



Dr. Jose-Carlos Segovia has focused his research on the study of hematopoietic stem cells (HSC), on their interaction with viral pathogens, on their ex vivo purification and manipulation and on the gene transfer on HSC, with the aim of developing protocols of gene therapy for the treatment of genetic diseases with hematopoietic pathology. He defended his doctoral thesis in April 1993 and is currently Head of the Cell Technology Division and the Flow Cytometry and Cell Separation Laboratory (LACISEP), at the Center for Energy, Environmental and Technological Research (CIEMAT). Throughout his scientific career, he has published more than 100 scientific articles in high-impact journals in the areas of Gene and Cell Therapy, he has participated in more than 45 projects, being Principal Investigator of more than 10 and he has obtained 7 patents, two of them already licensed to the biotechnology companies Rocket Pharmaceuticals (New York, USA) and DanausGT Biotechnology (Madrid, Spain), respectively. During the last years, he has focused his research on the development of gene therapy protocols for the treatment of Pyruvate Kinase Deficiency (PKD). He has achieved orphan drug designation by the European Agency and the American Drug Agency (EMA and FDA,

respectively) for an addition gene therapy drug, which is already being tested in a first global human clinical trial. He has coordinated international meetings with PKD patients, relatives, and physicians, in Spain and Europe to inform about the progress that is being made and to make this new therapy reach the largest number of patients. In recent years, he has applied new gene editing technologies for the treatment of PKD, the next frontier in the application of gene therapy, obtaining Orphan Drug Designation by EMA and FDA, as well. In addition, he is beginning its application to other anemias, such as Congenetic Dyserythropoietic Anemia. In this area, he has directed three workshops on gene editing, one of them of an eminently practical nature. Dr. José Carlos Segovia is also a collaborator in numerous master's degrees in biotechnology and biomedicine that are taught at various public and private universities. Lastly, he has been secretary of the Spanish Society of Gene and Cell Therapy and he is currently vice-president of the Iberian Society of Cytometry.