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Novel Therapeutics from Modern Biotechnology

From Laboratory to Human Testing

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Preface

A cover story of *Business Week Magazine* in January 1984 stated "Biotech Comes of Age". In February 1986, *Venture Magazine* had a cover article entitled "The Biotech Revolution is Here". This article went on to say "New Genetic Technologies Will Transform Our Lives". These announcements were made many years after the first biotechnology companies, such as Genentech, Cetus, Amgen and Biogen, were formed to commercialize the "New Biology".

At the time of writing this book, there are over 1300 biotech companies developing new technologies or identifying potential biotech drugs. Most of these companies were started in the height of the "high-technology hype", although companies are still forming as the technology advances.

A more recent survey showed only a relatively small number of Food and Drug Administration (FDA) approvals among over several hundred biotechnology products now in clinical trial. One could ask why it has taken so long to produce biotechnology products. Part of the reason is that each new class of biotech products brings with it a set of problems that need to be solved before they enter clinical trials. These problems are often unique to biotechnology products, such as peptides, proteins, monoclonal antibodies, nucleic acids and cellular therapies.

Although the "biotech" therapeutics represent unique scientific issues, there are now numerous examples that show that the principles of drug development apply to the newer systems as well as to development of novel small synthetic compounds. For example, there needs to be a well-controlled process for making the therapeutics, appropriate analytical methodologies to characterize the product, appropriate formulation to store and deliver the therapy to the patient, and a thorough characterization of the safety issues surrounding the new entity.

Our goal in this volume is to address the particular problems associated with several classes of biotechnology products and, at the same time, demonstrate that the principles are the same as in development of small new chemical entities. We have invited several scientists at biotechnology or pharmaceutical companies to share with us some of the unique problems that they encountered in getting biotech products into clinical trials and how they solved them.

The first chapter addresses FDA regulatory expectations for biotech products. The next several chapters discuss general issues common to each class of

biotech drug, such as proteins, peptides and nucleic acids. The balance of the chapters deals with specific biotech drugs that have successfully made it into clinical trials.

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