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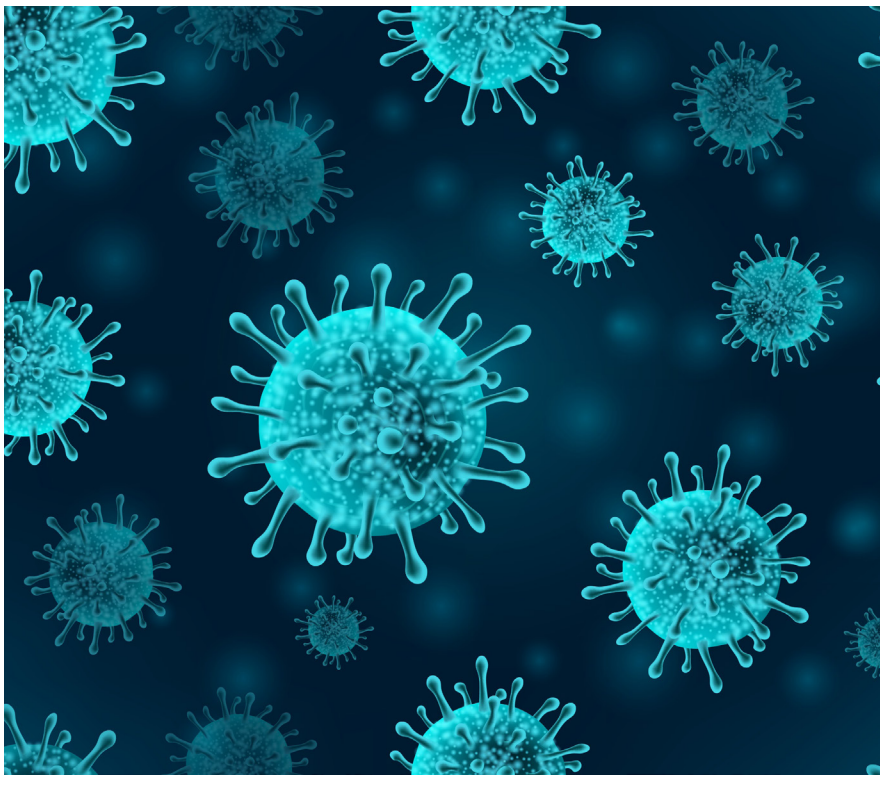
FDA approves first oral antiviral to treat adult patients with COVID-19



The US Food and Drug Administration (FDA) has announced that it has approved the oral antiviral Paxlovid (nirmatrelvir tablets and ritonavir tablets, co-packaged for oral use) for the treatment of adult patients with mild-to-moderate COVID-19 if they are at high risk for progression to severe COVID-19, including hospitalisation or death.

The approval of Paxlovid marks the fourth approval of a drug to treat COVID-19 in adults, although this is the first oral antiviral pill to be approved for this indication.

The drug has previously been manufactured and packaged under the emergency use authorisation (EUA) and distributed by the US Department of Health and Human Services, and this will remain available in order to ensure that access remains consistent for adults, as well as for paediatric patients between 12 and 18 who are not covered by this approval.

Patrizia Cavazzoni, MD, director of the FDA's Center for Drug Evaluation and Research, commented: "While the pandemic has been challenging for all of us, we have made great progress mitigating the impact of COVID-19 on our lives. Today's approval demonstrates that Paxlovid has met the agency's rigorous standards for safety and effectiveness, and that it remains an important treatment option for people at high risk for progression to severe COVID-19, including those with prior immunity. The FDA remains committed to working with sponsors to facilitate the development of new prevention and treatment options for COVID-19."

Future Focus

NICE recommends nine digital treatment options for depression and anxiety

The National Institute for Health and Care Excellence (NICE) has recommended six digitally enabled therapies for adult patients with anxiety disorders and three digitally enabled therapies for adult patients with depression for use within the NHS while further data is gathered.

It is thought that these therapies delivered via digital technology will free up clinical resources as they require less therapist time than standard care, meaning the time and resources can be used elsewhere within the NHS to increase access or reduce waiting times.

Digital therapies for depression on average need 90 minutes with a therapist compared to eight hours required in standard care. Similarly, digital therapies for anxiety require four hours of clinician or practitioner time compared to ten hours of standard care.

A formal assessment with an NHS Talking Therapies clinician or practitioner will be required prior to starting digital treatment in order to ensure the correct intervention is matched to clinical needs and the patient's preferences.

Mark Chapman, interim director of medical technology and digital evaluation at NICE, commented: "We know NHS Talking Therapies services are in demand and people are facing waits of several weeks. A part of the solution could be the use of digitally-enabled therapies recommended by our committee, which could increase the number of people receiving the treatment they need sooner. […] One of our priorities is to get the best care to people fast while at the same time ensuring value for money for the taxpayer – these digitally-enabled therapies do both. […] Every person seen by an NHS Talking Therapies clinician or practitioner is assessed so their needs can be fully understood. The choice of a digitally-enabled therapy must be the right one for the individual, ensuring that they get the care they need."

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Welcome to the July/August issue of *Pharmafocus*!

This issue covers the latest news, from a new study finding that the NHS may miss out on new drugs due to rising costs (page 4) and the European Parliament sharing its goal to tackle antimicrobial resistance (page 4), to the US Food and Drug Administration approving Pfizer's RSV vaccine (page 8) and scientists at McMaster University and MIT using AI to find new antibiotics (page 10).

Along with the latest pharma news, this issue includes an article from Galimedix Therapeutics in which Alexander Gebauer considers the link between Alzheimer's disease and age-related macular degeneration and how the treatment landscape for both diseases may develop in the future (page 16). Another article in this issue sees Ameet Nathwani from Dewpoint Therapeutics explain the use of biomolecular condensates and how they could change the treatment landscape for diabetes (page 14).

Finally, I examine how the ongoing cost of living crisis is impacting our health, where the crisis stemmed from and advice that charities have shared for maintaining good health in times of financial hardship (page 18).

As the weather heats up I hope you find time to relax in the sun and enjoy this issue, and we'll be back with the September edition of *Pharmafocus* at the end of August!

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Report finds NHS may miss out on new drugs due to rising costs

Figures from a team at the University of York, the London School of Economics and Political Science (LSE), and the London School of Hygiene and Tropical Medicine (LSHTM) have revealed that the total cost of prescription medicines to NHS England reached a new high of £17.2bn in 2021-22, and that since 2018, NHS spending on branded medicines have been increasing by over 5% annually.

Much of that growth is due to increased spending on hospital-prescribed medicines – this rose 35% from £6.7bn to £9.1bn between 2018 and 2022.

The Voluntary Scheme for Branded Medicines Pricing and Access (VPAS) sets an annual cap on the total allowed sales value of branded medicines to the NHS. Under the current scheme, which ends in 2023, the cap grows 2% annually, and any sales above the cap are paid back to the government. However, there is much contention between the pharmaceutical companies and the NHS around the VPAS, with companies arguing that the VPAS, in its current form, may limit or delay the availability of new medication in the UK.

The report contends that it would be costly for pharma companies to remove themselves from the UK market as the NHS has historically been a reliable market for them. It also raises points such as the Medicines and Healthcare Products Regulatory Agency (MHRA) being one of the fastest regulators globally; the National Institute for Heath and Care Excellence (NICE) recommending most new medicines it appraises to the NHS; and that new drugs are already exempt from VPAS for three years after their launch.

Dr James Lomas, from the University of York's Department of Economics and Related Studies, said: "We have to face the reality that the NHS does not have unlimited resources and the more money we spend on medicines, the less money we have for other medicines, treatments and services that already offer significant health benefits in the NHS."

Beth Woods, from the University of York's Centre for Health Economics, said: "Incentivising the development of new medicines is important, but the right balance needs to be struck given other NHS priorities – especially at a time when budgets are tight. Doing this requires sharing the value of medicines between providing rewards to the pharmaceutical industry and generating health benefits for patients in the NHS. The pharmaceutical industry is currently getting too big of a slice of the pie."

European Parliament shares goal to tackle antimicrobial resistance

The European Parliament has shared its recommendations for a 'coordinated EU response to health threats posed by antimicrobial resistance,' according to its press release.

Members of the European Parliament (MEPs) voted 525 in favour of measures to control this threat, with two voting against and 33 abstentions. Following this vote, MEPs say that "the successful tackling of antimicrobial resistance requires the prudent use of antibiotics for humans and animals, good infection prevention and control measures, and more research and development into novel antimicrobials and alternatives to antimicrobials."

It has also been agreed that if the above measures are insufficient then further EU-level legislation will become necessary.

EU countries are called on to implement and regularly update 'National Action Plans' to tackle antimicrobial resistance at least every two years, which is intended to become a priority for their national health systems.

Other measures include addressing antimicrobial consumption and support for research and prevention of medicines shortages, details of which are outlined in the European Parliament's press release.

EU member states were expected to adopt these measures in mid-June 2023.

FDA relaxes blood donation regulations for MSM

The US Food and Drug Administration (FDA) has released updated guidance for blood donation, which relaxes the rules for donations from men who have sex with men (MSM). This follows decades in which MSM have been rejected or discouraged from donation blood due to a fear of HIV transmission.

The new guidance has removed the time-based deferrals and screening questions aimed specifically at MSM and women who have sex with MSM, instead utilising individual risk-based questions that will be asked of every potential donor as a means to reducing the risk of transmitting HIV.

This policy change means MSM will now be eligible to donate blood as long as they meet the new donor criteria, and all people who have had sex with a new sexual partner or more than one sexual partner in the last three months will have to defer. Also anyone taking medication to treat or prevent HIV infection, such as antiretroviral therapy (ART), pre-exposure prophylaxis (PrEP) or post-exposure prophylaxis (PEP) will be deferred, despite the fact that these medications are safe and effective.

Peter Marks MD PhD, director of the FDA's Center for Biologics Evaluation and Research, commented: "The FDA has worked diligently to evaluate our policies and ensure we had the scientific evidence to support individual risk assessment for donor eligibility while maintaining appropriate safeguards to protect recipients of blood products. The implementation of these recommendations will represent a significant milestone for the agency and the LGBTQI+ community. The FDA is committed to working closely with the blood collection industry to help ensure timely implementation of the new recommendations and we will continue to monitor the safety of the blood supply once this individual risk-based approach is in place."

GRAIL and University of Oxford showcase first prospective study results for multi-cancer early detection test

US-based healthcare company GRAIL and the University of Oxford, UK, have announced encouraging first prospective study results at the American Society of Clinical Oncology (ASCO) Annual Meeting 2023 for their multi-cancer early detection (MCED) test for patients who were referred for diagnostic follow-up for suspicion of cancer.

SYMPLIFY is the first large-scale evaluation of an MCED, enrolling 6,238 patients aged 18 or older in England and Wales who were referred for urgent imaging, endoscopy or other diagnostic tests to investigate potential gynaecological, lung, lower or upper gastrointestinal tract (GI), or non-specific cancer symptoms. The most commonly reported symptoms included: unexpected weight loss (24.1%); change in bowel habit (22%); post-menopausal bleeding (16%); rectal bleeding (15.7%); abdominal pain (14.5%); pain (10.6%); difficulty swallowing (8.8%); and anaemia (7.1%).

The MCED test's predictions were compared with diagnoses obtained from traditional methods or investigated. It detected a cancer signal in 323 people, of which a cancer diagnosis was made in 244 – a positive predictive value (PPV) of 75.5%, negative predictive value (NPV) of 97.6%, and a specificity of 98.4%. The overall sensitivity was 66.3%, with a range from 24.2% in stage I cancers to 95.3% in stage IV, that also increased with age.

Sir Harpal Kumar, president of GRAIL Europe, stated: "GRAIL's earlier PATHFINDER study previously demonstrated that adding GRAIL's MCED testing to standard of care screening more than doubled the number of cancers detected compared with standard screening alone in adults with no symptoms or suspicion of cancer. Now, the SYMPLIFY data confirm the potential benefit of methylation-based MCED blood tests as a diagnostic aid for use in the symptomatic patient population. These exciting results will inform our development of an optimised classifier for use in symptomatic patients with a suspicion of cancer."

Merck invests €35m in biosafety testing in Scotland

Merck has announced that it has invested €35m in biosafety testing at its sites in Glasgow and Stirling, both Scotland. Biosafety testing is a crucial step in the drug development process, which ensures that new drugs are safe, efficacious and meet any regulatory requirements.

It is thought that this investment and the subsequent expansion will create approximately 500 new jobs, with the centrepiece of the investment taking the form of a new 1,200-square-metre facility in Glasgow, which will be home to molecular biology and sequencing services.

The company also plans to expand testing capability in its current buildings, including biosafety testing, analytical development and viral clearance suites. This investment follows other similar investments in Maryland, US, and Shanghai, China, both of which have seen recent testing expansions.

Dirk Lange, head of life science services at Merck Life Science, commented: "We remain committed to ensuring the safety of the world's medicines through our state-of-the-art testing solutions for our customers around the world that drive new treatments. Since mid-2022, we have announced investments of more than €350 million in our global testing network to meet the growing demand for these services."

Davis McClelland, site head and managing director for Merck's Scottish sites, added: "Merck has a wealth of testing expertise spanning over 75 years, including 33 years here in Scotland. The biosafety testing services at our sites in Glasgow and Stirling have been experiencing strong, double-digit growth for several years. This investment is a significant announcement for Merck in Scotland and an acknowledgment of the great work of our on-site teams."

More infections from contaminated eyedrops in US increase death toll

The US Centers for Disease Control and Prevention (CDC) have released updated data regarding contaminated eyedrops. As of May 2023, 81 patients in 18 states have tested positive for VIM-GES-CRPA, a rare strain of drug-resistant *Pseudomonas aeruginosa*.

In February 2023, 55 reports of eye infections, permanent vision loss and one death led to the US Food and Drug Administration (FDA) issuing a recall for EzriCare Artificial Tears and Delsam Pharma's Artificial Tears. The FDA then investigated Global Pharma's manufacturing plant in Chennai city, India, where it discovered several violations.

Some of these violations included failure to provide adequate, tamper-evident packaging, and the distribution of drugs without correct preservatives. The CDC confirmed a matching strain of *P. aeruginosa* in opened bottles to the one found in specimens taken from patients.

In March 2023 the CDC revealed 68 patients had been infected, eight people had vision loss, four underwent enucleation (surgical removal of the eyeball) and three people had died. As of May 2023, 14 people have suffered vision loss and four people have died.

The CDC recommends purchasers and prescribers of the eye drops to "immediately stop using and discard EzriCare Artifical Tears, Delsam Pharma Artifical Tears and Delsam Pharma Artificial Ointment." The FDA has asked "health professionals and patients to report adverse events or quality problems with any medicine to FDA's MedWatch Adverse Event Reporting programme."

AstraZeneca announces positive results from phase 3 trial for Tagrisso plus chemotherapy

AstraZeneca has announced positive results from the FLAURA2 phase 3 trial, which showed that Tagrisso (osimertinib), in combination with chemotherapy, demonstrated a statistically significant and clinically meaningful improvement in progression-free survival (PFS) when compared to Tagrisso alone in the treatment of patients with locally advanced (stage IIIB-IIIC) or metastatic (stage IV) epidermal growth factor receptor-mutated (EGFRm) non-small cell lung cancer (NSCLC).

The drug's safety results and discontinuation rates due to adverse events remained consistent with previously established profiles of the medicine.

Full data from the trial is expected to be presented at a forthcoming medical meeting, after which it will be shared with global health authorities.

Pasi A Jänne MD PhD, medical oncologist at Dana-Farber Cancer Institute and principal investigator for the FLAURA2 trial, commented: "As the global standard of care for EGFR-mutated non-small cell lung cancer, osimertinib monotherapy has transformed the treatment landscape allowing many patients the opportunity to achieve improved survival. FLAURA2 provides compelling evidence that the addition of chemotherapy to osimertinib can provide a new option for patients and clinicians that further improves outcomes compared to osimertinib alone and as such, can further delay treatment resistance and disease progression."

Susan Galbraith, executive vice president of Oncology R&D at AstraZeneca, added: "These significant FLAURA2 results show Tagrisso has the potential to offer patients in the first-line setting a new treatment option that can extend the time they live without their disease progressing. This meaningfully builds on successive trials which have demonstrated improved clinical benefit with Tagrisso in patients with EGFR-mutated lung cancer."

Sanofi announces positive phase 2 data for MS drug frexalimab

French pharmaceutical and healthcare company Sanofi has announced positive trial data from its phase 2 clinical study into its relapsing multiple sclerosis (MS) drug.

Frexalimab (SAR441344) is a second-generation investigational monoclonal anti-CD4OL antibody. It is believed to block the co-stimulatory CD40/CD40L cellular pathway necessary for adaptive (T- and B-cells) and innate (macrophages and dendritic cells) immune cell activation and function, without lymphocyte-depletion.

The phase 2 study was a randomised, double-blind, placebo-controlled trial, which evaluated the drug in patients with relapsing MS. In part A, there were 129 participants, which were randomised to receive either higher or lower doses of frexalimab or the matching placebo for 12 weeks. Open-label part 2, which is ongoing, saw the patients receiving the placebo switch to the respective frexalimab arm after 12 weeks.

The primary endpoint was indicated as the reduction in the number of new GdE T1-hyperintense MRI brain lesions after 12 weeks of treatment – at week 12, patients achieved an 89% decrease in the higher dose group and 79% in the lower dose. Both groups also showed reductions in new or enlarging T2-lesions and total GdE T1-lesions.

Erik Wallström MD PhD, global head of Neurology Development at Sanofi, said: "Building on our 20 years of research and development in MS, we are committed to growing our robust pipeline of MS therapies by exploring multiple treatment approaches with unique mechanisms of action (MOAs) that have the potential to slow or halt disability, which remains one of the greatest unmet medical needs in MS today."

Gavin Giovannoni MD PhD FCP FRCP FRCPath, chair of Neurology at Blizard Institute, Barts and The London School of Medicine and Dentistry, Queen Mary University of London commented: "Frexalimab has a unique mechanism of action, blocking the CD40/CD40L co-stimulatory pathway thought to regulate both adaptive and innate immune cell activation and function – a pathway that is pivotal in the pathogenesis of MS. We are thrilled with the results achieved with frexalimab in just three months, which shows that CD40L inhibition rapidly controls MS disease activity without lymphocyte depletion."

US-based Bellerophon shares top-line data from phase 3 trial of INOpulse for fILD

Bellerophon Therapeutics has announced top-line results from its phase 3 REBUILD trial evaluating the safety and efficacy of INOpulse for the treatment of fibrotic Interstitial Lung Disease (fILD). The trial did not meet its primary endpoint related to the change in moderate to vigorous physical activity (MVPA), however INOpulse was safe and well tolerated, remaining consistent with its overall safety profile.

The randomised, double-blind, placebo-controlled phase 3 study aimed to assess the safety and efficacy of INOpulse for patients with fILD; 145 patients were enrolled and treated with either INOpulse or a placebo. The primary endpoint of change in MVPA was not met, with patients treated with INOpulse performing worse than placebo by 5.49 minutes per day. The secondary endpoints demonstrated minimal difference between the two groups.

However, INOpulse was well tolerated and there were no safety concerns; this remains consistent with the established safety profile from prior phase 2 studies.

Peter Fernandes, Bellerphon's chief executive officer, commented: "The REBUILD study did not match the outcomes we saw in the exploratory phase 2 study in this patient population; however, the overall outcome of this pivotal validation study is conclusive and we do not see a path forward for continuing the REBUILD trial. On behalf of Bellerophon, I would like to thank all the patients, clinical trial sites and investigators for participating and supporting the conduct of this pivotal study, allowing us to bring closure to the REBUILD clinical study."

ISA Pharmaceuticals highlights positive clinical data at ASCO

Clinical stage biotechnology company ISA Pharmaceuticals has announced positive phase 1 data at the American Society of Clinical Oncology (ASCO) Annual Meeting for its combination therapy to treat recurrent and/or metastatic human papilloma virus type 16 (HPV16) positive oropharyngeal cancer (OPC).

The combination includes ISA101b (peltopepimut-S) and Regeneron's anti-PD-1 Libtayo (cemiplimab) and it was trialled in 26 patients who had progressed on pembrolizumab or nivolumab – including people who had not responded to previous anti-PD1 therapy – for six months. Patients were treated until disease progression, toxicity, treatment withdrawal for up to 24 months.

The ISA101b and Libtayo combination resulted in an overall response rate (ORR) or 15.4%, with long-term disease stabilisation being achieved in 26.9% of all patients.

Dr Anthony Kong, principal investigator and medical oncologist at King's College in London, said: "The initial results of this study are promising, given the high unmet medical need in this difficult-to-treat patient population."

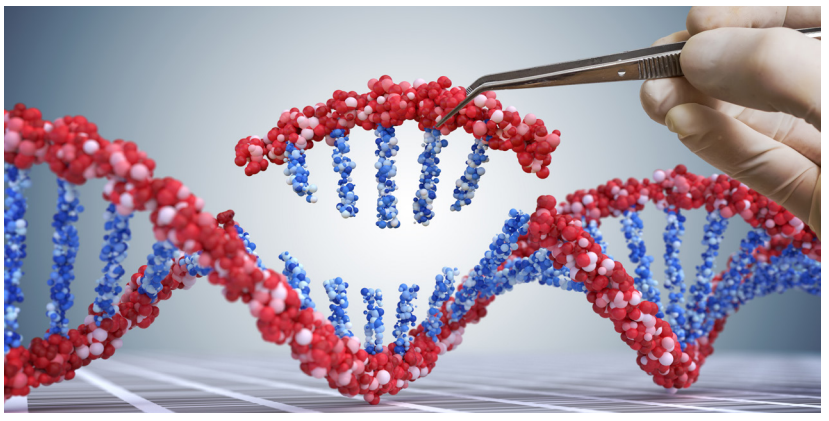
Leon Hooftman, chief medical officer of ISA Pharmaceuticals, said: "The most impressive element of this data set of anti-PD1 therapy-resistant head and neck cancer patients is the six months' Disease Control Rate (DCR): it appears that the targeted therapeutic cancer vaccine ISA101b together with an anti-PD1 antibody can have a real stabilising effect in a fair proportion of these sick patients."

SNIPR Biome reports positive findings for first-in-human, CRISPR-based microbial gene therapy

Danish CRISPR-based microbial gene therapy company SNIPR Biome has announced positive interim data from its phase 1 clinical trial with SNIPR001.

SNIPR001 is a CRISPR-armed phage therapeutic that targets and removes *Escherichia coli*, including antibiotic-resistant strains, from the human gastrointestinal tract. It is initially being looked at for patients with haematological malignancies who are undergoing haematopoietic stem cell transplants and are vulnerable to blood infections caused by the translocation of *E. coli* from the gut.

The study tested SNIPR001 in three levels of doses in 36 healthy people, with an aim to study the safety profile, SNIPR001 recovery and pharmacodynamics of the drug. The trial showed that oral dosing over seven days was well tolerated and that SNIPR001 numerically lowered gut *E. coli* levels. SNIPR001 could also be recovered in faeces from treated individuals in a dose-dependent manner.



Dr Christian Grøndahl, CEO and co-founder of SNIPR Biome, commented: "We are thrilled with these positive interim results from our phase 1 clinical trial of SNIPR001, which provide clinical validation for this innovative treatment. With the combined killing effects of bacteriophages and CRISPR-Cas technology, SNIPR001 has demonstrated the ability to target and eliminate antibiotic-resistant *E. coli* strains in the gut, providing a safe alternative to traditional treatments that do not work against antibiotic-resistant strains, while sparing the rest of the gut microbiome. This is a significant milestone in our mission to develop groundbreaking solutions in the fight against antimicrobial resistance, and we look forward to advancing SNIPR001 through further clinical studies to learn more and ultimately, we hope, to improve patient outcomes."

RadioMedix and Orano Med complete patient enrolment for neuroendocrine cancer trial

US-based RadioMedix and French Orano Med, two clinical stage radiopharmaceutical companies, have announced that the last patient has been enrolled in the phase 2 trial of their targeted alpha emitter therapy.

The therapy, AlphaMedix (212Pb-DOTAMTATE), is indicated for use in peptide receptor radionuclide therapy (PRRT) of naïve patients with somatostatin receptor-expressing neuroendocrine tumours (NET), regardless of the location of the tumour.

Forty-one patients with histologically confirmed NETs and positive somastostatin analogue imaging who had not received prior PRRT are enrolled in the phase 2, multicentre, single-arm, non-randomised, open-label basket trial. The treatment consists of four cycles of AlphaMedix at eight-week intervals.

The primary endpoint of the trial is the safety and effectiveness of AlphaMedix, with efficacy endpoints consisting of objective response rate (ORR) using RECIST v1.1 criteria, progression-free survival (PFS) and overall survival (OS).

Ebrahim Delpassand MD, chairman and CEO of RadioMedix stated: "The completion of the phase 2 trial enrolment is a significant milestone in the clinical development of our innovative targeted alpha-emitter radiotherapy, AlphaMedix, and brings us one step closer to having this drug available to patients. Previous studies have shown targeted alpha therapy (TAT) with AlphaMedix is well tolerated. The preliminary efficacy data seen to date are very promising, particularly achieving the planned ORR endpoint. As the trial progresses, we believe the ORR could improve further. We look forward to reporting data on the study in 2024, which we believe will show that AlphaMedix will provide substantial benefit over currently US Food and Drug Administration (FDA) approved therapies for patients with metastatic or inoperable SSTR-expressing NETs."

GSK receives FDA file acceptance for Jemperli plus chemotherapy for treating endometrial cancer

GSK has announced that the US Food and Drug Administration (FDA) has accepted the supplemental Biologics License Application (sBLA) for Jemperli (dostarlimab) in combination with chemotherapy for the treatment of adult patients with mismatch repair deficient (dMMR)/microsatellite instability-high (MSI-H) primary advanced or recurrent endometrial cancer.

If the sBLA is approved for this patient population, this could mark the first meaningful frontline treatment advancement in decades for patients with primary advanced or recurrent endometrial cancer.

The FDA granted Priority Review to this application as well as assigning a Prescription Drug User Fee Act action date of 23 September 2023.

Currently, Jemperli is approved in the US as a monotherapy in the treatment of dMMR recurrent or advanced endometrial cancer that has progressed on or following a prior platinum-containing regimen. If this sBLA is approved it is likely that the drug could be used earlier in treatment alongside platinum-containing chemotherapy in this patient group.

Hesham Abdullah, senior vice president and global head of Oncology Development at GSK, commented: "We are excited about this initial filing for this potential new indication for dostarlimab in the patient population that demonstrated the strongest treatment effect in the phase 3 RUBY trial. Long-term outcomes for patients with primary advanced or recurrent endometrial cancer remain poor, and there is an urgent need to evolve the current standard of care, which is platinum-based chemotherapy. We look forward to working with the FDA and other health authorities as they review this application."

FDA approves Pfizer's RSV vaccine for older adults

Global pharmaceutical company Pfizer has announced that the US Food and Drug Administration (FDA) has approved its respiratory syncytial virus (RSV) vaccine for the prevention of lower respiratory tract disease (LRTD) caused by RSV in patients aged 60 years or older.

Abrysvo is a bivalent RSV prefusion F (RSVpreF) vaccine that is unadjuvanted and composed of two preF proteins that have been chosen to optimise protection against RSV A and B strains.

The FDA's decision was based on data from the global, randomised, double-blind, placebo-controlled phase 3 RENOIR (NCT05035212) trial, designed to assess the efficacy, immunogenicity and safety of a single dose of the vaccine. The trial is still ongoing, with interim results having been published in *The New England Journal of Medicine*.

Annaliesa Anderson PhD, senior vice president and CSO of Vaccine Research and Development at Pfizer, commented: "A vaccine to help prevent RSV had been an elusive public health goal for more than half a century. Today's approval is a monumental step forward in delivering on Pfizer's commitment to help alleviate the significant burden of RSV in higher-risk populations, which includes older adults. Abrysvo will address a need to help protect older adults against the potentially serious consequences of RSV disease. We are extremely grateful to the clinical trial participants, study investigator teams and our dedicated Pfizer colleagues for their roles in making this vaccine available."

Edward E Walsh MD, professor of Medicine at University of Rochester Medical Center and principal RENOIR investigator, said: "This past RSV season demonstrated the serious consequences and potential health risks this virus poses for older adults. Today's FDA approval of Abrysvo recognises significant scientific progress and importantly helps provide older adults potential protection against RSV and an opportunity to improve community health by helping prevent the disease."

Bristol Myers Squibb's NDA accepted by FDA

Global pharmaceutical company Bristol Myers Squibb (BMS) has announced that its New Drug Application (NDA) has been accepted by the US Food and Drug Administration (FDA) for its ROS1-positive locally advanced or metastatic non-small cell lung cancer (NSCLC) treatment repotrectinib.

Repotrectinib (TPX-0005, BMS-986472) is a next-generation, potentially best-in-class tyrosine kinase inhibitor (TKI), designed to improve the durability of response with favourable properties for human brain penetration to enhance intracranial activity.

The acceptance was based on results from the TRIDENT-1 study, a phase 1/2 open label, multicentre, first-in-human clinical trial. Phase 1 evaluated the safety, tolerability, pharmacokinetics and anti-tumour activity of repotrectinib. Phase 2 had a primary endpoint of overall response rate (ORR) as assessed by Blinded Independent Central Review (BICR) using RECIST v1.1, and secondary endpoints of: duration of response (DOR); time to response (TTR); progression-free survival (PFS); overall survival (OS); and clinical benefit rate (CBR).

Repotrectinib demonstrated high response rates and clinically meaningful durability, with the safety profile being manageable. The study remains ongoing for long-term outcomes and additional endpoints.

Jonathan Cheng MD, senior vice president and head of oncology development at BMS, commented: "Patients with ROS1-positive NSCLC face a rare disease with a significant unmet medical need given the limited durability of benefit and emergence of resistance to approved therapies. The FDA's acceptance of this application marks an exciting milestone on our journey to bring this next-generation TKI to patients. If approved, this would represent a potential best-in-class option for TKI-naïve patients and a potential first-in-class option for patients with ROS1-positive NSCLC who have been previously treated with TKI, and for whom there are currently no approved targeted therapies available. We are eager to continue working closely with the FDA on the review of this precision medicine, which has shown unprecedented level of durability of responses and robust intracranial responses in patients with ROS1-positive NSCLC."

FDA approves prescription nasal spray for opioid overdose treatment

The US Food and Drug Administration (FDA) has announced that it has approved Opvee, the first nalmefene hydrochloride nasal spray for the emergency treatment of opioid overdose in adults and paediatric patients over the age of 12.

The drug, when administered quickly, can reverse the effects of opioid overdose, including respiratory depression, sedation and low blood pressure, with this product providing 2.7mg of nalmefene into the nasal cavity. It is available by prescription and intended for use in healthcare and community settings.

Opvee's approval follows various safety and pharmacokinetic studies, as well as a study that assessed how fast the drug worked in people who use opioids recreationally. Some adverse reactions were reported, these included nasal discomfort, headaches, nausea, dizziness, hot flushes, vomiting, anxiety, fatigue, nasal congestion and throat irritation, pain in the nose, decreased appetite, skin redness and excessive sweating.

It was also noted that the use of nalmefene hydrochloride in patients who are opioid dependent can result in opioid withdrawal, including symptoms such as body aches, diarrhoea, fast heart rate, runny nose, fever, sneezing, goosebumps, sweating, yawning, nausea or vomiting, nervousness, restlessness or irritability, shivering or trembling, abdominal cramps, weakness and increased blood pressure.

Robert M Califf MD, FDA commissioner, commented: "The agency continues to advance the FDA Overdose Prevention Framework and take actionable steps that encourage harm reduction by supporting the development of novel overdose reversal products. On the heels of the FDA's recent approval of the first over-the-counter opioid reversal agent, the availability of nalmafene nasal spray places a new prescription opioid reversal option in the hands of communities, harm reduction groups and emergency responders."

FDA approves new insulin pump and algorithm software for enhanced automated insulin delivery

The US Food and Drug Administration (FDA) has announced that it has cleared the Beta Bionics iLet ACE Pump and the iLet Dosing Decision Software for people over the age of six with type 1 diabetes. The devices, along with a compatible and approved integrated continuous glucose monitor (iCGM), will form a system known as the iLet Bionic Pancreas.

The automated insulin dosing (AID) system will use an algorithm to decide and trigger insulin delivery. The algorithm removes the need for patients to manually adjust insulin pump therapy settings and variables, making it considerably easier to operate than other AID systems.

The FDA reviewed both the iLet ACE Pump and the iLet Dosing Decision Software through the 501(k) premarket clearance pathway, and granted approval to both.

Jeff Shuren MD JD, director of the FDA's Center for Devices and Radiological Health (CDRH), commented: "Today's action will provide the type 1 diabetes community with additional options and flexibilities for diabetes management and may help to broaden the reach of AID technology. The FDA is committed to advancing new device innovation that can improve the health and quality of life for people living with chronic diseases that require day-to-day maintenance like diabetes through precision medicine approaches."

FDA approves Abbott's Assert-IQ insertable cardiac monitor

Abbott has announced that the US Food and Drug Administration (FDA) has granted its clearance for the Assert-IQ insertable cardiac monitor (ICM), providing doctors with a new diagnostic option for the evaluation and long-term monitoring of people with irregular heartbeats.

The clearance adds to Abbott's existing portfolio of connected medical devices, most of which aim to help doctors to manage and treat their patients remotely.

Unlike most available ICMs, which only work for a few years, the new Assert-IQ has two battery life options of at least three or six years, providing better flexibility for doctors to monitor their patients in a traditional or longer-term setting.

The device uses Bluetooth to connect to a transmitter – usually the patient's mobile phone – where it then checks the patient's heart rhythms every 20 seconds, transmitting the results in real time to the clinic's portal. There is also an option for remote programming, meaning the clinician would be able to adjust the settings of the device, such as optimising performance and limiting any unnecessary transmissions remotely, which would reduce unnecessary clinic visits by the patient.

Dhanunjaya Lakkireddy MD, medical director of the Kansas City Heart Rhythm Institute, US, commented: "The Assert-IQ ICM is a significant advancement amongst the tools that are currently available for the diagnoses of irregular hear rhythms. Given that the device is small and is inserted just under the skin, patients can go about their daily lives, enjoying the activities that they love, and the ICM does the work. With Assert-IQ ICM's advanced algorithms, it can detect even hard-to-spot irregularities and help physicians determine a treatment course. It can be a very valuable tool both for short-term and long-term management of cardiac arrhythmia disorders."

Leonard Ganz MD, divisional vice president of medical affairs and chief medical officer of Abbott's cardiac rhythm management business, added: "As the incidence of abnormal heart rhythms like atrial fibrillation continue to rise, more doctors are turning to UCM technology to monitor their patients remotely to better detect the cause of symptoms that can impact overall health and quality of life. Until now, insertable cardiac monitors have allowed for remote monitoring of patients but lacked the longevity needed to monitor them long term. Abbott's Assert-IQ ICM offers physicians a connected health device that will help them provide the best care for their patients while making more accurate and informed treatment decisions."

FDA committee votes in favour of Pfizer's RSV vaccine for maternal immunisation

Pfizer has announced that the Vaccines and Related Biological Products Advisory Committee (VRBPAC) from the US Food and Drug Administration (FDA) has voted in favour of the company's respiratory syncytial virus (RSV) vaccine for maternal immunisation.

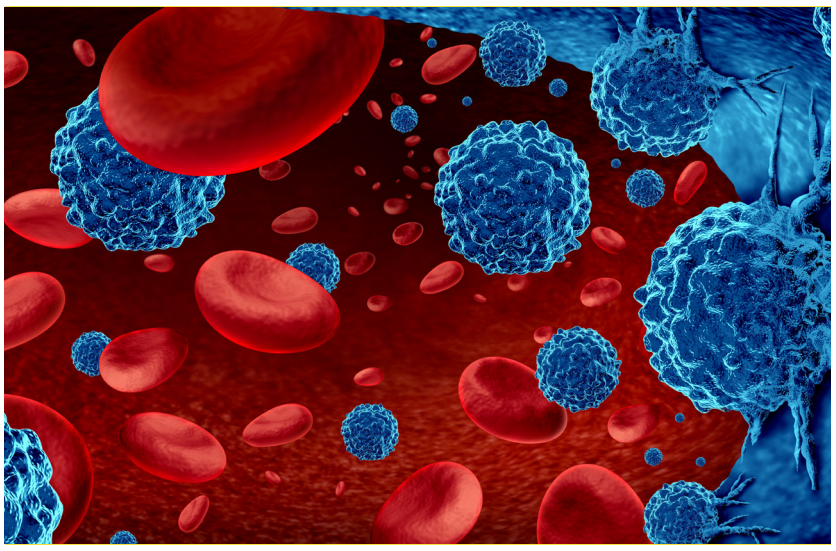
The committee voted 14 to 0 on effectiveness and 10 to 4 on safety, however the vaccine is currently still under FDA review for the prevention of medically attended lower respiratory tract disease (MA-LRTD) and severe MA-LRTD caused by RSV in infants from birth to six months of age through the immunisation of pregnant individuals.

The VRBPAC recommendation is based on Pfizer's evidence including primary analysis results from the phase 3 clinical trial MATISSE (MATernal Immunisation Study for Safety and Efficacy) that were announced in November 2022, and have recently been published in *The New England Journal of Medicine*.

Annaliesa Anderson PhD, senior vice president and chief scientific officer, Vaccine Research and Development at Pfizer, commented: "We are encouraged by the outcome of today's VRBPAC meeting as it is a critical step forward in the scientific community's long-sought-after goal to help prevent RSV disease in infants during their most vulnerable first six months of life. If approved, our RSV vaccine candidate has the potential to be the first maternal immunisation vaccine to help protect infants at first breath through their first six months of life from this potentially serious infection."

LaNova Medicines and AstraZeneca sign global exclusive licence agreement for preclinical ADC

Chinese biotechnology company LaNova Medicines and UK-based pharma company AstraZeneca have announced that they have signed a global exclusive licence agreement for a preclinical stage antibody drug conjugate (ADC).



LM-305 targets G protein-coupled receptor, class C, group 5, member D (GPRC5D). It consists of an anti-GRPC5D monoclonal antibody, a protease-degradable linker and a cytotoxic payload monomethyl auristatin E (MMAE). It is indicated for use in patients with multiple myeloma.

Within the agreement, AstraZeneca will be granted full exclusive global licence to research, develop and commercialise LM-305. LaNova will be eligible for upfront and near-term payments of up to $55m, as well as additional development and commercial milestone payments of up to $545m and tiered royalties on net sales.

Dr Crystal Qin, founder, chairman and CEO of LaNova Medicines commented: "LaNova Medicines has a strong focus on discovering and developing innovative medicines in the ADC and immuno-oncology fields. We're excited to reach this agreement with AstraZeneca. With the potential to become a first-in-class GPRC5D-directed ADC for multiple myeloma, LM-305 exemplifies our innovative and robust platform for ADC development. This agreement is further recognition of our exceptional pipeline assets and R&D capabilities. We are confident that AstraZeneca is the ideal company to advance LM-305 for the betterment of patients globally."

Nina Shah, global head of multiple myeloma, Haematology R&D at AstraZeneca, said: "We are pleased to have the opportunity to advance the development of LM-305, a novel GPRC5D-targeting ADC, as a potential new treatment option for relapsed/refractory multiple myeloma. LM-305 advances our leadership in ADCs and enriches our growing Haematology pipeline, helping us deliver against our broader ambition to transform clinical outcomes for patients living with blood cancers."

Scientists at MIT and McMaster University use AI to find new antibiotic to fight superbug

Scientists at McMaster University and the Massachusetts Institute of Technology (MIT) have utilised artificial intelligence (AI) in order to discover a new antibiotic, which, it appears, could be used to fight a drug-resistant 'superbug' that often circulates among vulnerable hospital patients.

The World Health Organization (WHO) has identified *Acinetobacter baumannii* as one of the world's most dangerous antibiotic resistant bacteria, which the researchers were attempting to treat.

Often, this bacterium is found in hospital settings, surviving on surfaces for long periods of time, as well as picking up DNA from other species of bacteria in its environment, for example antibiotic-resistant genes.

The study was published in *Nature Chemical Biology* and explains how the researchers utilised an AI platform to predict the structural classes of antibacterial molecules and ultimately identify a new antibacterial compound named abaucin.

Jonathan Stokes, lead author on the study and an assistant professor in McMaster University's Department of Biomedicine and Biochemistry, commented: "This work validates the benefits of machine learning in the search for new antibiotics. Using AI, we can rapidly explore vast regions of chemical space, significantly increasing the chances of discovering fundamentally new antibacterial molecules. […] We know broad-spectrum antibiotics are suboptimal and that pathogens have the ability to evolve and adjust to every trick we throw at them. AI methods afford us the opportunity to vastly increase the rate at which we discover new antibiotics, and we can do it at a reduced cost. This is an important avenue of exploration for new antibiotic drugs."

James J Collins, professor of medical engineering and science at MIT and Life Sciences faculty lead at the MIT Abdul Latif Jameel Clinic for Machine Learning in Health, added: "AI approaches to drug discovery are here to stay and will continue to be refined. We know algorithmic models work, now it's a matter of widely adopting these methods to discover new antibiotics more efficiently and less expensively."

Servier and Aitia enter into R&D collaboration for pancreatic cancer using Digital Twins

French pharmaceutical company Servier and US-based Causal AI and Digital Twins company Aitia have announced a collaboration to create Digital Twins as an aid to help treat pancreatic cancer. This collaboration builds on a previous multiple myeloma one that was announced in 2022.

Gemini Digital Twins brings together omics and patient data into an AI-powered system that is used to discover genetic and molecular pathways linked to clinical outcomes of a specific disease. It works by modelling what happens when a gene or protein is targeted, which can reveal new drug indications. The platform also allows the simulation of disease progression and response to drug therapies.

Under the terms of the agreement, Aitia and Servier will discover, validate and strive to develop novel drug targets through Aitia's Gemini Digital Twins and Servier's preclinical assays, therapeutic creation platforms and expertise. Servier will then receive an exclusive option to research, develop and commercialise products stemming from the collaboration.

Claude Bertrand, executive VP of Research and Development and chief scientific officer at Servier, stated: "I am convinced that scientific innovation combined with the power of digital technologies will help us bring new and innovative treatments faster to patients suffering from difficult and hard-to-treat cancers. Our expanded collaboration with Aitia will leverage discoveries from Gemini Digital Twins and harness the full potential of AI to make significant strides towards developing deeper biology of the disease and a better understanding of translational medicine questions such as biomarkers, patient stratifications or the discovery of drug targets. We believe this approach will generate novel mechanisms and hypotheses for potential new targeted therapies that can make a meaningful difference in the lives of patients with pancreatic cancer."

Colin Hill, CEO and co-founder of Aitia, commented: "Pancreatic cancer is one of the most devastating cancers that claims the lives of far too many people far too soon. Drug discovery and development efforts in pancreatic cancer to date have yielded very few treatment options for patients. We believe our Gemini Digital Twins, which are created from large quantities of multi-omic patient data and Causal AI, have the potential to bring significant disruption to the field of pancreatic cancer and pave the way for breakthrough discoveries. We're excited to collaborate with Servier to gain deeper insights into the underlying mechanisms driving this complex disease and accelerate the discovery of new therapies for people living with pancreatic cancer."

Asieris Pharmaceuticals and UroViu announce strategic collaboration to further develop integrated diagnosis and treatment for bladder

US-based biopharmaceutical company Asieris Pharmaceuticals and single-use endoscopic platform creator UroViu have announced that they have entered into a strategic collaboration to integrate the detection and treatment of bladder cancers.

As part of the collaboration, Asieris will obtain exclusive global rights to UroViu's patented portable single-use cystoscope, which it will use in combination with optical imaging agents to research the application of non-white light imaging technology in the diagnosis and postoperative monitoring of non-muscle invasive bladder cancer (NMBC). Asieris will also be responsible for the global commericalisation of UroViu's single-use cystoscope.

In 2021, the companies entered into a cooperation agreement where Asieris gained the exclusive registration and commercialisation rights of UroViu's single-use flexible white light cystoscopy system in mainland China, Taiwan, Hong Kong and Macau.

Dr Susan Wang, senior VP of Global Business Development and Strategic Partnership of Asieris, stated: "There is still a huge unmet need for more accessible and safer fluorescent cystoscopy in the diagnosis and surveillance of bladder cancer. We are thrilled to deepen and expand our collaboration with UroViu, which enables us not only to broaden our technology portfolio, but also to expedite the implementation of Asieris' integrated strategy for bladder cancer diagnosis and treatment, aiming to provide more effective disease management options for bladder cancer patients."

Bruce OuYang, founder and CEO of UroViu, commented: "We are excited about the possibilities that our expanded partnership with Asieris will create. Our unique and expanding portfolio of single use endoscopic products are fulfilling UroViu's promise to greatly improve both the patient and provider experience and elevate the standard of care in a field with rapidly advancing technologies."

US-based Nemours Children's Health selected to conduct first gene therapy for Morquio A syndrome

Nemours Children's Health, based in Delaware, US, has been chosen by the Foundation for the National Institutes of Health (FNIH) Accelerating Medicines Partnership Bespoke Gene Therapy Consortium (AMP BGCT) to conduct a first-of-its-kind gene therapy clinical trial for Morquio A syndrome.

Morquio A syndrome is a rare skeletal dysplasia caused by an inherited gene mutation. It affects one in 200,000 births and causes serious complications such as cervical spinal cord compression, short stature, flat feet, difficulty walking, tracheal obstruction, hearing loss and heart valvular disease.

FNIH AMP BGTC is a public-private partnership between the National Institutes of Health (NIH), US Food and Drug Administration (FDA), biopharmaceutical and life sciences companies and non-profit and other organisations, which has been created to help speed up the development and delivery of bespoke gene therapies. Currently, eight genetic diseases have been selected for clinical trials, where Nemours was selected for the Morquio A proposal, including being the clinical trial site.

For the rest of 2023, FINH and staff at Nemours will focus on refining the treatment protocol and hiring additional staff, with the aim to begin enrolment for the clinical trial in 2024.

Stuart Mackenzie MD, orthopaedic surgeon, director of the Skeletal Dysplasia Clinic, Nemours Children's Health, Delaware, commented: "The ultimate goal of our work is to help our patients. With the knowledge we gain during this trial, we believe Nemours Children's Health will be able to offer Morquio patients the newest and most innovative therapies available. Through this specialised public and private partnership with FNIH AMP, we are able to help realise our vision to create the healthiest generation of children."

Lonza to acquire Synaffix to strengthen ADC development

Global manufacturer for the pharmaceutical, biotech and nutraceutical markets, Lonza, has announced that it has acquired Synaffix, a biotech company focused on the commercialisation of its clinical stage technology platform for the development of antibody-drug conjugates (ADCs).

ADCs can be used to offer widespread and targeted treatment potential against cancer, however they present a variety of development and manufacturing challenges. Synaffix's technology platform is intended to enhance and extend Lonza's integrated ADC services.

This acquisition is intended to combine Lonza's development and manufacturing capabilities with Synaffix's ADC technology platform to provide customers and licensees with a comprehensive service, which aims to rapidly discover, develop, scale up and commercialise ADCs.

Ulrich Osswald, vice president of licensing at Lonza, commented: "The Synaffix ADC technology is the gold standard, helping clinical-stage developers to design potentially curative therapies in areas of high unmet medical need. The acquisition of Synaffix underlines the strategic position of bioconjugates within Lonza's portfolio, expands our offering in this fast-growing market and enhances our value proposition for clinical customers. With Synaffix, our combined industry-leading knowledge and capabilities have the capacity to support both clinical and commercial needs."

Peter van de Sande, Synaffix's CEO, added: "On our mission to bring Synaffix's ADC technologies to patients, we are excited to become part of Lonza and thus, through strong and immediate synergies, ensure a robust technology and manufacturing platform for our licensees as they progress into the late stage clinical and commercial development phase. We can now also leverage the potential offered by Lonza to fast-track technology innovations in bioconjugates beyond cytotoxic ADCs. We look forward to working closely with our colleagues at Lonza for the continued enablement of promising potential medicines for patients in need."

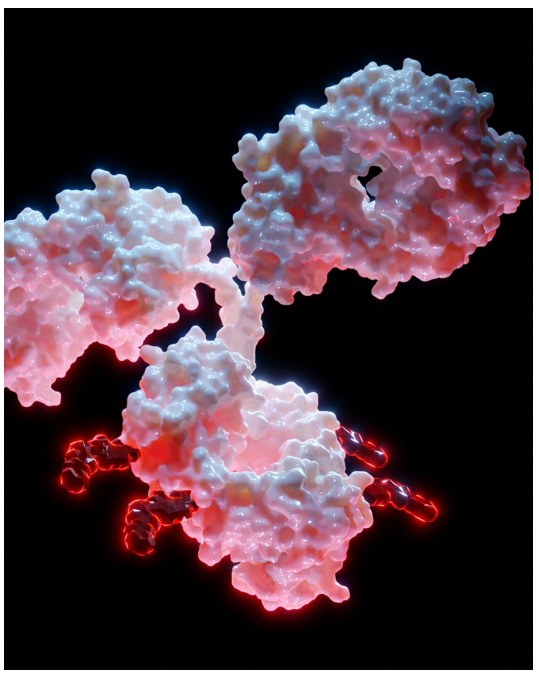
Astellas and Sony enter collaborative research agreement

Sony and Astellas Pharma have announced that they have entered into a collaborative research agreement in an attempt to discover a novel antibody-drug conjugate (ADC) platform in oncology, which will be based on Sony's polymeric material, KIRAVIA Backbone.

The collaboration aims to develop ADCs to selectively deliver anti-cancer drugs to target cells, increasing efficacy and reducing side effects often caused by anti-cancer cells attacking normal cells.

The two companies began exploring research into new linker technology to create a new ADC platform in July 2022, however this agreement now means they will develop and optimise a new ADC platform using the KIRAVIA Backbone as a linker. Astellas will conduct non-clinical trials of development candidates, and the two companies have agreed to continue discussions on expanding their research partnerships.

Katsunori Ogawa, head of the Life Science and Technology Business Unit at Sony Corporation, commented: "Sony's life science business has accumulated substantial knowledge in the field of cell analysis. Through this collaboration, Sony is striving to contribute to the medical and drug discovery fields and provide further social value by leveraging Sony's technological capabilities in the development of anti-cancer drugs therapy, which are expected to grow."



Yoshitsugu Shitaka PhD, chief scientific officer at Astellas Pharma, added: "We are pleased to enter into a joint research agreement with Sony. Astellas is working to create innovative drugs from a multifaceted perspective called the Focus Area approach, which identifies combinations of biology, therapeutic modality or technology and diseases with high unmet medical needs. The partnership will further strengthen our ability to utilise suitable modalities. It is our expectation that the collaboration will lead to the continuous creation of innovative drugs for patients around the world."

Pharmanovia and Stealth BioTherapeutics enter new licensing agreement for novel Barth syndrome treatment

Pharmanovia and Stealth BioTherapeutics have announced that they have entered into a new licensing agreement for the marketing and further development of elamipretide as a treatment for Barth syndrome in Europe and the Middle East and North Africa (MENA).

This agreement gives Pharmanovia the licensing rights to the drug in Europe and MENA, however it plans to work with Stealth on the final analyses of the studies demonstrating elamipretide's potential for this indication. Various clinical trials have already been carried out showing positive results and have demonstrated the drug's safety and efficacy, as well as its efficacy compared to control groups.

James Burt, Pharmanovia's CEO, commented: "This partnership is our third new chemical entity in as many months, further broadening our treatment portfolio, as we set our sights on improving the experience and lives of patients globally. […] We are delighted to have been recognised by Stealth BioTherapeutics as the right overseas partner to launch and potentially bring this novel treatment to patients in the EU, Switzerland, Norway, UK, Iceland and MENA. Beyond revitalising and adding value to iconic brands, utilising our extensive technical and commercial platform to address unmet needs with novel medicines that complement our existing portfolio across our core therapeutic areas, in this instance cardiology, is a key strategic priority for us. […] Only 5% or fewer rare diseases are estimated to have an approved treatment option, therefore we're especially excited to work with authorities and regulators on potentially bringing to market the first specific treatment option for those living with Barth syndrome."

Reenie McCarthy, Stealth BioTherapeutics' CEO, added: "We are committed to broadening global access to elamipretide for patients living with Barth syndrome. Partnering with Pharmanovia is a natural choice to achieve that goal given their experience in bringing medicines to patients across Europe and MENA and our mutual alignment on elamipretide's potential to address the unmet medical needs of patients living with Barth syndrome."

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What are biomolecular condensates, and will they be a breakthrough in diabetes treatment?

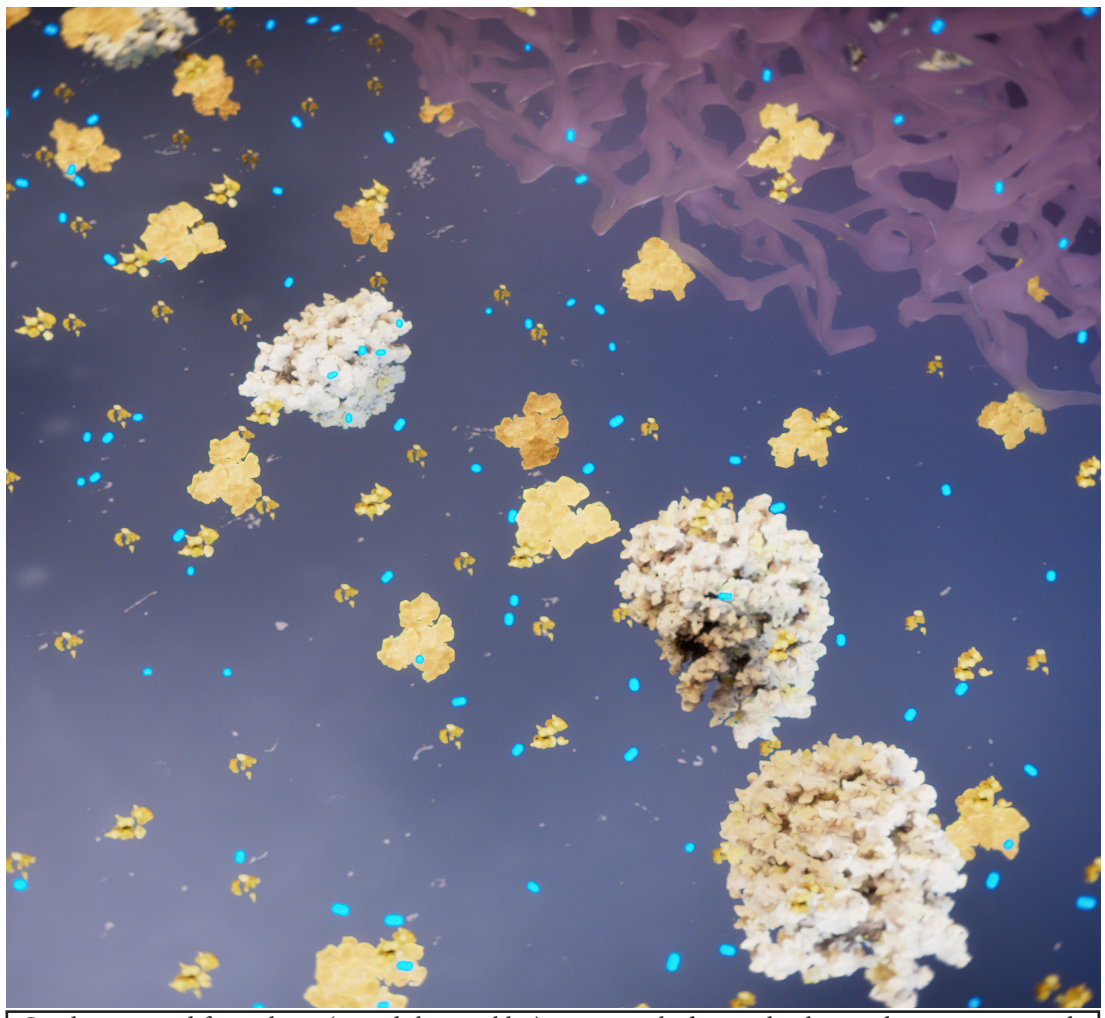
Ameet Nathwani at Dewpoint Therapeutics considers the current treatment landscape for both type 1 and type 2 diabetes, as well as evaluating the use of biomolecular condensates as a potential treatment option

***Pharmafocus*: What are the current standard-of-care treatments for type 1 and type 2 diabetes?**

Ameet Nathwani (AN): Type 1 diabetes, also known as juvenile diabetes or insulin dependent diabetes, is a chronic condition with its onset usually occurring in childhood, when the pancreas produces insufficient insulin due to an autoimmune destruction of the insulin-producing cells in the pancreas. Insulin is a vital hormone needed to allow sugar (glucose) to enter cells in the body to produce energy. Patients with type 1 diabetes require insulin replacement as their mainstay of treatment; this can be administered through injections or pumps and needs to be accompanied by frequent or continuous blood sugar monitoring.

Type 2 diabetes, by far the more common form of diabetes comprising 95% of all diagnoses, is also a chronic condition. Unlike Type 1 diabetes, it results from the body's inability to use the insulin effectively – a condition called insulin resistance – thereby leading to a relative insulin insufficiency. This is a progressive disease whereby the underlying insulin resistance not only leads to a progressive increase in blood sugar over time, but the insulin resistance itself contributes to additional complications including cardiovascular disease. One of the underlying risk factors for an increase in insulin resistance and type 2 diabetes is obesity, and in particular, central obesity – which is an excess accumulation of fat around key organs like the liver.

The mainstay of treatment in type 2 diabetes is a combination of intense lifestyle changes, regular blood sugar monitoring plus medication. The main first-line treatments include oral therapies such as metformin, but as the disease is relentlessly progressive, patients usually require multiple oral therapies over time including the addition of DPP-4 inhibitors, PPAR-gamma agonists and, in more recent years, the addition of SGLT2-inhibitors. However, these therapies are not considered disease modifying, and patients who continue to be poorly controlled, or who progress, will often transition to requiring insulin therapy or the addition of the newer class of injectable drugs such as GLP-1 agonists, which have shown promise in not only controlling blood sugar, but also in reducing weight and cardiovascular complications.



Condensate modifying drugs (c-mods here in blue) are uniquely designed to hit condensate targets and restore healthy condensate properties and function

Apart from weight loss surgery (bariatric surgery), which is reserved for the most severe patients, no treatment has shown the ability to truly reverse or prevent the progression of type 2 diabetes, which explains the high unmet need. Given the epidemic proportions of the disease (nearly 550 million patients worldwide) and nearly seven million deaths every year (one death every five seconds), the global unmet need to be able to prevent or modify the course of disease is very high.

***Pharmafocus*: What are biomolecular condensates and how could they improve current diabetes treatments?**

AN: A biomolecular condensate is a membrane-less compartment within a cell that forms when thousands of specific biomolecules, such as proteins and nucleic acids, come together in very specific ways to carry out specific

functions when the cell requires them. Condensates are often formed through a process called liquid-liquid phase separation – akin to the separation of oil and vinegar in vinaigrette – where certain molecules become more concentrated in a particular region of the cell, they compartmentalise and self-organise to carry out a myriad of cellular processes, such as gene expression, signal transduction and stress responses. Condensates are thought to be important for the majority of biological functions in the cell.

The discovery of biomolecular condensates has completely transformed our understanding of normal cellular function and organisation. But the most important realisation was that if these membrane-less compartments form to regulate normal function, dysregulation of certain biomolecular condensates could be the root cause of many diseases and could pave the way to developing novel therapies that target the aberrant condensate. For decades, scientists have been trying to discover the underlying cellular triggers that lead to the development of type 2 diabetes. We know that one of the major biological precursors to the onset of this form of diabetes is the development of insulin resistance, where the tissues in the body become more resistant to the effects of insulin, and the body, therefore, has to produce more insulin to have the same effect. Over time, this demand of higher insulin levels cannot be met and that's when the individual can transition to becoming diabetic. Research from MIT and Novo Nordisk found that the insulin resistance in tissues from patients with diabetes was caused by dysregulation of a condensate responsible for the efficient signaling of insulin in cells.

Industry collaboration in this area is focused on building upon these findings to discover what are the condensate dysregulations that drive the insulin resistance process, which then leads to diabetes. By doing so, we may finally be able to address a fundamental biological driver of type 2 diabetes and its progression. We are hopeful of being able to find small molecules or novel therapeutics that can modulate or normalise the insulin signaling process. If effective, these drugs could be added to any of the current therapies to improve glucose control and drastically slow down the progression of diabetes or, potentially, prevent the transition to full onset type 2 diabetes, if patients are diagnosed early enough in the prodromal or prediabetic stage.

***Pharmafocus*: How could AI be used to further expand the field of biomolecular condensates?**

AN: Companies can use AI extensively in an integrated way in discovery processes, leveraging AI to develop digital condensate signatures. Teams can then visualise these subcellular compartments, tagging the key components of condensates inside the cells and extracting several thousand features from these images, which can then be compared with a library of compounds. These can correlate the very subtle changes of these images to the desired functional change of the cell's response and can then quickly use this to find the chemical structures or even potential mechanisms that are causing favorable changes. From there, many companies will be using various AI tools to accelerate the optimal design of the molecules and their impact. Condensate images across multiple diseases and cell types and with thousands of compounds, help form the basis of a proprietary Knowledge Graph, which can be used to make predictions on linking genetics to condensates that are likely to be involved in diseases, and immediately predicting which types of compounds could be used to modulate the condensate, thereby accelerating the discovery process. The potential value of this Knowledge Graph to both academia and industry could be very significant, given the increasing realisation of the widespread involvement of condensates as central nodes of dysregulation in disease.

***Pharmafocus*: What are the possible obstacles to implementing biomolecular condensates as part of diabetes treatments?**

AN: The only specific obstacle to being able to implement a condensate-targeted drug for the management of diabetes will be having the physicians fully understand and appreciate the new biology and its implications, as this field is very new. However, as the drug development process is lengthy and we would need to generate the compelling clinical data through conventional trials showing the disease-modifying potential, there is plenty of time to both educate and demonstrate the utility of this approach.

***Pharmafocus*: How do you see the diabetes treatment landscape developing in the next five years?**

AN: It's a very exciting time in the diabetes field. The recent approvals of the GLP-1 agonist therapies in both diabetes and obesity have opened up the field of addressing the impact of obesity in cardio-metabolic disease. There are many trials looking at the longer-term impact of managing obesity on both cardiovascular disease and the development of diabetes, which could significantly change the landscape in the future.

The recent advent of the SGLT2 inhibitors with very promising effects on both cardiovascular and renal protection have also been a very welcome advance and are leading to interesting new scientific avenues to explore regarding the underlying mechanism of these benefits. Given the global magnitude of the diabetes problem, there is still a lot to do to really make an impact on the costs, complications and mortality of the disease.

Author Bio:

Since 2020, ***Ameet Nathwani*** *has been chief executive officer of* ***Dewpoint Therapeutics.*** *Nathwani has more than 25 years of experience in the pharmaceutical industry. Before joining Dewpoint, he was an executive committee member at Sanofi SA, where he also served as chief medical officer and chief digital officer and was responsible for enhancing Sanofi's strategy to integrate digital technologies and medical science to ultimately improve patient outcomes across the portfolio. Prior to that, Nathwani worked for over 11 years at Novartis Pharma AG, where he served as global head of medical affairs and as member of the pharma executive committee and held different senior development and commercial positions. Nathwani also worked in senior roles in R&D at GlaxoSmithKline, SmithKline Beecham and Glaxo Group Research in both the UK and US. He earned his medical degree from the University of London, specialised in cardiology and intensive care medicine, and holds a Master of Business Administration.*

Alzheimer's disease and age-related macular degeneration – how are they linked and what are the treatments?

Alexander Gebauer from Galimedix Therapeutics talks to *Pharmafocus* about the link between the two diseases and what the treatment landscape for both could look like in the future

***Pharmafocus*: How prevalent are Alzheimer's disease (AD) and age-related macular degeneration (AMD) both individually and as comorbidities?**

Alexander Gebauer: Alzheimer's Disease (AD) is the most common cause of dementia. The prevalence of AD increases with age, and it is estimated that about 5-8% of people aged 65 and older have AD worldwide. As the population ages, the number of individuals with AD is expected to rise.

Age-related macular degeneration (AMD) is a degenerative eye disease that affects the macula, the central part of the retina responsible for sharp, central vision. It can be classified into two main types: dry AMD and wet AMD, with dry AMD affecting approximately 80-90% of individuals with AMD. AMD is the leading cause of severe vision loss in individuals over the age of 50. Alongside AD, it is a disease of the elderly and, therefore, its prevalence increases with age. According to estimates, approximately 8.7% of the global population aged 45 and older has some form of AMD.

Current research on the comorbidity of AD and AMD is still limited. However, some studies suggest that there may be a higher prevalence of AMD among individuals with AD compared to the general population. Also, a recent study suggested that people who get AMD, cataracts and diabetes-related eye disease may have a higher risk for dementia.[1] In addition, detection of deposited amyloid beta in the retina is more and more accepted as a diagnostic marker for AD.

***Pharmafocus*: What are the symptoms of AMD and how can these symptoms be used as indicators for specific neurodegenerative diseases such as AD?**

AG: Symptoms of AMD may include:

- Blurred central vision: Objects in the centre of the visual field appear blurry, making it difficult to read, recognise faces or perform detailed tasks
- Distorted vision: Straight lines may appear wavy or bent, which is known as metamorphopsia. This can affect the perception of shapes and cause objects to appear distorted
- Dark or empty areas in the central vision: A blind spot or a dark spot may develop in the centre of the visual field, causing a loss of clarity and detail
- Reduced colour perception: Colours may appear less vibrant or washed out
- Difficulty adjusting to low light levels: The ability to adapt to changes in lighting may be reduced, leading to difficulties in seeing in dim environments.

These symptoms are specific to AMD and are related to the degeneration of the macula. It's important to note that they alone are not indicative of other specific neurodegenerative diseases such as AD. AMD primarily affects vision, while AD is a complex neurodegenerative disorder that primarily affects cognitive function. However, there is growing research interest in the potential links between the eyes and AD. The retina, located at the back of the eye, is considered an extension

> **A breakthrough in developing effective treatments for AMD that target a specific mechanism [...] could identify a promising therapeutic strategy for AD, too.**

of the central nervous system and shares similarities with brain tissue. Therefore, studying changes in the retina may provide insights into AD pathology and offer opportunities for early detection and monitoring of the disease and the other way around: Alzheimer's research can provide insights into retinal degeneration.

***Pharmafocus*: What is the current standard of care for AD and how can ophthalmic treatment options help in expanding the treatment of AD?**

AG: The current standard of care for AD involves a combination of pharmacological and non-pharmacological approaches to manage symptoms and slow disease progression.

On one hand, the primary pharmacological approach involves the use of cholinesterase inhibitors (donepezil, rivastigmine and galantamine) and an NMDA receptor antagonist (memantine). These drugs can help manage cognitive symptoms, such as memory loss and confusion, by modulating neurotransmitters in the brain.

Importantly, recent positive results with amyloid beta-targeting therapies have validated the importance of Aβ in treating AD. In June 2023, Lequembi, the first disease-modifying drug to treat AD was approved in the US, marking a new era in treating this disease. Aduhelm was previously approved but showed limited efficacy and significant side effects. Both of these drugs have to be infused, making their usability complicated. On the other hand, non-pharmacological interventions aim to improve the overall quality of life for individuals with AD. They include cognitive stimulation, physical exercise, social engagement and the creation of a supportive and structured environment. An increasing body of literature suggests that the same cell death mechanism – Aβ toxicity – is involved in both AD and retinal diseases. It therefore seems likely that a therapeutic approach showing a marked effect in one disease could also be of benefit against the other.

***Pharmafocus*: Given that there appears to be a connection between AMD and AD, when do you expect current research into AMD to have a decisive impact on research into AD treatments and how would this be implemented?**

AG: Both AMD and AD are age-related neurodegenerative diseases and there is a growing body of evidence suggesting that the neurodegenerative processes in the retina and the brain are very similar, potentially identical. Also, clinical epidemiological data points in this direction, showing that individuals with AMD may have an increased risk of developing AD or experiencing cognitive decline. Research into AMD and AD often share common ground due to the involvement of similar underlying mechanisms. Advances in understanding these shared mechanisms could potentially lead to insights and therapeutic strategies that may be beneficial for addressing both diseases.

If current research into AMD were to have a decisive impact on AD treatments, it would likely occur through the identification of shared pathways or targets. For example, a breakthrough in developing effective treatments for AMD that target a specific mechanism, such as reducing the formation of toxic amyloid beta oligomers and/or removal of existing toxic oligomers, could identify a promising therapeutic strategy for AD, too.

***Pharmafocus*: How do you see the treatment landscape for dry AMD and AD developing in the next five years?**

AG: *Dry Age-Related Macular Degeneration (dAMD):*

As one of the major factors for retinal cell death in dry AMD appears to be formation of toxic amyloid beta oligomers, therapeutic strategies that directly prevent the formation of these species and additionally remove existing oligomers, are seen as a highly promising approach. Nevertheless, clinical proof of concept still needs to be shown.

Another interesting approach that is being explored as a potential treatment is the use of complement inhibitors. These drugs aim to control the over-activation of the complement cascade, a part of the immune system suggested to have a role in dAMD. Large clinical trials have demonstrated that complement plays a certain role in the pathophysiology. The first anti-complement drug Syfovre has recently been approved by the FDA for the treatment of geographic atrophy secondary to AMD.

A second drug, Zimura, is expected to be approved by FDA this summer, with European approvals also expected soon. However, these drugs are applied by intraocular injection, which is burdensome for patients, and not acceptable for all. Therefore, a safe and effective non-injectable drug would offer a great improvement for patients. Ongoing clinical trials will provide insights into the efficacy and safety of these therapies in the coming years.

Alzheimer's Disease (AD):

Efforts to develop disease-modifying therapies for AD are ongoing. Several antibody drugs targeting beta-amyloid species and tau tangles are currently in clinical trials or have recently been approved, as previously mentioned. Within the next five years, we believe that we will gain a better understanding of the potential impact of these treatments on AD progression. However, even though big pharma is focused on the development of antibody therapies, they come with major and frequent side effects, such as headaches, changes in mental state and confusion, due to antibody-typical brain swelling and micro-bleedings (ARIA) seen in brain imaging, that limit their therapeutic window. Also, antibody-based therapeutics are expensive. Alternatively, small molecules might offer a certain advantage regarding patient safety and could be economically more accessible for patients.

Early detection and intervention is key for the treatment of AD. Researchers are currently focused on identifying biomarkers and developing reliable diagnostic tools to detect AD at its earliest stages. This may enable intervention before significant neuronal damage occurs. Advances in neuroimaging, blood tests and cerebrospinal fluid analysis may contribute to improved early detection methods.

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Author bio:

***Alexander Gebauer** is co-founder and executive chairman of **Galimedix Therapeutics.** A medical doctor by training, Gebauer has spent over 30 years in the pharmaceutical industry in various R&D leadership roles at international pharmaceutical companies, including Sun Pharmaceuticals, Hoechst AG, Aventis, Sanofi and Merz Pharmaceuticals. During his time at Merz, he served as CSO, head of Global Research & Development and was directly involved in developing GAL-101 and GAL-201, which are now being further advanced at Galimedix.*

The impact of the cost of living crisis on health

Betsy Goodfellow from *Pharmafocus* looks at the reasons for the current cost of living crisis and the resulting impact on health

Various factors, including the war in Ukraine and the COVID-19 pandemic, have caused food prices, energy bills and other costs to soar over the last year, resulting in a 'cost of living crisis'. Salaries are failing to rise with inflation, leaving many struggling to make ends meet. As well as the obvious financial concerns of such a crisis, there is also the added worry of how this may be impacting people's health.

According to the Office for National Statistics (ONS), in the UK, the price of consumer goods and services increased at the fastest rate in the last four decades in the year leading up to October 2022, and the annual inflation rate rose to 7.9% in the year to May 2023 from 7.8% in the year to April 2023.[1] In addition, private rental prices increased by 5% in the year to May 2023, up from 4.8% in the year to April 2023, marking the largest annual percentage change since the ONS began recording this data in January 2016.[1]

But how have these increases impacted people's health?

According to a recent poll commissioned by the Royal College of Physicians (RCP), 55% of the British population feel that their health has been negatively impacted by the rising cost of living,[2] with 84% citing increased heating costs, 78% citing the rising cost of food and 46% citing rising transport costs.[2]

Discussing the results of this poll, Dr Andrew Goddard, president of the RCP, said: "The cost of living crisis has barely begun so the fact that one in two people is already experiencing worsening health should sound alarm bells, especially at a time when our health service is under more pressure than ever before. […] We can't continue to see health inequality as an issue for health directives to solve. A cross-government approach to tackling the underlying causes of ill health will improve lives, protect the NHS and strengthen the economy."[2]

As the cost of living increases, this impacts health in various ways. People in lower income groups are less able to buy good quality, nutritious food, turning instead to lower cost food that can be of a lower quality. As of April 2022, there was an 11.5% increase in ready meal sales, suggesting that in times of financial burden people are turning to cheap and convenient food.[3] These pre-prepared meals not only save money, as purchasing raw ingredients can be costly, but also limit preparation costs, as a few minutes in the microwave is much less expensive than powering an oven or hob for long enough to cook a meal. It was also estimated in April 2022 that 7.3 million adults and 2.6 million children were 'food insecure', meaning they lacked reliable access to affordable and nutritious food.[4] It is clear that the cost of living crisis has led to an increase in food insecurity and a decrease in the quality of food that people are eating, and there is an obvious link between this and poorer health.

Additionally, heating prices have risen exponentially; between January and March 2023 inflation for gas and electricity reached 129.4% and 66.7% respectively,[5] with 56% of people reportedly using less fuel in their homes as a result.[5] Although this saves money, it was estimated in 2022 that when household temperatures in Britain drop below 18°C, each

> **The cost of living crisis is indisputably a health crisis too, and as the public calls on the government for assistance, help and advice are available from various charities.**

one degree drop in temperature corresponds to 3,500 additional deaths.[6] A lack of heating can make various underlying conditions worse, such as cardiovascular disease, asthma, respiratory infections and mental health issues, and can also make it more difficult to sleep. It is known that sleep is important for maintaining good health.[6]

AgeUK suggests batch cooking food to conserve energy rather than turning to less nutritious convenience food; having showers instead of baths and lighting only the room that is in use.[7] In addition, in winter, keep windows and doors shut, wear layers and keep moving to help reduce energy consumption.[7]

As well as these direct health impacts, the cost of living crisis is placing further strain on the already over-stretched NHS, with growing numbers of hospital admissions and appointments for those with health problems triggered by the increased cost of living.[7] The junior doctors and nurses strikes have also had an effect on the NHS's ability to cope with this rise in patient numbers.

Commenting on the cost of living crisis, Julian Tang, a clinical virologist at the University of Leicester, UK, said it "will culminate in higher admissions to the NHS for the exacerbation of these chronic conditions due to the cold, inability to heat their homes and inability to eat enough – with possible malnutrition, particularly in children, as people have to buy cheaper and less healthy foods due to rising food bills – and the additional burden of returning seasonal respiratory virus infections".[8]

The cost of living crisis is indisputably a health crisis too, and as the public calls on the government for assistance, help and advice are available from various charities.

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Move of the month

Simon Sinclair appointed as chief medical officer of Ondine Biomedical

Canadian life sciences company Ondine Biomedical has announced that it has appointed Dr Simon Sinclair as chief medical officer. This appointment comes as Ondine prepares to start its US phase 3 trial of its nasal photodisinfection technology.

Sinclair has over 20 years of pharma and medtech experience across translational medicine, clinical development, medical affairs, market access and medical safety and vigilance. He has worked as a non-executive director on Ondine's board of directors since 2021.

Previously, Sinclair has held senior positions at a myriad of major pharma and medtech companies, including Johnson and Johnson Medical Devices and Merck and Co (MSD in the US).

Commenting on his appointment, Sinclair stated: "Having been closely involved with Ondine at board level, I am excited to join the company in an operational capacity at this critical stage, as we plan for entry into the US phase 3 trial and see growing interest and adoption in other key markets. […] Multidrug-resistant infections are an existential global healthcare threat, and there has never been a more important time to focus on new, non-antibiotic solutions. I look forward to working with the Ondine team to bring its photodisinfection technology through the FDA regulatory process in the US and continuing to build usage and implementation across the countries where we already have approval, including Canada, EU and UK."

Ken Mariash appointed as Sinaptica's chief executive officer

Sinaptica Therapeutics has announced the appointment of Ken Mariash Jr as chief executive officer, bringing with him more than 25 years of life sciences leadership experience in strategy, corporate development and marketing across both medtech and biotech.

Mariash's experience spans roles at CSL Behring, Baxter BioScience and Boston Scientific, a decade of this experience being in the neuromodulation sphere.

Prior to joining Sinaptica, Mariash worked as founder and managing partner of Rubicon Strategy Partners, vice president of marketing at EBT Medical, director of strategy and new markets for the neuromodulation division of Boston Scientific. Before this he held roles in strategy and business development at Baxter BioScience (now known as Takeda), as well as working at CSL Behring and Charles River Associates in his early career.

Mariash commented: "I'm excited to take on this leadership role at Sinaptica at a time when our mechanistic understanding of AD pathology is evolving – the world is starting to appreciate new targets and new approaches like neuromodulation, which have the potential to significantly bend the curve of disease progression. After decades of drug failures, we applaud the recent progress of those targeting amyloid; however there remains a massive unmet need for safe, effective – and perhaps complementary – approaches."

Rich Macary, president of Sinaptica Therapeutics, added: "As Sinaptica prepares for a pivotal clinical trial of our closed-loop neuromodulation therapy, Ken is the ideal choice to lead the company through this important stage. He brings a rare combination of drug, device, neuromodulation, and AD experience together with a broad skill set ranging from product development to capital equipment commercialisation. We have incredible confidence in Ken's ability to take Sinaptica through the development stage toward commercialisation, and that his leadership will guide the company to realise the full potential of this transformative therapy to impact the lives of patients with Alzheimer's disease."

Immodulon appoints Josefine Roemmler-Zehrer as chief medical officer

Immodulon, a late-stage clinical company developing highly differentiated cancer immunotherapies to prime a patient's own immune system, has announced that it has appointed Dr Josefine Roemmler-Zehrer as chief medical officer.

Roemmler-Zehrer brings with her a strong track record of experience in clinical and medical affairs, with specific experience in drug development and leadership roles at pharmaceutical companies such as Celgene, Amgen and Ipsen. She has been involved in several successful product development programmes including Celgene's Abraxane for pancreatic cancer and non-small cell lung cancer and Ipsen's Cabometyx in renal cell carcinoma and thyroid cancer. She has also led teams spanning various disciplines in multiple therapeutic areas including oncology, inflammation, rheumatology, dermatology, neurology and rare diseases.

Gertjan Bartlema, chief executive officer of Immodulon, stated: "I am delighted to welcome incoming Josefine to the new leadership team at Immodulon as we prepare to advance our lead asset IMM-101 into a pivotal trial. Josefine brings a wealth of global experience in clinical and medical affairs from leading pharmaceutical companies and will be instrumental in our efforts to unlock the full potential of IMM-101. We look forward to benefiting from Josefine's insights and leadership as we finalise the design of our pivotal trial in pancreatic cancer and advance our mission to improve the lives of patients suffering from this difficult-to-treat cancer."

Commenting on her appointment, Roemmler-Zehrer added: "I am so pleased to be joining Immodulon at this pivotal time in the company's development. I have been excited and inspired by the new management's energy and commitment to bring IMM-101, a new, transformative, broad-spectrum immunotherapy to patients with pancreatic cancer. Pancreatic cancer has the highest mortality of any solid tumour and patients have few meaningful treatment options. I believe that IMM-101 can make a major contribution in addressing this fatal disease."

Engimmune Therapeutics appoints Annalisa D'Andrea to board of directors

Swiss biotech Engimmune Therapeutics has announced that it has appointed Dr Annalisa D'Andrea to its board of directors as a non-executive director, effective from 1 June 2023.

D'Andrea brings with her over 25 years of experience in the life sciences industry from immunology research and drug discovery and development to strategic leadership in biotechnology and pharmaceutical companies. She is also a venture partner at Longwood Fund and was previously president and chief scientific officer of ImmuneID. Before this she worked as chief scientific officer at Kiniksa Pharmaceuticals and vice president and global head of discovery for immunology and inflammation at Roche. She has also previously held various executive roles at SRI International, as well as working at Chiron Vaccines early in her career.

Bent Jakobsen PhD, chairman of Engimmune's board of directors, commented: "We are delighted to welcome Dr Annalisa D'Andrea to the board with her extensive proven experience of managing and growing novel healthcare start-up companies, developing important treatments to help patients. Her expertise and significant drug discovery experience will be invaluable as we continue to further develop our novel pipeline of soluble, multi-specific T-cell receptor therapies."

D'Andrea, added: "I am delighted to be joining Engimmune Therapeutics at an exciting and important phase of development as the Company strives to develop new multi-specific TCR therapies for the treatment and benefit of patients with cancer and autoimmune diseases. I look forward to working with the team and using my vast experience to assist with advancing its proprietary platform technologies through discovery and development of highly potent and specific TCR-based therapeutic products."

Five facts about income and health

1. As of 2019/20, 31% of adults under the age of 55 in the UK on the lowest tiers of income report 'less than good' health, including 10% reporting 'bad' or 'very bad' health, compared to 22% in those with middle incomes and 12% of those with high incomes.[1]
2. The percentage of people in poverty who report having 'less than good' health is higher in all age groups than those not in poverty; in the 16-24 age group, the gap is 10%; this widens to 15% in the 25-34 and 35-44 age groups and 19% in the 45-54 age group.[2] This gap remains even if unemployed people are not included, indicating that the gap does not stem from those in poor health being in poverty, as they cannot work due to their health.[2]
3. In 2019, deaths in the most deprived 10% of local areas were 1.76 times higher for men and 1.77 times higher for women than in least deprived 10% of areas.[3] In 2020, this disparity widened, with men in the most deprived areas 1.92 times more likely to die and women 1.83 times more likely to die than in the least deprived areas.[3]
4. Higher life expectancy is linked with higher average income; an increase in household income of £1,000 per annum is associated with a 0.7-year increase in female healthy life expectancy and a 0.5-year increase in male healthy life expectancy.[4,5]
5. As of 2020/21, 20.3% of people in the UK live in poverty, equating to approximately 13.6 million people.[6] This has a clear impact on health as those living on lower incomes struggle to afford basic necessities, especially in the winter, such as heating, nutritious food and warm clothing.[6]

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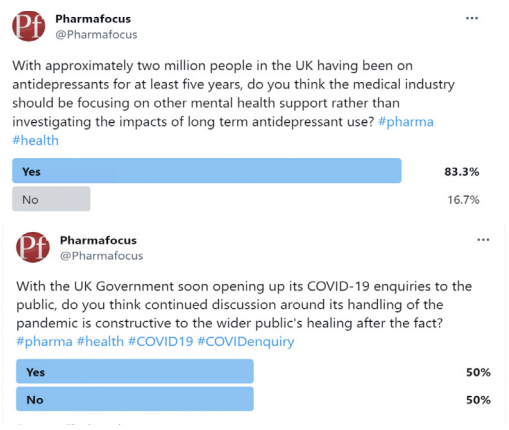

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