

**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-100409-PIP01-21

### **Scope of the Application**

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

autologous dendritic cells pulsed with autologous tumour cell lysate

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

Treatment of high grade glioma

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

Cell suspension for injection

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

Intradermal use

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

Northwest Biotherapeutics Inc.

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

Pursuant to the Human Medicines Regulations 2012, Northwest Biotherapeutics Inc. submitted to the licensing authority on 21/02/2022 17:38 GMT an application for a

The procedure started on 28/03/2022 11:31 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-100409-PIP01-21

Of 16/08/2022 15:36 BST

On the adopted decision for autologous dendritic cells pulsed with autologous tumour cell lysate (MHRA-100409-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 for autologous dendritic cells pulsed with autologous tumour cell lysate, Cell suspension for injection , Intradermal use .

This decision is addressed to Northwest Biotherapeutics Inc. , 4800 Montgomery Avenue, Bethesda, United States, MD 20814

## ANNEX I

### 1. Waiver

#### 1.1 Condition:

Treatment of high grade glioma The waiver applies / applied to: Paediatric Subset(s): The paediatric population weighing less than 6 kg body weight Pharmaceutical form(s): Cell suspension for injection Route(s) of administration: Intradermal use Reason for granting waiver: on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible

### 2. Paediatric Investigation Plan:

#### 2.1 Condition(s):

Treatment of high grade glioma

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

Adjuvant treatment of high grade glioma after tumour resection and/or standard of care therapy

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

The paediatric population from 6 kg body weight to less than 18 years of age

### 2.4 Pharmaceutical Form(s):

Cell 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 Open-label, single-arm study to assess the safety and efficacy of autologous dendritic cells pulsed with autologous tumour cell lysate (DCVax-L) in male and female children and adolescents weighing at least 6 kg to less than 18 years of age with recurrent resectable glioblastoma. Study 2 Open-label, single-arm study to assess the safety and efficacy of DCVax-L in male and female children and adolescents weighing at least 6 kg to less than 18 years of age with newly diagnosed resectable glioblastoma.
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:	No
Date of completion of the paediatric investigation plan:	31/07/2028
Deferral of one or more studies contained in the paediatric investigation plan:	Yes

