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(81) **Designated States** (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AO, AT, AU, AZ, BA, BB, BG, BH, BN, BR, BW, BY, BZ, CA, CH, CL, CN, CO, CR, CU, CV, CZ, DE, DJ, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IQ, IR, IS, IT, JM, JO, JP, KE, KG, KH, KN, KP, KR, KW, KZ, LA, LC, LK, LR, LS, LU, LY, MA, MD, MG, MK, MN, MU, MW, MX, MY, MZ, NA, NG, NI, NO, NZ, OM, PA, PE, PG, PH, PL, PT, QA, RO, RS, RU, RW, SA, SC, SD, SE, SG, SK, SL, ST, SV, SY, TH, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, WS, ZA, ZM, ZW.

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(54) **Title: NON-HUMAN ANIMALS COMPRISING A MODIFIED TRANSFERRIN RECEPTOR LOCUS**

Offical Synonym	NCBI Gene ID	RefSeq mRNA ID	UniProt ID	Genomic Assembly	Location
Mouse Tfrc	420942	NM_002357206.1	Q62451	GRCh38	Chr 16: 82,427,318-82,438,121 (+)
Human TFRC	70337	NM_001128348.3	P02786	GRCh38.p14	Chr 3: 196,049,284-196,082,050 (+)

	Genomic Build	Start	End	Length (bp)
5' mouse arm	GRCh38/mm10	chr3:32,354,579	32,618,239	263,661
Human Invert	GRCh38	chr3:196,075,456	chr3:196,045,415	26,240
3' mouse arm	GRCh38/mm10	chr3:32,690,774	chr3:32,659,232	28,459

FIGURE 1A

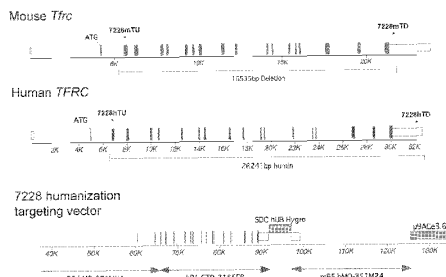


FIGURE 1B

(57) **Abstract:** Non-human animal cells and non-human animals comprising a humanized *Tfrc* gene, e.g., at an endogenous *Tfrc* locus, and methods of using such non-human animal cells and non-human animals are provided. Non-human animal cells or non-human animals comprising a humanized *Tfrc* gene express a human TfR protein or fragments thereof. Non-human animal cells and non-human animals comprising a humanized *Tfrc* gene and a knockout mutation in a *Gaa* gene, e.g., at an endogenous *Gaa* locus, and methods of using such non-human animal cells and non-human animals are also provided. Such animals are useful to screen anti-human-TfR binding protein based therapies of, e.g., Pompe disease.

SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, RU, TJ, TM), European (AL, AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HR, HU, IE, IS, IT, LT, LU, LV, MC, ME, MK, MT, NL, NO, PL, PT, RO, RS, SE, SI, SK, SM, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, KM, ML, MR, NE, SN, TD, TG).

Declarations under Rule 4.17:

- *as to applicant's entitlement to apply for and be granted a patent (Rule 4.17(ii))*
- *as to the applicant's entitlement to claim the priority of the earlier application (Rule 4.17(iii))*

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