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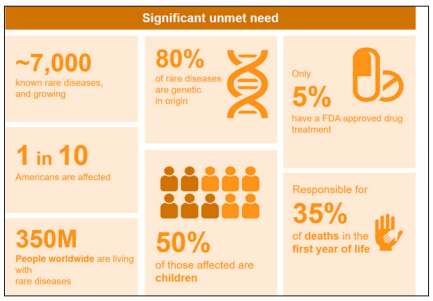
Insider insight on the orphan drug act



In the early 1980s, it became clear that while there are thousands of rare diseases without any therapies (about 8,000 at last count, when rare is defined as <200,000 persons prevalence in the US). Lawmakers had a fundamental choice in social policy: Do we make it easier for drugs for rare diseases to get approved based on less data (i.e. a lower evidentiary bar) and lower standards (decrease the costs), or do we make it more valuable for manufacturers to create these therapies through market exclusivity incentives? They chose the latter; Orphan Drug Act creates exclusivity that artificially increases the price of the drugs. Our United States-dominant biotechnology industry blossomed because the ODA directly incentivized tiny companies. Typically, these companies are constituted from a mom who won't just watch her kid die, a brilliant scientist, and a guy with a little bag of money to bring forth startlingly effective therapies. Orphan drugs are the most exciting opportunity in drug development. Today nearly 40% of all new product market approvals the FDA grants are for orphan products. Orphan drug development is different; it involves special regulatory affairs, special drugs, special financial metrics and special science. Unique experience is required to work with orphan drugs, experience not usually found amongst those who traditionally develop drugs for common diseases. This presentation helps learn how orphan drugs are managed with special deference by regulatory agencies such as the FDA and EMA, essentials of orphan drug designation, pediatric/tropical rare disease Priority Review Voucher (PRV), breakthrough therapies for orphans and special tenderness offered to orphan drugs by the FDA review divisions.

Biography

Kritika Khurana is a Business Development Manager at IQVIA's Orphan Drug Unit, overseeing the Asian and Eastern US markets. She has trained under the guidance of a former head of the FDA Office of Orphan Products Development and has been working in the orphan space for 3 years. She is a 2015 graduate of the MS Biotechnology program from Georgetown University and has experience in strategic business development and market research.

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