Personalised precision therapy is increasingly being seen as the future of medical care. This requires that drugs or diagnostics tailored to the specific needs of individuals be selectively targeted to the site of disease. To achieve this end, nucleic acid aptamer technology is one of the most promising approaches capable of delivering targeted diagnosis and therapy. Aptamers, often termed “chemical antibodies,” are short, synthetic single-stranded DNA or RNA oligonucleotides that can bind to a molecular target with high specificity and affinity because of their ability to adopt 3D shapes. The application of aptamers continues to grow across various fields and demonstrates its potential for targeted molecular recognition. This book provides an overview of aptamer technology through 13 well-crafted chapters written by international experts in the field. The chapters provide excellent insight into the aptamer history, development, and applications in targeted cancer therapy, neurological diseases, infectious diseases, drug delivery, medical diagnosis, molecular imaging, clinical development, and the scope of aptamer technology.

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Preface

Target-specific drug delivery and therapy still remains one of the holy grails of the drug development community. Of the many approaches used to date, antibody-based techniques are the most widely used, and this technology has matured over several decades. In the early 1990s a new class of targeting compounds emerged with very high target specificity and affinity, as an alternative to antibodies for targeted drug delivery and therapy. These compounds, aptamers (often referred to as “chemical antibodies”), are short, single-stranded functional nucleic acids that can fold into complex 3D shapes in solution for high-affinity recognition of defined molecular targets ranging from small molecules to large proteins and even whole cells. Typically, aptamers are developed through an in vitro reiterative combinatorial selection process called systematic evolution of ligands by exponential (SELEX) enrichment starting with a large pool of oligonucleotide sequences.

Since their discovery, nucleic acid aptamers have attracted considerable attention across various fields of medicine as a platform technology for targeted therapeutic development for a broad range of disease conditions as well as for molecular imaging and diagnosis. Aptamers possess certain qualities that potentially give them an edge over antibody-based approaches. In particular, they do not require live animals for production and can be synthesised on a large scale. Moreover, they possess high stability with long shelf lives, they can be chemically modified, and their functions can be neutralized using an antidote sequence. In 2004, the first aptamer drug targeting the vascular endothelial growth factor protein, Mucagen (Pegaptanib), was approved for clinical use by the US Food and Drug Administration (FDA) for the treatment of age-related macular degeneration (AMD). These developments
have been instrumental in reinforcing the potential of aptamers as a clinical tool and have led to a plethora of publications and patents exploring the use of aptamer technology in various fields. The primary purpose of this book is to review the history, development, and applications of aptamers and discuss their potential as a transformational technology for target-specific therapeutics and diagnostics.

This book covers all aspects of aptamers across 13 chapters. Chapters 1–4 provide a general introduction, while Chapters 5–7 describe the development of therapeutic aptamer candidates for cancer, neurological diseases, and infectious and parasitic diseases. Chapters 8 and 9 provide an overview of aptamers as tools for targeted drug delivery and nanotherapy. The application of aptamers as biosensors for medical diagnosis is described in Chapter 10, and Chapter 11 highlights the use of aptamers as a platform technology for target-specific molecular imaging. Finally, Chapters 12 and 13 are devoted to an in-depth analysis of the clinical development of aptamers and their scope, limitations, and future prospects. As the editor of this book, with many years of experience in the aptamer field, I remain convinced that aptamers have tremendous potential as a tool for delivering targeted nanotherapies and in molecular imaging. I feel immensely proud to have had the opportunity to edit this book and wish to thank the book’s many distinguished contributors. I hope that the book will encourage scientists and clinicians from a variety of fields to consider aptamer-based technologies as a tool in their own research.

Rakesh N. Veedu