

Web Table I Listing of Current Gene Therapy Clinical Trials for TDT in North America and EU

<i>Trial NCT number</i>	<i>Sponsor</i>	<i>Phase</i>	<i>Patient population</i>	<i>Vector or platform/DP name</i>	<i>MOA</i>	<i>References</i>
NCT 01745120 and 02151526	bluebird bio (HGB-204 and 205)	1/2	All TDT	SIN Lentiviral vector, BB305 (β^{A-T87Q})	Increase HbA ^{T87Q} levels	Thompson et.al, NEJM [24]
NCT 02906202	bluebird bio (HGB-207)	3	TDT with non- β^0/β^0 genotypes	SIN Lentiviral vector, BB305(β^{A-T87Q}) with process improvements	Increase HbA ^{T87Q} levels	Thompson et.al, 2019 [32]
NCT 03207009	bluebird bio (HGB-212)	3	β^0/β^0 genotypes	SIN Lentiviral vector, BB305(β^{A-T87Q}) with process improvements	Increase HbA ^{T87Q} levels	Lal et.al, 2019 [33]
NCT 02453477	IRCCS, San Rafael and Orchard Therapeutics	1/2	All TDT (6-35 years)	SIN Lentiviral vector (GLOBE)- β^A -globin; OTL-300	Increase HbA levels	Marktel et.al, [51]
NCT 03432364	Bioverative and Sanofi	1/2	All TDT (18-40 years)	Gene-editing of erythroid specific region of BCL11a with ZFN; ST 400	Increase HbF	No publication
NCT 03655678	Crispr Therapeutics and Vertex (CTX001-111)	1/2	All TDT (18-35 years)	Crispr-Cas9 gene-editing of erythroid specific BCL11a region; CTX001	Increase HbF	Corbacioglu et.al, 2020 [60]
Pre-clinical	Editas Medicine	TBD	TDT	Crispr-Cas12 gene-editing of β -globin locus that binds BCL11a	Recreate HPFH	No publication