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

***Date – version***

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

1. **PURPOSE OF THIS DOCUMENT**

This document:

1. Defines the rationale of the Compassionate use program with *product name* for the treatment of the single indication *indication.*
2. Describes the conditions of the Compassionate use program with *product name* set up by *company name* under which *product name* will be made available by *company name* free of charge following a request from Belgian physicians to receive the product for the treatment of an individual patient.
3. Defines the patients’ eligibility criteria for this Compassionate Use Program.
4. Defines the procedures for each individual initial and follow-up request for *product name*.
5. Provides instructions on how to report safety events.
6. States if a demand for cohort will be requested at INAMI/RIZIV.
7. Defines how to manage the unused medicinal products.
8. **RATIONALE FOR THE COMPASSIONATE USE PROGRAM**

*Description of the disease and justification of the unmet medical need (e.g. clinical trials references supporting the feasibility of the CUP with protocol and results produced or marketing authorisation dossier if available) or others.*

*In order to support the claim that an unmet medical need exists, the applicant is requested to provide:*

*• A critical review of available methods of prevention, medical diagnosis or treatment, highlighting an unmet medical need*

*• Quantification of the unmet medical need taking into account technical argumentation (e.g., quantifiable medical or epidemiologic data). Provide also the number of patients expected to be included in the program.*

*• A justification of the extent to which the medicinal product addresses the unmet medical need”*

* *Scientific data supporting the positive benefit-risk balance, in particular evidence from clinical trials.*

1. **SCOPE OF THE COMPASSIONATE USE PROGRAM**

The aim of this Compassionate use program is to make *product name* available to a group of patients who suffer from *indication* and, in the opinion and the clinical judgement of the treating physician, would benefit from a treatment with the product which does not have a marketing authorisation yet.

*Product name* will only be made available by *company name* in case  the responsible physician gives a positive advice on the admissibility of the patient upon an individual request submitted by the treating physician. The initiation and conduct of the treatment with *product name* for a particular patient will fall under the full and only responsibility of the treating physician.

Any data generated within the Compassionate Use Program will only be used in the scope of pharmacovigilance.

Only one indication can be envisaged per program.

1. **DRUG ELIGIBILITY CRITERIA FOR THIS COMPASSIONATE USE PROGRAM**

*Product name* is undergoing a clinical trial with EudraCT reference \_\_-\_\_\_\_\_\_-\_\_ entitled “…” or *company name* has submitted a marketing authorisation dossier for *Product name* by centralized procedure at EMA in the envisaged indication as requested in the law on medicines modified on 01/05/2006, art.6quater §1 2°.

1. **RECOMMANDATIONS**

*The applicant should describe what are the international clinical guidelines recommendations in the indication of the program.*

1. **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* and/or a clinical trial running in the envisaged indication of this program.
* The patient is not eligible for treatment or cannot be treated satisfactorily with alternative therapy commercially available in Belgium AND reimbursed for the concerned condition, in accordance with clinical guidelines, because of efficacy and/or safety issues.

*An overview of relevant clinical trials ongoing in Belgium in the envisaged indication with Product name or with another product should be provided (see the table below). For each trial it should be commented whether the patient population of the Compassionate Use Program is (partly) eligible for inclusion in that trial.*

*In case running clinical trials in the same indication are identified, and the patient population of the program with an unmet medical need is eligible for such trials, the existence of these trials must be clearly communicated to the treating physicians involved in the MNP as part of the MNP documentation and patients must be offered first the possibility to participate in the concerned trials. Amongst others, relevant sources are* [*www.clinicaltrials.gov*](http://www.clinicaltrials.gov) *and* [*www.clinicaltrialsregister.eu*](http://www.clinicaltrialsregister.eu).

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

Patients should have been clearly and completely informed by the treating physician and have signed the informed consent form , before the start of the treatment.

1. **AMENDMENT TO THE COMPASSIONATE USE PROGRAM**

*Company name* has the possibility to review the Compassionate use program. In case of substantial changes to the initial program, *company name* will submit the dossier and the FAMHP will review the proposed changes following the procedure for an initial review.

The history of the amendments will be summarized in this section including the listing of modified documents.

1. **DURATION OF THE COMPASSIONATE USE PROGRAM**

*Product name* will be provided free of charge by *company name* on an individual patient basis following the criteria stated in this program from … (e.g. the set-up of the Compassionate Use Program, dependent on cohort request or …) until … (e.g. the product will be commercially available in Belgium in the envisaged indication or specified date or …) provided that there is an unmet medical need.

**Note regarding end of program**:

The program has to end at the latest at the moment the medicinal product is commercially available in Belgium for the indication of the program.

At the moment the program ends patients who are included in the program should switch to the commercially available medicinal product. If the medicinal product is not commercially available in Belgium the applicant will continue to provide the medicinal product following the modalities of the closed program.

For the assessment of the unmet medical need only treatments that are commercially available and reimbursed in Belgium are taken into account

Treatment duration must be in line with the supporting clinical trials (e.g. for treatments which were tested on a limited timespan).

The applicant commits itself to let the FAMHP know if the product has got   the (partial) Marketing Authorization or if the Marketing Authorization has been rejected.

1. **Procedure of drug distribution**

*The following information should be present as a minimum in the program to describe the procedures to follow for the drug distribution.*

* The treating physician will check ongoing clinical trials which could fit to the patients and check the inclusion/exclusion criteria of the program.
* The motivated request (respect of unmet medical need definition) by the treating physician for an individual patient supply of *product name*, will be sent to the responsible physician (in writing or by an electronic way)
* The responsible physician will check the inclusion/exclusion criteria and motivation of treating physician to enrol this patient. In case of positive advice, the responsible physician will send its agreement to the responsible of the program who will make available the product name to the patient through the pharmacist and/or the treating physician).Please add specific timelines (for the time between the moment the treating physician sends his/her demand for inclusion of the patient to the responsible physician of the program AND the moment the responsible of the program takes its decision and if positive, makes available the medicinal product to the treating physician).

1. **DOCUMENTS TO BE ARCHIVED**

All documents related to this Compassionate use program (at least data registered in the central registry of included patients and unexpected suspected serious adverse events) will be archived by *company name* in Belgium for at least 10 years. The demands for patient inclusion with annexes should be archived by the responsible physician for at least 10 years.

1. **SAFETY REPORTING**

A list of expected adverse reactions is provided below.

*As for clinical trials, this should be done from the perspective of events previously observed, not on the basis of what might be anticipated from the pharmacological properties of a medicinal product. By this way it will be possible to define if an adverse reactions must be classified as a suspected unexpected serious adverse reactions (SUSAR) or not.*

The treating physician should report at least any SUSAR to the responsible physician specified in the front page of this Compassionate Use Program. A serious adverse event form should be annexed to the protocol.

The SUSAR registered in this Compassionate Use Program will be mentioned in the Development Safety Update Report (DSUR) and in the last Investigator Brochure version if applicable. To comply with the line listing requirement of art. 106 §5 alinea 3 of the modified Royal Decree dated 25/4/2014, additional SUSARs notified after the last DSUR publication will be provided in addition to this DSUR and the last Investigator Brochure version

Line listings should include SUSAR occurring worldwide in clinical trials with *product name* and in this Compassionate use program.

1. **MEDICATION**

*Chemical and Pharmaceutical Characteristics of the product, specific recommendations e.g. description of the product, form and presentation, dose regimen, storage conditions, administration route, handling of unused medication…*

Any unused medication needs to bereturned to *company name* or destroyed in an appropriate facility as soon as possible after the patient’s discontinuation from the Compassionate use program. The medication delivered for an individual patient request in the context of a Compassionate use program can only be used for that particular patient.

The mention « CU-cannot be sold » will be present 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 to be used in the Compassionate use program is the same product as the product referred in the rationale.**

A cohort *will or will not* be requested at INAMI/RIZIV.

For information, the *company name* has already a CUP/MNP ongoing with *product name* for the treatment of *”other indication”* that was approved by the famhp on \_\_ /\_\_ / \_\_\_\_*.*

1. **Financial aspects**

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

1. **Ethics Committee**

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

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

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

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

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

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

The federal agency for medicines and health products will transfer the informed consent form as well as this protocol to the proposed LEC *ethics committee* in order to obtain their feedback to take into account in the final approval decision.

It is highly recommended that the treating physician informs the EC of the hospital where he practices about the inclusion of his/her patient in the CUP.

1. **APPENDICES**

* Physician Declaration form
* ICF