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MedNous Editorial Board

Editorial

China beckons

Like most advanced economies, China has been supporting the development of biopharmaceutical companies for years in order to supply its citizens with modern medicines. Initially the emphasis was on products modelled after those in the west, namely 'me too' drugs. Lately however, Chinese innovation has shaken up this legacy. At an investor conference in Basel, Switzerland on 9 October Yuwen Liu, founding partner of the BOHE Angel Fund, said that 40% of global pharmaceutical deals now involve a Chinese asset. Looking to the future, she predicted that 35% of assets currently underpinning prospective US Food and Drug Administration submissions will originate from China.

One of the first reports on this new trend appeared in the February 2024 edition of the publication *Signal Transduction and Targeted Therapy*. Here the authors track targeted drug approvals in 2023 in the US and China, and highlight three FDA approvals of innovative drugs developed in China. The first is Loqtorzi (toripalimab), a PD-1 inhibitor for nasopharyngeal carcinoma, developed by Junshi Biosciences. This was the first drug to be approved by the FDA for this indication. The second is Fruzaqla (fruquintinib), developed by Hutchmed for colorectal cancer. This was the first drug to be approved in the US that inhibits three VEGF receptor kinases irrespective of a patient's disease signature. The third is Ryzneuta (efbemalenograstim), developed by Evive Biotech for neutropenia. At the time of approval, this drug was the only granulocyte colony-stimulating factor product authorised in both China and the US.

If anything, the innovation coming out of China has accelerated. The GlobalData consultancy reported on 14 October that in 2024 there was a sudden spike in out-licensing deals from China after the regulator, the National Medical Products Administration, introduced a pilot programme to expedite reviews for innovative therapies. Traditionally, Chinese talent has been concentrated on oncology. Recently, the focus has broadened to include immunology and metabolic disorders.

What are the implications? Panellists at the Basel forum said the surge in corporate activity and the large Chinese market have been a boon to the Hong Kong Stock Market, which has seen an avalanche of IPOs. According to KPMG China, the market had almost 300 active IPO applications in its pipeline at the end of the third quarter – for all industries.

"We are becoming more and more international." Minyue Dong, founder of the Asian Innovation and Finance Center, told the Basel meeting. This is set to continue.

– By Victoria English

Correction: In the article, *Pricing innovative medicine in the UK*, in the September edition of *MedNous*, a sentence describing the UK share of the global pharmaceutical market should have given the estimated size of the global market as 'approximately $1.6 trillion.'

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European Licensing and Collaboration Briefs

	Licensor/Vendor		Licensee/Acquirer	Deal or Acquisition	Comment	Date
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UK	Genomics Ltd	US	Mystra	Launch of AI-enabled genetics drug discovery platform	Enabled by large data collection	Oct-25
US	Akero Therapeutics Inc	DK	Novo Nordisk A/S	Acquisition of company with candidate drug for MASH	Deal value is $4.7b	Oct-25
US	Orbital Therapeutics Inc	US	Bristol Myers Squibb Co	Acquisition of company with *in vivo* CAR T asset	Deal value is $1.5b	Oct-25
US	MaxCyte Inc	US	Moonlight Bio Inc	Rights to technology for T cell therapy development	Undisclosed licencing fees	Oct-25
US	RTP Operating LLC	UK	Oxford Biomedica Plc	Acquisition of cell + gene therapy viral vector manufacturing site	F=$4.5m supported by revenue from site	Oct-25
US	Algen Biotechnologies Inc	UK	AstraZeneca Plc	Discover therapeutic targets in immunology	Potential deal value is $555m	Oct-25
US	Arbor Biotechnologies Inc	IT	Chiesi Farmaceutici SpA	Develop liver targeted therapies for rare diseases	F=up to $115m + M=up to $2b	Oct-25
US	SiVEC Biotechnologies Inc	US	RheumaGen Inc	HLA gene editing therapies including *in vivo* programme	Financial terms not disclosed	Oct-25
US	Gemma Biotherapeutics Inc	US	Rare Therapeutics Inc	Develop gene therapies for ultra-orphan diseases	Treatments to be delivered via public-private partnerships	Oct-25
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AT	Quantro Therapeutics GmbH	DE	Boehringer Ingelheim GmbH	Drugs targeting cancer-associated transcription factors	Deal value of up to €500m	Oct-25
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(F) front-end fee (M) milestone payments (A) research funding (L) licensing fees (R) royalties on net sales (E) equity investment, CEPI=Coalition for Epidemic Preparedness Innovations

Draghi urges faster action on reforms

Europe must start acting less like a confederation and more like a federation, believes former European Central Bank president and Italian Prime Minister Mario Draghi. Mr Draghi, who in 2024 authored a report addressing European competitiveness and the future of the EU, was speaking at a conference entitled *One year after: The Draghi Report – what has been achieved and what has changed,* held in Brussels in September.

The Draghi report identified three significant challenges to Europe's continued competitiveness. Firstly, its growth model had long been under pressure. Secondly, its resilience was threatened by a dependence on external sources of raw materials and technologies. And finally, it was at risk of failing to realise its climate, digital and security ambitions, or to fund its ageing societies, unless faster growth could be achieved. According to Mr Draghi, each of these challenges has intensified over the past year, and Europe's ability to expand world trade and high-value exports has further weakened.

In a keynote address to the *One year after* conference, Commission President Ursula von der Leyen outlined progress to date in meeting the challenges highlighted by Mr Draghi. The global competition for technological leadership is being driven by artificial intelligence (AI), she said, and although the race is not yet won, Europe is a leader in many fields that will define it. Massive investment in high-performance computing power has given Europe some of the best supercomputers in the world.

However, many obstacles remain. President von der Leyen acknowledged that the single market is far from complete, with the International Monetary Fund recently estimating that internal barriers are equivalent to a 45% tariff on goods and a 110% tariff on services. The EU's single market roadmap, published in September, is designed to address these barriers and complete the internal market by 2028. Measures contained in the roadmap include eliminating the 10 most harmful barriers to the single market, dubbed the 'terrible ten,' which include overly complex EU rules and a lack of common standards; providing targeted support for small and medium-sized enterprises and innovative start-ups; and simplifying the adoption of digital tools that can reduce bureaucracy and streamline operations.

One of the cornerstones of proposed action on overburdensome EU regulation is the suggestion to establish a '28th regime.' This would be an optional, harmonised set of EU-wide business laws that companies could adopt as an alternative to navigating individual national laws within the existing 27 member states. The modular approach would focus on the needs of innovative businesses, providing targeted measures to support them. It is hoped this would help make the EU a more attractive place for business compared with the US and Asia.

Mr Draghi responded by saying that he appreciated the decision to place competitiveness at the centre of the EU's response to his report, but warned that there is broad disappointment at how slowly the EU moves compared with other players. What some see as acceptance that the complex processes of the EU are necessarily time-consuming, and that they must respect the positions of many actors, is in reality merely complacency. Competitors in the US and China are far less restrained, he continued, and for Europe to carry on as usual is to resign itself to falling behind.

Welcoming the proposed 28th regime, Mr Draghi cautioned that backing from member states remains uncertain. Early-stage funding also needs stronger support: the Scaleup Europe Fund can help start-ups grow, but its size must match their financial needs. And while the planned budget increase in Horizon Europe is welcome, it will fall short unless additional resources are concentrated into priority programmes for breakthrough research.

Also in the area of regulation, Mr Draghi called for radical simplification of the GDPR (General Data Protection Regulation). This has been estimated to have pushed up the cost of data by about 20% for EU firms compared with their US peers, restricting the availability of the public web data needed for training AI models. Mr Draghi also emphasised the need for the EU AI Act, which sets out harmonised, risk-based rules for AI systems, to be proportionate and to support innovation and development. Furthermore, Mr Draghi suggested that the implementation of the next stage, which covers high-risk AI systems in healthcare and other areas, should be paused until the potential disadvantages are better understood.

On the subject of AI, Mr Draghi said that sectoral AI applications are even more critical than raw supercomputing power. However, Europe has a clear advantage in this area inasmuch as its companies hold more than half of the global market in industrial automation solutions, which are the bedrock of industrial AI. Nevertheless, only about 10% of European firms used AI in 2024. The Commission's forthcoming 'Apply AI' strategy, designed to reduce Europe's reliance on foreign AI providers, will be crucial in encouraging industry and governments to work together.

Other measures

Other measures proposed by Mr Draghi include ensuring that industry support by individual member states encourages the building of European businesses that are globally competitive, rather than locking activity within national borders, as sometimes happens at present; harmonisation of EU procurement rules in order to drive a proportion of public spending to European companies, thus strengthening strategic sectors and driving innovation; and a review of EU competition policy, including an assessment of current merger guidelines.

Mr Draghi emphasised that while the EU could use its existing powers more effectively, deeper reform is necessary in some areas, particularly financing. This process would take time, but if Europe is to function more like a federation it could make a start by concentrating projects, pooling resources, and considering common debt for shared projects.
– By Peter Charlish

Commentary: Jarryl D'Oyley and Catherine Stace

Redefining drug discovery using quantum physics

The world of pharmaceutical research is characterised by high budgets and decade-long timelines. But this may be on the cusp of change. Quantum physics, or the study of matter and energy at the most fundamental level, has the potential to deliver outcomes for drug discovery that are both more efficient and less costly than current methods. The application of this technology across all drug types is potentially profound. In this article, we describe how our company Kuano Ltd of the UK[1] is taking advantage of this opportunity.

In recent years, computer-based methods have made huge progress in drug design and development. Artificial intelligence plays a big role, with tools like AlphaFold and RoseTTAFold, by transforming how we predict protein structures. But despite their sophistication, these methods still rely on simplified models of chemistry and may not fully capture the real physical behaviour of the biological system.

One way to visualise this is to think about classic ball-and-stick diagrams – atoms fixed at points in space where only distance and position seem to matter. Most of today's advances in computer-based drug design, even the most powerful AI-driven tools, are built on this same picture of the world. But if we keep feeding our models the same kind of information, we'll keep running into the same limitations irrespective of how sophisticated the algorithms have become. By moving beyond the rigid classical descriptions and adopting quantum mechanical ones when they matter, we can reveal what was once hidden. Suddenly, what looked like an unsolvable problem becomes an opportunity for discovery.

Quantum-enhanced insight

Consider an enzyme and the reaction it drives. If we want to design the most potent and truly selective inhibitors, we have to go beyond a simple picture of shape and coordinates because many proteins in the same family can look almost identical. Instead, we need to understand the possibilities and probabilities of how that specific reaction unfolds, and how we might influence it. This requires stepping into the realm of quantum physics. This quantum-enhanced insight allows for the prediction of molecular behaviour with unprecedented accuracy, simulating complex chemical interactions before costly laboratory experiments begin, and identifying promising compounds that conventional screening might overlook.

When it comes to understanding covalent ligand reactivity, conventional approaches typically focus only on what happens between a single amino acid and the reactive chemical group, or warhead. In reality, the outcome depends on a system-wide interplay in which the protein, its environment, the warhead, and the ligand all influence each other. To capture this complexity and uncover a solution across the wider system, we need the tools of quantum physics.

Quantum physics throws the classical rule book out of the window. It is an alternative view of the world in which probabilities are considered. Parts of this realm are interconnected by non-local entanglement, enabling us to calculate outcomes that defy conventional expectations. It reveals what is normally invisible, such as transition states, and allows us to predict behaviours that might seem counterintuitive. The local world of classical models becomes more holistic and interactive, turning mysteries into explainable phenomena.

But here is the challenge: when a large number of alternatives in a quantum picture of the world need to be tracked simultaneously, the amount of data explodes and the computational demands quickly become impractical. How can we harness this quantum viewpoint? How can we organise such vast data and make simulations of complex systems truly scalable?

At Kuano we believe that this can be achieved through quantum-computing inspired algorithms that run on existing digital infrastructure; no quantum computer is needed. As quantum hardware matures however, our technology is ready to scale up seamlessly, harnessing even greater computational power.

This platform has been able to deliver accurate, scalable, quantum-level simulations in biologically relevant systems on existing infrastructure. At the same time, it is positioned to leverage quantum hardware when it becomes viable. This is the result of a fusion of quantum computing principles with AI and machine learning, to create what we believe to be something that exceeds the scale, accuracy and resolution of other physics-based approaches, such as Density Functional Theory (DFT), a computational method for predicting the electronic structure of molecules that is becoming increasingly commonplace. However, the next generation of these methods, such as Kuano's technology, blends DFT and other familiar tools with advanced theoretical concepts and cutting-edge techniques. For example, the company has been able to deploy specialised metrics to measure entanglement, and automated orbital selection to focus on aspects where quantum matters. Tensor networks, which are a mathematical framework for understanding complex data, are also used to exploit insights from quantum computing. These add up to providing Kuano with a sophisticated filtering and property optimisation system which integrates with generative AI for compound design. As a result, we have a platform that enables us to look at the biological world differently, unbounded by what went before. This enables us to explore new chemical space where quantum simulations can replace the absolute need for pre-existing experimental datasets.

Unlocking drug programmes

Conventional approaches often falter when faced with targets that are under-researched, structurally complex, or biologically obscure. Fragment screening, docking, and structure-based design typically focus on stable, well-characterised biomedical targets, leaving a vast array of promising targets unexplored. One such target is an enzyme

called Notum which is linked to Alzheimer's disease and colorectal cancer[2]. Despite its therapeutic promise, this enzyme has never reached clinical development. Repeated attempts using traditional methods such as advanced medicinal chemistry and fragment screening techniques, have failed to generate viable leads. As a result, Notum has been largely dismissed by the pharmaceutical industry as too difficult or obscure to pursue. Kuano however saw the enzyme as a way to demonstrate how quantum physics, fused with artificial intelligence, could turn targets that were thought to be undruggable into druggable ones.

The approach was to simulate the transition state, which is the fleeting, high-energy intermediate where reactions occur. This state is the key fingerprint of enzyme function which can only be simulated using quantum. It is the key to unlocking the potency, selectivity, and mechanism of action of a molecule. It also revealed chemical interactions that were invisible to conventional simulations, and enabled the company to identify potent inhibitors and demonstrate early *in vivo* efficacy. This was achieved with fewer than 100 compounds and demonstrated a potential for a new generation of precision therapies, even against the most elusive targets[3]. Another example is DNMT1, a methyltransferase which is implicated in multiple cancers and is known to present significant challenges. DNMT1 shares its natural substrate with its close relatives DNMT3a and DNMT3b, making the design of selective inhibitors difficult. Current drugs circumvent this by avoiding the active site and binding the DNA substrate instead. But this causes unwanted side effects. By contrast, in just two months, we were able to demonstrate selectivity for DNMT1 over DNMT3a/3b with only 40 synthesised compounds. This unveiled a totally new type of chemistry that avoided the nucleoside analogues that had blighted earlier therapies for this target[4].

While comparable projects typically require budgets exceeding £1 million and large teams, the Notum inhibitor progressed with just £350,000 and a lean, highly skilled team, achieving faster timelines than industry standards. This was particularly impressive given Notum's largely uncharted scientific territory. In the case of DNMT1, a longstanding, industry-wide conundrum was solved in a number of weeks. By comparison, the nearest reported alternative took months, if not years, of screening. Where traditional methods struggled, Kuano's platform generated viable inhibitors. It achieved this objective through systemic, data-driven analysis rather than brute-force experimentation. The platform, with quantum simulation at its core, extends from hit identification to lead optimisation. At every step it reveals new insights which are made possible by the quantum physics that underpins our technology. For us, breakthrough therapies emerge not from massive budgets, but from deep scientific insight enhanced by quantum intelligence.

We believe this technology is a new paradigm for drug discovery by integrating computational chemistry, AI, and quantum physics into a mature end-to-end design engine. We start with a quantum simulation, deploy a range of methods from our quantum toolbox, and translate this into putative chemical matter with our AI design engine. This isn't just another piece of modelling software, it is a revolutionary lens that reveals molecules as dynamic, reactive entities operating within a quantum web of forces.

This approach has been deployed in two of the industry's most sought-after advances: understanding the selectivity for phosphatases, and the elucidation of covalent ligand reactivity. Kuano's study of SHP1 and SHP2, two well-known and structurally similar phosphatases, revealed the determinants of selectivity observed for existing ligands. The quantum lens is not limited to the handful of residues in the active site. It was able therefore to reveal a key peripheral residue that, for the first time, explained the observed selectivity. This picture was further enhanced by the technology's ability to quantify both favourable and unfavourable interactions, whereas existing methods would only reveal one side of this picture[5].

For covalent ligands in two well-known use cases, we created 'quantum fingerprints' of known ligands and were able to distinguish between the influence of the different ligand chemistries and environment in describing the observed reactivity. This went beyond the usual 'residue+warhead' perspective. Importantly, Kuano's approach is transferable to residues other than cysteine. This has important implications for the rational design of covalently-reactive ligands in context.

As we expand our capabilities to model covalent reactivity, peptides, allosteric modulators, and protein-protein interfaces, the platform is poised for broad, cross-target scalability. And when quantum computers become viable, we believe that Kuano's quantum-native algorithms will unlock unprecedented accuracy, further pushing the frontiers of medical science.

Conclusion

The success with our initial targets is just the beginning. There is still plenty more work to be done. Today our approach can tackle a huge range of systems, but the largest and most complex may require the eventual arrival of quantum hardware to unlock at scale. Nevertheless, the potential of Kuano's current platform extends far beyond small-molecule inhibitors, enabling enzyme engineering for industrial biotech, biologics design, and mechanistic analyses that clarify structure-activity relationships for clinical compounds. This scalable approach applies across diverse target classes including enzymes, transporters, and receptors and unlocking therapeutic opportunities previously considered out of reach.

References: 1. https://www.kuano.ai. 2. van Driel MS, Linssen JDG, van Neerven SM, et al, (2023) Pleiotropic role of NOTUM in colorectal cancer, *Gut* 72 p2222-2223. 3. Bayle ED, Svensson F, Atkinson BN, Steadman D, Willis NJ, Woodward HL, Whiting P, Vincent JP, Fish PV, (2021) Carboxylesterase Notum Is a Druggable Target to Modulate Wnt Signaling. *J Med Chem*. 64(8) p4289-4311. 4. Zhang Zhixiong, Wang Guan, Li Yuyan, Lei Dongsheng, Xiang Jin, Ouyang Liang, Wang Yanyan, Yang Jinliang. (2022) Recent progress in DNA methyltransferase inhibitors as anticancer agents. *Frontiers in Pharmacology* 13:1072651. 5. Kuano Ltd, 2025 (unpublished data). Available at: https://www.kuano.ai/case-studies.

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Targeting neutrophils in stroke: Marc Dechamps

Stroke research progresses towards new frontier

Stroke has long been an underserved indication in the pharmaceutical industry. Yet research into the impact of neuroinflammation on neurological disorders has made significant progress over the past years. This opens up the prospect of developing new drugs with true blockbuster potential, given the large prevalence of stroke and the urgent unmet medical need.

This article describes research being conducted by our Belgium-based company Bioxodes SA into drugs for thrombotic and inflammatory diseases. The goal is to bring a new therapy for intracerebral haemorrhage (ICH) to the market as early as 2030. This follows promising clinical results for the company's lead asset, BIOX-101, in September.

A study published by *The Lancet Neurology* in 2024 showed that in 2021, neurological conditions were the leading cause of illness and disability worldwide, affecting one in three people[1]. Stroke was the worst offender in the top-10 of neurological conditions contributing to health loss that year, together with Alzheimer's disease, epilepsy, meningitis, and a number of other diseases.

Among this diverse line-up of diseases, stroke has a special place. It is currently the second-leading cause of death for those over 60, and the primary cause of disability. As the population ages, it is projected to become the second leading cause of death and disability overall, with 12 million deaths and 225 million disability-adjusted life years (DALYs) worldwide by 2050.

There are two types of stroke: ischaemic and intracerebral haemorrhage (ICH), which have a different origin, but share common neuroinflammatory symptoms. Triggered by a blood clot in the brain, ischaemic stroke accounts for the vast majority of cases. It is a dangerous disease, which kills 10% of patients within the first 30 days, after an often dramatically sudden onset. And yet the statistics for ICH – caused by a rupture of a blood vessel in the brain – are worse.

While non-traumatic ICH accounts for only approximately 10-15% of all strokes, it is responsible for nearly half of stroke-related deaths. Mortality approaches 50% at 30 days[2] and approximately half of all ICH-related deaths happen within the first 24 hours[3]. Survivors often face severe long-term disability, with fewer than 20% achieving functional independence after six months. The burden on public health systems is large: ICH contributes to 49% of global stroke-related disability-adjusted life years (DALYs)[4]. Such numbers show the long-term impact of this devastating condition. Yet despite its severity, patients with ICH are faced with limited therapeutic options. Furthermore, fragmented systems of care leave them without a clear path to recovery.

In ischaemic stroke, thrombolytic agents such as alteplase are standard of care, but they cannot be used in ICH because of the risk of increased bleeding. Therefore, ICH treatment consists largely of monitoring and stabilising patients, and, in some cases, surgery. The fact that doctors will readily describe the current practice as 'watch and pray,' underscores the need to raise awareness of the gravity of this disease and its epidemiology, catalyse innovation, and prioritise investment in research.

Box 1. BIOX-101 as a treatment for ICH

BIOX-101 is a recombinant version of a small protein found in the saliva of the tick (*Ixodes ricinus*), an external parasite which millions of years of evolution have enabled to overcome the challenges of feeding on the blood of a host without being detected. When feeding, it discharges substances that are both anticoagulative to enable it to feed on blood, and anti-inflammatory to avoid detection. BIOX-101's dual mode of action closely reflects this behavior. It targets the neutrophils – the first responders of the immune system – to avoid neuroinflammation, as well as Factors XIIa and XIa of the intrinsic coagulation pathway to prevent the formation of microthrombosis. Crucially, it displays this mode of action without increasing bleeding, an essential consideration in ICH.

A lack of awareness

A lack of awareness of ICH – among the general public, but unfortunately also in medical practice – aggravates the poor prospects for patients. To start with: ICH is often diagnosed late. Unlike ischaemic stroke, which presents itself with the clear FAST symptoms (Facial drooping, Arm weakness, Speech difficulties, Time to call emergency services), symptoms for ICH are both more variable, as well as less well known. As a result, many patients arrive late at hospitals, often 12 to 24 hours after the onset of symptoms. Such delays are often compounded in hospitals that are insufficiently prepared to deal with stroke, and where access to rapid neuroimaging, specialised care teams and standardised protocols may be limited. In such settings, even the initial diagnosis of ICH can come late, narrowing the already brief window for treatment.

While ischaemic stroke benefits from well-established systems of care such as stroke codes, telestroke networks, and clearly defined pathways for thrombolysis or thrombectomy, ICH lacks those options. The absence of a universally accepted acute treatment strategy for ICH has led to inconsistent management across institutions, reducing the sense of urgency and coordination which is critical not only for clinical outcomes, but also for research participation.

Stroke units play a pivotal role in addressing these gaps. By centralising expertise, ensuring rapid access to imaging and neurologic assessment, and streamlining care processes, stroke units can significantly improve the early recognition and management of ICH. Yet studies have shown that only about 30% of stroke patients are admitted to these specialised units.

Just how great the need for more awareness is became clear during a clinical trial into ICH we ran for our drug candidate BIOX-101. A key observation from the trial performed in stroke units across Belgium was the unexpectedly low

recruitment rate. Investigators noted a stark contrast in the hospital presentation patterns of ICH patients compared with those with ischaemic stroke. When we raised the maximum intake slot in our trial to 24 hours - still in line with the biology of the disease - we were able to complete recruitment without delay. These challenges revealed gaps in our healthcare system that need to be addressed. At the same time, the hope is that the change we made to our protocol will help investigators make future trials a success.

Historically stroke, particularly ICH, has suffered from limited funding and investment, largely due to the complexity of the condition and a perceived lack of therapeutic options. However, this gap highlights a major opportunity. With no approved disease-modifying therapies for ICH, the field is wide open for innovation. The absence of competition, combined with the high burden of disease and the urgent clinical need, positions this area as one of the few remaining frontiers in neurology with genuine blockbuster potential. Strategic investment now could yield significant returns, both in terms of patient outcomes and commercial impact.

The impact of neuroinflammation on neurological disorders, particularly stroke, has been increasingly studied over the past few years. Significant progress has been made in understanding the mechanisms underlying neuroinflammation, identifying novel therapeutic targets, and developing innovative drug delivery systems. These efforts aim to reduce the detrimental effects of inflammation in brain diseases, including ischaemic and haemorrhagic stroke, by addressing challenges such as the blood-brain barrier disruption, and neuronal degeneration. Advances in imaging techniques have allowed researchers to map brain inflammation following a stroke, providing insights into its progression from the subacute to chronic stages.

Despite these promising advances, effective treatments remain limited for neurological disorders. For example, only two approved therapies exist for ischaemic stroke, while there are currently no approved treatments for haemorrhagic stroke. If successful in clinical trials and approved, BIOX-101 would be the first treatment for this disease (see description of the drug in Box 1).

Neutrophils play a complex and dual role in stroke treatment, acting as both early responders to injury and potential contributors to further brain damage. Their prolonged activation can lead to excessive inflammation, releasing reactive oxygen species, cytokines, and neutrophil extracellular traps (NETs) — weblike structures composed of nuclear DNA/histones and granular content released by neutrophils — which exacerbate brain damage and disrupt the blood-brain barrier. To optimise stroke treatment, researchers are investigating neutrophil modulation with drugs like anti-NET therapies, inhibitors of neutrophils, and timing-specific interventions to reduce harmful effects, while preserving the protective role of the neutrophils. This approach aims to improve stroke recovery by minimising inflammation-driven secondary brain damage.

Looking ahead

As life expectancy in the population rises, so will the burden of ICH. Thus, the need to better characterise the pathophysiology of the disease and to develop specific therapies becomes urgent.

In the next five years, stroke care is expected to become more proactive, personalised, and technology-driven. Prevention will be a major focus, with artificial intelligence and wearable devices helping to identify individuals at high risk for stroke by enabling continuous monitoring of physiological parameters. Public awareness campaigns will continue to be essential in teaching people to recognise stroke symptoms early, including those of ICH. Reducing the time it takes to seek and receive medical help will remain critical.

Diagnosis and treatment will become faster and more accurate, thanks to mobile stroke units equipped with CT scanners and telemedicine capabilities, along with AI-powered triage systems in emergency departments. These tools will help distinguish between ischaemic and haemorrhagic stroke more rapidly, ensuring the correct treatment path is followed. Access to advanced interventions like clot-busting drugs and mechanical thrombectomy for ischaemic stroke will expand, while acute care for ICH will benefit from improvements in blood pressure management, surgical techniques, and critical care protocols. Efforts will also focus on early identification of patients with ICH who may benefit from minimally invasive surgery or other targeted therapies.

Holistic post-stroke care will be more integrated, with multidisciplinary teams addressing not only physical recovery but also emotional well-being, cognitive function, and reintegration into daily life. Mental health support, cognitive rehabilitation, and strategies to prevent recurrent strokes, including secondary prevention for those recovering from ICH, will become standard parts of care.

Overall, stroke care in five years will hopefully be smarter, faster, and more equitable, addressing the full spectrum of stroke types. Cooperative research efforts will be essential for addressing global stroke challenges and refining rehabilitation methodologies. The goal will be not only to save more lives but to help all stroke survivors regain their independence and improve their quality of life.

References:

1. Collaborators, G.B.D.N.S.D., *Global, regional, and national burden of disorders affecting the nervous system, 1990-2021: a systematic analysis for the Global Burden of Disease Study 2021*. Lancet Neurol, 2024. **23**(4): p. 344-381.

2. Fogelholm, R., et al., *Long term survival after primary intracerebral haemorrhage: a retrospective population based study*. J Neurol Neurosurg Psychiatry, 2005. 76(11): p. 1534-8.

3. Hemphill, J.C., 3rd, et al., *The ICH score: a simple, reliable grading scale for intracerebral hemorrhage*. Stroke, 2001. 32(4): p. 891-7.

4. Diseases, G.B.D. and C. Injuries, *Global incidence, prevalence, years lived with disability (YLDs), disability-adjusted life-years (DALYs), and healthy life expectancy (HALE) for 371 diseases and injuries in 204 countries and territories and 811 subnational locations, 1990-2021: a systematic analysis for the Global Burden of Disease Study 2021*. Lancet, 2024. 403(10440): p. 2133-2161.

This article was written by Marc Dechamps PhD, Chief Executive Officer of Bioxodes SA.

US & European Regulatory Actions					
Drug or Device	**Comment**	**Sponsor**	**Action**	**Agency**	**Date**
Tecentriq plus Zepzelca	Extensive-stage small cell lung cancer	Roche Group + Jazz Pharmaceuticals	AP	FDA	Oct-25
Libtayo (cemiplimab)	Cutaneous squamous cell carcinoma	Regeneron Pharmaceuticals Inc	NI	FDA	Oct-25
Kidney Klinrisk Algorithm	CE mark for AI-based kidney disease diagnostic	Roche Group	AP	EU	Oct-25
Enflonsia (clesrovimab)	Prevent RSV infection + lower respiratory tract disease	Merck & Co Inc	PO	EMA	Sep-25
Imaavy (nipocalimab)	Generalised myasthenia gravis	Johnson & Johnson Inc	PO	EMA	Sep-25
Kyinsu (insulin icodec / semaglutide)	Insufficiently controlled Type 2 diabetes	Novo Nordisk A/S	PO	EMA	Sep-25
Lynkuet (elinzanetant)	Moderate to severe vasomotor symptoms	Bayer AG	PO	EMA	Sep-25
Acvybra (denosumab)	Biosimilar for osteoporosis and bone loss	Reddy Holding GmbH	PO	EMA	Sep-25
Degevma (denosumab)	Biosimilar for giant cell tumour of bone	Teva Pharmaceutical Ltd	PO	EMA	Sep-25
Bimervax (Covid-19 vaccine)	Immunisation in people 12 years and older	Hipra Human Health SL	NI	EMA	Sep-25
Dupixent (dupilumab)	Treat spontaneous urticaria	Sanofi SA	NI	EMA	Sep-25
Keytruda (pembrolizumab)	Neoadjuvant agent for head and neck cancer	Merck & Co Inc	NI	EMA	Sep-25
Koselugo (selumetinib)	Patients from 3 years with neurofibromas	AstraZeneca Plc	NI	EMA	Sep-25
Tezspire (tezepelumab)	Chronic rhinosinusitis with nasal polyps	AstraZeneca Plc	NI	EMA	Sep-25
Uplizna (inebilizumab)	Immunoglobulin G4-related disease	Amgen Inc	NI	EMA	Sep-25
Inluriyo (imlunestrant)	ER+, HER2-, ESR-1 mutated breast cancer	Eli Lilly & Co	AP	FDA	Sep-25
Rexulti (brexpiprazole) + sertraline	Post-traumatic stress disorder	H Lundbeck A/S + Otsuka	CRL	FDA	Sep-25
Rhapsido (remibrutinib)	Chronic spontaneous urticaria	Novartis	AP	FDA	Sep-25
VCFix spinal system (device)	Vertebral compression fractures	Amber Implants BV	AP	FDA	Sep-25
Pacing leads (implant)	CE mark for symptomatic bradycardia	Boston Scientific Corp	AP	EU	Sep-25
Forzinity (elamipretide)	Treat Barth syndrome	Stealth Biotherapeutics Inc	AP	FDA	Sep-25
Eyeglass lenses (device)	Slow progression of paediatric myopia	Essilor of America Inc	AP	FDA	Sep-25

FDA approves drug for lupus

The US Food and Drug Administration has approved a new treatment for lupus nephritis, an autoimmune disease that affects the kidneys. The approval of Gazyva (obinutuzumab) represents a fifth indication for a drug that has been on the market since 2013 for haematological cancers. The latest approval is based on positive results from Phase 2 and Phase 3 studies, in which Gazyva was combined with a standard of care therapy.

In the REGENCY Phase 3 trial, which enrolled 271 people, 46.4% of the participants who received the combination therapy achieved a complete renal response compared with 33.1% on a standard therapy alone. The standard therapy was mycophenolate mofetil and glucocorticoids. This was accompanied by clinically meaningful improvements in complement levels, which are a measure of proteins in the blood, and reductions in proteinuria.

Lupus nephritis affects more than 1.7 million people globally, with a particular impact on women of colour and of childbearing age. If left untreated, up to one-third of these cases can progress to end-stage kidney disease.

Gazyva is a humanised monoclonal antibody which attaches to the CD20 protein found on certain B cells. According to Roche, data suggests that Gazyva depletes disease-causing B cells, helping to limit damage to the kidneys and potentially preventing or delaying disease progression. The FDA granted a 'breakthrough therapy' designation for the drug in 2019 based on data from the NOBILITY Phase 2 study, which showed that Gazyva, in combination, improved renal responses.

Peter Marks joins Eli Lilly

Peter Marks, a former director at the US Food and Drug Administration, has joined Eli Lilly and Company to oversee molecule discovery and head up work on infectious diseases – the second senior FDA official to cross over to the private sector following the start of the Trump Administration. Dr Marks was previously responsible for the regulation of biologics products, including vaccines, at the FDA's Center for Biologics Evaluation and Research. He left the agency on 5 April after the Administration decided to downsize the Department of Health and Human Services, the FDA's governing body.

This followed the departure, in February, of Patrizia Cavazzoni, former director of the agency's Center for Drug Evaluation and Research, who joined Pfizer Inc as chief medical officer. In this role she will lead the company's regulatory, pharmacovigilance, and evidence generation activities. Pfizer also employs Scott Gottlieb, a former FDA commissioner, who has been a member of the company's board of directors since 2019. Dr Gottlieb is also a partner of New Enterprise Associates, a venture capital financing company.

The *STAT* news agency, which was one of the first to report Dr Marks' new job at Lilly, questioned an executive at the company about apparent conflicts of interest arising from the move. Dan Skovronsky, the company's chief scientific officer, acknowledged the criticism, but said to block transfers from the public to private sectors would limit these officials' potential contributions to new medicines.

Antibody for IgG4 diseases

The European Medicines Agency has issued a positive opinion for a new indication of an antibody therapeutic that targets B cells in order to treat immunoglobulin G4 related diseases. These are autoimmune disorders caused when the body's own defence system attacks normal tissues. This can lead to fibrosis and inflammation in one or multiple organs of the body. The therapy, Uplizna (inebilizumab), has already been approved for the treatment of neuromyelitis optica spectrum disorder, an inflammatory disorder of the central nervous system. If the new indication is approved by the European Commission, the scope of coverage will widen for patients in the EU. The therapy is already approved for both indications in the US.

Announced on 19 September, the positive opinion is based on the results of a Phase 3, randomised, double blind, 52-week trial in 135 adult patients with active IgG4-related disease. Participants received either an intravenous administration of Uplizna or a placebo. The treatments were given on day 1, day 15 and week 26 of the trial. The results showed that the median time from administration of the drug to the first IgG4 related flare was significantly longer in patients receiving Uplizna than the placebo. Furthermore, of the 68 patients receiving Uplizna, only seven were treated for flares compared with 40 of the 67 patients in the placebo group. In addition, 58.8% of participants receiving the therapy achieved corticosteroid-free, flare-free, complete remission at week 52 compared with 22.4% of those on the placebo. The safety profile of the therapy was consistent with its use in patients with neuromyelitis optica spectrum disorder, according to the EMA.

Uplizna is owned by Amgen Inc, which acquired the therapy during its takeover of Horizon Therapeutics Plc in 2023. The original developer was Viela Bio, a spin-out of AstraZeneca's research and development unit, formally known as MedImmune.

Regulatory wins for Brinsupri

Brinsupri (brensocatib), a small molecule drug for non-cystic fibrosis bronchiectasis, a chronic, progressive lung disease, has received a positive opinion from the European Medicines Agency, only two months after getting a formal approval from the US Food and Drug Administration. The EMA opinion still has to be endorsed by the European Commission, a process that can take up to 67 days, but once obtained, patients across two continents are expected to have access to the product. Developed by Insmed Inc of the US, Brinsupri targets neutrophilic inflammation by inhibiting the dipeptidyl peptidase 1 enzyme (DPP1). The enzyme is involved in the activation of neutrophils, a type of white blood cell. If overactivated, neutrophil serine proteases can cause airway wall damage, excessive mucus and sustained inflammation, impairing the functioning of the immune system.

The EMA's positive opinion is based on the results of a double-blind, placebo-controlled trial in 1,767 patients where those on Brinsupri experienced a 19.4% reduction in the annual rate of pulmonary exacerbations and a 14-week delay in the median time to a first pulmonary exacerbation.

Initiation of US & European Clinical Trials					
Name of drug or device	**Sponsor**	**Treatment**	**Phase**	**Comment**	**Date**
Ilofotase alfa (recombinant enzyme)	AM-Pharma BV	Prevent cardiac surgery-associated renal damage	2	Recombinant alkaline phosphatase	Oct-25
DT-101 (small molecule)	Draig Therapeutics Ltd	Major depressive disorder	2	Positive allosteric modulator	Oct-25
SYX-5219 (small molecule)	Sitryx Therapeutics Ltd	Atopic dermatitis	1b	Targets pyruvate kinase M2 which regulates cell metabolism	Oct-25
ADI-001 (allogeneic cell therapy)	Adicet Bio Inc	Refractory rheumatoid arthritis	1	Study therapy across seven autoimmune conditions	Oct-25
TRX319 (allogeneic cell therapy)	Tr1X Inc	Progressive multiple sclerosis	1/2a	Dose escalation in early 2026	Oct-25
Prostate and blood pressure drugs	UK university consortium	Parkinson's disease	1	Test multiple medications in a single trial	Oct-25
O3R-5671 (small molecule)	Onco3R Therapeutics BV	Autoimmune diseases	1	Targets salt-inducible kinase 3 enzyme	Oct-25
Subretinal surabgene lomparvovec (gene therapy)	Regenxbio Inc	Wet age-related macular degeneration	3	Inhibits vascular endothelial growth factor	Oct-25
CMTX-101 (antibody therapy)	Clarametyx Biosciences Plc	Cystic fibrosis-associated pulmonary biofilm infections	2a	Being assessed on top of standard of care therapy	Oct-25
PET radiopharmaceutical	PeptiDream Inc	Prostate cancer	2	Targets prostate-specific membrane antigen	Oct-25
Ivonescimab (bispecific antibody)	Summit Therapeutics Inc	Unresectable metastatic colorectal cancer	3	Primary endpoint is progression free survival	Oct-25
CYT-101 (T cell therapy)	Cyteph Pty Ltd	Recurrent glioblastoma multiforme	1	Targets cytomegalovirus antigens	Oct-25
CTx001 (gene therapy)	Complement Therapeutics GmbH	Geographic atrophy secondary to age-related macular degeneraton	1/2	Trial informed by natural history study	Oct-25
NGN-401 (AAV9 gene therapy)	Neurogene Inc	Rett Syndrome	N/A	Intracerebroventricular delivery	Oct-25
ZI-MA4-1 (allogeneic cell therapy)	Zelluna ASA	Solid tumours	1	T cell receptor-based natural killer cells	Oct-25
Visugromab (monoclonal antibody)	CatalYm GmbH	Non-squamous non-small cell lung cancer	2b	In combination with chemoimmunotherapy	Sep-25
HTL0039732 (small molecule)	Nxera Pharma Co Ltd	Solid cancers in combination with immunotherapies	2a	In collaboration with Cancer Research UK	Sep-25
MB097 (live biotherapeutic product)	Microbiotica Ltd	Advanced melanoma	1b	In combination with pembrolizumab	Sep-25
AB-1005 (gene therapy)	AskBio Inc	Multiple system atrophy-Parkinsonian Type	1	Contains glial cell line-derived neurotrophic factor transgene	Sep-25

Clinical Trials: a round-up of recent advances and setbacks

New Phase 3 data released by **UK**-based **AstraZeneca Plc** on 19 October confirm the promise of the company's antibody-drug conjugate (ADC) for triple negative breast cancer, Datroway (datopotamab deruxtecan). Presented at the European Society for Medical Oncology Congress in Berlin, Germany, the data showed a 43% reduction in patients' risk of disease progression or death compared with chemotherapy, the standard of care for the disease. Like other ADCs, Datroway consists of an antibody, attached via a linker to a toxic payload, which is then delivered to the body. AstraZeneca is developing the therapy as a first-line treatment for metastatic triple negative breast cancer, the most aggressive breast cancer with one of the worst prognoses. Expected median overall survival for the disease is 12 to 18 months. In the Phase 3 trial, called TROPION-Breast02, Datroway not only reduced the risk of disease progression but also showed a five-month improvement in median overall survival compared with chemotherapy. "The TROPION-Breast02 results show for the first time that these triple negative breast cancer patients may have an alternative to chemotherapy in the first-line setting that can both delay the progression of their disease and prolong their lives," Susan Galbraith, head of oncology and haematology research and development, said in a prepared statement. AstraZeneca jointly developed Datroway with **Daiichi Sankyo Co Ltd** of **Japan**. The drug has already been approved in the US for HR positive, HER2 negative breast cancer and EGFR-mutated non-small cell lung cancer.

Abivax SA disclosed data from two Phase 3 induction trials of its candidate drug obefazimod for ulcerative colitis on 6 October which confirmed the treatment's promise as novel therapy for patients with chronic inflammatory diseases. In a presentation at the United European Gastroenterology (UEG) meeting in Berlin, the **French** company said that obefazimod had achieved clinically meaningful improvements in ulcerative colitis across all endpoints regardless of prior inadequate response to advanced therapies. In particular, the drug, at a 50 mg dose, demonstrated clinically meaningful improvements in patients who had failed to respond to a Janus kinase (JAK) inhibitor. JAK inhibitors are a standard of care for the disease. "The outstanding results shared today demonstrate meaningful improvements across the spectrum of patients with ulcerative colitis, ranging from those who were naïve to advanced therapies, to those who have failed up to four lines of prior advanced therapy, including JAK inhibitors," said Silvio Danese, the UEG president elect in a prepared statement. Developed by Abivax, obefazimod is a small molecule drug that upregulates a microRNA in order to stabilise the immune response in patients with ulcerative colitis.

Denmark-based **Genmab A/S** reported more positive data for rinatabart sesutecan, its antibody-drug conjugate for endometrial cancer, on 18 October at the European Society for Medical Oncology meeting. The data showed that treatment with the drug every three weeks resulted in a 50% confirmed objective response rate, including two complete responses, in heavily pretreated patients with advanced cancer. The patients' cancers had progressed despite treatment with platinum-based chemotherapy and an immune checkpoint inhibitor. Rinatabart sesutecan targets the folate receptor-alpha (FR-alpha) cell membrane protein which is overexpressed on multiple tumours, including endometrial cancer. The latest data showed that patients responded to the drug regardless of their FR-alpha expression levels. The US Food and Drug Administration has granted a 'breakthrough therapy' designation to rinatabart sesutecan, based on the early clinical data.

Switzerland-based **Roche Group** has generated new data for vamikibart, an investigational medicine for uveitic macular oedema, showing that the treatment has the potential to improve vision without the side effects generated by steroids, a common treatment for the disease. Vamikibart is a monoclonal antibody that targets interleukin-6 (IL-6), a cytokine involved in uveitic macular oedema, a complication of uveitis. It blocks the interaction of IL-6 with its receptor thereby reducing inflammatory signalling pathways that contribute to retinal leakage and swelling. According to the company, uveitic macular oedema has a disproportionate impact on vision loss and blindness. It is a leading cause of vision loss in people with uveitis, which accounts for 10 to 20% of cases of blindness in the US and Europe. In two Phase 3 studies, vamikibart delivered rapid improvements in vision compared with a sham treatment, alongside a low rate of side effects. Study investigator Eric Suhler from the Casey Eye Institute in the US, said the data suggest that vamikibart could provide a "clinically relevant, locally injectable non-steroid treatment option" for people with uveitic macular oedema.

A Phase 3 trial of Fasenra (benralizumab), an approved asthma medicine, failed to achieve statistical significance in the proposed new indication of chronic obstructive pulmonary disease (COPD), the developer **AstraZeneca Plc** announced on 17 September. The trial was designed to evaluate the efficacy and safety of the drug in patients with a history of worsening COPD episodes and a rise in white blood cells. The efficacy threshold was not reached in the trial but the study did confirm the drug's favourable safety profile. Fasenra is a monoclonal antibody which targets an interleukin-5 receptor subunit. It was first approved by the US Food and Drug Administration in 2017 to treat severe eosinophilic asthma, and subsequently for eosinophilic granulomatosis with polyangiitis, a rare immune disorder involving the respiratory system. The drug is also under regulatory review to treat hypereosinophilic syndrome, which describes organ damage or dysfunction caused by an excess of white blood cells. AstraZeneca developed Fasenra under a licensing agreement with a subsidiary of Kyowa Kirin Co Ltd of Japan.

European Biotech Financings					
	Recipient	Type of Finance	Use of Proceeds	Comment	Date
DE	Tubulis GmbH	€308 mln in Series C financing	Accelerate clinical development of lead ADC	Led by Venrock Healthcare Capital Partners	Oct-25'
FR	Adcytherix SAS	€105 mln Series A financing	Advance development of preclinical ADCs	Co-led by Andera Partners	Oct-25'
IL	Omnix Medical Ltd	$25 mln in Series C funding	Anti-infectives for Gram-negative bacteria	Co-led by Harel Insurance & Finance	Oct-25'
FR	Igyxos SA	€5.7 mln in grant funding	Monoclonal antibody to treat infertility	Bpifrance on behalf of French government	Oct-25'
FR	Step Pharma SAS	€38 mln in Series C financing	Expand dencatistat development into essential thrombocythaemia	V-Bio Ventures is new investor	Oct-25'
UK	Mission Therapeutics Ltd	$13.3 mln in additional venture finance	Develop MTX325 for Parkinson's disease	Investors include Pfizer, Roche venture funds	Oct-25'
UK	National Institue for Health and Care Research	£157 mln in grant funding over five years	Healthcare research and development	University, hospital, public sector partnerships	Oct-25'
CH	ADC Therapeutics SA	$60 mln private share placement	Commercial expansion of ADC drug Zynlonta	Led by investment group TCGX	Oct-25'
US	Kernal Biologics Inc	Up to $48 mln in grant funding	*In vivo* CAR T cell therapies using mRNA platform	Advanced Research Projects Agency for Health	Oct-25'
JP	Celaid Therapeutics Inc	$7.2 mln in Series B financing	*Ex vivo* haematopoietic stem cell therapy for paediatric diseases	Venture and government funding	Oct-25'
UK	Tres Alchemix Ltd	$4.4 mln in Series A financing	Advance AI drug design platform	Led by DNX Ventures	Oct-25'
UK	Trogenix Ltd	£70 mln in Series A financing	Advance drug for glioblastoma into the clinic	Led by IQ Capital	Oct-25
UK	Athernal Bio Ltd	£3.5 mln in start-up funding	Treat blood cancer precursor conditions	Launch funding from Delin Ventures	Oct-25'
UK	Owlstone Medical Ltd	Up to $49.1 mln in grant funding	Develop early detection tests for multiple cancers	Advanced Research Projects Agency for Health	Oct-25'
FR	SeaBeLife SAS	€2 mln pre-Series A funding round	Dry AMD and severe acute hepatitis programmes	Led by investor iXLife	Oct-25'
UK	Biotechs developing antibacterials	£6 mln for up to eight projects	Candidate therapies to treat antimicrobial resistance	Pathways to Antimicrobial Clinical Efficacy	Oct-25'
UK	Forge Genetics Ltd	£2 mln in post-launch funding	Develop new gene editing tool	Funding manager is Mercia Ventures	Oct-25'
US	Cartography Biosciences Inc	$67 mln Series B financing	T cell engaging bispecific and multispecific antibodies	Led by Pfizer Ventures	Oct-25'
FR	Sonomind SAS	€3 mln in seed and grant funding	Neuromodulation ultrasound device	Seed funds from Critical Path Ventures	Sep-25'
UK	Enhanced Genomics Ltd	Total $19 mln after Series A extension	Genetically validated targets for common diseases	Investors include BGF, Parkwalk	Sep-25'
FR	RDS SAS	€14 mln Series A funding round	Patch for remote patient monitoring	Led by SPI fund	Sep-25

FR	Genoskin SAS	$8.7 mln in Series A funding	Non-animal models for testing new drugs	Led by FPCI Occidev Impacts	Sep-25
NL	Cantoni Therapeutics BV	Financing of undisclosed size	Advance non-incretin obesity drug	Brabant Development Agency	Sep-25'
FR	Sanofi Ventures	$625 mln in additional capital	Accelerate investment in biotech + digital health	Sanofi SA	Sep-25'
CH	Basilea Pharmaceutica Ltd	$25 mln in grant funding	Advance development of antifungal medicines	BARDA of the US	Sep-25'
CH	Basilea Pharmaceutica Ltd	Non-dilutive funding of up to $159 mln	Develop novel Phase 3 ready antibiotic	BARDA of the US	Sep-25'
CH	Santhera Pharmaceuticals AG	CHF 20 mln from existing investors	Rollout Duchenne muscular dystrophy drug	Investors Highbridge + R-Bridge	Sep-25
US	Sparrow Pharmaceuticals Inc	$95 mln Series B financing round	New treatment for Type 2 diabetes	Co-led by Forbion	Sep-25

Source: Company announcements

Omnix secures funding

Omnix Medical Ltd, based in Israel, has raised $25 million from a Series C funding round to accelerate development of a pipeline of antimicrobials directed against multidrug-resistant infections. Announced on 15 October, the financing was co-led by Harel Insurance & Finance Group, one of Israel's largest financial institutions, and the European Innovation Council Fund, a venture initiative of the European Commission under the Horizon Europe research programme.

The financing will enable Omnix to complete Phase 2 proof-of-concept studies for its lead compound, OMN6, which is a bioengineered antimicrobial peptide for the treatment of life-threatening infections caused by Gram-negative bacteria, particularly *Acinetobacter baumannii*. This bacteria can cause infections in the blood, urinary tract, lungs or wounds. The funding will also support regulatory activities, manufacturing scale-up, and the advancement of Omnix's broader pipeline of engineered peptides. Following the latest round, the company will have raised a total of $43 million since its inception in 2015.

OMN6's mechanism of action is based on the destruction of bacterial cell membranes. This means that it is fast-acting and bactericidal. It is also effective regardless of the bacterial genotype or resistance phenotype. Based on trial results thus far, the US Food and Drug Administration has granted the compound both Fast Track and Qualified Infectious Disease Product designations.

In a statement, Niv Bachnoff, the company's chief scientific officer, said the funding will demonstrate OMN6's potential "to transform the treatment of multidrug-resistant infections."

Omnix Medical was founded in 2015 at the VLX Ventures incubator in Israel to address the threat of drug-resistant bacterial infections.

Tubulis raises €308 million

Tubulis GmbH, a German company with a portfolio of antibody-drug conjugates, has raised €308 million in a Series C financing round in what is being described as the largest C round for a European biotechnology company and the biggest financing globally for a private ADC developer. Announced on 15 October, the round was led by Venrock Healthcare Capital Partners with participation from additional new investors Wellington Management and Ascenta Capital. Proceeds from the round will be used to accelerate development of the company's lead product, TUB-040, which entered the clinic 16 months ago. TUB-040 is currently being investigated in patients with ovarian cancer and lung adenocarcinoma. The goal is to expand its reach into earlier lines of therapy and additional tumour indications.

"The new funding empowers us to execute on our vision of creating truly differentiated antibody-drug conjugates that are tailored to the biology of solid tumours and can deliver superior therapeutic value to patients," Dominik Schumacher, the company's chief executive and co-founder, said in a prepared statement.

The ADC construct has taken decades to develop. But intensive research on components of the product, such as the chemical compound that links the delivery vehicle, an antibody, to the toxic payload, has yielded results with an increasing number of companies entering the field. Tubulis says that its ADCs have "superior biophysical properties" relative to those of other companies. This was on the basis of preclinical studies. The company now has clinical data, which was disclosed on 19 October at the European Society for Medical Oncology meeting in Berlin. These showed that TUB-040 achieved an overall response rate of 59% and a disease control rate of 96%, with responses occurring early and deepening over time.

Q3 sees unexpected recovery in stocks

2025's third quarter saw an unexpected but certainly welcome recovery in stock prices for the majority of Europe's biotech companies, mirroring - as is usually the case - the performance of their US listed counterparts. This is already being hailed as marking the start of a recovery in biotech, which has felt under relentless financial pressure since 2022.

The closely watched XBI index, which follows the US small/mid cap space and is considered a bell weather of wider investor sentiment, notched up some solid gains. This was 3% in July, 5% in August, and 12% in September. It was attributed to a run of positive trial data readouts, a sudden resurgence of big pharma buyouts and, on the macro-economic side, the prospect of interest rate cuts. These share price gains have continued into October in spite of the US administration's tariff threats and potential US drug price reductions under most-favoured-nation deals.

In terms of the bigger European companies, three of the four largest – those with market capitalisations of $10 billion or more, namely argenx, Genmab and Ascendis - recorded solid stock price gains, with only BioNTech seeing a decline in the quarter. Thus, these relatively large players will have driven a very positive rise for Europe's biotech sector generally. But the stand-out performance was France-based Abivax, which reported highly positive data from a Phase 3 trial of obefazimod in ulcerative colitis in mid-July. This propelled its share price up more than ten-fold this year. Abivax's skyrocketing share price has allowed it to raise almost $750 million in a secondary financing – hitherto an almost unknown sum for a European biotech - from its now largely US shareholder base.

But Abivax was also not the only European biotech to show a strong performance in the quarter. uniQure reported encouraging results from a Phase 1/2 trial of its Huntington's disease gene therapy AMT-130 in September, which led to its share price rising 360%. As a result, it has been able to refinance debt and raise new equity. Many other companies have seen gains in the quarter, with other notable ones, including Nanobiotix, which develops physics-based nanotherapies, rising more than three-fold in the past month.

However, there were still some notable declines. One of the more closely followed was MoonLake Immunotherapeutics which reported disappointing results for sonelokimab in hidradenitis suppurativa at Phase 3.

One of the key drivers of the recent biotech stock performance has been buyouts by big pharma and some larger biotechs. At the end of September, Genmab announced a deal to acquire the Dutch biotech Merus for $8 billion, giving it rights to petosemtamab, a promising EGFR×LGR5 bispecific antibody that is in two Phase 3 trials in head and neck cancer. This deal should also promote Genmab back up to second position in the European market capitalisation space.

It is widely assumed the stock price gains and – perhaps increasing buyout activity – will eventually filter down to the smaller companies in Europe's biotech space. Although this has yet to be seen, it should make it easier for companies to go public and/or raise equity finance on more attractive terms, something that is desperately needed for a more vibrant industry. – *By Robin Davison*

EUROPEAN SPECIALTY PHARMACEUTICAL COMPANIES

Stock market value in Euros at FX rates on 30-Sep-25

		30-Sep-24	31-Dec-24	31-Mar-25	30-Jun-25	30-Sep-25	Annual Change	Quarter Change
BE	Argenx SE	29,440,000,000	35,880,000,000	32,847,000,000	29,621,000,000	40,000,000,000	**35.9%**	35.0%
DE	Evotec SE	1,102,000,000	1,515,000,000	1,076,000,000	1,289,000,000	1,110,000,000	**0.7%**	-13.9%
BE	Galapagos NV	1,691,000,000	1,748,000,000	1,532,000,000	1,582,000,000	1,960,000,000	**15.9%**	23.9%
DK	Genmab A/S	13,790,000,000	12,710,000,000	17,958,000,000	10,850,000,000	16,560,000,000	**20.1%**	52.6%
FR	Ipsen SA	9,205,000,000	9,156,000,000	9,010,000,000	8,709,000,000	9,880,000,000	**7.3%**	13.4%
DK	Lundbeck A/S	5,590,000,000	5,280,000,000	4,730,000,000	4,730,000,000	5,800,000,000	**3.8%**	22.6%
DK	Novo Nordisk A/S	470,000,000,000	371,677,000,000	220,000,000,000	265,336,000,000	160,000,000,000	**-66.0%**	-39.7%
DK	Novonesis A/S	26,790,000,000	25,380,000,000	22,400,000,000	28,330,000,000	21,610,000,000	**-19.3%**	-23.7%
FI	Orion Corp	6,900,000,000	6,005,000,000	7,720,000,000	8,916,000,000	9,320,000,000	**35.1%**	4.5%
SE	Swedish Orphan Biovitrum	9,690,000,000	9,500,000,000	9,330,000,000	8,790,000,000	9,600,000,000	**-0.9%**	9.2%
BE	UCB SA	32,240,000,000	36,550,000,000	31,970,000,000	31,709,000,000	47,870,000,000	**48.5%**	51.0%
DK	Zealand Pharma A/S	7,740,000,000	6,780,000,000	13,710,000,000	3,469,400,000	4,410,000,000	**-43.0%**	27.1%
	TOTALS	**614,178,000,000**	**522,181,000,000**	**372,283,000,000**	**403,331,400,000**	**328,120,000,000**		
	Quarterly change		**-15.0%**	**-28.7%**	**8.3%**	**-18.6%**		
	Cumulative change		**-15.0%**	**-39.4%**	**-34.3%**	**-46.6%**		

Market Capitalisation of European Biotech Companies

		Stock market value in Euros at FX rates on 30-Sep-25						
		30-Sep-24	31-Dec-24	31-Mar-25	30-Jun-25	30-Sep-25	12-mth	Quarter
FR	Abivax SA	634,000,000	450,600,000	364,660,000	418,800,000	5,430,000,000	756.47%	1196.6%
CH	Addex Therapeutics Ltd	9,280,000	7,810,000	11,010,000	12,230,000	12,090,000	30.28%	-1.1%
SE	Alligator Bioscience AB	89,000,000	16,490,000	29,150,000	20,020,000	14,570,000	-83.63%	-27.2%
UK	Allergy Therapeutics Plc	263,180,000	373,690,000	353,080,000	440,230,000	464,960,000	76.67%	5.6%
UK	Angle Plc	31,400,000	39,870,000	36,700,000	27,000,000	9,960,000	-68.28%	-63.1%
UK	Aptamer Group Plc	5,760,000	8,870,000	5,970,000	8,280,000	22,370,000	288.37%	170.2%
NO	ArcticZymes AS	74,400,000	57,770,000	74,540,000	71,980,000	120,000,000	61.29%	66.7%
UK	Avacta Group Plc	195,670,000	226,800,000	159,250,000	138,460,000	288,230,000	47.30%	108.2%
CH	Basilea Pharmaceutica Ltd	590,190,000	533,210,000	630,800,000	672,010,000	678,830,000	15.02%	1.0%
DK	Bavarian Nordic A/S	2,350,000,000	1,990,000,000	1,680,000,000	1,790,000,000	2,420,000,000	2.98%	35.2%
NO	BerGenBio ASA***	34,180,000	27,120,000	5,640,000	4,620,000	4,020,000	-88.24%	-13.0%
SE	Bioinvent International AB	260,000,000	220,000,000	180,000,000	207,300,000	170,000,000	-34.62%	-18.0%
DE	Biofrontera AG	14,433,000	13,065,000	14,610,000	15,366,000	17,020,000	17.92%	10.8%
DE	Biotest AG	1,359,000,000	1,347,000,000	1,460,000,000	1,397,000,000	1,397,000,000	2.80%	0.0%
FR	Cellectis SA	193,982,000	163,153,000	86,300,000	96,570,000	186,600,000	-3.81%	93.2%
IE	Cosmo Pharmaceuticals NV	1,280,000,000	1,090,000,000	1,070,000,000	1,072,380,000	1,210,000,000	-5.47%	12.8%
FR	Eurobio Scientific SA	256,576,000	253,177,000	250,580,000	253,369,000	254,680,000	-0.74%	0.5%
FI	Faron Pharmaceuticals Oy	282,520,000	265,748,000	279,010,000	283,031,000	224,750,000	-20.45%	-20.6%
UK	ImmuPharma Plc	9,100,000	6,180,000	14,720,000	10,670,000	76,440,000	740.00%	616.4%
FR	Innate Pharma SA	161,902,000	159,974,000	152,060,000	144,522,000	159,480,000	-1.50%	10.3%
SE	Medivir AB	28,760,000	27,760,000	13,040,000	15,970,000	15,380,000	-46.52%	-3.7%
SE	Mendus AB	35,500,000	34,230,000	28,020,000	35,170,000	32,710,000	-7.86%	-7.0%
IT	Newron Pharmaceuticals SpA	160,940,000	183,110,000	168,960,000	142,906,000	230,800,000	43.41%	61.5%
FR	Nicox SA	18,690,000	18,183,000	17,610,000	16,202,000	30,460,000	62.97%	88.0%
UK	Niox Group Plc	345,050,000	323,250,000	354,700,000	327,680,000	348,120,000	0.89%	6.2%
BE	Nyxoah SA	283,921,000	321,040,000	243,280,000	241,416,000	169,170,000	-40.42%	-29.9%
SE	Orexo AB	42,190,000	52,620,000	45,960,000	62,720,000	100,000,000	137.02%	59.4%
UK	Oxford BioDynamics Plc	11,040,000	5,730,000	10,820,000	7,720,000	10,420,000	-5.62%	35.0%
UK	OBX Plc (Oxford Biomedica)	479,220,000	539,790,000	372,570,000	390,090,000	809,620,000	68.95%	107.5%
UK	Oxford Nanopore Technologies Plc	1,800,000,000	1,500,000,000	1,149,170,000	1,540,000,000	1,700,000,000	-5.56%	10.4%
ES	Oryzon Genomics SA	117,430,000	89,032,000	206,428,000	207,735,000	246,850,000	110.21%	18.8%
NL	Pharming Group NV	492,485,000	657,308,000	533,670,000	623,580,000	891,550,000	81.03%	43.0%
CH	Santhera Pharmaceuticals Holding	118,800,000	177,990,000	202,040,000	163,610,000	154,610,000	30.14%	-5.5%
UK	Scancell Holdings Plc	150,640,000	134,410,000	100,570,000	113,700,000	115,160,000	-23.55%	1.3%
UK	Shield Therapeutics Plc	41,300,000	25,950,000	35,520,000	34,270,000	93,720,000	126.92%	173.5%
FR	Transgene SA	150,815,000	92,734,000	87,969,000	112,533,000	149,990,000	-0.55%	33.3%
FR	Valerio Therapeutics SA* **	14,368,000	11,548,000	10,008,000	3,605,000	19,720,000	37.25%	447.0%
DE	Vita 34 International AG	79,140,000	71,819,000	215,283,000	74,440,000	104,080,000	31.51%	39.8%
NO	Zelluna ASA	N/A	N/A	31,750,000	23,750,000	20,830,000	-	-12.3%
		30-Sep-24	31-Dec-24	31-Mar-24	30-Jun-25	30-Sep-25		
	Totals	12,464,862,000	11,517,031,000	10,685,448,000	11,220,935,000	18,404,190,000		
	Quarter-to-quarter		-7.6%	-7.2%	5.0%	64.0%		
	Cumulative		-7.6%	-14.3%	-10.0%	47.6%		

Sources: Yahoo Finance, company websites, *formerly Circassia Group Plc, ** formerly Onxeo SA, ***to merge with Oncoinvent ASA

European biotechs listed on NASDAQ (Quarterly change in market capitalisation in $m)								
Ticker	Company name		Market Cap 30-Jun-25	Price 30-June-25	Market Cap 30-Sept-25	Price 30-Sept-25	Quarterly change in market cap ($)	Quarterly change in market cap (%)
ABVX	Abivax SA	FR	$485	$7.65	$6,409	$84.90	$5,923.21	1220.2%
ACIU	AC Immune SA	CH	$201	$2.03	$301	$2.89	$99.93	49.8%
ADAP	Adaptimmune Therapeutics	UK	$62	$0.24	$37	$0.13	($25.28)	-40.9%
ADCT	ADC Therapeutics Ltd	CH	$266	$2.68	$456	$4.00	$190.39	71.6%
AFMD	Affimed NV	NL	$1	$0.08	$0	$0.00	($1.14)	-98.7%
AMRN	Amarin Corporation plc	IE	$336	$16.22	$335	$16.38	($1.03)	-0.3%
ARGX	Argenx SE	NL	$33,657	$551.22	$44,401	$737.56	$10,743.26	31.9%
ASND	Ascendis Pharma A/S	DK	$10,420	$172.60	$12,041	$198.81	$1,620.17	15.5%
ATAI	ATAI Life Sciences NV	DE	$440	$2.19	$1,092	$5.29	$652.40	148.4%
AUTL	Autolus Therapeutics plc	UK	$607	$2.28	$434	$1.63	($172.54)	-28.4%
BCYC	Bicycle Therapeutics	UK	$481	$6.95	$378	$7.74	($102.95)	-21.4%
BNTX	BioNTech SE	DE	$25,595	$106.47	$23,461	$98.62	($2,134.09)	-8.3%
CLLS	Cellectis SA	FR	$110	$1.53	$201	$2.85	$91.20	83.0%
CMPS	COMPASS Pathways Plc	UK	$260	$2.80	$536	$5.73	$276.29	106.3%
CRSP	CRISPR Therapeutics AG	CH	$4,201	$48.64	$5,852	$64.81	$1,651.02	39.3%
DBVT	DBV Technologies SA	FR	$250	$9.16	$268	$10.00	$17.59	7.0%
EVAX	Evaxion Biotech A/S	DK	$3	$2.45	$25	$3.76	$21.07	613.2%
EVO	Evotec SE	DE	$1,492	$4.20	$1,286	$3.63	($205.98)	-13.8%
GLPG	Galapagos NV	BE	$1,844	$27.99	$2,263	$34.64	$418.45	22.7%
GLTO	Galecto Inc	DK	$5	$3.41	$5	$3.72	$0.35	7.8%
GNFT	Genfit SA	FR	$185	$3.73	$189	$3.80	$3.93	2.1%
GNTA	Genenta Science SpA	IT	$56	$3.05	$58	$3.18	$2.55	4.6%
GMAB	Genmab A/S	DK	$12,707	$20.66	$18,734	$30.67	$6,026.76	47.4%
GHRS	GH Research Plc	IE	$756	$12.19	$873	$14.30	$117.23	15.5%
IMTX	Immatics NV	DE	$654	$5.38	$1,005	$8.52	$350.67	53.6%
IOBT	IO Biotech ApS	DK	$92	$1.39	$24	$0.36	($67.65)	-73.9%
IPHA	Innate Pharma	FR	$167	$1.82	$178	$1.93	$10.61	6.3%
IVA	Inventiva SA	FR	$444	$3.19	$814	$5.79	$370.41	83.5%
ITRM	Iterum Therapeutics Plc	IE	$40	$1.00	$30	$0.67	($9.51)	-23.9%
JAZZ	Jazz Pharmaceuticals Plc	IE	$6,421	$106.12	$7,796	$131.80	$1,374.44	21.4%
LVTX	LAVA Therapeutics NV	NL	$35	$1.32	$40	$1.56	$5.52	15.9%
MLTX	MoonLake Immunotherapeutics AG	CH	$2,996	$47.20	$391	$7.17	($2,605.45)	-87.0%
MREO	Mereo BioPharma Group plc	UK	$431	$2.71	$314	$2.06	($116.87)	-27.1%
MRUS	Merus BV	NL	$3,917	$52.60	$7,077	$94.15	$3,160.01	80.7%
MOLN	Molecular Partners AG	CH	$141	$3.82	$138	$3.71	($3.26)	-2.3%
NBTX	Nanobiotix SA	FR	$223	$4.73	$958	$18.89	$734.87	329.8%
NCNA	NuCana Plc	UK	$22	$0.06	$7	$4.64	($14.59)	-66.6%
PHVS	Pharvaris NV	NL	$959	$17.60	$1,571	$24.95	$611.56	63.8%
PRQR	ProQR Therapeutics NV	NL	$215	$2.04	$221	$2.13	$6.31	2.9%
PRTA	Prothena Corporation plc	IE	$327	$6.07	$526	$9.76	$199.19	61.0%
TLSA	Tiziana Life Sciences Plc	UK	$176	$1.58	$236	$2.16	$60.19	34.2%
QURE	Uniqure NV	NL	$763	$13.94	$3,541	$58.37	$2,777.32	363.9%
VALN	Valneva SE	FR	$482	$5.67	$996	$12.20	$513.91	106.6%
VRNA	Verona Pharma Plc	UK	$8,052	$94.58	$9,169	$106.71	$1,117.04	13.9%

Sources: Nasdaq and company statements. Market capitalisation figures are in millions of US dollars

Commentary: Femida Gwadry-Sridhar

Natural history studies underpin clinical research

Historians writing about the origin of clinical research often cite studies conducted in the UK as setting some of the fundamental standards in this field. The Medical Research Council conducted a trial of patulin for the common cold in 1943 which was the first double-blind controlled trial. Three years later it carried out a study to evaluate streptomycin in pulmonary tuberculosis – the first randomised controlled trial. Decades later, with more research taking place to treat rare diseases, clinical trials have evolved further to take into account patient data outside the clinical setting such as data from patient registries and health insurance claims.

This information has become known as real world data – or data from sources other than a clinical trial. The analysis of this raw data is called real world evidence. In addition, natural history studies, which collect information about the natural history of a disease, are part of the modern arsenal. These in turn have led to the creation of external control arms where data in certain populations is collected to supplement an existing randomly allocated control arm of a trial.

The last decade has seen a considerable shift in the acceptability of real word data by both the US Food and Drug Administration and the European Medicines Agency. Both agencies have issued guidance clarifying when this data may be used when a traditional randomised control trial may not be feasible. In 2019, Pfizer Inc used real world data to gain FDA approval of a new indication for Ibrance (palbociclib), a breast cancer drug. The indication was for use of the drug in men, a small patient population unable to support a traditional trial.

Other drugs that have been evaluated using real world data include Elevidys (delandistrogene moxeparvovec) for Duchenne muscular dystrophy, approved by the FDA in 2023, and Zolgensma (onasemnogene abeparvovec), approved for spinal muscular atrophy in 2019. Of particular interest is the use of real world data for external control arms where data in certain populations may not be available for randomisation. Here, the design of a natural history study can be important.

Natural history studies and external control arms are becoming more relevant as regulatory agencies, companies and payers seek faster, more cost effective ways to evaluate new therapies without compromising scientific rigour. The quality of the underlying natural history data often determines whether an external control arm can withstand regulatory and payer scrutiny. To achieve quality, some biotech investors are even insisting that a natural history study is initiated prior to Phase 1.

Patient deaths and serious adverse events that led to clinical holds and drug failures in 2024 and 2025 have generated support for the use of real world data in the context of natural history studies. These studies have become the scaffolding on which external control arms stand. When executed well, these arms align with the inclusion criteria, endpoints, and follow-up cadence of an interventional trial, while capturing the full arc of disease progression in the absence of treatment.

Rare diseases, oncology, and other high-unmet-need areas are leading this shift. Testing drugs for these diseases in traditional randomised trials is often neither feasible nor ethical. Instead, robust natural history studies using external control arms can achieve scientifically credible, clinically meaningful comparisons acceptable to regulators.

Patients with severe and rapidly progressive rare diseases, such as those with a lysosomal storage disease (LSD), often oppose placebo-controlled trials because receiving no active treatment can mean irreversible decline or death within a short period of time. In these small populations, retention in placebo arms is also a major challenge where even a few patient dropouts can drastically undermine statistical power and risk trial failure. As the UK advocacy group for LSD called the Cure & Action for Tay-Sachs Foundation has regularly stated, "there is nothing worse than a trial failing, not because the drug didn't work, but because the trial design was so bad." This highlights the need to use real world data or natural history cohorts as comparator arms to address these ethical and practical challenges, enabling every participant to access treatment while still providing credible, comparative evidence of efficacy.

Natural history studies, when designed prospectively and to regulatory standards, capture the patient journey before any therapeutic intervention. They document disease trajectory, progression variability, and presentation heterogeneity. Without this foundation, an external control arm risks being little more than retrospective data stitched together, risking inconsistent measures, missing outcomes and unmeasured confounders.

Recently, regulators have clarified what they expect. The FDA's draft guidance on externally controlled trials underscores several minimum requirements, including patient-level data, pre-specified analytic plans, clearly defined eligibility and outcome criteria, bias mitigation strategies, and contractual data access provisions[1]. More recently, on 25 July, the EMA issued a scientific guideline on the use of evidence generation in regulatory decision-making[2].

Retrospective analyses show that about 34 FDA and 41 EMA applications relied on external control arms between 2005 and 2017 of which 98% received an FDA approval and 79% an EMA positive opinion. In the period from 2016 to 2021 external control arms were used in 20% of oncology approvals in the EU and 82% of approvals for drugs for rare diseases.

References: 1. FDA, *Considerations for the Design and Conduct of Externally Controlled Trials for Drug and Biological Products*, February 2023. 2. EMA, *Development of a reflection paper on the use of external controls for evidence generation in regulatory decision-making* – Scientific guideline, 25 July 2025.

This article was written by Femida Gwadry-Sridhar, PhD, CEO and Founder of Pulse Infoframe Inc.

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Nobel for immune discovery

The 2025 Nobel Prize in Physiology or Medicine has been awarded jointly to Mary E. Brunkow, Fred Ramsdell, and Shimon Sakaguchi for their discoveries concerning peripheral immune tolerance, a breakthrough that has transformed understanding of how the immune system avoids attacking the body's own tissues.

Their pioneering research uncovered the identity and genetic control of regulatory T cells, the so-called "security guards" of the immune system that prevent autoimmune attack. "Their discoveries have been decisive for our understanding of how the immune system functions and why we do not all develop serious autoimmune diseases," said Olle Kämpe, chair of the Nobel Committee.

The immune system must strike a delicate balance: powerful enough to destroy invading microbes, yet restrained enough to avoid damaging the body itself. Until the 1990s, scientists believed this balance was maintained mainly through a process known as central tolerance, in which self-reactive immune cells are eliminated in the thymus.

However, Shimon Sakaguchi of Osaka University challenged this view. In 1995, he described a previously unknown subset of immune cells, characterised by the surface markers CD4 and CD25, that suppress excessive immune reactions. His findings, published in *The Journal of Immunology*, revealed that tolerance also operates in the body's periphery, mediated by what he named regulatory T cells. Sakaguchi's discovery faced early scepticism but would later prove foundational. As he continued to explore the mechanisms of immune regulation, others were unravelling the genetic basis of immune dysfunction.

In 2001, Mary E. Brunkow and Fred Ramsdell, then working at Celltech Chiroscience in Bothell, Washington, US, identified a mutation responsible for severe autoimmune disease in a long-studied strain of mice, known as scurfy. Through painstaking genetic mapping, they discovered that these mice carried defects in a previously unknown gene, which they named Foxp3. Their *Nature Genetics* paper not only pinpointed the cause of the mice's immune dysregulation but also linked mutations in the human Foxp3 gene to a rare, often fatal autoimmune disorder called IPEX (immune dysregulation, polyendocrinopathy, enteropathy, X-linked syndrome). Two years later, Sakaguchi demonstrated that *Foxp3* is the master regulator of the same T cells he had discovered earlier, proving that the gene controls the development of regulatory T cells. This connection completed a central puzzle in immunology and established the concept of peripheral immune tolerance as a fundamental principle of immune regulation.

The Nobel Assembly, which announced this year's prize on 6 October, noted that the laureates' discoveries have laid the foundation for a new field of research and spurred the development of new treatments, for example for cancer and autoimmune diseases. Current clinical trials are testing therapies that either boost or suppress regulatory T cell activity. In autoimmune diseases, researchers aim to expand these cells to calm an overactive immune response, while in cancer, the goal is to limit their activity to allow immune attack against tumours. *– By Rosie Bannister*

Metal-organic frameworks

The Royal Swedish Academy of Sciences has awarded the 2025 Nobel Prize in Chemistry to Susumu Kitagawa of Kyoto University, Richard Robson of the University of Melbourne, and Omar M. Yaghi of the University of California, Berkeley, for the development of metal-organic frameworks.

Their pioneering work has led to the creation of molecular materials with vast internal spaces that can capture, store and transform a range of substances - from water vapour to carbon dioxide and toxic gases.

"Metal-organic frameworks have enormous potential, bringing previously unforeseen opportunities for custom-made materials with new functions," said Heiner Linke, chair of the Nobel Committee for Chemistry.

The laureates' discoveries have transformed materials chemistry by enabling the construction of crystalline frameworks composed of metal ions linked by organic molecules. These networks, known as metal-organic frameworks (MOFs), form porous materials with precisely engineered cavities. Chemists can tailor the size, shape and functionality of these pores to suit specific industrial or environmental purposes - such as storing hydrogen, catalysing reactions or filtering pollutants.

The field's origins date back to 1989, when Richard Robson built the first prototype of a framework by combining copper ions with a four-armed organic molecule. The resulting crystal, although fragile, revealed an unexpectedly ordered structure filled with microscopic voids. Robson recognised the potential for using such architectures to develop materials with novel chemical properties.

Susumu Kitagawa and Omar Yaghi later provided the theoretical and practical foundation that transformed Robson's early concept into a robust and versatile platform. Between 1992 and 2003, both researchers independently made breakthroughs that established MOFs as a new class of functional materials.

Kitagawa demonstrated that gases could pass in and out of the porous frameworks and predicted their potential flexibility. Yaghi achieved stability and rational control over their structures, producing the now-classic MOF-5, a material whose internal surface area is comparable to that of a football pitch per few grams of substance.

MOFs are now produced in tens of thousands of variations, many with targeted applications. Some are designed to harvest water from desert air, while others separate per- and polyfluoroalkyl substances (PFAS) from drinking water, or capture carbon dioxide from industrial emissions. Certain MOFs can even absorb ethylene gas to slow fruit ripening, or encapsulate enzymes that break down residual antibiotics in wastewater.

Industrial interest in these materials is accelerating. Several companies are developing scalable methods for MOF production and testing their use in semiconductor manufacturing, gas storage and carbon capture. One of Yaghi's research groups has even demonstrated a MOF-based device that extracts potable water from arid desert air, powered solely by sunlight.

– By Rosie Bannister

Commentary: Maarten Meulenbelt

EU pharma reform meets US MFN pricing risks

The European Union is entering a decisive phase in the overhaul of its pharmaceutical legislation - the most extensive revision since 1965. The so-called Pharma Package, also referred to as the General Pharma Legislation (GPL), is now in trilogue negotiations between the European Commission, the European Parliament, and the Council of the EU.

The reform, consisting of a draft Pharmaceutical Directive (PR) and a Pharmaceutical Regulation (PR), has been presented as a modernisation to strengthen Europe's competitiveness and, at the same time, improve patient access, and it does include helpful attempts to streamline regulatory processes. However, some of its mechanisms – modulations of intellectual property (IP) and regulatory exclusivities, and potential obligations to launch across the EU – could have unintended negative effects, especially when viewed against the risk that the US might implement certain most-favoured nation (MFN) pricing policies. Together, these developments risk undermining both Europe's attractiveness for pharmaceutical innovation and global launch strategies.

Under current law, marketing authorisation holders are not required to launch their products in every country. They must ensure adequate supply once a product is launched, but there is no obligation to make it available across all EU markets. The Commission's proposal of April 2023 sought to change that balance through 'launch conditionality.' It proposed cutting baseline regulatory data protection (RDP) from eight to six years – a sizeable reduction – but offered companies the opportunity to regain two years by launching their product 'in sufficient quantities' in all member states within two or three years. Stakeholders pointed out that launching simultaneously across 27 member states, each with its own pricing and reimbursement regime, is unrealistic.

In response, the Parliament in April 2024 abandoned the conditionality approach but proposed an obligation to launch in member states that deliver a positive reimbursement decision. Yet the proposal fails to define positive - for example, whether a reimbursement level that is acceptable for a government but commercially unsustainable for a company would still trigger an obligation to launch.

The Council's June 2025 position went further still. It introduced a new Article 56a PD, compelling marketing authorisation holders to file pricing and reimbursement applications, to meet unspecified procurement 'requirements,' and establish a 'roll-out plan.' Failure to continuously supply a product in a member state within four years could lead to the loss of market protection, RDP, and orphan market exclusivity (OME) in that country. In addition, a new Article 5a PR would add an obligation for each company to ensure availability 'upon agreement with the respective Member State and within its responsibility.' A test of 'viability' of the company was proposed, but only in a recital to the proposed Regulation and not in the text itself; and it was not clear at what level the viability would be tested.

Such measures, without clear definitions or viability safeguards, can inject significant uncertainty into investment calculations. They risk lowering the risk-adjusted net present value (rNPV) of developing and launching innovative medicines in the EU.

The debate about obligations to launch has become more urgent by the possibility that the US might introduce MFN pricing, as proposed in the May 2025 Executive Order entitled Delivering Most-Favored Nation Prescription Drug Pricing to American Patients, aiming to align US prices with the lowest available in comparable OECD countries. Letters sent in July to 17 major pharmaceutical companies demanded substantial price reductions in the US market. The US government proposed comparisons with prices in countries with at least 60% of US GDP per capita (adjusted for purchasing power). Such a mechanism recalls the Trump administration's earlier MFN initiatives - defeated by US courts at the time - but signals renewed determination to explore linking US prices directly to foreign reference points.

If the EU were to impose obligations to launch in member states qualifying for US MFN benchmarking – which might occur either under the Trump administration, or a future administration – companies could face a difficult situation: depending on the data that would be available in the US about European prices, a discount offered in Europe could have an impact on the prices in the much larger US market.

The trilogue negotiations are not limited to launch obligations. All three EU institutions agree that baseline IP and exclusivity periods should be reduced. Under the current 8+2+1 system, products enjoy eight years of regulatory data protection and two years of market exclusivity, with an optional one-year extension for a new indication. The Commission's proposal would cut this to a 6+2+1 structure, the Parliament to 7.5+2+1, and the Council to 8+1+1. Orphan medicines would see reductions as well.

Another question relates to the robustness of protections such as RDP and OME (which are already facing baseline reductions), patents, and supplementary protection certificates (SPCs). There are several mechanisms that could erode protections, including a proposed expansion of the Bolar exemption permitting pricing and reimbursement procedures, and potentially even generic/biosimilar price listings, well before patent/SPC expiry; 'hospital exemption' rules for producing advanced therapy medicinal products (ATMPs) without an authorisation; and a proposed expansion of the possibilities to supply unauthorised pharmacy-compounded products.

The coming months will determine whether the EU's largest pharmaceutical reform in six decades strengthens competitiveness - or inadvertently weakens it.

This article was written by Maarten Meulenbelt, Partner, Sidley Austin LLP, Brussels

AZ in pricing deal with US

AstraZeneca Plc has become the second global pharmaceutical company to sign an agreement with the Trump Administration to lower the prices of its medicines sold in the US. In parallel it will increase its manufacturing presence in the country and upgrade its share listing. Pfizer Inc signed a similar agreement with the government on 30 September. The deals are applications of the World Trade Organization's most-favoured nation principle (MFN) under which favourable terms of trade, like lower tariffs, extended to one country are granted to other WHO members as well.

Announced on 11 October, the agreement with AstraZeneca stipulates that patients in the US will have access to medicines at prices that are equal to those in other wealthy countries. Prices in Europe are generally lower than those in the US. The company will therefore introduce a discount of up to 80% in the list price of prescription medicines for chronic diseases. It will further support a new platform, called TrumpRx.gov, where patients will be able to buy these medicines at the reduced prices. Separately, the US will delay the imposition of tariffs on the company's products for three years giving AstraZeneca time to increase its US manufacturing presence.

The agreement coincides with an upgrading of the company's US share listing. Currently, AstraZeneca is listed on the US Nasdaq market via American Depositary Receipts (ADRs). These are certificates issued by a US depositary bank confirming an investor's ownership of shares in a non-US company. This listing will be replaced by a direct listing of the company's shares on the New York Stock Exchange without the need for depositary documents. AstraZeneca said the change will enable investors to trade its stocks seamlessly across markets in New York, and in London and Stockholm where it is also listed. The company noted that the new structure will not change its status as a UK listed, headquartered, and tax resident company. "The company will remain bound by applicable UK governance principles and standards," it said.

AstraZeneca has committed to make $50 billion in manufacturing and R&D investments in the US over the next five years. Part of this is a planned outlay of $4.5 billion for a new manufacturing site in Virginia. The facility will produce the active ingredients for the company's weight management and metabolic portfolio, including oral glucagon-like peptide-1 receptor agonists, and combination small molecule products. The facility will employ 600 skilled workers and recruit a further 3,000 people to build the facility.

Genmab to acquire Merus

Genmab A/S is to take over the Dutch company Merus NV for $8 billion in one of the largest European biotech transactions to date, giving it new tools for treating a host of cancers. Merus has a portfolio of bispecific and trispecific antibodies which are recombinant molecules that bind to one or more targets at the same time. The company has one marketed product, Bizengri (zenocutuzumab), which has been approved for non-small cell lung and pancreatic cancers, and a second, petosemtamab, which is in development for head and neck and colorectal cancers. Petosemtamab has received two breakthrough therapy designations from the US Food and Drug Administration for head and neck cancer.

The takeover, announced on 29 September, is an all-cash transaction valuing Merus at $97 per share. It is expected to complete in early 2026. Both companies are listed on the US Nasdaq market.

Genmab is an established European biotech with a history of successful partnerships for antibody therapeutics. Since its founding in 1999 it has largely generated revenue through licensing income and royalties. In the 2025 first half, the company reported revenue from royalties of $1.4 billion, or 84% of the company's total revenue of $1.6 billion. The largest contributor to this figure was Darzalex (daratumumab), an antibody drug for multiple myeloma, discovered by Genmab and licensed to Janssen Pharmaceutical Companies. The Darzalex patent is set to expire in Europe between 2029 and 2032. The Merus acquisition, which follows Genmab's takeover of Profound Bio Inc of the US in 2024, will substantially bulk out its oncology portfolio.

Following the announcement, Genmab's share price rose by 22%. "The market saw how we wanted to tackle the patent cliff in a very strategic and practical way," Peter Louwagie, the company's head of corporate and business development, told an investor conference in Basel, Switzerland on 9 October.

Merus' marketed product Bizengri is a bispecific HER2 and HER3 directed antibody for the treatment of patients with lung and pancreatic cancers with a specific genetic abnormality called neuregulin 1 fusion positive. The next most advanced product, petosemtamab, is a bispecific antibody in Phase 3.

Merus reported revenue of $8.8 million in the second quarter, up by 20% from a year earlier, all of which was collaboration income. At the end of the quarter, the company had collaborations with Incyte Corp, Eli Lilly and Co, Gilead Sciences Inc, Ono Pharmaceutical Co Ltd, and Biohaven Ltd.

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Pfizer buys obesity assets

Pfizer Inc is to pay $4.9 billion to acquire a three-year old company, Metsera Inc, which is developing drugs for weight management giving it a selection of candidate products that can be delivered orally and by injection, and which include both amylin and incretin mimetics. The amylin-based treatments restore sensitivity to leptin, a satiety hormone that enables people to feel naturally full after eating. The incretins, which include the glucagon-like peptide-1 (GLP-1) drugs, mimic hormones in the gut that help regulate blood sugar and appetite.

Announced on 22 September, the acquisition is being priced at $47.50 per share in cash at closing, giving an enterprise value of $4.9 billion. In addition, the agreement includes contingency value rights for Metsera's shareholders valued at up to $22.50 per share, which would be paid if the company meets three clinical and regulatory milestones. If the contingency rights are exercised in full, the value of the deal could rise to up to $7.3 billion, according to *The Financial Times*. The transaction is expected to close in the fourth quarter.

The deal comes five months after Pfizer stopped a Phase 3 trial of a prospective drug for chronic weight management over a safety concern. The drug, danuglipron, was a small molecule GLP-1 receptor agonist initially developed for diabetes. During a weight management trial in April, one patient in the trial experienced a potential drug-induced liver injury, causing Pfizer to discontinue the programme.

In the newest deal, Pfizer will be acquiring four obesity programmes in clinical development and several pre-clinical candidates. The clinical programmes include weekly and monthly injectable GLP-1 receptor agonists and a monthly amylin analogue being evaluated as both a monotherapy and in combination with a GLP-1.

Novo acquires Akero Therapeutics

Novo Nordisk A/S is to acquire a US company with a late stage product for the treatment of metabolic dysfunction-associated steatohepatitis (MASH), a co-morbidity of obesity. Novo is building a global market presence in treatments for obesity and its latest acquisition, announced on 9 October, is expected to broaden the scope of its portfolio. MASH is a progressive metabolic disease affecting the liver. It is estimated that more than 40% of patients with MASH also have Type 2 diabetes and more than 80% are overweight or obese. Akero's lead product is efruxifermin, an analogue of fibroblast growth factor 21. Analogues of this growth factor are intended to lower blood glucose, improve insulin sensitivity and induce weight loss. Efruxifermin is currently in Phase 3 development for the treatment of patients with moderate to advanced liver fibrosis and cirrhosis.

Novo is to pay $54 per share in cash at closing for a deal value of $4.7 billion. Shareholders will also receive a contingent value right of $6 per share which, if exercised, would generate an additional $500 million. Akero is listed on the Nasdaq exchange.

Roche to acquire 89bio Inc

Roche is to acquire 89bio Inc of San Francisco, US, to expand its cardiovascular, renal and metabolic disease portfolio with a drug that is being developed for metabolic dysfunction-associated steatohepatitis (MASH). MASH (previously called non-alcoholic steatohepatitis) is a liver disease that develops when fat builds up and causes inflammation. Without treatment, it can lead to cirrhosis and liver cancer.

Roche is to pay up to $2.4 billion for the company at the closing, but this figure could rise to to $3.5 billion if milestones in the form of contingency value rights held by shareholders are exercised as part of the deal. The transaction is expected to close in the fourth quarter.

89bio's lead product, pegozafermin, is a recombinant protein currently in Phase 3 for the treatment of patients with MASH with advanced fibrosis. It is an analogue of fibroblast growth factor 21 which is a hormone that regulates important metabolic pathways. Pegozafermin is a glycopegylated version of the growth factor with an extended half-life, enabling it to increase sensitivity to insulin and reduce fats. In a Phase 2b placebo-controlled trial the drug showed an improvement in fibrosis and no worsening of non-alcoholic steatohepatitis, the predecessor to MASH. The trial results were published in *The New England Journal of Medicine* on 24 June 2023.

MASH is a comorbidity of obesity. In a statement on 18 September, Roche said that the acquisition opens the door to new treatments for overweight, obesity and MASH. It also unlocks opportunities for future combination treatments win incretins, which include glucagon-like peptide-1 receptor agonists.

Kailera gets financing for obesity

Kailera Therapeutics Inc, a US clinical stage biotech company, has received $600 million in Series B financing to support its portfolio of obesity drugs that include an asset poised for Phase 3. This is an injectable dual glucagon-like peptide-1 (GLP-1) receptor agonist and glucose-dependent insulinotropic polypeptide (GIP) receptor drug. These mechanisms of action work together to reduce obesity by decreasing caloric intake and activating receptors in the brain to curb appetite. The Phase 3 programme is expected to start by year-end and includes two trials in adults living with obesity or overweight with comorbidities, with and without Type 2 diabetes, and a trial in adults with a body mass index of 35 or more.

In a statement on 14 October, Kailera said that its lead product has achieved 'potentially best in category' weight loss results. The Series B financing was led by new investor Bain Capital Private Equity. Additional investors included Adage Capital Management LP, the Canada Pension Plan Investment Board, and Invus. The financing is expected to cover the Phase 3 programme as well as support a small molecule GLP-1 receptor agonist in Phase 2.

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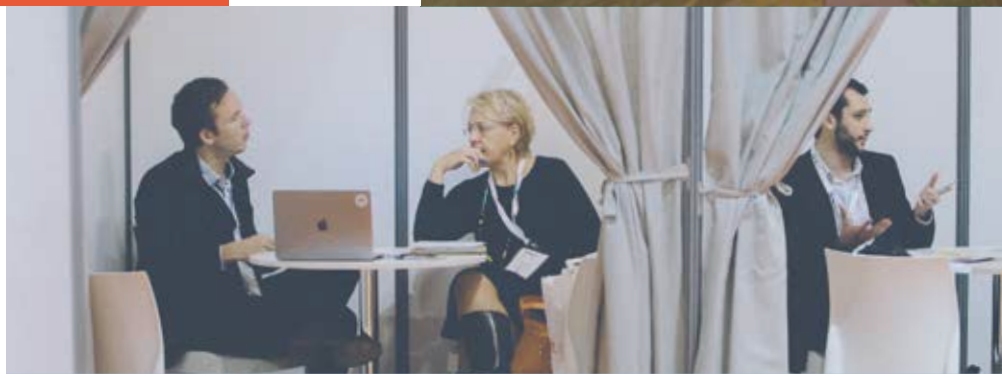

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Round-up of European biopharmaceutical company news

GSK Plc has appointed Luke Miels as chief executive officer designate, as well as a member of the company's board of directors from 1 January 2026. Emma Walmsley, the current CEO who has led the **UK** company since 2017, will step down from her position. However, she will remain with the business until her notice period ends on 30 September 2026. In a statement on 29 September, Ms Walmsley said that 2026 is the right moment for new leadership as the company defines its path for the decade ahead. "As CEO, you hope to leave the company you love – stronger than you found it and prepare for seamless succession. I'm proud to have done both – and to have created Haleon, a new world-leader in consumer health," she commented. One of Ms Walmsley's jobs during her tenure was to spin out Haleon, GSK's consumer health division in order to focus on proprietary medicine development. This took place in July 2022, leaving the company with a portfolio of vaccines and specialty medicines including drugs for respiratory diseases, cancer, and treatments for inflammatory disorders. The company currently has 15 candidate products that could potentially launch by 2031. It has set a sales target for the same year of more than £40 billion. Mr Miels joined GSK in 2017 and is currently chief commercial officer. He previously held senior positions at AstraZeneca Plc, the Roche Group and Sanofi SA. He holds a Bachelor of Science from Flinders University and a Master of Business Administration from Macquarie University, both in Australia.

Netherlands-based **VectorY Therapeutics BV** has negotiated an option and licensing agreement with a US manufacturer of adeno-associated viral (AAV) capsids ahead of making its first regulatory filings for clinical trials of a new drug for amyotrophic lateral sclerosis. The supplier of the tool is Shape Therapeutics Inc of Seattle, Washington, US, which is developing capsids for genetic medicines for difficult-to-reach tissues. Capsids are the protein shells of AAV viruses that can be used to carry a working copy of a gene into the nucleus of a target cell. Under the agreement, VectorY will receive an exclusive option on a brain penetrant AAV capsid from Shape to be used with a product from its preclinical vectorised antibody portfolio. The Dutch company will pay an upfront fee and potential milestones and other fees of up to $1.2 billion. If the option is exercised, the company could pay regulatory, development, and commercial milestones of up to $338 million for rare disease programmes, and up to $503.5 million for non-rare disease programmes. Potential royalties are also part of the package. Based in Amsterdam, VectorY has a preclinical portfolio of AAV vectors whose capsids have been engineered to incorporate the DNA of antibody fragments. When the vector is delivered to the body, the antibodies are produced *in vivo* and secreted. The company's lead product, VTx-002, targets a nuclear protein called transactive response DNA-binding protein 43 (TDP-43) which, in a disease setting, mislocates from the nucleus of a cell to the cytoplasm and forms aggregates that are toxic. VTx-002 is intended to target specific epitopes on the TDP-43 aggregated proteins in order to prevent proteinopathy in the cytoplasm, while protecting TDP-43 in the nucleus. The dysfunction of the TDP-43 protein is a feature of sporadic amyotrophic lateral sclerosis, according to the journal *Brain*. The disease, formerly known as Lou Gehrig's disease, is rare, but for those whom it affects, leads to a loss of mobility. VectorY's portfolio also includes candidate products for Huntington's disease and Alzheimer's disease. The company plans to submit clinical trial applications for VTx-002 to regulatory authorities in the US and Europe by the end of 2025.

An **Italian** university, hospital, and biotech and diagnostic companies, have joined forces to develop new treatments for lung cancer patients targeting the human epidermal growth factor receptor 3 (HER3). The collaboration is expected to identify HER3-positive circulating tumour cells with the goal of developing targeted therapies for the disease. It includes the **Sant'Andrea Hospital in Rome**, the **Department of Clinical and Molecular Medicine at Sapienza University**, also in Rome, the biotech company **Takis Srl**, and the diagnostics company Tethis SpA."Detecting and characterising HER3-positive cells in blood samples from lung cancer patients could lay the groundwork for developing new therapeutic solutions such as antibodies, antibody-drug conjugates, and CAR-T therapies that target HER3 as a specific molecular target," Claudia De Vitis of Sapienza University, said in a prepared statement on 3 October. HER3 is a protein on cell surfaces that, when paired with other members of the HER family of receptors, sends signals that control cell growth and survival. The protein amplifies signals from other receptors, especially HER2, to promote cancer cell growth, multiplication and spread, according to the US National Cancer Institute. Having established proof-of-concept for their project, the four collaborators are poised to enter clinical development where blood samples will be taken from lung cancer patients and used to produce slides which can identify HER3-positive circulating tumour cells. This is expected to be the starting point for developing HER3-targeted therapies and non-invasive diagnostics to select patients for treatment. Financial terms of the collaboration were not disclosed.

H. Lundbeck A/S of **Denmark** has entered into a collaboration with Contera Pharma A/S, another Danish company, to develop oligonucleotide-based medicines for neurological diseases. Oligonucleotide drugs are short DNA or RNA molecules that modulate gene and protein expression. Contera has proprietary RNA technology that can modulate RNA functions which can include protein synthesis, gene regulation, and enzymatic activity. "By joining forces with Lundbeck, we can create strong synergies and advance the potential of oligonucleotide therapeutics," said Thomas Sager, Contera's chief executive, in a prepared statement. The agreement features an upfront payment and research funding as well as milestone payments, the size of which were not disclosed.

Gene therapy advances

A gene therapy intended as a one-time treatment for Huntington's disease significantly slowed progression of the disorder in a pivotal Phase 1/2 trial, paving the way for a regulatory submission in the first quarter of 2026. The developer, uniQure NV, showed that it is possible to safely impact the disease by targeting both the disease-causing huntingtin protein and the normal protein using a vector-based gene therapy and a gene encoding a microRNA (miRNA).

uniQure announced top-line results from the trial on 24 September. They showed that the therapy, AMT-130, administered at a high dose, was able to achieve a 75% disease slowing at 36 months as measured by a Huntington's disease rating scale and compared with a score-matched external control. The trial enrolled 29 patients.

"I believe these ground-breaking data are the most convincing in the field to date and underscore potential disease-modifying effects in Huntington's disease where an urgent need persists," said Sarah Tabrizi, director of the University College London Huntington's Disease Centre, in a prepared statement.

Huntington's disease is a rare, inherited neurodegenerative disorder that can cause chorea, behavioural abnormalities, and cognitive decline, resulting in a progressive physical and mental deterioration. Mutations in the huntingtin gene lead to the production and aggregation of abnormal proteins in the brain. AMT-130 has been designed to carry and deliver a gene encoding a miRNA that will recognise, bind and non-selectively lower both the mutant and the normal huntingtin proteins. The therapy is administered to patients by way of a neurosurgical procedure.

Based in Amsterdam, the Netherlands, and Lexington, Massachesetts, US, uniQure has an approved gene therapy on the market for haemophilia B and a pipeline of candidate therapies for refractory temporal lobe epilepsy, amyotrophic lateral sclerosis, and Fabry disease.

Separately, the company announced the closing on 29 September of an upsized public offering of $345 million.

BMS invests in cell therapy

Bristol Myers Squibb Co has stepped up its commitment to cell therapies with plans to spend $1.5 billion to acquire a Cambridge, Massachusetts biotech which is developing a medicine to reprogramme the immune system and treat autoimmune diseases. Orbital's lead product, OTX-201, is an *in vivo* chimeric antigen receptor (CAR) T cell therapy that is being developed using ribonucleic acid (RNA) technology. The preclinical product consists of a circular RNA that encodes a CAR which targets cells expressing CD19, a B cell specific antigen. The RNA molecule is delivered to the body using lipid nanoparticles. Once delivered, the body manufactures CAR T cells internally in order to induce the immune system to reset in cases of an autoimmune disease.

On 22 July, Orbital presented preclinical data for OTX-201 which showed that it achieved the full depletion of B cells in blood, spleen and lymph nodes in an autoimmune setting. While B cells defend the body against pathogens they can also malfunction and contribute to the production of auto-antibodies.

Orbital, soon to be part of BMS, said it planned to start clinical development of OTX-201 in the first half of 2026. In a statement, Robert Plenge, BMS's chief research officer, said that *in vivo* CAR T therapies "could redefine how we treat autoimmune diseases." The *in vivo* cell therapy is a relatively new approach, following on from *ex vivo* CAR T cell therapies that were first approved by the US Food and Drug Administration in 2017. The first *ex vivo* treatment was Kymriah (tisagenlecleucel) for acute lymphoblastic leukaemia and produced by Novartis. Three more *ex vivo* therapies were then approved in a second wave. One of these was Breyanzi (lisocabtagene maraleucel) from BMS which was authorised in 2021 for refractory large B-cell lymphoma. A month later, BMS had a second *ex vivo* product approved for multiple myeloma. This was Abecma (idecabtagene vicleucel). In 2024 Breyanzi generated sales of $747 million and Abecma, $406 million.

Companies exit cell therapy

Restructuring across the biotech industry has resulted in decisions by three companies to end programmes in cell therapy after investing heavily in the sector for several years. On 30 September, Heartseed Inc of Japan announced that partner Novo Nordisk A/S, was ending a collaboration to create a stem cell-based therapy for heart failure. In separate announcements on 1 October, Takeda Pharmaceutical Co Ltd of Japan and Galapagos NV of the Netherlands announced plans to discontinue or divest cell therapy programmes in cancer.

In all three cases, the companies are restructuring their businesses for financial or strategic reasons. In announcing the measures, they did not critique the cell therapy technologies themselves.

Novo is in the midst of a restructuring that will reduce its workforce by 9,000 in order to concentrate resources on diabetes and obesity. The company has a new chief executive, Mike Doustdar, who is overseeing the reorganisation. Under a 2021 agreement, Novo and Heartseed Inc were developing a cell therapy for heart failure using cardiomyocytes derived from induced pluripotent stem cells. This collaboration is now ending.

Takeda's decision to exit cell therapy coincides with a narrowing of its therapeutic focus. Going forward, it will concentrate on developing small molecule drugs, biologics and antibody-drug conjugates. The company's cell therapy investment dates to 2017 when it took out an option to acquire GammaDelta Therapeutics Ltd of the UK. It bought the company in 2021 in order to investigate the potential of gamma delta T cells for treating cancers. The goal was to generate both blood and tissue-derived allogeneic therapies for haematological malignancies and solid tumours. In 2022, Takeda acquired another UK company, Adaptate Biotherapeutics Ltd, to expand the gamma delta T cell business. On 1 October, Takeda said it had wound down the business and was not running any clinical trials using the technology.

Galapagos, formerly a developer of small molecule drugs, entered the cell therapy field when Paul Stoffels, a former Johnson & Johnson Inc manager, became CEO in 2022. The plan was to develop autologous CAR T cell therapies for cancer. They would be manufactured locally and delivered to patients within seven days of drawing cells from the same patients. After another change in management in May, this plan was abandoned. On 1 October, Galapagos said that it was reviewing strategic alternatives for the cell therapy business including potential divestiture.

News Briefs

Italy's Chiesi in gene editing deal with Arbor

Italy-based Chiesi Farmaceutici SpA has entered into an option and licensing agreement with Arbor Biotechnologies Inc to use the US company's gene editing technologies to develop medicines for rare diseases. The deal focuses on a clinical-stage programme for primary hyperoxaluria Type 1 (PH1), but also includes access to Arbor's platform for other therapies targeting the liver. Based in Cambridge, Massachusetts, Arbor has developed a suite of gene editing technologies ranging from the knock-out of genes to large gene insertions. PH1 is a lifelong genetic disease caused by a mutation in the *AGXT* gene leading to an enzyme deficiency in the liver. This results in a build-up of oxalate crystals in the kidney which can eventually result in end-stage kidney disease. Arbor's product, ABO-101, is intended as a one-time treatment to reduce oxalate production. It recently entered a Phase 1/2 clinical trial in the US with plans to open additional trial sites in the UK and the EU. Chiesi, a family controlled, global pharmaceutical company, will pay up to $115 million upfront for rights to the product and technology. There are also potential milestone payments of up to $2 billion. "While this path holds immense promise, we know there is still a long journey ahead, and much to learn," said Giacomo Chiesi, the company's executive vice president, in a statement, welcoming the collaboration.

AZ partners with Algen Biotechnologies

AstraZeneca Plc is to team up with Algen Biotechnologies Inc of San Francisco, US, to discover new targets in immunology. This would be done by using Algen's technology platform which is able to identify RNA changes in disease-relevant cell types and link them to functional outcomes using high throughput gene modulation. According to Algen, this process has the potential of identifying gene targets that could reverse disease processes. The platform uses both AI and machine learning to enhance the discovery process. Under the agreement, Algen will receive up to $555 million including upfront, near-term payments and potential future milestone payments. "Truly transformative therapies begin with uncovering the right biological targets which have strong human translational relevance," Jim Weatherall of AstraZeneca, said in a statement on 6 October.

T cell therapies for solid tumours

The cell engineering company MaxCyte Inc has reached an agreement with Moonlight Bio Inc to develop ex vivo gene edited T cell based therapies for solid tumours. Moonlight has a pipeline of preclinical T cell therapies while MaxCyte has electroporation technology to genetically engineer cells at scale. The two companies have signed a strategic platform licensing agreement under which Moonlight will have non-exclusive rights to the electroporation technology in exchange for licensing fees and programme-related payments. Moonlight is based in Seattle, Washington, and MaxCyte in Rockville, Maryland, US.

On the Move

Yellowstone Biosciences Ltd, an Oxford University, UK, spinout developing selective cancer therapeutics, has appointed **James MacDonald** as chief executive officer. Mr MacDonald's more than 25 years of experience includes scaling high-growth biotech companies, advancing novel oncology platforms and strategic financing. He was most recently venture partner at Altitude Life Science Ventures and previously, he was co-founder, executive vice president and general counsel at Sana Biotechnology, where he played a key role in building and scaling the business, undertaking its private financings as well as its Nasdaq IPO.

Cumulus Oncology Ltd has appointed **Manuel Aivado**, a haematologist and oncologist, to its board, and **Brian Jones**, experienced in small molecule drug discovery, as a scientific advisor. In recent years Dr Aivado played a key role in six new drug approvals, including four in oncology, and has a strong track record in clinical development strategies as well as business management. Dr Jones will support the progression of Cumulus' portfolio as well as improving the identification of novel asset acquisition targets.

BICO Group AB's board has elected **Maria Rankka** as chair until a new chair is named at the company's shareholders' meeting. She succeeds Rolf Classon who has resigned for health reasons. Ms Rankka, an investor and entrepreneur mainly in health tech and life science, joined BICO's board in May. She is also board chair of a Swedish stem cells company Cellcolabs. Her new position at BICO supports the Swedish company's focus on its commercial strategy and in communications. BICO supports pharma companies, biopharma and academia, to bring innovations to market by automating life science laboratories through hardware and software.

Jon Wigginton, an expert in immunotherapy drug development, has joined the board of **Pathios Therapeutics Ltd** as a non-executive director. Dr Wigginton brings over 25 years of corporate and research experience in clinical oncology, including leadership positions at Bristol Myers Squibb where he led early clinical development of the company's immuno-oncology portfolio. He is currently president of research and development at Bright Peak Therapeutics, a clinical-stage biotechnology company. Pathios is working on a programme to counteract the polarisation of immune cells that can occur in the tumour microenvironment.

Zealand Pharma A/S has appointed **Rachel James-Owens** as vice president, corporate communications and media relations. She brings over 20 years of experience from Pfizer and Novartis spanning sales, commercial and communications. Most recently, she was executive director, global corporate communications at Novartis Global Health and Sustainability and prior to that, she led global executive oncology communications at Novartis and regional positions at Pfizer. Earlier this year Roche signed an agreement with the Danish company to co-develop a synthetic peptide analogue of amylin, a peptide hormone. The drug, petrelintide, is being developed for obesity.

Exciting Instruments Ltd, a developer of technology to accelerate drug discovery, has appointed **Jonathan Rigby** as chairman. Mr Rigby brings extensive experience in building and financing life sciences companies including SteadyMed Therapeutics, acquired by United Therapeutics, and co-founding Zogenix Inc, which was acquired by UCB Pharma for $2 billion. He is currently chairman and chief executive officer of Sernova, a regenerative medicine company. Exciting Instruments' benchtop single-molecule detection platform is designed to accelerate and improve the discovery of novel proteins and drug candidates. Mr Rigby will support the UK company's expansion and partnership strategy with academic researchers and pharmaceutical companies.

Epitopea Ltd has made four appointments to its scientific advisory board, bringing the SAB to six members along with company co-founder Pierre Thibault and chair Michael Kalos. The new members are: **Nahum Sonenberg**, a professor at McGill University, Canada and an expert in translational control; **Nina Bhardwaj**, a professor of medicine and urology at the Icahn School of Medicine, Mount Sinai, US, and an expert in human dendritic cell biology; **Margaret Callahan**, a professor of medicine and immunology at a UConn Health cancer centre with expertise in immuno-oncology, clinical trial design and translational research; and, **Craig L. Slingluff**, a professor of surgery at the University of Virginia, US, and an expert in melanoma immunology and cancer vaccine development. The appointments come as the UK/Canadian company advances its RNA-based cancer immunotherapies, focusing on tumour-specific antigens known as Cryptigen TSAs, towards the clinic.

The **European Commission** has appointed **Mikko Tapio Huttunen** as principal legal adviser to the trade team in the Commission's legal service. Mr Huttunen brings almost three decades of experience at the Commission and previously led the Commission's legal efforts, ensuring compliance with rules of the World Trade Organization and the European Court of Justice.

Siegfried Holding AG, a Swiss life sciences company, has proposed that **Beat Walti** be elected chairman at the company's annual shareholders meeting in April, 2026. Dr Walti is already a board member and would succeed Andreas Casutt, who has been Siegfried's chairman since 2014. Dr Walti, an attorney, chairs the board of trustees of the Ernst Göhner Foundation, Siegfried's largest shareholder. With board mandates at DSV A/S and Rahn AG, as chairman, he will drive the expansion of Siegfried's market position. The company manufactures pharmaceutical APIs and drug products for the pharmaceutical industry.

Companies and universities in this issue

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