20 years of ASGCT and gene therapy.

1995 1996 1997 1998 1999 2000 2001 2002 2003 2004 2005 2006 2007 2008 2009 2010 2011 2012 2013 2014 2015 2016 2017

1995

Report and Recommendations of the Panel to Assess the NIH Investment in Research on Gene Therapy.

Stuart H. Orkin, MD and Arno Motulsky, MD
Co-Chairs
December 7, 1995

- No evidence of clinical benefit to date despite 100 protocols
- Significant deficiencies in the basic science underpinning
- Only a few clinical trials designed to yield useful basic information
- Overselling of studies is rampant, which hinders confidence and progress

20 years of ASGCT and gene therapy.

1995 | 1996 | 1997 | 1998 | 1999 | 2000 | 2001 | 2002 | 2003 | 2004 | 2005 | 2006 | 2007 | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017

1996

American Society of Gene Therapy established

AMERICAN SOCIETY of GENE THERAPY

20 years of ASGCT and gene therapy.

1995 1996 1997 1998 1999 2000 2001 2002 2003 2004 2005 2006 2007 2008 2009 2010 2011 2012 2013 2014 2015 2016 2017

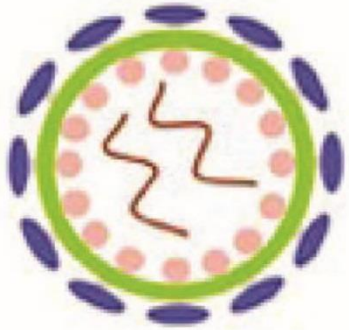
1996

Development of Lentivirus vectors

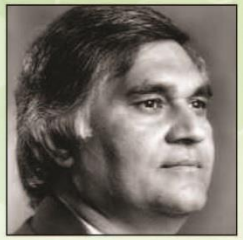
In Vivo Gene Delivery and Stable Transduction of Nondividing Cells by a Lentiviral Vector

Luigi Naldini, Ulrike Blömer, Philippe Gallay, Daniel Ory, Richard Mulligan, Fred H. Gage, Inder M. Verma,* Didier Trono

A retroviral vector system based on the human immunodeficiency virus (HIV) was developed that, in contrast to a murine leukemia virus–based counterpart, transduced heterologous sequences into HeLa cells and rat fibroblasts blocked in the cell cycle, as well as into human primary macrophages. Additionally, the HIV vector could mediate stable in vivo gene transfer into terminally differentiated neurons. The ability of HIV-based viral vectors to deliver genes in vivo into nondividing cells could increase the applicability of retroviral vectors in human gene therapy.



Science 272: 263. 1996.



Inder Verma, PhD
Salk Institute, La Jolla, California

Luigi Naldini, MD, PhD
Salk Institute, La Jolla, California

Didier Trono, PhD
Salk Institute, La Jolla, California

20 years of ASGCT and gene therapy.

1995 1996 1997 1998 1999 2000 2001 2002 2003 2004 2005 2006 2007 2008 2009 2010 2011 2012 2013 2014 2015 2016 2017

1996–99

Multiple crucial steps in preclinical development of AAV2 as a gene therapy vector, including these landmark papers in mice and in a canine disease model

Efficient Long-Term Gene Transfer into Muscle Tissue of Immunocompetent Mice by Adeno-Associated Virus Vector

XIAO XIAO,[1,2] JUAN LI,[1,2] AND RICHARD JUDE SAMULSKI[1,3]*

Gene Therapy Center[1] and Department of Pharmacology,[3] University of North Carolina at Chapel Hill, Chapel Hill, North Carolina 27599, and Somatix Therapy Corporation, Alameda, California 94501[2]

J Virol 70: 8098, 1996

Long-term correction of canine hemophilia B by gene transfer of blood coagulation factor IX mediated by adeno-associated viral vector

ROLAND W. HERZOG[1], EDMUND Y. YANG[2], LINDA B. COUTO[3], J. NATHAN HAGSTROM[1], DAN ELWELL[4], PAUL A. FIELDS[1], MELISSA BURTON[3], DWIGHT A. BELLINGER[4], MARJORIE S. READ[4], KENNETH M. BRINKHOUS[4], GREGORY M. PODSAKOFF[3], TIMOTHY C. NICHOLS[4], GARY J. KURTZMAN[3] & KATHERINE A. HIGH[1,5]

Nat Med 5: 56, 1999

Jude Samulski, PhD
University of North Carolina, Chapel Hill, North Carolina

Roland Herzog, PhD
University of Florida, Gainesville, Florida

20 years of ASGCT and gene therapy.

1995 | 1996 | 1997 | 1998 | 1999 | 2000 | 2001 | 2002 | 2003 | 2004 | 2005 | 2006 | 2007 | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017

1997

Effective suicide gene therapy transfer to T cells allowing mitigation of graft-versus-host disease following donor lymphocyte infusion in allogeneic transplantation recipients

Chiara Bonini, PhD
Ospedale San Raffaele, Milan, Italy

Claudio Bordignon, MD
Ospedale San Raffaele, Milan, Italy

HSV-TK Gene Transfer into Donor Lymphocytes for Control of Allogeneic Graft-Versus-Leukemia

Chiara Bonini, Giuliana Ferrari, Simona Verzeletti, Paolo Servida, Elisabetta Zappone, Luciano Ruggieri, Maurilio Ponzoni, Silvano Rossini, Fulvio Mavilio, Catia Traversari, Claudio Bordignon*

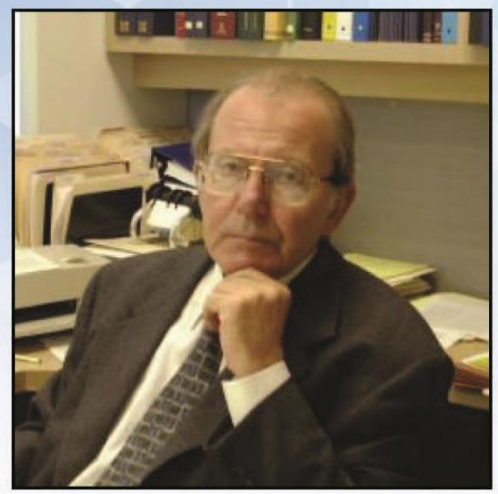
Science 272: 263. 1996.

20 years of ASGCT and gene therapy.

1995 | 1996 | 1997 | 1998 | 1999 | 2000 | 2001 | 2002 | 2003 | 2004 | 2005 | 2006 | 2007 | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017

1998

First ASGT annual meeting, Seattle



George Stamatoyannopoulos, MD, Dr.Sci
Founding President, ASGCT

20 years of ASGCT and gene therapy.

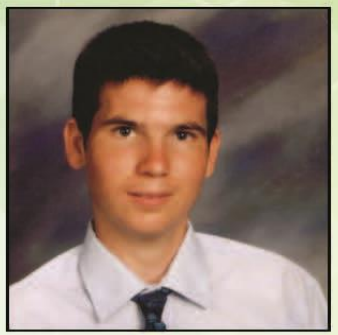
1995 | 1996 | 1997 | 1998 | 1999 | 2000 | 2001 | 2002 | 2003 | 2004 | 2005 | 2006 | 2007 | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017

1999

Patient Dies During a Trial Of Therapy Using Genes

New York Times, Sept. 29, 1999

Death of 18 year-old patient Jesse Gelsinger following administration of an adenoviral vector carrying the ornithine decarboxylase gene in a gene therapy protocol.



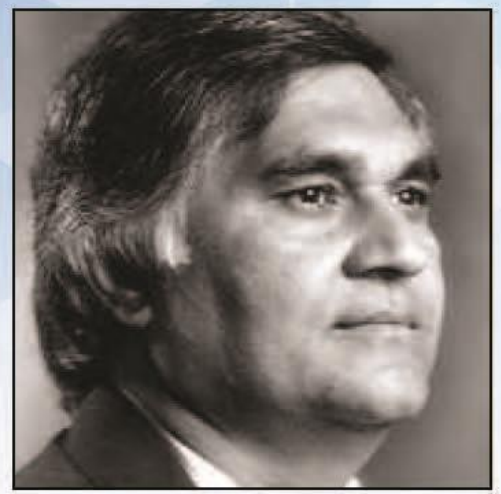
Jesse Gelsinger

20 years of ASGCT and gene therapy.

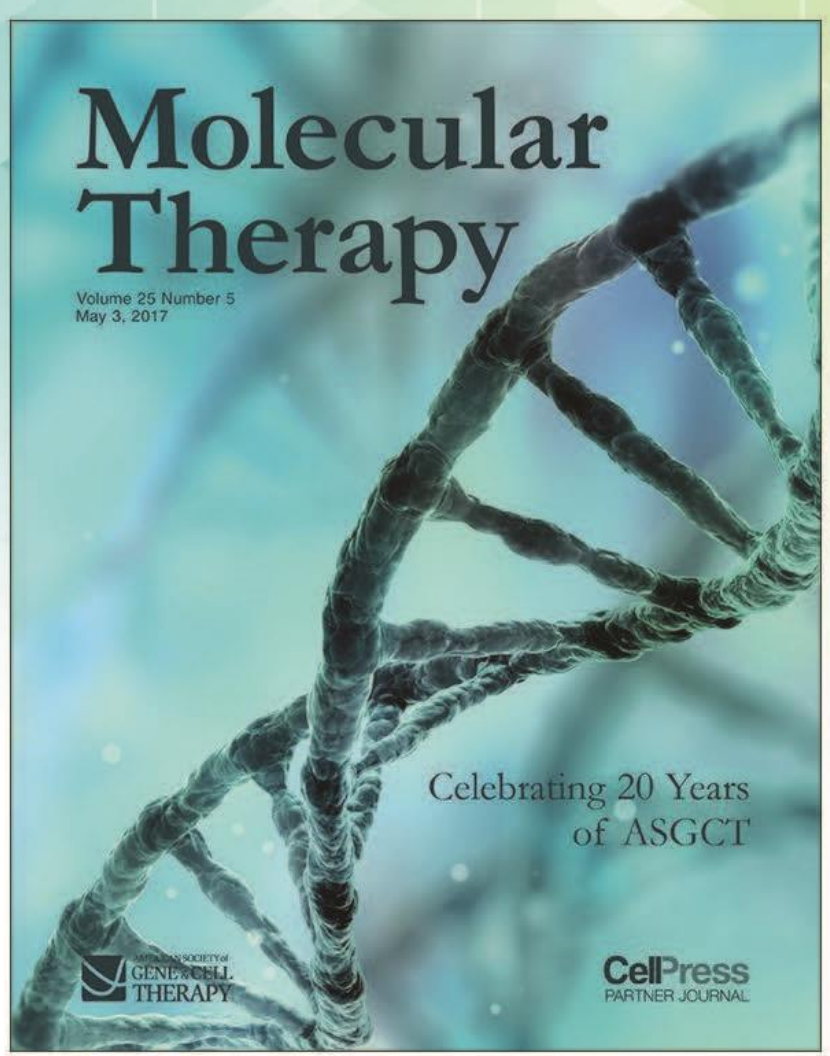
1995 | 1996 | 1997 | 1998 | 1999 | 2000 | 2001 | 2002 | 2003 | 2004 | 2005 | 2006 | 2007 | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017

2000

Launch of *Molecular Therapy*, official journal of ASGT



Inder Verma, PhD
Founding Editor-in-Chief



20 years of ASGCT and gene therapy.

1995 1996 1997 1998 1999 2000 2001 2002 2003 2004 2005 2006 2007 2008 2009 2010 2011 2012 2013 2014 2015 2016 2017

2002

Development of novel serotypes of AAV with increased efficiency and extended tropism as gene therapy vectors

James M. Wilson, MD
University of Pennsylvania, Philadelphia, Pennsylvania

Novel adeno-associated viruses from rhesus monkeys as vectors for human gene therapy

Guang-Ping Gao, Mauricio R. Alvira, Lili Wang, Roberto Calcedo, Julie Johnston, and James M. Wilson*

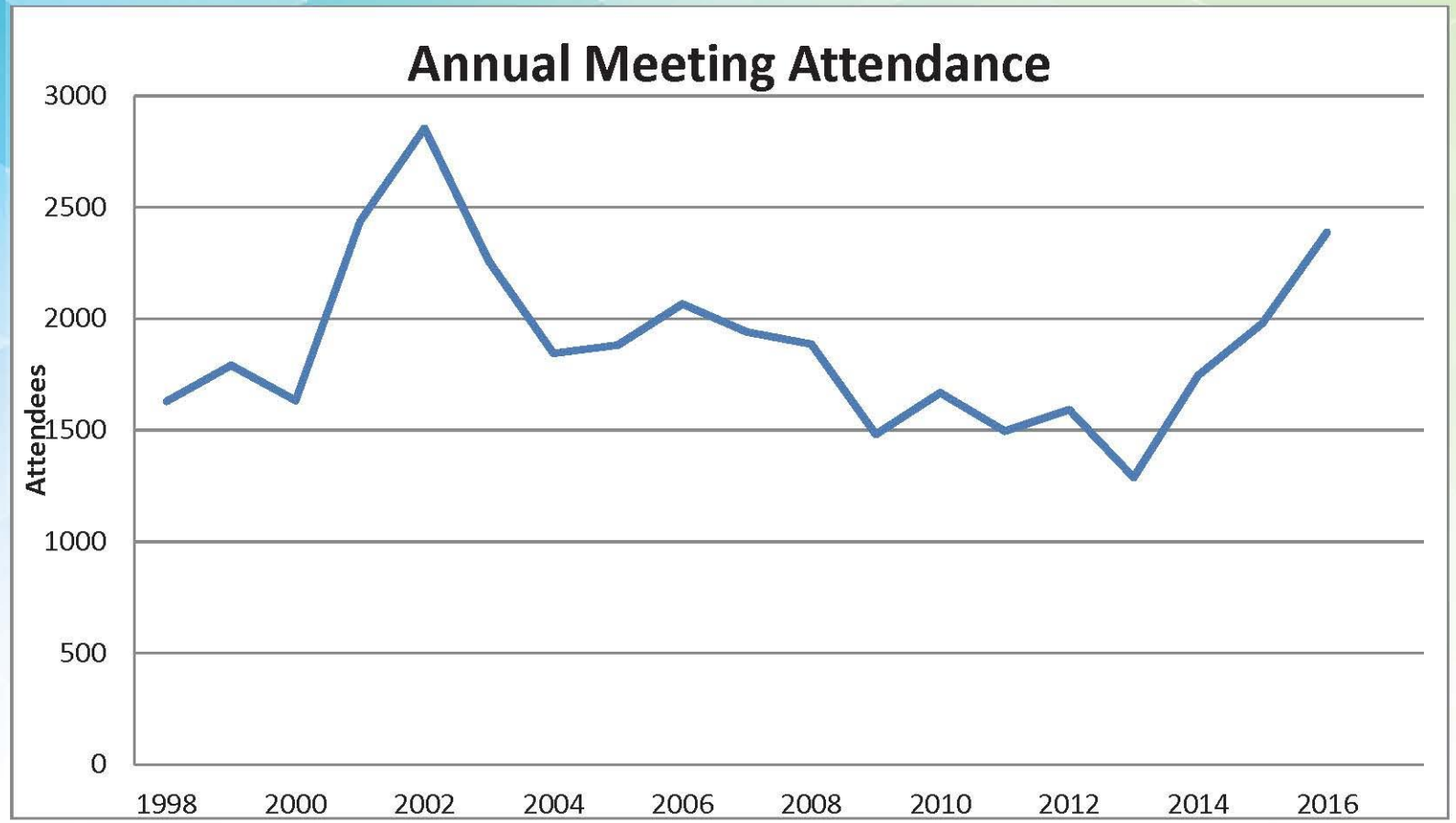
Guangping Gao, PhD
University of Massachusetts, Wocester, Massachusetts

20 years of ASGCT and gene therapy.

1995 | 1996 | 1997 | 1998 | 1999 | 2000 | 2001 | 2002 | 2003 | 2004 | 2005 | 2006 | 2007 | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017

2002

5th Annual Meeting attendance peaks with 2,885 attendees



20 years of ASGCT and gene therapy.

1995 1996 1997 1998 1999 2000 2001 2002 2003 2004 2005 2006 2007 2008 2009 2010 2011 2012 2013 2014 2015 2016 2017

2002

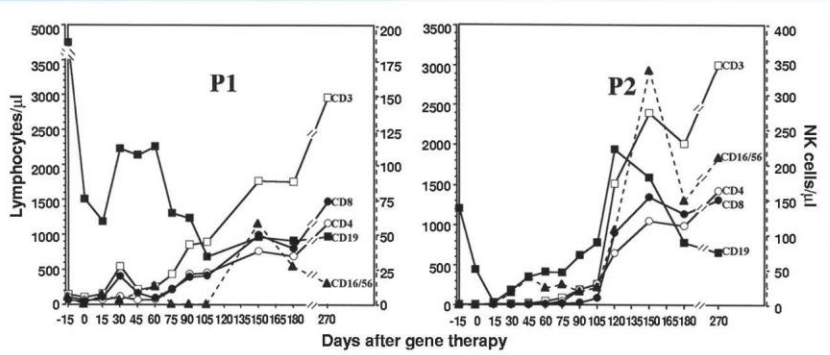
Clinical benefit of hematopoietic stem cell retrovirus-mediated gene therapy for X-SCID

Retroviral transfer of IL2R gene to hematopoietic stem and progenitor cells of boys with X-linked severe combined immunodeficiency (SCID)

Alain Fischer, MD and Marina Cavazzana-Calvo, MD, PhD
Hopital Necker, Paris, France

Gene Therapy of Human Severe Combined Immunodeficiency (SCID)–X1 Disease

Marina Cavazzana-Calvo,*[1,2,3] Salima Hacein-Bey,*[1,2,3] Geneviève de Saint Basile,[1] Fabian Gross,[2] Eric Yvon,[3] Patrick Nusbaum,[2] Françoise Selz,[1] Christophe Hue,[1,2] Stéphanie Certain,[1] Jean-Laurent Casanova,[1,4] Philippe Bousso,[5] Françoise Le Deist,[1] Alain Fischer[1,2,4]†



20 years of ASGCT and gene therapy.

1995 1996 1997 1998 1999 2000 2001 2002 2003 2004 2005 2006 2007 2008 2009 2010 2011 2012 2013 2014 2015 2016 2017

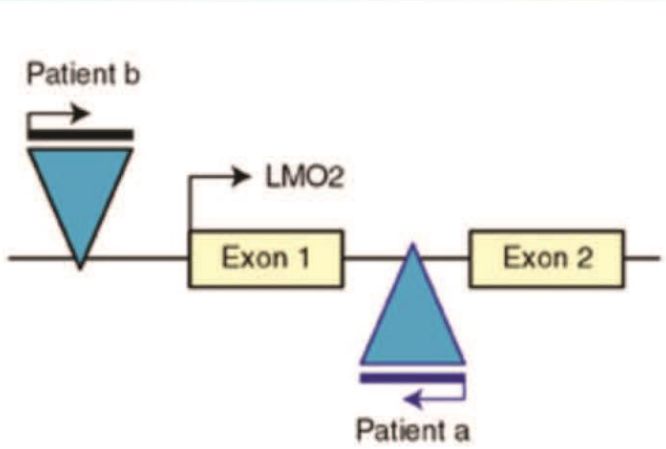
2003

Insertional mutagenesis and proto-oncogenic activation-mechanism of geno toxicity

Science 302: 415, 2003

LMO2-Associated Clonal T Cell Proliferation in Two Patients after Gene Therapy for SCID-X1

S. Hacein-Bey-Abina,[1,2*] C. Von Kalle,[6,7,8] M. Schmidt,[6,7] M. P. McCormack,[9] N. Wulffraat,[10] P. Leboulch,[11] A. Lim,[12] C. S. Osborne,[13] R. Pawliuk,[11] E. Morillon,[2] R. Sorensen,[19] A. Forster,[9] P. Fraser,[13] J. I. Cohen,[15] G. de Saint Basile,[1] I. Alexander,[16] U. Wintergerst,[17] T. Frebourg,[18] A. Aurias,[19] D. Stoppa-Lyonnet,[20] S. Romana,[3] I. Radford-Weiss,[3] F. Gross,[2] F. Valensi,[4] E. Delabesse,[4] E. Macintyre,[4] F. Sigaux,[20] J. Soulier,[21] L. E. Leiva,[14] M. Wissler,[6,7] C. Prinz,[6,7] T. H. Rabbitts,[9] F. Le Deist,[1] A. Fischer,[1,5†‡] M. Cavazzana-Calvo[1,2†]



Salima Hacein-Bey-Abina, PhD
Hopital Necker, Paris, France

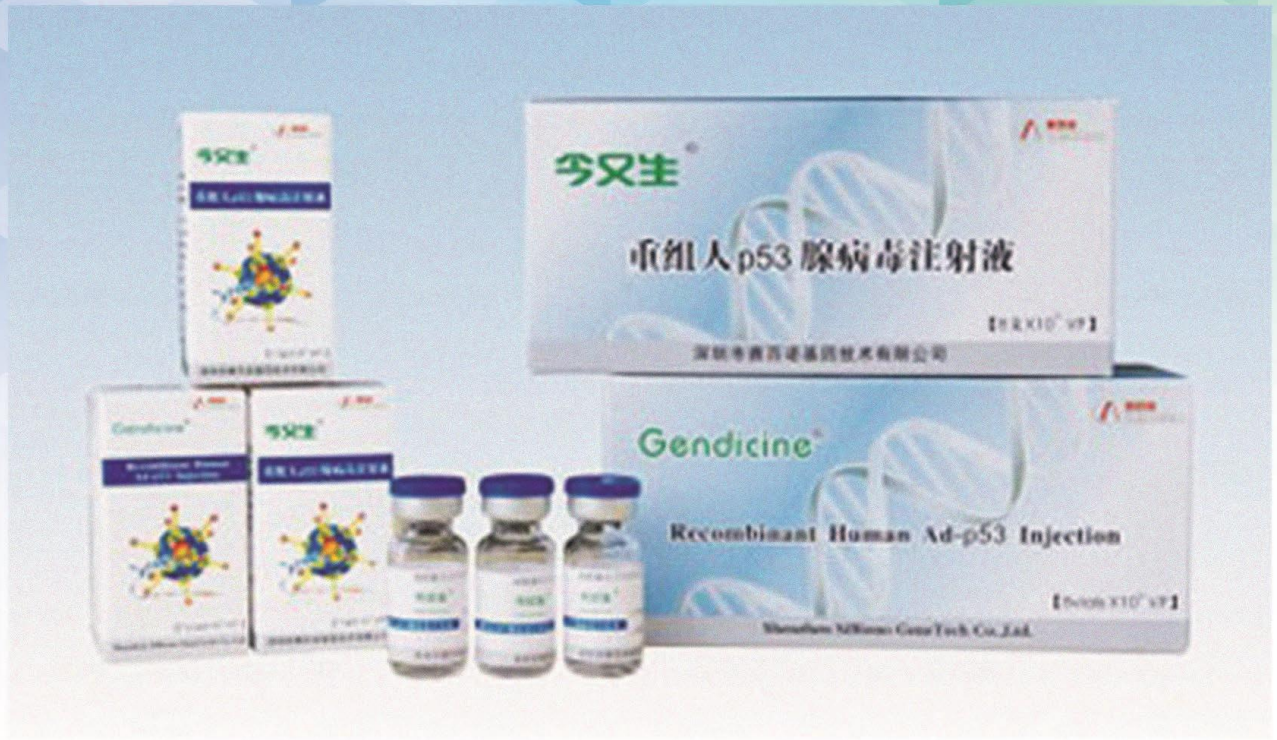
Christof Von Kalle, MD, PhD
University of Freiburg, Freiburg, Germany

20 years of ASGCT and gene therapy.

1995 | 1996 | 1997 | 1998 | 1999 | 2000 | 2001 | 2002 | 2003 | 2004 | 2005 | 2006 | 2007 | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017

2003

First regulatory approval of a gene therapy product



Gendecine, a cancer treatment consisting of a replication-incompetent adenovirus vector expressing wild-type p53 approved in China

20 years of ASGCT and gene therapy.

1995 1996 1997 1998 1999 2000 2001 2002 2003 2004 2005 2006 2007 2008 2009 2010 2011 2012 2013 2014 2015 2016 2017

2003–04

Elucidation of vector integration patterns

Transcription Start Regions in the Human Genome Are Favored Targets for MLV Integration

Xiaolin Wu,[1] Yuan Li,[2] Bruce Crise,[2] Shawn M. Burgess[1]*

Science 300: 1749, 2003

Distinct Genomic Integration of MLV and SIV Vectors in Primate Hematopoietic Stem and Progenitor Cells

Peiman Hematti[1☯¤1], Bum-Kee Hong[1☯], Cole Ferguson[1☯], Rima Adler[1], Hideki Hanawa[2], Stephanie Sellers[1], Ingeborg E. Holt[3], Craig E. Eckfeldt[4], Yugal Sharma[5], Manfred Schmidt[6], Christof von Kalle[7], Derek A. Persons[2], Eric M. Billings[5], Catherine M. Verfaillie[4], Arthur W. Nienhuis[2], Tyra G. Wolfsberg[3], Cynthia E. Dunbar[1]*, Boris Calmels[1¤2]

PLoS Biology: E423 2004

Retroviral DNA Integration: ASLV, HIV, and MLV Show Distinct Target Site Preferences

Rick S. Mitchell[1☯], Brett F. Beitzel[1☯], Astrid R. W. Schroder[2], Paul Shinn[3], Huaming Chen[3], Charles C. Berry[4], Joseph R. Ecker[3], Frederic D. Bushman[1]*

PLoS Biology: E234, 2004

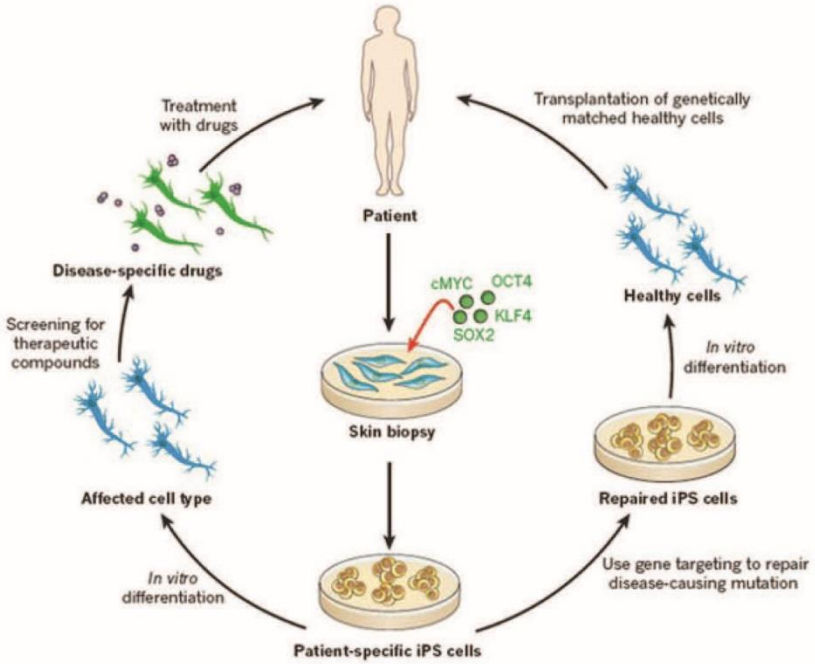
Fred Bushman, PhD
University of Pennsylvania, Philadelphia, Pennsylvania

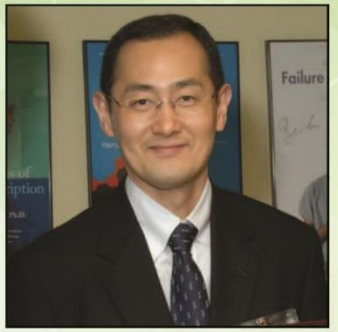
20 years of ASGCT and gene therapy.

1995 | 1996 | 1997 | 1998 | 1999 | 2000 | 2001 | 2002 | 2003 | 2004 | 2005 | 2006 | 2007 | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017

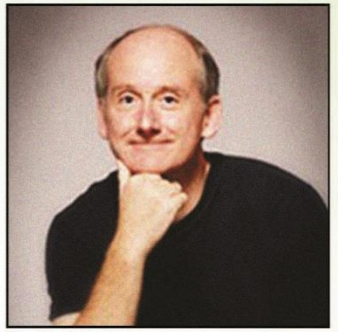
2008

Derivation of human induced pluripotent stem cells





Shinya Yamanaka, MD, PhD
Kyoto University, Kyoto, Japan



James Thomson, PhD
University of Wisconsin, Madison, Wisconsin

20 years of ASGCT and gene therapy.

1995 | 1996 | 1997 | 1998 | 1999 | 2000 | 2001 | 2002 | 2003 | 2004 | 2005 | 2006 | 2007 | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017

2008

Pioneering AAV gene therapy clinical trials for inherited form of blindness

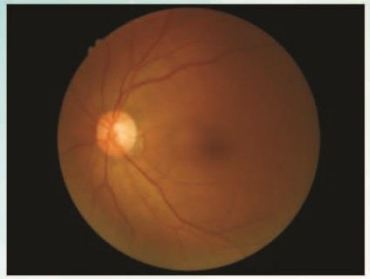
ORIGINAL ARTICLE

BRIEF REPORT

Safety and Efficacy of Gene Transfer for Leber's Congenital Amaurosis

Albert M. Maguire, M.D., Francesca Simonelli, M.D., Eric A. Pierce, M.D., Ph.D., Edward N. Pugh, Jr., Ph.D., Federico Mingozzi, Ph.D., Jeannette Bennicelli, Ph.D., Sandro Banfi, M.D., Kathleen A. Marshall, C.O.T., Francesco Testa, M.D., Enrico M. Surace, D.V.M., Settimio Rossi, M.D., Arkady Lyubarsky, Ph.D., Valder R. Arruda, M.D., Barbara Konkle, M.D., Edwin Stone, M.D., Ph.D., Junwei Sun, M.S., Jonathan Jacobs, Ph.D., Lou Dell'Osso, Ph.D., Richard Hertle, M.D., Jian-xing Ma, M.D., Ph.D., T. Michael Redmond, Ph.D., Xiaosong Zhu, M.D., Bernd Hauck, Ph.D., Olga Zelenaia, Ph.D., Kenneth S. Shindler, M.D., Ph.D., Maureen G. Maguire, Ph.D., J. Fraser Wright, Ph.D., Nicholas J. Volpe, M.D., Jennifer Wellman McDonnell, M.S., Alberto Auricchio, M.D., Katherine A. High, M.D., and Jean Bennett, M.D., Ph.D.

N Engl J Med 2008; 358:2240-2248 | May 22, 2008 | DOI: 10.1056/NEJMoa0802315



Jean Bennett, MD, PhD
University of Pennsylvania

ORIGINAL ARTICLE

BRIEF REPORT

Effect of Gene Therapy on Visual Function in Leber's Congenital Amaurosis

James W.B. Bainbridge, Ph.D., F.R.C.Ophth., Alexander J. Smith, Ph.D., Susie S. Barker, Ph.D., Scott Robbie, M.R.C.Ophth., Robert Henderson, M.R.C.Ophth., Kamaljit Balaggan, M.R.C.Ophth., Ananth Viswanathan, M.D., F.R.C.Ophth., Graham E. Holder, Ph.D., Andrew Stockman, Ph.D., Nick Tyler, Ph.D., Simon Petersen-Jones, Ph.D., Shomi S. Bhattacharya, Ph.D., Adrian J. Thrasher, Ph.D., M.R.C.P., F.R.C.P., Fred W. Fitzke, Ph.D., Barrie J. Carter, Ph.D., Gary S. Rubin, Ph.D., Anthony T. Moore, F.R.C.Ophth., and Robin R. Ali, Ph.D.

N Engl J Med 2008; 358:2231-2239 | May 22, 2008 | DOI: 10.1056/NEJMoa0802268

Robin Ali, PhD
University College,
London, United Kingdom

20 years of ASGCT and gene therapy.

1995 | 1996 | 1997 | 1998 | 1999 | 2000 | 2001 | 2002 | 2003 | 2004 | 2005 | 2006 | 2007 | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017

2009

Clinical benefit of hematopoietic stem cell retrovirus gene therapy for adenosine deamaninase-deficient SCID

Gene Therapy for Immunodeficiency Due to Adenosine Deaminase Deficiency

Alessandro Aiuti, M.D., Ph.D., Federica Cattaneo, M.D., Stefania Galimberti, Ph.D., Ulrike Benninghoff, M.D., Barbara Cassani, Ph.D., Luciano Callegaro, R.N., Samantha Scaramuzza, Ph.D., Grazia Andolfi, Massimiliano Mirolo, B.Sc., Immacolata Brigida, B.Sc., Antonella Tabucchi, Ph.D., Filippo Carlucci, Ph.D., Martha Eibl, M.D., Memet Aker, M.D., Shimon Slavin, M.D., Hamoud Al-Mousa, M.D., Abdulaziz Al Ghonaium, M.D., Alina Ferster, M.D., Andrea Duppenthaler, M.D., Luigi Notarangelo, M.D., Uwe Wintergerst, M.D., Rebecca H. Buckley, M.D., Marco Bregni, M.D., Sarah Marktel, M.D., Maria Grazia Valsecchi, Ph.D., Paolo Rossi, M.D., Fabio Ciceri, M.D., Roberto Miniero, M.D., Claudio Bordignon, M.D., and Maria-Grazia Roncarolo, M.D.

N Engl J Med 2009; 360:447-458 | January 29, 2009 | DOI: 10.1056/NEJMoa0805817

Alessandro Aiuti, MD, PhD
Ospedale San Raffaele,
TIGIT, Milan, Italy

Claudio Bordignon, MD
Ospedale San Raffaele,
TIGIT, Milan, Italy

Maria-Grazia Roncarolo, MD
Ospedale San Raffaele,
TIGIT, Milan, Italy

20 years of ASGCT and gene therapy.

1995 | 1996 | 1997 | 1998 | 1999 | 2000 | 2001 | 2002 | 2003 | 2004 | 2005 | 2006 | 2007 | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017

2009

ASGT changes name to reflect integration of gene and cell therapies

AMERICAN SOCIETY of
GENE & CELL
THERAPY

20 years of ASGCT and gene therapy.

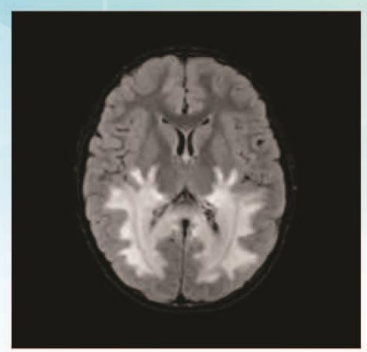
1995 | 1996 | 1997 | 1998 | 1999 | 2000 | 2001 | 2002 | 2003 | 2004 | 2005 | 2006 | 2007 | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017

2009,13

Positive clinical trial results using hematopoietic stem cell lentiviral gene therapy for central nervous system metabolic/storage disorders

Hematopoietic Stem Cell Gene Therapy with a Lentiviral Vector in X-Linked Adrenoleukodystrophy

Nathalie Cartier,[1,2]* Salima Hacein-Bey-Abina,[3,4,5]* Cynthia C. Bartholomae,[6] Gabor Veres,[7] Manfred Schmidt,[6] Ina Kutschera,[6] Michel Vidaud,[3] Ulrich Abel,[6] Liliane Dal-Cortivo,[3,5] Laure Caccavelli,[3,5] Nizar Mahlaoui,[8] Véronique Kiermer,[9] Denice Mittelstaedt,[10] Céline Bellesme,[2] Najiba Lahlou,[11] François Lefrère,[3] Stéphane Blanche,[8] Muriel Audit,[12] Emmanuel Payen,[13,14] Philippe Leboulch,[13,14,15] Bruno l'Homme,[1] Pierre Bougnères,[2] Christof Von Kalle,[6] Alain Fischer,[4,8] Marina Cavazzana-Calvo,[3,4,5]* Patrick Aubourg[1,2]*†



Science 326: 818, 2009

Nathalie Cartier, MD
University Paris-Descartes, Paris, France

Alessandra Biffi, MD
Ospedale San Raffaele, TIGIT, Milan, Italy

Lentiviral Hematopoietic Stem Cell Gene Therapy Benefits Metachromatic Leukodystrophy

Alessandra Biffi,* Eugenio Montini, Laura Lorioli, Martina Cesani, Francesca Fumagalli, Tiziana Plati, Cristina Baldoli, Sabata Martino, Andrea Calabria, Sabrina Canale, Fabrizio Benedicenti, Giuliana Vallanti, Luca Biasco, Simone Leo, Nabil Kabbara, Gianluigi Zanetti, William B. Rizzo, Nalini A. L. Mehta, Maria Pia Cicalese, Miriam Casiraghi, Jaap J. Boelens, Ubaldo Del Carro, David J. Dow, Manfred Schmidt, Andrea Assanelli, Victor Neduva, Clelia Di Serio, Elia Stupka, Jason Gardner, Christof von Kalle, Claudio Bordignon, Fabio Ciceri, Attilio Rovelli, Maria Grazia Roncarolo, Alessandro Aiuti, Maria Sessa, Luigi Naldini*

Science 341: 2003

Luigi Naldini, MD, PhD
Ospedale San Raffaele, TIGIT, Milan, Italy

20 years of ASGCT and gene therapy.

1995 | 1996 | 1997 | 1998 | 1999 | 2000 | 2001 | 2002 | 2003 | 2004 | 2005 | 2006 | 2007 | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017

2011

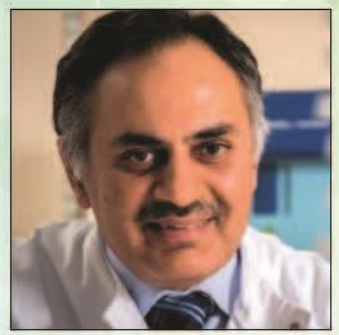
Successful AAV8 gene therapy for hemophilia B

ORIGINAL ARTICLE

Adenovirus-Associated Virus Vector–Mediated Gene Transfer in Hemophilia B

Amit C. Nathwani, M.B., Ch.B., Ph.D., Edward G.D. Tuddenham, M.B., B.S., M.D., Savita Rangarajan, M.B., B.S., Cecilia Rosales, Ph.D., Jenny McIntosh, Ph.D., David C. Linch, M.B., B.Chir., Pratima Chowdary, M.B., B.S., Anne Riddell, B.Sc., Arnulfo Jaquilmac Pie, B.S.N., Chris Harrington, B.S.N., James O'Beirne, M.B., B.S., M.D., Keith Smith, M.Sc., John Pasi, M.D., Bertil Glader, M.D., Ph.D., Pradip Rustagi, M.D., Catherine Y.C. Ng, M.S., Mark A. Kay, M.D., Ph.D., Junfang Zhou, M.D., Yunyu Spence, Ph.D., Christopher L. Morton, B.S., James Allay, Ph.D., John Coleman, M.S., Susan Sleep, Ph.D., John M. Cunningham, M.D., Deokumar Srivastava, Ph.D., Etiena Basner-Tschakarjan, M.D., Federico Mingozzi, Ph.D., Katherine A. High, M.D., John T. Gray, Ph.D., Ulrike M. Reiss, M.D., Arthur W. Nienhuis, M.D., and Andrew M. Davidoff, M.D.

N Engl J Med 2011; 365:2357-2365 | December 22, 2011 | DOI: 10.1056/NEJMoa1108046



Amit Nathwani, MB, ChB, PhD
University College, London, United Kingdom

Arthur W. Nienhuis, MD
St. Jude Children's Research Hospital, Memphis, Tennessee

Katherine A. High, MD
Spark Therapeutics

Andrew M. Davidoff, MD
St. Jude Children's Research Hospital, Memphis, Tennessee

20 years of ASGCT and gene therapy.

1995 1996 1997 1998 1999 2000 2001 2002 2003 2004 2005 2006 2007 2008 2009 2010 2011 2012 2013 2014 2015 2016 2017

2011–14

Dramatic clinical responses to chimeric antigen receptor engineered T Cells for CD19+ lymphoid malignancies

Brief report

Eradication of B-lineage cells and regression of lymphoma in a patient treated with autologous T cells genetically engineered to recognize CD19

James N. Kochenderfer,[1] Wyndham H. Wilson,[2] John E. Janik,[2] Mark E. Dudley,[1] Maryalice Stetler-Stevenson,[3] Steven A. Feldman,[1] Irina Maric,[4] Mark Raffeld,[3] Debbie-Ann N. Nathan,[1] Brock J. Lanier,[1] Richard A. Morgan,[1] and Steven A. Rosenberg[1]

Blood 116: 4099, 2010

ORIGINAL ARTICLE

BRIEF REPORT

Chimeric Antigen Receptor–Modified T Cells in Chronic Lymphoid Leukemia

David L. Porter, M.D., Bruce L. Levine, Ph.D., Michael Kalos, Ph.D., Adam Bagg, M.D., and Carl H. June, M.D.

N Engl J Med 2011; 365:725-733 | August 25, 2011 | DOI: 10.1056/NEJMoa1103849

ORIGINAL ARTICLE

Chimeric Antigen Receptor T Cells for Sustained Remissions in Leukemia

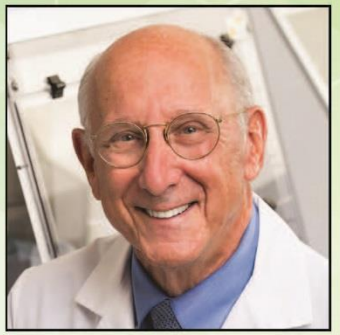
Shannon L. Maude, M.D., Ph.D., Noelle Frey, M.D., Pamela A. Shaw, Ph.D., Richard Aplenc, M.D., Ph.D., David M. Barrett, M.D., Ph.D., Nancy J. Bunin, M.D., Anne Chew, Ph.D., Vanessa E. Gonzalez, M.B.A., Zhaohui Zheng, M.S., Simon F. Lacey, Ph.D., Yolanda D. Mahnke, Ph.D., Jan J. Melenhorst, Ph.D., Susan R. Rheingold, M.D., Angela Shen, M.D., David T. Teachey, M.D., Bruce L. Levine, Ph.D., Carl H. June, M.D., David L. Porter, M.D., and Stephan A. Grupp, M.D., Ph.D.

N Eng J Med 371: 1507, 2014

Efficacy and Toxicity Management of 19-28z CAR T Cell Therapy in B Cell Acute Lymphoblastic Leukemia

Marco L. Davila,[1] Isabelle Riviere,[1,2,3,4] Xiuyan Wang,[4] Shirley Bartido,[4] Jae Park,[1] Kevin Curran,[5] Stephen S. Chung,[1] Jolanta Stefanski,[4] Oriana Borquez-Ojeda,[4] Malgorzata Olszewska,[4] Jinrong Qu,[4] Teresa Wasielewska,[4] Qing He,[4] Mitsu Fink,[4] Himaly Shinglot,[4] Maher Youssif,[4] Mark Satter,[4] Yongzeng Wang,[4] James Hosey,[4] Hilda Quintanilla,[1] Elizabeth Halton,[1] Yvette Bernal,[1] Diana C. G. Bouhassira,[2] Maria E. Arcila,[6] Mithat Gonen,[7] Gail J. Roboz,[8] Peter Maslak,[1] Dan Douer,[1] Mark G. Frattini,[9] Sergio Giralt,[1,2] Michel Sadelain,[1,2,3]* Renier Brentjens[1,2,3]*

Sci Transl Med 6: 224, 2014



Steven A. Rosenberg, MD, PhD
National Cancer Institute

Michel Sadelain, MD, PhD
Memorial Sloan Kettering Cancer Center

20 years of ASGCT and gene therapy.

1995 | 1996 | 1997 | 1998 | 1999 | 2000 | 2001 | 2002 | 2003 | 2004 | 2005 | 2006 | 2007 | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017

2012

Launch of *Molecular Therapy Nucleic Acids*, an open-access ASGCT journal

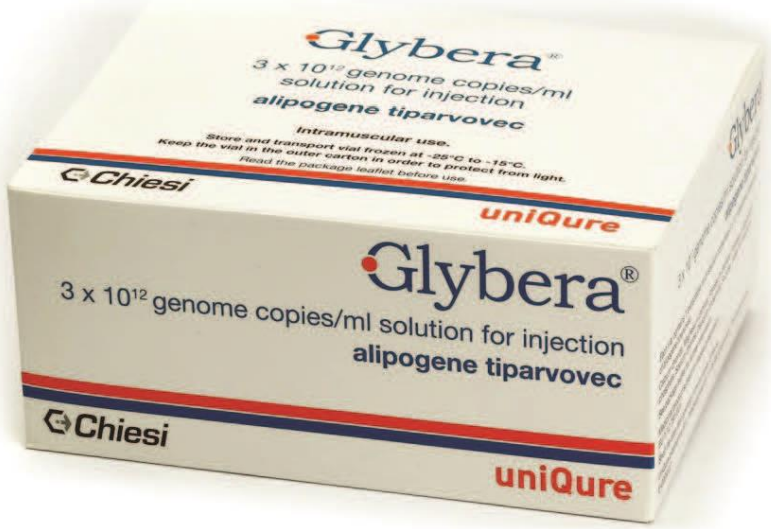
John Rossi, PhD
Founding Editor-in-Chief

20 years of ASGCT and gene therapy.

1995 | 1996 | 1997 | 1998 | 1999 | 2000 | 2001 | 2002 | 2003 | 2004 | 2005 | 2006 | 2007 | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017

2012

European Commission approves Glybera, the first approved gene therapy in Europe



AAV Vector delivering the lipoprotein lipase gene to patients with genetic hypercholesterolemia

20 years of ASGCT and gene therapy.

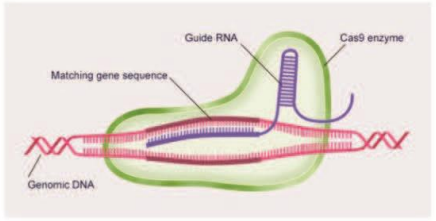
1995 | 1996 | 1997 | 1998 | 1999 | 2000 | 2001 | 2002 | 2003 | 2004 | 2005 | 2006 | 2007 | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017

2012–13

Discovery of CRISPR/Cas9 and utilization of this system to perform targeted gene editing in mammalian cells

Multiplex Genome Engineering Using CRISPR/Cas Systems

Le Cong,[1,2*] F. Ann Ran,[1,4*] David Cox,[1,3] Shuailiang Lin,[1,5] Robert Barretto,[6] Naomi Habib,[1] Patrick D. Hsu,[1,4] Xuebing Wu,[7] Wenyan Jiang,[8] Luciano A. Marraffini,[8] Feng Zhang[1]†



Science 339: 819, 2013

A Programmable Dual-RNA–Guided DNA Endonuclease in Adaptive Bacterial Immunity

Martin Jinek,[1,2*] Krzysztof Chylinski,[3,4*] Ines Fonfara,[4] Michael Hauer,[2]† Jennifer A. Doudna,[1,2,5,6]‡ Emmanuelle Charpentier[4]‡

RNA-Guided Human Genome Engineering via Cas9

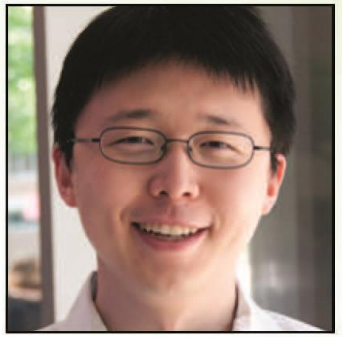
Prashant Mali,[1*] Luhan Yang,[1,3*] Kevin M. Esvelt,[2] John Aach,[1] Marc Guell,[1] James E. DiCarlo,[4] Julie E. Norville,[1] George M. Church[1,2]†

Science 339: 823, 2013

Jennifer Doudna, PhD
University of California, Berkeley, California

Emmanuelle Charpantier, PhD
Umea Center for Microbial Research, Umea Sweden

George Church, PhD
Harvard Medical School, Boston, Massachusetts



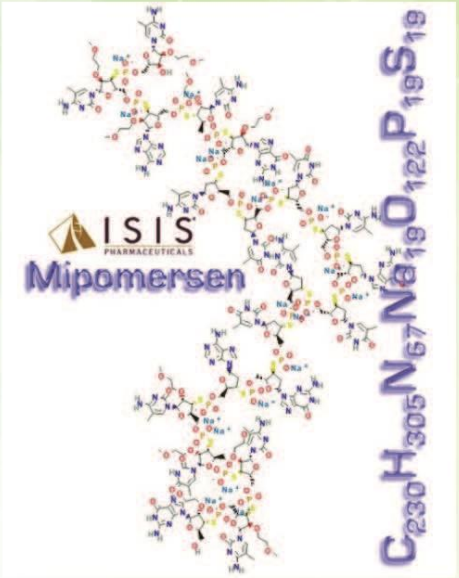
Feng Zhang, PhD
MIT, Cambridge, Massachusetts

20 years of ASGCT and gene therapy.

1995 1996 1997 1998 1999 2000 2001 2002 2003 2004 2005 2006 2007 2008 2009 2010 2011 2012 2013 2014 2015 2016 2017

2013

FDA approval of Kynamro (mipomesen), an antisense oligonucleotide inhibitor of ApoB-100, for use in patients with hypercholesterolemia



Articles

Mipomersen, an apolipoprotein B synthesis inhibitor, for lowering of LDL cholesterol concentrations in patients with homozygous familial hypercholesterolaemia: a randomised, double-blind, placebo-controlled trial

Prof Frederick J Raal, MD, Raul D Santos, MD, Dirk J Blom, MD, Prof A David Marais, MD, Min-Ji Charng, MD, William C Cromwell, MD, Robin H Lachmann, MRCP, Daniel Gaudet, MD, Ju L Tan, MB BS, Scott Chasan-Taber, PhD, Diane L Tribble, PhD, JoAnn D Flaim, PhD, Stanley T Crooke, MD

Lancet 375: 998, 2010

20 years of ASGCT and gene therapy.

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2014

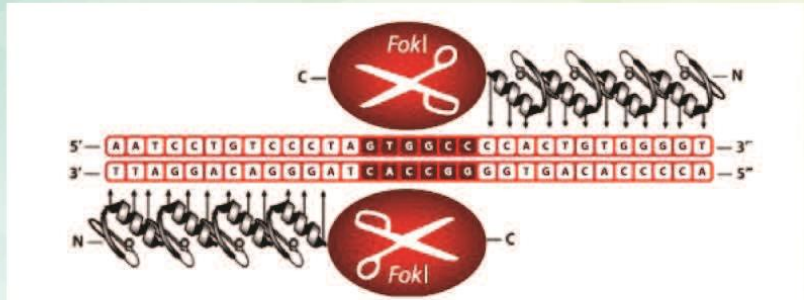
First clinical trial of genome editing

ORIGINAL ARTICLE

Gene Editing of *CCR5* in Autologous CD4 T Cells of Persons Infected with HIV

Pablo Tebas, M.D., David Stein, M.D., Winson W. Tang, M.D., Ian Frank, M.D., Shelley Q. Wang, M.D., Gary Lee, Ph.D., S. Kaye Spratt, Ph.D., Richard T. Surosky, Ph.D., Martin A. Giedlin, Ph.D., Geoff Nichol, M.D., Michael C. Holmes, Ph.D., Philip D. Gregory, Ph.D., Dale G. Ando, M.D., Michael Kalos, Ph.D., Ronald G. Collman, M.D., Gwendolyn Binder-Scholl, Ph.D., Gabriela Plesa, M.D., Ph.D., Wei-Ting Hwang, Ph.D., Bruce L. Levine, Ph.D., and Carl H. June, M.D.

N Engl J Med 2014; 370:901-910 | March 6, 2014 | DOI: 10.1056/NEJMoa1300662



Pablo Tebas, MD *Carl June, MD* *Bruce L. Levine, PhD*

20 years of ASGCT and gene therapy.

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2014

Launches of *Molecular Therapy Methods and Clinical Development* and *Molecular Therapy Oncolytics*, two additional open-access ASGCT journals

Matthew Porteus, PhD
Founding Editor-in-Chief, MTMCD

Yuman Fong, MD
Founding Editor-in-Chief, MTO

20 years of ASGCT and gene therapy.

1995 1996 1997 1998 1999 2000 2001 2002 2003 2004 2005 2006 2007 2008 2009 2010 2011 2012 2013 2014 2015 2016 2017

2014

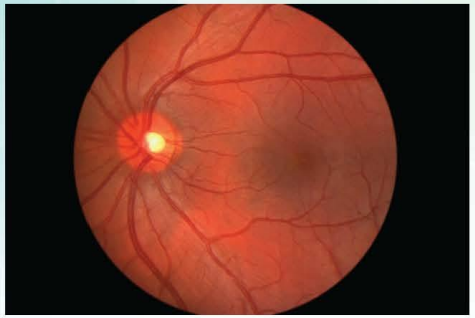
First clinical trial of iPSC-derived cells to regenerate tissue

Massayo Takahashi, PhD
RIKEN, Kobe, Japan

Autologous Induced Stem-Cell–Derived Retinal Cells for Macular Degeneration

Michiko Mandai, M.D., Ph.D., Akira Watanabe, Ph.D., Yasuo Kurimoto, M.D., Ph.D., Yasuhiko Hirami, M.D., Ph.D., Chikako Morinaga, Ph.D., Takashi Daimon, Ph.D., Masashi Fujihara, M.D., Ph.D., Hiroshi Akimaru, Ph.D., Noriko Sakai, B.S., Yumiko Shibata, M.S., Motoki Terada, Yui Nomiya, M.S., Shigeki Tanishima, B.S., Masahiro Nakamura, M.D., Ph.D., Hiroyuki Kamao, M.D., Ph.D., Sunao Sugita, M.D., Ph.D., Akishi Onishi, Ph.D., Tomoko Ito, Kanako Fujita, Shin Kawamata, M.D., Ph.D., Masahiro J. Go, Ph.D., Chikara Shinohara, Ph.D., Ken-ichiro Hata, D.D.S., Ph.D., Masanori Sawada, M.D., Ph.D., Midori Yamamoto, Sachiko Ohta, Yasuo Ohara, B.S., Kenichi Yoshida, M.D., Ph.D., Junko Kuwahara, Yuko Kitano, M.S., Naoki Amano, M.S., Masafumi Umekage, M.S., Fumiyo Kitaoka, Ph.D., Azusa Tanaka, Ph.D., Chihiro Okada, M.S., Naoko Takasu, M.S., Seishi Ogawa, M.D., Ph.D., Shinya Yamanaka, M.D., Ph.D., and Masayo Takahashi, M.D., Ph.D.

N Engl J Med 2017; 376:1038-1046 | March 16, 2017 | DOI: 10.1056/NEJMoa1608368



20 years of ASGCT and gene therapy.

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2015

Drs. Alain Fischer and Theodore Friedmann are awarded the prestigious Japan Prize for the proposal of the concept of gene therapy and its clinical applications

Alain Fischer, MD, PhD
Institut Imagine, Paris, France

Theodore Friedmann, MD
University of California, San Diego, California

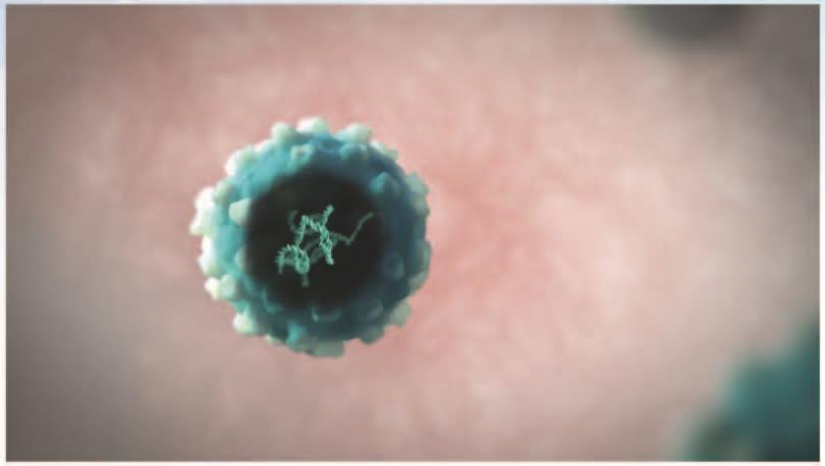
20 years of ASGCT and gene therapy.

1995 | 1996 | 1997 | 1998 | 1999 | 2000 | 2001 | 2002 | 2003 | 2004 | 2005 | 2006 | 2007 | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017

2016

European Commission approves Strimvelis, the first ex vivo gene therapy product worldwide

Strimvelis



Retrovirus vector expressing the adenosine deaminase gene to treat patients with ADA-deficient SCID

20 years of ASGCT and gene therapy.

1995 1996 1997 1998 1999 2000 2001 2002 2003 2004 2005 2006 2007 2008 2009 2010 2011 2012 2013 2014 2015 2016 2017

2016

FDA approval of the oligonucleotide drug Spinraza for spinal muscle atrophy

Articles

Treatment of infantile-onset spinal muscular atrophy with nusinersen: a phase 2, open-label, dose-escalation study

Dr Richard S Finkel, MD, Claudia A Chiriboga, MD, Jiri Vajsar, MD, John W Day, MD, Jacqueline Montes, EdD, Darryl C De Vivo, MD, Mason Yamashita, MD, Frank Rigo, PhD, Gene Hung, MD, Eugene Schneider, MD, Daniel A Norris, PhD, Shuting Xia, MS, C Frank Bennett, PhD, Kathie M Bishop, PhD

Lancet 388: 3017, 2016

20 years of ASGCT and gene therapy.

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2017

Use of "universal donor" TALEN-engineered CAR-T cells to achieve remission in a patient with refractory CD19+ acute leukemia

Remission in infant with refractory acute leukemia

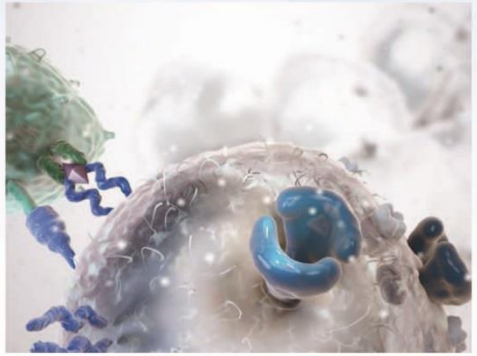
REPORTS | CANCER

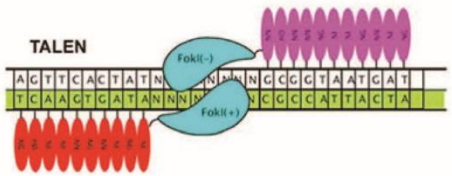
Molecular remission of infant B-ALL after infusion of universal TALEN gene-edited CAR T cells

Waseem Qasim[1,2,*], Hong Zhan[1], Sujith Samarasinghe[2], Stuart Adams[2], Persis Amrolia[1,2], Sian Stafford[1], Katie Butler[1], Christi...

+ See all authors and affiliations

Sci Trans Med: 2017





Waseem Qasim, PhD
Great Ormond Street Hospital/University Collegve, London, United Kingdom

Adrian Thrasher, PhD
Great Ormond Street Hospital/University Collegve, London, United Kingdom