

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

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## **Decision Cover Letter**

### **Decision of the licensing authority to:**

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

MHRA-101521-PIP01-24

### **Scope of the Application**

#### **Active Substance(s)**

Derivative of 6-(piperidine-1-carbonyl)pyridine-3-ol

#### **Condition(s)**

Treatment of glomerulonephritis and nephrotic syndrome

#### **Pharmaceutical Form(s)**

Film-coated tablet; Age appropriate oral solid dosage form

#### **Route(s) of Administration**

ORAL USE

#### **Name / Corporate name of the PIP applicant**

Boehringer Ingelheim International GmbH

#### **Basis for the Decision**

Pursuant to the Human Medicines Regulations 2012, Boehringer Ingelheim International GmbH submitted to the licensing authority on 25/06/2024 14:16 BST an application for a

The procedure started on 03/09/2024 08:31 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-101521-PIP01-24

Of 08/10/2024 17:21 BST

On the adopted decision for Derivative of 6-(piperidine-1-carbonyl)pyridine-3-ol (MHRA-101521-PIP01-24) 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 for Derivative of 6-(piperidine-1-carbonyl)pyridine-3-ol, Film-coated tablet; Age appropriate oral solid dosage form , ORAL USE .

This decision is addressed to Boehringer Ingelheim International GmbH, Binger Strasse 173, Ingelheim am Rhein, GERMANY, 55216

## ANNEX I

### 1. Waiver

#### 1.1 Condition:

Treatment of glomerulonephritis and nephrotic syndrome The waiver applies / applied to:  
Paediatric Subset(s): The paediatric population from birth to less than 1 year of age  
Pharmaceutical form(s): Film-coated tablet  
Age appropriate oral solid dosage form  
Route(s) of administration: ORAL USE  
Reason for granting waiver: on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments

### 2. Paediatric Investigation Plan:

#### 2.1 Condition(s):

Treatment of glomerulonephritis and nephrotic syndrome

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

Treatment of steroid-resistant nephrotic syndrome (SRNS) due to primary focal-segmental glomerulosclerosis (FSGS), minimal-change disease (MCD), diffuse mesangial proliferative disease (DMP) or IgM-nephropathy (IgMN) in patients from 1 year to less than 18 years of age.

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

The paediatric population from 1 year to less than 18 years of age

## 2.4 Pharmaceutical Form(s):

Film-coated tablet Age appropriate oral solid dosage form

## 2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of an age-appropriate oral solid dosage form. Study 2 Food compatibility and in-use stability study.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 3 Open-label, randomised, active-controlled trial to evaluate pharmacokinetics, safety and efficacy of BI 764198 compared to standard-of-care in children from 1 year to less than 18 years of age with steroid-resistant nephrotic syndrome due to focal-segmental glomerulosclerosis, minimal-change disease, diffuse-mesangioproliferative disease, IgM-nephropathy or C1q deposit nephropathy with or without mesangial proliferation.
Extrapolation, Modeling & Simulation Studies	2	Study 4 Modelling and simulation study to select the dose of BI 764198, sample size and plasma sampling schedule for PIP study 3. Study 5 Population PK/PD model and exposure-response analysis.
Other Studies	0	Not applicable.
Other Measures	0	Not applicable.

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

<b>Concerns on potential long term safety and efficacy issues in relation to paediatric use:</b>	No
<b>Date of completion of the paediatric investigation plan:</b>	29/02/2036
<b>Deferral of one or more studies contained in the paediatric investigation plan:</b>	Yes