Orphan Drugs

Possibility and Peril

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Orphan drugs - possibility and peril

Recent controversies surrounding triple-digit price increases for some prescription drugs have put the need for assessing the risks surrounding the manufacturing and marketing of drugs and medical devices into sharp focus. These risks are especially acute for orphan drugs treating rare, but highly visible, diseases that affect very small populations.

350 million people globally — more than half of them children[1] — are afflicted with one of 7,000 known rare diseases. However, treatments exist for less than 5 percent of these diseases[2], leaving the vast majority of sufferers without options. These are known as orphan drugs, and these treatments have become a booming business - projected to have worldwide sales of over $100 billion in 2016. The growth in the orphan drug market was driven by the passage of the Orphan Drug Act of 1983, which helped get these life-saving drugs to market quicker and granted manufacturers a seven year period of sales exclusivity. Patient advocacy groups then forced health insurers to put these expensive medications on their formulary, forming close, cordial partnerships with orphan drug manufacturers in the process.

However, changes in the public's perception of drug manufacturers as profiteering and the rise of "villain" CEOs have driven a pervasive, active, and largely hostile negative public perception that poses an increasing threat to orphan drug manufacturers. Twice in the last two years, enormous public controversies arose over the radical price increases for two common, but necessary, generics. The subsequent blaze of social media outrage spilled out into mainstream editorials, on-line petitions, congressional and agency investigations, and lawsuits against the manufacturers.

Like other controversial drugs recently in the media, orphan drugs are also in high demand, and are extremely expensive with costs ranging from $75,000 to $350,000 per year. While their special status from the FDA has helped more of these drugs get to market, the shortened testing process and seven year exclusivity also raise questions about collusion and the potential dangers of the reduced testing requirements. Patient advocacy groups could come to see themselves as exploited and start to turn against manufacturers whose motives may now seem mainly financial, not altruistic.

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[1]Atlantis Healthcare Infographic, https://globalgenes.org/rare-diseases-facts-statistics/

[2]Atlantic Healthcare Infographic, http://www.fromhopetocures.org/fighting-rare-diseases

Pitfalls

Although orphan drugs serve much smaller populations than mainstream medications for common afflictions, orphan drug developers are exposed to the same potentially catastrophic products and professional liability claims that plague conventional pharmaceutical operations:

Drugs may not work as expected (limited testing may skew perceptions) and manufacturers can be viewed as providing false hope to a vulnerable population. Side effects may be perceived as incommensurate with benefit. Orphan drug companies are also increasingly likely to be painted as preying on the seriously ill for profit by setting high prices for drugs that have no competition.

Communicating clearly and managing expectations

Four risk management fundamentals are key to mitigating risk and preparing orphan drug companies to mount a strong defense of a claim.

1. Transparent, straightforward communication. Companies should be meticulous in setting expectations of what a drug can and cannot do. Is it potentially curative or does it simply provide better quality of life? Is the expected improvement 10%, or much larger? All known potential side effects should be clearly articulated.

2. Active monitoring of use and side effects. Companies involved with orphan drugs need to vigilantly gather and analyze information on how patients are faring, monitoring for efficacy and side effects. Current data should be actively and publicly shared, increasing understanding and trust in patients, doctors, and the public.

3. Communication and alignment with patient advocacy groups. These grass roots, often family-driven, organizations are important links in the chain of communication and expectation-setting for orphan drugs.

4. Broad, tailored insurance. Insurance should target the specific product and professional liabilities of orphan drug companies; in particular, the potential for bodily injury arising out of known and unknown side effects , as well as mental distress of patients. Also necessary are products recall and coverage for the expense to medically monitor patients who feel they may have been harmed.

Insurance and risk management critical to success:

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Orphan drug makers are credited with some of the greatest pharmaceutical innovations of the past two decades, giving hope, saving lives, and enhancing the quality of life for disease suffers who had previously been ignored and without resource. As companies continue working on alleviating the suffering caused by rare diseases, they must also keep a close watch on their pricing, risk management and insurance practices. This will allow them to continue their mission of helping patients in an increasingly volatile liability climate with heightened public scrutiny.

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