

gene therapy

| NCT Number | | Title | Authors | Description | Identifier | Dates |
|------------|-----------------|---|--|---|--|---------------------------------|
| 1 | pubmed:36084427 | Long non-coding RNA LINC00992 promotes hepatocellular carcinoma cell proliferation, metastasis, and invasiveness by downregulating MicroRNA miR-361-5p expression to increase levels of the transcription factor twist1 | Ning-Lei Li Gang Xiao Yi-Yi Jin Yun-Yao Deng Yu-Jiao Liu Liang-Chun Yin | Hepatocellular carcinoma (HCC) is one of the most common cancers, and has an extremely poor prognosis. Our previous study confirmed that the microRNA miR-361-5p inhibited the proliferation, metastasis, invasiveness, and epithelial-to-mesenchymal transition (EMT) process of HCC by targeting the transcription factor Twist1. Long non-coding RNAs (lncRNAs) are key regulators of processes such as cell differentiation, inflammation, tumor formation, and immune escape. However, the underlying... | pmid:36084427 doi:10.1016/j.prp.2022.154115 | Fri, 09 Sep 2022 06:00:00 -0400 |
| 2 | pubmed:36084637 | In vitro modeling and rescue of ciliopathy associated with IQCB1/NPHP5 mutations using patient-derived cells | Kamil Kruczek Zepeng Qu Emily Welby Hiroko Shimada Suja Hiriyanna Milton A English Wadih M Zein Brian P Brooks Anand Swaroop | Mutations in the IQ calmodulin-binding motif containing B1 (IQCB1)/NPHP5 gene encoding the ciliary protein nephrocystin 5 cause early-onset blinding disease Leber congenital amaurosis (LCA), together with kidney dysfunction in Senior-Løken syndrome. For in vitro disease modeling, we obtained dermal fibroblasts from patients with NPHP5-LCA that were reprogrammed into induced pluripotent stem cells (iPSCs) and differentiated into retinal pigment epithelium (RPE) and retinal organoids. Patient... | pmid:36084637 doi:10.1016/j.stemcr.2022.08.006 | Fri, 09 Sep 2022 06:00:00 -0400 |
| 3 | pubmed:36084652 | High-performance multiplex drug-gated CAR circuits | Hui-Shan Li Nicole M Wong Elliot Tague John T Ngo Ahmad S Khalil Wilson W Wong | Chimeric antigen receptor (CAR) T cells can revolutionize cancer medicine. However, overactivation, lack of tumor-specific surface markers, and antigen escape have hampered CAR T cell development. A multi-antigen targeting CAR system regulated by clinically approved pharmaceutical agents is needed. Here, we present VIPER CARs (versatile protease regulatable CARs), a collection of inducible ON and OFF switch CAR circuits engineered with a viral protease domain. We established their... | pmid:36084652 doi:10.1016/j.ccell.2022.08.008 | Fri, 09 Sep 2022 06:00:00 -0400 |
| 4 | pubmed:36084658 | Tisagenlecleucel therapy for relapsed or refractory B-cell acute lymphoblastic leukaemia in infants and children younger than 3 years of age at screening: an international, multicentre, retrospective cohort study | Sara Ghorashian Elad Jacoby Barbara De Moerloose Susana Rives Denise Bonney Geoff Shenton Peter Bader Nicole Bodmer Agueda Molinos Quintana Blanca Herrero Mattia Algeri Franco Locatelli Kim Vettenranta Berta Gonzalez Andishe Attarbaschi Stephen Harris Jean Pierre Bourquin André Baruchel | BACKGROUND: Children aged younger than 3 years were excluded from the ELIANA phase 2 trial of tisagenlecleucel in children with acute lymphoblastic leukaemia. The feasibility, safety, and activity of tisagenlecleucel have not been defined in this group, the majority of whom have high-risk (KMT2A-rearranged) infant acute lymphoblastic leukaemia and historically poor outcomes despite intensification of chemotherapy, and for whom novel therapies are urgently needed. We aimed to provide real-world... | pmid:36084658 doi:10.1016/S2352-3026(22)00225-3 | Fri, 09 Sep 2022 06:00:00 -0400 |

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|------------|-----------------|--|--|---|---|---------------------------------|
| 5 | pubmed:36084874 | Theranostic silk sericin/SPION nanoparticles for targeted delivery of ROR1 siRNA: Synthesis, characterization, diagnosis and anticancer effect on triple-negative breast cancer | Armina Shirangi Fatemeh Mottaghtalab Sajad Dinarvand Fatemeh Atyabi | Triple-negative breast cancer (TNBC) has the worst prognosis among breast cancer subtypes. The lack of proper treatments prompted scientists to find a practical targeted therapy to treat this type of tumor. Based on previous studies, tyrosine kinase-like orphan receptor (ROR1) is overexpressed in TNBC cells. Here, we designed a system consisting of superparamagnetic iron oxide nanoparticles (SPIONs) decorated with silk sericin (SS NPs) for the targeted delivery of ROR1 siRNA, a gene silencer... | pmid:36084874 doi:10.1016/j.ijbiomac.2022.09.020 | Fri, 09 Sep 2022 06:00:00 -0400 |
| 6 | pubmed:36084885 | Functional analysis of the Cystatin F gene response to SGIV infection in orange-spotted grouper, Epinephelus coioides | Suifeng Xu Yuexuan Wang Chengzong Han Yunxiang Jiang Qiwei Qin Shina Wei | Cystatin F (CyF), an inhibitor of cysteine protease, was widely studied in immune defense and cancer therapy. However, the function of CyF and its latent molecular mechanism during virus infection in fish remain vacant. In our research, we cloned the open reading frame (ORF) of CyF homology from orange-spotted grouper (Ec-CyF) consisting of 342 nucleotides and encoding a 114-amino acid protein. Ec-CyF included two cystatins family sequences containing one KXXVG sequence without the signal... | pmid:36084885 doi:10.1016/j.fsi.2022.09.003 | Fri, 09 Sep 2022 06:00:00 -0400 |
| 7 | pubmed:36084888 | Zebrafish as an emerging tool for drug discovery and development for thyroid diseases | Poonam Yadav Lopmudra P Sarode Ravinder Reddy Gaddam Puneet Kumar Jasvinder Singh Bhatti Amit Khurana Umashanker Navik | Zebrafish is a useful model for understanding human genetics and diseases and has evolved into a prominent scientific research model. The genetic structure of zebrafish is 70% identical to that of humans. Its small size, low cost, and transparent embryo make it a valuable tool in experimentation. Zebrafish and mammals possess the same molecular mechanism of thyroid organogenesis and development. Thus, thyroid hormone signaling, embryonic development, thyroid-related disorders, and novel genes... | pmid:36084888 doi:10.1016/j.fsi.2022.09.001 | Fri, 09 Sep 2022 06:00:00 -0400 |
| 8 | pubmed:36085025 | Decision-making about gene therapy in transfusion dependent thalassemia | Maa-Ohui Quarmyne Diana Ross Cynthia Sinha Nitya Bakshi Jeanne Boudreaux Lakshmanan Krishnamurti | CONCLUSION: There is tempered excitement about GT in patients/families with TDT with a general willingness to accept transfusions reduction as the outcome. | pmid:36085025 doi:10.1186/s12887-022-03598-3 | Fri, 09 Sep 2022 06:00:00 -0400 |
| 9 | pubmed:36085074 | Association of novel MUC16, MAP3K15 and ABCA1 mutation with giant congenital melanocytic nevus | Renpeng Zhou Qirui Wang Jialin Hou Danru Wang Yimin Liang | CONCLUSION: The MUC16, MAP3K15 and ABCA1 may serve as novel biomarkers for predicting malignant transformation and targets for the diagnoses and therapy for the GCMN. | pmid:36085074 doi:10.1186/s41065-022-00247-8 | Fri, 09 Sep 2022 06:00:00 -0400 |
| 10 | pubmed:36085138 | In vivo restoration of dystrophin expression in mdx mice using intra-muscular and intra-arterial injections of hydrogel microsphere carriers of exon skipping antisense oligonucleotides | Shani Attias Cohen Orit Bar-Am Claudia Fuoco Galit Saar Cesare Gargioli Dror Seliktar | Duchenne muscular dystrophy (DMD) is a genetic disease caused by a mutation in the X-linked Dytrophin gene preventing the expression of the functional protein. Exon skipping therapy using antisense oligonucleotides (AONs) is a promising therapeutic strategy for DMD. While benefits of AON therapy have been demonstrated, some challenges remain before this strategy can be applied more comprehensively to DMD patients. These include instability of AONs due to low nuclease resistance and poor tissue... | pmid:36085138 doi:10.1038/s41419-022-05166-0 | Fri, 09 Sep 2022 06:00:00 -0400 |

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|------------|-----------------|---|--|---|--|---------------------------------|
| 11 | pubmed:36085167 | A RUNX-targeted gene switch-off approach modulates the BIRC5/PIF1-p21 pathway and reduces glioblastoma growth in mice | Etsuko Yamamoto Hattori Tatsuya Masuda Yohei Mineharu Masamitsu Mikami Yukinori Terada Yasuzumi Matsui Hirohito Kubota Hidemasa Matsuo Masahiro Hirata Tatsuki R Kataoka Tatsutoshi Nakahata Shuji Ikeda Susumu Miyamoto Hiroshi Sugiyama Yoshiki Arakawa Yasuhiko Kamikubo | Glioblastoma is the most common adult brain tumour, representing a high degree of malignancy. Transcription factors such as RUNX1 are believed to be involved in the malignancy of glioblastoma. RUNX1 functions as an oncogene or tumour suppressor gene with diverse target genes. Details of the effects of RUNX1 on the acquisition of malignancy in glioblastoma remain unclear. Here, we show that RUNX1 downregulates p21 by enhancing expressions of BIRC5 and PIF1, conferring anti-apoptotic properties on... | pmid:36085167 doi:10.1038/s42003-022-03917-5 | Fri, 09 Sep 2022 06:00:00 -0400 |
| 12 | pubmed:36085259 | Fibrinogen-like protein 2: Its biological function across cell types and the potential to serve as an immunotherapy target for brain tumors | Sheng Zhang Ganesh Rao Amy Heimberger Shulin Li | Brain tumors are among the 10 leading causes of cancer-related death and present unique treatment challenges due to their critical location, genetic heterogeneity, and the blood-brain barrier. Recent advances in targeted immunotherapy and immune checkpoint blocking therapy provide alternative therapeutic strategies for brain tumors. Fibrinogen-like protein 2 (FGL2), which induces transformation from low-grade glioma to high-grade glioblastoma, is a type II membrane protein that is highly... | pmid:36085259 doi:10.1016/j.cytogfr.2022.08.004 | Fri, 09 Sep 2022 06:00:00 -0400 |
| 13 | pubmed:36085288 | In vivo tumor immune microenvironment phenotypes correlate with inflammation and vasculature to predict immunotherapy response | Aditi Sahu Kivanc Kose Lukas Kraehenbuehl Candice Byers Aliya Holland Teguru Tembo Anthony Santella Anabel Alfonso Madison Li Miguel Cordova Melissa Gill Christi Fox Salvador Gonzalez Piyush Kumar Amber Weiching Wang Nicholas Kurtansky Pratik Chandrani Shen Yin Paras Mehta Cristian Navarrete-Dechent Gary Peterson Kimeil King Stephen Dusza Ning Yang Shuaitong Liu William Phillips Pascale Guitera Anthony Rossi Allan Halpern Liang Deng Melissa Pulitzer Ashfaq Marghoob Chih-Shan Jason Chen Taha Merghoub Milind Rajadhyaksha | Response to immunotherapies can be variable and unpredictable. Pathology-based phenotyping of tumors into 'hot' and 'cold' is static, relying solely on T-cell infiltration in single-time single-site biopsies, resulting in suboptimal treatment response prediction. Dynamic vascular events (tumor angiogenesis, leukocyte trafficking) within tumor immune microenvironment (TiME) also influence anti-tumor immunity and treatment response. Here, we report dynamic cellular-level TiME phenotyping in vivo... | pmid:36085288 doi:10.1038/s41467-022-32738-7 | Fri, 09 Sep 2022 06:00:00 -0400 |

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|------------|-----------------|---|---|---|---|---------------------------------|
| 14 | pubmed:36085390 | Impact of BRCA Mutation Status on Tumor Dissemination Pattern, Surgical Outcome and Patient Survival in Primary and Recurrent High-Grade Serous Ovarian Cancer: A Multicenter Retrospective Study by the Ovarian Cancer Therapy-Innovative Models Prolong Survi | Jacek Glajzer Dan Cacsire Castillo-Tong Rolf Richter Ignace Vergote Hagen Kulbe Adriaan Vanderstichele Ilary Ruscito Fabian Trillsch Alexander Mustea Caroline Kreuzinger Charlie Gourley Hani Gabra Eliane T Taube Oliver Dorigo David Horst Carlotta Keunecke Joanna Baum Timothy Angelotti Jalid Sehouli Elena Ioana Braicu | CONCLUSIONS: Patients with BRCAmut disease showed a more aggressive course of disease with earlier onset and more extensive tumor dissemination at pOC. However, surgical outcome and OS were significantly better in patients with BRCAmut disease compared with patients with BRCAwt disease. We therefore propose to consider BRCAmut status in regard to patient selection for cytoreductive surgery, especially in rOC. | pmid:36085390 doi:10.1245/s10434-022-12459-3 | Sat, 10 Sep 2022 06:00:00 -0400 |
| 15 | pubmed:36085499 | HSV-1's contribution as a vector for gene therapy | Alberto L Epstein | No abstract | pmid:36085499 doi:10.1038/s41587-022-01449-1 | Sat, 10 Sep 2022 06:00:00 -0400 |
| 16 | pubmed:36085540 | Overexpression of PYGO1 promotes early cardiac lineage development in human umbilical cord mesenchymal stromal/stem cells by activating the Wnt/-catenin pathway | Jie Shen Xiushan Wu Ping Zhu Jian Zhuang Bin Qin Fang Sun Wuzhou Yuan Xiongwei Fan Zhigang Jiang Fang Li Yongqing Li Yuequn Wang Mingyi Zhao | Cardiovascular disease still has the highest mortality. Gene-modified mesenchymal stromal/stem cells could be a promising therapy. Pygo plays an important role in embryonic development and regulates life activities with a variety of regulatory mechanisms. Therefore, this study aimed to investigate whether the overexpression of the PYGO1 gene can promote the differentiation of human umbilical cord-derived mesenchymal stromal/stem cells (HUC-MSCs) into early cardiac lineage cells and to... | pmid:36085540 doi:10.1007/s13577-022-00777-3 | Sat, 10 Sep 2022 06:00:00 -0400 |
| 17 | pubmed:36086707 | Identification of a predictive gene signature related to pyroptosis for the prognosis of cutaneous melanoma | Zhaoyang Shi Jiaying Gu Yi Yao Zhengyuan Wu | Pyroptosis is a form of inflammatory programmed cell death. However, because of no specific molecular biomarker, pyroptosis has not been considered as a novel therapeutic method to treat cutaneous melanoma (CM). Here, we identified pyroptosis genes that associate with the prognosis of CM patients and constructed an effective model for the prognostic prediction of CM patients. To identify genes related to pyroptosis that are differentially expressed in CM, we obtained gene expression data of CM... | pmid:36086707 doi:10.1097/MD.00000000000030564 | Sat, 10 Sep 2022 06:00:00 -0400 |

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|------------|-----------------|--|--|---|---|---------------------------------|
| 18 | pubmed:36086782 | Ethnicity influences phenotype and clinical outcomes: Comparing a South American with a North American inflammatory bowel disease cohort | Tamara Pérez-Jeldres Benjamín Pizarro Gabriel Ascui Matías Orellana Mauricio Cerda-Villablanca Danilo Alvares Andrés de la Vega Macarena Cannistra Bárbara Cornejo Pablo Baéz Verónica Silva Elizabeth Arriagada Jesús Rivera-Nieves Ricardo Estela Cristián Hernández-Rocha Manuel Álvarez-Lobos Felipe Tobar | Inflammatory bowel disease (IBD), including ulcerative colitis (UC) and Crohn disease (CD), has emerged as a global disease with an increasing incidence in developing and newly industrialized regions such as South America. This global rise offers the opportunity to explore the differences and similarities in disease presentation and outcomes across different genetic backgrounds and geographic locations. Our study includes 265 IBD patients. We performed an exploratory analysis of the databases of... | pmid:36086782 doi:10.1097/MD.00000000000030216 | Sat, 10 Sep 2022 06:00:00 -0400 |
| 19 | pubmed:36086817 | Feasibility and preclinical efficacy of CD7-unedited CD7 CAR T-cells for T-cell malignancies | Norihiro Watanabe Feiyan Mo Rong Zheng Royce Ma Vanesa C Bray Dayenne G van Leeuwen Juntima Sritabal-Ramirez Hongxiang Hu Sha Wang Birju Mehta Madhuwanti Srinivasan Lauren D Scherer Huimin Zhang Sachin G Thakkar LaQuisa C Hill Helen E Heslop Chonghui Cheng Malcolm K Brenner Maksim Mamonkin | CAR-mediated targeting of T-lineage antigens for the therapy of blood malignancies is frequently complicated by self-targeting of CAR T-cells or their excessive differentiation driven by constant CAR signaling. Expression of CARs targeting CD7, a pan-T cell antigen highly expressed in T-cell malignancies and some myeloid leukemias, produces robust fratricide and often requires additional mitigation strategies, such as CD7 gene-editing. In this study, we show fratricide of CD7 CAR T-cells can be... | pmid:36086817 doi:10.1016/j.ymthe.2022.09.003 | Sat, 10 Sep 2022 06:00:00 -0400 |
| 20 | pubmed:36086889 | Construction of Degradable and Amphiphilic Triblock Polymer Carriers for Effective Delivery of siRNA | Yunfeng Yan Fangtao Zhu Huahui Su Xiaomin Liu Qidi Ren Fangqian Huang Wenbo Ye Mengdan Zhao Yunchun Zhao Junpeng Zhao Qi Shuai | The development of effective and safe delivery carriers is one of the prerequisites for the clinical translation of siRNA-based therapeutics. In this study, we constructed a library of 144 functional triblock polymers using ring-opening polymerization (ROP) and thiol-ene click reaction. These triblock polymers are composed of hydrophilic poly (ethylene oxide) (PEO), hydrophobic poly (-caprolactone) (PCL), and cationic amine blocks. Three effective carriers were discovered by high-throughput... | pmid:36086889 doi:10.1002/mabi.202200232 | Sat, 10 Sep 2022 06:00:00 -0400 |

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|------------|-----------------|---|---|--|---|---------------------------------|
| 21 | pubmed:36087071 | Clinical Significance of Clonal Hematopoiesis in the Setting of Autologous Stem Cell Transplantation for Lymphoma | Tracy Lackraj Sharon Ben Barouch Jessie J F Medeiros Stephanie Pedersen Arnavaz Danesh Mehran Bakhtiari Michael Hong Kit Tong Jesse Joynt Andrea Arruda Mark D Minden John Kuruvilla Sita Bhella Vishal Kukreti Michael Crump Anca Prica Christine Chen Yangqing Deng Wei Xu Trevor J Pugh Armand Keating John E Dick Sagi Abelson Robert Kridel | Autologous stem cell transplantation (ASCT) remains a key therapeutic strategy for treating patients with relapsed or refractory non-Hodgkin and Hodgkin lymphoma. Clonal hematopoiesis (CH) has been proposed as a major contributor not only to the development of therapy-related myeloid neoplasms but also to inferior overall survival in patients who had undergone ASCT. Herein, we aimed to investigate the prognostic implications of CH after ASCT in a cohort of 420 lymphoma patients using ultra-deep,.... | pmid:36087071 doi:10.1002/ajh.26726 | Sat, 10 Sep 2022 06:00:00 -0400 |
| 22 | pubmed:36087225 | Three atypical BCR/ABL transcripts detected simultaneously in a Philadelphia-positive acute lymphoblastic leukemia patient showing resistance to tyrosine kinase inhibitors | Haimin Sun Zeying Yan Sujiang Zhang | The Philadelphia (Ph) chromosome with a BCR/ABL fusion gene is a characteristic feature of chronic myeloid leukemia (CML) and partial acute lymphoblastic leukemia (ALL) patients, with different breakpoints of the BCR and ABL genes. Here, we report the case of a Ph-positive ALL patient with poor prognosis in whom simultaneous different BCR/ABL transcripts named e1a3, e1a4, and e1a5 were detected by RNA-seq analysis but not traditional RT-PCR. To our knowledge, this is the first report to describe... | pmid:36087225 doi:10.1007/s12185-022-03451-4 | Sat, 10 Sep 2022 06:00:00 -0400 |
| 23 | pubmed:36087301 | The DNAm levels of CREB5 (cg11301281) were associated with clopidogrel resistance | Jiyi Li Jin Yang Qinglin Yu Lian Chen Xiliang Shi Jia Su Keqi Zhu | CONCLUSIONS: Our findings were likely to provide fresh understanding for the new mechanism of platelet inhibition failure and promote individualized antiplatelet therapy. | pmid:36087301 doi:10.1002/jcla.24690 | Sat, 10 Sep 2022 06:00:00 -0400 |
| 24 | pubmed:36087315 | Reduction of sporadic and neurofibromatosis type 2-associated vestibular schwannoma growth in vitro and in vivo after treatment with the c-Jun N-terminal kinase inhibitor AS602801 | Mark C Dougherty Seiji B Shibata J Jason Clark Franklin J Canady Charles W Yates Marlan R Hansen | CONCLUSIONS: The data suggest that JNK inhibition with AS602801 suppresses growth of sporadic and neurofibromatosis type 2-associated VSs. As such, AS602801 is a potential systemic therapy for VS and warrants further investigation. | pmid:36087315 doi:10.3171/2022.7.JNS22934 | Sat, 10 Sep 2022 06:00:00 -0400 |