

There are treatments available for Orphan or Rare Diseases



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On the rare occasion I fall ill, I often assume and take for granted that there is some sort of medication available that will cure me or at least improve my symptoms. For common illnesses I find there are a variety of therapies and many different brands of similar products on the market, as much research and development is spent on improving medicines for the most common and well established illnesses. However, those suffering from rare or orphan diseases often can be left with very few treatment options.

A disease is classified as rare when there are less than a designated number of recorded cases. This number varies dependent on which country but as an example, within the EU a rare disease is classified by having less than 10,000 cases, and in the USA it is less than 200,000. Rough estimates suggest that there are 350 million people worldwide living with an orphan or rare disease. Globally, there have been 6000-8000 orphan diseases identified, yet only an estimated 500 of them have treatment options, not all of which are successful. That leaves a large number of patients

who have little to no treatment options. In particular, the Middle East has many unmet needs for orphan disease drugs. I recently read that among the estimated population of 400 million, around 2.8 million patients in the Middle East are suffering from a rare or orphan disease.

The pharmaceutical industry has actually witnessed a significant shift towards research and development of drugs for orphan and rare disease indications. Putting the patient first is at the heart of the industry, and with this in mind, most regulatory agencies across the world have allowed some flexibility in the supply process in order to provide patients-in-need access to life-changing medicines, without having to wait for approval or commercial launch. These methods come under the umbrella term 'Managed Access Programs' (MAPs). MAPs are a method of bridging the gap between clinical trials and commercial availability. They are put in place in order to provide patients with no other viable treatment options and an unmet medical need, the ability to access potentially lifesaving medicines at various stages of their product lifecycle.

The high cost of drug development, stringent regulations and a low return on investment can discourage pharmaceutical companies from investing money into the research and development of drugs for small populations of patients. Because of this, government involvement is necessary to support the orphan drug market. According to www.orphan-drugs.org, some governments have assisted hospitals in the GCC region with importing medication from other countries by offering reimbursement. Government incentives such as the FDA Orphan Grants Program, and the 10 years' market exclusivity and reduced regulatory fees offered by the European Medicines Agency, have allowed the orphan drug market to become an increasingly viable financial option for pharmaceuti-

cal companies. In addition to this, clinical trials involving orphan drugs tend to be smaller and shorter, and successful treatments are pushed through regulatory processes substantially quicker than medicines for more common indications. This means that orphan drug clinical trials are typically a less financially demanding investment.

MAPs can not only assist an innovator in providing potentially lifesaving treatment, the programs can also help identify or confirm patient populations, key physicians and opinion leaders, and provide an opportunity to gain access to real world treatment and usage patterns while collecting supplementary data. Durbin has been running Managed Access Programs and worldwide distribution programs for pharmaceutical and biotech companies on an exclusive basis for over 25 years. We have extensive knowledge of the regulatory requirements in the EMEA markets and rest of the world. I am extremely proud to say we have assisted in saving the lives of many people and will always strive to continue doing so! 

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