

Juan A. Bueren

Brief statement of support for my nomination:

- Organizer of one of the 2013 ESGCT Meeting, in collaboration with the Spanish Society for Gene and Cell Therapy
- Coordinator of a European FP7 Grant (EUROFANCOLEN)
- Active Team Leader in the field of hematopoietic stem cells (123 publications in the field)

Why I would like to join the ESGCT Board:

I consider that Europe is currently leading the field of Gene Therapy at the international level. Additionally, the ESGCT is promoting the collaboration between several European teams, and this has been particularly useful for our team. I would be interested in further promoting such type of interaction among other qualified teams in the context of the ESGCT. Also I would like to help to the ESGCT Board based on our experience with the organization of an ESGCT Meeting.

What skills and expertise I would bring to ESGCT:

My participation in the organization of the collaborative Meeting of the ESGCT meeting and the Spanish Society for Gene and Cell Therapy (SETGyC) in Madrid (October 2013); together with my duty as President of the SETGyC, that expired in 2013, allowed me to get experience in the organization of tasks that may promote the collaboration between European teams interested in the field of Gene and Cell Therapy.

Outline of any areas of work/ideas I would like to introduce to the work of the ESGCT.

- Further improvements in the development of tasks to be conducted in the different Sections/Committees of the ESGCT
- Collaboration to further improve the webpage of the ESGCT Webpage and ESGCT Congresses
- Development of additional activities for ESGCT PhD students
- Promotion of basic and translational studies to be conducted by ESGCT Partners in the field of Genetic Instability Syndromes

CURRICULUM VITAE: Juan Antonio Bueren

POSITION: Head of the Hematopoietic Innovative Therapies Division. CIEMAT and Biomedical Network Centre for research on rare Diseases (CIBERER). Av. Complutense, 40. 28040 Madrid. SPAIN. Ph.: +34-91-346 651; Fax.: 34-91-346 6484 ; E-mail: juan.bueren@ciemat.es

EDUCATION:

MASTER DEGREE	CENTER	DATE
Pharmacy	Complutense University. Madrid	1979
PhD		
Pharmacology	Complutense University. Madrid	1982

ACADEMIC POSITIONS:

Institution	Degree	Year
Junta de Energía Nuclear	PhD student	1980-82
CIEMAT	PhD	1982-92
CIEMAT	Senior Scientist. Cell Biology Unit	1992-2002
CIEMAT	Head of the Hematopoiesis Division	2002-present
CIBERER	Team leader	2008-present
Fundación Jimenez Díaz	Head of the Advanced Therapies Unit	2014-present

HONORS AND AWARDS

- Award of Appreciation: Honoring Initiative in Fanconi Anemia Research and Family Support. Fanconi Anemia Research Foundation (FARF). 2004
- Corresponding member of the Academy of Pharmacy of Galicia. 2006
- Corresponding member of the Royal national Academy of Pharmacy. 2008
- Distinguished Service Award. Fanconi Anemia Research Foundation (FARF). 2011.
- Award of Appreciation: Honoring of discovery of FANCC. Fanconi Anemia Research Foundation (FARF). 2014

RESEARCH INTEREST:

- Development of advanced therapies for the treatment of hereditary and acquired diseases based on genetic manipulation and stem cell transplantation.
- Improving the efficacy and the safety of cell reprogramming for the generation and expansion of hematopoietic stem cells through the generation of induced pluripotent stem cells (iPS cells)
- Use of mesenchymal stem cells for the treatment of severe inflammatory diseases, including graft versus host disease and autoimmune diseases.

- Development of assays for the hematotoxic screening of new drugs with anticancer and antiviral activity.

PARTICIPATION IN INTERNATIONAL PROJECTS (Last 10 years):

European Commission

Novel Approaches in the management of the radiation injury.

FIGH-CT-2001-157

2002-2004

Coordinator: Juan A. Bueren

European Commission

Concertad Safety and Efficiency Evaluation of Retroviral transgenesis in Gene Therapy of Inherited Diseases "Programme of Life Sciences, Genomics and Biotechnology for Health"

LSHB-CT-2004-5242

2004-2008

Coordinator : G. Wagemaker. Erasmus Univ.

European Commission

Optimization and prevalidation of an in vitro test strategy for predicting acute toxicity

LSHB-CT-2004-512051

2005-2008

European Commission

Persisting Transgenesis: PERSIST

Grant Agreement Number: 222878

Associated Contractor

2009-2012

Coordinator: Luigi Naldini

European Commission

Phase I/II Gene therapy trial of Fanconi anemia patients with a new Orphan Drug consisting of a lentiviral vector carrying the FANCA gene: A Coordinated International Action

2012-2017

Coordinator: Juan A. Bueren

INTERNATIONAL JOURNALS WITH IMPACT FACTOR (Last 10 years; 71 out of 121)

1. Pipaon, C., Casado, J. A., Bueren, J. A., Fernández-Luna, J. L. *Jun-N-terminal kinase activity and early growth-response factor-1 gene expresión are downregulated in Fanconi anemia group A lymphoblasts. Blood. 103:128-132 (2004).*
2. Lamana, M. L., Bueren, J. A., Vicario, J. L., Balas, A. *Functional and Phenotypic Variations in Human T Cells Subjected to Retroviral-Mediated Gene Transfer. Gene Therapy. 11:474-482 (2004).*
3. Callen E, Tischkowitz M, Creus A, Marcos R., Bueren JA, Casado JA, Mathew C and Surrallés J *Quantitative PCR analysis reveals a high incidence of large intragenic deletions in the FANCA gene in Spanish Fanconi anemia patients. Cytogenet and Genome Research 104:341-345 (2004).*

4. Bueren, J. A., Guenechea, G, Casado, J. A., Lamana, M. L., Segovia, J. C. *Genetic Modification of Hematopoietic Stem Cells: Recent Advances in the Gene Therapy of Inherited Diseases*. Review. **Arch. Med. Res.** **34:589-599 (2004)**.
5. Almarza, E., Segovia, J. C., Guenechea, G., García, S., Ramírez, A., Bueren, J. A. *Regulatory Elements of the vav Gene Drive Transgene Expresión in Hematopoietic Stem Cells from Adult Mice*. **Exp. Hematol.** **32:360-364 (2004)**.
6. Grande, T. Bueren, J. A. *A New Approach to Evaluate the Total Reserve of Hematopoietic Progenitors After Acute Irradiation*. **Radiat. Res** **162:397-404 (2004)**.
7. Rio P, Martínez-Palacio J, Ramírez A, Bueren JA and Segovia JC. *Efficient Engraftment of In-Utero Transplanted Mice with Retrovirally Transduced Hematopoietic Stem Cells*. **Gene Ther.** **2005. 12:358-63**.
8. Callén E, Casado JA, Bueren JA, Creus A, Marcos R, Dasí A, Estella JM, Muñoz A, Ortega JJ, Joenje H, de Winter JP, Schindler D, Hanenberg H, Tischkowitz MD, Mathew CG, and Surrallés J. *A common founder mutation in FANCA underlies the world highest prevalence of Fanconi anemia in gipsy families from Spain*. **Blood.** **105:1946-1949 (2005)**
9. Ramirez M, Regidor C, Marugan I, Garcia-Conde J, Bueren JA, Fernandez MN. *Engraftment kinetics of human CD34+ cells from cord blood and mobilized peripheral blood co-transplanted into NOD/SCID mice*. **Bone Marrow Transplant.** **35:271-5 (2005)**
10. Garcia-Castro J, Martinez-Palacio J, Lillo R, Garcia-Sanchez F, Alemany R, Madero L, Bueren JA, Ramirez M. *Tumor cells as cellular vehicles to deliver gene therapies to metastatic tumors*. **Cancer Gene Ther.** **12:341-9. (2005)**
11. Casado JA, Nuñez MI, Segovia JC, Ruiz de Almodóvar JM and Bueren JA. *Non-Homologous End Joining Defect in Fanconi Anemia Hematopoietic Cells Exposed to Ionizing Radiation*. **Radiat Res.** **164: 635-41 (2005)**
12. Quintana-Bustamante, A. Álvarez-Barrientos, A.V. Kofman, J.A. Bueren, N.D. Theise, J.C. Segovia *Mobilization of hematopoietic progenitors increases the presence of bone marrow derived hepatocytes through in vivo fusion events in a murine model of CCl4-induced liver damage*. **Hepatology** **43:108-16 (2006)**.
13. Jacome A, Navarro S, Casado JA, Rio P, Madero L, Estella J, Sevilla J, Badell, Ortega JJ, Olivé T, Hanenberg H, Segovia JC, Bueren JA. *A Simplified Approach to Improve the Efficiency and Safety of Ex Vivo Hematopoietic Gene Therapy in Fanconi Anemia Patients*. **Human Gene Therapy** **17:245-50 (2006)**
14. [Grande T, Bueren JA](#). *The mobilization of hematopoietic progenitors to peripheral blood is predictive of the hematopoietic syndrome after total or partial body irradiation of mice*. **Int J Radiat Oncol Biol Phys.** **1:612-8 (2006)**.
15. Navarro S, Meza NW, Quintana-Bustamante O, Casado JA, McAllister K, Puerto S, Surrallés J, Segovia JC and Bueren JA. *Hematopoietic dysfunction in a mouse model for fanconi anemia group D1*. **Molecular Therapy.** **14:525-35 (2006)**.
16. [Meza NW, Puyet A, Perez-Benavente S, Quintana-Bustamante O, Diez A, Bueren JA, Segovia JC, Bautista JM](#). *Functional analysis of gammaretroviral vector transduction by quantitative PCR*. **J Gene Med.** **8:1097-104 (2006)**.

17. Rosa Yañez, María Luisa Lamana, Javier García-Castro, Isabel Colmenero, Manuel Ramírez and Juan A. Bueren. *Adipose Tissue-Derived Mesenchymal Stem Cells (Ad-Mscs) have in vivo Immunosuppressive Properties Applicable for the Control of the Graft-Versus-Host Disease (GVHD)*. **Stem Cells: 24: 2582-2591, (2006)**.
18. Laufs S, G. Guenechea., Gonzalez-Murillo A, Nagy KZ. Lozano L, Hotz-Wagenblatt A, Jens Zeller W, Bueren JA, Fruehauf S. *Lentiviral vector integration sites in human NOD/SCID repopulating cell*. **J. Gene Med. 8:1197-207, (2006)**.
19. [Casado JA](#), [Callén E](#), [Jacome A](#), [Río P](#), [Castella M](#), [Lobitz S](#), [Ferro T](#), [Muñoz A](#), [Sevilla J](#), [Cantalejo A](#), [Cela E](#), [Cervera J](#), [Sánchez-Calero J](#), [Badell I](#), [Estella J](#), [Dasí A](#), [Olivé T](#), [José Ortega J](#), [Rodriguez-Villa A](#), [Tapia M](#), [Molinés A](#), [Madero L](#), [Segovia JC](#), [Neveling K](#), [Kalb R](#), [Schindler D](#), [Hanenberg H](#), [Surrallés J](#), [Bueren JA](#). *A comprehensive strategy for the subtyping of patients with Fanconi anaemia: conclusions from the Spanish Fanconi Anemia Research Network*. **J. Med. Genetics. 44: 241-249, (2007)**
20. [Kalb R](#), [Neveling K](#), [Hoehn H](#), [Schneider H](#), [Linka Y](#), [Batish SD](#), [Hunt C](#), [Berwick M](#), [Callen E](#), [Surralles J](#), [Casado JA](#), [Bueren J](#), [Dasi A](#), [Soulier J](#), [Gluckman E](#), [Zwaan CM](#), [van Spaendonk R](#), [Pals G](#), [de Winter JP](#), [Joenje H](#), [Grompe M](#), [Auerbach AD](#), [Hanenberg H](#), [Schindler D](#). *Hypomorphic Mutations in the Gene Encoding a Key Fanconi Anemia Protein, FANCD2, Sustain a Significant Group of FA-D2 Patients with Severe Phenotype*. **Am. J Hum Genet. 80:895-910 (2007)**.
21. [Almarza E](#), [Río P](#), [Meza NW](#), [Aldea M](#), [Agirre X](#), [Guenechea G](#), [Segovia JC](#), [Bueren JA](#). *Characteristics of lentiviral vectors harboring the proximal promoter of the vav proto-oncogene: a weak and efficient promoter for gene therapy*. **Molecular Therapy.15:1487-94 (2007)**
22. [Meza NW](#), [Quintana-Bustamante O](#), [Puyet A](#), [Rio P](#), [Navarro S](#), [Diez A](#), [Bueren JA](#), [Bautista JM](#), [Segovia JC](#). *In vitro and in vivo expression of human erythrocyte pyruvate kinase in erythroid cells: a gene therapy approach*. **Hum Gene Ther.18: 502-14 (2007)**
23. Río P and Bueren JA. *Fanconi Anemia Core Complex Moves to Chromatin*. Invited Inside Comment. *Blood*: 111: 4837-4838, (2008).
24. [Casado JA](#), [Río P](#), [Marco E](#), [García-Hernández V](#), [Domingo A](#), [Pérez L](#), [Tercero JC](#), [Vaquero JJ](#), [Albella B](#), [Gago F](#), [Bueren JA](#). *Relevance of the Fanconi anemia pathway in the response of human cells to trabectedin*. **Mol Cancer Ther. 7:1309-18 (2008)**.
25. [López-Bueno A](#), [Segovia JC](#), [Bueren JA](#), [O'Sullivan MG](#), [Wang F](#), [Tattersall P](#), [Almendral JM](#). *Evolution to pathogenicity of the parvovirus minute virus of mice in immunodeficient mice involves genetic heterogeneity at the capsid domain that determines tropism*. **J Virol.;82:1195-203 (2008)**.
26. [Gonzalez-Murillo A](#), [Lozano ML](#), [Montini E](#), [Bueren JA](#), [Guenechea G](#). *Unaltered repopulation properties of mouse hematopoietic stem cells transduced with lentiviral vectors*. **Blood. 112:3138-47 (2008)**.
27. Agudo J, Ayuso E, Jimenez V, Salavert A, Casellas A, Tafuro S, Haurigot V, Ruberte J, Segovia JC, Bueren JA, Bosch F. *IGF-I mediates regeneration of endocrine*

pancreas by increasing beta cell replication through cell cycle protein modulation in mice. **Diabetología. 51:1862-72, 2008.**

82. Río, P, Meza N, González-Murillo A, Navarro S, Álvarez L, Surrallés J, Castella M, Guenechea G, Segovia JC, Hanenberg H, Bueren JA. *In vivo proliferation advantage of genetically corrected hematopoietic stem cells in a mouse model of Fanconi anemia FA-D1.* **Blood. 112:4853-61 (2008).**

29. Jacome A, Navarro S, Río P, Yañez R, González-Murillo R, Lamana ML, Lozano ML, Sevilla J, Olivé. T, Díaz-Heredia C, Badell I, Estella J, Madero L, Casado JA, Güenechea G, Segovia JC, Bueren JA. *Lentiviral-mediated genetic correction of hematopoietic and mesenchymal stem cells from Fanconi anemia patients.* **Molecular Therapy. 17:1083-92 (2009).**

30. Ángel Raya, Ignasi Rodríguez-Pizà, Guillermo Guenechea, Rita Vassena, Susana Navarro, María José Barrero, Antonella Consiglio, Paula Río, Eduard Sleep, Gustavo Tiscornia, Elena Garreta, Trond Aasen, Anna Veiga, Inder M. Verma, Juan Bueren & Juan Carlos Izpisua Belmonte. *Generation of disease-free hematopoietic progenitors from Fanconi anemia-specific induced pluripotent stem cells.* **Nature. 460: 53-59 (2009).**

31. Haurigot V, Villacampa P, Ribera A, Llombart C, Bosch A, Nacher V, Ramos D, Ayuso E, Segovia JC, Bueren JA, Ruberte J, Bosch. *Increased intraocular insulin-like growth factor-I (IGF-I) triggers blood-retinal barrier breakdown.* **F.J. Biol Chem. 284:22961-9 (2009).**

32. Ibáñez A, Río P, Casado JA, Bueren JA, Fernández-Luna JL, Pipaón C. *Elevated levels of IL-1beta in Fanconi Anemia group A patients due to a constitutively active PI3K-AKT pathway are capable of promoting tumor cell proliferation.* **Biochem J. 422:161-70 (2009).**

33. Cerrato L, Valeri A, Bueren JA, Albella B. *In vitro sensitivity of granulomonocytic progenitors as a new toxicological cell system and endpoint in the ACuteTox Project.* **Toxicol Appl Pharmacol. 238:111-9 (2009)**

34. Meza NW, Alonso-Ferrero ME, Navarro S, Quintana-Bustamante O, Valeri A, Garcia-Gomez M, Bueren JA, Bautista JM, Segovia JC. *Rescue of pyruvate kinase deficiency in mice by gene therapy using the human isoenzyme.* **Molecular Therapy 17:2000-9 (2009).**

35. Ute Modlich, Susana Navarro, Daniela Zychlinski, Tobias Maetzig, Sabine Knoess, Martijn H. Brugman, Axel Schambach, Sabine Charrier, Anne Galy, Juan Bueren, Christopher Baum. *Insertional transformation of hematopoietic cells by self-inactivating lentiviral and gammaretroviral vectors.* **Molecular Therapy. 17:1919-28 (2009).**

36. [García-Bravo M](#), [Morán-Jiménez MJ](#), [Quintana-Bustamante O](#), [Méndez M](#), [Gutiérrez-Vera I](#), [Bueren J](#), [Salido E](#), [Segovia JC](#), [Fontanellas A](#), [de Salamanca RE](#). *Bone marrow-derived cells promote liver regeneration in mice with erythropoietic protoporphyria.* **Transplantation. 88:1332-40 (2009).**

37. Gonzalez-Murillo A, Lozano M, Alvarez L, Jacome A, Almarza E, Navarro S, Segovia JC, Hanenberg H, Guenechea G, Bueren J, Río P. *Development of lentiviral*

vectors with optimized transcriptional activity for the gene therapy of Fanconi anemia patients. **Hum Gene Ther** 21:623-30 (2010).

38. Villegas-Mendez A, Garin MI, Pineda-Molina E, Veratti E, Bueren JA, Fender P, Lenormand JL. *In Vivo Delivery of Antigens by Adenovirus Dodecahedron Induces Cellular and Humoral Immune Responses to Elicit Antitumor Immunity*. **Molecular Therapy**. 18:1046-53 (2010).

39. Raya A, Rodríguez-Pizà I, Navarro S, Richaud-Patin Y, Guenechea G, Sánchez-Danés A, Consiglio A, Bueren J, Izpisua Belmonte JC. *A protocol describing the genetic correction of somatic human cells and subsequent generation of iPS cells*. **Nature Protocols**. 5:647-60. (2010).

40. García-Gómez I, Elvira G, Zapata AG, Lamana ML, Ramírez M, Castro JG, Arranz MG, Vicente A, Bueren J, García-Olmo D. *Mesenchymal stem cells: biological properties and clinical applications*. **Expert Opin Biol Ther**.10:1453-68 (2010).

41. Yañez R, Oviedo A, Aldea M, Bueren JA, Lamana ML. *Prostaglandin E2 plays a key role in the immunosuppressive properties of adipose and bone marrow tissue-derived mesenchymal stromal cells*. **Exp Cell Res** 316:3109-23 (2010)

42. Valeri A, Alonso-Ferrero ME, Cerrato L, Martínez S, Bueren JA, Albella B. *Development of an in vitro model for the simultaneous study of the efficacy and hematotoxicity of antileukemic compounds*. **Toxicol Lett**. 199:317-22 (2010)

43. Alonso-Ferrero ME, Valeri A, Yañez R, Navarro S, Garin MI, Ramirez JC, Bueren JA, Segovia JC. *Immunoresponse against the transgene limits hematopoietic engraftment of mice transplanted in utero with virally transduced fetal liver*. **Gene Ther**. 2010 Dec 23. [Epub ahead of print]

44. Valeri A, Alonso-Ferrero ME, Río P, Pujol MR, Casado JA, Pérez L, Jacome A, Agirre X, Calasanz MJ, Hanenberg H, Surrallés J, Prosper F, Albella B, Bueren JA. *BCR/ABL interferes with the Fanconi anemia/BRCA pathway: implications in the chromosomal instability of chronic myeloid leukemia cells*. **PLoS One**. 2010 Dec 28;5(12):e15525.

45. Almarza E., Zhang F., Santilli G., Blundel M.P, Howe S.J., Thornhill S.I, Bueren J.A., and Thrasher A.J. *Correction of SCID-X1 using an enhancerless vav promoter*. **Human Gene Ther**. 19:122-32 (2011). Paper with an Editorial Commentary from C. Baum and Axel Schambag in the same Journal.

46. G. Santilli, E. Almarza, C. Brendel, C. Beilin, M. Blundell, S Haria, K. Parsley, C. Kinnon, J.A Bueren, M. Grez and A..J. Thrasher. *Biochemical correction of X-CGD by a novel chimeric promoter regulating high levels of transgene expression in myeloid cells*. **Mol Ther**. 19:122-132 (2011).

47. Mukherjee S, Santilli G, Blundell MP, Navarro S, Bueren JA, Thrasher AJ. *Generation of functional neutrophils from a mouse model of x-linked chronic granulomatous disorder using induced pluripotent stem cells*. **PLoS One**. 3; 6(3):e17565.2011

48. Castella M, Pujol R, Callén E, Ramírez MJ, Casado JA, Talavera M, Ferro T, Muñoz A, Sevilla J, Madero L, Cela E, Beléndez C, Díaz de Heredia C, Olivé T, Sánchez de Toledo J, Badell I, Estella J, Dasí A, Rodríguez-Villa A, Gómez P, Tapia

M, Molinés A, Figuera A, Bueren JA, Surrallés J. *Chromosome fragility in patients with Fanconi anaemia: diagnostic implications and clinical impact*. **J Med Genet.** **48:242-50 (2011)**

49. Castella M, Pujol R, Callén E, Trujillo JP, Casado JA, Gille H, Lach FP, Auerbach AD, Schindler D, Benitez J, Porto B, Ferro T, Muñoz A, Sevilla J, Madero L, Cela E, Beléndez C, Díaz de Heredia C, Olivé T, Sánchez de Toledo J, Badell I, Torrent M, Estella J, Dasí A, Rodríguez-Villa A, Gómez P, Barbot J, Tapia M, Molinés A, Figuera A, Bueren JA, Surrallés J. *Origin, functional role and clinical impact of Fanconi anemia FANCA mutations*. **Blood** **117:3759-69 (2011)**

50. Valeri A, Martínez S, Casado JA, Bueren JA. *Fanconi anaemia: from a monogenic disease to sporadic cancer*. **Clin Transl Oncol** **13:215-21 (2011)**

51. Martrat G, Maxwell CA, Tominaga E, Porta M, Bonifaci N, Gomez-Baldo L, Bogliolo M, Lazaro C, Blanco I, Brunet J, Aguilar H, Fernandez-Rodriguez J, Seal S, Renwick A, Rahman N, Kuhl J, Neveling K, Schindler D, Ramirez MJ, Castella M, Hernandez G, Embrace ES, Easton DF, Peock S, Cook M, Oliver CT, Frost D, Platte R, Evans DG, Lalloo F, Eeles R, Izatt L, Chu C, Davidson R, Ong KR, Cook J, Douglas F, Hodgson S, Brewer C, Morrison PJ, Porteous M, Peterlongo P, Manoukian S, Peissel B, Zaffaroni D, Roversi G, Barile M, Viel A, Pasini B, Ottini L, Putignano AL, Savarese A, Bernard L, Radice P, Healey S, Spurdle A, Chen X, Beesley J, Kconfab KC, Rookus MA, Verhoef S, Tilanus-Linthorst MA, Vreeswijk MP, Asperen CJ, Bodmer D, Ausems MG, van Os TA, Blok MJ, Meijers-Heijboer HE, Hogervorst FB, Hebon HB, Goldgar DE, Buys S, John EM, Miron A, Southey M, Daly MB, Bcfr BC, Swe-Brca SB, Harbst K, Borg A, Rantala J, Barbany-Bustanza G, Ehrencrona H, Stenmark-Askmal M, Kaufman B, Laitman Y, Milgrom R, Friedman E, Domchek SM, Nathanson KL, Rebbeck TR, Johannsson OT, Couch FJ, Wang X, Fredericksen Z, Cuadras D, Moreno V, Pientka FK, Depping R, Caldes T, Osorio A, Benitez J, Bueren J, Heikkinen T, Nevanlinna H, Hamann U, Torres D, Caligo MA, Godwin AK, Imyanitov EN, Janavicius R, Gemo GG, Sinilnikova OM, Stoppa-Lyonnet D, Mazoyer S, Verny-Pierre C, Castera L, de Pauw A, Bignon YJ, Uhrhammer N, Peyrat JP, Vennin P, Fert Ferrer S, Collonge-Rame MA, Mortemousque I, McGuffog L, Chenevix-Trench G, Pereira-Smith OM, Antoniou AC, Ceron J, Tominaga K, Surralles J, Pujana MA. *Exploring the link between MORF4L1 and risk of breast cancer*. **Breast Cancer Res.** **2011, 13:R40.**

52. Tolar J, Antoniou M, Bartholomä C, Blazar BR, Bueren J, Carroll T, Cavazzana-Calvo M, Dagleish R, Galy A, Gaspar HB, Hanenberg H, Von Kalle C, Kiem H-P, Naldini L, Navarro S, Renella R, Rio P, Sevilla J, Schmidt M, Verhoeyen E, Wagner JE, Williams DA, Thrasher A. *Stem Cell Gene Therapy for Fanconi Anemia: Report from the 1st International Fanconi Anemia Gene Therapy Working Group Meeting. Review*. **Molecular Therapy**, **19:1193-8 (2011)**.

53. Frecha C, Costa C, Nègre D, Amirache F, Trono D, Rio P, Bueren J, Cosset FL, Verhoeyen E. *A novel lentivector targets gene transfer into hHSC in marrow from patients with BM-failure-syndrome and in vivo in humanized mice*. **Blood.** **119:1139-50 (2011)**.

54. Tolar J, Becker PS, Clapp DW, Hanenberg H, de Heredia CD, Kiem HP, Navarro S, Qasba P, Río P, Schmidt M, Sevilla J, Verhoeyen E, Thrasher AJ, Bueren J. [Gene Therapy for Fanconi Anemia: One Step Closer to the Clinic](#). **Hum Gene Ther.** **23:141-4 (2012)**

55. Río P, Agirre X, Garate L, Baños R, Álvarez L, San José-Enériz, E, Badell I, Casado JA, Garín M, Prósper F and Bueren JA. *Downregulated Expression of hsa-*

miR-181c in Fanconi Anemia Patients: Implications in TNF α Regulation and in the Proliferation of Hematopoietic Progenitor Cells. **Blood.** **119:3042-9 (2012)**

56. Valeri A, Río P, Agirre X, Prosper F and Bueren JA. *Unraveling the Role of FANCD2 in Chronic Myeloid Leukemia. (Letter)* **Leukemia** **26:1447-8 (2012)**

57. Cuesta-Domínguez Á, Ortega M, Ormazábal C, Santos-Roncero M, Galán-Díez M, Steegmann JL, Figuera Á, Arranz E, Vizmanos JL, Bueren JA, Río P, Fernández-Ruiz E. *Transforming and tumorigenic activity of JAK2 by fusion to BCR: molecular mechanisms of action of a novel BCR-JAK2 tyrosine-kinase.* **PLoS One.** **2012;7(2):e32451.**

58. Oviedo A, Yañez R, Colmenero I, Aldea M, Rubio A, Bueren JA, Lamana ML. *Reduced efficacy of mesenchymal stromal cells in preventing graft-vs-host-disease in an in vivo model of haploidentical bone marrow transplant with leukemia.* **Cell Transplant.** **2012 Oct 4.** Cell Transplant. 2012 Oct 4. [Epub ahead of print]PMID:23044223

59. Tremblay JP, Xiao X, Aartsma-Rus A, Barbas C, Blau HM, Bogdanove AJ, Boycott K, Braun S, Breakefield XO, Bueren JA, Buschmann M, Byrne BJ, Calos M, Cathomen T, Chamberlain J, Chuah M, Cornetta K, Davies KE, Dickson JG, Duchateau P, Flotte TR, Gaudet D, Gersbach CA, Gilbert R, Glorioso J, Herzog RW, High KA, Huang W, Huard J, Joung JK, Liu D, Liu D, Lochmüller H, Lustig L, Martens J, Massie B, Mavilio F, Mendell JR, Nathwani A, Ponder K, Porteus M, Puymirat J, Samulski J, Takeda S, Thrasher A, VandenDriessche T, Wei Y, Wilson JM, Wilton SD, Wolfe JH, Gao G. [Translating the genomics revolution: the need for an international gene therapy consortium for monogenic diseases.](#) **Mol Ther.** **2013 Feb;21(2):266-8.** doi: 10.1038/mt.2013.4.

60. Oviedo A, Yañez R, Colmenero I, Aldea M, Rubio A, Bueren JA, Lamana ML. *Reduced efficacy of mesenchymal stromal cells in preventing graft-versus-host disease in an in vivo model of haploidentical bone marrow transplant with leukemia.* **Cell Transplant.** **2013;22(8):1381-94.** doi: 10.3727/096368912X657666. Epub 2012 Oct 4.

61. Martínez S, Pérez L, Galmarini CM, Aracil M, Tercero JC, Gago F, Albella B, Bueren JA. *Inhibitory effects of marine-derived DNA-binding anti-tumour tetrahydroisoquinolines on the Fanconi anaemia pathway.* **Br J Pharmacol.** **2013 Oct;170(4):871-82.** doi: 10.1111/bph.12331.

62. Molina-Estevez FJ, Lozano ML, Navarro S, Torres Y, Grabundzija I, Ivics Z, Samper E, Bueren JA, Guenechea G. *Impaired cell reprogramming in nonhomologous end joining deficient cells.* **Stem Cells.** **2013 Aug;31(8):1726-30.** doi: 10.1002/stem.1406.

63. Bogliolo M, Schuster B, Stoepker C, Derkunt B, Su Y, Raams A, Trujillo JP, Minguillón J, Ramírez MJ, Pujol R, Casado JA, Baños R, Río P, Knies K, Zúñiga S, Benítez J, Bueren JA, Jaspers NG, Schärer OD, de Winter JP, Schindler D, Surrallés J. *Mutations in ERCC4, encoding the DNA-repair endonuclease XPF, cause Fanconi anemia.* **Am J Hum Genet.** **2013 May 2;92(5):800-6.** doi: 10.1016/j.ajhg.2013.04.002. Epub 2013 Apr 25.

64. S Navarro, V Moleiro, F.J. Molina-Estevez, M.L. Lozano, R Chinchon, E Almarza, O Quintana-Bustamante, G Mostoslavsky, T Maetzig, M Galla, N Heinz, B Schiedlmeier, Y Torres, U Modlich, E Samper, P Río, J.C. Segovia, A Raya, G

Güenechea, J.C. Izpisua-Belmonte and J.A. Bueren. Generation of iPSCs from genetically corrected *Brca2* hypomorphic cells: Implications in cell reprogramming and stem cell therapy. **Stem Cells. Accepted manuscript online: 29 OCT 2013.** DOI: 10.1002/stem.1586

65. Tremblay JP, Aartsma-Rus A, Bogdanove A, Ferreira MB, Bueren J, Huard J. Development of a web course on gene therapy by the international consortium of gene therapy. **Mol Ther. 2014 Mar;22(3):482. doi: 10.1038/mt.2014.11.**

66. Rio P, Baños R, Lombardo A, Quintana-Bustamante O, Alvarez L, Garate Z, Genovese P, Almarza E, Valeri A, Díez B, Navarro S, Torres Y, Trujillo JP, Murillas R, Segovia JC, Samper E, Surralles J, Gregory PD, Holmes MC, Naldini L, Bueren JA. Targeted gene therapy and cell reprogramming in Fanconi anemia. **EMBO Mol Med. 2014 May 23;6(6):835-48. doi: 10.15252/emmm.201303374.**

67. Leon-Rico D, Aldea M, Sanchez R, Segovia JC, Weiss LA, Hidalgo A, Bueren JA, Almarza E. Reduced expression of CD18 leads to the in vivo expansion of hematopoietic stem cells in mouse bone marrow. **Stem Cells. 2014 Jun 6. doi: 10.1002/stem.1762.**

68. Modeling Fanconi Anemia pathogenesis and therapeutics using integration-free patient iPSCs. G-H Liu, K Suzuki, M Li, J Qu, N Montserrat, C Tarantino, Y Gu, F Yi, X Xu, W Zhang, S Ruiz, N Plongthongkum, K Zhang, S Masuda, E Nivet, Y Tsunekawa, R D Soligalla, A Goebel, E Aizawa, N Y Kim, J Kim, I Dubova, Y Li, R Ren, C Benner, A del Sol, J Bueren, JP Trujillo, J Surralles, E Cappelli, C Dufour, C Rodriguez-Esteban and JC Izpisua-Belmonte. **Nature Comm Commun. 2014 Jul 7;5:4330. doi: 10.1038/ncomms5330.**

69. Conversion of Human Fibroblasts into Monocyte-Like Progenitor Cells. Pulecio J, Nivet E, Sancho-Martinez I, Vitaloni M, Guenechea G, Xia Y, Kurian L, Dubova I, Bueren J, Laricchia-Robbio L, Izpisua Belmonte JC. **Stem Cells. 2014 Aug 30. doi: 10.1002/stem.1800. [Epub ahead of print]**

70. F. J. Molina-Estevez, A. Nowrouzi, M.L. Lozano, A. Galy, S. Charrier, C. von Kalle, G. Guenechea, J. A. Bueren*, M. Schmidt*. Lentiviral Gene Therapy in Fanconi Anemia Mice Reveals Long-term Engraftment and Continuous Turnover of Corrected HSCs. **Molecular therapy. Enviado.**

71. M. Fernández-García, R.M. Yañez, R. Sánchez-Domínguez, J.C. Segovia, J.A. Bueren and M.L. Lamana. Mesenchymal Stromal Cells Enhance the Engraftment of Hematopoietic Stem Cells in an Autologous Mouse Transplantation Model. **Molecular Therapy. Enviado**

ORPHAN DRUGS

Lentiviral vector carrying the Fanconi anaemia-A (*FANCA*) gene for the treatment of Fanconi anaemia type A. . Committee for Orphan Medicinal Products. P.I: Juan A. Bueren. PROMOTOR: CIEMAT and CIBERER

SUPERVISION OF DOCTORAL THESIS

1. M^a Teresa Grande Azañedo (CIEMAT).

Estimulación hematopoyética de células madre hematopoyéticas de ratón por actividades criculantes producidas durante la regeneración de un daño hematopoyético radioinducido.

Universidad Complutense de Madrid. Facultad de Biología
1988
Sobresaliente *cum laude*

2. Elvira Cuenllas (F. Veterinaria)

Expresión del daño residual radioinducido sobre la funcionalidad de las células del sistema hematopoyético de ratón.

Universidad Complutense. Facultad de Farmacia
1992
Apto *cum laude*

3. José Carlos Segovia (CIEMAT)

Mecanismo de patogénesis del parvovirus en el sistema hematopoyético del ratón.

Universidad Autónoma. Facultad de Biología
1993
Apto *cum laude*

4. Florencio Varas. (CIEMAT)

Marcado genético de médula ósea por vectores retrovirales.

Universidad Autónoma. Facultad de Biología
1995
Apto *cum laude*

5. Javier G^a Castro. (Becario CICYT FPI)

Purgado de médula ósea contaminada con leucemia mediante vectores virales: Un modelo preclínico.

Universidad Complutense. Facultad de Biología
2001
Apto *cum laude*

6. Paula Río. (Becaria CICYT FPI)

Terapia génica de la Anemia de Fanconi en un modelo murino.

Universidad Complutense. Facultad de Biología
2002
Sobresaliente *cum laude*

7. Susana García. (Becaria Pharmamar)

Predicción de la hematotoxicidad de fármacos antitumorales de origen marino.

Universidad Autónoma. Facultad de Biología
2004
Apto *cum laude*

8. Elena Almarza. (Becaria F. Botín)

Estudio del promotor vav para la expresión de genes en células del sistema hematopoyético

Universidad Complutense. Facultad de Biología
2007
Tesis Europea. Apto *cum laude*

9. Africa González. (Becaria CIEMAT)

Seguridad y Eficacia de vectores lentivirales para la terapia génica de enfermedades genéticas hereditarias.

Universidad Complutense. Facultad de Biología
2009
Apto *cum laude*

- 10. Ariana Jacome.** (Becaria CICYT FPI)
Modelos preclínicos de terapia génica de la anemia de Fanconi.
Universidad Complutense. Facultad de Biología
2009
Tesis Europea. Apto *cum laude*
- 11. Maria Eugenia Alonso** (Becaria CIEMAT)
Terapia Génica prenatal en modelos de ratón
Universidad Complutense. Facultad de Biología
2009
European Thesis. Apto *cum laude*
- 12. Sandra Fernández** (Becaria Pharmamar)
Implicaciones de la ruta de la anemia de Fanconi en la respuesta de tumores a fármacos tetraisoquinolónicos que interaccionan con el DNA
Universidad Complutense. Facultad de Biología
2013
Apto *cum laude*
- 13. Javier Molina** (Becario CIEMAT)
Gene Therapy and Cell reprogramming in mouse models of monogenic hematopoietic stem cell diseases
Universidad Autónoma de Madrid. Facultad de Biología
2013
European Thesis. Apto *cum laude*
- 14. Rocío Baños** (Becario MINECO)
Nuevas aproximaciones para el estudio y terapia génica de la anemia de Fanconi
Universidad Complutense de Madrid. Facultad de Farmacia
2014
Apto *cum laude*
- 14. Victoria Moleiro** (Becario MINECO)
Gene Therapy and cell reprogramming in a mouse model with mutations in *Fancd1/Brca2*
Universidad Complutense de Madrid. Facultad de Farmacia
2014
European Thesis. Apto *cum laude*
- 15. Antonio Valeri** (Becario CIEMAT)
Implicaciones de la ruta de la anemia de Fanconi en Leucemia Mieloide Crónica
Universidad Complutense de Madrid. Facultad de Biología
2014
Apto *cum laude*