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Translate Bio announces FDA clinical hold on IND

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The Company submitted the IND in December 2018 to support the initiation of a Phase 1/2 clinical trial in patients with OTC deficiency



Translate Bio, a clinical-stage messenger RNA (mRNA) therapeutics company developing a new class of potentially transformative medicines to treat diseases caused by protein or gene dysfunction has announced that the Company has received verbal notification from the U.S. Food and Drug Administration (FDA) about completion of the review of Company's Investigational New Drug Application (IND) submission for MRT5201.

The Company submitted the IND in December 2018 to support the initiation of a Phase 1/2 clinical trial in patients with OTC deficiency. The FDA is placing the IND on clinical hold until these questions are resolved. The Company expects to receive formal written communication with additional information from the FDA in the near future and plans to work with the FDA in an effort to resolve its questions as promptly as possible.

MRT5201 is designed to treat patients with OTC deficiency by intravenous delivery of mRNA encoding fully functional OTC enzyme to the liver to enable the hepatocytes, the predominant type of liver cell, to produce the normal OTC enzyme. MRT5201 has been granted orphan drug designation for the treatment of OTC deficiency in the U.S. and EU.