



IPOPI 5TH REGIONAL ASIAN PID MEETING

24-25 MARCH 2024

TOKYO, JAPAN

an **IPOPI** event

COLLABORATION



SUPPORTED BY





WI-FI

User: **SCCC_RoomA**
Password: **solacity01**



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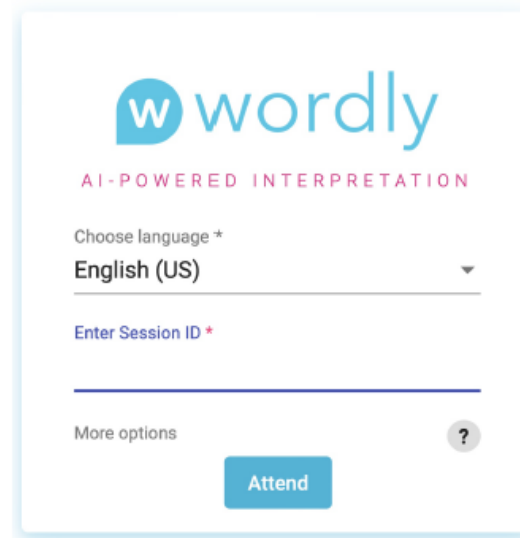
Live Translation

Step 1



Scan QR Code or Go To:
<https://attend.wordly.ai/join/KPYA-6720>

Step 2

A screenshot of the Wordly web interface. At the top is the Wordly logo (a blue circle with a white 'W' followed by the word 'wordly' in blue). Below the logo is the text 'AI-POWERED INTERPRETATION' in pink. There are two input fields: 'Choose language *' with a dropdown menu showing 'English (US)' and a downward arrow, and 'Enter Session ID *' with a text input field. Below these fields is a link 'More options' and a question mark icon. At the bottom is a blue button labeled 'Attend'.

Choose Language
Click Attend

Step 3



Read Captions on Device
Use Headset for Audio

SESSION 2 | Effective PID patient care

Moderator:

Mr Johan Prevot | IPOPI Executive Director



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Latest developments on the state of the art treatment in PIDs

Dr Pamela Lee | Hong Kong

Associate Professor, Department of Paediatrics and Adolescent Medicine. The University of Hong Kong Honorary Consultant. Queen Mary Hospital and Hong Kong Children's Hospital, HKSAR. HKU Shenzhen Hospital, Shenzhen, China



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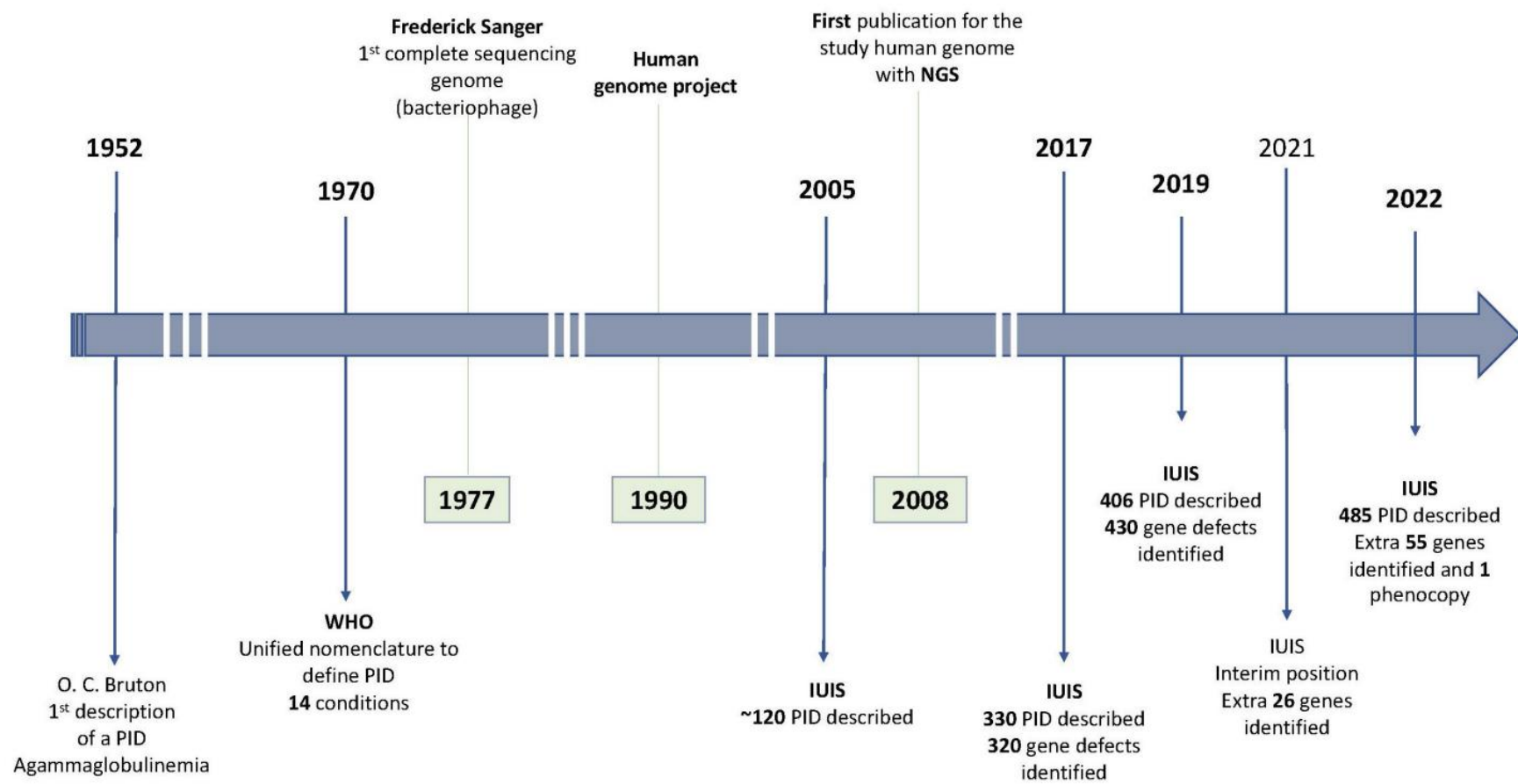


SUPPORTED BY



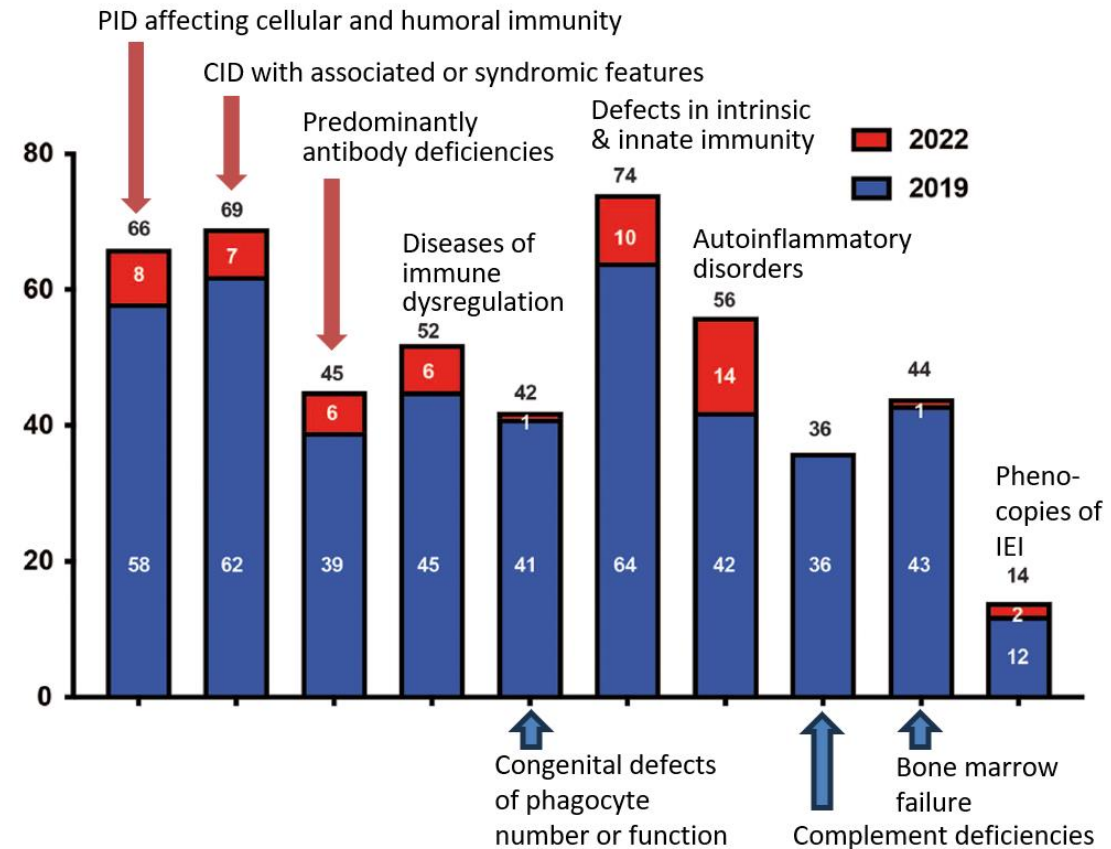
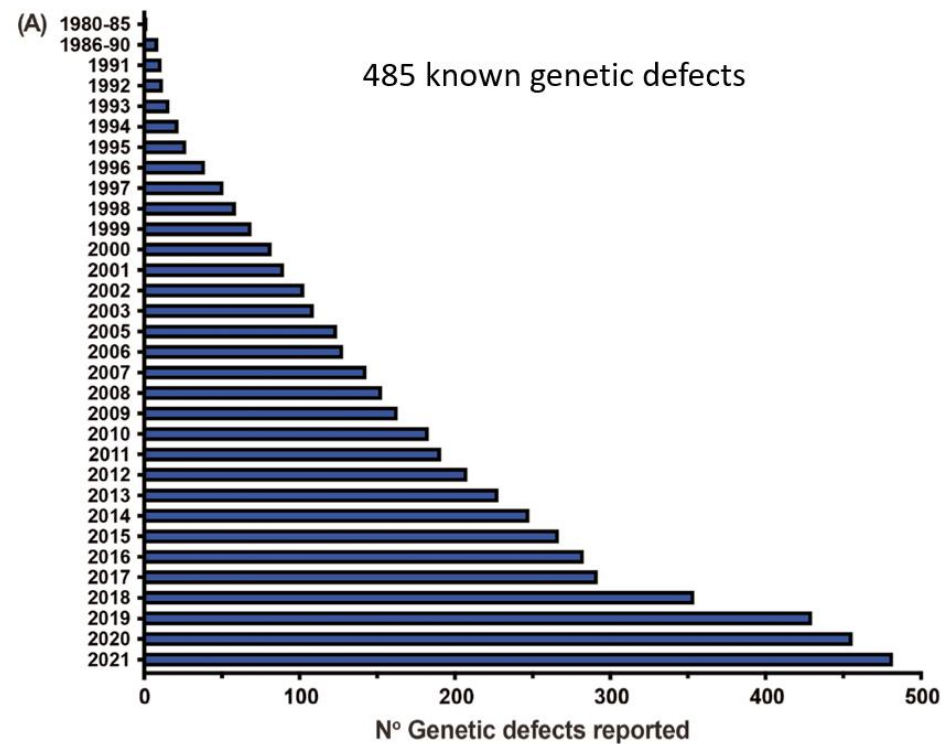
PID discovery: Chronology and Landmarks

Pinto MV and Neves JF (2022) Precision medicine: The use of tailored therapy in primary immunodeficiencies. Front. Immunol. 13:1029560.

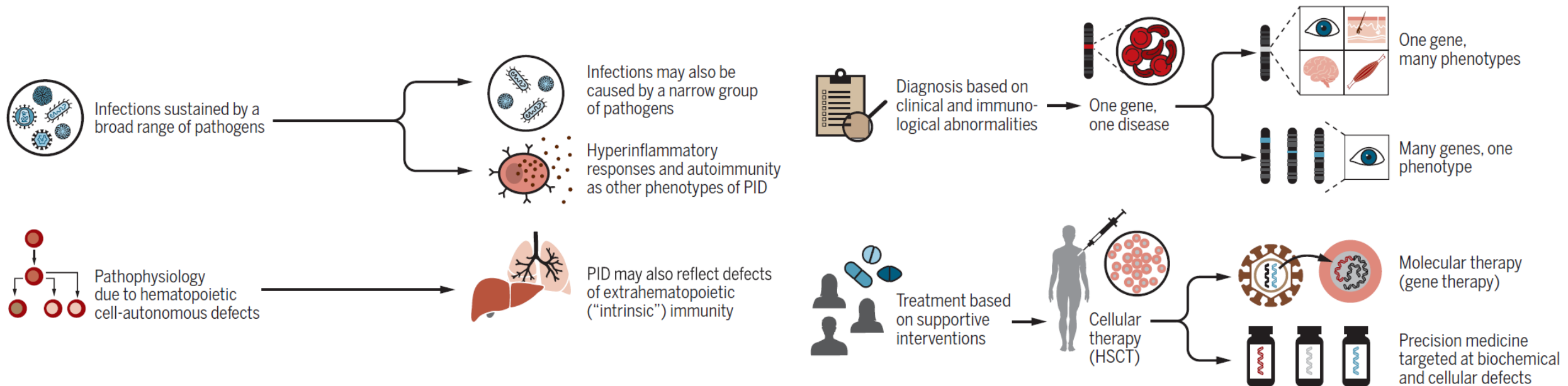


Human Inborn Errors of Immunity: 2022 Update on the Classification from the International Union of Immunological Societies Expert Committee

Stuart G. Tangye^{1,2} · Waleed Al-Herz³ · Aziz Bousfiha⁴ · Charlotte Cunningham-Rundles⁵ · Jose Luis Franco⁶ · Steven M. Holland⁷ · Christoph Klein⁸ · Tomohiro Morio⁹ · Eric Oksenhendler¹⁰ · Capucine Picard^{11,12} · Anne Puel^{13,14} · Jennifer Puck¹⁵ · Mikko R. J. Seppänen¹⁶ · Raz Somech¹⁷ · Helen C. Su⁷ · Kathleen E. Sullivan¹⁸ · Troy R. Torgerson¹⁹ · Isabelle Meyts²⁰



Evolution of clinical, pathophysiological, diagnostic, and therapeutic approach to inborn errors of immunity.



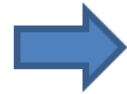
Notarangelo *et al.*, *Sci. Immunol.* **5**, eabb1662 (2020)

SCIENCE IMMUNOLOGY | REVIEW

Primary Immunodeficiencies – Principles of management

Problems

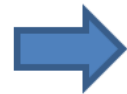
Deficient



Strategies

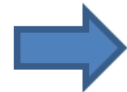
Replace

Dysregulated



Regulate

Dysfunctional

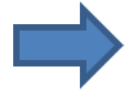


Reconstitute

Primary Immunodeficiencies – Principles of management

Problems

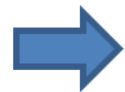
Deficient



Strategies

Replace

Dysregulated



Regulate

Dysfunctional



Reconstitute

General considerations

Protect e.g. Prophylactic antimicrobials, G-CSF, protective isolation as indicated

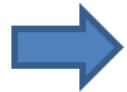
Prevent e.g. precautions against exposure, avoidance of live vaccination as indicated

Prompt action e.g. contingency plan and specific patient care pathway to facilitate prompt medical attention, aggressive Tx

Primary Immunodeficiencies – Principles of management

Problems

Deficient



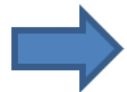
Strategies

Replace

Modalities

e.g. Immunoglobulin, cytokines

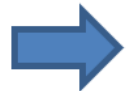
Dysregulated



Regulate

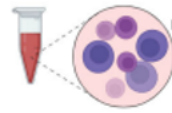
Immunomodulators e.g. corticosteroids, immunoglobulin, biologics, mTOR inhibitors, JAKINIBS

Dysfunctional



Reconstitute

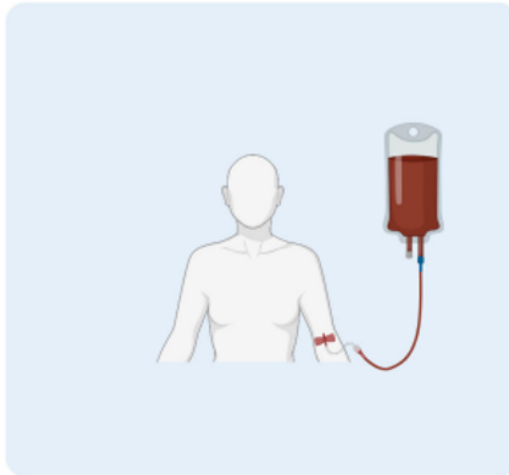
e.g. Haematopoietic stem cell transplantation, gene therapy



Target therapies in PID

Pinto MV and Neves JF (2022) Precision medicine: The use of tailored therapy in primary immunodeficiencies. Front. Immunol. 13:1029560.

HSCT



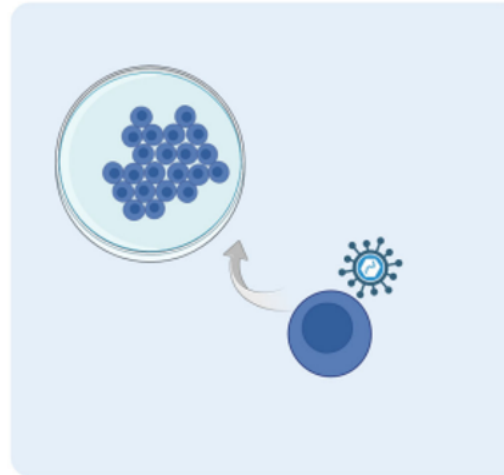
Pros:

- Curative
- Well known technique
- Experience in different conditioning regimes

Cons:

- GVHD
- Risk of adverse events (like infection) during conditioning or after transplant

Gene therapy



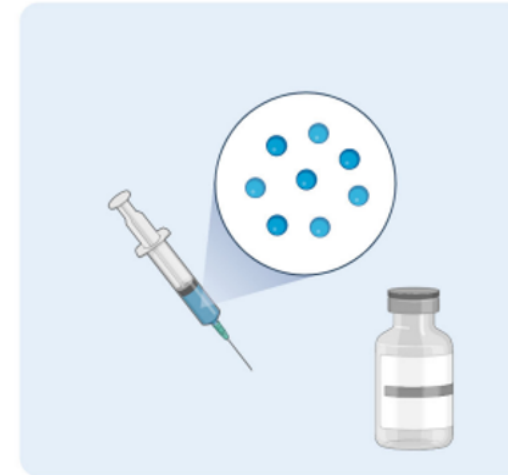
Pros:

- Potentially curative
- Not limited by donor availability
- Eliminates GVHD
- Reduced risk associated to conditioning regimes

Cons:

- Currently still not applicable to the majority of PID
- Lack or low availability
- Long term side effects not fully established

Biologic or small molecules



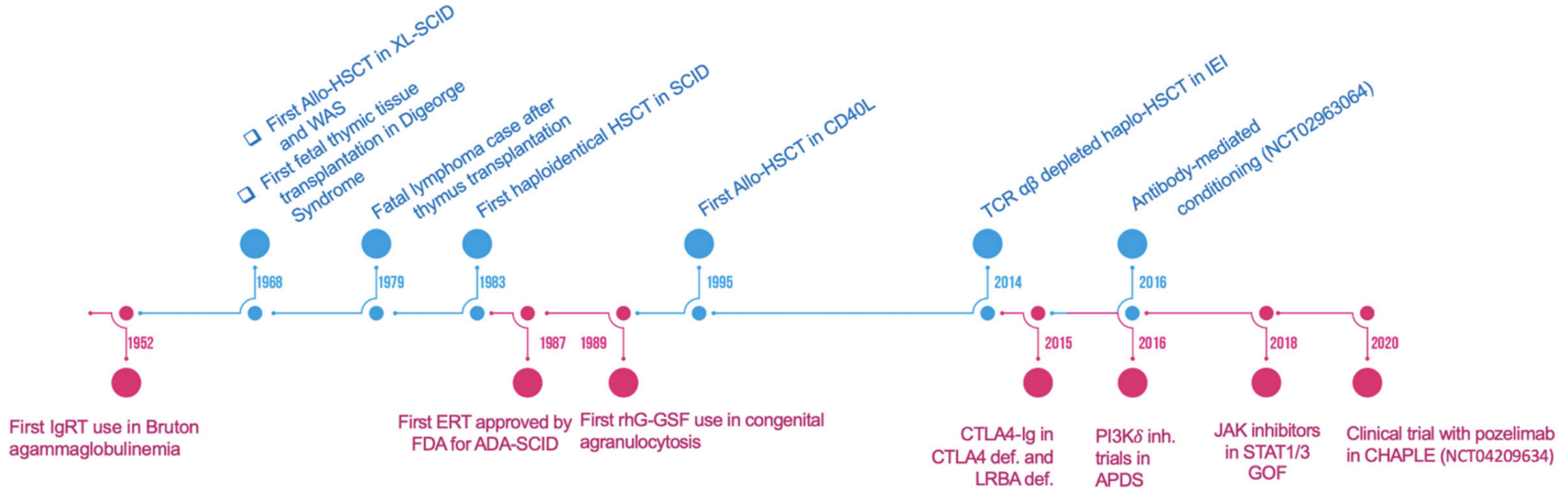
Pros:

- Target only the molecules involved
- Quicker control of disease symptoms
- Easier access in a higher number of centers
- Transitioning treatment for HSCT

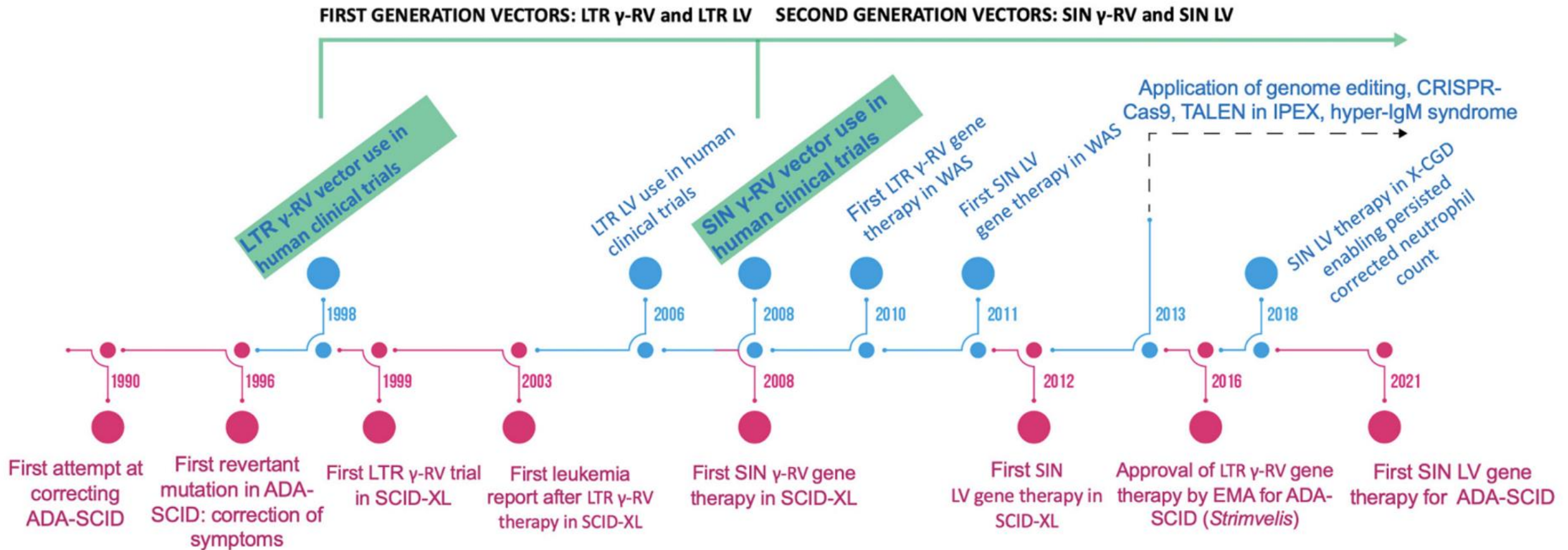
Cons:

- No definitive cure
- Long term side effects not fully established
- Risk of adverse events (like infection) during treatment

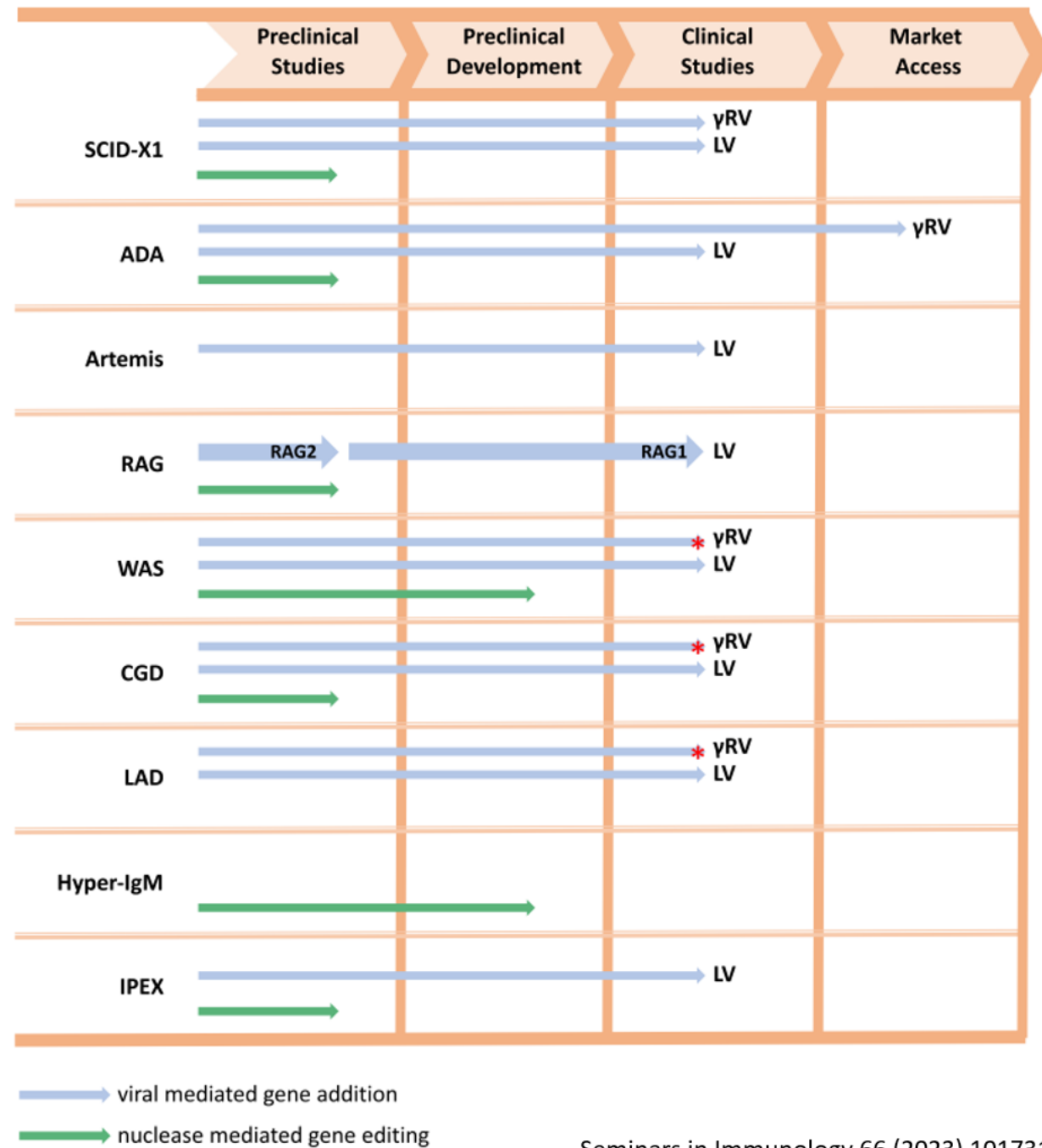
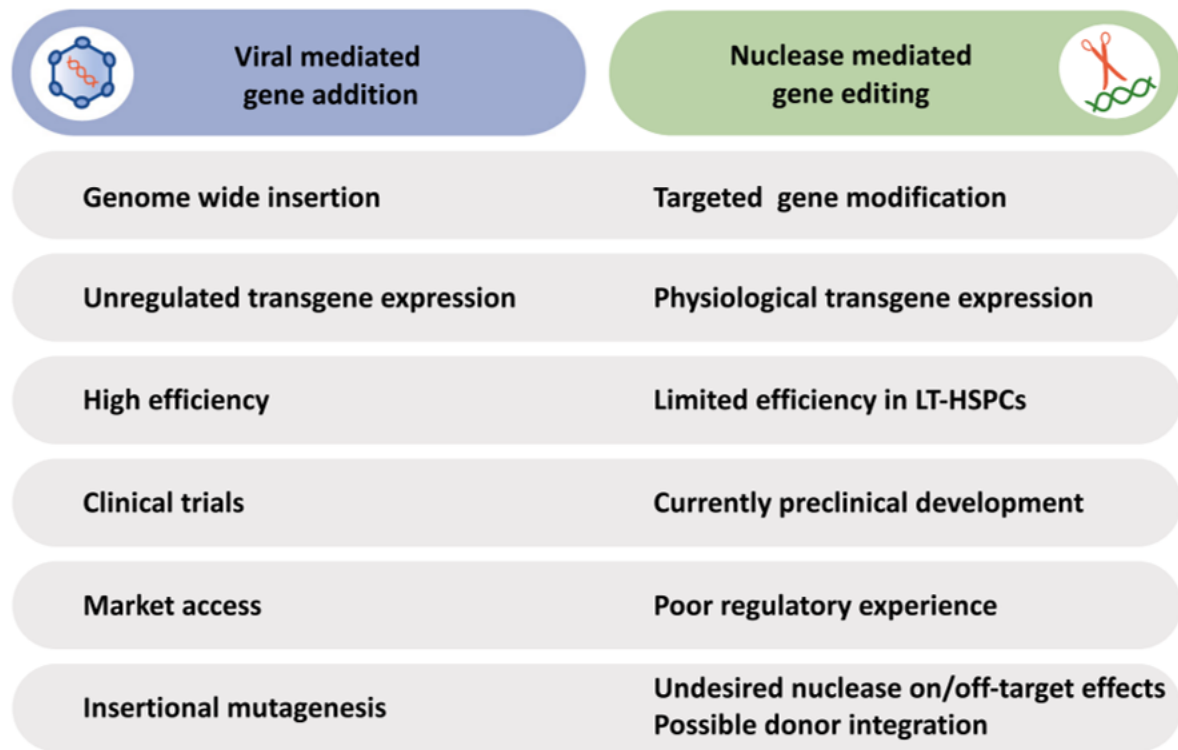
Timeline showing the major therapeutic advances in PID: Pharmacotherapy and Cellular Therapy



Timeline showing the major therapeutic advances in PID: Gene Therapy

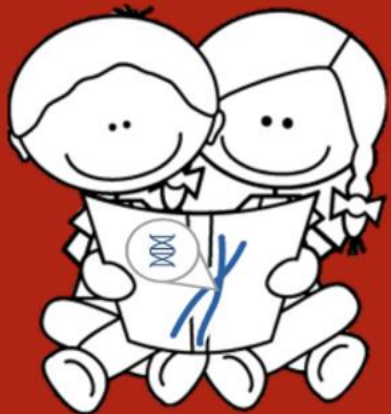


Gene addition and gene editing for PIDs: Current status



Towards Precision Medicine and a Personalized Approach to Hematopoietic Stem Cell Transplantation and Cellular Therapy for Inborn Errors of Immunity

Early diagnosis



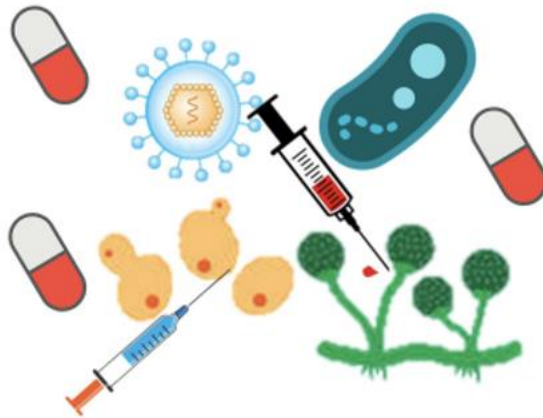
Precise molecular
diagnosis

Optimizing of disease control

Targeted therapy
Monoclonal antibody



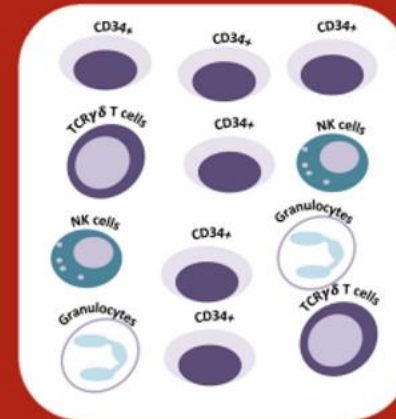
Surveillance, prophylaxis and
treatment of infection prior to
and during HSCT



Reduced toxicity
conditioning

+

Individualized dosage
optimization of conditioning
(Pharmacokinetic study)



Precise graft prescription
Graft engineering

Post transplant care

Cellular therapy to boost
immune recovery

New therapy to treat transplant-
related complications

Vaccination

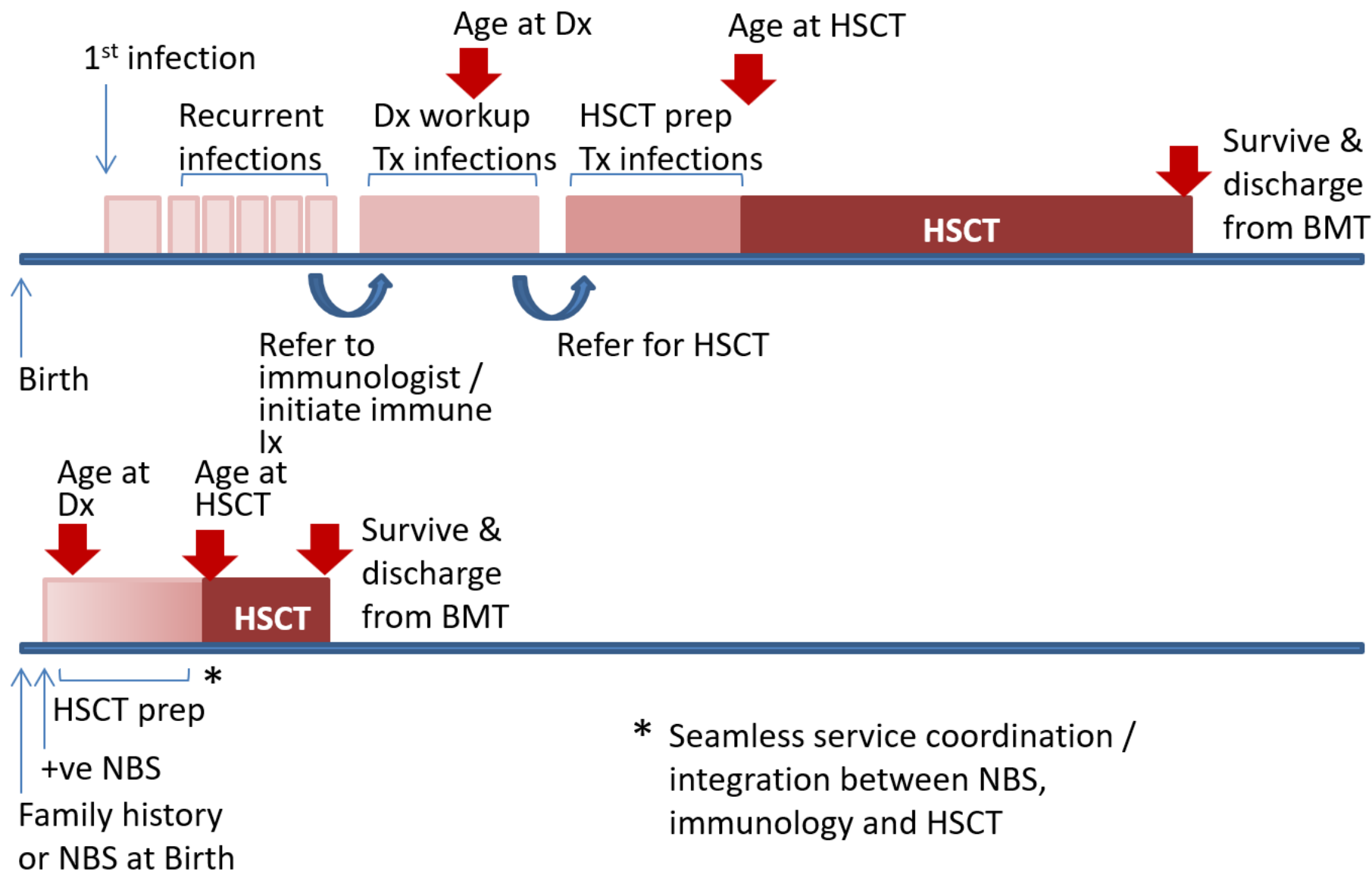
Survivorship program

Screening for late effects





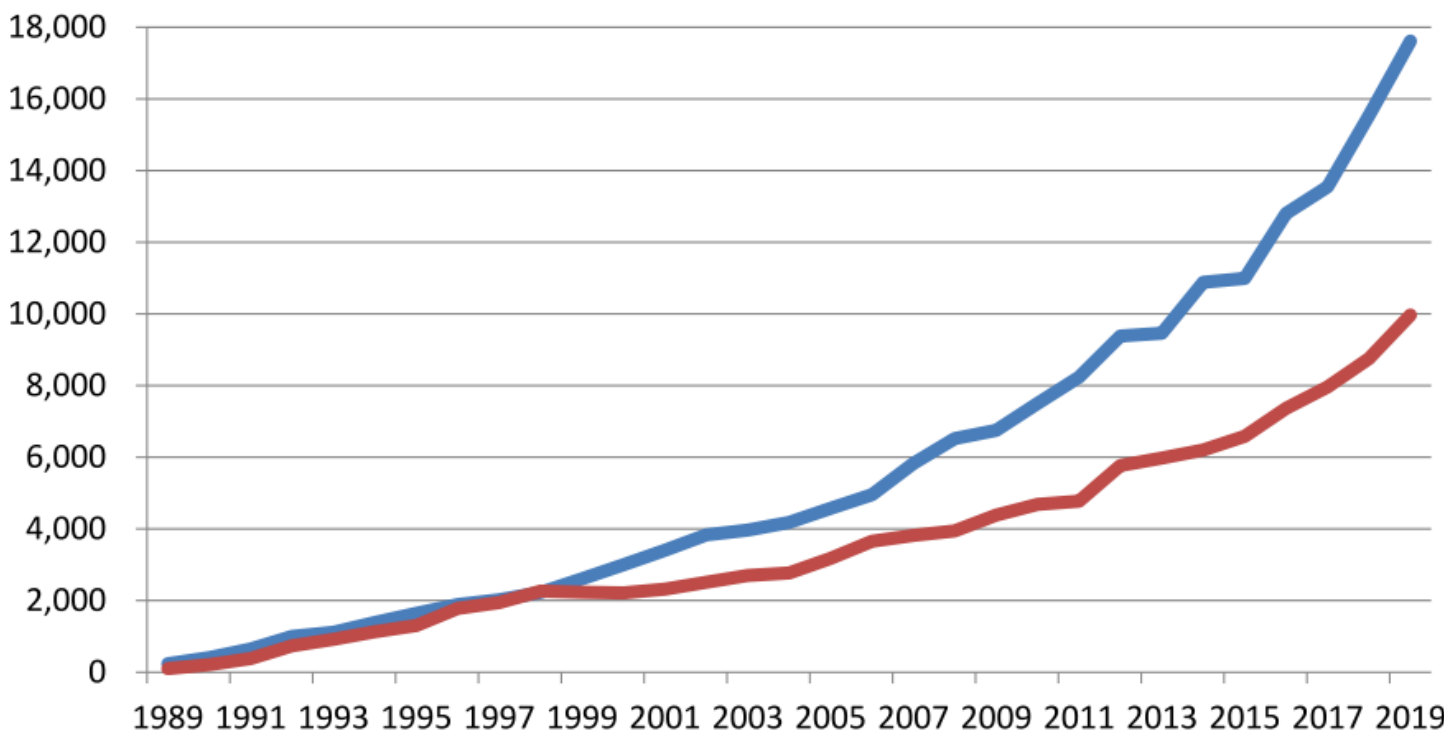
The journey to survival and cure for an infant with SCID: Early Dx → Early HSCT



Original Article



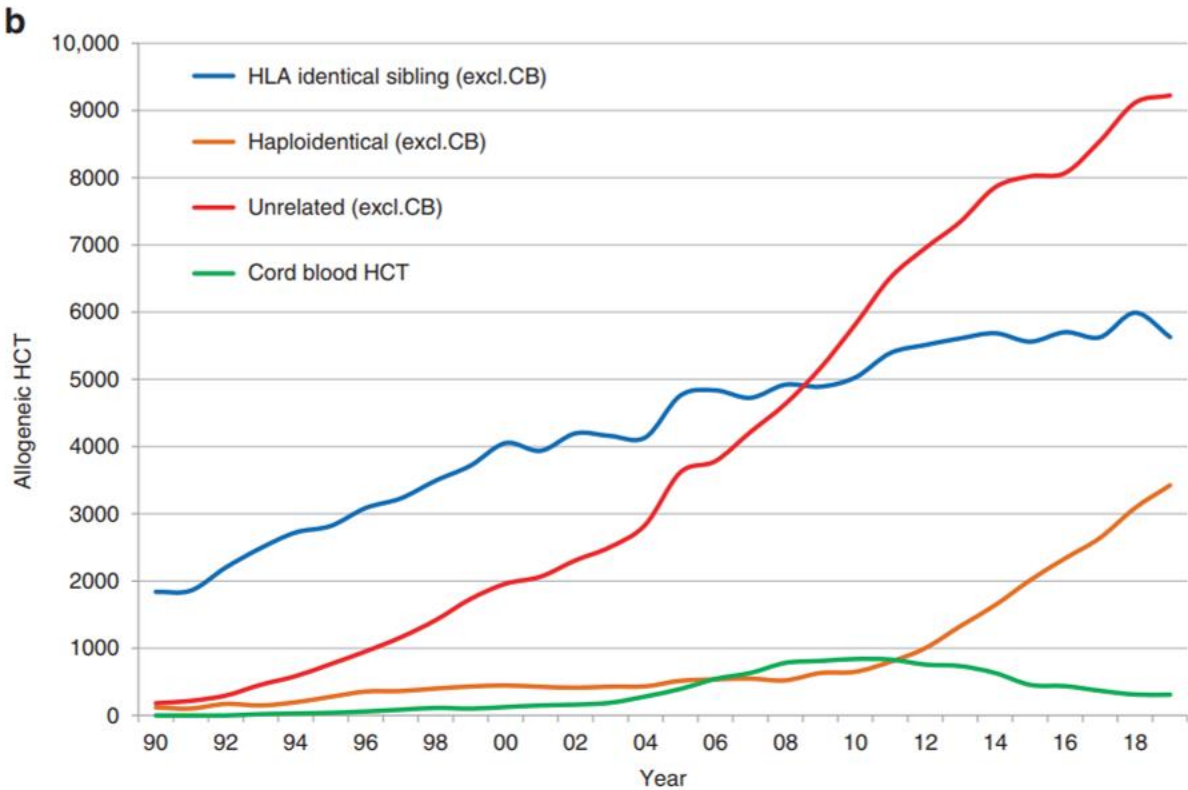
Report on hematopoietic cell transplantations performed in 2018/2019 focusing on the trends of selection of stem cell sources in the Asia-Pacific region: APBMT Activity Survey



Number of HSCTs and Centers Participating in Activity Survey 2019		
	Centers	HSCTs
Australia	42	1,872
Bangladesh	2	20
China	149	11,367
Hong Kong	2	152
India	81	2,432
Indonesia	3	5
Iran	10	1,080
Japan	312	5,860
Korea	45	2,707
Malaysia	15	188
Mongolia	1	6
Myanmar	2	6
Nepal	1	25
New Zealand	6	301
Pakistan	3	177
Philippines	6	44
Singapore	3	244
Sri Lanka	4	73
Taiwan	5	390
Thailand	9	342
Vietnam	2	103
Total	703	27,394

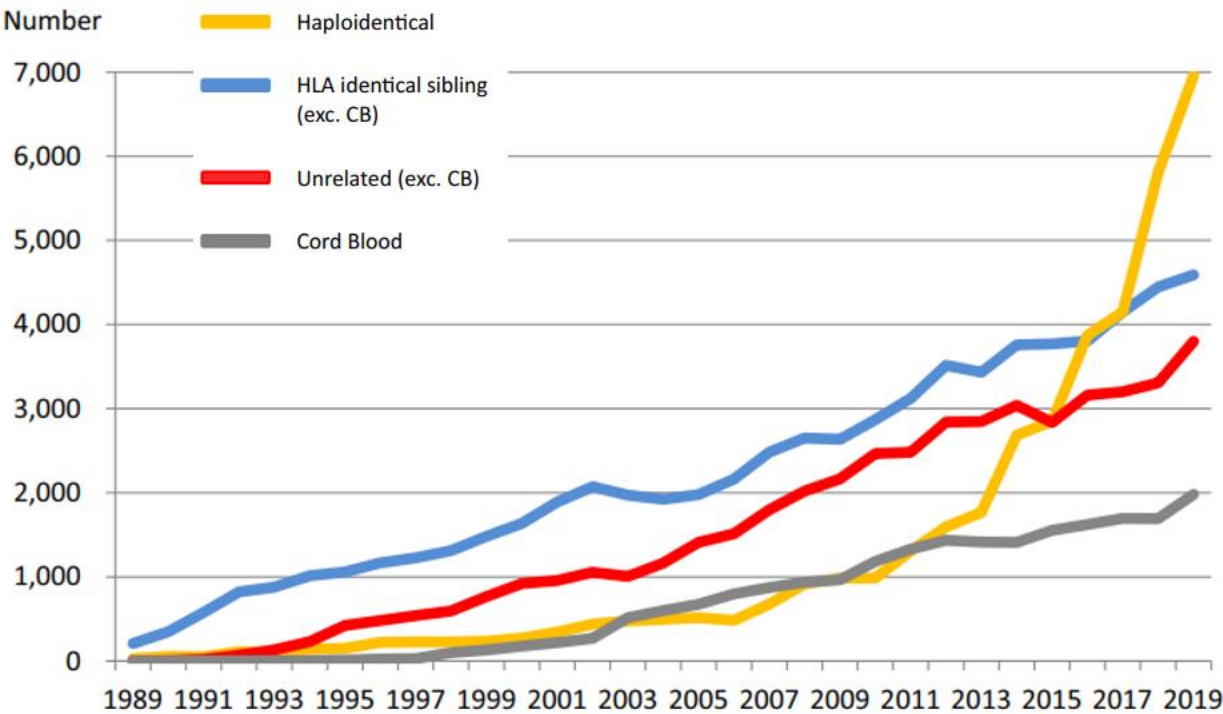
Stem cell source

EBMT survey 2019



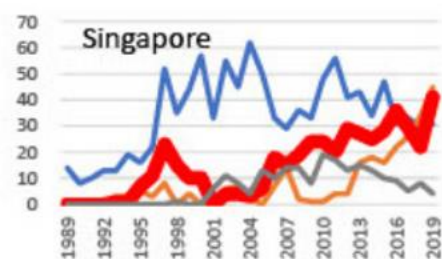
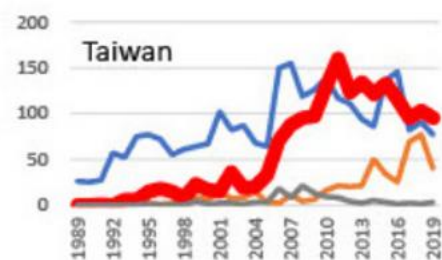
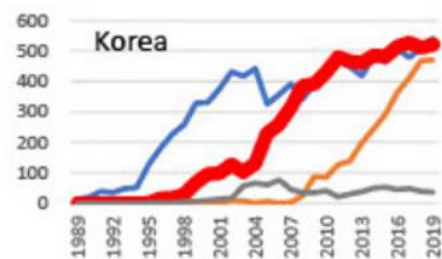
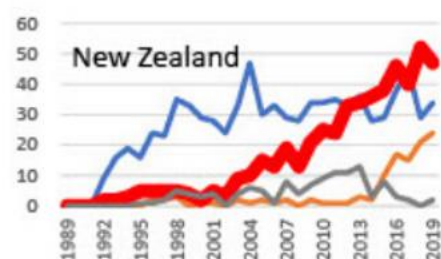
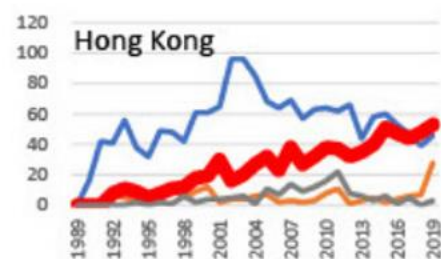
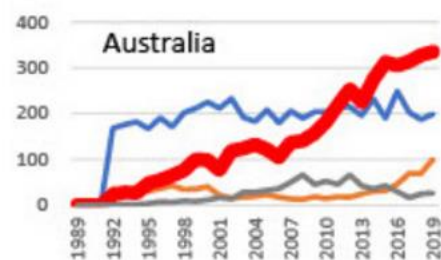
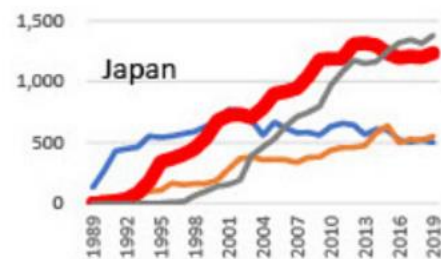
Bone Marrow Transplantation (2021) 56:1651–1664

APBMT survey 2019

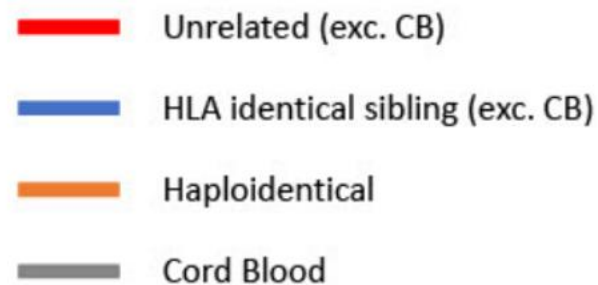
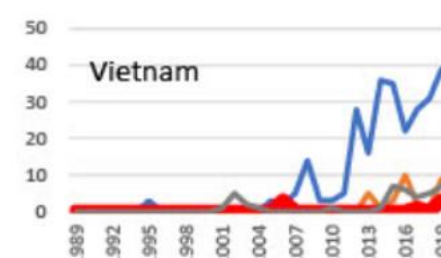
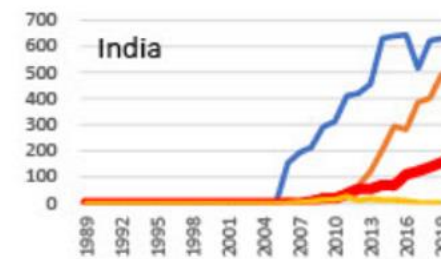
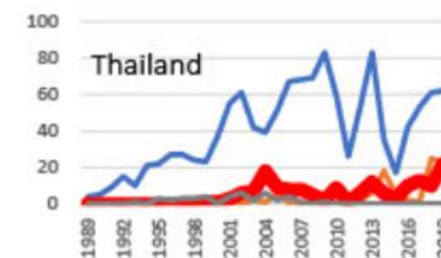
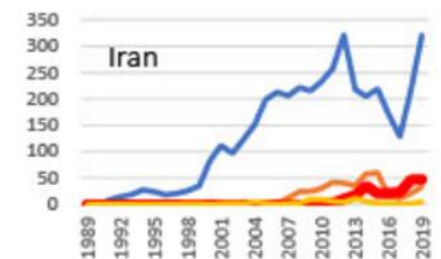
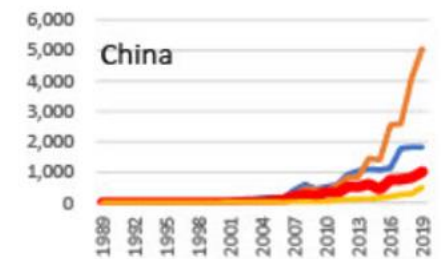


[Blood Cell Ther.](#) 2023 Nov 25; 6(4): 114–123

A



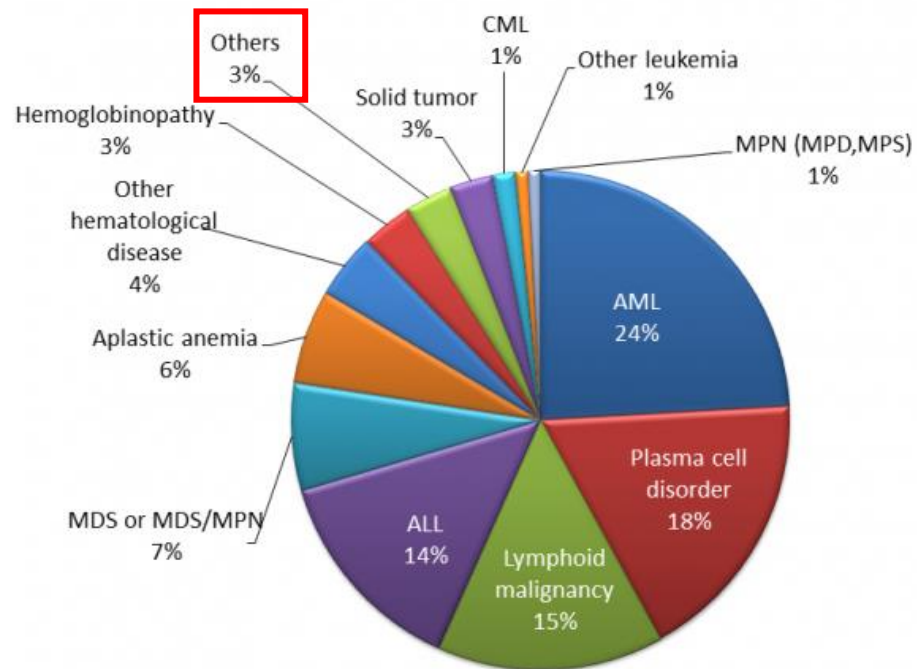
B



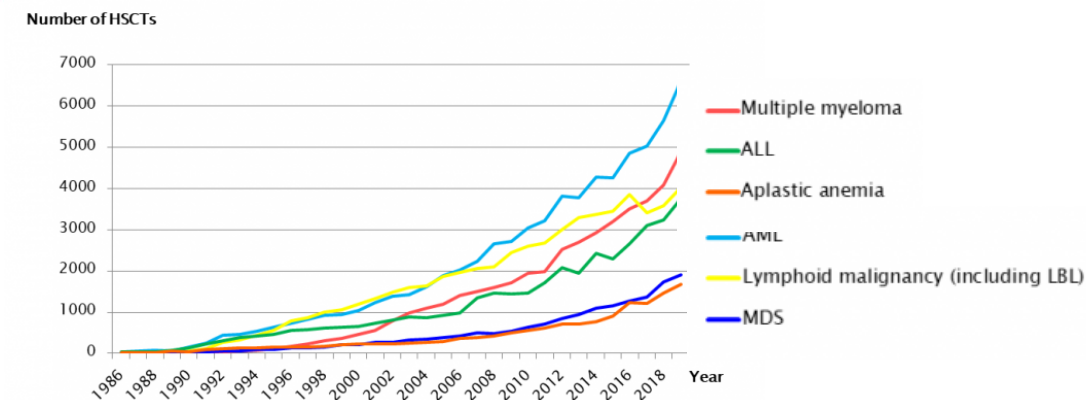


Report on hematopoietic cell transplantations performed in 2018/2019 focusing on the trends of selection of stem cell sources in the Asia-Pacific region: APBMT Activity Survey

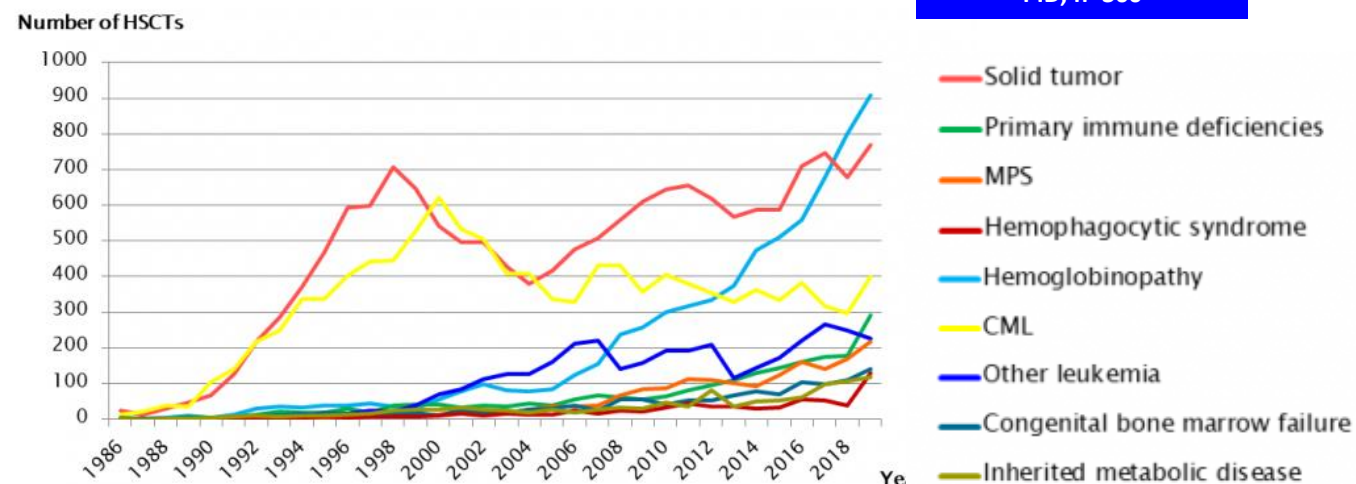
Main indication of total HSCTs



Disease indications (over 1,000)



Disease indications (100 – 1,000)



PID, n=300

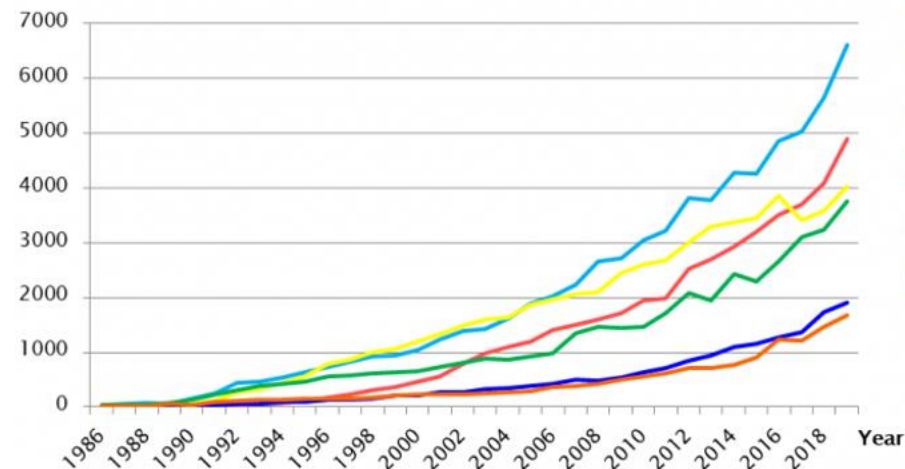
<https://www.apbmt.org/research/registry/survey>

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Total	703	27,394

Disease indications (over 1,000)

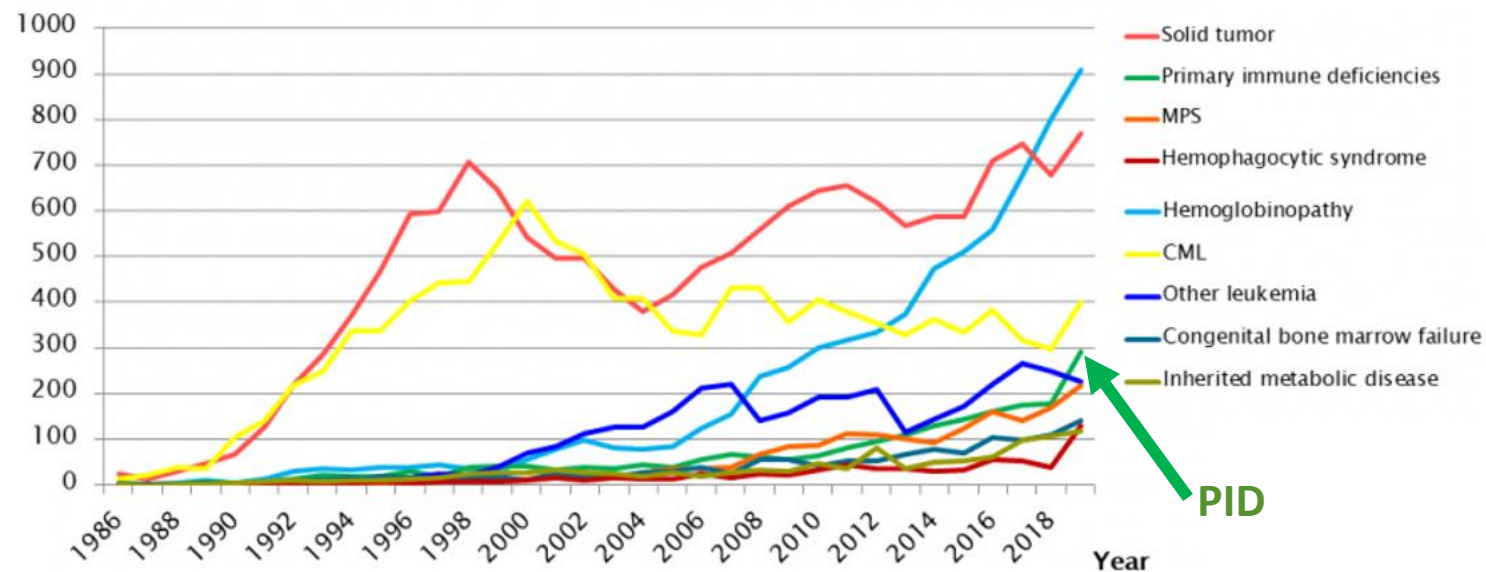
Number of HSCTs



Disease indications (100 - 1,000)

PID, n=300

Number of HSCTs



HSCT and cellular therapy survey of the EBMT: 2019

Bone Marrow Transplantation (2021) 56:1651–1664

Total no. of allo-HSCT =
19798

Total no. of allo-HSCT for
PID = 719

719/19798 = 3.6%

Table 1 Numbers of HCT in Europe 2019 by indication, donor type and stem cell source.

	Transplant activity 2019																	
	No. of patients																	
	Allogeneic												Autologous			Total		
	Family									Unrelated						Allo	Auto	Total
	HLA-id			Twin	Haplo ≥ 2MM		Other family						BM	BM+				
	BM	PBPC	Cord	All	BM	PBPC	BM	PBPC	Cord	BM	PBPC	Cord	Only	PBPC	Cord			
Myeloid malignancies	294	2562	9	13	367	1359	14	73	0	417	5292	118	0	245	1	10,518	246	10,764
Acute myeloid leukemia	227	1799	5	12	268	986	9	56	0	253	3305	87	0	237	0	7007	237	7244
1st Complete remission	144	1163	1	9	154	523	4	37	0	153	1852	38	0	193	0	4078	193	4271
Not 1st complete remission	58	427	4	3	74	327	4	16	0	72	848	35	0	41	0	1868	41	1909
AML therapy-related or myelodysplasia-related changes	25	209	0	0	40	136	1	3	0	28	605	14	0	3	0	1061	3	1064
Chronic myeloid leukemia	7	103	1	1	6	46	0	0	0	20	209	2	0	0	0	395	0	395
Chronic phase	3	54	1	0	2	13	0	0	0	7	91	2	0	0	0	173	0	173
Not chronic phase	4	49	0	1	4	33	0	0	0	13	118	0	0	0	0	222	0	222
MDS or MD/MPN overlap	52	475	3	0	64	259	4	14	0	130	1283	26	0	7	1	2310	8	2318
MPN	8	185	0	0	29	68	1	3	0	14	495	3	0	1	0	806	1	807
Lymphoid malignancies	312	1321	8	6	207	841	9	51	1	346	2093	60	30	22,610	0	5255	22,640	27,895
Acute lymphatic leukemia	267	745	6	0	124	478	7	34	1	286	1044	52	0	66	0	3044	66	3110
1st Complete remission	149	535	3	0	54	243	5	23	0	144	686	24	0	62	0	1866	62	1928
Not 1st complete remission	118	210	3	0	70	235	2	11	1	142	358	28	0	4	0	1178	4	1182
Chronic lymphocytic leukemia	4	40	0	0	6	19	0	0	0	3	99	0	0	11	0	171	11	182
Plasma cell disorders—MM	2	89	0	3	5	24	0	1	0	5	140	0	7	13,245	0	269	13,252	13,521
Plasma cell disorders—other	0	5	0	0	5	3	0	0	0	0	14	0	0	442	0	27	442	469
Hodgkin lymphoma	11	113	0	1	32	112	1	3	0	12	147	2	10	2175	0	434	2185	2619
Non Hodgkin lymphoma	28	329	2	2	35	205	1	13	0	40	649	6	13	6671	0	1310	6684	7994
Solid tumors	3	2	0	0	3	14	0	0	0	2	4	1	18	1529	0	29	1547	1576
Neuroblastoma	2	2	0	0	3	9	0	0	0	0	1	0	10	489	0	17	499	516
Soft tissue sarcoma/Ewing	1	0	0	0	0	2	0	0	0	0	1	1	2	245	0	5	247	252
Germinal tumors	0	0	0	0	0	0	0	0	0	0	1	0	2	431	0	1	433	434
Other solid tumors	0	0	0	0	0	3	0	0	0	2	1	0	4	364	0	6	368	374
Nonmalignant disorders	731	343	25	7	123	205	65	55	1	530	461	58	7	561	1	2604	569	3173
Bone marrow failure—SAA	185	140	0	5	21	54	5	10	0	181	138	10	0	1	1	749	2	751
Bone marrow failure—other	56	28	2	1	20	18	8	9	0	75	52	4	0	0	0	273	0	273
Thalassemia	154	45	9	0	2	11	17	9	1	42	52	0	1	0	0	342	1	343
Sickle cell disease	163	87	8	0	26	9	12	1	0	11	7	0	0	0	0	324	0	324
Primary Immune deficiencies	136	28	6	0	44	103	21	19	0	174	169	19	4	8	0	719	12	731
Inh. disorders of Metabolism	32	12	0	1	10	9	2	5	0	46	33	25	2	13	0	175	15	190
Autoimmune disease—MS	0	0	0	0	0	0	0	0	0	0	1	0	0	442	0	1	442	443
Autoimmune disease—SSC	0	0	0	0	0	0	0	0	0	0	0	0	0	55	0	0	55	55
Autoimmune disease—other	5	3	0	0	0	1	0	2	0	1	9	0	0	42	0	21	42	63
Others	21	14	0	0	11	17	5	6	0	36	42	5	0	16	0	157	16	173
Total patients	1361	4242	42	26	711	2436	93	185	2	1331	7892	242	55	24,961	2	18,563	25,018	43,581
Re/additional transplants	36	155	0	2	59	332	2	11	1	68	547	22	5	3691	0	1235	3696	4931
Total transplants	1397	4397	42	28	770	2768	95	196	3	1399	8439	264	60	28,652	2	19,798	28,714	48,512

	Indication	Allogeneic																		Autologous			Total	
		Related														Unrelated							Allo	Auto
		HLA id sibling				Haplo(≥ 2 loci mismatch)			Other related				Twin											
		BM	PB	CB	other mixtures	BM	PB	other mixtures	BM	PB	CB	other mixtures	BM	PB	other mixtures	BM	PB	CB	other mixtures	BM	PB	other mixtures		
Leukemias	AML 1st CR	45	894	0	62	8	714	662	0	26	0	0	0	2	0	189	490	305	38	1	173	6	3,435	180
	AML non 1st CR	20	466	0	86	24	596	640	6	40	0	0	0	1	0	162	363	473	30	0	107	0	2,907	107
	ALL 1st CR	47	510	0	55	7	330	482	2	16	0	0	0	2	0	132	262	193	21	0	43	1	2,059	44
	ALL non 1st CR	20	251	1	51	14	330	523	1	19	0	0	0	0	1	59	196	172	16	0	19	1	1,654	20
	CML 1st CP	7	31	0	2	0	26	8	0	1	0	0	0	1	0	19	19	16	2	0	0	0	132	0
	CML non 1st CP	2	62	0	4	1	60	57	0	9	0	0	0	0	0	19	31	20	4	0	0	0	269	0
	MDS or MDS/MPN	29	375	0	49	21	341	317	5	12	0	0	0	0	0	208	299	225	26	0	6	0	1,907	6
	MPN (MPD,MPS)	2	58	0	3	0	39	10	0	1	0	0	0	1	0	28	63	13	0	0	0	0	218	0
	CLL inclu.PLL	0	9	0	0	0	4	3	0	0	0	0	0	0	0	4	11	3	0	0	5	0	34	5
	Other leukemia	2	31	0	4	1	31	57	1	2	0	0	0	0	0	15	20	21	0	0	4	0	185	4
Lymphoid Malignancies	PCD-Myeloma	2	44	0	5	2	20	0	0	1	0	0	0	0	0	11	16	7	0	1	4,570	9	108	4,580
	PCD-other	0	2	0	0	0	1	1	0	0	0	0	0	0	0	1	3	0	0	0	239	0	8	239
	Hodgkin lymphoma	3	35	0	0	0	24	1	0	3	0	0	0	0	0	8	10	6	0	1	703	1	90	705
	Lymphoblastic lymphoma	1	27	0	3	1	24	28	1	1	0	0	0	0	0	17	7	23	4	0	79	0	137	79
	Mature B cell lymphoma	3	63	0	0	0	72	4	0	4	0	0	0	0	0	29	48	69	0	3	1,899	1	292	1,903
	Mature T/NK cell lymphoma	12	77	0	1	4	92	46	1	5	0	0	0	0	0	49	64	131	0	0	418	0	482	418
	Other LPD	1	22	0	8	0	34	43	0	1	0	0	0	0	0	5	17	14	11	1	761	13	156	775
	Neuroblastoma	1	2	0	0	1	12	1	0	0	0	0	0	0	0	0	0	14	0	4	274	0	31	278
Solid Tumors	Germ cell tumor	0	1	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	74	0	1	74
	Breast cancer	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0
	Ewing	0	0	0	0	0	2	0	0	0	0	0	0	0	0	0	0	0	0	0	44	0	2	44
	Other solid tumor	0	1	0	0	0	3	1	0	0	0	0	0	0	0	1	0	1	0	9	341	0	7	350
	Non-Malignant Hematological Disorders	BM failure-SAA	79	317	0	109	33	210	435	6	27	0	0	1	2	0	45	220	139	48	0	0	0	1,671
Aquired pure red cell anemia		1	1	0	0	0	1	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	3	0
PNH		2	11	0	1	0	3	2	0	1	0	0	0	0	0	1	4	3	0	0	0	0	28	0
Congenital bone marrow failure		10	32	0	0	2	19	18	2	7	0	0	0	0	0	14	29	8	0	0	0	0	141	0
BM failure-other		2	10	0	0	1	2	2	0	6	0	0	0	0	0	3	9	5	0	0	0	0	40	0
Hemoglobinopathy-Thalassemia		120	168	2	78	8	115	104	12	33	0	2	0	0	0	7	201	4	7	0	0	0	861	0
Hemoglobinopathy-other		3	24	1	0	1	19	1	0	0	0	0	0	0	0	0	0	0	0	0	0	0	49	0
Other hematological disease		2	1	0	1	0	0	1	0	1	0	0	0	0	0	0	1	0	0	0	0	0	7	0
Non-Malignant Hematological Diseases	Primary immune deficiencies	20	32	2	0	15	54	27	3	16	0	0	0	0	0	36	55	38	0	0	0	0	298	0
	Inherited metabolic disease	5	12	0	0	0	15	13	0	6	0	0	0	0	0	8	21	37	0	0	1	0	117	1
	Autoimmune disease	2	1	0	0	0	0	0	1	2	0	0	0	0	0	0	3	0	0	0	40	0	9	40
	EBV related disorders	4	8	0	0	1	12	17	0	0	0	0	0	0	0	8	17	16	0	0	3	0	83	3
	Hemophagocytic syndrome	2	17	0	3	2	36	39	0	0	0	0	0	0	0	1	21	8	0	0	2	0	129	2
	Langerhans cell histiocytosis	0	1	0	0	1	1	0	0	0	0	0	0	0	0	0	1	1	0	0	1	0	5	1
	Others	1	7	0	1	1	10	14	0	2	0	0	0	0	0	7	5	10	0	1	110	1	58	112
	Total	450	3,603	6	526	149	3,252	3,557	41	242	0	2	1	9	1	1,086	2,506	1,975	207	21	9,916	33	17,613	9,970
	Sub total 1	4,585				6,958			285				11			5,774				9,970			27,583	
	Sub total 2	11,839																						
	Sub total 3																							
		17,613																						

Q&A



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Panel discussion: Achieving optimal patient care for PID patients

Patients: Mr Jitchanwichai Narumon (*Thailand*) - Mr Bruce Lim (*Malaysia*)

Doctors: Prof Surjit Singh (*India*) - Dr Nizar Mahlaoui (*France*)



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How to manage daily life with a PID?

Dr Intan Hakimah (*Malaysia*)

Mr Bruce Lim (*IPOPI Vice-Chair*)



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Report Back

Mr Bruce Lim (*IPOPI Vice-President*)



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Conclusions

Mr Bruce Lim (*IPOPI Vice-President*)



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