**Compassionate Use Program with** ***product name* for the treatment of *indication***

***Date – version***

|  |  |
| --- | --- |
| **Responsible of the program** | ***Name***  ***Address***  ***Phone***  ***Email*** |
| **Responsible physician of the program** | **Dr.**  ***Address***  ***Phone***  ***Email*** |

**TABLE OF CONTENTS**

[1. ABBREVIATIONS 2](#_Toc137570739)

[2. PURPOSE OF THIS DOCUMENT 2](#_Toc137570740)

[3. RATIONALE FOR THE COMPASSIONATE USE PROGRAM 2](#_Toc137570741)

[4. SCOPE OF THE COMPASSIONATE USE PROGRAM 3](#_Toc137570742)

[5. DRUG ELIGIBILITY CRITERIA FOR THIS COMPASSIONATE USE PROGRAM 3](#_Toc137570743)

[6. RECOMMENDATIONS 3](#_Toc137570744)

[7. PATIENTS ELIGIBILITY CRITERIA FOR THIS COMPASSIONATE USE PROGRAM 4](#_Toc137570745)

[8. AMENDMENT TO THE COMPASSIONATE USE PROGRAM 4](#_Toc137570746)

[9. DURATION OF THE COMPASSIONATE USE PROGRAM 5](#_Toc137570747)

[10. PROCEDURE OF DRUG DISTRIBUTION 5](#_Toc137570748)

[11. DOCUMENTS TO BE ARCHIVED 5](#_Toc137570749)

[12. SAFETY REPORTING 5](#_Toc137570750)

[13. MEDICATION 6](#_Toc137570751)

[14. FINANCIAL ASPECTS 7](#_Toc137570752)

[15. ETHICS COMMITTEE 7](#_Toc137570753)

[16. APPENDICES 7](#_Toc137570754)

# ABBREVIATIONS

CUP Compassionate Use Program

DSUR Development Safety Update Report

FAMHP Federal Agency for Medicines and Health Products

ICF Informed Consent Form

IMP Investigational Medicinal Product

MA Marketing Authorisation

# PURPOSE OF THIS DOCUMENT

1. To define the rationale of the Compassionate Use Program (CUP) with *product name* for the treatment of *the single* *indication*
2. To describe the conditions of the CUP with *product name* set up by *company name*
3. To define which patients are eligible for the CUP
4. To determine the procedures for each individual initial and follow-up request for *product name*
5. To provide instructions for reporting safety events
6. To determine the management of unused medicinal product
7. To indicate whether a cohort application will be requested from the INAMI/RIZIV

# RATIONALE FOR THE COMPASSIONATE USE PROGRAM

*Description of the disease and justification of the unmet medical need.*

*For the assessment of the unmet medical need only pharmaceutical treatments commercially available in Belgium and that are reimbursed with respect to the CUP indication should be considered.*

*To support the claim that an unmet medical need is met, the applicant is requested to provide the following:*

* *A critical review of available methods of prevention, medical diagnosis and treatment including a discussion of the (remaining) unmet medical need.*
* *A quantification of the unmet medical need taking into account technical arguments (e.g., quantifiable medical or epidemiological data). In addition, the number of patients estimated to be enrolled in the program on an annual basis is to be provided.*
* *A justification of the extent by which the medicinal product meets the unmet medical need through a critical discussion of scientific data, such as clinical trial data, that support a positive benefit-risk ratio of the medicinal product in the CUP indication.*

# SCOPE OF THE COMPASSIONATE USE PROGRAM

With this CUP, *product name* becomes available to a group of patients with *indication* who, according to the clinical judgement of the treating physician, could benefit from treatment with *product name*.

*Product name* is made available by *company name* only if  the responsible physician of this program, following an individual request from the treating physician, considers the patient to be eligible for the program. The initiation and implementation of the treatment with *product name* for a particular patient is the sole and complete responsibility of the treating physician.

Data generated within the CUP can be used for pharmacovigilance purposes only.

Only one indication can be considered per program.

# DRUG ELIGIBILITY CRITERIA FOR THIS COMPASSIONATE USE PROGRAM

☐ Clinical trial(s) is/are running with *Product name* :

|  |  |
| --- | --- |
| EudraCT reference | Title of clinical trial |
|  |  |

*or*

☐a Marketing Authorisation Application (MAA) has been submitted to EMA for *Product name* for the CUP indication.

*Please tick the appropriate statement*

# RECOMMENDATIONS

*The applicant must provide a critical overview of the relevant (international) clinical recommendations and guidelines for the CUP indication.*

# PATIENTS ELIGIBILITY CRITERIA FOR THIS COMPASSIONATE USE PROGRAM

Inclusion/Exclusion criteria

*As mandatory inclusion criteria :*

* The patient is not eligible for a clinical trial running with *Product name* or another Investigational Medicinal Product (IMP) in the CUP indication.
* The patient is not eligible for treatment or cannot be adequately treated according to clinical guidelines, because of efficacy and/or safety issues, with an alternative pharmaceutical therapy that is commercially available in Belgium AND that is reimbursed for the CUP indication.

*Inclusion/Exclusion criteria must be consistent with the recruitment criteria and results from clinical trials.*

*An overview of relevant clinical trials ongoing in Belgium for the CUP indication with Product name or with another IMP should be provided (see the table below). For each trial it should be indicated whether or not the CUP patient population is (partially) eligible for inclusion.*

|  |  |  |  |  |
| --- | --- | --- | --- | --- |
| EudraCT Number | Title of clinical trial | Phase | Indication | CUP population potentially eligible for inclusion in this trial? |
|  |  |  |  | Yes / No |

*In case ongoing clinical trials for the same indication are identified, and the CUP patient population is eligible for such trials, the existence of these trials should be clearly communicated to the treating physicians involved in the CUP as part of the CUP documentation and patients should first be offered the opportunity to participate in these trials.*

Additionally, prior to considering a patient for enrolment in the CUP, the following websites must be consulted for relevant clinical trials: [www.clinicaltrials.gov](http://www.clinicaltrials.gov)and [www.clinicaltrialsregister.eu](http://www.clinicaltrialsregister.eu)*.*

Patients must have been clearly and completely informed by the treating physician and have signed the informed consent form before treatment begins.

# AMENDMENT TO THE COMPASSIONATE USE PROGRAM

*Company name* has the option to amend the CUP. In case of substantial amendments to the initial program, *company name* should submit a file for amendment and the FAMHP will process the file according to the same procedure as the initial program application.

The history of amendments should be summarized in this section including the list of amended documents (date & version).

# DURATION OF THE COMPASSIONATE USE PROGRAM

The CUP will run from … (e.g. establishing the CUP or depending on cohort application or certain date or …) until … (e.g. the product is commercially available in Belgium in the envisaged indication or certain date or …) or for an estimated duration of … months, provided there is an unmet medical need.

*The program must end at the latest when the medicinal product is commercially available in Belgium for the indication of the program.*

At the time the program ends patients participating in the program must switch to the commercially available medicinal product. However as long as the medicinal product is not commercially available in Belgium for the indication of the program the applicant shall continue to provide the medicinal product to these patients that were already included in the program, according to the modalities of the closed program unless otherwise decided by the competent authority for scientific reasons.

Of note, the modalities of a program can be adapted upon request of the competent authorities at any time e.g. in case scientific data would necessitate such change.

The applicant should notify the FAMHP on any regulatory decision/outcomes with regard to the marketing authorisation status of the indication relevant to the CUP.

# PROCEDURE OF DRUG DISTRIBUTION

*As a minimum, the following information should be present describing the drug distribution procedure to be followed.*

* The treating physician checks ongoing clinical trials that could be suitable for the patients and checks the inclusion and exclusion criteria of the CUP.
* The treating physician submits a motivated request to the responsible physician, in written or electronic, to supply *product name* for an individual patient.
* The responsible physician verifies the inclusion/exclusion criteria and the treating physician’s motivation for enrolling this patient. In case of positive opinion, the responsible physician sends his/her agreement to the responsible of the program who will make *product name* available to the patient through the pharmacist. *Please add specific timelines (for the time between when the treating physician sends his/her request for inclusion of the patient to the responsible physician of the program AND when the responsible of the program makes his/her decision and if positive, makes available the medicinal product to the treating physician**).*
* *Please also describe the procedure for the follow-up request.*

# DOCUMENTS TO BE ARCHIVED

All documents related to this CUP (at least data registered in the central register of admitted patients and the Suspected Unexpected Serious Adverse Reactions (SUSARs)) will be archived by *company name* in Belgium for at least 10 years. Applications for admission of patients must be archived by the responsible physician for at least 10 years.

# SAFETY REPORTING

*A list of expected serious adverse reactions (Reference Safety Information or RSI) should be provided as illustrated in the table below:*

|  |  |  |  |  |
| --- | --- | --- | --- | --- |
| **System Organ Class**  **(MedDRA)** | SARs | Number of subjects exposed (N) = … | | |
| All SARs | Occurrence of fatal SARs | Occurrence of life threatening SARs |
| n\* (%) | n (%) | n (%) |
|  |  |  |  |  |

n = number of subjects who have experienced the SAR

*As with clinical trials, previously observed events should be relied upon rather than what might be expected from the pharmacological properties of a medicinal product. In this way it can be determined whether or not a serious adverse reactions should be classified as a suspected unexpected serious adverse reaction (SUSAR).*

*Please add a sentence regarding the fact that section … of the IB provides further details on the safety profile of the drug.*

As a minimum the treating physician must report any SUSAR to the responsible physician listed on the front page of this CUP. A serious adverse event form should be attached to the protocol.

SUSARs recorded in this CUP will be listed in the Development Safety Update Report (DSUR) and in the latest version of the Investigator Brochure if applicable. To comply with the line listing requirement of art. 106 §5 alinea 3 of the Royal Decree dated 14/12/2006, additional SUSARs notified after the latest version of the DSUR will be provided in addition to this DSUR and the latest version of the Investigator Brochure.

Line listings should include SUSARs that occur worldwide in clinical trials with *product name* and in this CUP.

# MEDICATION

*Chemical and Pharmaceutical Characteristics of the product, specific recommendations such as product description, form and presentation, dosage schedule, storage conditions, route of administration, handling of unused medication…*

All unused medication should bereturned to *company name* or destroyed in an appropriate facility as soon as possible after the patient’s discontinuation from the CUP. Medication provided for an individual patient request under the CUP should be used for that specific patient only.

The statement « CU – cannot be sold » must be displayed on the secondary packaging in the 3 national languages, in addition to the requirements of annex 13 of the Good Manufacturing Practices Volume 4.

**The product intended for use in the Compassionate use program is the same product as that referred to in the rationale.**

# FINANCIAL ASPECTS

*Product name* must be provided to the patient free of charge by *company name*.

A cohort *is/ is not* requested from the INAMI/RIZIV.

# ETHICS COMMITTEE

*Company name* proposes the following Leading Ethics Committee (LEC):

Name …………………………………………………………………………………………………

Address……………………………………………………………………………………………….

………………………………………………………………………………………………..

Telephone …………………………………………………………………………………………….

E-mail…………………………………………………………………………………………

The proposed Ethics Committee gives its opinion, at the request of the FAMHP, regarding the ICF and the ethical admissibility of the program. Approval of the program by the FAMHP implies a positive opinion from the EC.

The treating physician is advised to check whether the facility requires the notification of the local EC of his/her patient’s inclusion in the CUP.

# APPENDICES

* Physician Declaration form
* ICF
* Serious Adverse Event form