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Challenges and opportunities in development of orphan drugs

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Development of orphan drugs for rare diseases is fraught with opportunities and challenges globally, in legislative policies, research and development, clinical trials, time to reach the market and disparity in affordability and accessibility. The opportunities include incentivizing of researchers and manufacturers in fee reduction or no fees for protocol assistance, preauthorization inspections, marketing authorization, grant funding, priority review voucher for rare pediatric disease and granting of market exclusivity. These were stimulated by the US 1983 Orphan Drug Act and heightened awareness of the public health impact and ramifications in many countries. The number of orphan drug designations has increased in recent years and so is the number of marketing approvals. However, there challenges that could limit the development include understanding the disease and sometimes the co-morbidities, establishing the clinical relevance and cost effectiveness, difficulties in setting up clinical trials for the small populations and high cost of bringing a new product to market especially an orphan drug with limited target population and market opportunities. The purpose of the presentation is to underscore the opportunities, successes in orphan drug development and challenges using relevant case studies. Review of orphan drugs categories and designations for rare diseases in several countries, opportunities that include legislative and regulatory incentives, challenges in development and recent successes were done. Using USA as a reference country, examples of opportunities and challenges in the development of pediatric orphan drugs for rare diseases such as pediatric HIV/AIDS and sickle cell disease were given. Perspectives on academic-industry-government collaboration relating to the opportunities and challenges were also presented. The significance in the successes in orphan drug approvals, ongoing and increasing awareness of impact of development of orphan drugs on the life of the patients and the low market opportunities and difficulty in bringing the products to market were emphasized.

Biography

Moji C Adeyeye is the Founding Chair of Biopharmaceutical Sciences Department and Professor of Pharmaceutics and Drug Product Evaluation at the College of Pharmacy, Roosevelt University (RU) in Schaumburg, Illinois. Her research interest include pre-formulation, pediatric and adult drug product (solids, liquids and semisolids) development and evaluation investigational new drug application-driven bench-to-bedside translational research, preclinical and clinical trials, analytical/bioanalytical assay development, bioavailability and bioequivalence quantitation, fixed dose combination dosage forms for various drug classes including antiretrovirals, anti-malarials and anti-sickling agents. She is a Senior William J. Fulbright Scholar and Specialist, 2016 Nigeria National Academy of Science Fellow and 2008 American Association of Pharmaceutical Scientists (AAPS) Fellow. She has earned her BS and MS/PhD from the University of Nigeria, Nsukka, Nigeria and University of Georgia, Athens, GA, respectively. She has 5 patents, 57 peer-reviewed manuscripts, book chapters and books, more than 150 scientific presentations and has successfully mentored many MS and PhD candidates.

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