**Appendix 1**

**Instructions for Completing the Compassionate Use Application Form**

The request form consists of four pages containing five sections. Applicants/requesting Physicians are required to complete all five sections of the form each time a request is made for compassionate use of a product. It includes requests for renewals. The five sections are as follows:

**SECTION A: APPLICANT, PHYSICIAN AND SHIPPING INFORMATION**

**Applicant’s Details:** First and last name of the applicant, designation, contact information (for group CUAs)

**Physician’s Details:** If different from the applicant (for group CUAs)**,** first and last name of the responsible physician, and contact information. For an individual patient CUA the applicant is the responsible physician.

**Note:** The physician must be authorized by law to treat patients with any drug or drug substance intended for human use and requiring a prescription.

**Hospital or Clinic Name:** Full name of clinic or hospital where investigational medicinal product (IMP) or unregistered novel product is to be delivered when imported.

**Address:** Address of the physician’s office/clinic or hospital pharmacy where the IMP/un-registered novel product is to be delivered, including the city, and postal code.

**Contact Person:** Full name and position (e.g. Pharmacist, Nurse, Resident, etc.) of the person completing the form, if other than the requesting physician.

**Contact Telephone number:** A telephone number including an area code and extension (if applicable) where the practitioner or contact person can be reached if further information or follow-up is required.

**Contact’s email address:** An email address for the contact person should they need to be reached if further information or follow-up is required.

**SECTION B: INVESTIGATIONAL MEDICINAL PRODUCT/UN-REGISTERED NOVEL PRODUCT AND MANUFACTURER INFORMATION**

**Brand Name/Other Name:** Full name of IMP/ un-registered novel product, including international nomenclature (INN) and company designated code.

**Name of Manufacturer:** Full name of the manufacturer and location. Name and contact details (telephone number and email address) for the manufacturer or sponsor’s contact person. The manufacturer or sponsor’s contact person has agreed to supply the IMP/ un-registered novel product to the requesting physician. Evidence of this agreement should be attached to the application form.

**Route of Administration/Dosage Form:** Check the boxes that apply, or specify “other” if applicable.

**SECTION C: PATIENT INFORMATION FOR INDIVIDUAL OR GROUP REQUEST**

**Individual Request**

**Initials:** First, middle (if applicable) and last initials of the patient

**Note**: To ensure confidentiality, please do not indicate the patient’s full name.

**DOB:** specify the date of birth in order of date, month, year order (i.e. DD/MM/YYYY).

**Sex:** Check off the applicable box for the specified patient- **M**ale or **F**emale.

**Indication:** Exact medical indication for which the drug is being requested.

**New or Repeat Patient:** Check the applicable box indicating whether this represents an initial or repeat. request for the patient for the specific IMP/ un-registered novel product.

**Dosage and Duration:** Prescribed dosage including planned duration of therapy.

**Strength:** Required strength or combination of strengths.

**Quantity:** Precise number of tabs, vials, etc. requested for each patient.

**Total:** Sum of the quantities for all patients **Note:** Specify the exact amount requested (e.g. number of tabs, vials, units, etc.). **Your request will be returned if the amount is not clearly stated.**

**Group Request**

**Identity of the group:** with approximate number and age range, and any distinctive details.

**Indication:** Exact medical indication for which the drug is being requested.

**New or Repeat Group request:** Check the applicable box indicating whether this represents an initial or repeat request for the group for the specific IMP/ un-registered novel product.

**Dosage and Duration:** Prescribed dosage including planned duration of therapy.

**Strength:** Required strength or combination of strengths.

**Quantity:** Precise number of tabs, vials, etc. requested for the group.

**Total:** Sum of the quantities for all patients. Note: Specify the exact amount requested (e.g. number of tabs, vials, units, etc.). **Your request will be returned if the amount is not clearly stated.**

**SECTION D: CLINICAL RATIONALE**

**Question 1a) New Patients:**

Provide information about the patient(s)’s medical history, including the severity of their condition, prognosis as well as treatments considered, failed, unsuitable or unavailable to achieve an adequate response. Include a rationale indicating what about the requested IMP/ un-registered novel product makes it the best choice for your patient(s) (i.e. mechanism of action, dosage form, drug class)[[1]](#footnote-1)

**Question 1b) Repeat Patients:**

Provide information on your patient(s)’s condition since treatment was initiated, including a rationale for continued access. **Note:** this section should be updated each time a renewal is requested to ensure that the patient(s)’s current medical state is well described.

**Question 2) References:**

Provide **specific** data/references with respect to the safety and efficacy of the product that support the requesting physician’s decision to prescribe the IMP/un-registered novel product for the specified indication. This can be in the form of medical literature, clinical protocols, investigator brochures etc. Append copies of the reference(s) to the request form, and please tick the appropriate box.

**SECTION E: PHYSICIAN’S ATTESTATION**

Section E consists of three attestations for the requesting and /or responsible physician to acknowledge and sign off on for the CUA to be valid.

**Physician’s Signature:** Requesting/ responsible physician’s signature

**License number:** Requesting/responsible physician’s licence number (i.e. license to practice medicine as issued by the national licensing authority)

**Date:** Date when request is signed and submitted to the national regulatory authority (NRA).

**Processing of CUA Requests**

The applicant/requesting physician should submit the complete application form to the NRA, with a brief cover letter addressed to the head of the NRA.

The NRA and National Ethics Committee (NEC) will make every effort to jointly process a request as rapidly as possible, if possible, Group requests may require additional time. After consideration of a request, approval may be granted. The NRA will send an approval letter to the manufacturer/sponsor and a copy to the requesting physician. The requesting physician will be notified in the event that a request is denied.

Due to the urgency of CUA requests, applications should ideally, be submitted to, and approval letters received from NRAs electronically.

It is the responsibility of the applicant/requesting physician to contact the NRA in advance for related administrative information including submission options, the procedure, and possible fees.

**COMPASSIONATE USE APPLICATION**

**FORM A – PATIENT SPECIFIC REQUEST**

|  |  |
| --- | --- |
| **SECTION A: APPLICANT’S AND PHYSICIAN’S INFORMATION** | |
| Applicant’s Name: | |
| Applicant’s designation: | |
| Applicant’s telephone number: | |
| Applicant’s Email Address: | |
| If different from above  Responsible Physician’s name: | |
| Responsible Physician’s Telephone number: | |
| Responsible Physician’s Email address: | |
| Hospital or Clinic Name: | |
| Address: (shipping address only) | |
| City: | Postal Code: |
| Contact Person at Hospital/Clinic: (if other than responsible physician) | Contact Telephone number: |
| Contact’s Email Address: | |

| **SECTION B: IPM/un-registered novel product and Manufacturer Information** | |
| --- | --- |
| Brand Name: | Other Name/INN: |
| Manufacturer: | |
| Route of Administration: ORAL I.V**.**  I.M. TOPICAL S.C. OTHER: | |
| Dosage Form: TAB CAP LIQUID POWDER CREAM OINT. PATCH OTHER: | |

| **SECTION C: Patient Information** | | | | | | | | | |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  | | | | | | | | | |
| Patient Initials  (e.g. A.B.C.) | DOB  (DD/MM/YYYY) | | | Gender | Indication for Use of Drug | **N**ew or **R**epeat patient via the CUA for this IMP/un-registered novel product? | Dosage and Duration  (milligrams and days) | Strength  (mg) | Quantity  (e.g. number of tablets/vials/other units) |
|  |  |  |  | M  F |  | N  R |  |  |  |
|
|  |  |  |  | M  F |  | N  R |  |  |  |
|
|  |  |  |  | M  F |  | N  R |  |  |  |
|
|  |  |  |  | M  F |  | N  R |  |  |  |
|
| **Total:** | | | | | | | | |  |
| Please specify the EXACT AMOUNT of requested IMP/un-registered novel product (e.g. number of tabs, vials, units, etc.). | | | | | | | | | |

|  |
| --- |
| 1. Informed Consent:   Patient(s) has/have been informed about the risk/benefit of the IMP/ un-registered novel product and has/ have given appropriate consent.  Yes/No………………………………….   1. Proposed date for initiation of treatment:…………………. 2. Criteria for discontinuation of the treatment: |

| **SECTION D: CLINICAL RATIONALE** |
| --- |
| 1a) For each **new** patient, provide specific information about your patient(s)’s medical history including conventional therapies considered, ruled out and/or failed or that are unsuitable and/or unavailable to achieve an adequate response. What specifically about this drug (e.g. mechanism of action, drug class, dosage form) makes it the best choice for your patient(s)? Please explain.  Tick here if additional sheets are attached |
|  |
| 1b) For **repeat** patients, describe your patient(s)’s response to the drug relative to the initial treatment goal(s) and provide a rationale for requesting continued access.  Tick here if additional sheets are attached |
|  |
| 2. Please provide **SPECIFIC** data, references and/or resources in your possession, with respect to the use, safety and efficacy that support your decision to prescribe this IMP/un-registered novel product. For citations include journal/article titles, author(s), volume, issue, date and page information.  Tick here if additional sheets are attached |
|  |

| **SECTION E: Attestation** | |
| --- | --- |
| I, ………………………………………………………………………. the requesting physician/applicant for this CUA, am accessing this IMP/un-registered novel product for use in the emergency treatment of a patient/ patients under my care. | |
| I, …………………………………………………….………………the requesting physician/ applicant, am aware that by accessing this IMP/ un-registered novel product, I am responsible for the use of the IMP/ un-registered novel product. I have informed the patient(s) about the risks and benefits associated with the use of the IMP/un-registered novel product. The patient(s) has/ have given their written consent. I have attached a copy of the informed consent form used. | |
| I,… …………………………………………………………………the requesting physician/responsible physician, agree to provide a report on the results of the use of the IMP/un-registered novel product including information on Adverse Drug Reactions and, on request, to account for quantities of the IMP/ un-registered novel product received. | |
| **Practitioner’s Signature:** | **License number:** |
| **Date:** |

|  |  |
| --- | --- |
| **For Official Use** | |
| Date Received |  |
| Date Approved |  |
| Details: | |

1. In instances when a CUA request is for more than 4 patients, additional copies of the form should be filled out. All rationales should be patient specific. In such cases where additional pages are added, please number the pages appropriately. [↑](#footnote-ref-1)