



Silence Therapeutics submits Clinical Trial Application for SLN124 Phase Ib First-in-Human study in  $\beta$ -Thalassemia and MDS patients, with the first patient expected to enter the clinic in Q3 2019

March 27, 2019

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, today announces that a Clinical Trial Application (CTA) for the Company's lead candidate SLN124 has been submitted to the UK Medicines and Healthcare products Regulatory Agency (MHRA).

SLN124, which has been shown to lower serum iron levels, modulate tissue iron distribution and ameliorate anemia in preclinical models for  $\beta$ -Thalassemia and Hereditary Hemochromatosis (HH), represents a highly promising therapeutic candidate medicine for patients with iron overload disorders, such as  $\beta$ -Thalassemia, Myelodysplastic syndrome (MDS) and HH. Subject to approval from the MHRA, Silence intends to begin a Phase Ib First-in-Human study in  $\beta$ -Thalassemia and MDS patients, with the first patient expected to enter the study in Q3 2019.

In January 2019, SLN124 was 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. Receipt of Orphan Drug Designation means that SLN124 is eligible for expedited clinical development and ten years of market exclusivity, subject to approval.

**Dr. David Horn Solomon, Chief Executive Officer of Silence Therapeutics, commented:** *"This is a very positive step forward for Silence Therapeutics as we continue preparations for our return to the clinic. With robust data generated in several preclinical disease models, favorable safety profile and patient-friendly administration, we believe that SLN124 is well positioned against current standard of care and other medicines in development for the treatment of iron overload disorders. The filing of this CTA with the MHRA is another positive step towards progressing this promising candidate into the clinic in Q3 2019."*

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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/>