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Unique challenges and opportunities in conducting pharmacovigilance for orphan drugs

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Diseases that manifest in patient populations representing at the maximum 6–8% of the world population are defined as rare diseases or orphan diseases. These are pathologies whose incidence at birth is less than 1 in 2000. Orphan diseases are often so rare that a physician may observe only 1 case a year or less, and proper treatment may only be a personalized encounter between doctor and patient. One way to fill this therapy vacuum is by developing orphan drugs. Orphan drugs are a significant part of personalized medicine. In order to optimize the benefit-risk of these medicines, innovative pharmacovigilance methods need to be put in place that take into consideration the unique challenges of drug development for orphan indications as well as the realities of the patient population. The presentation will provide a summary of the existing methodologies for performing targeted pharmacovigilance for orphan drugs, as well as new innovative methods developed to address some of these challenges. The efficacy and safety of medicines may be different in children compared to adults. The available documentation at the time of approval is, in general more sparse in children and long term data collection may be needed in order to clarify the safety profile in children and particularly to detect any long-term or delayed toxicities in the developing child. Therefore, there is a need to carefully consider how pharmacovigilance is conducted for medicines used by children and whether there are any aspects of the pharmacovigilance system that need to be enhanced to ensure adequate protection of public health. Furthermore, many medicines used to treat children are not licensed for such use (off-label use) or are not licensed at all (unlicensed use). This may further limit reporting of suspected adverse reactions to the pharmacovigilance system.

Biography

Cristina Damatarca has over 17 years of experience in the biopharmaceutical industry, across all stages of development and product commercialization. In her current position as Vice President of Medical Affairs and Pharmacovigilance at Agility Clinical, she provides oversight for all medical affairs and drug safety activities for Agility Clinical, which focuses on rare diseases. Before joining Agility Clinical, she was Vice President and Head of Drug Safety and Pharmacovigilance at Clovis Oncology, where she provided oversight for all safety aspects of the clinical development program for Clovis Oncology molecules. Before that, she served as Executive Director and Head of Safety at Avanir Pharmaceuticals, where her responsibilities included oversight of clinical trials and postmarketing safety, pharmacovigilance, medical review, signal detection, aggregate reporting, and risk management. Previous functions included senior roles in Pharmacovigilance as Therapeutic Area Head for the Oncology Signaling Franchise at Genentech-Roche and before that, as Global Safety Officer and Global Safety Forum Chair at Amgen for various products in Oncology, Oncology–Hematology, and Inflammation-Internal Medicine. While at Amgen, she also served as the Chair of the Global Safety Forum, an internal advisory board for safety issues. In addition to her work experience in drug safety and pharmacovigilance, she spent several years of her career in Medical Education as Executive Director of Medical Education, Patient Outcomes, and Risks Management at the Neuroscience Education Institute. Other roles she held included positions within drug safety and medical affairs with a few other companies in the industry. She has published in peer-reviewed journals, including several publications in the area of drug safety, and has had speaking engagements at several conferences and meetings.

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