N Engl J Med. 1995;332:1418-1424
2. Ubel et al., Arch Intern Med. 2003;163(14):1637-41.
3. Jönsson., Pharmacoeconomics 2004;22 Suppl 4:S5-S10
4. Fattore per AIES., Pharmacoeconomics–Italian Research Articles 2009;11:83-93
5. Martone et al., Global & Regional Health Technology Assessment 2014;1(2):31-43

Come giudicare l'accettabilità del costo per anno di vita o QALY salvato incrementale? - II

- **Altre proposte Italia:**

- media PIL pro capite su più anni (sterilizzazione fluttuazioni di breve periodo; minore impatto sulla copertura sanitaria);
- disponibilità a pagare (***Willingness-To-Pay — WTP***) per QALY incrementale (criticità statistiche e metodologiche);
- disinvestire da tecnologie sanitarie obsolete (funziona per i farmaci, anche se, in generale, i denari spesi non sono recuperabili).

Adattato da Jommi & Iorio. Pharmacoeconomics–Italian Research Articles 2012;14:3-27

Come giudicare l'accettabilità del costo per anno di vita o QALY salvato incrementale? - III

- Appraisal Committee, National Institute for Health and Clinical Excellence (NICE), UK: valori-soglia utilizzati:[1]

Importo ΔC/ΔLYS*	Accettazione programma sanitario
<UK£ 20.000	Sì
UK£ 20.000 - 30.000	Sì, ma con limitazioni
>UK£ 30.000	No, ma con alcune eccezioni esplicitamente normate[2]

* LYS: Life Years Saved (anni di vita salvati).

Recenti suggerimenti al NICE: UK£13.000 per QALY incrementale.[3]

1.http://www.nice.org.uk/media/b52/a7/tamethodsguideupdatedjune2013.pdf
2. http://www.nice.org.uk/media/88a/f2/supplementaryadvicetaceol.pdf
3. Claxton et al. Health Technol Assess 2015;19:1-504

CEA ed analisi di sensibilità probabilistica - I

- L' analisi di sensibilità probabilistica indaga l'incertezza della CEA di base mediante:

➢ curva di accettabilità del rapporto costo-efficacia incrementale (*Cost-effectiveness acceptability curve* – CEAC);

➢ frontiera di accettabilità del rapporto costo-efficacia incrementale (*Cost-effectiveness acceptability frontier* – CEAF);

➢ valore atteso dell'informazione perfetta (*Expected value of perfect information* – EVPI);

- CEAC, CEAF ed EVPI: richiedono il ricorso a procedure statistiche di ricampionamento dei costi e dell'efficacia (es.: simulazione Monte Carlo "frequentista" o bayesiana).

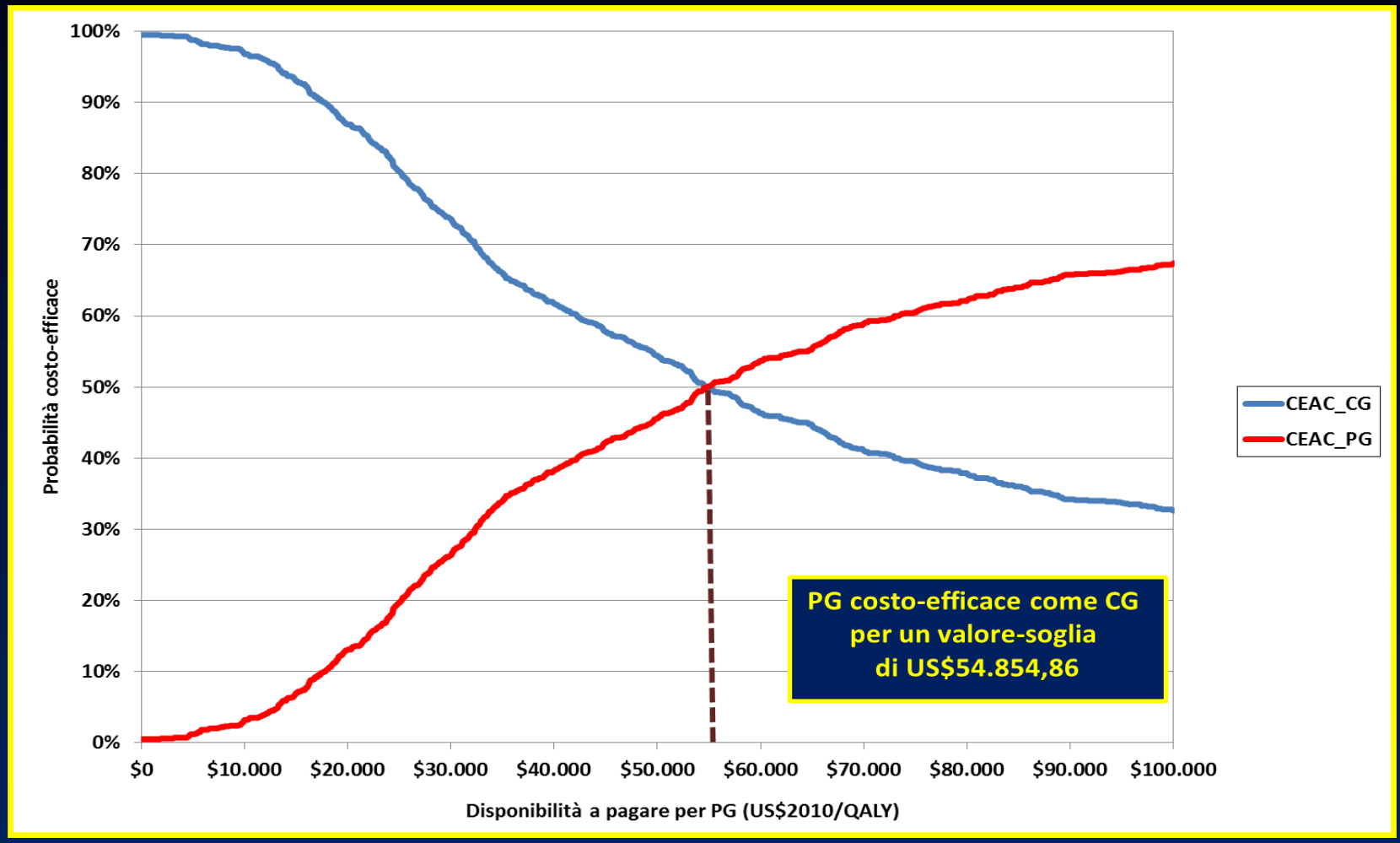
- Si tratta di strumenti richiesti/utilizzati dalle Autorità Regolatorie di Paesi che, come l'Italia, hanno adottato il Servizio Sanitario Nazionale (SSN) quale modello di erogazione di prestazioni a tutela della salute degli utenti (es.: Regno Unito; Svezia).

CEA ed analisi di sensibilità probabilistica - II

- La **CEAC** consente di **rappresentare graficamente, alla luce delle evidenze disponibili,** la **probabilità** che il programma sanitario oggetto di studio sia **costo-efficace** rispetto alle alternative, per **diversi valori-soglia di accettabilità del rapporto costo-efficacia incrementale.**
- **Razionale impiego CEAC**: **il vero valore-soglia** di accettabilità del rapporto costo-efficacia incrementale **utilizzato dal decisore è ignoto.**

CEA Plerixafor plus GCSF (PG) vs Cyclophosphamide plus GCSF (CG) in MM- III

- *Cost-effectiveness acceptability curves* - CEACs



Elaborato da Tuffaha et al., Biol Blood Marrow Transplant 2012; 18(2): S248

CEA Plerixafor plus GCSF vs GCSF alone in NHL

Am J Manag Care. 2012 January ; 18(1): 33–41.

Economic Evaluation of a Plerixafor for Stem Cell Mobilization

SM Kymes, **I Pusic**, **DL Lambert**, **M Gregory**, **KR Carson**, and **JF DiPersio**
Center for Economic Evaluation in Medicine, Washington University School of Medicine, St. Louis, MO 63110-1093, USA

Abstract

Introduction—Autologous peripheral stem cell transplantation (ASCT) with high-dose chemotherapy is a preferred treatment for relapsed non-Hodgkin's lymphoma (NHL) patients. Estimated failure rates with current stem cell mobilization (SCM) regimens are 5% to 30%. Granulocyte colony-stimulating factor (G-CSF) with plerixafor (G+P) is superior to G-CSF alone for SCM in heavily pretreated NHL patients. We conducted a cost-utility evaluation of G+P versus G-CSF as a method for SCM in patients with diffuse large B-cell lymphoma (DLBCL), the most common subtype of NHL.

Methods—A Markov model simulated the care process of DLBCL patients undergoing ASCT using data from the Washington University site of the plerixafor Phase III study. Other data and utilities were taken from the literature. Costs were Medicare allowable. Using microsimulation we estimated the incremental cost-utility ratio (ICUR) over the patient's remaining lifetime.

Results—The expected lifetime cost of providing care for DLBCL patients using G+P was $25,567 more than G-CSF, but they accumulated 1.74 more quality adjusted life years (QALYs) for an ICUR of $14,735/QALY. In sensitivity analyses this result was robust to clinically relevant changes in assumptions.

Conclusion—Using G+P for SCM in ASCT of patients with DLBCL meets accepted standards of cost-effectiveness, primarily because of its effectiveness in SCM.

Kymes et al., Am J Manag Care 2012; 18(1): 33-41

CEA ed analisi di sensibilità probabilistica - III

- La CEAF si basa sulla manipolazione algebrica del rapporto costo-efficacia incrementale definita **Beneficio Monetario Netto (*Net Monetary Benefit* – NMB)**.

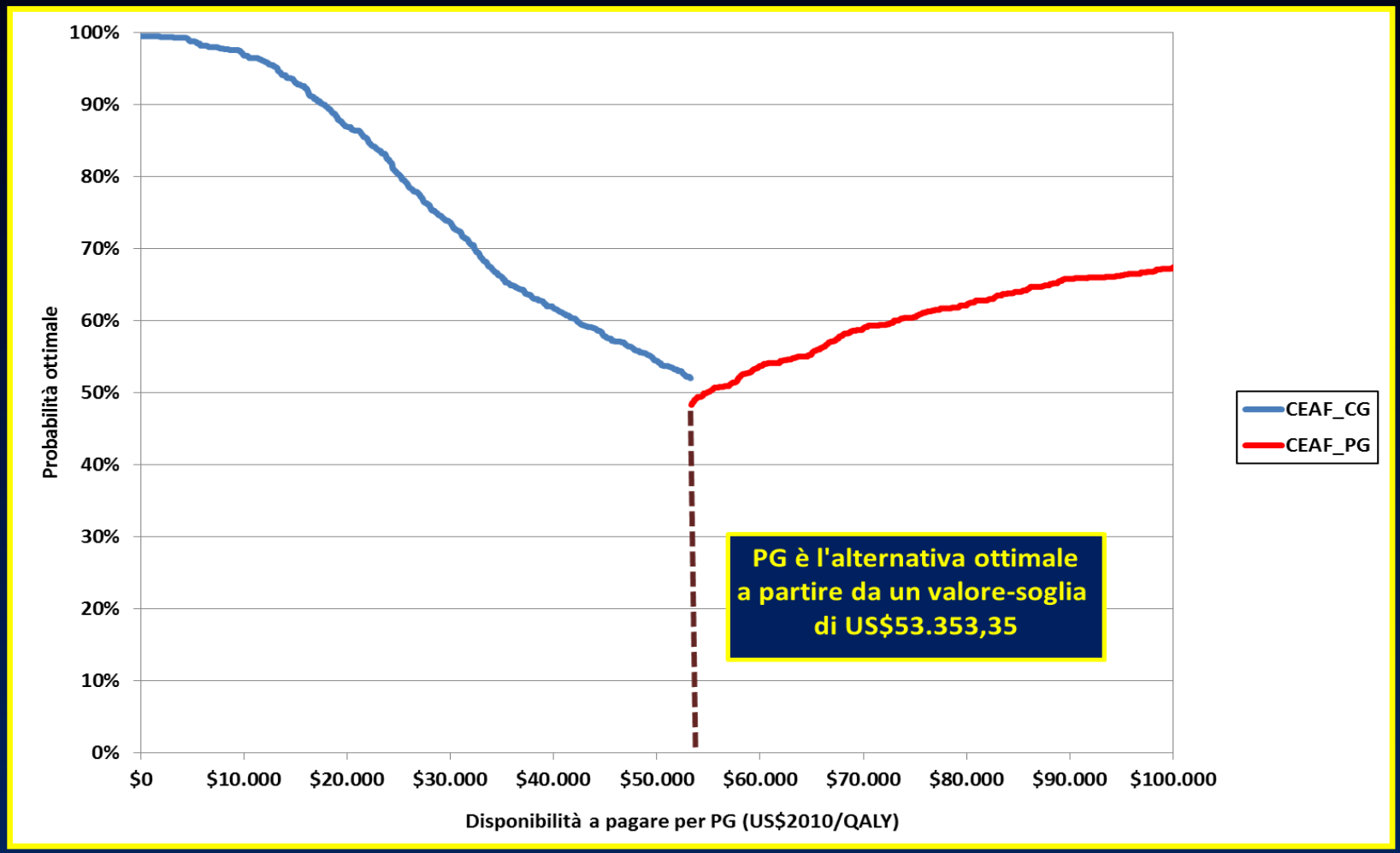
- Posto un determinato valore-soglia (λ), un programma sanitario è costo-efficace se:
  - **Rapporto costo-efficacia incrementale: ICER = ΔC/ΔE< λ**
  - **Beneficio Monetario Netto incrementale: ΔNMB = [(λ *ΔE)-ΔC]>0**

- Razionale impiego CEAF: per ciascuno dei valori-soglia selezionati dal decisore, individuare l'alternativa che ha la maggiore probabilità di essere **ottimale poiché massimizza il NMB atteso** (cioè: **ΔNMB atteso>0**).

- **CEAC e CEAF possono fornire al decisore informazioni diverse poiché indagano fenomeni diversi.**

CEA Plerixafor plus GCSF (PG) vs Cyclophosphamide plus GCSF (CG) in MM - IV

- *Cost-effectiveness acceptability frontier* - CEAF



Elaborato da Tuffaha et al., Biol Blood Marrow Transplant 2012; 18(2): S248

CEA ed analisi di sensibilità probabilistica - IV

- "Information is valuable because it reduces the expected costs of uncertainty surrounding a clinical decision".
- "The expected costs of uncertainty is determined by the probability that a treatment decision based on existing information will be wrong and the consequences if the wrong decision is made".
- "The expected costs of uncertainty can also be interpreted as the expected value of perfect information (EVPI) since perfect information (an infinite sample) can eliminate the possibility of making the wrong decision".

Claxton, Health Econ 1999;8(3):269-74

CEA ed analisi di sensibilità probabilistica - V

- La ricerca clinica è un bene pubblico puro (non rivalità né escludibilità dei potenziali beneficiari).

- Pertanto, l'EVPI decisionale deve essere calcolato per popolazione:

  - quantificando la vita attesa di una nuova tecnologia sanitaria (*Health Technology* - HT);
  - calcolando i pazienti (gli episodi) incidenti/anno che potrebbero beneficiare della nuova tecnologia sanitaria.

- L'EVPI decisionale per popolazione valuta la *potenziale* costo-efficacia di produrre nuove evidenze sulla nuova HT tramite nuova ricerca.

CEA ed analisi di sensibilità probabilistica - VI

International Journal of Epidemiology 2001;30:771–776

THEORY AND METHODS

The economics of 'more research is needed'

Carl V Phillips

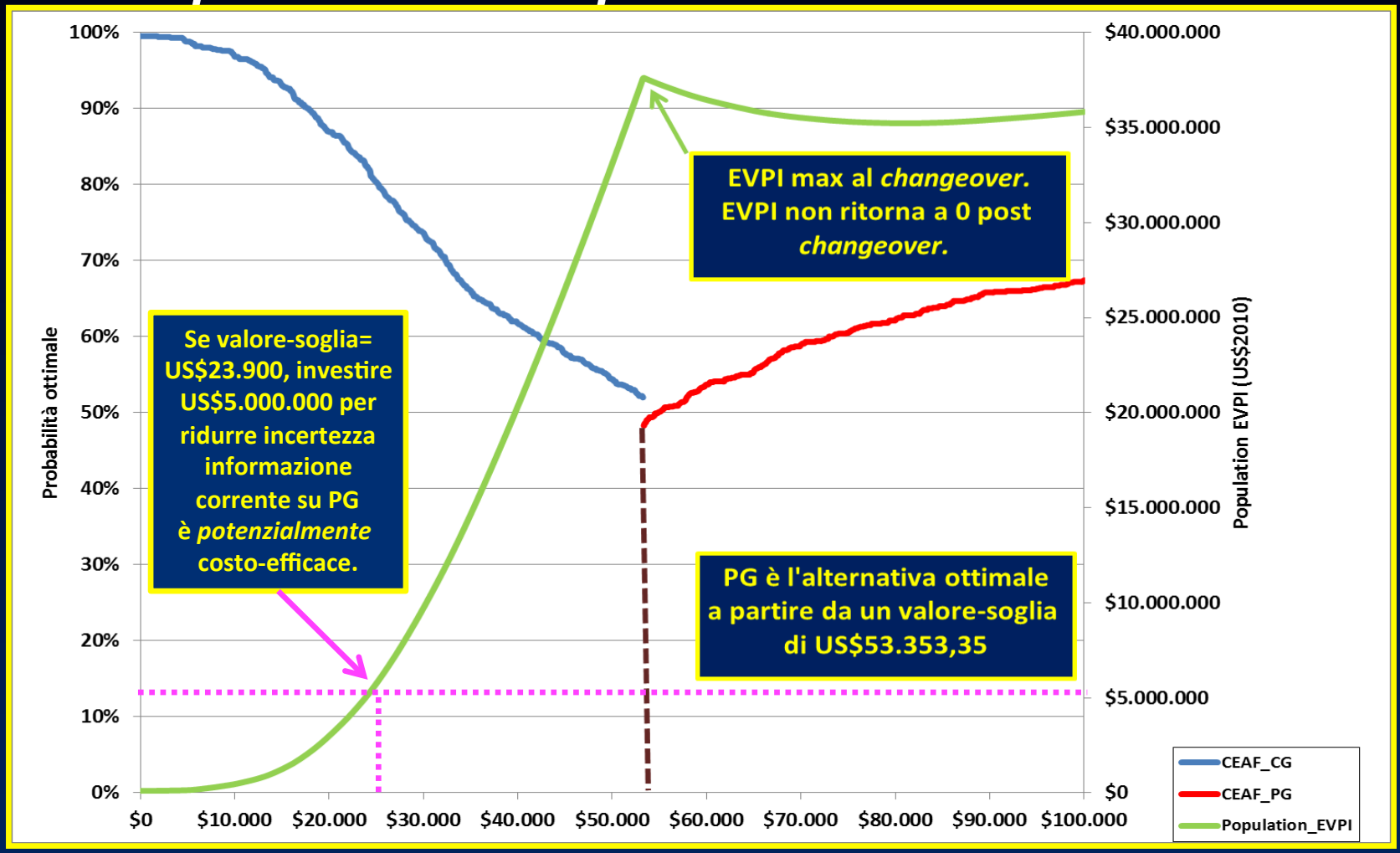
Background	Results from epidemiology and other health research affect millions of life-years and billions of dollars, and the research directly consumes millions of dollars. Yet we do little to assess the value of research projects for future policy, even amid the ubiquitous assertions that 'more research is necessary' on a given topic. This methodological proposal outlines the arguments for why and how *ex ante* assessments can inform us about the value of a particular piece of further research on a topic.
Methods	Economics and decision theory concepts—cost-benefit analysis and probability-weighted predictions of outcomes—allow us to calculate the payoff from applied health research based on resulting decisions. Starting with our probability distribution for the parameters of interest, a Monte Carlo simulation generates the distribution of outcomes from a particular new study. Each true value and outcome are associated with a policy decision, and improved decisions are valued to give us the study's contribution as applied research.
Results	The analysis demonstrates how to calculate the expected value of further research, for a simplified case, and assess whether it is really warranted. Perhaps more important, it points out what the measure of the value of a further study ought to be.
Conclusions	It is quite possible to improve our technology for assessing the value of particular pieces of further research on a topic. However, this will only happen if the need and possibility are recognized by methodologists and applied researchers.

Keywords	Epidemiology methods, cost-benefit analysis (CBA), economic valuation, uncertainty, Monte Carlo simulation
Accepted	26 June 2000

Phillips, Int J Epidemiol. 2001;30(4):771-6

CEA Plerixafor plus GCSF (PG) vs Cyclophosphamide plus GCSF (CG) in MM - V

- *Expected value of perfect information* - EVPI



Elaborato da Tuffaha et al., Biol Blood Marrow Transplant 2012; 18(2): S248

CEA Plerixafor plus GCSF (PG) vs GCSF with or without chemotherapy (CG) in lymphoma & MM

J Oncol Pharm Pract. 2014 Apr;20(2):130-6. doi: 10.1177/1078155213484785. Epub 2013 May 15.

View Full-Text Article at SAGE Publications

GCSF with or without chemotherapy compared to Plerixafor with GCSF as salvage mobilization regimen in patients with multiple myeloma and lymphoma: collection effectiveness and cost effectiveness analysis.

Abdel-Rahman F[1], Tuffaha HW, Sharma S, Jazar HA, Hussein N, Saad A, Al Rawi O, Hussein A.

Author information

Abstract

INTRODUCTION: Plerixafor is a novel mobilizing agent of peripheral blood stem cells (PBSCs) in lymphoma and multiple myeloma (MM) patients whose cells mobilize poorly. Due to the substantial cost associated with its use, we aimed to compare the effectiveness and cost effectiveness of Plerixafor + GCSF (PG) versus GCSF ± Chemotherapy (GC) as salvage mobilization regimens.

METHODS: The charts of consecutive lymphoma and MM patients who had undergone at least one previous attempt of PBSCs mobilization that failed or resulted in an insufficient cell dose for transplant between 2007 and 2010 were retrospectively reviewed. Patients identified received salvage mobilization with GC (prior to 2009) or PG after Plerixafor's FDA approval. Data collected included demographics, medical histories, apheresis yields and transplant outcome. The cost effectiveness analysis was from the perspective of the Jordanian Ministry of Health. The incremental cost effectiveness ratio (ICER) was calculated by dividing the difference in cost by the difference in effectiveness for the two regimens.

RESULTS: Five patients received GC and twelve received PG. A minimum CD34+ cell dose of 2 × 10(6) cells/kg was collected from 8 patients (67%) in the PG group compared to 3 (60%) in the GC group (p=0.79). The average costs were US$8570 and US$25,700 for the GC group and the PG group, respectively. The ICER was US$244,714 per successful stem cell collection.

CONCLUSION: Salvage Plerixafor use showed a non-significant improvement in PBSCs collection with a significant increase in cost. Prospective comparative effectiveness studies are warranted to inform the optimal salvage mobilization regimen. To our knowledge, this is the first study from the Middle East to describe the effectiveness and cost effectiveness of Plerixafor.

KEYWORDS: Plerixafor; cost effectiveness; mobilization; transplant

Adattato da Abdel-Rahman et al., J Oncol Pharm Pract 2014;20(2):130-6

CEA Plerixafor plus GCSF (PG_1 & PG_2) vs GCSF with or without chemotherapy (CG) in HL, MM, NHL - I

Biol Blood Marrow Transplant 19 (2013) 87–93

Cost-Effectiveness Analysis of a Risk-Adapted Algorithm of Plerixafor Use for Autologous Peripheral Blood Stem Cell Mobilization

Ivana N.M. Micallef [1,*], Shirshendu Sinha [2], Dennis A. Gastineau [1], Robert Wolf [3], David J. Inwards [1], Morie A. Gertz [1], Suzanne R. Hayman [1], William J. Hogan [1], Patrick B. Johnston [1], Martha Q. Lacy [1], Stephen M. Ansell [1], Francis Buadi [1], David Dingli [1], Angela Dispenzieri [1], Mark R. Litzow [1], Luis F. Porrata [1], Jeffrey L. Winters [4], Shaji Kumar [1]

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Key Words:
Plerixafor
Cost effective analysis
Risk-adapted algorithm
Stem cell mobilization

A B S T R A C T

Historically, up to 30% of patients were unable to collect adequate numbers of peripheral blood stem cells (PBSCs) for autologous stem cell transplantation (ASCT). Plerixafor in combination with granulocyte colony-stimulating factor (G-CSF) has shown superior results in mobilizing peripheral blood (PB) CD34+ cells in comparison to G-CSF alone, but its high cost limits general use. We developed and evaluated risk-adapted algorithms for optimal utilization of plerixafor. In plerixafor-1, PBSC mobilization was commenced with G-CSF alone, and if PB CD34 on day 4 or day 5 was $<10/\mu L$, plerixafor was administered in the evening, and apheresis commenced the next day. In addition, if on any day, the daily yield was $<0.5 \times 10^6$ CD34/kg, plerixafor was added. Subsequently, the algorithm was revised (plerixafor-2) with lower thresholds. If day-4 PB CD34 $<10/\mu L$ for single or $<20/\mu L$ for multiple transplantations, or day-1 yield was $<1.5 \times 10^6$ CD34/kg, or any subsequent daily yield was $<0.5 \times 10^6$ CD34/kg, plerixafor was added. Three time periods were analyzed for results and associated costs: January to December 2008 (baseline cohort; 319 mobilization attempts in 278 patients); February to November 2009 (plerixafor-1; 221 mobilization attempts in 216 patients); and December 2009 to June 2010 (plerixafor-2; 100 mobilization attempts in 98 patients). Plerixafor-2 shows a significant improvement in PB CD34 collection, increased number of patients reaching minimum and optimal goals, fewer days of apheresis, and fewer days of mobilization/collection, albeit at increased costs. In conclusion, although the earlier identification of ineffective PBSC mobilization and initiation of plerixafor (plerixafor-2) increases the per-patient costs of PBSC mobilization, failure rates, days of apheresis, and total days of mobilization/collection are lower.

Micallef et al., Biol Blood Marrow Transplant 2013;19(2):87-93

CEA Plerixafor plus GCSF (PG_1 & PG_2) vs GCSF with or without chemotherapy (CG) in HL, MM, NHL - II

Table 3
First Mobilization Results

Patient Cohorts	Baseline (n = 278)	Plerixafor-1 (n = 216)	Plerixafor-2 (n = 98)	*P* Value
CD34 collected ($\times 10^6$/kg)				
Median	5.6	6.1	7.8	
Range	0-26	0.1-28	1.6-29.3	<.001
Mean	5.9	7	8.3	
$\geq 4 \times 10^6$/kg (%)	201 (72%)	181 (84%)	91 (93%)	<.001
$\geq 2 \times 10^6$/kg (%)	226 (81%)	206 (95%)	97 (99%)	<.001
Day-1 apheresis yield	2.3 (0-26)	2.4 (0.03-28)	3.7 (0.5-29)	<.001
Mobilization failures	52 (19%)	10 (5%)	1 (1%)	<.001
Remobilizations	39 (14%)	5	1	<.001
Plerixafor use	0	82 (38%)	57 (58%)	<.001
Indication for plerixafor use				
Day-4 CD34 <10	-	9	34	
Day-4 CD34 <20	-	-	10	
Day-5 CD34 <10	-	33	-	
Day-1 apheresis yield $<1.5 \times 10^6$/kg	-	-	10	
Any apheresis yield $<0.5 \times 10^6$/kg	-	40	3	
Days of plerixafor				
Median	-	3	2	.007

Micallef et al., Biol Blood Marrow Transplant 2013;19(2):87-93

CEA Plerixafor plus GCSF (PG_1 & PG_2) vs GCSF with or without chemotherapy (CG) in HL, MM, NHL - III

Table 4
Cost Analysis

	Baseline	Plerixafor-1	Plerixafor-2	*P* Value
Patients	280	219	98	
Total cost per patient*				
Median	$12,500	$12,500	$20,000	
Minimum	$3,000	$5,000	$5,500	
Maximum	$146,750	$93,500	$89,750	.01
Mean	$17,150	$21,532	$20,617	

* Cost analysis includes remobilization costs and has been added to the original mobilization regardless of when the remobilization occurred.

Micallef et al., Biol Blood Marrow Transplant 2013;19(2):87-93

CEA Plerixafor plus GCSF (PG_1 & PG_2) vs GCSF with or without chemotherapy (CG) in HL, MM, NHL - IV

- **Analisi di base - I**

Programma sanitario	Costi (US$2008)	Mobilizza-zioni fallite (%)	Δ Costi	Δ Efficacia	ICER
CG	17.150	19%	-	-	
PG_1	21.532	5%	**PG_1 dominato in senso forte da PG_2 (PG_1 eliminato)**		
PG_2	20.617	1%			

ICER – ***Incremental Cost-Effectiveness Ratio.***

Adattato da Micallef et al., Biol Blood Marrow Transplant 2013;19(2):87-93

CEA Plerixafor plus GCSF (PG_1 & PG_2) vs GCSF with or without chemotherapy (CG) in HL, MM, NHL - V

- **Analisi di base - II**

Programma sanitario	Costi (US$2008)	Mobilizza-zioni fallite (%)	Δ Costi	Δ Efficacia	ICER
CG	17.150	19%	-	-	
PG_2	20.617	1%	3.467	18%	19.261,11*

ICER – *Incremental Cost-Effectiveness Ratio.*

***Ogni mobilizzazione riuscita ottenuta con PG_2 vs CG costa al finanziatore US$ 19.261,11.**

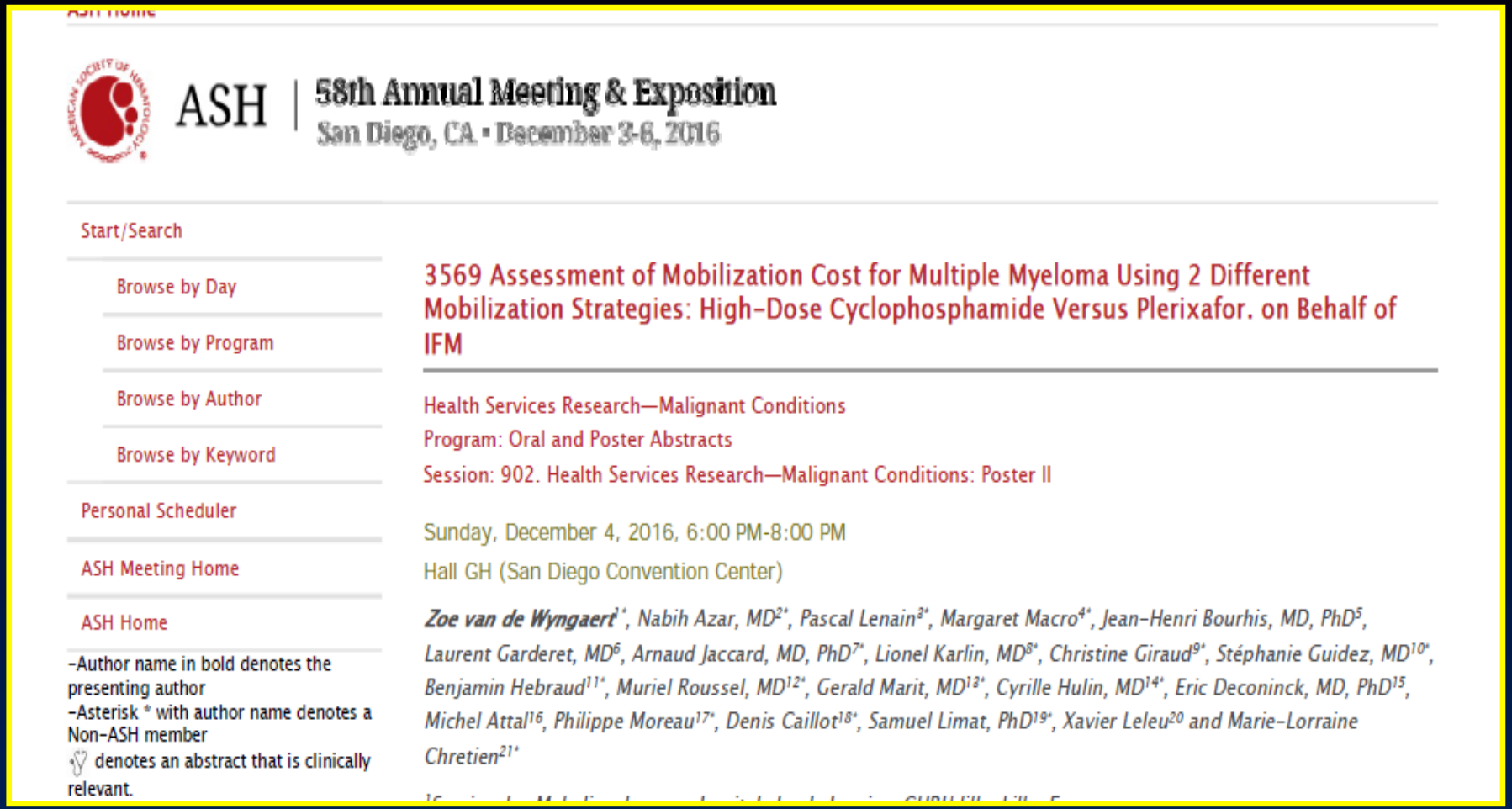
Adattato da Micallef et al., Biol Blood Marrow Transplant 2013;19(2):87-93

Analisi dei costi e delle conseguenze - I

- "[Cost-consequences analysis] may present an array of output measures alongside cost and live it to decision-makers to form their own view on the relative importance of these."

Drummond et al., 2005

Analisi dei costi e delle conseguenze - II



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denotes an abstract that is clinically relevant.

3569 Assessment of Mobilization Cost for Multiple Myeloma Using 2 Different Mobilization Strategies: High-Dose Cyclophosphamide Versus Plerixafor. on Behalf of IFM

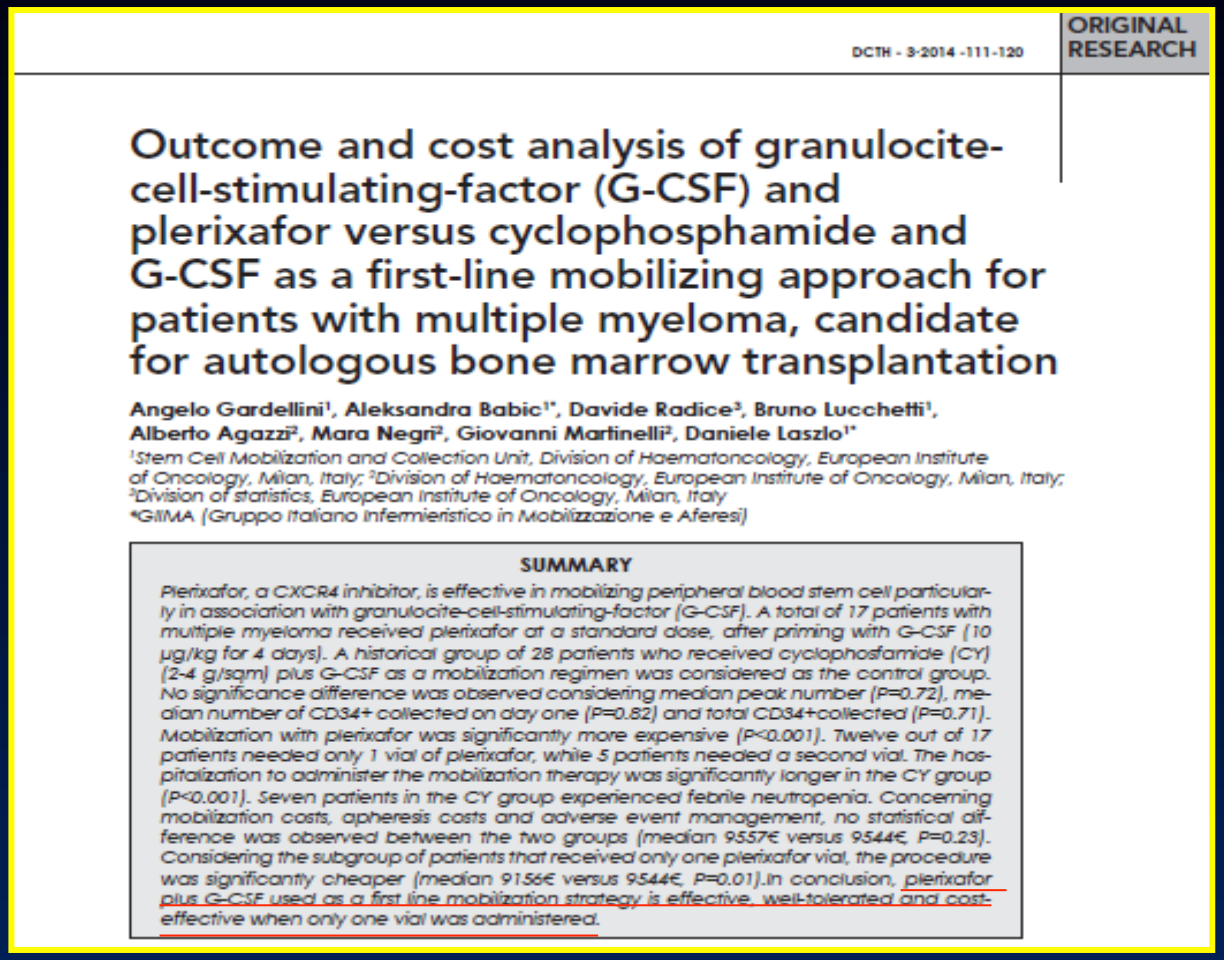
Health Services Research—Malignant Conditions
Program: Oral and Poster Abstracts
Session: 902. Health Services Research—Malignant Conditions: Poster II

Sunday, December 4, 2016, 6:00 PM-8:00 PM
Hall GH (San Diego Convention Center)

Zoe van de Wyngaert[1*], *Nabih Azar, MD*[2*], *Pascal Lenain*[3*], *Margaret Macro*[4*], *Jean-Henri Bourhis, MD, PhD*[5], *Laurent Garderet, MD*[6], *Arnaud Jaccard, MD, PhD*[7*], *Lionel Karlin, MD*[8*], *Christine Giraud*[9*], *Stéphanie Guidez, MD*[10*], *Benjamin Hebraud*[11*], *Muriel Roussel, MD*[12*], *Gerald Marit, MD*[13*], *Cyrille Hulin, MD*[14*], *Eric Deconinck, MD, PhD*[15], *Michel Attal*[16], *Philippe Moreau*[17*], *Denis Caillot*[18*], *Samuel Limat, PhD*[19*], *Xavier Leleu*[20] *and Marie-Lorraine Chretien*[21*]

van de Wyngaert et al., ASH 2016

Analisi dei costi e delle conseguenze - III



DCTH - 3-2014 -111-120

ORIGINAL RESEARCH

Outcome and cost analysis of granulocite-cell-stimulating-factor (G-CSF) and plerixafor versus cyclophosphamide and G-CSF as a first-line mobilizing approach for patients with multiple myeloma, candidate for autologous bone marrow transplantation

Angelo Gardellini[1], Aleksandra Babic[1*], Davide Radice[3], Bruno Lucchetti[1], Alberto Agazzi[2], Mara Negri[2], Giovanni Martinelli[2], Daniele Laszlo[1*]

[1]Stem Cell Mobilization and Collection Unit, Division of Haematoncology, European Institute of Oncology, Milan, Italy; [2]Division of Haematoncology, European Institute of Oncology, Milan, Italy; [3]Division of statistics, European Institute of Oncology, Milan, Italy

[*]GIIMA (Gruppo Italiano Infermieristico in Mobilizzazione e Aferesi)

SUMMARY

Plerixafor, a CXCR4 inhibitor, is effective in mobilizing peripheral blood stem cell particularly in association with granulocite-cell-stimulating-factor (G-CSF). A total of 17 patients with multiple myeloma received plerixafor at a standard dose, after priming with G-CSF (10 µg/kg for 4 days). A historical group of 28 patients who received cyclophosfamide (CY) (2-4 g/sqm) plus G-CSF as a mobilization regimen was considered as the control group. No significance difference was observed considering median peak number (P=0.72), median number of CD34+ collected on day one (P=0.82) and total CD34+collected (P=0.71). Mobilization with plerixafor was significantly more expensive (P<0.001). Twelve out of 17 patients needed only 1 vial of plerixafor, while 5 patients needed a second vial. The hospitalization to administer the mobilization therapy was significantly longer in the CY group (P<0.001). Seven patients in the CY group experienced febrile neutropenia. Concerning mobilization costs, apheresis costs and adverse event management, no statistical difference was observed between the two groups (median 9557€ versus 9544€, P=0.23). Considering the subgroup of patients that received only one plerixafor vial, the procedure was significantly cheaper (median 9156€ versus 9544€, P=0.01).In conclusion, plerixafor plus G-CSF used as a first line mobilization strategy is effective, well-tolerated and cost-effective when only one vial was administered.

Gardellini et al., DCTH 2014;3:111-20

Analisi dei costi e delle conseguenze - IV

TABLE 3 • Mobilization outcomes.

	Plerixafor + G-CSF n=17	CY + G-CSF n=28	P-value
Patient with 10 cells/μL CD34+ on day 5, #, (%)	17/17 (100)	27/28 (96)	0.3
Patient with 20 cells/μL CD34+ on day 5, #, (%)	16/17 (94)	21/28 (75)	0.1
Peak peripheral blood CD34+ cell count (cells/μL) on day 1, Mean (range)	69.6 (14-138)	98.9 (9-397)	0.72
CD34+ cells x 10^6/kg collected on day 1, median (range)	5.0 (1.2-10.0)	7.1 (0.8-22.8)	0.82
Total CD34+ cells x 10^6/kg collected, #, (range)	6.3 (3-11)	8.1 (1.6-22.8)	0.71
Patients collecting ≥2x10^6CD34 + cells/kg, # (%)	17(100)	26 (92.9)	0.52
Patients collecting ≥4x10^6CD34 + cells/kg, # (%)	12 (70.6)	17 (60.7)	0.54

G-CSF, granulocite-cell-stimulating-factor; CY, cyclophosfamide.

Gardellini et al., DCTH 2014;3:111-20

Analisi dei costi e delle conseguenze - V

TABLE 4 • Cost analysis.

Mobilization		
Plerixafor: Total plerixafor vial, median (range) G-CSF vials, #, (range)	 1 (1-2) 12 (10-17)	
CY: g of CY, median (range) g of Mesna, median (range) G-CSF vials, #, (range)	 6.8 (3.4-8.0) 6.8 (3.4-8.0) 12 (6-21)	
Hospitalization days Plerixafor group, median (range) CY group, median (range)	 2 (2-3) 3 (2-4)	P<0.001
Total cost of mobilization: Plerixafor plus G-CSF, median (range) CY plus G-CSF, median (range)	 9091€ (8690-16477) 3015€ (1432-5083)	P<0.001
Apheresis procedure Total number of apheresis procedures: Plerixafor plus G-CSF, median range CY plus G-CSF, median, range Patients requiring weekend apheresis Plerixafor plus G-CSF, # (%) CY plus G-CSF, # (%) Central venous catheter insertion Plerixafor plus G-CSF, # (%) CY plus G-CSF, # (%), # (%)	 1 (1-2) 1 (1-3) 0 2 (7) 6 (35.3) 27 (96.4)	 P=0.48 P<0.001
Total cost of mobilization: Plerixafor plus G-CSF, median (range) CY plus G-CSF, median (range)	 466 € (466-866) 2066€ (733-3132)	 P<0.001
Adverse events management CY plus G-CSF, median days of hospitalization,#, range Plerixafor plus G-CSF, median days of hospitalization,#, range	 6 (4-8) 0	
Total cost of Adverse eventsmanagement: Plerixafor plus G-CSF, median (range) CY plus G-CSF, median (range)	 0 3396 € (2796-4105)	
Total procedure cost (mobilization, apheresis, AE management) Plerixafor plus G-CSF, median (range) CY plus G-CSF, median (range)	 9557€ (9157 -17343) 9544€ (7278-9946)	 P=0.23
Total mobilization cost only 1 plerixafor vial Plerixafor plus G-CSF, median range CY plus G-CSF, median (range)	 9156 € (9157-9956) 9544€ (7178-9946)	 P=0.01

G-CSF, granulocite-cell-stimulating-factor; CY, cyclophosfamide; AE, adverse events.

Gardellini et al., DCTH 2014;3:111-20

Analisi dei costi e delle conseguenze - VI

In conclusion, plerixafor plus G-CSF used as a first line mobilization strategy is effective, well-tolerated, potentially safer than CY plus G-CSF, and cost-effective when only one vial was administered. Further randomized studies looking at G-CSF plus plerixafor *versus* CY plus G-CSF would be necessary to better understand the optimal and most cost-effective methods of mobilization and to confirm these preliminary data.

Gardellini et al., DCTH 2014;3:111-20

Analisi dei costi e delle conseguenze - VII

ORIGINAL RESEARCH

Efficacy and Cost Analysis of a Plerixafor Protocol for Peripheral Blood Stem-Cell Mobilization in Patients with Multiple Myeloma or Non-Hodgkin Lymphoma

Victoria H. Wehr, PharmD; Jill M. Comeau, PharmD, BCOP

Background: Plerixafor mobilizes stem cells for autologous stem-cell transplantation, and produces a predictable peak of CD34+ cells approximately 10 hours after administration. Despite the predictable response—resulting in target CD34+ levels in fewer apheresis sessions—the cost of plerixafor has limited its use. An institutional protocol was implemented to restrict the use of plerixafor to patients who are at risk for poor mobilization. To our knowledge, no studies have analyzed mobilization costs before and after the implementation of a risk-based plerixafor protocol.
Objectives: To determine the median number of stem-cells collection days before and after implementing a risk-based plerixafor protocol, and to analyze the cost-effectiveness of the protocol.
Methods: This single-center, retrospective study was conducted at a tertiary-care medical center. Adults with multiple myeloma or with non-Hodgkin lymphoma who underwent stem-cell collection before and after the plerixafor protocol were included. Retrospective data collected to evaluate the primary objective included mobilization regimen, CD34+ levels, number of collection and clinic/hospital days, and previous therapies. The cost data collected to evaluate secondary end points included the costs of CD34+ level testing, line placement, apheresis sessions, and mobilization medications, as well as the number of collection, and clinic and hospital days.
Results: A total of 101 patients were included in the analysis and categorized into 2 groups: preprotocol (n = 39) and postprotocol (n = 62). The median number of collection days before and after protocol implementation was 2.0 (P = .192). Using the protocol reduced plerixafor costs by 32% (pre- vs postprotocol), and decreased plerixafor use by 77%.
Conclusion: Using a risk-based protocol for plerixafor reduced medication costs and produced no significant difference in the number of collection days.

J Hematol Oncol Pharm.
2016;6(4):139-143
www.JHOPonline.com

Disclosures are at end of text

Wehr & Comeau, J Hematol Oncol Pharm 2016;3(4):139-43

Analisi dei costi e delle conseguenze - VIII

Table 2 Resource Costs

Resource	Cost, $	Source
Collection of CD34+ cells	154.02	*CPT* codes
Apheresis session	8537.40	—
Line placement (tunneled central venous catheter)	6708.84	—
Plerixafor[a]	7103.61	Pharmacy wholesaler price
Filgrastim[b]	900.82	—
Cyclophosphamide[c]	1220.81	—

[a]Price per 24-mg vial.
[b]Price per 960-µg dose (2 vials of 480 µg each).
[c]Price based on a 2500-mg dose (average of doses given).
CPT indicates *Current Procedural Terminology*.

Wehr & Comeau. J Hematol Oncol Pharm 2016;3(4):139-43

Analisi dei costi e delle conseguenze - IX

Table 4 Collection Days and Mobilization Response

Variable	Before protocol (N = 39)			After protocol (N = 62)		
Diagnosis	NHL (n = 19)	MM (n = 20)	All (n = 39)	NHL (n = 18)	MM (n = 44)	All (n = 62)
Mean no. of cells collected	5.80×10^6	9.22×10^6	8.33×10^6	6.91×10^6	7.40×10^6	7.26×10^6
Mean no. of collection days	2.10	2.35	2.23	2.00	1.80	1.85
Median daily dose						
Plerixafor, mg/kg	0.26	0.31	0.29	0.25	0.25	0.25
G-CSF, µg/kg	10.99	10.41	10.70	10.18	10.48	10.33
Dose-adjusted plerixafor						
Renal insufficiency, CrCl ≤50 mL/min	1	1	2	5	6	11

CrCl indicates creatinine clearance; G-CSF, granulocyte colony-stimulating factor; MM, multiple myeloma; NHL, non-Hodgkin lymphoma.

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Analisi dei costi e delle conseguenze - X

Risk-based use of plerixafor did not lengthen the duration of stem-cell collection. Although the use of the protocol had no significant effect on the median number of collection days, it did reduce the administration and cost of plerixafor.

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Alcune ipotesi di ricerca per contestualizzazione CEA al caso italiano

- Prospettiva analisi: SSN e/o collettività.
- Studi empirici o modellistici (modelli di Markov).
- Dati di *gross-costing* da letteratura (tariffe per risorse sanitarie diverse dal farmaco) e casistica clinica strutture ospedaliere (prospettiva del SSN).
- Dati di *micro-costing* e casistica clinica strutture ospedaliere (prospettiva della collettività).
- Dati di utilità empirici o da letteratura.

Conclusioni

- Profilo di costo-efficacia di Plerixafor: richiede approfondimenti (ma risultati attuali parzialmente positivi per mobilizzazione positiva).
- Necessario costruire un valore-soglia per mobilizzazione positiva (implicazioni di razionamento diverse da costo per anno di vita salvato).
- Definire ruolo determinazione di impatto budgetario a livello nazionale e, soprattutto, locale.

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