gene therapy

	NCT Number	Title	Authors	Description	Identifier	Dates
1	pubmed:36113182	Inhibiting S100A8/A9 attenuates airway obstruction in a mouse model of heterotopic tracheal transplantation	Dai Shimizu Mikio Okazaki Seiichiro Sugimoto Rie Kinoshita Kentaro Nakata Shin Tanaka Kohei Hashimoto Kentaroh Miyoshi Masaomi Yamane Akihiro Matsukawa Masakiyo Sakaguchi Shinichi Toyooka	Although bronchiolitis obliterans syndrome (BOS) is a major cause of death after lung transplantation, an effective drug therapy for BOS has not yet developed. Here, we assessed the effectiveness of a neutralizing anti-S100 calcium binding protein (S100) A8/A9 antibody against BOS. A murine model of heterotopic tracheal transplantation was used. Mice were intraperitoneally administered control IgG or the S100A8/A9 antibody on day 0 and twice per week until they were sacrificed. Tissue sections...	pmid:36113182 doi:10.1016/j.bbrc.2022.08.087	Fri, 16 Sep 2022 06:00:00 -0400
2	pubmed:36113555	Dystrophin restoration after AAV-U7-mediated dmd exon-skipping is modulated by muscular exercise in the severe D2-mdx DMD murine model	Alexandra Monceau Dylan Moutachi Mégane Lemaitre Luis Garcia Capucine Trollet Denis Furling Arnaud Klein Arnaud Ferry	Duchenne muscular dystrophy (DMD) is a severe neuromuscular disease caused by Dmd mutations resulting in the absence of dystrophin in skeletal muscle, with a greater susceptibility to damage during contraction (exercise). In this study, we evaluated whether voluntary exercise impacts a Dmd exon skipping approach and muscle physiology in a severe DMD murine model. We intramuscularly injected an AAV-U7snRNA that aimed to correct Dmd reading frame in D2-mdx mice and they voluntary ran in a wheel...	pmid:36113555 doi:10.1016/j.ajpath.2022.07.016	Fri, 16 Sep 2022 06:00:00 -0400
3	pubmed:36113627	Insights into the multi-faceted role of Pioneer transcription factors in glioma formation and progression with targeting options	Angeliki-Ioanna Giannopoulou Dimitrios S Kanakoglou Athanasios G Papavassiliou Christina Piperi	Pioneer transcription factors (TFs) present an important subtype of transcription factors which are vital for cell programming during embryonic development and cellular memory during mitotic growth, as well as cell fate reprogramming. Pioneer TFs can engage specific target binding sites on nucleosomal DNA to attract chromatin remodeling complexes, cofactors, and other transcription factors, ultimately controlling gene expression by shaping locally the epigenome. The priority of binding that they...	pmid:36113627 doi:10.1016/j.bbcan.2022.188801	Fri, 16 Sep 2022 06:00:00 -0400
4	pubmed:36113724	Size-tunable lipid vectors for controlled local delivery of siRNA from gene activated matrix	Jeremy Salvador Jade Berthelot Claire Bony Baptiste Robin Josephine Lai Kee Him Danièle Noël Emmanuel Belamie Marie Morille	Tissue engineering aims to restore or replace different types of biological tissues through the association of cells, biologic factors and biomaterials. Currently, stem cells arise as a major cell source for many therapeutic indications, and their association with 3D scaffolds allow increasing regenerative medicine efficiency. In this context, the use of RNA interference to enhance or control stem cell differentiation into the desired phenotype appears as a promising strategy. However, achieving...	pmid:36113724 doi:10.1016/j.actbio.2022.09.016	Fri, 16 Sep 2022 06:00:00 -0400
5	pubmed:36113779	A phase II trial of hypofractionated high-dose proton beam therapy for unresectable liver metastases	Kangpyo Kim Jeong Il Yu Hee Chul Park Gyu Sang Yoo Do Hoon Lim Jae Myung Noh Woo Kyoung Jeong	CONCLUSIONS: Hypofractionated PBT with a BED >100 GyRBE for liver metastasis is safe and effective, given the high rate of 6-month FFLP without grade 3 treatment-related toxicities. However, further improvements are required for larger tumors and/or those without prior systemic therapy.	pmid:36113779 doi:10.1016/j.radonc.2022.09.003	Fri, 16 Sep 2022 06:00:00 -0400

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6	pubmed:36114027	HER2 in Uterine Serous Carcinoma: Testing platforms and implications for targeted therapy	Tenley R Klc Sharon Wu Annelise M Wilhite Nathaniel L Jones Matthew A Powell Alex Olawaiye Eugenia Girda Jubilee Brown Allison Puechl Rouba Ali-Fehmi Ira S Winer Thomas J Herzog W Michael Korn Britt K Erickson	CONCLUSIONS: There was high concordance between HER2 positivity based on CISH and IHC. Rate of HER2 positivity is the lowest by NGS. Ultimately these testing platforms need to be validated by response to targeted therapy.	pmid:36114027 doi:10.1016/j.ygyno.2022.09.006	Fri, 16 Sep 2022 06:00:00 -0400
7	pubmed:36114310	Magnetically responsive nanoplatform targeting circRNA circ_0058051 inhibits hepatocellular carcinoma progression	Song You Zijin Luo Niangmei Cheng Ming Wu Yongping Lai Fei Wang Xiaoyuan Zheng Yingchao Wang Xiaolong Liu Jingfeng Liu Bixing Zhao	Circular RNAs (circRNAs) are a class of highly stable and closed-loop noncoding RNA that are involved in the occurrence and development of hepatocellular carcinoma (HCC). However, little is known about the therapeutic role of circRNAs in HCC. We found that high circ_0058051 expression was negatively correlated with the prognosis of HCC patients. Circ_0058051 knockdown attenuated the proliferation and colony formation, meanwhile inhibited migration of HCC cells. Circ_0058051 may be used as a...	pmid:36114310 doi:10.1007/s13346-022-01237-z	Fri, 16 Sep 2022 06:00:00 -0400
8	pubmed:36114353	Genetic correction of haemoglobin E in an immortalised haemoglobin E/beta-thalassaemia cell line using the CRISPR/Cas9 system	Kongtana Trakarnsanga Nontaphat Thongsin Chanatip Metheetrairut Chartsiam Tipgomut Saiphon Poldee Methichit Wattanapanitch	-thalassaemia is one of the most common genetic blood diseases worldwide with over 300 mutations in the HBB gene affecting red blood cell functions. Recently, advances in genome editing technology have provided a powerful tool for precise genetic correction. Generation of patient-derived induced pluripotent stem cells (iPSCs) followed by genetic correction of HBB mutations and differentiation into haematopoietic stem/progenitor cells (HSPCs) offers a potential therapy to cure the disease....	pmid:36114353 doi:10.1038/s41598-022-19934-7	Fri, 16 Sep 2022 06:00:00 -0400
9	pubmed:36114375	Design, construction and in vivo functional assessment of a hinge truncated sFLT01	Fahimeh Zakeri Hamid Latifi-Navid Zahra-Soheila Soheili Mehdi Sadeghi Seyed Shahriar Arab Shahram Samiei Ehsan Ranaei Pirmardan Sepideh Taghizadeh Hamid Ahmadieh Ali Hafezi-Moghadam	Gene therapy for the treatment of ocular neovascularization has reached clinical trial phases. The AAV2-sFLT01 construct was already evaluated in a phase 1 open-label trial administered intravitreally to patients with advanced neovascular age-related macular degeneration. SFLT01 protein functions by binding to VEGF and PlGF molecules and inhibiting their activities simultaneously. It consists of human VEGFR1/Flt-1 (hVEGFR1), a polyglycine linker, and the Fc region of human IgG1. The IgG1 upper...	pmid:36114375 doi:10.1038/s41434-022-00362-1	Fri, 16 Sep 2022 06:00:00 -0400
10	pubmed:36114448	Bioinformatics analysis reveals the potential target of rosiglitazone as an antiangiogenic agent for breast cancer therapy	Adam Hermawan Herwandhani Putri	CONCLUSION: This study explored the potential targets of RGZ as antiangiogenic agents in breast cancer therapy and highlighted FABP4, ADIPOQ, PPARG, PPARGC1A, CD36, and CREBBP as potential targets of RGZ. These findings require further validation to explore the potential of RGZ as an antiangiogenic agent.	pmid:36114448 doi:10.1186/s12863-022-01086-2	Fri, 16 Sep 2022 06:00:00 -0400

	NCT Number	Title	Authors	Description	Identifier	Dates
11	pubmed:36114512	Xeno-free induced pluripotent stem cell-derived neural progenitor cells for in vivo applications	Ruslan Rust Rebecca Z Weber Melanie Generali Debora Kehl Chantal Bodenmann Daniela Uhr Debora Wanner Kathrin J Zürcher Hirohide Saito Simon P Hoerstrup Roger M Nitsch Christian Tackenberg	CONCLUSION: We describe the generation of transgene- and xeno-free NPCs. This simple differentiation protocol combined with the ability of in vivo cell tracking presents a valuable tool to develop safe and effective cell therapies for various brain injuries.	pmid:36114512 doi:10.1186/s12967-022-03610-5	Fri, 16 Sep 2022 06:00:00 -0400
12	pubmed:36114514	The role of DNA demethylation in liver to pancreas transdifferentiation	Adi Har-Zahav Daniela Lixandru David Cheishvili Ioan Valentin Matei Ioana Raluca Florea Veronica Madalina Aspritoiu Inna Blus-Kadosh Irit Meivar-Levy Andreea Madalina Serban Irinel Popescu Moshe Szyf Sarah Ferber Simona Olimpia Dima	CONCLUSIONS: Transdifferentiation is associated with global DNA hypomethylation, and with increased expression of specific demethylated genes. A combination of epigenetic modulators may be used to increase chromatin accessibility of the pancreatic transcription factors, thus promoting the efficiency of the developmental process.	pmid:36114514 doi:10.1186/s13287-022-03159-6	Fri, 16 Sep 2022 06:00:00 -0400
13	pubmed:36114670	Targeted genomic translocations and inversions generated using a paired prime editing strategy	Jiyeon Kweon Hye-Yeon Hwang Haesun Ryu An-Hee Jang Daesik Kim Yongsub Kim	A variety of cancers have been found to have chromosomal rearrangements and the genomic abnormalities often induced expression of fusion oncogenes. To date, a pair of engineered nucleases including ZFNs, TALENs, and CRISPR-Cas9 nucleases have been used to generate chromosomal rearrangement in living cells and organisms for disease modeling. However, these methods induce unwanted indel mutations at the DNA break junctions, resulting in incomplete disease modeling. Here, we developed Prime editor...	pmid:36114670 doi:10.1016/j.ymthe.2022.09.008	Sat, 17 Sep 2022 06:00:00 -0400
14	pubmed:36114756	ROS inhibits ROR degradation by decreasing its arginine methylation in liver cancer	Hyuntae Im Hee-Ji Baek Eunbi Yang Kyeongkyu Kim Se Kyu Oh Jung-Shin Lee Hyunkyung Kim Ji Min Lee	Retinoic acid receptor-related orphan receptor (ROR) is a transcription factor involved in nuclear gene expression and a known tumor suppressor. ROR was the first identified substrate of lysine methylation-dependent degradation. However, the mechanisms of other post-translational modifications (PTMs) that cause ROR remain largely unknown, especially in liver cancer. Arginine methylation is a common PTM in arginine residues of non- and histone proteins and affects substrate protein function...	pmid:36114756 doi:10.1111/cas.15595	Sat, 17 Sep 2022 06:00:00 -0400
15	pubmed:36114955	Effect of FGFR2 Alterations on Overall and Progression-Free Survival in Patients Receiving Systemic Therapy for Intrahepatic Cholangiocarcinoma	Ghassan K Abou-Alfa Kristen Bibeau Nikolaus Schultz Amin Yaqubie Brittanie Millang Haobo Ren Luis Féliz	CONCLUSIONS: Patients with intrahepatic cholangiocarcinoma and FGFR2 fusions may have a better prognosis than those without FGFR2 alterations in terms of overall survival, and progression-free survival on second-line, but not first-line systemic therapy. Progression-free survival improvement on second-line chemotherapy may imply an important impact of prior chemotherapy as first line.	pmid:36114955 doi:10.1007/s11523-022-00906-w	Sat, 17 Sep 2022 06:00:00 -0400