A Whitepaper from DDi

Direct-to-Patient (D2P): Clinical Supplies for Expanded Access

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mIRT

Direct-to-Patient (D2P):
Clinical Supplies for Expanded Access

D2P services have a main motto which is on *'Patient Centricity'* that would help in increase patient participation and retention in any clinical trial. This D2P support will be highly beneficial for clinical trials with drug therapies focused on Orphan Indications, Novel Therapies, Geriatric patient population, Emergency Situations and for '***Expanded Access Programs*** (EAPs)' also called as '***Compassionate Use Programs*** (CUPs)' for unmet medical needs. Expanded Access is for individual patients, including for emergency use, for intermediate-size patient populations and for widespread use.

Investigational Product (IP) should be used as part of a clinical trial. However, if patient enrollment is not possible (e.g., patient ineligibility, lack of ongoing clinical trials) or enrollment in a clinical trial is not feasible (e.g., distance to a trial precludes access), Compassionate Use offers a possible route for gaining access to an IP.

In a Compassionate Use scenario, D2P service is on an exception basis that would meet a critical need as the IND/ANDA are not approved yet prior to safety and efficacy establishment for market release. Some patient population may not be eligible for the clinical studies such as in Oncology and they would need medication to treat such emergency solutions as per Clinical Investigator's discretion/judgement. Compassionate Use is not restricted to cancerous conditions alone. Requests for such access are also received for nonlife threatening, but disabling conditions.

For such scenarios, D2P with Interactive Response Technology (IRT) platform (mIRT XPRESS tool of DDi) would be the best possible approach for study drug traceability, accountability and patient comfort/adherence. mIRT XPRESS supports 'IP Supplies Distribution' that can be managed at Site level as well as from Depot level with patient data privacy maintained at these said levels.

D2P approach will be a boon for EAP as most of the country regulations support such requirement on humanitarian/compassion grounds for best possible patient medical treatment. Compassionate Use regulations have been introduced in the US, Canada, many European countries, Australia and Brazil, and treatment on a compassionate use basis may be performed in Japan and China. Here are some of the definitions stated by various country regulatory authorities on EAP/CU/SAS/SAP.

According to the US FDA, 'Expanded Access' sometimes called "compassionate use", is a potential pathway for a patient with **an immediately life-threatening condition or serious disease or condition** to gain access to **an investigational medical product** (drug, biologic, or medical device) for treatment outside of clinical trials when no comparable or satisfactory alternative therapy options are available.

The World Health Organization (WHO) defines compassionate use (CU) as a "program that is intended to provide potentially life-saving experimental treatments to patients suffering from a disease for which no satisfactory authorized therapy exists and/or who cannot enter a clinical trial. For many patients, these programs represent their last hope."

The European Union (EU) has the CU program and the named patient program in place. The European Medicines Agency (EMA) provides non-legally binding recommendations through the Committee for Medicinal Products for Human Use.

CHINA

For China, compassionate drug use was first introduced in the second draft amendments to Drug Administration Law (DAL), which provided that if a clinical institution is conducting clinical trial of a drug to treat life-threatening disease with no comparable treatment options, the drug being tested can be available free of charge to other patients with the same conditions in the same clinical trial institution, provided that the drug being tested is likely to have benefits and has passed the ethical review and informed consent has been obtained from the patients. However, the "free of charge" condition has been removed from the final amendments to the DAL (Article 23).

BRAZIL

For Brazil, the Expanded Access Program (EAP) consists in providing a new, promising drug, still without Brazil, National Health Surveillance Agency (ANVISA) registration or not commercially available in Brazil, that is in developing or completed phase III study, aimed at a group of patients who are carriers of serious debilitating and/or life threatening diseases and with no satisfactory therapeutic alternative with registered products.

The Compassionate Use program consists in providing new promising drug, for personal use of patients and non-participants of expanded access program or clinical trials, still without ANVISA's registration, that is in process of clinical development, aimed at patients carriers of serious debilitating and/or life-threatening diseases and with no satisfactory therapeutic alternative with products registered in Brazil.

AUSTRALIA

For Australia, the Special Access Scheme (SAS) allows certain health practitioners to access therapeutic goods (such as medicines, medical devices or biologicals) that are not included in the Australian Register of Therapeutic Goods (ARTG) for a single patient. Therapeutic goods that are not included in the ARTG (and are not otherwise exempt from being in the ARTG) are described by us as 'unapproved'.

CANADA

For Canada, the Special Access Programme (SAP) allows practitioners to request access to drugs that are unavailable for sale in Canada. This access is limited to patients with serious or life-threatening conditions on a compassionate or emergency basis when conventional therapies have failed, are unsuitable, or are unavailable.

JAPAN

For Japan, 'compassionate use' system was established in 2016 pursuant to a notice of the Ministry of Health, Labour and Welfare (MHLW). Under that system, an unapproved drug that is being tested in a certain phase of a clinical trial (under the PMD Act) may be administered to a patient suffering from a disease that is not related to the indication specified in the clinical trial, provided that the requirements specified in the MHLW notice are met.

According to CDSCO, a new drug approved outside India can receive a waiver of clinical trial in the Indian population only in cases of national emergency, extreme urgency, epidemics, for orphan drugs in rare diseases, and conditions which have no therapy.

Compassionate use programs can only be put in place for medicines that are expected to help patients with life-threatening, long-lasting or seriously disabling illnesses. Such programs are expected to benefit seriously ill patients who currently cannot be treated satisfactorily with authorized medicines, or who have a disease for which no medicine has yet been authorized. The compassionate use route may be a way for patients who cannot enroll in an ongoing clinical trial to obtain treatment with a potentially life-saving medicine.

For instance, patients who have been treated with the medicine during a clinical trial and wish to continue treatment may be able to do so via an EAP. These programs are often authorized by national authorities in the same way as clinical trials, and patients are followed in the same way as patients in a clinical trial. Country Regulatory Agencies are increasing awareness about its 'EAPs'/ 'CUPs' process and the procedures for obtaining access to investigational drugs, biologics, and medical devices.

The implementation of IRT (mIRT XPRESS tool) for the above stated scenarios/situations shall give remarkable outcomes with D2P services.

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