

Stem Cell Reports, Volume 18

Supplemental Information

History and current status of clinical studies using human pluripotent stem cells

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Supplementary Information for:

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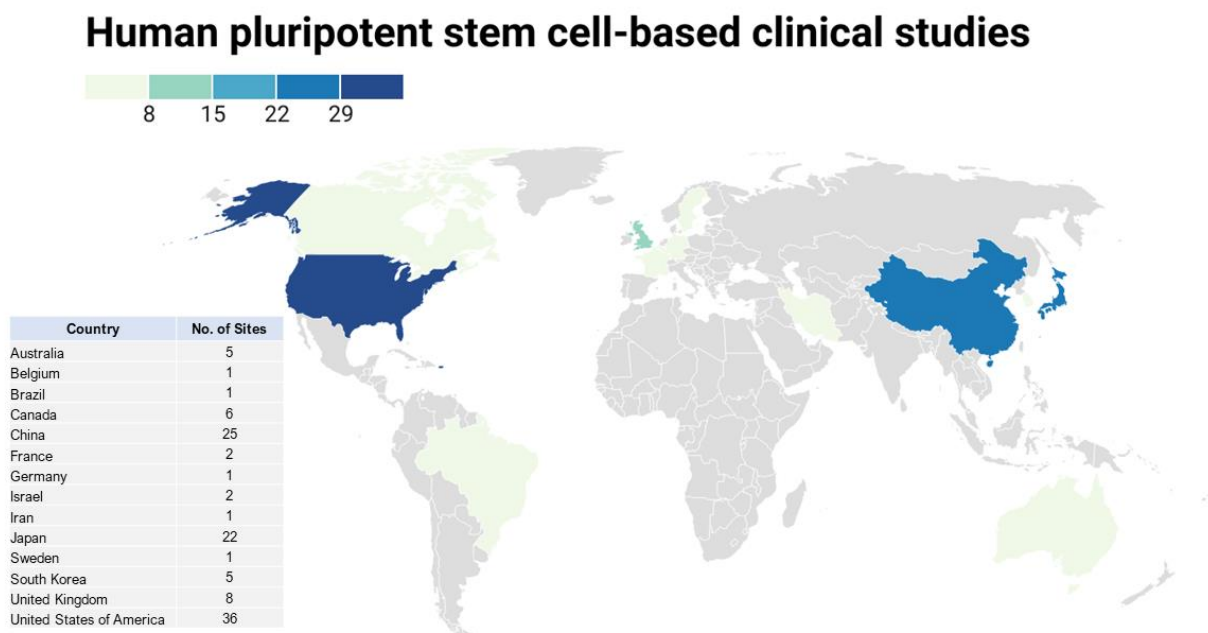
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Figure S1. World map of clinical study sites, related to section “Where and who carries out clinical studies”.



Data as of December 13, 2022

Created with Datawrapper

Table S1. PSC lines used as starting materials for the production of cell-based therapeutics, related to section “Traceability of the source PSC lines employed in PSC-based therapies”.

PSC line common name	hPSCreg® cell line ID	Year of First Publication of the Cell Line	No. of Clinical Trials
Human embryonic stem cells			
MA09	ACTe002-A	2006	13
CyT49	VCYTe001-A	2006	6
H1	WAe001-A	1998	3
H9	WAe009-A	1998	2
Shef-1	AXORe001-A	2004	2
HAD-C 100	HADe008-A	2012	1
HAD-C 102	HADe007-A	2012	1
I6	TECHe003-A	2002	1
RC17	RCe021-A	2016	1
RC9	RCe013-A	2012	1
Q-CTS-hESC-2	Not registered	2016	1
Unknown ESC			19
Human induced pluripotent stem cells			
QHJI	Not registered	2019	3
Unknown iPSC			51

Table S2. Target diseases of PSC-derived cell therapies, related to section “Cell types and their target indications for PSC-based therapies”.

Disease (ICD-10 classification)	Cellular Origin of Cell Product				Total
	hESC	hiPSC	hpSC	hSCNT	
Diseases of the eye and adnexa	24	9	0	1	34
Degeneration of macula and posterior pole: Senile macular degeneration, dry AMD	11	3		1	
Other retinal disorders	4	2			
Hereditary retinal dystrophy: Stargardt disease	5				
Hereditary retinal dystrophy: retinitis pigmentosa	2	1			
Hereditary retinal dystrophy	1				
Bullous keratopathy		1			
Other specified disorders of cornea		1			
Degeneration of macula and posterior pole: wet age-related macular degeneration		1			
Myopia	1				
Neoplasms	1	17	0	0	18
Malignant neoplasms		5			
Myeloid leukaemia		3			
Other and unspecified types of non-Hodgkin lymphoma		2			
Chronic lymphocytic leukaemia of B-cell type		2			
Malignant neoplasm of ovary		2			
Head, face and neck		1			
Malignant neoplasm of bronchus and lung	1				
Malignant neoplasm of breast		1			
Multiple myeloma		1			
Diseases of the circulatory system	3	13	0	0	16
Ischaemic cardiomyopathy		7			
Cerebral infarction, unspecified	1	2			
Heart failure, unspecified		2			
Left ventricular failure	1				
Ischaemic heart diseases	1				
Congestive heart failure		1			
Dilated cardiomyopathy		1			
Diseases of the nervous system	5	3	2	0	10
Parkinson disease	2	3	2		
Multiple sclerosis	1				
Epilepsy	1				
Motor neuron disease	1				

Disease (ICD-10 classification)	Cellular Origin of Cell Product				Total
	hESC	hiPSC	hpSC	hSCNT	
Endocrine, nutritional and metabolic diseases	9	2	0	0	11
Unspecified diabetes mellitus with multiple complications with diabetic foot syndrome, controlled		1			
Type 1 diabetes mellitus	7	1			
Disorders of urea cycle metabolism	1				
Primary ovarian failure	1				
Injury, poisoning and certain other consequences of external causes	3	4	0	0	7
Injury of spinal cord, level unspecified	3	1			
Tear of articular cartilage of knee, current		2			
Bone-marrow transplant rejection		1			
Diseases of the blood and blood-forming organs and certain disorders involving the immune mechanism	0	4	0	0	4
Beta thalassaemia		2			
Thrombocytopenia, unspecified		1			
Other aplastic anaemias		1			
Provisional assignment of new diseases of uncertain etiology or emergency use	2	2	0	0	4
COVID-19	2	2			
Diseases of the genitourinary system	2	0	0	0	2
Intrauterine synechiae	1				
Interstitial cystitis (chronic)	1				
Arthropathies	0	1	0	0	1
Osteoarthritis of knee, unspecified		1			
Diseases of the digestive system	1	0	0	0	1
Acute and subacute hepatic failure	1				
Diseases of the musculoskeletal system and connective tissue	1	0	0	0	1
Derangement of meniscus due to old tear or injury	1				
Totals	51	55	2	1	109

Table S3. Cell types used for PSC-derived cell-based therapies (last 10 years) and target indications, related to section “Cell types and their target indications for PSC-based therapies”.

Cell types used for therapy	2013	2014	2015	2016	2017	2018	2019	2020	2021	2022	Total
retinal pigment epithelial cell	4		5	2	2	3		2	2	2	22
age related macular degeneration	2		2	2	2	2		1	1	1	13
dystrophies primarily involving the retinal pigment epithelium									1		1
macular degeneration			2			1					3
myopic macular degeneration	1										1
retinitis pigmentosa			1					1			2
RPE impaired disease										1	1
Stargardt disease	1										1
natural killer cell							3	6	7	2	18
acute myeloid leukemia							1	1	1		3
advanced malignant solid neoplasm							2	1	1	1	5
B-Cell malignant neoplasm										1	1
breast cancer									1		1
chronic lymphocytic leukemia								1	1		2
COVID-19								1			1
head and neck cancer								1			1
multiple myeloma									1		1
non-Hodgkin lymphoma								1			1
ovarian cancer									2		2
mesenchymal stem cell				1	1		2	5	2	1	12
adhesions of uterus								1			1
age related macular degeneration				1							1
COVID-19								3			3
diabetic foot ulcer										1	1
graft versus host disease					1						1
interstitial cystitis								1			1
meniscus							1				1
multiple sclerosis									1		1
osteoarthritis									1		1
primary ovarian insufficiency							1				1
cardiac muscle cell							3	2	3	4	12
cardiomyopathy							1	1		1	3
congestive heart failure									1		1
heart failure							1	1	2	2	6
ischemic heart disease							1			1	2
progenitor cell of endocrine pancreas		1		1	2		1			1	6
type 1 diabetes mellitus		1		1	2		1			1	6
neural stem cell				1			2	1	1		5
ischemic cerebrovascular accident							1		1		2
Parkinson's disease				1			1				2

Cell types used for therapy	2013	2014	2015	2016	2017	2018	2019	2020	2021	2022	Total
spinal cord injury								1			1
dopaminergic neuron					1	2			1	1	5
Parkinson's disease					1	2			1	1	5
corneal epithelial cell			1				1				2
limbal stem cell deficiency			1				1				2
islet of Langerhans							1		1		2
type 1 diabetes mellitus							1		1		2
platelet							1		1		2
aplastic anemia							1				1
thrombocytopenia									1		1
dendritic cell, human						1	1				2
lung non-small cell carcinoma						1					1
retinitis pigmentosa							1				1
cartilage tissue								1		1	2
articular cartilage disease								1		1	2
hematopoietic stem cell			1				1				2
beta thalassemia			1				1				2
hepatocyte						1				1	2
acute liver failure										1	1
urea cycle disorder						1					1
endothelial progenitor cell						1					1
ischemic cerebrovascular accident						1					1
cardioblast	1										1
ischemic heart disease	1										1
interneuron										1	1
epilepsy										1	1
neural progenitor cell									1		1
spinal cord injury									1		1
retina								1			1
retinitis pigmentosa								1			1
astrocyte						1					1
amyotrophic lateral sclerosis						1					1
corneal endothelial cell									1		1
bullous keratopathy									1		1
oligodendrocyte precursor cell			1								1
spinal cord injury			1								1
Grand Total	5	1	8	5	6	9	16	18	20	14	102