

# Medicines and Healthcare products Regulatory Agency

## MANUFACTURER'S AUTHORISATION

<b>1: Authorisation Number</b>	UK MIA(IMP) 17328
<b>2: Name of authorisation holder</b>	GREAT ORMOND STREET HOSPITAL FOR CHILDREN NHS FOUNDATION TRUST
<b>3: Address(es) of manufacturing site(s)</b>	ZAYED CENTRE FOR RESEARCH INTO RARE DISEASE IN CHILDREN, 20 GUILFORD STREET, LONDON, WC1N 1DZ, UNITED KINGDOM
<b>4: Legally registered address of authorisation holder</b>	GREAT ORMOND STREET HOSPITAL FOR CHILDREN NHS FOUNDATION TRUST, GENE AND CELL THERAPY, GREAT ORMOND STREET, LONDON, WC1N 3JH, UNITED KINGDOM
<b>5: Scope of authorisation and dosage forms</b>	ANNEX 1 and/ or ANNEX 2
<b>6: Legal Basis of authorisation</b>	Part 6 of The Medicines for Human Use (Clinical Trials) Regulations 2004 [SI 2004/1031]
<b>7: Name of responsible officer of the competent authority of the member state granting the manufacturing authorisation</b>	Confidential
<b>8: Authorisation Date</b>	11/10/2024
<b>9: Annexes attached</b>	Annex 1 and/or Annex 2

### SCOPE OF AUTHORISATION

#### Annex 2

Name and address of the site:

**ZAYED CENTRE FOR RESEARCH INTO RARE DISEASE IN CHILDREN, 20 GUILFORD STREET, LONDON, WC1N 1DZ, UNITED KINGDOM**

Human Investigational Medicinal Products
Authorised Operations
MANUFACTURING OPERATIONS (according to part 1) IMPORTATION OF MEDICINAL PRODUCTS (according to part 2)
<b>Part 1 - MANUFACTURING OPERATIONS</b> <b>[ 1.1 ] Sterile Investigational Medicinal Products</b> [ 1.1.1 ] Aseptically prepared (processing operations for the following dosage forms) [ 1.1.1.4 ] Small volume liquids

<ul style="list-style-type: none"> <li>[ 1.1.3 ] Batch certification</li> </ul>
<p><b>[ 1.3 ] Biological investigational medicinal products</b></p> <ul style="list-style-type: none"> <li>[ 1.3.1 ] Biological medicinal products <ul style="list-style-type: none"> <li>[ 1.3.1.3 ] Cell therapy products</li> <li>[ 1.3.1.4 ] Gene therapy products</li> </ul> </li> <li><b>Special Requirements</b> <ul style="list-style-type: none"> <li>Viral vectors <ul style="list-style-type: none"> <li>[ 1.3.1.5 ] Biotechnology products</li> </ul> </li> </ul> </li> <li>[ 1.3.2 ] Batch certification <ul style="list-style-type: none"> <li>[ 1.3.2.3 ] Cell therapy products</li> <li>[ 1.3.2.4 ] Gene therapy products</li> </ul> </li> </ul>
<p><b>Special Requirements</b></p> <ul style="list-style-type: none"> <li>Viral vectors <ul style="list-style-type: none"> <li>[ 1.3.2.5 ] Biotechnology products</li> </ul> </li> </ul>
<p><b>[ 1.4 ] Other investigational medicinal products or manufacturing activity</b></p> <ul style="list-style-type: none"> <li>[ 1.4.2 ] Sterilisation of active substances/excipients/finished products: <ul style="list-style-type: none"> <li>[ 1.4.2.1 ] Filtration</li> </ul> </li> </ul>
<p><b>[ 1.5 ] Packaging</b></p> <ul style="list-style-type: none"> <li>[ 1.5.2 ] Secondary packaging</li> </ul>
<p>Part 2 - IMPORTATION OF MEDICINAL PRODUCTS</p>
<p><b>[ 2.3 ] Other Importation Activities</b></p> <ul style="list-style-type: none"> <li>[ 2.3.2 ] Importation of Intermediate which undergoes further processing</li> <li>[ 2.3.4 ] Other <ul style="list-style-type: none"> <li>Importation of QP-certified IMPs from a country on the approved country for import list</li> </ul> </li> </ul>

**SCOPE OF AUTHORISATION**

**Annex 2**

Name and address of the site:

**GREAT ORMOND STREET HOSPITAL FOR CHILDREN NHS FOUNDATION TRUST, GENE AND CELL THERAPY, GREAT ORMOND STREET, LONDON, WC1N 3JH, UNITED KINGDOM**

Human Investigational Medicinal Products
Authorised Operations
<p>MANUFACTURING OPERATIONS (according to part 1)</p> <p>IMPORTATION OF MEDICINAL PRODUCTS (according to part 2)</p>
<p><b>Part 1 - MANUFACTURING OPERATIONS</b></p> <p><b>[ 1.1 ] Sterile Investigational Medicinal Products</b></p> <ul style="list-style-type: none"> <li>[ 1.1.1 ] Aseptically prepared (processing operations for the following dosage forms) <ul style="list-style-type: none"> <li>[ 1.1.1.4 ] Small volume liquids</li> </ul> </li> <li>[ 1.1.3 ] Batch certification</li> </ul> <p><b>[ 1.3 ] Biological investigational medicinal products</b></p>

[ 1.3.1 ] Biological medicinal products

[ 1.3.1.3 ] Cell therapy products

[ 1.3.1.4 ] Gene therapy products

[ 1.3.2 ] Batch certification

[ 1.3.2.3 ] Cell therapy products

[ 1.3.2.4 ] Gene therapy products

**[ 1.5 ] Packaging**

[ 1.5.2 ] Secondary packaging

**[ 1.6 ] Quality control testing**

[ 1.6.2 ] Microbiological: non-sterility

**Part 2 - IMPORTATION OF MEDICINAL PRODUCTS**

**[ 1.1 ] Sterile Investigational Medicinal Products**

[ 1.1.1 ] Aseptically prepared (processing operations for the following dosage forms)

[ 1.1.1.4 ] Small volume liquids

[ 1.1.3 ] Batch certification

**[ 1.3 ] Biological investigational medicinal products**

[ 1.3.1 ] Biological medicinal products

[ 1.3.1.3 ] Cell therapy products

[ 1.3.1.4 ] Gene therapy products

[ 1.3.2 ] Batch certification

[ 1.3.2.3 ] Cell therapy products

[ 1.3.2.4 ] Gene therapy products

**[ 1.5 ] Packaging**

[ 1.5.2 ] Secondary packaging

**[ 1.6 ] Quality control testing**

[ 1.6.2 ] Microbiological: non-sterility

**[ 2.3 ] Other Importation Activities**

[ 2.3.4 ] Other

Importation of QP-certified IMPs from a country on the approved country for import list