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

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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). **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 in public family-directed UCB banking for SCD from 1995-2014.

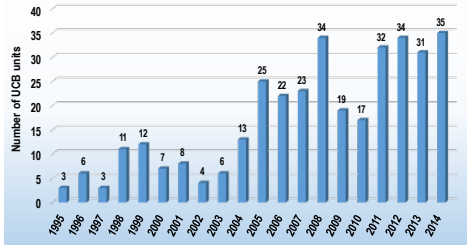
Eligibility criteria:

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

- Participation was voluntary & free of charge.
- All mothers underwent a panel of serologic donor screening assays for infectious diseases.
- UCB units were collected in remote sites, cryopreserved and stored in 2 public banks*
- HLA typing of UCB were not performed routinely unless requested by the physician.

Family-directed UCB banking for SCD	
UCB collection period	1995 - 2014
UCB units collected, N	**345**
Participating centers, N	27
Participating families, N	309
UCB units collected per family, N (%)	
1 unit	276 (80%)
2 units	27 (8%)
3 units	5 (2%)
Potential recipients per family, N (%)	
1 affected sibling	327 (95%)
2 affected siblings	12 (4%)
≥3 affected siblings	2 (2%)
Median recipient age at harvest	6 (11mo-15y)

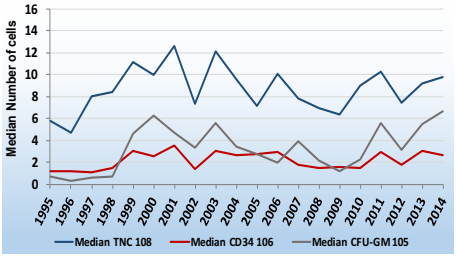
SCD - Familial UCB units (n) collected per year
N=345



Results

- The **hemoglobin genotype** of the banked UCB units was assessed through the neonatal screening program.
- All UCBs were negative for HIV.
- **HLA-typing performed for 106 (31%) UCB**
  - 43 were HLA-identical to the sibling
  - 63 were non HLA-identical

SCD - Familial UCB - Collected cell dose
N=345

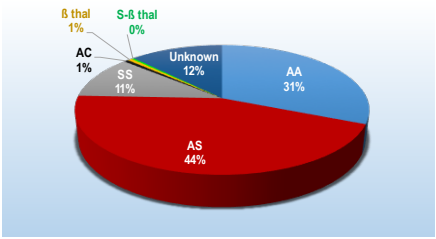


Utilization of banked UCB units

☞ **35/345 (10%) UCB were released for SCT:**

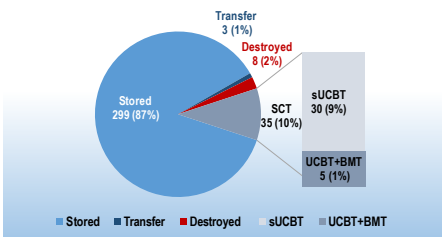
- Median TNC count was $7.0x10^8$ ($3.0x10^8$-$21.8x10^8$).
- 30 patients were transplanted using a single UCB (sUCB).
- 5 patients with the sibling's BM and UCB.
- **Post-transplant data were available for 33/35 patients:** all had stable engraftment of donor cells and are alive and free of SCD.

SCD - Familial UCB - Hb Genotype
N=345



Characteristics of collected UCB units	
Total UCB collected, N	**345**
Median UCB volume collected (ml)	90 (23-196)
Median TNC count ($x10^8$)	8.6 (0.7-75)
Median CD34+ count ($x10^6$)	2.5 (0.05-61)
Median CFU-GM count ($x10^5$)	3.5 (0.01-63)
Median cryopreservation period	7 y (1-20)

SCD - Familial UCB utilization
N=345



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 remains low despite the good results of sibling transplantation in SCD. The 10% utilization rate might increase if HLA typing was performed upon UCB collection thus allowing to early identify HLA-compatible units. Therefore, we must think about the cost-effectiveness of this approach when an HLA identical sibling donor is available. Finally, the stored UCB units with SS genotype might be used in the future for gene therapy approach.

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No relevant conflicts of interest to disclose.