Patient Wellness First

Issue: 1 June 2025

BioSciTech biweekly NEWS

NEWS & COMMENTARIES

Candel Therapeutics Receives FDA Regenerative Medicine Advanced Therapy Designation for CAN-2409 for the Treatment of Prostate Cancer

U.S. Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designation to CAN-2409 (aglatimagene besadenovec), the Company's biological immunotherapy lead candidate, for the treatment of newly diagnosed localized prostate cancer in patients with intermediate-to-high-risk disease

https://www.globenewswire.com/news-release/2025/05/28/3089438/0/en/Candel-Therapeutics-Receives-FDA-Regenerative-Medicine-Advanced-Therapy-Designation-for-CAN-2409-for-the-Treatment-of-Prostate-Cancer.html

Streamling Recombinant AAV Production A Single Plasmid Approach for higher Yields and Reduced Cost

by Alina Venereo-Sanchez,

- Developing innovative technologies for AAV manufacturing | CEO & Founder at VVector Bio
- Cut your plasmid costs for AAV by at least 66%.
- That's what we just showcased on our poster.

But there's more:

We showcased that doubling the concentration of the single plasmid resulted in a 5x increase in yield.

https://www.linkedin.com/posts/alina-venereo-sanchez_cut-your-plasmid-costs-for-aav-by-at-least-activity-7329174854195187714-OLhJ?utm_source=share&utm_medium=member_desktop&rcm=ACoAAA-ogHUBaoxAlJDPfBf9EfJVJbgiz1j2nPI

FDA clears first blood test for diagnosing Alzheimer's

The test from Japan-based Fujirebio Diagnostics could help patients get treatment faster. The Food and Drug Administration approved on Friday the first blood test for diagnosing Alzheimer's disease, opening up a quicker way for patients to get detected for the neurological condition and receive treatment.

https://www.statnews.com/2025/05/16/alzheimers-fujirebio-fda-approval/

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Cambridge Biomedical Campus:£1 billion BioNTech investment sets way for jobs, growth, breakthroughs

Covid-19 vaccine pioneer BioNTech SE has a committed to invest up to £1bn in the UK, including a new base on the Cambridge Biomedical Campus. The announcement, made today by the Government, includes plans to set up a new R&D centre on Campus, focused on genomics, oncology, structural biology, and regenerative medicine.

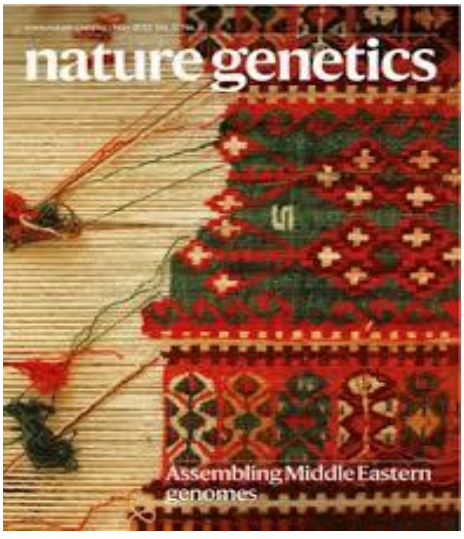
https://www.gov.uk/government/news/1-billion-biontech-investment-sets-way-for-jobs-growth-breakthroughs

Sanofi has just announced that it will acquire Vigil Neuroscience, a Boston-based biotech, in a $470 million deal to expand its neurology pipeline.

Sanofi adds an oral Alzheimer's drug (VG-3927) to its pipeline. Unlike other therapeutics, it does not target amyloid or tau but instead modulates the immune system via microglia. This makes it a first-in-class oral small molecule TREM2 agonist.

https://www.sanofi.com/en/media-room/press-releases/2025/2025-05-21-23-15-31-3086232

SELECTED PUBLICATIONS



Near-complete Middle Eastern genomes refine autozygosity and enhance disease-causing and population-specific variant discovery

Mohammadmersad Ghorbani------Younes Mokrab , 2025

https://doi.org/10.1038/s41588-025-02173-7

Using long-read sequencing, researchers generated near-complete, phased genomes from six Middle Eastern trios with neurodevelopmental conditions. These assemblies uncovered 42.2 Mb of novel sequence, 75 new HLA/KIR alleles, and high levels of inbreeding. Crucially, 23 candidate disease-causing variants were identified that were missed by standard references. The study highlights the importance of population-specific genome assemblies for improving variant detection and understanding genetic disease in underrepresented populations.

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Patient-Specific In Vivo Gene Editing to Treat a Rare Genetic Disease

K. Musunuru et al., 2025

DOI: 10.1056/NEJMoa2504747

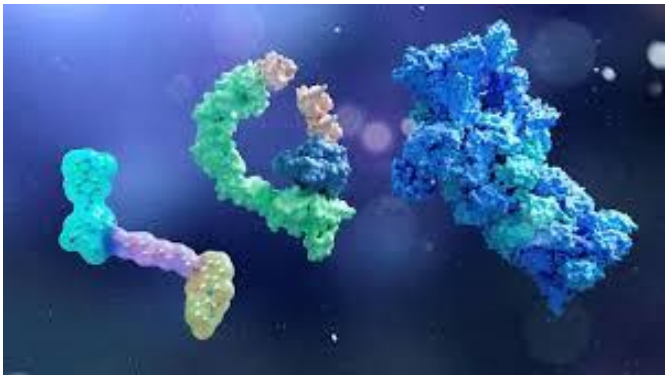
In a groundbreaking case, a custom base-editing therapy delivered via lipid nanoparticles was developed and administered within months of diagnosing a newborn with severe carbamoyl-phosphate synthetase 1 deficiency, a condition with high early mortality. After two infusions, the infant showed improved tolerance to dietary protein and reduced reliance on medication, with no serious adverse events reported. This first-in-human application of base editing in an infant highlights the rapid potential of precision genetic therapies, though continued monitoring is needed to confirm long-term safety and efficacy.

Identification of nanoparticle infiltration in human breast milk: Chemical profiles and trajectory pathways

Qing Yang et al., 2025

https://doi.org/10.1073/pnas.2500552122

A new study reveals that nanoparticles are present in human breast milk, detected in 42 of 53 samples at concentrations up to 1.12×10^{11} particles/mL. These NPs contain elements like oxygen, silicon, iron, copper, and aluminum. Researchers mapped how NPs infiltrate breast milk, crossing biological barriers via transcytosis or immune cell transfer. Smaller, neutrally charged particles showed the highest infiltration ability. This study provides the first detailed chemical profiling of NPs in breast milk and highlights the need to understand and regulate infant exposure to engineered nanomaterials.



Discovery of an LSD1 PROTAC degrader

Amir Hosseini et al., 2025

DOI:10.1073/pnas.2425812122

To overcome the limitations of traditional LSD1 inhibitors in acute myeloid leukemia, researchers developed MS9117, a PROTAC-based degrader that targets LSD1 for proteasomal degradation via cereblon. MS9117 eliminates both catalytic and noncatalytic functions of LSD1, showing superior anti-leukemic activity compared to existing inhibitors. It also enhances sensitivity to retinoic acid in non-APL AML cells. These findings position MS9117 as a promising therapeutic candidate and a powerful tool for future LSD1-targeted cancer therapies.

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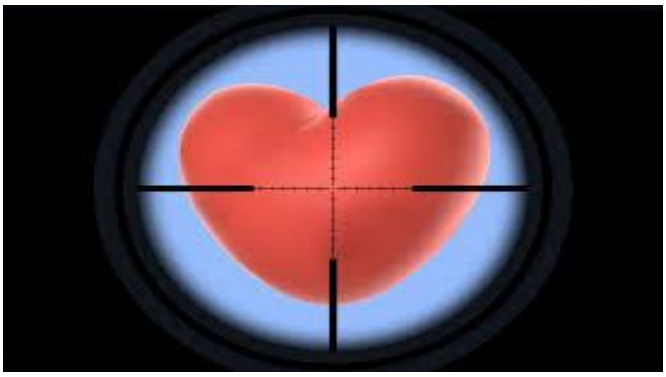

Thymic Bmi-1 hampers γδT17 generation and its derived RORγt-IL-17A signaling to delay cardiac aging

Qiuyi Wang et al., 2025

DOI:10.1073/pnas.2414717122

A new study highlights thymic Bmi-1 as a promising target for preventing senescence-associated pathological cardiac hypertrophy. Researchers found that Bmi-1 delays T cell aging by promoting thymic T cell development and suppressing pro-inflammatory γδT17 cell differentiation, thereby reducing IL-17A levels, a key driver of SA-PCH. Mechanistically, Bmi-1 regulates Notch signaling and facilitates RORγt degradation via the Bmi-1-RING1B complex. Both RORγt inhibition and IL-17A neutralization improved cardiac function in aged models. These findings open new therapeutic avenues targeting immunosenescence to combat age-related heart disease.

High continuity of forager ancestry in the Neolithic period of the eastern Maghreb

Mark Lipson et al., 2025

https://doi.org/10.1038/s41586-025-08699-4

A new *Nature* study reveals that ancient populations in the eastern Maghreb (modern-day Algeria and Tunisia) maintained strong genetic continuity with local foragers during the transition to farming. Genome-wide analysis of nine individuals dating from the Later Stone Age to the Neolithic shows that the distinctive "Maghrebi" ancestry extended across space and time. While minor genetic inputs from European hunter-gatherers, early farmers, and Levantine groups were detected, eastern Maghreb communities remained far less influenced by external migrations than their Mediterranean neighbors. These findings highlight a unique demographic path in North Africa's deep history.

Pharmacological targeting of BMAL1 modulates circadian and immune pathways

Hua Pu, Laura et al., 2025

https://doi.org/10.1038/s41589-025-01863-x

Researchers developed a small molecule, CCM, that selectively targets BMAL1 to reshape its structure and activity. This disrupts circadian signalling and reduces immune responses in macrophages. The study opens up BMAL1 as a druggable target for circadian and inflammatory regulation.

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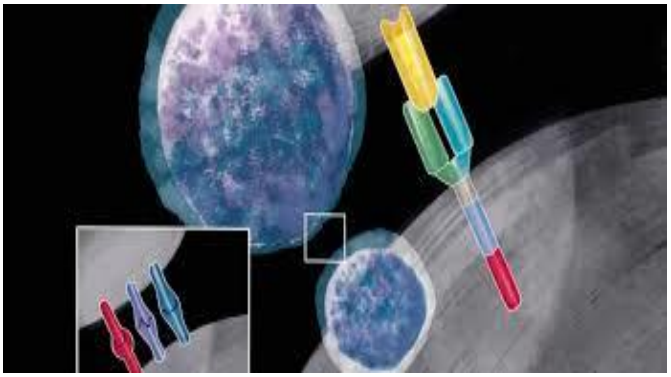


Novel strategies to manage CAR-T cell toxicity

Arthur Mulvey et al., 2025

https://doi.org/10.1038/s41573-024-01100-5

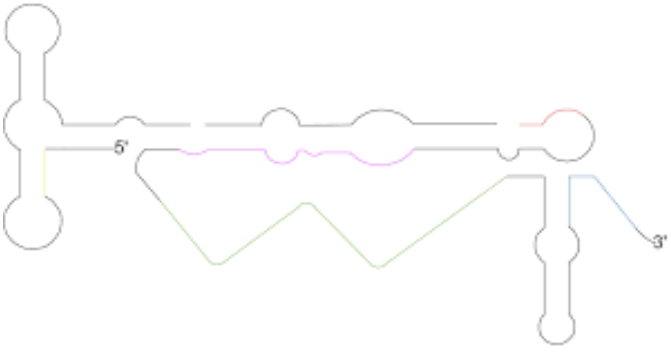
CAR-T therapies can cause severe side effects, and current treatments don't always work. This review explores how fine-tuning immune signalling or engineering CAR-T cells may reduce toxicity. Strategies include low-affinity receptors and reversible control systems.

GLP-1 receptor agonists for the treatment of alcohol use disorder

Gavin N. Petrie and Leah M. Mayo, 2025

DOI:10.1172/JCI192414

Semaglutide, a GLP-1RA drug for diabetes, may help reduce alcohol consumption. Human and animal studies show promise, unlike DPP-4 inhibitors. These findings suggest a novel approach for managing alcohol use disorder through metabolic medications.



Long noncoding RNA BCYRN1 promotes cardioprotection by enhancing human and murine regulatory T cell dynamics

Ke Liao et al., 2025

DOI:10.1172/JCI179262

The lncRNA BCYRN1 boosts regulatory T cell activity by silencing key microRNAs, enhancing immune tolerance. Enriched in extracellular vesicles, it reduces heart damage in mice after heart attacks.

Progressive Elevation of Store-Operated Calcium Entry-Associated Regulatory Factor (SARAF) and Calcium Pathway Dysregulation in Multiple Sclerosis

Safa Taha and Mohamed Belhocine, 2025

DOI:10.3390/ijms26104520

MS patients show rising SARAF levels and altered calcium signalling genes, linked to disease progression. This study in Bahraini patients suggests SARAF may be a marker and target in MS. Dysregulated calcium entry could underlie neuroinflammation in MS.

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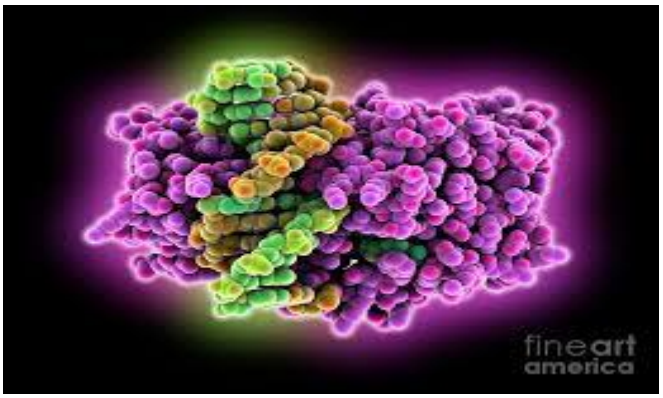

DNA methyltransferase 1 modulates mitochondrial function through bridging m5C RNA methylation

Jing Wang et al., 2025

DOI:10.1016/j.molcel.2025.04.019

DNMT1 not only maintains DNA methylation but also modulates RNA m5C methylation by recruiting NSUN2. This affects mitochondrial genes and their stability. Mutations in DNMT1 disturb this process, leading to mitochondrial dysfunction and neurodegeneration.

JOBS CORNER

Scientist – High Throughput Screening (Antibody Discovery)– Under the leadership of Nick Tribble

Reports to: Principal Scientist, Antibody Discovery

Location: Cori Building, Granta Park, Cambridge

Closing date: 26th May 2025 23:55.

https://cancerresearchuk.wd3.myworkdayjobs.com/External_Careers/job/Granta-Park/Scientist--High-Throughput-Screening--Antibody-Discovery-_R031108-1

Sr. Scientist, Computational Biology

The Altos Labs Institute of Computation (IoC) is seeking an independent and highly motivated Computational Biology Scientist to build, maintain, and support our hybrid AI initiative to model cellular processes.

https://job-boards.greenhouse.io/altoslabs/jobs/5525253004

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