Título del Proyecto	**Regenerative medicine for Fanconi anemia: generation of disease-free patient-specific iPS cells, and iPSC-derived hematopoietic progenitors and platelets**
Nº de expediente asignado	121431
Abstract	Fanconi anemia (FA) is a rare disorder caused by mutations in any of the 15 genes identified in the FA pathway. The principal outcomes of FA patients are bone marrow failure, pancytopenia and cancer predisposition. The transfusion of heterologous platelets is one of the supportive therapies for FA patients against thrombocytopenia. It has been shown that somatic cells from FA patients, upon correction of the genetic defect, can be reprogrammed to patient-specific induced pluripotent stem cells (iPSC) that may be differentiated into hematopoietic progenitor cells (HPC). Recently, the direct conversion of adult fibroblasts into HPC has been reported. Our main objective is to obtain patient-specific megakaryocytes derived from iPSC and HPC, being able to generate in vitro, autologous platelets that may be exploited for new therapeutic approaches.
Entidad Financiadora	Fundación La Marató de TV3
Convocatoria:	Ayudas a proyectos de investigación sobre regeneración y trasplante de órganos y tejidos
Importe de la ayuda	100.000€
Fechas de ejecución del proyecto	2013-2015

	
Enlaces:	https://www.ciberisciii.es/areas-tematicas/grupo-de-investigacion?id=17087