

Patent Owner's Exhibit List

IPR2023-00070

Patent 7,541,179

Exhibit No.	Description
2001	Exclusive Licensee Agreement Between Sloan Kettering Institute for Cancer Research and San Rocco Therapeutics, LLC
2002	Declaration of Dr. James Riley
2003	October 2020 Declaration of Dr. Michel Sadelain
2004	Petitioner's October 2020 Letter Submitting Dr. Sadelain's October 2020 Declaration in New York State Court
2005	Joint Defense Agreement
2006	January 2023 Declaration of Michel Sadelain
2007	Declaration of Chad May
2008	Declaration of Stefano Rivella
2009	Declaration of Lucio Luzzatto
2010	Sorrentino, "One step closer to gene therapy for hemoglobinopathies"
2011	Caterina et al., "Human beta-globin locus control region: analysis of the 5' DNase I hypersensitive site HS2 in transgenic mice." Proc Natl Acad Sci U S A. 1991 Mar 1;88(5):1626-30. doi: 10.1073/pnas.88.5.1626. PMID: 2000371; PMCID: PMC51077
2012	Judson, "Glimmering Promise of Gene Therapy"
2013	Jackson, et al., (1996), "Role of D N A sequences outside the cores of DNase hypersensitive sites (HSs) in functions of the p-globin locus control region." Domain opening and synergism between HS2 and HS3. J Biol Chem. 271:11871-8
2014	Philipsen, et al. "The β -globin dominant control region: hypersensitive site 2." EMBO J. (1990) 9:2159-67
2015	Hardison, et. al., "Locus control regions of mammalian β -globin gene clusters: combining phylogenetic analyses and experimental results to gain functional insights." (1997) Gene. 205:73-94.
2016	Persons, D. A., A. W. Nienhuis. 2000. "Gene therapy for the hemoglobin disorders: past, present, and future." Proc Natl Acad Sci U S APNAS. 97:5022-4
2017	Kafri, "Lentiviral Vectors: Regulated Gene Expression"
2018	Amado, "Lentiviral Vectors — the Promise of Gene Therapy within Reach?"

Exhibit No.	Description
2019	Chada, et al., "Specific expression of a foreign β -globin gene in erythroid cells of transgenic mice." <i>Nature</i> . (1985) 314:377-80
2020	Townes, et al., "Expression of human β -globin genes in transgenic mice: effects of a flanking metallothionein-human growth hormone fusion gene." (1985) <i>Mol Cell Biol</i> . 5:1977-83
2021	Dzierzak, et al., "Lineage-specific expression of a human β -globin gene in murine bone marrow transplant recipients reconstituted with retrovirus-transduced stem cells." (1988) <i>Nature</i> . 331:35-41
2022	Bodine, et. al., "Combination of interleukins 3 and 6 preserves stem cell function in culture and enhances retrovirus-mediated gene transfer into hematopoietic stem cells." (1989) <i>Proc Natl Acad Sci USA</i> . 86: 8897-901
2023	Bender, et al., "A majority of mice show long-term expression of a human β -globin gene after retrovirus transfer into hematopoietic stem cells." (1989) <i>Mol Cell Biol</i> . 9:1426-34
2024	Sadelain et al., <i>Proc. Nat'l Acad. Sci. (USA)</i> 92:6728-6732 (1995)
2025	GenBank Accession No. Z84721 (March 19, 1997)
2026	GenBank Accession No. NM_000517 (October 31, 2000)
2027	Hardison <i>et al.</i> , <i>J. Mol. Biol.</i> (1991) 222(2):233-249
2028	A Syllabus of Human Hemoglobin Variants (1996), by Titus <i>et al.</i> , published by The Sickle Cell Anemia Foundation in Augusta, Georgia (available online at http://globin.cse.psu.edu)
2029	GenBank Accession No. J00179 (August 26, 1993)
2030	Tagle <i>et al.</i> , <i>Genomics</i> (1992) 13(3):741-760
2031	Li <i>et al.</i> , <i>Blood</i> (1999) 93(7):2208-2216
2032	Slightom <i>et al.</i> , <i>Cell</i> (1980) 21(3):627-638
2033	Excerpts from Inventor Notebooks
2034	Excerpts from Inventor Notebooks
2035	October 2020 Declaration of Dr. Isabelle Rivière
2036	Verma et al., "Gene Therapy: Twenty-First Century Medicine," <i>Annu Rev. BioChem</i> (2005). 74:711-38
2037	Blau, et al., "Molecular Medicine, Gene Therapy – A Novel Form of Drug Delivery," <i>The New England Journal of Medicine</i> (1995).

Exhibit No.	Description
2038	Morris et al., "MHC class II gene silencing in trophoblast cells is caused by inhibition of CIITA expression," American Journal of Reproductive Immunology (1998)
2039	Physical mapping of the globin gene deletion in β -thalassemia, Benards (1979)
2040	Ryan, T. M., R. R. Behringer, N. C. Martin, T. M. Townes, R. D. Palmiter, R. L. Brinster. 1989. A single erythroid-specific DNase I super-hypersensitive site activates high levels of human beta-globin gene expression in transgenic mice. Genes Dev. 3: 314-23
2041	Pasceri, P., D. Pannell, X. Wu, J. Ellis. 1998. Full activity from human beta-globin locus control region transgenes requires 5'HSl, distal beta-globin promoter, and 3' beta-globin sequences. Blood. 92:653-63
2042	Hardison, R., J. L. Slightom, D. L. Gumucio, M. Goodman, N. Stojanovic, W. Miller. 1997. Locus control regions of mammalian beta-globin gene clusters: combining phylogenetic analyses and experimental results to gain functional insights. Gene. 205: 73-94
2043	Hacein-Bey-Abina, et. al., "Vector mediated transformation," (2003) N. Engl. J. Med. 348:255-256. [PubMed])
2044	Pfeifer, Gene Therapy: Promises and Problems
2045	Declaration of Wanda French-Brown in Support of Motion for <i>Pro Hac Vice</i>
2046	Declaration of James H. McConnell in Support of Motion for <i>Pro Hac Vice</i>
2047	Declaration of Howard S. Suh in Support of Motion for <i>Pro Hac Vice</i>