

Register of Genetically Modified Organisms (GMOs) Users in Ireland

The name and address of the notifier	uniQure Biopharma BV Paasheuvelweg 25A 1105 BP Amsterdam Netherlands
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 or clinical trial.	St James's Hospital James's Street Dublin 8
Notification Ref. No.	B/IE/18/01
GMO Register No.	G0667-01
The date or dates of a deliberate release	1 st January 2019 – 31 st December 2021 Patients will be followed up for a further 5 years after treatment i.e. until 31 st December 2026.
The description and intended uses of each GMO involved	Recombinant serotype 5 adeno-associated virus (AAV) based vector containing the Padua variant of a codon-optimised human coagulation factor IX gene (AAV5-hFIXco-Padua). The gene encoding a normal human clotting FIX will be expressed in the hepatocytes of patients with severe or moderately severe haemophilia B. The gene is codon optimised to enhance hFIX expression and clotting activity and thus enable sustained levels of plasma FIX activity.
The purpose of the deliberate release	To test whether a single administration of AAV5-hFIXco-Padua is efficacious (i.e. that it has the ability to produce the desired or intended result) and that it is safe in patients with haemophilia B.
The date of receipt of a notification or amended notification	28 th June 2018
The date of publication of a notice under article 15(1)	5 th July 2018

The number of representations, if any, received under article 16(1)	0
The date of any request by the Agency for further information	31 st August 2018 14 th September 2018 17 th October 2018
The date of receipt by the Agency of any further information	10 th September 2018 16 th October 2018 1 st November 2018
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 th November 2018 – Approved 21 st November 2018 – Consent Conditions issued