Register of Genetically Modified Organisms (GMOs)
Users in Ireland

The name and address of the notifier	Intellia Therapeutics, Inc. 40 Erie Street Cambridge MA 02139 USA
The location (including, where necessary, the name of townland or townlands) of a deliberate release proposed under, or granted consent in accordance with, Part II of the GMO (Deliberate Release) Regulations 2003, S.I. No. 500 of 2003 - Part B release.	Beaumont Hospital Beaumont Rd. Dublin 9 & St. James' Hospital James St. Dublin 8
Notification Ref. No.	B/IE/24/01
GMO Register No.	G0876-01
The date or dates of a deliberate release	December 2024 to 31 March 2030
The description and intended uses of each GMO involved	NTLA-3001 is an investigational medicinal product derived from a recombinant, replication incompetent adeno associated virus (AAV) viral vector with a serotype 8 capsid (AAV8), incorporating the following elements: • An expression cassette flanked by the wild type AAV serotype 8 (AAV8) inverted terminal repeats (ITRs) • Two unique copies of the *SERPINA1* gene encoding human alpha-1 antitrypsin (hA1AT) protein • A polyadenlylation (polyA) signal NTLA-3001 harnesses a CRISPR-mediated double strand DNA break to facilitate insertion of the transgene into a safe harbour locus to leverage an endogenous genomic promoter for transcription and protein expression.
The purpose of the deliberate release	NTLA-3001 will be administered to patients with Alpha-1 Antitrypsin Deficiency (AATD)-associated lung disease, caused by mutations in the *SERPINA1* gene, which encodes alpha-1 antitrypsin (A1AT) protein. The overall objective of the study is to evaluate the safety and tolerability of NTLA-3001 following a single treatment

	infusion in adult participants with AATD-associated lung disease.
The date of receipt of a notification or amended notification	31 August 2024
The date of publication of a notice under article 15(1)	
The number of representations, if any, received under article 16(1)	
The date of any request by the Agency for further information	
The date of receipt by the Agency of any further information	
The date of receipt, or the date on which the Agency otherwise became aware, of any information or any other matter referred to in article 22(1)	
The date of any exercise by the Agency of its powers under article 22(1)	
The date and nature of any decision by the Commission of the European Communities under Article 18 (1) or 23(2) of the Directive	
The date of withdrawal of a notification or an amended notification	
The date and nature of the decision by the Agency on a notification or an amended notification	