Valder R Arruda

List of Publications by Year
in descending order

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109 papers	11,700 citations	76294 40 h-index	60583 81 g-index
109 all docs	109 docs citations	109 times ranked	6510 citing authors

#	Article	IF	Citations
1	Safety and Efficacy of Gene Transfer for Leber's Congenital Amaurosis. New England Journal of Medicine, 2008, 358, 2240-2248.	13.9	1,941
2	Successful transduction of liver in hemophilia by AAV-Factor IX and limitations imposed by the host immune response. Nature Medicine, 2006, 12, 342-347.	15.2	1,865
3	Evidence for gene transfer and expression of factor IX in haemophilia B patients treated with an AAV vector. Nature Genetics, 2000, 24, 257-261.	9.4	971
4	AAV-mediated factor IX gene transfer to skeletal muscle in patients with severe hemophilia B. Blood, 2003, 101, 2963-2972.	0.6	707
5	Hemophilia B Gene Therapy with a High-Specific-Activity Factor IX Variant. New England Journal of Medicine, 2017, 377, 2215-2227.	13.9	549
6	Induction of immune tolerance to coagulation factor IX antigen by in vivo hepatic gene transfer. Journal of Clinical Investigation, 2003, 111, 1347-1356.	3.9	363
7	Sustained phenotypic correction of hemophilia B dogs with a factor IX null mutation by liver-directed gene therapy. Blood, 2002, 99, 2670-2676.	0.6	333
8	X-Linked Thrombophilia with a Mutant Factor IX (Factor IX Padua). New England Journal of Medicine, 2009, 361, 1671-1675.	13.9	298
9	The Mutation Ala677â†'Val in the Methylene Tetrahydrofolate Reductase Gene: A Risk Factor for Arterial Disease and Venous Thrombosis. Thrombosis and Haemostasis, 1997, 77, 0818-0821.	1.8	265
10	Long-term correction of inhibitor-prone hemophilia B dogs treated with liver-directed AAV2-mediated factor IX gene therapy. Blood, 2009, 113, 797-806.	0.6	247
11	Induction of immune tolerance to coagulation factor IX antigen by in vivo hepatic gene transfer. Journal of Clinical Investigation, 2003, 111, 1347-1356.	3.9	242
12	Modulation of tolerance to the transgene product in a nonhuman primate model of AAV-mediated gene transfer to liver. Blood, 2007, 110, 2334-2341.	0.6	218
13	Evidence of Multiyear Factor IX Expression by AAV-Mediated Gene Transfer to Skeletal Muscle in an Individual with Severe Hemophilia B. Molecular Therapy, 2006, 14, 452-455.	3.7	196
14	Assessing the potential for AAV vector genotoxicity in a murine model. Blood, 2011, 117, 3311-3319.	0.6	196
15	Muscle-Directed Gene Transfer and Transient Immune Suppression Result in Sustained Partial Correction of Canine Hemophilia B Caused by a Null Mutation. Molecular Therapy, 2001, 4, 192-200.	3.7	186
16	Lack of Germline Transmission of Vector Sequences Following Systemic Administration of Recombinant AAV-2 Vector in Males. Molecular Therapy, 2001, 4, 586-592.	3.7	152
17	Influence of Vector Dose on Factor IX-Specific T and B Cell Responses in Muscle-Directed Gene Therapy. Human Gene Therapy, 2002, 13, 1281-1291.	1.4	149
18	Prevalence of the Prothrombin Gene Variant (nt20210A) in Venous Thrombosis and Arterial Disease. Thrombosis and Haemostasis, 1997, 78, 1430-1433.	1.8	148

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19	Safety and efficacy of factor IX gene transfer to skeletal muscle in murine and canine hemophilia B models by adeno-associated viral vector serotype 1. Blood, 2004, 103, 85-92.	0.6	147
20	Regional intravascular delivery of AAV-2-F.IX to skeletal muscle achieves long-term correction of hemophilia B in a large animal model. Blood, 2005, 105, 3458-3464.	0.6	144
21	Eradication of neutralizing antibodies to factor VIII in canine hemophilia A after liver gene therapy. Blood, 2010, 116, 5842-5848.	0.6	144
22	AAV liver expression of FIX-Padua prevents and eradicates FIX inhibitor without increasing thrombogenicity in hemophilia B dogs and mice. Blood, 2015, 125, 1553-1561.	0.6	143
23	Risk and Prevention of Anti-factor IX Formation in AAV-Mediated Gene Transfer in the Context of a Large Deletion of F9. Molecular Therapy, 2001, 4, 201-210.	3.7	133
24	Posttranslational modifications of recombinant myotube-synthesized human factor IX. Blood, 2001, 97, 130-138.	0.6	123
25	Efficacy and Safety of Long-term Prophylaxis in Severe Hemophilia A Dogs Following Liver Gene Therapy Using AAV Vectors. Molecular Therapy, 2011, 19, 442-449.	3.7	116
26	Inherited Thrombophilia as a Risk Factor for the Development of Ischemic Stroke in Young Adults. Thrombosis and Haemostasis, 2000, 83, 229-233.	1.8	108
27	Peripheral transvenular delivery of adeno-associated viral vectors to skeletal muscle as a novel therapy for hemophilia B. Blood, 2010, 115, 4678-4688.	0.6	104
28	The efficacy and the risk of immunogenicity of FIX Padua (R338L) in hemophilia B dogs treated by AAV muscle gene therapy. Blood, 2012, 120, 4521-4523.	0.6	100
29	Novel approaches to hemophilia therapy: successes and challenges. Blood, 2017, 130, 2251-2256.	0.6	95
30	Safety and Efficacy of Regional Intravenous (RI) Versus Intramuscular (IM) Delivery of rAAV1 and rAAV8 to Nonhuman Primate Skeletal Muscle. Molecular Therapy, 2008, 16, 1291-1299.	3.7	89
31	Prevalence of the mutation C677 ? T in the methylene tetrahydrofolate reductase gene among distinct ethnic groups in Brazil. , 1998, 78, 332-335.		79
32	Gene therapy for hemophilia: what does the future hold?. Therapeutic Advances in Hematology, 2018, 9, 273-293.	1.1	79
33	Factor IX variants improve gene therapy efficacy for hemophilia B. Blood, 2005, 105, 2316-2323.	0.6	71
34	Minimal modification in the factor VIII B-domain sequence ameliorates the murine hemophilia A phenotype. Blood, 2013, 121, 4396-4403.	0.6	70
35	Safety of AAV Factor IX Peripheral Transvenular Gene Delivery to Muscle in Hemophilia B Dogs. Molecular Therapy, 2010, 18, 1318-1329.	3.7	66
36	Factor V Leiden (FVQ 506) is common in a Brazilian population. American Journal of Hematology, 1995, 49, 242-243.	2.0	64

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37	Inadvertent Germline Transmission of AAV2 Vector: Findings in a Rabbit Model Correlate with Those in a Human Clinical Trial. Molecular Therapy, 2006, 13, 1064-1073.	3.7	62
38	Strategies to Modulate Immune Responses: A New Frontier for Gene Therapy. Molecular Therapy, 2009, 17, 1492-1503.	3.7	56
39	Recombinant canine B-domainâ€“deleted FVIII exhibits high specific activity and is safe in the canine hemophilia A model. Blood, 2009, 114, 4562-4565.	0.6	55
40	In vivo efficacy of platelet-delivered, high specific activity factor VIII variants. Blood, 2010, 116, 6114-6122.	0.6	54
41	A Novel Platform for Immune Tolerance Induction in Hemophilia A Mice. Molecular Therapy, 2017, 25, 1815-1830.	3.7	52
42	Protein-Engineered Coagulation Factors for Hemophilia Gene Therapy. Molecular Therapy - Methods and Clinical Development, 2019, 12, 184-201.	1.8	39
43	Timing of Intensive Immunosuppression Impacts Risk of Transgene Antibodies after AAV Gene Therapy in Nonhuman Primates. Molecular Therapy - Methods and Clinical Development, 2020, 17, 1129-1138.	1.8	34
44	Emerging therapies for hemophilia: controversies and unanswered questions. F1000Research, 2018, 7, 489.	0.8	29
45	Omental implantation of BOECs in hemophilia dogs results in circulating FVIII antigen and a complex immune response. Blood, 2014, 123, 4045-4053.	0.6	28
46	Obstacles and future of gene therapy for hemophilia. Expert Opinion on Orphan Drugs, 2015, 3, 997-1010.	0.5	28
47	Circumventing furin enhances factor VIII biological activity and ameliorates bleeding phenotypes in hemophilia models. JCI Insight, 2016, 1, e89371.	2.3	28
48	Safety of Liver Gene Transfer Following Peripheral Intravascular Delivery of Adeno-Associated Virus (AAV)-5 and AAV-6 in a Large Animal Model. Human Gene Therapy, 2011, 22, 843-852.	1.4	25
49	Successful Phenotype Improvement following Gene Therapy for Severe Hemophilia A in Privately Owned Dogs. PLoS ONE, 2016, 11, e0151800.	1.1	25
50	Association of severe haemophilia A and factor V Leiden: report of three cases. Haemophilia, 1996, 2, 51-53.	1.0	24
51	Hyperactivity of factor IX Padua (R338L) depends on factor VIIIa cofactor activity. JCI Insight, 2019, 4, .	2.3	24
52	Gene therapy for hemophilia: Progress to date and challenges moving forward. Transfusion and Apheresis Science, 2019, 58, 602-612.	0.5	23
53	Translational Potential of Immune Tolerance Induction by AAV Liver-Directed Factor VIII Gene Therapy for Hemophilia A. Frontiers in Immunology, 2020, 11, 618.	2.2	22
54	Complete correction of hemophilia B phenotype by FIX-Padua skeletal muscle gene therapy in an inhibitor-prone dog model. Blood Advances, 2018, 2, 505-508.	2.5	21

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55	Guest Editor: G. Castaman GENE THERAPY FOR HEMOPHILIA: FACTS AND QUANDARIES IN THE 21ST CENTURY. Mediterranean Journal of Hematology and Infectious Diseases, 2020, 12, e2020069.	0.5	18
56	Factor IX assay discrepancies in the setting of liver gene therapy using a hyperfunctional variant factor IXâ€Padua. Journal of Thrombosis and Haemostasis, 2021, 19, 1212-1218.	1.9	17
57	Update on gene therapy for hereditary hematological disorders. Expert Review of Cardiovascular Therapy, 2003, 1, 215-232.	0.6	16
58	The search for the origin of factor VIII synthesis and its impact on therapeutic strategies for hemophilia A. Haematologica, 2015, 100, 849-850.	1.7	16
59	Safety of Recombinant Adeno-Associated Viral Vectors in a Large Animal Model.. Blood, 2007, 110, 2586-2586.	0.6	13
60	Evolutionary insights into coagulation factor IX Padua and other high-specific-activity variants. Blood Advances, 2021, 5, 1324-1332.	2.5	12
61	Gene Therapy for Inherited Bleeding Disorders. Seminars in Thrombosis and Hemostasis, 2021, 47, 161-173.	1.5	11
62	B cellâ€“activating factor modulates the factor VIII immune response in hemophilia A. Journal of Clinical Investigation, 2021, 131, .	3.9	10
63	Genetic variability of platelet glycoprotein IbÎ± gene. American Journal of Hematology, 2004, 77, 107-116.	2.0	6
64	Inhibitorsâ€”Recent insights. Haemophilia, 2021, 27, 28-36.	1.0	6
65	Padua FIXa resistance to Protein S and a potential therapy for hyperactive FIXa. Thrombosis Research, 2018, 170, 133-141.	0.8	5
66	Factor VIII and IX Genes Polymorphisms in a Brazilian Black Population. Thrombosis and Haemostasis, 1993, 70, 371-371.	1.8	5
67	A Novel Method of Regional Intravenous Delivery of AAV Vector to Skeletal Muscle Results in Correction of Canine Hemophilia B Phenotype.. Blood, 2004, 104, 3179-3179.	0.6	5
68	The Role of Immunosuppression in Gene- and Cell-Based Treatments for Duchenne Muscular Dystrophy. Molecular Therapy, 2007, 15, 1040-1041.	3.7	4
69	Induction of Immune Tolerance to Canine FVIII in Hemophilia a Dogs with Inhibitors Using AAV-Mediated Expression of Canine FVIII. Blood, 2008, 112, 243-243.	0.6	4
70	Understanding Ectopically Expressed Factor VIII (F8) In Megakaryocytes: Implications for Optimum Platelet-Delivered F8 Activity for Gene Therapy. Blood, 2010, 116, 2205-2205.	0.6	4
71	Zymogen-Like Factor Xa Variants Restore Thrombin Generation and Effectively Bypass the Intrinsic Pathway in Vitro. Blood, 2008, 112, 240-240.	0.6	3
72	FIX-R338L (FIX Padua) as a Successful Alternative for the Treatment of Canine Severe Hemophilia B.. Blood, 2009, 114, 694-694.	0.6	2

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73	Rabbit Anti-Thymocyte Globulin (rATG) Administrated Concomitantly with Liver Delivery of AAV2-hFIX Can Promote Inhibitor Formation In Rhesus Macaques.. Blood, 2010, 116, 3765-3765.	0.6	2
74	Why is AAV FVIII gene therapy not approved by the US Food and Drug Administration yet?. Blood Advances, 2021, 5, 4313-4313.	2.5	2
75	Gene therapy matures to medicines. Human Molecular Genetics, 2019, 28, R1-R2.	1.4	1
76	A Novel Role of Coagulation Proteases on Viral-Based Gene Transfer Efficacy.. Blood, 2004, 104, 691-691.	0.6	1
77	Insights Into the Mechanism of Zymogen Protein C Protection Against Cancer Progression. Blood, 2012, 120, 3350-3350.	0.6	1
78	Unexpected Role of PACE/Furin Cleavage Site in FVIII Biology: Implications for Hemophilia a Therapy. Blood, 2014, 124, 105-105.	0.6	1
79	Protease-Activated Receptor-2 (PAR-2) as a Novel Target for Modulating Immune Responses to Neo Antigens Following In Vivo Gene Transfer.. Blood, 2005, 106, 1296-1296.	0.6	1
80	Proteaseâ^'Activated Receptor 2 (PARâ^'2) as a Novel Target To Prevent Inhibitor Formation to FIX.. Blood, 2006, 108, 763-763.	0.6	1
81	Muscle Gene Therapy for Hemophilia. Journal of Genetic Syndromes & Gene Therapy, 2013, S1, .	0.2	1
82	Complete Correction of Severe Canine Hemophilia B By Skeletal Muscle Directed AAV-Based FIX-Padua Gene Therapy in Inhibitor-Prone Dogs. Blood, 2015, 126, 3487-3487.	0.6	1
83	Factor IX Padua: From Biochemistry to Gene Therapy. Blood, 2016, 128, SCI-9-SCI-9.	0.6	1
84	In Vivo Evidence of Modulation of the Hemophilia Phenotype by the Factor V Leiden.. Blood, 2004, 104, 693-693.	0.6	0
85	Co-Inheritance of FV Leiden and High Levels of FIX Results in Novel Models for Thrombophilia and Spontaneous Fetal Loss.. Blood, 2005, 106, 1946-1946.	0.6	0
86	Characterization of the Immune Response to Canine Factor IX Following AAV-Mediated Intravascular Gene Delivery to Skeletal Muscle in Hemophilia B Dogs.. Blood, 2005, 106, 1297-1297.	0.6	0
87	Long Term Dose-Dependent Correction of Hemophilia A Dogs Using AAV-8 and AAV-9-Mediated FVIII Gene Transfer.. Blood, 2006, 108, 999-999.	0.6	0
88	An Essential Role of the Factor VIII Light Chain in Facilitating Heavy Chain Secretion.. Blood, 2006, 108, 4034-4034.	0.6	0
89	Heterogeneous In Vivo Role of Clotting Factors FVIII and FIX in Atherogenesis. Blood, 2007, 110, 3730-3730.	0.6	0
90	Successful Production of Canine FVIII: Biochemical and Functional Characterization in Hemophilia A Dogs.. Blood, 2007, 110, 495-495.	0.6	0

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91	Factor Xa Variants as Novel Bypass Agents for the Treatment of Hemophilia in Murine Models. Blood, 2008, 112, 239-239.	0.6	0
92	Successful Long Term Therapeutic Expression of Factor VIII in Hemophilia A Dogs After Administration of AAV-cFVIII Using a Two-Chain or Single Chain Delivery Approach.. Blood, 2009, 114, 546-546.	0.6	0
93	Assessment of Insertional Mutagenesis Risk Following AAV Vector-Mediated Factor IX Gene Transfer in Mice.. Blood, 2009, 114, 2465-2465.	0.6	0
94	The Development of Novel Hemostatic Bypassing Molecules.. Blood, 2009, 114, SCI-20-SCI-20.	0.6	0
95	Naturally-Occurring Antibodies to Human AAV In Sheep: A New Large Animal Model for Immune Aspects of AAV Gene Transfer.. Blood, 2010, 116, 3762-3762.	0.6	0
96	Zymogen Protein C as a Novel Modulator of Cancer Progression In Murine Models. Blood, 2010, 116, 718-718.	0.6	0
97	Protease-Activated Receptor 4 (PAR4) Plays a Critical Role in Fetal Loss and Peripheral Thrombosis in a Mouse Model of Thrombophilia. Blood, 2011, 118, 373-373.	0.6	0
98	Hemophilia Gene Therapy. Blood, 2011, 118, SCI-48-SCI-48.	0.6	0
99	The Role of Activated Protein C in Cancer. Blood, 2011, 118, SCI-18-SCI-18.	0.6	0
100	Bioengineering Factor VIII B-Domain Sequences Improves Function and Efficacy in Hemophilia A Models.. Blood, 2012, 120, 2208-2208.	0.6	0
101	Delivery of a Modified U1 Small Nuclear RNA Alleviates Splicing-Defective Coagulation Factor VII Expression in Mouse Models. Blood, 2012, 120, 754-754.	0.6	0
102	Platelet Factor VIII-Induced Megakaryocyte Apoptosis: Implications for Hemophilia A Gene Therapy. Blood, 2012, 120, 2051-2051.	0.6	0
103	Tolerance Induction To FIX Padua With AAV Liver Gene Transfer In Inhibitor-Prone Hemophilia B Dogs. Blood, 2013, 122, 4203-4203.	0.6	0
104	VKORc1 Is Under-Expressed in Skeletal Muscle of Humans, Dogs and Mice: Potential Implications for Ectopic Coagulation Factor Expression in Pre-Clinical and Therapeutic Applications. Blood, 2014, 124, 1477-1477.	0.6	0
105	Towards the Care of Hemophilia a Patients Using Induced Pluripotent Stem Cell (iPSC)-Derived Megakaryocytes (iMks) Expressing Coagulation Factor (F) VIII. Blood, 2015, 126, 2266-2266.	0.6	0
106	Infusion of Coagulation Factor VIII-Containing Induced Pluripotent Stem Cell (iPSC)-Derived Megakaryocytes (iMks) Shows Potential As Hemophilia a Treatment. Blood, 2016, 128, 2559-2559.	0.6	0
107	Stopping bleeding is not enough to FIX hemarthropathy. Blood, 2017, 129, 2048-2049.	0.6	0
108	The Complete Dependence of Factor IX Padua (R338L) Hyperactivity on Factor VIIIa Cofactor Activity Supports Its Safety As a Transgene for Hemophilia B Gene Therapy. Blood, 2018, 132, 3486-3486.	0.6	0

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109	Extra-Splenic Role of B Cell Activating Factor Blockade in Prevention of Factor VIII Inhibitors. Blood, 2021, 138, 1025-1025.	0.6	0