



α -gal A siRNA (m): sc-140596

BACKGROUND

α -galactosidase A (α -gal A) functions as a lysosomal hydrolase. α -gal A forms an active homodimer that acts upon a glycolipid substrate, globotriaosylceramide (Gb3). Inherited mutations in this gene cause an X-linked recessive glycolipid storage disorder known as Fabry's disease. In Fabry patients, α -gal A deficiencies lead to an accumulation of Gb3 in the body. The numerous clinical manifestations of the disease include renal and cardiac impairment, severe pain in the extremities and cutaneous lesions known as angiokeratomas. Enzyme replacement therapy using recombinant α -gal A effectively treats the symptoms of Fabry disease.

REFERENCES

1. Kint, J.A. 1970. Fabry's disease: α -galactosidase deficiency. *Science* 167: 1268-1269.
2. Sweatman, A.K., et al. 1994. Physical mapping in the region of the Bruton's tyrosine kinase and α -galactosidase A gene loci in proximal Xq22. *Hum. Genet.* 94: 624-628.
3. Schiffmann, R., et al. 2000. Infusion of α -galactosidase A reduces tissue globotriaosylceramide storage in patients with Fabry disease. *Proc. Natl. Acad. Sci. USA* 97: 365-370.
4. Ioannou, Y.A., et al. 2001. Fabry disease: preclinical studies demonstrate the effectiveness of α -galactosidase A replacement in enzyme-deficient mice. *Am. J. Hum. Genet.* 68: 14-25.
5. Eng, C.M., et al. 2001. A phase 1/2 clinical trial of enzyme replacement in fabry disease: pharmacokinetic, substrate clearance, and safety studies. *Am. J. Hum. Genet.* 68: 711-722.
6. Breunig, F., et al. 2003. Fabry disease: Diagnosis and treatment. *Kidney Int. Suppl.* 84: 181-185.

CHROMOSOMAL LOCATION

Genetic locus: GlA (mouse) mapping to X E3.

PRODUCT

α -gal A siRNA (m) is a pool of 3 target-specific 19-25 nt siRNAs designed to knock down gene expression. Each vial contains 3.3 nmol of lyophilized siRNA, sufficient for a 10 μ M solution once resuspended using protocol below. Suitable for 50-100 transfections. Also see α -gal A shRNA Plasmid (m): sc-140596-SH and α -gal A shRNA (m) Lentiviral Particles: sc-140596-V as alternate gene silencing products.

For independent verification of α -gal A (m) gene silencing results, we also provide the individual siRNA duplex components. Each is available as 3.3 nmol of lyophilized siRNA. These include: sc-140596A, sc-140596B and sc-140596C.

RESEARCH USE

For research use only, not for use in diagnostic procedures.

PROTOCOLS

See our web site at www.scbt.com for detailed protocols and support products.

STORAGE AND RESUSPENSION

Store lyophilized siRNA duplex at -20° C with desiccant. Stable for at least one year from the date of shipment. Once resuspended, store at -20° C, avoid contact with RNases and repeated freeze thaw cycles.

Resuspend lyophilized siRNA duplex in 330 μ l of the RNase-free water provided. Resuspension of the siRNA duplex in 330 μ l of RNase-free water makes a 10 μ M solution in a 10 μ M Tris-HCl, pH 8.0, 20 mM NaCl, 1 mM EDTA buffered solution.

APPLICATIONS

α -gal A siRNA (m) is recommended for the inhibition of α -gal A expression in mouse cells.

SUPPORT REAGENTS

For optimal siRNA transfection efficiency, Santa Cruz Biotechnology's siRNA Transfection Reagent: sc-29528 (0.3 ml), siRNA Transfection Medium: sc-36868 (20 ml) and siRNA Dilution Buffer: sc-29527 (1.5 ml) are recommended. Control siRNAs or Fluorescein Conjugated Control siRNAs are available as 10 μ M in 66 μ l. Each contain a scrambled sequence that will not lead to the specific degradation of any known cellular mRNA. Fluorescein Conjugated Control siRNAs include: sc-36869, sc-44239, sc-44240 and sc-44241. Control siRNAs include: sc-37007, sc-44230, sc-44231, sc-44232, sc-44233, sc-44234, sc-44235, sc-44236, sc-44237 and sc-44238.

RT-PCR REAGENTS

Semi-quantitative RT-PCR may be performed to monitor α -gal A gene expression knockdown using RT-PCR Primer: α -gal A (m)-PR: sc-140596-PR (20 μ l). Annealing temperature for the primers should be 55-60° C and the extension temperature should be 68-72° C.