EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

APPLICATION FOR PAEDIATRIC INVESTIGATION PLAN / WAIVER

This application is submitted for a:

☒ Medicinal Product for which the Applicant does not hold a marketing authorisation in the EU, according to the Global Marketing Authorisation concept. (Article 7 of Regulation (EC) No 1901/2006). Note: this also covers future applications for a PUMA (in accordance with art. 30 of Regulation [EC] No 1901/2006), if the Applicant is not the marketing authorisation holder.

☐ Medicinal Product: 1) for which the Applicant already holds a Marketing Authorisation in the EU (according to the Global Marketing Authorisation concept); and 2) which is protected either by a Supplementary Protection Certificate (SPC) or by a patent which qualifies for the granting of a SPC; and 3) for which a future regulatory application will include one or more of the following (please specify all that will apply):

- ☐ New indication(s) (in adults and/or children)

  Please specify:

- ☐ New route(s) of administration

  Please specify:

- ☐ New pharmaceutical form(s) (intended for children or not)

  Please specify:

(Article 8 of Regulation EC No 1901/2006)

☐ Medicinal Product: 1) which is not covered by a Supplementary Protection Certificate or a patent which qualifies for the granting of a SPC; and 2) for which a Paediatric Use Marketing Authorisation will be sought (in accordance with Article 30 of Regulation EC No 1901/2006), when the applicant is the marketing authorisation holder.

IDENTIFICATION OF THE APPLICATION, DECLARATION and SIGNATURE

Name of the Active Substance(s) *(including salt, hydrate, ester or prodrug form, mixture of isomer, isomer if relevant. Do not use a proposed INN here; please use the recommended INN, EU Pharmacopoeia name, common name, or exact scientific/chemical name, in this order of descending preference). A proposed INN may be entered in A.3. (Please, use one line per active substance)*

Proposed Invented Name(s)
Please write "not available at present" if no invented name has been chosen yet

Pharmaceutical Form(s) that are authorised or under development *target populations*

Route(s) of administration
(corresponding to the pharmaceutical form)

Condition(s) (in adults and children) for which the product is being developed

Proposed Paediatric Investigation Plan Indication, if any
(corresponding to the condition)

Proposed Indications
(for a marketing authorisation application covering adults and/or paediatric population)

Population

Applicant

This application is related to another PIP application : ☐ Yes ☐ No

Please Indicate EMEA PIP number: Status:

Active substance name(s) in that PIP:

Language
The language of the PIP application, including the PDCO Opinion, is English. The language of the Agency Decision will also be English unless the applicant requests the Decision in one of the official languages of the Member State jurisdiction in which he is based.

If so, please specify language here:

The authentic text of the Opinion annexed to the Decision is the English version.

It is hereby confirmed that all existing data which are relevant for the request for agreement of the Paediatric Investigation Plan and request for Waiver have been included in the application and that the documentation provided in the request is an accurate account of the data obtained by the applicant.

_____

Signature of person authorised to act on behalf of the applicant

_____

Place and Date

Application Form

This form is to be used for a paediatric investigation plan, according to Chapter 3 of Regulation EC No 1901/2006, as amended. Please consult the `*Commission guideline on the format and content of applications for agreement or modification of a paediatric investigation plan and requests for waivers or deferrals and concerning the operation of the compliance check and on criteria for assessing significant studies*' when completing this form.

Please complete the following sections as appropriate.

THIS APPLICATION:
(SEVERAL BOXES CAN BE TICKED AS APPROPRIATE)

- ☐ includes a plan for development in the paediatric population or some subsets thereof (parts A, B, D, and F to be compiled)
- ☐ includes a Request for deferral(s) for one or more of the measures contained in the above plan for development (parts A, B, D, E and F to be compiled)
- ☐ includes a Request for waiver(s) (parts A, B, C, [D], [E] and [F] to be compiled)

Are you developing also in a **class-waived** condition? ☐ Yes ☐ No

If yes, please provide notes/additional information below

Part A - Administrative and Product Information

A.1 Name or Corporate Name and Address of the Applicant and Contact Person

Applicant

Name or corporate name of applicant:

Address (*P.O. boxes not acceptable*):

Postcode: City:

Country: Within EEA: ☐ Yes ☐ No

Does the applicant have SME status ? ☐ No ☐ Yes SME Number:

If different from Applicant
Person or Company responsible for Research and Development of the Medicinal Product

Name or corporate name:

Address:

Postcode: City:

Country:

Telephone: Telefax:

E-mail:

Please separate email addresses with a comma

Person authorised to communicate with the Agency during the procedure and after the decision

Name:

Company name:

Address:

Postcode: City:

Country:

Telephone: Telefax:

E-mail:

Please separate email addresses with a comma

The letter of authorisation by the applicant should be provided in the annex

Person authorised to upload 3rd country clinical trial data to EudraCT Copy from above

Name:

Company name:

Address:

Postcode: City:

Country:

Telephone: Telefax:

E-mail:

The letter of authorisation by the applicant should be provided in the annex.

Contact point of the applicant for public enquiries from interested parties

Contact point's E-mail:

Please separate email addresses with a comma

Country:

Telephone: Telefax:

A.2 Type of Medicinal Product

Please specify the type of product

- ☐ **Chemical**
  - ☐ Radiopharmaceutical
  - ☐ Antisense
  - ☐ Other
- ☐ **Bio(techno)logical**
  - ☐ Classical biological
    - ☐ Vaccine ☐ Enzyme derivative ☐ Blood derived ☐ Other biological
  - ☐ Recombinant DNA derived product
    - ☐ Vaccine ☐ Cytokine ☐ Hormone ☐ Monoclonal antibody
    - ☐ Transgene derived (animal/biopharm) ☐ Other recombinant
  - ☐ Nucleic acid-based
    - ☐ Gene Therapy ☐ DNA vaccine ☐ Oncolytic virus
  - ☐ Cell-based
    - ☐ Autologous ☐ Allogenic ☐ Xenogenic ☐ Transfected Cells
  - ☐ Tissue-based
    - ☐ Allogenic graft
- ☐ **Other** Please specify:

ATC Code:

☐ Assigned ☐ Pending ☐ Not yet assigned

Pharmacotherapeutic group (assigned or proposed):

Therapeutic field:

☐ Neurology ☐ Psychiatry ☐ Haematology-Hemostaseology ☐ Uro-nephrology
☐ Pain ☐ Oto-rhino-laryngology ☐ Gastroenterology-Hepatology ☐ Infectious Diseases
☐ Oncology ☐ Pneumology-Allergology ☐ Dermatology ☐ Diagnostic
☐ Vaccines ☐ Cardiovascular Diseases ☐ Ophthalmology ☐ Anaesthesiology
☐ Neonatology-Paediatric Intensive Care ☐ Nutrition
☐ Immunology-Rheumatology-Transplantation
☐ Endocrinology-Gynaecology-Fertility-Metabolism
☐ Other Please specify:

A.3 Name of the Active Substance(s)/Strength Unit(s)

The name of the active substance will be included on every PIP-related document exactly as shown below including on the final, published Decision.

Active substance	Nomenclature	Proposed INN	*Strength Unit*

A.4 Details of Medicinal Product

Proposed pharmaceutical form

Length of treatment ☐ Single dose ☐ Short term (<= 3 months) ☐ Long term (>3 months)

Dosages

Active Substance	Min. foreseen dose	Max. foreseen dose	Unit	Specification

New drug delivery system ◯ Yes ◯ No

Multi particulate (e.g: mini-tablets, granules) ◯ Yes ◯ No

Type of formulation proposed

Please select the type of formulation

☐ New paediatric formulation developed

☐ Extemporaneous formulation (i.e. magistral preparation) ☐ Adapted from adult formulation

☐ Industry verified (i.e. clinical trial enabling formulation) ☐ Same as adult formulation

Composition

Strength 1: - + -

Active substance	Quantity	Unit

Excipient Name	Quantity	Unit
		mg

General category for route of administration

Exact route of administration

A.5 Regulatory information on clinical trials related to the condition and to the development in the paediatric population

Please provide information on studies that will NOT be part of the PIP. Studies that are part of the PIP are to be submitted using the separate PDF file

*- For clinical trials conducted inside EU, please provide a table of **completed** clinical trials relevant to the condition(s) in paediatrics; report studies in adults **only** if relevant to the development in the paediatric population.*

*- For clinical trials conducted outside EU, please provide a table of **completed** clinical trials performed in paediatric patients only, and relevant to the condition(s).*

Reference Number *(e.g. EudraCT number)*

Region where the clinical trial is conducted

Protocol Number

Clinical trial status

Protocol title/ objectives

Study done according to GCP criteria?: ☐ Yes ☐ No

A.6 Marketing Authorisation status of the medicinal product

The product is not authorised anywhere in the world: ☐

Type of Marketing Authorisation

☐ Outside the EEA

Has there ever been a refusal / withdrawal / restriction of a marketing authorisation or extension application for this medicinal product in the EEA?

☐ Yes ☐ No

If yes, please specify ☐ Centralised ☐ Decentralised ☐ Mutual recognition ☐ National *(Tick all that apply)*

If yes, please specify the reasons for the refusal / withdrawal / restriction:

Outside EEA

Country		MA status	
Authorisation date		Invented name	
Full Indication (Adults/ Paediatrics)			
Strength		Pharmaceutical form	
Route of administration			

A.7 Advice from any Regulatory Authority, relevant to the development in the paediatric population

The applicant should list in this section all advice(s) received, or pending or planned, from any regulatory authorities, which is relevant to the development in the paediatric population; this includes advice(s) received for the adult population which may be of relevance for children, even if specific questions on paediatric development were not asked. If no advice/opinion/decision has been received, this should be specified.

Type

Please provide the corresponding documentation in the Annex

A.8 Orphan Drug Designation in the EEA

Do you intend to seek Orphan designation for this medicinal product?

☐ Yes ☐ No If yes, indicate planned submission date:

Has this medicinal product been designated as an Orphan drug by the European Commission?

☐ Yes, please indicate the Community Register of Orphan Medicinal Products number

☐ No

☐ Pending, please indicate Orphan designation procedure number

A copy of the decision on Orphan designation should be provided in the annex.

A.9 Planned application for Marketing Authorisation

Planned submission dates of application for marketing authorisation and future variations in the EEA

Date of completion of the adult pharmacokinetic studies specified in Section 5.2.3 of Part I of Annex I to Directive 2001/83/EC:

.
Please provide a justification below when no date can be provided *(either the date of completion or the justification must be entered)*:

Planned submission date of marketing authorisation application:

Intended route of submission: ☐ Centralised ☐ Mutual Recognition/Decentralised ☐ National MA (single MS) ☐ Not yet known

Planned submission dates of future regulatory procedures (if any):

Date	Type of submission	Indication

A.10 Annexes related to the administrative documentation where appropriate

- ☐ Letter of authorisation for the person authorised to communicate on behalf of the Applicant
- ☐ List and copies of literature references
- ☐ Copy of Scientific Advice given by CHMP
- ☐ Copy of Scientific Advice given by National Competent Authorities in EU
- ☐ Copy of Advice/Opinion/Decision given by competent authorities of third countries
- ☐ Copy of FDA written request
- ☐ Copy of the Commission Decision on Orphan Designation
- ☐ Copy of previous Agency decision on Paediatric Investigation Plan
- ☐ Risk Management Plan
- ☐ Investigator's Brochure

When completed, please save this form to your PC, then copy the saved file to a CD or DVD. The form should also be printed, signed in original, and then scanned as a separate (graphic) PDF file and saved. The scientific documentation listed below (B-E) and the PDF form for the proposed non-clinical and clinical studies should also be provided electronically, as separate document(s). The complete application (including attachments) should be submitted to the Agency as a CD or DVD containing all the files, via courier or registered mail (and also via Eudralink if urgent). For security reasons do not send the application by regular E-mail.Please note that no printed copies are necessary.

PART B - OVERALL DEVELOPMENT OF THE MEDICINAL PRODUCT INCLUDING INFORMATION ON THE TARGET DISEASES / CONDITIONS

B.1 SIMILARITIES AND DIFFERENCES

B.1.1 Discussion on similarities and differences of the disease/condition between populations (including information on prevalence/incidence)
B.1.2 Pharmacological rationale and explanation (including structure, absorption, PK, pharmacodynamics, metabolism, elimination; mechanism of action; similarities and differences of the safety and efficacy profile)

B.2 CURRENT METHODS OF DIAGNOSIS, PREVENTION OR TREATMENT IN PAEDIATRIC POPULATIONS

B.3 SIGNIFICANT THERAPEUTIC BENEFIT /FULFILMENT OF THERAPEUTIC NEEDS

PART C - APPLICATIONS FOR PRODUCT SPECIFIC WAIVER(S)

C.1 OVERVIEW OF THE WAIVER REQUEST(S)

C.2 GROUNDS FOR A PRODUCT SPECIFIC WAIVER

C.2.1 Grounds based on lack of efficacy or safety
C.2.2 Grounds based on the disease or condition not occurring in the specified paediatric subset(s)
C.2.3 Grounds based on lack of significant therapeutic benefit

PART D - PAEDIATRIC INVESTIGATION PLAN

D.I EXISTING DATA AND OVERALL STRATEGY PROPOSED FOR THE PAEDIATRIC DEVELOPMENT

D.I.a Paediatric Investigation Plan indication
D.I.b Selected paediatric subset(s)
D.I.c Information on the existing quality, non-clinical and clinical data

D.II QUALITY ASPECTS

D.II.a Strategy in relation to quality aspects
D.II.b Outline of each of the planned and/or ongoing studies and steps in the pharmaceutical development

D.III NON-CLINICAL ASPECTS

D.III.a Strategy in relation to non-clinical aspects
D.III.b Overall Summary Table of all non-clinical studies
D.III.c Synopsis/outline of protocol of each of the planned and/or ongoing non-clinical studies (use PDF table)

D.IV CLINICAL ASPECTS

D.IV.a Strategy in relation to clinical aspects
D.IV.b Overall Summary Table of all clinical studies
D.IV.c Synopsis/outline of protocol of each of the planned and/or ongoing clinical studies (use PDF table)

D.V TIMELINE OF MEASURES IN THE PAEDIATRIC DEVELOPMENT PLAN

PART E - REQUEST FOR DEFERRAL(S)

PART F - ANNEXES

Please provide:
- a list and copies of the literature references
- a copy of the investigator brochure (if available)
- a copy of the latest approved EU risk management plan (if available)

NB: this outline is based on the EU Commission "Guideline on the format and content of applications for agreement or modification of a paediatric investigation plan and requests for waivers or deferrals", which should be consulted before preparation of the application. Please note that in the present outline the numbering and order for the items in part D is slightly modified, for simplicity.