GENESIS REVIEW

A review of our annual Genesis conference bringing together key Life Science opinion leaders and stakeholders to debate key trends, instigate deals and generate a vision of the future. Including an update from companies on the One Nucleus stand at BIO-Europe Spring 2024.

London, UK

GENESIS 2023 OVERVIEW

For over two decades the Genesis conference in London has served as an ideal opportunity to assemble key stakeholders and thought leaders from across the Life Sciences sector to take stock of recent trends and discuss what influences, challenges and opportunities may lie ahead. The discussions at Genesis evolve year on year, largely reflecting the changes in the biomedical research and development field and the associated financing landscape. Reflecting on the Winners & Losers of the past year at the start of programme, expertly compiled by veteran industry commentator Mike Ward is always a great place to start!

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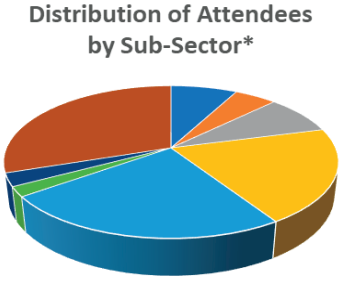
305 Attendees

208 Companies

38 Speakers

22 Exhibitors



*Sub-Sector	Organisation types
Business Support Groups	Membership Groups; Economic Development Associations, Government Bodies; Trade Associations
Facilities	Science Parks; Innovation Centres; Real Estate Investors; Accelerators
Academia	University Research Labs; Technology Transfer; NHS Bodies; Research Charities
R&D Services	CRO; CDMO; CMO; Lab Tech Suppliers
Primary R&D	Pharma, Biotech, MedTech, Digital Health
Investors	Venture Capital; Public; Wealth Managers; Investment Banks
Press	Industry publications; commentators; Business News Channels
Professional Services	Legal, Regulatory Advisers; Intellectual Property; Communication; Business Consultants

The cross-section of views and expertise illustrated above is what informs and shapes the formal and networking debate at Genesis and why the content evolves with the sector. The December 2023 programme explored key issues including Investment Trends; Accessing Patient Data; Business Models for Growth; Aligning Health-span, Life Span and Sustainability for Affordable Longevity; A Patient's Perspective on Modern Illnesses and Hot Dealmaking Areas for 2024; and The Dealmaker's Anatomy.

In this review of Genesis 2023, you'll find a summary of the key messages arising from the conference and the associated On-line Innovation Workshops and Genesis Fringe sessions. If you wish to delve deeper into the detail, you are able to find recordings of the Keynote Sessions and Innovation Workshops via the One Nucleus YouTube channel.

The Genesis 2023 Thought Leaders:

Mike Ward
Winners & Losers 2023

Alexandra Gray
A Patient's Perspective

Panel: Bio-Dollar Deal Briefing

Eleanor Malone
Citeline

Will Walker-Arnott
Charles Stanley

Rowan Gardner
PrecisionLife

Maina Bhaman
Sofinnova Partners

Bradley Hardiman
Astellas Pharma

Panel: Buy, Borrow or Grow-Your-Own?

Dianne Lee
DLRC

Robert Grundy
Intelligent OMICS

Francesca Crawford
ViaNautis Bio

Neil Torbett
PhoreMost

Daniel Rooke
Start Codon

Panel: Incorporating Real World Evidence into R&D Strategy

Victoria English
MedNous

Daniel Prieto Alhambra
University of Oxford
DARWIN-EU Deputy Director

Steve Gardner
PrecisionLife

Jaspreet Grewal
AxialBridge

Chris Wayman
EY

Panel: BioPharma Dealmaker Outlook

Sue Charles
Charles Consultants

Nuno Alves
Astellas Pharma

Khatereh Ahmadi
MSD

Lilian Alcaraz
J&J Innovation

Panel: Investing in Longevity – Making Better Patient Outcomes Affordable

Lisa Urquhart
Healthcare Journalist

Anji Miller
LifeArc

Alex Blyth
LIfT Biosciences

Roz Bird
Anglia Innovation Partnership

Pedro de Noronha Pissarra
Chrysea Labs

Panel: The Anatomy of a Great Dealmaker

Mike Ward
Clarivate

Caroline Austin
AstraZeneca

Suzy Dilly
ValiRx

Prashant Shah
o2h ventures

Rachel Bradley
Penningtons Manches Cooper

Seven Key Take-Aways from Seven Keynote Sessions:

Winners & Losers 2023:

Mike Ward, Global Head of Life Science & Healthcare Though Leadership, Clarivate, set the scene with his 'Winners & Losers' keynote presentation to open the day. Reflecting on a challenging year for life science companies, Mike highlighted key data on investment, deals and performances in the BioPharma industry in 2023. Whilst the data and specifics are in the full recording, the key messages included:

Out of control – Many of the global factors impacting the life science industry were outside of its control such as the Inflation Reduction Act; tightening of patent claim coverage; global conflicts; and impending General Elections in 2024.

Breakthroughs – Lilly and Novo Nordisk gaining approval for anti-obesity products, a condition estimated to cost $170Bn in 2019 in the US alone saw them become the industry's two largest companies. Evidence for activity outside of obesity bodes well, and may well see other Pharma striving to enter the race. Equally, the MHRA being the first to approve a CRISPR-Cas9 designed drug (Vertex) and FDA and other approvals expected in early 2024, then that is expected to trigger deal flow also.

Watch out for the cliff – Much is often made of the patent cliff facing Pharma. In 2023 we saw how the emergence of biosimilars impacted revenues for the top-selling drug in the world, Humira. As the patent expired at the start of the year, no less than eight biosimilars launched since (another coming) saw revenues drop by 31%.

Ask the public – Whilst indices such as the S&P 500, NASDAQ Composite and others showed rises year-on-year (Y-o-Y), NASDAQ Pharma showed a modest 2.26% rise and the NASDAQ Biotech Index was 73% down. That said, whilst IPO appetite was low, follow-ons and other forms had the third best year ever, bio-venture capital saw the fourth best year ever, venture funds have raised money and Pharma are sitting on large war chests, so the outlook may be very good.

Risers and fallers – 8/10 of the biggest share price rises Y-o-Y were in the US and led by Eyepoint Pharmaceuticals. Orchard Therapeutics being the leading UK performer. 7/10 of the biggest drops Y-o-Y were in the US also with Adaptimmune being the biggest UK faller.
Friends needed – Analysis of the top five products for six top tier Pharma (BMS, Johnson & Johnson, Gilead Sciences, Pfizer, Abbvie and Merck & Co) it was striking that 83% of the products came from externally sourced innovative products and >50% of the revenues in all those companies. The 'not invented here' syndrome is well and truly over and deals are here to stay whether driven by a need to fill pipeline, or just as commonly in order to enter new therapeutic or mode of action areas.
Indicators of the future – Notable during the year were some case studies that may well be covered in business school textbooks in years to come. These include the impact of the Sanofi CEO making what seemed like an industry norm statement about their forward-looking focus on innovation and spinning of the consumer business which led to a one-day hit of over $20Bn on the share price. Y-o-Y the share price was down just under 5% though. Equally, a once poster child of the digital health emergence, Babylon Health went from a $4Bn IPO valuation to bankruptcy.

View the recording HERE or via

Bio Dollar Briefing:

Moderated by **Eleanor Malone**, Editor-in-Chief, Commercial Insights, Citeline the panel explored reasons to be optimistic about the bio-financing outlook in Life Sciences, along with the challenges that remain to be overcome. Some of the examples and data given can be viewed in the recording but key messages included:

It's getting there - Looking at some of the underlying reasons why there may be justification to be cautiously optimistic then from a macro-economic perspective falling inflation in key markets and anticipated further interest rate cuts are leading to re-pricing in public markets and generally will support a greater risk appetite among investors.

Public impact in private – whilst the majority of successful investments in life sciences exit via M&A, one should not forget the impact public market trends have on the private investors. This includes their ability to raise capital as well as their assessment of an innovative company pitch in terms of can they see a pathway to an exit at an acceptable valuation.

Basic thinking – the fundamentals remain unchanged in terms of how innovation drives the sector in the UK. The science base is very strong, there is investment capital out there and Pharma needs to do deals and for the Corporate Venture Funds the plan doesn't really change.

Being awesome – Raising money is hard, for investors and entrepreneurs. Significant investors will see in excess of 100 pitches a week, so standing out is key. What's needed is fairly consistent. Be focussed and intentional in your financing strategy; be creative in deal structures and sources of capital; do your homework to know which investors to pitch to and why; be a great storyteller; have a great team and don't forget you need to be lucky too. But doing the other things right can make you more lucky.

A rich ecosystem – Discussing US vs UK/EU situation. The gap in deal sizes isn't necessarily getting bigger, even if that could be felt when reading the headline deals. The drivers are similar, ex-US companies may offer greater value given the pricing, US has more capital but US also has a richness of the deal enablers in their ecosystem compared to UK/EU. Those enablers that connect, advise, assess valuations and inform others are key to transactions getting done. Dynamics in e.g. London have meant a less rich

cadre of specialists in the ecosystem which is detrimental and has been contributed to by the de-equitisation of the London capital market over recent decades. The Mansion House reforms aim to address this.

Building Mansions – The UK Government move to reform pension fund regulation to release 5% of their holding into non-public companies represents a potential £50Bn capital pool to pitch to. The life science sector needs to be ready to compete against others sectors for that money by aligning the right opportunities with the right investors in terms of timelines, size and focus. The UK life science sector needs to compete well to ensure the chosen fund managers deploy the capital in the UK for impact.

Using intelligence - Entrepreneurship has become increasingly professionalised over recent years and that helps match the opportunity to the investor more effectively and efficiently. Whether using artificial intelligence can help this is questionable when so much of the deal-making process is a human chemistry dynamic as well. Just because you fit the criteria of the fund, doesn't mean you are the right opportunity. Personal networks can help those pitching stand out in the first sift to at least get in the door. There are available lists of investors and their criteria, so entrepreneurs need to do their homework thoroughly, be smart about who they pitch to, and use what they have to load the luck dice in their favour: your insight into the opportunity, your storytelling and your passion; your network and don't forget to read the situation. Do not ignore the sign of a quick 'no' because the investor is being polite.

View the recording HERE or via

Buy, Borrow or Grow-Your-Own:

Dianne Lee, CEO, DLRC Ltd, led the panel through a discussion on how growing Life Science companies make the decision of when to grow their internal team, outsource to expert providers, or acquire new capacity through M&A. Key insights shared by the panel included:

We are all different! – Every decision in a growing business has to be made on its merits for that situation and set of options. There is not a one-size-fits-all solution to how best to scale a company. Key ingredients to consider are available capital, the correct company culture, the impact on profitability and what key parts of the business are considered core to need scaling, e.g. the science.

Capital efficiency – All stakeholders wish to see this and investment directed appropriately. In deciding between in-house, outsourcing or M&A routes then best use of capital is a key aspect. This is stage-dependent too, when start-ups and early-stage companies may have no option but to outsource to access key equipment and skills as they generate early data and value.

Good neighbours – The choice can be influenced by the richness of the ecosystem in which you operate and what competences, scale and flexible options may be on offer externally. The UK, for example, has a very diverse, skilled, and deep provision of services across the drug discovery and development pipeline. In such an instance, where good relationships can be built locally, outsourcing may feel like an obvious choice.

Who decides? – Considering who the business leaders turn to for advice and consultation, there are some key cohorts that must be engaged. The senior management team needs to be as one in deciding which elements of the business can be outsourced and which cannot as well as the rationale behind the choice. The senior scientists need to be engaged in identifying who has the expertise to manage the external discussions with providers from a technical point of view. Investors of course, will want to be, and should be, engaged in the more significant decision so best keep them informed on the strategy.

External to internal – One of the challenges is striking the right balance between wanting to generate and secure good data quickly and with the time it may take to build an internal capacity to do the work. Time and cost to build internally on the one hand, and cost and delay in securing the data in-hand on the other. Viewing the services provider as a true partner can be valuable as can the willingness to be open with the provider about your longer-term plans to take this in-house. Good providers who are a fit with your culture and aspirations will not be offended by the fact that you ultimately want to take it in-house. Far from it, they are likely to be great partners in helping you train, recruit and build the in-house capacity in your plans whilst you use them to bridge the gap. Providers are far less happy when communication has not been open and an abrupt move of the capacity in-house is sprung on them.

Be whatever you want – It is vital not to lose sight of what type of company you wish to be. The focus of what you see yourself as is important in deciding between external vs internal. For most, if you want to be a drug discovery company then focus on building that. Alternatively, if you wish to be a manufacturer then be a manufacturer. You may wish to be a FIPCO like Pharma, but this is less likely.

Can we do that? – After considering all the business needs in coming to a decision on outsourcing, M&A or in-house growth to scale the company, then comes the question of feasibility. Cash and focus already considered, there are often logistical challenges to get over too. Are the right people available to recruit to the team? Is there appropriate lab space available on suitable terms? Or has there been a change in the way geographic clusters offer pools of the right talent post-Covid, which may have moved the goal posts.

View the recording HERE or via

Incorporating Real World Evidence into R&D Strategy:

Victoria English, Co-Founder & Editor, MedNous explored with the panel the challenges of how to leverage the growing availability of data from multiple sources to inform, innovate and improve the R&D process leading to better medicines. Key messages emerging from the discussion included:

Definitions – Real World Evidence is the outcome of analysing Real World Data that has been collected outside of a controlled clinical trial environment. Combining with medical data can inform and enhance progress towards the ultimate goal for all involved: better outcomes.

Integrity – A concern often raised is how one can guarantee the quality and integrity of the data given the diverse sources and varying regulatory environments. There are clear safeguards that evolve all the time when data is accepted into the large datasets to exclude 'rogue' data. Integrity of access and use governed by, e.g. 'Secure Data Environment' approaches that allow access based on the technical and ethical compliance of using the anonymised data.

Regulation – Often held up as a barrier to innovation or even value creation by those who seek to leverage the data sets. Being open source and transparent with data does not necessarily inhibit innovation since it provides access to help inform decisions at all stages of the research and care pathways. Clarity on who owns the data is key and due diligence should be done not just by those seeking to access the data but by those contributing to the data set, sometimes inadvertently when buying some form of genealogy service.

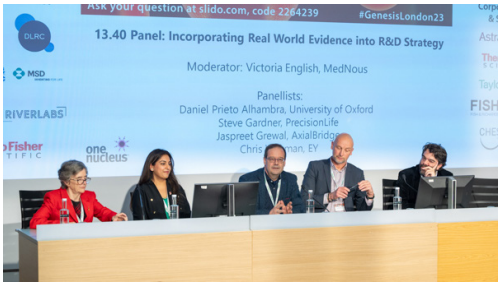
Epidemiology matters – An obvious statement may be when it crystalises how a disease presents, how it is treated and how it changes over time. Considered in the context of a medicine's regulator, then collections of validated epidemiological data sets, as we saw in e.g. Covid-19, provides great evidence and guidance on decisions such as which drug development programmes to grant accelerated approval routes, outcome measures sought, and more.

Gatekeeper's questions – Whether to grant access is driven by whether the entity seeking access is able to address two key elements. First, is the access applicant asking the right question of that data? Second, is the data being accessed the right data to answer the question being asked? Whilst using RWE can benefit many stakeholders in enhancing aspects such as clinical decision-making, regulatory guidance, care pathway efficiency and healthcare practice/costs, quite appropriately the attention is ultimately focussed on patients and what benefits there are to them from agreeing to have the data shared. If we are unable to show better outcomes as a result, one could argue we are asking the wrong questions.

Synthetic Data – The ability to safeguard the level of traceability back to patients and how anonymous the data is, one approach is to effectively create an identical twin of that population data that can be used to train and test new algorithms which can later be transferred and tested in the real world such that predictability in patient stratification and efficacy can be increased. Sounds great in theory and has uses but one must caution against inherent biases becoming baked in and ensure it is the right data being collected in the first instance.

Worth the effort? – Whilst there are many challenges to ensuring effective and valid uses of RWE in R&D, as well as ensuring an equitable sharing of value (not necessarily financial) to those contributing that data, then the investment in areas such as the technology advances to enable us to collect more data, the development of secure environments, the training of AI algorithms and disruption of the norm do allow us to move along the value change in R&D from safety, to efficacy to effectiveness. More effectiveness meaning better outcomes for patients which can be priceless.

View the recording here or via



BioPharma Dealmaker Outlook:

Sue Charles, Founder and Director, Charles Consultants explored with leading Pharma partnering leaders which therapeutic areas and treatment modalities will likely attract attention in deals in the coming year. Accepting there is a comprehensive and broad spread of interests across the industry, the panel, exemplified by the mention of a range of specific deals, picked out key messages from their own company perspectives:

Therapy Areas – Oncology remains a strong interest for pretty much everyone, often >50% of the portfolio. Other areas highlighted were ophthalmology (particularly delivery technologies); neuromuscular disease; protein homeostasis; phage therapies and microbiome-related areas; neurodegenerative disorders such as Alzheimers (AD) and Parkinson's (PD) diseases; psychiatric disorders; immunological diseases.

Deal stages – This very much depends on the programme and opportunity, but there was a strong sense among the companies that the pressures of patent cliffs, the IRA (Inflation Reduction Act) and competition for innovation meant they were on the whole stage and modality agnostic within their respective strategies.

ADC's – Despite the first iterations being around since the start of this millennium, e.g. Mylotarg which was subsequently removed voluntarily by Pfizer in 2010, ADC's (antibody-drug conjugate) continue to evolve, and they remain a key hot modality. Advances in the linker technologies to bring efficacy are also key. Moving to tri- and even quad-specific antibodies is starting to emerge as an interesting approach. Achieving success as a monotherapy for the product remains the strong goal to achieve a 'prevent, treat and cure' holy grail.

Cell & Gene Therapies – There is a strong interest in this space as the drive to optimise CAR-T approaches remains by using delivery and formulation advances to increase efficacy and reduce, e.g. immune rejection. Addressing monogenic diseases, using next generation AAV delivery technology, gene writing and insertion approaches and non-viral delivery strategies that bring scale-ability all got mentioned. Note here

that a counter-mention was made that despite e.g. the IRA impact, there was still a critical role, and hence a strong interest in small molecule programmes given the cost and complexity advantages.

AI/ML – It would have been odd not to hear this mentioned. The growing use of generative AI across the breadth of R&D, from target discovery and molecular structures to clinical trial design and patient stratification, is here to stay. Pharma has invested heavily in both building internal capacity and, increasingly, now through partnering strategies. Accessing the appropriate patient data, including the use of resources such as the UK BioBank and a soon-to-be launched proteomics consortium all contributing to the focus in this area.

mRNA – A significant increase in interest in recent years as expected and much attention is now being paid to this area of modality in e.g. cancer vaccines. Understanding continues to grow but there does appear to be a sense that harnessing the host's own molecular machinery is impacting drug efficiency. The bottom line here is that as with any new area, the progress and corresponding deal appetite will be a very data-driven relationship.

Opening the door – addressing the question of how best to approach the potential Pharma partner, it was noted that Pharma is more transparent in their wish lists and who to speak to than ever before. There was strong consensus across the panel that the key characteristics to keep in mind when approaching a potential Pharma partner included: Present your data clearly about your science and programme (not generic market data); be open about the gaps that will build trust; do your homework on the strategic focus you need to align to; for any claims you make, be sure you have the data to back them up; be sure to do your research on how that partner works; engage early to build the relationship and trust (as well as get free advice); maximise the opportunity to start and nurture those relationships; and do your homework by attending industry networking events where you can informally engage with the Pharma lead.

View the recording HERE or via

Investing in Longevity – Better and More Affordable Patient Outcomes When Health-span and Innovative Treatments Collide:

Lisa Urquhart, Healthcare Journalist, led one of the more diverse and challenging panel discussions of the day when opinion leaders from biopharma, precision nutrition, technology transfer, and innovation ecosystems debated the potential opportunities and challenges in aligning prevention and cure approaches to enable affordable longevity for populations. Several interesting themes emerged that require solutions, including:

Start here – Considering the current models and systems in healthcare. Most systems are shaped like an inverted pyramid, whereby those that get increasingly unwell get more attention (expenditure) as the population filters down. Healthcare systems know it is unaffordable, existing care pathways flow that way and business models that incentivise investment are based upon it. In an ideal world, we would not start here, but start at the top of that inverted pyramid, intervening earlier at lower cost per individual but helping at volume towards longevity of health-span.

Application of knowledge – In the UK, and in other life science centres of research excellence, a great deal is known about the positive impact certain behaviours, better foods, diet, microbiome, and more can have on longevity. A great deal is also known about the pathophysiology of ageing disorders, including biomarkers of said conditions. Yet these pools of information rarely get integrated through effective collaboration and joined-up thinking.

Value capture – There is a well-accepted path for innovators and their associated funders and technology transfer offices to harness the value of innovation through IP protection, business creation, and commercialisation. The primary measure of success often being financial. In wellness and prevention, the route to impact can be seen more as creating change for the better in society, so disseminating the knowledge widely and freely is the way to go. Not running with the traditional currency and pathways

making it difficult to create the right vehicles and business models to attract investment and adoption.

Empowerment – Underpinning the creation of any societal change, and perhaps the market opportunity to accompany it, is a set of needs in the shape of information, education, and communication. Increasingly, the information to show correlative links between, e.g. exercise and improved outcomes observed in observational rather than clinical trials. The greater trust being put into Real World Evidence is starting to gain traction among health leaders on these topics also. This needs to lead to the education of consumers so that they can make informed choices. If communicated to empower and not scare, such education can encourage change.

WIIFM – What incentives could be applied to address the 'what's in it for me?' questions. Providing revenue incentives e.g. Pharma through prevention metrics along the pathway to treatments and demonstration that their products are more effective when needed, making it a Pharma problem, consumer desire to create demand, incentives for life insurance companies to maybe engage – these could all be discussed where the profit or savings can be demonstrated.

Who's driving – There are key stakeholder groups who need to drive change. Citizens, before they become patients, can drive change by being informed and have a desire to not want to get ill. Governments that need to reduce health costs, as evidenced by the introduction of the Inflation Reduction Act in the US, have a major need. Corporations and leaders in the biopharma and healthcare industry perhaps need change to meet their primary passion to defeat unmet medical need, believing doing the right thing will in the end deliver sustainable shareholder value.

The Budget Pot Challenge – Affording longevity is a problem for many, including healthcare providers, citizens, employers and governments. The challenge is that there is nobody owning the problem. No IP protection and no ownership of 'wellness reimbursement', translates to no investment and business model for biopharma companies or venture capitalists. The challenge is how to bring the two together, across multiple stakeholders, to address the eg £100Bn UK economy hit by obesity, through a reimbursement mechanism that provides value creation across an integrated wellness and healthcare system that removes siloed budget pots, provides ownership, and drives collaboration.

View the recording HERE or via

The Anatomy of a Great Dealmaker:

Mike Ward, Global Head of Life Science & Healthcare Though Leadership, Clarivate, has interviewed innumerable biopharma dealmakers who have been involved in some of the most transformative transactions in the sector. In a sector underpinned by partnering and collaboration, a dynamic to which dealmakers are pivotal, Mike led a discussion by dealmakers about dealmakers on what characteristics, influences and role models enable them to deliver:

Personality Traits – The dealmaker needs to retain humility in knowing their own limits and knowledge, be resilient with common sense, appreciate what both parties seek from the deal and communicate well. Formal qualifications such as a PhD or MBA may open the right doors, but there is no checklist when recruiting a dealmaker; personality, aptitude, excellent soft skills, and experience are key.

Multi-tasking vs Team – Context is important. Within a large corporate, one would expect a variety of expert colleagues to form a team with the deal lead, each bringing their expertise. In a small company, one person may be charged with overseeing all aspects, but being able to assemble a team, perhaps external in make-up, and manage the process well means team players skills are essential in both contexts. There is no given size threshold for when it is right to build in-house expertise rather than out-source, it just depends on context.

The attraction – A key factor is the desire for the dealmaker to want to be in that business development role. What attracts them is often the diversity of day-to-day, the challenge, interest in seeing the research translated, interacting with people, learning from colleagues, and the challenge to succeed.

Networking – Seen as key for a dealmaker, but some feel more comfortable than others. It is based upon the art of conversation, and techniques to help can be learned. Aspiring dealmakers should take every opportunity to grow their network, including being bold at events ('picking off the lone prey acceptable'), leveraging their current network and colleagues and consider seeking a mentor to help introduce them.

Metrics – How a dealmaker's performance is measured can be complex if delving deeper than simply deals signed and the financial success triggered. Leads identified, communication performance, keeping all stakeholders motivated and focussed to get to signature can all be assessed against expectation.

Be informed – It's impossible for one person to stay completely aware of all details in all aspects of the deal and due diligence, such as market sizes, clinical development routes, regulatory, and IP. The key for the dealmaker is to ensure they follow the trends well enough to seek more detail from expert colleagues or advisers at the right time. Staying informed is most often achieved through networking, complemented report reading, and subscription to commercial intelligence sources.

Efficiency – The best dealmakers are efficient in their approach, using all the tools available to get to the most desired leads, agreements, and signatures without distraction; staying focussed is the key. This applies to many of the other points covered, such as staying focussed in your networking goals; knowing your limits and when to buy-in advice, communicate to maintain momentum across all parties, and staying abreast of all the moving parts that need to get done for the deal to conclude.

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London BioScience Innovation Centre	**LBIC** provides a focus for life sciences activity in London, offering exceptionally high standard laboratory facilities and a professional front door that cannot fail to impress. https://www.lbic.com

Genesis 2023 Exhibitors

LINK-J Life Science Innovation Network Japan	**Link-J** partners with national and international organisations and companies to jointly host programs to facilitate collaboration in and out of Japan. https://www.link-j.org/en/
Medicilon	**Medicilon** is an integrated contract research organisation providing comprehensive one-stop services for pharmaceutical enterprises ad scientific research institutions around the world. https://www.medicilon.com/
CATAPULT Medicines Discovery	**Medicines Discovery Catapult**, part of the UK Government-funded network of Catapults, is an independent, not-for-profit organisation that industrialises and drives the adoption of new tools and technologies for the sector. https://md.catapult.org.uk/
NHS North East Innovation Lab Part of Newcastle Hospitals	**The North East Innovation Lab**, part of Newcastle Hospitals, and works with organisations, supporting the development of next-generation diagnostics. https://commercial.newcastle-hospitals.nhs.uk/our-services/innovation-lab/
PELAGO BIOSCIENCE	**Pelago Bioscience** is a Drug Discovery Research Partner supporting preclinical drug discovery and development with their unique core technology: the Cellular Thermal Shift Assay (CETSA® by Pelago Bioscience) https://www.pelagobio.com/
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Genesis Fringe: Online and In-Person

It is the case some things are created and developed by design from the outset. Sometimes however, activities develop through a serendipity and evolution that was not envisaged at the outset. 2024 will mark ten years since One Nucleus started delivering and supporting the Genesis Fringe. This is series of separate seminars and receptions across London that occur in the days around the main Genesis conference day.

Attended by those attending Genesis and non-attendees, the Fringe events add depth to the content and networking opportunities available to visitors. The Covid-19 pandemic, and associated restrictions saw events move to the virtual world. For One Nucleus, this prompted the creation of the online Innovation Workshops as part of our conferences which also ran on the days leading up to those main events.

Post pandemic, combining the best of both worlds, 2023 saw the most dynamic Genesis Fringe to date, with no less than eight satellite in-person events in London and a further eight online sessions accessible to all. Considering the other activities happening that week also, it would seem a form of 'London Life Science Week' has landed.

Delivering the Genesis Fringe Sessions were:

Appleyard Lees
Intellectual property law

BIO PRO
Baden-Württemberg GmbH

Blue Link
WORLDWIDE

Danforth
Advisors

CEBR
Council of European BioRegions

CANADIAN
TECHNOLOGY
ACCELERATOR

CELLUSION

CANADA–
UNITED KINGDOM
Chamber of Commerce

FLANDERS
INVESTMENT &
TRADE

GTM GLOBAL
business expansion

LINK-J
Life Science Innovation Network Japan

Mathys&Squire

mha
An independent member of
bakertilly
INTERNATIONAL

Marks&Clerk

Innovation Workshops 4, 5, 6 December 2023:

- Does the Early-stage Biotech Funding Landscape in the UK Need Disrupting?
- Advancing Innovation Through Clusters
- Innovation opportunities in Baden-Württemberg's Healthcare sector
- Getting the most from your IP Strategy
- USA Bio Invest – Coming to the USA
- Soft-landing Opportunities for Japanese Life Science Startups and the Ecosystems
- Listing in London – An Update on Activity from the London Stock Exchange
- Development of CECSi as an alternative to Corneal Transplantation for Patient Suffering from Bullous Keratopathy

Catch the recordings at YouTube

In-Person Fringe Events 5, 6, 8 December 2023:

- Breakfast Roundtable – How Are the Markets Working for Life Sciences
- A Smarter Way Into the USA
- A UK-China Approach to Developing the Right Strategies for Early-stage Life Science Company Growth
- The Good Housekeeping Guide to Dealmaking
- Innovation Through Collaboration: How Canada & the UK are Championing the Life Sciences
- Revolutionising Healthcare: AI Unleashed in Pharma, Medtech and IP
- Access to Patient Data & Genesis Welcome Reception
- Digitalization of Health and Social Care – Opportunities and Challenges in Canada and the UK

BioNewsRound Award Finalists

And the winner

PrecisionLife and LifeArc announce strategic MND/ALS partnership

BIO-EUROPE SPRING®

one nucleus

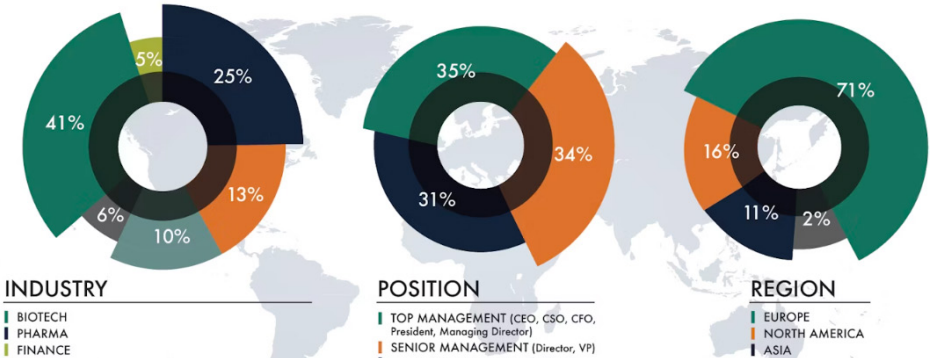
ONE NUCLEUS MEMBERS AT BIO-EUROPE SPRING 2024

Highlighting our members at BIO-Europe Spring 2024.

Barcelona, Spain

Our Collaborators at BIO-Europe Spring

We are returning to BIO-Europe Spring with an enhanced profiling opportunity for our members to help showcase them at this event in Barcelona, Spain on 18-20 March 2024. An unrivalled forum for companies across the biotech value chain to meet and do business.



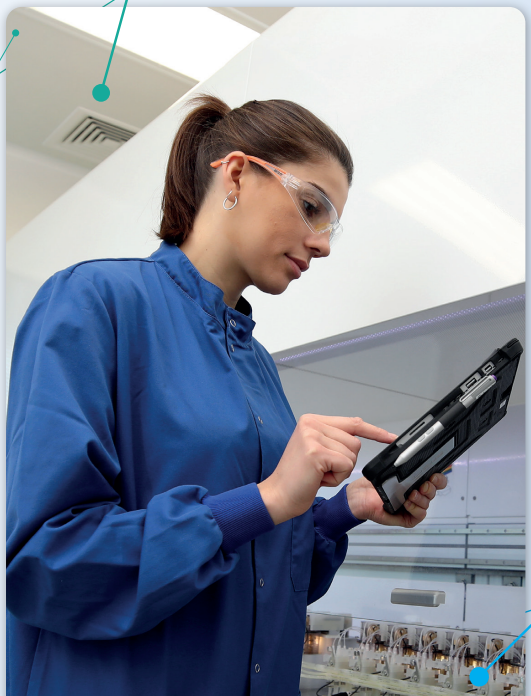
Who Attends?
Over the years, BIO-Europe Spring has become Europe's largest springtime partnering event. Its international reach makes it a one of a kind offering and gateway to the global life science community.

The event caters to the entire value chain, start-up and innovator educational programmes, industry trends and outlooks from KOLs, company pitches, partnering meetings as well as various networking opportunities. Read more on the BIO-Europe Spring website.

One Nucleus is proud to represent the following organisations at BIO-Europe Spring: Riverlabs, Ikarovec, DefiniGEN, Vernalis, CPI.

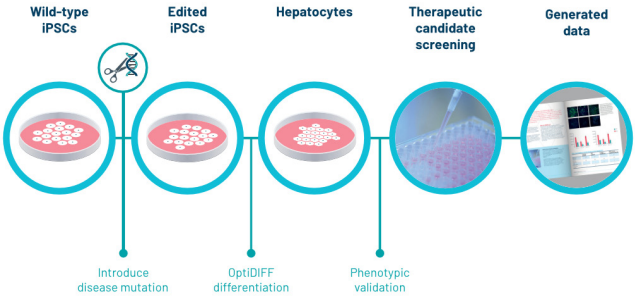
One Nucleus Members attending BIO-Europe Spring this year include: Abzena, Agility Life Sciences, Alloy Therapeutics, Alveron, Amphista Therapeutics Ltd, Antikor Biopharma, Apollo Therapeutics Limited, Asahi Kasei Pharma, Astellas Pharma Europe Ltd, Astex Pharmaceuticals Ltd, bit bio Ltd, Bristol Myers Squibb (BMS), Catalent Pharma Solutions, Cerevance Ltd, Charles River, Constructive Biology Ltd, DefiniGEN, Insmed Innovation UK Ltd, Ipsen Biopharm Ltd, Isogenica Ltd, Kadans Science Partner - Canary Wharf, LabConnect Europe BV, LabGenius, LifeArc, Medicines Discovery Catapult, PharmaVentures Ltd, PharmEnable, Quotient Sciences Ltd, Scendea Ltd, Shionogi B.V., Simcere UK Limited, SRG, Storm Therapeutics Ltd, Sygnature Discovery Ltd, Thermo Fisher Scientific, tranScrip, University College London (UCL), ValiRx Plc, Vernalis Ltd

Read on to find out more about the companies we are profiling on Stand 102B!



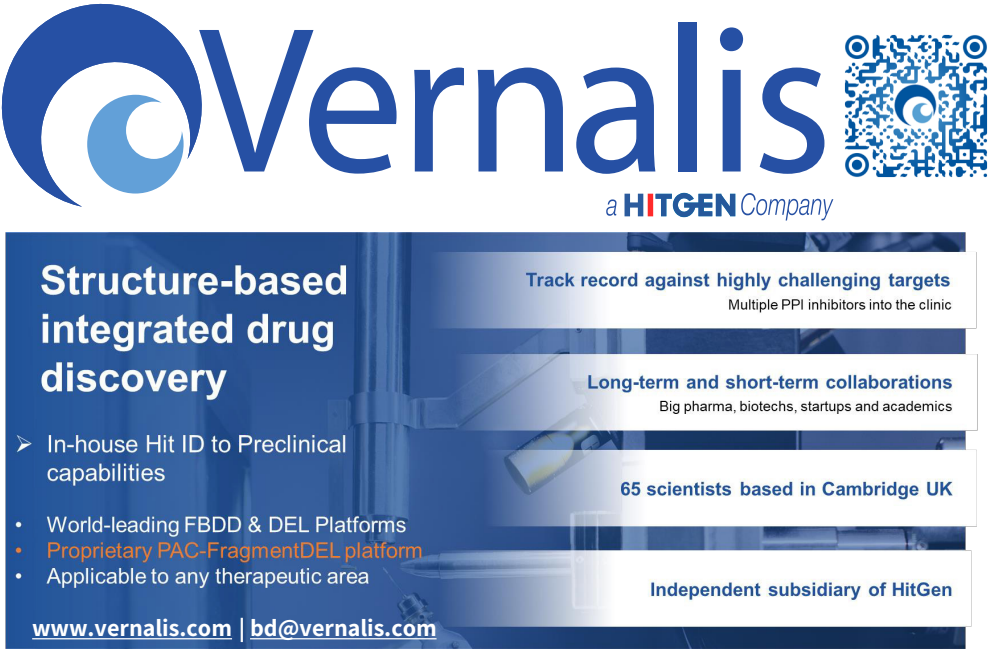
DefiniGEN is a game-changing company with a platform that enables the large-scale generation of iPSC-derived hepatocytes (Opti-Heps) with functional relevance comparable to primary human hepatocytes.
Opti-Heps successfully replicate all aspects of hepatocyte pathophysiology, allowing them to replace primary liver cells and hepatocellular carcinoma cell lines in many aspects of toxicity testing. This breakthrough provides scientists with a reliable and consistent supply of highly functional hepatocytes for the first time. By integrating CRISPR/Cas9 technology, DefiniGEN can replicate disease phenotypes catering to unmet needs for in vitro efficacy testing and enabling the study of previously inaccessible rare monogenic liver diseases.

Visit www.definigen.com



Ikarovec is a pioneering UK-based biotechnology company that has been operational since 2020 and is committed to the development of cutting-edge gene therapies to treat chronic eye diseases. Ikarovec aims to radically alter current standards of care for AMD patients that currently involve regular eye injections to deliver a one-time therapy that will lead to a meaningful and long-lasting improvement to patients' sight and quality of life. Ikarovec has selected two unique AAV-based gene therapies each with multiple therapeutic components to address the unmet clinical need and complex nature of AMD and is seeking Series A to support clinical evaluation.

https://ikarovec.com



INAPHAEA
BIOLABS

Inaphaea BioLabs is a translational contract research organisation, offering cell-based assays specialising in oncology and women's health. In the oncology space, we offer a range of patient derived cells (500+). These cells, across a range of cancers, are available for use in our services, including screening, or can be purchased under license from us for use in your own laboratories. Data associated with our biobank samples is also available for licensing for data applications or used for cell line selection. This includes RNAseq data, for select cell lines (300+), considering RNA expression levels across different cell types for a large number of genes.

Find out more on our website

康龙化成
PHARMARON

Driven by Science to Help our Partners Succeed

Laboratory Services

- Synthetic & Medicinal Chemistry
- Analytical Chemistry
- Computational Chemistry
- DMPK/ADME
- Biology & Pharmacology
- Safety Assessment

Chemistry, Manufacturing & Control

- Process Chemistry R&D
- Material Science
- GMP API Manufacturing
- Formulation Development
- Drug Product Manufacturing
- Commercial-scale Manufacturing

Clinical Development

- Radiolabelled Sciences
- Clinical Bioanalysis
- Clinical Pharmacology
- End-to-end Clinical Development
- Clinical SMO

Biologics & CGT

- Discovery Biologics
- Biologics CDMO
- CGT Lab Services
- GT CDMO

Pharmaron is a premier service provider for the life sciences industry that offers a broad spectrum of research, development and manufacturing service capabilities throughout the entire drug discovery, preclinical and clinical development process across multiple therapeutic modalities, including small molecules, biologics and CGT products. With state-of-the-art labs in China, the US, and the UK, we deliver unparalleled service and innovative drug products worldwide.

Contact us for more information

bd@pharmaron.com
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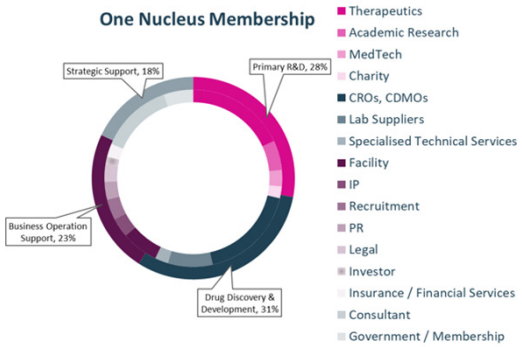
About One Nucleus

One Nucleus is a not-for-profit Life Sciences & Healthcare membership organisation headquartered in Cambridge, UK. We support institutions, companies and individuals in the Life Sciences sector providing local, UK-wide and international connectivity.

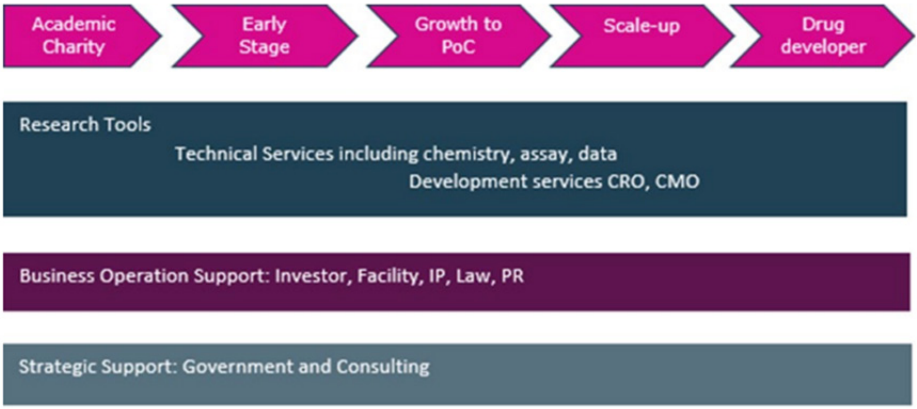
One Nucleus' mission is to bring the right people together to exchange knowledge and collaborate to enable the translation of great innovation into great products that markedly improve patient outcomes. Attracting and enabling the best people to engage is at the heart of what we do and what we continually strive to deliver.

One Nucleus has approximately 400 members concentrated (~70%) in the Greater Cambridge (East of England) and Greater London clusters. The One Nucleus membership base is diverse and represents the full breadth of the Life Science sector, each playing vital roles in the creation, protection, investment, infrastructure and accommodation of an ecosystem responsible for translating world class invention and innovation into patient benefit, economic development and returns on investment.



One Nucleus is committed to highlighting the crucial role that each component of the sector plays within the Life Science value chain. The value chain ranges from academic research to large drug developers and all stages and model of company in-between. Developing new health interventions requires technical services, business support and strategic support to enable innovation, scalability and success.

Accelerating the speed and efficiency of traversing the process from ideation to patient, as represented below, is the mutual goal of both One Nucleus our members and our collaborators.



One Nucleus as a Virtual Innovation Centre (VIC)

One Nucleus provides support to the Life Science ecosystem in key areas that are critical for innovation being translated into successful businesses, products and services. Delivery of such support can best be broadly conceptualised as One Nucleus acting as a virtual innovation centre. Unlike a traditional innovation centre, where the breadth and depth of the ecosystem and physical capacity is a factor, viewing One Nucleus as an innovation centre without walls encapsulates the immense spread of complementary excellence One Nucleus is able to facilitate access to for our members.

One Nucleus acts as a comprehensive ‘Sat Nav’ to experts who can advise on the dynamic challenges that innovators face throughout their translational research and business creation journey, all the way from their initial ideas to exit.

One Nucleus’ innovation support is delivered in a variety of forms through our Innovation Seminars, Events and Conferences, which offer comprehensive for peer-to-peer knowledge-exchange and operational support services around Savings, Learning & Development and Facilities Consultancy.

Key Services for Our Members
The diagram and text below outline the main elements of One Nucleus support included in One Nucleus membership.

- Raise Your Profile
One Nucleus members benefit from enhanced visibility to their target audience and from the opportunity to be seen in our network alongside approximately 400 other members. We do this through our online directory listing, website and social media channels, newsletters and speaker opportunities at our events.

- Connect with Our Network
A key advantage to any company joining One Nucleus is the ability to connect with you next potential partner, client or investor. Our diverse range of events and conferences is a key tool for enabling these interactions.

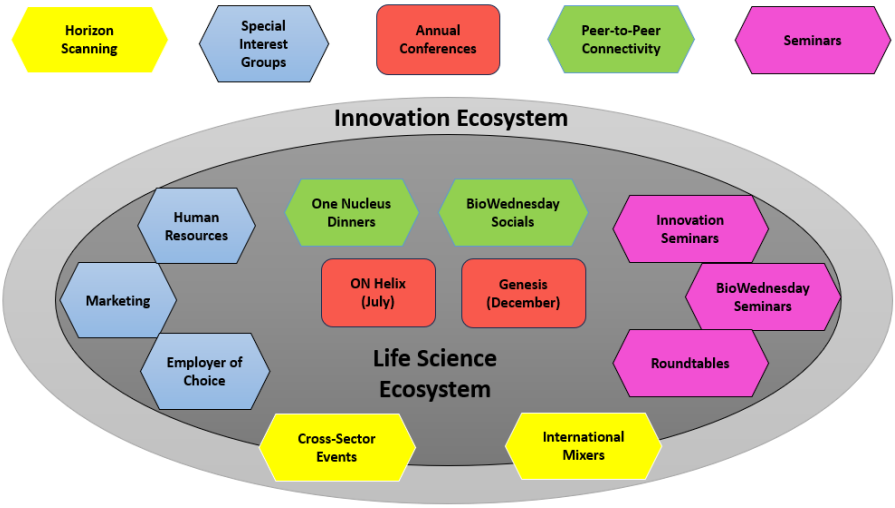
- Build the Best Teams
We help our members to develop and grow their teams through our focussed Life Science training portfolio (offered to members at a discount), Lunch & Learn series, Building Life Science Adventures (BLSA) panel discussions, mentoring initiative and One Nucleus jobs page. The One Nucleus Training Sat Nav is the most recent initiative that One Nucleus has created to help life science companies identify training opportunities for staff efficiently.

- Discounts for Members
One Nucleus members make significant savings through a Group Purchasing Scheme and Member-to-Member Marketplace, as well as numerous discounts across third party conferences and member discounts on facility management.

- Finding and Running a Lab
We support our members with their innovation by reducing the cost on fit outs and related services, as well as saving them time on finding the right lab space.

- Access Innovation Support
We help our members to become operational in areas such as raising investment, building partnerships, recruitment, establishing facilities or accessing business support by providing access to the right experts, further resources and One Nucleus services.

Peer-to-peer Knowledge Sharing via One Nucleus Events and Conferences



The portfolio of One Nucleus events is completely structured around encouraging the key conversations, meetings and knowledge-sharing that enables deal flow whether those deals are investment, R&D collaborations, service procurement, recruitment or other. Membership is the most efficient and cost-effective option to engage with our network via our events.

Conferences: The focal points of the One Nucleus calendar are our Genesis and ON Helix conferences. With discounted rates for members to attend, these larger events are an ideal opportunity to contribute, raise profile, and network at scale with the One Nucleus ecosystem.

Seminars: These may be built in partnership with third parties or One Nucleus led. They can include a mix of presentations and/or debate around key technical or business challenges in the sector. These sessions are often free to attend and may be invite only, apart from our BioWednesdays which are free to One Nucleus members and chargeable to non-members.

Peer-to-peer Connectivity: Our Regional Life Science dinners started in 2022 and have demonstrated a very successful track record of bringing a targeted group together for informal networking to nurture relationships. Our BioWednesday Socials are an opportunity for members and non-members to come together to network, often in the bar where many new ideas are forged!

Special Interest Groups: Our SIGs enable peer-to-peer interactions on topics such as HR, Marketing and Employer of Choice. They are an ideal opportunity to connect with those in similar roles to discuss solutions to shared challenges and stay up-to-date on current regulation. Discounted to members.

Horizon Scanning: Facilitating the convergence of our network with complementary networks from related technology fields or different geographies is an effective means to help our members build those all-important new relationships. One Nucleus maintains strong collaborative relationships with a multitude of such groups, both across the UK and internationally.

One Nucleus Membership Benefits

Benefit	Gold	Silver	Non-Member
Listed in Online Membership Directory and in Biotechgate Directory	Yes	Yes	No
Post News, Jobs, Events to website	Free	Free	£75 + VAT
ON Helix	£325 + VAT	£435 + VAT	£545 + VAT
Genesis	£325 + VAT	£435 + VAT	£545 + VAT
BioWednesday Seminars	Free	Free	£50 + VAT
Innovation Seminars	Free	Free	£50 + VAT
Special Interest Group	Free	£30 + VAT	£50 + VAT
Training Courses	30% Discount	15% Discount	List Price
Facilities Management Consultancy (Day Rate)	£810 + expenses + VAT	£980 + expenses + VAT	£1,155 + expenses + VAT
Preferred Supplier Discounts	Yes	Partial	No
Savings on Third Party Industry Events	Yes	Yes	No
Access to Member-to-Member Marketplace Discounts	Yes	Yes	No
Banner Advert in eNews	£500 + VAT	£500 + VAT	£800 + VAT

prices subject to change

Become a One Nucleus Member

Along with bespoke Corporate Sponsorship and Partner packages, we offer two levels of membership – Silver and Gold. Silver Members receive a range of entry-level benefits. Gold Members enjoy an enhanced range of exclusive services and benefits. Follow these five simple steps to become a One Nucleus member...

One Nucleus Website
Visit onenucleus.com/membership to check if your organisation is already a member and/or apply for membership.

Sign-in / Create Account
To join One Nucleus, you must be logged in to our website. If you are a first time visitor you will be prompted to create an account.

Apply for Membership
Enter your company details including the company size (number of full time employees) and select the level of membership that you would like to apply for (Silver or Gold).

Submit Application
Once you have submitted your application, the One Nucleus accounts team will review and contact you directly regarding next steps and payment.

Welcome to the One Nucleus Ecosystem
Once your application has been approved and paid for, you can now enjoy the benefits of One Nucleus membership!

Find The Right Course For You And Your Team With Our

ONE NUCLEUS TRAINING SAT NAV

Are you...

Congratulations to the Winners of the Boston Bootcamp 2024 Competition!

The Boston Bootcamp is a competition aimed at supporting UK companies looking to expand to the MA cluster. Following an exciting Presentations Session on 20 February where all finalists presented their pitch the 4 winners Carocell Bio, Enhanc3D Genomics, Ikarovec and Storm Therapeutics were announced. The benefits of the programme will include:

- Return Premium Economy Flights LHR-BOS
- 3 nights hotel stay
- UK preparatory sessions by life science experts with Greater Boston experience
- Participation in One Nucleus Boston Bootcamp on 22-25 April 2024
- Free delegate place at the One Nucleus Annual Awards Dinner 2024
- Participating in a keynote panel session at ON Helix 2024

The programme out in Boston will cover the following themes:

Introduction to MA Ecosystem and Navigating the Landscape • An overview of the MA Life Sciences sector • How to navigate the ecosystem • Support available to UK companies seeking to do business in MA	**Best Operational Strategies** • Intellectual property • Facilities leasing • Building your leadership team • Reporting compliance
Raising Investment • Financing landscape • Business model and value proposition • Terms sheets • Due diligence	**R&D Collaborations** • Identifying a MA-based partner • Pitching to partners • Working with academia • Clinical trials

Sponsored by:

200+
Exclusive attendees celebrating achievements in our ecosystem

8 Awards
Recognising all stakeholders

Innovation Equity
Accessible, Inclusive, Sustainable Solutions

With the sustained success of the sector, it feels appropriate that with there now being such critical mass One Nucleus should launch an annual awards dinner recognising success in its community. For the first time, life science innovators, companies and their supporters were able to enter to celebrate their success in one or more categories of the One Nucleus Annual Awards.

The One Nucleus Award categories are structured to reflect that breadth of R&D, investment, service provision and enabling support across the entire One Nucleus ecosystem. “Performance of the Year” being recognised in each category as follows:

- Primary R&D Company
- Investor
- Provider
- Technology Transfer Office
- Professional Services Company
- Facilities Provider
- Non-Profit Research Organisation
- Non-Profit Life Science Innovation Enablers

Good luck to all the finalists!

Visit www.onenucleusawards.com

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Sciad
Bringing innovation to life

ON Helix and Genesis in 2024

ON Helix and Genesis offer a high content mix of plenary talks and panels from key opinion leaders; 1-2-1 Partnering; an Exhibition assembling an array of providers supporting the life science sector and ample networking opportunities. The topics covered are those that our network wants to engage with and can contribute to.

New Horizons for Bio Innovation

As the Life Science sector constantly evolves, introducing new methods to develop treatments and therapies for improved patient outcome, new translational challenges arise that require collective input to address. The annual ON Helix conference explores the key R&D developments helping to guide the advancement of research and technology to deliver on the sector's vision of better patient treatments for all.

ON Helix 2024 will explore a range of significant challenges currently shaping our sector including: emerging modalities; biomarker technologies; techbio and the importance of connecting globally for innovation.

Visit www.onhelix.com

Maximising Returns from Life Science Innovation

Genesis is the annual conference that brings together the One Nucleus network of key industry leaders and innovators to reflect on the past year's most significant innovation trends, as well as dealmaking, corporate and patient considerations. The 2024 Keynote Programme at Genesis will explore how innovation in life science R&D, technology, dealmaking and leadership are translating innovation into patient, economic and sustainability benefits with value creation for all stakeholders.

Visit www.genesisconference.com

Shooting for the Stars in Life Science Innovation

By **Jasmin Bannister,** Business Development Manager, One Nucleus

A successful life science ecosystem is much like a spaceship shooting for the stars; there may be many moving parts but none are redundant, if they didn't serve a vital function they wouldn't be there.

Bringing the best people together is key to translating great innovation into great products that markedly improve patient outcomes and drive economic development. One Nucleus is at the forefront, driving both local and international connectivity. This article reflects on the impact of the life sciences ecosystem and our valuable members.

The One Nucleus community is multifaceted, as demonstrated by the visual shown here. There are various benefits of being visible on this colourful wheel, but what each organisation shares is impact of its vital role in supporting the life sciences value chain. The value chain ranges from public research to large drug developers with all stages of companies in between. In order to discover and develop new therapies, they require the highly specialised technology providers, technical services, business and strategic support services that enable innovation, growth and success. The Membership Directory Search feature facilitated by Biotechgate, is a powerful tool for understanding and presenting the strength of our network.

2024 sees the delivery of new initiatives such as the Annual Awards Dinner, Boston Bootcamp, and Training Sat Nav, to support our members and the wider network with achieving their goals whether it be:

- increasing visibility
- savings
- making connections
- learning and development
- championing success

As our members' needs change, One Nucleus consistently strives to adapt to be the best it can be for our sector.

Shooting for the Stars in Life Science Innovation

Recognising the benefits that international connectivity can provide locally, membership of groups such as CEBR, strong collaborations in North America and Asia with like-minded associations and having EBD Group as a Partner is important to One Nucleus helping our members. The Boston Bootcamp will play a key role in supporting our members expand their reach, gain valuable new connections and insights, supporting them in their innovation journey. More details in the 'Building Bridges in Life Sciences' article co-authored by my colleagues Alicia Gailliez and Tony Jones.

Working with EBD Group, One Nucleus looks forward to supporting its members again this year at Bio Europe Spring 2024. Events such as Bio Europe and BIO are excellent opportunities for life science communities to grow their networks. One Nucleus members can receive significant discounts on registration or join our Enhanced Profiling Opportunity at Bio Europe Spring 2024 for increased visibility and presence without breaking the bank.

The 'Advancing Innovation Through Clusters' Innovation Workshop at Genesis 2023, promoted the benefits of exchanging good practice on supporting life science and healthcare companies between various bio clusters in Europe to enable success. The session was hosted in collaboration with CEBR (Council of European BioRegions).

Whilst One Nucleus offers a portfolio of first-class formal courses, there is an array of technical and softer skills that staff may need to develop outside of that portfolio. Yet the portals through which to search for such training provision can be limited. To address this, the Training Sat Nav will clearly identify opportunities for people to develop specific skills relevant to their career development; reflecting the needs that we are made aware of by our member companies and the onward research to identify credible providers of such courses.

In conclusion, One Nucleus is more than just a membership organisation; we are passionate about supporting and celebrating each and every remarkable individual and organisation within our community.

Unveiling New Horizons in Paediatric Medicine Through Engagement

By Alicia Gailliez, Business Development Manager, One Nucleus

In a study by Sedova et al., entitled 'Do those who talk more learn more?', increased participation correlated with improved learning outcomes, which supports the importance of active engagement (as opposed to passively listening for example) in learning. In a technical field such as Life Sciences however, it can be equally important for audiences to be able to absorb sufficient data and insights to be able to have an informed and valuable panel discussion. The balance of format is likely role and topic specific, or perhaps Epictetus, the Greek philosopher was correct in his assertions that we have two ears and one mouth for a reason. The goal for One Nucleus events is not to essentially suggest there is a one-size fits all for knowledge-sharing, whether in the auditorium or during the networking, but to create an environment where data, insights and challenges are transferred between speaker and listener with each having engaged intellectually in the process. For an event such as ON Helix, keynote presentations are complemented by panel discussions that probe the trends, challenges and solutions in translational research, whether those areas are technical, financial or infrastructural. This format, whilst setting the scene via informing presentations, results in a dynamic mix of viewpoints being aired and a more conversational style to enhance all participants' learning under the Sedova hypothesis above.

An example of such a panel discussion that took place at ON Helix 2023 is summarized below on the topic of 'New Horizons in Paediatric Medicines'. The panel looked at what is helping to encourage more investment into paediatric medicines and what are the tools that will help us to serve this population better.

Photo credit PHandB.com

Expertly moderated by Paul Branthwaite (tranScrip), who was joined by Anna Moore (NIHR BioResource), Beatrice Panico (Scendea) and Becky Birch (The Brain Tumour Charity).

Paediatic drug development is a tough space to be in. Many childhood diseases are often severe and life-threatening and poorly addressed in terms of effective therapeutic interventions. The approach by the panel was to perform a SWOT analysis particularly focusing on identifying the strengths of the UK, in terms of providing resources to researchers, examining the regulatory hurdles and highlighting opportunities in the field.

Beatrice and Anna highlighted the uniqueness of the paediatic population. For example, they are not a homogeneous group and therefore, a tailored drug development strategy is needed, taking into account different developmental stages as well as differences in physiology and psychology between perinatal, neonatal, infant and adolescent stages. Unwanted side effects are currently an issue as highlighted by Becky who has witnessed this in numerous paediatric brain tumour cases where developers have gone down the repurposing route.

Unveiling New Horizons in Paediatric Medicine Through Engagement

In this instance, even when the disease itself is cured, there can be long-term debilitating side effects.

On the regulatory perspective, the importance of engaging both patients and caregivers in paediatric drug development strategies and clinical trial planning was highlighted. Beatrice pointed out that some regulatory bodies require documentation of this engagement as part of the clinical trial application process. The consensus was that successful patient engagement goes beyond a mere tick-box exercise. It necessitates meaningful involvement of patients and families and the development of engagement activities with input from patients. Paul raised the use of Paediatic Investigational Plans (PIPs), which must be submitted to the MHRA to support a medicine's authorisation for use in children. This serves as a good example of an approach that goes beyond a check-box mentality.

Paediatric drug development faces a significant hurdle when dealing with small patient populations. Beatrice talked about adaptative trial designs which can be used to overcome this challenge through allowing information gathered during the trial to be used to make statistically robust adjustments to modify the trials' course. Becky substantiated this approach referring to neuro-oncology in paediatrics, where limited patient numbers are available for clinical trials due to a lower incidence rate. In cases where adaptative trials are implemented, Becky stressed the value of a collaborative network, such as that in the UK, for sharing outcomes. This is especially valuable if the trial does not achieve the desired level of success, and to avoid repeating similar mistakes in future.

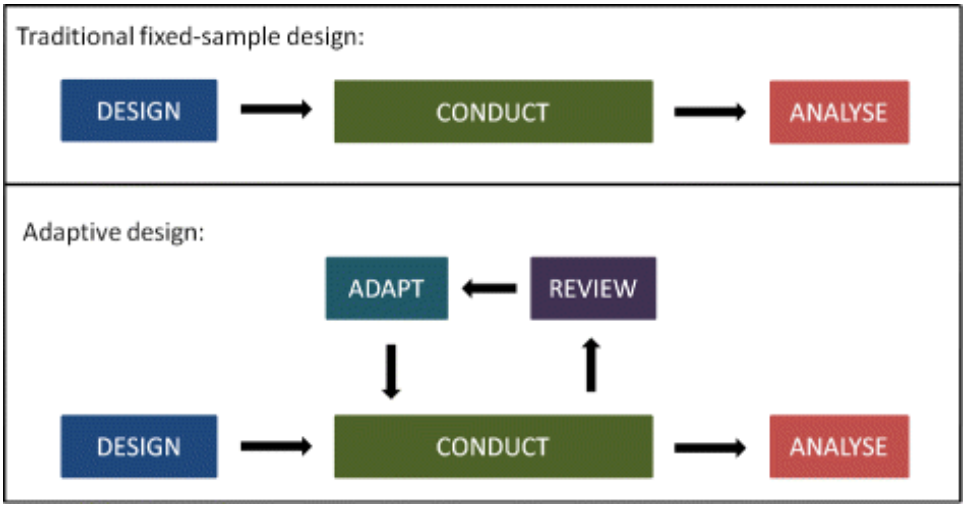

Photo source: Pallmann, P., Bedding, A.W., Choodari-Oskooei, B. et al. Adaptive designs in clinical trials: why use them, and how to run and report them. BMC Med 16, 29 (2018). https://doi.org/10.1186/s12916-018-1017-7

Unveiling New Horizons in Paediatric Medicine Through Engagement

An audience member raised a question about the significance of genetics in paediatric clinical research, indicating that it was a topic of considerable interest. A point that aligned fully with the panel's initial plan. Anna highlighted the Next Generation Children study, a Cambridge-based study run by Lucy Raymond, Professor of Medical Genetics and Neurodevelopment at the University of Cambridge and Honorary Consultant in Medical Genetics at Cambridge University Hospital, UK. The study found one in four children in intensive care had a genetic disorder, leading sometimes to a change in the treatment plan. The NIHR BioResource is building a game-changing repository of genetic profiles from willing pediatric participants, providing a transformative resource for research.

The lack of approved medicines for rare pediatric conditions underscores the need for more evidence-based therapeutics. Currently, orphan medicines benefit from ten years of market exclusivity once they receive a marketing authorisation in the European Union (EU). It is clear that regulators remain open to innovative approaches, such as gene therapy. A good example is how the use of a gene therapy administered via eye drops successfully regained the vision of Antonio Vento Carvajal, a teenager born with dystrophic epidermolysis bullosa, an uncommon genetic disorder. The treatment was developed by Pittsburg start-up Krystal Biotech.

Paul highlighted challenges associated with recruiting patients for paediatric trials, which can be more difficult due to parental reluctance. Beatrice discussed a potential avenue for overcoming challenges in paediatric therapeutics development by exploring the use of Machine Learning (ML) models in clinical trials. She described a validated methodology that uses ML tools to analyse patient data, thereby creating predictive patient trajectories. This means that patients can effectively serve as their own controls for comparison and limited participation becomes less of an issue.

The importance of good quality public involvement, including young people and those from underserved backgrounds, was identified as a critical aspect to effective trial design. Paul turned to the audience asking them to reflect on their own challenges in this space and encouraged everyone involved to think about accessing various resources including: Connect4children network; NIHR Clinical Research Network and NIHR BioResource D-CYPHER.

Unveiling New Horizons in Paediatric Medicine Through Engagement

To address the challenge of co-creation involving young people and parents, the University of Cambridge and Anna Freud have recently launched CA:RING. This provides access to a diverse community of children and parents who are eager to support paediatric research though patient involvement activities. It has been designed to safely manage recruitment and safeguarding concerns that are often raised as barriers to involving young people in trial design.

Professional facilitators are available to work with research groups and run co-design workshops, making feedback on paediatric trial design much more accessible for academia and industry. The initiative has been created in partnership with the University of Cambridge, Anna Freud and NIHR Applied Research Collaboration East of England, funded by Cambridge BRC, NIHR BioResource, UKRI and Alan Turing Institute.

An audience member posed a question regarding progress in advanced therapeutics in light of the recent success with the first gene therapy approved for Duchenne Muscular Dystrophy. This question prompted considerations for dosing and off-target effects. Several important points were raised by the panel, including the need for longer/lifelong follow-up after administration of advanced therapeutics; strategies to mitigate potential risks; and the value of initiatives such as D-CYPHER which provides access to pre consented data and can address some of the issues around long-term follow up.

In conclusion, the ON Helix panel discussion “New Horizons in Paediatric Medicines” took the audience on journey of the challenges and opportunities in this field. Overall, the discussion emphasised the importance of tailoring the drug strategy for the paediatric population, collaborative patient engagement and the value of emerging approaches such as adaptive trial design, genetics, gene therapy and ML for progress in this field.
We would like to extend our thanks to all panellists and moderator for their valuable contributions.

Click here to watch the recording from 6 July 2023.

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