

Media contacts:

Joyce Peterson, The Jackson Laboratory, 207-288-6058, joyce@jax.org

Bryan deCastro, SMA Foundation, 631-495-9177, bdecastr@optonline.net

Jackson Laboratory to Distribute Mouse Models for Spinal Muscular Atrophy – the Leading Genetic Cause of Infant and Toddler Death

Bar Harbor, Maine – October 21, 2004 – The Jackson Laboratory is pleased to announce that it has received support from the Spinal Muscular Atrophy Foundation to make available the first group of mouse models for spinal muscular atrophy (SMA), a neuromuscular disease and the leading genetic cause of death among infants and toddlers. The mice will be available to the scientific community from the Laboratory in the first half of 2005.

Spinal Muscular Atrophy is a genetic motor neuron disease caused by the progressive degeneration of nerve cells in the spinal cord and brainstem, leading to muscle weakness, respiratory complications and premature death. Having been identified by researchers as the neurological disease closest to a treatment, the National Institute of Neurological Disorders and Stroke (NINDS) has selected SMA to serve as the prototype for a \$22 million translational research project that is expected to yield drug candidates for Investigational New Drug Application (IND) filing within three to five years. The SMA Foundation estimates that there are currently 25,000 to 55,000 people suffering from SMA in the United States, Europe and Japan and that the annual market potential for a drug to treat SMA could exceed \$500 million.

Three of the new mouse models were donated by Dr. Arthur Burghes of Ohio State University, and the fourth by Dr. Hung Li of the Institute of Molecular Biology in Taipei. Each of the models include a targeted mutation of the endogenous mouse *Smn* gene (survival motor neuron), combined with transgenes involving various forms of human *SMN2* and/or *SMN1*.

The donated models are named:

- FVB.Cg-Tg(SMN2*delta7)4299Ahmb Tg(SMN2)89Ahmb *Smn*^{tm1Msd}/J (Stock Number 005025)
- FVB.Cg-Tg(SMN2)89Ahmb Tg(SMN1*A2G)2023Ahmb *Smn*^{tm1Msd}/J (Stock Number 005026)
- FVB.Cg-Tg(SMN2)89Ahmb *Smn*^{tm1Msd}/J (Stock Number 005024)
- FVB.Cg-Tg(SMN2)1Hung *Smn*^{tm1Hung}/J (Stock Number 005058)

Researchers interested in obtaining any of the JAX[®] Mice should register interest using the respective stock number by visiting – http://jaxmice.jax.org/jaxmicedb/html/model_119.shtml. Additional information on SMA is available on the SMA Foundation web site, www.smafoundation.org.

About SMA Foundation

The SMA Foundation is a nonprofit organization founded in 2003 dedicated to finding a treatment and potential cure for Spinal Muscular Atrophy (SMA). The Foundation provides funding for the full range of research from basic to clinical work conducted in academic laboratories as well as corporate therapeutics development. Since its inception, the SMA Foundation has committed more than \$15 million to fund research. In addition, the Foundation is committed to raising awareness, education and increased federal funding and support. For more information on the Spinal Muscular Atrophy Foundation, visit www.smafoundation.org or call (646) 253-7100.

About Jackson Laboratory

The independent, nonprofit Jackson Laboratory, founded in 1929, is the world's largest mammalian genetics research facility. The Laboratory is also the source of more than 2,800 stocks and strains of genetically defined mice, the home of the Mouse Genome Database and many other publicly available information resources, and an international hub for scientific courses, conferences, training and education.

#