



Press Release

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Daiichi Sankyo Announces Phase 1/2 Clinical Trial Results for DS-5141 (Therapeutic Agent for Duchenne Muscular Dystrophy) in Japan

Tokyo, Japan (April 25, 2018) - Daiichi Sankyo Company, Limited (hereafter, Daiichi Sankyo) today announced the top-line results of the Phase 1/2 clinical trial in Japan (hereafter, the study) of DS-5141^{*1} (treatment drug for Duchenne muscular dystrophy, hereafter, the drug), which Daiichi Sankyo is jointly developing with the Orphan Disease Treatment Institute Co., Ltd. (ODTI)^{*2}.

The study is the first clinical trial to examine the safety and efficacy of the drug, which was administered subcutaneously once weekly for 12 weeks to patients with Duchenne muscular dystrophy (DMD)^{*3}.

No safety concerns, such as discontinuation or clinically significant adverse events, were observed in the study. The expression of dystrophin protein, the primary endpoint of efficacy, was partially identified, but was not clearly detected as a whole. However, the secondary endpoint of efficacy, the production of messenger RNA with exon 45 skipping of the dystrophin gene, was found in all patients.

Daiichi Sankyo will continue to develop DS-5141 to offer a new treatment option to patients with DMD as soon as possible since both its safety and the exon skipping activity of the dystrophin gene in all patients were observed in the study.

References

***1 DS-5141**

DS-5141, a nucleic acid drug expected to treat muscular dystrophy, skips exon 45 splicing, producing an incomplete but functional dystrophin protein during messenger RNA processing from the dystrophin gene in patient's myocytes. In addition, it contains ENA® oligonucleotide, Daiichi Sankyo's proprietary modified nucleic acid, as an active ingredient, and received the SAKIGAKE designation in April 2017.

***2 Orphan Disease Treatment Institute Co., Ltd. (ODTI)**

ODTI was founded in 2013 through joint investment with a fund run by the Innovation Network Corporation of Japan and Mitsubishi UFJ Capital Company Limited.

***3 Duchenne muscular dystrophy (DMD)**

Duchenne muscular dystrophy is a severe rare hereditary disease with an incidence of about one in 3,500 male newborns regardless of ethnicity. It is caused by the deficiency of dystrophin protein production in the patient's muscle cells, significantly limiting possible treatments and their effects.