

# Gene Therapy

## Bibliography of One Hundred Key Papers

- 
- Anderson WA.** *Human gene therapy.*  
**Science.** 1992;256:808-813.
- 
- Autieri MV, Yue L, Ferstein GZ, Ohlstein E.** *Antisense oligonucleotides to the p65 subunit of NF- $\kappa$ B inhibit vascular smooth muscle cell adherence and proliferation and prevent neointima formation in rat carotid arteries.*  
**Biochem Biophys Res Commun.** 1995;213:827-836.
- 
- Barr E, Kalynych AM, Tripathy SK, Kozarsky K, Wilson JM, Leiden JM.** *Efficient catheter-mediated gene transfer into the heart using replication-defective adenovirus.*  
**Gene Ther.** 1994;1:51-58.
- 
- Barr E, Leiden JM.** *Systemic delivery of recombinant proteins by genetically modified myoblasts.*  
**Science.** 1991;254:1507-1509.
- 
- Bennett MR, Anglin S, McEwan JR, Jagoe R, Newby AC, Evan GI.** *Inhibition of vascular smooth muscle cell proliferation in vitro and in vivo by c-myc antisense oligonucleotides.*  
**J Clin Invest.** 1994;93:820-828.
- 
- Berkner KL.** *Expression of heterologous sequences in adenoviral vectors.*  
**Curr Top Microbiol Immunol.** 1992;158:39-66.
- 
- Bielinska A, Schivdasani RA, Zhang L, Nabel GJ.** *Regulation of gene expression with double-stranded phosphothioate oligonucleotides.*  
**Science.** 1990;250:997-1000.
- 
- Brody SL, Crystal RG.** *Adenovirus-mediated in vivo gene transfer.*  
**Ann N Y Acad Sci.** 1994;716:90-101.
- 
- Buttrick PM, Kass A, Kitsis RN, Kaplan ML, Leinwand LA.** *Behavior of genes directly injected into the rat heart in vivo.*  
**Circ Res.** 1992;70:193-198.
- 
- Chang MW, Barr E, Seltzer J, et al.** *Cytostatic gene therapy for vascular proliferative disorders with a constitutively active form of the retinoblastoma gene product.*  
**Science.** 1995;267:518-522.
- 
- Chowdhury JR, Grossman M, Gupta S, Chowdhury NR, Baker JR, Wilson JM.** *Long-term improvement of hypercholesterolemia after ex vivo gene therapy in LDLR-deficient rabbits.*  
**Science.** 1991;254:1802-1805.
- 
- Cone RD, Mulligan RC.** *High-efficiency gene transfer into mammalian cells: generation of helper-free recombinant retrovirus with broad mammalian host range.*  
**Proc Natl Acad Sci USA.** 1984;81:6349-6353.
-

**Bibliography of One Hundred Key Papers**

---

- Cox JL, Chiasson DA, Gotlieb AI. *Stranger in a strange land: the pathogenesis of saphenous vein graft stenosis with emphasis on structural and functional differences between veins and arteries.* **Prog Cardiovasc Dis.** 1991;34:45-68.
- 
- Crystal RG. *Transfer of genes to humans: early lessons and obstacles to success.* **Science.** 1995;270:404-410.
- 
- Crystal RG, McElvaney NG, Rosenfeld MA, et al. *Administration of an adenovirus containing the human CFTR cDNA to the respiratory tract of individuals with cystic fibrosis.* **Nat Genet.** 1994;8:42-51.
- 
- Culver KM, Osborne WR, Miller AD, et al. *Correction of ADA deficiency in human T lymphocytes using retroviral-mediated gene transfer.* **Transplant Proc.** 1991;23:170-171.
- 
- Curiel DT, Agarwal S, Wagner E, Cotten M. *Adenovirus enhancement of transferrin-polylysine-mediated gene delivery.* **Proc Natl Acad Sci USA.** 1991;88:8850-8854.
- 
- Danos O, Mulligan RC. *Expression of retroviral trans-acting functions from complementary crippled genomes: a system for helper-free packaging of retroviral vectors.* **J Cell Biochem.** 1988;12:172-178.
- 
- Dhawan J, Pan LC, Pavlath GK, Travis MA, Lanctot AM, Blau HM. *Systemic delivery of human growth hormone by injection of genetically engineered myoblasts.* **Science.** 1991;254:1509-1512.
- 
- Dichek DA, Neville RF, Zwiebel JA, Freeman SM, Leon MB, Anderson WF. *Seeding of intravascular stents with genetically engineered endothelial cells.* **Circulation.** 1989;80:1347-1353.
- 
- Dzau VJ. *The role of mechanical and humoral factors in growth regulation of vascular smooth muscle and cardiac myocytes.* **Curr Opin Nephrol Hypertens.** 1993;2:27-32.
- 
- Dzau VJ, Braunwald E. *Resolved and unresolved issues in the prevention and treatment of coronary artery disease: a workshop consensus statement.* **Am Heart J.** 1991;121:1244-1263.
- 
- Dzau VJ, Gibbons GH, Cooke JP, Omoigui N. *Vascular biology and medicine in the 1990s: scope, concepts, potentials, and perspectives.* **Circulation.** 1993;87:705-719.
- 
- Dzau VJ, Morishita R, Gibbons GH. *Gene therapy for cardiovascular disease.* **Trends Biotechnol.** 1993;11:205-210.
- 
- Epstein SE, Speir E, Unger EF, Guzman RJ, Finkel T. *The basis of molecular strategies for treating coronary restenosis after angioplasty.* **J Am Coll Cardiol.** 1994;23:1278-1288.
- 
- Felgner PL, Gader TR, Holm M, et al. *Lipofectin: a highly efficient, lipid-mediated DNA-transfection procedure.* **Proc Natl Acad Sci USA.** 1987;84:7413-7417.
-



- 
- Felgner PL, Rhodes G.** *Gene therapeutics.*  
**Nature.** 1991;349:351-352.
- 
- Ferry N, Duplessis O, Houssin D, Danos O, Heard JM.** *Retroviral-mediated gene transfer into hepatocytes in vivo.*  
**Proc Natl Acad Sci USA.** 1991;88:8377-8381.
- 
- Gibbons GH, Dzau VJ.** *The emerging concept of vascular remodeling.*  
**N Engl J Med.** 1994;330:1431-1438.
- 
- Grossman M, Rader DJ, Muller DWM, et al.** *A pilot study of ex vivo gene therapy for homozygous familial hypercholesterolemia.*  
**Nat Med.** 1995;1:1148-1154.
- 
- Guzman RJ, Lemarchand P, Crystal RG, Epstein SE, Finkel T.** *Efficient and selective adenovirus-mediated gene transfer into vascular neointima.*  
**Circulation.** 1993;88:2838-2848.
- 
- Guzman RJ, Lemarchand P, Crystal RG, Epstein SE, Finkel T.** *Efficient gene transfer into myocardium by direct injection of adenovirus vectors.*  
**Circ Res.** 1993;73:1202-1207.
- 
- Hamamori Y, Samal B, Tian J, Kedes L.** *Myoblast transfer of human erythropoietin gene in a mouse model of renal failure.*  
**J Clin Invest.** 1995;95:1808-1813.
- 
- Indolfi C, Avvedimento EV, Rapacciuolo A, et al.** *Inhibition of cellular ras prevents smooth muscle cell proliferation after vascular injury in vivo.*  
**Nat Med.** 1995;1:541-545.
- 
- Isner JM, Walsh K, Symes J, et al.** *Arterial gene therapy for therapeutic angiogenesis in patients with peripheral artery disease.*  
**Circulation.** 1995;91:2687-2692.
- 
- Kaneda Y, Iwai K, Uchida T.** *Increased expression of DNA cointroduced with nuclear protein in adult rat liver.*  
**Science.** 1989;243:375-378.
- 
- Kaneda Y, Iwai K, Uchida T.** *Introduction and expression of the human insulin gene in adult rat liver.*  
**J Biol Chem.** 1989;264:12126-12129.
- 
- Kaneda Y, Morishita R, Tomita N.** *Increased expression of DNA cointroduced with nuclear protein in adult rat liver.*  
**J Mol Med.** 1995;73:289-297.
- 
- Kass-Eisler A, Falck-Pedersen E, Alvira M, et al.** *Quantitative determination of adenovirus-mediated gene delivery to rat cardiac myocytes in vitro and in vivo.*  
**Proc Natl Acad Sci USA.** 1993;90:11498-11502.
- 
- Kato K, Nakanishi M, Kaneda Y, Uchida T, Okada Y.** *Expression of Hepatitis B virus surface antigen in adult rat liver.*  
**J Biol Chem.** 1991;266:3361-3364.
- 
- Kay MA, Rothenberg S, Landen CN, et al.** *In vivo gene therapy of hemophilia B: sustained partial correction in factor IX-deficient dogs.*  
**Science.** 1993;262:117-119.
-

**Bibliography of One Hundred Key Papers**

---

- Kitsis RN, Buttrick PM, Mc Nally EM, Kaplan ML, Leinwand LA.** *Hormonal modulation of a gene injected into rat heart in vivo.* **Proc Natl Acad Sci USA.** 1991;88:4138-4142.
- 
- Knowles MR, Hohneker KW, Zhou Z, et al.** *A controlled study of adenoviral-vector-mediated gene transfer in the nasal epithelium of patients with cystic fibrosis.* **N Engl J Med.** 1995;333:823-831.
- 
- Koh GY, Kim S, Klug MG, Park K, Soonpaa MH, Field LJ.** *Targeted expression of transforming growth factor- $\beta_1$  in intracardiac grafts promotes vascular endothelial cell DNA synthesis.* **J Clin Invest.** 1995;95:114-121.
- 
- Koh GY, Soonpaa MH, Klug MG, et al.** *Stable fetal cardiomyocyte grafts in the hearts of dystrophic mice and dogs.* **J Clin Invest.** 1995;96:2034-2042.
- 
- Kopfler WP, Willard M, Betz T, Willard JE, Gerard RD, Meidell RS.** *Adenovirus-mediated transfer of a gene encoding human apolipoprotein A-I into normal mice increases circulating high-density lipoprotein cholesterol.* **Circulation.** 1994;90:1319-1327.
- 
- Kozarsky KF, McKinley DR, Austin LL, Raper SE, Stratford-Perricaudet LD, Wilson JM.** *In vivo correction of low density lipoprotein receptor deficiency in the Watanabe heritable hyperlipidemic rabbit with recombinant adenoviruses.* **J Biol Chem.** 1994;13:13695-13702.
- 
- Kupfer JM, Ruan XM, Liu G, Matloff J, Forrester J, Chaux A.** *High-efficiency gene transfer to autologous rabbit jugular vein grafts using adenovirus-transferrin/polylysine-DNA complexes.* **Hum Gene Ther.** 1994;5:1437-1443.
- 
- Lim CS, Chapman GD, Gammon RS, et al.** *Direct in vivo gene transfer into the coronary artery and peripheral vasculatures in the intact dog.* **Circulation.** 1991;83:2007-2011.
- 
- Lin H, Parmacek MS, Morle G, Bolling S, Leiden JM.** *Expression of recombinant gene in myocardium in vivo after direct injection of DNA.* **Circulation.** 1990;82:2217-2221.
- 
- Lynch CM, Clowes MM, Osborne RA, Clowes AW, Miller AD.** *Long-term expression of human adenosine deaminase in vascular smooth muscle cells of rats: a model for gene therapy.* **Proc Natl Acad Sci USA.** 1992;89:1138-1142.
- 
- Mann MJ, Gibbons GH, Kernoff RS, et al.** *Genetic engineering of vein grafts resistant to atherosclerosis.* **Proc Natl Acad Sci USA.** 1995;92:4502-4506.
- 
- Mendell JR, Kissel JT, Amato AA, et al.** *Myoblast transfer in the treatment of Duchenne's muscular dystrophy.* **N Engl J Med.** 1995;333:832-838.
- 
- Messina LM, Podrazik RM, Whitehill TA, et al.** *Adhesion and incorporation of lacZ-transduced endothelial cells into the capillary wall in the rat.* **Proc Natl Acad Sci USA.** 1992;89:12018-12022.
-



- 
- Milano CA, Allen LF, Rockman HA, et al. *Enhanced myocardial function in transgenic mice overexpressing the  $\beta_2$ -adrenergic receptor.*  
**Science.** 1994;264:582-586.
- 
- Morishita R, Gibbons GH, Ellison KE, et al. *Single intraluminal delivery of antisense cdc 2 kinase and proliferating cell nuclear antigen oligonucleotides results in chronic inhibition of neointimal hyperplasia.*  
**Proc Natl Acad Sci USA.** 1993;90:8474-8478.
- 
- Morishita R, Gibbons GH, Ellison KE, et al. *Intimal hyperplasia after vascular injury is inhibited by antisense cdk 2 kinase oligonucleotides.*  
**J Clin Invest.** 1994;93:1458-1464.
- 
- Morishita R, Gibbons GH, Ellison KE, et al. *Evidence for direct local effect of angiotensin in vascular hypertrophy. In vivo gene transfer of angiotensin converting enzyme.*  
**J Clin Invest.** 1994;94:978-984.
- 
- Morishita R, Gibbons GH, Horiuchi M, et al. *A novel molecular strategy using cis element "decoy" of E2F binding site inhibits smooth muscle proliferation in vivo.*  
**Proc Natl Acad Sci USA.** 1995;92:5855-5859.
- 
- Morishita R, Gibbons GH, Kaneda Y, Ogihara T, Dzau VJ. *Novel and effective gene transfer technique for study of vascular renin angiotensin system.*  
**J Clin Invest.** 1993;91:2580-2585.
- 
- Morishita R, Gibbons GH, Kaneda Y, Ogihara T, Dzau VJ. *Pharmacokinetics of antisense oligodeoxynucleotides (cyclin B1 and cdc2 kinase) in the vessel wall in vivo: enhanced therapeutic utility for restenosis by HVJ-liposome delivery.*  
**Gene.** 1994;149:13-19.
- 
- Nabel EG, Plautz G, Boyce FM, Stanley JC, Nabel GJ. *Recombinant gene expression in vivo within endothelial cells of the arterial wall.*  
**Science.** 1989;244:1342-1344.
- 
- Nabel EG, Plautz G, Nabel GJ. *Site-specific gene expression in vivo by direct gene transfer into the arterial wall.*  
**Science.** 1990;249:1285-1288.
- 
- Nabel EG, Shum L, Pompili VJ, et al. *Direct transfer of transforming growth factor- $\beta_1$  gene into arteries stimulates fibrocellular hyperplasia.*  
**Proc Natl Acad Sci USA.** 1993;90:10579-10763.
- 
- Nabel EG, Yang Z, Liptay S, et al. *Recombinant platelet-derived growth factor B gene expression in porcine arteries induce intimal hyperplasia in vivo.*  
**J Clin Invest.** 1993;91:1822-1829.
- 
- Nabel EG, Yang Z, Plautz G, et al. *Recombinant fibroblast growth factor-1 promotes intimal hyperplasia and angiogenesis in arteries in vivo.*  
**Nature.** 1993;362:844-846.
- 
- Nathan A, Nugent MA, Edelman ER. *Tissue engineered perivascular endothelial cell implants regulate vascular injury.*  
**Proc Natl Acad Sci USA.** 1995;92:8130-8134.
-

**Bibliography of One Hundred Key Papers**

---

- Ohno T, Gordon D, San H, et al.** *Gene therapy for vascular smooth muscle cell proliferation after arterial injury.*  
**Science.** 1994;265:781-784.
- 
- Okada Y.** *Sendai virus-induced cell fusion.*  
**Methods Enzymol.** 1993;221:18-41.
- 
- Partridge TA, Morgan JE, Coulton GR, Hoffman EP, Kunkel LM.** *Conversion of mdx myofibres from dystrophin-negative to -positive by injection of normal myoblasts.*  
**Nature.** 1989;337:176-179.
- 
- Rando TA, Blau HM.** *Primary mouse myoblast purification, characterization, and transplantation for cell-mediated gene therapy.*  
**J Cell Biol.** 1994;125:1275-1287.
- 
- Riessen R, Isner JM.** *Prospects for site-specific delivery of pharmacologic and molecular therapies.*  
**J Am Coll Cardiol.** 1994;23:1234-1244.
- 
- Ross R.** *The pathogenesis of atherosclerosis: a perspective for the 1990s.*  
**Nature.** 1993;362:801-809.
- 
- Sawa I, Suzuki K, Bai HZ, et al.** *Efficiency of in vivo gene transfection into transplanted rat heart by coronary infusion of HVJ-liposome.*  
**Circulation.** 1995;92(suppl):II-479-II-482.
- 
- Shi Y, Fard A, Galeo A, et al.** *Transcatheter delivery of c-myc antisense oligomers reduces neointimal formation in a porcine model of coronary artery balloon injury.*  
**Circulation.** 1994;90:944-951.
- 
- Shubeita HE, Thorburn J, Chien KR.** *Microinjection of antibodies and expression vectors into living myocardial cells. Development of a novel approach to identify candidate genes that regulate cardiac growth and hypertrophy.*  
**Circulation.** 1992;85:2236-2246.
- 
- Simons M, Edelman ER, DeKeyser JL, Langer R, Rosenberg RD.** *Antisense c-myc oligonucleotides inhibit intimal arterial smooth muscle cell accumulation in vivo.*  
**Nature.** 1992;359:67-70.
- 
- Simons M, Edelman ER, Rosenberg RD.** *Antisense proliferating cell nuclear antigen oligonucleotides inhibit intimal hyperplasia in a rat carotid artery injury model.*  
**J Clin Invest.** 1994;93:2351-2356.
- 
- Soonpaa MH, Koh GY, Klug MG, Field LJ.** *Formation of nascent intercalated disks between grafted fetal cardiomyocytes and host myocardium.*  
**Science.** 1994;264:98-101.
- 
- Stevenson SC, Marshall-Neff J, Teng B, Lee CB, Roy S, McClelland A.** *Phenotypic correction of hypercholesterolemia in apo E-deficient mice by adenovirus-mediated in vivo gene transfer.*  
**Arterioscler Thromb Vasc Biol.** 1995;15:479-484.
- 
- Stribling R, Brunette E, Liggitt D, Gaensler K, Debs R.** *Aerosol gene delivery in vivo.*  
**Proc Natl Acad Sci USA.** 1992;89:11277-11281.
-



- 
- Sullenger BA, Gallardo HF, Ungers GE, Gilboa E.** *Overexpression of TAR sequences renders cells resistant to human immunodeficiency virus replication.*  
**Cell.** 1990;63:601-608.
- 
- Takehita S, Bauters C, Asahara T, et al.** *Physiologic assessment of angiogenesis by arterial gene therapy with vascular endothelial growth factor.*  
**Circulation.** 1994;90:1-90. Abstract.
- 
- Takehita S, Zheng LP, Asahara T, et al.** *In vivo evidence of enhanced angiogenesis following direct arterial gene transfer of the plasmid encoding vascular endothelial growth factor.*  
**Circulation.** 1993;88:1-476. Abstract.
- 
- Thompson L.** *Gene therapy. Monkey tests spark safety review.*  
**Science.** 1992;257:1854.
- 
- Tomita N, Higaki J, Kaneda Y, et al.** *Hypertensive rats produced by in vivo introduction of the human renin gene.*  
**Circ Res.** 1993;73:898-905.
- 
- Tomita N, Higaki J, Morishita R, et al.** *Direct in vivo gene introduction into rat kidney.*  
**Biochem Biophys Res Commun.** 1992;186:129-134.
- 
- Vane JR, Änggård EE, Botting RM.** *Regulatory functions of the endothelium.*  
**N Engl J Med.** 1990;323:27-36.
- 
- Varmus H.** *Retroviruses.*  
**Science.** 1988;240:1427-1435.
- 
- von der Leyen HE, Gibbons GH, Morishita R, et al.** *Gene therapy inhibiting neointimal vascular lesion: in vivo gene transfer of endothelial-cell nitric oxide synthase gene.*  
**Proc Natl Acad Sci USA.** 1995;92:1137-1141.
- 
- von Harsdorf R, Schott RJ, Shen YT, Vatner SF, Mahdavi V, Nadal-Ginard B.** *Gene injection into canine myocardium as a useful model for studying gene expression in the heart of large animals.*  
**Circ Res.** 1993;72:688-695.
- 
- Wagner E, Zatloukal K, Cotten M, et al.** *Coupling of adenovirus to transferrin-polylysine/DNA complexes greatly enhances receptor-mediated gene delivery and expression of transfected genes.*  
**Proc Natl Acad Sci USA.** 1992;89:6099-6103.
- 
- Williams RS, Johnston SA, Riedy M, Devit MJ, McElligott SG, Sanford JC.** *Introduction of foreign genes in top tissues of living mice by DNA-coated microprojectiles.*  
**Proc Natl Acad Sci USA.** 1991;88:2726-2730.
- 
- Wilson JM.** *Gene therapy for cystic fibrosis: challenges and future directions.*  
**J Clin Invest.** 1995;96:2547-2554.
- 
- Wilson JM, Birinyi LK, Salomon RN, Libby P, Callow AD, Mulligan RC.** *Implantation of vascular grafts lined with genetically modified endothelial cells.*  
**Science.** 1989;244:1344-1346.
- 
- Wilson JM, Grossman M, Cabrera JA, Wu CH, Wu GY.** *A novel mechanism for achieving transgene persistence in vivo after somatic gene transfer into hepatocytes.*  
**J Biol Chem.** 1992;267:11483-11489.
-

**Bibliography of One Hundred Key Papers**

**Wu GY, Wilson JM, Shalaby F, Grossman M, Shafritz DA, Wu CH.**

*Receptor-mediated gene delivery in vivo. Partial correction of genetic analbuminemia in Nagase rats.*

**J Biol Chem.** 1991;266:14338-14342.

---

**Yao SN, Kurachi K.**

*Expression of human factor IX in mice after injection of genetically modified myoblasts.*

**Proc Natl Acad Sci USA.** 1992;89:3357-3361.

---

**Yao SN, Smith KJ, Kurachi K.**

*Primary myoblast-mediated gene transfer: persistent expression of human factor IX in mice.*

**Gene Ther.** 1994;1:99-107.

---

**Zoldhelyi P, McNatt J, Xu X, et al.**

*Prevention of arterial thrombosis by adenovirus-mediated transfer of cyclooxygenase gene.*

**Circulation.** 1996;93:10-17.

---