Pharmafocus
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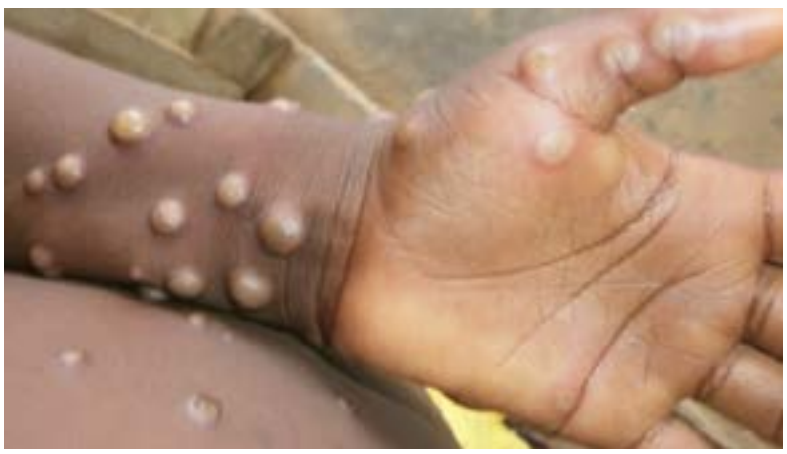
Monkeypox antiviral tecovirimat put to the test

The antiviral was developed to treat poxviruses



Around 500 patients will take part in the PLATINUM trial at the University of Oxford to study an antiviral which aids in monkeypox recovery.

The virus has been declared a global health emergency, and is spreading rapidly in other countries, as well as the UK. Monkeypox infection usually improves on its own without treatment, but some cases can be severe, and can require hospitalisation. Recovery from infection can take weeks, and there can be serious complications involved.

Meanwhile, the UK government has bought stocks of the smallpox vaccine to try to halt its spread. The virus can be spread through close contact with an infected person. It has not been scientifically described as a sexually-transmitted infection, but anyone with the virus should abstain from sex while they have symptoms.

As part of the PLATINUM trial, some participants will be treated twice-daily with tecovirimat tablets while they recuperate from the virus in their own home. Others will receive a placebo instead.

Tecovirimat, also known as Tpoxx, prevents the virus from leaving infected cells, thereby stopping its spread within the body. The antiviral can help people with monkeypox recover more quickly, and shorten the time that they are infectious to others.

Tecovirimat was initially developed to treat illness caused by poxviruses. While it has been shown to improve recovery from monkeypox in animals, there is currently limited data on its use in human infections, according to the PLATINUM team.

However, the drug has been administered on over 400 healthy people in order to test its safety, and no concerns have been identified. It was approved as the first medicine to treat smallpox in 2018.

"The aim is to find a treatment that can help people get better quicker and get out of quarantine," said Professor Sir Peter Horby, one of the PLATINUM trial researchers at the University of Oxford.

Minister for Public Health, Maggie Throup, said: "This government-funded study is an important step to finding a treatment which can help speed up the recovery of those who have monkeypox."

She added: "Vaccines remain our best defence against the spread of monkeypox – we urge all those eligible to come forward when contacted, and report any symptoms to NHS 111."

First disease-modifying treatment for AADC deficiency

The EC has approved the first gene therapy directly infused into the brain

The European Commission (EC) has granted a marketing authorisation for Upstaza (eladocagene exuparvovec), the world's first disease-modifying treatment for aromatic L-amino acid decarboxylase (AADC) deficiency.

Upstaza is additionally the first marketed gene therapy directly infused into the brain.

The treatment, formerly known as PTC-AADC, has been approved for patients 18 months and older.

"Today's approval from the EC for Upstaza, for the treatment of AADC deficiency, is momentous for patients, for PTC, as well as for the larger gene therapy community," commented Stuart W Peltz, PhD, CEO, PTC Therapeutics. "We are proud to bring this innovative therapy to the marketplace so that patients may benefit. Upstaza is the first and only approved disease-modifying treatment for patients living with AADC deficiency. We are ready to deliver this long-awaited treatment to patients as soon as possible."

The approval is based on several clinical studies, in which patients went from not achieving any developmental

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motor milestones, to demonstrating clinically meaningful skills, from as early as three months following treatment. Transformational improvements were shown to continue up to ten years after treatment.

AADC deficiency is a very rare, fatal genetic disorder that typically causes severe disability and suffering from the first months of life, with many impacts – physical, mental, and behavioural. The suffering of children with AADC deficiency may be exacerbated by episodes of distressing seizure-like oculogyric crises, causing the eyes to roll up in the head, frequent vomiting, behavioural problems, and difficulty sleeping.

The disorder is characterised by decreased activity of aromatic I-amino acid decarboxylase, an enzyme involved in the synthesis of dopamine and serotonin, which are responsible for the communication between neurons in the nervous system.

"Before treatment, our daughter had not met any development milestones. She suffered from oculogyric crises that evolved into hours of pain, and we were told she would be bedridden for life," said patient organisation Teach RARE founder, Richard Poulin, whose daughter was treated as part of a clinical trial. "We're thrilled with the EMA approval, and the hope that this milestone brings to other children and families impacted by AADC deficiency."

Future Focus

Bausch + Lomb begins TikTok challenge for Lumify, asking customers to 'dance with their eyes'

A major pharma company has kickstarted a marketing campaign for their new eye drop Lumify, which taps into the current craze for social media platform TikTok. The campaign asks users to dance with their eyes instead of their feet.

Bausch + Lomb is looking to accelerate their marketing with the new LUMIFYEyeDance challenge. The OTC eye drop was approved in 2018 to reduce eye redness, and made over $100 million last year.

The company aims for potentially thousands of people to become more aware of its brand, Lumify, either through taking part in the dance-off challenge, or by viewing the videos.

The hashtag for the drop has "organically amassed nearly 14 million views to date," according to a press release issued by Bausch + Lomb.

"We believe the Lumify Eye Dance Challenge will help build upon the existing excitement on TikTok and inspire consumers to celebrate what makes them and their eyes unique," Joe Gordon, President, Global Consumer, Surgical and Vision Care of Bausch + Lomb, said in the press release.

The Lumify challenge was inspired by Salix, marking the first TikTok campaign for Salix, and was focused on constipation. Salix, which sells Relistor for several types of constipation, turned to five healthcare workers who use TikTok in their attempt to break the stigma of the condition.

Contents

Words from the Editor

Hi readers,

Welcome to the September issue of *Pharmafocus*!

We've been experiencing record temperatures here in the UK, and across Europe – we hope you've been coping with the heat and enjoying your summer, wherever you are in the world.

There have been some major changes across the health and pharma landscape since our last issue. For one, the WHO activated its highest alert for the growing monkeypox outbreak on 23 July – declaring the virus a global health emergency. The UK is currently waiting for 100,000 vaccines to treat monkeypox, but, according to the UKHSA, only 50,000 have been received thus far due to supply issues.

On a more positive note, HealthTrackRx has developed a monkeypox-specific PCR test, with next-day results. This will be available to thousands of customers across the US, to combat the recent surge in cases across the country. Read more about this on page 8.

In this issue, we also delve into some exciting new developments in technology. Cognito Therapeutics has unveiled new data showing that six months' use of its digital therapeutic could potentially reduce the rate of atrophy within the brain's white matter in patients with Alzheimer's. You can check out the full story in our digital news section, on page 12.

That's it from us! We hope you enjoy reading through our latest issue as much as we enjoyed putting it together.

Pop open a cold drink, sit back in the shade, and enjoy *Pharmafocus* September!

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MPs warn NHS England facing worst staffing crisis in history

17,000 care workers paid below minimum wage of £9.50 an hour in England

A recent report by MPs says that the large number of unfilled NHS job vacancies is posing a serious risk to patient safety.

England is now short of 12,000 hospital doctors and over 50,000 nurses and midwives, with the report calling this the worst workforce crisis in NHS history. The report also said that a reluctance to decisively address the staffing gap could impair plans to tackle the COVID-19 treatment backlog.

Former Health Secretary, Jeremy Hunt, has said: "Persistent understaffing in the NHS poses a serious risk to staff and patient safety, a situation compounded by the absence of a long-term plan by the government to tackle it."

There is evidence that almost a million new jobs will need to be filled in health and social care by the beginning of the next decade. The report is calling for HMRC to be more proactive in enforcing the minimum wage, amid concerns that 17,000 care workers were paid below the legal minimum of £9.50 an hour.

"Without the creation of meaningful professional development structures, and better contracts with improved pay and training, social care will remain a career of limited attraction, even when it is desperately needed," the report said.

Patricia Marquis, England director at the Royal College of Nursing, said the risk to staff and patients from low staffing levels should "shock ministers into action".

"On pay the committee was very clear, saying it is unacceptable that some NHS nurses are struggling to feed their families, pay their rent, and travel to work," she said.

Potential shortage of 140,000 nurses in NHS England by 2030

Leading nurses describe the projected numbers as "apocalyptic"

According to new analysis, NHS England could face a shortfall of almost 40,000 nurses by 2024 and, under a pessimistic scenario, will be faced with a shortage of 140,000 nurses by 2030/31.

It has been estimated that the health service will be short of 38,000 full-time equivalent registered nurses by 2023/2024, according to the analysis from the Health Foundation.

While the government set a target of recruiting an additional 50,000 nurses by the end of the parliamentary term, the target failed to recognise the increase in demand for care, such as those with more complex health needs.

Leading nurses have described the 2030 figure as "apocalyptic".

In the last year alone, almost 20,000 nurses left the health service in England, and the government target still fell short of tens of thousands to meet patient need at a pre-pandemic level of care. Further, the government target failed to consider bringing in nurses to specialities where the biggest gaps exist, including social and community care.

Anita Charlesworth, director of research and REAL Centre, commented: "The NHS in England appears to be on track to recruit the additional 50,000 nurses promised by the government by 2023/24, but this relies heavily on sustaining historically high levels of international recruitment – very much a 'quick fix' – and does not replace the need to train and retain more nurses in the UK."

Charlesworth continued: "The 50,000 target is arbitrary, and not based on the number of nurses the NHS needs; nor does it ensure that nurses are recruited to the areas and types of care where the need is greatest. 50,000 extra nurses will still leave the NHS almost 40,000 short of what is needed."

Patricia Marquis, director for England at the Royal College of Nursing, also commented on the figures: "These projections show the apocalyptic impact inactivity from ministers could have on the NHS in England – a potential shortfall of 140,000 nurses would be devastating for patient care."

£70 million in fines for pharma firms that overcharged NHS

The CMA has found that Pfizer and Flynn charged unfairly high prices for phenytoin sodium capsules for over four years, which were ultimately paid for by the NHS.

The firms de-branded the epilepsy drug, previously known as Epanutin, meaning that it was no longer subject to price regulation, and the firms could set prices at their discretion. Pfizer and Flynn were the dominant suppliers of the drug in the UK at the time, and the NHS had no choice but to pay the inflated final price for this important medicine, according to a press release from the Gov website.

Over the ensuing four years, Pfizer charged prices between 780% and 1,600% higher than previously.

Pfizer then supplied the drug to Flynn, which then sold the capsules on to wholesalers and pharmacies, at a price between 2,300% and 2,600% higher than the prices previously charged by Pfizer.

This led to NHS annual costs for phenytoin capsules increasing from £2 million in 2012, to approximately £50 million the following year.

The CMA has now determined that the companies' behaviour was an abuse of their dominant positions, and that they charged unfair prices for phenytoin capsules.

Andrea Coscelli, CEO of the CMA, said: "Phenytoin is an essential drug relied on daily by thousands of people throughout the UK to prevent life-threatening epileptic seizures. These firms illegally exploited their dominant positions to charge the NHS excessive prices and make more money for themselves – meaning patients and taxpayers lost out.

UK scientists say they have found cancer driver in junk DNA

The study suggests that junk DNA is potentially destructive to cells

Scientists in the UK think they have understood the mechanism behind junk DNA, which is implicated in cancer. Junk DNA is a term used to describe the 97% of the genetic sequence in human cells found between the 3% coding for our 20,000 genes, once thought to be inert.

In the past, studies have focused on repetitive sequences of DNA that account for around half of our DNA – sometimes called the 'repeatome', and thought to originate from ancient viral infections – have suggested they could interfere with the replication and repair of the genome.

To study the process behind junk DNA, scientists reconstituted the entire process of DNA replication in a test tube, so that they could examine it in detail. They showed how repetitive patterns of DNA are copied during replication, and found evidence that they can stall the process entirely, increasing the risk of errors that can be an early driver of cancer, according to a paper published in *Nature Communications*.

The research team found that when the DNA replication machinery encountered repetitive DNA, it was able to unwind the DNA strands, but it sometimes failed to copy the opposite DNA strand. This error could cause replication to stall, resulting in collapse of the replication machinery in a manner similar to that induced by DNA damage, potentially allowing mutations to accumulate.

"We wanted to understand why it seems more difficult for cells to copy repetitive DNA sequences than other parts of the genome," said study leader Dr Gideon Coster.

"Our study suggests that so-called junk DNA is actually playing an important and potentially damaging role in cells, by blocking DNA replication and potentially opening the door to cancerous mutations."

New reforms aim for better NHS dental service access

NICE guidance recommends patients should recieve oral checkup based on health risk

Patients across the country will benefit from improved access to dental care under changes announced by NHS England, the organisation has shared. The first new reforms to the dental contract in 16 years have taken place.

These reforms mean NHS dentists will be paid more for treating more complex cases, such as people who need three fillings or more.

Dental therapists will also be able to accept patients for NHS treatments, providing fillings, sealants, and preventative care for adults and children, which will free up dentists' time for urgent and complex cases.

Chief Dental Officer for England, Sara Hurley, commented: "The NHS is determined to overhaul dental provision, with a focus on increasing access to necessary dental care and supporting prevention. Today's reforms are the first step on that journey.

"NHS dental staff are working hard to recover services, but the key to delivering this will be reform – these changes announced today will help teams carry out even more treatments, and help address the inevitable backlogs that have built up during the pandemic."

In order to ensure more accessible services, under the new changes, dentists must update the NHS website and directory of services, so that patients can easily find the availability of dentists in their local area.

High-performing dental practices will also have the opportunity to increase their activity by a further 10%, in order to see as many patients as possible.

"Anyone with concerns about their dental health should contact their local dentist as they usually would or seek advice from NHS 111," Hurley continued. "Infection prevention and control measures to protect staff and patients were introduced during the pandemic, limiting the number of procedures that NHS dentists could carry out."

The new reforms will ensure that dentists who are operating at full capacity for the first time in two years will be able to recover dental services following the impact of the pandemic.

NICE guidance states that dental teams should see patients for an oral check-up based on their health risk which can be once every two years instead of every six months. This will ensure appointments are given to those most in need.

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Round-up

EMA responds to monkeypox public health emergency

The EMA has initiated a series of actions to respond to the ongoing monkeypox outbreak, which was declared a Public Health Emergency of International Concern (PHEIC) by WHO on Saturday 23 July.

The EMA Executive Steering Group on Shortages and Safety of Medicinal Products (MSSG), established by the new regulation, will produce and maintain a formal list of critical medicines for the monkeypox public health emergency. This list will be drawn up in a collaborative process involving Member States, healthcare professionals, patients, and consumers.

An Emergency Task Force (ETF) has also been formally extended to deal with both COVID-19 and monkeypox. The ETF was initially set up during the pandemic to bring together expertise in the EU medicines regulatory network.

EMA recommends treatment for multiple myeloma patients with limited options

The EMA has recommended a conditional marketing authorisation in the EU for Tecvayli (teclistamab) for the treatment of adult patients with relapsed and refractory multiple myeloma, who have received at least three prior therapies.

Multiple myeloma is a rare cancer of a type of white blood cells called plasma cells. Plasma cells are found in the bone marrow, and are an important part of the immune system, as they make the antibodies that enable the body to recognise and attack viruses or bacteria. In multiple myeloma, cancerous plasma cells accumulate in the bone marrow and crowd out healthy blood cells. Instead of producing helpful antibodies, the cancer cells produce abnormal proteins which can cause complications.

NICE issues positive appraisal for advanced kidney cancer treatment

Lenvatinib is indicated or treatment for adults with advanced renal cell carcinoma

NICE has published an appraisal consultation document (ACD) recommending the use of lenvatinib as a therapeutic option for untreated intermediate or poor risk advanced renal cell carcinoma (RCC) in adults.

The inhibitor has been recommended only if nivolumab with ipilimumab would otherwise be offered.

The ACD follows the Scottish Medicine Consortium (SMC) positive recommendation of the combination for advanced RCC in June 2022. The combination will receive interim funding in England via the Cancer Drugs Fund, until publication of the technology appraisal guidance, which is expected in early 2023.

"We are happy to have supported Eisai's clinical trial programme, to help develop this alternative first-line combination therapy and are delighted to see NICE deliver a positive ACD, now making this treatment available to patients across England," said Professor Thomas Powles, Director of Barts Cancer Centre at St. Bartholomew's Hospital. "With more treatment options available to patients in more countries, we can provide personalised care plans that align more closely to their needs."

Lenvatinib is indicated for the treatment of adults with advanced renal cell carcinoma in combination with pembrolizumab, as a first-line treatment.

RCC is the most common type of kidney cancer, accounting for approximately nine in 10 kidney cancer cases in the UK. It also has the highest mortality rate of the genitourinary cancers, as more than a third of patients will die from the disease. Between 2013 and 2017, approximately one third (36%) of people in the UK diagnosed with kidney cancer were diagnosed at an advanced or metastatic stage of the disease (stage 3 or 4).

"We are delighted that NICE is recommending this alternative first-line combination treatment option for patients living with advanced kidney cancer across England. We know through our annual Kidney Cancer UK patient survey that 42% of patients in the UK are diagnosed at an advanced stage when survival rates are typically lower. Having this combination option available through the Cancer Drugs Fund is great news which will be welcomed." said Nick Turkentine, Chief Operating Officer, Kidney Cancer UK.

Medics in Ukraine work amid shelling and worsening health crisis

Ukraine's health emergency is worsening, WHO have shared as the conflict with Russia continues, and staff face burnout and increased shelling. So far, there have been 434 attacks on healthcare facilities in the country.

615 such attacks on healthcare have been reported worldwide this year, WHO shared via a tracker.

With winter approaching, the health organisation's concerns for healthcare in the region have been exacerbated.

In June, Mariupol had a population standing at around 100,000, where prior to the war, around 430,000 lived in the city.

"It's kind of falling off the news in a way...but this is an emergency of public health," WHO's Ukraine emergency coordinator Heather Papowitz told Reuters.

"Getting access is the biggest issue, it is what keeps us up at night," said Papowitz. Currently, global healthcare services such as WHO are facing challenges getting medicines into areas of conflict for people with chronic conditions, or to treat physical and mental trauma. Papowitz shared that WHO's biggest concern was for the areas currently

inaccessible to its teams, due to conflict or Russian occupation. These areas include the Eastern Donbas region and Kherson, to the south.

In May, Reuters published the statistic that at least 3,000 people had died in Ukraine due to the inability to access treatments for chronic diseases. This problem of access was directly caused by the February invasion of the country by Russian forces.

Now, disease control is playing a part in the worsening health crisis of the country. There have, further, been concerns over the risk of cholera in the region, though Papowitz underlined that no cholera outbreaks have yet been reported.

Monkeypox declared global health emergency

The move marks the seventh declared global health emergency since 2009

For the second time in two years, WHO has declared a global health emergency, this time for monkeypox. The declaration is the strongest call to action the agency can make.

Monkeypox has spread in just a few weeks to dozens of countries, and infected tens of thousands.

It is the seventh time such a declaration has been made since 2009, the most recent being for COVID-19. The declaration follows a meeting of a committee of experts on Thursday.

WHO Director General Dr Tedros Adhanom Ghebreyesus shared: "WHO's assessment is that the risk of monkeypox is moderate globally and in all regions, except in the European region, where we assess the risk as high."

"In short, we have an outbreak that has spread around the world rapidly through new modes of transmission about which we understand too little, and which meets the criteria in the international health regulations," Dr Tedros continued. "For all of these reasons I have decided that the global monkeypox outbreak represents a global health emergency of international concern."

The move apparently marks the first time that the director general had sidestepped his advisers to declare an emergency.

Dr Boghuma Titanji, an Infectious Diseases physician at Emory University in Atlanta, US, commented that the move was overdue, and with the delay, "one can argue that the response globally has continued to suffer from a lack of coordination with individual countries working at very different paces to address the problem."

"There is almost capitulation that we cannot stop the monkeypox virus from establishing itself in a more permanent way," she added.

"We've now unfortunately really missed the boat on being able to put a lid on the outbreak earlier," Dr James Lawler, co-director of the University of Nebraska's Global Centre for Health Security, said. "Now it's going to be a real struggle to be able to contain and control spread."

Lawler estimated that it may take a year or more to control the outbreak, by which point the virus is likely to have infected hundreds of thousands of people. Additionally, monkeypox may have already now permanently entrenched itself in some countries.

Global monkeypox cases rise to 26,000

Between five and 10 million vaccine doses needed to protect high-risk groups

WHO have shared that the window to stop the spread of monkeypox is closing, with cases currently doubling every two weeks. There have been over 26,000 cases of monkeypox reported globally from 78 countries, with the majority of cases in Europe. The virus is additionally in the process of being renamed.

Around 10% of patients have been hospitalised in the current outbreak. Five of these have died, WHO reported; all of them in Africa. The virus generally causes mild to moderate symptoms, including fatigue, fever, and painful skin lesions. These tend to resolve within a few weeks.

The US has shared it is to distribute 800,000 doses of monkeypox vaccine. Distribution was withheld pending an FDA review, which has now cleared the additional doses of vaccine for use. Stores of Jynneos, the monkeypox vaccine, have been constricted since the beginning of the outbreak.

While the new doses will expand the US supply, experts have questioned whether it will provide sufficient immunisation to meet demand. Since May, the US has confirmed nearly 7,000 cases, among the highest tallies in the world. This figure is believed to underestimate the true number of positive cases.

80 of the countries reporting cases of the virus have not historically reported monkeypox.

Dr Tedros Adhanom Ghebreyesus, WHO Director-General, has shared that there are around 16 million doses of approved vaccine available, but only in bulk. This means it will take several months to transfer vaccine doses into vials.

The organisation estimates that between 5 million and 10 million doses of vaccine will be needed to protect all high-risk groups.

American officials have now ordered nearly seven million doses, which will arrive in batches over the coming months.

Round-up

FDA approval for neurostimulation device to treat diabetic neuropathic pain

DyAnsys has received FDA approval for its First Relief device, a wearable tool which monitors pulses of a low-level electrical current over several days, made for diabetic peripheral neuropathy pain. The device is intended for multiple treatments for up to 56 days.

First Relief is a percutaneous electrical neurostimulation (PENS) device, intended for multiple treatments for symptomatic relief of chronic, intractable pain from diabetic peripheral neuropathy.

"We are excited to have the FDA clearance of First Relief so that this device, which has been proven effective, can now be used to treat patients who have been experiencing pain related to diabetic neuropathy," said DyAnsys CEO Srini Nageshwar.

The approval was based on a study testing First Relief against a placebo, and another device previously cleared by the FDA, and was conducted in the Jeevak Multispeciality Hospital in Warangal, India. This hospital is renowned for the treatment of diabetes.

Neuropathic pain is caused by damage or injury to the nerves whichtransfer information between the brain and spinal cord from the skin, muscles, and other parts of the body.

FDA approves new treatment for rare tumour

The FDA has approved crizotinib for adult and paediatric patients aged one year and older with unresectable, recurrent, or refractory inflammatory anaplastic lymphoma kinase (ALK)-positive myofibroblastic tumours (IMT).

IMT appears in organs such as the lungs, stomach, bladder, or liver. These tumours are not cancerous, and do not usually spread. However, local growth within organs can cause problems for patients.

The drug is already approved to treat metastatic non-small cell lung cancer (NSCLC) in patients whose tumours are positive for ALK or ROS1 mutations, alongside for ALK-positive anaplastic large cell lymphoma in children and young adults. Crizotinib, marketed as Xalkori, was first introduced over a decade ago. The recommended dose for adults in 250mg orally twice daily, until disease progression or unacceptable toxicity.

HealthTrackRx develops monkeypox-specific PCR test with next-day results

HealthTrackRx is now offering a molecular test for detection of the human monkeypox virus.

The test was developed by the company's research and development team, and is available to the thousands of HealthTrackRx customers across the country in response to this rapidly growing public health emergency.

The HealthTrackRx assay is a pan-monkeypox test that detects both the West African and Congo basin strains of the virus, using the TaqMan multiplex real-time PCR technology from Thermo Fisher Scientific.

Martin Price, CEO and Chairman of HealthTrackRx stated: "In light of The WHO declaring the monkeypox outbreak a global health emergency, and the proliferation of cases in our communities, we're pleased to deploy a means of rapidly detecting this virus. Applying molecular diagnostics to detect infectious diseases and getting those results in healthcare providers' hands by the next day is our mission, and is where we can make a difference in containing this crisis."

Dr Vijay Singh, who led the research and development teams' effort to develop and validate the assay stated:

"Testing is one of the first and most crucial strategies for containment of an infectious disease outbreak or pandemic. The non-endemic spread of human monkeypox is an urgent public health threat. The rapid development of the pan-Monkeypox Assay at HealthTrackRx, alongside our industry partners, demonstrates our commitment to public health by delivering solutions that accelerate results to patients."

WHO recently declared monkeypox a global health emergency. The widespread availability of a human monkeypox test follows the company's announcement last week that it has been working in partnership with the CDC to conduct an epidemiologic study that will contribute to understanding the spread of human monkeypox

Micronesia's first COVID-19 outbreak sees steep rise in cases

Micronesia's first outbreak of COVID-19 has grown in one week to over 1,000 cases, causing significant alarm in the Pacific Island nation.

Last week, Micronesia became the final nation in the world with a population of more than 100,000 to experience an outbreak of COVID-19 after avoiding it for over two years, thanks to geographic isolation and border controls.

Individuals who flew into the country with COVID-19 were unable to spread the disease, as all new arrivals were required to quarantine.

Micronesia is an island country consisting of four states spread across the western Pacific, around 2,900km (1,802 miles) north of eastern Australia. Health officials of the Pacific Island nation have reported that cases are rapidly increasing. As of 2 August, there were over 6,000 cases of the virus, with 11 deaths.

The last week of July saw over 1,200 positive cases, including some cases caught at the border prior to the outbreak.

Several top politicians and senior officials have caught the disease, including Vice President Yosiwo George, who has been admitted to hospital. Officials have shared that his condition is improving. The initial outbreak arrived less than two weeks before Micronesia planned to end its quarantine restrictions and reopen its international borders on 1 August.

Last year, Micronesia became one of the few countries to impose a broad mandate which requires all eligible citizens get vaccinated against the coronavirus. The government threatened to withhold federal funds from any individuals or business owners who refused vaccination. Health officials have shared that 75% of people aged 5 and over were fully vaccinated.

Elsewhere in the Pacific, the Omicron variant has spread the virus to several small nations for the first time this year, including Kiribati, Tonga, Samoa, and Nauru.

Recent hepatitis linked to adeno-associated virus AAV2

Recent acute hepatitis cases of unknown origin in children have now been linked to the virus AAV2 in two new UK studies, with no evidence of a direct link to SARS-CoV-2 infection.

The two studies were led independently, with one examining cases from Scotland by the MRC-University of Glasgow Centre for Virus Research (CVR) and the Royal Hospital for Children in Glasgow, in partnership with Public Health Scotland and ISARIC (International Severe Acute Respiratory and emerging Infections Consortium) WHO Clinical Characterisation Protocol UK (CCP-UK).

The second studied cases from across the UK at Great Ormond Street Hospital and the UCL Great Ormond Street Institute of Child Health (UCL GOS ICH), in partnership with the UK Health Security Agency.

Professor Emma Thomson, Clinical Professor and Consultant in Infectious Diseases at the MRC-University of Glasgow Centre for Virus Research (CVR), and senior author of the Scottish study, commented: "The presence of the AAV2 virus is associated with unexplained hepatitis in children. This virus can only replicate in the presence of another virus (usually an adenovirus). AAV2 may cause disease itself, or it may be a useful biomarker of recent adenovirus infection which may be the main underlying pathogen, but which can be harder to detect.

"There are many unanswered questions, and larger studies are urgently needed to investigate the role of AAV2 in paediatric hepatitis cases. We also need to understand more about seasonal circulation of AAV2, a virus that is not routinely monitored - it may be that a peak of adenovirus infection has coincided with a peak in AAV2 exposure, leading to an unusual manifestation of hepatitis in susceptible young children."

Since April 2022, a number of young children worldwide have developed jaundice and acute severe hepatitis of unknown origin. WHO has reported at least 1,010 probable cases in 35 countries.

Children with the condition have commonly had to be hospitalised for a number of days, with 11 children in England and one in Scotland requiring a liver transplant.

In the UK, most of the 268 cases have been under the age of five years old, with nearly 40% of hospitalised cases (74 of 189) requiring admission to intensive care.

Initiative to improve next generation cancer diagnostics

The Medical Device Innovation Consortium (MDIC) has formally launched its Somatic Reference Samples (SRS) Initiative with a pilot project.

The new initiative will aim to improve the validation and regulatory review process for sequencing-based cancer diagnostics.

The initiative will seek to develop, manufacture, and validate publicly available somatic reference samples, and create public genomic datasets with the potential to be used by sponsors and regulators.

Next generation sequencing (NGS) is a parallel sequencing technology that offers extremely high throughput, scalability, and speed.

"Through the MDIC SRS Initiative, we are developing reference samples and data sets that can be used globally by test developers and regulators, to bring more consistency to NGS-based cancer diagnostic development, increasing the confidence and accuracy of these tests, which will ultimately lead to more accuracy in diagnosis and treatment for patients," said Andrew Fish, President and CEO, MDIC.

MDIC will lead a collaboration with the FDA, the National Institute of Standards and Technology (NIST), National Institutes of Health (NIH), and industry stakeholders, to manufacture, validate, and distribute SRSs to simplify and support validation of NGS-based cancer diagnostics.

"There is a need for appropriately consented, highly characterised, and broadly available reference materials that may improve the accuracy, reliability, and transparency of NGS-based oncology tests, and support the generation of validation data for use in regulatory submissions. The reference samples and datasets being created by the MDIC Somatic Reference Samples Initiative can help fulfil this need," said Wendy Rubinstein, MD, PhD, Director, Personalised Medicine, Centre for Devices and Radiological Health, FDA.

The initiative also includes the goal to create a publicly available global genomic data resource library of datasets with the potential to be used by sponsors and regulators.

Home-based brain stimulation study on stroke survivors finds mixed results

26 stroke survivors were given four weeks of remotely supervised transcranial direct current stimulation (RS-tDCS) in an at-home cognitive rehabilitation pilot study, which revealed this method to be feasible, but not wholly effective.

The Korean version of the Montreal Cognitive Assessment (K-MoCA) was used to characterise the results, finding that the therapy improved general cognitive function but not significantly more so than computerised cognitive therapy alone. This was reported by Yun Hee Kim, MD, PhD, along with colleagues at Samsung medical centre etc.

The researchers also noted that only stroke survivors with moderate cognitive impairment, and those with left hemispheric lesions, showed an improvement on the K-MoCA, compared with controls.

Randomisation was used to allocate either RS-tDCS or a sham computerised cognitive therapy to patients. Both involved the survivors wearing an electrode-embedded soft cap on their heads. They could not control the settings themselves – a remote supervisor-controlled session duration and intensity.

However, in both protocols, patients and caregivers had training for correct tDCS self-application to see if it would be possible for this treatment to be used within the home without supervision.

A major flaw in the study was the lack of a control group who didn't undergo any type of cognitive therapy. There was also a small sample size, making it hard to extrapolate the results.

Kim stated: "Despite the preliminary nature of this study on the efficacy of home-based tDCS, the present study provides new hope for patients with stroke and their families, for accessible and continuous rehabilitation of cognitive symptoms."

In studies relating to Alzheimer's disease, major depressive disorder (MDD), and schizophrenia, RS-tDCS has been shown to be effective in lessening symptoms. A recent trial also showed home-based tDCS improved attention in people with attention deficit-hyperactive disorder (ADHD) who weren't taking stimulant medications.

New method involving musical tests can enable early detection of cognitive decline

Researchers at Tel Aviv University have created a method which uses musical tests and a portable brain activity-measuring device to detect cognitive decline in older people.

The method measures 15 minutes of electrical brain activity while the user is performing simple musical tasks.

"Our method enables routine monitoring and early detection of cognitive decline, in order to provide treatment and prevent rapid, severe deterioration," said the researchers involved in the study.

During the test, the user wears a portable electroencephalography (EEG) device, with only three electrodes attached to their forehead. They are then fed instructions through headphones which ask them to perform musical-cognitive tasks. For example, pressing a button each time every instrument is played in a melody, then pressing it only when the violin is being played.

The test also includes musically-guided meditation that is designed to bring the brain to a resting state; this state can indicate cerebral functioning.

Neta Maimon, the lead researcher and PhD student at the School of Psychological Sciences and the Buchmann-Mehta School of Music, explained how music is known to be a mood stimulant, particularly of positive emotions, yet when we are concentrating, music can also be quite cognitively challenging. Combining these two sides creates a test which is both useful for the researchers and pleasant for the subjects.

Included in the study was an experiment at the Dorot-Netanya Geriatric Medical Center. New patients at the centre undergo a standard test – the 'mini-mental' test – which evaluates their cognitive condition. Patients can score up to 30, with a higher score indicating a more normal cognitive function level.

The experiment tested 50 patients at Dorot-Netanya who had scored 18-30 on the mini-mental test, using the method designed by Maimon and her team. This allowed "mathematical indices to be identified that were precisely correlated with the mini-mental test scores."

The researchers shared: "Today, millions of people around the world already suffer or are liable to suffer soon from cognitive decline and its dire consequences, and their number will only increase in the coming decades.

"Our method could pave the way towards efficient cognitive monitoring of the general population, and thus detect cognitive decline in its early stages, when treatment and prevention of severe decline are possible," they concluded.

MiroBio to be acquired by Gilead Sciences

This partnership will accelerate MiroBio's candidates into treatments for patients

Medicxi, a European life sciences investment firm, has announced that its portfolio company, MiroBio Limited, has entered into a definitive agreement with Gilead Sciences to be acquired for approximately $405 million in cash, subject to customary adjustments.

MiroBio is a privately-held, UK-based biotechnology company, focused on restoring immune balance with agonists targeting immune inhibitory receptors. It was spun out of the University of Oxford in 2019 to develop a new class of medicines known as immune checkpoint agonists, to treat autoimmune diseases by restoring balance to the immune system.

Oxford Science Enterprises and Samsara BioCapital co-led a £27 million Series A investment in 2019. Medicxi invested in the company in June 2022, when it led an £80 million Series B financing.

Nick Williams, Partner at Medicxi, commented: "The acquisition of MiroBio is a testament to the best-in-class discovery engine and highly innovative pipeline they have built. Despite recent advances, significant clinical unmet needs remain in inflammatory and autoimmune diseases with the majority of patients not achieving remission with current therapies."

Williams was recently appointed as Partner at Medicxi. He oversaw the investment and joined MiroBio's board.

"By restoring immune homeostasis, MiroBio's checkpoint agonists have the potential to provide novel and differentiated treatments to these patients," Williams continued. "We are extremely excited to be partnering with an experienced organisation like Gilead, which will accelerate MiroBio's candidates into treatments for patients."

Giovanni Mariggi, co-founder and Partner at Medicxi, commented: "Over a decade of foundational research into inhibitory receptors at Oxford University provided MiroBio with world-leading domain expertise in checkpoint agonism. We are proud to have backed the company as they transition into the clinic and delighted for Nick, our newly-appointed Partner at Medicxi, on his continued success in creating and investing in biotech companies with the aim to deliver meaningful treatments for patients."

US government buys 66 million doses of Moderna's Omicron booster

The US government has confirmed its plans to begin a booster vaccination campaign this autumn, and has placed a $1.74 billion order for 66 million doses of an updated version of Moderna's Spikevax shot.

This updated vaccine will target the BA.4 and BA.5 subvariants of Omicron, which are responsible for most of the COVID-19 cases around the world currently.

Moderna's current clinical data is for a shot based on the original Wuhan strain of SARS-CoV-2, and the first Omicron subvariant (BA.1) that emerged towards the end of 2021. However, the FDA's Vaccines and Related Biological Products Advisory Committee (VRBPAC) concluded in a recent meeting that future booster campaigns should ideally include jabs which target BA.4 and BA.5. Moderna's initial BA.1-based booster showed lesser activity against the new subvariants.

Moderna is now advancing both BA.1 and BA.4/5 candidates into clinical testing "based on different population health security strategies in different countries".

This order comes after the Department of Health and Human Services (HHS) ordered 105 million doses of Pfizer and BioNTech's updated vaccine in June.

The second booster doses will be available for Americans 50 years and older, as well as for younger people who are more vulnerable to COVID-19 due to underlying health issues.

Approximately 107 million people have now received a first COVID-19 booster jab, according to data from the Centres for Disease Control and Prevention (CDC). According to Moderna, the supply contract also includes an option to purchase another 234 million doses of its booster shot.

Sanofi makes €300 million investment in Innovent for cancer

Two key oncology medications will be brought to patients as rapidly as possible

Sanofi has announced a €300 million investment in Chinese biotech Innovent, as part of a collaboration to bring two new cancer therapies as quickly as possible to patients.

The deal includes an option on a second €400 million investment, and will see the two companies jointly developing tusamitamab ravtansine (SAR408701), a CEACAM5-targeting antibody-drug conjugate (ADC), as well as a pegylated formulation of interleukin-2, codenamed SAR444245 in China.

Sanofi has already taken its ADC into Phase III testing outside of China as a second or third-line therapy for non-small cell lung cancer (NSCLC), pitching at regulatory fillings in the US and Europe next year.

The company is also testing it in Phase II trials in gastric cancer and other solid tumours, as a monotherapy, and in combination with other drugs.

Innovent is responsible for developing the drug in China, picking up exclusive marketing rights there, with Sanofi in line for up to €80 million in milestone payments as well as royalties on sales if it gets approved. Sanofi is taking the lead on commercialisation, while Innovent stands to receive up to €60 million in milestones, plus royalties.

"This strategic collaboration with Innovent will not only accelerate the development, market access, and future commercialisation of two of our key oncology medicines in selected combinations with sintilimab, but also bolster our overall presence in oncology in China," commented Sanofi's head of R&D, John Reed.

PCI Pharma Services announces multi-million-dollar expansion to UK manufacturing facility

PCI has announced a significant expansion of its facility in Tredegar in Wales, UK, designed to help keep up to date with the market growth of powerful, targeted oncology therapies.

The expansion includes two new facilities dedicated to the manufacturing and packaging of solid oral-dose tablets and capsules. A second contained manufacturing building will double large-scale processing capacity, including dispensing and fluid bed granulation of high-potency solid-dose products at a commercial scale. There is also a new multi-product packaging facility with primary and secondary blistering and bottling suites.

"We're excited to announce the latest expansion in Tredegar that will address the growing and urgent need for specialty global manufacturing services within the oncology arena," said Salim Haffar, CEO of PCI Pharma Services.

"As the market expands for potent therapies and highly complex, concentrated formulations that can present unique challenges to manufacturing and packaging," Haffer continued. "We're proud to be one of the few providers with the global capabilities to manage this specialty at both clinical and, importantly, commercial scale."

"The rapid evolution of oncology pipelines has coincided with the continued globalisation of clinical development," commented Rebecca Coutts, PhD, General Manager, Tredegar, PCI Pharma Services.

"This latest investment, along with the existing analytical and formulation capabilities, combines a clinical and commercial scale packaging facility to complement the existing clinical and increased commercial scale manufacturing capabilities, placing end-to-end services for these high-potency molecules under one roof to better serve our client's evolving needs."

CDMO wins tech transfer and production contract for HIV drug candidate

Societal CDMO will execute appropriate technology transfer activities

Societal CDMO, a contract development and manufacturing organisation (CDMO), has been selected to provide CDMO services to support the ongoing clinical development of a novel HIV drug candidate in Europe.

The compound is a solid, oral dose, anti-viral therapy approved for the prevention and treatment of HIV in certain countries in Europe. Societal has shared that its work will be focused on supporting the expansion of the product's indications.

Societal CDMO is dedicated to solving complex formulation and manufacturing challenges primarily in small molecule therapeutic development.

Under the terms of the new agreement, Societal CDMO will execute appropriate technology transfer activities, followed by cGMP manufacture of clinical trial material, to support the initiation and execution of Phase II studies of the drug candidate.

This work will include production, packaging, and labelling of both the active compound and matching placebo for the study.

There were approximately 1.5 million new HIV infections in 2021, according to a new report by UNAIDS, a joint UN programme advocating for global action to address the HIV and AIDS epidemic. Its data shows HIV infections have increased since 2015 in 38 countries globally, and that every day 4,000 people become infected with HIV, including 1,100 young people aged 15 to 24 years old.

"We are proud to be trusted by our partner to execute the tech transfer, manufacturing, and packaging services that will be necessary for the initiation of its planned Phase II study in Europe," said David Enloe, CEO of Societal CDMO. "We continue to be pleased with our progress in leveraging our end-to-end CDMO service offerings to grow our business and expand our client base."

F2G announce development and commercialisation for rare antifungal treatment

F2G, a clinical-stage biopharmaceutical company, has announced that it can now bring a fourth antifungal class to the market. Currently, there are only three classes of antifungal treatments.

F2G has secured $70 million in financing to produce the new antifungal treatment, though it has yet to unveil data from its open Phase IIb study in patients with rare and resistant moulds. The company has shared that it plans to unveil results in October 2022.

The funding will enable F2G to advance late-stage development and commercialisation in the US of olorofim, a novel oral antifungal therapy to treat invasive aspergillosis (IA) and other rare mould infections.

However, the results from the Phase IIb trial have already secured the company FDA breakthrough therapy, qualified infectious disease product, and orphan drug designations.

F2G is focused on the discovery and development of novel therapies to treat life-threatening rare fungal infections with a high unmet medical need.

Francesco Maria Lavino, CEO of F2G, said: "We are delighted to attract this additional capital from such high calibre late-stage investors to F2G, and welcome Nanna and Joe to the Board. Their experience will be invaluable as we move to our next stage of growth.

"This is a pivotal year for the Company. We are building a world class team with commercial, operational, and deal-making experience as we prepare for final development and commercialisation of olorofim in the US. If approved, olorofim is expected to be the first new class of anti-fungal with a novel, differentiated mechanism of action in more than 20 years and will address genuine unmet needs in conditions with high morbidity and mortality."

Nanna Lüneborg, General Partner at Forbion, commented: "The Forbion Growth Opportunities Fund II focuses on promising late-stage European life sciences companies like F2G. We have been very impressed with the significant progress achieved to date, and we are pleased to support this highly impactful company in its next stage of growth as it pursues commercialisation of olorofim in the US."

Olorofim exerts fungicidal activity through inhibition of the pyrimidine synthesis pathway. It is anticipated to be used to treat patients with a serious, invasive, rare fungal disease where existing treatments are inappropriate or no longer effective.

Joe Anderson, Partner at Sofinnova Partners, said: "We aim to support outstanding companies developing innovative treatments for life-threatening disease. With its highly experienced team, F2G has made significant progress over recent years, and is now close to bringing its breakthrough product to market for patients with limited treatment options. We look forward to working with management, the board, and our co-investors, as the company moves into this important phase in its development and growth."

Cognito's light-and-sound therapy slows brain tissue atrophy in Alzheimer's study

The company plans to launch new trials into Alzheimer's disease this year

Cognito Therapeutics has revealed new data showing that a six-month use of its digital therapeutic could potentially reduce the rate of atrophy within the brain's white matter in patients with Alzheimer's, according to Fierce Biotech.

The white matter ferries electrical signals around the brain's large regions of grey matter. Deteriorating white matter is a normal sign of ageing, but the atrophy is more pronounced in Alzheimer's patients.

Through using a headset that delivers pulsing lights and sounds at fast and specific frequencies, the company aims to stimulate the activity of immune cells inside the brain, known as microglia. The aim is to help the central nervous system clear out certain proteins which can lead to different neurological diseases and dementia.

Cognito said that it plans to launch new, pivotal trials in Alzheimer's disease this year.

The company delivered the latest findings at the Alzheimer's Association International Conference in San Diego. In the Phase II study, named Overture, participants wore the company's GammaSense headset at home for one hour per day for six months. The analysis compared participants treated in the Overture study, which enrolled 76 people, to historical data collected from a separate, decade-long, global study, conducted by the Alzheimer's Disease Neuroimaging Institute. The company said that it saw less white matter shrinkage after half a year – even slightly increasing over baseline readings, by about 0.4%, compared to the start of the Overture trial.

Cognito CEO Brent Vaughan commented: "These results continue to build on our knowledge and understanding of how gamma frequency patterns in the brain are disrupted in patients with Alzheimer's disease, and how our technology restores the levels of neural activity to improve cognition."

Digital physical therapy app OneStep launches fall risk detection using a smartphone

OneStep, an app-based physical therapy service, has expanded its technology to include fall risk detection capabilities.

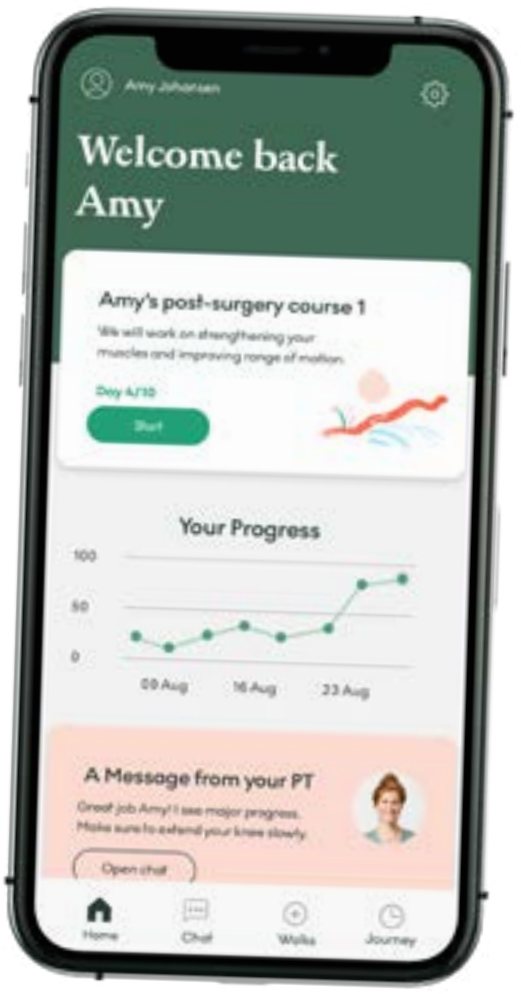
The company leverages smartphone sensors to continuously analyse walking and functional movement and provide lab-level gait analysis.

Patrick Tarnowski, Chief Commercial Officer at OneStep, commented in an interview that gait is a key health indicator, and monitoring it can prevent future health problems, such as falls. "We know that gait speed, which has been dubbed the sixth vital sign, is able to track how a patient is doing over time."

When there is a decrease in gait speed, or an increase in gait variability, it likely indicates an impairment that should be addressed for mobility, safety, and overall wellness.

Through OneStep, providers alerted of a trend that may indicate increased probability of falls can take immediate action to correct gait impairments, provide assistive devices, and put in place fall safety education before a medical event occurs.

"We can tell the provider through the dashboard about changes in Ms Jones and indicate that she is likely to fall very soon," Tarnowski said. "We can reach out through the OneStep platform and deploy one of your clinicians to see her in her home. It's exciting for providers because they can move upstream in their care continuum. It's not just being reactive to a fall. The technology doesn't just notify someone when a fall has occurred, but looks at trends that she is going to fall."



ResMed buys Mementor for prescription digital health app

ResMed has acquired Mementor to add an insomnia digital health application to its portfolio of products in Germany.

Mementor is the developer of Somnio, a prescription that uses cognitive behavioural therapy to try to improve the sleeping patterns of people with insomnia within three months.

The new deal gives ResMed ownership of the only permanently approved digital health application in the field of sleep medicine in Germany. Approximately 10% of Germans are affected by clinically relevant insomnia. Access to in-person treatment for insomnia is limited, which has led Mementor to create an app that scales the service and increases accessibility.

In a randomised trial of 56 participants, run by two of the founders of Mementor, use of a web-based unguided self-help programme with automated feedback was associated with significant decreases in sleep-related cognitions, safety behaviours, depression, and somatisation. Some of these effects persisted through a 12-month follow-up assessment.

After Mementor is integrated into ResMed, Noah Lorenz, the co-founder and CEO of the startup, and Katherina Jekerle, will lead the division. Jekerle previously worked as ResMed's Senior Marketing Director in Germany. Lorenz and Jekerle will work on further digital health developments, according to MedTechDive.com.

The acquisition adds to the digital expertise that ResMed has cultivated in recent years through internal programmes and other takeovers, including its $225 million buyout of connected inhaler company Propeller Health.

Personalised healthcare is key to a sustainable NHS – and pharma can help

Doina Ionescu, General Manager at Merck, UK & Ireland, explores the importance of personalised healthcare and supported self-management, and highlights the work that Merck is doing in these areas

***Pharmafocus:* Why do you feel so passionately about personalised healthcare?**

Doina Ionescu: To me, the optimisation of people's individual treatment needs must be a priority, not a luxury. More personalised approaches to healthcare today are vital to achieve the best and most sustainable health management in the future. According to recent data, supported self-management can result in 19% fewer GP appointments and 38% fewer A&E attendances. There are clearly financial implications that are positive for the NHS budget. But it's not only about cost savings – it's also about enabling people to live better and healthier lives.

What is the current state of play?

The NHS Long-Term Plan outlines the aim that 2.5 million people are receiving personalised healthcare by 2024.

They concluded that personalised healthcare is critical in helping to reduce health inequalities, and support the future sustainability of our healthcare system.

COVID-19 put a break on personalised healthcare, but it has also accelerated the need for it, as prevention and proactive management of patients (and longer-term cost savings) will be more important post-COVID-19 than pre-COVID-19. While there is mounting evidence of the positive impact of personalised healthcare initiatives (particularly in mental health and learning disabilities) in primary care, personalised healthcare has not yet become systematically embedded in secondary care.

That's why, at Merck, we want to focus on that issue, as well as the wider needs around personalised medicine and better/equitable genomic testing across the UK, so that newer, more targeted medicines reach the right patients.

What is Merck doing to further embed personalised healthcare specifically?

We have created a personalised healthcare framework of accountability, supported by a set of guiding principles that will be embedded within our businesses, and all future personalised healthcare activity we initiate, or support will be evaluated against them. The framework and the principles were based on insights collected from a multistakeholder survey, which was completed by over 100 individuals working within the NHS, pharma, patient organisations, and academia. We then took a deep dive into the survey findings with multidisciplinary experts, and explored how Merck could support the implementation of the personalised healthcare agenda, with a particular focus on shared decision making and supported self-management.

How does the work Merck is doing in the UK & Ireland reflect the PHC Framework and Guiding Principles you have developed?

If we take multiple sclerosis (MS) as an example, we know that patient outcomes are better when patients have a key role to play in their ongoing treatment, and we believe this supports the NHS Recovery Plan post-COVID-19. And yet, depending on where patients live, their ability to be a part of their healthcare decisions will vary. MS patient numbers are only increasing, and services are already stretched. New models of care are needed. We want to support clinicians to make shared decision-making and supported self-management an embedded part of MS patient care, and we are working on some exciting plans to offer practical solutions and ideas for the sustainability of quality care for patients in supported self-management.

Merck also has a long-standing heritage in fertility, and we are about to launch a campaign to empower patients to become more informed, empowered, and play a more vocal role in their fertility treatment and care.

What needs to happen in the future?

What I found striking at the outset was that there seemed to be no clarity on which organisation would be accountable for ensuring that there is a systematic approach to planning and delivering services, particularly in secondary care. We shared our Framework and Guiding Principles with NHS leaders, and the response was really positive. There is an appreciation that pharma has a key role to play. Pharma has skills and capabilities such as R&D and access to big data, which can help to enhance health and close the gap in health inequalities. I think that we need a task force – just like the Vaccine Task Force – that has one consistent goal and that brings people together from industry, Integrated Care Systems (ICS), the NHS and patient organisations. Merck hopes to be able to play a pivotal role in driving additional initiatives forward in partnership with the NHS. The NHS can't do it all by itself.

Doina Ionescu has been Merck's General Manager for UK & Ireland since May 2020. Since joining Merck as a research scientist in 1998, she has held a diverse range of roles within the company including in Corporate Business Development, Corporate Mergers & Acquisitions and Commercial Operations. In June last year, she was elected to the Board of Directors for the Association of British Pharmaceutical Industry. She also represents Merck on the European Medicines Group, contributing towards the development of its policy positions and advocacy efforts. Doina is a physicist by background, and holds a Master's degree in nuclear physics from the University of Bucharest, Romania, a PhD in physics from the University of Southampton, UK, and an Executive MBA from Ashridge Business School, UK.

To find out more, visit: www. merckgroup.com/uk-en/expertise/news.html

Up close and personal: The potential of precision medicine in mental health

Personalised medicine has revolutionised treatments and kept patients at the fore, across an array of therapeutic areas. Lina Adams illuminates the applications that this has, and is continuing to have, in the field of mental healthcare

As technology has advanced exponentially over the years, the landscape of clinical practice has continually evolved to adapt to these changes. The principles of personalised medicine have always been integral to clinical practice, since the very first efforts to classify disorders, and to prescribe a specific treatment on the basis of a precise diagnosis.

The proliferation of this method across many therapeutic areas could allow clinicians to prevent disease at the onset, before clinical symptoms appear.[1] They can then select the appropriate therapy for the individual and save costs in both clinical trials and healthcare.

Personalised medicine tailors treatment to individuals based on genetic factors and clinical information. This has applications in a broad range of medical fields, such as cancer genomics, and in the diagnoses process of an array of conditions. Research has also shown that personalised medicine can be used for those with, and at risk of, heart and circulatory diseases.

There are significant applications for precision medicine in mental health, as everyone has unique biological factors that impact their neurology. Recent research has shown, in particular, promise in mapping the genetics of clinical depression and major depressive disorder (MDD).

Mind matters

Pharmafocus spoke to Javier Garcia Palacios, PhD, Head of Personalised Healthcare Integrated Solutions, Roche, who shared: "Personalised healthcare means better health at a lower cost for people and society, by shifting from a one-size-fits-all approach to the best care for each person. With personalised medicines, treatments target the underlying biology of a disease and advanced diagnostics help doctors find the right treatment for the right patient.

"Personalised healthcare more broadly is a holistic approach to healthcare that aims to integrate the components of care (prevention, diagnosis, treatment, monitoring) into a seamless experience that helps people achieve optimal outcomes while reducing complexity and costs."

Recent research has shown promise in mapping the genetics of clinical depression.[2] According to Ness Labs: "The China Oxford and VCU Experimental Research on Genetic Epidemiology study analysed the cases of more than 5,000 women suffering from major depressive disorder (MDD) and used low coverage sequencing to genotype both these women and a control group of equivalent size; they identified two genome-wide significant loci contributing to the risk of MDD, one near the SIRT1 gene, and the other in an intron of the LHPP gene."[1]

This study is encouraging, as the results suggest that low sequence coverage of a large number of individuals can be an effective way to infer genetic signals, supporting research that depression is largely genetic.

Javier added: "Using advances in data, analytics and technology, a personalised approach to healthcare has the potential to provide a more precise, evidence-based, holistic care tailored to each individual, helping patients achieve better outcomes throughout their lives. At Roche, we leverage advancing and emerging digital technologies to enable more personalised care, and aim to be transversal to mental health across disease areas. For example, anxiety and depression are common among people with chronic diseases, with the prevalence of depression during cancer treatment being approximately 14%. Therefore, having a holistic, personalised approach for oncology patients, including mental health, can lead to better outcomes overall.

"Additionally, with a strong need for resilience in facing chronic diseases, we see the impact positive mental health, including appropriate stress and sleep management, can have on therapy adherence and compliant behaviours. Many apps focus uniquely on mental health, and we now see them expanding to encompass mental health through chronic disease management."

Personalised medicine for mental health can also come in the form of virtual therapy platforms. In recent years, many apps have come to the forefront of digital health to personalise care for each individual and ensure that each patient's needs are being met. Smartphone apps create ease of accessibility in areas such as diagnosis, symptom tracking, and self-management. The latter, in particular, has allowed patients to access support regularly and at their fingertips.

Chronic health can inevitably take a toll on a patient's wellbeing, due to concerns about financial costs, health anxiety, and individual circumstances. Heal is an app providing urgent care as well as mental health support, offering at-home care through telemedicine, remote appointments, and remote monitoring. Similarly, the app AmWell offers online doctor consultations and healthcare support, as well as options for therapy. These options enable clinicians to scale therapy and offer personalised support for those with mental illnesses. As new therapeutic interventions emerge, technology continues to be pivotal in the management and treatment of mental health.

Rising to the Challenge

Developing personalised medicines for mental health is not a streamlined process, and there are inevitable security concerns that need to be considered. Healthcare systems and pharma companies need to work collaboratively to ensure that data is being efficiently stored and managed.

For the May issue of *Pharmafocus*, we spoke to Dr Andy Blackwell, Chief Scientific and Strategy Officer of data-driven AI healthcare company ieso, about challenges in developing smartphone apps for mental health. Dr Blackwell told us: "Digital innovation in mental health is currently transforming access to mental healthcare. However, access is not enough – there must also be a focus on quality and outcomes.

"We believe digital innovations, that engage patients and show real-world evidence of efficacy, will ultimately be industry leaders

in the future of mental health care. Further, the insights from a data-rich sector like digital therapeutics can generate will help clinicians to make more accurate diagnoses, to optimise and personalise treatment decisions ,and to eliminate unnecessary or ineffective practices."

Javier, at Roche, also shared with Pharmafocus: "The healthcare industry generates the world's largest volume of data, and medical knowledge doubles every few months. This creates challenges both for healthcare systems that can't effectively and efficiently capture and deliver value from underutilised sources of data, and providers and patients navigating an overload of information. Additionally, delivering truly personalised healthcare can be challenged by the need to achieve patient trust, which is paramount to fully realising the value of data and data-enabled technologies. To earn and maintain patient trust, we must be transparent and uphold the highest standards for data privacy, de-identification, and security. It's also essential to incorporate the patient's perspective to design truly patient-centred, user-friendly digital tools that solve for distinct patient needs."

Patient trust is of utmost importance when developing personalised medicines, as individuals need to know that they can trust their healthcare provider. In June, I wrote a feature in which I explored the rising role of technology and AI in data management. Electronic Data Capture (EDC) methods, such as Bluetooth or similar technology, as accurate data can be obtained directly from the patient, increasing patient convenience through allowing real-time data to be synced into the clinical database. It is important to mitigate security concerns when managing and storing data from participants in trials. This can be done through using reputable cloud service providers to offer continuous security monitoring and incident response, as they can quickly identify security issues and can apply patches.

If healthcare providers can work in conjunction with pharma companies to mitigate cybersecurity concerns, they can significantly increase patient trust and therefore keep the patient's needs at the core of treatment.

Looking Ahead

Personalised medicine still has plenty of room for application in mental health, but due to the complexities of the brain and the pathophysiology of its disorders, this is no easy feat. Each patient's neural networks are different, and what works for one individual may have little effect on another.[3] Research needs to advance in order to enhance the clinical management of mental illnesses, especially in high-risk and complex patient populations, such as women with children under 12 months, and in the elderly.

Biomarker development and precision medicine interventions need to be integrated, to enhance patient outcomes. These biomarkers can help in detecting suicidal tendencies, sometimes reaching about 95% accuracy.[4] Whilst there is currently limited evidence supporting biomarkers in mental health research, there is still ongoing research to support their role in identifying disease subtypes, with the end goal of enhancing prevention and diagnosis. The future of precision medicine in mental healthcare, and beyond, is certainly looking promising.

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Access all areas: increasing health equity with early access

Time is an invaluable resource in healthcare, and bringing lifesaving therapies to the patient is paramount. Ana Ovey explores the importance of early access to medicine schemes in deploying treatments to the patients who need it, and other areas this programme can be applied

Since the outbreak of a pandemic across the globe, we've grown used to governments and healthcare providers resorting to investigational and repurposed drugs in order to treat patients in need of care. Initiatives such as the UK's RAPID-19 aimed to get treatments for COVID-19 to NHS patients in a drastically accelerated timeframe. Other programmes established by the FDA allowed clinicians in the US to gain access to investigational therapies during the pandemic, and programmes such as the expanded access (EA) programme, and emergency use authorisation (EUA) programme, have allowed for the rapid deployment of potential therapies for investigational treatments with emerging evidence.

COVID-19 highlighted the reality that time is an invaluable resource in healthcare, and the delivery of lifesaving therapies to patients hinges on the timescale they can be delivered in.

Early Access in the UK and Rare Disease Innovation

Early access schemes also play a vital role in delivering treatment and management for rare diseases. The ABPI has shared that "nearly 40% of the medicines now in development are for rare or very rare conditions, including many cell and gene therapies which are personalised to individual patients."[1]

Considering that only 10% of known rare diseases currently have an approved treatment,[2] it is clear the impact early access schemes may have on patients living with conditions for which no approved treatment, or even management option, currently exist. There may be medicines in the development pipeline for these conditions not yet approved for marketisation, but with clear clinical benefit.

"The Early Access to Medicines Scheme (EAMS), the Innovative Medicines Fund, Accelerated Access Collaborative and Innovative Licensing and Access Pathway all hold the potential to improve the situation for patients with rare and very rare conditions – as well as those for more common conditions," the ABPI shares.

In the UK, there are a number of initiatives aiming to bring patients early access to lifesaving medicines. The Accelerated Access Collaborative (AAC) is a fast-track route into the NHS for 'breakthrough' medicines and technologies. It is a national group made up of partners including NHS England and NHS Improvement, the Government, NICE, industry associations, research organisations, patient representatives, and other health and care partners.

The Innovative Licensing and Access Pathway (ILAP) aims to streamline patient access to safe, financially sustainable, and innovative medicines, including new chemical entities, biological medicines, new indications, and repurposed medicines. The Accelerated Access Collaborative, EAMS, Innovative Licensing, and Access Pathway are all mechanisms designed to fast-track promising medicines safely through the approval process, so that they get to patients who could benefit as soon as possible. The ABPI believes that "the Early Access to Medicines Scheme (EAMS), the Innovative Medicines Fund, Accelerated Access Collaborative, and Innovative Licensing and Access Pathway all hold the potential to improve the situation for patients with rare and very rare conditions – as well as those for more common conditions," but maintains, however, that "the opportunity of these initiatives needs to be realised for rare diseases, as well as more common ones."

Ebola and Early Access

Following the outbreak of Ebola virus in West Africa in 2014-16, WHO issued a statement sharing that, in healthcare emergencies, it was ethical to provide patients access to investigational drugs not approved by regulators, in order to save lives.

Johnson & Johnson announced that it would donate Ebola vaccine regimens in support of a WHO early access clinical programme. The programme was launched in response to an outbreak of the virus in Guinea, and aimed at preventing Ebola in West Africa. It began by vaccinating health and other frontline workers, alongside those at increased risk of exposure to the virus in Sierra Leone.

By the end of 2021, over 250,000 patients participating in clinical trials and vaccination initiatives had received at least the first dose of the vaccine regimen for Ebola by J&J. Further, over 200,000 had been fully vaccinated.

There were limitations to the study, however, including the fact that there were more men than women in the adult trial, and the measurement of Ebola antibody concentration levels was only in a subset of participants. Other limitations of the study include the fact that it only focused on safety and immunogenicity, as it was implemented when the West Africa Ebola outbreak had been brought under control. It was therefore not possible to evaluate the efficacy of the regimen.

EAMS in the time of COVID-19

The Early Access to Medicines Scheme (EAMS) was responsible for the select use of the first COVID-19 medicine, Gilead's remdesivir. This drug worked to save lives, decrease disease severity and thereby decreasing health issues such as long-COVID-19, and lessening the burden faced by the NHS in a time of crisis. In the UK, the Early Access to Medicines Scheme (EAMS) works to give those living with seriously debilitating or life-threatening diseases, early access to new medicines that have not yet received a marketing authorisation, where there is significant and unmet medical need.

Further, following analysis of the six-week consultation by the MHRA in 2021, the UK regulator has prepared a Government response, outlining key legislative changes, which will provide the UK with an opportunity to maximise the Scheme's impact. This will be done through accelerating availability of medicines for patients, reducing the burden on manufacturers supplying EAMS medicines, and facilitating the collection of real-world data, which may be used as evidence to support regulatory decision-making for future authorisations.

All this will work to help support more patients benefiting from important EAMS medical products, and the healthcare practitioners delivering them.

Early Access to ART for All

WHO recommends antiretroviral treatment (ART) for all HIV-positive patients, regardless of CD4 count or disease stage. This initiative is referred to as "Early Access to ART for All" (EAAA). Early HIV infection is the period up to 6 months after infection with HIV.

HIV is treated with antiretroviral medicines, which stop the virus replicating in the body. ART involves taking a combination of HIV medicines every day, and while it cannot cure HIV, they reduce a person's viral load to an undetectable level. This suppression of the virus allows the immune system to repair itself, and prevent

> **It's possible that we'll see more early access schemes in the years to come than ever before**

further damage. A combination of HIV drugs is used, as HIV can quickly adapt and become resistant. For such reasons, it can be extremely important to equip patients with the best and most effective and individualised care for them as possible. Various Early Access schemes can help deliver such healthcare to patients.

Without access to antiretroviral medicines, the virus is able to attack and destroy the infection-fighting CD4 cells (CD4 lymphocyte) of the immune system, the loss of which makes it more difficult for the body to fight off infections, as well as certain HIV-related cancers.

EAAA saw a "substantial effect on retention and a large effect on viral suppression", according to the Final Report on the study published in December 2018. "During 'Standard of Care' and 'Early Access to ART for All' respectively, the 12-month retention rates were 80% and 86%, and the viral suppression rates at 12 months post-ART initiation among those we were retained that long were 4% and 79%."

The report shared: "The MaxART Early Access to ART for All implementation study has been a unique project, testing the real-life implications of providing early antiretroviral treatment (ART) to all people living with HIV through the government managed health system of the Kingdom of Eswatini. The results show that early ART is acceptable, feasible, affordable and effective in preventing new HIV transmissions and improves the health and well-being of people living with HIV. The potential of early ART opens a new era in HIV prevention and treatment, with the possibility of ending HIV and AIDS."[3]

In the stepped-wedged, randomised, controlled trial, 14 public sector health facilities in Eswatini were paired, and randomly assigned, to stepwise transition from standard of care (SoC) to EAAA. The MaxArt Consortium trial additionally saw an increase in testing coverage in the community, where compared with baseline data gathered three months prior to the start of the trial, there was a 273% proportional increase in HIV test conducted among adult males, adolescent females, and adult men. The study also found a 722% increase over baseline for adolescent males.

Additionally, because of the MazART trial, the health service in Eswatini was able to identify areas for further investment, demonstrating an often-unconsidered benefit to early access schemes for patients. This benefit included the health service in the country being able to address the system-side barriers to routine viral load monitoring, also designing and implementing innovative community-based approaches, reaching people living with HIV who were not necessarily routinely accessing HIV testing or counselling services.[4]

Enhancing equity

Early Access Programmes (EAPS) of a number of forms are being adopted by more institutions and companies every day, delivering vital and lifesaving medicines to patients who have no treatment options otherwise available to them. Different early access programmes might aim to combat the spread or severity of a particular illness, such as EAAA, while others offer pathways for ethical, controlled mechanisms of access for investigational drugs prior to commercial launch, not in a trial setting.

With new issues to global health constantly arising, from monkeypox to this month's news about tomato flu, it's possible that we'll see more early access schemes in the years to come than ever before.

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10 steps closer to the patient – The pharma industry post-COVID-19

Emma Banks, CEO, Ramarketing, delves into the connection between patients and the companies in the pharmaceutical supply chain, and shares how this relationship was strengthened by the pandemic

Pfizer, AstraZeneca, Moderna, Johnson and Johnson, the list goes on – we're now as familiar with these brand names as we are with Apple, Coca Cola, and Nike. This has completely changed the playing field for pharmaceutical companies when it comes to growth.

Before the COVID-19 pandemic, the general public was never so acutely aware of which organisations created their medicines or vaccines. National vaccination programmes are not new. The population receives routine vaccines from a few weeks old and, with the flu injection, into their later life. We're used to vaccines. We're used to getting them. And several times over. What we were until very recently not used to, is knowing so much about who is making them, what is in them, where they are manufactured, and how those responsible are making vaccines as safe as possible, and as quickly as possible, to serve a global population.

Because of the unparalleled need to make COVID-19 vaccines and get them into the arms of people as rapidly as humanly possible, people are so much more aware now of the pharma industry – and the pharma supply chain that has been instrumental in achieving this. And, while the patient population might not know too much more about the supply chain, the pharmaceutical supply chain is a lot more aware of their direct impact on their end-user – people.

Before the coronavirus pandemic, although all the companies involved in the pharma supply chain understood the purpose of the medicines or vaccines they were helping develop, that immediate impact on the population was never seen.

COVID-19 generated constant media coverage of vaccines going into arms, demonstrated how being vaccinated unlocked freedom for many populations, and how the injection has directly saved lives. The speed that the entire pharma supply chain worked at to enable the delivery of a global vaccination programme is phenomenal – and has changed the sector forever.

This complete shift to being closer to the patient has been transformational.

Elements of the pharma supply chain that didn't traditionally engage with patients are now doing so and, from a patient point of view, expected to. And it's not just COVID-19 that's changed the game. As technology advances, more pioneering medicines are being made. Medicines that are highly precise and personalised to treat very bespoke conditions that patients suffer from, make the difference between life and death.

Suddenly, people care who the therapy manufacturer is, where it comes from, how medicines or vaccines are stored, and what the side effects are. And this provides an immediate and huge opportunity for pharma companies to proactively market themselves, enabling continued growth.

The pharma industry is built around human life – the quality of life and saving lives. Now it can actively humanise, moving away from the stereotypical pharma perception of a sea of blue medicinal packets stacked high in a storage facility, to the development of a more mainstream, accessible image.

So, how can the sector capitalise on this opportunity?

Reputation – raising their profile

The current landscape enables pharma companies to raise their profile and develop their reputation, by being transparent in how they work and why they deliver the work they do.

We're now in a position, as a sector, to lay bare our personalities, and show people what's behind the business and what drives us. To deliver the COVID-19 vaccines at scale took real passion. For many pharmaceutical firms, the amount of investment and risks taken to get the COVID-19 vaccines to market was astronomical. If something had gone wrong, it would have been the end for many businesses. That's what people don't see, and that's where the opportunity is.

Science is not black and white, regardless of how it is perceived. It's a nebulous pursuit, in that you could go down one path, and find that it's not right, so you have to modify something, and try again. It's time for pharmaceutical companies to embrace being closer to the population it serves, utilise the greater societal contact the industry has, and be open about challenges, successes, and failures.

Pharma companies should highlight specific activities around good governance – or robust sustainability practices and strong ethics, connecting profits to purpose and, in turn, drive a stronger public image.

Scaled capabilities

COVID-19 has shown all organisations, big and small, involved in the pharma supply chain, what they're truly capable of. Never before has a vaccine been developed, tested, approved and rolled out on a global scale in such a short, record-breaking timeframe. From the manufacturers, to the regulators and logistics transportation companies, to those storing the vaccines, and the licensing bodies responsible for ensuring vaccines meet the regulations of each sovereign country, it is truly a phenomenal achievement, and one that creates a huge opportunity moving forwards for growth.

The industry has shown what it can do against the odds. Now is the time to take these capabilities forward, learn from what went well and what didn't, and apply it to other therapeutic processes and developments to drive and scale business growth.

Investment interest

Although we don't generally hear about pharmaceutical companies boasting about the incredible work they've done, or the vital role they have played in monumental milestones in public health, their efforts have not gone unnoticed by investors. The speed to market, the unwavering pursuit of several vaccines which were safe and effective to use in a hugely diverse global population, is extraordinary.

Consequently, this has created interest from investors and, most recently, revitalised mergers and acquisitions activity. This is because pharma companies have shown their worth and, with an unexpected catastrophe like the COVID-19 pandemic, they have demonstrated their resilience, reliability, and innovation to deliver against the grain. The pharma industry has always been high risk from an investment perspective, given its pioneering and experimental nature, but the space has more than illustrated what it's capable of doing which, in turn, has increased investor confidence. Whether this proves to be short lived or not, only time will tell – but the industry has certainly raised awareness, cemented its credibility, and attracted interest.

Overall, the industry is in a position to be brave, truly brave, about its brand.

Pharmaceutical companies now have the opportunity to make their brand more accessible, and more mindful of the patient while streamlining efficiencies, costs, and processes, to enable the next stage of growth.

The future for pharma looks inspiring.

Emma Banks is CEO of ramarketing.

The evolving landscape of RA treatments

Professor Rieke Alten, Head of the Department of Internal Medicine at Schlosspark-Klinik, illuminates the benefits of subcutaneous infliximab in treating rheumatoid arthritis

***Pharmafocus*: What are the benefits of subcutaneous infliximab?**

Professor Rieke Alten: Especially under pandemic conditions, it is necessary that physicians adopt a more tailored approach when it comes to deciding whether a patient should receive intravenous (IV) or subcutaneous (SC) infliximab, according to each individual's condition or disease state. For instance, an IV formulation could be used to induce a rapid response in patients, whilst SC can be utilised to maintain a better drug survival rate.

All patients with immune-mediated diseases have to commit to a treatment for years, often decades. Due to this, patient adherence is a key issue.

Therefore, especially under pandemic conditions, a SC formulation of infliximab offers patients a better quality of life by releasing them from the burden of having to travel to treatment for IV infusions. Self-administration reduces demand on healthcare systems, keeping patients out of clinics and providing clinicians and nurses with additional time.

What challenges do people with rheumatoid arthritis face in their daily lives?

Rheumatoid arthritis (RA) is a chronic, progressive, inflammatory autoimmune disease. It is characterised by painful, tender, and swollen joints that can significantly affect a patient's quality of life, productivity, and participation, due to pain, stiffness, and sleep disturbances.

Morning stiffness is one of the most prevalent problems for people with RA, affecting the majority of those experiencing even low disease activity. For people living with RA, fatigue also remains a major constraint, and unmet need.

People with RA can experience higher rates of depression and anxiety, as well as periods of exacerbation of the disease known as flares.

How does subcutaneous infliximab compare to existing treatments for RA?

In clinical studies, Remsima SC showed clinical advantages in terms of pharmacokinetics compared to the IV formulation, implying that it is a biobetter. A biobetter is defined as a 'modified version of a specific, approved biologic, that enhances clinical outcomes (e.g., improved efficacy) and/or drug pharmacology (e.g., pharmacokinetics and/or pharmacodynamics).

Remsima SC has been shown to have a stable potency, and patients with RA on Remsima SC showed lower rates of ADA, compared with patients receiving Remsima IV. With the availability of both an IV formulation and the novel SC formulation of infliximab, clinicians can choose the optimal treatment for their patients, providing a more personalised and convenient treatment plan.

What are the greatest obstacles in developing treatments for RA?

When developing treatments for RA, it is key to balance safety, efficacy, and convenience for the patient, particularly as life-long treatment is mandatory.

In RA, disparities between high and low GDP countries still play a major role in treatment allocation.

What are your hopes for the future treatment of RA?

Having enough and adequate resources to treat all patients who need an advanced therapy to achieve remission or at least LDA.

Shared decision-making is an integral part of T2T. This should always be an integral part of the art of medicine.

Professor Rieke Alten, Head of the Department of Internal Medicine, Rheumatology, Clinical Immunology and Osteology at Schlosspark-Klinik, Teaching Hospital of Charité, Berlin, Germany

Immunisation or hospitalisation: what RWE tells us about vaccines

Pharmafocus sat down with Joaquin Mould, Global HEOR & Value Strategy Director, Seqirus, to get to the heart of what invaluable insights RWE can give us on vaccine efficacy

***Pharmafocus*: What are the benefits of real-world evidence (RWE) in assessing the economic impacts of vaccines, particularly influenza vaccination?**

Joaquin Mould: RWE allows us to evaluate influenza vaccine effectiveness on a continual basis and provides an ever-growing data set to evaluate real-world outcomes, offering larger, more diverse populations of patients and healthcare settings. In addition, RWE using claims databases allows us to compare direct medical costs with real vaccinated subjects, so we are able to estimate the level of protection from the vaccines as well as measure the economic impact (savings) in a direct and more credible way. The latter, as an example, compares to regular epi or cost-effectiveness models that include a large number of assumptions and high levels of uncertainties. With RWE economic analyses, we can estimate real cost differences by assessing real subjects and their claims. In influenza, with RWE you may not only estimate the proportion of influenza-related hospitalisations or outpatient visits, but also the mean costs for influenza-related hospitalisations or average costs for ambulatory and pharmacy services, per influenza vaccine type.

What is the economic burden of seasonal flu on global healthcare systems?

As the influenza viruses change, so does the burden of disease. In the US, it's estimated that there have been 140,000-710,000 hospitalisations and 12,000-52,000 deaths annually between 2010 and 2020, all of which come with associated healthcare costs and economic burden. Because the circulating strains of influenza virus can change from year to year, annual reformulation and revaccination are necessary to help protect the public against influenza. From a publication in 2018 (Putri et al), the estimated average annual total economic burden of influenza on the healthcare system and society was $11.2 billion. Direct medical costs were estimated to be $3.2 billion and indirect costs to be $8.0 billion. These total costs were based on the estimated average numbers of:

i) Ill, non-medically attended patients (21.6 million)
i) Office-based outpatient visits (3.7 million)
iii) Emergency department visits (0.65 million)
iv) Hospitalisations (247.0 thousand)
v) Deaths (36.3 thousand)
vi) Days of productivity lost (20.1 million).

This study suggests that substantial costs from influenza remain despite the vaccination efforts in the US. The total direct and indirect costs of influenza were equal to $34.8 per capita annually (direct $10.0 and indirect $24.9) with the total costs equal to approximately 0.35% of US per capita health expenditure.

How can RWE and health economics and outcomes research (HEOR) support National Immunisation Technical Advisory Groups (NITAG) around the world?

Economic evaluations in health can be a very useful complement to the decision-making process, so methodological approaches should be continually refined and improved. Caution must be exercised in interpreting the results of economic evaluations performed in a given setting and in extrapolating to a different population, location, healthcare system, and resource use. It's strongly suggested that economic evaluations should be performed on a regular basis to ensure that the results are valid, up-to-date, and consistent with payer's views and priorities of the societies which are under research. RWE, aside from clinical outcomes, may also include economic outcomes (vaccination cost comparisons) which are helpful for NITAG's to decide which populations the vaccines are more cost-effective for, and which provide better value for the money paid.

How can RWE and HEOR inform how data are leveraged for country/region-specific applications?

Data collected from RWE and HEOR can be beneficial in country/region-specific scenarios by allowing us to evaluate vaccine effectiveness and estimate influenza-related costs and benefits. With RWE we may be able to identify future vaccine effectiveness that could be later used as an input within cost-effectiveness models. Alternatively, if it's feasible, you may estimate the mean healthcare costs per vaccinated subject directly through RWE, and compare those costs across the different vaccines within your analysis. The latter approach is stronger due to the assessment's use of real healthcare expenses from real patients and comparing those depending on which vaccine was received. These comparisons can be done across regions (South vs North), to evaluate if there are any with higher costs than others, or for estimating any cost differences across different healthcare providers (e.g., Medicare vs Medicaid), and analysing which provider has higher influenza mean costs.

What is COVID-19 and influenza co-circulation?

Just as COVID-19 mitigation measures helped reduce the spread of the virus and other infectious pathogens like influenza; as many countries continue to decrease such measures, it is expected that COVID-19, influenza, and other respiratory viruses will increase in viral activity simultaneously. We are already observing pre-pandemic level resurgences of influenza in the Southern Hemisphere, which are anticipated to be mirrored in the Northern Hemisphere this coming influenza season.

How can influenza immunisation rates impact overall hospital resources during co-circulation?

Flu vaccination prevents tens of thousands of hospitalisations each year. For example, during 2019-2020, flu vaccination prevented an estimated 105,000 flu-related hospitalisations.

A 2021 study showed that, among adults, influenza vaccination was associated with a 26% lower risk of ICU admission and a 31% lower risk of death from influenza compared to those who were unvaccinated.

A 2018 study showed that, among adults hospitalised with influenza, vaccinated patients were 59% less likely to be admitted to the ICU than those who had not been vaccinated. Among adults in the ICU with influenza, vaccinated patients on average spent 4 fewer days in the hospital than those who were not vaccinated. All these preventions can easily be extrapolated into monetary savings and reflect not only the clinical, but also the economic benefits of influenza vaccines.

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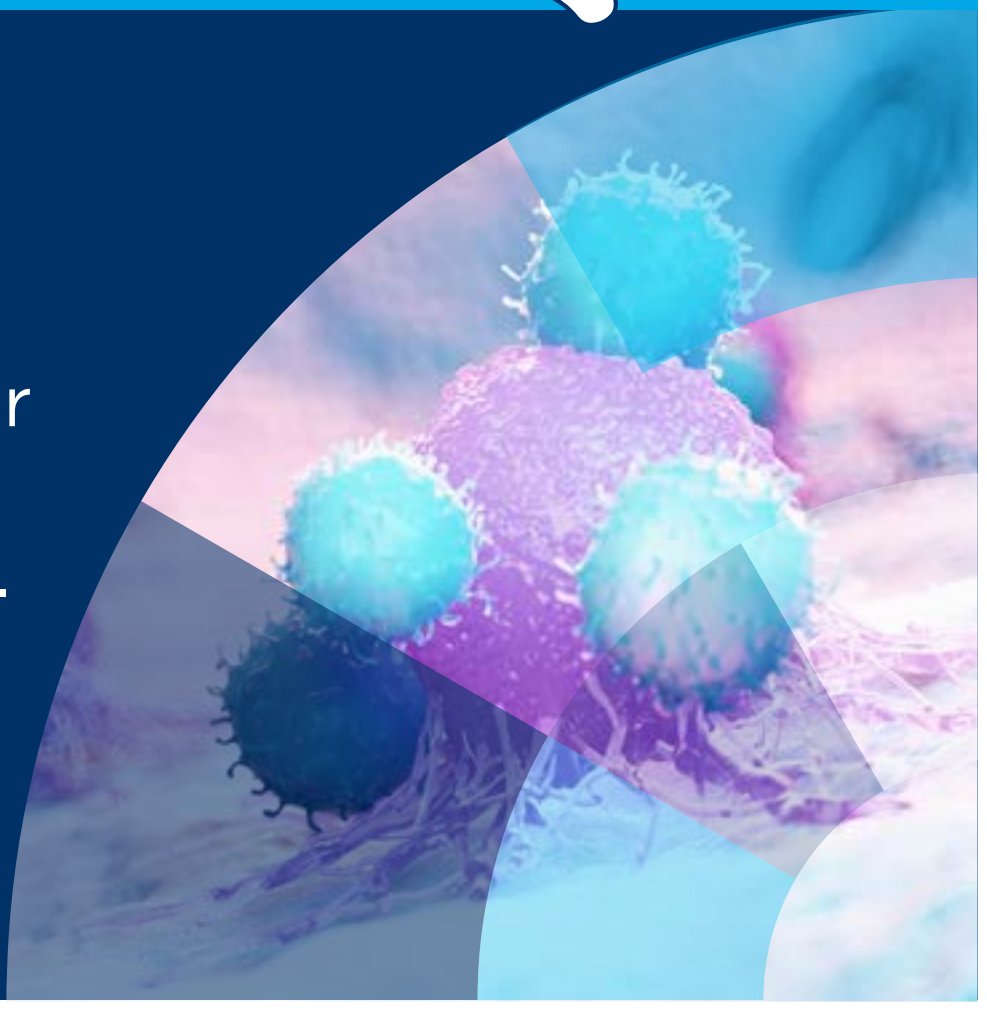

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Move of the month

Janssen promoted Maria Walsh to Business Unit Director

Janssen has announced the appointment of Maria Walsh as Business Unit Director, Oncology & Haemotology, Cell & Gene Therapy and Pulmonary Hypertension for Janssen UK.

Speaking about her new role, Maria said: "I am thrilled to start my new role as Business Unit Director. Over the last two years, I have proudly witnessed first-hand the hard work and dedication shown by the Janssen team as they successfully collaborate to deliver excellent outcomes. I look forward to leading the Oncology, Haematology, Cell & Gene Therapy and Pulmonary Hypertension franchises for Janssen UK, guided by our Credo to continue our longstanding commitment to the patients we serve, and our employees."

Gaëtan Leblay, Managing Director, Janssen UK and Ireland, comments, "Maria is not only an experienced leader in Oncology, she is also a strategic thinker with an empathic and thoughtful management style. This is something she has previously drawn upon to navigate her team through complex and uncertain times with outstanding results. On behalf of the Janssen UK leadership team, I would like to extend a warm welcome to Maria Walsh and wish her every success in her new role".

COMPASS Pathways appoints Kabir Nath as CEO

COMPASS Pathways has announced the appointment of Kabir Nath as CEO as of 1 August. Kabir brings decades of experience in the healthcare industry into his new role.

COMPASS is a mental health care company dedicated to accelerating patient access to evidence-based innovation in mental health. In is role, Kabir will continue building on COMPASS' success and work advancing patient access to evidence-based, technology-enabled innovative care models, combining pharmacological, psychological, and digital solutions.

George Goldsmith, current CEO and Chairman of COMPASS Pathways, said: "We founded COMPASS Pathways to transform mental health care by creating a personalised, predictive and preventative model of care. As we enter the next stage of development, Kabir brings a track record of successful strategic growth, and a deep dedication to this mission.

"Working alongside our talented leadership team, he will leverage his extensive background in leading the commercialisation of innovative therapies to bring COMP360 psilocybin therapy to the large number of patients who are not currently being helped by existing treatments."

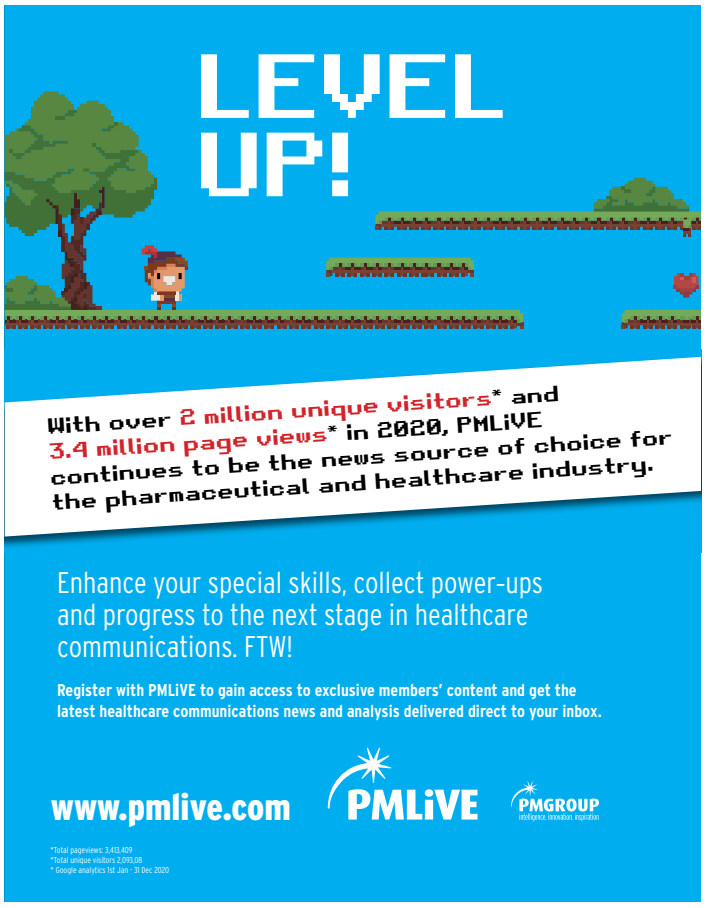
Chris Tower joins Euromed USA as new general manager

Euromed USA, a leading producer of standardised herbal extracts, has announced the appointment of Chris Tower to the position of General Manager. Chris is a highly experienced, senior level executive, with an extensive range of skills and over 25 years of successful sales, business development, and operations expertise in the US.

"We are very happy to have Chris join our team," says Javier Roig, General Manager of Euromed SA. "He is a proven leader with extensive knowledge of our industry on every level, and we look forward to his leadership for years to come."

Guy Woodman, outgoing General Manager of Euromed USA Inc, adds: "We took more than a year to find the ideal candidate to fill this vital role, and I feel totally comfortable handing over the reins to Chris as I know the company will be in very capable hands."

Speaking about his new role, Chris enthuses: "I highly value the level of careful consideration Euromed invested in the selection process, and it is my great pleasure and honour to join this exceptional company which I've admired and respected throughout my career. I could not be more excited, not to mention proud, to join Euromed – an organisation which I know to be deeply rooted in quality and devoted to continuous innovation."



5 examples of personalised medicine

Personalised medicine is a type of medical treatment customised for an individual patient. In this section, we explore how this has been applied across numerous therapeutic areas.

1.) Tumour marker testing can be used to diagnose and treat cancer, as well as to predict how aggressive the cancer is likely to be. Researchers at the University of Bristol have recently found mathematical models that helped them examine and compare new biomarkers and tests for brain tumours as they emerged. This means that an affordable and rapid test to detect a tumour could be on the horizon.

2.) Diabetes management apps have played an important role in self-management for patients in recent years. Individuals can now monitor their blood glucose and insulin levels from their smartphone, and some apps even allow the option to integrate with other devices, such as your insulin pump, or other diabetes management tools.

3.) Virtual therapy platforms, such as Sensa, help individuals in managing their mental health. Sensa is developed with assistance from experts and is heavily based on cognitive behavioural therapy (CBT). The app offers the user 27 questions, centred on their goals for therapy and what they hope to learn, which helps customise the individual's experience.

4.) Genome/DNA sequencing involves sequencing many human genomes to understand how genomic differences relate to different traits. This process helps tailor a person's medications based on their genome, meaning that DNA sequencing plays a pivotal role in identifying what medications work best for each individual. Researchers are continuing to gain a greater understanding of how diseases work, in order to develop new treatments and make new diagnoses.

5.) Health supplements, such as Vitamin D tablets and fish oil pills, are another example of personalised medicine. Vitamin D regulates the absorption of calcium and phosphorus, which encourages healthy immune function. It is also important for maintaining healthy bones, muscle function, and brain activity. It is important to consult with your doctor, if you are unsure about what supplements are best suited to your needs.

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Do you think that the current UK response to the monkeypox pandemic is sufficient?

Yes – 44.4%
No – 55.6%

Do you agree with the decision to pause COVID-19 testing in NHS England and care home settings?

Yes – 0%
No – 100%

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