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New Crohn's disease treatment could come from neonatal stem cells, research shows

Recent studies have shown that human neonatal stem cells can be used as a treatment for symptoms caused by Crohn's disease, page 10

GSK's RSV vaccine approved by MHRA

GSK has announced that the Medicines and Healthcare products Regulatory Agency (MHRA) has authorised its Arexvy respiratory syncytial virus (RSV) vaccine for active immunisation for the prevention of lower respiratory tract disease (LRTD) caused by RSV in adults over the age of 60.

This approval marks the first time an RSV vaccine for older adults has been approved by the MHRA for use in Great Britain.

The approval follows data from the pivotal AReSVi-006 phase 3 vaccine efficacy trial, which was published in the *New England Journal of Medicine*. In this trial, the vaccine showed high overall efficacy against RSV-LRTD, including in patients with underlying medical conditions. The vaccine was generally well-tolerated, with the most frequently reported adverse events being injection site pain, fatigue, myalgia, arthralgia and headaches – however, these were all generally mild to moderate and transient.

Neale Belson, senior vice president and general manager UK at GSK, commented: "We are very excited by today's announcement. Our ambition is to help protect adults 60 years of age and older in the UK who are at risk from RSV disease, including those with underlying medical conditions, who drive the majority of RSV hospitalisations. This authorisation for Arexvy means eligible adults can be vaccinated against RSV disease for the first time, reinforcing GSK's long history of vaccine innovation."

Future Focus

UKHSA opens new vaccine research centre in Wiltshire to prepare for future pandemics

Ministers have opened a new vaccine research centre at the UK Health and Security Agency's (UKHSA) Porton Down campus in Wiltshire, intended to prepare for 'disease X', the next possible pandemic pathogen.

The state-of-the-art Vaccine Development and Evaluation Centre will house live viruses in specialist containment facilities allowing scientists to access pathogens that do not have vaccines or those that have vaccine programmes that could be improved, such as the flu or monkeypox.

The decision to open this centre followed an announcement from the COVID-19 inquiry that previous governments were ill-prepared for the pandemic, and focused too much on flu outbreaks rather than other viruses.

Professor Dame Jenny Harries commented: "What we're trying to do now is capture that really excellent work from COVID-19 and make sure we're using that as we go forward for any new pandemic threats. […] What we try to do here is keep an eye on the ones that we do know. For example, with COVID-19 we are still here testing all the new variants with the vaccines that have been provided to check they are still effective. But we are also looking at how quickly we can develop a new test that would be used if a brand-new virus popped up somewhere."

Professor Isabel Oliver, UKHSA's chief scientific officer, added: "We know that through scientific advancement, we could detect and control these spreads before they have the impact that COVID-19 had on our lives. It's not easy, but we know that if we strengthen surveillance and if we accelerate the development of diagnostics, vaccines and therapeutics, we could do so much better. We need to be prepared for all threats, including those that have not been detected yet."

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As well as the latest news, this issue also includes an article from Sanofi on how indication-based agreements can remove barriers to oncology treatments (page 16), as well as an article from Phastar considering how research innovations can help improve rare disease treatments (page 14). There is also an article from Veeva about how healthcare practitioners can boost their engagement through field behavioural change (page 19).

In addition, in my article about community pharmacies, I look at why so many have closed their doors and how this is impacting local communities (page 20).

As we move to the end of summer and the nights draw in, I hope you find the opportunity to read this issue while enjoying the last of the sun. We'll be back with the October issue of *Pharmafocus* next month!

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ISSN: 1465-5403

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All subscriptions payable to Samedan. Back copies can be purchased for £15.00 each. *Pharmafocus* is published by Samedan Ltd.

Merck's ebola vaccine, Ervebo, approved by FDA for children

On 3 August 2023, the US Food and Drug Administration (FDA) approved Merck's Ervebo vaccine for children over 12 months. The Ebola Zaire vaccine was approved for over 18s in December 2019 initially, however the Committee for Medicinal Products for Human Use (CHMP) recommended granting further approval to allow children to receive the vaccine.

The duration of protection that the vaccine provides for Zaire Ebolavirus is unknown but will be determined with time, along with its effectiveness. Developers of the vaccine have confirmed that it does not protect against any other species of Ebolavirus, and should not be given to individuals with a history of severe reactions to any of its components.

In January 2021, Merck made a deal with UNICEF to establish the first global Ebola vaccine stockpile to protect against any Zaire Ebolavirus breakouts in the future and aid response efforts. In March 2023, over 500,000 doses of the vaccine were delivered to the stockpile, administered by the International Coordinating Group on Vaccine Provision.

Dr Eliav Barr, senior vice president at Merck Research Laboratories, commented: "Ebola virus disease is contagious and potentially deadly in both children and adults. We're proud of the approval of Ervebo for the prevention of disease caused by Zaire Ebolavirus in children as young as 12 months old, which is another milestone in our continued commitment to help address the global health threat caused by Zaire Ebolavirus."

Treatment programme for CVD to be developed as part of partnership between Novartis and Ionis

Ionis Pharmaceuticals has announced that it has entered into a new collaboration and licence agreement with Novartis, aiming to discover, develop and commercialise a novel medicine for patients with lipoprotein(a) or Lp(a)-driven cardiovascular disease (CVD).

The companies have an existing collaboration for the development and commercialisation of pelacarsen, which Novartis is currently assessing in its phase 3 trial. The new drug is expected to be a potential follow-on to pelacarsen.

Under the terms of the new agreement, Ionis will receive an upfront payment of $60m from Novartis and will be eligible for further development, regulatory and commercial milestone payments as well as tiered royalties. Novartis is solely responsible for the development, manufacturing and commercialisation of the novel therapy.

Brett P Monia PhD, Ionis' chief executive officer, commented: "We are pleased to expand our productive collaboration with Novartis aimed at delivering transformative therapies to patients with elevated Lp(a) who are at high risk of cardiovascular events. This collaboration is designed to leverage Ionis' advancing RNA-targeting platform technologies to deliver a novel Lp(a)-targeting therapy that we expect will provide industry-leading efficacy and dosing frequency."

Contaminated cough syrup leads to WHO Medical Product Alert

The World Health Organization (WHO) has shared a Medical Product Alert for an additional contaminated cough syrup medicine identified in Africa.

The product alert refers to a Naturcold syrup identified in Cameroon and first reported to WHO on the 13 March 2023. The active ingredients in the cough syrup are listed as paracetamol, phenylephrine hydrochloride and chlorpheniramine maleate, a combination often used to relieve symptoms of the common cold, flu and allergic rhinitis.

Samples of the Naturcold syrup were provided to WHO on the 27 June 2023, which were assessed in a WHO contracted and prequalified laboratory. This assessment found that the syrup contained unacceptable levels of diethylene glycol as a contaminant. The samples contained levels of the contaminant of up to 28.6%, while acceptable levels are no more than 0.10%.

The manufacturer of the product is listed as UK-based, operating under the name Fraken International. However, the UK's Medicines and Healthcare products Regulatory Agency (MHRA) has confirmed that this company does not exist in the UK, meaning the origin of the product is still unknown. Inquiries are underway to determine its origin, however at present the manufacturer has not provided any guarantees to WHO on the safety or quality of the products.

Various other cough syrups have also been identified by WHO as having been contaminated, making this the latest in a line of similar Medical Product Alerts.

RNAi treatment for hypertension developed by Alnylam and Roche

Alnylam Pharmaceuticals has announced that it has entered a strategic agreement with Roche for the development and commercialisation of zilebesiran, Alnylam's investigational RNAi therapeutic for the treatment of hypertension in patients with high cardiovascular risk.

The drug is currently in phase 2 of its development, with this partnership allowing for additional development aiming to disrupt the hypertension treatment paradigm globally.

Under the terms of the agreement, Alnylam is expected to receive an upfront payment of $310m and will be eligible to receive further milestone payments in the coming years.

Yvonne Greenstreet MBChB, Alnylam's CEO, commented: "We are thrilled to announce this collaboration, as it combines Alnylam's proven track record in RNAi therapeutics with Roche's global commercial reach, commitment to innovation and desire to transform the landscape for patients with severe cardiovascular diseases. With this collaboration, we now can develop zilebesiran in a more robust way, allowing us to have cardiovascular outcomes data in hand at launch to ensure results relevant not only for health authorities but also for access and clinical practice in order to ultimately reach as many patients as possible."

Teresa Graham, CEO of Roche Pharma, added: "We are excited to work together with Alnylam and leverage our strong R&D capabilities, our leadership in cardiovascular diagnostics and our global commercial footprint to further develop and provide this promising therapy with best-in-disease potential to patients. Throughout our history, we have redefined the standard of care across various disease areas. Together with a strong partner like Alnylam, we are looking forward to making a significant impact for patients living with hypertension at high cardiovascular risk and potentially other cardiovascular indications."

GSK's novel gonorrhoea vaccine gains Fast Track designation from FDA

GSK has announced that the US Food and Drug Administration (FDA) has granted Fast Track Designation for its investigational vaccine for gonorrhoea, which is currently in an ongoing phase 2 trial assessing the efficacy of the vaccine in healthy adults aged 18 to 50 who are considered at risk of contracting gonorrhoea.

As gonorrhoea is the second most prevalent sexually transmitted infection (STI) worldwide, with around 82 million new cases globally each year, the acceleration of vaccine development though the Designation will allow a large percentage of the global population access to an increasingly prevalent drug.

As well as cases in the US increasing 118% from 2009 to 2021, antimicrobial resistance (AMR) to gonorrhoea has also increased over the last 80 years, meaning many antibiotics used against it are now ineffective. With this in mind, vaccines against the STI could help prevent the increasing cases.

Phil Dormitzer, global head of vaccines research and development at GSK, commented: "We welcome the FDA's decision to grant Fast Track designation for our new vaccine candidate against *Neisseria gonorrhoeae* infection. With a high and growing incidence, gonorrhoea is a major concern for sexual and reproductive health around the globe. This designation recognises the potential for a vaccine that could help protect millions of people across the world a gainst the serious health consequences of infection with a bacterium that is considered a 'high priority' pathogen by the World Health Organization (WHO)."

New open innovation hub opened by Astellas for tumour microenvironment research

Astellas Pharma and Mitsui Fudosan have announced that Astellas plans to establish a tumour microenvironment (TME) open innovation hub, intended to open in October 2023. The hub will be based at MITSUI LINK-Lab Kashiwa-no-ha 1, in Kashiwa City, Japan, and operated by Mitsui Fudosan.

The hub, TME iLab, will function as an open innovation hub for research into TME, with the two companies planning to collaborate in order to maximise the benefits of the Kashiwa-no-ha area, which is located close to various leading Japanese medical facilities, such as the National Cancer Center Japan. The companies aim to gain new insights about the TME while producing new innovations based on their research.

Yoshitsugu Shitaka PhD, chief scientific officer at Astellas, commented: "We are pleased to reach an agreement with Mitsui Fudosan. In our new hub of open innovation, we are seeking a wide range of partners with innovative expertise and technologies in tumour microenvironment research. By combining Astellas' capabilities in drug discovery with the knowledge of partners, including academia and venture companies, we expect to accelerate anti-cancer drug research and create innovative drugs. Through these activities, we will also contribute to further growth of the ecosystem in Kashiwa-no-ha smart city."

Kazunori Yamashita, executive managing officer and general manager at Mitsui Fudosan, added: "We sincerely welcome Astellas to establish a hub in Kashiwa-no-ha. In the Kashiwa-no-ha area, a smart city is being built through public-private-academic collaboration, and in the life science area, an ecosystem that generates innovation is being created through the formation of communities to support both cross-collaboration between academia and industry on medical and healthcare-related initiatives and the social implementation of research and development seeds. In the life science area as well, the creation of an ecosystem that fosters innovation is progressing. We, too, will contribute to the development of TME iLab and industrial growth, including the creation of innovative drugs, by working to create a place for promoting innovation and building a community."

Data from Eli Lilly's two phase 3 tirzepatide studies shared

Eli Lilly has announced results from its two phase 3 tirzepatide studies in adult patients with obesity or who are overweight with weight-related comorbidities, excluding type 2 diabetes.

SURMOUNT-3 and SURMOUNT-4 met all primary and key secondary objectives for tirzepatide when compared to a placebo dose. Participants of both trials on the drug who followed intensive lifestyle intervention or who continued tirzepatide treatment, achieved up to 26.6% mean weight loss.

The overall safety profile remained consistent with previously reported SURMOUNT and SURPASS trials and to similar therapies already approved for the treatment of obesity. The most commonly reported adverse events were gastrointestinal-related and were mainly mild to moderate in severity.

Jeff Emmick MD PhD, senior vice president of product development at Eli Lilly, commented: "The results of SURMOUNT-3 and -4 showed the highest level of weight loss observed in the SURMOUNT programme to date. Whether taking tirzepatide for 88 weeks in SURMOUNT-4 or taking tirzepatide for 72 weeks following intensive caloric restriction in SURMOUNT-3, participants achieved similar mean weight reduction – about 26%. The findings from SURMOUNT-3 challenge the notion that patients living with obesity or overweight can achieve their weight loss goals with diet and exercise alone. Additionally, the findings from SURMOUNT-4 reinforce that obesity should be regarded like other chronic diseases where chronic therapy may be needed to maintain treatment benefits."

First official long COVID-19 treatment could emerge from the NIH's RECOVER initiative

As of 31 July 2023, The National Institutes of Health (NIH) launched the first trials of its 'Researching COVID to Enhance Recovery' (RECOVER) initiative. This consists of four trials named RECOVER-NEURO, RECOVER-VITAL, RECOVER-SLEEP and RECOVER-AUTONOMIC. The studies have recruited over 24,000 participants and Dr Kanecia Zimmerman from Duke University, US has confirmed that there has been "good feedback from the US Food and Drug Administration (FDA), as well as others".

RECOVER-NEURO is one of the first to open enrolment and intends to reduce brain fog and memory-related problems using a web-based brain training programme that improves cognitive function. RECOVER-VITAL, also one of the first to be launched, focuses on targeting SARS-CoV-2, the virus responsible for causing COVID-19.

The remaining two trials are set to be launched within the next few months; RECOVER-SLEEP will test the effectiveness of drugs such as melatonin, modafinil and solriamfetol in improving sleep, as well as an educational coaching system. The final trial, RECOVER-AUTONOMIC, will examine treatments for the autonomic nervous system; for example, using intravenous immune globulin to aid the immune system.

The trials were initially supposed to launch at the beginning of 2023, however it took researchers a while to understand long COVID-19 well enough to formulate treatments for it. In addition, the study design had to approved by numerous governing bodies before it was officially released to the public.

While this should lead to positive outcomes, the Long-COVID Alliance has expressed its concern that the NIH has not presented a solid timeline for the trial results, meaning it will be at least a year before any positive results come through.

Dr Ziyad Al-Aly, from Washington University. US, commented: "It is a bit too late, but it's certainly helping us move the ball forward… I wish they had the sense of urgency to get us to this stage two years ago."

Study shows Wegovy by Novo Nordisk reduces cardiovascular risk

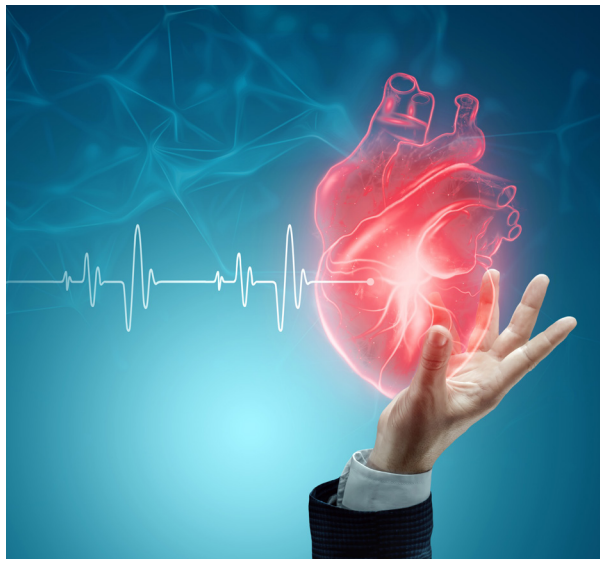
Novo Nordisk has shared results from the SELECT cardiovascular outcomes trial, which show that a once-weekly subcutaneous dose of Wegovy (semaglutide 2.4mg) reduced the risk of major adverse cardiovascular events (MACE) by 20% in overweight or obese adults over the age of 45 with established cardiovascular disease (CVD) and no prior history of diabetes.

The double-blind trial enrolled 17,604 adult patients and compared the drug to a placebo dose, showing a statistically significant and superior reduction in MACE of 20% for those treated with Wegovy compared to the placebo.

Throughout the trial, the drug appeared to be safe and well-tolerated, consistent with previous trials.

Martin Holst Lange, executive vice president for Development at Novo Nordisk, commented: "People living with obesity have an increased risk of CVD but to date, there are no approved weight management medications proven to deliver effective weight management while also reducing the risk of heart attack, stroke or cardiovascular death. Therefore, we are very excited about the results from SELECT showing that semaglutide 2.4mg reduces the risk of cardiovascular events. SELECT is a landmark trial and has demonstrated that semaglutide 2.4mg has the potential to change how obesity is regarded and treated."

The company expects to file for regulatory approvals of Wegovy for this indication expansion in the US and EU before the end of 2023, with detailed results from this trial to be presented later this year.



Phase 2 trial data from GBS maternal vaccine candidate shared by Pfizer

Pfizer has announced new data from its phase 2 study, which assessed its hexavalent capsular polysaccharide (CPS) conjugate Group B Streptococcus (GBS) vaccine candidate, GBS6, in development for maternal vaccination in order to protect infants against invasive GBS disease.

This phase of the study enrolled 360 healthy pregnant volunteers. GBS6 triggered robust maternal antibody responses against all six GBS CPS serotypes included in the vaccine. The antibodies were efficiently transferred to the infants at ratios of between 0.4-1.3 depending on GBS6 group.

Results from the study were published in *The New England Journal of Medicine (NEJM)*, and will inform the company's planned phase 3 clinical development programme.

The safety profile of the vaccine was similar between the vaccine and placebo groups, with local reactions generally remaining mild or moderate and of short duration.

Annaliesa Anderson PhD, senior vice president and chief scientific officer of vaccine research and development at Pfizer, commented: "Group B Streptococcus can cause potentially devastating diseases in infants, including sepsis, pneumonia and meningitis. Annually, there are nearly 400,000 cases of infant disease and approximately 138,000 stillbirths and infant deaths worldwide due to GBS. The findings published in NEJM provide hope that maternal vaccination with GBS6 may protect infants against GBS, potentially helping to prevent thousands of cases of illness annually, if it is successfully developed and approved. Building on decades of expertise and knowledge in vaccines, we are committed to helping protect newborns and young infants through maternal immunisation."

Phase 3 trial results for datopotamab deruxtecan for NSCLC treatment announced by AstraZeneca

AstraZeneca has announced results from its TROPION-Lung01 phase 3 trial for its TROP2-directed antibody drug conjugate, developed in collaboration with Daiichi Sankyo. The drug demonstrated statistically significant improvement in progression-free survival compared to standard chemotherapy in patients with previously treated locally advanced or metastatic non-small cell lung cancer (NSCLC) treated with at least one prior therapy.

The dual primary endpoint of overall survival (OS) did not have sufficient supporting evidence, as the data was not mature and an early trend was observed in favour of the drug, which did not appear to meet the prespecified threshold for statistical significance at this interim analysis. The trial is expected to continue to assess OS with greater maturity.

The drug's safety profile remained consistent with previous clinical trials, and no new safety signals were identified.

Susan Galbraith, executive vice president of Oncology R&D at AstraZeneca, commented: "With TROPION-Lung01, we met the dual primary endpoint of progression-free survival, challenging the entrenched standard of care in a previously treated and unselected patient population that has long deserved an alternative to chemotherapy. These first phase 3 trial results from the datopotamab deruxtecan clinical programme provide compelling evidence for the potential role this TROP2-directed antibody drug conjugate can play in treating patients with lung cancer."

Ken Takeshita, MD and global head of Oncology R&D at Daiichi Sankyo, added: "We are encouraged by the statistically significant results of the dual primary endpoint of progression-free survival seen with datopotamab deruxtecan and look forward to the final overall survival analysis. We plan to share these data with regulatory authorities to discuss next steps."

Breast cancer treatment phase 3 trial results shared by Merck

Merck, also known as MSD outside of the US and Canada, has announced positive results from the phase 3 KEYNOTE-756 trial, which assessed Keytruda in combination with chemotherapy for the treatment of patients with high-risk, early-stage oestrogen receptor-positive, human epidermal growth factor receptor 2-negative (ER+/HER2-) breast cancer.

The drug met one of its primary endpoints of pathological complete response (pCR) rate in ER+/HER2- breast cancer patients, and demonstrated a statistically significant improvement in pCR rate compared to neoadjuvant placebo plus chemotherapy.

The trial will now continue to assess the other primary endpoint of event-free survival (EFS).

Gursel Aktan, vice president of global clinical development at Merck Research Laboratories, commented: "This is the first positive phase 3 study evaluating an immunotherapy-based regimen for patients with high-risk, early-stage ER+/HER2- breast cancer, and an important milestone in our efforts to advance research in early-stage breast cancer. We look forward to sharing the detailed results with the medical community and thank the patients and investigators for their important contributions to this study."

Fatima Cardoso, director of the Breast Unit of the Champalimaud Clinical Centre, Portugal and co-principal investigator on the trial, added: "While significant advancements have been made in the treatment of ER+/HER2- breast cancer, people diagnosed with high-risk disease as assessed by clinical and pathologic criteria typically have a worse prognosis and limited options before surgery. Data from KEYNOTE-756 suggest that adding pembrolizumab [Keytruda] to neoadjuvant chemotherapy before surgery can significantly improve the pCR rate compared to neoadjuvant chemotherapy alone for people with high-risk, early-stage ER+/HER2- breast cancer."

Jemperli by GSK approved to treat endometrial cancer

GSK has announced that the US Food and Drug Administration (FDA) has approved Jemperli (dostarlimab) in combination with carboplatin and paclitaxel for the treatment of adult patients with primary advanced or recurrent endometrial cancer that is mismatch repair deficient (dMMR) or microsatellite instability-high (MSI-H).

The company previously submitted a supplemental Biologics License Application (sBLA) supporting the new indication, which received Priority Review and was approved ahead of its Prescription Drug User Fee Act action date.

This approval means Jemperli is now indicated earlier in the treatment programme along with chemotherapy for patients with dMMR/MSI-H primary advanced or recurrent endometrial cancer.

Hesham Abdullah, senior vice president and global head of Oncology Development at GSK, commented: "Today's expanded approval of Jemperli redefines the treatment landscape for patients with dMMR/MSI-H primary advanced or recurrent endometrial cancer. Until now, chemotherapy alone has been the standard of care with many patients experiencing disease progression. In the RUBY trial, Jemperli plus chemotherapy demonstrated a 71% reduction in the risk of disease progression or death versus chemotherapy in this patient population, providing a statistically significant and clinically meaningful benefit. These results and today's approval underscore our belief in the potential for Jemperli to transform cancer treatment as a backbone immuno-oncology therapy."

Wenora Johnson, president of the board of directors of Facing Our Risk of Cancer Empowered (FORCE), added: "The endometrial cancer community is thrilled by today's news, which changes the treatment paradigm for a population with long-term unmet needs. FORCE is grateful for the many participants and researchers who contributed to this important study. As an endometrial cancer survivor, I know how much this approval offers hope for patients with primary advanced or recurrent dMMR/MSI-H endometrial cancer."

Alzheimer's disease drug Leqembi given full approval by FDA

Japanese Eisai and US-based biotechnology company Biogen have announced that the US Food and Drug Administration (FDA) has given their Alzheimer's disease (AD) drug Leqembi (lecanemab-irmb) full approval through the approval of a supplemental Biologics License Application (sBLA). This will allow wider medical insurance coverage in the US.

Leqembi is a humanised immunoglobulin gamma 1 (IgG1) monoclonal antibody directed against aggregated soluble and insoluble forms of amyloid beta (Aβ). It works by targeting and clearing the most neurotoxic form of Aβ that accumulates on the brain, as well as removing existing plaque. While the drug cannot reverse memory loss and other related side effects, it has shown to slow clinical decline.

In order to receive the approval, the companies conducted the phase 3 CLARITY AD multicentre, randomised clinical trial, which enrolled 1,795 patients with AD to verify the drug's effectiveness. The primary endpoint was the global cognitive and functional scale, Clinical Dementia Rating Sum of Boxes (CDR-SB), with the secondary endpoint being AD Cooperative Study-Activities of Daily Living Scale for Mild Cognitive Impairment (ADCS MCI-ADL), measured by the carers of the patients. Overall the trial found a 'statistically significant and clinically meaningful reduction' in cognitive decline in patients taking Leqembi.

Haruo Naito, CEO at Eisai, commented: "Today, the FDA approved Leqembi under the traditional approval pathway, making [it] the first and only approved anti-amyloid AD treatment shown to reduce the rate of disease progression and to slow cognitive impairment in the early and mild dementia stages of the disease. As a research and development-focused company based on our human healthcare (HHC) concept, we are proud that the results of Eisai's AD research over the past 40 years have been recognised and delivered to people living with this disease in the US. AD is a progressive, fatal disease that greatly impacts not only the people living with it, but also their loved ones, care partners and society. We continue to work to create broad and simple access to Leqembi for patients and to support diagnosis and treatment at the early stage of the disease. Eisai will diligently work to educate physicians on the safe and appropriate use of Leqembi to maximise its benefit to people living with early AD and their families."

Christopher A Viehbacher, president and CEO of Biogen, stated: "Today marks a breakthrough in the treatment of AD, and we are proud to be at the forefront of ushering in a new era of advances for a disease that was previously considered untreatable. We would like to express our sincere appreciation to those who have worked tirelessly to find a treatment for this unrelenting disease, without whom this progress would not be possible. Our focus is now on the path forward, working alongside Eisai with the goal of making Leqembi accessible to eligible patients as soon as possible."

First over the counter contraceptive pill approved by FDA

The US Food and Drug Administration (FDA) has announced that it has approved the Opill (norgestrel) tablet for non-prescription use to prevent pregnancy. This approval makes Opill the first daily oral contraceptive approved for use in the US without a prescription, meaning it will be available over the counter (OTC) at pharmacies, supermarkets and other stores, as well as online.

The efficacy of the drug was established in 1973 when norgestrel was approved for prescription use, however HRA Pharma has applied to switch this to an OTC product. For an OTC approval, the FDA requires proof that the product can be used by consumers safely and effectively, relying only on the non-prescription drug labelling. Studies showed that consumer understanding was high overall for Opill.

Common side effects of Opill include irregular bleeding, headaches, dizziness, nausea, increased appetite, abdominal pain, cramps and bloating.

Patrizia Cavazzoni MD, director of the FDA's Center for Drug Evaluation and Research, commented: "Today's approval marks the first time a non-prescription daily oral contraceptive will be an available option for millions of people in the United States. When used as directed, daily oral contraception is safe and is expected to be more effective than currently available non-prescription contraceptive methods in preventing unintended pregnancy."

This approval is intended to reduce barriers to access by allowing individuals to access contraceptives without needing to see their healthcare providers first.

Bristol Myers Squibb's Sotyktu recommended to treat adult patients with psoriasis on the NHS

Bristol Myers Squibb (BMS) has announced that the National Institute for Health and Care Excellence (NICE) has recommended Sotyktu (deucravacitinib) for use on the NHS in England as a new treatment option for adult patients with moderate-to-severe plaque psoriasis.

The once-daily oral tablet will be available to patients whose Psoriasis Area and Severity Index (PASI) is ten or more and the Dermatology Life Quality Index (DLQI) is ten or more, and if their condition has not responded to other treatments including ciclosporin, methotrexate and phototherapy, or these treatments are contraindicated or not tolerated.

This NICE recommendation follows marketing authorisation in Great Britain in May 2023 for this indication.

Professor Chris Griffiths, emeritus professor at the University of Manchester, commented: "Today's announcement marks another step forward for people with psoriasis. This complex condition can affect each person differently, therefore it is my hope that access to a greater variety of treatments, such as deucravacitinib, will enable eligible patients to have more choice, with therapies that may suit their daily needs and lifestyle."

Laura Stevenson, deputy chief executive at Psoriasis Association, added: "It is estimated that psoriasis affects over a million people across the UK and for some, it can have a significant life impact. It is therefore hugely important for the community to have access to a variety of treatments, including new therapies such as deucravacitinib. The availability of deucravacitinib for adults with moderate-to-severe plaque psoriasis may make a real difference for eligible people with psoriasis and add to their potential treatment options. The Psoriasis Association is a national charity, here to provide patients with important new information as well as support with their condition."

FDA approves first cellular therapy for treatment of patients with type 1 diabetes

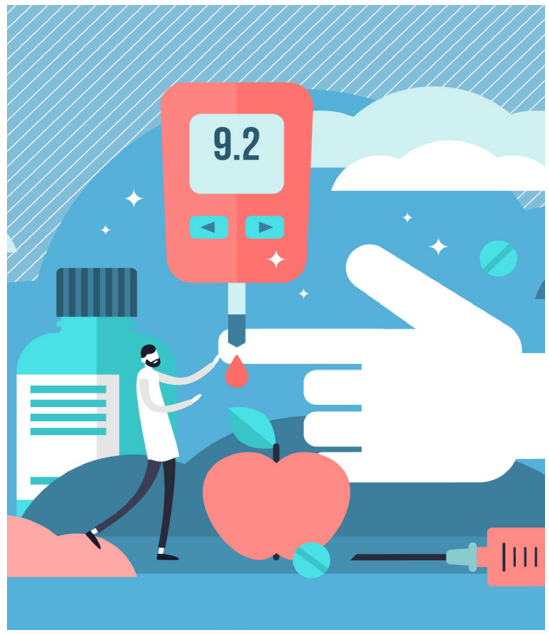
The US Food and Drug Administration (FDA) has announced that it has approved Lantidra, the first allogeneic (donor) pancreatic islet cellular therapy made from deceased donor pancreatic cells for the treatment of adult patients with type 1 diabetes.

The drug is approved for adult patients with type 1 diabetes who are unable to approach target glycated haemoglobin (average blood glucose levels) due to current repeated episodes of severe hypoglycaemia (low blood sugar) regardless of other diabetes management and education.

Some patients struggle to manage the insulin doses they need daily to prevent hyperglycaemia (high blood sugar) without causing hypoglycaemia, and others may develop hypoglycaemic unawareness meaning they are unable to detect a drop in their blood glucose levels – giving them little chance to prevent this from further dropping and making it difficult to correctly dose insulin. This approval of Lantidra provides a new potential treatment option for these patients.

The safety and efficacy of the drug was assessed in two non-randomised, single-arm studies which comprised 30 participants with type 1 diabetes and hypoglycaemic unawareness. Of these patients, 21 did not need to take insulin for at least a year, with 11 not needing it for one-to-five years and ten participants not needing it for more than five years.

Peter Marks MD PhD, director of the FDA's Center for Biologics Evaluation and Research, commented: "Severe hypoglycaemia is a dangerous condition that can lead to injuries resulting from loss of consciousness or seizures. Today's approval, the first-ever cell therapy to treat patients with type 1 diabetes, provides individuals living with type 1 diabetes and recurrent severe hypoglycaemia an additional treatment option to help achieve target blood glucose levels."



CHMP grants positive opinion to ViiV Healthcare's cabotegravir for HIV prevention

GSK has announced that ViiV Healthcare has received a positive opinion from the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP), recommending marketing authorisation for cabotegravir long-acting (LA) injectable and tablets for HIV prevention.

Cabotegravir is recommended in combination with safe sex practices as a pre-exposure prophylaxis (PrEP) with the aim of reducing the risk of sexually acquired HIV-1 infection in high-risk adults and adolescents who weigh over 35kg.

This decision is based on data from two phase 2b/3 muticentre, randomised, double-blind, active controlled studies, which assessed the drug's safety and efficacy as PrEP for HIV-negative men who have sex with men, transgender women and cisgender women who were at an increased risk of contracting HIV.

Kimberly Smith MD, head of research and development at ViiV Healthcare, commented: "The expansion of prevention options is critical if we are to end the HIV epidemic. LA options have the potential to play an important role in reducing challenges such as inconsistent adherence to taking daily pills, and stigma associated with oral PrEP use that can be faced by people who could benefit from PrEP. At ViiV Healthcare we are at the forefront of cutting-edge science, developing innovative solutions to address the biggest unmet needs in HIV prevention. With the CHMP positive opinion, we are hopeful that people in Europe will soon be able to benefit from greater choice."

Data shared by Sanofi about new drug for treatment of haemophilia A in children

Sanofi has shared new data from the phase 3 XTEND-Kids study, which assessed Altuviiio (Antihemophilic Factor (Recombinant), Fc-VWF-XTEN Fusion Protein) once-weekly prophylaxis in patients under the age of 12 with previously treated severe haemophilia A.

This data was presented at the Annual Meeting of the International Society on Thrombosis and Haemostatis (ISTH) in Montreal, Canada.

The drug is a first-in-class, high-sustained factor VIII replacement therapy indicated for once-weekly treatment, compared to standard of care for paediatric patients who often need injections two to four times per week.

Having been approved by the US Food and Drug Administration (FDA) for routine prophylaxis and on-demand treatment for adults and children with haemophilia A in February 2023, Altuviiio has now met the primary endpoint of the phase 3 trial with no inhibitor development to factor VIII detected, as well as meeting key secondary endpoints.

Lynn Malec MD, medical director of the Comprehensive Center for Bleeding Disorders, associate investigator at The Versiti Blood Research Institute and associate professor of Medicine and Pediatrics at The Medical College of Wisconsin, US, commented: "The results from XTEND-Kids mark an important breakthrough as we strive for optimised bleed protection as the standard of care. Achieving high-sustained factor activity with once-weekly dosing means a freedom from the trade-offs between treatment burden and efficacy we often see in treating severe haemophilia A. The reliable and consistent bleed protection Altuviiio provides offers confidence for children living with haemophilia and their families to manage haemophilia with less worry."

Karin Knobe MD PhD, therapeutic area head, Rare Diseases and Rare Blood Disorders at Sanofi, added: "In an effort to reduce their risk of bleeding episodes, many children living with haemophilia A are currently limited in their ability to fully participate in daily activities. This burden is compounded by the challenge of administering prophylactic treatments intravenously multiple times a week. Today's XTEND-Kids results reinforce the ability of Altuviiio to provide effective bleed protection with once-weekly dosing and reinforce our commitment to developing new treatment options designed to redefine the standard of care for people living with rare blood disorders."

American Heart Association grants $2.1m for research into possible link between CVD, migraines and strokes

The American Heart Association has announced that it will fund seven new scientific research studies to learn more about the link between migraines, strokes and cardiovascular disease (CVD).

Prior research has shown that some types of migraine can increase the risk of strokes, as well as there being evidence to suggest that they can lead to other types of CVD. Therefore, the American Heart Association is providing $2.1m in grants for seven research projects from Brigham and Women's Hospital in Massachusetts; Mayo Clinic in Minnesota; Mayo Clinic in Arizona; Duke University School of Medicine in North Carolina; Rhode Island Hospital in Providence; the University of South Carolina in Columbia; and Yeshiva University in New York City, all US.

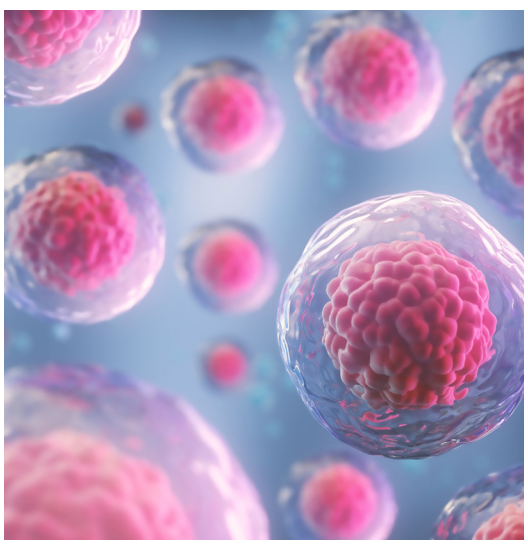
The projects began on 1 July 2023, and are funded for up to two years.

Mitchell S V Elkind MD MS FAHA, chief clinical science officer of the American Heart Association, commented: "According to the American Migraine Foundation, 39 million people in the US experience migraines. And while evidence suggests that migraines increase the risk of strokes and possibly other types of CVDs, there is still much we don't know about what causes this increased risk. With these new research grants, we hope to create a collaborative group of experts in migraine, cardiovascular disease, stroke, biostatistics and data science to explore these unanswered questions about the cardiovascular complications of migraine."

New Crohn's disease treatment could come from neonatal stem cells, research shows

Recent studies from the Ann & Robert H Lurie Children's Hospital in Chicago have discovered that human neonatal cardiac-derived mesenchymal stem cells can induce wound-healing and reduce inflammation in the digestive system, caused by Crohn's disease.

The researchers used a mouse model to trial the potential treatment and found that injecting the cells into the inflammatory lesions was a successful method of administration. However, this requires surgical procedures; in order for this treatment to be used officially with humans, researchers would have to develop a safer way for the cells to be received. Doctors intend to inject the stem cells through a vein in the arm, although more animal trials will be necessary before clinical trials can begin.



While alternative treatments for Crohn's disease are available, such as various medication or surgeries for the digestive system, the neonatal mesenchymal stem cells would avoid the severe side effects that typically come with the medication, such as gastrointestinal dysfunction.

Arun Sharma PhD, from the Stanley Manne Children's Research Institute, commented: "Our results are encouraging and definitely provide a new platform to potentially treat aspects of chronic inflammatory bowel diseases." He confirmed that the goal is to "utilise cell type as treatment and as a preventive measure before symptoms can develop", and is sure that the "potential is enormous".

Researchers on the project believe these cells could be used as possible treatment for other inflammatory diseases and are excited for what this development could mean for the future.

Beclu carbon neutral asthma inhaler launched by Lupin Healthcare

UK-based subsidiary of Lupin Limited, Lupin Healthcare, has announced the UK launch of its carbon-neutral pressurised metered dose inhalers (pMDI) for the treatment of adult and paediatric patients with asthma.

The inhaler, Beclu (beclometasone dipropionate), is available in both 100 micrograms (mcg) and 200mcg, with Beclu 100 indicated for adults and children and Beclu 200 indicated solely for adult use.

This launch is intended to provide cost savings for the NHS, as well as being a certified carbon-neutral inhaler due to the company's carbon offsetting. Currently, around 5.4 million people in the UK are receiving asthma treatments, equalling one in every 12 adults and one in every 11 children, which places a burden of approximately £3bn on the NHS each year.

The company has undertaken an independent Life Cycle Assessment (LCA) by Carbon Footprint Ltd, which calculated the carbon footprint of all Beclu inhalers sold in the UK, allowing it to ensure that it can offset this footprint. This makes Beclu pMDI the only carbon-neutral beclometasone dipropionate pMDIs currently available.

Ben Ellis, general manager at Lupin Healthcare, commented: "This additional pMDI launch demonstrates Lupin's commitment to respiratory and to delivering value to the NHS. Whilst carbon reduction is core to our long-term carbon reduction plan, providing this carbon-neutral product we believe demonstrates our environmental commitments."

AI-enabled drug discovery developments trigger partnership between Recursion and NVIDIA

Clinical stage biotech company Recursion has announced a $50m investment by NVIDIA, intended to accelerate the development of its AI foundation models for biology and chemistry, working in collaboration with NVIDIA to optimise and distribute these AI models to biotechnology companies through NVIDIA's cloud services.

Recursion intends to use its biological and chemical data set to accelerate the training of foundation models on NVIDIA DGX Cloud for commercial licence/release on BioNeMo (NVIDIA's cloud service for generative AI in drug discovery). NVIDIA is also expected to help optimise and scale the foundation models using its AI and computing expertise.

Chris Gibson PhD, co-founder and CEO of Recursion, commented: "Our collaboration with NVIDIA represents two best-in-class companies coming together to help solve one of the world's most difficult challenges, drug discovery. With our powerful data set and NVIDIA's accelerated computing capabilities, we intend to create groundbreaking foundation models in biology and chemistry at a scale unlike anything that has ever been released in the biological space."

Jensen Huang, founder and CEO of NVIDIA, added: "Generative AI is a revolutionary tool to discover new medicines and treatments. We are delighted to collaborate with Recursion's world-class team, which is doing pioneering work in digital biology and chemistry with NVIDIA DGX and NVIDIA AI software to accelerate the development of the world's largest biomolecular generative AI models and speed drug discovery for biotech and pharmaceutical companies."

3D printed pills to enable time-controlled release in development

A team of scientists at the Max Planck Institute (MPI) for Informatics, Germany, and the University of California, US, have developed a process of 3D printing pills in unusual shapes, allowing a time-controlled release as the object's shape means it will dissolve in a predetermined manner.

This development aims to address issues with controlling patients' levels of pharmaceutical drugs, which is often problematic with orally administered drugs. Advances in 3D printing have allowed this team of scientists to create complex shapes and free-form drugs, with the aim to regulate constant distribution of the biochemicals.

This project has been led by Dr Vahid Babaei, from the MPI for Informatics, and Professor Julian Panetta at the University of California. The 3D printing technology has allowed their teams to produce pills that dissolve in specific amounts of time, releasing their content in more controlled ways, allowing them to set predetermined drug concentrations in orally delivered drugs.

According to the press release: "Since no external influence is possible after ingestion in the digestive tract, the desired time-dependent drug release must be generated by the shape (active surface that dissolves) of the specimen. With some effort, the time-dependent dissolution can be calculated from a given geometric shape."

The release concludes that this technology is not only useful for pharmaceuticals but "further possibilities include the production of catalytic bodies or even coarse granular fertilisers".

Rare disease treatments to be researched in partnership between Pfizer and AstraZeneca's Alexion

Alexion, AstraZeneca's Rare Disease division, has announced that it has entered a definitive purchase and licence agreement with Pfizer. The agreement covers a portfolio of preclinical gene therapy programmes and enabling technologies, and furthers the company's commitment to advancing next-generation genomic medicines.

Under the terms of the agreement, Alexion will receive a number of novel adeno-associated virus (AAV) capsids, which have been proven to work effectively at delivering therapeutic gene cargos for gene therapy and editing.

This agreement is intended to strengthen Alexion and AstraZeneca's capabilities in genomic medicine, which has recently been supported by the acquisition of LogicBio. AstraZeneca additionally hopes to acquire talent from Pfizer associated with the gene therapy portfolio.

Alexion's chief executive officer, Marc Dunoyer, commented: "Today's announcement represents another major step forward in Alexion and AstraZeneca's ambition to be an industry leader in genomic medicine, which has potential to be transformative and even curative for patients with devastating diseases. We look forward to continuing our work to develop enhanced platforms and technologies with broad therapeutic application while integrating best-in-class expertise to accelerate promising therapeutics into the clinic."

Biogen plans to acquire Reata Pharmaceuticals for $7.3bn

Biogen and Reata Pharmaceuticals have announced that they have entered a definitive agreement under which Biogen will acquire Reata for approximately $7.3bn, or $172.50 per share.

Reata has a focus on developing therapeutics to regulate cellular metabolism and inflammation in serious neurologic diseases; the company's drug Skyclarys (omaveloxolone) is the only treatment to be approved by the US Food and Drug Administration (FDA) for the treatment of Friedreich's ataxia (FA). The company is also in the process of developing a portfolio of products for various neurological disorders.

Christopher Viehbacher, Biogen's president and CEO, commented: "With extensive expertise in rare disease product development and global commercialisation, as demonstrated by Spinraza

and the recent launch of Qalsody, we believe Biogen has the foundation in place to accelerate the delivery of Skyclarys to patients around the world. This is a unique opportunity for Biogen to bolster our near-term growth trajectory, and Skyclarys is an excellent complement to our global portfolio of treatments for neuromuscular and rare disease."

Warren Huff, chairman and CEO of Reata, added: "Biogen's expertise and commercial footprint make it the optimal choice to help Skyclarys realise its full potential. With its clear understanding of the rare disease patient journey and existing commercial infrastructure, we believe Biogen will establish Skyclarys as the standard of care in the treatment of this devastating genetic disease."

Eli Lilly shares plans to acquire Versanis Bio for $1.9bn

Eli Lilly and Versanis Bio have announced a definitive agreement for Lilly to acquire Versanis, a private clinical-stage biopharmaceutical company focusing on the development of new medicines to treat cardiometabolic diseases.

Under the terms of the companies' agreement, Versanis shareholders could receive up to $1.925bn, including an upfront payment as well as subsequent payments dependent on the achievement of some development and sales milestones.

Versanis' lead asset is bimagrumab, a monoclonal antibody that binds activin type 2 A and B receptors to bloc activin and myostatin signaling. This antibody is currently being evaluated in the phase 2b BELIEVE study, both alone and in combination with semaglutide in

adults who are overweight or obese.

Ruth Gimeno, PhD, group vice president of diabetes, obesity and cardiometabolic research at Lilly, commented: "Lilly is committed to investigating potential new medicines to fight cardiometabolic diseases, including obesity, a chronic disease that affects over 100 million Americans. By unifying the knowledge and expertise in incretin biology at Lilly with the deep understanding of activin biology at Versanis, we aim to harness the potential benefits of such combinations for patients."

Mark Pruzanski, MD, Versanis' chairman and CEO, added: "It has been a privilege for our team to advance bimagrumab to address one of the greatest health crises of our time. As a global leader developing life-changing medicines, Lilly is ideally positioned to realise the potential of bimagrumab in combination with its incretin therapies to benefit people living with cardiometabolic diseases."

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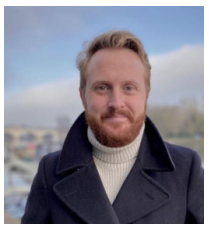
Indication-based agreements – A key enabler of UK access to combination therapies in oncology



Jonathan Bowen from Sanofi UK and Ireland explains how indication-based agreements can remove barriers to patient access to combination cancer therapies

Combination therapies can provide important clinical benefits, yet NHS reimbursement remains a challenge and cancer patients are missing out on access to some of these treatments.[1,2] Indication-based agreements have the potential to remove one of the barriers to patient access to combination therapies in cancer. At Sanofi, we would welcome the opportunity to work with the Department of Health and Social Care, NHS England, the National Institute for Health and Care Excellence (NICE), the All Wales Medicines Strategy Group (AWMSG), the Scottish Medicines Consortium (SMC), patient groups, the pharmaceutical industry and other interested stakeholders to develop a framework for combination therapies, which reflects the need for new combinations or treatment options to be assessed fairly.

Combination therapies represent the future of cancer care

Around one in two people will develop some form of cancer during their lifetime.[3] Despite considerable progress in prevention, diagnosis and treatment, the burden of cancer is continuing to increase.[4] Every two minutes, someone in the UK is told "you have cancer", and between now and 2040, UK cancer cases are projected to increase by 2%.[4] In 2020, more than one in four UK deaths were attributed to cancer, which is the equivalent to a life lost to cancer every four minutes.[5]

These figures make it clear why cancer is a national priority, with the NHS providing cancer care to millions of people around the clock. The NHS Long Term Plan has set a goal that by 2028, an additional 55,000 people per year will survive their cancer for at least five years after diagnosis.[6]

Combination therapies are becoming increasingly central to cancer treatment, with many life sciences companies focusing research and development efforts on investigating different options.[7] Bringing together two or more medicines that work in different ways can enhance the effectiveness of treatments compared to each medicine alone.[1,8,9] The use of combination therapies will be key to improving patient outcomes in the future and achieving the NHS's long-term ambitions in cancer.

Combination therapies are unable to fit within the UK's pricing and reimbursement framework

> **Combination therapies often bring significant clinical benefit to patients and even now represent a cornerstone of cancer care**

While positive efforts have been made to support access to innovative medicines on the NHS, the evolution of the UK's pricing and reimbursement framework has not always kept pace with scientific advances.

A key challenge is that when two or more medicines – one of which has likely been assessed by NICE in the indication already – are brought together, it is only possible to negotiate the price of the new component. This makes it difficult, and sometimes impossible, for a combination to be considered cost-effective so it can be routinely prescribed to UK patients. As a result, the UK is falling behind in access to innovative combination therapies compared to similar countries across Europe.[10]

In the UK, a medicine can only have a single net price across all its indications, which is challenging for combination therapies

As drug development has evolved, more therapies are being used to treat multiple cancers at different stages of disease and in combinations that may not always be the same, depending on the indication.

NHS policy states that once the price of a medicine has been agreed, it must be applied to all its uses, both retrospectively (to previously agreed indications) and prospectively (to all future indications), whether the indication is for combination oncology or used on its own in any other disease area. Flexibility can be considered in exceptional circumstances under NHS England's Commercial Framework. However, the criteria for pricing flexibility are challenging to meet, particularly for cancer combinations, since treatments must fall significantly below the standard NICE threshold of cost-effectiveness to qualify.

Demonstrating cost-effectiveness within the current NICE framework can be challenging for cancer combinations. The extended survival often provided means patients are treated for longer and receive both (or more) medicines for a greater amount of time. The costs of the new component(s) are therefore added to the increased costs of the existing component. The inability to price each component within the combination to appropriately reflect its value under the current framework may mean that these medicines won't ever reach patients. In some instances, for example, where the existing component is already reimbursed in the indication at the upper limit of the cost-effectiveness threshold, even if the new component was given away at zero price, the combination still wouldn't be cost-effective.[11]

While the Association of the British Pharmaceutical Industry (ABPI) and the pharmaceutical industry are engaging to find a solution that allows manufacturers of the different components of a combination to enter into pricing dialogue in a way that is compliant with competition law, these efforts will fail if the current system continues to prevent the agreement of a combination-specific price.

Rigid pricing arrangements do not incentivise companies to invest in combinations, if they know NHS reimbursement is not possible. The outcome of this is that the UK is prevented from realising the full value of innovations and patients are losing out on promising new treatments. In fact, since 2016, around half of terminated NICE appraisals for cancer treatments have been combinations.[2]

Indication-based agreements are a key part of the solution to increasing patient access to cancer combination therapies

Indication-based agreements refer to a mechanism whereby the price of a medicine is agreed based on its clinical benefits when it is used in different ways.[12] This pricing arrangement provides greater flexibility, enabling the NHS to recognise the varying value of a medicine in each indication. Indication-based agreements will allow the true potential of innovative new therapies to be realised and will restore the UK as a destination where cancer patients can access the latest treatments.

Indication-based agreements are expected to improve access to combination therapies because they will help companies set a combination-specific price for their medicine, reflective of its value when used with another treatment. The ability to do so will increase the likelihood of the combination being considered cost-effective, in turn providing cancer patients with more options they might not previously have had.[12]

At the same time, investment into research and development will likely be encouraged to explore more niche indications, with smaller patient populations, since the prospect of a combination-specific price might mean these indications have a better probability of obtaining reimbursement.

A policy and infrastructure shift are required, but it is possible and much needed

Combination therapies often bring significant clinical benefit to patients and even now represent a cornerstone of cancer care.[1,8,9] Introducing indication-based agreements would mean any changes in price required to demonstrate cost-effectiveness for the component medicines in the combination do not affect their price in other indications, and have the potential to be a key enabler in bringing many combination therapies to cancer patients. Given the move towards improved use of data across the NHS, we have a great opportunity to implement new targeted systems that can track the patient's disease at the point of care. Doing so would facilitate access to value-based and personalised interventions such as cancer combination therapies.

Author bio

Dr Jonathan Bowen *trained at UCL Medical School before transitioning to a career within Medical Affairs in the pharmaceutical industry. He currently works as the medical lead for Haemato-Oncology at* ***Sanofi*** *for the UK and Ireland affiliate.*

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How statistical and data analysis can support research innovations in rare diseases

Giles Partington, Lindsay Govan, Paddy O'Hara, Emily Foreman and Jennifer Visser-Rogers from Phastar consider how data can be used to improve research innovations in rare diseases

Introduction

A rare disease is defined by the European Union (EU) as one that affects fewer than five in 10,000 of the general population and in the United States (US) as one that affects fewer than 200,000 of its population.[1] There are between 5,000 and 8,000 known rare diseases; approximately 80% of rare diseases have a genetic component, 75% affect children and 30% of rare disease patients die before the age of five.[2,3]

A single rare disease may affect up to 30,000 people in the UK alone, meaning research into these diseases is urgently needed.[1] However, research is often hindered by several factors: diagnosis is often difficult, resulting in lack of proper diagnosis or a delay in receiving a diagnosis; the population affected is sparse and spread over a wide geographical area; clinical research centres specialising in rare diseases are often limited in number and, in almost all cases, most of the patient care is provided locally.[4]

These issues mean that conducting clinical trials in the rare disease space is difficult. Rare disease trials are more likely to have smaller target sample sizes, be early-phase, recruit to a single arm, be non-randomised and be unblinded.[4] Recent research into the treatment of rare diseases has focused on gene therapy or cell therapy. Gene therapy is particularly relevant to rare disease patients, as more than 80% of rare diseases have a known monogenic (single-gene) cause.[5] Traditionally, drugs in rare diseases often work by minimising the symptoms, or managing the condition, whereas gene therapy has the potential to correct the underlying genetic defects.

Optimising data

Due to the nature of rare disease trials, it is often hard to reach necessary sample sizes to achieve appropriate powers for frequentist trials. The International Rare Diseases Research Consortium, European Medicines Association and Parmar et al (2016) have introduced frameworks to follow when designing small population trials.[6]

These covered several key areas: participant recruitment (considering broadening eligibility criteria, increasing recruitment timelines, expanding scope to multicentre or more international trials including working with specific disease networks); expanding the data available from participants (can patients be re-randomised? Could you perform a repeated measures trial? Could the design be set as a crossover, sequential or n-of-1 trial?); considering how to ensure outcomes are information rich (continuous rather than binary outcomes, larger differences between arms, stratifying patients); or considering including other available information sources through Bayesian methods.

A targeted review of rare disease and small population trials by Partington et al (2021), looking at trials from 2009 onwards, found that few studies were following these methods and that many either failed to meet their needed sample size or reduced their power to make their trials possible.[7] Few decided to use Bayesian methods, which would have avoided many of these issues by enriching their trials with previous information available through historic trials, case studies or expert opinions.

Historical controls may make the recruitment for studies of rare diseases easier, by reducing required patient numbers. A 2020 publication by Ghadessi et al found that most confirmatory clinical trials using historical controls have indications in rare disease.[8] However, using external historical controls in clinical trials involves careful analysis and skilful adjustment. Stringent methodological requirements are needed, including rigorous patient selection criteria, record of refusals (inasmuch as the intent-to-treat principle is even more important), identification of external controls in the protocol before any analysis, formalisation of statistical considerations as in a conventional randomised trial and proper selection of endpoints (response, duration of response, survival).[9]

Not only can the use of historical controls be useful in increasing patient numbers receiving treatment, but also could be considered necessary if the treatment is curative. If dramatic beneficial effects (eg, a cure) are likely, then it can be unethical to randomise patients within a trial to an alternative treatment so, the use of external (historical) controls is necessary. The US Food and Drug Administration (FDA) states in its guidelines that the use of natural history data as a historical comparator for patients treated in a clinical trial is often of interest, but it recognises that there are challenges associated with the use of historical controls.[10] It recommends that historical controls can be used in clinical development programmes for rare

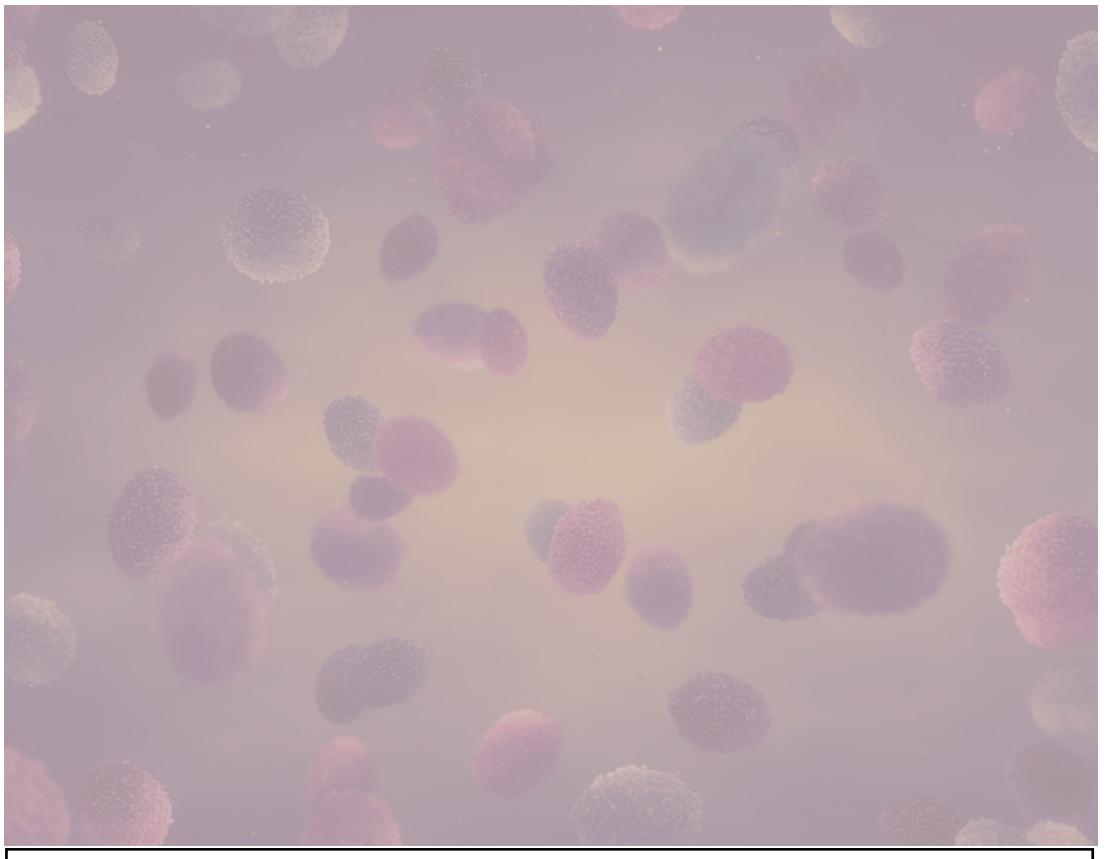

Illustration of rare disease cells

diseases, comparing patients on known covariates, or in studies where the observed effect is large in comparison to variability in disease course (eg, a substantial improvement in outcome is observed with treatment in a disease that does not naturally remit). In general, the FDA states that, provided the study design permits a valid comparison, the use of historical controls may be used in limited or special circumstances.

Rare disease trials are often underfunded, and not seen as priority in comparison to more profitable disease areas.[11] Progress in these trials could be expanded through work with charities due to their familiarity with networks of patients with specific diseases and their more focused look at areas regardless of profitability. Therefore, adding expertise across the industry to such projects in a pro bono setting can be vital to ensure rare diseases do not get overlooked. Similarly, an industry-wide push to ensure data has been optimised and trials are considering the available frameworks is key to ensure that trials in these areas are not wasted opportunities to produce vital research where they are most needed.

Case study

One rare disease that has seen breakthroughs in treatment due to innovative gene therapy research is adenosine deaminase severe combined immunodeficiency (ADA-SCID), an inherited metabolic disorder that causes abnormalities of the immune system and is usually diagnosed before 12 months of age.[12] Patients suffering from ADA-SCID often experience failure to thrive, frequent opportunistic infections and without treatment rarely survive beyond one to two years unless immune function is restored or contact with pathogens is avoided by creating a sterile environment around the patient (the so-called 'bubble-children').

Historically, the most successful treatment option was haematopeic stem cell transplant (HSCT), but this is not available to most patients with ADA-SCID and often leads to complications.[13] Now, gene therapy offers a single treatment option intended to cure the condition. During treatment, a correctly functioning copy of the ADA gene is introduced into haematopoietic stem cells (HSCs) that have been harvested from the patients using a gene transfer vector. These transduced cells are then returned to the patient where they initiate immune reconstitution much like HSCs from a healthy donor.[14]

Statistical support for gene therapy studies can include the submission for initial approval aimed at demonstrating superiority in survival over a historical control group of subjects treated with HSCT.[15] Alongside comparisons to the historical control data, this study also looked at within-subject comparisons from before and after treatment. Together, these comparisons optimised the amount of information that could be generated given the limited sample size, while ensuring that all eligible patients were enrolled onto the treatment arm of the study.

Given the success of proving the efficacy of the fresh formulation of gene therapies, new studies have since focused on studying the safety and efficacy of a cryopreserved formulation. The cryopreserved option improves shelf life and allows the treatment to be produced and administered in separate locations, reducing the distance that patients need to travel to receive the treatment. One of these studies determining the safety and efficacy of this cryopreserved treatment is currently taking place at Great Ormond Street Hospital. This research is a significant contribution to the scientific community and will bring benefits to children with this life-threatening condition.

Conclusion

Although, by definition, rare diseases affect far fewer people compared to other conditions, their numbers are still not insignificant and these diseases can have a substantial impact on individuals and their families. Conservative estimates suggest there are 300 million people worldwide living with more than 6,000 clinically defined rare diseases (not accounting for rare cancers).[16] This is between 3.5% and 5.9% of the global population at any given time.

Optimising rare disease clinical trials by enabling effective and efficient data analysis remains of paramount importance given the complexities associated with them. Biometrics expertise can be deployed to support vital research innovations, increase trial success rates and help those living with rare diseases. Continuing developments and improvements are transforming the lives of children with ADA-SCID and countless other rare diseases.

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Giles Partington is a Bayesian statistician with several years of research work into Bayesian methods, focusing on rare disease trials and Bayesian expert elicitation.

Lindsay Govan

Lindsay Govan is a statistics manager with a PhD and First-Class Hons degree in Statistics. She has over 13 years' experience as a statistician in medical research, including over seven years of experience in clinical trial development activities within CROs. Her role as a statistical lead and consultant involves leading studies for a variety of therapeutic including rare diseases.

Paddy O'Hara

Paddy O'Hara is a statistician at Phastar whose expertise lies in studies focused on rare diseases. With an impressive four years of experience in the clinical sector, Paddy has honed their skills in preparing regulatory submissions, ensuring adherence to stringent standards.

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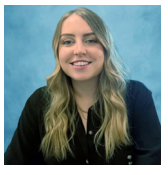
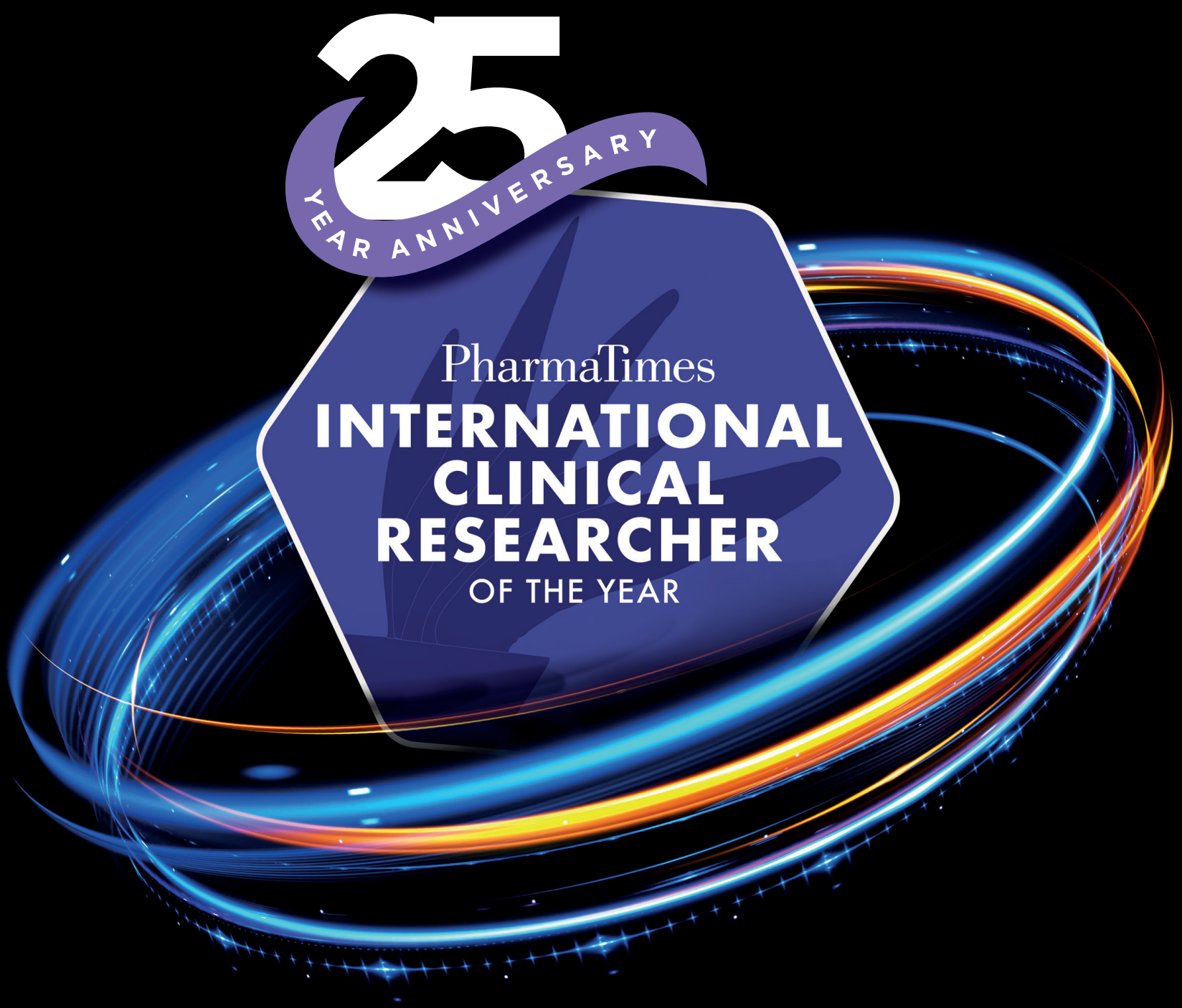


Emily Foreman is a statistician at Phastar with experience leading rare disease studies and an interest in research work into Bayesian methods. Emily holds an MSc in Statistics and has three years of experience in clinical trial development activities within a CRO as well as a year's experience within pharmaceutical consultancy.

Jennifer Visser-Rogers

Prof Jennifer Visser-Rogers is vice president, Statistical Research and Consultancy at specialist CRO, Phastar, and has a broad portfolio of achievement, particularly in the development of clinical trial methodologies. She directs the research strategy at Phastar and provides leadership and advice to statistical consultancy activities. Jen came to Phastar from the University of Oxford, where she was an associate professor and director of Statistical Consultancy Services. Jen did her BSc and MSc at Lancaster University before moving to the University of Warwick to do her PhD.



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Seven ways to boost HCP engagement through field behavioural change

Veeva talks to *Pharmafocus* about the best ways that biopharma companies can boost their engagement

The rising importance of digital channels has caught many biopharma companies off guard. Not all field teams have benefited as digital engagement increases its share of the channel mix: Veeva Pulse data shows 65% of accessible healthcare professionals (HCPs) in Europe meet with three or fewer biopharma companies.

Biopharma organisations must provide the right level of practical support for field teams to adapt and succeed in this new environment. Unfortunately, change management and training are often rushed and fail to focus on the behavioural change required to realise value.

To embed new skills and behaviours, leaders should communicate a value proposition that answers learners' core questions: 'What's in it for me?' and 'Why now?' Soft skills are as important (if not more so) than tools and tactics in a training curriculum. Leaders can accommodate different training preferences by deploying diverse ways of learning. Ensuring change is measurable will build confidence and belief in the programme.

1. Start with the 'why'

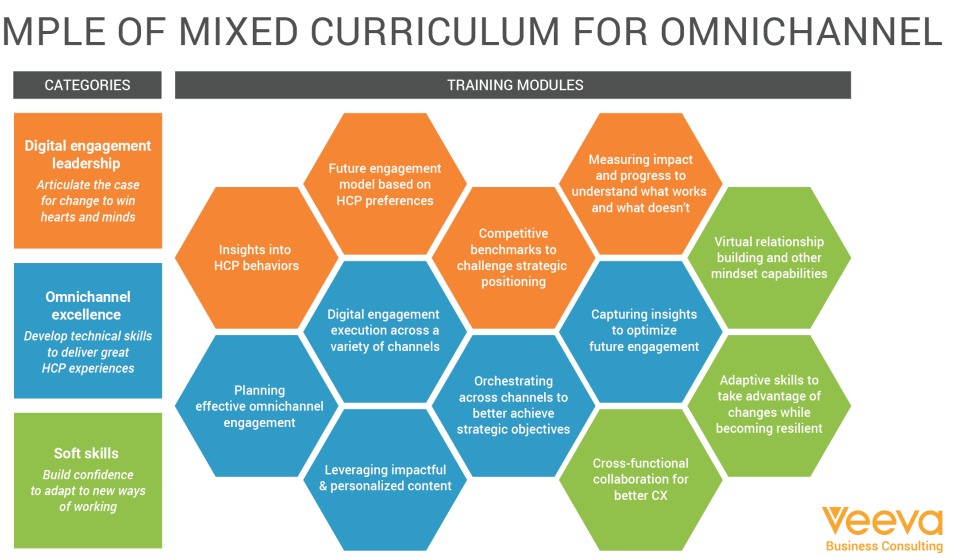
When teams understand why omnichannel is important to the business and its impact on HCPs, they are more likely to want to take the 'right' action for each customer, rather than defaulting to certain tools. This nurtures the willingness to learn about other highly effective engagement tools (including remote call and email platforms), explore new ways of partnering with customers and capture and use insights.

2. Update and upgrade your training curriculum

Training for field teams has historically been anchored around brand promotion and gaining access to HCPs. However, with so many field teams now engaging with HCPs through video calls, emails and events, access has changed significantly. A curriculum for the omnichannel field team needs to reflect the new skills required while being flexible to the different engagement needs of brands and markets. Focus first on foundational approaches and behaviours that should span all parts of the organisation.

3. Personalise the learning journey

Training needs to be designed to increase capability, whatever the starting point. Learning paths should reflect every individual's current capabilities, role needs and learning preferences. This approach helps field teams to identify different maturity levels among the HCPs they target and support them during the transition to desired behaviours. This is particularly important when deploying training to markets with different levels of omnichannel maturity.



4. Varied learning formats to engage the modern learner

Flexibility and the ability to learn on the go are musts. Consider delivering bite-sized content, across a range of formats suitable for mobile devices, so that field teams can easily combine and consume content on demand.

5. Align to support the required behaviours

Leadership has an important role to play in supporting capability development and avoiding the return of old behaviours. A good example is making sure that the incentives and objectives of field teams are aligned with the new omnichannel behaviours as well as business processes, such as customer targeting and multichannel cycle planning.

6. Lead from the top and continuously reinforce change

Foster a sense of community by celebrating key milestones (like programme kick-offs or 'graduations') so that learners are rewarded for their achievements. First-line managers play a key role, by coaching field teams, recognising progress and encouraging knowledge exchange. Creating a safe space to ask questions – perhaps within a network of experts or 'refresher' training sessions – means learners can test or give examples from their practice.

7. Measure change to show impact on behaviours

Transformation programmes tend to be long and complex and need strong governance to track implementation and adoption over time. It's as important to keep the programme alive as it is to measure the impact on individuals and the whole organisation. This demands a measurement framework that goes beyond the classic implementation metrics and assesses behavioural change (and corresponding value).

Conclusion

Field teams will continue to play a critical role in how biopharma goes to market. However, the nature of what they do and how they do it will have to evolve. Behavioural change and capability building take time, so there is no better time to start this journey – or if you are already on the journey, to reinvigorate and accelerate your approach.

Those that do so will build a competitive advantage through better-quality customer engagement. With the amount of money invested in field teams, it's too important to leave it to chance.

Contact Veeva Business Consulting to improve how your organisation engages with customers.

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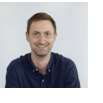

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Why are so many community pharmacies closing and what does this mean for local communities?

Betsy Goodfellow from *Pharmafocus* considers the importance of community pharmacies as part of primary care in the UK and the significance of their closure

Community pharmacies are an important part of primary care in the UK, using their wealth of clinical knowledge to help patients with acute or chronic conditions as an integrated part of a primary healthcare team.[1] These pharmacies provide a range of health services, from advice and consultations to prescription-only medications and flu vaccines.[1] However, in the last few years, an ever-increasing number of community pharmacies have closed, leaving a widening gap in local community access to primary healthcare.

Over the last seven years, pharmacies have seen a 30% cut in government funding (even when accounting for inflation) and an estimated shortfall of £1.1bn in funding, meaning there are now just over 11,000 community pharmacies in England, the lowest number since 2015.[2] This fall is due to rising operational costs and staff shortages, along with the reduced government support.[2] In addition to the reduced funding, there has been an expansion of the role that community pharmacies are expected to play, in order to reduce the strain on general practitioners (GPs) and the wider NHS, with the government introducing a plan to allow pharmacists to prescribe antibiotics and routine tests.[3] This includes treating simple conditions such as earache, sore throat, sinusitis, impetigo, shingles, infected insect bites and uncomplicated urinary tract infections in women.

In addition to the existing pharmacy closures, 252 in-store supermarket pharmacies are at risk of closure this year, with Lloyds Pharmacy announcing in January that it will close its branches in 237 Sainsbury's stores.[4] In the same month, Asda announced that it will close seven of its 241 in-store pharmacies, and Tesco has also announced that it will close eight of its 351 in-store pharmacies.[4] Even though Asda and Tesco are currently only closing a fraction of their in-store pharmacies, these closures will still impact local communities. While these closures will greatly reduce the number of easily accessible and convenient pharmacies, they will also likely impact those who are already vulnerable, as they may struggle to access a pharmacy elsewhere, leading to difficulty in receiving their prescriptions and accessing the other health services that community pharmacies provide.

It has also been calculated that 40% of permanent pharmacy closures have occurred in 20% of the most deprived areas, with 63% happening in the Northwest, West Midlands, Yorkshire and the Humber.[5] This has only served to increase the existing barriers to accessing good healthcare due financial insecurity in these areas. In 2014, 99% of people in the most deprived areas lived within a 20-minute walk of a community pharmacy, compared to 90% in the least deprived areas, although it is likely that this number will have fallen for both groups during the last nine years.[5]

But what impact will these closures have on the health of local communities?

The combination of closing a large proportion of pharmacies and giving greater prescribing powers and responsibilities to the remaining pharmacies can only lead to a lower level of care

for the majority of patients, as they become overstretched. Although this reduces strain on GP services, the knock-on effect of these increased responsibilities, along with the closures, will lead to pharmacies struggling to cope with an increased demand from the overspill of patients previously treated at now closed branches and from patients expecting additional services such as consultations and prescriptions for simple infections or conditions.

Experts have also expressed their concern that many more pharmacies could close over the coming years due to this increased pressure and workload, which can only make the issue worse.[2]

Sanjeev Panesar, who owns Pan Pharmacy in Birmingham, UK, commented: "Things are in serious jeopardy. It's our worst year ever, where we've made a loss. We have to make some really tough calls and decisions now."[2] He also added that he would love to help relieve the burden on the NHS and GPs specifically, however this is not possible currently with ongoing financial constraints.[2]

In 2019, the Department of Health and Social Care (DHSC), NHS England and the Pharmaceutical Services Negotiating Committee (PSNC) agreed on a Community Pharmacy Contractual Framework running from 2019 to 2024, which lays out how pharmacies are expected to help with the NHS Long Term Plan.[6] They pledged £2.592bn to be spent on community pharmacies between 2019 and 2024, in the hopes of providing stability and reassurance to community pharmacies, thus allowing them to continue to provide primary healthcare efficiently, especially with their newly extended responsibilities.[6]

On the whole, it is evident that, as more pharmacies close, the strain on those remaining only increases. This, combined with the additional roles and responsibilities given to pharmacies in order to reduce the strain on GP surgeries and the wider NHS, has greatly increased pressure on pharmacies. Along with the lack of government funding, this has inevitably led to mass closures with 670 pharmacies closing since 2015, which has impacted the ability of local communities to easily access pharmacies, resulting in a potentially negative effect on the population's health.[7]

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Move of the month

Reacta appoints Kevin Hawkins as new head of regulatory affairs

Reacta Healthcare, an early-stage food allergy diagnostic company, has announced the appointment of Kevin Hawkins as head of regulatory affairs.

Hawkins has extensive leadership experience including in R&D, regulatory affairs, analytical development and project leadership, having previously worked as senior director and head of development at Teva Pharmaceuticals for more than ten years. He has also worked in regulatory affairs for the last 15 years, building an R&D team at Teva with experience in multiple dosage form areas such as inhalation, ophthalmic, parenteral and nasal sprays.

Dr Paul Abrahams, CEO of Reacta Healthcare, said: "We are delighted to welcome Kevin to the Reacta Senior Leadership Team at this critical juncture in the company's development. Kevin is a highly skilled and respected senior leader with a strong track record in R&D and Regulatory Affairs. His experience in dealing with regulators and his practical approach to regulatory matters will be a great asset to Reacta and will be invaluable in contributing to the growth of the company."

Professor Ashley Woodcock, chairman at Reacta, added: "Our ultimate strategic goal is to achieve a Marketing Authorisation for our challenge meal products and Kevin will play an instrumental role in progressing this strategy, particularly given recent positive feedback from several Health Authorities to our product and approach."

Commenting on his appointment, Hawkins stated: "I am thrilled to be joining Reacta at this critical time as the company progresses its regulatory strategy with Health Authorities globally. I look forward to supporting the Executive Team in their continued efforts to build Reacta as a leading food allergy diagnostics company seeking to improve the lives of food allergy sufferers."

Olav Hellebø to join Cytovation's board of directors

Cytovation ASA, a clinical stage immune-oncology company with a focus on targeted tumour membrane immunotherapies, has announced that it has appointed Olav Hellebø to its board of directors.

Hellebø brings with him extensive leadership experience from US- and Europe-based pharmaceutical and biotechnology companies, having previously acted as chief executive officer at ReNeuron and Clavis Pharma. Prior to these roles, Hellebø headed up the global immunology franchise at UCB Pharma, also having worked as head of UK commercial operations at Novartis.

He has also held senior roles at Schering-Plough.

Currently, Hellebø serves on the board of Antev, a UK-based private biotech company with a focus on prostate cancer.

Stein Christian Mohn, chairman at Cytovation, stated: "We are very pleased to welcome Olav to our board of directors. We believe that his extensive and broad-ranging experience in the industry will prove invaluable for the advancement of CyPep-1 and the continuing growth of the company as we continue to make excellent progress in our ongoing clinical programme both in Europe and the US."

Commenting on his new role, Hellebø added: "CyPep-1 has great potential as an innovative treatment for solid tumours where there is significant unmet medical need despite recent advances in cancer treatment. I look forward to working with the talented team at Cytovation to help bring this unique therapy to patients."

Helmholtz Centre for Infection Research appoints Josef Penninger to new scientific director

The Helmholtz Centre for Infection Research (HZI), Germany, has appointed Professor Josef Penninger as scientific director.

Penninger has previously worked as founding director at the Institute for Molecular Biotechnology of the Austrian Academy of Sciences, Vienna, Austria; professor at the Department of Medical Genetics at the University of British Columbia, Canada; and director of the Life Sciences Institute at the University of British Columbia, Canada.

Professor Otmar D Wiestler, president of the Helmholtz Association, added: "We would like to express a warm welcome to Josef Penninger to the Helmholtz Association and wish him good luck and success with his new responsibilities at the HZI. His expertise in genetics and molecular biology and his international experience are excellent preconditions for strengthening internal health research at Helmholtz. With his ambitious plans, Josef Penninger will actively take part in building the bridge from foundational research to medical applications."

Penninger stated: "I am very excited about the new challenges and opportunities at HZI. The HZI is a great place for research in infection biology with great scientists and a great organisation. Now it is time to make the next step – to position HZI as the best international centre for infection research in the world. To accomplish this, we now build the right steps to execute on this vision, including the creation of multiple new research positions to refresh the HZI community with the best young minds from all around the world. We will also develop a unique programme on technology fellows, to make Germany a world-leader in research innovation in infection biology with the potential to develop radically new and personalised therapies and to establish unicorn companies."

Alfonso Quintás-Cardama to join Foghorn Therapeutics as new chief medical officer

The biotechnology company Foghorn Therapeutics has announced the addition of the new chief medical officer, Alfonso Quintás-Cardama, who will be replacing Sam Agresta after his retirement on 11 September 2023. The clinical-stage biotech company specialises in correcting abnormal gene expression in order to treat diseases.

Adrian Gottschalk, president and chief executive officer at Foghorn, commented: "I want to thank Sam for his leadership and many contributions to Foghorn over the past four years. He joined the

company at a critical time and built our clinical team which advanced our first programmes to the clinic. […] We are excited to welcome Alfonso to the Foghorn team. Alfonso's experience and expertise in oncology and as a leukaemia expert will be integral to our plans as we continue to advance our pipeline and develop medicines for patients."

Dr Agresta will remain available to provide consulting services until early 2024.

Dr Quintás-Cardama was previously the head of clinical development for cell therapies at GSK, and also worked as the clinical leader at Novartis, where he led the development of the first FDA-approved CAR T-cell therapy. Most recently, he was the chief medical officer at TCR2, where he led the development of the company's cell therapy platform from 2017.

Quintás-Cardama said, "As a leader in understanding and drugging chromatin biology, Foghorn is uniquely positioned to develop the next class of cancer medicines. I am excited to join Foghorn given the company's broad pipeline coupled with a platform that has enabled the drugging of some of the most compelling targets in cancer. I am looking forward to being part of the Foghorn journey as the company continues to address unmet needs in cancer and brings potential therapies to people who need it the most."

Five facts about rare diseases

1. In most countries, rare diseases typically affect fewer than one in 2,000 people, however they are significantly more common in Canada, affecting one in 12 people.[1] This means there are approximately three million Canadians and their families currently living with a rare disease.[1]
2. There are over 6,000 unique rare diseases across the world, with almost 70% exclusively occurring in children and over 10% exclusively occurring in adults.[1]
3. Approximately 95% of the known rare diseases in the world currently have no treatment and many have not yet been studied by medical researchers.[2] Patients are typically treated off-label, meaning the treatment is not approved by the US Food and Drug Administration (FDA).[2]
4. Around two-thirds of patients with rare diseases are children.[3] These children often develop these rare diseases due to genetics, but other causes can be from infections, exposure to toxic substances, nutritional deficiencies, injuries or adverse effects of treatment from other illnesses.[3]
5. With prenatal testing becoming more advanced, it is now possible to test for rare diseases as early as the tenth week of pregnancy – the procedure consists of a simple blood test that screens for multiple diseases, such as Edwards's syndrome and Patau syndrome.[4]

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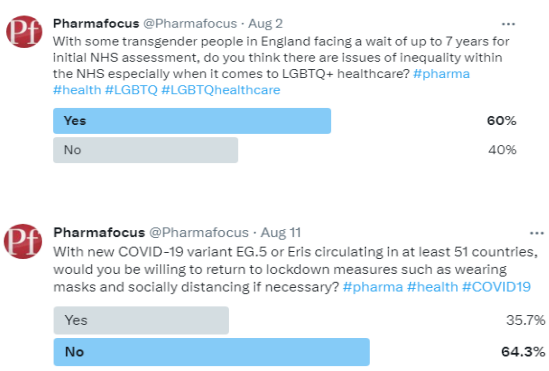

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