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ASIA EDITION

Japan approves Sarepta's gene therapy to treat Duchenne Muscular Dystrophy

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Company is eligible to receive up to $103.5 M in near-term regulatory and commercial milestone payments



US-based Sarepta Therapeutics, Inc. has announced that the Japanese Ministry of Health, Labour, and Welfare (MHLW) has approved ELEVIDYS (delandistrogene moxeparvovec) for the treatment of Duchenne muscular dystrophy (DMD) under the conditional and time-limited approval pathway in Japan.

ELEVIDYS is approved for individuals ages 3- to less than 8-years-old, who do not have any deletions in exon 8 and/or exon 9 in the DMD gene, and who are negative for anti-AAVrh74 antibodies. This is the first global approval to include individuals younger than 4 years of age.

The approval is based on the efficacy and safety data for ELEVIDYS, which includes muscle health and longer-term functional results from the ELEVIDYS clinical programmes, including the two-year data from the global Phase 3 EMBARK clinical trial (Study SRP-9001-301).

The conditional and time-limited approval pathway in Japan provides for marketing authorisation in Japan for up to seven years for innovative medicines to treat serious conditions. To be eligible for the pathway, certain additional criteria must also be met, including early clinical trial results that have demonstrated significant efficacy and safety.

As part of a collaboration agreement signed in 2019, Sarepta is working with Roche to transform the future for the Duchenne community, enabling those living with the disease to maintain and protect their muscle function. Sarepta is responsible for regulatory approval and commercialisation of ELEVIDYS in the US, as well as manufacturing. Roche is responsible for regulatory approvals and bringing ELEVIDYS to patients across the rest of the world. Commercialisation of ELEVIDYS in Japan is through Chugai Pharmaceuticals via its alliance with Roche.