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

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Decision Cover Letter

Decision of the licensing authority to:

grant a product specific waiver

MHRA-100848-PIP01-23

Scope of the Application

Active Substance(s)

messenger RNA encoding Cas9, single guide RNA targeting the human TTR gene

Condition(s)

Treatment for transthyretin amyloidosis (ATTR)

Pharmaceutical Form(s)

Suspension for infusion

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

Intellia Therapeutics, Inc.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Intellia Therapeutics, Inc. submitted to the licensing authority on 14/02/2023 20:26 GMT an application for a Waiver

The procedure started on 13/06/2023 20:20 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 grant a product specific 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-100848-PIP01-23

Of 12/07/2023 16:53 BST

On the adopted decision for messenger RNA encoding Cas9, single guide RNA targeting the human TTR gene (MHRA-100848-PIP01-23) in accordance with the Human Medicines Regulations 2012.

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

Granting a waiver in all age groups for the listed condition(s)

This decision applies to a Waiver for messenger RNA encoding Cas9, single guide RNA targeting the human TTR gene, Suspension for infusion , INTRAVENOUS USE .

This decision is addressed to Intellia Therapeutics, Inc., 40 Erie Street, Suite 130, Cambridge, UNITED STATES OF AMERICA, 02139

ANNEX I

1. Waiver

1.1 Condition:

Treatment of Transthyretin Amyloidosis (ATTR).

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of Transthyretin Amyloidosis (ATTR).

2.2 Indication(s) targeted by the PIP:

Treatment of Transthyretin Amyloidosis (ATTR).

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

The paediatric population from birth to less than 18 years of age.

2.4 Pharmaceutical Form(s):

Suspension for infusion

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures		
Non-Clinical Studies		
Clinical Studies		
Extrapolation, Modeling & Simulation Studies		
Other Studies		
Other Measures		

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

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	
Date of completion of the paediatric investigation plan:	
Deferral of one or more studies contained in the paediatric investigation plan:	