



alpha-L-iduronidase siRNA (h): sc-89031

BACKGROUND

Alpha-L-iduronidase, also known as IDUA (iduronidase, alpha-L-) or MPS1, is a 653 amino acid protein belonging to the glycosyl hydrolase 39 family. Encoded by a gene that maps to human chromosome 4p16.3, alpha-L-iduronidase localizes to lysosome and is ubiquitously expressed. Alpha-L-iduronidase hydrolyzes terminal alpha-L-iduronic acid residues of two glycosaminoglycans, dermatan sulfate and heparan sulfate, thereby resulting in lysosomal degradation of both glycosaminoglycans. Alpha-L-iduronidase defects are linked to mucopolysaccharidosis type 1H (MPS1H), also known as Hurler syndrome, a rare lysosomal storage disease characterized by progressive physical deterioration, urinary excretion of dermatan sulfate and heparan sulfate, hepatosplenomegaly, skeletal deformities, corneal clouding, severe mental retardation, obstructive airways disease, respiratory infection and cardiac complications.

REFERENCES

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2. Scott, H.S., et al. 1992. Structure and sequence of the human alpha-L-iduronidase gene. *Genomics* 13: 1311-1313.
3. Scott, H.S., et al. 1992. Alpha-L-iduronidase mutations (Q70X and P533R) associate with a severe Hurler phenotype. *Hum. Mutat.* 1: 333-339.
4. Bach, G., et al. 1993. Molecular analysis of Hurler syndrome in Druze and Muslim Arab patients in Israel: multiple allelic mutations of the IDUA gene in a small geographic area. *Am. J. Hum. Genet.* 53: 330-338.
5. Scott, H.S., et al. 1993. Identification of mutations in the alpha-L-iduronidase gene (IDUA) that cause Hurler and Scheie syndromes. *Am. J. Hum. Genet.* 53: 973-986.
6. Clarke, L.A., et al. 1993. Two novel mutations causing mucopolysaccharidosis type I detected by single strand conformational analysis of the alpha-L-iduronidase gene. *Hum. Mol. Genet.* 2: 1311-1312.
7. Scott, H.S., et al. 1993. Multiple polymorphisms within the alpha-L-iduronidase gene (IDUA): implications for a role in modification of MPS-I disease phenotype. *Hum. Mol. Genet.* 2: 1471-1473.

CHROMOSOMAL LOCATION

Genetic locus: IDUA (human) mapping to 4p16.3.

PRODUCT

alpha-L-iduronidase siRNA (h) 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 alpha-L-iduronidase shRNA Plasmid (h): sc-89031-SH and alpha-L-iduronidase shRNA (h) Lentiviral Particles: sc-89031-V as alternate gene silencing products.

For independent verification of alpha-L-iduronidase (h) gene silencing results, we also provide the individual siRNA duplex components. Each is available as 3.3 nmol of lyophilized siRNA. These include: sc-89031A, sc-89031B and sc-89031C.

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

alpha-L-iduronidase siRNA (h) is recommended for the inhibition of alpha-L-iduronidase expression in human 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 alpha-L-iduronidase gene expression knockdown using RT-PCR Primer: alpha-L-iduronidase (h)-PR: sc-89031-PR (20 μ l). Annealing temperature for the primers should be 55-60° C and the extension temperature should be 68-72° C.

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.