1. Record Nr. UNINA9910508447303321

Titolo	Rare Disease Drug Development : Clinical, Scientific, Patient, and Caregiver Perspectives / / edited by Raymond A. Huml
Pubbl/distr/stampa	Cham : , : Springer International Publishing : , : Imprint : Springer, , 2021
ISBN	3-030-78605-6
Edizione	[1st ed. 2021.]
Descrizione fisica	1 online resource (417 pages)
Disciplina	615.19
Soggetti	Medicine Clinical medicine - Research Pharmacology Immunology Therapeutics Pharmacovigilance Clinical Medicine Clinical Research Drug Safety and Pharmacovigilance Malalties rares Desenvolupament de medicaments Quimioteràpia Llibres electrònics
Lingua di pubblicazione	Inglese
Formato	Materiale a stampa
Livello bibliografico	Monografia
Nota di contenuto	Introduction to Rare Diseases and Market Overview -- The Patient Perspective -- Select Patient Narratives -- The Caregiver Perspective -- The Critical, Multidimensional Role of Patient Advocacy Groups in Rare Disease -- A Mental Health Perspective -- Investment Decisions Related to Rare Disease Drug Development -- Optimizing Rare Disease Registries and Natural History Studies -- Novel Approaches to Clinical Trials in Rare Diseases -- Patient Benefits from Innovative Designs in Rare Diseases -- Central Nervous System Rare Disease Drug Development -- Oncologic Rare Disease Drug Development --

Hematologic Rare Disease Drug Development -- Lessons From Rare Disease and Gene Therapy Clinical Studies in Ophthalmology -- Rare Diseases in the Pediatric Population -- Cell and Gene Therapy in Rare Diseases -- The Feasibility Assessment -- The Evolving Regulatory Space and the Advent of Patient-Focused Drug Development -- Operational Aspects of Rare Disease Drug Development -- Accelerating Rare Disease Drug Development -- Select Rare Disease Drug Approvals: Lessons Learned -- A Rapid Market Access Strategy for Orphan Medicinal Products (OMPs) with Highlights Regarding the Pricing and Reimbursement Process and Barriers to Patient Use -- Integrated Life Cycle Management for Rare and Orphan Products -- The Case for Real-World Data and Real-World Evidence Generation in Rare and Orphan Medicinal Drug Development -- Closing Remarks -- .

Sommario/riassunto

This book provides a broad overview of rare disease drug development. It offers unique insights from various perspectives, including third-party capital providers, caregivers, patient advocacy groups, drug development professionals, marketing and commercial experts, and patients. A unique reference, the book begins with narratives on the many challenges faced by rare disease patient and their caregivers. Subsequent chapters underscore the critical, multidimensional role of patient advocacy groups and the novel approaches to related clinical trials, investment decisions, and the optimization of rare disease registries. The book addresses various rare disease drug development processes by disciplines such as oncology, hematology, pediatrics, and gene therapy. Chapters then address the operational aspects of drug development, including approval processes, development accelerations, and market access strategies. The book concludes with reflections on the authors' case for real-world data and evidence generation in orphan medicinal drug development. Rare Disease Drug Development is an expertly written text optimized for biopharmaceutical R&D experts, commercial experts, third-party capital providers, patient advocacy groups, patients, and caregivers.