Establishment of a national thematic Collection of biological samples pre- and post-Allogeneic Stem Cell Transplantation for the study of Graft-versus-Host Disease

1 INTRODUCTION

Allogeneic Hematopoietic Stem Cell Transplantation (HSCT) is the only curative treatment for many patients with hematologic diseases. Unfortunately, the transplanted immune system may react against the recipient, inducing Graft-versus-Host Disease (GvHD). GvHD is the leading cause of treatment related mortality and morbidity following HSCT and is expected to increase in the forthcoming years. Pathophysiology and long-term determinants of GvHD are still largely unknown in humans. The French Society for Stem Cell Transplantation and Cell Therapy (SFGM-TC) is the project promoter. CryoStem brought together 21 HSCT Units and 14 Biological Resources Centers that will contribute to a networked, prospective, longitudinal and standardized HSCT samples collection with concomitant well documented clinical data. CryoStem has been selected and funded to the extent of 5,5 millions € over a period of 9 years by the French Government’s Investissements d’Avenir program.

2 CRYOSTEM PARTNERSHIP

3 GENERAL ORGANISATION

4 SAMPLING PROCESS

Patient sampling is scheduled before and after HSCT, depending on the occurrence or not of acute and/or chronic GvHD. 3 types of pre-analytical products are processed and stored, according to standardized techniques: viable cells in DMSO, dried cell pellets and plasma. Samples and clinical data are anonymized and centralized in a CryoStem-dedicated web-based database application.

5 COLLECTION STATUS

On less than one year, all centers were activated. Until the end of 2014, the majority of French HSCT Units will be part of the CryoStem Network.

Evolution of inclusions since August 2012

Number of frozen aliquots as of September 1st, 2013

- 573 Patients and 245 Related Donors
- Samples: 1,210
- 13,905 Cryopreserved aliquots
  - Cells in DMSO: 5,022
  - Dried pellets: 4,681
  - Plasma: 4,202

6 CONCLUSION

National projects are the only way to acquire the high number of samples needed for statistically significant results in a reasonable time, especially in the field of rare diseases. Indeed, CryoStem expects to draw more than 10,000 cryopreserved aliquots per year.

The first project partner center is including patients since July 2012, and the others have been progressively opened on the whole territory. Currently, more than eight hundred patients and related donors have been included within 21 centers.

An embargo has been put on the collection until 2015. Then aliquots will be lay on by requests for proposal.

Aliquots quality is our main concern. Therefore the network is committed to a quality approach towards ISO 9001 certification in 2014.

CryoStem is a crucial step toward progress in GvHD research: it will allow collaborative studies between leading French and international research organizations and foster technology transfer with potential industrial players whenever possible.