



Notes and acknowledgements

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Citing this report

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Notice

This report is intended to provide a broad introduction to collaborative working between charities and industry and to raise and discuss related questions. As a result, please be aware that AMRC cannot take responsibility for any actions taken with reference to the report. Each charity must consider its own circumstances and priorities before deciding on its most appropriate course of action. The legal position surrounding collaborative working, in particular in relation to agreements and contracts is complex and this document is not a substitute for specific legal advice.

Foreword



Aisling Burnand
chief executive
Association of Medical Research Charities

The Association of Medical Research Charities (AMRC) represents over 130 charities that together fund more than a third of all publicly funded medical research in the UK. The diversity of our members and their wide-ranging aims and objectives means that AMRC member charities fund across the entire research landscape, at all stages of the research process. Increasingly, charities are looking to maximise their impact by developing partnerships that encourage the translation of research from 'bench to bedside'. This, combined with the patient-centric focus of charity-funded research, is what can really bring charities and industry together, as both share the common goal of bringing new treatments to patients more quickly.

An Essential Partnership is something of a landmark for AMRC – it updates a previous guide that set the scene for collaborative working and builds on the fantastic progress we have made in recent years to drive forward innovative research. One of the most important roles of AMRC is to try to predict how the environment will look over the coming years. We hope that this publication sets the direction, as well as explaining how charities can go about working with industry. We're also very interested in connecting our members with charity and non-charity partners. Throughout this guide, we have illustrated the varied and novel ways that our charities are doing just that. It's this sharing of best practice, ideas and insights that we hope everyone will benefit from.

I'm delighted to see this document come to fruition and hope it will help all charities think about their own ways of working. By working together and developing our own 'essential partnerships', we can all contribute to a strong sector-wide movement to address unmet patient need.



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The AMRC Industry–Charity Advisory Group was brought together to help us support members in developing relationships with the different parties involved in commercialisation. As co-chairs of the group, we hope this document will be an important tool for showing charities how to go about working with industry.

Charities are increasingly looking to collaborate at all stages of the medicines, diagnostics and devices development pathway in order to bring new treatments and therapies to patients sooner. As we learn more about treating disease and managing long-term conditions, we need to join together across the sector to address the most challenging research questions. Charities and industry have a very real opportunity to do this together in partnerships, each bringing their unique strengths and perspectives.

The landscape is already changing – both in terms of the type of research we are undertaking and the environment in which it's being done. Charities and industry working together can bring the treatments so urgently needed to patients as quickly and effectively as possible.

Executive summary

Collaborations between medical research charities and the pharmaceutical, biotechnology, diagnostic and devices industries can deliver significant benefits for patients and for wider society, speeding up the development of new treatments and therapies and improving existing ones to produce better health outcomes. Despite their diverse nature, charities and industry have unique strengths to bring to collaborations, and it is on this basis that the most effective partnerships can be formed.

An Essential Partnership is a guide for medical research charities wishing to undertake research collaborations with industry. We describe how the changing landscape can lead to new opportunities for collaboration and illustrate the different ways in which charities are working with industry through case studies. We also cover what charities should consider when developing research partnerships and the types of agreements and contracts that might need to be in place.

This guide complements a document¹ published by National Voices and the Association of the British Pharmaceutical Industry (ABPI), which sets out principles for collaboration and the standards expected of charities, patient groups and industry. We hope that, together, these documents will help all sectors work more collaboratively than ever before.

While there isn't a 'one size fits all' model for developing collaborations, this guide helps explain how the sector is currently performing. As the information is likely to change over time, we will review and update the document periodically. If you would like to share your experience for future editions, please get in touch.²

Throughout the guide, we refer to three principles that underpin all aspects of collaborative working:

- **integrity:** both parties should act honestly and with integrity at all times
- **independence:** charities should maintain their independence from the company concerned
- **transparency:** charities should be entirely open about their collaborations with industry

By building relationships where integrity, independence and transparency are central, charities should feel comfortable in pursuing links with industry to further their charitable aims.

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1. Introduction



- **Charities and commercial organisations play vital roles in the research landscape and bring different but complementary strengths to collaborations.**
- **Charities should be aware of the complexities when bringing new drugs, diagnostics and devices to the market. Charities and industry can work together to facilitate this process.**
- **Charities are directly connected to patients and are well placed to express patient need accurately and effectively.**

Collaboration between medical research charities and the pharmaceutical, biotechnology, diagnostic and devices industries (collectively referred to as 'industry') is essential to ensure that treatments and interventions achieve maximum benefit to patients by being fit for purpose.

Charities collaborate with industry in a variety of ways:

- Research and development: partnerships with pharmaceutical, biotechnology and medical technology companies, providing patient insights and co-funding research
- Policy and advocacy: influencing policy in the research sphere across the healthcare landscape
- Sponsorship: industry working with charities to sponsor events such as conferences and meetings, or supporting other forms of communication (eg patient information, educational materials)
- Campaigning: working together on specific campaigns or lobbying (eg to highlight patient need or to campaign for the adoption of a new drug by the NHS)

This guide specifically addresses research and development collaborations. Traditionally, many charities fundraise on the basis that increased funds will support research, which in turn can support the development of new treatments for patients. By working with industry, charities can maximise their impact by encouraging the translation of research from 'bench to bedside'.

1.1 Why might charities work with industry?

Increasingly, charities are looking to collaborate with a variety of partners, and those wishing to increase treatment options for their patient groups need to consider working with industry. The costs and expertise required to bring new therapies to market cannot be provided by charities alone – industry is often the main driver. It's on this basis that successful collaborations between charities and industry can be built.

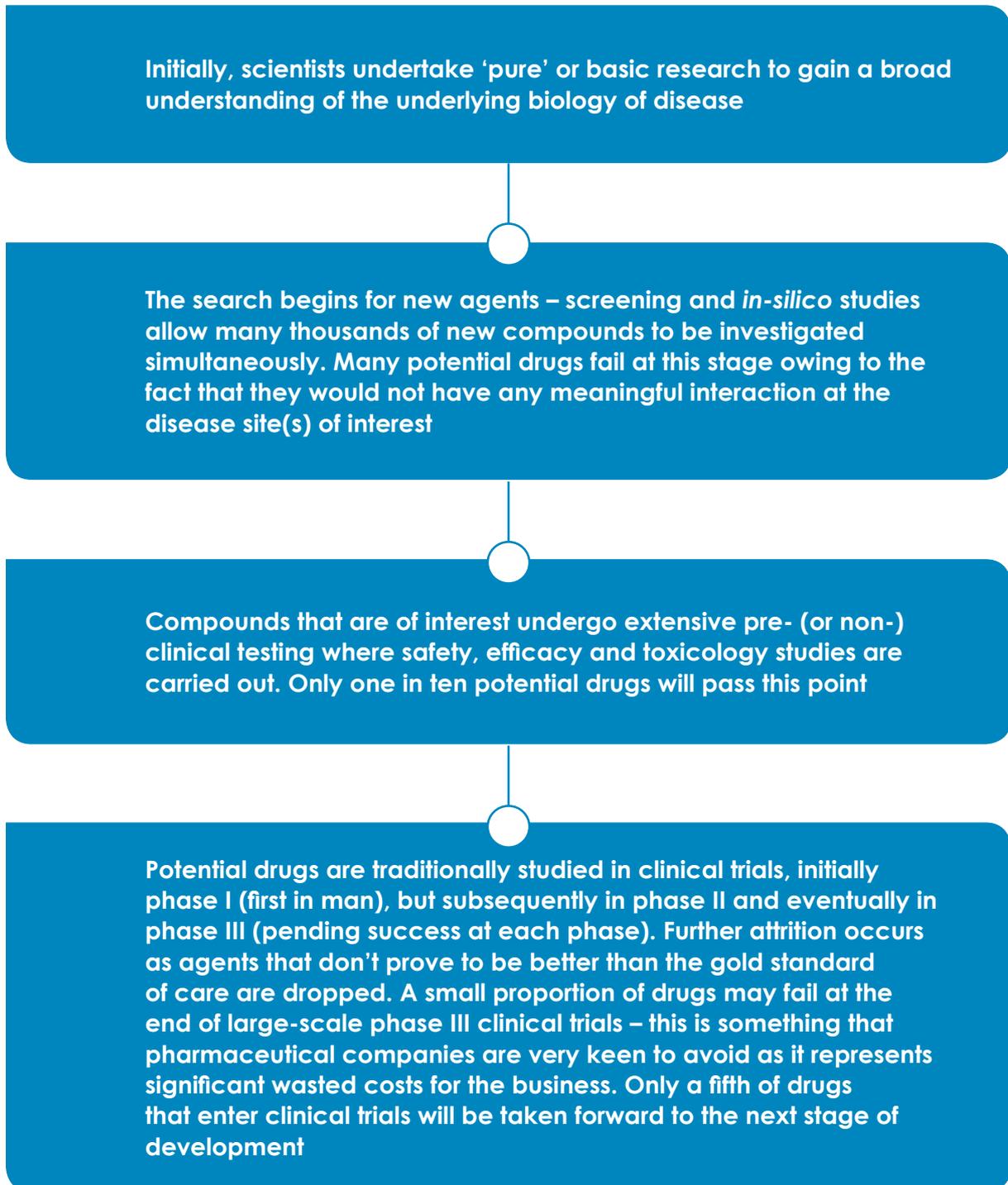
In spite of the advantages, some charities prefer not to work with industry. This is particularly true of those whose focus is to increase research capacity (perhaps through studentships and fellowships) or to fund types of research that aren't immediately relevant to industry (eg some forms of psychosocial or epidemiological research). This type of research is absolutely crucial and this document in no way means to diminish the value of such work.

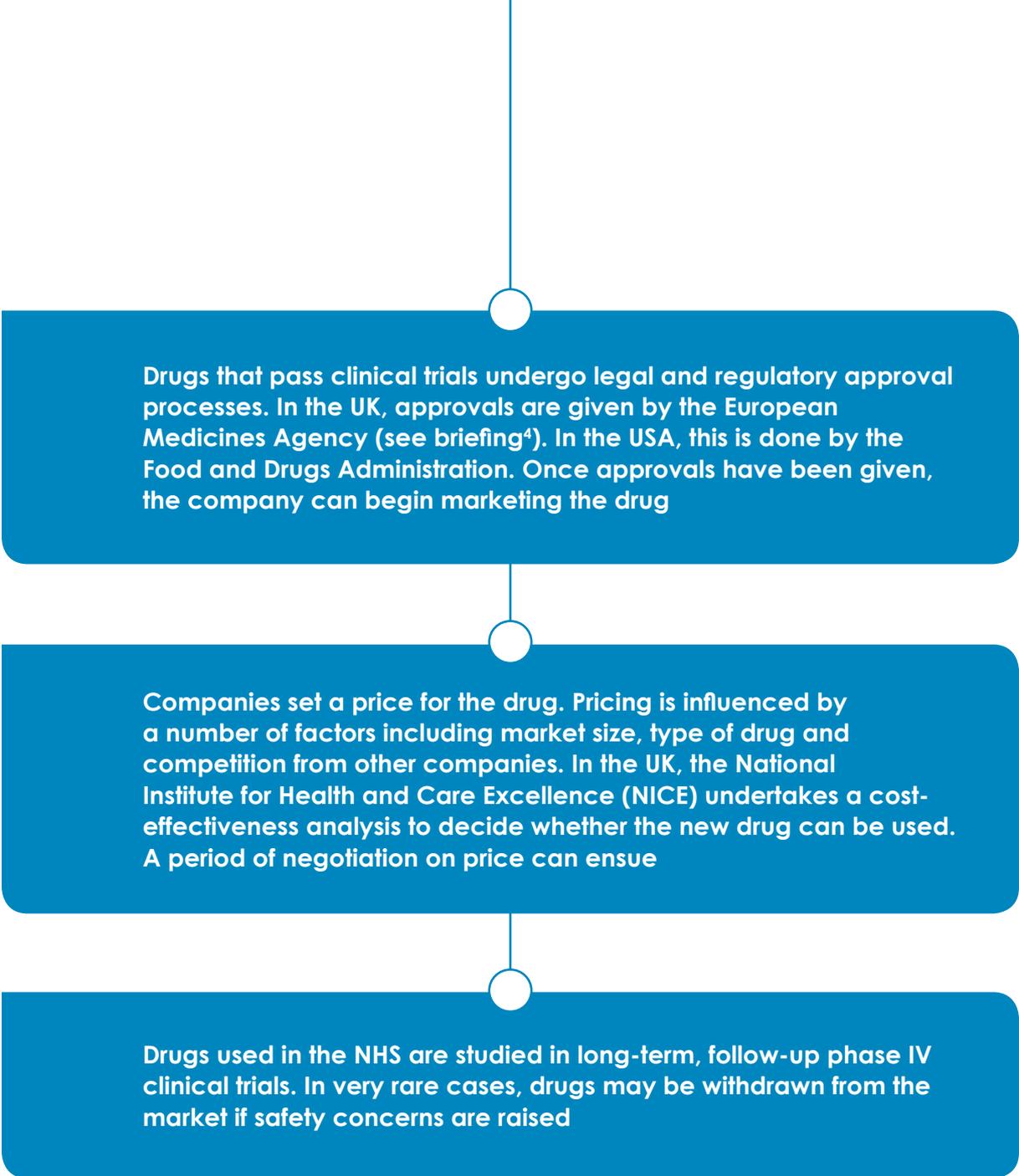
1.1.1 Developing drugs for adoption in the NHS

Developing a drug from concept to market takes on average 12 years and costs from £30m to over £1bn.³ Only a handful of the many thousands of potential new drugs that start out on the drug development pathway will end up in the clinic. It's for these reasons that the pathway is widely referred to as being 'too slow, too low'. To understand this further, it's important to explore the current state of play in the drug development pathway (figure 1).

FIGURE 1:

OVERVIEW OF THE TRADITIONAL MEDICINAL PRODUCT DEVELOPMENT PATHWAY





Drugs that pass clinical trials undergo legal and regulatory approval processes. In the UK, approvals are given by the European Medicines Agency (see briefing⁴). In the USA, this is done by the Food and Drugs Administration. Once approvals have been given, the company can begin marketing the drug

Companies set a price for the drug. Pricing is influenced by a number of factors including market size, type of drug and competition from other companies. In the UK, the National Institute for Health and Care Excellence (NICE) undertakes a cost-effectiveness analysis to decide whether the new drug can be used. A period of negotiation on price can ensue

Drugs used in the NHS are studied in long-term, follow-up phase IV clinical trials. In very rare cases, drugs may be withdrawn from the market if safety concerns are raised

When a drug reaches the market, pharmaceutical companies begin to recoup the costs spent on its development as well as the costs of investigating other potential drugs that fail along the pathway. Companies normally have approximately ten years exclusivity on the market, after which other companies can offer generic compounds at a much lower price.

However, as the cost of drug discovery and development continue to rise, this can influence how much a company may charge for a medicine and, in some cases, it can negate the incentive for further drug development. Evidence is already emerging that the number of new medicines produced per billion dollars spent is falling.⁵ In response, some companies are looking to work with other organisations, including charities, to define new therapies and treatments.

Although companies may wish to charge higher prices for new drugs to cover their costs, this is unlikely to be feasible. The Pharmaceutical Price Regulation Scheme⁶ defines how much profit a company can make from selling its drugs to the NHS. The decision to purchase a branded drug is based on the relevant NICE technology appraisal guidance, which includes a cost-effectiveness analysis and clinical evaluation. This informs an assessment of how many additional years of life and improvements to a patient's quality of life the drug may provide, known as a quality adjusted life year (QALY) score. NICE calculates the cost of the drug per QALY. In general terms, treatments costing more than £20,000 to £30,000 per QALY are not recommended as cost-effective by NICE; however, end-of-life treatments can receive approvals at around twice this amount. Drugs deemed too expensive by NICE will not be adopted by the NHS, as shown in the case study below.

CASE STUDY 1

Kadcyla not recommended for use in the NHS

Kadcyla, a targeted chemotherapy drug for secondary breast cancer developed by Roche, was not recommended for routine use⁷ in the NHS by NICE owing to its expense. Kadcyla is a combination drug comprising Herceptin and the chemotherapy drug DM1 that allows specific targeting of HER2-positive cancerous cells, reducing damage to healthy cells and thereby reducing side effects. The drug costs around £90,000 per patient per year and adds on average six months of life to women with terminal breast cancer.

The assessment of new medicines, medical devices and other technologies was recently reviewed as part of a consultation on Value Based Assessment⁸ which examined whether the definition of 'value' should be broadened to include the burden of illness and wider societal benefits. Following the consultation, it is unlikely that NICE will make changes to the technology appraisal methodology in the short term, but it will give further consideration to the use of QALYs as a means of quantifying the burden of illness. Further changes are likely in the future.

As well as financial considerations, there has to be sufficient patient need if a drug is to be adopted by the NHS. Patient need varies depending on how many people are affected by a disease, the severity of the symptoms and whether other drugs are available on the market. Understanding the patient voice is at the heart of all these considerations and charities can play a significant role in bringing these views to discussions with NHS regulators and payers. For more information, see our briefing on the value of the patient voice.⁹ Patients are also increasingly being asked to get involved in the design of research projects to ensure the results are of clinical significance; see the next case study.

CASE STUDY 2

IMI looking to include patient voice

The Innovative Medicines Initiative¹⁰ (IMI) supports collaborative research projects and builds networks of industrial and academic experts to boost pharmaceutical innovation in Europe. IMI aims to improve the drug development process by supporting more efficient ways of new medicine discovery and development. The most recent round of calls included specific provisions for patient groups¹¹ to be an active part of partnerships and, in a recent project examining novel endpoints in eye diseases, patients, users and caregivers played a vital role in establishing the value of new clinical endpoints.

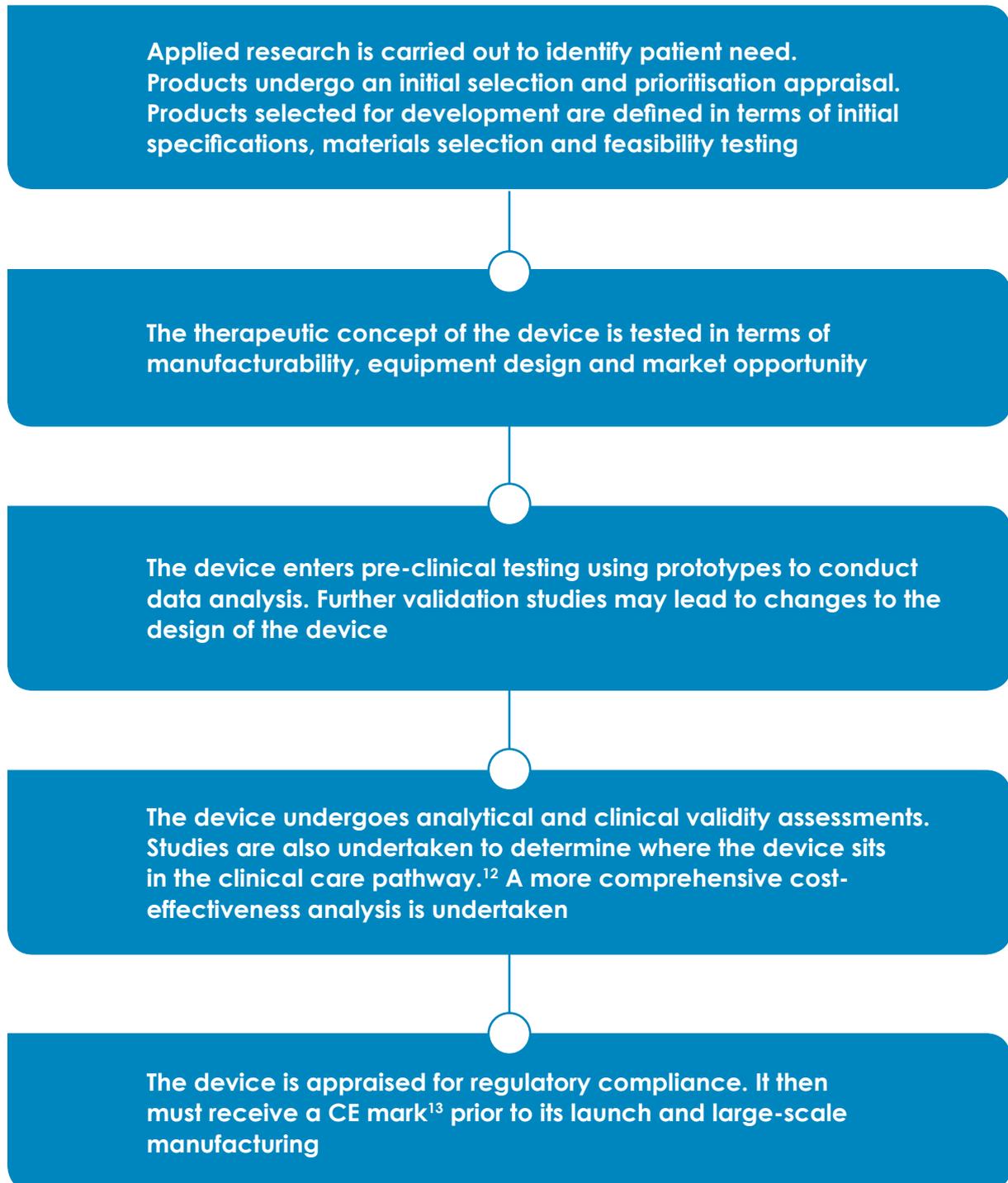
1.1.2 Developing devices for adoption in the NHS

When thinking about developing new treatments and therapeutics, it's important to remember the vital role that medical devices play. They cover a broad range of innovative products that can be used in the clinic for treating and monitoring many diseases and conditions, enhancing the quality and effectiveness of healthcare. They may range from a simple bandage to highly complicated life support equipment. Outside the clinic, huge technological advances are bringing new devices directly to patients in their own homes. A wide array of products are available – from assistive technologies supporting patients with Alzheimer's disease to mobility aids to help older people live more independent lives.

Medical devices therefore represent a significant opportunity for charities to support new and innovative research that addresses patient need. As with pharmaceuticals, charities thinking about developing partnerships with medical device companies should have a good understanding of how devices are developed for adoption in the NHS. There are some important differences to the steps seen in drug development, as explained in figure 2.

FIGURE 2:

OVERVIEW OF THE DEVICES DEVELOPMENT PATHWAY



Further work in terms of marketing, sales, production and clinical adoption is carried out. An output report is produced for dissemination across the NHS. In some cases, this could involve a NICE Medtech Innovation Briefing¹⁴

Long-term economic and patient benefit assessments are carried out to inform the impact on the healthcare system

When companies develop new devices, they need to consider how difficult it will be to get them adopted by the NHS. All too often, uptake is infrequent and slow. This can be due to a number of issues, including an unclear pathway to engage with NHS partners or failure in uptake of innovations across the organisation. One charity, recognising that this can be a significant disincentive for companies involved in the devices sector to develop new technologies, is examining how they can tackle this: see below.

CASE STUDY 3

Royal College of Surgeons: facilitating innovation and adoption of medical devices in the NHS

The Royal College of Surgeons Clinical Research Initiative (RCS CRI) is working with partners, including the NIHR Office for Clinical Research Infrastructure (NOCRI), to develop a Technology Evaluation Pathway to create a clear route for device companies to get their products into the NHS. Using clinical networks, the pathway aims to coordinate a group of healthcare experts to take forward new innovations and promote best practice across the NHS. The RCS CRI helps throughout the development pathway of a new product. In the early stages, it plays an important role in assessing clinical need and utility, as well as the impact the device may have. It helps in the preclinical testing assessment and clinical evaluation of the product and also plays a role in disseminating information across the NHS to inform the organisation of new technologies that might benefit patients. It's hoped that this initiative will make the process of adopting new products across the NHS more efficient and successful, encouraging medical device companies to invest in this area.

2. Understanding the changing research landscape

- **The changing landscape brings many new opportunities for charities to collaborate with industry. Charities should examine their portfolios to see how they might benefit from such opportunities.**
- **Collaborations in early-stage research to determine proof of concept and reduce the risk involved in early projects are increasingly prevalent. They may take place through an equal partnership structure in terms of shared decision-making, risk and financial input.**
- **Charities should be aware of the many initiatives they can undertake to facilitate collaborative research, including funding early-stage research, supporting translational research and developing new funding streams to complement commercial research.**

The research landscape has undergone significant changes in recent times, driven by technological advancement, a changing policy and regulatory climate, new licensing frameworks that promote innovation and a developing NHS and healthcare sector. In this chapter, we take a look at changes across the landscape and how they might influence partnerships between charities and industry.

2.1 New opportunities for drug development

As well as looking to develop new drugs, devices and diagnostics, industry is becoming increasingly interested in new opportunities for drug and device development. 'Biosimilars' are of particular interest to pharmaceutical companies. These agents, produced after the original patent has expired, are subsequent versions of biological products, similar but not necessarily identical to the original version, with the same mechanism of action. This could help to introduce competition into the drug market and force price reductions once the original cost of innovation of the product has been recouped in the protection period. For more information on biosimilars and how charities can work with them, see our briefing document.¹⁵

Industry may also be interested in repurposed drugs. These are defined as previously approved medicines used to treat one disease or condition, which are subsequently proposed for use in other diseases or conditions. An example of a repurposed drug is described in the case study below. This example illustrates how repurposed drugs that may have been initially investigated perhaps 40 years ago may be relevant for new indications today. For more information, see our briefing document.¹⁶

CASE STUDY 4

NICE approves MS drug developed by University of Cambridge researchers

NICE has recently approved a new biologic medicine for use in people with relapsing-remitting multiple sclerosis. Alemtuzumab (marketed as Lemtrada¹⁷ by the pharmaceutical company Genzyme) has been shown to reduce disease activity and limit disability. Research on Lemtrada dates back to the 1970s when it was known as Campath-1H – originally developed as an immunosuppressant to prevent rejection of bone marrow transplants. Campath-1H was identified as a potential treatment for multiple sclerosis in the late 1980s and the first MS patient was treated with it in 1991. Recent publication of phase III clinical trial results¹⁸ has confirmed that the drug is effective in relapsing-remitting MS patients. It does, however, have side effects – roughly one-third of patients treated with it go on to develop thyroid diseases. Further studies are currently underway to determine whether a particular subgroup of patients is particularly vulnerable to such complications.

Nick Rijke, director for policy and research at the MS Society, says: 'The NICE approval of Lemtrada is a major step forward in the treatment of people with multiple sclerosis. This drug has taken decades to develop, and while it's not without risk, it's proven to be a highly effective medicine for people with relapsing-remitting MS. We look forward to seeing it made available to those who could benefit.'

'Drug rescue' is another term used to describe small molecules and biologics that have been abandoned because they weren't found to be effective for the purpose for which they were initially intended or because the side effects were so severe that the drug was withdrawn from use. Some of these agents may be effective in treating other diseases for which they haven't been tested. One example of drug rescue is described in the case study below.

CASE STUDY 5

Drug rescue: thalidomide as a treatment for myeloma

Thalidomide was initially used in the 1950s to treat morning sickness and as a sedative for insomnia but was withdrawn in 1961 after it was found to cause severe birth defects. It is thought that this was caused by the anti-angiogenic action of the drug, which prevents new blood vessels from forming, thereby affecting limb development. In recent years, interest in the mechanism of action of thalidomide has grown and the drug is now routinely used as a treatment for some types of cancer, in particular myeloma.¹⁹

As well as these non-traditional routes of developing medicines, research is increasingly focusing on combination therapies, which combine medicines with other pharmaceuticals or therapies to treat diseases and manage long-term conditions. A well-known example of combination therapy has been for the treatment of HIV/ Aids, but increasingly research is investigating this type of therapy for other diseases such as cancer,²⁰ malaria and cystic fibrosis.²¹ It is important that charities are aware of these opportunities to develop pharmaceuticals or biologics to bring new treatments to patients.

2.1.1 Changing how trials are undertaken

Industry is also looking for ways to accelerate the development of new treatments and therapies. One way of doing this is by changing how clinical trials are undertaken. Traditionally, clinical trials have been focused on the primary end point – a clinically significant treatment difference that is pre-specified in early planning of the study. Success of the trial is influenced by how well the original assumptions were made. However, new types of clinical trial are beginning to gain traction. One example is adaptive trial designs,²² which allow modifications to how trials are run based on the review of accumulating evidence within a study. This introduces greater flexibility and increases the likelihood that the trial will successfully answer the question for which it was originally intended. Adaptive designs also have the potential to speed up the drug development process. They are therefore of huge significance to industry as new agents could potentially be offered to patients sooner. An example of how one charity is funding a clinical trial with an adaptive design is described in the case study below.

CASE STUDY 6

Adaptive trial designs in cancer research

A trial²³ supported by an MRC-NIHR partnership with Cancer Research UK (CRUK) is testing different treatments for different subtypes of bowel cancer. The trial, FOCUS4,²⁴ has an adaptive trial design, meaning that it can be modified to enhance flexibility, ceasing randomisation to a specific arm, as well as prospectively and retrospectively changing a treatment arm if a new hypothesis comes to light. This can be achieved by amending the protocol while the rest of the trial continues, thereby speeding up the process by reducing unnecessary delays. Trials will be conducted across the UK, including through the Experimental Cancer Medicine Centres (ECMC) Network, a joint initiative between CRUK and the four UK health departments to support early-phase trials.

2.1.2 Changes to the regulatory environment

In order for the UK to remain an attractive place for research, it's important to maintain a flexible regulatory system that can respond to new and innovative research methods. Changes to the regulatory and drug discovery environment also have the potential to influence the way in which charities can engage with industry. By creating an environment more conducive to partnerships and encouraging research collaboration, the regulatory environment offers great potential to speed up the access to new drugs by patients. There are a number of initiatives that are currently being trialled:

Early access to medicines

Traditional clinical trials are the main way severely ill patients access the newest treatments, but many are excluded from trials because they have complicating factors, such as multiple pre-existing conditions. In April 2014, the government launched the Early Access to Medicines Scheme²⁵ to help make the UK a more attractive place for research. The scheme aims to help people suffering from some of the most life-threatening or debilitating conditions without effective treatments to receive new drugs sooner. The scheme, made up of three stages, involves:

1. Promising innovative medicine (PIM) designation: a PIM designation will be granted by the Medicines and Healthcare Products Regulatory Agency (MHRA) for treatments in areas of unmet clinical need that show early promising signs in phase I and II clinical trials. This may be for conditions for which there are no treatments available or where there is an identifiable subset of patients who don't respond to the current treatment. PIMs can be given to new biological or chemical entities as well as repurposed or recently approved drugs licensed for other conditions. Products with a PIM designation will be subject to early review and early clinical data will be available to MHRA and NICE to assess the benefits and risks of the treatment.
2. An early access to medicines scientific opinion: using the data collected during stage one, MHRA will issue treatments a benefit-risk scientific opinion at the end of phase II clinical trials. A positive opinion will allow prescribers to offer the medicine to patients where there are no therapeutic alternatives. As the system currently stands, medicines will be made available to patients free of charge by the sponsoring company. Carrying out the benefit-risk scientific opinion at the end of phase II clinical studies (rather than as now after phase III) could speed up the development of a drug by several months or even years.
3. Licensing and rapid commissioning: a coordinated NICE technology appraisal and NHS England commissioning process using data collected in the earlier stages will allow the assessment of medicines developed through the early access scheme to be fast-tracked. The new process will also allow manufacturers to make use of Pharmaceutical Price Regulation Scheme provisions for flexible pricing.²⁶ The medicines will typically be commissioned by NHS England through its specialised commissioning processes, meaning they will be available nationally.

Adaptive licensing

Adaptive licensing aims to speed up the licensing process for a specific group of drugs where there is a discrete patient need. It differs from the early access scheme, which focuses on drug availability before licences have been granted, but is similar in that both schemes are attempting to get drugs to patients sooner.

The European Medicines Agency (EMA) is undertaking an adaptive licensing pilot project²⁷ for drugs in early phase II clinical trials. Companies will be able to work with a wide group of interested parties such as sponsors, regulators, payers, patient groups and professional bodies if a drug is being developed for a serious medical condition where there is a high unmet need. The pilot will allow a prospective plan to be agreed that makes best use of existing flexibility in the regulations to ensure faster approvals, balancing this with the need to collect sufficient data about benefit-risk balance prior to launch. The plan will allow the medicine to be licensed for use first in a restricted patient population, with new patient groups/indications added as data about the drug are developed. The pilot will use existing legislation, and will also be used by the European Commission to examine whether there is any need for change in the existing EU legal framework.

As this project is currently in pilot, information is likely to change. For the latest details, see our briefing.²⁸

2.1.3 The importance of the patient voice

Traditionally, industry has had limited contact with patients but, in recent times, it has shown much stronger interest in gaining a thorough understanding of the 'patient voice'. At the same time, patient advocacy groups have grown in number and influence, and in some areas have become opinion leaders on key policy issues to campaign for better therapies. An example of how one such group has done this and is changing the research landscape is explained in the case study below.

CASE STUDY 7

Parent advocacy groups: influencing the landscape

The muscular dystrophies are a group of genetic disorders, which cause muscle weakness. Duchenne muscular dystrophy (DMD) predominately affects boys and men. Symptoms begin in early childhood and typically lead to death by the late twenties due to the failure of cardiac and respiratory muscles. There is currently no cure.

Parent Project Muscular Dystrophy is an advocacy group founded by family members of DMD patients. Frustrated by a lack of research on DMD, this group enlisted a committee of over 80 parents, researchers, clinicians and pharmaceutical executives to write guidance²⁹ for pharmaceutical companies on developing and researching new drugs. This is the first patient-initiated guidance that has been submitted to the Food and Drugs Administration (FDA) for consideration. It outlines common problems in undertaking clinical trials in DMD such as overly restrictive patient inclusion criteria, inappropriate clinical end points and excessive tissue biopsies that put patients off participating as their justification is often poorly explained.³⁰

It's clear that patients are also becoming much more informed about their conditions, changing the dynamic of the traditional doctor-patient relationship. Industry has long since recognised that there is little to be gained in developing new treatments that patients don't want or need and this change in the landscape has encouraged some industry groups to provide support to patient advocacy groups to explore their needs. Charities can play a key role in representing the patient voice because they are in the unique position of having direct contact with patients while at the same time funding medical research. Many charities go to great lengths to gain valuable insights into current treatments and the experiences of patients to identify where treatment gaps exist and where improvements are needed most, as explained in the next case study.

CASE STUDY 8

Leukaemia & Lymphoma Research: survey of patient need

Leukaemia & Lymphoma Research is undertaking a programme of work to understand the needs of blood cancer patients and gaps in current provision. The Prioritisation of Patient Need³¹ project aims to put patients at the heart of every investment it makes. Alongside an analysis of existing epidemiological and clinical research data, the charity has conducted a survey³² with patients, carers, family members and friends, as well as a number of focus groups across the country, to understand their needs and experiences. The questionnaire focused on areas of patient need during every stage of the blood cancer journey, assessing need in a variety of areas. National evidence suggests that there are varying levels of support and experience across the treatment pathway for patients with blood cancers, such as access to clinical nurse specialists and understanding explanations of what is wrong. Lauren Dias, research manager at Leukaemia & Lymphoma Research, says: 'We've been listening carefully to blood cancer patients, giving us an invaluable understanding of where improvements need to be made in the patient journey. The large-scale information we will gain from this comprehensive study will help us build on this insight and clearly identify areas where more support is needed.'

2.1.4 Patient records

The data stored in patient records represents another area where the changing landscape could influence industry–charity partnerships. AMRC has a position statement³³ on the importance of patient data as a valuable resource for health research. Access to these data allows researchers to understand disease better and to develop new treatments.

In the past, regulations governing the use of patient data have been ambiguous and, as a result, it can be difficult for researchers to follow them. The Health and Social Care Information Centre was established in April 2013 with the aim of collecting, analysing and presenting national health and social care data to improve patient care. This has the potential to be an excellent resource for research as it informs how the NHS can meet the greatest patient need. But there have been some well-documented concerns about how this information will be collected, stored and used. Building trust with the general public in relation to these issues is key to making this a success. Both charities and industry can encourage the safe and secure sharing of this information to facilitate research while protecting patient confidentiality.

2.1.5 Personalised medicine: will stratification make everything rare?

Personalised medicine classifies diseases according to their genetic make-up rather than their physical characteristics. This has allowed new insights into diseases and is enabling many conditions to be explored and differentiated more specifically so that treatments can be more accurately targeted, thereby replacing traditional 'broadbrush' therapies. Some charities are directly funding research in this area, as explained in the next case study.

CASE STUDY 9

Cancer Research UK: the Catalyst Club

The Cancer Research UK Catalyst Club³⁴ is a pioneering venture that's raising £10m towards research aiming to drive the development of personalised cancer treatment. Philanthropic members of the group are supporting a number of cutting-edge projects, including giving scientists access to advanced genetic technologies, the complete genetic mapping of oesophageal cancer, the charity's innovative Stratified Medicine programme³⁵ and TRACERx, the first ever study to track the genetic evolution of cancer.

While personalised medicine is potentially great news for patients, it presents new challenges for the research community. Researchers focusing on rare diseases are often faced with having insufficient patient numbers to drive clinical research – greater stratification will do little to help already small patient populations in research studies. Industry may also be anxious that smaller markets may result in higher per-patient prices, thereby increasing the need for strong justification to NHS payers. It's important that charities can step into this breach and offer researchers (where appropriate) access to their networks of patients and disease registries to reduce barriers to clinical research. An example of this is described in the case study below.

CASE STUDY 10

Muscular Dystrophy Campaign: Summit announces start of Duchenne muscular dystrophy trial

Summit Corporation plc (a biopharma company based in Oxford) has announced³⁶ that its phase Ib clinical trial of SMT C1100 in boys with Duchenne muscular dystrophy has started. SMT C1100 is designed to increase levels of utrophin in the muscles and researchers and clinicians believe this may compensate for the lack of functional dystrophin observed in Duchenne and Becker muscular dystrophy, regardless of mutation. This is the first time that a drug with the potential to increase utrophin levels has been tested in boys with Duchenne muscular dystrophy.

The trial aims to test whether different doses of the drug are safe and how well they are tolerated. It will take place at four sites around the UK and researchers aim to recruit 12 boys with Duchenne muscular dystrophy aged between five and 12 who will each receive one of three doses of the potential drug for ten days. As well as monitoring the safety of the boys, clinicians will measure the amount of the drug that enters the bloodstream – a crucial piece of information that will help the company plan a phase II trial, scheduled to start next year. Muscular Dystrophy Campaign is helping with the recruitment of patients to this trial to ensure the work can progress as planned.

2.1.6 Government sources of funding for innovative research

Innovate UK (formerly known as the Technology Strategy Board) is the UK's innovation agency that supports business development across a broad range of activities including health and care. Charities should be aware of a range of initiatives they offer:

- Biomedical Catalyst:³⁷ A joint initiative between Innovate UK and MRC offering funding to small and medium-sized commercial businesses and researchers to work individually or in collaboration to tackle important health challenges. The Biomedical Catalyst has awarded more than £180m since it was formed in 2012. More than 240 small and medium-sized companies and universities have received innovation support.
- Cell Therapy Catapult:³⁸ Established in 2012, the Cell Therapy Catapult focuses on building a world-leading cell therapy industry in the UK. The Catapult helps cell therapy organisations translate early-stage research into commercially viable products, and this is one of several with a specific focus on cellular medicine.
- Advancing regenerative medicines and cell therapies:³⁹ Innovate UK provides up to £8m in a single investment to a company to support research and development in regenerative medicines and cell therapies.
- Assisted Living Innovation Platform:⁴⁰ This wide-ranging programme enables the ageing population and those with long-term health conditions to live independent lives.
- Detection and identification of infectious agents:⁴¹ This initiative aims to encourage the development, uptake and adoption of clinically useful and commercially viable diagnostics for detection.
- Stratified Medicine Innovation Platform:⁴² This five-year programme aims to accelerate the development and uptake of stratified medicine in the UK. Up to £200m will be available to support innovative research and development in cancer profiling and treatment, biomarker identification, validation and adoption, and the uptake of medicines and diagnostics in the NHS.

While these initiatives are primarily aimed at businesses, charities should also be aware of them. Partnering with industry may provide an opportunity for charities to access these vital funding streams, so that new therapies can be developed to a stage where they are commercially viable.

2.2 Understanding why industry may not fund in certain areas

Although both charities and industry want to bring new medicines to market, the drivers can be very different. For charities, the health outcomes for patients is their primary concern. Industry, however, must also consider whether their investment will yield a sufficient profit and must therefore assess the costs of drug development, market size, sufficient and justifiable patient need and competition from other companies. This may mean that, for some disease areas, there are fewer opportunities for collaborations with industry.

Even in those areas where industry is interested, it may make a strategic decision not to fund in specific research areas because the project is deemed 'too early' or 'too high risk' to be pursued. Historically, this type of research was often conducted by industry using in-house facilities. In today's climate, such early-stage work is done in academic units, either as contract research or as pre-competitive research where the results will be made available in the public domain. This creates a diverse ecosystem of companies supporting drug, biotech and devices development.

Spin-outs often rely on short-term seed funding and investment from private donors and business angels who want to see relatively fast returns on their investment. The failure rate of spin-outs is high – sometimes because the research fails to show anything commercially interesting and sometimes because companies are started too soon, taking on high-risk, early-stage basic research that is too early for translation and not ready for commercialisation within the timeframes expected (typically five years). It's therefore important that ideas are developed to the stage where they have a 'proof of concept' and that as much risk as possible has been reduced to maximise the chances of commercial viability and the potential for additional funding to be secured if required.

2.3 What can charities do to help?

Charities can play a significant role in making otherwise unattractive research appealing to industry. While there isn't a one size fits all model, the following examples may give you some ideas about how your charity might entice industry to invest in your research area.

2.3.1 Funding early-stage research to de-risk ideas

Some charities have taken the strategic decision to fund early-stage research to reduce the commercial risks to industry. This may include funding to increase the accuracy of diagnostics, provide greater molecular definition of new drugs and their targets, or perhaps investigate the efficacy of repurposed drugs in new diseases. Some charities are doing this, as explained in the case studies below.

CASE STUDY 11

Cure Parkinson's Trust: funding pilot screening trials of repurposed drugs

The Cure Parkinson's Trust⁴³ has taken a pragmatic approach to supporting translational research. By examining existing drug targets and biochemical pathways, it identifies drugs that may be useful in Parkinson's disease (PD) and works with the relevant industrial partner to develop trials and funding models. Its Linked Clinical Trial initiative⁴⁴ carries out a number of parallel small pilot screening trials to determine which treatments merit larger phase III studies. Supported by an executive group of funders, and an international group of scientific experts who decide on which repurposed drugs or compounds should be moved into proof-of-concept phase II clinical trials, this initiative allows many clinical neurology centres around the world to be involved in one or more of approximately 20 different drug studies in PD patients.

CASE STUDY 12

Arthritis Research UK and NIHR: expressions of interest for first-in-disease and experimental medicine studies

Arthritis Research UK has issued a competitive call for proposals⁴⁵ to encourage partnerships between industry and academia to conduct first-in-disease clinical trials of investigational drugs for arthritis and other rheumatic diseases. This initiative has been developed in partnership with the NIHR Office for Clinical Research Infrastructure (NOCRI) and the Translational Research Partnership. It is hoped that the trials funded under this scheme will advance the understanding of arthritis and identify experimental drugs that may be developed into new treatments through proof of efficacy and mechanism of action studies. It is expected that the research projects will make use of Arthritis Research UK and NIHR-supported specialist infrastructure.

Professor Alan Silman, medical director and director of policy and health promotion at Arthritis Research UK, says: 'The pharmaceutical industry, particularly in North America, is the major source of innovative novel therapies for arthritis and rheumatic diseases. This new partnership will ensure that these first-in-man drugs for arthritis are thoroughly investigated for effectiveness by the leading academic clinical groups in UK universities. It could result in some truly exciting new breakthroughs in the treatment of many different types of arthritis.'

2.3.2 Developing funding streams to support translational research

Many charities are aware of the funding gap to develop important laboratory discoveries into viable treatments in the clinic. Some funders have therefore introduced schemes to help to bridge this gap, as discussed in the following case studies.

CASE STUDY 13

Wellcome Trust: funding schemes to support innovation

Innovations is a division of the Wellcome Trust offering a suite of funding opportunities⁴⁶ to support researchers to develop early-stage ideas. Proposals must address unmet medical needs and secure healthcare benefits by developing novel technologies and products. Funding decisions are based on the validity of the scientific proposal and the benefit to patients, not on the potential profitability of the end product. The Trust encourages researchers from academic institutions, companies, and clinical and end-user settings to collaborate during the award life-cycle. There are several funding schemes on offer, four of which are listed below:

- Translation awards:⁴⁷ to develop innovative and paradigm-shifting new technologies in the biomedical area
- Seeding drug discovery:⁴⁸ to develop drug-like, small molecules that will be the springboard for further research and development by the biotech and pharmaceutical industry in areas of unmet medical need
- Pathfinder awards:⁴⁹ to encourage effective partnerships between the public sector and a company partner to undertake pilot studies, simulate product development in orphan or neglected diseases, and to share knowledge to de-risk projects
- Health Innovation Challenge Fund⁵⁰: a partnership between the Wellcome Trust and the Department of Health to progress innovative healthcare ideas from proof of concept to early phase clinical studies in man.

CASE STUDY 14

Cancer Research UK: funding translational research

Cancer Research UK has a number of funding initiatives to increase translational research. The Translational Cancer Research Prize⁵¹ recognises an outstanding translational research team that has made seminal cancer research discoveries at the cutting edge of scientific novelty. The prize-winning team is expected to be multidisciplinary, comprise both clinical and non-clinical members, and may belong to different institutions, although a significant proportion of work must be carried out in the UK.

2.3.3 Investigating drug company 'back catalogues'

Science has progressed so rapidly in recent years that there are more compounds available than there are commercial resources to investigate them. The time and investment needed to develop therapies means that only the most promising are taken on by industry. Charities can play a role in investigating these alternative compounds (often referred to as 'back catalogues') to determine whether they are of clinical interest, as described in the next case study.

CASE STUDY 15

Cancer Research UK: utilising drug company 'back catalogues': AZD0424

In 2006, Cancer Research UK and Cancer Research Technology, the charity's development and commercialisation arm, established a clinical development partnerships⁵² (CDP) initiative to increase the number of successful new treatments for cancer by taking undeveloped anti-cancer agents from industry and putting them into clinical trials. The initiative is primarily targeted at leading pharmaceutical and biotechnology companies which have a large pool of molecules that may have anti-cancer properties. These companies have to prioritise the agents they take into clinical development, which leaves potentially effective treatments on pharmaceutical companies' shelves. CDP will take promising but 'de-prioritised' anti-cancer drugs into early-stage clinical trials through Cancer Research UK's Centre for Drug Development (formerly Drug Development Office). Effectively, the charity 'borrows' a drug from a company and conducts early clinical trials at no cost to the company. If the drug looks promising, the company retains the option to develop and market the drug, with the charity receiving key milestone payments and a share of any revenues. One such trial⁵³ involves an AstraZeneca drug called AZD0424, which may offer treatment for solid tumours that have continued to grow despite other treatment or for which no standard treatment is available. AZD0424 works by slowing down or stopping the activity of proteins called Src and ABL1 involved in cell growth. The trial aims to establish the highest safe dose of AZD0424 and investigate its side effects.

2.3.4 Joint funding schemes between charities and industry

Joint funding schemes between industry and charities are less common but examples do exist where collaboration at this level has been managed successfully. These are often referred to as public-private partnerships, which aim to generate innovative approaches to tackle research questions by marrying expertise from the public and private sectors. Public-private partnerships use public, private and voluntary sector funding for industry and academically led research that would not normally be undertaken by each party independently. It can therefore be seen as a facilitator for research, combining knowledge and funding to collaborative research projects in pursuit of a common goal, as illustrated below.

CASE STUDY 16

Medicines for Malaria Venture

The Medicines for Malaria Venture⁵⁴ (MMV), whose contributors include the Bill and Melinda Gates Foundation, the Wellcome Trust and the International Federation of Pharmaceutical Manufacturers & Associations, provides support for the discovery, development and delivery of new medicines to treat and prevent malaria. MMV has worked with more than 300 partners in over 50 countries from the public sector, the private sector, NGOs and non-profit organisations, as well as from clinical centres, to tackle key malaria research questions.

2.3.5 Funding pre-competitive research

Some charities are funding 'pre-competitive' research with industry. Traditionally, drug companies are known to be quite secretive about their research programmes, but in recent times they have become much more open with researchers and charities to validate new targets. This 'pre-competitive' work has opened up new opportunities for collaboration, as discussed in the case studies below.

CASE STUDY 17

Alzheimer's Research UK Dementia Consortium: bringing different sectors together

The Dementia Consortium⁵⁵ is a pre-competitive research partnership that aims to speed up the development of new drugs for dementia by supporting research on novel targets for neurodegeneration. It brings together the Alzheimer's Research UK, Eisai, Lilly and MRC Technology to tackle the growing dementia problem, closing the gap between fundamental academic research and the pharmaceutical industry's drug discovery programmes to develop a new dementia treatment. The consortium provides funding, expertise and resources to support new drug targets emerging from academic research that hold the promise of patient benefit.

CASE STUDY 18

Charities funding pre-competitive research

The Structural Genetics Consortium⁵⁶ (SGC) is a pre-competitive public-private partnership that has released 3D structures of over 1,500 medically important proteins and developed 20 protein inhibitors – all of which are freely available publicly. SGC is partnering with the CHDI Foundation,⁵⁷ an American not-for-profit organisation that develops drugs to slow to progression of Huntington's disease. The agreement in this partnership includes legal provisions against intellectual property (IP) – ie the research is patent free. The CHDI Foundation will support two scientists over two years (up to the value of \$500,000) to work at SGC studying protein structures and potential therapeutic agents at the University of Toronto and the University of Oxford.

Some UK charities are involved in similar partnerships.⁵⁸ The Institute of Cancer Research (ICR), Newcastle University and the Oxford SGC, with funding from Cancer Research UK and the Avon Foundation, are researching new histone demethylase enzyme targets that have been implicated in various types of cancer. If successful, a lead drug compound could be tested in early-phase clinical trials without any IP claims. This patent-free arrangement is, however, only likely to be compatible under certain circumstances when the research is cure focused and linked with a not-for-profit organisation.

2.3.6 Supporting research infrastructure

As well as funding research directly, some charities also support infrastructure such as laboratories, specialised equipment, clinical trial units and patient registries. This can be very attractive to industry, particularly those looking to invest in specific disease fields.

Patient registries contain a huge array of information that may allow research to be undertaken that otherwise would not be possible. They can also be of great importance to industry looking to invest in new areas, particularly in rare diseases. The case study below describes one example of a patient registry where the data collected is informing research on long-term trends in a rare disease area and how well different types of treatment work.

CASE STUDY 19

Society for Endocrinology: the UK Acromegaly Register

The Society for Endocrinology manages the UK Acromegaly Register,⁵⁹ which was established in 1997 to facilitate epidemiological and therapeutic research in acromegaly – a rare disease caused by a growth hormone secreting pituitary tumour. Acromegaly is associated with increased morbidity and mortality. If the condition initiates in childhood, it leads to gigantism. The registry has recruited over 3,200 patients from 32 endocrine centres across the UK. Its aims are to gather prospective and retrospective data on a large series of patients with acromegaly to inform on treatment options and long-term mortality and morbidity in patients with the condition. The registry will also help to provide data on treatment outcomes (medical treatment, surgery and radiotherapy), and to disseminate information across the research landscape, including patient support groups. The registry is supported by the pharmaceutical company IPSEN.⁶⁰

2.3.7 Charities funding with industry

For neglected diseases or those without large markets, the commercial drivers may be too weak to encourage industry investment. As a result, some charities, particularly in the USA, have provided funding directly for biotechnology firms in the hope that they will step into the translational research gap left by a cautious pharmaceutical industry.

CASE STUDY 20

JDRF: funding KalVista

UK biotech company KalVista and JDRF have formed a research partnership⁶¹ to develop a plasma kallikrein inhibitor for the treatment of diabetic macular edema (DME) - the leading cause of visual loss for people with type 1 diabetes. A candidate called KVD001 was selected from a series of novel small-molecule plasma kallikrein inhibitors that yielded promising results in preclinical studies. It has just entered a phase I, first-in-human clinical trial in an ascending dose-escalation study to determine safety, tolerability and pharmacodynamic activity.

JDRF will provide up to \$2.2m in financial support and research expertise to KalVista. JDRF's investment is a structured, milestone-driven agreement; payments are only made to KalVista on delivery of each milestone. Although JDRF's investment is a relatively small proportion of the total needed to support this element of KalVista's research, it demonstrates the charity's faith in the project and its willingness to co-fund a pharmaceutical company to help develop this potential treatment as a viable option for patients suffering from DME in the future.

Although uncommon in the UK, there are examples of charities funding with an industry partner to develop products that will benefit patients and advance their charitable objectives, as discussed below.

CASE STUDY 21

Action on Hearing Loss: funding Otomagnetics LLC

Through its Translational Research Initiative for Hearing (TRIH), Action on Hearing Loss funded a three-year project to develop a new method to deliver steroids to the inner ear for the treatment of sudden sensorineural hearing loss. The project, carried out between Otomagnetics LLC and the University of Maryland, USA, will investigate a novel system that uses a magnetic field to 'push' steroid-carrying nanoparticles across the inner ear membranes and into parts of the ear that are not normally accessible. This is one of three projects funded under TRIH supporting projects in the early stages that aim to turn research discoveries into potential new treatments for hearing loss and tinnitus.

3. Developing the foundations for a research partnership

- **Charities should discuss their principles for partnerships with trustees, supporters and stakeholders before beginning discussions with potential industry collaborators.**
- **It is important to have a clearly defined research strategy that sets out what a charity wishes to achieve and with whom.**
- **Charities should be open and transparent with the public about the partnerships they form.**
- **Charities should consider the best way to attract an industry partner. Some approach industry through their academic networks, while others prefer a more direct route through organised networking and partnering events.**
- **Charities should aim to have an established point of contact in their partner company and a specific member of staff who leads on managing the relationship.**

This chapter looks at the foundations required for developing research collaborations with industry. We've included guidance on talking to your trustees and on how to ensure that your aims of working collaboratively are reflected in your research strategy. If you are thinking about working with an industrial partner, you should have a thorough understanding of the company prior to seeking support. We encourage you to think carefully about what you are trying to achieve and whether this complements your potential partner's objectives. You may wish to consider:

- The size of your charity and industry partner and the practicalities of entering partnerships
- What your proposed partner specialises in – drug development, medical technologies or novel therapeutics, for example. These should match your objectives and the research you want to support.

3.1 Talking to your trustees

Before formalising any collaboration, you must have the support of the charity's trustees because they hold overall responsibility for the charity's operations. Before entering such discussions, you should have considered a number of factors:

- How working with an industry partner will help you meet your research strategy
- How you intend to approach or attract industry to your charity
- Which companies (if any) you already have in mind
- Potential risks and rewards and how you will manage these
- Contingency plans in the unlikely event that the partnership runs into difficulties.

3.2 Talking to the public and your supporters

It's also important to consider public perception of working with industry and how you may handle any potential questions. Some members of the public may take the view that industry is focused purely on profit rather than patients and may question why your charity is pursuing such links. Our three principles of partnering – integrity, independence and transparency – should help provide reassurance for the public that such partnerships are important for the charity to meet its aims. Our recent survey with charities on working with industry (appendix 1) also revealed that public perception of working with industry was of less concern than other challenges such as finding the right industry partner and balancing risk and reward.

An example of how one charity is talking to the public about their industry partnerships is described in the next case study. This highlights the importance of transparency when explaining your reasons for pursuing such links.

CASE STUDY 22

Myeloma UK: informing the public about how it works with industry

Myeloma UK is open with the public and its supporters about its work with industry. The charity's webpage⁶² and associated guidance⁶³ set out how such relationships are formed and how collaborations with pharmaceutical companies help fund its Clinical Trial Network.⁶⁴ Established in 2009, the network takes a structured and prioritised approach to early-phase myeloma clinical studies to shorten the time to bring new treatments to patients. The network also plays a role in bringing together the NHS, the NIHR Health Technology Assessment, patients and the pharmaceutical industry in a collaborative and strategic manner. So far, seven trials have been developed through the network, which has attracted collaboration with six industry partners. Funding from pharmaceutical companies helps to support the running costs of the trials, the running of the clinical trials office and the costs incurred by the participating hospitals. All trials are investigator led; the overall design and conduct of the trials therefore rest with clinicians rather than with industry. Myeloma UK remains completely independent throughout the process and articulates this arrangement to provide reassurance to its supporters and the public.

3.3 Your research strategy: attracting industry involvement

All AMRC members have a published research strategy setting out priorities for the research they wish to fund and why. A good research strategy links funding activities with the charity's mission and objectives, allowing supporters to see how research funding will make a difference to the charity, its wider goals and, most important, the people it supports. It also puts a charity's funding in context of other funders in the research sector.

Before setting out to build collaborations with industry, you should ensure that your strategy is comprehensive and up to date. If working with industry is part of the charity's plans to deliver its research strategy, you should be explicit about this and explain why. Two case studies of charities with research strategies emphasising the importance of working collaboratively with industry follow.

CASE STUDY 23

Alzheimer's Research UK: a strategy for collaboration

Alzheimer's Research UK's research strategy⁶⁵ has a specific focus on connecting researchers with the pharmaceutical industry and on developing partnerships and collaborative working. Its strategy sets out four key priorities:

1. **Responsive and targeted funding:** supporting basic and clinical research to improve the understanding of all causes of dementia, as well as contributing to better diagnosis, prevention and treatment.
2. **New drug discovery:** funding for the first critical phases of drug discovery to find promising new targets; this involves connecting researchers with pharmaceutical companies to speed up drug development. The charity also encourages collaborations with other funders.
3. **Strategic projects:** funding to develop new opportunities, including a longitudinal study to help detect the earliest stages of disease.
4. **Partnerships:** working nationally and internationally to pool expertise and make better progress through collaborative working.

CASE STUDY 24

Cystic Fibrosis Trust: leveraging support from working with industry

Cystic Fibrosis Trust's research strategy⁶⁶ outlines what the charity wants to achieve over the next five years and the ways in which it plans to meet its targets. One mechanism involves it awarding venture and innovation grants. These awards will be used to leverage funding from external sources including industry and NIHR. Examples could be 'pump-priming' funding to demonstrate project feasibility – something that industry may be particularly keen on if a proof of concept study has already been undertaken. The charity also plans to build capacity for undertaking clinical trials. This is another step towards attracting industry to cystic fibrosis research and the charity's pre-existing networks for the running of large-scale clinical trials.

Cystic Fibrosis Trust's strategy outlines how the charity will stay alert to new developments in the field through 'research sandpits', designed to bring together researchers, industry and other funding bodies from a wide spectrum to stimulate new thinking and innovation. This offers further opportunities for it to engage with industry to solve the most challenging research questions.

3.4 Making the approach

Developing a partnership requires time and commitment from each partner, as well as a 'shared language' and understanding of each other's aims and objectives. But it can be difficult for charities new to this space, which may be unsure about how to begin discussions with industry.

3.4.1 Attracting industry to your charity

Many charities find it difficult to know which companies to approach and how to attract them. There is no one size fits all approach, and many partnerships are born of different circumstances, each specific to an individual collaboration. On the following page are just a few examples of how charities are attracting industry to their respective fields.

CASE STUDY 25

Action on Hearing Loss: acting as a broker between researchers and industry

Some charities find that there is little industry involvement in developing treatments for the condition they represent and therefore want to find ways in which they can encourage and support industry involvement. Action on Hearing Loss⁶⁷ spends £1.6m each year on biomedical research on treatments to protect and restore hearing and silence tinnitus. It recognises that the involvement of industry is vital to achieving its goals but that, without a well-trodden path through clinical trials to market, companies are often reluctant to invest in developing promising lines of research.

In response, the charity set up the Translational Research Initiative for Hearing (TRIH),⁶⁸ designed to bring together universities, pharmaceutical companies and people with hearing loss to strengthen and encourage translational research. The initiative acts as a catalyst by:

- Funding translational research that will de-risk and add value to promising new treatments, making them more commercially attractive to industry
- Sharing with industry opportunities to collaborate and invest in promising lines of research
- Providing industry with market intelligence and with links to researchers and clinicians to strengthen their research and business plans
- Providing industry with access to people with hearing loss to speed up patient recruitment to clinical trials and help them understand patient needs.

CASE STUDY 26

Leukaemia & Lymphoma Research: improving existing infrastructure to attract industry

The Trials Acceleration Programme⁶⁹ (TAP) is Leukaemia & Lymphoma Research's innovative long-term investment to deliver more promising treatments to blood cancer patients through early-phase clinical trials. The network, worth around £8m over the next five years, consists of 13 leading research centres around the UK, coordinated by a central hub in Birmingham. This investment supports an expert team of scientists based at the hub, skilled at setting up clinical trials, and a range of nationwide research support staff to run the trials smoothly locally. The hub team prioritises and leads the set-up and governance of trials and ensure that new studies open at each of the 13 treatment centres efficiently and simultaneously. The hub and spoke model allows a large collective catchment area to recruit adequate numbers of patients more efficiently, so more patients have the opportunity to access emerging treatments, wherever they live in the UK. This targeted acceleration aims to see blood cancer clinical trials being completed within two years – a significantly shorter time than most early-phase clinical trials. It also creates space for more clinical trials than would have happened otherwise, especially those in rarer diseases and patient subgroups. There is a strong incentive for industry partners to undertake and contribute to clinical studies using this network, significantly speeding up the research process and reducing industry costs.

CASE STUDY 27

UK Kidney Research Consortium: facilitating trials through clinical study groups

The UK Kidney Research Consortium⁷⁰ (UKKRC) was set up by the Renal Association and Kidney Research UK in 2007 to support the development of clinical and translational research in kidney diseases in the UK. The consortium set up a platform for clinical study groups to generate and assess studies in specific renal specialities and, where appropriate, to secure interest and collaborative funding from a variety of sources. UKKRC has an important relationship with the UK Clinical Research Network (UKCRN) Renal Specialty Group, which supports clinical research and helps to facilitate the conduct of trials in the NHS. A major remit of the group is to review potential studies, particularly from industry, for inclusion on the UKCRN portfolio. This could be of particular interest to industry as portfolio studies are eligible for infrastructure and other support from each comprehensive local research network.

3.4.2 Approaching industry through your academics

Industry often has strong links with academia, allowing it access to the wider scientific community and cutting-edge academic research. As charities fund predominantly in an academic environment, universities can provide the 'hotbed' where charities and industry overlap in terms of research activity. It's also in academia's interest to attract investment from multiple sources. In some cases, academic institutions market themselves as 'open for business' to boost collaborative working. This may attract external investment, as explained in the case study below.

CASE STUDY 28

Imperial College London: SME collaborations

Imperial College London is encouraging collaborations⁷¹ with SME organisations as part of Innovate UK's (formerly the Technology Strategy Board) innovation voucher⁷² scheme and the European Commission Horizon 2020⁷³ funding call. In a letter to SMEs, it particularly welcomed industry involvement in specific disease areas and outlined the types of studies for which it envisaged applying for funding. This is a really positive example of how universities can attract SMEs to work with academia, acting as a conduit between charities and industry to foster collaborations.

In our recent survey (appendix 1), many charities reported that they had developed collaborations with industry through their academic contacts. These collaborations were often independent arrangements, with charities providing funding and industry committing to provide drugs, educational grants or support for labelling, packaging and clinical trials administration. In these circumstances, it's normally the academic researchers who instigate collaborations and deal directly with the industry partner. This offers charities the opportunity to find out more about developing research partnerships with industry before committing to more formal collaborative working. In the example below, one charity also received funding from industry through their academic contact.

CASE STUDY 29

Fight for Sight: partnering with industry through academia

In 2013, Fight for Sight peer reviewed a project grant that was deemed worthy of funding but the charity had insufficient funds to support it. The applicants then contacted Novartis, which had previously supported their research and, after negotiations, they agreed to co-fund the project up to £137,000. This enabled the project to go ahead, showing how academic contacts can leverage industry investment.

3.4.3 Organised networking and partnering events

Some charities prefer to engage with potential industry partners through organised networking and partnering events. During 2013 and 2014, AMRC held several workshops with the BioIndustry Association and OBN bringing charities and companies together to stimulate new collaborations. We will continue to host these in future. Some organisations arrange more formal events, for example:

- OBN's BioTrinity Biopartnering and Investment Conference⁷⁴ attracts investors, pharma, drug development, technology and R&D companies. Such platforms offer charities an opportunity to meet companies with a view to developing productive research collaborations.
- Bio-Europe Spring⁷⁵ is a partnering event aimed at providing life science companies with partnering opportunities.
- In the USA, events such as Partnering for Cures⁷⁶ bring together organisations from across the medical research sector to initiate collaborations and inform philanthropic investment.

The experience of two AMRC member charities at these meetings are summarised in the case studies below.

CASE STUDY 30

British Heart Foundation: BioTrinity and FasterCures

The British Heart Foundation's (BHF) translation research team has attended BioTrinity and FasterCures⁷⁷ partnering meetings since 2012. Its main objective has been to gain greater understanding of the translational research environment and how best to structure a new BHF award to meet milestones targeting the needs of both industry and the regulatory authorities. These meetings have enabled BHF to meet clinical research organisations (CROs), venture capital companies (VCs) and potential industrial partners investing in cardiovascular disease. BHF sees this as increasingly important because charities, CROs and VCs fund most early-stage 'high-risk' research, with industry entering only at a later stage of development.

From these meetings, BHF has gained a better understanding of the strategic business priorities of industry, the conditions that need to be in place for a project to be a desirable investment and how best to achieve this by harnessing appropriate CROs and VCs at the right stage of a project.

CASE STUDY 31

Parkinson's UK: Partnering for Cures

Parkinson's UK attended Partnering for Cures in 2013, where it presented plans for the use of big data to support the discovery of biomarkers. It has subsequently become a member of FasterCures' TRAIN network,⁷⁸ a group of not-for-profit organisations aiming to tackle cross-disease challenges to catalyse innovation. The experience has been important for the charity as it develops a new research strategy, providing it with a clearer vision of the role of patient organisations in pulling together the whole community as 'honest brokers' and driving change. Attending the meeting also put it in touch with various partners in the drug development process, and charity representatives have subsequently been invited to speak on a range of platforms, particularly in biotech, allowing them to engage with new audiences.

3.5 Beginning discussions with industry

Early on in discussions, it's important to consider who you will be dealing with and whether they will be your regular point of contact. Charities collaborating with pharmaceutical companies should bear in mind that these are often global enterprises with many different departments and some use public affairs consultancies to deal with particular aspects of their business. During the first stages of discussions, it's worth establishing who the main point of contact is (for both industry and charity) and agreeing a channel of regular and direct communication. We also recommend following the ABPI code of practice,⁷⁹ which is very helpful for understanding the considerations of your industry partner.

National Voices and ABPI have produced a guide for charities and patients groups wishing to collaborate with the pharmaceutical industry.⁸⁰ This document works in harmony with our guide, focusing on the principles of collaboration and the way in which charities, patient groups and industry are expected to work when undertaking collaborative projects.

There are also some other points you may wish to consider, discussed below.

3.5.1 Knowing your partner

As with any collaboration, it's important to research the company thoroughly before entering any partnership. If your potential industry partner is funding organisations or institutes whose ethos and principles don't align with yours, you may wish to reconsider whether this is an appropriate choice because it could introduce conflicts. Some charities have formal policies outlining where they will or will not develop collaborations, as discussed in the case study below.

CASE STUDY 32

British Heart Foundation: relationships with tobacco companies

The British Heart Foundation (BHF) has a policy⁸¹ not to have corporate partnerships or relationships with tobacco companies owing to the strong association between smoked tobacco and ill health, including coronary heart disease. The activities of the tobacco industry act in opposition to the BHF mission of tackling heart disease and its vision of a world in which no one dies prematurely of heart disease. As such, partnerships of this type would undermine BHF's role in tobacco control issues.

BHF doesn't accept any matched funding from tobacco companies or funds raised by tobacco companies through fundraising. Furthermore, BHF policy states that researchers funded by the tobacco industry cannot use its equipment and facilities. Similarly, when BHF considers major new funding awards, any association of the applicant or their institution with the tobacco industry is taken into account.

3.5.2 Being clear about funding processes

All AMRC members use peer review⁸² in their allocation of research funding and therefore appoint a panel of external experts to assess funding applications. The membership of these committees is drawn largely from the academic community, although many charities also involve other stakeholders such as patients. Being transparent about your funding processes will help you explain to potential industry partners how you support research and that all projects are assessed to the same standard. It's important that the same level of peer review is applied to all research (including projects carried out in collaboration with industry). As long as these principles are followed and the quality of the funding process is maintained, charities should feel confident in partnering with industry.

Funders usually have 'reactive' funding mechanisms in place – ie responding to specific areas of unmet need. Some charities are beginning to take more proactive measures, such as commissioning research to answer specific patient-focused questions or developing specific research themes to support strategic needs. Industry–charity partnerships often fall into these categories. Including industry experts in these discussions can help shape the way the charity undertakes research so that it's ready for commercialisation.

4. Developing the agreement and managing the partnership

- Collaboration with industry should be subject to a clear agreement outlining each partner's expectations. Agreements may differ depending on the types of partner involved.
- Partnerships based in academia may involve a dedicated research services team with experience in contract negotiations. In some cases, this will involve a technology transfer office. Standard agreements are often preferred.
- Collaborations based in the NHS may involve interactions at local, regional and national levels, for example through an academic health science centre/network or directly with NHS bodies. Where possible, trusts use Department of Health-approved model agreements for clinical research.
- Charities should consider issues around conflicts of interests, intellectual property (particularly in terms of publishing and ownership of results) and endorsement of products. They should also be clear about the use of their logo and any of their materials by the industry partner.
- Charities should discuss the logistics of monitoring the collaboration and payment schedules with the industry partner early on in the partnership. If you have agreed that your scientific advisory committee will monitor progress, they may need to sign confidentiality disclosure agreements.
- Charities should report their research collaborations and the financial contributions received from industry in their annual reports and accounts. Similarly, industry should publicly indicate financial supports for research partnerships in line with the ABPI code of practice.

After you have agreed with an industry partner to work together, the next step is to form an agreement that clearly sets out the main aims, roles, responsibilities and expectations of everyone involved. In this chapter, we take a look at the common types of agreement and things you may wish to consider when undertaking collaborative working. Before doing so, it's important to point out the difference between an agreement and a memorandum of understanding. Both these terms are used frequently but mean slightly different things:

- A memorandum of understanding (MoU) describes the agreement between parties and sets out the working principles of the relationship. MoUs are often a preliminary document to the contract and generally aren't meant to be legally binding but you should check this before signing.
- An agreement (often called a research contract) sets out the roles and responsibilities of each partner on a specific research project or set of projects. It defines what work will be undertaken and includes the terms and conditions governing the conduct of the project.

4.1 Forming the agreement

Written agreements should be drawn up at the beginning of a partnership and work should not begin until they have been agreed by both parties and signed. The purpose of an agreement is to set out the obligations of the host institution, the funders and all other parties in the collaboration – this could include the charity, the industry partner, government bodies, the university and/or the NHS and any other body involved. Common elements in agreements include:

- Defining the partnership or collaboration
- Confidentiality
- Research activity to be performed including timescales of the research
- Budget and financial considerations
- Intellectual property (IP) rights
- Publications
- Conflicts of interest
- Exploitation
- Working with other partners and exclusivity clauses
- Limits of liability
- Acknowledging support.

Forming the agreement can be a tricky process and is often perceived as a stumbling block. Our recent survey (appendix 1) suggested that charities often instigate discussions on developing these agreements. This may be linked to the fact that many have policies in place for many of these areas, including the handling of conflicts of interest, IP and publication expectations. Regardless of who is leading, it's important that the finalised agreements are given a thorough check by a legal team to check that the language and content is suitable before signing.

It's worth pointing out that the process of developing an agreement may differ depending on where the research is being undertaken and which organisations are involved. In the next section, we look at some of these differences you may come across.

4.2 Working in academia

Many charities fund research in academia and will therefore need to develop agreements with industry partners with the academic context in mind. When developing these types of partnerships, the university's research service department will usually deal with contractual frameworks, although in some universities, technology transfer offices (TTOs) may also be involved.

4.2.1 Research service departments

A university's research service department (sometimes called the research operations office or research and development office) assists with the administration of externally funded research, managing grants from the academic side throughout the cycle. The department will be involved in the agreement process, negotiating terms with all parties involved to make sure everyone's rights and responsibilities are clear. Usually acting on behalf of the university, it also deals with contractual issues including IP, publication and liability.

4.2.2 Technology transfer offices

Some universities have a technology transfer office (TTO) to identify research that could become commercially interesting. TTO staff often range across disciplines, including economists, lawyers, marketing specialists and in-house IP experts. They help protect the IP of commercially interesting research and work with researchers to ensure that early-stage ideas are sufficiently developed to the point where they become attractive and economically viable products for industry.

TTOs may have numerous roles in developing new technologies – they can help to set up joint ventures and partnerships, raise venture capital to fund the development process and directly invest in spin-out companies. They can also help in licensing out new technologies for further development to the stage where commercialisation becomes a viable option. How one such TTO operates is illustrated in the case study below.

CASE STUDY 33

Imperial Innovations: a technology transfer office

Imperial Innovations⁸³ was initially founded as the TTO for Imperial College London before becoming a wholly owned subsidiary of the university. It works with the NHS trusts linked to the university, including Imperial College NHS and North West London Hospital Trusts. Imperial Innovations commercialises academic research and has made investments in early-stage technology businesses based on IP developed with the universities of Oxford and Cambridge and University College London. Over the past ten years, it has raised over £206m from investors, enabling it to support a range of spin-out companies. One particular success is RespiVert, a small molecule drug discovery company, which was sold to what is now Janssen Biotech, resulting in £9.5m gross cash proceeds.

4.2.3 Types of agreement in academia

When working in an academic environment, it can be helpful to use model agreements. There are many different types of template, designed for use across a range of partnerships. Some universities prefer to use their own versions developed in-house and often have a suite of model agreements depending on the research project and type of partner. There are, however, several standard cross-disciplinary agreements that can be helpful.

Brunswick agreements

The Brunswick Group⁸⁴ has a number of template agreements for use in academia. These are in the public domain and can be used freely and adapted as necessary. There are three types:

- Material transfer:⁸⁵ for use between two universities (or similar) for the transfer of materials. These have a minimal approach and aren't suitable for clinical materials where there may be IP considerations, or for the transfer of materials between a university and commercial partner (Lambert agreements are used for these – see below). There are two subtypes of transfer templates: one for materials, the other for human tissue.
- Research collaboration:⁸⁶ for use predominantly where two or more universities receive a joint grant. These may be useful where your collaboration involves multiple academic partners.
- Studentship:⁸⁷ for use between a university and a company for supporting a postgraduate research student.

Lambert agreements

The Intellectual Property Office⁸⁸ (IPO) is the government body responsible for granting IP rights in the UK. IPO has developed a suite of model agreements for collaborative research projects in academia. These Lambert agreements⁸⁹ cover key elements including ownership and the right to use the results from a project, financial (and any other) contributions made by the commercial sponsor and the university's right to use the results of a study for academic purposes. There are various different types of Lambert agreement for use in different circumstances:

- Five model research collaboration agreements⁹⁰ provide a range of options in relation to the right to publish and ownership and exploitation of IP.
- Four model consortium agreements⁹¹ are used when more than two parties are collaborating (either more than one university or more than one industry partner).

Choosing the right agreement will vary depending on the circumstances of the partnership. However, IPO has produced a decision guide⁹² and guidance notes⁹³ on each agreement to help identify and resolve issues early in discussions.

4.3 Working in the NHS

Undertaking research in the NHS can be complex, involving multiple bodies, networks and organisations, and often overlays academia and industry. It's this aspect that can be difficult for funders and researchers to navigate, as the challenge of coordinating organisations can increase the time spent and costs involved in setting up a research study. To avoid this becoming a disincentive to conducting clinical research, it's important to have a good understanding of the main infrastructure of the NHS before undertaking collaborative working.

Currently, 77 AMRC member charities fund research in the NHS, accounting for 30 per cent of research undertaken in this setting.⁹⁴ Charities support a diverse array of clinical infrastructure such as labs, centres of excellence, tissue banks and clinical trial units. When deciding where it would be best to undertake clinical research, you should consider whether these vital charity-funded resources could facilitate your partnership.

The government also supports a significant amount of research in the NHS. NIHR is the funding mechanism through which the Department of Health supports research in the NHS, with an annual budget of nearly £1bn. During 2011/12, this budget included £202.2m for research,⁹⁵ across a broad range of programmes and initiatives, and £609.5m for infrastructure.⁹⁶ There is a large number of NIHR organisations, covering different aspects of research coordination and delivery at a national and local level, some of which are described in our directory of organisations.⁹⁷

MRC is also a significant funder of research infrastructure.⁹⁸ If you are undertaking a partnership in a specific disease area, you may wish to consider whether working with or alongside MRC can help you to progress your collaboration with industry.

Regardless of the funder, clinical research infrastructure is often specialised in specific disease areas and can be linked to separate or integrated academic and clinical providers (for example, the NHS trust may not be same as the university). When planning your collaborative work in a clinical setting, it's worth spending time working out where you need to work to be most effective.

4.3.1 Academic health science networks

Building partnerships in an NHS setting has traditionally been challenging. In response, the government set up 15 academic health science networks⁹⁹ (ASHNs), each with a different focus,¹⁰⁰ to bring together everyone involved in healthcare, including local NHS organisations, universities, industry and charities, to foster innovation in the NHS. ASHNs are intended to change the way in which the NHS identifies, develops and adopts new technologies across all areas of healthcare to improve patient and population health outcomes. An initiative from one AHSN is described in the case study below.

CASE STUDY 34

AHSNs: providing the infrastructure for industry working in the NHS

Kent, Surrey and Sussex AHSN are working to provide industry with clear points of access to the NHS through their Navigator Advisory Service¹⁰¹ to enable the best ideas to be spread and adopted across the region. They also hold partnership days focusing on a range of specialised topics that bring together companies keen to develop and introduce innovations in the NHS. This commitment to research and adoption of innovations can help the NHS deliver new treatments to benefit patients and share best practice.

4.3.2 Model agreements for clinical research

NIHR has developed a series of model agreements¹⁰² for clinical research in partnership with, and specifically for, industry. If you are partnering with industry for clinical research, these may be very helpful, saving both time and money, as they remove the need for drawing up site-by-site reviews and local legal agreements. This can also speed up the approval process, as many NHS trusts have a 'fast-track' system for unmodified model agreements so that trials can start earlier.

There are currently a suite of model agreements with associated guidance setting out how they should be used:

Agreement	Description
Model clinical trial agreement (mCTA) for pharmaceutical research	Aims to speed up the contracting process for pharmaceutical industry-funded trials in NHS hospitals
Contract research organisation model clinical trial agreement (CRO mCTA) for pharmaceutical research	Sets out the contracting process when the management of a contract commercial clinical trial is outsourced to a contract research organisation
Primary care model clinical trial agreement (primary care mCTA)	Designed to simplify the process for signing off and initiating pharmaceutical and biopharmaceutical clinical trials involving patients in primary care
Model clinical investigation agreement (mCIA) for medical technology industry	Aims to speed up the contracting process for medical technology industry-funded trials in NHS hospitals
Contract research organisations model clinical investigation agreement (CRO mCIA) for medical technology industry	A tripartite agreement for use when the management of a contract commercial clinical investigation is outsourced to a contract research organisation
Model industry collaborative research agreement (mICRA)	Aims to support clinical research collaborations involving the pharmaceutical and biotechnology industries, academia and NHS organisations across the UK

4.4 Other common types of agreements

In addition to the model agreements described above, there are many others that you may come across:

- Investigator-led clinical trial agreements: for academic clinical trials sponsored by the employer. Where pharma are providing funding or other contributions, such as free drug or placebo or support for packaging and labelling, a further agreement between the host institution and the company is often needed to ensure compliance with laws, regulations and codes of practice.
- Commercially-led clinical trial agreements: for those designed, sponsored and funded by pharmaceutical companies. These can also involve CROs.

- Collaboration agreements: these are required for a project involving at least one other research partner. They set out the roles and responsibilities of collaborating parties working on a specific research project, or set of projects, and how they will be managed. These types of agreement are often drawn up following the awarding of joint research funding, and normally all parties are bound by the same terms and conditions. They can also include sections on avoiding and resolving conflict.
- Material transfer agreement (MTA): this contract governs the transfer of research materials between two organisations and describes the rights relating to the use of the material, confidentiality, publication and ownership of IP. These agreements don't usually contain payment for material other than any associated transport costs.
- Confidentiality or non-disclosure agreement (CDA/NDA): this regulates how confidential information (including IP) may be disclosed. They also set out whether information should be returned to the provider or destroyed after use. Examples include any information, results or know-how owned by someone, which the owner wishes to be kept secret. CDAs must be signed before disclosure of confidential information.
- Drug manufacturing agreement: used when an institution wishes to contract a pharmaceutical company to produce a medicine. These are required for the production of new drugs as well as repurposed medicines. Drug manufacturing agreements should include regulatory compliance (such as good manufacturing practice (GMP) standards, as well as UK, EU and/or FAD regulations) but also warranties and indemnities.
- Investigational medicinal product (IMP) or drug data transfer agreements: used when external parties make in-kind contributions for investigator-led studies, which might include the supply of drugs or medical devices. Transfer agreements are generally held between the host institution and the supplier, outlining each party's role and responsibility as well as regulatory compliance.

4.5 Considerations when developing the agreement

When developing your agreement, it's important to give careful consideration to some of the wider implications and potential challenges you may encounter. Some of these considerations are discussed below.

4.5.1 Intellectual property

Increasingly, researchers are asked to indicate whether their work may result in IP at the funding application stage as this provides an early warning system to the charity. If you are collaborating with industry, it's important to be clear whether you are seeking IP information in the application process and why. Our survey revealed that charities deal with IP in agreements in different ways. Some have a specific clause in their agreement while others use their standard terms and conditions of grant. AMRC has produced guidance on benefiting from innovations and managing IP,¹⁰³ as well as a set of basic clauses that charities may wish to use in their terms and conditions¹⁰⁴ to protect IP.

In some cases, issues can arise relating to IP. For instance, researchers often expect to build on the results of their research in future studies but the industry partner stipulates that they must have full rights to the results in order to license an application. Issues can also arise when a researcher wants to publish findings but the industry partner wishes to postpone publication for a variety of reasons, often to allow for patenting

or to protect the findings for successful future exploitation. To avoid tension, early discussions about ownership of results and publishing rights should be held so that all parties are aware of each other's positions.

Sometimes companies may allow researchers to have access to materials, such as new compounds, drugs, devices or equipment. Such 'gifts' usually have a two-way advantage – the researchers obtain valuable tools for their research at little or no direct cost while the company gains vital feedback on its product. Despite this, companies may impose tight restrictions on any research outcomes arising from use of their products, including restrictions on publishing and/or ownership of IP. Most universities and NHS bodies are aware of this issue and may wish to negotiate such arrangements in an MTA. While the university will seek to secure an MTA that is not overly restrictive, charities should be aware that IP rights quite often remain with the provider in these circumstances.

4.5.2 Issues around conflicts of interest

As well as checking for obvious conflicts of interest, such as partnering with industry that doesn't complement the aims of the charity, there are more subtle conflicts that may arise. For instance, charities are increasingly being asked to be involved in discussions about regulatory approval and health technology assessments. While including these perspectives should be welcomed, this could raise conflicts of interest where the charity has supported early stages of research that is being assessed, especially if it is actively benefiting from the innovation or IP through the work it funded.

To counter this, charities should be fully transparent about collaborations with industry. This includes clearly explaining the role the charity is playing, any funding received or provided, and the possible financial benefits that result from partnerships. Setting out how potential conflicts will be managed is something that is normally clarified in the agreement.

4.5.3 Endorsement of products and acknowledging support

Care should be taken if a charity could be seen to endorse a particular product or treatment from a specific company. Any motivation to do this should be clearly set out in the agreement and discussed early in the partnership. Charities should ensure, in promoting any treatment, that they do so only after full consultation with the scientific and medical expertise available to them, so they can make fully informed decisions. This helps to assure patients that the charity is acting independently and in their best interests.

It's also important to consider how both parties will acknowledge each other's support. This could mean reporting on your collaborations in your annual reviews, scholarly publications, press releases and website. For companies that wish to use a charity's brand or logo, this should be considered carefully, particularly if there is a risk of negative associations with the charity name and reputation. You should discuss under what circumstances a charity's logo would be used and on what materials. We encourage you to check the ABPI code of practice if you have any concerns in this regard.

4.6 Monitoring the collaboration

As with any other activity, it's important that you closely monitor the progress of the collaboration. Many charities do this through an annual reporting cycle, requesting information on what has been achieved, any difficulties or unexpected delays, preliminary findings and some financial accounting. When thinking about the logistics of monitoring progress, you may wish to consider:

- Whether the charity or industry partner will lead on the monitoring of progress of the research project.
- How this will be undertaken – some funders use online tools such as Researchfish¹⁰⁵ to monitor progress.
- How often you will ask for updates and whether this will change as results become available. Your industry partner may have specific interests at certain time points (eg after a proof of concept experiment or after results from first-in-human clinical trial) that don't fit with an annual reporting 'model'.
- The payment schedule and whether this is subject to submission of satisfactory progress. If this is the case, you should discuss what this constitutes, how it will be assessed and the circumstances under which withholding payment may be considered.
- How everyone will be kept informed about the outcomes of monitoring and possible next steps.

You should also think about how progress will be assessed in terms of:

- Whether your scientific advisory committee will review the progress of the collaboration. If this is the case, it should be agreed with the industry partner beforehand and processes to manage conflicts of interest adhered to. Committee members may be asked to sign confidentiality disclosure agreements.
- If the research involves the development of a new drug, industry may be particularly interested in the preliminary findings. You should discuss whether industry will want to access data arising from experiments and the relevant timescales. This could also have significant implications on publishing rights (see 4.5.1).
- If the results of the research collaboration have yielded positive results and will be developed further, it's important that an appropriate communication plan is agreed. Industry may wish to lead on certain aspects, especially if they have greater experience in the regulatory and approval arena, but the charity should be kept informed about progress.

4.7 Additional support throughout your partnership

Many of the issues raised in this chapter are covered by published resources, including:

4.7.1 ABPI Code of Practice for the Pharmaceutical Industry and guidance for collaboration

The ABPI Code of Practice for the Pharmaceutical Industry (second edition: 2012)¹⁰⁶ covers the relationship between pharmaceutical companies and charities. It also provides advice on the promotion of prescription medicines. Although specific to pharmaceuticals, the code applies to other types of industry, offering some general principles that are applicable to a wide range of industry partners.

National Voices and ABPI have also produced a guide for charities and patients groups wishing to collaborate with the pharmaceutical industry.¹⁰⁷ This guide works in harmony with AMRC's *An Essential Partnership*, focusing on the principles of collaboration and the framework that should be in place to do this well.

4.7.2 Guidance from the Charity Commission

Members should be aware of the Charity Commission's guidelines on working with companies and professional fundraisers,¹⁰⁸ which recommend that:

- Charities should recognise that their name is a valuable asset and should take steps to protect it by maintaining independence when entering commercial partnerships.
- Charities should thoroughly research a company before entering a partnership to minimise reputational risk.
- All work is undertaken according to a legally binding written contract.
- Charities and commercial interests should clearly define their aims and expectations before entering a partnership.
- Charities should consider the appropriate use of their name, brand and logo, as well as shared roles and responsibilities and any funding arrangements.

4.7.3 Other organisations

There are a number of other organisations that you might wish to contact to facilitate collaborative working with industry:

MRC Technology

MRC Technology was formed in 2000 to help increase translational research by bridging the gap between basic research and industry collaborations. They can help charities develop research collaborations with industry in a number of ways:

- Reviewing your terms and conditions to make sure they are fit for purpose to support the development of new treatments.
- Identify specific elements in your research strategy likely to be of particular interest to pharmaceutical companies.
- Giving advice on IP to protect research and maximise the chances of it reaching patients.
- Developing promising early-stage research and provide partnering with industry.

During the past 12 years, MRC Technology (MRC-T) has helped launch 12 drugs to market, negotiated around 400 commercial licences and generated over £600m in royalty revenues for academic and charity partners. Below is a case study where MRC-T worked with an AMRC member charity to help them manage IP.

CASE STUDY 35

MRC-T and Worldwide Cancer Research

Worldwide Cancer Research has a close partnership with MRC Technology¹⁰⁹ in managing IP arising from the research it funds. This ensures that the IP is appropriately exploited to maximise patient benefit and, if any financial value is attached to that exploitation, Worldwide Cancer Research receives its share to fund further research. MRC-T helps in this process by:

- Assessing IP potential at the outset of each grant
- Closely monitoring 'high IP' grants
- Facilitating commercial development work if necessary and where possible
- Actively pursuing revenue share agreements when patents are filed.

Charity-led commercialisation services

Some charities also provide their own commercialisation services: an example is Cancer Research Technology (CRT), which develops and commercialises exciting new discoveries for Cancer Research UK and oncology institutes worldwide. CRT often acts as the meeting point between academia and industry and has substantial expertise in translating promising research into commercial propositions for the greatest patient benefit and maximum financial return.

In 2013, CRT leveraged £14m in industry funding to progress promising cancer research. The organisation currently has exclusive rights to £300m of cancer research funding every year. It has three major industry partnerships – with AstraZeneca, Teva and FORMA – and has helped launch three new drugs to market.

4.7.4 Examples of other policies

All charities view partnerships with care and it's important to keep the objectives of the charity and wishes of the trustees and patients at the forefront when partnering with industry. Many charities have developed their own guidelines and policies for such work, including:

- Prostate Cancer UK: Working with pharmaceutical and medical device companies¹¹⁰
- Sarcoma UK: Policy statement on working with pharmaceutical and medical device companies¹¹¹
- Kidney Research UK: Collaborations and partnerships¹¹²
- Parkinson's UK: Policy statement on working with industry¹¹³
- MS Society: Policy statement on working with the medicines and healthcare products industry.¹¹⁴

5. Conclusion and future perspectives

We hope this document is a useful starting point for charities considering developing partnerships with industry. To conclude, we examine some of the future challenges and opportunities for charities and industry working together.

Healthcare has changed significantly over the past 50 years. Only through research can we continue to tackle the most challenging research questions. Working with industry across the drugs and devices development pipeline is crucial to research success and many charities are already thinking about how they can provide more and better treatments for an increasingly informed patient group.

Working collaboratively with multiple cross-disciplinary funders is likely to have a big impact on the way in which new therapies are developed. As long as these partnerships are conducted according to the principles of integrity, independence and transparency, charities should feel confident in pursuing such ventures.

Gene therapies and regenerative medicines

In the future, gene therapies and regenerative medicines may be able to offer a 'cure' for certain diseases, radically changing the way in which we think about treatments. But this will come with new challenges. How will industry be reimbursed for such treatments, considering the high costs of developing these therapies and the fact that patients will no longer need long-term treatments? It's important that everyone works together to create a framework to incentivise these developments, as well as tackling issues around their implementation. Some charities are already beginning to fund gene therapy research with the potential to transform the lives of patients. The next case study shows that research is clearly moving in this direction, so charities and industry need to position themselves carefully in order to undertake such research.

CASE STUDY 36

British Heart Foundation: world first gene therapy trial

The British Heart Foundation has funded a clinical trial¹¹⁵ assessing treatment for patients with left ventricular assist devices (LVADs). In severe cases of heart failure, patients may be fitted with LVADs – battery-operated mechanical pumps to help the failing heart restore normal blood flow around the body. However, this comes with its own risks: implantation of an LVAD requires open chest surgery, which can lead to complications including thrombosis and infection. Furthermore, failure of an LVAD would be catastrophic for the patient and alternatives are therefore sought. BHF and Celladon Corporation are funding a trial to investigate whether gene therapy could help patients with LVADs recover, thereby offering them an alternative form of treatment.

Professor Peter Weissberg, medical director of BHF, says: 'Heart failure devastates the lives of hundreds of thousands of people in the UK. Despite major advances in treating heart attacks, we're still some way off a treatment that restores function in hearts damaged by one. This cutting-edge trial offers genuine hope of an effective treatment in the near future. The trial has been made possible by decades of BHF funding for laboratory research and demonstrates the importance of translating promising laboratory findings into effective treatments for heart patients. Our new strategy puts greater emphasis on providing funding to ensure that even more lab discoveries make it to clinical trials. Improved treatments for heart failure are urgently needed. That's why we launched our Mending Broken Hearts appeal to fund regenerative medicine research which could lead to treatments that replace areas of the heart damaged after a heart attack.'

Personalised medicine

Personalised (sometimes called stratified) medicine is already offering new insights into disease subtypes and allowing drugs to be tailored to suit an individual patient's genetic profile. This will dramatically increase the efficiency of administering treatments and may open up new possibilities to treat orphan (or 'ultra-orphan') and neglected diseases. Cancer Research UK, in partnership with industry and the government, is supporting the stratified medicine programme, as discussed in the next case study.

CASE STUDY 37

Cancer Research UK: stratified medicine programme

Cancer Research UK is working with Astra Zeneca, Pfizer and the UK government's Innovate UK with the aim of supporting the delivery of national, standardised high-quality and cost-effective genetic testing services for patients with cancer. By profiling an individual's tumour, if and when targeted treatments become available, it is hoped that patients will be able to have a genetic test to help clinicians choose the most suitable treatment for them. This involves building a national database of tumour genetic information, treatments and outcomes to help researchers design more effective cancer treatments.

The first phase of the programme¹¹⁶ involved a pilot study in which over 9,000 people with melanoma, breast, bowel, lung, prostate and ovarian cancer had their tumours tested. It also helped demonstrate how the NHS can provide molecular diagnosis for all cancer types routinely. The programme is now moving into the second phase, where it is anticipated that up to 2,000 non-small cell lung cancer patients will be screened annually to identify key genetic faults so that patients can be matched to the best treatments in the National Lung Matrix Trial.¹¹⁷

Tailoring treatments to patient need

In the future, it's likely that there will be further changes in terms of how we think about treatments – both in terms of making treatments kinder to patients (with fewer side effects) and perhaps age-specific drug formulations or therapies (eg babies and children being offered different modes of treatment from older age groups). Charities could play a key role in providing the patient voice for these discussions, acknowledging the need for newer, kinder treatments with differing age-specific requirements of a medicine or technology.

New types of methodology

Changes to how clinical trials are undertaken using adaptive designs is already helping to reduce the time and costs of developing new drugs and therapies. Charities need to be mindful of this and consider such proposals carefully in their peer review processes. This may mean that additional experts need to be invited onto your scientific advisory committee to ensure that new trial designs are appraised rigorously and fairly. In future, charities and industry should be aware of new and innovative types of clinical trial design aimed at speeding up clinical research to bring new treatments to patients sooner.

There are also likely to be changes to the ways research is funded and undertaken. For instance, crowd funding represents a more open approach to carrying out research by engaging the public to find and fund new therapies. This could play an increasingly popular role in research, particularly as patients and supporters become more engaged in raising funds and awareness. However, it does come with its own challenges as the money raised for projects directly by the public bypasses peer review. (If a scientist has an idea that receives enough support from the public, this work can be funded despite not knowing whether the science is of high quality.) Some charities have already recognised the importance of crowd funding in supporting research, as explained in the next case study.

CASE STUDY 38

Cancer Research UK: crowd unding through MyProjects

Cancer Research UK offers various ways in which the public can decide how they want their money to be spent. MyProjects¹¹⁸ is one online example, which allows people to support work that means the most to them. It lets the public choose to donate directly to a particular type of cancer, area of work or even a specific research project that they find relevant or interesting. They are then encouraged to spread the word through social media and fundraising websites, and can visit the project page to see the donation total meet its target and stay abreast of project developments.

New types of medicine

The future may also see the emergence of different types of medicine such as nanotherapies to specifically deliver drugs at the right dosage at highly specific sites and at the optimum time in treatment. This could open up the possibility of a single agent being prescribed to deliver a multitude of site-specific drugs in sequence – something that would be of particular importance for long-term complex diseases or those with significant comorbidities.

These and other innovations have the potential to create new opportunities for treatments and open up new ethical and practical challenges. It is only by working together that charities, government and industry can ensure they meet common goals.

Appendix 1: AMRC survey of charities

In 2014 we surveyed 120 AMRC member charities to find out how charities work with industry. In total, 41 charities representing various sizes completed the survey. Below is a summary of the main findings.

Type of collaborations

Charities collaborated with industry in a number of ways. Those with research collaborations with pharmaceutical companies did not necessarily collaborate with biotechnology, medical technology or others such as umbrella groups, consortia or trade groups.

Industry organisation type	Research collaboration (co-funding for a research project, in-kind support)	Advocacy and campaigning collaboration	Other (educational material, patient info, advertisement, events sponsorship, fundraising)	We don't currently have any collaborations
Pharmaceutical companies	9	5	11	17
Biotechnology	9	1	6	17
Other: umbrella groups, consortia, trade groups and med technology	4	3	4	21

When asked to describe the benefits of working with industry, the collaborating charities said that the collaborations were crucial in helping them meet their charitable objects. Common themes included:

- Extra funding allowed the charity to undertake more research
- Collaborations helped develop, manufacture and supply vaccines - something that couldn't have been possible working alone
- Partnering with industry helped the charity to develop new therapies by supporting translational research
- Collaboration provided access to expertise not represented in the charity
- The partnership helped charities organise and fund (through sponsorship) conferences and symposium and provide educational material
- Partnerships helped support PhD studentships and fellowships.

Research collaborations and agreements

In total, 11 charities had a research collaboration with industry (predominantly pharmaceutical and biotech companies). In most cases, the charity made the initial approach in developing the collaboration (n=6), although researchers also played a part in instigating partnerships (n=3). Two charities reported that there was mutual interest.

Nine out of the 11 research collaborations had some kind of formal agreement in place. One charity had used Lambert agreements. Primarily, it was the charity that led the development of these agreements (n=6), although two were developed mutually by the charity and its industry partner. Technology transfer offices also led two collaborations and one charity was led by MRC-T.

Four charities reported that they used the ABPI code of practice when developing their agreement and four didn't (although one reported that this was because the collaboration was with a medical technology rather than a pharmaceutical company). Three charities were not aware of the code.

Intellectual property

The survey revealed that charities dealt with IP in different ways. One had a specific clause in its agreement but did not specify how this was developed. Two charities reported that IP was dealt with in accordance with their standard grant terms and conditions and three said that IP was owned by the university or the industry partner. One agreement included a clause on IP developed by a TTO, while another was developed by MRC-T.

Most respondents said that IP clauses varied on a case-by-case basis, but the filing, maintenance and enforcement of IP was not the charity's responsibility. One charity mentioned that they had the option of exploiting IP if the company chose not to pursue it. In such cases, they agreed with the company to transfer licences and gain access to regulatory data and filings. They also had a steering group responsible for vetting publications and presentations to ensure that IP filing strategy was not adversely affected.

Conflicts of interest

Charities dealt with conflicts of interest differently. The majority said that they assessed conflicts on a case-by-case collaboration, although two said that they used their standard conflicts of interest policy throughout. One charity said that the TTO dealt with conflicts of interest whereas another reported that the type of collaboration (a consortium with a minimum of two industry partners coordinated by MRC-T) minimised the potential risk for conflicts.

One charity reported that its main concern in working with industry was to ensure that a programme was not halted for strategic rather than scientific reasons. This charity managed this potential conflict with step-in rights and diligence provisions. They talked through this aspect before embarking on a collaborative project with industry. One charity reported that they took careful steps not to endorse particular products.

Type of support

Six charities reported on their research collaborations with industry in 2013, accounting for 61 collaborations between them.

How many research awards in 2013 involved collaboration with industry?				
Project	Programme	Clinical trial	Studentships/ fellowships	Other
3	1	31	2	24

These collaborations mainly provided financial support; however, in around half of cases, they also involved access to novel technologies, methodologies and equipment or other expert advice or specific technology.

What form of support did these research collaborations with industry take?

Financial support towards research costs	Educational grants	Free drug, placebo or other agent	Support for (drug) packaging, shipping, labelling, etc	Access to novel technologies, methodologies and equipment	Other
6 / 6	1 / 6	2 / 6	1 / 6	3 / 6	3 / 6: expert advice, access to other technology

One charity reported on the direct funding it provided to industry. It explained that it operated a revenue share structure on all products receiving charitable funding and that this was governed by a predetermined proportion based on the contribution given.

Transparency

Twenty charities said that they reported industry links in their annual report or review. However, the details published varied, with some reporting the global sum received, whereas others gave a breakdown on a company-by-company basis. One charity gave details of the companies involved, the value and what the money had been put towards. Some funders preferred to highlight a subset of industry collaborations likely to be of interest to their supporters.

Do you employ any other mechanism of publicly demonstrating your relationship with industry?

Statement on website	Press release	Scholarly publication	Other
17	14	6	11: newsletters, patient information, marketing material, blogs, consortiums, social media

Perspectives

The majority of respondents said that they anticipated an increase in partnerships with industry in the future, although around a quarter did not. Most of those who anticipated an increase viewed this as being very important, particularly for clinical trials and translational research, but finding the right industry partner was the biggest challenge.

What do you think are the most important issues and challenges for charities forming research collaborations with industry? Please score each issue 1-5, with 1 being unimportant, and 5 being very important.

Finding the right partner	Maintaining independence in partnership	Balancing risk and reward	Intellectual property ownership and protection	Negative public perception of working with industry	Other
4.8 / 5	4.2 / 5	4.0 / 5	3.6 / 5	3.1 / 5	Maintaining independence, integrity, changing attitudes, establishing clear roles and responsibilities

The greatest policy concerns that collaborating with industry could tackle were the funding of translational research, innovation in the NHS and access and uptake of new treatments. Public trust in the regulation of medical research, medicines and medical devices also scored relatively highly.

What do you believe are the most important policy concerns that collaborations with industry can tackle together? Please score each issue 1-5, with 1 being unimportant, and 5 being very important.

Funding translational research	Innovation in the NHS	Access to new treatments and uptake	Public trust in the regulation of medical research, medicines and medical devices	Appropriate use of patient data	Lack of flexibility in the regulation of medicines	Issues with repurposing drugs	Value of patient voice to regulators	Other
4.0 / 5	3.7 / 5	3.7 / 5	3.5 / 5	3.5 / 5	3.4 / 5	3.4 / 5	3.4 / 5	Public trust in industry

Appendix 2: AMRC survey of industry

In addition to the survey of AMRC members, we also conducted a similar survey of industry. There were 13 respondents representing companies of a variety of sizes. The majority were pharmaceutical companies but biotechnology and medical technology firms and one contract research organisation also completed survey.

Type of collaborations

The majority of respondents were aware of the different types of support that medical research charities could offer (n=8), and many companies reported that they had worked with UK medical research charities in the past. Several companies reported more than one collaboration and just two said that they hadn't worked with charities before.

Please indicate whether your company has worked or is working with medical research charities in the UK on any of the following activities				
Research collaboration (co-funding for a research project, in-kind support)	Advocacy and campaigning collaboration	Supporting patient groups	We haven't worked with medical research charities before	Other (eg production of educational material, information for patients, advertising, events sponsorship, fundraising)
6	2	3	2	2

When asked what influenced the company's decision to work with charities, the majority said that this was down to opportunities to co-fund research (n=7), having access to patients to take part in research (n=5), access and the desire to understand the patient voice/need (n=5). Other reasons included the opportunity to increase the company's visibility and brand in the patient population (n=4), access to scientists and expertise in academia and the NHS (n=3), access to research infrastructure (eg tissue banks) (n=2) and for charities to support navigation of the regulatory pathway (n=2). One company also reported that it worked with charities to access high-quality peer review funding processes.

Research collaborations

On research collaborations, six companies reported such collaborations, although it was not possible to accurately determine how many collaborations with charities had taken place over the past five years. The support that companies provided to research collaborations varied widely. These included access to novel technologies/methodologies, access to research infrastructure, support for packaging/labelling/shipping, access to analytical/computational tools, access to scientific expertise and other forms of financial support (such as lab equipment, research staff costs and running expenses). Companies also provided free drug/placebo, access to medical devices, as well as knowledge and help navigating the regulatory pathway.

Intellectual property

When asked about the handling of IP, responses were somewhat limited (n=3). Those that did respond said that this varied on a case-by-case basis but predominantly the IP was negotiated between the company and the academic partner.

Transparency

In terms of transparency, most of the companies that responded said that they reported their links with charities, although two did not. The most popular way was through a statement on the company's website but some also did this through press releases, their annual review and scholarly publications. The information published varied but usually included either just the charity name or the charity name and the contributions from the company. To some extent, this depended on the nature of the collaboration and the arrangement.

How do you report your links to medical research charities?					
Through annual report or review	Statement on website	Press release	Scholarly publication	We don't report such links	Other communications
3	5	4	3	2	0

What information do you provide on reporting your links with charities?			
Charity name only	Charity name and its contributions to the collaboration	Charity name and contributions from both the charity and industry to the collaboration	Other: please specify, if possible provide weblinks to information you publish on collaborations with charities
4	4	1	None entered

Perspectives

When asked whether they thought that partnerships with medical research charities would increase in the future, all respondents to this question agreed they would. Some said that this was because charities were beginning to focus on commercial research, filling the void by supporting more early-stage, high-risk projects. Further to this, one respondent reported that pharma was focusing resources on disease areas that had the highest probability of scientific, medical and commercial success, and that all drug discovery and clinical development programmes were designed to offer a distinct medical advantage to patients. In order for them to do this, they had to work more collaboratively across academia and medical research charities.

Most respondents also reported that charities played the most significant role in providing them with access to patients, but they also had major input into encouraging more clinical research.

How do you think partnerships with medical research charities could help to deliver your goals?					
Encouraging more discovery research	Encouraging more pre-clinical research	Encouraging more clinical research	Providing access to patients	These types of partnerships cannot really help us	Other
3	2	6	8	0	1

Finally, when asked about what major barriers industry faced in collaborating with medical research charities, respondents gave a variety of responses including: lack of awareness of the charitable sector, difficulty in finding the right charity, a lack of opportunities for research collaborations and that fact that some research projects represented too high risk for industry collaboration. All these factors were scored the most important in equal measure. Another particularly important barrier reported by several respondents was the lack of understanding of the drivers for commercial research and the importance of working with industry to support research that brings benefit directly to patients.

What are the main barriers that industry faces in collaborating with medical research charities in the UK? Please score each issue 1-5, with 1 being unimportant, and 5 being very important.

Lack of awareness of the charitable sector	Finding the right charity	IP ownership and protection	Lack of opportunities for research collaborations	Lack of research infrastructure	Research projects that represent too high risk for industry collaboration	Adhering to ABPI code of practice	Other (please specify)
2.8 / 5	2.7 / 5	2.4 / 5	2.7 / 5	2.6 / 5	2.7 / 5	1.9 / 5	Lack of commercial awareness

The most important policy areas identified included access to new treatments and their uptake in the NHS, the value of the patient voice to regulators, innovation in the NHS and the pricing of new drugs.

What are the most important policy concerns that collaborations with UK charities can tackle together? Please score each issue 1-5, with 1 being unimportant, and 5 being very important.

Lack of flexibility in regulation of medicines	Pricing of new drugs	Issues with repurposing drugs	Access to new treatments and their uptake	Innovation in the NHS	Funding for translational research	Appropriate use of patient data	Value of patient voice to regulators	Public trust regulating medical research, medicines and devices	Other (please specify)
2.8 / 5	3.4 / 5	2.3 / 5	4.2 / 5	3.7 / 5	2.9 / 5	2.9 / 5	3.9 / 5	3.1 / 5	None entered

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