**Medical Need 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*** |

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# ABBREVIATIONS

MNP Medical Need Program

PSUR Periodic 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 Medical Need Program (MNP) with *product name* for the treatment of *the single* *indication*
2. To describe the conditions of the MNP with *product name* set up by *company name*
3. To define which patients are eligible for the MNP
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 MEDICAL NEED 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 reimbursed with respect to the MNP 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 annually in the program 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 balance of the medicinal product in the MNP indication.*

# SCOPE OF THE MEDICAL NEED PROGRAM

With this MNP, *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 MNP can be used for pharmacovigilance purposes only.

Only one indication can be considered per program.

# DRUG ELIGIBILITY CRITERIA FOR THE MEDICAL NEED PROGRAM

The medicinal product for human use is authorised in Belgium through a marketing authorisation (MA) and

a demand to obtain the MNP indication is being evaluated in a MA application, *or*

the MA has been granted for the MNP indication but the medicinal product is not yet marketed with the MNP indication, *or*

clinical evidence has been generated in (ongoing) clinical trials for the MNP indication.

*Please tick the appropriate statements*

# RECOMMENDATIONS AND GUIDELINES

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

# PATIENT ELIGIBILITY CRITERIA FOR THE MEDICAL NEED 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 MNP 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 MNP indication.

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

*An overview of relevant clinical trials ongoing in Belgium for the MNP 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 MNP patient population is (partially) eligible for inclusion.*

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

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

Additionally, prior to considering a patient for enrolment in the MNP, the following websites must be consulted for relevant clinical trials: <http://www.clinicaltrials.gov> and www.clinicaltrialsregister.eu.

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

# AMENDMENT OF THE MEDICAL NEED PROGRAM

*Company name* has the option to amend the MNP. In case of substantial amendments of 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 summarised in this section including the list of amended documents (date & version).

# DURATION OF THE MEDICAL NEED PROGRAM

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

***Please take these rules into consideration regarding the end of the program:***

*The program must end at the latest when the medicinal product is commercially available in Belgium for the indication of the program unless the medicinal product is commercially available for another indication and the reimbursement procedure for the indication of the program is ongoing. In this case, the program must stop when the reimbursement procedure has ended (regardless of its outcome) or when the reimbursement file has been withdrawn.*

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 for free the medicinal product to those patients that were already included in the program, according to the modalities of the closed program unless decided otherwise by the competent authority for scientific reasons.

Of note, the modalities of a program can be adapted upon request from 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/outcome with regard to the marketing authorisation status of the indication relevant to the MNP.

# PROCEDURE OF DRUG DISTRIBUTION

*As a minimum, the following information should be present describing the drug distribution procedures 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 MNP.
* 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 the patient. In case of a 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 and realistic 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 MNP (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 reaction should be classified as a suspected unexpected serious adverse reaction (SUSAR).*

*If the indication is not yet authorised, please add a sentence regarding the fact that section … of the IB provides further details on the safety profile of the drug.*

*If the indication is already authorised, please insert the information of section 4.8 of the SmPC as RSI.*

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

SUSARs recorded in this MNP will be listed in the Periodic Safety Update Report (PSUR) and in the latest version of the Investigator Brochure. To comply with the line listing requirement of art. 108 §5 alinea 3 of the Royal Decree of 14/12/2006, additional SUSARs notified after the latest version of the PSUR will be provided in addition to this PSUR 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 MNP.

# 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 MNP. Medication provided for an individual patient request under the MNP should be used for that specific patient only.

The packaging of *product name* must be the same as that of the *product name* authorised in Belgium.

**The product intended for use in the MNP 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 INAMI/RIZIV.

# ETHICS COMMITTEE

*Company name* proposes the following Leading Ethics Committee:

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 Ethics Committee.

The treating physician must check whether the facility requires the notification of the local Ethics Committee of his/her patient’s inclusion in the MNP.

# APPENDICES

* Physician Declaration form
* ICF
* Serious Adverse Event form