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-100339-PIP01-21

Scope of the Application

Active Substance(s)

Autologous selected renal cells

Condition(s)

Treatment of chronic kidney disease

Pharmaceutical Form(s)

Suspension for injection

Route(s) of Administration

Percutaneous use

Name / Corporate name of the PIP applicant

ProKidney LLC

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, ProKidney LLC submitted to the licensing authority on 22/11/2021 12:33 GMT an application for a Paediatric Investigation Plan

The procedure started on 18/02/2022 10:12 GMT

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-100339-PIP01-21

Of 24/03/2022 16:30 GMT

On the adopted decision for Autologous selected renal cells (MHRA-100339-PIP01-21) 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 Autologous selected renal cells, Suspension for injection , Percutaneous use .

This decision is addressed to ProKidney LLC, 8020 Arco Corporate Dr. Ste 118, Raleigh, United States, NC 27617

ANNEX I

1. Waiver

1.1 Condition:

Treatment of chronic kidney disease The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Suspension for injection Route(s) of administration: Percutaneous 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 chronic kidney disease

2.2 Indication(s) targeted by the PIP:

Treatment of patients with progressive chronic kidney disease resulting from congenital anomalies of the kidney and urinary tract aged 2 years to less than 18 years

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

The paediatric population from 2 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Suspension for injection

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable
Non-Clinical Studies	0	Not applicable
Clinical Studies	2	Study 1 (REGEN-005) Open-label, single-arm study to investigate the safety, tolerability, and effectiveness of renal autologous cell therapy (REACT) in children and adolescents from 2 years to less than 18 years of age with CKD Study 2 (REGEN-011) Open-label, dual-arm study to investigate the safety and efficacy of REACT in terms of superiority versus standard-of-care (SOC) in children and adolescents from 2 years to less than 18 years of age with CKD.
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:	31/12/2034
Deferral of one or more studies contained in the paediatric investigation plan:	Yes