

For immediate release

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**Daiichi Sankyo Announces Development of Nucleic Acid Treatment for Duchenne Muscular Dystrophy Utilizing Proprietary Technology**

**Tokyo, Japan (February 14, 2013)** —Daiichi Sankyo Co., Ltd. (hereafter, Daiichi Sankyo) announced today that it will establish a new company, Orphan Disease Treatment Institute Co., Ltd. (hereafter, Orphan Disease Treatment Institute), with Innovation Network Corporation of Japan (hereafter, INCJ) and Mitsubishi UFJ Capital Co., Ltd. (hereafter, MUC). Daiichi Sankyo will collaborate with the new company to undertake development of a treatment for Duchenne muscular dystrophy (hereafter, DMD) with the active ingredient ENA® oligonucleotide\*, a modified nucleic acid made using proprietary technology owned by Daiichi Sankyo.

INCJ will underwrite third party allocation of new shares for the new company with a maximum investment of 1.65 billion yen. The new company will also issue new shares by third party allocation for a fund managed by MUC. Daiichi Sankyo will invest in the new company and mainly conduct development with the goal of achieving proof of concept (POC) for clinical drug development.

DMD is known as a disease that affects one in 3,500 new-born males regardless of ethnicity. The onset of the disease occurs between the age of two and five, at first slightly affecting the ability to be self-reliant. DMD is associated with muscular atrophy which progresses with age, causing various impairments to mobility and finally resulting in death for many in their 20s and 30s. It is an extremely serious and rare hereditary X-linked recessive genetic disorder. It is known that DMD occurs because muscle cells do not produce dystrophin, but there is no fundamental or effective therapy available.

In 2006, professor Masafumi Matsuo (Kobe Gakuin University Department of Medical Rehabilitation) and designated professor Yasuhiro Takeshima (Kobe University Graduate School of Medicine Department of Pediatrics) were the first in the world to demonstrate the effectiveness of anti-sense oligonucleotides to restore dystrophin expression in DMD sufferers through the mechanism known as exon skipping (Pediatr. Res., 59:690-694, 2006). Daiichi Sankyo and Orphan Disease Treatment Institute will jointly conduct clinical and non-clinical studies with the cooperation

and support of these two professors with the aim of achieving POC.

\*ENA® oligonucleotides

ENA® is an ethylene-bridged nucleic acid in which ethylene is bridged at the furanose sugar ring at 2'-*O* and 4'-*C* ends. Short-chain nucleic acids and ENA® oligonucleotides found in ENA® demonstrate high binding force with complementary DNA and RNA as well as superior thermal stability and nuclease resistance. ENA® is a registered trademark of Daiichi Sankyo.