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978-0-521-69084-3 - Gene Transfer and the Ethics of First-in-Human Research: Lost in Translation

Jonathan Kimmelman

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McGill University / Biomedical Ethics Unit and Social Studies of Medicine



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Cambridge, New York, Melbourne, Madrid, Cape Town, Singapore, São Paulo, Delhi, Dubai, Tokyo

Cambridge University Press

The Edinburgh Building, Cambridge CB2 8RU, UK

Published in the United States of America by

Cambridge University Press, New York

www.cambridge.org

Information on this title: www.cambridge.org/9780521690843

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First published 2010

Printed in the United Kingdom at the University Press, Cambridge

A catalogue record for this publication is available from the British Library

Library of Congress Cataloguing in Publication data

Kimmelman, Jonathan.

Gene transfer and the ethics of first-in-human research : lost in translation / Jonathan Kimmelman.

p. cm.

Includes bibliographical references and index.

ISBN 978-0-521-69084-3 (pbk.) 1. Gene therapy--Moral and ethical aspects.

2. Clinical trials--Moral and ethical aspects. I. Title.

[DNLM: 1. Gene Transfer Techniques--ethics. 2. Human Experimentation--ethics.

3. Public Policy. WB 60 K49g 2010]

RB155.8.K56 2010

615.8'95--dc22 2009029830

ISBN 978-0-521-69084-3 Paperback

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For Chichester and Gooch

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We grow accustomed to the Dark --
When light is put away --
As when the Neighbor holds the Lamp
To witness her Goodbye --

A Moment – We uncertain step
For newness of the night --
Then – fit our Vision to the Dark --
And meet the Road – erect --

And so of larger – Darkness --
Those Evenings of the Brain --
When not a Moon disclose a sign --
Or Star – come out – within --

The Bravest – grope a little --
And sometimes hit a Tree
Directly in the Forehead --
But as they learn to see --

Either the Darkness alters --
Or something in the sight
Adjusts itself to Midnight --
And Life steps almost straight.

Emily Dickinson

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Acknowledgments

This book began inauspiciously enough when, newly arrived in Montreal with a very bad haircut and a Ph.D. in molecular genetics, I approached Kathy Glass about post-doctoral studies in bioethics. I am grateful to Kathy for taking a risk by hiring me (first as a post-doc, and later as an assistant professor) and supporting this research.

I also thank Nancy King for her inspiring work in research ethics, as well as her support and critical feedback at key junctures. My colleague Nick King (no relation) provided a fine-grained vetting of this book. Alex London was a ready and more than able intellectual sparring partner throughout the writing process. Rebecca Dresser and Eric Juengst were gratuitously generous for agreeing to read my manuscript.

Numerous other oversubscribed colleagues, friends, and strangers made important contributions. These include: James Anderson, Françoise Baylis, Alberto Cambrosio, Marina Emborg, Dean Fergusson, Abe Fuks, Margaret Lock, Josephine Nalbantoglu, Tim Ramsey, Jason Scott Roberts, Thomas Schlich, Stan Shapiro, Charles Weijer, George Weisz, and Allan Young. I thank the many gene-transfer researchers, clinical investigators, and laboratory scientists (too numerous to name – and many probably preferring anonymity anyway) who graciously and patiently entertained my questions over the years. I also thank my editors at Cambridge, Katie James, Nick Dunton, Matt Davies and John Forder.

I received feedback from many anonymous commentators through peer review of various articles relating to this book. Some of these individuals should be shot (you know who you are). But many others volunteered elaborate and thoughtful criticisms that went well beyond the call of duty. Their anonymous service is an inspiration.

Through its main health granting agency, Canada “been very very good to me.” I thank the Canadian Institutes of Health Research for supporting this work with various grants and awards, including the Maud Menten New Investigator Prize, a CIHR New Investigator Award (Ethics in Translational Research: Navigating the Interface between the Bench and Clinic, MSH 87725), and several operating grants (States of Mind: Emerging Issues in Neuroethics, NNF 80045; Therapeutic Hopes and Ethical Concerns: Clinical Research in the Neurosciences, MOP 77670; Investigators as Inventors: An Empirical Study of Patent Holding and Ethics in Human Gene Transfer Research, MOP 79288; Advancing the Ethics of Clinical Trials: Enhancing Participant Protection and Scientific Rigour and a New Investigator Award, MOP 68835). This funding has allowed me to employ two resourceful and reliable research assistants over the years, David McLaughlin and Kat Duckworth.

This book emphasizes the role of social and technical networks for managing uncertainty and risk. What is true for novel medical interventions also applies for “risky” book projects like this. My cup runneth over with social infrastructure. First thanks go to my parents, Harold and Joyce Kimmelman, for enthusiasm and support. Harold also helped with the graphics and cover design. My wife, Sara Laimon, has been tireless in helping to maintain and restore order and morale throughout the book writing. She further offered very useful intellectual and stylistic feedback. I also thank several close friends for their care and feedback on various issues. They include: Cristiano Martello, Sean Hecht, Todd Krichmar, Naomi Schrag, and Laura Wolf-Powers. Finally, my daughters Rachel and Tamara had small hands in this project; I thank them for sustaining me with their humor, humanity, and reprieve.

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Every effort has been made to ensure the accuracy of this book’s content. None of the above acknowledgments should be interpreted as suggesting endorsement, sanction, confirmation, collusion, blandishment, complicity, or financial interest in this work or its author. Despite the use of high standards and care in writing this book, certain errors of analysis might have occurred. Money for harm or wages lost as a result of faulty analysis is not available. By reading this statement, you understand that I take full responsibility for any faults, foreseeable or unforeseeable, in the analysis.

Abbreviations

AAV	adeno-associated virus
AAMC	Association of American Medical Colleges
ADA	adenosine deaminase
AHCJ	Association of Health Care Journalists
ASGT	American Society of Gene Therapy
CIOMS	Council for International Organizations of Medical Sciences
CNS	Central Nervous System
CRADA	cooperative research and development agreement
CTEP	cancer therapy evaluation program
CTSC	Clinical and Translational Science Center
DARPA	Defense Advanced Research Project Agency
DNA	deoxyribonucleic acid
DSMB	Data Safety Monitoring Board
ESGT	European Society of Gene Therapy
FDA	Food and Drug Administration
FH	familial hypercholesterolemia
GAD	glutamic acid decarboxylase
GIPAP	Glivec International Patient Assistance Program
hESC	human embryonic stem cell
HGTS	Human Gene Therapy Subcommittee
HHS	Health and Human Services
HIC	high-income country
HIV	human immunodeficiency virus
IBC	Institutional Biosafety Committee
IHGT	Institute for Human Gene Therapy
IND	investigational new drug
IRB	institutional review board
LCA	Leber’s congenital amaurosis
LMIC	low-and middle-income country
MPS-VII	mucopolysaccharidosis type VII
MPTP	1-methyl-4-phenyl-1,2,3,6-tetrahydropyridine
NCI	National Cancer Institute
NGVB	National Gene Vector Biorepository
NGVL	National Gene Vector Laboratories
NHF	National Hemophilia Foundation
NHLBI	National Heart Lung and Blood Institute
NIH	National Institutes of Health
NUCDF	National Urea Cycle Disorders Foundation
OBA	Office of Biotechnology Activities
OTCD	ornithine transcarbamylase deficiency

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Abbreviations

PI	principal investigator
RAC	Recombinant DNA Advisory Committee
RNA	ribonucleic acid
RNAi	inhibitory ribonucleic acid
RT-PCR	reverse transcriptase polymerase chain reaction
SCID	severe combined immunodeficiency
siRNA	small interfering RNA
STAIR	Stroke Therapy Academic Industry Roundtable