The mice were created using the “plug and socket” method of gene targeting in murine embryonic stem cells to replace the two (cis) murine adult ß globin genes with a single copy of the human ßIVS-2-654 gene. The ßIVS-2-654 C-->T mutation accounts for approximately 20% of ß thalassemia mutations in southern China and causes aberrant RNA splicing and leads to ß0 thalassemia. The generated mice provide an animal model in which the antisense and other types of therapy can be tested in vivo and the first animal model of any disease resulting from a known human splicing mutation.

These mice are available at The Jackson Laboratory and can be found at https://www.jax.org/strain/003250.

Related Publications:

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