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(54) Title: RECOMBINANT HUMAN NAGLU PROTEIN AND USES THEREOF

Human NaGlu Amino Acid Sequence (signal peptide: 1-23, underlined)

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HEAVAVAAAV GVLLLAGAGG AAGDEAREAA AVRALVARLL GPGPAADFSV SVERALAARK 60
GLDTYSLGGG GAARVRVRS TGVAAGAGLH RYLDFGCH VAWSGSQLRL PPPLPAVPGE 120
LTEATPNRYR YQNVCTQSY SFVWMDWARW EREIDWMLN GINLALAWSC QEATWQRVYL 180
ALGLTQAEIN EFFTGPAPLA WGRMGNLHTW DGPLPPSWHT KQYLYQHRVL QQMRSPGMTP 240
VLPAPAGHVP EAVTRVFPQV NVTKMGSGWH FNCSYSCSFL LAPEDPIFPI IGSUFLREL 300
KEFGTDHIYG ADTFNEMQPP SSEPSYLAAG TTAVYEAMTA VDEAVWLLQ GWLFQHQPF 360
WGPAQIRAVL GAVPRGRLLV LDLPAESQPV YTRTASFQGG PFIWCMLEHF GGNHGLFGAL 420
EAVNGGPEAA RLFPNSTMVG TGMAPEGISQ NEVVYSLMAE LGWRKDPVPD LAAWTSPFAA 480
RRYGVSHFDA GAARLLLRV VYNCSEACR GHNRSPVLRV PSLQMNSTIW YNRSDVFEAW 540
RLLLTSAPSL ATSPAFRYDL LDLTRQAVQE LVSLYEEAR SAYLSKELAS LLRAGGVLAY 600
ELLPALDEVL ASDSRFLFGS WLEQARAAAV SEAEADFYEQ NSRYQLTLWG PEGNILDYAN 660
KQLAGLVANY YTPRWRLFLE ALVDSVAQGI PFQHQFQDN VFQLEQAFVL SKQRYPSQPR 720
GDTVDLAKKI FLKYYPWVA GSW 743

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{SEQ ID NO:1}

Fig. 1

(57) Abstract: The present invention provides compositions comprising an isolated mixture of recombinant human NaGlu proteins in which a substantial amount of the NaGlu proteins in the mixture has increased levels of phosphorylated mannose that confer the proteins to be efficiently internalized into human cells. The present invention also provides methods of producing such mixture of NaGlu proteins, vectors used in transgenesis and expression, host cells harboring such vectors, and methods of isolating and purifying the mixture of NaGlu proteins. The invention further provides methods of treating NaGlu associated diseases.