Pharmaceutical Medicine (2022) 36:279–286
https://doi.org/10.1007/s40290-022-00442-y

CURRENT OPINION

Medical Research Charities and Biopharmaceutical Companies as Partners in Patient-Centred R&D

Tina Flatau[1] · Julie Greenfield[2] · Brian Dickie[3] · Oli Rayner[4] · Helen Matthews[5] · John Wise[6]

Accepted: 7 July 2022 / Published online: 12 August 2022

Abstract

Life science research and development (R&D) companies are all too aware of the importance of patient perspectives but also of the barriers to engaging directly with patients, not least compliance, complex technical and regulatory issues, and the need to meet multifaceted expectations. Medical research charities (MRCs), highly technical and professional organisations, work directly with patients; they represent an expert resource for the science of their field, for disease-related patient advocacy issues and to advise and assist R&D companies in devising meaningful trials. The Pistoia Alliance, a non-profit organisation facilitating life sciences R&D, gathered a number of UK MRCs focused on complex lifelong conditions. The group used workshops and an opinion questionnaire for a snapshot of how the charities believe their knowledge and patient experiences could contribute insights and efficiencies to commercial R&D. MRCs argued that for chronic conditions, the patient perspective is vital in facilitating and de-risking trials, promoting patient motivation, compliance and study viability. MRCs and the patients they represent want to see successful trials, and it is in everyone's interest that well considered studies can proceed. Today, with remote assessments, consumer wearables and digital health technologies, MRCs and patients are already collating substantial data sets that are relevant to quality-of-life benefits, regulatory and value assessments, all of great interest to biopharmaceutical companies. In turn, MRCs would benefit from the experience of biopharma in generating clinical data and implementing novel technologies.

1 Introduction

Medical research charities (MRCs) invest significantly in research, in 2020 contributing £1.7Bn which paid for nearly half of all publicly funded medical research in the UK [1].

Many MRCs are mature research and development (R&D) organizations, working independently, educating, funding and collaborating in scientific studies, and representing patients in healthcare and payer/regulator interactions.

Key Points

The Pistoia Alliance surveyed the opinions of medical research charities (MRCs) focused on lifelong conditions about opportunities to improve biopharmaceutical research and development (R&D).

MRCs see huge value in embedding the patient voice and lived experience into the clinical trials process, right from the early stages of programme design. They agreed that patient-centric approaches offer de-risking and efficiencies for clinical development but are not a daily reality for biopharma companies.

The COVID-19 pandemic and data technologies have shown how patients can join in research and contribute to clinical data sets.

MRCs see clear opportunities for patients to positively input to biopharma R&D throughout, from defining requirements to informing cost–benefit and helping optimize use of new therapeutics.

✉ John Wise
john.wise@pistoiaalliance.org

1 Action Duchenne, London, UK

2 Ataxia UK, London, UK

3 Motor Neurone Disease Association, Northampton, UK

4 Rare disease patient advocate with Cystic Fibrosis, Plymouth, UK

5 Cure Parkinson's, London, UK

6 The Pistoia Alliance, 401 Edgewater Pl, Wakefield, Massachusetts 01880, USA

Early patient input is essential, directly through MRC members and collaboratively in the UK (e.g. the Patient-Led Research Hub [plrh.org]), helping develop new research ideas with patient input. In the same user-centric way as the development of consumer technologies, patient input helps in understanding needs, designing creative solutions and validating with real-world experience.

Patient-centred healthcare informs and engages patients and their representatives in care and treatment decisions that respect their needs, and patient-centricity takes this further, with "integrated measures for listening to and partnering with patients, and placing patient well-being at the core of all initiatives" [2]. Patient-centricity is not straightforward for biopharma, cautious about the patient interface, managing expectations and regulatory compliance. MRCs are ideally placed to help biopharma improve patient-centricity with excellent patient communication to understand needs, motivate patients about research and importantly to embed effective communication strategies into all clinical programmes.

The Pistoia Alliance is a global non-profit organization with the mission to lower barriers to innovation in life science R&D, including bringing together stakeholders to promote patient-centric practices in clinical development.

Since 2019, the Pistoia Alliance has been working with leading MRCs on engagement with biopharma to encourage R&D for chronic and lifelong conditions. Participating charities to date have been Action Duchenne, Alzheimer's Research UK, Ataxia UK, Autistica, Cure Parkinson's, Cystic Fibrosis Trust, Findacure, Institute of Cancer Research, LAM Action, Motor Neurone Disease Association and Muscular Dystrophy UK. The group created an opinion survey, based upon jointly agreed key themes, to explore MRC capabilities to inform research programmes, contribute insights and highlight opportunities to de-risk and lower barriers to R&D. Furthermore, the survey covered aspects of patient-centric R&D programme design, such as relevant outcomes measures and the utility of patient-contributed data. The charities' views and experiences were also surveyed relating to MRC-curated registries, as well as opportunities and examples of patients improving R&D design and the future of real-world data to reflect patients' lived experiences. Survey responses have been used to inform the commentary below, select comments are reproduced in italics.

2 Putting Patient-Centricity at the Heart of Biopharmaceutical Research and Development

The biopharmaceutical industry must balance costly and complex science with a timely return on investment. The priority of early R&D is to optimize the technology opportunity, rather than focus on user requirements. Patient-centred therapy design can de-risk R&D, but most patients become involved only later in R&D to participate in large clinical trials. Many patients volunteer altruistically, to help others and advance disease understanding. Good practices, such as simple information on trial design and governance, are not always present. Patients are indispensable as participants for clinical studies, but as individual end-users they rarely define key user requirements or contribute to product design.

> *"Discovering and defining the problem to be solved, by directly asking patients and designing a solution with patients as you go, is what I would call patient-centred innovation".* (Cystic fibrosis patient advocate)

2.1 The Role of Patients in Defining User Requirements and Relevant Measures

Patients and their representatives are proactive and knowledgeable; through social media and patient organizations they have grouped together to form eloquent and influential lobbies. Biopharma companies dedicate significant effort to patient recruitment and retention in clinical trials. They use marketing companies for patient input to Health Technology Assessment (HTA) agencies such as the National Institute for Health and Care Excellence (NICE). Regulators and payers, the key decision makers for product commercialization, have long engaged with patients to testify about the safety, effectiveness, and value of products. Patient engagement initiatives span national boundaries, such as the US Food and Drug Administration (FDA) and European Medicines Agency (EMA) Patient Engagement Cluster [3], and are a core feature of Medicines and Healthcare products Regulatory Agency (MHRA) regulatory strategic planning [4]. In their survey response, Alzheimer's Research UK commented that biopharmaceutical companies "*still use patient engagement mostly to get patients to enrol in the study"* and *"there are opportunities right from study design to engage better with patients and patient organisations".*

For true patient-centricity, MRCs seek early involvement in identifying patient requirements for new products and meaningful clinical trial outcomes, arguing this would improve patient interest in joining studies and avoid unnecessary difficulties leading to dropouts and non-compliance.

> *"Monthly visits to a trial site mean a child misses a lot of school. For some families that prevents them participating in trials".* (Action Duchenne)
> *"Patients distracted after contact gel used in EEG measurements leaves them feeling 'dishevelled' afterwards. If they felt uncomfortable they couldn't*

engage properly in cognitive tests". (Muscular Dystrophy UK)

The COVID-19 pandemic has introduced remote monitoring and telemedicine into trials, with study and clinic visits combined. Home monitoring means fewer invasive and complex tests, more patient-reported outcomes measures (PROMs) and offers more user-friendly assessments.

With more decentralized trials, good patient communication at all stages is even more critical. For e-Consents (electronic informed consents), providing information and opportunity for discussion is an integral part of good consenting practice. MRCs stress the importance of ongoing trial information, feedback and updates to maintain motivation, and that the rollout of telemedicine requires equal access to technology for special groups and older patients [5].

2.2 The Increasing Importance of MRC Registries and Natural History Data to Understand Patient Experience

To understand patient experience, MRC natural history studies recruit as a formal trial, collecting clinical measures in the normal course of a progressive condition. In their registries, MRCs broadly set out standard measures and observations, including natural history data, contributed by clinicians and patients to an ongoing, longitudinal database. This is useful in feasibility assessment, design, identifying patient sub-populations for trials and pharmacovigilance. Registries have a wide scope, but therapy trials often target a narrow population to show a measurable difference over the defined course of the study.

"Duchenne trials typically take place in children aged 4–7 but that's a fraction of the populations and restricting trials [to this age] has led to drugs being approved for very limited age groups". (Action Duchenne)

"Dialogue between pharma companies, registries and patients to better understand the art of the possible and the kinds of data that are needed and why, might be a good starting point". (Muscular Dystrophy UK)

Future registries will use informatics best practices to collate patient-reported and clinician-reported data on compatible platforms, delineate disease subpopulations, maintain data security, and allow data sharing for AI analytics.

"Ataxia UK and the US charity Friedreich's Ataxia Research Alliance have a global patient registry using a platform designed for rare diseases and cancers, curated by medical informatics provider Pulse Infoframe". (Ataxia UK)

Validated natural history data, whether untreated or with existing standard of care, are already being accepted for enhancing or replacing a placebo arm in trials where ethically justified. With the FDA issuing guidance [6], biopharma R&D and MRCs could collaborate on registry and natural history standards across diseases, future proofed by FAIR [7, 8] data standards.

2.3 Making Trial Data Meaningful

According to MRCs, patient-relevant measures for chronic diseases are of a *real-world* nature, very different from the tests and biochemical markers typical as surrogate indicators.

"Outcome measures of more direct relevance to patients carry no economic import and they tend to influence QALY[9] *models in extremely indirect and inefficient ways.' …. 'For us the most health-economically impactful outcome measure is pulmonary exacerbation rate but, unfortunately, these require larger/longer studies to capture enough events"*. (Cystic fibrosis patient advocate)

Everyday movement/activity, sleep/fatigue, cognitive ability/decline have been expensive and risky measures in clinical trials, difficult to prioritise in an HTA context. Early disease factors may be physical, patients with advanced disease may focus on mental well-being. Real-time measuring tools provide qualitative insights, and the MRCs surveyed all promote the collection of longitudinal on-treatment and off-treatment data for patient-relevant outcomes, such as

- Self-reported or proxy-reported quality of life
- Mental health
- Exacerbations leading to hospitalisation
- Sleep quality/pattern
- Everyday activities/fatigue/fitness
- Measures showing treatment advantages

MRCs promote work to validate meaningful measures that are relevant for patients. Examples include the handheld dynamometer for grip strength or correlating MRI for muscle deterioration and fibrosis with deteriorating health.

Importantly, patients want to see quality of life (QoL) factors reflected strongly in trial outcomes. One issue here is that PROMs, where the patient records their experiences such as pain intensity, can be difficult to translate into regulatory and labelling claims. As a result, 'old' primary end points persist, requiring many patients for statistical significance, and limiting the contribution of relevant subpopulations.

2.4 Many Sources of Data—Joining the Dots

Modern data standards and modelling could bring everyday patient experiences into data sets. In the meantime, patients are realistic that meaningful data for a clinical trial, regulatory approval and HTA assessment do not necessarily reflect desired improvements in their own life experience.

> *"We desperately need better measures of Quality of Life. The current instruments do not map onto patients' quality of life factors. There is also potential to decompose QoL into more specific domains and convert them from qualitative to quantitative using wearables and digital technologies".* (Cystic fibrosis patient advocate)

This highlights the importance of daily patient experience in registry and natural history data, where novel technologies could support outcomes with direct and impactful relevance to QALY models.

> *"One young adult told us that now he is no longer able to walk, what matters most is the ability to meet his friends and drink a pint with them."* (Action Duchenne)
> *"PROMs are valuable but it is the clinical measures that need to change to reflect patient experience and priorities."* (Cure Parkinson's)

Data that have been generated in any biopharma trial could more widely inform disease aetiology, time course, progression and management. Genetic characterisations, comorbidities and various standards of care, all recorded and followed in biopharma trials, are siloed within the study. Patient consents are not yet routinely considering mechanisms for contributing individual trial data to future searches with the facility for retracing/recontacting patients for their benefit and for the potential benefit of all patients.

3 Medical Research Charities in Chronic Disease R&D

In the UK, 15 million people, some 22% of the population, live with an illness requiring long-term care [10] and approximately 3.5 million people live with a rare genetic disease [11].

MRCs oversee significant medical R&D and are trusted to hold large sets of directly contributed patient data that can complement and contextualize biopharma libraries. MRCs address important unanswered questions where commercial R&D may be uneconomic. Lifelong conditions may be syndromic and poorly understood, so MRCs compile a wide range of relevant information to enable clinical studies and illustrate the natural course of the disease, measures and outcomes. MRCs including Alzheimer's Research UK, Cure Parkinson's and Motor Neurone Disease Association run extensive programmes. Smaller rare disease charities, such as Action Duchenne and Ataxia UK collect data and promote disease understanding and trial opportunities.

Since MRCs are not commercial institutions, they fund open-access technologies, use off-patent drugs, repurposed medicines and treatment combinations that innovating biopharmaceutical companies are unlikely to pursue. MRC Scientific Advisory Boards, which include patients as well as experts, have strategic priorities for purely exploratory research, biomarkers, outcomes and therapy clinical trials, all with a patient-centred philosophy.

> *"I think of the 'patient-centric' in innovation as referring to certain qualities of the process of innovation and the role of patients in that process."* (Cystic fibrosis patient advocate)

Biopharma companies are bound by international guidelines such as the International Federation of Pharmaceutical Manufacturers & Associations (IFPMA) Code of Practice [12] on communications with the public and patients. As communication intermediaries, MRCs could be central in helping patients contribute to the design of biopharma treatments and tools.

3.1 Disease Modification is a Difficult Goal

For chronic diseases, to have any hope of success towards disease modification requires expertise, deep pockets and unshakeable tenacity. Research charities working in Parkinson's are dealing with a chronic, debilitating, and progressive disease, costed in the USA at US$52 billion per year [13]. Trials are long and onerous, slow to recruit and often fail to accommodate the needs of participants. No disease-modifying treatments exist, and this is a profound unmet need [14].

The UK charity Cure Parkinson's is dedicated to developing disease-modifying treatments for Parkinson's disease, accomplishing these aims through international research programmes [15] looking at both novel therapeutics and repurposed treatments, running pre-clinical studies and its own clinical trials. The international Linked Clinical Trials initiative (cureparkinsons.org.uk), established in 2012, aims to prioritise repurposed drugs with potential as disease-modifying therapies for Parkinson's. Cure Parkinson's progresses these repurposed candidates into clinical trials for Parkinson's disease, with over 50 candidates identified

to date. More than a third of all current Parkinson's disease-modifying clinical trials globally, including two extensive phase III programmes, are a direct result of this Cure Parkinson's initiative.

3.2 Venture Philanthropy and Capital Reinvestment

Cystic fibrosis (CF) is often the exemplar of how a life-limiting, incurable and devastating disease can be reframed by determined, focused non-commercial R&D efforts with patients at the core. Basic science funded by the Cystic Fibrosis Foundation led to the cloning of the CFTR gene in 1989. Until the Foundation laid out a vision with venture philanthropy to go from gene to therapy, CF had too many risks and unknowns for biopharma drug developers.

Today many of the therapies approved for CF were directly supported by the Cystic Fibrosis Foundation. In addition to providing funding for basic science and clinical research, the Foundation supports therapeutic development through a unified patient data registry, research tools, scientific advice, patient perspectives, and an integrated international clinical trials network dedicated to CF. By de-risking early studies and enabling promising science to move forward, the Foundation has effectively kick-started and directed a market for CF therapies.

Over 30 years, advances in disease understanding, standards of care and outcomes in CF have been profound. Life expectancy has increased from mid-teens to 47 years, although median age at death is still around 28 years. The promise of new treatments such as CFTR modulators is beginning to change what it means to have cystic fibrosis, despite an uphill struggle with reimbursement. However, not everyone with CF has a genotype that responds or fully responds to the new drugs so far. The CFTR modulators do not cure, people still have CF, experience pulmonary exacerbations, inflammation, and gradual respiratory decline. Continuing serious symptoms and complications of CF all still require additional treatments, albeit slowed, and treatments come at a high cost. The challenge remains to deliver affordable therapies for patients with remaining unmet needs, treat the underlying cause of disease in 100% of people with CF and, ultimately, deliver a cure.

Such goals are not unrealistic; successful venture philanthropy brings the Cystic Fibrosis Foundation significant royalties from approved therapies. These funds are being recycled to support and empower competition in the CFTR modulator space, encourage treatments for unmet needs and make therapies more curative and accessible for all. This shows what is achievable when patient organizations unite around a bold vision, leverage capitalism, invest in infrastructure and embrace a portfolio approach to risk.

3.3 Lowering Barriers to Entry for Biopharma

Motor neurone disease (MND) can be viewed as a model for age-related neurodegeneration, often rapidly progressing within 2 years of diagnosis. With quantifiable measures of disease trajectory, such as motor function, muscle strength and survival, and emerging biomarker candidates for early diagnosis, prognosis, stratification of disease and assessment of treatments, research charities want to see more biopharma interest.

MND charities have increased collaborative basic science activity over the past decade to lower barriers to entry for biopharma. Over 10% of MND cases have a strong genetic basis. The identification of numerous causative and disease-modifying genes over the past decade, through ambitious collaborative initiatives such as Project MinE (projectmine.com), has catalysed development of more relevant laboratory disease models, often funded by the MND research charities themselves. This has accelerated the discovery of multiple therapeutic targets. While small molecule-based approaches dominate the therapy development pipeline, gene therapy and antisense strategies offer hope for those families with inherited forms of the disease. Targeting disease-related genes not only offers the opportunity to treat those diagnosed with familial MND, but also enables the study of preventative strategies for younger 'at risk' family members (ClinicalTrials.gov identifier: NCT04856982).

MND charities and academic clinical researchers study repurposed drugs using novel multi-arm trial design, exploratory biomarkers and PROMs, all important aspects permitting more comprehensive stratification of trial cohorts. The Motor Neurone Disease Association is working with the Medicines Discovery Catapult and research funders to develop a 'roadmap' that aims to accelerate upstream drug discovery and development through precompetitive academic–industry partnerships.

Focusing on quality of life, the TONiC initiative (tonic.thewaltoncentre.nhs.uk) is the world's largest study to understand the physical and psychosocial aspects of MND. The hope is that the findings of TONiC can drive the development of new, validated PROMs that can be widely adopted in clinical trials to assess patients' lived experiences.

3.4 De-Risking Clinical Trials at the Design Stage

Chronic degenerative diseases tend to be multifactorial, and Cure Parkinson's is committed to developing novel trial designs such as multi-arm, multi-stage platform trials to accelerate clinical testing and generate efficiencies and cost savings for Parkinson's disease projects. Platform trials necessitate deep research initiatives such as whole genome

sequencing, biomarker programmes and the algorithms for subtle changes from wearables and imaging. The aim is to accurately measure aspects of Parkinson's disease rather than simply measuring movement. A holistic approach involves people living with Parkinson's in the trial process from the earliest stage of establishing understanding, baseline disease and meaningful measures. This co-creation, collaborative approach ensures trials are practical and feasible with a clear definition of the potential burden, understood and supported by potential study participants. With a philanthropic funding basis, non-commercial status and deep expertise in Parkinson's R&D, Cure Parkinson's can support industry in their plans and help de-risk drug development in Parkinson's disease.

Not all MRCs can run extensive trials, but they can help de-risk studies using their networks, registries, disease knowledge and proximity to patients. In the rare disease space, Action Duchenne works with biopharma companies to encourage patient-centred research in Duchenne Muscular Dystrophy (DMD), improve study designs and widen trial opportunities for patients. Rare genetic disease charities represent patients of all ages from children to adults and expanding trials across age groups and abilities is vital for all to access licensed treatments. Action Duchenne encourages biopharma to involve patients and their families early in planning clinical programmes, to engage patients and reflect DMD as experienced, ranging from tests of motor skills in physically active children, to new measures for older, wheelchair-bound patients and eventually ventilator users. Measures of retained abilities are particularly important to older, non-ambulant patients who wish to protect their remaining muscle function, such as being able to drink without assistance or use a keyboard. Action Duchenne has supported the development of useful outcome measures applicable to all patients, such as the ActiMyo® device accepted in 2019 by the European Medicines Agency as a secondary outcome measure. Wider engagement facilitated by MRCs could increase enrolment, compliance and retention, all vital to de-risk rare disease research trials.

3.5 Deliver Continuous R&D Improvement Through Patient-Centric R&D

Like the DMD charities, Ataxia UK works to provide enabling data for biopharma, collaborating with other MRCs to make patient registry data available and promoting patient-relevant measures for important everyday activities such as speech intelligibility and feeding. Ataxia UK supports families, engages them with research advisory boards, and is involved in patient-centric research consortia such as the Ataxia Global Initiative (ataxia-global-initiative.net) and the Critical Path to Therapeutics for the Ataxias (c-path.org/programs/cpta/) that are striving to achieve trial-readiness and regulatory approval of outcome measures. The charity gathers information from carers and people with ataxia to help researchers understand the condition, pathways in diagnosis and care, burden of illness, experience of trials and barriers to trial participation [16]. Working with other European ataxia organizations (Euro-ataxia), a Patient Charter describes patient engagement, why it matters and how it should be implemented (euroataxia.org).

In cystic fibrosis, clinical trial networks directly involve patients and their representatives in early development, contributing basic science funding and country-specific registries. As much as this enabling approach helps biopharma companies to work in CF, this alone does not guarantee patient-centricity in subsequent drug development. When projects are de-risked in this way, biopharma is much less reliant on patient organizations, so CF patients have less input to the design of therapies or what it means for them to say a therapy 'works'. This is changing, as a greater proportion of people with CF survive into adulthood with independent lives, they use technology to come together to build relationships, lead, support and contribute. Where parents once were the entire CF community, now well-informed adults with CF are active online in patient forums and organizations.

Rather than the voice of one 'expert patient', biopharma companies and other stakeholders can have ongoing consultation with larger more representative groups of real patients, and MRCs can help to broker this input in flexible and user-friendly ways. MRCs such as Ataxia UK also engage with regulators directly and through the UK MHRA patient group consultative forum. Initiatives such as the FDA's 'Patient-Focused Drug Development' in the US [17], offer ideal opportunities for regulators, pharma and stakeholders to hear the patient perspective. Similar initiatives in the UK would be welcomed and UK-based MRCs would be well placed to lead them.

4 Data Technologies as Enablers of Patient-Centricity

Digital health data libraries are growing exponentially from many sources. MRCs already use consumer technologies (smart phones, activity trackers) to collate user-contributed real-world data [18] for research purposes. In public health, the DETECT trial (NCT04336020) used various wearable trackers for early indication of influenza and other viral infections. By 2021 telemedicine was routine, with UK-based HealthHero the largest telemedicine provider in Europe, covering some 22 million people [19]. The chronic disease crowdsource platform StuffThatWorks (stuffthatworks.health) uses AI to collate and learn from people who

contribute their own disease, treatment and comorbidity information to inform management choices for all. The vast amounts of longitudinal data from consumer devices, the growth of telehealth and the new willingness of the public to contribute health information, expands opportunities for modelling to identify meaningful therapeutic benefits. The digital health sector is growing fast and attracted over US$50Bn in global funding in 2021 [20].

The big technology companies including Meta, Google and Amazon all have ambitions in healthcare and see the opportunities for improved efficiency and patient experience. Google Cloud has already partnered with the National Institutes of Health (NIH) in the US to enable sharing and analytics under the STRIDES initiative (datascience.nih.gov/strides). A significant challenge is managing the ownership of health data when captured and processed by unregulated and competing commercial data companies. Under International Conference on Harmonisation (ICH) guidelines, biopharma companies, and the regulatory agencies that oversee them, have long experience and well-established control systems for managing patient data, and ensuring scientific data inputs are of high quality, ethically sourced, validated, secure and, where informed patient consent allows, reusable. The FAIR data principles are being actively deployed. As such, biopharma is well placed to manage digital health data responsibly.

For people with long-term conditions, the Covid-19 pandemic accelerated the adoption of 'smart' technologies for remote patient monitoring. In time, data standards, validated algorithms and larger data sets will overcome the variability of real-world data in adaptive study designs and subgroup metanalyses.

Biopharma companies have established collaborations such as the Digital Medicine Society (dimesociety.org) for good digital data practices in health and medical applications, specifically including real-world data. In this new world of enhanced collaboration in health science, with large-scale health data and high-performance computing, patients with hard-to-treat conditions might anticipate an acceleration in progress towards meaningful therapies for them.

In a wearable, technology-enabled future, all patients will be able to monitor and submit health information reflecting how effective their treatments are.

> *"… so while a trial may tell us that a medicine is safe and effective in principle, a real-world system would tell us whether that medicine actually works for a given individual in as scientific a way as possible. Machine Learning and wearables could help. Before starting a new treatment, patients should ask what job is this medicine going to do for me and how will we know if it is working*?" (Cystic fibrosis patient advocate)

5 Conclusions

Medical research charities understand patient priorities, they have professional R&D operations, budgets and the freedom to explore therapy options, tools and combinations. MRCs can see the advantages of engaging with biopharma to help focus on real, patient-perceived advantages. As consumer technology companies start to realise their ambitions in the healthcare market, the future is for biopharma to move beyond the old constraints of the medical communication function, to start connecting with patient networks and embracing technology for new models of involvement. Biopharma can elevate patient-centricity into two-way relationships with communities, shaping the design and development of products to solve the problems that really matter to patients and the people around them.

Acknowledgements The authors would like to thank the following people and their organizations for advice, participating in the survey: Neil Bennett, Action Duchenne; Michael Karran, Alzheimer's Research UK; Lorcan Kenny, Autistica; Adrian Cottrell, Institute of Cancer Research; Jo Pisani, LAM and Findacure; Kate Adcock, Muscular Dystrophy UK and for contributing quotations to the paper: Neil Bennett, Action Duchenne; Lorcan Kenny, Autistica; Kate Adcock, Muscular Dystrophy UK; Lucy Allen, Cystic Fibrosis Trust.

Declarations

Funding Action Duchenne, Ataxia UK, the Motor Neurone Disease Association and Cure Parkinson's are all members of the Pistoia Alliance. The Pistoia Alliance provided funding for a consultant, John Wise, as a coordination resource for the questionnaire and joint authorship of this paper.

Conflicts of interest The authors listed below are volunteers or employees of charities consulted in this manuscript, they declare no affiliations with, or direct or indirect financial interest in the subject matter of this manuscript: Tina Flatau, Julie Greenfield, Brian Dickie, Oli Rayner, Helen Matthews.

Ethics approval Not applicable.

Consent to participate Not applicable.

Consent for publication Not applicable.

Availability of data and material Not applicable.

Code availability Not applicable.

Authors' contributions All authors contributed to the questionnaire conception and design. Material preparation, collection of inputs and analysis were performed by John Wise and Tina Flatau. The first draft of the manuscript was written by Tina Flatau and all authors commented on previous versions of the manuscript. All authors read and approved the final manuscript. The authors confirm that they have all agreed with the content and gave explicit consent to submit.

References

1. AMRC. 2021: Our sector's footprint. 2021. https://www.amrc.org.uk/our-sectors-footprint-in-2020. Accessed 28 Feb 2022.
2. du Plessis D, Sake JK, Halling K, et al. Patient centricity and pharmaceutical companies: is it feasible? Ther Innov Regul Sci. 2017;51(4):460–7. https://doi.org/10.1177/2168479017696268.
3. US Food and Drug Administration. FDA and European Medicines Agency Patient Engagement Cluster. 2018. https://www.fda.gov/patients/learn-about-fda-patient-engagement/fda-and-european-medicines-agency-patient-engagement-cluster. Accessed 28 Feb 2022.
4. Medicines and Healthcare products Regulatory Agency. Proposed Patient and Public Involvement Strategy 2020-25. 2021. https://www.gov.uk/government/consultations/mhra-patient-involvement-strategy-consultation/proposed-patient-and-public-involvement-strategy-2020-25 Accessed 26 Feb 2022.
5. Eberly LA, Kallan MJ, Julien HM, et al. Patient characteristics associated with telemedicine access for primary and specialty ambulatory care during the COVID-19 pandemic. JAMA Netw Open. 2020;3(12): e2031640. https://doi.org/10.1001/jamanetworkopen.2020.31640.
6. US Food and Drug Administration. FDA—Rare Diseases: Natural History Studies for Drug Development—Draft Guidance for Industry. 2020 https://www.fda.gov/regulatory-information/search-fda-guidance-documents/rare-diseases-natural-history-studies-drug-development. Accessed 26 Feb 2022.
7. Wilkinson M, Dumontier M, Aalbersberg I, et al. The FAIR Guiding Principles for scientific data management and stewardship. Sci Data. 2016;3: 160018. https://doi.org/10.1038/sdata.2016.18.
8. Wise, JCM, Grebe de Barron A, Splendiani A, et al. Implementation and relevance of FAIR data principles in biopharmaceutical R&D. Drug Discovery Today. 2019;24(4):933–8. https://doi.org/10.1016/j.drudis.2019.01.008.
9. National Institute for Health and Care Excellence. How NICE measures value for money in relation to public health interventions. 2013. https://www.nice.org.uk/Media/Default/guidance/LGB10-Briefing-20150126.pdf Accessed 26 Feb 2022.
10. Department of Health Policy paper. Long Term Conditions Compendium of Information: Third Edition. 2012. https://www.gov.uk/government/publications/long-term-conditions-compendium-of-information-third-edition. Accessed 26 Feb 2022.
11. European Commission. Rare diseases. 2022. https://ec.europa.eu/health/non-communicable-diseases/steering-group/rare-diseases_en. Accessed 28 Feb 2022.
12. International Federation of Pharmaceutical Manufacturers & Associations, Code of Practice. 2019. https://www.ifpma.org/subtopics/new-ifpma-code-of-practice-2019/ Accessed 26 Feb 2022.
13. Marras C, Beck JC, Bower JH, et al. Prevalence of Parkinson's disease across North America. NPJ Parkinson's Disease. 2018;4:21. https://doi.org/10.1038/s41531-018-0058-0.
14. Chaudhuri KR, Bhidayasiri R, van Laar T. Unmet needs in Parkinson's disease: New horizons in a changing landscape. Parkinson Relat Disord. 2016;33(supp1):s2–8. https://doi.org/10.1016/j.parkreldis.2016.11.018.
15. Brundin P, Wyse RK. The Linked Clinical Trials initiative (LCT) for Parkinson's disease. Eur J Neurosci. 2019;49(3):307–15. https://doi.org/10.1111/ejn.14175.
16. Giunti P, Greenfield J, Stevenson AJ, et al. Impact of Friedreich's Ataxia on health-care resource utilization in the United Kingdom and Germany. Orphanet J Rare Dis. 2013;8:38. https://doi.org/10.1186/1750-1172-8-38.
17. US Food and Drug Administration. CDER patient-focused drug development. 2021. https://www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development. Accessed 28 Feb 2022.
18. Mahajan R. Real world data: additional source for making clinical decisions. Int J Appl Basic Med Res. 2015;5(2):82. https://doi.org/10.4103/2229-516X.157148.
19. Telehealth Cos Are Seeing A Wave Of Consolidation. Here's what that means for the future of virtual care. CB Insights Newsletter. 2021. https://www.cbinsights.com/research/telehealth-healthcare-mergers-acquisitions-trends-analysis/?utm_source=CB+Insights+Newsletter&utm_campaign=d7cdd34691-newsletter_general_Tues_20210518&utm_medium=email&utm_term=0_9dc0513989-d7cdd34691-88164293. Accessed 26 Feb 2022.
20. How health care is turning into a consumer product. The Economist. 2022. https://www.economist.com/business/how-health-care-is-turning-into-a-consumer-product/21807114. Accessed 26 Feb 2022.