

SICUREZZA ED EFFICACIA DELL'USO DI FILGRASTIM NELLA MOBILIZZAZIONE DELLE CELLULE CD34+ IN QUATTRO CASI DI PAZIENTI CON ANEMIA FALCIFORME CANDIDATI A GENOME EDITING

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	Full Analysis Set ^a N = 46
Number of mobilization cycles, median (range)	2.0 (1.00, 6.00)
Exa-cel dose: 10 ⁶ x CD34 ⁺ cells/kg, mean (range)	4.7 (2.9, 14.4)
Duration (months) of follow-up after exa-cel infusion ^b , mean (range)	28.2 (8.2, 57.4)



Patient	Gender	Age (years)	Genotype	Beta mutation (if any)	Hydroxyurea (HU)	HU dose (mg/kg)	Transfusion support	Chronic complications	VOCs/year	Concomitant medications
Patient 1	Male	30	HbS/ β^+	IVS2-745	Yes	20	Chronic EEX every 3 months	Silent stroke, vertebral infarcts (L3-L5), osteopenia	2	N/A
Patient 2	Male	24	HbSS	N/A	Yes	30	Extemporany EEX	Avascular necrosis (femoral head), ischemic retinopathy, benign paroxysmal vertigo in left canalolithiasis	5	Ketorolac, paracetamol, morphine
Patient 3	Male	21	HbSS	N/A	No	N/A	Chronic EEX every 2-3 weeks	N/A	6	N/A
Patient 4	Female	32	HbSS	N/A	No	N/A	Chronic EEX every 2-3 weeks	N/A	12	N/A

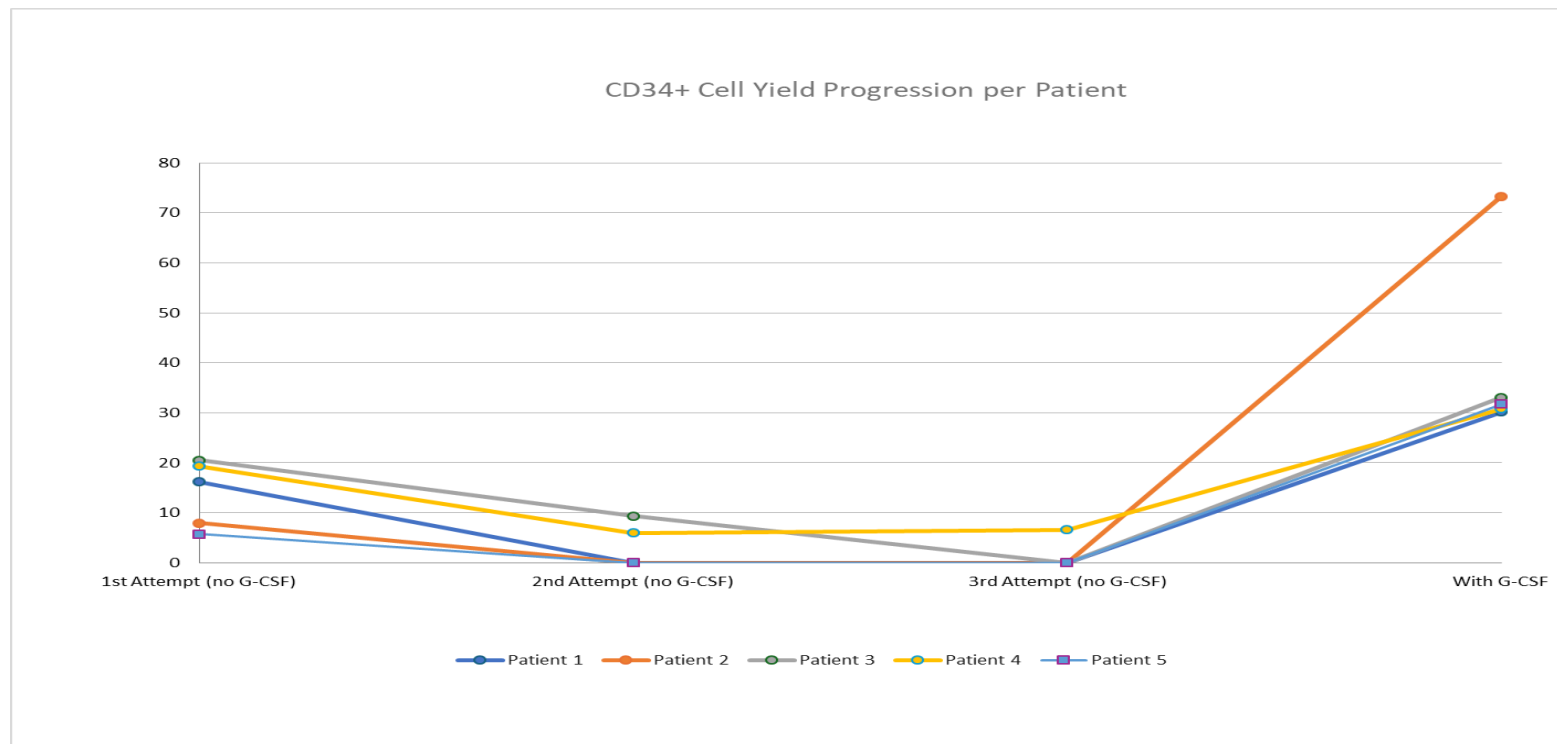
Caratteristiche basali e storia del trattamento dei quattro pazienti

Strategie di pre-mobilizzazione (EEX) e antitrombotiche

Patient	Mobilization	HbS pre (%)	Anticoagulant/Antiplatelet	Days since last EEX
Patient 1	1st	12,6	Enoxaparin + Heparin	3
	2nd	11,7	Enoxaparin	3
Patient 2	1st	16,89	Enoxaparin	4
	2nd	8,34	Enoxaparin	3
Patient 3	1st	7,5	Enoxaparin	3
	2nd	6,9	Enoxaparin	5
	3rd	4,7	Enoxaparin	3
Patient 4	1st	9,4	Apixaban + Enoxaparin	2
	2nd	4,7	Apixaban + Enoxaparin	4
	3rd	5,2	Apixaban + Enoxaparin	5
	4th	6,4	Apixaban + Enoxaparin	5

Patient ID	Apheresis #	Mobilization	Total days of plerixafor	Total days of G-CSF	Total CD34+ yield (x10 ⁶ /kg)	Fold Increase from last mobilization without G-CSF (%)
Patient 1	1	Plerixafor	3	N/A	16.2	N/A
	2	Plerixafor + G-CSF	2	5	30.19	1.9 (86.35%)
Patient 2	1	Plerixafor	3	N/A	7.8	N/A
	2	Plerixafor + G-CSF	2	6	73.3	9.4 (839.74%)
Patient 3	1	Plerixafor	3	N/A	20.52	N/A
	2	Plerixafor	2	N/A	9.31	N/A
	3	Plerixafor + G-CSF	2	5	33.1	3.5 (255.53%)
Patient 4	1	Plerixafor	3	N/A	19.31	N/A
	2	Plerixafor	2	N/A	6	N/A
	3	Plerixafor	2	N/A	6.59	N/A
	4	Plerixafor + G-CSF	2	5	30.8	4.7 (367.37%)

Questa serie di casi supporta la fattibilità e la sicurezza della combinazione di plerixafor e filgrastim per la mobilizzazione di CD34⁺ nella SCD



GRAZIE PER L'ATTENZIONE

