Children's Memorial Health Institute's experience in management of PID patients qualified for haematological stem cell transplantation

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Primary Immunodeficiencies in CMHI registry 1980 – 2006

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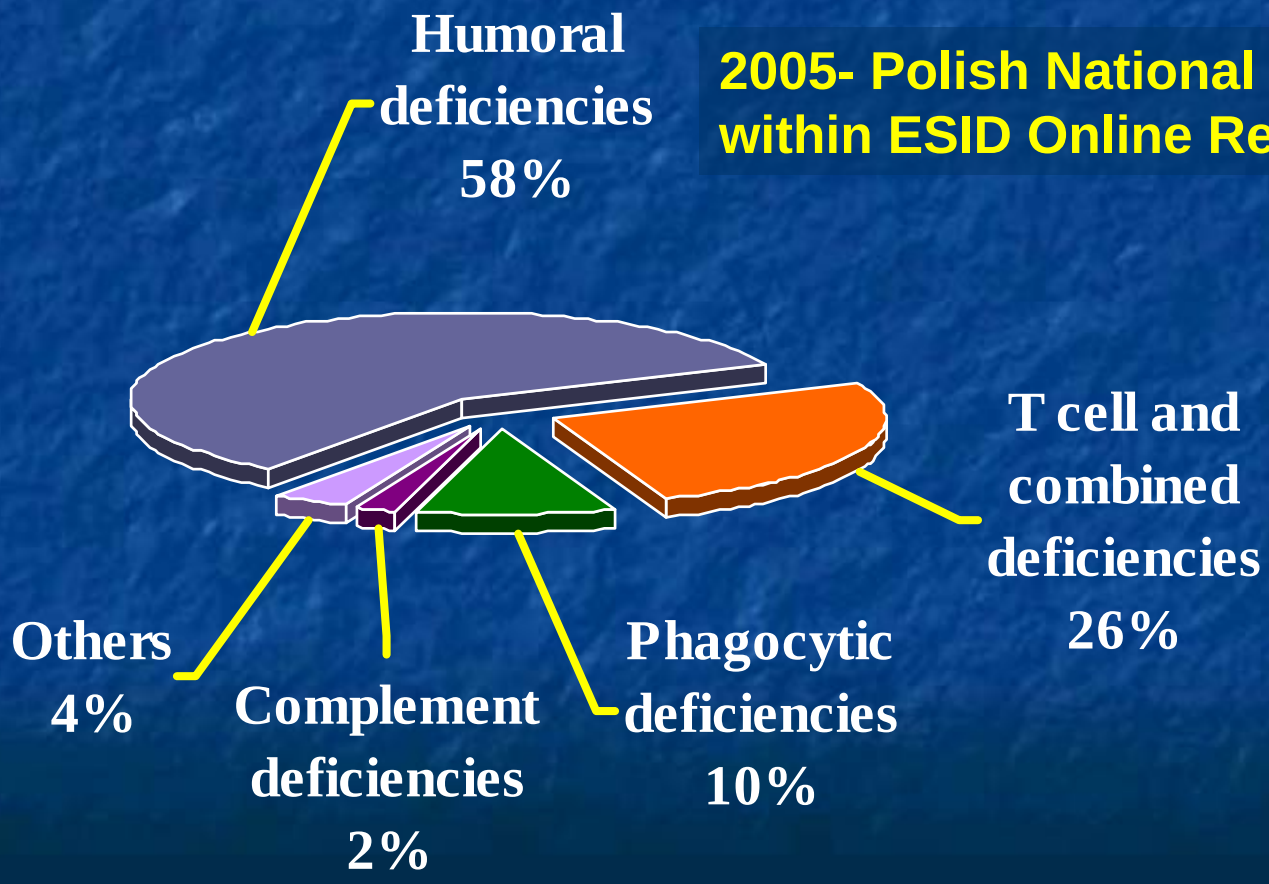


2005- Polish National Registry within ESID Online Registry

Absolute indications for HSCT:

- severe combined immunodeficiencies
- death before 2nd year of age in natural course of PID

Realtive indications for HSCT :

- increasing PID number for HSCT as alternative treatment
- individual qualification based on:
  - PID type
  - clinical course
  - HSCT donor availability
  - age of patient
  - social and psychological aspects

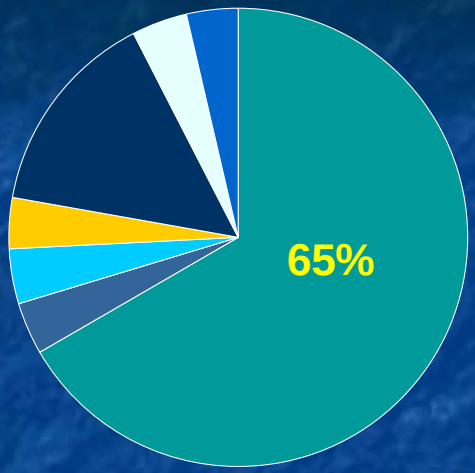
Realtive indications for HSCT

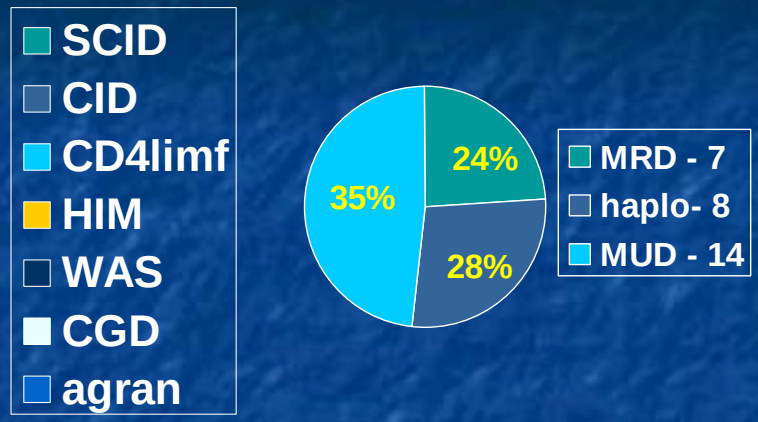
- **Wiskott-Aldrich syndrome**
  (trombocytopenia, infections, risk of malignancies)
- **Hiper IgM syndrome**
  (infections, liver insufficieniency – Cryptosporidial infection – cholangitis scleroticans, high risk of malignancy)
- **Chronic granulomatous disease**
  (infections, chronic pulmonary disease)

Others PID :..... DNA breakage disorders.....?????

- 1968 – HLA discovery
- 1968 – first bone marrow transplantation from sibling donors in SCID and WAS patients
- 1997 – first haploidentical BMT in SCID patient in Poland – BMT Unit Wroclaw

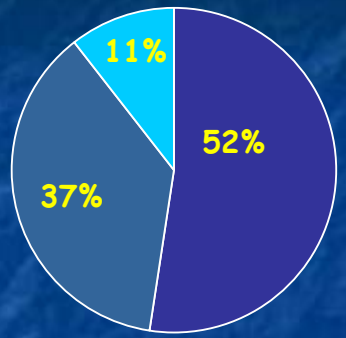
HSCT in PID's - 29 patients





PID type	Number of patients
Severe combined immunodeficiencies (SCID)	18
Combined immunodeficiency (CID)	1
Primary CD4 lymphopenia (CD4 limf)	1
Hiper IgM syndrome (HIMS)	3
Wiskott – Aldricha syndrome (WAS)	4
Chronic granulomatous disease (CGD)	1
Severe agranulocytosis	1

Age of patients on HSCT



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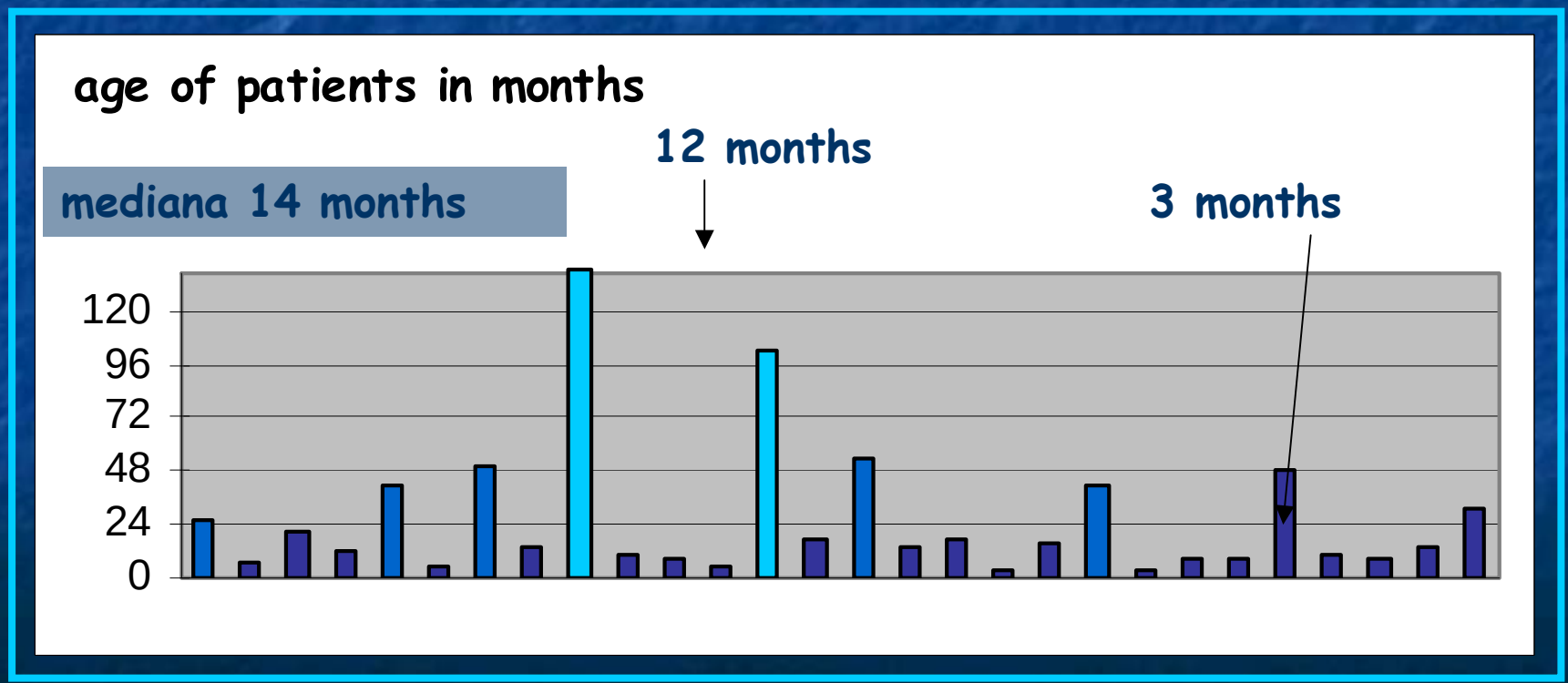


Initials	Sex	Date of birth	Year of diagnosis	Diagnosis	HSCT	Outcome
ML	F	1985-11-03	1986	SCID	No	Died
SE	F	1986-11-24	1987	SCID	No	Died
MK	F	1994-11-09	1995	SCID	No	Died
PM	**M**	**1995-07-18**	**1996**	**SCID**	**1997**	**Alive**
MJ	M	1996-04-28	1996	SCID	No	Died
EB	F	1997-03-07	1997	OS	No	Died
KK	M	1997-10-24	1998	SCID	Yes	Alive
SU	M	1997-12-25	1998	SCID	Yes	Alive
DS	M	1998-09-23	1998	SCID	Yes	Alive
MT	F	1998-11-19	1998	OS	No	Died
KK	F	1999-03-08	1999	OS	Yes	Died
MD	M	1999-07-04	2000	SCID	No	Died
LW	F	2000-03-14	2000	SCID	Yes	Alive
MK	M	2000-11-06	2001	SCID	Yes	Alive
DS	F	2001-01-25	2001	SCID	Yes	Died
SK	M	2001-04-01	2001	OS	Yes	Alive
AS	M	2001-04-16	2001	SCID	No	Died
KJ	M	2002-01-09	2002	SCID	Yes	Alive
GS	F	2002-01-08	2002	SCID	Yes	Alive
FW	M	2002-07-26	2003	SCID	Yes	Alive
MS	M	2002-12-30	2003	SCID	Yes	Alive
SD	M	2003-06-07	2003	SCID	Yes	Alive
JP	F	2004-09-13	2004	SCID	Yes	Alive
MN	F	2004-07-01	2004	OS	Yes	Alive
DW	M	2004-03-03	2004	SCID	No	Died
WW	F	2004-03-25	2004	SCID	Yes	Alive
GN	M	2005-10-21	2005	OS	Yes	Died

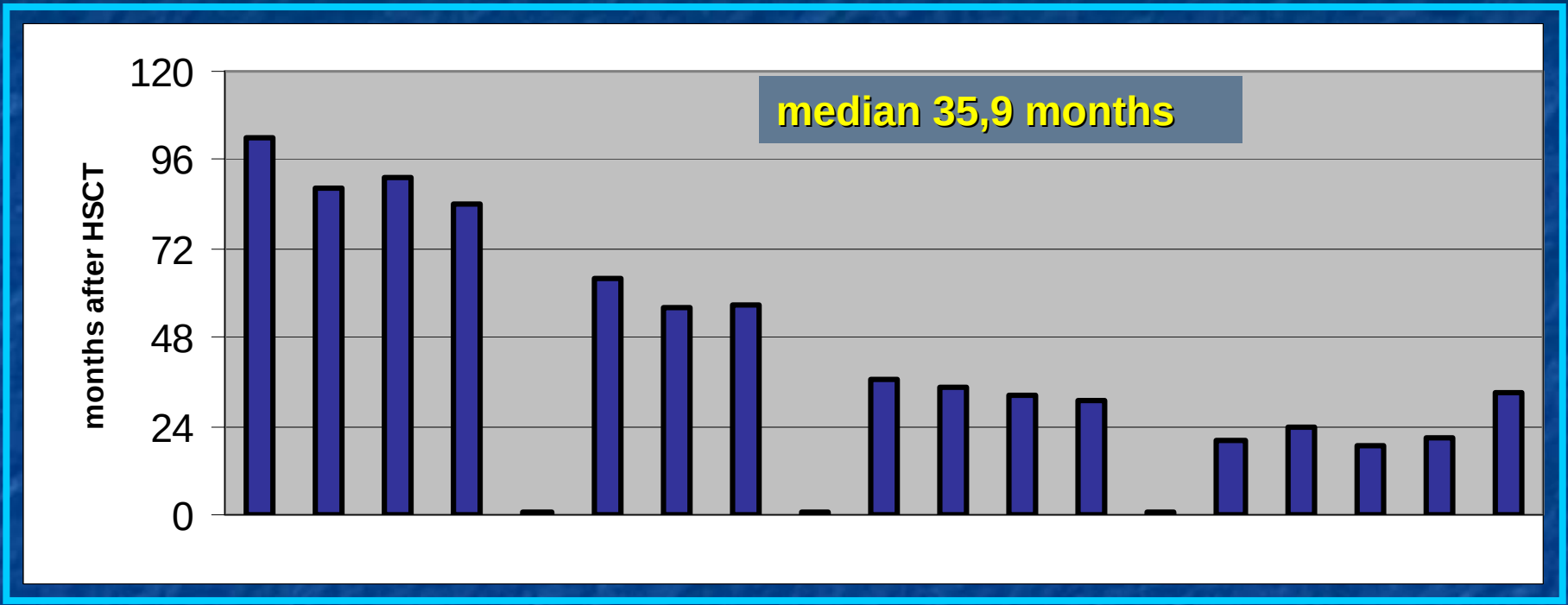
SCID

27 patients:
21 SCID / 6 OS

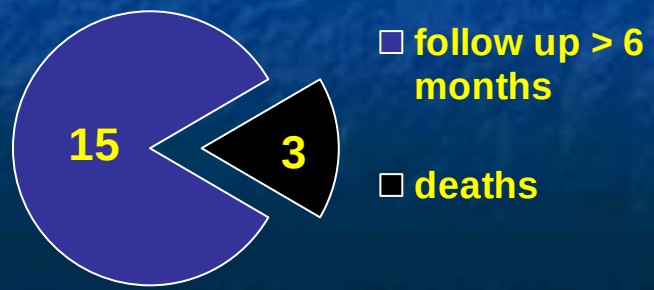
OUTCOME
9 died before HSCT
3 died after HSCT
15 alive after HSCT

Causes of deaths before HSCT:
BCG itis – 3
CMV – 3
P.carini - 1
Aspergillosis - 2

Follow up of 18 SCID patients after HSCT



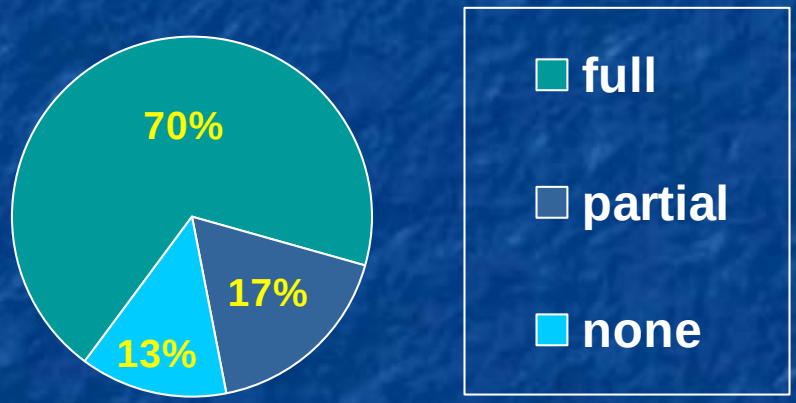
Causes of deaths	Number of patients
GvHD IVst.	1
Sepsis	1
Renal insufficiency	1



Follow up of patients other than SCID after HCST

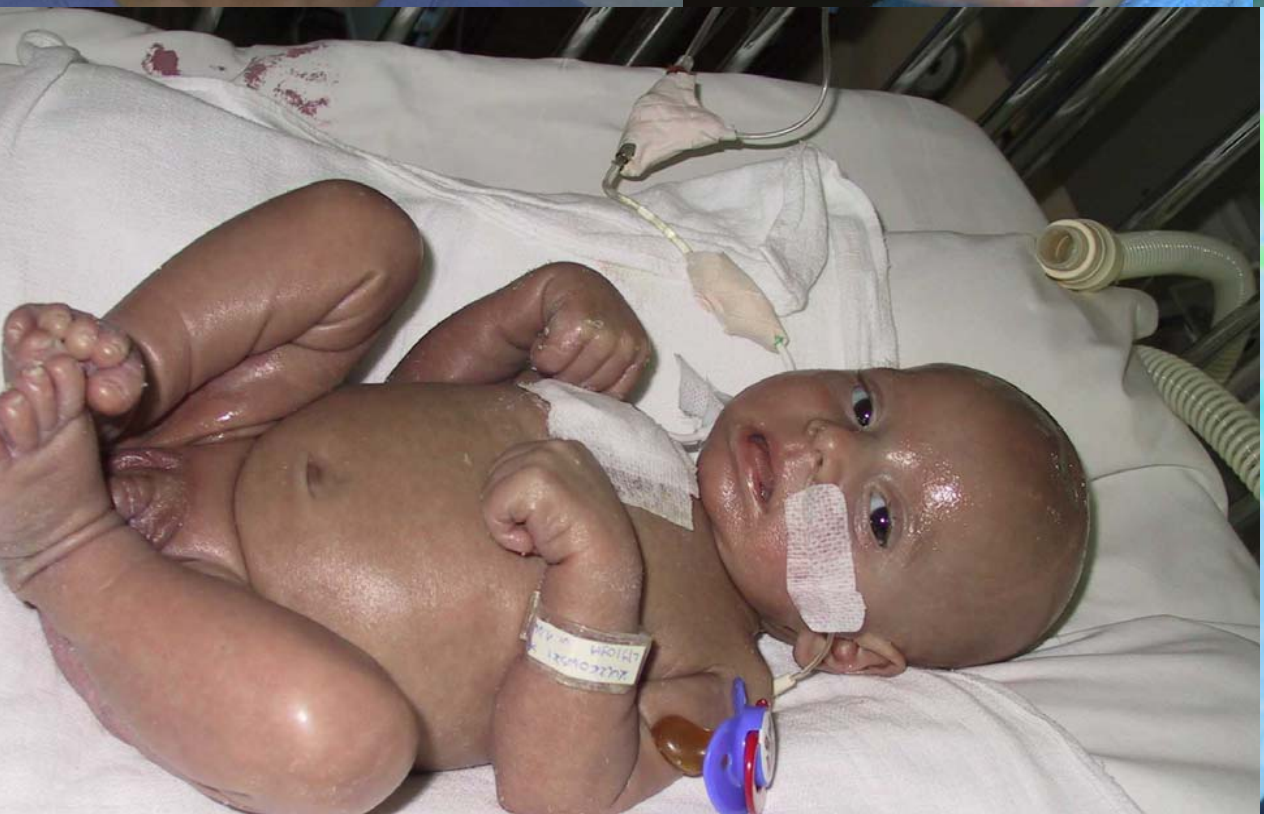
Combined immunodeficiency (CID) 1 patient	1 -died 2 weeks afer HSCT liver insufficiency
Primary CD4 lymphopenia (CD4 limf) 1 patient	1 - alive and well
Hiper IgM syndrome (HIMS) 3 patient	1 - alive and well 1 - died 2 weeks after HSCT –GvHD 1 - graft rejection, expecting second procedure
Wiskott – Aldricha syndrome (WAS) 4 patient	2 - full PID correction 1 - partial PID correction, neurological sequelae 1- gratf rejection, expecting second transplant
Chronic granulomatous disease (CGD) 1 patient	1- graft rejection
Severe agranulocytosis 1 patient	1 - died 3 weeks after HSCT – GvHD

Immunological reconstitution in 23 patients



PID correction	Number of patients	Donor chimerism		
		CC	MC	AR
full	**16**	10	6	
partial	**4**		4	
none	**3**			3




Thank you very much for your attention