

Fig 1: Timeline showing key developments in human gene therapy for PID

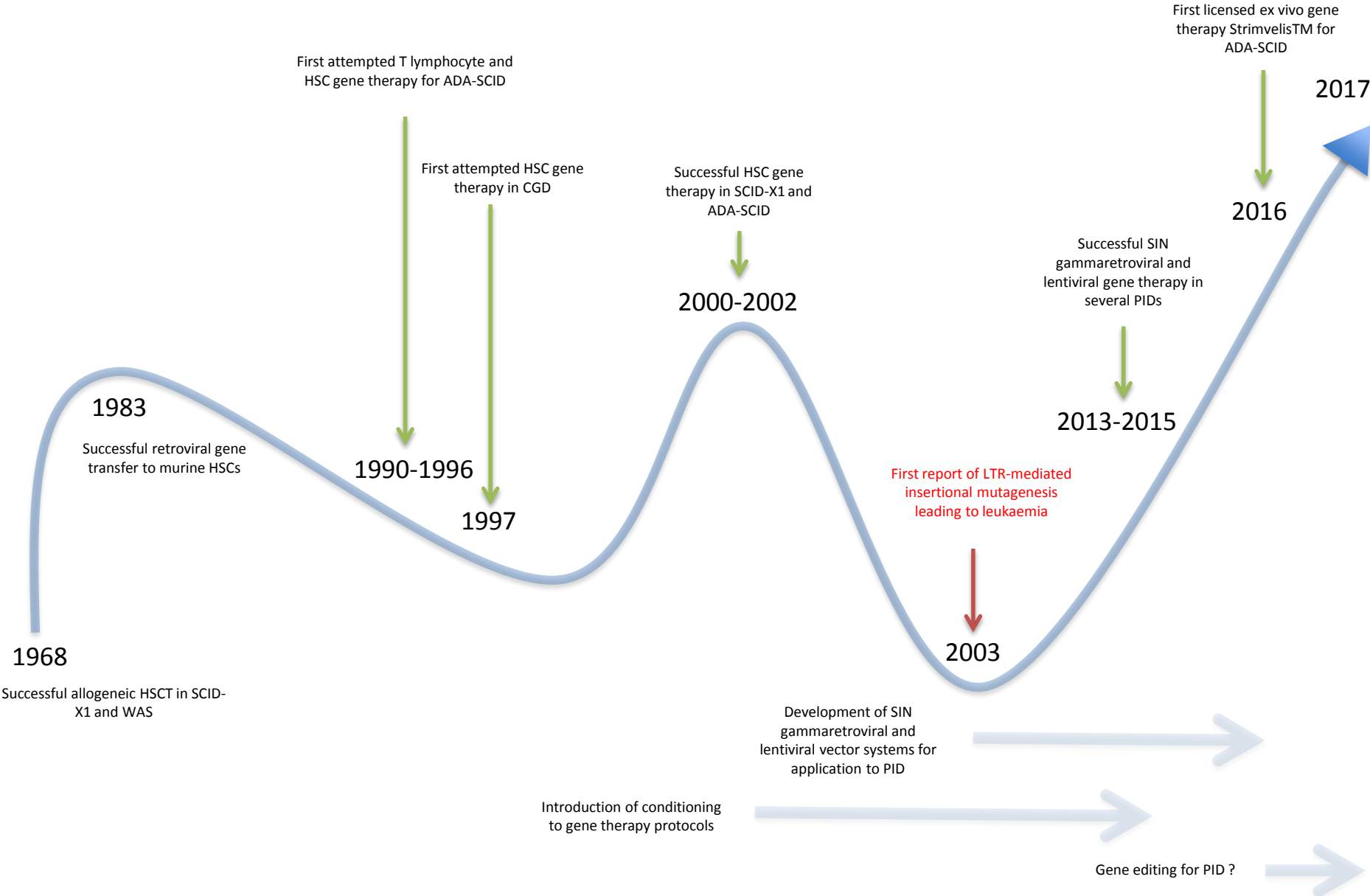


Fig 2: Published and ongoing clinical trials for PID

<i>Disease</i>	<i>Mutated gene/ protein</i>	<i>Target cell/Vector</i>	<i>Conditioning</i>	<i>Year reported reported/reference</i>
ADA-SCID	ADA (adenosine deaminase)	PBL/RV	N	1995(21)
		PBL,HSC/RV	N	1995(22)
		CB/RV	N	1995 (23)
		HSC/RV	N	1996 (24)
		HSC/RV	Y (Bu RIC)	2002 (25,27,28,30)
		HSC/RV	Y (Melp Bu RIC)	2006 (26,32)
		HSC/RV	N/Y (Bu RIC)	2012(29,31)
		HSC/LV	Y (Bu RIC)	ongoing
SCID-X1	IL2RG (gc)	HSC/RV	N	2000(43,45,49,52,)
		HSC/RV	N	2000 (44,46,50,51)
		HSC/RV	N	2007 (66)
		HSC/SIN-RV	N	2014 (63)
		HSC/LV	Y (Bu RIC)	2016 (67) ongoing
CGD	NCF-1/p47phox CYBB (gp91phox)	HSC/RV	N	1997(77)
		HSC/RV	Y (Bu RIC)	2006(78)
		HSC/RV	Y (Bu RIC)	2010(79)
		HSC/RV	Y (Bu/Flu RIC)	2012 (80)
		HSC/LV	Y (Bu FIC)	ongoing (82)
WAS	WASP (WASp)	HSC/LV	Y (Bu/Flu RIC)	2013(90,91) ongoing
		HSC/LV	Y (Bu/Flu FIC)	2015(90,92) ongoing
		HSC/LV	Y (Bu/Flu FIC)	ongoing (90,US)

PBL, peripheral blood lymphocytes; CB, cord blood; HSC, haematopoietic stem cells

Bu, Busulphan; Flu, fludarabine, Melp, Melphalan; RIC, reduced intensity conditioning; FIC, full myeloablative conditioning