SLN124 granted Orphan Drug Designation by EMA for the treatment of β-Thalassemia

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LONDON, Silence Therapeutics, PLC (LON: SLN) ("Silence" or "the Company") a leader in the discovery, development and delivery of novel RNA therapeutics for the treatment of serious diseases, announces that its lead medicine candidate SLN124 has been granted Orphan Drug Designation by the Committee for Orphan Medicinal Products (COMP), the European Medicines Agency's (EMA) committee responsible for recommending orphan designations of medicines for rare diseases. This positive COMP decision has been adopted by the European Commission.

Following an application by Silence last year, the COMP concluded that SLN124 will be of significant benefit to those affected by the chronic and potentially life-threatening condition, β-Thalassemia. By receiving Orphan designation, SLN124 can benefit from expediated clinical development and ten years of market exclusivity, subject to approval. The COMP decision follows positive feedback from the UK Medicines and Healthcare products Regulatory Agency (MHRA) Scientific Advice meeting in June last year.

SLN124, which has been shown to lower serum iron levels and modulate tissue iron distribution in rodent models for β-Thalassemia and hereditary hemochromatosis, represents a promising therapeutic candidate for treating patients with iron overload disorders, including β-Thalassemia, myelodysplastic syndrome (MDS) and hereditary hemochromatosis.

Dr. David Horn Solomon, Chief Executive Officer of Silence Therapeutics, commented:
"We are pleased to receive Orphan Drug Designation for our iron overload disorder candidate, SLN124, which will assist in expediting clinical development as we progress our Phase Ib trial planned to begin in H2 2019. We believe that this innovative product offers significant promise for patients with iron overload disorders such as β-Thalassemia, MDS and hereditary hemochromatosis and we look forward to rapidly advancing SLN124 through clinical development."

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About Silence Therapeutics plc

Silence Therapeutics is developing a new generation of medicines by harnessing the body's natural mechanism of RNA interference, or RNAi, within its cells. Its proprietary technology can selectively inhibit any gene in the genome, specifically silencing the production of disease-causing proteins. Using its enabling delivery systems, it has achieved an additional level of specificity by delivering its therapeutic RNA molecules exclusively to target cells. Silence's proprietary RNA chemistries and delivery systems are designed to improve the stability of our molecules and enhance effective delivery to target cells, providing a powerful modular technology well suited to tackle life-threatening diseases. For more information, please visit: https://www.silence-therapeutics.com/