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NICE recommends more digital mental health therapies for NHS use

NICE has reccommended eight new digital therapies to be used by the NHS for the treatment of various mental health disorders

Eight more digital therapies (DTx) for mental health disorders have been recommended by NICE for use by the NHS, following a push from the health technology assessment (HTA) agency to utilise treatment options which can relieve pressure on the health service.

With one in six people experiencing common mental health problems, including anxiety or depression, the burden on the NHS is huge, so new treatments to reduce this burden and alleviate symptoms for the patients can only be a positive step.

The eight new DTx have the potential to help up to 40,000 patients, according to NICE, covering conditions including depression, anxiety, post-traumatic stress disorder (PTSD) and body dysmorphia.

The new therapies draw on cognitive behavioural therapy (CBT) techniques, and have now received conditional backing from NICE until further evidence around their use can be compiled.

The tools can only be used by patients following a formal assessment by an NHS talking therapist.

Mike Chapman, interim director of medical technology and digital evaluation at NICE, suggested that the DTx have "demonstrated […] the potential to provide effective treatments to the many thousands of people who live with these conditions."

Elizabeth Mullenger, a lay specialist member of NICE's appraisal committees, commented: "It can be incredibly isolating to be on a long waiting list for in-person treatment. You might know that help is coming, you just don't know when. […] Having access to a digital therapy could help prevent this lonely feeling. Sometimes, people need support most in the middle of the night, or after a busy day at work, and its hard to know where to turn. Having access to digital therapy can give people the help they need, when they need it."

Future Focus

iNtRON confirms efficacy of 'Inhalation Anthrax'-fighting drug

iNtRON, a bio-new drug developing venture company, has announced the completion of an efficacy evaluation study of BAL200. It has previously been awarded Orphan Drug Designation (ODD) by the FDA.

BAL200 is a novel bacteriophage-derived endolysin-based biologic for the treatment of 'Inhalation Anthrax'. It provides complete bactericidal activity, rapidly eradicating infected anthrax strains from the body.

The study evaluated BAL200's antibacterial activity against various anthrax strains, in particular its lytic activity for how quickly it lyses the target strain. It found that BAL200 had the most potent bactericidal ability than any other Post-Exposure Prophylaxis (PEP) antibiotics on the market.

iNtRON has stated its intention to promote an out-licensing process based on the efficacy

evaluation results and has already secured safety data. It also plans to conduct required studies to be subject to the Animal Efficacy Rule with a new partner afterwards.

Dr Kang, Sang Hyeon, CTO of iNtRON said, "We believe that the recently secured efficacy results are very important and required data to seek a licensing partner for the collaboration of the further developments. We will prepare a data package that emphasises the competitiveness of BAL200 well so it leads to the successful out-licensing deal… Since the existing drugs act in a way that inhibits the physiological mechanism of bacteria, the effectiveness of the existing drugs is not rapid enough to prevent occurrence of bacterial resistance, and is limited by the relationship between the number of drug molecule and bacteria. On the other hand, BAL200 provides a non-stoichiometric characteristic due to its completely different MOA, which makes BAL200 to be a drug that can provide robust therapeutic effects with only a small amount of dose."

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This month has seen some exciting developments in pharma, from Scottish researchers using waste molecules from certain types of algae to cure the common cold (page 5) and Ethicon undertaking the first robot-assisted kidney stone removal (page 9), to the bankruptcy petition filed by a unit of Johnson & Johnson (page 8) and AI research that discovered a drug candidate for liver cancer in only 30 days (page 12).

In this issue we bring you an interview with Amarin's Scott Curley that focuses on cardiovascular disease (CVD). CVD is the current leading cause of death worldwide and in the article Scott talks about the lessons learned during COVID-19 about the best ways to support patients with CVD (page 14).

Also in this issue, Nikunj Desai from ACG explains the importance of efficient supply chains in the pharma industry and the resulting improvements (page 17), while Rhian Linney from Galapagos UK and Ireland shares her thoughts about the vital role of the patient voice in autoimmune diseases and the work the company is doing in this area, and its focus on rheumatoid arthritis (page 16).

I hope you enjoy this issue.

Betsy Goodfellow

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Cambridge's Experimental Cancer Medicine Centre to receive funding

The Experimental Cancer Medicine Centre (ECMC) in Cambridge is one of a selection of organisations to receive a share of £47.5m over the next five years to research and develop new cancer treatments.

The funding is part of a partnership between Cancer Research UK, the NIHR, the Little Princess Trust and 17 adult ECMCs, including those in Belfast, Birmingham, Cardiff, Edinburgh, Glasgow, Leicester, Liverpool, Manchester, Newcastle, Oxford, Southampton and five in London.

There is also a further £2.2m set aside for research at 12 paediatric ECMCs across the UK, with a total of £6.6m for this research over the next five years. This funding is expected to employ new research staff, such as nurses and data managers, according to Cancer Research UK.

Dr Iain Foulkes, executive director of research and innovation at Cancer Research UK, commented: "We are proud to be supporting an expansion of our successful ECMC network, bringing together vast medical and scientific expertise to translate the latest scientific discoveries from the lab into the clinic. […] The ECMC network is delivering the cancer treatments of the future, bringing new hope to people affected by cancer. […] The trials taking place today will give the next generation the best possible chance of beating cancer."

Health Minister Helen Whately, added: "A cancer diagnosis can be devastating but the earlier the diagnosis, the better the chance to treat it and beat it. We are already picking up more cancers early by screening but we can do even better. […] This partnership between Cancer Research UK, the National Institute for Health and Care Research and the Little Princess Trust will fund innovative trials that could lead to new life-saving treatments. […] Every life lost to cancer is devastating and I'm pleased that across the country, people will be given renewed hope – especially children and young people – that we can beat this awful disease."

Eli Lilly and AbbVie leave UK VPAS; blow to UK pharma industry

Eli Lilly and AbbVie have announced their departure from the voluntary scheme for branded medicines pricing and access (VPAS), causing worry that NHS patients may miss out on breakthrough drug treatments.

VPAS is an agreement that was set up in 2019 between the Government, NHS and medicine manufacturers designed to limit the cost of drugs for the health service while supporting industry innovation. It caps the health services branded medicines bill, which means that all drug manufacturers would face a charge if the bill rose more than 2% annually.

However, the charge has risen too rapidly for many companies over the recent years. As of December 2022, the payback rate was set at 26.5% – before the pandemic, the payback rate was about 5%.

Dr Richard Torbett, the chief executive at the Association of the British Pharmaceutical Industry (ABPI), said that demand for new medicines has grown "much faster than industry pre-pandemic projections – driving up the repayment rates far beyond sustainable levels" owing to the record backlog of patients waiting for NHS treatment. The current VPAS deal means that "despite demand for branded medicines rising sharply, driven by clinical decisions and patient need, the money spent on them has declined by 14% in real terms over the last decade," he added.

Because of this, Eli Lilly and AbbVie have become the first to leave the VPAS in favour of an alternative statutory scheme, which will be imposed by law and have higher repayment rates. The ABPI has said that this highlights the "depth of feeling" over the failures of VPAS.

This has raised fears that NHS patients will struggle to access cutting-edge treatment in the future, as firms may feel deterred from investing in the UK.

Laura Steele, Eli Lilly's president and general manager for northern Europe, said: "We simply cannot stay signed up to a scheme, which has such a punishing impact on innovation. We want to see action on a new settlement that allows life sciences to thrive in the UK now and over the long term, to boost investment in the UK and ensure patients here can benefit rapidly from cutting-edge clinical trials and medicines."

Merck's London research hub to begin construction in April 2023

Merck has been planning a $1.3bn, 220,000 square foot research hub in London for six years but will finally begin construction in April this year, according to Mace, the newly appointed principal contractor.

The new hub is set to be located within Belgrove House on Euston Road, near King's Cross railway station. This sets Merck within what is known as London's Knowledge Quarter.

The new research facility is expected to house 800 researchers and office staff, including people working in marketing, finance and administration. The majority of these staff will be acquired from the company's existing sites around the country, however the development will open up 120 new jobs for scientists and technicians.

It is expected that researchers at the new facility will undertake early-stage research into age-related diseases, making it the only one of Merck's labs to conduct this work outside of the US.

The facility will also be home to an education and outreach centre, a publicly accessible auditorium, a step-free entrance from King's Cross as well as an educational outreach programme collaboration between Merck and Camden STEAM.

Merck's press release states that "it has been designed using sustainable materials and includes an innovative double-skin façade to reduce solar gain. Carbon emissions generated through construction, operation and future fit-out will be reduced."

NHS to offer artificial pancreas to type 1 diabetes patients

The NHS will soon be offering an artificial pancreas to more than 100,000 people with type 1 diabetes across England and Wales. It is hoped the device will help them manage their condition more effectively, improving quality of life and reducing risk of long-term health issues.

The device is a combination of an insulin pump and a continuous glucose monitor (CGM). A sensor is placed under the skin, which automatically measures blood glucose levels. These readings are then sent to a pump, which calculates the amount of insulin required and administers it. All of this is tracked through an app, where patients can also input food intake to make the process more accurate.

Both insulin pumps and CGMs are used now, however at the moment the rate of delivery on the pump needs to be manually adjusted. This means regular checking of the patient's glucose levels by finger prick, CGM or flash glucose monitor.

Although NICE is recommending the device, it says that a cost-effective price is still needed to be negotiated with the manufacturers. At the moment, the cost-per-device is £6,000, but NICE wants to agree on a price that will be "fair to taxpayers".

Professor Partha Kar, a national specialty adviser for diabetes at NHS England, said, "This technology has been proven to give the best control for managing type 1 diabetes and should make things like amputations, blindness and kidney problems possibly a thing of the past. The quality of life this technology gives to those using it is huge."

Scottish biotech aims to cure common cold

Scottish biotech company ScotBio has announced that it has seen "promising initial results" from its attempts to extract a cure for the common cold from algae.

The Livingston-based company is assessing how waste molecules from Spirulina, an alga often consumed as a superfood, could be used as a cure for the common cold, COVID-19 and various other viruses.

This research comes in collaboration with the University of Edinburgh, Robert Gordon University and the Industrial Biotechnology Innovation Centre (IBioIC). The researchers are aiming to utilise the antiviral properties of the alga's waste molecules.

Spirulina is currently used in the production of various other products including dietary supplements and food colourants. Separating the molecules of this substance into different chemical groups allows researchers to identify which molecules have more efficient antiviral properties to treat viruses such as COVID-19, the common cold or influenza.

Joe Palmer, ScotBio's development manager, commented: "Working with the project consortium has enabled us to fractionate Spirulina into distinct chemical groups and then screen the extracts in a safe environment. Through that, we have identified compounds that could be particularly effective against a broad range of viruses from what was previously seen as waste. We can now work on recovering and purifying these molecules as high-value ingredients."

"The next step for us is to better understand the market opportunities for these molecules and to fully resolve the relationship between molecule chemistry and their antiviral mechanisms," Palmer continued. "Our aim is for this project to be a catalyst that will unlock a large and untapped market, helping in the fight against common viruses and future pandemics."

UCL to begin new trial to assess cough medicine's impact on Parkinson's disease

Researchers led by a team at University College London (UCL) have began researching the impacts of ambroxol, a common ingredient in cough syrup, as a treatment for Parkinson's disease. The team is expected to launch a phase 3 clinical trial to assess the efficacy of this treatment in 330 patients with the disease.

Ambroxol works as a cough medicine by thinning the phlegm build-up caused by many respiratory diseases. A study published in 2009 found that the drug also increased levels of an enzyme called glucocerebrosidase (GCase) in patients who had a rare genetic disorder called Gaucher disease. Patients with this disease are also more likely to develop Parkinson's, although scientists still aren't sure why.

A common symptom of Parkinson's is a build-up of Lewy bodies, groups of a protein called α-synuclein. When the levels of this protein go up, levels of GCase decrease, and the 2009 study proved that ambroxol increased GCase in Gaucher disease patients, so scientists began to consider whether there could be a potential application for the treatment of Parkinson's.

A smaller human trial has already been undertaken, and results from this appear promising – the drug made it to the brain and increased levels of GCase, possibly decreasing the level of α-synuclein. The drug was also demonstrated to be safe and well-tolerated in Parkinson's patients, despite the higher than usual dose.

Will Cook, chief executive officer of Parkinson's charity, Cure Parkinson's, commented: "Once the ambroxol trial is underway, it will be one of only six phase 3 trials on public record of potentially disease-modifying drugs in Parkinson's, worldwide. This trial is a big step forward in the search to finds new treatments for Parkinson's."

Professor Anthony Schapira, who is leading the study, added: "I am delighted to be leading this exciting project. This will be the first time a drug specifically applied to a genetic cause of Parkinson's disease has reached this level of trial and represents ten years of extensive and detailed work in the laboratory and in a proof of principle clinical trial."

AstraZeneca moves manufacturing to Ireland due to Britain's tax regime

Big pharma giant, AstraZeneca, has announced that its plans to open a new facility in northwest England have now changed, due to the "discouraging" corporation tax increase. Now opening in Ireland, the company will avoid the increase from 19% to 25%, which is set for April.

Initially, AstraZeneca's chief executive officer, Sir Pascal Soriot, said that the company had planned to build the facility close to its other sites in the North West of England, however the increasing corporation tax led the company to adapt its plans, with Ireland looking more promising.

The decision to leave the UK will likely hinder the UK Government's aim to become a life sciences "superpower", according to Soriot, who added: "You need an environment that gives you good returns and incentive to invest."

Tom Keith-Roach, AstraZeneca's UK president, has previously warned that Britain will lose out on AstraZeneca's investment following the tax rate increase, as the company plans to investigate more competitive countries.

The new facility will be a next-generation active pharmaceutical ingredient (API) manufacturing plant for small molecules, based at the Alexion Campus in College Park Dublin, Ireland, with the company set to invest $360m in its development.

Soriot commented: "This is a tremendously proud moment for us all at AstraZeneca and I am delighted that we are bringing this very significant investment to Dublin, which, with the support of the IDA, will create highly skilled jobs, nurture the country's dynamic life sciences sector and allow for the development of high value-added medicines."

Europe plans further antibiotic shortage countermeasures

With respiratory infections increasing, an ongoing cost of living crisis and the Russia-Ukraine conflict, officials throughout Europe are beginning to look into contingency plans to deal with the concerning shortages of certain antibiotics.

The shortages seem to have most significantly hit the liquid formulations of the drugs usually used to treat children, such as amoxicillin, with officials calling this an "ongoing public health concern". This issue has also impacted the US, with the FDA warning of similar shortages of the oral solution.

To add to this situation, Europe is also experiencing some manufacturing delays and production capacity issues, making the shortages even more significant.

The Executive Steering Group on Shortages and Safety of Medicinal Products (MSSG) has been observing the shortages with the hope to get ahead of the problem. Along with its government partners, MSSG is hoping to boost manufacturing capacity throughout Europe and therefore resolve the supply issues. Currently, MSSG estimates that the "situation will improve in the coming months."

In a joint press release with the European Medicines Agency (EMA) and the European Commission, MSSG stated: 'MSSG and the SPOC working party will continue to closely monitor the situation together with the European Commission and EU Member States. Based on current information from companies and stakeholders, it is expected that the situation will improve in the coming months. Patients and healthcare professionals are reminded that alternatives are available in case of shortages.

'It is important that antibiotics are used prudently to maintain their efficacy and avoid antimicrobial resistance. Antibiotics, including amoxicillin, should only be prescribed to treat bacterial infections. They are not suitable for treating viral infections such as cold and flu, where they are not effective.'

Genoscience Pharma received Orphan Drug Designation for hepatocellular carcinoma drug

French clinical-stage biotech company Genoscience has received FDA Orphan Drug Designation (ODD) for its lead candidate ezurpimtrostat. ODD qualifies the drug for a period of seven years of market exclusivity after approval.

Hepatocellular carcinoma (HCC) is a largely fatal cancer, with the median survival time for patients being between four and eight months. A combination treatment of atezolizumab and bevacizumab has more than doubled this timeline, however progression-free survival remains short.

Ezurpimtrostat is a first-in-class, first-in-human autophagy inhibitor whose anticancer activity is linked to PPT-inhibition, both as a solo treatment and in combination with immune checkpoint inhibitors. When tested in HCC *in vivo* models, ezurpimtrostat showed high liver tropism and potent anti-tumour activity against a panel of human cancer cells.

The ODD was granted after positive data from a phase 1 trial on primary and secondary liver tumours, where ezurpimtrostat was shown to be both feasible and well-tolerated as a monotherapy. A phase 2b ABE-Liver trial will now be conducted to test it in combination with an anti-PDL1 and an anti-angiogenic in up to 196 patients.

"FDA Orphan Drug Designation is a significant milestone for both Genoscience and for our product, ezurpimtrostat. It recognizes that our treatment has the potential to improve the lives of individuals living with HCC. We have recently launched our phase 2b clinical trial using ezurpimtrostat in conjunction with the standard atezolizumab/bevacizumab treatment. We are looking forward to sharing the intermediate results in 2024," Professor Philippe Halfon, CEO of Genoscience Pharma commented.

Australia's TGA approves psychedelics to treat mental health conditions

Australia's Therapeutic Goods Administration (TGA) has announced its approval of the use of psychedelics to treat certain mental health conditions. From 1 July 2023, medicines containing the psychedelic substances psilocybin (found in hallucinogenic fungi, also known as magic mushrooms) and MDMA can be prescribed by authorised psychiatrists to treat selected mental health conditions.

There is currently evidence that psychedelics have benefits for treating patients with post-traumatic stress disorder (PTSD) and depression, so it is only these two conditions for which the drugs can be prescribed. MDMA will be approved for PTSD while psilocybin will be approved for treatment-resistant depression.

This approval marks the TGA's acknowledgement of the lack of treatments for some treatment-resistant mental health conditions, meaning the drugs will be able to be used therapeutically in a controlled medical setting.

Only psychiatrists approved under the TGA's Authorised Prescriber Scheme will be able to prescribe these drugs, however there are currently no approved products containing the substances that have been assessed for safety, efficacy and quality, so psychiatrists will have access to 'unapproved' medicines to prescribe to their patients. For medical purposes the substances will be listed as Schedule 8 (controlled drugs) medicines in Australia's Poisons Standard, but for all other uses they remain classified as Schedule 9 (prohibited substances).

Sarah-Catherine Rodan, PhD student at the University of Sydney, commented: "Safety and efficacy have been demonstrated in other indications such as nicotine/alcohol dependence, obsessive compulsive disorder and end-of-life distress. Further, depression is often co-occurring with these psychiatric disorders. The TGA clearly acknowledges that it does have therapeutic value and states that these substances are relatively safe when administered in a medically controlled environment."

Professor Peter Duggan, senior principal research scientist at CSIRO believes this news is "really promising" for patients with these difficult-to-treat conditions, adding that it will "also be great encouragement for a number of local Australian companies whose goal is to commercialise psychedelic therapies based on psilocybin or MDMA."

Drug-resistant bacterium found in eye drops has infected 55 people in 12 states

A strongly drug-resistant bacterium has been found in multiple brands of artificial tears eyedrops; the most commonly reported was EzriCare Artificial Tears, sold by Walmart, Amazon and other retailers. The bacterium has infected 55 people throughout 12 US states, having killed one patient and leaving others hospitalised or with permanent vision loss.

The CDC has released an alert warning of the bacteria, although the FDA is yet to announce any product recalls. The CDC is recommending that clinicians and patients stop using the products in lieu of any official guidance from regulatory authorities.

EzriCare's manufacturer has announced that it is planning to recall the product.

The bacterium is a strain of Pseudomonas aeruginosa, which is a very versatile, drug-resistant bacterium often found in freshwater. It can cause skin, wound, burn, lung and systemic infections, but most often affects immune-compromised people, for example those with cystic fibrosis.

Currently, 35 of the 55 infected were found in four clusters of cases within healthcare facilities, among these four clusters EzriCare was the only product consistent between them all.

The strain circulating at the moment is Verona Integron-mediated Metallo-ß-lactamase (VIM) and Guianan-Extended Spectrum-ß-Lactamase (GES)-producing carbapenem-resistant P. aeruginosa, also known as VIM-GES-CRPA. This is the first time VIM-GES-CRPA has spread in the US and it is resistant to countless antibiotics, including: cefepime, ceftazidime, piperacillin-tazobactam, aztreonam, carbapenems, ceftazidime-avibactam and various others. It currently appears that the strain is still susceptible to cefiderocol, a new antibiotic that received FDA approval in 2019.

Cases have so far been reported in California, Colorado, Connecticut, Florida, New Jersey, New Mexico, New York, Nevada, Texas, Utah, Washington and Wisconsin.

EzriCare has stated: "As of today, we are not aware of any testing that definitively links the Pseudomonas aeruginosa outbreak to EzriCare Artificial Tears. Nonetheless, we immediately took action to stop any further distribution or sale of EzriCare Artifical Tears. To the greatest extent possible, we have been contacting customers to advise them against continued use of the product."

The company is so far cooperating with the CDC and FDA on the investigation.

First Indian intranasal COVID-19 vaccine recently launched

Indian biotech company Bharat Biotech launched its new intranasal COVID-19 vaccine iNCOVACC on the 26 January 2023 – the vaccine is the first of its kind to be developed in India.

The vaccine is already approved by the Central Drugs Standard Control Organisation (CDSCO) in India for the treatment of patients over 18 years of age, and can be used as a heterologous booster dose, meaning those who are already vaccinated with other variations can take the nasal vaccine as their booster. The vaccine can be given six months after the second dose of the initial vaccination programme.

The vaccine will be available to both the government and private vaccination centres, priced at 325 rupees ($3.99) per shot for the government, and 800 rupees ($9.83) per shot for private vaccination centres.

Nasal vaccines can be particularly effective as the nose is often the entry point for the virus, so by creating an immune response at the point of entry, a patient is protected against disease, infection and transmission.

Dr Krishna Ella, Bharat Biotech's executive chairman, has commented: "We have achieved the goals we set for ourselves during this pandemic. We have developed COVAXIN and iNCOVACC, two COVID-19 vaccines from two different platforms, with two different delivery systems. The vectored intranasal delivery platform gives us the capability for rapid product development, scale-up, easy and painless immunisation during public health emergencies and pandemics."

Johnson & Johnson subsidiary loses US bankruptcy bid

A US federal appeals court in Philadelphia has denied pharma giant Johnson & Johnson's (J&J) subsidiary's bankruptcy petition, which aimed to transfer thousands of legal claims against its subsidiary LTL management regarding the claim that its talcum powder causes cancer.

The three-judge panel ruled in favour of the cancer victims who stated that J&J wrongly put LTL management into bankruptcy, allowing it court protection, so courts were unable to hear the 40,000 claims against it.

This method has been called the 'Texas Two-Step Bankruptcies', where companies create a subsidiary to absorb the liabilities then file under Chapter 11 for bankruptcy. J&J is one of four giant companies to try to use this method.

J&J has already lost a number of similar cases, including one that was escalated to the Supreme Court and ended with J&J paying more that $2bn to one group of victims. Following this most recent loss, J&J will potentially have to pay further huge sums in court fees and payouts to claimants.

Judge Thomas Ambro wrote, 'Good intentions – such as to protect the Johnson & Johnson brand or comprehensively resolve litigation – do not suffice alone [to file for bankruptcy]. What counts to access the Bankruptcy Code's safe harbour is to meet its intended purposes. Only a putative debtor in financial distress can do so. LTL was not. Thus, we dismiss its petition.'

J&J has said it will appeal the ruling, and that its bankruptcy was filed in good faith to "equitably resolve" the talcum powder claims. This appeal will first go to a full panel at the Philadelphia appeals court, and will then be escalated to the Supreme Court if an agreement cannot be reached.

Stand Up To Cancer announces four teams dedicated to increasing diversity in clinical trials

Stand Up To Cancer (SU2C) has announced four teams that will focus on increasing diversity in early phase cancer clinical trials. The scheme is sponsored by the Janssen Pharmaceutical Companies of Johnson & Johnson, and aims to address the need to engage underserved communities in phase 1 and 2 clinical trials.

The goals of the new teams include addressing cancer disparities and encouraging the development of new treatments with the potential to benefit its patients.

The Diversity in Early Development Clinical Trials Program, part of SU2C's Health Equity Initiative, includes research teams from Chicago, Dallas, Los Angeles and Philadelphia, all in the US. The teams are aiming to address specific issues in their local region while sharing information to ensure a broader outreach.

According to data from the FDA in 2020, 73% of cancer trial patients are white, 14% are Asian, 6% are Hispanic and only 5% are Black, so an increased level of diversity is evidently needed in trials.

Russell Chew, president and CEO of SU2C, commented: "In the United States, cancer clinical trial participation remains significantly lower for people of diverse race and ethnicity, and for people in medically underserved communities. The lack of diverse involvement in therapy development makes it challenging for the healthcare industry to provide evidence-based treatments for all cancer patients. We are grateful to collaborate with Janssen on this vital effort, with the shared goal of achieving equity for everyone impacted by cancer."

Jeffrey Infante MD, global head of Oncology Early Clinical Development and Translational Research at Janssen Research & Development, LLC, added: "Our goal in sponsoring SU2C is to enable innovative research teams to develop initiatives at the community level that have a measurable impact on enhancing access to and engagement in cancer clinical trials. We look forward to seeing the results of these programmes so we can continue to help improve health equity and develop therapies and clinical programmes that benefit patients with cancer."

WHO issues a 'call to action' after cough syrup deaths

WHO has released an urgent 'call to action' to all 194 WHO member states in order to try to prevent, detect and respond to incidences of illegitimate medical products.

Since October 2022, three countries – the Gambia, Indonesia and Uzbekistan – have reported children dying from Acute Kidney Injury (AKI), which resulted from them taking cough syrup containing diethylene glycol and ethylene glycol. WHO issued global medical alerts for each country, leading to products being recalled for public safety and testing by authorities.

In the Gambia, four products made by Maiden Pharmaceuticals were found to have high levels of the chemicals after 66 children died. In Uzbekistan, an analysis conducted by Uzbekistan's Ministry of Health identified "unacceptable amounts of diethylene glycol and/or ethylene glycol" in products made by Marion Biotech.

As part of its call to action, the WHO has asked regulators to, amongst other things, "detect and remove from circulation in their respective markets any substandard medical products that have been identified in the WHO medical alerts…" and "increase marketing surveillance including risk-based targeting testing for medical products released in their respective markets…"

They have also asked manufacturers to, amongst other things, "only purchase pharmaceutical grade excipients from qualified and bona fide suppliers" and "provide assurance of product quality including through certificates of analyses based on appropriate testing results".

TheracosBio's oral drug approved by FDA for adults with type 2 diabetes

TheracosBio has announced that the first oral SGLT2 inhibitor, bexaglifozin, has been approved by the FDA for the treatment of adult patients with type 2 diabetes. The drug was originally indicated for cats with diabetes.

The once-daily treatment is recommended for adult patients with type 2 diabetes but is not indicated for those with type 1 diabetes or in diabetic ketoacidosis, however it can also be used for patients with stage 3 chronic kidney disease (CKD).

The approval follows 23 clinical trials, assessing the drug in over 5,000 patients with type 2 diabetes. The phase 3 trials showed significant HbA1c and fasting glucose reductions by the 24th week when used as a monotherapy, in combination with metformin, or as an additional treatment for those already being treated with sulfonylureas, metformin, insulin and DPP-4 inhibitors.

Mason Freeman MD, of Massachusetts General Hospital in Boston, US, commented: "As a class of drugs, SGLT2 inhibitors have shown tremendous benefit in treating adults with type 2 diabetes. Being involved in all of the clinical trials for Brenzavvy, I am greatly impressed with the efficacy of the drug in reducing blood glucose levels, and I believe it is an important addition to the SGLT2 inhibitor class of drugs."

Johnson & Johnson's Ethicon undertakes first robot-assisted kidney stone removal

Johnson & Johnson (J&J) medtech company, Ethicon, has announced its first robotic-assisted kidney stone removal using its Monarch platform.

The Monarch platform was developed by Auris Health, a subsidiary of Ethicon, and received FDA clearance for endourological procedures in May 2022. The University of California, Irvine's UCI Health utilised this technology to undertake the first procedure with the Monarch platform as part of a clinical study.

The procedure was the first robotically assisted electromagnetic (EM)-guided percutaneous access and mini-percutaneous nephrolithotomy (PCNL) procedure. The clinical study was collaborative with co-investigator Dr Mihir Desai from the University of Southern California (USC).

Dr Jaime Landman, chair of the UCI School of Medicine Department of Urology and director of the UCI Health Kidney Stone & Kidney Disease Services, commented: "This clinical study is the first in the world to research and demonstrate potential for improved navigation, access, clearance and control in mini-PCNL procedures using the Monarch platform for urology. In addition to potentially helping urologists achieve stone-free patients in a single procedure, this approach could help reduce the need for retreatment after kidney stone removal and decrease risks and complication rates."

"After years of work, we are thrilled to be a part of this first clinical series, which introduces a new treatment to improve outcomes for patients in need," Landman continued.

Dr Mihir Desai, USC, added: "The prevalence of kidney stones remains high, and many urologists seek a new treatment option that reduces overall retreatment and complication rates. In patients who require treatment through surgery, close to one in two will require retreatment within five years."

SynaptixBio secures FDA Rare Paediatric Disease designation to develop treatments for TUBB4a leukodystrophy

SynaptixBio, a UK-based biotech firm, has been granted FDA Rare Paediatric Disease (RPD) designation – an accolade that encourages the development of new drugs that are indicated for rare diseases with highly unmet needs.

The designation is also the first step to obtaining a Priority Review Voucher (PRV), which can accelerate market access for therapeutics and allow them to be sold or traded by sponsors, including large pharma companies.

SynaptixBio is focusing on TUBB4a leukodystrophy, a genetic and debilitating condition that mainly affects babies and young children. It disrupts myelin, leading to an interruption of the signals between the nerve cells in the brain, which can cause seizures, muscle contractions, hearing and speech difficulties and uncontrollable limb movements. At its worst, it can lead to a regression of motor skills learned at an earlier age, as well as significant impairment which affects walking, sitting up and swallowing.

Leukodystrophies affect one in 7,663 births, according to the University of Utah, meaning every year 20,000 people could develop a leukodystrophy, with more than 2,220 of those being TUBB4a.

To develop a treatment, SynaptixBio is focusing on antisense oligonucleotides (ASOs), which have previously been used to treat Duchenne muscular dystrophy and spinal muscular dystrophy. Last year, it entered into a sponsored research agreement with the Children's Hospital of Philadelphia (CHOP), which will allow it to translate CHOP's research into first-in-human clinical trials, which SynaptixBio hopes will begin in 2024. The agreement also includes worldwide patent rights.

SynaptixBio CEO and co-founder Dr Dan Williams stated: "To be granted an RPD will enable us to accelerate our research into TUBB4a treatments, while ensuring the work being done to tackle it remains a focus within medical communities around the world. With significant need to directly address mutations in the TUBB4a gene, we believe this is a monumental step forward in our mission to develop the world's first treatment. Obtaining a PRV will not only validate the rarity and importance of the disease, it will lower the commercial risk for partners and investors, and potentially fund ongoing drug development. As our data set and understanding of the disease grows, the closer to securing a PRV we become."

Dr Adeline Vanderver, programme director of the Leukodystrophy Center at CHOP said: "ASOs provide the potential to stabilise, improve quality of life and extend life expectancy of children suffering from the condition. Successful prevention of leukodystrophy progression would be revolutionary, life-saving and life-enriching."

AbbVie expects 37% decline in Humira sales following new biosimilars hitting the US market

AbbVie Inc has announced that it expects to see a 37% decline in sales of its flagship rheumatoid arthritis drug Humira, as new competition emerges in the form of cheaper biosimilar drugs. The company expects its sales to stabilise by the end of 2024.

New drugs Skyrizi and Rinvoq also treat autoimmune disorders, so are part of the reason that AbbVie's sales have dropped.

Investors will be reassured to learn that the company expects its 2024 earnings to be no lower than $10.70 per share, similar to its 2023 predictions of $10.70 to $11.10 per share. AbbVie's shares were also up more than 3% despite the 2023 profit outlook falling short of Wall Street estimates of $11.65 per share.

According to vice chairman Robert Michael, the sales decline is likely to be driven by both volume erosion and price erosion, adding: "Now it's a question of what will the volume erosion look like?"

Commenting on the company's financial year, Richard A Gonzalez, chairman and chief executive officer of AbbVie, said, "2022 was another highly productive year capping a decade of outstanding performance. Since our inception, we have built a diverse portfolio of growth products with significant leadership positions, developed a robust pipeline of innovative assets and created a culture of strong execution. Looking forward, we have a solid foundation, which will allow us to absorb the US Humira loss of exclusivity, return to strong top-line growth in 2025 and drive top-tier financial performance over the long term."

Boston Scientific fined $42m over patent infringement

A five-year legal battle between Boston Scientific and TissueGen has culminated in a $42m fine, which Boston Scientific must pay to TissueGen regarding a patent infringement.

The technology in question – extruded fibre that delivers drugs though an implanted vascular stent – was created and patented by Kevin Nelson PhD, while he was a faculty member at the University of Texas. Nelson then founded TissueGen, which, in 2013, launched biodegradable Elute drug-loaded fibres that can be customised to deliver a variety of drugs and fit into a device-maker's existing materials.

In 2015, Boston Scientific followed with FDA clearance for its own Synergy system – a drug-covered stent used to treat coronary artery disease. The court case began in Texas in 2017 when TissueGen and

the University of Texas filed a complaint, claiming Boston Scientific had infringed on two patents licensed to TissueGen regarding 'drug-releasing biodegradable fibers.'

In early February 2023, Delaware's jury voted in favour of TissueGen, stating that the Synergy technology infringed on TissueGen's patent, meaning Boston Scientific should pay $42m in lost royalties to the company.

Nelson, currently the chief scientific officer of the company, commented: "TissueGen does not control what Boston Scientific does with its resources, which are endless by comparison to TissueGen. If Boston Scientific respects the court who provided both sides a fair opportunity to present their cases and the citizens who took time from their lives to render a judgment, the recovery will be poured back into research at UT System and TissueGen to save lives and relieve suffering."

"Boston Scientific respectfully disagrees with the jury's verdict and plans to appeal," a company spokesperson has said.

Garuda Therapeutics closes series B funding with $62m

Cambridge-based Garuda Therapeutics announced it closed a $62m series B funding round to support its off-the-shelf, self-renewing blood stem cell technology.

Garuda launched 15 months ago with $72m in funding, with the aim to use its cellular therapy to potentially cure over 70 diseases. Since then, it has identified a further 50 that could benefit from stem cell treatment.

Its therapy uses proprietary hematopoietic stem cells, which will be faster and more effective, and lead to greater patient accessibility. Using a patient's own depleted cells or seeking out a biological donor match is both costly and time-prohibitive.

The series B funding will be shared between two programmes: transfusion-dependent beta-thalassemia (TDBT) in Europe, and bone marrow failure syndrome in Europe, South America and North America.

It's also notable that having off-the-shelf gene therapies will help close the racial divide in treatments. According to a 2019 study, white Americans have a 77% chance of finding a donor on the National Marrow Donor Program's 'Be The Match' registry, whereas Black patients have around a 23% chance.

Dhvanit Shah PhD, co-founder and CEO of Garuda stated: "Garuda is built upon the foundation of what we consider to be the most de-risked and highly validated blood stem cell-based cell therapy approach, and has the potential to overcome challenges to the current standard of care, including lack of consistency, scalability, durability, affordability and availability of suitable healthy donors. We are grateful for the continued strong financial support from our elite institutional investors that will help enable us to bring our off-the-shelf durable blood stem cell and immune cell programmes to patients in need. Garuda's series B investment translates directly into the advancement of two lead programmes for haematology and oncology indications. This funding milestone is another crucial step to delivering life-changing cellular therapeutics to patients around the world afflicted by treatable, and often curable, diseases."

Daiichi Sankyo to build Japan's first factory for mRNA COVID-19 vaccines

Daiichi Sankyo has announced its plans to build Japan's first factory for mRNA COVID-19 vaccines. The new facility is expected to have a capacity of 20 million doses per year by the fiscal year of 2024.

Production equipment has already been installed at a plant currently run by a subsidiary, Daiichi Sankyo Biotech, which is based in Kitamoto near Tokyo. In January, the company applied for approval for its COVID-19 vaccine that is currently in production.

The new factory is intended to enhance Japan's self-sufficiency in terms of its coronavirus vaccines as the nation aims to convert from treating COVID-19 as a health emergency to learning to live with the virus. Early in the pandemic, Japan struggled to secure supplies of the Pfizer and BioNTech, and Moderna mRNA vaccines, which use messenger RNA to teach the body how to make proteins to trigger immune responses to the virus.

While other Japanese drugmakers are also working on different vaccines against COVID-19, Daiichi Sankyo is planning to use government subsidiaries to add another mRNA vaccine production wing to its existing factory by the fiscal year of 2027.

According to its January press release, 'Daiichi Sankyo is striving to establish mRNA-vaccine-related technologies and the production and supply system in Japan to ensure a prompt provision of vaccines in the event of outbreaks of emerging and re-emerging infectious diseases, thereby continuing to protect safety and security in society and people's health.'

Eli Lilly plans to invest $450m at production site in Research Triangle Park

US-based pharmaceutical company, Eli Lilly and Company, has announced plans to invest $450m in its manufacturing facilities at Research Triangle Park in North Carolina, US.

Lilly's expansion is set to include the addition of further parenteral filling, device assembly and packaging capacity in order to meet the growing demand for the company's incretin-based diabetes treatments and medications. The facility is expected to become operational in 2027 and will create over 100 new jobs for manufacturing personnel.

Since 2020, Lilly has invested almost $4bn, $1.7bn of which has gone towards the development and expansion of its campus at the Research Triangle Park. The preliminary manufacturing is expected to begin this year at the newly expanded site, with preparations for FDA inspections under way.

The expansion is expected to allow the company to continue to supply patients with new medications and aims to set up the global distribution of its products.

Edgardo Hernandez, Lilly's Manufacturing Operations executive vice president and president, commented: "As we move into 2023, Lilly is focused on finding innovative solutions to meet the growing demand for our medicines. […] Expanding our operations at Research Triangle Park will accelerate the rate at which we can produce medicines that patients can rely on to address serious health challenges like diabetes. […] We're on track to achieve the goal we shared in November 2022 of doubling incretin capacity by the end of this year, but this investment is key to ensuring even more patients will have access to medicines they need in the future."

Johnson & Johnson scale back COVID-19 vaccination production

US-based Johnson & Johnson (J&J) has begun scaling back production of its COVID-19 vaccine due to demand falling over the past few months. As part of this, it's started terminating manufacturing agreements with companies who helped it produce the vaccine, such as Catalent and Sanofi.

J&J's shot was welcomed by health authorities due to its fewer cold-chain storage requirements. However, manufacturing issues meant J&J suffered availability problems. According to the Centers for Disease Control and Prevention, only 19 million doses of J&J's vaccine were administered, compared to around 400 million of the Pfizer-BioNTech shot and approximately 250 million doses of the Moderna one.

There was also a safety risk involving the rare but serious blood-clotting issue, which led to J&J's initial one-dose shot being age-restricted in some countries.

Prashant Yadav, a supply-chain expert and senior fellow at the Center for Global Development, a think tank focused on cutting poverty, commented: "They were trying to do this the best way possible to meet global demand, but all of those plans fell apart."

There are currently hundreds of millions of remnant J&J doses, meaning the vaccine will still be available where it is needed. J&J has not commented to whether it will cease manufacturing the vaccine altogether, though, following the scale-back.

Joe Wolk, CFO at J&J stated: "Right now, we are, like we would do for any product, right-sizing our manufacturing footprint, our R&D programmes, to correlate better with the demand that's out there. We're going to meet our regulatory, as well as our commercial, commitment and just right-sizing it for […] the business prospects that are out there."

AI finds liver cancer drug candidate in only 30 days

With most drug discovery and development processes taking years, if not decades, a team of scientists have broken records with their new AI which has found a novel drug candidate for liver cancer in only 30 days.

Liver cancer is one of the leading causes of cancer mortality, with over 830,000 deaths worldwide in 2020, according to the WHO, so this breakthrough is highly significant.

The discovery may mark the beginning of a new era in drug discovery, with potential to renew research processes within the biotech, pharma, healthcare and life sciences industries. The research was recently published in *Chemical Science*.

The researchers used AI to identify the best 20 targets using ten data sets of data around hepatocellular carcinoma, the most common form of liver cancer. The targets were filtered for safety, tissue specificity, accessibility by biologics, small molecule accessibility and novelty. Following this process, cyclin-dependent kinase 20 (CDK20) was selected as the researchers' initial target.

Alex Zharvoronkov, senior author on the study, founder and chief executive officer at Insilico Medicine, commented: "In 2022, Insilico nominated nine preclinical candidates out of its AI engine, eight for internal and one for a partner. If you compare this total with any big pharmaceutical company's performance, it is very impressive since it is a comparable number, which comes at a tiny fraction of the cost. Many of these are novel targets and some are challenging targets demonstrating that the generative AI can now perform well in both biology and chemistry. We also got the top line data from the first AI-discovered and AI-designed antifibrotic."

BD showcases new-generation robotic track system for microbiology labs

US-based Becton, Dickinson and Company (BD) has revealed a new-generation robotic track system for its BD Kiestra microbiology laboratory solution, which will automate lab specimen processing, helping to reduce wait times and the need for manual labour.

The third-gen Kiestra Total Lab Automation (TLA) system is customisable and flexible, with multiple BD Kiestra modules. Labs can select the automation entry point and configure the system according to their individual workflows.

It allows routine specimen inoculation to incubation, imaging and colony selection, using BD's Synapsys Informatics solution to trace each step of the lab's diagnostic pathway. The BD's Synapsys Informatics solution is a life diagnostics information platform designed to aid laboratories.

BD microbiology vice president and general manager Cecilia Soriano said: "The track-based configuration options are designed to eliminate the manual sorting of plates and walking the specimen from module to module, which helps to ensure culture integrity and results in streamlined workflows. The modular tracks use a sophisticated robotic highway on/off ramp design to help eliminate bottlenecks, traffic jams and plate collisions."

BD integrated diagnostic solutions president Brooke Story commented, "With our 3rd Generation Total Lab Automation System, labs with workflows impeded by room layouts can break free from space and configuration constraints to build the system that best meets their needs today and be ready to expand tomorrow."

DrugBAN AI expected to reduce costs and speed up drug discovery

In a collaboration between Sheffield University and pharma giant, AstraZeneca, a new AI has been developed that could help deliver new medicines at a faster rate and lower cost. DrugBAN AI has been developed by Professor Haiping Lu and PhD student Peizhen Bai from the University's Department of Computer Science, alongside AstraZeneca's Dr Filip Miljković and Dr Bino John.

The research has been published in *Nature Machine Intelligence*. The study proved that DrugBAN AI could predict whether candidate drugs can interact with the intended target protein molecules within the human body.

AIs to predict whether drugs will reach their targets already exist, however this new AI has been developed to do this with increased accuracy while also providing extra insights to help scientists learn how the drugs will interact with their protein partners on a molecular level.

Haiping Lu, professor of machine learning (ML) at Sheffield University, commented in a statement: "We designed the AI with two primary objectives. Firstly, we want the AI to capture how drugs interact with their targets at a finer scale, as this could provide useful biological insights to help researchers understand these interactions on a molecular level. Secondly, we want the tool to be able to predict what these interactions will be with new drugs or targets to help accelerate the overall prediction process. The study we've published… shows our AI model does both of these."

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Improving cardiovascular disease care and awareness

Pharmafocus talks to Scott Curley about the risks of CVD and the importance of getting the right treatment at the right time

***Pharmafocus*: How did the COVID-19 pandemic impact care for patients with cardiovascular disease?**

Scott Curley (SC): One of the main impacts is the rising number of heart-related deaths due to cardiovascular disease (CVD). A recent article published by the British Heart Foundation (BHF) suggests that the pandemic could be linked to up to 30,000 additional heart deaths. We all know that the number of excess deaths related to COVID-19 is far greater than that, but this is still a significant number of potentially avoidable deaths due to CVD.

A second impact of the pandemic was the inability to get an appointment, which unfortunately led to a lot of missed or delayed diagnoses. Research done by Heart UK looking at the evolving system of healthcare shows the impact of COVID-19 over the last 12 months, while also benchmarking it against the previous 12 months. This research estimates that, during this time, there were more than a million missed health checks, including blood tests and general patient health checks. This, of course, includes checks for CVD.

We generally find that those who have been diagnosed and are being treated have had their healthy heart check-ups. However, it's estimated that up to eight out of ten patients who are eligible for these check-ups have either not been contacted or have not yet had this check-up, which includes important lipid profile tests. Unfortunately, even some who were being treated and were getting regular check-ups haven't had follow-up appointments. This also presents a huge challenge as, if they've already had a primary event, a high number of these patients could have a secondary, catastrophic event.

Another impact of the pandemic, despite the excellent services the NHS provides, is that waiting lists are at an all-time high, even as much as 50% higher than they were before the pandemic. So, when the ticking time bomb that is CVD starts to make its way into an already pressurised NHS, it will take some time to work through.

What steps can the NHS and the UK government take to improve CVD care?

SC: First, I would like to say that, during the pandemic, we all faced unprecedented challenges under exceptional circumstances. Those in the NHS did the very best they could, all the while putting themselves on the line and facing a disease that wasn't yet fully understood, and tragically many of the staff succumbed to COVID-19 in the early stages.

In recognition of the outstanding work the NHS was doing, so many of us stood outside our houses applauding their heroic efforts – it's important that we don't forget that and that we continue to show our appreciation for the NHS.

A recent publication by NHS England, the Secondary Prevention Programme, looks at patients who have had an event and who have received hospital treatment, and have then gone back into the community. There's a huge risk for these patients and if we don't continue to monitor them, it's possible they will have a secondary event, which may be far worse than the initial one.

The NHS is looking at high-impact interventions through patient identification and medicine optimisation clinics to treat cardiovascular and other diseases – developments and prevention strategies that they are already supporting. There is a focus on secondary prevention and ensuing that patients who are most at risk get the treatment they need. Through NHS England, in partnership with NICE, new integrated care systems (ICSs) and integrated care boards (ICBs) have developed resource packs detailing the most impactful interventions that can be implemented to delay or reduce secondary events.

However, it's clear that one of the most pressing challenges currently is that the NHS is already in crisis – underfunded, under-resourced and striking for the right to decent pay – so implementing new processes and procedures will be extremely difficult in such turbulent times.

Is the UK government's response to CVD different from responses seen in the US or Asia?

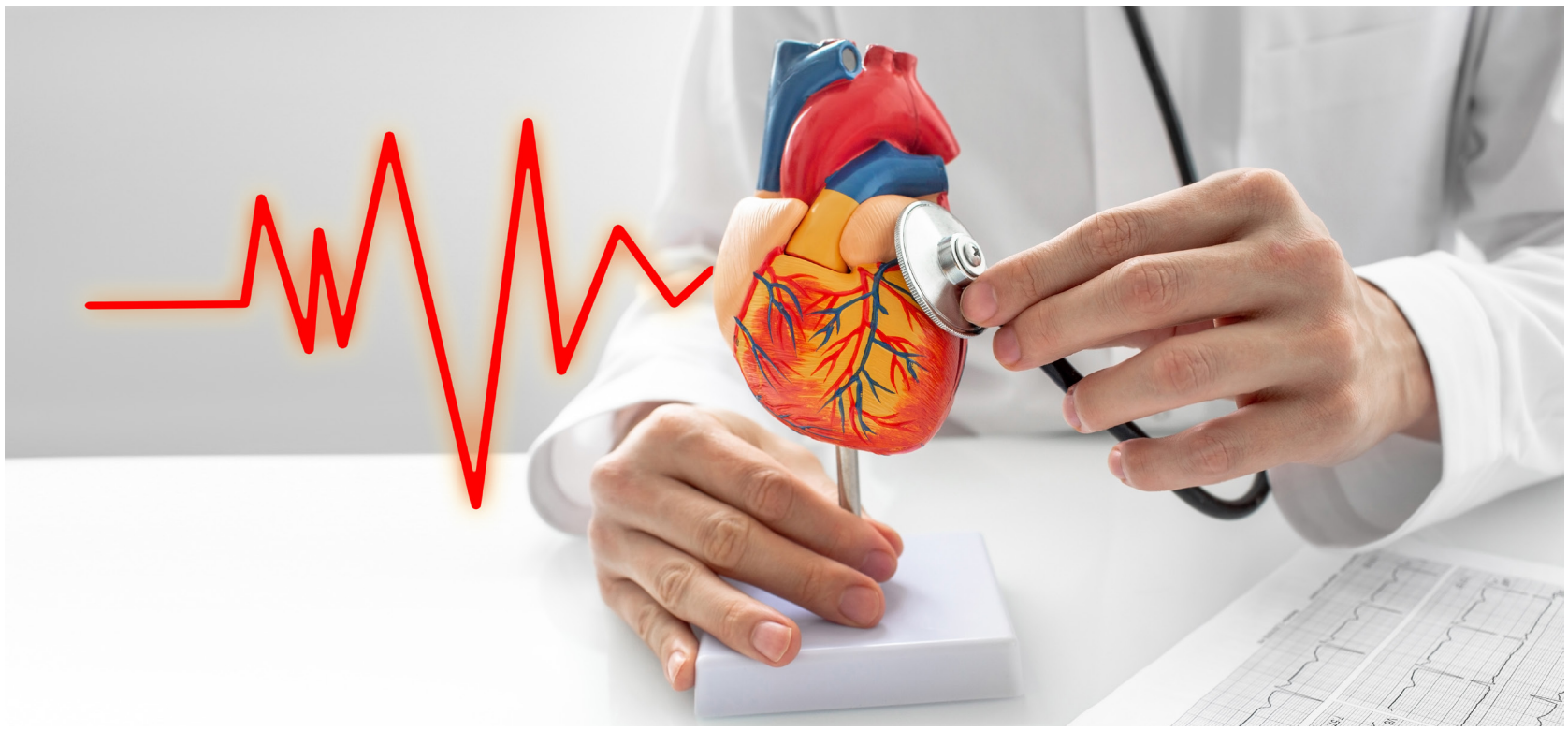
SC: I worked in China, Hong Kong and Asia for a number of years and, in many instances, these countries look at the interventions made by Western countries, because they're on a different path – that is certainly what I've seen historically. Over the last five years, it looks like NHS England, NICE and the new ICSs and ICBs are leading the way, to some extent, in terms of how we tackle the potential burden of the excess deaths related to CVD. When speaking to colleagues in these other markets, I haven't seen an approach that's quite as focused or as determined. I have very high hopes that we can lead the way and that others may then benefit from this.

How can pharma companies and healthcare providers work together to lessen the impact of CVD?

SC: I wish I had a definitive answer. There's definitely a need to completely rethink CVD and the typical patient profile if we are going to be successful in tackling the rising mortality rates. NICE recommends that high-quality cardiovascular risk assessments need to be done, and the recently produced new national guidelines that support this include more robust, detailed lipid profiles, which will give a better indication of each patient's risk.

We also need to be innovative through, for example, the use of point-of-care testing and identifying ways to better educate patients. Organisations such as the European Society of Cardiology are looking to support the development of national strategies such as those from NHS England on secondary prevention and recently, more pharma companies have partnered with the NHS.

Historically, collaborations have been more focused on R&D, for example working together on new innovative medicines, new trials and new opportunities to support patients at high



risk, but what we've seen more locally are collaborations looking at the expertise and resources that pharma companies have, and using these collaborations to identify the most at-risk patients and provide them with the right level of care.

After new guidelines have been made available and new protocols have been introduced, the right support mechanisms need to be in place to ensure that eligible patients at higher risk get the right treatment at the right time. This requires collaboration and trust, and working together to help as many patients as possible, in order to have a really positive impact on current CVD mortality rates, which are a legacy of the COVID-19 pandemic.

What are the best steps to take to protect against an increased risk of CVD?

SC: In a nutshell – behaviour change. But it is extremely difficult to encourage people to make the changes needed to lead a healthy lifestyle, such as eating healthy food, limiting alcohol intake, stopping smoking, exercising and maintaining a healthy weight. People live fast-paced lives – they often don't have the time and energy to implement these changes, and this is understandable.

It's important to recognise just how difficult these lifestyle changes are and the huge efforts they require to implement and maintain. Everyone would like to lead healthier lives, but that's not always possible. This is where cognitive behavioural change comes in, as it can help people understand how much difference even small changes can make.

One other key thing is to book an appointment with your GP to ensure that you get the necessary check-ups. In addition to this, people should always be encouraged to seek good medical advice. This is available through services like 111 and GP surgeries, and there are also charities in the UK, such as Heart UK and the British Heart Foundation (BHF) in particular that can provide helpful information. It's important to take control, as understanding your existing and future health risks can enable you to take the right steps and get the right treatment at the right time.

We need to remember that the outlook for CVD has changed considerably from previous years. It used to be seen as something that was associated with your grandparents' generation, with a generally accepted view that it affected older men – but it affects women as much as it affects men, and it also affects younger people, as well as those who are older. Atherosclerotic disease can affect those in their 30s, so there needs to be much more awareness of this, to enable people to take the right steps to protect their health.

As mentioned, it's easy to suggest lifestyle interventions and advise people to 'eat more healthy foods', but one of the most important things is to get those check-ups done. Like most diseases, when CVD is caught early, there are effective treatments and interventions that can help to delay or prevent later events.

Behaviour change is always difficult and no one should be blamed for developing CVD. What's important is to help people access the different healthcare options available to enable them to live a longer, healthier life.

What is the forecast for CVD treatments over the next few years?

S**C:** What we do know is that this disease is a silent killer – many people don't realise they have CVD until it's too late, and it's important to recognise that. For outcomes to improve, it's vital for people to have the right check-ups and the right tests, to enable them to receive treatment, medication or advice on lifestyle changes.

There is an important opportunity here to completely rethink the treatment paradigms. NICE offers world-class health technology appraisals of new medicines and, when it believes that a new innovative treatment can make a huge difference, it backs that treatment and publishes its guidelines. At this point, there is a need for the ICSs, ICBs and other health boards to implement these guidances much more rapidly.

As new, innovative treatments are developed, it's up to NICE to decide if they will be added to the available treatments for CVD. If that answer is yes, the changes to the protocols and pathways that are needed to implement these new medicines need to be made as quickly as possible.

The NHS needs to identify those who are most at risk, so they can be offered treatment that may prevent or delay that first event. This is even more important for those who already had an event, as they are more likely to have a secondary event.

There are many innovative medicines on the horizon, a number of which have only recently been launched. Together with the NHS, we can make a huge difference to the impact of CVD on patients' lives.

*Written by **Iona Everson,** Group Managing Editor at **PMGroup***

Scott Curley** was interviewed by **Betsy Goodfellow** and **Charlie Blackie-Kelly** at **Samedan Ltd

***Scott Curley** is general manager of **Amarin**, UK and Ireland. He has worked in the pharma industry for more than 25 years and has spent more than half of his career working in the cardiovascular and metabolic disease space. Previously, he held roles at AstraZeneca and GlaxoSmithKline, and was based in the UK, China, Hong Kong and Asia.*

Talking Point

The patient voice in autoimmune conditions

Galápagos
Pioneering for patients

Rhian Linney, Associate Communications Director at Galapagos, UK and Ireland, tells Pharmafocus about the significance of the patient voice in autoimmune conditions, focusing on rheumatoid arthritis

Personal

Working in the healthcare industry runs in my family – it's in my blood. My father started his working life as a medical sales representative and my mother was a pharmacist for many years before they started up their own independent pharmacy. Saturday mornings and many school holidays were spent in the shop, watching my mother prepare and dispense medicines, talking to patients and interacting with people from all walks of life. It was a truly great experience and one that cemented my ambition to work in the healthcare industry.

Throughout my communications career I've had the pleasure of collaborating with several different patient groups, on both a local and global scale – it's the part of my job that I enjoy the most and something I'm very passionate about. This is why I work at Galapagos. I am empowered and supported with the right tools and resources to work and collaborate with patient advocacy groups in the inflammation space.

Pioneering

The chronic nature of most autoimmune conditions makes the role of patient support programmes even more critical – when thinking about how to make these as effective as possible, there is much to consider. Specifically, they need to be delivered sensitively and with care, to facilitate personal confidence and give those who feel like they've been silenced, a voice.

However, too often chronic conditions are 'left behind', both when it comes to treatment advancements, or provision of useful information and holistic support for patients.

What stood out to me about Galapagos was its commitment to bringing innovation to inflammation, but also its dedication to provide ongoing, valuable support for patients. This pioneering mindset, which Galapagos employs across every facet of the business, presented something that I wanted to be part of.

Priority

Looking at rheumatoid arthritis (RA) specifically, Galapagos wanted to challenge how the condition is perceived by the public and how the people affected are supported. It wanted to make RA a priority.

The 'We R.A. Priority' (WRAP) campaign was launched in 2020 based on direct insights from the RA community, gained via a survey of 300 people living with RA. Developed in partnership with the National Rheumatoid Arthritis Society (NRAS), the campaign changed the face of RA. Common misconceptions about RA mean it is often seen as a condition that affects 'old people'. By sharing the stories of people from different backgrounds and ages, WRAP gives a voice to the people who are actually living with RA: people of different ages, ethnicities, genders and backgrounds.

The campaign, which continues to live on today, has evolved year on year – adapting to best serve the RA community at any given time. One aspect that has remained constant is that the patient voice has remained at the heart of the campaign.

Kaoru, We R.A. Priority ambassador and violinist for the Royal Opera House

We have learned first-hand from the community that it is the stories of other people, who have the shared experience of living with RA, which offer hope and reassurance to those struggling with aspects of life with RA. For this connection to be made, it is crucial that individuals can see themselves in the person whose story they are looking to for inspiration.

Through the diverse voices of our WRAP ambassadors, the campaign also helps to empower others to find their voice, and with that voice ask the public, employers, relatives and friends to make RA a priority.

Positive

The impacts of RA can extend across all aspects of a person's life. Through NRAS' strong connection to the RA community, we are able to better understand what the most pertinent challenges are and partner with allies of the RA community to provide expert advice and holistic support across areas including: sleep, relationships, technology and accessibility, fashion, legislation and sex.

Now in its third year, I am immensely proud of this award-winning campaign and the impact it continues to have on the RA community.

No two people are the same, but they can find commonality and community by raising their voices together. Where we can support one patient with information about looking good and feeling good, another might find they can shout louder about requiring better healthcare or have the confidence to speak up about their needs at work. The combination of these individual voices, singing together about their needs, creates a noise so loud that it can help to propel a patient community forward into positive change. It's heartening to know that the work we do helps to provide an opportunity for their voices to be the loudest in the room; for once, they are a priority.

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Industry Insight

Removing Uncertainty in the Supply Chain

Nikunj Desai, head of supply chain at ACG, explores how supply chains can be made more secure and the impacts this may have on the pharma industry

Supply chain volatility

The current crisis in Europe has led to increasing energy insecurity, with the potential of power outages under discussion for the remainder of this winter.

These uncertainties are set to exacerbate existing issues that have been enduring since March 2020, when the COVID-19 pandemic caused drastic upheavals to supply chains. Shortages of raw materials are continuing to yield severe pharmaceutical manufacturing supply line disruptions.

In some cases, European manufacturers are experiencing a doubling of supplier lead times, uncertain and fluctuating prices and orders that are often subject to delays.

Add to this soaring inflation and global talent shortages, these numerous sector threats have left many manufacturers with plants not operating to full capacity – compounded by increases to overall running costs.

Looking to Darwin's philosophical reasoning 'It is not the most intellectual of the species that survives; it is not the strongest that survives, but the species that survives is the one that is able best to adapt and adjust to the changing environment in which it finds itself.'

Perhaps now, during one of the greatest manufacturing crises on record, is a time to consider doing things differently rather than accepting the scarcity of multiple resources and running a leaner operation to match.

Resilient supply chain

A resilient supply chain is defined by its capacity for resistance and recovery. However, the most modern and resilient supply chains are designed to do more than simply resist and recover. These supply chains are developed and built on strong foundations of robust processes and systems that enable their stakeholders to have full visibility and hence plan proactive actions.

Managing risk

In the long history of supply and distribution, risk management has been a challenge. Considering the complex interoperability of each service provider in the chain, even a small problem can cause vast disruption.

Mitigation planning must also anticipate other potential disruptions such as seismic changes in consumer behaviour, sudden market movement, an unpredictable trade environment, fluctuating tariffs or political uncertainty.

In building resilience to adapt to unforeseen events, supply chain custodians have had to review and streamline their logistics and warehousing networks or work with more third-party fulfilment providers to adapt.

Track and Trace

Whilst not yet standardised, being able to identify products' whereabouts in the supply chain is also key – from production through to delivery. Depending on the logistics partners in the supply chain, having visibility of the location of a consignment, the condition in which it is transported and monitoring the temperature all add to the security of the supply chain. Being able to monitor the route of a vehicle and escalating an issue can make a significant difference to journey timings. Schedules are critical for both cost management and operational efficiency, which can ultimately impact a brand's profitability.

Working to exception reporting

Although it is widely understood that strong supply chain management is founded on having a flexible contingency plan that helps the manager respond quickly to operational disruptions, in my experience the most resilient supply chain must learn from historical data and events to anticipate or even forecast. Mapping scenarios-based strategies helps teams to identify risks such as port closures, handling vendor disruption, logistic hub failure or having contingent alternative modes of transport or routes. These are also instrumental in identifying key performance indicators (KPIs) – to monitor instances of failure that will help to isolate an incident and make an appropriate corrective decision.

Working to exception reporting should enable the avoidance of disruption altogether. Supply chain control relies on having clear communications alongside powerful, yet flexible systems. Building transparency and having true visibility drives sound business decisions and creates agility.

Process automation

Beyond systems and communications, greater resilience and agility are achieved via process automation that reduces errors and increases accuracy and precision. Freeing staff from repetitive tasks and effectively adopting automation removes an element of risk.

During these difficult times, not only has it become vitally important to proactively manage risk to create resilient supply chains, but it has also become paramount to be acutely risk aware. Moving away from the traditional model of stand-alone systems and working in silos, it is essential to develop partnerships that are transparent, adaptive and responsive. It is the combination of the number of suppliers, the spread of geography and customers that creates a natural hedge and protection against the crisis.

European manufacturers must embrace what is needed now, allowing them to quickly and easily collaborate, tap into and benefit from established supply chains and distribution networks, which would otherwise take years of relationship building and investment.

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Move of the month

Chiesi Farmaceutici hires new CEO after $1.48bn Amryt acquisition

Chiesi Farmaceutici, a family-owned Italian pharmaceutical company, has appointed Giuseppe Accogli as group CEO. This comes after 2020's $1.48bn acquisition of rare disease specialist Amryt.

Accogli has 25 years' experience in the pharma industry, most recently at Baxter in its renal sector. Before then, he was at Italian medtechs Medtronic and Tyco Healthcare. Accogli's background spans sales, marketing, product innovation, R&D and M&A, where he focused on the EMEA and US markets.

Giuseppe Accogli stated: "I am honoured to lead Chiesi into its new stages of growth and I thank the Board of Directors for their trust and the opportunity they are giving me to be part of such an exciting journey. At its core, Chiesi is a group of innovative and highly committed professionals who care for the health and well-being of people around the world leaving a positive impact in the environment and the communities in which the company operates. As CEO, I strive to enable our leaders, people and partners to achieve even greater success in fulfilling this commitment to be a sustainable force for good in the biopharmaceutical industry."

Alberto Chiesi, President of Chiesi Group, says, "We are thrilled to have Giuseppe in the CEO role. He is a proven leader with the vision and the strength to expand on the success Chiesi has built over the past 85 years and take us into our next phase of growth and innovation."

Amryt, another Italian company, currently has three FDA-approved drugs: Juxtapid for homozygous familial hypercholesterolemia, Myalept for lipodystrophy and Mycapssa for acromegaly. It also has Filsuvez, which was approved in the EU in 2022.

Ingenza appoints Dr Mark Chadwick as strategic business advisor

Ingenza has announced the appointment of Dr Mark Chadwick to its team as strategic business advisor. Mark brings extensive industry experience to his new role, following his 30 years' worth of roles in the pharma sphere.

Dr Chadwick has previously worked at small growth companies, covering areas including biotechnology, drug discovery and drug development. He holds a PhD in molecular biology from the University of Newcastle as well as an MBA from Imperial College London.

Dr Chadwick has gained valuable experience in both discovery and development businesses through his roles at BioFocus (now part of Charles River Laboratories), pharmaceutical services company Excelsyn and his role leading the commercial team at Arcinova.

Commenting on his new role, Dr Chadwick stated: "Ingenza has an illustrious track record of engineering diverse biological systems that help companies of all sizes to address challenges in human health and environmental sustainability. It is also committed to developing green biomanufacturing processes and together, these factors attracted me to work with the team. I'm very much looking forward to helping Ingenza to drive their growth strategy."

Jaymin Amin, chief business officer at Ingenza, added: "Mark's broad skill set will be a resource for us as we seek to turbocharge our new business development strategy and extend our collaborator network in the pharmaceutical field across discovery, development and manufacturing areas. We're confident Mark will play an essential role in helping Ingenza to continue building its international reputation as a leading engineering biology company through his in-depth industry insights."

Medivir AB appoints Pia Baumann as chief medical officer

Pharmaceutical company Medivir AB has announced that Pia Baumann has taken the role of chief medical officer. Medivir focuses on the development of innovative cancer treatments in areas of high unmet medical need.

Pia Baumann now takes a role on Medivir's management team, meaning her responsibilities include the continued clinical development of the candidate drug fostroxacitabine bralpamide (fostrox).

Baumann earned her PhD at the Karolinska Institute and has extensive experience in the drug development sphere, specifically within the cancer field. She has experience of clinical work at Karolinska Hospital as well as having experience at both large pharmaceutical companies and smaller biotech companies.

Recently, Baumann has worked at AstraZeneca as vice president medical with global responsibility for the company's Tagrisso and Lung Cancer franchise. Prior to this she worked at Takeda, Incyte and ARIAD Pharmaceuticals holding various leading global positions.

Jens Lindberg, chief executive officer at Medivir AB, commented: "I am very pleased that Pia now has taken up the role as chief medical officer. She is joining us at an exciting time, considering we have just communicated that fostrox is now entering the expansion phase (phase 2a) in combination with Lenvima. Her experience in global drug development as well as interaction and cooperation with regulatory authorities will be very important for the continued clinical development of fostrox."

Vishal Kapoor appointed to Jasper Therapeutics' Board of Directors

Biotechnology company, Jasper Therapeutics, has appointed Vishal Kapoor to its Board of Directors. The company focuses on developing novel antibody therapies targeting c-Kit (CD117) to address diseases including chronic spontaneous urticaria and lower to intermediate risk myelodysplastic syndromes (MDS), along with novel stem cell transplant conditioning regimes.

Kapoor has been a partner of Avego Management, an affiliate of Velan Capital, since January 2021, having led its life sciences investment venture. He has previously been president of Amplitude Healthcare Acquisition Corporation, until its merger with Jasper Therapeutics in September 2021. Before this he worked as chief business officer of Iveric bio (previously Ophthotech), where he oversaw acquisitions of gene therapy and therapeutic assets in ophthalmology. Kapoor has also worked at NPS Pharmaceuticals, Genentech and Pfizer. He holds an MBA in Finance and Management from Columbia Business School and a BA in biology from Columbia University.

Ron Martell, president and chief executive officer of Jasper Therapeutics, commented: "With the completion of our successful public offering last month, we are well-positioned to begin carrying out our priority development programmes for briquilimab as a c-Kit targeting therapeutic in chronic diseases and as a novel conditioning agent for stem cell transplant. With substantial resources in place and a clear plan for achieving near-term clinical milestones, it is an excellent time to welcome someone of Vishal's calibre to our board. His track record of successful industry experience speaks for itself, and we look forward to benefiting from his strategic counsel."

Five facts about cardiovascular disease (CVD)

1. CVD is the leading cause of death globally, with one person dying of CVD every 34 seconds in the US. In 2019, approximately 17.9 million people died from CVD worldwide, equating to 32% of all deaths.
2. The main risk factors of CVD, including heart attack and stroke, include unhealthy diet, physical inactivity, tobacco use, excessive alcohol use, stress, family history of CVD, being overweight or obese, ethnic background, high blood pressure and cholesterol; most CVDs can be prevented by reducing these risk factors.
3. Men are more likely to get CVD earlier than women, and the older a patient is the more likely they are to have CVD, and over three-quarters of CVD deaths take place in low- or middle-income countries.
4. There are various types of CVD, including coronary heart disease, cerebrovascular disease, peripheral arterial disease, rheumatic heart disease, congenital heart disease, deep vein thrombosis and pulmonary embolism.
5. The main symptoms of CVD are: chest pain; pain, weakness or numb legs and/or arms; breathlessness; very fast or slow heartbeat, or palpitations; feeling dizzy, lightheaded or faint; fatigue; and swollen limbs.

References:

1. *Visit: cdc.gov/heartdisease/facts.htm*
2. *Visit: who.int/health-topics/cardiovascular-diseases#tab=tab_1*
3. *Visit: who.int/news-room/fact-sheets/detail/cardiovascular-diseases-(cvds)*
4. *Visit: bhf.org.uk/informationsupport/conditions/cardiovascular-heart-disease*

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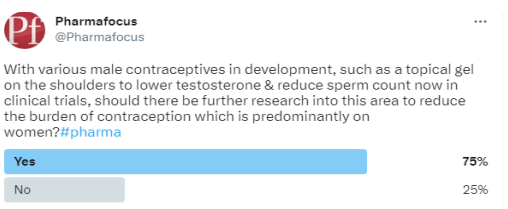

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