# The Patient Cannot Afford To Wait

*Keith Watts. July 2016*

I have spent the overwhelming majority of my professional life in the pharmaceutical industry. I have watched, and to some degree engaged in the pharmaceutical industry's evolution over many years. It is my view that most pharmaceutical manufacturers are good corporate citizens. Most of these companies have a decent track record of protecting broad stakeholder interests. However, patients outside the United States who need urgent access to newly FDA approved medications, long before those products are commercially available in the patients' home country, are forgotten or simply ignored. Each year there are thousands of patients that have used up all commercially available medicines to treat a severely debilitating or life-threatening disease. Often the only remaining hope is for the treating physician to request the host country government to allow the patient to temporarily buy the product ahead of market approval in the host country. There is good news! Most countries, including the United States, recognize the need to give doctors temporary authorization to prescribe a product for a patient who has exhausted all available treatment alternatives. So, a compliant regulatory path often already exists allowing access to the needed medicines before these products receive market authorization and before commercialization. The bad news is that few pharmaceutical manufacturers understand these pathways, or refuse to recognize they exist. Even fewer have effective procedures in place to allow for urgent access to pre-licensed medicines.

I am actively involved with One-World Inc. (OWI), a company that specializes exclusively in navigating these temporary and special regulatory pathways that give doctors pre-commercialization access to treat patients who have exhausted existing medical alternatives. OWI is a respected and licensed exporter of specialty pharmaceuticals. Many manufacturers understand that OWI operates only through specific rules set up to allow temporary access to needed medicines before market authorization in a host country and in countries where market authorization or commercialization is not planned. These laws protect manufacturers' interests by allowing a compliant, although temporary, supply channel for needed medicines for which critically ill or disabled patients have exhausted all treatment alternatives. It is unlawful to promote or market these products during this period of temporary access to needed medicines or at any time before a company receives market authorization. OWI neither markets nor commercializes pharmaceutical products through this channel. In those countries where a product receives market authorization for an intended launch, OWI helps the transition to commercialization, but has no continued involvement.

I frequently hear of patients unable to access life-saving medicines, and with very little time to wait for market authorization, who could immediately benefit from existing temporary access regulations. Manufacturers often direct these patients to begin an application for compassionate use, or similar program with a different name, because it is the pharmaceutical company's policy

and an attempt to offer pre-license product access. Application to these programs tends to be cumbersome and time consuming. This process, however well-meaning, can be a death sentence for patients with little or no time left. OWI can provide product access more quickly and securely and report every data point expected and needed to secure the pharmaceutical supply chain.

A recent case occurred with a patient in a European country suffering from recurrent, metastatic colorectal cancer (CRC). As is typically the case, this patient failed on other drug regimens and had no alternatives. A regulatory compliant request came in from an authorized hospital clinic for a newly approved product, Lonsurf, for which the patient had already received one dose. The provider was having difficulty obtaining the product for the patient and called OWI, as OWI is a known licensed and qualified specialist serving this channel. I received a message the manufacturer had no process to address this need and no company serving these special access channels could buy the product, including OWI. The manufacturer seemingly has no provision for specialty distribution to address this situation. At this time, no dialogue between OWI and the manufacturer was achieved. OWI received communication on July 1, 2016 that the patient had died after waiting weeks to receive a second dose. Unintentional or intentional ignorance of special access pathways is inexcusable for patients who are running out of time! For example, the mean doubling time for CRC tumors is 92.4 days.[1] This translates into the possibility that CRC tumors could grow by 8% after only eight days without treatment. Of course, tumors do not grow uniformly or in conformity to means and statistics. However, forcing patients in dire need to wait when ethical and regulatory approved temporary access pathways exist is unnecessary and too often results in a patient death.

Over the past 18 months, OWI has documented at least 12 cases in which patients have died after failing to receive timely access to a well-known PD-L1 antagonist for treatment of metastatic melanoma requiring special handling. In each case, the manufacturer did not understand, or simply refused, specialty distribution's role in access to needed treatment. Ignorance, whether unintentional or forced by corporate policy, drives some patients to seek access through unauthorized suppliers with regard for special handling requirements. Ignorance of these specific, temporary regulatory pathways to quickly address urgent need can be deadly. Experienced specialty distributors devoted to this unique and important supply channel can cut out discrimination against a patient that precludes buying a needed medicine simply based on where the patient currently lives. There is a need for a secure and compliant supply chain that connects newly approved and innovative medicines to patients in countries legislating a lifeline for severely ill patients with no alternatives. These countries, large and small, recognize the need for a quick regulatory response allowing temporary access to needed medicine.

The takeaway for those cases OWI identified, there are no economic or regulatory barriers to buying the product. There is simply manufacturers' misunderstanding or seemingly willful ignorance of existing rules that run outside normal and familiar channels, designed specifically to

[1] Nomura, K., Miyagawa, S., Harada, H., Kitamura, H., Seki, H., Shimada, R., . . . Kawasaki, S. (1998). Relationship between doubling time of liver metastases from colorectal carcinoma and residual primary cancer. *Digestive Surgery, 15*(1), 21-24.

help patients who have no treatment choices left. There are cases of clearly designed business policy to withhold product in regions where there is no planned market authorization and commercialization. Pre-license product access for many pharmaceutical companies begins with a call to a toll-free phone number and expert help at the other end of the line. Unfortunately, patients often endure unneeded complexity, delays, and failure to obtain timely access to a product that already has an ethical access pathway legislated in the patient's home country. OWI, on behalf of critically ill patients, and for no compensation, has repeatedly tried to access product through compassionate use and existing processes required by certain manufacturers without success. Why then, are life-saving and life altering drugs blocked from reaching patients simply because of where they live and not because of economic or regulatory hurdles?