An exciting outlook for pre-approval access and compassionate use

How to deal with the ethical and practical considerations of the demand for promising new drugs that are in development

Most pharma and biotech companies are still getting to grips with how they will deal with the increasing demand from patients, patient's families and healthcare professionals for access to their promising new drugs that are in development.

The ethical and practical considerations are vast and complex in this area. However, up until recently, companies have been laser focused on researching and developing drugs, gaining marketing approval, managing the subsequent launch and, finally, guaranteeing widespread patient access.

Companies have been forced to rethink their development strategies over recent years as the environment has changed around them. Stronger and more united patient advocacy efforts, among other trends, have resulted in a situation where it is no longer deemed acceptable by many for companies not to have clear and transparent policies in place on how they will deal with patients seeking medicines that are in development outside the clinical trial setting.

The topic is high on the political agenda across global markets but no more so than in the US, where the 'Right to Try' movement is shining a spotlight on this issue. While there is an active debate as to whether or not this particular initiative is ultimately helpful to patients, it has certainly galvanised interest in and advocacy around the topic of patient access to drugs that are in development.

The challenges surrounding pre-approval access vary not only from country to country but also between large and small pharma companies. For a successful internal and external policy to be implemented successfully, there must be total organisational alignment, a task that is both resource intensive and extremely challenging for vast multinational pharma companies. The resources demanded (both in terms of manpower and money) are much more limited in smaller organisations. Particularly for pre-revenue companies, the associated risks of providing access to drugs in development may mean life or death, not only for patients, but also for these companies. And, of course, for a company to fail in the aftermath of the pre-approval access debacle may have immeasurable potential negative impact on future patient lives. These are, therefore, weighty issues, with both immediate and future implications.

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But there is a new wave of optimism and innovation in this space that is exciting to be a part of.


TOM WATSON

As more and more companies are realising, preparing for requests from patients and healthcare providers should be considered early on in development planning in order for robust plans to be put into place to address these needs and to avoid challenges further down the line.

Various projects across the industry are currently underway to establish fair and transparent mechanisms and recommendations on extremely difficult ethical decisions related to early access, including Janssen's independent review panel CompAC, run by the New York University School of Medicine's Division of Medical Ethics.

In addition, the power of patient groups in this area is one that is being utilised to great effect.

While opinion is often divided in this highly emotive area, continued multistakeholder collaboration is critical in order to provide patients with safe and effective treatments, both for an immediate (and often desperate) need and in the long term. Companies are realising that pre-approval access needs to be considered as a fundamental element of all early routine planning alongside clinical trial development.

I am constantly reminded of the dramatic positive impact that early access to critical treatments can have on the lives of patients and their families, if programmes are designed well and risks are managed effectively.

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