Fecha del CVA	04/10/2019

Parte A. DATOS PERSONALES

Nombre y Apellidos	Juan Bueren Roncero			
DNI	50.801.002 M		Edad	62
Núm. identificación del investigador	Researcher ID	L-6112-2014		
	Scopus Author ID			
	Código ORCID	0000-0002-3228-7013		

A.1. Situación profesional actual

Organismo	Rocket Pharma			
Dpto. / Centro	/			
Dirección	Av. Complutense, 40, 28040, Madrid			
Teléfono	(34) 696614806	Correo electrónico	juan.bueren@ciemat.es	
Categoría profesional	Consultant		Fecha inicio	2016
Espec. cód. UNESCO				
Palabras clave				

A.2. Formación académica (titulo, institución, fecha)

Licenciatura/Grado/Doctorado	Universidad	Año
Doctor en Farmacia	Universidad Complutense de Madrid	1982
Licenciado en Farmacia	Universidad Complutense de Madrid	1979

A.3. Indicadores generales de calidad de la producción científica

Six-year research: 5
Directed doctoral theses: 21
Sum of the Times Cited: 6165
Articles with Citation Data: 470
Average citations per item: 13.1
Articles in PubMed: 154
h-index: 38
Principal Investigator in 34 National Projects, 14 International.
Author in 9 patents and 4 Orphan Drugs.

Parte B. RESUMEN LIBRE DEL CURRÍCULUM

Prof Bueren is the Head of the Division of Hematopoietic Innovative Therapies of CIEMAT and CIBER of Rare Diseases of the ISCIII and Coordinator of the Mixed Unit of Advanced Therapies CIEMAT/IIS-Jiménez Díaz Foundation. He has participated in 14 European Programs, and coordinated two of them "Novel Approaches for the Management of the Radiation Syndrome (FP-5) and “Phase I/II Gene Therapy Trial on Patiens with Fanconi anemia A (FP-7). Between the years 2001-2004 he participated in two European Projects (CONSERT; LSHB-CT-2004-5242 and PERSIST; Grant Agreement Number: 222878) focused on the optimization of hematopoietic stem cell gene therapy. Thereafter he focused his attention in the implementation of an ambitious translational program of gene therapy for patients with Fanconi anemia. Hence, he participated as coordinator of a National Network on "Applications of molecular and cellular biology to the diagnosis and treatment of Fanconi anemia", funded by the Ministry of Health and Genoma España. In 2010, he participated in the development of an Orphan Drug approved by the European Medicines Agency and thereafter by Federal and Drug Administration of the USA named: “Lentiviral vector carrying the FA-A gene for the treatment of Fanconi anemia patients”. In 2014 he served as coordinator of a National Grant from the Ministry of Health for the gene therapy of FA (Call for Independent Clinical Investigation),

which was extended to other European Countries by the EUROFANCOLEN Project. In 2016 he signed a Master Research Agreement and a License Agreement with Rocket Pharma for the development of two gene therapy programs in Europe and in the USA for Fanconi anemia patients and for patients with the primary Immunodeficiency LAD-I.

Dr Bueren has supervised 21 Doctoral Theses, participated as Principal Investigator in 34 National Projects and 14 European Projects related to hematopoietic stem cells and hematopoietic gene therapy, and has published 154 research papers that appear in the PubMed data base.

Other distinctions:

· Corresponding Academician of the Royal National Academy of Pharmacy and the Galician Academy of Pharmacy.

· Former President of the Spanish Society of Gene and Cell Therapy (2011-2013)

· VicePresident of the European Societty for Gene and Cell Therapy (2018-)

· Consultant of Rocket Pharma Inc.(2016-)

· Distinguished Service Award by Fanconi Anemia Reseearch Fund (FARF). October 2011.

· Member of the “Data Safety Monitoring Board of the Gene Therapy trial for Beta Thalassemia”at the HSR-TIGET, in Milan (IPs: Alessandro Aiuti and Fabio Ciceri). 2015-2018

· Member of the Hematologic and Immunologic Gene and Cell Therapy Committee of the American Society for Gene and Cell Therapy (2015-2018) and of the International Committee of the American Society for Gene and Cell Therapy (2018-2020)

· Member of the Scientific Advisory Board of the European Projects SCIDNET and RAG-2 Gene Therapy (2017-2020)

· Former President of the Organizing Committee of the Collaborative Congress of the "European Society for Gene and Cell Therapy" and the "Spanish Society of Gene and Cell Therapy". Madrid 24-28 October 2013.

Parte C. MÉRITOS MÁS RELEVANTES (ordenados por tipología)

C.1. Publicaciones

1 **Artículo científico**. Roman-Rodriguez, F. J.; et al. 2019. NHEJ-mediated repair of CRISPR-Cas9-induced DNA breaks can efficiently correct mutations in HSPCs from patients with Fanconi Anemia Cell Stem Cell. In press.

2 **Artículo científico**. Rio, P; et al. 2019. Successful Engraftment of Gene Corrected Hematopoietic Stem Cells in Non-conditioned Fanconi Anemia Patients Nature Medicine. In press.

3 **Artículo científico**. Rio, Paula; Navarro, Susana; Bueren, Juan A.2018. Advances in Gene Therapy for Fanconi Anemia HUMAN GENE THERAPY. 29-10, pp.1114-1123. ISSN 1557-7422.

4 **Artículo científico**. Lopez-Santalla, Mercedes; et al. 2018. Comparative Analysis between the In Vivo Biodistribution and Therapeutic Efficacy of Adipose-Derived Mesenchymal Stromal Cells Administered Intraperitoneally in Experimental Colitis INTERNATIONAL JOURNAL OF MOLECULAR SCIENCES. MDPI. 19-7. ISSN 1422-0067.

5 **Artículo científico**. Almarza Novoa, Elena; et al. 2018. Leukocyte adhesion deficiency-I: A comprehensive review of all published cases JOURNAL OF ALLERGY AND CLINICAL IMMUNOLOGY-IN PRACTICE. 6-4, pp.1418-+. ISSN 2213-2198.

6 **Artículo científico**. Holstein, Marta; et al. 2018. Efficient Non-viral Gene Delivery into Human Hematopoietic Stem Cells by Minicircle Sleeping Beauty Transposon Vectors MOLECULAR THERAPY. CELL PRESS. 26-4, pp.1137-1153. ISSN 1525-0016.

7 **Artículo científico**. Diez, Begona; et al. 2017. Therapeutic gene editing in CD34(+) hematopoietic progenitors from Fanconi anemia patients EMBO MOLECULAR MEDICINE. WILEY. 9-11, pp.1574-1588. ISSN 1757-4676.

8 **Artículo científico**. Levy, Camille; et al. 2017. Measles virus envelope pseudotyped lentiviral vectors transduce quiescent human HSCs at an efficiency without precedent BLOOD ADVANCES. AMER SOC HEMATOLOGY. 1-23, pp.2088-2104. ISSN 2473-9537.

9 **Artículo científico**. Rio, Paula; et al. 2017. Engraftment and in vivo proliferation advantage of gene-corrected mobilized CD34(+) cells from Fanconi anemia patients BLOOD. AMER SOC HEMATOLOGY. 130-13, pp.1535-1542. ISSN 1528-0020.
10 **Artículo científico**. Reina-Castillon, Judith; et al. 2017. Detectable clonal mosaicism in blood as a biomarker of cancer risk in Fanconi anemia BLOOD ADVANCES. AMER SOC HEMATOLOGY. 1-5, pp.319-329. ISSN 2473-9537.
11 **Artículo científico**. Pulecio, Julian; et al. 2016. Direct Conversion of Fibroblasts to Megakaryocyte Progenitors CELL REPORTS. CELL PRESS. 17-3, pp.671-683. ISSN 2211-1247.
12 **Artículo científico**. Leon-Rico, Diego; et al. 2016. Lentiviral Vector-Mediated Correction of a Mouse Model of Leukocyte Adhesion Deficiency Type I HUMAN GENE THERAPY. MARY ANN LIEBERT, INC. 27-9, pp.668-678. ISSN 1043-0342.
13 **Artículo científico**. Garcia, Marta; et al. 2016. Long-term skin regeneration in xenografts from iPSC teratoma-derived human keratinocytes EXPERIMENTAL DERMATOLOGY. WILEY-BLACKWELL. 25-9, pp.736-738. ISSN 1600-0625.
14 **Artículo científico**. Rio, Paula; Bueren, Juan A. 2016. TGF-beta: a master regulator of the bone marrow failure puzzle in Fanconi anemia.Stem cell investigation. 3, pp.75. ISSN 2306-9759.
15 **Reseña**. Bueren, Juan A.; et al. 2019. Advances in the gene therapy of monogenic blood cell diseases CLINICAL GENETICS. ISSN 0009-9163.

C.2. Proyectos

1 GENCURE-Nuevas aproximaciones terapéuticas para el tratamiento de enfermedades hereditarias de fallo de médula ósea-RTI2018-097125-B-I00 G. Güenechea. (Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas). 01/01/2019-31/12/2021. 266.200 €.
2 AvanCell-CM: Una nueva generación de medicamentos celulares más eficaces y seguros J. A. Bueren. (Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas). 01/01/2018-31/12/2021.
3 FANCOLEN. "Ensayo clínico Fase I/II para evaluar la seguridad de la infusión de células CD34+ autólogas movilizadas con mozobil y filgrastim y transducidas con un vector lentiviral portador del gen FANCA (medicamento huérfano) para pacientes…" Julián Sevilla Navarro. (Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas). 01/01/2012-31/12/2021. 1.052.640 €.
4 AVANCELL: Terapias avanzadas de precisión en regeneración y reparación celular y tisular Francisco Fernandez Avilés. (Hospital Gregorio Marañón/CIEMAT/and other public Institutions). 01/01/2016-31/12/2019. 867.900 €.
5 mRNA engineered mesenchymal stromal cells: A second generation of MSCs for the treatment of inflammatory diseases DAMIAN GARCIA OLMO. (IIS. Fundación Jiménez Díaz and CIEMAT). 01/01/2016-31/12/2019. 867.900 €.
6 FANCOSTEM. Ensayo clínico Fase I/II para evaluar la seguridad y eficacia de la infusión de células CD34+ autólogas movilizadas con mozobil y filgrastim y transducidas con un vector lentiviral portador del gen FANCA (medicamento huérfano) para pacientes con Anemia de Fanconi del Subtipo A. EC11-060 Ministerio de Sanidad. Convocatoria de Ensayos Clínicos Independientes.. Cristina Díaz de Heredia. (Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas). 01/01/2012-31/12/2019. 437.580 €. Coordinador.
7 FAMOCURE: T. Génica y otras T. innovadoras para el tratamiento de los síndromes congénitos con fallo de médula Ósea Paula Rio Galdo. (Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas). 01/01/2016-31/12/2018.
8 EUROFANCOLEN. 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 Juan Antonio Bueren. (Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas). 01/01/2013-31/12/2018. 5.320.000 €.
9 Terapia celular y génica dirigida en anemia de Fanconi “Target”. Ref SAF 2012-39834 Ministerio de Economía y Competitividad. Plan Nacional. Juan Antonio Bueren Roncero. (Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas). 01/01/2013-31/12/2015. 301.000 €.

10 CellCAM - Una nueva generación de medicamentos celulares más eficaces y seguros . Ref S2010/BMD-2420 Comunidad de Madrid.. Juan Antonio Bueren Roncero. (Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas). 01/01/2012-31/12/2015. 1.014.960 €. Coordinador.

11 Una nueva generación de medicamentos celulares más eficaces y seguros (CellCAM). Juan Antonio Bueren Roncero. (Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas). 01/01/2012-31/12/2015. 1.014.960 €.

C.3. Contratos

1 License Agreement between Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas, Centro de Investigación Biomédica en Red, Fundación Instituto de Investigación Sanitaria Fundación Jiménez Díaz, Fundación para la Investigación Biomédica del Hospital del Niño Jesús and Rocket Pharmaceuticals, Ltd. for the Gene Therapy of Fanconi Anemia Rocket Pharmaceuticals. Juan Antonio Bueren Roncero. Desde 01/01/2016.

2 Master Research Agreement Rocket Phamaceuticals. Juan Antonio Bueren Roncero. 01/01/2016-P5Y. 5.150.000 €.

3 Creación de la Unidad Mixta de Terapias Avanzadas Fundación Jiménez Díaz/Ciemat Fundación Jiménez Díaz. Juan Antonio Bueren Roncero. Desde 01/01/2014.

4 Application of Modern Biology in the Development of Improved Diagnostic Tools and More Efficient Therapies for Patients with Mutated Fanconi Anemia/BRCA Genes Fundación Genoma España/Pharmamar/Asociación Española Anemia de Fanconi/CNIO/Universidad Autónoma Barcelona/Genzyme. Juan Antonio Bueren Roncero. 01/01/2007-P4Y. 750.000 €.

C.4. Patentes

1 Juan Antonio Bueren Roncero; Paula Rio Galdo; Susana Navarro Ordoñez; Jose Carlos Segovia Sanz; José Antonio Casado Olea; Julián Sevilla Navarro; Africa González Murillo. RTWI-002/01US 326219- 0000 (62412028). Gene therapy for patients with Fanconi anemia Estados Unidos de América. 08/09/2017. CIEMAT/CIBERER/IIS Fundación Jiménez Dïaz/Fundación Hospital Niño Jesús.

2 Segovia JC; Quintana-Bustamante O; Garate Z; Bueren JA. : EP15382545 - PCT/EP2016/076893. Knock-In Induced Pluripotent Stem Cells (Ipscs) Derived From Peripheral Blood Mononuclear Cells And Uses Thereof. 18/11/2016. CIEMAT / CIBERER / IIS-FJD OTHER COUNTRIES WHICH THE PATENT HAS BEEN EXTENDED TO: INTERNATIONAL COMPANIES THAT ARE LICENSING IT:.

3 Elena Almarza Novoa; Juan Antonio Bueren Roncero. EMA/COMP/662962/2010. ORPHAN DRUG: Hematopoietic stem cells modified with a lentiviral vector containing the CD18 (integrin beta 2) gene for the treatment of leukocyte adhesion deficiency type I España. 09/11/2016. CIEMAT/CIBER/IIS Fundación Jiménez Dïaz.

4 Elena Almarza Novoa; Juan Antonio Bueren Roncero. FDA #16/5430_2016. ORPHAN DRUG: Hematopoietic stem cells modified with a lentiviral vector containing the CD18 (integrin beta 2) gene for the treatment of leukocyte adhesion deficiency type I España. 09/11/2016. CIEMAT/CIBER.

5 Juan Antonio Bueren Roncero; Paula Rio Galdo; Susana NAvarro Ordoñez. FDA #16/5193_2016. ORPHAN DRUG: Lentiviral vector carrying the Fanconi anaemia-A (FANCA) gene for the treatment of Fanconi anaemia type A España. 02/05/2016. CIEMAT/CIBERER.

6 Juan Antonio Bueren Roncero; Paula Río Galdo; Susana Navarro Ordoñez. EMA/COMP/662962/2010. ORPHAN DRUG: Lentiviral vector carrying the Fanconi anaemia-A (FANCA) gene for the treatment of Fanconi anaemia type A España. 21/12/2010. CIEMAT/CIBERER/IIS Fundación Jiménez Dïaz.