Date of the CVA	16/02/2021

Section A. PERSONAL DATA

Name and Surname	Susana Navarro Ordóñez		
DNI	51070101G	Age	45
Researcher's identification number	Researcher ID		
	Scopus Author ID		
	ORCID	0000-0002-0764-5384	

* Obligatorio

A.1. Current professional situation

Institution	Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas			
Dpt. / Centre	/			
Address	Avda. Principe de Asturias 29 Bajoa A, Majadahonda, 28221, Madrid			
Phone	(+34) 620955865	Email	s.navarro@ciemat.es	
Professional category	Investigador Científico de los Organismos Públicos de Investigación.		Start date	2020
Keywords				

A.2. Academic education (Degrees, institutions, dates)

Bachelor/Master/PhD	University	Year
Doctor en Programa Oficial de Bioquímica y Biología Molecular	Universidad Complutense de Madrid	2004
Doctorado y Diploma de Estudios Avanzados en Programa Oficial de Bioquímica y Biología Molecular	Universidad Complutense de Madrid	2002
Licenciado en Biología, Especialidad de Biosanitaria	Universidad Complutense de Madrid	1999

A.3. General quality indicators of scientific production

Research ID: L-9300-2014. **Orchid code:** http://orcid.org/0000-0002-0764-5384

Publications in Web of Science

81

Sum of times cited

1.239

H-index

15 ☐

Average citations per item

15.3

Average citations per year

65.2

Section B. SUMMARY OF THE CURRICULUM

Since her PhD Susana Navarro has been working on the development and improvement of Gene Therapy for rare diseases focused in anemias and bone marrow failure syndromes such as porphyrias, Fanconi Anemia (FA) and Pyruvate kinase Deficiency (PKD). In 2003 she joined the Division and Innovative Therapies of the Hematopoietic System at CIEMAT/CIBERER/IIS-FJD and she´s been involved in the study of animal models for both FA and PKD and specially in the study of human bone marrow samples from patients with both of these diseases and the development of transduction protocols to restore their clinical manisfestations. Additionally, she has participated in the generation of 2 lentiviral vectors that carry the FANCA and PKLR gene respectively, and in particular, in efficacy and safety studies of the vectors generated (stay in the hematology laboratory of the Hannover Medical School, directed by Dr. Christopher Baum, 2007). Due to this she has participated in 4 Orphan Drug Designations (ODD) both in Europe and the USA for therapeutic FANCA (**EMA/COMP/662962/2010 and #DRU-2016-5193**) and PKLR (**EMA/COMP/865665/2014 and DRU-2016-5168**) lentiviral gene therapy vectors and the subsequent presentation of the IMPD according to EMA guidelines for the subsequent development of a Phase I/II gene therapy trials on these diseases. All this has allowed her to participate as an associate researcher in the clinical trial for the treatment of patients with AF through cells corrected with this therapeutic lentiviral vector carrying the FA gene and in phase II of this study that is carried out under the Sponsor of the company Rocket Pharmaceuticals as well as she´s also participating in the manufacturing of the medicinal product as Supervisor of the Clinistem GMP facility at the CIEMAT. Likewise, she is also currently participating as a researcher in the first Phase I and global clinical trial for the treatment of this disease "A Phase I Clinical Trial to Evaluate the Safety of the Infusion of Autologous CD34 + Cells Transduced With a Lentiviral Vector Carrying the Codon Optimized Red Cell Pyruvate Kinase (coRPK) Gene in Adult and Pediatric Subjects With PKD ".Aditionally during some year she intended to new sources of hematopoietic cells, workinh in the generation of iPSCs and in the differentiation of these to hematopoietic progenitors, as well as in the development of new gene therapy strategies working in the field of gene editing for Fanconi cells.

Due to this expertise she´s currently coordinating a research national project from CIBERER and co-cordinating a European Project from EJPRD in gene therapy for Blackfan Diamond anemia with a permanent position as “Investigador Científico de OPIS at CIEMAT”.

Section C. MOST RELEVANT MERITS (ordered by typology)

C.1. Publications

AC: Autor de correspondencia; (nº x / nº y): posición firma solicitante / total autores

1 **Scientific paper**. Juan Bueren; Oscar Quintana-Bustamante; Elena Almarza; Susana Navarro; Paula Río; Jose Carlos Segovia; Guillermo Güenechea. 2019. Advances in the gene therapy of monogenic blood cell diseases Clin Genet.wileyonlinelibrary.com.
2 **Scientific paper**. Susana Navarro; Paula Río;. Advances in the Gene Therapy for Fanconi Anemia Human Gene Therapy.
3 **Scientific paper**. Paula Río; Susana Navarro; Wei Wang; et al;. Successful Engraftment of Gene Corrected Hematopoietic Stem Cells in Non-conditioned Fanconi Anemia Patients Nature Medicine. Nature. 25.
4 **Scientific paper**. Pino-Barrio; Giménez; Villanueva; et al; Navarro. TALEN Mediated Gene Editing in a Mouse Model of Fanconi Anemia Sci Rep. 2020. Apr 24;-10(1):, pp.6997.

5 Paula Río*; Susana Navarro*; Guillermo Guenechea; et al; Julián Sevilla and Juan Bueren. Engraftment and In Vivo Proliferation Advantage of Gene Corrected Mobilized CD34+ cells from Fanconi Anemia Patients TÍTULO: Blood. 2017 Sep 28;130(13):1535-1542. doi: 10.1182/blood-2017-03-774174. Epub 2017 Aug 11.Blood. 2017 Sep 28;130(13):1535-1542. doi: 10.1182/blood-2017-03-774174. Epub 2017 Aug 11.
6 Navarro S; Giorgetti A; Raya A; Tolar J.Induced Pluripotency and Gene Editing in Fanconi Anemia TÍTULO Current Gene Therapy, 2016, 16, 321-328.
7 Susana Navarro; Paula Río Juan A Bueren. Perspectives on gene therapy for Fanconi anemia TÍTULO Expert Opinion on Orphan Drugs, 3:8, 899-910. (2015).
8 Garcia-Gomez M; Calabria A; Garcia-Bravo M; et al; Segovia JC.Safe and Efficient Gene Therapy for Pyruvate Kinase Deficiency. TÍTULO Mol Ther. 2016 Aug;24(7):1187-98. doi: 10.1038/mt.2016.87.

C.2. Participation in R&D and Innovation projects

1 Lentiviral-mediated gene therapy for Diamond Blackfan Anemia: Preclinical Safety and Efficacy Studies (DBAGeneCure) Instituto de Salud Carlos III. Juan Bueren Juan Bueren. (Fundación Jiménez Díaz). 2021-2024. 1.717.357 €.
2 Nuevas aproximaciones terapeuticas para el tratamiento de enfermedades hereditarias de fallo en médula osea. Guillermo Gúenechea. (CIEMAT/CIBERER/FJD-IIS). 2019-2022. 266.200 €.
3 Preclinical studies to demonstrate the efficacy and the safety of an ex vivo gene therapy approach in Diamond Blackfan Anemia with lentiviral vectors (CIBERER17-EERR-15) (CIEMAT/CIBERER/FJD-IIS). 18/04/2018-18/04/2021. 149.830 €.
4 Fancolen (CIEMAT (Comunidad de Madrid)). 2012-2017.
5 Fancostem (CIEMAT (Comunidad de Madrid)). 2012-2014.
6 FAMOCURE (CIEMAT (MINECO)). 2015-2008.

C.3. Participation in R&D and Innovation contracts

C.4. Patents

1 Julián Sevilla; Jose Antonio Casado; Jose Carlos Segovia; Susana Navarro; Paula Río; Juan Bueren. RTWI-002/01US 326219- 0000 (62412028). Gene therapy for patients with Fanconi anemia United States of America. 08/09/2017. CIEMAT/CIBERER/IIS Fundación Jiménez Díaz/Fundación Hospital Niño Jesús.
2 María García Gomez; Maria Garcia Bravo; Juan Bueren; Susana Navarro; Jose Carlos Segovia. RTWI-001/00US (326219-2001). Compositions and methods for enhanced gene expression of PKLR United States of America. 20/04/2016. CIEMAT/CIBERER/IIS Fundación Jiménez Dïaz/Fundación Hospital Niño Jesús.
3 DESIGNACIÓN DE MEDICAMENTO HUÉRFANO (FDA#DRU-2016-5193) 2016. CIEMAT/CIBERER/IIS-FJD.
4 DESIGNACIÓN DE MEDICAMENTO HUÉRFANO (FDA # DRU-2016-5168) 23/03/2015. CIEMAT/CIBERER/IIS-FJD.
5 DESIGNACIÓN DEL MEDICAMENTO HUÉRFANO (EMA/COMP/865665/2014) 21/05/2014. CIEMAT / CIBERER / IIS-FJD.
6 DESIGNACIÓN DEL MEDICAMENTO HUÉRFANO EMA / COMP / 662962/2010 2010. Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas.
7 1; Juan Bueren; Jose Carlos Segovia; Paula Río; Susana Navarro; Elena Almarza; Oscar Quintana; Juan Bueren. PCT/US19/44237. METHODS FOR GENE MODIFICATION OF HEMATOPOIETIC CELLS United States of America. CIEMAT/CIBERER/FJD-IIS/ROCKET PHARMACEUTICALS.