Orchard Statement on Strimvelis®, a Gammaretroviral Vector-Based Gene Therapy for ADA-SCID

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Orchard Therapeutics was notified earlier this week that a patient treated under a compassionate use program in 2016 with Strimvelis®, a gammaretroviral vector-based gene therapy approved by the European Medicines Agency (EMA) for the treatment of ADA-SCID, has been diagnosed with lymphoid T-cell leukemia. Preliminary findings suggest this diagnosis may be attributable to an insertional event related to treatment with Strimvelis. The patient is undergoing treatment for the leukemia at a specialty center, and we are conducting a full investigation to determine potential causality. Our thoughts are with the patient and family during this time.

Leukemia arising from the insertion of gammaretroviral vectors into the genome, a process known as insertional oncogenesis (or mutagenesis), is a known risk factor for gammaretroviral vector-based gene therapy and is described in the Strimvelis product information as a potential risk of treatment.

Strimvelis is the only gammaretroviral vector-based gene therapy in Orchard's portfolio. Each of Orchard's other pipeline therapies employ a self-inactivating (SIN) lentiviral vector-based approach that has been specifically designed to avoid insertional oncogenesis after administration. No evidence of insertional oncogenesis related to lentiviral vector-based hematopoietic stem cell (HSC) gene therapy has been reported in any indication.

Patient safety continues to be our highest priority, and Orchard has notified EMA and relevant local European regulatory authorities of this adverse event. Sixteen patients have been treated with Strimvelis since its approval in 2016, and no additional patients will be treated with the therapy before the investigation is complete. The company will determine the future of Strimvelis following discussions with relevant stakeholders and will provide further updates as appropriate.

For more information about Strimvelis, see the EMA website.