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Revision No: 0

MINISTRY OF HEALTH

PHARMACY AND POISONS BOARD

GUIDELINES ON SUBMISSION OF DOCUMENTATION FOR COMPASSIONATE USE AUTHORIZATION (CUA) OF HEALTH PRODUCTS AND TECHNOLOGIES

JUNE, 2025

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HPT/PER/GUD/025	Guidelines on Submission of Documentation for Compassionate Use Authorization (CUA) of Health Products and Technologies	Revision :0	Effective date: 02/06/2025 Review Date: 01/06/2029

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ABBREVIATIONS

CUA	Compassionate Use Authorization
DPER	Department of Product Evaluation and Registration
EMA	European Medical Agency
GCP	Good Clinical Practice
GLP	Good Laboratory Practice
GMP	Good Manufacturing Practice
GxP	Good Practices e.g., GCP, GLP
ICH	International Council on Harmonization of requirements of Pharmaceuticals for Human Use
IMP	Investigational Medicinal Product
PPB	Pharmacy and Poisons Board
PS	Product Safety Department
PV/PMS	Pharmacovigilance/ Post Market Surveillance
RRA	Reference Regulatory Authority
SAE	Serious Adverse Events
SmPC	Summary of product characteristics
SRA	Stringent Regulatory Authority
SUSAR	Suspected Unexpected Serious Adverse Reaction
WHO	World Health Organization

QAO Quality Assurance Officer

GLOSSARY

For this guideline, the following definitions shall apply:

Compassionate use means approval for use in isolated cases of the individual(s) or subpopulations i.e., where an unregistered medicinal product is made available to "patients with a chronically or seriously debilitating disease, or life-threatening disease or condition, including chemical, biological or radiological harmful exposure and who cannot be treated satisfactorily using a registered product and who are not eligible or participant(s) in a clinical trial.

Marketing Authorization means approval i.e., license/ certificate of registration for a product to be marketed in a country. The term product registration may be used synonymously.

The Board- Pharmacy and Poisons Board

ACKNOWLEDGEMENTS

PPB acknowledges all the authors, stakeholders, and organizations who contributed to the preparation of this document.

FOREWORD

The Compassionate Use Authorization (CUA) Guideline has been developed to address situations where patients with serious, life-threatening, or rare conditions may require access to investigational or otherwise unauthorized health products or technologies for which no satisfactory authorized therapies are available. This guideline provides a regulatory framework that enables such access outside of clinical trials, under conditions that ensure patient safety, ethical oversight, and appropriate regulatory control. It seeks to balance the urgent medical needs of patients with the necessity of maintaining oversight of products that have not yet completed the formal registration process.

Rule 15 of the Pharmacy and Poisons Rules 2022 makes allowance for Compassionate Use Authorization. This legal provision underpins the compassionate use pathway and affirms the Board's commitment to facilitating timely access to critical health products, that are still under investigation or not yet registered in Kenya. The CUA guideline outlines the eligibility criteria, application process, supporting documentation, and post-authorization responsibilities required to operationalize this pathway effectively

1.0 INTRODUCTION

The Pharmacy and Poisons Board (hereinafter referred to as "the Board") is mandated under the Pharmacy and Poisons Act Cap 244 Laws of Kenya (hereinafter referred to as "the Act") to regulate Health Products and Technologies.

Ordinarily, before any consideration for the Marketing Authorization of a product, sufficient scientific and clinical evidence must be collected to demonstrate that it is safe, efficacious, and of suitable quality. The scientific evidence includes quality data, and safety and efficacy result from human clinical trials or non-clinical studies; it should be evident that the benefits of the product outweigh the risks associated. However, less sufficient information on quality, safety, efficacy/immunogenicity/performance may be accepted for individual health needs where there is no other available treatment/intervention. In such circumstances, additional mechanisms like; a risk-management plan, pharmacovigilance, and post-market surveillance for compliance verifications as well as investigations of potential health hazards shall be implemented.

Compassionate use Authorization shall be applied for use in isolated cases of individual (s) or subpopulations i.e., a group of patients, where an investigational medicinal product is made available to "patients with a chronically or seriously debilitating disease, or life-threatening disease or condition, and who cannot be treated satisfactorily using a registered product. Notably, this does not require a declaration of public health emergency to apply rather, it can be implemented whenever a situation occurs.

Products for compassionate use may be allowed to be used outside of clinical trials when: the disease is serious and life-threatening, no alternative treatment for the disease, the individual is not part of or eligible for the clinical trial, and the doctor justifies that there are no other options, and the experimental treatment may be of help.

The Board may grant compassionate use authorization, based on evidence of such approval by an SRA or WHO. In case the investigational medicinal

product is undergoing clinical trial studies within the country and where there has been no authorization by SRA or acceptance by WHO, the Board may approve for compassionate use upon advice from the mandated advisory scientific committees and/ or WHO.

This guideline shall be read with international guidelines on quality, safety, and efficacy, such as the international council on Harmonization of requirements of Pharmaceuticals for Human Use (ICH) guidelines.

1.1 Legal Framework

The Board is statutorily empowered to undertake various duties in the execution of its mandate regarding the regulation of health products and technologies. This includes regulation of clinical trials and marketing authorization. The regulation for the conduct of clinical trials is governed under the provisions of the Pharmacy and Poisons Act Cap 244 Laws of Kenya (hereinafter referred to as "the Act"), and the Subsidiary Legislation thereunder.

With respect to Marketing Authorization and Clinical Trials, the Board is empowered amongst others, under Section 3A of the Act to:

a) Grant or withdraw authorization for conducting clinical trials of medical products.
b) Grant or withdraw marketing authorization for medicinal products subject to appropriate conditions and revise such conditions for marketing as necessary.
c) Prescribe the standards appropriate for new medical products; new uses, dosages, and formulations of existing medical products; and such other categories as may be appropriate.
d) Constitute technical and expert advisory committees.

Further Section 3B of the Act mandates the Board to undertake the following with respect to clinical trials and compassionate use:

a) Approve the use of any unregistered medicinal substance for purposes of clinical trials and compassionate use.

b) Collaborate with other national, regional, and international institutions on medicinal substance regulation.
c) Advise the Cabinet Secretary on matters relating to the control, authorization, and registration of medicinal substances.

Rule 15 of the Pharmacy and Poisons Rules 2022 makes allowance for Compassionate Use Authorization.

This guideline shall be applicable to investigational medicinal products or technologies for purposes of compassionate use.

1.2 Scope

This guideline is intended to provide general considerations and guidance on content and format for required information for regulatory submission of Health products and Technologies for compassionate use authorization in Kenya.

Products for compassionate use may be allowed to be used outside of clinical trials when: the disease is serious and life-threatening, no alternative treatment for the disease, the individual is not part of or eligible for the clinical trial, and the doctor justifies that there are no other options, and the experimental treatment may be of help.

Compassionate Use Authorization shall be applied for use in isolated cases of individual (s) or subpopulations i.e., a group of patients, where an investigational medicinal product is made available to "patients with a chronically or seriously debilitating disease, or life-threatening disease or condition, and who cannot be treated satisfactorily using a registered product

Compassionate use is not a substitute for properly conducted trials. Compassionate use should therefore not slow down the implementation or continuation of clinical trials intended to provide essential information relative to the benefit/risk balance of a medicinal product.

Patients should always be considered for inclusion in clinical trials before being offered compassionate use programmes

Compassionate use does not refer to the use of an authorised medicinal product for an indication different from the one mentioned in section 4.1 of the summary of product characteristics (SPC), i.e. off-label use. Thus, this guideline does not cover the off-label use of health products and technologies.

1.3 Eligibility Of Candidate Products

The product categories to be reviewed under compassionate use shall include but are not limited to:

a) Medicines (therapeutics)
   i. Blood and Blood Product
   ii. Biotherapeutics products
   iii. Chemical products
b) Vaccines
c) Medical devices & In-vitro diagnostics (IVDs)

The product categories shall each have specific requirements for eligibility for evaluation under the CUA procedure.

To qualify for assessment under the CUA procedure the following criteria must be met: -

a) The disease for which the product is intended is serious, immediately life-threatening, and there are no registered products for the indication or a critical subpopulation.
b) The potential benefits of the product must outweigh the potential risks.
c) The product is manufactured in compliance with Good Manufacturing Practices (medicines & Vaccines) and under a functional Quality Management system (ISO standards) in the case of IVDs and Medical devices, and
d) Where applicable, the applicant undertakes to complete the development of the product (clinical trials in case of medicines & vaccines and validation and verification in case of IVDs).

2.0 APPLICATION SUBMISSION

2.1 Submission of applications

The applicant (for CUA) shall submit an application with a cover letter to the Pharmacy and Poisons Board, Department of Product Evaluation and Registration.

For the compassionate Use Authorization application process, a cover letter and supporting documentation shall be submitted. Please refer to the application form (Annex II and Annex III). Additionally, the applicant shall be required to submit an informed consent form (Annex IV), together with supporting documents as outlined in section 3 of this guideline.

The Board shall acknowledge receipt through email for all submissions.

The Board shall issue a rejection letter for applications that do not meet eligibility criteria.

2.2 Post Authorization Phase

The Board shall continually review submitted quality and safety reports submitted by the QPPV appointed by the applicant to collect and analyze reports on safety surveillance, efficacy/effectiveness/ performance monitoring, quality complaints, and other relevant data that may impact the validity of the Authorization.

The existing Pharmacovigilance and Post-marketing surveillance mechanisms shall be applied in effecting collection and dissemination of quality and safety surveillance, efficacy/effectiveness/ performance monitoring, quality complaints, and other relevant data.

The applicant should provide the following post-approval commitments in addition to meeting other Pharmacovigilance obligations as stipulated in the Guidelines on the safety and vigilance of medical products and health technologies (https://pharmacyboardkenya.org/pharmacovigilance)

1. Periodic benefit-risk evaluation report in accordance with ICH Guideline E2C(R2) Clinical Safety Data Management: Periodic benefit-risk evaluation report.
2. Applicants shall provide information on any ongoing phase I/II/III/IV studies or on any active monitoring of the safety profile that is taking place i.e
    a) Risk management plan.
    b) Vigilance reports to be frequently provided;
        i. Suspected unexpected Serious Adverse Reactions (SUSARS) including Adverse events following Immunization (AEFIs) shall be reported within 24 hours.
        ii. Serious Adverse Events (SAEs) shall be reported within seven (7) days.
        iii. The Board may institute active Surveillance or targeted Spontaneous reporting.

3.0 CUA APPLICATION PROCESS

3.1 CUA Application Minimum Information Requirements

In general, the following minimum information shall be submitted in any application for a CUA: -

a) A description of the product and its intended use (the serious or life-threatening disease, how the product is anticipated to be used, and /or the populations/individual for which the product is to be used.

b) Description of PPB's registration status i.e., whether the product is NOT registered or if registered the requested CUA is for an unapproved use. Whether the product or intended use is under an Investigational application (with PPB or SRA country), whether the product is authorized in an SRA country or WHO.

c) Detailed justifications should be provided to support the claim that the medicinal product meets the criteria for compassionate use.
   i. Available safety and effectiveness information.
   ii. A discussion of risks and benefits.
   iii. Product information equivalent to the Product information requirements as per established product specific guidelines.
   iv. Information on product stability, anticipated storage, and handling conditions.
   v. With regard to safety information;
      a) For Unapproved uses of already registered products. If the new indication uses a similar dose (or dose range as established through previous clinical trials), duration, route of administration, or mechanism of action, and the intended patient population is similar to the approved product, a right of reference to the registered product is applicable.
      b) Unapproved products. The available data may vary considerably. It is recommended that a CUA application should include preclinical testing data i.e., in vitro testing and animal toxicology data. The applicant is also encouraged to submit human safety information from clinical trials and individual patient experiences, if available. If only animal data (including data on

non-human primates) is available an extrapolation to humans shall be provided. Any safety information on humans on related compounds or devices should be provided.

c) In terms of efficacy, the assumptions for compassionate use may be based on mature randomised phase III trials.

d) However, acceptable assumptions may rely on promising early data observed in exploratory trials (e.g. uncontrolled phase II trials). In terms of safety, submission of all available data, which may contribute to refinement of the conditions for use defined in the opinion, is encouraged

vi. A discussion on Risk-benefit analysis shall include the following: -

a) Measures are taken to mitigate risks.

b) Uncertainties and data gaps.

c) Contraindications.

vii. The applicant shall provide a Pharmacovigilance plan and Risk management Plan.

viii. The applicant/facility/program shall obtain informed consent from the patient(s), individual(s), or Guardian.

ix. Any additional conditions/requirements as per the Pharmacy and Poisons (Registration of Health Products and Technologies) Rules, Part V (Miscellaneous) regarding Compassionate Use.

3.2 CUA application

The assessment of CUA applications shall involve the selection of key experts within the PPB, strategic planning, and oversight of systems to facilitate the implementation of CUA, particularly in cases where the application is made for a group of patients and involves more than one institution.

3.3 Format of submissions for CUA:

A CUA application shall include the following:

1. Cover letter

Applicants shall include a cover letter with all applications. A copy of the letter shall be placed at the beginning of the application. The cover letter should include details on the institution/manufacturer providing the health product

and/or technology, the recipient institution, and a list of all the documents submitted including their version numbers and date. Additionally, the cover letter shall include the statement to confirm that the material provided is true and accurate and shall be signed and dated.

2. Supporting documentation

The following additional documentation shall be submitted by the applicant:

a) A filled application form as per the format outlined in Annex II of this guideline.

b) A filled Applicant, Physician and Patient Information form outlines in Annex III of this guideline.

c) An informed consent from the patient(s), individual(s), Guardian, or a legal representative as per the format outlined in Annex IV of this guideline.

d) Patient(s) prescriptions, treatment protocol, and duration of treatment.

e) Any additional documentation outlined in the relevant clauses, and in section 3.1 mentioned above.

f) Any additional conditions/documentation requirements as per the Pharmacy and Poisons (Registration of Health Products and Technologies) Rules, Part III (Miscellaneous) regarding registration for compassionate use.

g) The applicant shall be required to provide a Pharmacovigilance plan and Risk management plan (Guidelines on the safety and vigilance of medical products and health technologies (https://pharmacyboardkenya.org/pharmacovigilance)

4.0 REGULATORY PROCESS

Upon submission of the application, screening of the application shall be conducted for acceptance of the application. Screening shall be done within the stipulated timelines in the service charter. Successful applications shall proceed for evaluation under the CUA. If no queries are raised the application shall be recommended for Compassionate use authorization.

After the initial submission of the CUA application with all the mandatory information for initial assessment, applicants are requested to promptly submit any additional information on the development of the product to the Board. Any unsatisfactory application may be rejected upon screening or an unsatisfactory response and feedback provided to the applicant. An applicant may request for withdrawal of the application at any point of the evaluation process in written form.

The compassionate use authorization shall be subject to but not limited to the following conditions: -

1. The allowable CUA quantities of product being requested shall primarily be based on the physician prescription and submitted treatment protocol. The maximum allowable CUA quantities/period of use shall be determined by pharmacy and poisons board on case-by-case basis to a maximum use period of 6 months. This includes:
    a) Therapeutic dose, frequency, and duration of treatment per patient.
    b) The proposed strength of the investigational product in relation to the therapeutic dose.
2. The applicant shall adhere to all commitments including submission of safety reports, risk management plans, and adherence to regulatory requirements, and safety and vigilance requirements on health products and technologies.

5.0 REGULATORY PROCESS TIMELINES

The timelines for this process shall be in accordance with the PPB Service Charter.

6.0 TERMINATION OF CUA

A CUA has a one-off use validity; renewable whenever applicable based on set conditions, or as determined by the Board as per the Pharmacy and Poisons (registration of drugs) rules.

7.0 REFERENCES

1. Guideline on compassionate use of medicinal products, pursuant to Article 83 of regulation (EC) No 726/2004 (EMEA/27170/2006)
2. Guidance document on Expanded Access to Investigational Drugs for Treatment Use, USFDA (November 2022)
3. AVAREF Guidance and considerations on Compassionate Use Access
4. Guideline for Compassionate Use in Austria (L_I217, 14/02/2025)

8.0 REVISION HISTORY

Revision No:	Date	Author/ Reviewer	Section(s) revised	Description of change
Rev 0	15th May 2025	QAO		Separation of EUA and CUA guideline. This guideline has been introduced to provide exclusive information on the Compassionate Use Authorization Procedure

9.0 LIST OF CONTRIBUTORS AND REVIEWERS

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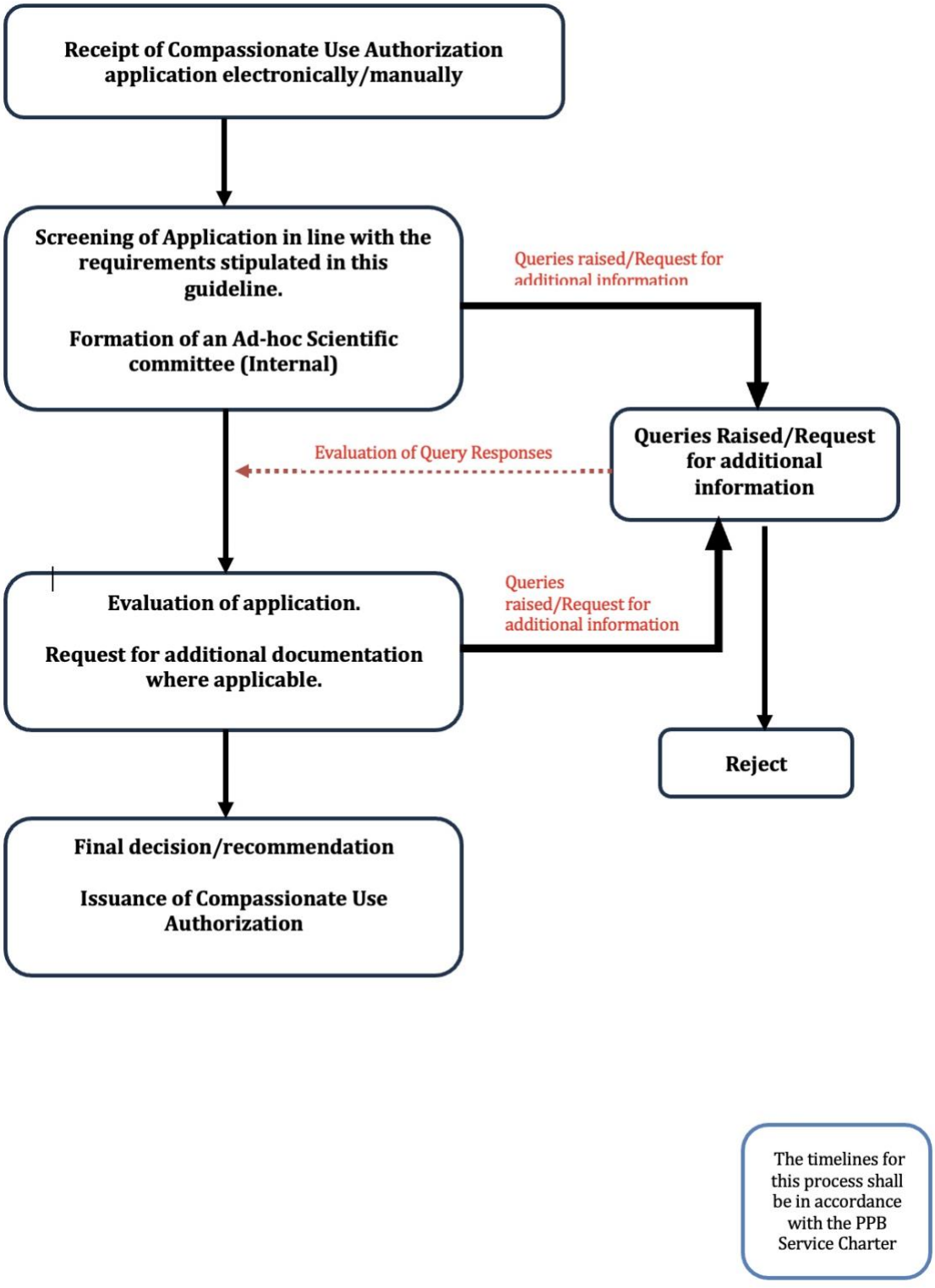
8. Dr. Anushree Patel Regulatory officer, DPER, PPB.

10.0 ANNEXES

Annex I. Flow chart of the CUA process.



Annex II: Application form for CUA

Application Form for Compassionate Use Authorization
Application No:
Active Substance:
Indication:
1. Description of the condition under which the HPT is to be used 1.1. Details of the condition 1.1.1 Definition 1.1.2 Aetiology 1.1.3 Specific characteristics; pathophysiological, histopathological, clinical characteristics 1.1.4. Classification 1.1.5 Diagnosis and symptoms 1.2. Proposed indication 1.3. Medical plausibility 1.3.1. Active substance: description of the medicinal product, pharmacological class and mode of action 1.3.2. Plausibility of the condition; data with the specific product as applied for designation in specific models or in patients affected the condition 1.4. Justification of the life-threatening or debilitating nature of the condition 2. Prevalence of the condition 2.1. Prevalence of the disease or condition in the Kenya 2.2. Prevalence and incidence of the condition in the Kenya 3. Other methods for diagnosis, prevention or treatment of the condition 3.1. Details of any existing diagnosis, prevention or treatment methods 3.2. Justification as to why methods are not satisfactory (Applicable/Not applicable. (Delete as appropriate) (Note that sections 3.2 and 3.3 are mutually exclusive.) 3.3. Justification of significant benefit Applicable/Not applicable. (Delete as appropriate) 4. Description of the stage of development 4.1. Summary of the development of the product 4.1.1 Quality aspects 4.1.2 Non-clinical aspects 4.1.3 Proof-of concept in relevant model 4.1.4 Pharmacology 4.1.5 Pharmacokinetics 4.1.6 Toxicology 4.1.7 Clinical aspects

4.1.8 Pharmacokinetics 4.1.9 Pharmacodynamics 4.1.10 Clinical efficacy 4.1.11 Dose-response studies and main clinical studies 4.1.12 Clinical studies in applied condition 4.1.13 Planned clinical studies 4.1.14 Clinical safety 4.1.15 Adverse events 4.1.16 Serious adverse events and deaths 4.2. Details of current regulatory status and marketing history in the Kenya and other countries
5. Applicant's position:

Annex III. Applicant, Physician and Patient Information form.

PHARMACY AND POISONS BOARD	FORM FOR COMPLETING THE COMPASSIONATE USE AUTHORIZATION APPLICATION	FOM 001/PER/MED/GUD/024 Rev. No.0

PLEASE READ THIS SECTION CAREFULLY BEFORE COMPLETING THIS FORM

i. The application form must be completed by an applicant (medical officer, pharmacist, or any authorized person by the respective Hospital Ethics Committee)

ii. Every application must be accompanied by the relevant supporting documents

iii. The Complete Application form shall be filled out for each request for Compassionate Use of a medicinal product, including Renewals.

SECTION A:

APPLICANT, PHYSICIAN, AND SHIPPING INFORMATION

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

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

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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 the investigational medicinal product (IMP) or unregistered novel product is to be delivered when imported.

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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.

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

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

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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.

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SECTION B:

UN-REGISTERED NOVEL PRODUCT AND MANUFACTURER INFORMATION

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

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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 unregistered novel product to the requesting physician. Evidence of this acceptance by the manufacturer/ sponsor should be attached to the application form.

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Route of Administration/Dosage Form: Key in the relevant information

SECTION C:

PATIENT INFORMATION FOR INDIVIDUAL OR GROUP REQUEST

1. Individual Request

Initials: First, middle (if applicable), and last initials of the patient. Note: To ensure confidentiality, indicate the inpatient number, outpatient number, or the unique patient identifier

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

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Sex: Check off the applicable box for the specified patient- Male or Female.

☐ Male ☐ Female

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

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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.

☐ New ☐ Repeat Patient

Dosage and Duration: Prescribed dosage including planned duration of therapy. Strength: Required strength or combination of strengths.

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

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1. Group Request

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

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

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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.

☐ New ☐ Repeat Patient(s)

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

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Strength: Required strength or combination of strengths.

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

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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 quantity is not clearly stated.*

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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)

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NB: Provide as attached the detailed information

Question 1b) Repeat Patients: Provide information on your patient(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.

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NB: Provide as attached the detailed information

Question 2) References: Provide specific data/references with respect to the safety and efficacy of the product that supports 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.*

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SECTION E:

PHYSICIAN'S ATTESTATION

This section consists of three attestations for the requesting and /or responsible physician to acknowledge and sign.

Physician's Signature: Requesting/ responsible physician's signature

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License number: Requesting/responsible physician's license number (i.e. license to practice medicine as issued by the KMPDC)

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Date:

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SECTION F:
INFORMED CONSENT

The application should be accompanied by a consent form for each patient.

Processing of CUA Requests

The applicant/requesting physician should submit the complete application form to the PPB with a brief cover letter addressed to the Board's CEO.

The Board shall make every effort to process a request as rapidly as possible. Group requests may require additional time. After consideration of a request, approval may be granted. The Board shall send an approval letter or notice of decline to the applicant /sponsor or requesting physician.

Due to the urgency of CUA requests, applications shall ideally, be submitted to, and approval letters received from the Board electronically/through hard copy. It is the responsibility of the applicant/requesting physician to contact the Board in advance for related administrative information including submission options, the procedure, and applicable fees.

Annex IV. Consent form for CUA

PHARMACY AND POISONS BOARD	INFORMED CONSENT FORM FOR COMPASSIONATE USE AUTHORIZATION	FOM002/PER/MED/GUD/025 Rev. No.0

INFORMED CONSENT FORM FOR COMPASSIONATE USE

If the patient in this compassionate use is under 18 years of age, the parental/guardian consent will be required.

1. Information about Compassionate Use Treatment and this Document

It has been determined that you have [Condition] which is of public health importance/life threatening/ severely debilitating. We believe that the [Health Product and Technology] may help you. There is currently no other available treatment that we believe would be as helpful for you.

[Health Product and Technology] is an investigational product. An investigational product is one that researchers are still studying to find out whether it is safe and effective. Because [Health Product and Technology] is under investigation, the Pharmacy and Poisons Board (PPB) has not yet registered it for general use.

[Health Product and Technology are not registered by the Pharmacy and Poisons Board (PPB) for your condition and therefore the use is under Compassionate Use.

The purpose of this consent form is to give you information about [Health Product and Technology] and to allow you to decide whether you want us to use it to treat you.

Please read this information carefully. It provides you with important information about the use of [Health Product and Technology]. This information shall be reviewed with you and if you have any questions, you can ask at any time.

To help you decide if you want to take part, you should know:

I. Consent for use of [Health product and technology] is voluntary.
II. You can choose to say NO.
III. You are free to change your mind at any time even after you have accepted.
IV. Your decision won't cause any penalties or loss of benefits to which you are otherwise entitled to.
V. Your decision won't change the access to the medical care you get now or in the future if you decide to say "NO or YES" now but change your mind later.

If you agree to the use of [Health Product and Technology], you need to sign this consent form to show that you want to take part. We shall give you a signed copy of this form to keep. A copy of this form shall be put in your medical records, and another copy shall be submitted to the Pharmacy and Poisons Board.

Include the below information for cognitively impaired adults taking part in this compassionate use:

I. The person being asked to take part in this compassionate use may not be able to give consent for the use. You are therefore being asked to permit this person as his/her decision maker.
II. While you are taking [Health Product Technology], we shall tell you if we learn of any new information that may cause you to change your mind about allowing this compassionate use.

NB: *Before you sign this form, be sure you understand how [Health Product Technology]relates to your condition, as well as the risks and possible benefits of using it.*

2. Information about the treatment

Dr. [Include name, address, phone] would like to treat your [condition] using the [Health Product and Technology], [if the medical device, include a

description of the device]. However, [Health Product and Technology] is not registered by the PPB for use in treating [condition]. Dr. [Name] is recommending the use of [Health Product and Technology] because S/he believes this is the best option to treat/manage your [condition] currently.

What will happen to you?
[Add information – in lay terms – of any procedures, blood tests, etc. that the patient will undergo as part of the treatment. Include an estimation of how long the treatment and any related follow-up shall last (e.g., duration of participation).]

3. Risks, Benefits, and other Alternatives

What are the risks of being treated with [Drug /Medical Device]?
[Describe reasonably foreseeable risks (including any risks to fetuses, if applicable). Include a statement that some risks may be unforeseeable. Include a statement about who to notify/what to do if side effects occur for example reporting to the PPB Pharmacovigilance reporting details] in case of ADRs.

What are the possible benefits of being treated with [Drug /Medical Device]?
[Describe anticipated benefits (e.g., cure of condition, minimizing severity/effects of a condition). Include disclosure that it is possible that the patient may not benefit]

What are the options if one does not want to allow compassionate use with (Drug/Medical Device)?
Inform the patient that s/he does not have to allow the compassionate use of the drug/medical device and that if s/he decides not to allow the use of the drug/medical device, his or her care at the hospital will not be affected.

What is usually done for patients who have this type of disease or condition?

Standard treatments for [Condition] include [list and describe standard treatments, if any]. We shall be glad to talk to you about your other treatment options.

You are free to stop using this [Health Product and Technology] at any time, and your treatment with it is voluntary. Before stopping, you should discuss your choice with your doctor, as stopping its use may pose additional risks to you that your doctor may need to manage. If you stop treatment before it is finished, there shall be no penalty or loss of benefits to which you may otherwise be entitled. If you decide to stop treatment before it is finished, please tell one of the persons listed in Section 6 "Contact Information" (below).

4. Costs associated with this treatment

[Select one of the following then delete the other option as well as all bracketed instructions:]

[if manufacturer is providing free Health Product and Technology:]

The [Health Product and Technology] shall be provided to you at no cost. You or your insurance company shall be responsible for the remaining costs related to this treatment,

including the cost of treatment if the [Health Product and Technology] makes you sick or causes you injury. You shall be responsible for any costs your insurance does not cover. If you have any questions about these costs, or what out-of-pocket expenses you may be responsible for, contact your insurance company.

(if the patient will be responsible for ALL costs)

You or your insurance shall be responsible for the cost of all care associated with the procedure[s] and the [Health Product and Technology] itself. This includes the cost of treatment. If [Health Product and Technology] makes you sick or causes you injury, it is possible that your insurance shall not pay for the cost of the *[include as applicable* Health Product and Technology*, the procedure to implant the device]* because the [Health Product and Technology]

is considered CUA. If that occurs, you shall be responsible for all costs, and these costs may be substantial.

By signing this form, you do not give up your right to seek compensation in case of professional negligence if you are harmed in the process of this treatment.

Shall you be paid to take part in this procedure?
You shall not be paid for taking part in this procedure.

5. Sharing of your information

If you give us permission to use [Health Product and Technology], we shall need to provide information about you, your condition, and your treatment to [Sponsor/Manufacturer Name], which is the manufacturer or supplier of the [[Health Product and Technology]] and to the Pharmacy and Poisons Board (PPB).

[Sponsor/Manufacturer Name] and the PPB require this information as part of the approval process for treatment use of [Health Product and Technology] and to monitor safety.

[Hospital Name], the Pharmacy and Poisons Board (PPB), and/or other ministry of health officials may also need to review your medical records to make sure that the [Health Product and Technology] is used in a safe and proper manner.

6. Contact Information

Who can I contact about this treatment?
Please contact the doctor to get the information listed below:

i. Obtain more information about the [Health Product and Technology]
ii. Ask a question about the [Health Product and Technology].
iii. Any adverse events that may be related to the use of the drug/device.

iv. Talk about treatment-related costs to you or your health plan.
v. Report an illness, injury, or other problem.
vi. Stop the treatment before it is finished.
vii. Express any concern.

(you may also need to tell your other doctors)

Doctor Overseeing Compassionate Use: [Name]
Mailing Address: [Address]
Telephone: [Phone]

When you call or write about a concern, please provide as much information as possible, including the name of the doctor providing treatment with the [HealthProduct and Technology], the title (at the top of this form), and details about the problem. This will help us look into your concern. When reporting a concern, you do not have to give your name unless you want to.

7. Signature

Consent

I have read (or it has been read to me) and fully understood the information in this consent document. I have had an opportunity to ask questions and all of my questions thus far have been answered to my satisfaction. If I have more questions or concerns, I may contact the person (s) listed in Section 6 of this consent document. I voluntarily agree to the use of [Health Product and Technology] for my treatment and understand that I can change my mind at any time. I do not give up any of my legal rights by signing this consent document.

Name:

Signature:

Date(dd/mm/yy):

If the patient is not able to consent themselves to the use of this investigational/ CUA, use the following signature block to obtain permission from a legally authorized representative or a parent

Guardian or Parent Permission

I have read (or it has been read to me) and fully understood the information in this consent document. I have had an opportunity to ask questions and all of my questions thus far have been answered to my satisfaction. If I have more questions or concerns, I may contact the person (s) listed in Section 6 of this consent document. By signing this form, I am voluntarily providing permission for the use of [Health Product and Technology] to treat [Patient Name] and understand that I can change my mind at any time. I do not give up any legal rights by signing this consent document.

Name:

_____

Signature:

_____

Date (mm/dd/yy):

_____

Relationship to patient: ☐ Parent ☐ Spouse ☐ Child
☐ Sibling ☐Legal Guardian ☐ Other (*specify*)

If "Other," explain the reason the patient is unable to consent: _____

Doctor

I have provided this patient and/or his/her legally authorized representative(s) with information about this compassionate use that I believe to be accurate and complete. The patient and/or his/her legally authorized representative(s) indicated that he or she understands the nature of the treatment, including the risks and benefits of its use.

Name:

_____

Signature:

Date(dd/mm/yy):

Annex V: Approval Letter for CUA

MINISTRY OF HEALTH

PHARMACY AND POISONS BOARD

Telegrams: "MINIHEALTH", Nairobi
PHARMACY AND POISONS BOARD HOUSE
Telephone: Nairobi 020 2716905/6 , 3562107
LENANA ROAD
Cellphone: 0733 – 884411/0720608811
P.O. BOX 27663-00506
Fax: 2713409
NAIROBI

When replying please quote
Ref. No.
Date:

Applicant/Sponsor/secretary hospital ethics committee

Dear Sir/Madam,

Re: **Compassionate use Authorization of ["health product and technology]" in Treatment/Diagnosis of [Disease /Condition**].

Reference is made to the above and your letter dated [].

The Board is cognizant that currently there is no registered medication or vaccine for treatment or prophylaxis of [Condition].

In view of this, the Board has considered and granted your request for Compassionate Use of (Product Name & strength) in [condition] cases in Kenya. The product details are hereby listed as follows:
Name of the product (brand Name):
INN name:
Name of the FPP manufacturer:
FPP site/s:
API site/s:

Yours Sincerely,

The office bearer & Signature

Title

<u>Conditions of authorization [page 2]:</u>

The Local Technical Representative (LTR):

1. The LTR shall ensure that all Adverse Events relating to the product, both at the authorized hospitals and out of the country are reported to the PPB promptly. Any such reports shall include any clinical data, including clinical trial data obtained in and out of the country.
2. The LTR shall maintain all such information and records in compliance with good records and documentation practice in case of any regulatory and other GXP inspections.

Authorized Hospitals:

1. The hospital shall ensure that all the patients or legal guardians sign the PPB-prescribed informed consent forms and as approved by the ethics committee in your institution, before administration of the product (Refer to Annexure V).
2. All adverse events shall be documented and reported every week, taking note of the requirement to report all fatal cases within 48 hours. Any such reports shall include concomitant medication(s) used. These reports shall be submitted through the Pharmacy and Poisons Board

Pharmacovigilance electronic reporting system at https://pv.pharmacyboardkenya.org. In addition to the individual case safety reports (ISCRs) submitted, a summary line list shall be submitted to the PV department (Refer to Annexure VI);

3. A cumulative summary of the number of patients given the product, patient outcomes, including negative conversion rate, time to body temperature normalization, death, need for ventilation and radiological progression where monitored, shall be submitted on a fortnight basis.
4. The hospital shall maintain and report information on product accountability.
5. Compassionate use of this medicine shall be well documented in the routine patient registry.
6. The quantities imported shall be aligned to the number of prescriptions or LPO submitted.
7. If a different therapy is registered (i.e., a marketing authorization has been issued) by the Board or there is compelling evidence against the use [health product and technologies] in treatment of [condition] the Board reserves the right to terminate this authorization.
8. Kindly note that this does NOT constitute a marketing authorization for the use of ((Product Name & strength).

Annex VI: Safety Monitoring guidance.

PHARMACY AND POISONS BOARD	REQUIREMENTS FOR SAFETY MONITORING	FOM 002/PER/MED/GUD/025
		Rev. No.0

Objective

The objective of the safety monitoring plan is to outline procedures for active pharmacovigilance during treatment of [condition] to ensure that healthcare professionals report all suspected adverse events (AEs) associated with medicines issued with Compassionate Use Authorization to facilitate early detection of safety signals to promote patient safety.

Monitoring Aspects

1. All adverse events shall be documented and reported as detailed in the National Pharmacovigilance guideline: (https://pharmacyboardkenya.org/pharmacovigilance)
   a. Suspected unexpected Serious Adverse Reactions (SUSARS) including Adverse events following Immunization (AEFIs) should be reported within 24 hours
   b. Serious Adverse Events (SAEs) should be reported within seven (7) days.

2. Any such reports should include concomitant medication(s) used. These reports shall be submitted through the Pharmacy and Poisons Board Pharmacovigilance electronic reporting system at https://pv.pharmacyboardkenya.org/. In addition, information on patients treated with any of the medicines during pregnancy shall also be reported with follow-up on the outcome of the pregnancy. To assist the PPB in the assessment of suspected adverse events reports, as much information as possible should be provided in the initial report, particularly under listed information (use the Adverse events reporting tools in the Pharmacovigilance Electronic Reporting System)

   Suspected drug

   I. The age and gender of the patient
   II. Description of the adverse reaction (including an indication of seriousness)

III. Patient's medical history (including any previously diagnosed/recently diagnosed conditions) e.g. diabetes, heart disease, chronic liver disease, asthma, HIV, active tuberculosis.

IV. Any concomitant medications, whether supportive or already prescribed

V. The outcome of the reaction (resolved, revolving, death)

VI. State whether the medicine was discontinued as a result of the adverse reaction

3. A line list of all the reported AEs should be prepared on an excel sheet using the format on the table below and submitted monthly.
4. All necessary laboratory parameters monitored must be documented and a list of out-of-range parameters reported before and after commencement of the use of [Medical Product and Health Technology].
5. Cumulative summary of the total number of patients (Including full demographic data) put on the Medical Product and Health Technology and the outcomes, e.g., negative conversion rate, time to body temperature normalization, death, need for ventilation, and radiological progression where monitored shall be submitted on a fortnight basis to Pharmacovigilance Division.
6. Information on product accountability shall be maintained and reported.
7. Your [Compassionate use] of this medicine should be well documented in the routine patient registry.
8. Adherence by the hospital to Good Records and Documentation practices to facilitate the reconstruction of the history of any related data in case of any regulatory decision and other GxP inspections.

LINE LIST FOR ADEs REPORTED WITH USE OF PRODUCT

No	Case Number	Case Report Type	Facility of Occurrence	Reporter Type	Age	Gender	List of Suspect Product Names	List of Concomitant Product Names	List of events	Case Level Serious	Case Relatedness	Case Narrative/ Description of the reaction	Case Outcome	Treatment Outcome
1														
2														
3														
4														
5														
6														
7														
8														
9														
10														

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Tel: +254-02-12345/6789, Fax: +254-02-12345,
Website: www.Pharmacyboardkenya.org.ke,
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