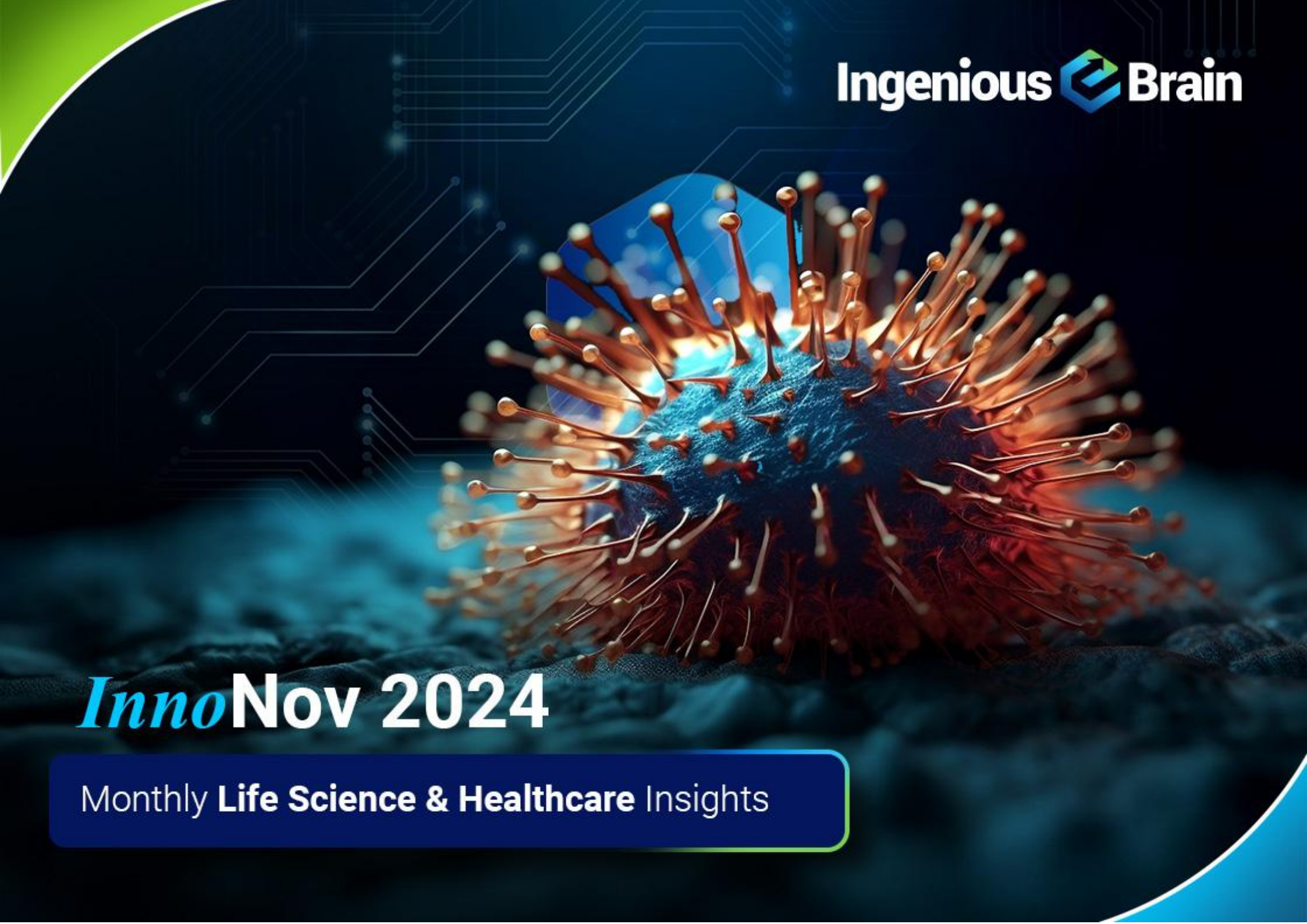
Ingenious Brain



*Inno*Nov 2024

Monthly **Life Science & Healthcare** Insights

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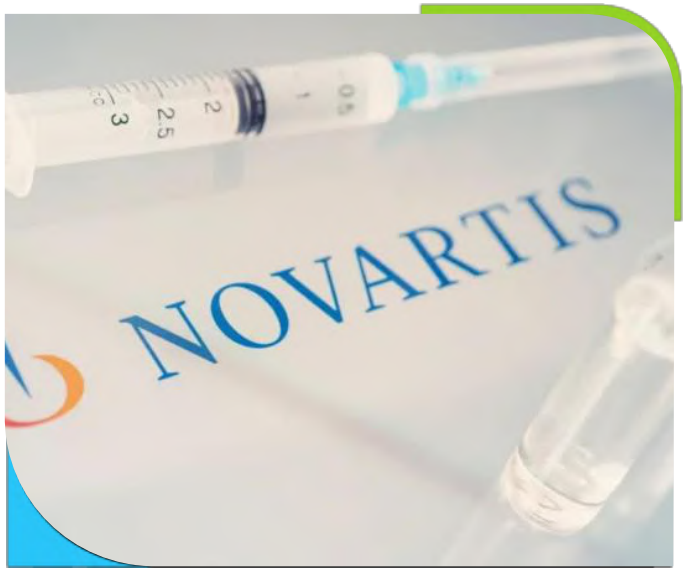


Novartis' Scemblix granted FDA accelerated approval in newly diagnosed CML

Novartis' Scemblix (asciminib) has received accelerated FDA approval to treat newly diagnosed adults with Philadelphia chromosome-positive chronic myeloid leukemia in the chronic phase (Ph-positive CML-CP), significantly expanding its eligible patient population. Approval is based on the ASC4FIRST trial, where Scemblix showed superior major molecular response rates at 48 weeks compared to tyrosine kinase inhibitors (TKIs) like imatinib. Approximately 9,280 new CML cases are expected in the US this year, with most involving the Philadelphia chromosome. Despite TKIs transforming CML into a chronic condition, challenges in efficacy, safety, and treatment tolerance remain, underscoring the need for improved therapeutic options.

AI model predicts progression of breast cancer better than standard hospital tests, study claims

Startup Ataraxis AI has developed an AI model to predict breast cancer progression rates up to 30% more accurately than standard tests like Oncotype DX. Published on arXiv, the model uses machine learning trained on extensive tumor progression imagery and patient data from partnered hospitals. By averaging predictions from multiple models, the approach reduces errors. Tested on historical data from 3,500 patients, the model aims to optimize treatment planning by addressing variability in cancer progression. The team plans to enhance accuracy further and develop additional diagnostic tools, with software expected to be available to healthcare facilities next year.

Merck acquires oncology spinout Modifi Biosciences in a deal worth $1.3bn

Merck & Co, operating as MSD outside the US and Canada, has acquired oncology-focused Yale University spinout Modifi Biosciences for $1.3 billion. The deal includes $30 million upfront and milestone payments. Modifi's innovative preclinical compounds exploit DNA repair defects in hard-to-treat cancers by targeting MGMT-deficient cancer cells, including glioblastomas and gliomas. With approximately half of glioblastomas and up to 80% of gliomas lacking MGMT, this marks a promising therapeutic approach. Merck's David Weinstock praised the approach's potential against resistant cancers. Modifi co-founder Ranjit Bindra expressed confidence in Merck's capabilities to advance their groundbreaking oncology innovations to clinical trials and commercialization.

Novo Nordisk's GLP-1 drug semaglutide shows promise in fatty liver disease MASH

Novo Nordisk's late-stage ESSENCE trial showed promising results for semaglutide in treating metabolic dysfunction-associated steatohepatitis (MASH). Among patients with MASH and moderate-to-advanced liver fibrosis, 37% treated with once-weekly semaglutide 2.4mg improved liver fibrosis without worsening steatohepatitis, compared to 22.5% on placebo. Additionally, 62.9% achieved steatohepatitis resolution with no fibrosis worsening, versus 34.1% on placebo. MASH, linked to obesity and type 2 diabetes, is a progressive liver disease with risks of advanced liver damage. Semaglutide, marketed as Ozempic and Wegovy, has already been approved for other conditions. Novo plans to seek regulatory approval for MASH treatment in the US and EU by mid-2025.

AstraZeneca's Fasenra approved by EC to treat rare inflammatory disease EGPA

AstraZeneca's Fasenra (benralizumab) has received European Commission approval as an add-on treatment for eosinophilic granulomatosis with polyangiitis (EGPA), a rare inflammatory disease. EGPA can damage multiple organs, and patients often rely on high-dose corticosteroids, risking relapses when tapering off. Fasenra, already approved for severe eosinophilic asthma, works by targeting the interleukin-5 receptor to reduce eosinophils and basophils. The approval follows the MANDARA study, where Fasenra showed similar remission rates to mepolizumab, with 41% of patients tapering off steroids compared to 26% for mepolizumab. According to AstraZeneca's Ruud Dobber, its convenient monthly injection marks a significant advancement for managing EGPA.

Johnson & Johnson's Balversa granted MHRA approval for bladder cancer

Johnson & Johnson's Balversa (erdafitinib), a pan-FGFR tyrosine kinase inhibitor, has received MHRA approval as an oral monotherapy for adults with unresectable or metastatic urothelial carcinoma (UC) harboring FGFR3 genetic alterations. Eligible patients must have received prior therapy with PD-1 or PD-L1 inhibitors. UC accounts for over 90% of bladder cancer cases in the UK, with FGFR3 alterations affecting up to 20% of metastatic cases. Approval follows the THOR trial, where Balversa showed superior outcomes compared to chemotherapy, including improved median overall survival (12.1 vs. 7.8 months), progression-free survival (5.6 vs. 2.7 months), and objective response rate (35.3% vs. 8.5%).

Sanofi/Regeneron's Dupixent approved by EC for younger eosinophilic oesophagitis patients

Sanofi and Regeneron's Dupixent (dupilumab) has received European Commission approval to treat eosinophilic oesophagitis (EoE) in children aged 1–11 years weighing at least 15kg. This makes Dupixent the first and only EU-approved medicine for this age group, adding to its existing approval for patients aged 12 and older. EoE, a chronic inflammatory condition causing difficulty swallowing and pain, significantly improved in the phase 3 EoE KIDS trial. Dupixent achieved 68% histological disease remission at week 16 versus 3% for placebo, with consistent safety. It targets interleukin-4 and interleukin-13 pathways, addressing type 2 inflammation in EoE and related diseases.

WHO announces priority endemic pathogens for urgent vaccine development

The WHO study highlights 17 endemic pathogens urgently needing vaccine development, marking the first global effort to prioritize them based on antimicrobial resistance, disease burden, and socioeconomic impact. Long-standing priorities like malaria and HIV vaccines are reaffirmed alongside emerging focuses such as Group A Streptococcus and Klebsiella pneumoniae. The initiative aims to save lives in vulnerable communities and reduce medical costs, shifting vaccine development from profit-driven goals to health impact. Vaccines for pathogens like hepatitis C, Cytomegalovirus, and Norovirus are in varying development stages. The study emphasizes reducing disease impact globally while addressing current vaccine research and innovation gaps.

World Health Organization

GSK expands immunology pipeline by acquiring Chimagen's CMG1A46 in a deal worth $850m

GSK is expanding its immunology pipeline by acquiring CMG1A46, a clinical-stage T cell-engager from Chimagen Biosciences, in an $850M deal. GSK will pay $300M upfront for global rights, with $550M in milestone payments. CMG1A46, targeting CD20 and CD19 on B cells, is in early trials for leukemia and lymphoma, with plans for a phase 1 lupus trial in 2025. The drug aims to treat B cell-driven autoimmune diseases, including systemic lupus erythematosus (SLE) and lupus nephritis. Lupus affects 5 million globally, causing inflammation, fatigue, and more, with SLE accounting for 70% of cases. CD19 offers a differentiated therapeutic approach.

FDA approves Autolus Therapeutics' Aucatzyl to treat acute lymphoblastic leukemia

The FDA has approved Autolus Therapeutics' Aucatzyl (obecabtagene autoleucel), a CD19-directed CAR-T cell therapy, to treat relapsed or refractory B-cell precursor acute lymphoblastic leukemia (ALL) in adults. It is the first CAR-T therapy approved without requiring a risk evaluation mitigation strategy. ALL affects 8,400 adults annually in the US and EU, with poor survival rates in the relapsed/refractory setting. Supported by positive phase 2 FELIX trial data, Aucatzyl achieved a 42% complete remission rate within three months, with a median remission duration of 14.1 months. The therapy aims to improve safety and efficacy compared to existing CAR-T treatments.

Novavax announces FDA clinical hold lift on phase 3 COVID-19/flu vaccine study

The FDA has lifted its clinical hold on Novavax's late-stage trials for its COVID-19/influenza combination and stand-alone influenza vaccine candidates. The hold was imposed in October 2023 due to a serious adverse event, initially reported as motor neuropathy, later reclassified as amyotrophic lateral sclerosis, a condition unrelated to vaccination. Novavax provided additional data that satisfied the FDA's concerns, enabling the company to proceed with its phase 3 trial. Novavax is working with trial investigators to resume activities swiftly. Chief Medical Officer Robert Walker expressed gratitude for the FDA's thorough review and support during this process.

Lupin to develop near-zero global warming inhalers

Lupin Healthcare UK Ltd plans to reformulate its pMDI inhalers with a near-zero global warming potential propellants, targeting net-zero emissions by 2045. This initiative aligns with European and UK regulations, aiming to reduce the environmental impact of asthma and COPD treatments. Ben Ellis, Lupin's UK General Manager, anticipates development completion by 2026, with regulatory filing to follow. CEO Vinita Gupta highlighted Lupin's commitment to sustainable, patient-centric healthcare innovation. Professor Mona Bafadhel emphasized the importance of greener inhalers for environmental and patient needs. Current pMDI propellants significantly contribute to climate change, accounting for 3% of the NHS's carbon footprint in the UK.

GSK and Vesalius partner on new Parkinson's treatments in a deal worth $650m

GSK and Vesalius Therapeutics have partnered in a $650M deal to develop treatments for Parkinson's disease (PD) and another neurodegenerative condition. Vesalius will leverage its proprietary platform, combining human genetics, genomics, AI, and stem cell models, to identify novel therapeutic targets. GSK gains global rights to a preclinical PD-focused small molecule program and potential therapies. Vesalius will receive $80M upfront, with up to $570M in milestone payments and tiered royalties. This collaboration underscores GSK's focus on advanced technology to address the root causes of diseases, aiming to benefit over 10 million PD patients worldwide through innovative intervention strategies.

Commission fines Teva €462.6 million over misuse of the patent system and disparagement to delay rival multiple sclerosis

The European Commission fined Teva €462.6 million for abusing its market dominance to delay competition for its multiple sclerosis drug, Copaxone. Teva extended Copaxone's patent protection artificially and spread misleading information to hinder the market entry of cheaper alternatives. The investigation revealed Teva's anti-competitive practices in Belgium, Czechia, Germany, Italy, the Netherlands, Poland, and Spain. By targeting the glatiramer acetate market, Teva aimed to prolong Copaxone's exclusivity and suppress competition. These actions violated EU antitrust rules, restricting the availability of affordable medicines and undermining fair competition in the pharmaceutical sector.

Merck gains rights to LaNova's PD-1/VEGF bispecific antibody in a deal worth over $3.2bn

Merck & Co (MSD outside the US and Canada) has signed a global licensing agreement worth over $3.2 billion with LaNova Medicines for LM-299, a PD-1/VEGF bispecific antibody. The deal includes an upfront payment of $588 million and up to $2.7 billion in milestone payments. Merck gains rights to develop, manufacture, and commercialize LM-299, undergoing Phase 1 trials in China for advanced solid tumors. Preclinical studies showed strong tumor inhibition and a favorable safety profile. LM-299, a cornerstone therapy, can be combined with immuno-oncology drugs, targeted therapies, ADCs, and T-cell activators for next-generation cancer immunotherapy.

Teva and Immunai partner to improve clinical decision-making

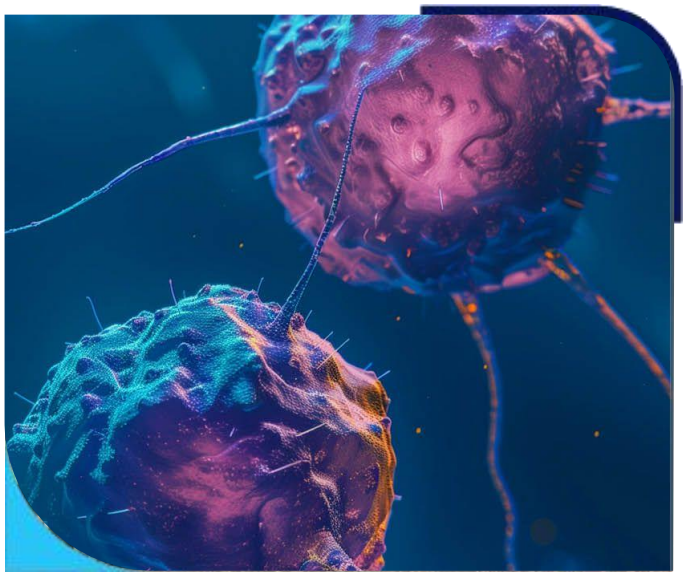
Teva Pharmaceuticals has partnered with AI-driven Immunai to optimize immunology and immuno-oncology clinical trials. The multi-year collaboration leverages Immunai's immune cell atlas (AMICA) and Immunodynamics Engine (IDE) to enhance clinical decision-making, focusing on drug mechanisms, dose selection, and biomarker analyses. The partnership may expand to include broader areas of Teva's R&D portfolio. Teva's Eran Harary highlighted the collaboration's potential to improve patient outcomes, while Immunai's CEO, Noam Solomon, emphasized its ability to streamline drug development. This partnership follows Immunai's recent collaboration with AstraZeneca, further advancing the use of AI in clinical trial optimization. Financial terms were not disclosed.

Biogen/Eisai's Alzheimer's drug lecanemab receives CHMP recommendation

The European Medicines Agency's CHMP has recommended Biogen and Eisai's Alzheimer's drug, lecanemab (Leqembi), for treating early-stage Alzheimer's disease (AD) in adults with mild cognitive impairment or mild dementia who have one or no copies of the apolipoprotein E4 gene. This follows Eisai's successful appeal against a prior negative opinion. The recommendation is based on the Clarity AD trial, where lecanemab reduced clinical decline by 31% over 18 months. Administered intravenously, Leqembi targets amyloid beta plaques in the brain. The European Commission will make a final decision. AD affects 6.9 million Europeans, progressively impairing memory, thinking, and daily tasks.

USFDA Approves FoundationOne®Liquid CDx as a Companion Diagnostic for TEPMETKO® (tepotinib) to Identify Patients with MET Exon 14 Skipping Alterations in Non-Small Cell Lung Cancer

Foundation Medicine's FoundationOne® Liquid CDx has become the first FDA-approved liquid biopsy companion diagnostic for TEPMETKO® (tepotinib), a targeted therapy by EMD Serono for metastatic non-small cell lung cancer (NSCLC) with MET exon 14 skipping alterations (METex14). TEPMETKO received traditional FDA approval in February 2024. METex14 alterations, found in 3-4% of NSCLC cases, are linked to advanced disease and poor prognosis. FoundationOne Liquid CDx provides genomic insights from a simple blood sample, analyzing over 300 cancer-related genes. With 19 FDA-approved NSCLC indications, Foundation Medicine leads in comprehensive genomic profiling and companion diagnostics, advancing precision medicine for lung cancer patients.



Ardena's expanded nanomedicine facility granted full GMP approval

Ardena has received full GMP approval from the Dutch Healthcare Authority for its expanded nanomedicine facility in Oss, Netherlands. The €20 million, 45,000 sq ft facility features GMP-compliant Grade C and D cleanrooms, advanced warehouse spaces, and dedicated laboratories for process development and analytics. The facility incorporates automated manufacturing flows and integrated analytical capabilities for complex formulations and is designed to handle a wide range of nanomedicines, including lipid-based, polymeric, and metal nanoparticles. It adheres to strict regulatory standards, including the latest EU Annex 1 guidelines for sterile manufacturing, strengthening Ardena's capabilities as a pharmaceutical CDMO in nanomedicine production.

Novartis and Ratio enter a radiotherapeutics partnership worth $745m

Novartis and Ratio Therapeutics have entered a $745M global license and collaboration agreement to develop a radiotherapeutic targeting somatostatin receptor 2 (SSTR2) for cancer, including neuroendocrine tumors, breast cancer, and head and neck carcinoma. The partnership leverages Ratio's technology platforms to optimize radiopharmaceuticals for therapy and imaging, enhancing drug availability and tumor targeting. Novartis will lead development, manufacturing, and commercialization after preclinical candidate selection. Ratio will receive up to $745M in upfront and milestone payments, along with tiered royalties. This alliance aims to advance targeted radiotherapies with improved pharmacokinetics and tumor efficacy.

J&J and Protagonist share promising phase 3 results for icotrokinra in plaque psoriasis

Johnson & Johnson (J&J) and Protagonist Therapeutics reported positive phase 3 trial results for icotrokinra (JNJ-2113), a targeted oral peptide for moderate-to-severe plaque psoriasis. In the ICONIC-LEAD trial, 64.7% of icotrokinra-treated patients achieved clear/almost clear skin (IGA 0/1) and 49.6% showed ≥90% improvement (PASI 90) at week 16, significantly outperforming placebo. Improvements continued through week 24. Similar success was seen in the ICONIC-TOTAL trial. Icotrokinra selectively blocks IL-23, a key factor in psoriasis-related immune response. Despite 125 million global cases, many eligible patients lack access to advanced therapies, underscoring the significance of these findings for unmet treatment needs.

Roche expands cell therapy capabilities with $1.5bn Poseida acquisition

Roche is acquiring Poseida Therapeutics in a $1.5 billion deal, gaining access to its allogeneic CAR-T cell therapy portfolio and platform technologies. This builds on their partnership to develop off-the-shelf CAR-T therapies for hematological malignancies. Poseida's lead candidate, P-BCMA-ALLO1, targets multiple myeloma and has shown a 91% overall response rate in a Phase 1 trial, with 100% efficacy in BCMA-naïve patients. The acquisition includes $9 per share plus milestone payments, valuing Poseida at $1 billion upfront. This move aligns with Roche's strategic focus on advancing cell therapies across oncology, immunology, and neurology, following its recent $850 million deal with Regor Pharmaceuticals.

Novartis' Kisqali receives EC approval to treat early breast cancer patients

The European Commission (EC) has approved Novartis' Kisqali (ribociclib) as an adjuvant therapy for early-stage hormone receptor (HR)-positive, HER2-negative breast cancer patients at high risk of recurrence. Based on positive NATALEE trial results, Kisqali combined with endocrine therapy reduced disease recurrence risk by 25.1% compared to endocrine therapy alone, benefiting stage 2 and 3 patients, including those node-negative. This CDK4/6 inhibitor already treats advanced breast cancer. Novartis aims to address unmet needs and improve outcomes for a broader patient group. Recent collaborations include Ratio Therapeutics ($745M) and Schrödinger ($2.4B+) to advance oncology-focused therapeutics.

FDA recommends collecting ovarian toxicity data in cancer drug trials

The FDA has issued draft guidance urging cancer drug developers to collect ovarian toxicity data during clinical trials involving premenopausal adults. This stems from concerns that cancer treatments may lead to infertility, early menopause, and increased risks of cardiovascular disease. Released on 26 November, the guidance emphasizes including ovarian toxicity as a safety endpoint in trials and incorporating it into drug development for premenopausal patients. The FDA highlights a significant data gap regarding long-term reproductive and health impacts on patients post-treatment, underscoring the need for clinical measures and biomarkers to better understand and address these risks in cancer drug development.

AstraZeneca announces $3.5bn US investment to boost research and manufacturing

AstraZeneca (AZ) will invest $3.5bn in the US by 2026 to expand its R&D and manufacturing footprint, including a new R&D center in Cambridge, Massachusetts, and a biologics facility in Maryland. The funds will enhance cell therapy manufacturing on both coasts and specialty manufacturing in Texas, creating over 1,000 skilled jobs. CEO Pascal Soriot highlighted the US's innovation and talent as key drivers. This investment aligns with AZ's ambition to achieve $80bn in revenue by 2030, fueled by growth in oncology, biopharmaceuticals, and rare diseases, and the launch of 20 new medicines. AZ also plans a $1.5bn ADC facility in Singapore.

New injection offers hope for asthma and COPD attacks

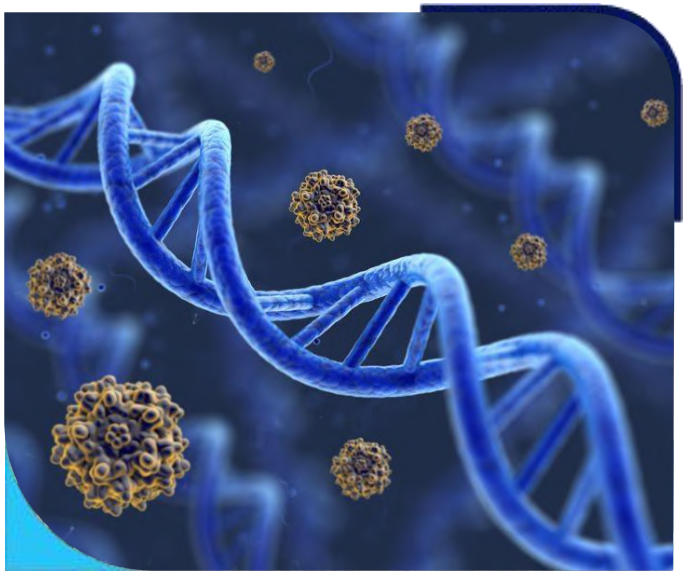
A new injection treatment for asthma and COPD attacks, benralizumab, has shown to reduce the need for further care by 30%, offering a potentially groundbreaking improvement over traditional steroid tablets. Published in The Lancet Respiratory Medicine, the study highlights the injection's effectiveness in targeting eosinophilic exacerbations, responsible for nearly 50% of asthma and 30% of COPD attacks. These conditions cause significant morbidity and mortality, with the NHS spending £5.9 billion annually on their management. Developed by researchers at King's College London and the University of Oxford, the phase 2 ABRA trial demonstrated improved respiratory symptoms and quality of life.

The European Commission (EC) has approved Pfizer's Hympavzi (marstacimab) to treat hemophilia A or B in adult and pediatric patients

Pfizer's Hympavzi (marstacimab) has been approved by the European Commission to treat hemophilia A or B in patients aged 12 and older, weighing at least 35kg, without factor inhibitors. As the first anti-tissue factor pathway inhibitor approved in the EU, it also features a convenient pre-filled auto-injector pen. Administered once weekly via subcutaneous injection, it reduces tissue factor pathway inhibitor levels, enhancing thrombin generation for better blood clotting. The approval follows positive BASIS trial results, showing a 35% reduction in annualized bleeding rates compared to routine prophylaxis. This breakthrough offers significant benefits over frequent intravenous infusions in managing hemophilia.

FDA Approves First Gene Therapy for Treatment of Aromatic L-amino Acid Decarboxylase Deficiency

The FDA has approved Kebilidi (eladocagene exuparvovec-tneq), the first gene therapy for aromatic L-amino acid decarboxylase (AADC) deficiency, a rare genetic disorder impairing neurotransmitter production, causing motor, developmental, and cognitive delays. Administered via pediatric stereotactic neurosurgery, Kebilidi delivers gene therapy directly to the brain, restoring AADC expression and increasing dopamine production. In clinical trials, it improved gross motor function in 8 of 12 pediatric patients, showing promise compared to untreated cases. Adverse effects include dyskinesia, fever, and procedural complications. Approved under Accelerated Approval, ongoing trials aim to confirm its long-term clinical benefits and treatment durability.



Stay tuned for more such updates in the coming months!

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