

[Library ebook] Orphan: The Quest to Save Children with Rare Genetic Disorders

Orphan: The Quest to Save Children with Rare Genetic Disorders

Philip R. Reilly

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Orphan

THE QUEST TO SAVE CHILDREN WITH RARE GENETIC DISORDERS



PHILIP R. REILLY, MD, JD

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#328266 in Books 2015-11-01Original language:EnglishPDF # 1 6.20 x 1.20 x 9.30l, .0 #File Name:
1621821374408 pages | File size: 32.Mb

Philip R. Reilly : Orphan: The Quest to Save Children with Rare Genetic Disorders before purchasing it in order to gage whether or not it would be worth my time, and all praised Orphan: The Quest to Save Children with Rare Genetic Disorders:

1 of 1 people found the following review helpful. Comprehensive discussion of Genetics, past, present futureBy Hammer75Although I'm not a biologist, I found this book immensely interesting from the historical background to so many disorders, the state-of-the art treatments of today and projections for the future treatment of these rare genetic

disorder. As the author notes towards the end of the book, if we look into our own ancestry, we are bound to find any number of 'familial traits' which likely have a genetic cause. So although most of the book delves into Orphan diseases, the very techniques used to treat/cure Orphan diseases are crucial in treating cancers, heart disease, etc.. The author's genetic background emerges as he time and again notes his presence at pivotal points in genetic discovery and his personal meetings and affiliations with so many of the key researchers in genetics. His writing style was quite engaging, occasionally making note of his own genetics and poignant anecdotal quips. Finally, the author's grasp of the legal and ethical nuances of such discoveries left me with an appreciation of all that medical researchers do to improve the quality of our lives, despite the legal and ethical hurdles that must be overcome in the process. 1 of 1 people found the following review helpful. The definitive story of rare diseases By Customer Simply superb. Reilly weaves together a series of intimate vignettes into a moving and informative tapestry that depicts the most vulnerable and the most venerable of humanity. A must read for anyone interested in rare diseases, genetics, or medicine. 1 of 2 people found the following review helpful. Five Stars By Customer Great!

Orphan is about the struggle to save the lives of children who, because of an unlucky roll of the genetic dice, are born with any one of several thousand rare genetic disorders. Many are burdened with diseases that carry mysterious names, some of which you can read about for the first time in this book, along with compelling stories about the physicians, scientists, and parents who have taken them on. The diseases include phenylketonuria, sickle cell anemia, dystrophic epidermolysis bullosa, X-linked hypohidrotic ectodermal dysplasia, and Friedreich's ataxia—just a few of the more than 1000 genetic disorders that are well-described and many more that are not. Many manifest in infancy. Some show up in mid-childhood, others later in childhood, and still others among adults. They touch almost every extended family. Orphan is more than a book about disease and research—it gives voice to thousands of people who, all too often, have endured terrible illnesses, bravely faced arduous clinical trials, and, sometimes, have gained victories, almost always in silence. This book recounts extraordinary breakthroughs and hopes for the future. Many of the disorders that will end our lives are in some part genetically influenced. We really are all orphans, and this book is for all of us.

"A physician, attorney, and biotech entrepreneur, Philip Reilly clearly illuminates rare genetic disorders, the struggle to understand and treat them, and the promise of the latest advances in genetic testing and therapy. Engaging and compassionate, Orphan brings the exciting advances in biomedical research to life. This excellent volume is enriched by the often heroic stories of parents and physician-scientists determined to find answers." - David J. Skorton, MD, Secretary, The Smithsonian Institution "A fascinating, engaging, and at times inspiring look at the past, present, and future of genetic medicine told through the stories of scientists, doctors, patients, and parents who were heroes in the fight against genetic disease. Thoroughly enjoyable." - Hank Greely, Stanford Law School, an authority on public policy issues in genetics "Reilly's Orphan provides a very interesting and readable history of the various approaches used in efforts to first understand and then treat children who suffer from rare genetic diseases. Research efforts to identify rare genetic changes that cause disease used to require decades and large families, but with the rapid advances in genome sequencing over the past 15 years, this can now be done in weeks. Orphan discusses how these new discoveries might be coupled with gene editing or cell therapy to offer new hope to families." - J. Craig Venter, PhD, Chairman and CEO, J. Craig Venter Institute, and a pioneer in human genomics "Written with clarity and from first hand experience by a pioneer who is helping to develop new breakthrough therapies for children with rare genetic diseases, this book takes you by the hand and pulls you into the world of these children and their heroic parents, many of whom helped to champion new treatments working with equally heroic scientists and physicians. The story is truly compelling." - Donald Ingber, MD, PhD, Founding Director of the Wyss Institute for Biologically Inspired Engineering at Harvard University About the Author Philip R. Reilly earned his undergraduate degree at Cornell University, studied human genetics at the University of Texas Graduate School of Biomedical Sciences, and graduated from Yale Medical School in 1981. He did his medical residency at Boston City Hospital. He earned board certification in internal medicine and clinical genetics, and a law degree at Columbia University. He has served on the Board of Directors of the American Society of Human Genetics, and he is a Founding Fellow of the American College of Medical Genetics. He twice served as President of the American Society of Law, Medicine, and Ethics. During the 1990s, Reilly was the Executive Director of the Eunice Kennedy Shriver Center for Mental Retardation in Waltham, Massachusetts, a nonprofit that worked on understanding childhood and adult neurological disorders. Dr. Reilly has held faculty positions at Harvard Medical School and Brandeis University. Since 2009 he has worked as a venture partner at Third Rock Ventures in Boston where he focuses on helping to start companies to develop innovative therapies for orphan genetic diseases. Over the years he has published six books and many articles about the impact of advances in genetics. Reilly frequently works with patient groups who are concerned with rare genetic disorders.