Patient Explainer: Compassionate Use

What is Compassionate Use?

Compassionate Use (referred to in this document as “CU”) helps people with serious and/or life-threatening illnesses get experimental, new medicines, when:

- Existing treatment options are limited or no other treatments are working, and
- Timely treatment is important.

These new medicines are not yet fully studied or approved for general use but most countries have CU programs that allow patients to get new medicines that could be helpful, especially for diseases like TB.

CU may also be called “pre-approval access”, or “expanded access programme.” It is not a clinical trial, meaning you do not have to participate in an experimental study to get the new medicine.

How does the Compassionate Use process work?

- Your doctor / a health organization have requested the CU medicine for you.
- Their request will be reviewed by a group of people (a committee of medical and affected community experts)—who will consider things like your condition, what medicines are an option for your situation, needs and preferences you might have for treatment, and so on.
- After carefully studying your situation, the committee will make recommendations to the drug company that made the medicine. Based on this the drug company will either:
    - Approve your application if the CU programme is suitable for you—meaning you can get the medicine through CU.
      OR
    - Recommend against giving you the CU medicine if it is not suitable or safe for your case (for example, if you are taking a medicine that could interact with the CU medicine and increase the risk of side effects).

What happens if you get access to the drug?

Process overview

- Your doctor will explain what happens in the CU programme so you can decide if you want to be part of the CU programme.
- If you agree to participate you will be given an informed consent form, which is a form that:
    - Gives you the information (benefits, risks etc) you need to decide whether you want to get a certain test or treatment; and
    - Records your permission for said test/treatment through their signature/thumb print, if you agree to the treatment being offered.
- Once you’ve signed this form, you will be part of the CU programme. You will be asked to take [Name of drug/drugs to be added as needed] along with your standard TB medication, as directed by your doctor/health provider.

- Your doctor will tell you when you have to take [Name of drug/drugs to be added as needed], in combination with which other TB medicines, for how long, and where you can receive the treatment (for example, at home or a health center/hospital).
- For the duration of the CU programme, in addition to your regular tests, your doctor may also prescribe certain other follow-up visits and tests. This is to see how the new medicine is working and to protect you from possible side effects.
- Your doctor may share relevant information, including test results, with [Name of the Drug Company to be added] as part of the CU programme. But all such information will be shared with your privacy protected and with your permission.
- The new medicine is free. However, you or the clinic may still have to cover regular doctor visits and standard TB tests. Please talk to your doctor/healthcare provider if you are worried about these costs.

Can the Compassionate Use medicine be bad for you?

- As of now there is no evidence to suggest this medicine is harmful in the long-term. But the medication has not been studied in the long-term, so this information is not fully known.
- The medicine may have these short term side effects: (insert drug-specific side effects). Because this medicine has only been formally studied for a short time, there may be other short-term side effects that we do not know of yet.
- That's why for your safety and well-being, your doctor will watch you closely through: a minimum of monthly visits, asking about your symptoms, testing your sputum/blood/other samples, and any other tests necessary.

What happens if you do NOT get access to the drug?

If you do not get the CU medicine, you may be worried about what to do next. But you're not alone. Speak with your doctor so you can both find a way ahead, together**. Even if you do not get access to the CU medicine, your doctor will still continue to provide you routine care. Your day-to-day care will not be affected.**

** If you have any questions about CU, please contact your doctor.* ***Please do not make any changes to your treatment without consulting your doctor/healthcare provider****.*