Annual report 2013

Pediatric Hematology, Oncology and Stem Cell Transplantation Unit

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ABBREVIATIONS

ALL  acute lymphoblastic leukemia
AML  acute myeloid leukemia
NOPHO  Nordic Organization for Pediatric Hematology and Oncology
allogeneic  from a healthy donor
autologous  transplantation with the patient's own cells
CML  chronic myeloid leukemia
MDS  myelodysplastic syndrome
NBL  neuroblastoma
NHL  non-Hodgkin lymphoma
MUD/URD  unrelated HLA matched donor
SIB  HLA identical sibling
SAA  severe aplastic anemia
TRM  transplant-related mortality, deaths related to recurrence of the disease excluded

Cover photo (leukemic blasts) by Kim Vettenranta, October 2014
UNIT INTRODUCTION

The Pediatric Hematology, Oncology and Stem Cell Transplantation Clinic is Finland’s largest unit specialized in pediatric cancer care and hematological diseases, as well as allogeneic stem cell transplantation for children. The unit comprises the Pediatric Cancer Ward, an outpatient clinic and a day-beds unit.

The unit is responsible for the diagnosis and treatment of pediatric cancers and hematological diseases in its area. Additionally, the unit carries out all of Finland’s pediatric allogeneic bone marrow transplantations, and all the respective training as part of specialization in pediatric oncology and hematology. The unit also bears the key national responsibility for international cooperation in pediatric hematology-oncology as well as stem cell transplantation.

The divisional staff includes six consultants in pediatric hematology-oncology and stem cell transplantation, a hematology-oncology fellow, a pediatric resident as well as a pediatric anesthesiology consultant, over 60 nurses, five ward clerks as well as supportive staff including ward pharmacists, physical therapists, a rehabilitation coordinator, nutritionist, consultant in adolescent psychiatry, psychologist, social worker, preschool teacher/teacher, hospital pastor, instrument technician and ward domestics. All specialists in each department have completed a Degree in Specialist Medicine (MD) and three have a docent’s competence (equivalent to Assistant Professor) in pediatric hematology and oncology.

The hospital sees approximately 5,000 in-patient days, 1,200 day clinic visits and 1,300 procedures that require anesthesia annually. There are approximately 4,000 outpatient clinic visits and around 1,000 home visits each year.

CONVENTIONAL CANCER THERAPY

1. KEY FIGURES

![New patients 2004-13](image)

Image 1. Pediatric cancer cases treated in the unit 2004-2013. In 2013, 43 new patients were admitted, 11 with leukemia.
2. THE ACUTE LEUKEMIAS

Image 2. Treatment results in pediatric ALL (Kaplan-Meier analysis). The results employing the current NOPHO-ALL-2008 protocol remain quite favorable with the horizontal bar giving the results of the entire protocol.

Image 3. Treatment results in pediatric AML do internationally not attain the level of those in ALL. The results obtained with the most recent protocol (AML-NBH-2012) appear satisfactory, but the follow-up currently remains short. Yet, our results obtained with the previous protocol (NOPHO-AML-2004) appear to exceed those of the protocol in total (bar).
3. SOLID TUMORS


Image 5. Cumulative survival for patients treated in 2000-2013 in the four key diagnostic groups. The respective Nordic results (NOPHO) are given at the 5-year-mark with the colored bars.
STEM CELL TRANSPLANTATION

1. KEY FIGURES

A total of 420 allogeneic bone marrow transplants and 329 cases of intensive chemotherapy followed by autologous stem cell rescue have been carried out at the Children’s Hospital by the end of 2013. This brings the total number of stem cell transplantations carried out to 749. A total of 38 cord blood stem cell transplantations have been carried out.
1. ALLOGENEIC STEM CELL TRANSPLANTATION

Image 9. The diagnostic grouping of patients with allogeneic transplants in 2004-13 with the leukemias constituting up the largest single group.
3. AUTOLOGOUS STEM CELL RESCUE

Indications for autologous stem cell rescue in 2004-13


KEY DATA FOR 2013

1. A total of 5 allogeneic bone marrow grafts were harvested
2. Umbilical cord blood was harvested once
3. Autologous grafts were harvested for 10 patients, once from the marrow and 9 times from peripheral blood.
4. The engraftment (ANC > 0.5 E9/l)
   - **Allogeneic**
     - sib bm graft: median D+20 (min 10, max 27)
     - mud bm graft: median D+20.5 (min 12, max 46)
     - mud umbilical cord D+37
     - mud PBSC D+23
   - **Autologous**: median D+10 (min 10, max 13)

<table>
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<th>Age and gender of the transplantation patients</th>
<th>0-5 y.</th>
<th>6-10 y.</th>
<th>&gt; 10 y.</th>
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<tr>
<td><strong>Allogeneic</strong>: male 10, female 9</td>
<td>10</td>
<td>5</td>
<td>4</td>
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<tr>
<td><strong>Autologous</strong>: male 4, female 3</td>
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Indications for transplantation in 2014

<table>
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<tr>
<th>Diagnosis</th>
<th>Amount</th>
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<tr>
<td><strong>Allogeneic 19 transplantations</strong></td>
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<td>ALL 1. remission</td>
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<tr>
<td>ALL &gt;2. remission</td>
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<td>AML &gt;2. remission</td>
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<td>Fanconi anemia</td>
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<tr>
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</tr>
<tr>
<td>XLP2</td>
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</tr>
<tr>
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<tr>
<td>Histiocytosis</td>
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<tr>
<td><strong>Autologous 7 transplantations</strong></td>
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<tr>
<td>Brain tumor</td>
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<tr>
<td>Neuroblastoma</td>
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<tr>
<td>Rhabdomyosarcoma</td>
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</table>

STEM CELL TRANSPLANTATION RESULTS IN 2000-2013

Image 11. Cumulative survival rate of transplants for ALL by donor type
Image 12. Transplant-related mortality (TRM) among transplants for ALL by donor type.

Image 13. Cumulative survival in transplants for AML by remission status.

The experience of the Pediatric Hematology-Oncology and Stem Cell Transplantation Clinic in the treatment of childhood cancer and hematological diseases is extensive. In October 2014 we reached the 40-year mark of allogeneic stem cell transplantations launched in Helsinki, Finland and the Nordic countries in 1974. As Finland's largest pediatric center, the unit is responsible for treating approximately one-third of the national, annual total. The consultants at the Children's Hospital are intensively involved in international collaboration in the field (NOPHO, EBMT, other treatment program cooperation etc.). In stem cell transplantation the unit is among the three largest in the Nordic countries and medium-sized by European standards. The unit was the first pediatric center in the Nordic countries to receive the JACIE accreditation for stem cell transplantation and remains the only one in Finland. The use of cord blood stem cells was initiated in 1994, again as the first in the Nordic countries. With nearly 40 cord blood transplants completed, the HUCH Children's Hospital has an unparalleled experience in the field in the Nordic countries.

Our results in the treatment of pediatric ALL and AML have exceeded those obtained by other centers employing the same treatment protocols. One of our consultants leads (PI) the work to draft the new Nordic ALL treatment program (NOPHO ALL-2016) and the novel Nordic AML treatment program (NOPHO-NBH-AML-2012) has just recently been adopted.

The unit is the sole pediatric allogeneic transplant center in the country and our transplant results have been good in the treatment of ALL and excellent in AML by international standards (Images 8-12).

Our results in the treatment of lymphomas and nephroblastoma (Image 4) remains excellent. With regard to neuroblastoma we joined international SIOPEN-HR-NBL-1.5 treatment program in 2013, expected to bring about a significant improvement in treatment results.

We will continue to work in close international collaboration in the area of solid tumors in 2014-2015 by joining key European research and treatment protocols at least in lymphomas, soft tissue sarcomas, and some brain tumors, as well as in allogeneic stem cell transplantation for ALL.

We also remain determined to develop our service: one of our consultants recently went through focused training in the treatment of congenital immune deficiencies with at the Great Ormond St. Children’s Hospital in London (2013) and another will go through the same for hemoglobinopathies at the Ospedale Pediatrico Bambino Gesu in Rome in 2015. Our nurses also actively participate in national and international pediatric oncology and stem cell transplantation collaboration and training.

Extracorporeal photochemotherapy service for patients with graft-vs-host disease has been launched late 2014.

At the beginning of 2015, we will initiate the fourth two-year-long Clinical Nurse Specialist training program for pediatric hematology and oncology, as well as stem cell transplantation in cooperation with a university of applied sciences in order to maintain and develop treatment expertise.

We remain adamant in striving for state-of-the-art results in both the conventional therapy and stem cell transplantation treatment of key pediatric malignancies as well as hematological diseases, both malignant and non-malignant.
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