

Advances in Experimental Medicine and Biology 895
American Society of Gene & Cell Therapy

Toni Cathomen
Matthew Hirsch
Matthew Porteus *Editors*

Genome Editing

The Next Step in Gene Therapy



Springer

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This book is dedicated to the memory of Carlos F. Barbas III. Among his incredibly long list of accomplishments, he was best known in the field of genome engineering for his invaluable contributions in designing zinc-finger arrays that have been used in artificial transcription factors, zinc-finger nucleases, and custom recombinases. “It has been a dream of mine to develop drugs that make a difference” (Carlos Barbas, 2008)

Preface

In the early 1900s, human genetic engineering remained the fanciful speculation of science fiction writers, scientists, and philosophers. The surfaced ideas perceived consequences that, already more than 100 years ago, invoked ethical dilemmas: Would cavalier applications of genetic engineering interrupt “natural” evolution? Would engineered creatures be capable of destroying us? Would an altered DNA blueprint make us susceptible to presently harmless “pathogens”? The concerns appeared limitless. In our day, most of our genome editing efforts are driven by the allure of potentially curing genetic diseases. In fact, some newly developed genome editing technologies are so effective and easy to use that the genes of nonhuman primate embryos could be altered. In view of that, it is conceivable that the same technique, which is used to cure genetic disorders, could also be employed to introduce genetic changes in the human germline in order to enhance human qualities, like intelligence or good looks. Where do we draw the line? To give us more time for public discussion and to better understand how safe the current genome engineering tools are, scientist and ethicist have called for a moratorium on human germline editing.

Human genetic engineering is at the forefront of disease therapy research based on seminal observations that have collectively increased the frequency of the ability of a cell to “process” its DNA. Depending on the cellular decision, both chromosomal disruption and the precise tailoring of a native locus with endogenous or exogenous DNA remain possible. To complement our ability to induce DNA alterations, great strides have been made to deliver nucleic acids efficiently throughout the human body. As we are on the crest of this genetic tsunami, it appears timely to coalesce the current understanding of the early twentieth century.

We feel happy that some of the world’s most prominent geneticists, biologists, and bioinformaticians, each having an expertise we felt will continue to shape our understanding and our ability of human genome engineering, have contributed to this book. In attempts to make this volume relevant to a broad target audience, the authors of the individual chapters and the editors have made an effort to provide

sufficient background for the respective genre. The final product represents the most comprehensive work on the many facets of human genetic engineering and our stepwise progression toward a dream of a disease-free existence. Please enjoy.

Freiburg, Germany
Chapel Hill, NC
Palo Alto, CA

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