



FAMILY-DIRECTED CORD BLOOD BANKING FOR SICKLE CELL DISEASE: A 20-YEAR EXPERIENCE

On behalf of Eurocord-Monacord and the International Sickle Cell Disease Observatory



Hanadi Rafii, Françoise Bernaudin, Marina Cavazzana, Valérie Vanneau, Audrey Cras, Valérie Gauthereau, Aurélie Stanislas, Hélène Rouard, Christèle Ferry, Chantal Kenzey, Barbara Cappelli, Annalisa Ruggeri, Mariane De Montalembert, Claire Rieux, Mathieu Kuentz, Robert Girot, Jérôme Larghero, Eliane Gluckman

Background

Cord blood transplantation (CBT) from a related family member is an effective therapy for patients with Sickle Cell Disease (SCD) resulting in encouraging outcomes with similar or superior survival to adult donor transplant. Efforts to implement family-directed umbilical cord blood (UCB) banking have been developed in the past two decades for siblings requiring stem cell transplantation (SCT). **Public umbilical cord blood banks** are faced with the challenge regarding the units to be stored or to be discarded or used for other endeavors such as research

Materials and Methods

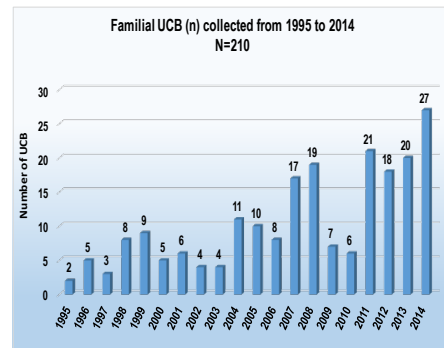
We report here our 20-year experience of family-directed UCB banking for SCD from 1995-2014.

Eligibility criteria: Mothers having a child with SCD, and expecting the birth of a sibling.

- Participation was voluntary and free of charge.
- All mothers underwent a panel of serologic donor screening assays.
- UCB units were collected in remote sites, cryopreserved and stored in a single bank.
- HLA typing on the UCB were not routinely performed unless requested by the physician.

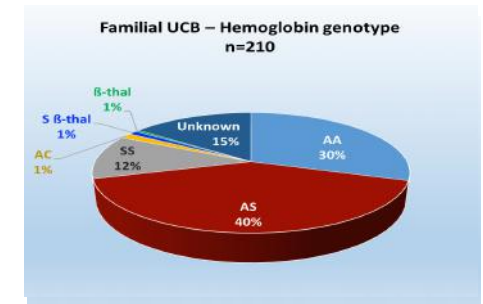
Family-directed UCB banking for SCD

UCB collection period	1995 - 2014
UCB units collected, N	210
Participating centers, N	27
Participating families, N	189
UCB units collected per family, N (%)	
1 unit	172 (91%)
2 units	13 (7%)
3 units	4 (2%)
Potential recipients per family, N	
1 affected sibling	191
2 affected siblings	12
≥3 affected siblings	2
Median age of recipient at harvest	6 (11mo-15y)



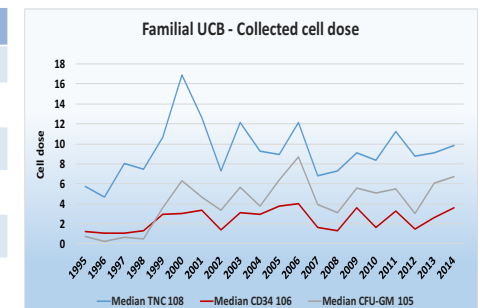
Results

- The **hemoglobin genotype** of the banked UCB units was assessed through the neonatal screening program.
- All UCBs were negative for HIV.
- 64 UCBs (30%) had positive anti-HBs and/or anti-HBc with negative HBsAg.



Characteristics of collected UCB units

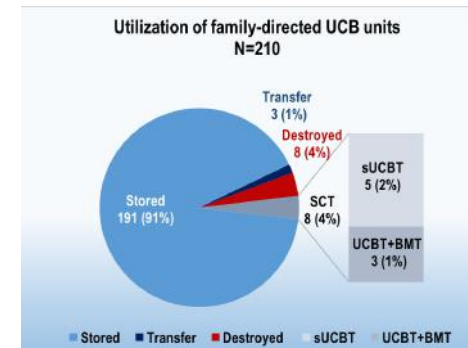
Total UCB collected, N	210
Median UCB volume collected (ml)	92.5 (33-194)
Median TNC count (x10 ⁸)	9.2
Median CD34+ cell count (x10 ⁶)	4.5
Median CFU-GM cell count (x10 ⁵)	4.5
Median cryopreservation duration	7 y (1-20)



Utilization of banked UCB units

8/210 (4%) were released for SCT:

- Median TNC count was 7.0×10^8 (3.0×10^8 - 21.8×10^8).
- Five patients were transplanted using a single UCB (sUCB)
- 3 patients with the sibling's bone marrow and UCB.
- Post-transplant data were available for 6 patients: all of them had stable engraftment of donor cells and are alive, free of SCD.



Conclusion

Our data showed that family-directed UCB banking is feasible and yields good quality cord blood units for sibling transplantation. However, the number of CBT performed is disappointing despite the good results of sibling transplantation in SCD. Therefore, we must think about the cost-effectiveness of this approach when HLA identical sibling donor is available.

No relevant conflicts of interest to disclose.