**MHRA**
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

**gov.uk/mhra**

# Decision Cover Letter

## Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral and grant a waiver

MHRA-102538-PIP01-26

# Scope of the Application

## Active Substance(s)

ECUR combination of two nonreplicating recombinant adeno-associated virus vectors, libenucgene parvec (ECUR-506A) and rezotcagene parvec (ECUR-506D)

## Condition(s)

Treatment of ornithine transcarbamylase deficiency

## Pharmaceutical Form(s)

Solution for infusion

## Route(s) of Administration

INTRAVENOUS

## Name / Corporate name of the PIP applicant

iECURE

## Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, iECURE submitted to the licensing authority on 19/06/2026 17:51 BST an application for a Paediatric Investigation Plan

The procedure started on 26/06/2026 15:13 BST

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to agree a paediatric investigation plan and grant a deferral and grant a waiver

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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# Final Decision Letter

MHRA-102538-PIP01-26

Of 08/10/2026 09:27 BST

On the adopted decision for ECUR combination of two nonreplicating recombinant adeno-associated virus vectors, libenucgene parvec (ECUR-506A) and rezotcagene parvec (ECUR-506D) (MHRA-102538-PIP01-26) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on a paediatric investigation plan

This decision applies to a Paediatric Investigation Plan for ECUR combination of two nonreplicating recombinant adeno-associated virus vectors, libenucgene parvec (ECUR-506A) and rezotcagene parvec (ECUR-506D), Solution for infusion , INTRAVENOUS .

This decision is addressed to iECURE, 1777 Sentry Parkway, Building # 14, Suite 200, Blue Bell, UNITED STATES OF AMERICA, 19422

## ANNEX I

### 1. Waiver

### 1.1 Condition:

| |
|---|
| Treatment of ornithine transcarbamylase deficiency The waiver applies / applied to: Paediatric Subset(s): The paediatric population from 12 years to less than 18 years of age Pharmaceutical form(s): Solution for infusion Route(s) of administration: Intravenous use Reason for granting waiver: on the grounds that the specific medicinal product is likely to be unsafe. |

### 2. Paediatric Investigation Plan:

### 2.1 Condition(s):

| |
|---|
| Treatment of ornithine transcarbamylase deficiency |

## 2.2 Indication(s) targeted by the PIP:

| |
|---|
| Treatment of ornithine transcarbamylase deficiency |

## 2.3 Subset(s) of the paediatric population concerned by the paediatric development:

| |
|---|
| The paediatric population from birth to less than 12 years of age |

## 2.4 Pharmaceutical Form(s):

| |
|---|
| Solution for infusion |

## 2.5 Studies:

| Study Type | Number of Studies | Study Description |
|---|---|---|
| **Quality Measures** | 0 | Not applicable. |
| **Non-Clinical Studies** | 0 | Not applicable. |
| **Clinical Studies** | 3 | Study 1 (ECUR-506-OTC-101) Open-label, dose escalation and dose expansion trial to evaluate safety and efficacy of a single intravenous dose of ECUR-506 in male neonates and infants from birth to less than 9 months of age with genetically confirmed neonatal onset ornithine transcarbamylase deficiency (OTCD). Study 2 (ECUR-506-OTC-102) Open-label, dose escalation and dose expansion trial to evaluate the safety and efficacy of a single intravenous dose of ECUR-506 in male and female neonates and infants from birth to less than 24 months of age with genetically confirmed neonatal and non-neonatal onset OTCD. Study 3 (ECUR-506-OTC-201[LC1.1] [MT1.2]) Open-label, dose escalation (if required) trial to evaluate the safety and efficacy of a single intravenous dose of ECUR-506 in male and female children from 2 years to less than 12 years of age with genetically confirmed OTCD. |
| **Extrapolation, Modeling & Simulation Studies** | 0 | Not applicable. |

| | | |
|---|---|---|
| **Other Studies** | 0 | Not applicable. |
| **Other Measures** | 0 | Not applicable. |

## 3. Follow-up, completion and deferral of a PIP:

| | |
|---|---|
| **Concerns on potential long term safety and efficacy issues in relation to paediatric use:** | Yes |
| **Date of completion of the paediatric investigation plan:** | 31/12/2036 |
| **Deferral of one or more studies contained in the paediatric investigation plan:** | Yes |