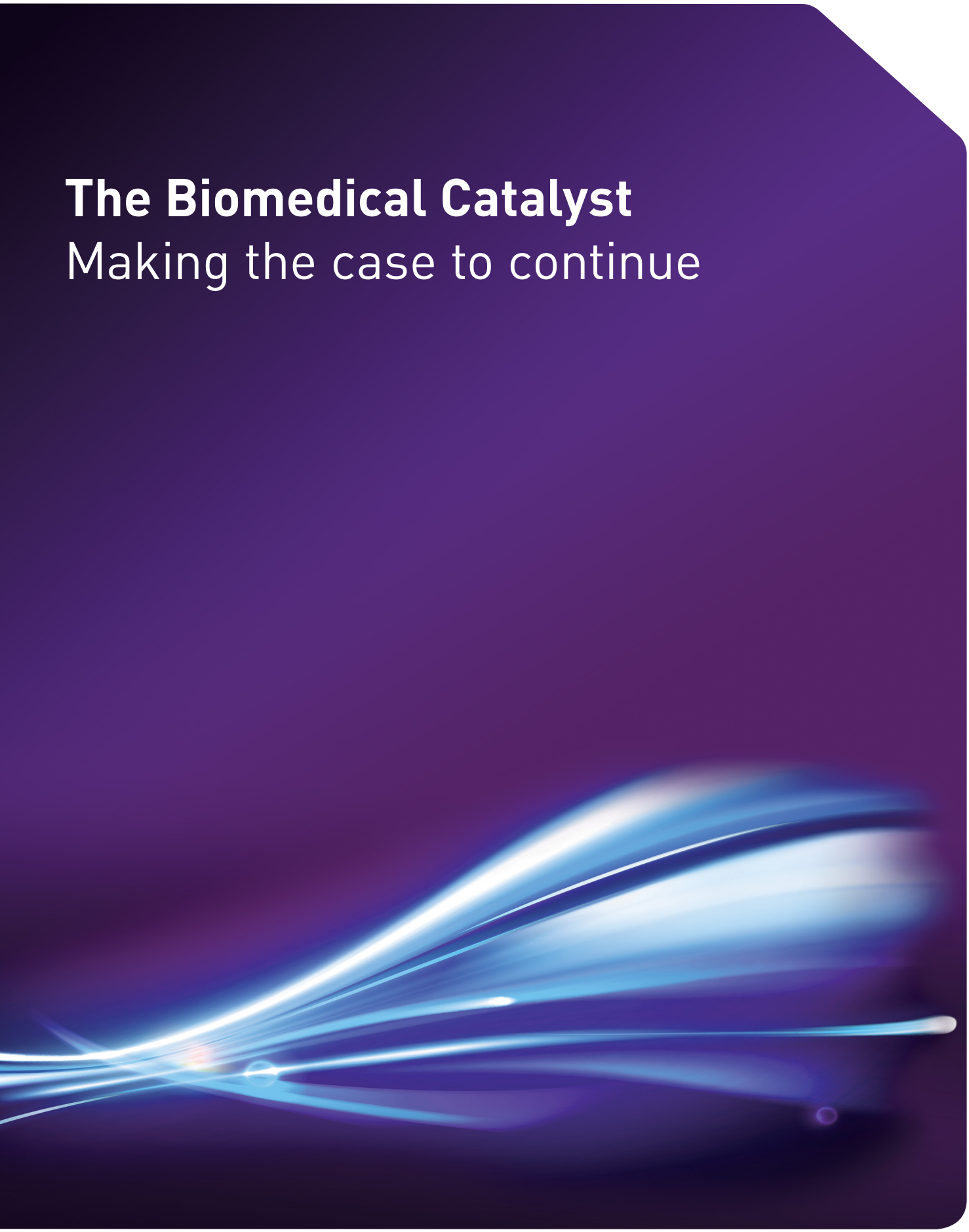
bionow® Bio Partner MediWales one nucleus BIA UK BioIndustry Association

The Biomedical Catalyst

Making the case to continue



Executive Summary

Since the Biomedical Catalyst (BMC) opened for applications in spring 2012, the scheme has made a game-changing impact on the ability to translate innovative scientific ideas into tangible products and therapies that can help patients in practice.

Launched by Prime Minister David Cameron in 2011 with *"the explicit aim (of) getting the best ideas through the proof of concept stage so we can get them into clinical development",* the joint Innovate UK and Medical Research Council (MRC) programme has made available funding to innovative small and medium sized businesses (SMEs) and researchers looking to work either individually or in collaboration to develop solutions to healthcare challenges.

To date the scheme has awarded over £250 million to accelerate medical research. Over 180 business-led projects have been supported with funds worth over £130 million and with a total project value of over £240 million. This means that over £100 million of additional private capital has been raised through the fund for project specific work. This does not include the additional private capital raised beyond funds linked specifically to those projects.

As the 2015 Spending Review approaches, the UK BioIndustry Association (BIA) publishes this report, alongside its United Life Sciences partners Bionow, BioPartner UK, MediWales and One Nucleus, to set out why the BMC scheme must be refilled and committed to in order to sustain the UK life sciences sector.

Enclosed within this report are 10 case studies, drawn from companies represented by the United Life Sciences partnership. They are but a snapshot of the full output of the investment made through the BMC but give an insight into the type of life-changing innovation that has already occurred during just three years:

1. Investment into the development of vaccines to prevent and treat bacterial infections, including MRSA (**Absynth Biologics** page 6).
2. Support for the development of technologies to formulate novel, patient-friendly, high doses of current medicines used to treat cancer and arthritis (**Arecor**, page 7).
3. Facilitated evaluation of a compound already shown to be effective in improving lung function and quality of life for asthmatic patients, to see how it might apply to severe forms of eczema (**Atopix Therapeutics** page 8).
4. Enabled exploration of a company's novel ion channel mechanism in tinnitus and schizophrenia (**Autifony Therapeutics**, page 9).
5. Investment into the discovery of new commercially viable antibiotics to treat bacterial infection (**Discuva**, page 10).
6. Support for a trial of a new test to confirm correct and safe nasogastric tube placement, with the potential to remove the need for costly and unpleasant chest radiography for patients (**Ingenza**, page 11).
7. Investment into the development of a series of novel treatments for rheumatoid arthritis, including a drug that has the unique potential to directly protect bone from the damage caused by the immune system (**Modern Biosciences**, page 12).
8. Support for the development of an orally administered drug that has the potential to treat all patients with the fatal genetic disease Duchenne Muscular Dystrophy (**Summit Therapeutics** page 13).
9. Support for new therapies to treat unmet clinical needs in cancer and ophthalmology, including the leading cause of blindness in the developed world (**Vasgen**, page 14).
10. Enabling examination of a promising compound in the treatment of inflammatory skin disease (**Ziarco**, page 15).

This snapshot of the outputs of the BMC scheme showcase the array of activity underway to deliver against unmet medical needs where the potential to both deliver patient outcomes and positively impact the health service's ability to deliver are clear.

Although the range of issues that BMC awarded projects address is wide and varied, as these case studies demonstrate, there are some benefits that are common across the scheme including:

1. **The ability to leverage private investment**: The scheme enables significant private capital, in the form of matched funding, to be leveraged as a condition of the grant award. To date this has totaled over £100m for the funded projects allowing them to go further, and providing a higher level of return, than would have been possible with public funding alone.
2. **BMC award acts as a badge for further investment later on**: In addition to leveraging match funding for the specific BMC-backed projects, the stories here alongside others, also demonstrate how the BMC can act as a "badge of honour" for further investment with investors viewing a BMC award as a compelling green light behind the underlying science and technology of the business. Post-award funded companies and academics have realised in excess of a further billion pounds in the form of additional private finance, grant funding, via licencing or through acquisition. Several companies highlighted here including Autifony Therapeutics, Discuva and Modern Biosciences illuminate where scientific innovation has occurred through investment, which would not have happened without BMC funding.
3. **Nurtures innovation from both academic and commercial sectors and facilitates collaboration between the two:** The way in which the scheme has been established allows collaborations to be easily considered and the enclosed case studies show a range of ongoing and evolving collaborations even beyond the scheme. For example, following the completion of a BMC-backed project undertaken by Absynth Biologics to evaluate vaccines to prevent different types of bacterial infection, the *Streptococcus pyogenes* vaccine is now the subject of an MRC Collaborative Awards in Science and Engineering (CASE) studentship between the University of Sheffield and Abysnth Biologics starting in September/October 2015.
4. **Supports wider collaboration within the industry:** Case studies such as those from Arecor and Atopix Therapeutics demonstrate how BMC-backed schemes are leading to new collaborations across the life sciences ecosystem, opening up new partnerships between biotech and pharmaceutical companies.
5. **Creates jobs:** BMC awards also allow for the possibility to create high-skilled jobs which would not have occurred otherwise. Here Vasgen, Ziarco and others demonstrate this.
6. **Has an impact throughout the UK:** BMC money has been invested throughout the UK as illustrated by the infographic on page 5. With over 1100 companies represented through the United Life Sciences partnership, there is great future potential reach of the scheme.
7. **Can have a transformative effect on a business:** BMC funding can truly transform a company. For example, the story of Modern Biosciences included here, where a £176 million option and licensing deal with Johnson and Johnson's Janssen pharmaceutical division would not have likely occurred without the initial developments BMC funding supported. Or Ingenza, where without BMC backing, their innovative product which was slightly tangential to their main company focus would have likely been shelved as there was no obvious route to develop the product further.
8. **Makes a real difference to patients in need**: Most importantly of all, the BMC scheme is supporting innovation that can save lives. One need only reflect on stories included here such as Summit Therapeutics' work on Duchenne Muscular Dystrophy or Autifony Therapeutics' strides to deliver desperately needed drugs to treat schizophrenia to understand this.

We publish this report with a strong and clear message: the Biomedical Catalyst scheme is a successful government policy that is underpinning the ongoing growth of the UK life sciences sector and it must be continued.

The cyclical investment environment of 2015 differs to that of 2011 with a small number of developed UK bioscience companies recently able to deliver Initial Public Offerings (IPOs) in the last couple of years. However the BMC fills a crucial structural gap in the UK investment pathway earlier in company development where private sector investors will not venture alone.

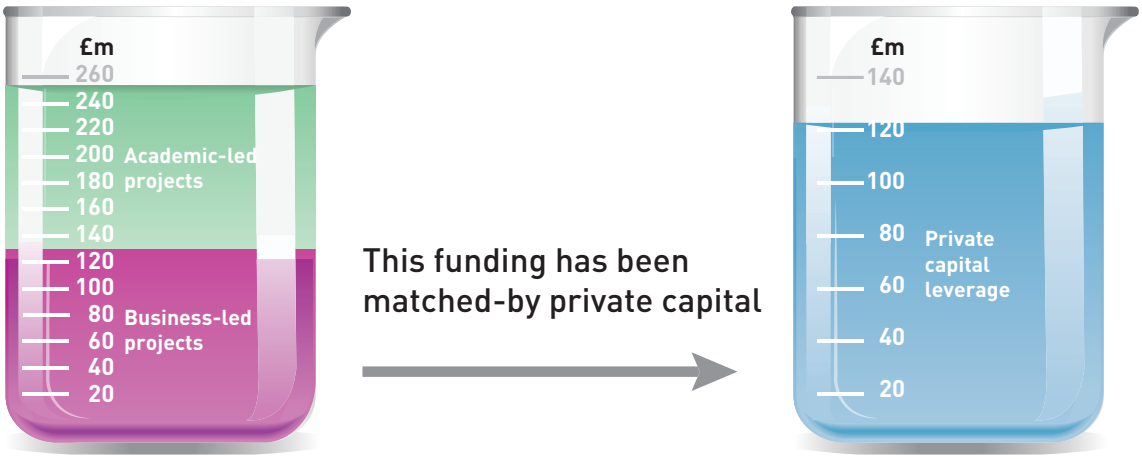
Other markets such as the US and Germany take technology closer to market through specific innovation and funds. If the UK fails to sustain this mechanism that de-risks a promising innovation to a stage that Venture Capital and other forms of financing will come in, then the UK stands to lose its position as Europe's leader in this global and innovative industry.

Whilst we see other areas of market weakness in the sector, such as the need for further support for scale up financing to build and sustain a strong tier of mid-size companies here in the UK; this is an additional and complementary need not a substitutional one.

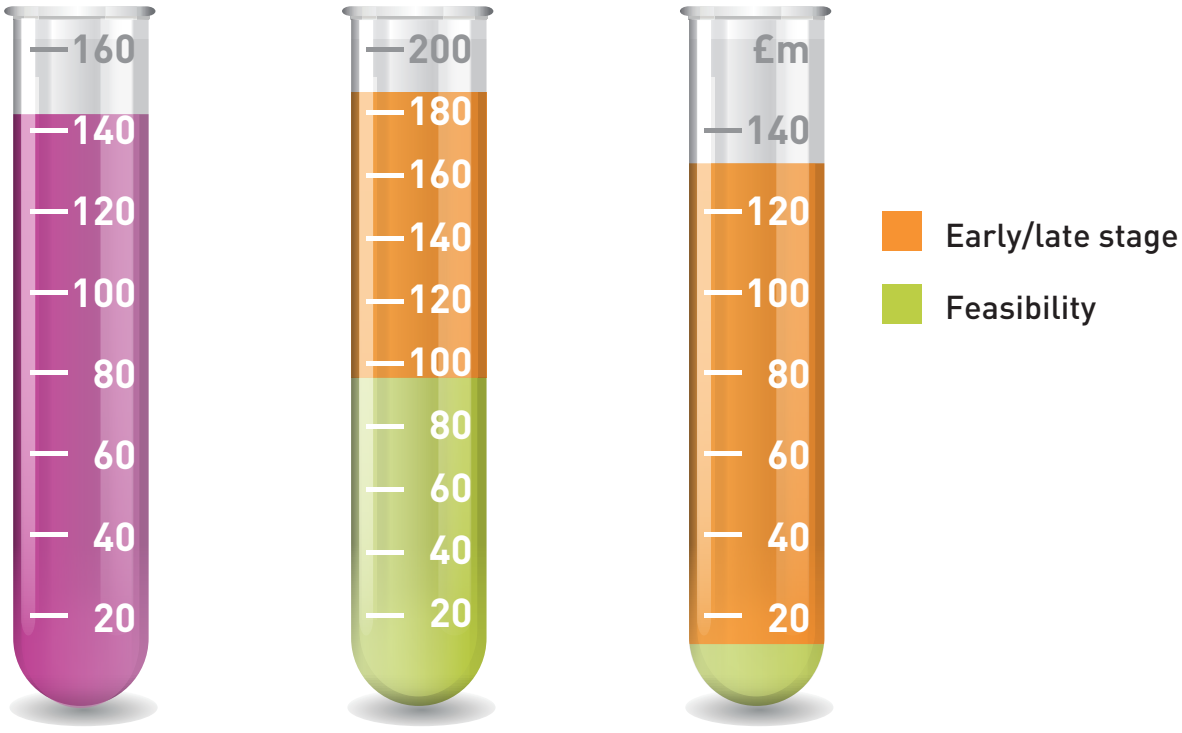
The bioscience sector requires a sustained funding ladder where promising innovations and companies have the most optimal environment from start up to liquidity. The Biomedical Catalyst scheme has proved to be an integral rung on that ladder to achieve the objectives of the current Life Sciences Strategy. If it is taken away or made less secure, already invested SMEs will fall again into the funding valley of death and the whole life sciences ecosystem, and UK economic growth will suffer.

The Biomedical Catalyst: a snapshot of

Total Award



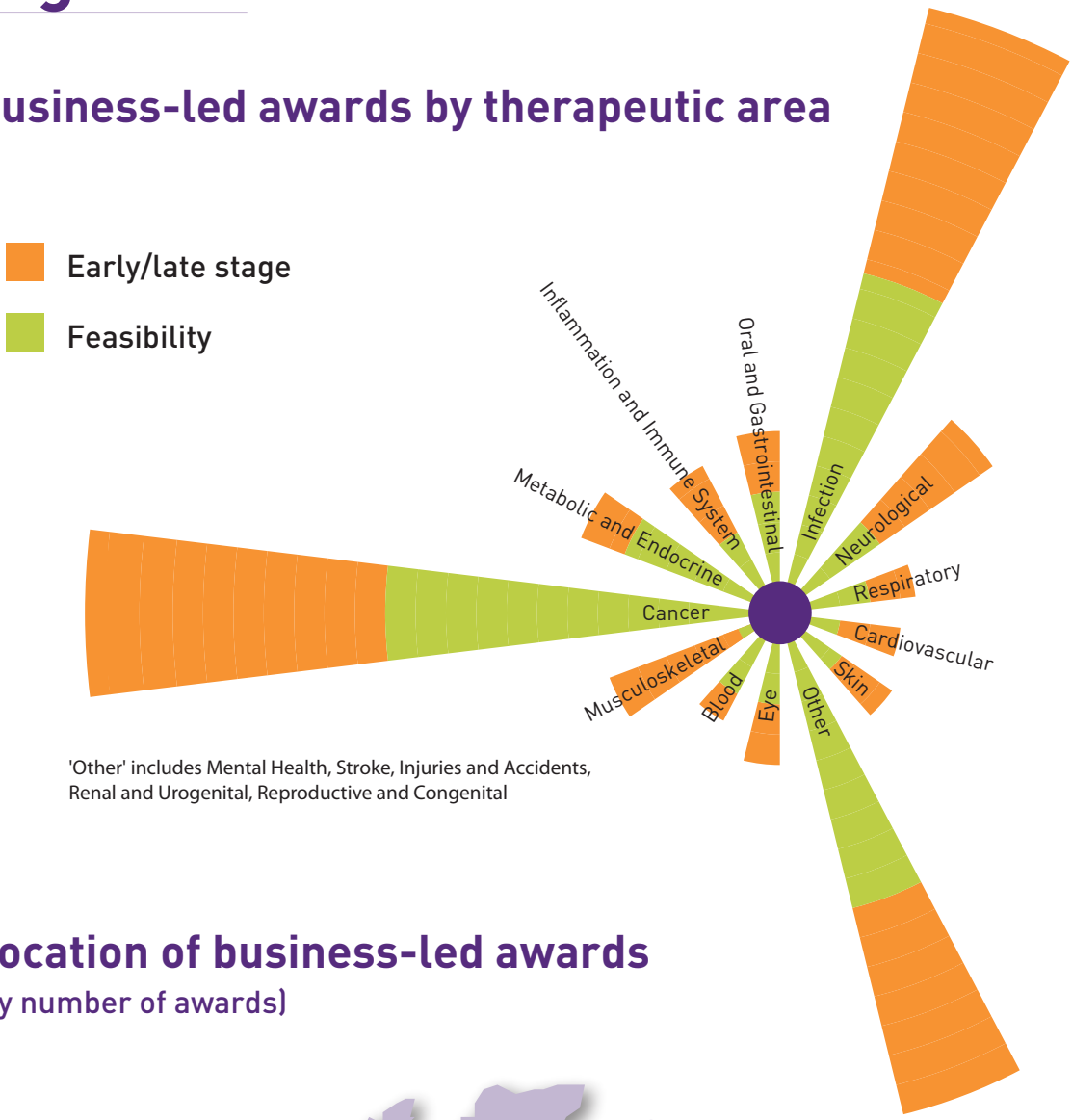
Business-led awards by amount



1. Data supplied by Innovate UK and the Medical Research Council up to and including Round 8 and accurate as of time of publication. Information on award totals is subject to change. Not all awards have been finalised and may be subject to certain requirements being met.

key figures[1]

Business-led awards by therapeutic area



'Other' includes Mental Health, Stroke, Injuries and Accidents, Renal and Urogenital, Reproductive and Congenital

Location of business-led awards

(by number of awards)

Dr Fiona Marston
Chief Executive
Absynth Biologics

Absynth Biologics Alderley Park North West England	ABsynth Biologics™
ROUND 1, FEASIBILITY	**GRANT:** £150 000 **LEVERAGED:** £51,103
ROUND 3, EARLY	**GRANT:** £2,013,706 **LEVERAGED:** £1,035,313
Development of a novel, effective vaccine for *Staphylococcus aureus* infection	

Absynth Biologics discovers and develops vaccines and antibodies to prevent and treat bacterial infections; based on a platform of proprietary protein antigens working by a novel dual-action mechanism. Originally founded with investment from Biofusion plc (now part of IP Group plc, a leading UK intellectual property commercialisation company), Absynth has established a vaccine pipeline targeting the pathogens *Staphylococcus aureus, Clostridium difficile and Streptococcus pyogenes.* There are no marketed vaccines for any of these pathogens and a limited number of candidates in clinical trials. Consequently, each of Absynth's pipeline programmes addresses an unmet market need.

Absynth's most advanced programme targets *S. aureus,* including its more difficult-to-treat drug-resistant form, methicillin-resistant *S. aureus (MRSA).* The market for an MRSA vaccine or therapy is large and growing in the UK and US; hospital acquired infections are an increasing public health concern with the advance of antibiotic resistance and together with the increase in those vulnerable to community-associated disease may be responsible for a rise in the number of deaths.

Absynth Biologics was awarded an Early Stage Biomedical Catalyst grant (Round 3), for its lead product programme – a prophylactic vaccine for *S. Aureus*. The award helped Absynth to secure private investment in both 2013 and 2014. With this combined grant and private funding, the company has recruited a small R&D team (now nine in total) and secured their own laboratories. By the end of the project in 2016, the product will be advanced to a value-added inflection point (candidate selection; pre-clinical tox. ready), which will help with future fundraising and/or partnering.

The BMC has also helped Absynth to establish its pipeline programmes. A Technology Strategy Board (now Innovate UK) Proof of Market grant in 2013 funded market assessment, including key opinion leader interviews, which identified three pathogens where there are unmet needs for a prophylactic vaccine: *C. difficile*, *S. pyogenes* and *S. pneumoniae*. This led to a successful BMC Feasibility grant application (Round 1) to use the technology developed for *S. aureus* to make and evaluate vaccines to prevent *C. difficile* and *S. pyogenes* infections. The project concluded successfully in 2014 with data to support both vaccines. The *S. pyogenes* vaccine is now the subject of an MRC CASE studentship between the University of Sheffield and Absynth that commences in Sept/Oct 2015.

"The award helped Absynth to secure private investment in both 2013 and 2014. With this combined grant and private funding, the company has recruited a small R&D team (now nine in total) and secured their own laboratories."

Dr Sarah Howell
Chief Executive Officer
Arecor

Arecor Cambridge East England	
ROUND 1, EARLY	**GRANT:** £785,418 **LEVERAGED:** £523,613

Novel excipients to allow superior liquid antibody formulation at high concentration

Arecor, backed by Unilever Ventures, was founded in 2007, around technologies for improving stability of protein-based therapeutics. Since its inception Arecor has further refined the technologies and revolutionized the way protein-based therapeutics are formulated, with a considerable social and commercial impact. For example, the novel way of formulating these life-saving medicines and vaccines allows their use outside the cold-chain, which is the only way of delivering them to the parts of the world where they are most needed. Similarly, the reformulated products allow markedly simplified methods of their administration which in turn leads to patients' comfort and improved compliance. Protein-based therapeutics, also known as biotherapeutics, represent the fastest growing segment of the pharmaceutical industry and they dominate the development pipelines of most pharmaceutical companies.

Arecor has a highly talented team with a variety of expertise including protein formulation and analysis, manufacture, intellectual property and regulatory approval. Arecor is based on the Cambridge Science Park, giving it access to cutting edge research and development through its strong ties to Cambridge University and the broader biotechnology community. Arecor has partnered with the world's largest pharmaceutical and biotechnology companies to enhance the commercial value and practical utility of many different types of biotherapeutics in many different applications such as vaccines and medicines used to treat cancer or diabetes.

Arecor is continuously refining its proprietary technologies to allow its successful application to new types of products. The Biomedical Catalyst grant has provided essential financial support for Arecor's research and development into novel excipients that enable a new generation of ultra-stable liquid biopharmaceutical drugs. One of the most important classes of biotherapeutics are antibodies. Because of the high doses of antibodies required in treatment of serious diseases such as cancer or arthritis, they have to be formulated at very high concentrations. In this grant the application of Arecor's novel excipient technology has been successfully demonstrated in formulating novel, patient-friendly, high doses of current medicines used in both oncology and arthritis treatment. Without the financial contribution from the Biomedical Catalyst grant this research would not have been possible.

The exciting data generated from this work is currently being used to engage in new collaborations with pharmaceutical companies as well as build on relationships with existing clients. These collaborations are critical for Arecor's success and represent the most efficient way of translating the research results into clinical development and production of therapeutics, benefiting patients around the world. The new Arecor product formulations developed in this Biomedical Catalyst funded project will provide safer and higher quality medicines for patients in the future, enabling the development of high concentration forms of antibodies to simplify and reduce the costs of current treatments, resulting in fewer patient visits to hospitals for administration.

Arecor's experience with the Biomedical Catalyst funding has been excellent. In return, the UK economy is already benefiting from the investment through Arecor's commercial collaborations and license deals with international pharmaceutical companies.

"Arecor's experience with the Biomedical Catalyst funding has been excellent. In return, the UK economy is already benefiting from the investment through Arecor's commercial collaborations and license deals with international pharmaceutical companies."

Tim Edwards
Chairman
Atopix Therapeutics

Atopix Therapeutics
Abingdon
South East England

ROUND 2, LATE

GRANT: £1,753,000
LEVERAGED: £1,209,555

Evaluating a novel anti-Th2 therapy for moderate to severe atopic dermatitis

Atopix Therapeutics Ltd is a biotech company located in the Innovation Centre, Milton Park, Abingdon. Atopix has pioneered the discovery and development of oral anti-Th2 therapies for the treatment of asthma, atopic dermatitis and related disorders.

In asthma, the company's lead compound, OC459, has been shown to be effective in improving lung function and quality of life in asthmatic patients with an eosinophilic Th2 dominant form of disease which can be serious and poorly treated.

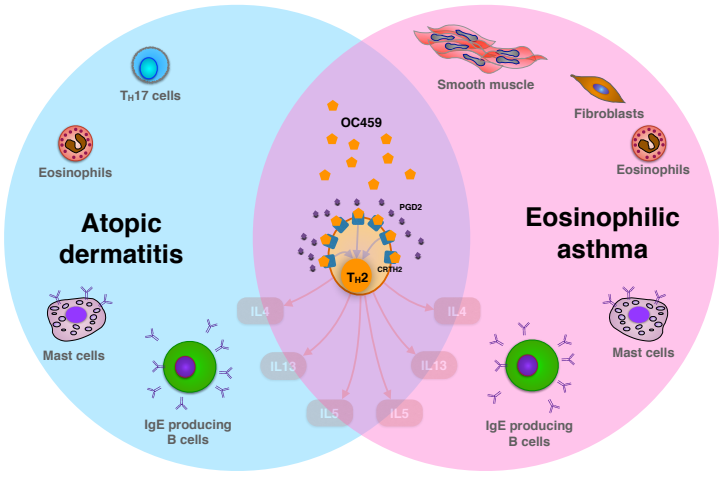
With respect to atopic dermatitis, in April 2013 Atopix received a Biomedical Catalyst award worth £1.7 million to study the effect of OC459 in the moderate-to-severe form of the disease.

There is a pressing need for a safe oral therapy to treat moderate-to-severe atopic dermatitis. Current oral therapies include cyclosporin, methotrexate, azathioprine and steroids, all of which can have serious side effects. A positive outcome in this indication with OC459 would be of major benefit to patients, especially as it seems likely that OC459 would have a much improved safety profile compared to existing systemic therapies.

The Biomedical Catalyst supported study involves centres of dermatology excellence in the UK, France, Germany, Finland and Eastern Europe and should provide definitive information on the efficacy of OC459. The scale of the study would not have been possible without support from Innovate UK which has enabled Atopix to unlock the potential of OC459 in this disease.

Atopix has conducted considerable development on OC459, having completed long term toxicology studies and developed a commercially viable tablet formulation that delivers an attractive once-a-day pharmacokinetic profile. A positive outcome from the clinical study in atopic dermatitis would position OC459 as an excellent candidate for further development, most likely in collaboration with a pharmaceutical partner.

This is a clear example of how funding from the Biomedical Catalyst is enabling the growth of the UK biotech sector and helping bring forward new medicines for the benefit of patients.



"This is a clear example of how funding from the Biomedical Catalyst is enabling the growth of the UK biotech sector and helping bring forward new medicines for the benefit of patients."

Dr Charles Large
Chief Executive Officer
Autifony Therapeutics

Autifony Therapeutics London England	autifony THERAPEUTICS
ROUND 2, EARLY	**GRANT:** £1,940,438 **LEVERAGED:** £836,879
ROUND 4, LATE	**GRANT:** £2,183,417 **LEVERAGED:** £1,079,778
ROUND 7, LATE	**GRANT:** £2,421,116 **LEVERAGED:** £846,439
Exploring potential of the company's novel ion channel mechanism in tinnitus and schizophrenia	

Biomedical Catalyst funding from Innovate UK and the MRC has been transformational for Autifony Therapeutics, allowing the company to grow beyond its initial core area of age-related hearing loss. It has enabled Autifony to explore the potential for the company's novel ion channel mechanism in other areas of high unmet medical need, such as schizophrenia and tinnitus. The funding has helped Autifony to build its team and to collaborate extensively with academic groups and with Clinical Research Organisations (CROs) around the UK. Initial results have already been presented at international scientific conferences, attracting considerable interest from both major pharma companies and the academic community.

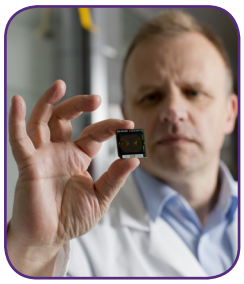
Autifony Therapeutics was founded to develop new drugs to treat hearing disorders using pioneering science that targets specific ion channels (called Kv3 ion channels) which regulate neuronal activity within the auditory system. Autifony's lead drug was designed to treat age-related hearing loss, and is currently in a Phase IIa trial for this indication. No one had previously developed drugs targeting Kv3 ion channels, which play an important role in the central nervous system (CNS) where they are critical to the regulation of many brain functions, including auditory processing and cognition. Consequently, drugs targeting Kv3 channels have potential as novel treatments for a wide range of serious CNS disorders, in particular schizophrenia, for which better drugs are desperately needed, and tinnitus, for which there are currently no effective treatments.

Autifony was originally funded by its venture capital investors to take its first drug through to clinical Proof of Concept for one hearing indication – age related hearing loss. The company had also generated encouraging preclinical data to support a tinnitus indication, which affects over 10% of the population. However, there was insufficient funding available to conduct a trial for this complex disorder. Thanks to a Biomedical Catalyst Late Stage award, Autifony was able to set up a fully powered Phase IIa study of tinnitus. The QUIET-1 study is currently recruiting at 14 sites across the UK.

Innovate UK and MRC funding has also been crucial in enabling Autifony to explore the potential for Kv3 modulator drugs outside the company's initial core area. The Biomedical Catalyst funded an Early Stage programme which enabled selection and preclinical development of a differentiated Kv3 drug for the treatment of schizophrenia. This project included a fruitful collaboration with the Universities of Manchester and Newcastle to explore the Kv3 mechanism in model systems relevant to the disorder. The new drug will shortly begin Phase I clinical trials, for which further support has been obtained in the form of a Late Stage Biomedical Catalyst award, creating an additional clinical stage asset for Autifony.

Autifony Therapeutics Limited is a UK-based company formed in 2011 as a spin-out from GlaxoSmithKline. The company is privately funded by leading venture capital investors, who have provided the matched funding to Autifony's Biomedical Catalyst supported programmes, in areas outside the company's original core area, but which the investors would not have been willing to support alone.

"Innovate UK and MRC funding from the Biomedical Catalyst has been transformational for Autifony Therapeutics, allowing the company to grow beyond its initial core area of age-related hearing loss."



Dr David Williams
Chief Executive Officer
Discuva

Discuva
Cambridge
East England

ROUND 1, EARLY	**GRANT:** £1,620,000 **LEVERAGED:** £1,080,000

Discovery of new commercially viable antibiotics to treat bacterial infection

Discuva is a Cambridge, UK-based drug discovery company which was established to address the major gaps that exist in the current treatment of infective disease with antibiotics and meet the current critical unmet medical need in the clinic. The company has proprietary technologies that allow the rapid identification and development of targeted, next generation antibiotics against emerging and drug-resistant pathogens.

Discuva currently has several specific antimicrobial programmes, mainly focused on Gram negative bacteria that cause major hospital and community-based infections. Their technology provides a powerful platform to discover new classes of antibiotics which have major advantages over conventional antibiotics; reduced development of resistance, fewer side effects and longer market life.

The company was awarded an Early Stage Biomedical Catalyst grant as part of Round One with the objective of progressing compounds to lead status, creating commercially attractive candidates ready for optimisation and development.

For Discuva, obtaining a Biomedical Catalyst award was a truly transformative event. The immediate effect, from the positive review of the company's science, was that it enabled further fundraising from existing investors. The combined additional funding meant that the company could immediately enhance their infrastructure build, employ more scientists and ultimately take more risks in the drug discovery process.

No longer restricted by a lack of funding, they were able to finalise the development of their technology and fully resource the research on a sensible number of potential drugs. This approach led to Discuva signing the largest pre-clinical antibiotic collaboration with a major pharmaceutical company, putting them in the position as one of the world's most innovative and productive antibiotic companies. The most important aspect of this additional non-dilutive funding was that it allowed the company the opportunity to innovate.

"For Discuva, obtaining a Biomedical Catalyst award was a truly transformative event... The most important aspect of this additional non-dilutive funding was that it allowed the company the opportunity to innovate."

Dr Ian Fotheringham
Managing Director
Ingenza

Ingenza
Roslin
Midlothian, Scotland

ROUND 6, LATE

GRANT: £829,336
LEVERAGED: £209,248

A novel enzymatic means to confirm correct and safe nasogastric tube placement

Ingenza Ltd is an industrial biotechnology company with a broad customer base across the chemicals, pharmaceuticals, food, feed and fuel industries. Located in Edinburgh and with a team of 42 people, Ingenza applies synthetic biology to the manufacture of industrial products including enhanced biofuels, sustainable manufacturing of chemicals and the production of protein therapeutics.

In 2010 Ingenza became aware of a very serious medical error problem in the UK - nasogastric feeding tube misplacement. A 2010 UK National Health Service report on deaths and severe harm associated with misdirecting NG feeding tubes into the lungs, identified misinterpretation of placement assays as the most common reason for errors. Current healthcare guidelines recommend safety checks, based on a pH test of tube aspirates, to define an upper pH cut-off for safe NG tube feeding, beyond which a confirmatory chest X-ray is required. To compound the situation, across the EU up to 42% of hospital patients receive antacid medications that render the results of pH test paper falsely negative. Consequently, according to most EU national medical guidelines, patients on antacid medication receiving NG tube-mediated feeding require many unnecessary chest radiographs to confirm correct tube placement. However, daily chest radiography is undesirable because this involves increased radiation exposure to patients, including children and represents significant additional costs/time to healthcare providers.

In July 2010 Ingenza started a SMART funded feasibility project with St. Mary's Hospital/Imperial Healthcare Trust, which was highly successful, demonstrating a 97.2% success rate versus a 65% success rate of the current test in NHS practice. However, the sample set of 28 patients that could be funded in the feasibility study was small and a larger study was deemed necessary to provide the necessary evidence to support adoption of the test by the NHS and healthcare systems worldwide.

Ingenza could not internally fund the £1M+ larger collaborative study with the same team at St. Mary's, particularly since this product is somewhat tangential to the company's more typical business in synthetic biology. Additional funding of £1.2M was therefore sought and obtained (November 2014) from the Innovate UK and the MRC's Biomedical Catalyst programme, which will fully support a much larger trial at the hospital and also will 60% fund Ingenza's internal development of a Phase 1 and Phase 2 device to greatly improve patient safety. This trial is now in progress and, upon success, is designed to provide the necessary statistically significant data to support Phase 1&2 product testing within the NHS. The current study will also optimise ISO13485 compliant manufacture of the modified device itself, address relevant barriers to adoption, economic, usability and competitive analyses. The current study is also highly appropriate since the St. Mary's Principal Investigator (PI) and gastric surgeon, Professor George Hanna co-authored the government report that highlights the problems of the current test and the pressing need for a superior test, such as that being developed by Ingenza.

Prior to being awarded the Biomedical Catalyst funding this project had been shelved at Ingenza as there was no obvious route to develop the product further. The BMC award by Innovate UK was critical to the ongoing development of this programme, which is protected by issued and pending worldwide patents on the assay.

"Prior to being awarded the Biomedical Catalyst funding this project had been shelved at Ingenza as there was no obvious route to develop the product further. The BMC award by Innovate UK and the MRC was critical to the ongoing development of this programme which is protected by issued and pending worldwide patents."

Dr Sam Williams
Chief Executive Officer
Modern Biosciences

Modern Biosciences London England	modern biosciences
ROUND 1, EARLY	**GRANT:** £1,562,595 **LEVERAGED:** £1,282,413
ROUND 5, LATE	**GRANT:** £2,400,000 **LEVERAGED:** £2,608,200
Novel, oral anti-inflammatory and bone-protecting compounds for the treatment of rheumatoid arthritis	

Modern Biosciences plc (MBS) is a London-based SME company focussed on developing treatments for autoimmune and inflammatory disorders. MBS runs an outsourced model of drug development, with experiments planned at its offices in the City and contracted to specialist companies around the world. Its lead product is a novel agent for the treatment of rheumatoid arthritis (RA), the debilitating auto-immune disorder, which is due to enter Phase 1 clinical studies this year. Based on IP licensed from the University of Aberdeen in 2007, this drug appears to do something unique in the treatment of RA, which is to directly protect bone from damage caused by the immune system. Bone destruction is the most debilitating facet of RA and MBS' molecule has the potential to not only inhibit the progression of this destruction, but also to reverse it, something that no existing therapies do.

In November 2014, MBS signed an Option and Licence Agreement for the RA programme with Janssen Biotech Inc., a division of Johnson & Johnson. This has enabled MBS to significantly expand the scope of its Phase 1 studies, which now include a small number of RA patients to test for early signs of efficacy. However, it is worth noting that the programme would not have reached its current stage of development or attracted the attentions of J&J without BMC funding.

Until the 2012 award, MBS was run on a tight budget, with its two-man team working with a single chemistry consultant during lead optimisation. This changed when MBS won an early-stage BMC award for £1.6m in 2012, which was followed by a late-stage award for £2.4m in 2014.

With the BMC money, the company was able to build an extensive network of expert consultants, including those covering toxicology, pharmacokinetics, formulation and manufacturing. It also enabled MBS to justify increased funding from its main shareholder, IP Group plc, and take the programme to a point at which it could be presented to the J&J Innovation Centre in London with a robust clinical candidate. Most importantly, it has given MBS and J&J the chance to bring a drug to the clinic that might help change the lives of the millions of patients worldwide that live with this awful, crippling disease.

"Most importantly, it has given MBS and J&J the chance to bring a drug to the clinic that might help change the lives of the millions of patients worldwide that live with this awful, crippling disease."

Glyn Edwards
Chief Executive Officer
Summit Therapeutics

Summit Therapeutics Oxford South East England	summit
ROUND 1, FEASIBILITY	**GRANT:** £150,000 **LEVERAGED:** £55,019
ROUND 3, LATE	**GRANT:** £2,400,000 **LEVERAGED:** £5,854,278
Using pioneering technology to develop novel drugs for rare diseases including the fatal genetic disorder Duchenne Muscular Dystrophy	

Summit Therapeutics ('Summit') is a biopharmaceutical company founded as a spin-out from the University of Oxford with a focus on developing treatments for the fatal genetic disease Duchenne Muscular Dystrophy ('DMD').

DMD is the most common and severest form of muscular dystrophy and there are approximately 1,500 boys and young men with the disease in the UK. It is caused by different genetic mutations affecting the dystrophin gene, resulting in progressive muscle wasting. Currently there is no approved therapy applicable to all DMD patients that seeks to slow or stop the progression of the disease. Average life expectancy is in the late twenties.

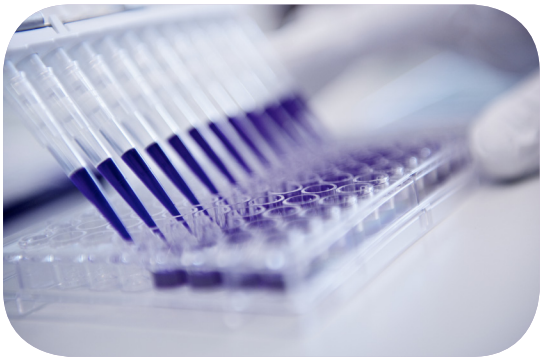
Summit is seeking to treat all boys affected with DMD using its pioneering utrophin modulation technology. The company's most advanced utrophin modulator is SMT C1100, an orally administered drug that has the potential to treat all patients with DMD, irrespective of the underlying genetic fault causing the disease. The concept of utrophin modulation was originally developed by Professor Dame Kay Davies at the University of Oxford.

The award from the BioMedical Catalyst has played a pivotal role in advancing the development of SMT C1100 by catalysing a comprehensive programme of work towards establishing it as a viable treatment. In summary, the funding has enabled Summit to commence UK clinical trials of SMT C1100, the first such studies of a utrophin drug to be undertaken in patients with DMD. The company is also developing new biomarkers that have the potential to help quantify more effectively the benefits of SMT C1100 in patients; the new biomarkers will be used in proof of concept clinical trials that are expected to start over the next 12 months.

The support of the BMC has also enabled Summit to attract significant new investment into the company to further support its leading-edge research and development. The endorsement received through the BMC award has enabled the company to raise over £40 million in funds from specialist healthcare investors to support the DMD programme. This has allowed Summit to expand as it seeks to meet the clinical and regulatory needs of the advancing drug programme, while the increased research and development investment has benefited a number of UK-based contractors and academic groups.

A successful outcome from Summit's research has the potential to significantly improve the quality of life of patients with DMD, reduce the current financial burden of the disease on NHS and maintain the UK's reputation as a country of science and innovation.

"The award from the BioMedical Catalyst has played a pivotal role in advancing the development of SMT C1100 by catalysing a comprehensive programme of work towards establishing it as a viable treatment. In summary the funding has enabled Summit to commence UK clinical trials of SMT C1100, the first such studies of a utrophin drug to be undertaken in patients with DMD."



Dr Salman Rahman
CEO and co-founder
Vasgen

Vasgen
London
England

ROUND 5, FEASIBILITY

GRANT: £173,434
LEVERAGED: £26,562

New monoclonal antibody therapy to treat age-related macular degeneration

Vasgen Ltd is a privately owned, London-based biotech SME developing innovative therapies to treat unmet clinical needs in ophthalmology and cancer medicine.

The company was formed by ex-academics Drs Salman Rahman and Yatin Patel in 2011 to commercialise their research from King's College London, devoting their full-time to the venture. The company is using a novel proprietary antibody technology called AbIMP® to generate a new, first-in-class, monoclonal antibody therapy to prevent the dysregulated growth and leakage of blood vessels in patients with wet age-related macular degeneration (AMD), the leading cause of blindness in developed countries.

Currently, only 35% of patients with wet AMD regain lost vision following the standard of care therapy and require monthly intra-ocular injections. Vasgen's aim is to see enhanced outcomes across all patients, particularly in the 65 % of those whose vision is not restored. By combining the company's new therapy with the current standard of care, it's anticipated that a greater proportion of patients will regain lost vision and will do so requiring fewer injections, reducing the overall cost of care for the NHS and other payers.

In collaboration with the UCL Institute of Ophthalmology, Vasgen was the recipient of a Biomedical Catalyst Feasibility award in late 2014 that is allowing development of a lead therapeutic candidate and experimental studies to support proof of its mechanism of action. This work is essential to place the company in a position to obtain private investment to finance this important therapeutic programme towards clinical trials.

Vasgen's new therapy will also be evaluated as a treatment for aggressive prostate and breast cancers for which current therapies have limited impact and the work from the Biomedical Catalyst award will provide important data to support follow-on financing for a further cancer programme.

The current Biomedical Catalyst funding has allowed Vasgen to set-up initial operations, establish an important collaboration with a leading academic centre, employ a full-time scientist and contract several UK companies. It is hoped that successful completion of this work will allow the company to apply for further funds from the Biomedical Catalyst competition to support pre-clinical development of our novel therapy to first-in-man studies.

"It is hoped that successful completion of this work will allow the company to apply for further funds from the Biomedical Catalyst competition to support pre-clinical development of our novel therapy to first-in-man studies."

Dr Mike Yeadon
CEO and co-founder
Ziarco Pharma

Ziarco Pharma
Sandwich
South East England

ROUND 4, LATE

GRANT: £1,701,527
LEVERAGED: £1,134,351

Development of oral treatment for itching and inflammation in atopic dermatitis

Ziarco is an independent, clinical stage biotechnology company progressing new and different therapies for inflammatory and allergic diseases, using both oral and externally applied routes of delivery. The company is UK based and has offices at Discovery Park, Kent.

By exploiting shared disease mechanisms in inflammatory and allergic pathways, coupled with high human confidence in the methods for application, Ziarco's programmes can be used to treat multiple disorders affecting the airways, skin and other organs. The company's current focus is on chronic, inflammatory skin diseases, specifically atopic dermatitis and psoriasis. With Ziarco's lead compound now in a formal clinical trial in Europe, including centres in the UK, the company hope to confirm their belief that ZPL-389 will become the first oral treatment for atopic dermatitis which will be effective in relieving both itch and skin inflammation (excema) as well as safe and well-tolerated.

Following this lead compound, the company has several other very promising compounds which are expected to yield medicines for a range of poorly-treated, chronic inflammatory disorders. Ziarco's highly-experienced team, who invented and developed these programmes, is passionately committed to advancing them and delivering new medicines with outstanding profiles of efficacy and safety.

The BIA network has helped Ziarco by providing access to key industry investors including Innovate UK, from whom they secured a Biomedical Catalyst award. The importance of access to funding opportunities is crucial and the company was pleased to have received an award, which it is currently engaged in using in their ongoing inflammatory skin disease clinical trial.

News of the BMC award was very helpful - as was its timing - as a kind of external validation of the company's technology and approach. As such, this validation was helpful to the company in raising fresh finance late in 2014, which has secured approximately 10 skilled positions for a number of years.

"News of the BMC award was very helpful - as was its timing - as a kind of external validation of the company's technology and approach. As such, this validation was helpful to the company in raising fresh finance late in 2014, which has secured approximately 10 skilled positions for a number of years."

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