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Orphan drugs: Getting arms around rare diseases

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Finding ways to bring drugs for rare diseases to patients is an important public health challenge. The complexity of the rare diseases, incomplete understanding of disease pathophysiology, limited population and heterogeneity of the patients suffering from rare conditions, difficulties in diagnosis, 'rare physicians' availability, high-cost of the R&D of orphan drugs, regulatory risks in the road of clinical trials conduct, marketing authorization and reimbursement are all factors having an impact on this challenge. Undoubtedly, the most challenging part of this is the clinical trials conduct on the orphan drug molecules. The limited number of patients together with the diversity of the rare conditions makes the protocol design a sensitive topic to consider; access to the rare patients, getting interest and the engagement of the physicians are also amongst the challenges in this process. There are a number of stakeholders playing key roles in the Orphan drug R&D including regulatory authorities, policymakers, patient advocacy groups, scientists and clinical investigators, research institutes, academic or non-academic associations. In the speech, we will focus on the ways to overcome all difficulties, discussing how to find the most cost-effective and having the least or no regulatory risks during the clinical trials conduct and evaluate the value of local and global expertise and the cooperation of the stakeholders.

Biography

Irmak Duygu Kuyuncu has joined the clinical research industry in 2003 and worked as CRA. She has worked as a Clinical Operations Manager for more than 5 years in CROs and Pharma having responsibilities in clinical resources management, ensuring successful and compliant clinical operations, management of the process improvement in Turkey and in Middle East Countries. She was an Advisory Board Member and Consultant of the Scientific and Technological Research Council of Turkey in 2013. She has been a Board Member of the Clinical Research Association in Turkey and one of the Board Members in the Neuromuscular Diseases Research Association in Turkey. She has been involved in publication projects with close co-authoring with key opinion leaders from regulatory, academy and industry. She is currently leading the INC Research Turkey as Clinical Operations Associate Director.

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