

Register of Genetically Modified Organisms (GMOs) Users in Ireland

The name and address of the notifier	Wellcome HRB Clinical Research Facility St James's Hospital James's Street Dublin 8 Children's Hospital Ireland Temple Street Rotunda Dublin 1
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.	Wellcome HRB Clinical Research Facility St James's Hospital James's Street Dublin 8 Patients will be followed up at: Children's Health Ireland Temple Street Hospital Temple Street Dublin 1
Notification Ref. No.	B/IE/20/01
GMO Register No.	G0726-01
The date or dates of a deliberate release	March 2020 - December 2020
The description and intended uses of each GMO involved	AVXS-101 is a recombinant, non-replicating, non-integrating, adeno-associated virus serotype 9 (AAV9) capsid shell containing the cDNA of the human Survival Motor Neuron (SMN) gene under the control of the cytomegalovirus (CMV) enhancer/chicken- β -actin-hybrid promoter (CB) as well as two AAV inverted terminal repeats (ITR) from the AAV serotype 2 (AAV2).
The purpose of the deliberate release	To administer AVXS-101 gene therapy treatment to paediatric patients diagnosed with spinal muscular atrophy (SMA). Patients will be treated and followed-up as part of the AveXis global Managed Access Programme.
The date of receipt of a notification or amended notification	25 th February 2020

The date of publication of a notice under article 15(1)	27 th February 2020		
The number of representations, if any, received under article 16(1)			
The date of any request by the Agency for further information	09/03/2020	Request for Further information	
The date of receipt by the Agency of any further information	10/03/2020	Further information Received	
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	13/03/2020	Consent conditions	Approved.
	13/03/2020	Consent conditions issued.	