text. For example, microfilaments are incorrectly introduced as being “~35 nm in diameter,” but the same chapter subsection also provides the correct relative diameters of microtubules and intermediate filaments. Perhaps subsequent editions of the textbook will address these issues.

The strength of the textbook lies in its ability to promote active learning by applying acquired physiological knowledge to clinical cases. The initial case and open-ended questions of each section, as well as case-based “Applying What You Know” questions at the end of each chapter, are engaging even for the non-medical student and provide a broad conceptual framework that helps the reader fit pieces of information together. Importantly, answers to all questions and a concise case analysis from both clinical and physiological perspectives are given at the end of each section. Furthermore, an access code is provided for a supplementary interactive online study aid, WinkingSkull.com PLUS, which includes two additional clinical cases on the cardiovascular and respiratory systems and 814 images from the Atlas of Anatomy with a timed test feature. The online program also gives readers the option of displaying anatomy labels in English or Latin. Taken together, these qualities make Fundamentals of Medical Physiology a valuable resource.

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Small Molecule Therapy for Genetic Diseases. Edited by Jess G. Thoene. New York: Cambridge University Press; 2010. 223 pp. US $110 Hardcover. ISBN 978-0521517812.

Small Molecular Therapy for Genetic Diseases is a collection of articles that commemorate the Orphan Drug Act that was passed in 1983 after sustained effort from patient advocacy groups. The legislation gave pharmaceutical companies various incentives, such as tax credits, expedited review, flexible clinical trial requirements, and exclusive marketing to develop treatments that would otherwise be unprofitable. A recent search in the on-line Orphan Drug database (http://www.accessdata.fda.gov/scripts/opdlisting/oopd/index.cfm) reveals that 370 drugs have been approved for 180 diseases, with approximately 2,000 more drugs in the pipeline. This turnout makes former Office of Orphaned Drugs Director Marlene Haffner proudly conclude at the end of the first chapter that the “taxpayer’s dollars have been well spent.” The majority of the approved products are biological agents such as enzymes, antibodies, or hormones. Another significant portion of drugs are cytotoxic agents for malignancies or autoimmune diseases. Only 24 agents are small molecule drugs for genetic diseases, but it is this category of drugs that provides the focus for the rest of the book. Although macromolecular therapy may dominate the post-genomic world, the authors remind us that small molecules have many distinct advantages, such as easy delivery, straightforward pharmacokinetics, and minimal immune interactions.

After an introductory section covering legislative backgrounds, infrastructures, and pharmacology principles, the book reviews 11 examples of effective small molecule therapy. They are grouped by the molecules’ modality of action as co-factors of enzymatic activities, activators of alternative pathways to circumvent metabolic defects, and metal conjugates. In each chapter, an overview of the pathogenesis and epidemiology of the condition is followed by clinical trial and case study data of the drug.

The book targets three types of readers: clinicians and clinical scientists, patients and patient advocates, and policy makers. The sense of hope and encouragement that permeates the pages enables the book to motivate physicians and researchers to use and develop small molecule therapies. But someone familiar with biomedical sciences will likely find the articles rather rudimentary. Although the writing is void of jargon and explains the clinical data in more palatable terms than a Cochran Review, it would be helpful to know what scientific inquiries
led to these therapies and to see more molecular details of both the pathological and therapeutic effects. Only one chapter showed actual structures of the drugs; none had diagrams illustrating the pathways involved. In addition, almost all of the diseases mentioned are inborn errors of metabolism. The geneticist is left to wonder whether small molecules can be used in other conditions such as prevalent neuro-developmental disorders. For patients, this book provides valuable resources for self-education about pharmacology and gateways to search for clinical trials and investigational drugs. Whether the examples given are useful would likely vary among patients.

Ultimately, *Small Molecular Therapy for Genetic Diseases* may prove to be the most beneficial for policy makers. The book’s format and tone resemble government progress reports, and it offers a concise and convincing sampling of the fruits of the Orphan Drug Act while highlighting the accomplishments of their legislative endeavors.

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